UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-K

☒ ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the year ended December 31, 2023

OR

☐ TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the transition period from ____to____

Commission File Number: 001-41475

PAXMEDICA, INC.
(Exact name of registrant as specified in its charter)

Delaware	85-0870387
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
303 South Broadway, Suite 125, Tarrytown, NY	10591
(Address of principal executive offices)	(Zip Code)

(914) 987-2876
(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Trading Symbol**	**Name of each exchange on which registered**
Common Stock, Par Value $0.0001 Per Share	PXMD	The Nasdaq Capital Market

Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. ☐ Yes ☒ No

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. ☐ Yes ☒ No

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. ☒ Yes ☐ No

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). ☒ Yes ☐ No

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
Emerging growth company	☒		

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☐

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to §240.10D-1(b). ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). ☐ Yes ☒ No

The aggregate market value of the voting and non-voting common equity held by non-affiliates for the most recently completed second fiscal quarter is $4.1 million

As of March 11, 2024, there were 7,408,067 shares of the registrant's common stock outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive proxy statement relating to its 2024 Annual Meeting of Stockholders are incorporated by reference in Part III of this Annual Report on Form 10-K. The definitive proxy statement will be filed with the U.S. Securities and Exchange Commission within 120 days after December 31, 2023, pursuant to Regulation 14A of the Securities Exchange Act of 1934, as amended.

PAXMEDICA, INC.
FORM 10-K
FOR THE YEAR ENDED DECEMBER 31, 2023

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PART I

In this Annual Report on Form 10-K, "we," "our," "us," "Paxmedica, Inc." "Paxmedica" and "the Company" refer to Paxmedica Inc. unless the context requires otherwise.

Cautionary Note Regarding Forward-Looking Statements

This Annual Report on Form 10-K, as well as information included in oral statements or other written statements made or to be made by us, contain statements that constitute "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements are neither historical facts nor assurances of future performance. Instead, they are based on our current beliefs, expectations and assumptions regarding the future of our business, future plans and strategies, and other future conditions. Forward-looking statements can be identified by words such as "anticipate," "believe," "envision," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," "ongoing," "contemplate" and other similar expressions, although not all forward-looking statements contain these identifying words. Examples of forward-looking statements include, among others, statements we make regarding our growth and future financial and operating results and financial position, regulatory approval and pathways, clinical trial and study timing and plans, the achievement of clinical, developmental and commercial milestones, sale and demand for our product candidates, and our liquidity and availability of capital resources. These forward-looking statements are based on our current expectations and beliefs and are subject to a number of factors, risks and uncertainties that could cause actual results to differ materially from those described in the forward-looking statements. Such factors, risks and uncertainties include, but are not limited to:

- our lack of operating history;
- the expectation that we will incur significant operating losses for the foreseeable future and will need significant additional capital, including through future sales and issuances of equity securities which could also result in substantial dilution to our stockholders;
- our current and future capital requirements to support our development and commercialization efforts for our product candidates and our ability to satisfy our capital needs;
- our dependence on our product candidates, which are still in preclinical or early stages of clinical development;
- our, or our third-party manufacturers', ability to manufacture cGMP batches of our product candidates as required for pre-clinical and clinical trials and, subsequently, our ability to manufacture commercial quantities of our product candidates;
- whether we will be successful in obtaining a priority review voucher, or PRV, for PAX-101 and the commercial value to be realized from any such PRV, if any;
- our relationship with TardiMed, an affiliated entity that provides office space and important administrative services to us, as well as our ability to attract and retain key executives and medical and scientific personnel;
- our ability to complete required clinical trials for our product candidates and obtain approval from the FDA or other regulatory agencies in different jurisdictions;
- our lack of a sales and marketing organization and our ability to commercialize our product candidates if we obtain regulatory approval;
- our dependence on third parties to manufacture our product candidates;
- our reliance on third-party CROs to conduct our clinical trials;
- our ability to obtain, maintain or protect the validity of our intellectual property, including our granted or potential future patents;
- our ability to internally develop new inventions and intellectual property;

- interpretations of current laws and the passages of future laws;
- acceptance of our business model by investors;
- adverse developments affecting the financial services industry;
- the accuracy of our estimates regarding expenses and capital requirements; and
- our ability to adequately support organizational and business growth.

We discuss many of these factors in greater detail under "Item 1A: Risk Factors." These factors, risks and uncertainties are not exhaustive and other sections of this Report may include additional factors which could adversely impact our business and financial performance. Given these uncertainties, you should not place undue reliance on these forward-looking statements.

You should read this Report and the documents that we reference in this Report and have filed as exhibits completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of the forward-looking statements in this Report by these cautionary statements. Except as required by law, we undertake no obligation to publicly update any of these forward-looking statements, whether as a result of new information, future events or otherwise.

Market and Industry Data

This Report contains estimates, projections and other information concerning our industry, our business, and the markets for our product candidates, including data regarding market research, estimates and forecasts prepared by our management. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances that are assumed in this information. Unless otherwise expressly stated, we obtained this industry, business, market and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data and similar sources. In some cases, we do not expressly refer to the sources from which this data is derived. In that regard, when we refer to one or more sources of this type of data in any paragraph, you should assume that other data of this type appearing in the same paragraph is derived from the same sources, unless otherwise expressly stated or the context otherwise requires.

ITEM 1. BUSINESS.

Overview

We are a clinical stage biopharmaceutical company focusing on the development of anti-purinergic drug therapies ("APT") for the treatment of disorders with intractable neurologic symptoms, ranging from neurodevelopmental disorders, including autism spectrum disorder ("ASD"), to neurodegenerative disorders such as Fragile X Tremor Ataxia Syndrome (FXTAS) and post-viral Myalgic Encephalomyelitis/Chronic Fatigue Syndrome ("ME/CFS"), which causes physical and cognitive decline in affected adults. APTs have been shown to block the effects of excess production and extracellular receptor activity of adenosine triphosphate ("ATP"), which acts as both the main energy molecule in all living cells and a peripheral and central nervous system neurotransmitter via receptors that are found throughout the nervous system. Excess purinergic signaling can offset homeostasis and trigger immune responses that result in localized and systemic increases in inflammatory chemokines and cytokines, ultimately stimulating ATP production. APTs may also impact immunologic and inflammatory mechanisms that may be causing or exacerbating symptoms in these seemingly unrelated disorders, which may be caused in part by similar mechanisms of ATP overproduction.

One of our primary points of focus is currently the development and testing of our lead program, PAX-101, an intravenous formulation of suramin, in the treatment of ASD and the advancement of the clinical understanding of using that agent against other disorders such as fragile X syndrome (FXS), fragile X-associated tremor/ataxia syndrome (FXTAS), and ME/CFS.

In November 2023, we announced the publication in a peer-reviewed medical journal of the results of our Phase 2 dose-ranging clinical trial evaluating PAX-101 (commonly known as intravenous suramin) for the treatment of the core symptoms of ASD, as described in more detail below.

In July 2023, we announced topline data from a Phase 3 retrospective trial evaluating the efficacy of suramin in the treatment of the fatal parasitic infection commonly known as African sleeping sickness ("HAT"), collecting exclusively licensed real-world evidence for suramin's historical use in treating Human African Trypanosomiasis outside of the United States. The retrospective, non-randomized, externally controlled, interventional efficacy and safety study of suramin for the treatment of Stage 1 TBR HAT demonstrated better health outcomes when compared with a natural history control group of patients evaluated and treated from 1900-1910, prior to the availability of suramin in Africa. The adverse event profile of suramin observed in the study was consistent with what has been widely reported in published medical and clinical literature. We intend to submit data to support a New Drug Application (an "NDA") for PAX 101 under the Neglected Rare Tropical Disease Priority Voucher Program of the U.S. Food and Drug Administration (the "FDA") for the treatment of Human African Trypanosomiasis in 2024.

On October 24, 2023, we entered into an Asset Purchase Agreement (the "Asset Purchase Agreement") with Rediscovery Life Sciences AKI LLC ("RLS"). Pursuant to the Asset Purchase Agreement, we acquired substantially all of the assets of RLS, including but not limited to its intellectual property, equipment, and inventory. The total purchase price for the assets was $100,000, payable in cash at closing, along with a one-time payment of 1% of gross proceeds earned by us in connection with any potential sale of a Priority Review Voucher issued by the FDA in connection with the potential approval of an NDA for HAT. The acquisition was financed through our existing cash reserves.

We are also pursuing the development of next generation APT product development candidates for neurodevelopmental indications. These candidates include PAX-102, our proprietary intranasal formulation of suramin, as well as other new chemical entities that are more targeted and selective antagonists of particular purine receptor subtypes. We believe our lead drug candidate (suramin), if approved by the FDA, may be a significant advancement in the treatment of ASD and a potentially useful treatment for FXS, FXTAS, ME/CFS and LCS.

We were formed as a Delaware limited liability company in April 2018 and converted into a Delaware corporation in April 2020. Our website address is www.paxmedica.com. The information contained on, or that can be accessed through, our website is not part of, and is not incorporated into, this Report.

Our Development Strategy

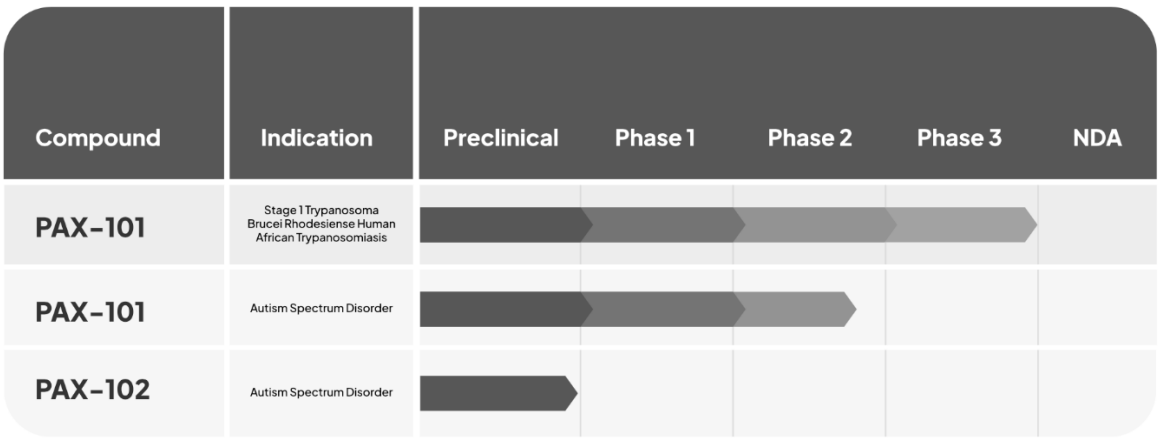
Current Clinical Development Plan

Our clinical development plan seeks to obtain initial U.S. approval of PAX-101 for the treatment of East African HAT, which is caused by the parasite *Trypanosome brucei rhodesiense*, and, using the FDA's 505(b)(2) regulatory pathway, leverage such approval, if

achieved, to facilitate an accelerated development program for PAX-101 for certain neurologic indications including ASD, ME/CFS and LCS. Based on our pre-IND meeting with the FDA in March 2021 and, in part, on an analysis of the data that we have exclusively licensed from the Ministry of Health, Republic of Malawi and Lwala Hospital (Soroti, Uganda) relative to East African HAT patients treated with suramin, we believe we have created a strong development strategy that we plan to employ in seeking the approval of PAX-101 for the treatment of East African HAT. Based on our prior interactions with the FDA, including our pre-IND and Type B meeting with the FDA, we further believe that an approval, if any, in East African HAT could confer upon us the potential receipt of a priority review voucher ("PRV") by the FDA, which we could potentially monetize to fund our future clinical programs. We expect further clinical studies of PAX-101 for the treatment of ASD, FXS, FXTAS, and ME/CFS will be required and similar clinical development is needed for PAX-102 to reach the commercial stage. In November 2020, the FDA granted orphan drug designation to PAX-101 for the treatment of East African HAT. However, there can be no assurance that we will receive FDA approval for PAX-101 for the treatment of East African HAT and, even if PAX-101 is approved by the FDA, there can be no assurance that we will receive a PRV. For more information on the PRV process and how we may benefit from it, see the section of this Annual Report on Form 10-K. "Item 1. *Business – Governmental Regulation - The Priority Review Voucher Program.*"

Development Pipeline

The following table summarizes our current product candidate and indication pipeline.



PAX-101 (intravenous suramin)

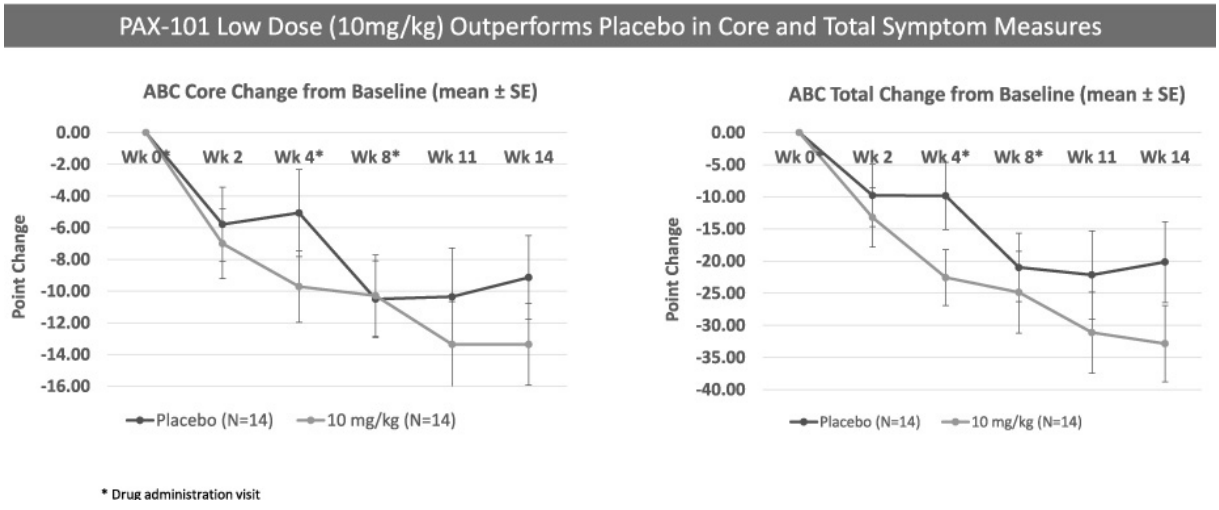
Our lead product candidate under development is PAX-101, an intravenous formulation of suramin, which we are developing for multiple indications, including East African HAT, ASD, and ME/CFS.

The most advanced indication for which we are developing PAX-101 is for treatment of "Stage 1" *Trypanosoma brucei rhodesiense (East African)* HAT, the stage of the clinical course of HAT in which the parasite is found in the peripheral circulation, but it has not yet infiltrated the CNS. We maintain exclusively licensed worldwide rights to patient-level data on the use of suramin in the treatment of Stage 1 East African HAT, which we intend to leverage for demonstration of the safety and efficacy of PAX-101. We have met with the FDA in three formal meetings regarding the execution and development of PAX-101 for this indication. Pursuant to those conversations, and to satisfy the FDA's requirement of demonstrating substantial effectiveness, we conducted a new prospective clinical trial. We completed an analysis and presentation of retrospective data from East African HAT patients previously treated with suramin from 2000 to 2020, for which we have the exclusive license, in July 2023. In addition to these retrospective data, we will also complete preclinical and clinical safety studies to support submission of an NDA for PAX-101's East African HAT indication. We expect that such work will be completed over the next 10 months, with the intention of filing an NDA in second half of 2024. Additionally, we are completing the development of a proprietary supply chain of drug substance and drug product which will form the basis of our NDA filing. Without establishing this supply chain, we will not be able to submit an NDA for the East African HAT indication. See "Manufacturing" for additional information about our expected timeline for establishing our supply chain. In November 2020, the FDA granted orphan drug designation to PAX-101 for the treatment of East African HAT. It is expected that PAX-101, if approved by the FDA for the East African HAT indication, will qualify for new chemical entity exclusivity (providing sole marketing rights in the United States to the Company with respect to any product that contains suramin for up to seven years), in addition to orphan drug exclusivity,

and potentially a tropical disease PRV. However, even if PAX-101 is approved by the FDA for the East African HAT indication, there can be no assurance that we will receive a tropical disease PRV.

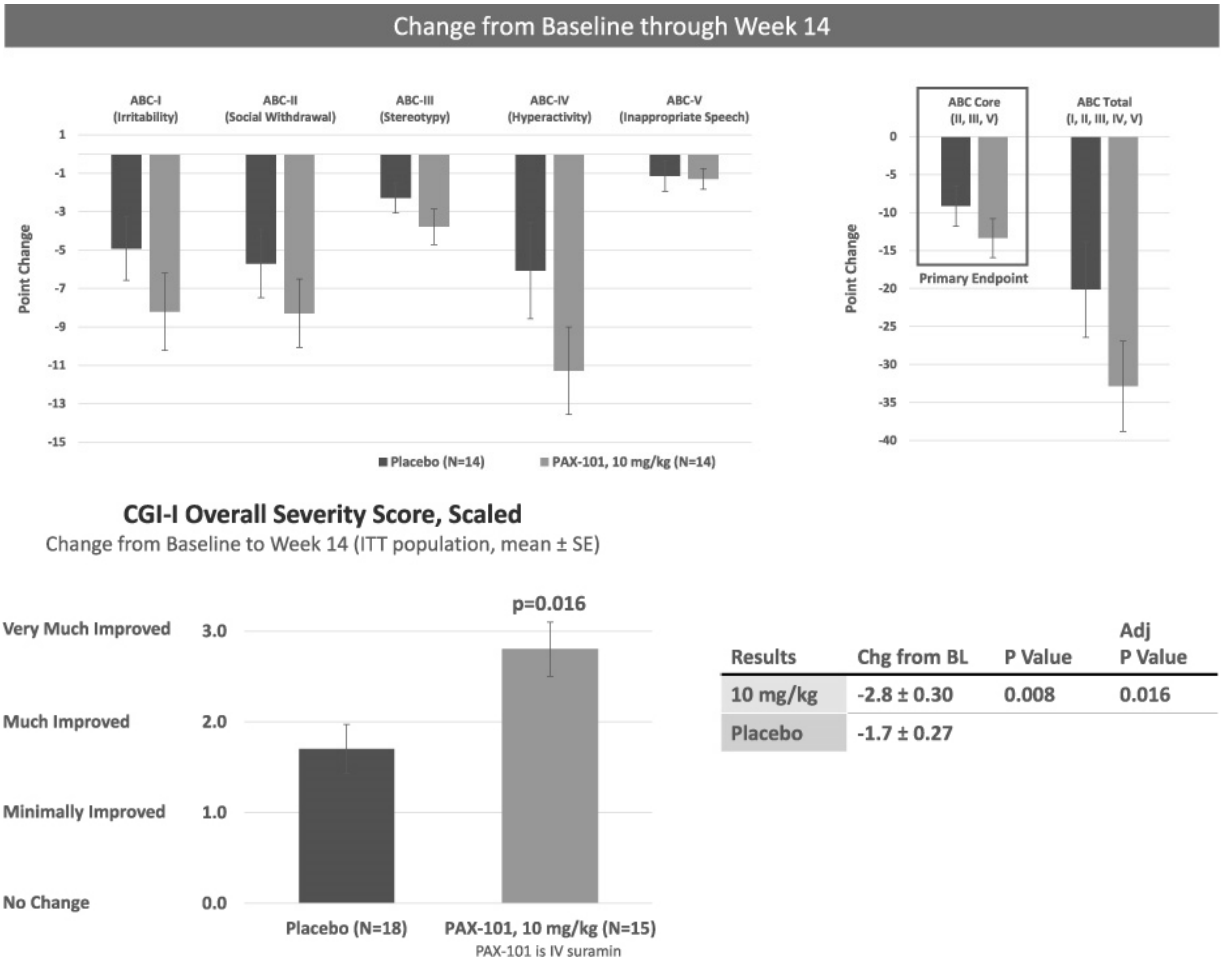
Phase 2 Clinical Trial

Our lead neurologic indication for PAX-101 is for use in treating core symptoms of ASD. In 2021, we conducted a Phase 2 clinical trial at six sites in South Africa with respect to this indication (the "Phase 2 Trial"). The trial was a randomized, double-blind, placebo-controlled design, where we studied two doses of drug versus placebo over a 14-week treatment period. Dosing was at baseline and at the end of weeks 4 and 8. The study population included a patient population with diverse ethnicity and a mean age of approximately 8.4 years. Forty-four of the fifty-two enrolled subjects completed the study, with five withdrawals due to COVID-19 lockdowns, one for an adverse event and three for other reasons. The study evaluated a number of different clinically validated endpoints used in the assessment of the core symptoms of ASD. The primary endpoint of the study was the change between baseline and Week 14 in the Aberrant Behavior Checklist ("ABC") composite score of core symptoms ("ABC Core") including ABC-II (lethargy/social withdrawal), ABC-III (stereotypy) and ABC-V (inappropriate speech).



PAX-101 10mg/kg demonstrated greater improvement through the 14-week treatment period compared to placebo in several assessment measures, including the ABC Core and the Clinical Global Impression - Global Improvement Scale ("CGI-I"). At Week 14, there was a mean improvement from baseline of 12.3 points in the ABC Core in subjects on 10 mg/kg vs. 8.4 points in subjects on placebo (p=0.37). The study was not fully powered for efficacy. However, at Week 14, the subjects treated with 10 mg/kg of PAX-101 demonstrated a mean improvement from baseline in the CGI-I overall symptom severity score of 2.8 points versus 1.7 points on placebo. This change in CGI-I was statistically significant (p=0.016). An improvement in the CGI-I overall symptom severity score of 2 points or more is generally considered to be a clinically relevant change. Certain key subpopulations demonstrated even further improvements on these and other assessments. This trial was designed as a robust dose-ranging study to confirm and expand upon the initial data from

a prior-published single dose, single site, pilot study, but was not designed to demonstrate statistical significance across all efficacy endpoints.



Results	Chg from BL	P Value	Adj P Value
10 mg/kg	-2.8 ± 0.30	0.008	0.016
Placebo	-1.7 ± 0.27		

The full analysis included data from primary, secondary, and exploratory endpoints evaluated in the trial, safety and laboratory data, and an analysis of the pharmacokinetic data. In July 2021, we completed a pre-IND meeting with the FDA to review the results of this trial where we agreed to obtain additional information about the pharmacokinetic profile of PAX-101 in children in different age groups. We intend to meet with the European Medicines Agency (the "EMA") and refine the program's development plan for global registration based on additional work required. We intend to seek permission from South African regulatory authorities for an additional PAX-101 study in South Africa to develop additional data in younger female subjects and other protocol refinements before submitting an IND to the FDA, which we currently expect to do in 2024 or 2025. We are also conducting pre-clinical and other required toxicologic studies to support this IND submission.

Phase 3 Clinical Trial

The PAX-HAT-301 study is a retrospective, non-randomized, externally controlled, interventional efficacy and safety study comparing medical records data from a cohort of patients with Stage 1 Trypanosoma Brucei Rhodesiense (S1 TBR HAT) evaluated and treated from about 2000 – 2020 at one medical site in Uganda and two medical sites in Malawi (referred to as the suramin-treated cohort), with medical records data from a cohort of patients from 1900-1910 evaluated and treated during the TBR HAT epidemic in Uganda (referred to as the natural history cohort). These records included data from a few weeks of hospitalization while they were being evaluated and the diagnosis of TBR HAT confirmed. As their conditions began to deteriorate, patients were often treated with arsenic or related compounds, sent to a Sleeping Sickness Hospital, or sent home to die with their families. The natural history records do not include long term outcomes data for many of the patients. The study was designed in consultation with the U.S. Food and Drug

Administration (FDA) and to ensure that the historical control group of patients had TBR HAT (not the chronic TBG form) and were in Stage 1 of the disease.

The primary objective of the study was to determine whether standard of care treatment with suramin, as currently practiced in Uganda and Malawi, from 2000-2020, led to better health outcomes in patients with S1 TBR HAT, than outcomes observed in a natural history cohort from the epidemic >100 years ago. The secondary objective was to evaluate the safety and tolerability of suramin. The primary endpoint of the study was survival and not meeting any of the supportive descriptive criteria (i.e., death, progression of the disease from Stage 1 to Stage 2, or becoming "moribund" [discharged to a sleeping sickness hospital, physician or patient giving up hope, or being close to death with no hope of recovery]). An independent study adjudication committee was established to review the suramin-treated and natural history cases for study eligibility, and to confirm the clinical endpoints. The committee was comprised of three physicians experienced in the treatment of TBR HAT in Malawi and Uganda.

The PAX-HAT-301 Study Results

The outcomes observed in the suramin-treated cohort were both statistically significant and clinically meaningfully different from the outcomes observed in the natural history cohort. The suramin-treated patients had a far lower rate of death or progression to Stage 2 compared with the natural history cohort. In addition, many of the longer-term outcomes from the natural history cohort pointed towards death as the inevitable outcome of TBR HAT without the benefit of suramin treatment.

In the study population there were 349 patients, 145 in the suramin-treated cohort and 204 patients in the natural history cohort. There were 121 suramin-treated and 42 natural history patients with sufficient data and that met all eligibility criteria for the primary analysis. The suramin-treated patients had a mean age of 31.1 years (range from 2 to 85 years) and 64% male. The natural history patients had a mean age of 22 (range from 3 to 40 years) and 79% male. Racial and ethnicity data were not available and weight was only available for about half of the suramin-treated patients.

The suramin-treated patients presented with a variety of commonly reported HAT related symptoms. The most common symptoms were fever/chills, severe headache, aching joints, extreme fatigue, and swollen lymph nodes. The natural history cohort patients had presenting symptoms recorded in 27/42 (64%) of cases. The reported symptoms were similar including headache, "feeling ill", drowsiness, cough, weakness, chest pain, diarrhea, and enlarged lymph nodes. One suramin-treated patient tested positive for HIV (only 23% tested) and 16/76 (21%) of patients tested were positive for malaria. No comparable data is available for the natural history cohort.

The outcomes for the two cohorts differed substantially. Of the suramin patients, 114 (94%) survived and successfully completed the treatment. Three patients (2%) had "Other" listed as the reason for stopping suramin treatment and 4 (3%) had no reason for stopping suramin treatment recorded. No patients required rescue medications for progression from Stage 1 to Stage 2.

In the natural history cohort, 6 (14%) were recorded as cured, improved, or discharged. Three (7%) patients died, 10 (24%) experienced clinical worsening, and 17 (40%) achieved moribund status (near death and in terminal clinical decline). It is anticipated that if all of these patients were followed for up to 6 months, that nearly all of them would have died.

The primary efficacy analysis revealed that the health outcomes in the suramin-treated cohort were statistically significantly better than those in the natural history cohort. According to the definition of the primary endpoint, the proportion of patients in the suramin-treated group that was alive and not meeting any supportive descriptive criteria of death, any clinical worsening or moribund status was 92% vs. 50% in the natural history cohort. The estimated proportion (95% CI) was 0.442 (0.277, 0.600). The two-sided p-value for the Fisher's exact test was <0.001

Type-B Meeting for HAT-301

On October 26, 2023, we completed a type-B meeting with the FDA, where we discussed the results of our recent data from our PAX-HAT-301 study of suramin in HAT. We received constructive feedback which will aid in the completion of the remaining work necessary to file a New Drug Application expected in the second half of 2024. See "Risk Factors – "Clinical and preclinical drug development is a lengthy and expensive process with uncertain outcomes that may lead to delayed timelines and increased cost, which may prevent us from being able to complete clinical trials". Most of the work to achieve this important milestone will focus on completing the production of commercial lots of PAX-101 under CMC regulatory guidelines, underway now and scheduled to conclude in the first half of 2024.

Clinical Trial of Suramin for Management of ASD

In 2017, the Suramin Autism Treatment ("SAT-1") trial, a double-blind, placebo-controlled, translational pilot study to examine the safety and activity of low-dose suramin in children with ASD, was completed by Naviaux et al. (2017) at the University of California, San Diego. The study included 10 boys aged 4-17 who lived in the San Diego, California area. None of the boys were less than the 5th percentile in weight for their age, on prescription medications, or had laboratory evidence of liver, kidney, heart, or adrenal abnormalities. Additionally, boys who lived more than 90 minutes from the testing site were excluded from the study to prevent bias due to aberrant behavior resulting from extended travel to and from the University. Boys who had ASD due to a DNA mutation or a chromosomal copy number variation were also excluded from the study. The boys' parents were instructed to maintain the boys' current therapies (e.g. supplements, speech, and behavioral therapies) throughout the course of the study.

The trial consisted of a treatment arm of five boys and a placebo arm of five boys. All 10 boys received a 50 mg test dose of suramin in 5 mL of saline or 5 mL of saline only, followed by a 10 mL saline flush. One hour later, boys in the treatment arm received 20 mg/kg of suramin, less the 50 mg test dose, in 50mL of saline, up to a maximum of 1 g, and boys in the placebo arm received 50 mL of saline, each administered intravenously. A 10 mL saline flush followed the second dose's administration as well.

In the SAT-1 study, the mean age was 9.1 years (range: 5-14 years), the mean nonverbal Leiter IQ was 80 (range: 66-92), and the mean ADOS-2 comparison score was 9.0 (range: 7-10).

No serious toxicities (common terminology criteria for adverse events grades three through five) were encountered. Peripheral neuropathy was not noted in the subject patients. Free cortisol, hemoglobin, white blood cell count, platelets, liver transaminases, creatinine, and urine protein levels were not statistically significantly different among the boys in the treatment arm and those in the placebo arm.

All five boys who received suramin experienced a self-limited, evanescent, asymptomatic, fine macular, patchy, morbilliform rash over 1%-20% of their body that peaked one day after infusion and disappeared without intervention between days two and four after infusion. No serious adverse events were encountered. On review by an independent data and safety monitoring board, no safety concerns were noted.

Instruments Factor or behavior	Treatment (days)	Difference from baseline (mean ± SD)	95% CI	*d*	*N*	*P*	*P*	Difference from baseline (mean ± SD)	95% CI	*d*	*N*	*P*	*p*
Primary outcomes													
ADOS-2 Comparison	45	-1.6 ± 0.55	-2.3 to -0.9	2.9	5	0.0028	0.038	-0.4 ± 0.55	-1.1 to + 0.28	0.7	5	0.18	0.16
Raw	45	-4.6 ± 1.9	-7.0. to -2.2	2.4	5	0.0062	0.039	-0.4 ± 1.8	-2.7 to +1.9	0.22	5	0.65	0.58
Social	45	-3.2 ± 1.9	-5.6 to -0.8	2.4	5	0.020	0.043	0.0 ± 1.7	-2.2 to +2.2	0	5	0.99	0.71
Rest/Rep	45	-1.4 ± 0.89	-2.5 to -0.29	1.6	5	0.025	0.059	-0.4 ± 2.1	-3.0 to +2.2	0.19	5	0.69	0.58
EOWPVT Vocabulary	45	-4.2 ± -8.3	-14.50 to +6.1	-0.51	5	0.32	0.50	+2.0 ± 4.6	-3.8 to +7.8	0.43	5	0.39	0.50
Secondary Outcomes													
ABC Stereotypy	7	36 ± 2.1	-6.2 to -1.0	1.7	5	0.018	0.043	+0.4 ± 1.9	-2.0 to +2.5	-0.21	5	0.67	0.68
Stereotypy	45	-4.0 ± 2.3	-6.9 to -1.1	1.7	5	0.019	0.042	+1.0 ± 4.3	4.3 to +6.3	-0.23	5	0.63	0.69
ATEC Total	7	-10 ± 7.7	-20 to -0.46	1.3	5	0.044	0.043	+7.2 ± 14	-10 to +25	-0.51	5	0.32	0.35
Language	7	-2.2 ± 1.5	-4.0 to -0.36	1.4	5	0.021	0.059	0.0 ± 4.1	-5.0 to +5.0	0	5	0.99	0.89
Sociability	7	-3.6 ± 2.6	-6.8 to -0.36	1.4	5	0.025	0.063	-0.8 ± 2.8	4.3 to +2.6	0.29	5	0.55	0.58
Language	45	-2.0 ± 1.4	-2.7 to -0.49	1.4	5	0.034	0.059	-0.2 ± 2.9	-3.8 to +3.4	0.07	5	0.88	0.79
CGI Overall ASD	45	-1.8 ± 1.04	-3.4 to -0.15	1.7	5	0.05	n/a	0.0 ± 0.34	-0.55 to +0.55	0	5	0.99	n/a
E. Language	45	-2.0 ± 1.04	-3.6 to -0.35	1.9	5	0.01	n/a	0.0 ± 0.34	-0.55 to +0.55	0	5	0.99	n/a
Social Inter.	45	-2.0 ± 1.04	-3.6 to -0.35	1.9	5	0.01	n/a	0.0 ± 0.34	-0.55 to +0.55	0	5	0.99	n/a
RBQ Total	45	-3.2 ± 5.8	-10.4 to +4.0	0.55	5	0.28	0.22	-0.8 ± 3.3	-4.9 to 3.3	0.24	5	0.62	0.47

ADOS-2, autism diagnostic observation schedule, 2nd edition; EOWPVT, Expressive One-Word Picture Vocabulary Test; ABC, aberrant behavior checklist; ATEC, autism treatment evaluation checklist; CGI, clinical global impression survey; RBQ, repetitive behavior questionnaire; Restr/Rep, restricted or repetitive behaviors; Overall ASD Sx, overall ASD symptoms; E. Language, expressive language; Social Inter., social interaction. *Analysis.* ADOS, EOWPVT, ABC, ATEC, and RBQ scores were analyzed by paired analysis before and after treatment using each subject as their own control. CGI was analyzed by two-way ANOVA (symptom x time before and after treatment) with post hoc correction. Nonparametric *P* values were not calculated (n/a). *Interpretation.* ADOS, ABC, ATEC, CGI, and RBQ are severity score; negative differences from baseline reflect decreased severity, that is, improvement. EOWPVT is a performance score; negative differences reflect a decrease.

(1) A positive Cohen's d reflects improvement, and a negative d reflects a decrease by convention. Cohen's d is likely an overestimate of the actual treatment effect based on the large mean differences and small standard deviations found before and after treatment in this small study.

(2) p value from parametric paired t-test analysis.

(3) *p* value from nonparametric paired Wilcoxon signed-rank sum analysis.

Parents reported that, after suramin treatment, the rate of language, social, behavioral, and developmental improvements increased over three weeks, then gradually decreased toward baseline over the next three weeks. ADOS-2 comparison scores at six weeks improved by an average of -1.6 +/- 0.55 points (mean +/- SD; n= 5; 95% CI= -2.3 to -0.9; Cohen's d = 2.9; P = 0.0028) (note: lower score associated with less concern). The mean ADOS comparison score in the suramin-treated group was 8.6 +/- 0.4 at baseline and 7.0 +/- 0.3 at six weeks. An improvement in ABC, Autism Treatment Evaluation Checklist, and Clinical Global Impression of Improvement scores was also noted among boys who received suramin.

PAX-102 (intranasal suramin)

PAX-102, a proprietary intranasal formulation of suramin, is also being developed for neurologic indications. The rationale for this program is the potential to better target the suramin molecule to the CNS, which may potentially allow us to deliver similar potency to that achieved using PAX-101 and reduce the dose needed and improve the tolerability profile of the drug, ultimately offering patients a more convenient delivery system versus intravenous infusion. We have developed a proprietary intranasal formulation, and based on our in vitro nasal membrane permeation studies using the cultured EpiAirway (Mattek) membrane model, as well as more targeted CNS delivery in vivo, we believe our intranasal formulation has the potential to demonstrate rapid and efficient uptake across the nasal membrane. We expect to move forward towards an Investigational New Drug application (an "IND") on PAX-102 pending further funding or partnerships.

Suramin is not orally bioavailable and has historically been administered intravenously and at very high doses separated by a few days between infusions. Suramin intravenously administered in high doses for treatment and eradication of the parasite that causes East African HAT can cause significant side effects, including rash, nausea, vomiting, diarrhea, abdominal pain, and a feeling of general discomfort. Other side effects include skin sensations such as crawling or tingling sensations, tenderness of the palms and soles, numbness of the extremities, watery eyes, and photophobia. In addition, when administered in high doses or as a continuous infusion, suramin has been shown to cause nephrotoxicity and peripheral neuropathy. Regarding pharmacokinetics, suramin is approximately 99-98% protein bound in the serum and has a half-life of 41-78 days, with an average of 50 days. Also, it is not extensively metabolized and is eliminated by the kidneys.

Despite strong early results from early animal and human studies, we believe more research is needed to provide safe and effective delivery of APTs, such as suramin, for treating neurologic conditions. We believe it may be necessary to deliver appropriate levels of the drug in brain tissue while also minimizing blood and other tissue levels. While it is difficult to deliver drugs across the blood-brain barrier (the "BBB"), which is a natural protective mechanism of most mammals, including humans, such delivery is even more challenging for higher molecular weight compounds, such as suramin. A possible route to maximize delivery across the BBB is to use intranasal delivery to provide higher levels of a drug to the upper nasal mucosa to allow for nose-to-brain transport along the olfactory and trigeminal nerves. We believe our proprietary intranasal formulations and methods of delivering suramin to mammals have been shown, in in-vivo preclinical studies, to deliver suramin to the brain in ways that may reduce systemic exposure, and our development plan calls for clinical trials to test this potential for reduced systemic exposure in humans.

The PAX-101 and PAX-102 development programs in neurologic disorders may be filed with the FDA as a supplement to the initial 505(b)(2) NDA, assuming PAX-101 is approved for the treatment of East African HAT. As discussed in more detail below, a

505(b)(2) NDA is a special type of NDA that enables the applicant to rely, in part, on the FDA's findings of safety and efficacy of an existing or previously approved product, or published literature, in support of its application. 505(b)(2) NDAs often provide an alternate path to FDA approval for new or improved formulations or new uses of previously approved products. Using a 505(b)(2) NDA, we expect to reduce the cost, time and risk that would otherwise be associated with bringing these programs to market. See "*The 505(b)(2) NDA Regulatory Pathway*" below for more information.

Early Program in Selective APTs

In addition to the PAX-101 and PAX-102 development programs, we have begun an early discovery program targeting the development of highly selective APTs. We have identified multiple compounds that are selective for certain purinergic receptor subtypes and have engaged in preclinical work with these compounds in an animal model of ASD. We expect to complete additional preclinical work and open an IND with respect to one or more of these compounds in 2023 or 2024. In the future, we intend to pursue opportunities to develop products, either alone or in partnership with other pharmaceutical companies, related to these APTs.

In June 2023, we entered into a Research Collaboration Agreement with PoloMar Health ("PoloMar") for Phase II clinical trial in Autism Spectrum Disorder (ASD) for emodin, is a naturally occurring anthraquinone derivative. In 2019, we completed several in vivo animal studies in a mouse model of autism and demonstrated positive results for emodin in several measures of cognition, memory, and behavior. These findings were the basis of a patent filing that was initiated that same year by the Company. PoloMar will be the sponsor of the U.S.-based trial for [•], and will, provide the funding for the trial. Under the terms of the agreement PoloMar retains rights to develop and commercialize any non-prescription supplement form of the product, while we retain exclusive rights to develop and commercialize a highly purified form of emodin as a prescription pharmaceutical product following FDA and other regulatory authorities' guidance. PoloMar is a related party due to their ownership by TardiMed Sciences.

Our Markets

Market for ASD

According to the U.S. Center for Disease Control and Prevention (the "CDC"), ASD affects between 1% and 2% of the world's population, including more than 3.5 million people in the United States. Prevalence of autism in eight-year-old U.S. children increased by approximately 316% from 2000 (1 in 150) to 2020 (1 in 36).

No pharmacological therapies exist for the treatment of the core symptoms of ASD, such as lethargy/social withdrawal, stereotypy, or repetitive or ritualistic behaviors, and inappropriate speech. Pharmacological therapies that have been approved to date, such as aripiprazole and risperidone, only treat the non-core symptom of irritability associated with ASD. There are also a number of new therapies for ASD in development by small and large companies with variable levels of clinical data, although none has proven efficacy and safety in large, randomized and controlled trials.

The global ASD therapeutics market is projected to grow from $2.01 billion in 2023 to $3.42 billion by 2030, at a CAGR of 7.9%, according to a research report by Fortune Business Insights. We believe a drug that can demonstrate strong safety and efficacy in the treatment of the core symptoms of ASD would generate strong market demand because there are no comprehensive treatment options available to address these important aspects of this condition.

Market for FXAS and FXTAS

Fragile X-associated tremor/ataxia syndrome (FXTAS) is a condition that specifically impacts Fragile X premutation carriers, typically manifesting in individuals aged 55 and above. This neurological disorder, akin to other prevalent adult-onset conditions like Amyotrophic Lateral Sclerosis (ALS or Lou Gehrig's disease), underscores its significance in the realm of neurodegenerative diseases. The estimated prevalence of FXTAS is approximately 1 in 8,000 males aged 50 and above within the general population.

Despite its impact, there are currently no medications available to halt or decelerate the progression of FXTAS. Neurologists commonly resort to prescribing medications tailored to the specific symptoms exhibited by individual patients. Notably, the management of FXTAS symptoms involves the use of medications such as primidone, gabapentin, topiramate, levetiracetam, amantadine, buspirone, varenicline, and riluzole, which have demonstrated efficacy in a limited number of patients. Furthermore, in cases where FXTAS patients exhibit symptoms resembling Parkinson's disease, carbidopa/levodopa may be recommended. The absence of targeted therapies emphasizes the pressing need for continued research and the development of interventions to address the unique challenges posed by FXTAS.

Market for ME/CFS

Prior to the COVID-19 pandemic, little attention had been paid to a potentially related post-acute infection disorder known as ME/CFS which, in light of the recent observation and identification of LCS, has received renewed interest in the medical and patient advocacy community. Like LCS, ME/CFS sufferers have nearly identical physical symptoms which in extreme cases have been documented to last for years, resulting in affected individuals becoming house-, if not bed-bound. Suicidality is also a common concern of clinicians who care for these patients. The cause of ME/CFS is unknown but research points to the possibility that many cases of ME/CFS resulted from a prior viral infection, which may or may not have had overt physical symptoms at the time, and an immune response to this infection that continues to induce an inflammatory response, despite the lack of any virus or similar infectious invader. Although some ME/CFS sufferers can and do tolerate some ME/CFS symptoms, many seek help through various unproven diets, supplements and prescription drugs. In some cases, complex spinal fusion therapy has been shown to be beneficial for ME/CFS patients whose symptoms may be related to recovery from physical trauma.

There is significant opportunity within the global ME/CFS market. ME/CFS can cause significant impairment and disabilities that have negative economic consequences at both the individual and the societal level. At least one-quarter of ME/CFS patients are house- or bed-bound at some point in their lives. The direct and indirect economic costs of ME/CFS to society have been estimated at $17 to $24 billion annually, $9.1 billion of which has been attributed to lost household and labor force productivity.

Market for East African HAT

The market for using PAX-101 to treat East African HAT is expected to be largely restricted to Sub-Saharan Africa, where suramin is already in use for Stage 1 East African HAT. Further, we may donate product for use in this indication to the WHO, either as a replacement for current limited supplies or as a supplementary source of suramin, if the WHO requests us to do so. If we obtain a U.S. approval for PAX-101 to be used in the treatment of East African HAT, we could potentially qualify to earn a tropical disease PRV from the FDA, which we would intend to monetize to raise funds to support the later-stage development and commercialization of PAX-101 and PAX-102 in the treatment of ASD, and ME/CFS. The estimated total cost to gain FDA approval, if any, for the HAT indication and qualify for the PRV is estimated to be between $11.0 to $13.0 million, with some portion of these expenses shared across our pipeline indications and formulations programs that would extend beyond the initial HAT approval, if any. There can be no assurance that we will receive a PRV, and even if we do obtain a PRV, there can be no assurance that we will receive sufficient funds from its sale to fund the clinical and commercial development of our drug candidates. If we are unable to obtain a PRV, or if the amount we obtain from its sale is insufficient to fund our operations, we may be required to fund the later-stage development and commercialization of PAX-101 and PAX-102 in the treatment of ASD, and ME/CFS through sales of our equity or debt securities, strategic collaborations with third parties or other similar transactions.

Manufacturing Activities

The synthesis of PAX-101 is a complex multi-step process and involves a global supply chain. We do not own or operate manufacturing facilities for the production of PAX-101, nor do we have plans to develop our own manufacturing facility for clinical or commercial manufacture of PAX-101 in the foreseeable future. We depend on third-party Contract Manufacturers (“CMOs”) and Contract Laboratories (“CLOs”) to manufacture and test all our critical intermediates, as well as our active pharmaceutical ingredients. We will utilize clinical service organizations to package, label and distribute clinical trial quantities of PAX-101. In 2019 and 2020, we entered into multiple agreements with CMOs for the development and cGMP- compliant production of suramin for use in our clinical trials and to support the future commercial supply chain of PAX-101. Pursuant to these agreements, we have paid upfront fees to our CMOs and will be required to make additional payments upon various milestones throughout the project.

There was no readily available source of suramin for use in clinical trials in the United States. There is currently one manufacturer of suramin, Bayer, which does not manufacture suramin on a regular basis and, when it does, generally only manufactures small quantities in response to outbreaks of HAT. We have engaged two independent contract development manufacturing organizations to develop, validate and scale our supply chain for suramin and the shelf stable drug product that is ultimately distributed to pharmacies.

We have completed the necessary steps to begin producing suramin in late 2023 and began the final development phase in November 2023, including the production and final release testing process, that will produce suramin for both clinical and registrational purposes. We have produced suramin API and delivered initial batches for animal testing and we anticipate delivering suramin API for final drug product development in the third quarter of 2023. Development, validation and scaling of the final drug product manufacturing and release process is expected to be initiated in the first half of 2024 and drug product is expected to be placed in stability testing immediately following Quality Control release. We estimate expenses required to complete the remaining to be between $2.5 million

and $2.8 million. We continue to explore alternative manufacturing sources and partnerships in an effort to ensure that we will have an uninterrupted supply chain and maintain sufficient manufacturing capacity in order to meet potential demand for any of our product candidates. We plan to work with our CMOs to establish and safeguard our supply chain for any products we successfully develop. We cannot guarantee, however, that our efforts to develop and maintain such an uninterrupted supply chain will be successful and, until we have a reliable supply, our clinical trial progress will continue to be delayed, including delaying our NDA filing for the HAT indication until we have sufficient supply. Unless and until we can complete the NDA for the HAT indication, we will not be eligible for a priority review voucher.

To comply with FDA requirements for approval of our pharmaceutical products, we will audit all of our CMOs and CLOs to ensure they are compliant with cGMP, including with respect to their document control procedures, manufacturing facilities, environment, and equipment, quality control procedures, quality assurance procedures, and personnel management policies (together, "Quality Systems"). These Quality Systems must be qualified and approved by the FDA prior to approval and release of product on an ongoing basis.

Sales and Marketing

We currently have no marketing, sales or distribution capabilities. In order to market and sell products that are approved for commercial sale, we must either develop our own sales, marketing and distribution infrastructure or collaborate with third parties that have such commercial infrastructure and relevant marketing and sales experience. We will consider the merits of developing our own sales, marketing and distribution infrastructure for the U.S. market. If we elect to develop our own sales and marketing organization, we do not intend to begin to hire sales and marketing personnel unless and until any of our product candidates are in Phase 3 clinical trials or closer to NDA submission, and we do not intend to establish our own sales organization in the United States unless and until shortly prior to FDA approval of PAX-101 or any of our other product candidates for neurologic indications. We do not intend to establish a sales organization for selling PAX-101 as a treatment for East African HAT in any market. Therefore, at the time of our anticipated commercial launch of PAX-101, assuming regulatory approval of the drug by the FDA for neurologic indications, any sales and marketing team, if we decide to have one, will have worked together for only a limited period of time.

In June 2023, we entered into an exclusive specialty benefit manager agreement with Vox Nova, LLC ("Vox Nova") pursuant to which Vox Nova will act as the exclusive United States distributor for PAX-101 for a period of up to seven years. Vox Nova will provide certain distribution management, pharmacy benefit management, sales and supply monitoring services to us with respect to PAX-101, in the event PAX-101 receives FDA approval. The distribution agreement also provides for an exclusivity fee payable to us of up to $2.0 million, payable in installments based on various time and regulatory approval parameters.

Competition

The pharmaceutical industry is characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. We face competition from many different sources, including commercial pharmaceutical and biotechnology enterprises, academic institutions, government agencies and private and public research institutions. Many of these competitors have far greater human and financial resources and may have product candidates in more advanced stages of development. Furthermore, these competitors' products will likely reach the market before our product candidates. Competitors may also develop products that are more effective or safe, less expensive or that have better tolerability or provide for more convenient administration. Although we believe that our intellectual property, experience and knowledge in our areas of focus provide us with competitive advantages, these potential competitors could reduce our commercial opportunities.

Competition for PAX-101 in ASD

There is currently no known cure for ASD, and no FDA approved medication to treat the core symptoms of ASD. We are aware, however, of several companies that are working to develop drugs that might compete against our product candidates. Our current and potential competitors in ASD include CureMark LLC, which is in Phase 3 studies for CM-AT for ASD, Yamo Pharmaceuticals, which is in Phase 2 studies for LI-79 for ASD, GW Pharmaceuticals, which is in Phase 2 studies for Cannabidivarin for ASD, Harmony Biosciences, which is in Phase 3 studies for CBD gel for ASD, QBioMed, which is developing a preclinical asset called QBM-001 for rare pediatric nonverbal ASD, Kuzani Therapeutics, Inc., which has announced that it is in clinical development for the treatment of the core symptoms of ASD in children, and Axial Therapeutics, which is in Phase 2 studies for AB-2004 for irritability in ASD. There are two treatments that have been approved by FDA to treat the non-core symptom of irritability in ASD: Risperdal® (risperidone) and Abilify® (aripiprazole). Both risperidone and aripiprazole are generic medications. For more information relating to competition risks, please see the risk factor with the heading "*We face competition from other biotechnology and pharmaceutical companies and our operating results will suffer if we fail to compete effectively.*"

Competition for PAX-101 in East African HAT

Sanofi, DNDi and the HAT-r-ACC consortium are working on Fexinidazole Winthrop as first oral treatment of acute form of sleeping sickness (rhodesiense). They received a positive opinion is for the treatment in adults and children six years of age or older and weighing at least 20 kg, of both first-stage (haemo-lymphatic) and second-stage (meningo-encephalitic) Trypanosoma brucei (T.b.) rhodesiense sleeping sickness from the European Medicines Agency (EMA) Committee for Medicinal Products for Human Use (CHMP).

Competition for PAX-101 in ME/CFS

For treatment of ME/CFS, AIM ImmunoTech has an approval for rintatolimod in Argentina, and is in development for the drug in the US.

Research and Development

We spent approximately $3.9 million and $1.7 million during the fiscal years ended December 31, 2023 and 2022, respectively, on research and development activities, which activities have been undertaken by our CROs and other third-party vendors. These expenses include cash and non-cash expenses related to the development of our clinical and pre-clinical programs. From time to time, as needed, we will employ consultants to support our various business and research and development activities.

Intellectual Property

Our commercial success depends in part on our ability to obtain and maintain proprietary protection for product candidates and any of our future product candidates, novel discoveries, product development technologies and know-how; to operate without infringing on the proprietary rights of others; and to prevent others from infringing our proprietary rights. Our policy is to seek to protect our proprietary position by, among other methods, filing on or in-licensing U.S. and foreign patents and patent applications related to our proprietary technology, inventions and improvements that are important to the development and implementation of our business. We also rely on trademarks, trade secrets, know-how, continuing technological innovation, regulatory and marketing exclusivity such as orphan drug exclusivity, and potential in-licensing opportunities to develop and maintain our proprietary position.

As of December 31, 2023, we own rights to at least four families of patent applications (discussed further in the following three paragraphs). Our patent applications related to our product development candidates currently in development are projected to expire no earlier than 2039, not including any patent term adjustments, patent term extensions, supplementary protection certificates, or other term extensions that might be available in a particular jurisdiction. We also plan to file further patent applications covering our technology and products. Additionally, we own the exclusive rights to patient data in certain East African hospitals that is necessary for our HAT NDA filing.

On May 2, 2020 we filed PCT international patent Application No. PCT/US2020/031217 entitled Compositions and Methods for Treating Central Nervous System Disorders. This application claims priority to US Provisional Patent Application Serial No. 62/858,621, filed on June 7, 2019. The PCT application published as WO 2020/247127 on December 10, 2020. The PCT patent application relates to compositions and methods for treating cognitive, social, or behavioral disabilities, and neurodevelopmental disorders. These disabilities and disorders include, ASD and other central nervous system disorders such as fragile X syndrome (FXS), fragile X-associated tremor/ataxia syndrome (FXTAS), chronic fatigue syndrome (CFS), and post-traumatic stress syndrome (PTSD). The patent application also includes further embodiments of ASD selected from autistic disorder, childhood disintegrative disorder, PDD-NOS, and Asperger syndrome. This patent application also relates to compositions for delivering a therapeutically effective amount of an APT, for example suramin, and pharmaceutically acceptable salts, esters, solvates, and prodrugs of these agents. In some embodiments, the APT is delivered by intranasal administration. All designated states (all PCT treaty member countries) were selected upon filing of the PCT patent application. National and regional stage applications have been filed in and are pending in the United States, Australia, Canada, China, the European Patent Office, Israel, and Japan. The application has also been registered in Hong Kong off of the Chinese application.

On October 20, 2021 we filed two PCT international patent applications. The first of these two PCT patent applications is PCT international patent Application No. PCT/US2021/55908 which is entitled Intranasal Administration of Suramin for Treating Nervous System Disorders. This application claims priority to US Provisional Patent Application Serial No. 63/104,350, filed on October 22, 2020. The PCT application published as WO 2022/087174 on April 28, 2022. This PCT patent application relates to methods and compositions for the intranasal administration of suramin for treating cognitive, social, or behavioral disabilities, and

neurodevelopmental disorders. All designated states (all PCT treaty member countries) were selected upon filing of the PCT patent application. National and regional stage applications have been filed in and are pending in the United States, Australia, Canada, China, the European Patent Office, Israel, and Japan. The application has also been registered in Hong Kong off of the European Patent application. The second of these two PCT patent applications is PCT international patent Application No. PCT/US2021/55911 which is entitled Administration of Antipurinergic Compositions for Treating Nervous System Disorders. This application claims priority to US Provisional Patent Application Serial No. 63/104,357, filed on October 22, 2020. The PCT application published as WO 2022/087176 on April 28, 2022. This PCT patent application relates to methods and compositions for the administration of antipurinergic compositions for treating cognitive, social, or behavioral disabilities, and neurodevelopmental disorders. All designated states (all PCT treaty member countries) were selected upon filing of the PCT patent application. National and regional stage applications have been filed in and are pending in the United States, Australia, Canada, China, the European Patent Office, Israel, and Japan. The application has also been registered in Hong Kong off of the European Patent application.

On August 22, 2022 we filed PCT international patent Application No. PCT/US2022/041050 entitled Methods for Treating Central Nervous System Disorders with Antipurinergic Agents. This application claims priority to US Provisional Patent Application Serial No. 63/236,155, filed on August 23, 2021. The PCT application published as WO 2023/027994 on March 2, 2023. The application relates to further aspects of our work for the administration of the antipurinergic agents based on pharmaco-kinetic/pharmaco-dynamic parameters and other learnings from our pre-clinical and clinical work. All designated states (all PCT treaty member countries) were selected upon filing of the PCT patent application. We are in the process of and intend to file national and regional stage applications in the United States, Australia, Canada, China, the European Patent Office, Israel, and Japan, and to file a Hong Kong registration off of the European Patent application.

While we seek broad coverage under our existing patent applications, there is always a risk that an alteration to our products or processes may provide sufficient basis for a competitor to avoid infringing our patent claims. In addition, patents, if granted, expire and we cannot provide any assurance that any patents will be issued from our pending or any future applications or that any potentially issued patents will adequately protect our products or product candidates.

Individual patents extend for varying periods depending on the date of filing of the patent application or the date of patent issuance and the legal term of patents in the countries in which they are obtained.

Generally, patents issued for regularly filed applications in the United States are granted a term of 20 years from the earliest effective non-provisional filing date. In addition, in certain instances, a patent term can be extended to recapture a period due to delay by the USPTO in issuing the patent as well as a portion of the term effectively lost as a result of the FDA regulatory review period. However, as to the FDA component, the restoration period cannot be longer than five years and the total patent term including the restoration period must not exceed 14 years following FDA approval. The duration of foreign patents varies in accordance with provisions of applicable local law, but typically is also 20 years from the earliest effective non-provisional filing date. However, the actual protection afforded by a patent varies on a product-by- product basis, from country to country, and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory-related extensions, the availability of legal remedies in a particular country and the validity and enforceability of the patent.

Our commercial success will also depend in part on not infringing upon the proprietary rights of third parties. It is uncertain whether the issuance of any third-party patent would require us to alter our development or commercial strategies for our products or processes, or to obtain licenses or cease certain activities. Our breach of any license agreements or failure to obtain a license to proprietary rights that we may require to develop or commercialize our future products may have an adverse impact on us. If third parties prepare and file patent applications in the United States that also claim technology to which we have rights, we may have to participate in interference or derivation proceedings in the USPTO to determine priority of invention. For more information, please see “Risk Factors — Risks Related to Our Intellectual Property.”

We are aware of PCT international patent application PCT/US2018/017674, titled “Methods for Autism Spectrum Disorder Pharmacotherapy”, which lists Perfect Daylight Limited and The Regents of the University of California as Applicants, filed on February 9, 2018, published as WO 2018/148580 on August 16, 2018, and claiming priority to U.S. provisional patent application no. 62/457,120, filed on February 9, 2017. The patent application describes compositions of antipurinergic agents, such as suramin, and methods of use for treating cognitive developmental disorders and autism spectrum disorder. From publicly available databases, we are aware that a U.S. nonprovisional patent application of this PCT patent application, U.S. application Serial No. 16/537,397, was filed in the United States and was subsequently abandoned in favor of U.S. application Serial No. 18/323,375, filed Mary 24, 2023 and U.S. application Serial No. 18/414,171, filed January 16, 2024. The European equivalent of the application was granted as EP3579836 on December 15, 2021, and was validated in Belgium, France, Germany, Great Britain, and Switzerland. A Chinese application, CN201880024535.9, is

also pending. Because patent applications of PCT/US2018/017674 are still pending at least in the United States and China, it is not certain if any patents will ultimately issue from these applications nor is it possible to predict the resultant claim scope of any such issued patent. We will continue to monitor the prosecution of these patent applications from publicly available documents.

We are also aware of PCT international patent application PCT/US2018/017200, titled "Antipurinergic Compounds and Uses thereof," which lists CSP Pharma, Inc. as Applicant, filed on February 7, 2018, published as WO 2018/148262 on August 16, 2018, and claiming priority to U.S. provisional patent application no. 62/456,438, filed on February 8, 2017. The patent application describes compositions and methods for treating neurodevelopmental disorders. The compositions contain an APT, such as suramin, and a carrier formulated for non-intravenous administration. The neurodevelopmental disorders include ASD. From publicly available databases, we are aware that a national phase application of this PCT patent application, U.S. application Serial No. 16/484,284 was filed in the United States. However, the US Patent Office issued a Notice of Abandonment on August 12, 2021 for applicant's failure to respond to the office action of January 14, 2021. No further child applications are listed as pending.

We are also aware of PCT international patent application PCT/US2017/041932, titled "Diagnostic and Methods of Treatment for Chronic Fatigue Syndrome and Autism Spectrum Disorders," which lists The Regents of the University of California as Applicant, filed on July 13, 2017, published as WO 2018/013811 on January 18, 2018, and claiming priority to U.S. provisional patent application nos. 62/ 464,369, filed on February 27, 2017 and 62/362,564, filed on July 14, 2016. The patent application describes biomarkers for diagnosing and predicting the development of chronic fatigue syndrome and methods of treating a mitochondrial disease or disorder, such as ASD, by administering an effective amount of an APT, such as suramin. Publicly available databases show no pending national or regional phase patent applications.

License Agreements

On October 10, 2018 and November 9, 2018, we obtained the rights to worldwide, exclusive licenses to the patient data from the Ministry of Health, Republic of Malawi (the "Malawi License Agreement") and Lwala Hospital (Soroti, Uganda) (the "Lwala License Agreement"), respectively, in connection with the treatment of East African HAT patients.

Under each of the Malawi License Agreement and the Lwala License Agreement (collectively, the "License Agreements"), we obtained an exclusive worldwide license to the medical data and information (in the form of patient medical files) related to patients who have been diagnosed with and/or treated for East African HAT. We intend to use these data to support our PAX-101 regulatory filings in the United States and Europe for the treatment of Stage 1 East African HAT. Pursuant to each of the License Agreements, we pay a fee to each licensor for its services in facilitating our access to, and analysis of, these data and we are obligated to make additional payments in the future based on the level of each licensor's participation. As of December 31, 2023, we have paid an aggregate of $29,717 under the License Agreements. Each of the License Agreements has an indefinite term, but may be terminated by each party thereto upon material breach of the other party, if such breach is not remedied by the breaching party within 30 days after being given notice of such breach by the non-breaching party. We anticipate that we will pay a total of approximately $50,000 during the life of the Malawi License Agreement, and a total of approximately $50,000 during the life of the Lwala License Agreement.

Governmental Regulation

Government authorities in the United States, at the federal, state and local level, and in other countries extensively regulate, among other things, the research, development, testing, manufacture, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing and export and import of products such as those we are developing. The pharmaceutical drug product candidates that we develop or acquire must be approved by the FDA before they may be marketed and distributed in the United States.

In the United States, the FDA regulates pharmaceutical products under the Federal Food, Drug, and Cosmetic Act, and implementing regulations. Pharmaceutical products are also subject to other federal, state and local statutes and regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant to administrative or judicial sanctions. FDA and related enforcement activity could include refusal to approve pending applications, withdrawal of an approval, a clinical hold, warning letters, product recalls, product seizures, total or partial suspension of production or distribution injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties. Any agency or judicial enforcement action could have a

material adverse effect on us. The process required by the FDA before a pharmaceutical product may be marketed in the United States generally involves the following:

- Completion of preclinical laboratory tests, animal studies and formulation studies according to Good Laboratory Practices or other applicable regulations;
- Submission to the FDA of an IND, which must become effective before human clinical studies may begin;
- Performance of adequate and well-controlled human clinical studies according to the FDA's GCP, to establish the safety and efficacy of the proposed pharmaceutical product for its intended use;
- Submission to the FDA of an NDA for a new pharmaceutical product;
- Satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the pharmaceutical product is produced, to assess compliance with cGMP, to assure that the facilities, methods and controls are adequate to preserve the pharmaceutical product's identity, strength, quality and purity;
- Potential FDA audit of the preclinical and clinical study sites that generated the data in support of the NDA; and
- FDA review and approval of the NDAs.

The lengthy process of seeking required approvals and the continuing need for compliance with applicable statutes and regulations require the expenditure of substantial resources and approvals, and continued compliance is inherently uncertain.

Before testing any compounds with potential therapeutic value in humans, the pharmaceutical product candidate enters the preclinical testing stage. Preclinical tests include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to assess the potential safety and activity of the pharmaceutical product candidate. These early proof-of-principle studies are done using sound scientific procedures and thorough documentation. The conduct of the single and repeat dose toxicology and toxicokinetic studies in animals must comply with federal regulations and requirements including good laboratory practices. The sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA has concerns and notifies the sponsor. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical study can begin. If resolution cannot be reached within the 30-day review period, either the FDA places the IND on clinical hold or the sponsor withdraws the application. The FDA may also impose clinical holds on a pharmaceutical product candidate at any time before or during clinical studies for various reasons. Accordingly, we cannot be sure that submission of an IND will result in the FDA allowing clinical studies to begin, or that, once begun, issues will not arise that suspend or terminate such clinical study.

Clinical studies involve the administration of the pharmaceutical product candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the clinical study sponsor's control. Clinical studies are conducted under protocols detailing, among other things, the objectives of the clinical study, dosing procedures, subject selection and exclusion criteria, how the results will be analyzed and presented and the parameters to be used to monitor subject safety. Each protocol must be submitted to the FDA as part of the IND. Clinical studies must be conducted in accordance with GCP. Further, each clinical study must be reviewed and approved by an independent IRB at, or servicing, each institution at which the clinical study will be conducted. An IRB is charged with protecting the welfare and rights of study participants and considers such items as whether the risks to individuals participating in the clinical studies are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the informed consent form that must be provided to each clinical study subject or his or her legal representative and must monitor the clinical study until completed.

Human clinical studies are typically conducted in three sequential phases that may overlap or be combined:

Phase 1: The pharmaceutical product is initially introduced into healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution and excretion. In the case of some products for severe or life-threatening diseases such as cancer, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients, with a goal of characterizing the safety profile of the drug and establishing a maximum tolerable dose ("MTD").

Phase 2: With the maximum tolerable dose established in a Phase 1 trial, the pharmaceutical product is evaluated in a limited patient population at the MTD to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases, to determine dosage tolerance, optimal dosage and dosing schedule and to identify patient populations with specific characteristics where the pharmaceutical product may be more effective.

Phase 3: Clinical studies are undertaken to further evaluate dosage, clinical efficacy and safety in an expanded patient population at geographically dispersed clinical study sites. These clinical studies are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for product labeling. The studies must be well controlled and usually include a control arm for comparison. One or two Phase 3 studies are usually required by the FDA for an NDA approval, depending on the disease severity and other available treatment options. In some instances, an NDA approval may be obtained based on Phase 2 clinical data with the understanding that the approved drug can be sold subject to a confirmatory trial to be conducted post-approval.

Post-approval studies, or Phase 4 clinical studies, may be conducted after initial marketing approval. These studies are often used to gain additional experience from the treatment of patients in the intended therapeutic indication. The FDA also may require Phase 4 studies, Risk Evaluation and Mitigation Strategies ("REMS") and post-marketing surveillance, among other things, to monitor the effects of an approved product or place conditions on an approval that could restrict the distribution or use of the product.

Progress reports detailing the results of the clinical studies must be submitted at least annually to the FDA and written IND safety reports must be submitted to the FDA and the investigators for serious and unexpected adverse events or any finding from tests in laboratory animals that suggests a significant risk for human subjects. Phase 1, Phase 2 and Phase 3 clinical studies may not be completed successfully within any specified period, if at all. The FDA or the sponsor or its data safety monitoring board may suspend a clinical study at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical study at its institution if the clinical study is not being conducted in accordance with the IRB's requirements or if the pharmaceutical product has been associated with unexpected serious harm to patients.

Concurrent with clinical studies, companies may complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the pharmaceutical product as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the pharmaceutical product candidate and, among other things, must develop methods for testing the identity, strength, quality and purity of the final pharmaceutical product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the pharmaceutical product candidate does not undergo unacceptable deterioration over its shelf life.

The results of product development, preclinical studies and clinical studies, along with descriptions of the manufacturing process, analytical tests conducted on the chemistry of the pharmaceutical product, proposed labeling and other relevant information are submitted to the FDA as part of an NDA requesting approval to market the product. The submission of an NDA is subject to the payment of substantial user fees. A waiver of such fees may be obtained under certain limited circumstances.

The FDA reviews all NDAs submitted before it accepts them for filing and may request additional information rather than accepting an NDA for filing. Once the submission is accepted for filing, the FDA begins an in-depth review of the NDA. Under the goals and policies agreed to by the FDA under the Prescription Drug User Fee Act ("PDUFA"), the FDA has 10 months after the 60-day filing date in which to complete its initial review of a standard review NDA and respond to the applicant, and six months after the 60-day filing date for a priority review NDA. The FDA does not always meet its PDUFA goal dates for standard and priority NDAs. The review process and the PDUFA goal date may be extended by three months if the FDA requests or if the applicant otherwise provides additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date.

After the NDA submission is accepted for filing, the FDA reviews the NDA application to determine, among other things, whether the proposed product is safe and effective for its intended use, and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, strength, quality and purity. The FDA may refer applications for novel pharmaceutical products or pharmaceutical products which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the pharmaceutical product approval process, the FDA also will determine

whether a REMS is necessary to assure the safe use of the pharmaceutical product. If the FDA concludes that a REMS is needed, the sponsor of the NDA must submit a proposed REMS; the FDA will not approve the NDA without a REMS, if required.

Before approving an NDA, the FDA will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA will typically inspect one or more clinical sites as well as the site where the pharmaceutical product is manufactured to assure compliance with GCP and cGMP. If the FDA determines the application, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information. In addition, the FDA will require the review and approval of product labeling.

The NDA review and approval process is lengthy and difficult and the FDA may refuse to approve an NDA if the applicable regulatory criteria are not satisfied or may require additional clinical data or other data and information. Even if such data and information is submitted, the FDA may ultimately decide that the NDA does not satisfy the criteria for approval. Data obtained from clinical studies are not always conclusive and the FDA may interpret data differently than we interpret the same data. The FDA will issue a complete response letter if the agency decides not to approve the NDA. The complete response letter usually describes all of the specific deficiencies in the NDA identified by the FDA. The deficiencies identified may be minor, for example, requiring labeling changes, or major, for example, requiring additional clinical studies. Additionally, the complete response letter may include recommended actions that the applicant might take to place the application in a condition for approval. If a complete response letter is issued, the applicant may either resubmit the NDA, addressing all of the deficiencies identified in the letter, or withdraw the application.

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. In addition, the FDA may require Phase 4 testing which involves clinical studies designed to further assess pharmaceutical product safety and effectiveness and may require testing and surveillance programs to monitor the safety of approved products that have been commercialized.

The 505(b)(2) NDA Regulatory Pathway

Most drug products obtain FDA marketing approval pursuant to a full Section 505(b)(1) NDA or an ANDA. A third alternative is a Section 505(b)(2) NDA, which enables the applicant to rely, in part, on studies not conducted by, or for, the applicant and for which the applicant has not obtained a right of reference or use, such as the FDA's findings of safety and/or effectiveness for a similar previously approved product, or published literature, in support of its application.

505(b)(2) NDAs often provide a path to FDA approval for new or improved formulations or new uses of previously approved products. Section 505(b)(2) permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by, or for, the applicant and for which the applicant has not obtained a right of reference. If the 505(b)(2) applicant can establish that reliance on the FDA's previous approval is scientifically appropriate, it may eliminate the need to conduct certain preclinical or clinical studies of the new product. The FDA may also require companies to perform additional studies or measurements to support the change from the approved product. The FDA may then approve the new product candidate for all, or some, of the label indications for which the referenced product has been approved, as well as for any new indication sought by the Section 505(b)(2) applicant.

The FDCA also provides three years of marketing exclusivity for an NDA, 505(b)(2) NDA or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example, new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the conditions of use associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the original active agent. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

To the extent that the Section 505(b)(2) applicant is relying on studies conducted for an already approved product, the applicant is required to certify to the FDA concerning any patents listed for the approved product in the Orange Book to the same extent that an ANDA applicant would. Thus approval of a 505(b)(2) NDA can be stalled until all the listed patents claiming the referenced product have expired, until any non-patent exclusivity, such as exclusivity for obtaining approval of a new chemical entity, listed in the Orange Book for the referenced product has expired, and, in the case of a Paragraph IV certification and subsequent patent infringement suit,

until the earlier of 30 months, settlement of the lawsuit or a decision in the infringement case that is favorable to the Section 505(b)(2) applicant.

Expedited Development and Review Programs

The FDA has various programs intended to expedite or facilitate the process for developing and reviewing new pharmaceutical products that meet certain criteria. For example, new pharmaceutical products are eligible for fast track designation if they are intended to treat a serious condition and demonstrate the potential to address unmet medical needs for the condition. Fast track designation applies to the combination of the product and the specific indication for which it is being studied. Unique to a fast track product, the FDA may consider for review sections of the NDA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA, if the FDA determines that the schedule is acceptable and if the sponsor pays any required user fees upon submission of the first section of the NDA.

Any product submitted to the FDA for market, including a fast track program, may also be eligible for other FDA programs intended to expedite development and review, such as priority review and accelerated approval. Any product is eligible for priority review if it is intended to treat a serious condition and it offers a significant improvement in the treatment, diagnosis or prevention of a disease compared to marketed products. The FDA will attempt to direct additional resources to the evaluation of an application for a new pharmaceutical product designated for priority review in an effort to facilitate the review. Additionally, accelerated approval may be available for a product intended to treat a serious condition that provides meaningful therapeutic benefit over existing treatments, which means the product may be approved on the basis of adequate and well-controlled clinical studies establishing that the product has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on an intermediate clinical endpoint. As a condition of accelerated approval, the FDA may require the sponsor to perform adequate and well-controlled post-marketing clinical studies. In addition, the FDA currently requires pre-approval of promotional materials for products receiving accelerated approval, which could impact the timing of the commercial launch of the product. fast track designation, priority review and accelerated approval do not change the standards for approval but may expedite the development or approval process.

Post-Approval Requirements

Any pharmaceutical products for which we receive FDA approvals are subject to continuing regulation by the FDA, including, among other things, cGMP compliance, record-keeping requirements, reporting of adverse experiences with the product, providing the FDA with updated safety and efficacy information, product sampling and distribution requirements, complying with certain electronic records and signature requirements and complying with FDA promotion and advertising requirements, which include, among others, standards for direct-to-consumer advertising, prohibitions on promoting pharmaceutical products for uses or in patient populations that are not described in the pharmaceutical product's approved labeling (known as "off-label use"), industry-sponsored scientific and educational activities and promotional activities involving the internet. Failure to comply with FDA requirements can have negative consequences, including adverse publicity, enforcement letters from the FDA, actions by the U.S. Department of Justice and/or U.S. Department of Health and Human Services' Office of Inspector General, mandated corrective advertising or communications with doctors, and civil or criminal penalties. Although physicians may prescribe legally available pharmaceutical products for off-label uses, manufacturers may not directly or indirectly market or promote such off-label uses.

We expect to rely on third parties for the production of clinical and commercial quantities of our products. Manufacturers of our products are required to comply with applicable FDA manufacturing requirements contained in the FDA's cGMP regulations. cGMP regulations require, among other things, quality control and quality assurance, as well as the corresponding maintenance of records and documentation. Pharmaceutical product manufacturers and other entities involved in the manufacture and distribution of approved pharmaceutical products are required to register their establishments with the FDA and certain state agencies and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer or holder of an approved NDA, including withdrawal of the product from the market. In addition, changes to the manufacturing process generally require prior FDA approval before being implemented and other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

The Priority Review Voucher Program

The FD&C Act section 524, authorizes FDA to award priority review vouchers (PRVs) to sponsors of approved tropical disease product applications that meet certain criteria. In July 2020, the FDA updated its guidance providing information on the implementation of section 524 of the FD&C Act — Tropical Disease Priority Review Vouchers Guidance for Industry.

To qualify for a PRV, a sponsor's application must be for a drug or biological product for the prevention or treatment of a "tropical disease," must otherwise qualify for priority review, and must contain no active ingredient (including any salt or ester of an active ingredient) that has been approved in any other application under section 505(b)(1) of the FD&C Act or section 351 of the Public Health Services Act.

While the FDA aims to complete a standard review in about 10 months, a PRV may result in the acceleration of review and approval of a product candidate by up to four months. However, despite the issuance of a PRV, the FDA is not obligated to, and may not, accelerate the timing of a review.

The Food and Drug Administration Reauthorization Act of 2017 made changes to the eligibility criteria for receipt of a tropical disease priority review voucher. Applications submitted after September 30, 2017 must also contain reports of one or more new clinical investigations (other than bioavailability studies) that are essential to the approval of the application and conducted or sponsored by the sponsor. In addition, the applicant must provide in the application an attestation that such report(s) were not submitted as part of an application for marketing approval or licensure by a regulatory authority in India, Brazil, Thailand, or any country that is a member of the Pharmaceutical Inspection Convention or the Pharmaceutical Inspection Cooperation Scheme prior to September 27, 2007.

Once the sponsor obtains a PRV, the voucher can be used to obtain priority review designation for a subsequent application that does not itself qualify for priority review as described in the guidance.

HAT is on FDA's current list of tropical diseases. We are not aware of any clinical trial activity in East African HAT, other than the activities we are conducting.

Exclusivity

Under various statutes, products approved by FDA may qualify for market exclusivity. For example, orphan drug exclusivity prevents, with some exceptions, FDA approval for seven years of another product with the same active moiety for the same rare disease. Hatch-Waxman exclusivity provides new chemical entities five years of protection against FDA approval of ANDAs relying on the application of the new chemical entity. Under another form of Hatch-Waxman exclusivity, products that are not new chemical entities may obtain three years of exclusivity against approval of ANDAs relying on the application of that product.

While we anticipate that we will be eligible to be awarded FDA market exclusivity for each program that we bring to market, there can be no certainty that any of our products will be eligible for or obtain any form of FDA exclusivity.

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any pharmaceutical product candidates for which we may obtain regulatory approval. In the United States and in markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend in part upon the availability of reimbursement from third-party payers. Third-party payers include government payers such as Medicare and Medicaid, managed care providers, private health insurers and other organizations. The process for determining whether a payer will provide coverage for a pharmaceutical product may be separate from the process for setting the price or reimbursement rate that the payer will pay for the pharmaceutical product. Third-party payers may limit coverage to specific pharmaceutical products on an approved list, or formulary, which might not, and frequently does not include all of the FDA- approved pharmaceutical products for a particular indication. Third-party payers are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. A payer's decision to provide coverage for a pharmaceutical product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development. In addition, in the United States there is a growing emphasis on comparative effectiveness research, both by private payers and by government agencies. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain the FDA approvals. Our pharmaceutical product candidates may not be considered medically necessary or cost-effective. To the extent other drugs or

therapies are found to be more effective than our products, payers may elect to cover such therapies in lieu of our products and/or reimburse our products at a lower rate.

The marketability of any pharmaceutical product candidates for which we may receive regulatory approval for commercial sale may suffer if the government and third-party payers fail to provide adequate coverage and reimbursement. In addition, emphasis on managed care in the United States has increased and we expect this will continue to increase the pressure on pharmaceutical pricing. Coverage policies and third- party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we may receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

International Regulation

In addition to regulations in the United States, we will be subject to a variety of foreign regulations governing clinical trials and commercial sales and distribution of our future drugs. Whether or not we obtain FDA approval for a drug, we must obtain approval of a drug by the comparable regulatory authorities of foreign countries before we can commence clinical trials or marketing of the drug in those countries. The approval process varies from country to country, and the time may be longer or shorter than that required for FDA approval. The requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly from country to country.

Under European Union regulatory systems, marketing authorizations may be submitted either under a centralized or mutual recognition procedure. The centralized procedure provides for the grant of a single marketing authorization that is valid for all European Union member states. The mutual recognition procedure provides for mutual recognition of national approval decisions. Under this procedure, the holder of a national marketing authorization may submit an application to the remaining member states. Within 90 days of receiving the applications and assessment report, each member state must decide whether to recognize approval.

In addition to regulations in Europe and the United States, we will be subject to a variety of foreign regulations governing clinical trials and commercial distribution of our future drugs.

Employees and Human Capital

As of December 31, 2023, we had six full-time employees and one part-time employee. Each of these employees is located in the United States.

We are subject to local labor laws and regulations with respect to our employees in the jurisdictions in which they are employed. These laws principally concern matters such as paid annual vacation, paid sick days, length of the workday and work week, minimum wages, pay for overtime, and insurance for workers' compensation.

Our employees are not represented by a labor union, and it is our understanding that our relations with our employees are satisfactory.

Our values – high performance, compliance, integrity and collaboration – are built on the foundation that our people and the way we treat one another promote creativity, innovation and productivity, which spur the Company's success. Providing market competitive pay and benefit programs, opportunities to participate in the success they help create, we create a culture in which all colleagues have the opportunity to thrive.

ITEM 1A. RISK FACTORS.

Investing in our common stock involves a high degree of risk. These risks include, but are not limited to, those described below, each of which may be relevant to an investment decision. You should carefully consider the risks described below, together with all of the other information in this Annual Report on Form 10-K, including our financial statements and related notes and Item 7. "Management's Discussion and Analysis of Financial Condition and Results of Operations." before deciding to invest in our common stock. The realization of any of these risks could have a significant adverse effect on our reputation, business, financial condition, results of operations and growth, and our ability to accomplish our strategic objectives. In that event, the market price of our common stock could decline, and you may lose part or all of your investment. Additional risks and uncertainties not presently known to us or that we currently deem immaterial may also impair our business operations and the market value of our common stock.

Summary of Risk Factors

Our business and an investment in our company is subject to numerous risks, many of which are discussed in the section entitled "Risk Factors" set forth in this Annual Report on Form 10-K. Some of these risks include:

- We are an early clinical stage pharmaceutical company with a limited operating history.
- We have never generated revenue from operations, are unlikely to generate revenues for several years, and our recurring losses from operations have raised substantial doubt regarding our ability to continue as a going concern. We may never become profitable or, if we achieve profitability, be able to sustain profitability.
- We need to raise additional capital to fund our operations, which may not be available on acceptable terms, if at all.
- Future sales of shares by existing stockholders or us could cause our stock price to decline.
- The sale of our common stock through our Lincoln Park Purchase Agreement may cause substantial dilution to our existing stockholders, and such sales, or the anticipation of such sales, may cause the price of our common stock to decline. Moreover, the terms of the Lincoln Park Purchase Agreement limit the amount of shares of common stock we may issue to Lincoln Park, which may require us to utilize more costly and time-consuming means of accessing the capital markets, which could materially adversely affect our liquidity and cash position.
- Our independent auditor's report for the fiscal year ended December 31, 2023 includes an explanatory paragraph regarding substantial doubt about our ability to continue as a going concern.
- We have identified material weaknesses in our internal control and if our remediation of such material weaknesses is not effective, or if we fail to develop and maintain an effective system of disclosure controls and internal control, our ability to produce timely and accurate financial statements or comply with applicable laws and regulations could be impaired.
- Our research and development are primarily focused on the drug suramin, leaving us subject to the risk of a lack of diversity in the active pharmaceutical ingredients we utilize in our business. We do not know whether we will be successful in our efforts to build a pipeline of product candidates or if we will be able to develop any products of commercial value.
- We cannot be certain that PAX-101 or any other product candidates that we may develop or acquire will receive regulatory approval, and without regulatory approval we will not be able to market any of our product candidates. Any delay in the regulatory review or approval of any of our product candidates will materially or adversely harm our business.
- Even if we obtain regulatory approval for our product candidates, if we are unable to successfully commercialize our products, it will limit our ability to generate revenue and will materially adversely affect our business, financial condition and results of operations.
- We have received deficiency letters from Nasdaq relating to non-compliance with Nasdaq's continued listing requirements. Our common stock could become subject to delisting from Nasdaq if we fail to regain compliance.

- While we believe we may be eligible to receive a tropical disease PRV for the use of PAX-101 for the treatment of HAT, there is a risk that we will not receive such PRV, which would require us to find alternative sources of funding for our later stage clinical programs.

- It is difficult and costly to protect our intellectual property rights, and we cannot ensure the protection of these rights.

- PAX-101 and our other product candidates may infringe the intellectual property rights of others, which could increase our costs and delay or prevent our development and commercialization efforts.

- Clinical and preclinical drug development is a lengthy and expensive process with uncertain outcomes that may lead to delayed timelines and increased cost, which may prevent us from being able to complete clinical trials.

- If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our drug candidates.

- We face competition from other biotechnology and pharmaceutical companies and our operating results will suffer if we fail to compete effectively.

- Since certain of our directors and officers are employed by and/or consult for other companies, their other activities could compete for time on, or create conflicts of interest with, our activities.

- Adverse developments affecting the financial services industry, such as actual events or concerns involving liquidity, could adversely affect our current and projected business operations and our financial condition and results of operations.

Risks Related to Our Financial Position and Need for Capital

Our independent auditor's report for the fiscal year ended December 31, 2023 includes an explanatory paragraph regarding substantial doubt about our ability to continue as a going concern.

Due to the uncertainty of our ability to meet our current operating and capital expenses, in its report on our audited annual financial statements as of and for the year ended December 31, 2023, our independent auditors included an explanatory paragraph regarding concerns about our ability to continue as a going concern. Recurring losses from operations raise substantial doubt about our ability to continue as a going concern. If we are unable to continue as a going concern, we might have to liquidate our assets and the value we receive for our assets in liquidation or dissolution could be significantly lower than the values reflected in our financial statements.

We are an early clinical stage pharmaceutical company with a limited operating history.

We are an early clinical stage pharmaceutical company with a limited operating history. We must complete clinical studies and receive regulatory approval of an NDA before commercial sales of a product can commence. The likelihood of success of our business plan must be considered in light of the problems, substantial expenses, difficulties, complications and delays frequently encountered in connection with developing and expanding early-stage businesses and the regulatory and competitive environment in which we operate. Pharmaceutical product development is a highly speculative undertaking, involves a substantial degree of risk and is a capital-intensive business.

Accordingly, you should consider our prospects in light of the costs, uncertainties, delays and difficulties frequently encountered by companies in the early stages of development, especially early-stage clinical pharmaceutical companies such as ours. Potential investors should carefully consider the risks and uncertainties that a company with a limited operating history will face. In particular, potential investors should consider that we cannot assure you that we will be able to, among other things:

- successfully implement or execute our current business plan, and we cannot assure you that our business plan is sound;

- successfully manufacture our clinical products and establish commercial drug supply;

- successfully complete the clinical trials necessary to obtain regulatory approval for the marketing of our drug candidates, including PAX-101;

- secure, maintain and, as necessary, defend our intellectual property rights;
- secure market exclusivity and/or adequate intellectual property protection for our drug candidates;
- attract and retain an experienced management and advisory team;
- secure acceptance of our drug candidates in the medical community and with third-party payors and consumers;
- launch commercial sales of our drug candidates, whether alone or in collaboration with others;
- raise sufficient funds in the capital markets or otherwise to effectuate our business plan; and
- utilize the funds that we do have and/or raise in this offering or in the future to efficiently execute our business strategy.

If we cannot successfully execute any one of the foregoing, our business may fail and your investment will be adversely affected.

We have never generated revenue from operations, are unlikely to generate revenues for several years, and our recurring losses from operations have raised substantial doubt regarding our ability to continue as a going concern. We may never become profitable or, if we achieve profitability, be able to sustain profitability.

We have never generated revenue from operations, are unlikely to generate revenues for several years, and are currently operating at a loss and expect our operating costs will increase significantly as we incur further costs related to preclinical development and the clinical trials for our drug candidates. We expect to incur substantial expenses without corresponding revenues unless and until we are able to obtain regulatory approval and successfully commercialize any of our drug candidates. We may never be able to obtain regulatory approval for the marketing of our drug candidates in any indication in the United States or internationally. Even if we are able to commercialize our drug candidates, there can be no assurance that we will generate significant revenues or ever achieve profitability. We have incurred recurring losses since inception and have an accumulated deficit of approximately $52.0 million as of December 31, 2023, which recurring losses have raised substantial doubt regarding our ability to continue as a going concern.

We anticipate operating losses to continue for the foreseeable future due to, among other things, costs related to research funding, development of our product candidates and preclinical and clinical programs, regulatory clearances, strategic alliances, the development of our administrative organization, as well as costs to comply with the requirements of being a public company operating in a highly regulated industry. As of December 31, 2023, we had $4.7 million of cash and cash equivalents. Our forecast of the period of time through which our current financial resources will be adequate to support our operations and the costs to support our general and administrative, sales and marketing, research and development activities and costs to comply with the requirements of being a public company operating are forward-looking statements and involve risks and uncertainties. The financial statements do not include any adjustments that might be necessary should we be unable to continue as a going concern.

We are uncertain when or if we will be able to achieve or sustain profitability. If we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods. Failure to become and remain profitable would impair our ability to sustain operations and adversely affect the price of our common stock and our ability to raise capital.

We need to raise additional capital to fund our operations, which may not be available on acceptable terms, if at all.

Our ability to continue as a going concern, maintain our listing on Nasdaq, and continue our operations is dependent on our ability to raise additional capital and should we be unable to raise sufficient additional capital, we may be required to undertake cost-cutting measures including delaying or discontinuing certain clinical activities. We need to raise significant additional capital to continue to fund the clinical trials for PAX-101, and our other product candidates. We will likely seek to sell common equity, including pursuant to the Lincoln Park Purchase Agreement (as defined below), preferred equity or convertible debt securities, enter into a credit facility or another form of third-party funding, or seek other debt financing. In addition, the sale of equity and convertible debt securities may result in dilution to our stockholders and certain of those securities may have rights senior to those of our common stock. If we raise additional funds through the issuance of preferred stock, convertible debt securities or other debt financing, these securities or other debt could contain covenants that would restrict our operations, fund raising capabilities or otherwise. Any other third-party funding arrangement could require us to relinquish valuable rights.

The source, timing and availability of any future financing will depend principally upon market conditions, and, more specifically, on the progress of our clinical development programs. Funding may not be available when needed, at all, or on terms acceptable to us. Lack of necessary funds may require us, among other things, to delay, scale back or eliminate some or all of our planned clinical trials, development programs or operations. These factors among others create a substantial doubt about our ability to continue as a going concern.

Future sales of shares by existing stockholders or us could cause our stock price to decline.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock.

We may issue additional shares of our common stock or securities convertible into our common stock from time to time in connection with a financing, acquisition, investments or otherwise. Any such issuance could result in substantial dilution to our existing stockholders and cause the trading price of our common stock to decline.

Specially, if and when we sell shares of common stock to Lincoln Park pursuant to the Lincoln Park Purchase Agreement, Lincoln Park may resell all, some or none of such shares at any time or from time to time in its discretion, subject to compliance with applicable securities laws. Therefore, sales to Lincoln Park by us could result in substantial dilution to the interests of other holders of our common stock. Additionally, the sale of a substantial number of shares of common stock to Lincoln Park, or the anticipation of such sales, could make it more difficult for us to sell equity or equity-related securities in the future at a time and at prices that it might otherwise wish to effect such sales.

Sales of our common stock may be made by holders of our public float or by holders of restricted securities in compliance with the provisions of Rule 144 of the Securities Act of 1933, or the Securities Act. There were 7,401,242 shares of common stock outstanding as of December 31, 2023, substantially all of which are freely tradable, without restriction, in the public market. We registered up to 770,718 shares of common stock pursuant to the Lincoln Park Purchase Agreement, of which 549,896 remain available, and, if and when we sell shares to Lincoln Park thereunder, they will be freely tradeable pursuant to such registration statement. We have also registered 98,619 shares of our common stock underlying existing grants or grants that we may issue under our equity compensation plan.

Also, in general, under Rule 144, a non-affiliated person who has satisfied a six-month holding period in a company registered under the Exchange Act, as amended, may, sell their restricted common stock without volume limitation, so long as the issuer is current with all reports under the Exchange Act in order for there to be adequate common public information.

Affiliated persons may also sell their common shares held for at least six months, but affiliated persons will be required to meet certain other requirements, including manner of sale, notice requirements and volume limitations. Non-affiliated persons who hold their common shares for at least one year will be able to sell their common stock without the need for there to be current public information in the hands of the public. Future sales of shares of our public float or by restricted common stock made in compliance with Rule 144 may have an adverse effect on the then prevailing market price, if any, of our common stock.

The sale of our common stock through our Lincoln Park Purchase Agreement may cause substantial dilution to our existing stockholders, and such sales or issuances, or the anticipation of such sales or issuances, may cause the price of our common stock to decline. Moreover, the terms of the Lincoln Park Purchase Agreement limit the amount of shares of common stock we may issue to Lincoln Park, which may require us to utilize more costly and time-consuming means of accessing the capital markets, which could materially adversely affect our liquidity and cash position.

On November 17, 2022, we entered into a Purchase Agreement (the "Lincoln Park Purchase Agreement") with Lincoln Park Capital Fund, LLC ("Lincoln Park"), under which, from time to time, we may cause Lincoln Park to purchase shares of our common stock. Although we have the right to control whether we sell any shares, if at all, under the Lincoln Park Purchase Agreement, and we generally have the right to control the timing and amount of any such sales, we are subject to certain restrictions, including those that limit the number of shares we may sell. We may not sell shares to Lincoln Park if it would result in Lincoln Park beneficially owning more than 9.99% of our then outstanding shares of common stock. Accordingly, we may not be able to utilize the Purchase Agreement to raise additional capital when, or in the amounts, we desire. If we cannot sell the full amount of the shares of common stock that Lincoln Park has committed to purchase because of these limitations, we may be required to utilize more costly and time-consuming means of accessing the capital markets, which could materially adversely affect our liquidity and cash position.

The 549,896 shares of common stock that remain to be resold into the public markets pursuant to the prospectus that is part of the registration statement on Form S-1 (File No. 333-268882) that was declared effective by the SEC in December 2022, represents approximately 7.4% of the shares of common stock outstanding as of December 31, 2023. While we generally have the right to control the timing and amount of any future sales of our shares to Lincoln Park, additional sales of our common stock, if any, to Lincoln Park will depend upon market conditions and other factors to be determined by us. We may ultimately decide to sell to Lincoln Park all, some or none of the additional shares of our common stock that may be available for us to sell pursuant to the Lincoln Park Purchase Agreement. If and when we do sell shares to Lincoln Park, after Lincoln Park has acquired the shares, Lincoln Park may resell all, some or none of those shares at any time or from time to time in its discretion. Therefore, sales to Lincoln Park by us could result in substantial dilution to the interests of other holders of our common stock. Additionally, the sale of a substantial number of shares of our common stock to Lincoln Park, or the anticipation of such sales, could make it more difficult for us to sell equity or equity-related securities in the future at a time and at a price that we might otherwise wish to effect sales.

Risks Related to Product Development, Regulatory Approval, Manufacturing and Commercialization

Our research and development are primarily focused on the drug suramin, leaving us subject to the risk of a lack of diversity in the active pharmaceutical ingredients we utilize in our business. We do not know whether we will be successful in our efforts to build a pipeline of product candidates or if we will be able to develop any products of commercial value.

Any product candidates that we develop or acquire may not be effective for the target indications and we may not be successful in using our intellectual property to build a pipeline of product candidates and progress these product candidates through clinical development for the treatment of any medical conditions**.** Moreover, our business plan currently is focused primarily on the use of the drug suramin, leaving us subject to the risk of a lack of diversity in our product pipeline and the active pharmaceutical ingredients we utilize in our business.

Even if we are successful in continuing to build our pipeline, we may not be able to develop or acquire other product candidates that are safe and effective. Our research programs may initially show promise in creating potential product candidates, yet fail to yield viable product candidates for further clinical development for a number of reasons, including as a result of being shown to have harmful side effects or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance. Our research programs to identify new product candidates will require substantial technical, financial and human resources. In addition, we may focus our efforts and resources on one or more potential product candidates that ultimately prove to be unsuccessful in clinical trials testing efficacy and safety. If we are unable to identify suitable additional compounds for preclinical and clinical development, our ability to develop product candidates and obtain product revenues in future periods could be compromised, which could result in significant harm to our financial position and adversely impact our stock price.

We cannot be certain that PAX-101 or any other product candidates that we may develop or acquire will receive regulatory approval, and without regulatory approval we will not be able to market any of our product candidates. Any delay in the regulatory review or approval of any of our product candidates will materially or adversely harm our business.

We expect to invest most of our capital in the development of PAX-101 and PAX-102. Our ability to generate revenue related to product sales, which we do not expect will occur for at least the next several years, if ever, will depend on the successful development and regulatory approval of one or more of our product candidates. All of our product candidates require regulatory review and approval prior to commercialization. Any delays in the regulatory review or approval of our product candidates would delay market launch, increase our cash requirements and result in additional operating losses. This failure to obtain regulatory approvals would prevent our product candidate from being marketed and would have a material and adverse effect on our business.

The process of obtaining FDA and other required regulatory approvals, including foreign approvals, often takes many years and can vary substantially based upon the type, complexity and novelty of the products involved. Furthermore, this approval process is extremely complex, expensive and uncertain. We may be unable to submit any new drug application, or NDA, in the United States or any marketing approval application in foreign jurisdictions for any of our products. If we submit an NDA including any amended NDA or supplemental NDA, to the FDA seeking marketing approval for any of our product candidates, the FDA must decide whether to accept or reject the submission for filing. We cannot be certain that any of these submissions will be accepted for filing and reviewed by the FDA, or that the marketing approval application submissions to any other regulatory authorities will be accepted for filing and review by those authorities. We cannot be certain that we will be able to respond to any regulatory requests during the review period in a timely manner, or at all, without delaying potential regulatory action. We also cannot be certain that any of our product candidates will receive favorable recommendations from any FDA advisory committee or foreign regulatory bodies or be approved for marketing by the FDA or foreign regulatory authorities. In addition, delays in approvals or rejections of marketing applications may be based upon

many factors, including regulatory requests for additional analyses, reports, data and studies, regulatory questions regarding data and results, changes in regulatory policy during the period of product development and the emergence of new information regarding such product candidates.

The results of preclinical studies and clinical trials may not result in the demonstration of safety or efficacy of the products we are developing. Further, the data from these studies are subject to different interpretations, which could delay, limit or prevent regulatory review or approval of any of our product candidates. Furthermore, regulatory attitudes towards the data and results required to demonstrate safety and efficacy can change over time and can be affected by many factors, such as the emergence of new information, including on other products, policy changes and agency funding, staffing and leadership. We do not know whether future changes to the regulatory environment will be favorable or unfavorable to our business prospects.

In addition, the environment in which our regulatory submissions may be reviewed changes over time. For example, average review times at the FDA for NDAs have fluctuated over the last ten years, and we cannot predict the review time for any of our submissions with any regulatory authorities. Review times can be affected by a variety of factors, including budget and funding levels and statutory, regulatory and policy changes. Moreover, in light of widely publicized events concerning the safety risk of certain drug products, regulatory authorities, members of the U.S. Government Accountability Office, medical professionals and the general public have raised concerns about potential drug safety issues. These events have resulted in the withdrawal of drug products, revisions to drug labeling such as boxed warnings and precautions that further limit use of the drug products, and establishment of Risk Evaluation and Mitigation Strategy ("REMS") measures that may, for instance, restrict distribution of drug products. Drug safety issues may result in a more cautious approach by the FDA to clinical trials. Data from clinical trials may receive greater scrutiny with respect to safety, which may make the FDA or other regulatory authorities more likely to terminate clinical trials before completion, or require longer or additional clinical trials that may result in substantial additional expense and a delay or failure in obtaining approval or may result in approval for a more limited indication than originally sought.

Clinical and preclinical drug development is a lengthy and expensive process with uncertain outcomes that may lead to delayed timelines and increased cost, which may prevent us from being able to complete clinical trials.

Clinical testing is expensive, can take many years to complete, and its outcome is inherently uncertain. The results of preclinical and clinical studies of our product candidates may not be predictive of the results of later-stage clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy despite having progressed through preclinical studies and initial clinical trials. A number of companies in the pharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier studies, and we cannot be certain that we will not face similar setbacks.

In addition, there may be third party individuals or groups that publish data from experiments using suramin that may reflect, either positively or negatively, on our clinical development program despite that we have no affiliation with or control over such individuals or groups. For example, we are aware of other suramin-related research that has been conducted in the autism indication at the University of California, San Diego as well as in other unrelated indications within and outside of the United States. Our clinical development programs could be negatively impacted by adverse events reported in such third party studies.

With respect to FXS, FXTAS, ME/CFS and LCS, no company, to our knowledge, has yet been successful in its efforts to obtain regulatory approval in the United States or Europe of treatment for these conditions. The mechanism of disease for these conditions has not been scientifically confirmed, and as a result, the mechanism of action for PAX-101 in potentially treating these diseases is unknown. In addition, LCS is potentially a self-resolving disease in some people, as well as a disease that increases and decreases in severity. As such, there may not be sufficient biomarkers or validated behavioral scoring metrics that could be used to support potential approval for PAX-101 in these diseases, and clinical trials will be difficult to design, conduct and assess.

This will make our development and potential approval of PAX-101 for these indications very difficult, and we may not be successful.

We cannot be certain that clinical trials for PAX-101 or any of our other product candidates will be completed, or completed on schedule, or that any other future clinical trials for PAX-101 or any of our other product candidates, will begin on time, not need to be redesigned, enroll an adequate number of patients on time or be completed on schedule, if at all, or that any interim analyses with respect to such trials will be completed on schedule or support continued clinical development of the associated product candidate. In particular, the basis for our submission of an NDA for approval of PAX-101 in HAT is historical data that is limited and not complete, and FDA may not agree that our study design is adequate or the data sufficient for approval. Because of the difficulties inherent in designing

clinical trials for a universally fatal disease, we may not be able to provide FDA with additional data (regarding safety and effectiveness) or analyses adequate for approval if requested by the FDA, which could prevent us from ever getting approval for PAX-101 in HAT.

We could also encounter delays if a clinical trial is suspended or terminated by us upon recommendation of the data monitoring committee for such trial, by the institutional review board ("IRB") of the institutions in which such trials are being conducted, or by the FDA or other regulatory authorities. Such authorities may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, site misconduct or deviations from Good Clinical Practice, major findings from an inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions, or lack of adequate funding to continue the clinical trial.

If we experience delays in the completion of, or termination of, any clinical trial of our product candidates, the commercial prospects of our product candidates may be harmed, and our ability to generate revenue from the sale of any of these product candidates will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow down our product candidate development and approval processes, and jeopardize our ability to commence product sales and generate revenue. Any of these occurrences may significantly harm our business, financial condition and prospects.

If the FDA does not conclude that our product candidates satisfy the requirements for the 505(b)(2) regulatory approval pathway, or if the requirements for approval of any of our product candidates under Section 505(b)(2) are not as we expect, the approval pathway for our product candidates will likely take significantly longer, cost significantly more and encounter significantly greater complications and risks than anticipated, and in any case may not be successful.

We intend to seek FDA approval through the 505(b)(2) regulatory pathway for each of our product candidates. The Drug Price Competition and Patent Term Restoration Act of 1984, also known as the Hatch-Waxman Act, added Section 505(b)(2) to the Federal Food, Drug, and Cosmetic Act, or FDCA. Section 505(b)(2) permits the filing of an NDA where at least some of the information required for approval comes from studies that were not conducted by or for the applicant.

If the FDA does not allow us to pursue the 505(b)(2) regulatory pathway for our product candidates as anticipated, we may need to conduct additional clinical trials, provide additional data and information and meet additional standards for regulatory approval. If this were to occur, the time and financial resources required to obtain FDA approval for our product candidates would likely substantially increase. Moreover, the inability to pursue the 505(b)(2) regulatory pathway could result in new competitive products reaching the market faster than our product candidates, which could materially adversely impact our competitive position and prospects. Even if we are permitted to pursue the 505(b)(2) regulatory pathway for a product candidate, we cannot assure you that we will receive the requisite or timely approvals for commercialization of such product candidate.

In addition, notwithstanding the approval of a number of products by the FDA under Section 505(b)(2) over the last few years, certain competitors and others have objected to the FDA's interpretation of Section 505(b)(2). We expect that our competitors could file citizens' petitions with the FDA in an attempt to persuade the FDA that our product candidates, or the clinical studies that support their approval, contain deficiencies. If the FDA's interpretation of Section 505(b)(2) is successfully challenged, the FDA may be required to change its Section 505(b)(2) policies and practices, which could delay or even prevent the FDA from approving any NDA that we submit under Section 505(b)(2).

Delays in the commencement, enrollment and completion of our clinical trials could result in increased costs to us and may delay or limit our ability to obtain regulatory approval for PAX-101 and our other product candidates.

Delays in the commencement, enrollment and completion of clinical trials could increase our product development costs or delay the regulatory approval of our product candidates. The commencement, enrollment and completion of clinical trials can be delayed for a variety of reasons, including:

- the inability to reach agreements on acceptable terms with prospective CROs and trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;

- the inability to maintain necessary supplies of study drug and comparator to maintain predicted enrollment rates at clinical trial sites;

- regulatory authority objections to commencing a clinical trial;

- the inability to obtain ethics committee or IRB approval to conduct a clinical trial;

- difficulty recruiting and enrolling subjects to participate in clinical trials for a variety of reasons, including meeting the enrollment criteria for our study and competition from other clinical trial programs for the same indication as our product candidates;

- difficulty obtaining informed consent in some patient populations who may be under 18 years of age and may not have the capacity to consent;

- the inability to retain subjects in clinical trials due to the treatment protocol, personal issues, side effects from the therapy or lack of efficacy; and

- difficulty in importing and exporting clinical trial materials and study samples.

We currently do not have quantities of suramin sufficient to support all of our clinical trial needs and we will need to rely on third-party manufacturers to meet the demand of future production.

There is currently one manufacturer of suramin, Bayer, which does not manufacture suramin on a regular basis and, when it does, generally only manufactures small quantities in response to outbreaks of HAT.

We have engaged two independent contract development manufacturing organizations to develop, validate and scale our supply chain for suramin and the shelf stable drug product that is ultimately distributed to pharmacies. We have completed the necessary steps to begin producing suramin in early 2022 and began the final stage of the development phase in the first quarter 2024, including the production and final release testing process, that will produce suramin for both clinical and registrational purposes. However, this final development phase is expected to take a significant amount of time and we cannot conduct our clinical trials until we have sufficient suramin supply. We currently do not expect to have quantities of suramin sufficient to support submission of an NDA for PAX-101's East African HAT indication until the fourth quarter of 2024.

If our suppliers of active ingredients to manufacture suramin are unable or unwilling to perform for any reason, we may not be able to locate alternative acceptable manufacturers or formulators or enter into favorable agreements with them. Any inability to acquire sufficient quantities of suramin or other active pharmaceutical ingredients we may utilize in a timely manner from third parties could delay clinical trials and prevent us from developing our products in a cost-effective manner or on a timely basis. In addition, manufacturers of our product candidates are subject to current Good Manufacturing Practices ("cGMP") and similar foreign standards and we would not have control over compliance with these regulations by our manufacturers. If one of our contract manufacturers fails to maintain compliance, the production of our products could be interrupted, resulting in delays and additional costs. In addition, if the facilities of such manufacturers do not pass a pre-approval or post-approval plant inspection, the FDA will not grant approval and may institute restrictions on the marketing or sale of our products.

We are reliant on third-party manufacturers and suppliers to meet the demands of our clinical supplies, particularly for suramin**.** Delays in receipt of materials, scheduling, release, custom's control, and regulatory compliance issues may adversely impact our ability to initiate, maintain, or complete clinical trials that we are sponsoring**.** Commercial manufacturing and supply agreements have not been established. Issues arising from scale-up, environmental controls, equipment requirements, or other factors, may have an adverse impact on our ability to manufacture our product candidates.

Even if we obtain regulatory approval for our product candidates, if we are unable to successfully commercialize our products, it will limit our ability to generate revenue and will materially adversely affect our business, financial condition and results of operations.

Even if we obtain regulatory approval for our product candidates, our long-term viability and growth depend on the successful commercialization of products which lead to revenue and profits. Pharmaceutical product development is an expensive, high risk, lengthy, complicated, resource intensive process. In order to succeed, among other things, we must be able to:

- identify potential drug product candidates;

- design and conduct appropriate laboratory, preclinical and other research;
- submit for and receive regulatory approval to perform clinical studies;
- design and conduct appropriate preclinical and clinical studies according to good laboratory and good clinical practices;
- select and recruit clinical investigators;
- select and recruit subjects for our studies;
- collect, analyze and correctly interpret the data from our studies;
- submit for and receive regulatory approvals for marketing;
- secure market and formulary access from payors; and
- manufacture the drug product candidates according to cGMP.

The development program with respect to any given product may take many years and thus delay our ability to generate profits. In addition, potential products that appear promising at early stages of development may fail for a number of reasons, including the possibility that the products may require significant additional testing or turn out to be unsafe, ineffective, too difficult or expensive to develop or manufacture, too difficult to administer, or unstable. Failure to successfully commercialize our products will adversely affect our business, financial condition and results of operations.

The market for PAX-101's lead indication, HAT, is extremely small, as the majority of usage would be in Sub-Saharan Africa. Further, we would likely donate any product for use in this indication to the WHO for use by patients in Africa.

If our preclinical and clinical studies do not produce positive results, if our clinical trials are delayed or if serious side effects are identified during such studies or trials, we may experience delays, incur additional costs and ultimately be unable to commercialize our product candidates.

Before obtaining regulatory approval for the sale of our product candidates, we must conduct, generally at our own expense, extensive preclinical tests to demonstrate the safety of our product candidates in animals, and clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Preclinical and clinical testing is expensive, difficult to design and implement and can take many years to complete. A failure of one or more of our preclinical studies or clinical trials can occur at any stage of testing. We may experience numerous unforeseen events during, or as a result of, preclinical testing and the clinical trial process that could delay or prevent our ability to obtain regulatory approval or commercialize our product candidates, including:

- our preclinical tests or clinical trials may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional preclinical testing or clinical trials or we may abandon projects that we expect to be promising;
- regulators or ethics committees/IRBs may not authorize us to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- conditions imposed on us by the FDA or any non-U.S. regulatory authority regarding the scope or design of our clinical trials may require us to resubmit our clinical trial protocols to ethics committees/IRBs for re-inspection due to changes in the regulatory environment;
- the number of patients required for our clinical trials may be larger than we anticipate and recruitment of the target population may be more difficult than we anticipate, or participants may drop out of our clinical trials at a higher rate than we anticipate;
- our third-party contractors or clinical investigators may fail to comply with regulatory requirements or fail to meet their contractual obligations to us in a timely manner;

- we might have to suspend or terminate one or more of our clinical trials if we, the regulators or the ethics committees/IRBs determine that the participants are being exposed to unacceptable health risks;

- regulators or ethics committees/IRBs may require that we hold, suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements;

- the cost of our clinical trials may be greater than we anticipate;

- the supply or quality of our product candidates or other materials necessary to conduct our clinical trials may be insufficient or inadequate or we may not be able to reach agreements on acceptable terms with prospective clinical research organizations; and

- the effects of our product candidates may not be the desired effects or may include undesirable side effects or the product candidates may have other unexpected characteristics. For example, suramin can cause significant side effects, including nausea, vomiting, diarrhea, abdominal pain, and a feeling of general discomfort. Other side effects include skin sensations such as crawling or tingling sensations, tenderness of the palms and soles, numbness of the extremities, watery eyes, and photophobia. In addition, nephrotoxicity is common, as is peripheral neuropathy when the drug is administered at high doses.

In addition, if we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete our clinical trials or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining, or may not be able to obtain, marketing approval for one or more of our product candidates;

- obtain approval for indications that are not as broad as intended or entirely different than those indications for which we sought approval;

- the product labeling may be very restrictive and lead to limitations in commercial value; or

- have the product removed from the market after obtaining marketing approval.

Our product development costs will also increase if we experience delays in testing or approvals. We do not know whether any preclinical tests or clinical trials will be initiated as planned, will need to be restructured or will be completed on schedule, if at all. Significant preclinical or clinical trial delays also could shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. Such delays could allow our competitors to bring products to market before we do and impair our ability to commercialize our products and product candidates.

If we cannot enroll enough patients to complete our clinical trials, such failure may adversely affect our business, financial condition and results of operations.

The completion rate of clinical studies of our products is dependent on, among other factors, the patient enrollment rate. Patient enrollment is a function of many factors, including:

- securing sufficient numbers of investigators and clinical trial sites;

- investigator identification and recruitment of appropriate patients;

- ethics committees/IRBs and regulatory approvals to initiate study sites;

- patient population size;

- the nature of the protocol to be used in the trial;

- patient availability and proximity to clinical sites;

- the eligibility criteria for the study;
- competition from other companies' clinical studies for the same patient population; and
- the ability to obtain comparator drug/device.

We believe our procedures for enrolling patients have been appropriate; however, delays in patient enrollment would increase costs and delay ultimate commercialization and sales, if any, of our products. Such delays could have a material adversely affect our business, financial condition and results of operations.

If we are not successful in discovering, developing and commercializing additional product candidates, our ability to expand our business and achieve our strategic objectives would be impaired.

Research programs to identify product candidates require substantial technical, financial and human resources, whether or not any product candidates are ultimately identified. Our research programs may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development for many reasons, including the following:

- the research methodology used may not be successful in identifying potential product candidates;
- we may be unable to identify viable product candidates in our screening campaigns;
- competitors may develop alternatives that render our product candidates obsolete;
- product candidates we develop may nevertheless be covered by third parties' patents or other exclusive rights;
- a product candidate may, on further study, be shown to have harmful side effects or other characteristics that indicate it is unlikely to be effective or otherwise does not meet applicable regulatory criteria;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all;
- a product candidate may not be perceived or accepted as safe and effective by patients, the medical community or third-party payors; and
- the development of resistance to potential product candidates may render them ineffective against target infections with respect to our development program in HAT.

If we are unsuccessful in identifying and developing additional product candidates, our potential for growth may be impaired.

Even if we receive regulatory approval for PAX-101 or any other product candidates we may develop or acquire, we still may not be able to successfully commercialize it and the revenue that we generate from its sales, if any, may be limited.

If approved for marketing, the commercial success of PAX-101 or any other product candidates we may develop or acquire will depend upon acceptance by the medical community, including physicians, patients and health care payors. The degree of market acceptance of PAX-101 or such other product candidate will depend on a number of factors, including:

- demonstration of clinical safety and efficacy of such product candidate;
- relative convenience and ease of administration;
- the prevalence and severity of any adverse effects as well as the cost and convenience of monitoring and treating them;
- the willingness of physicians to prescribe such product candidate and of the target patient population to try new therapies;
- pricing and cost-effectiveness;

- the inclusion or omission of such product candidate in applicable treatment guidelines;
- the effectiveness of our or any future collaborators' sales and marketing strategies;
- limitations or warnings contained in FDA-approved labeling;
- our ability to obtain and maintain sufficient third-party coverage or reimbursement from government health care programs, including Medicare and Medicaid, private health insurers and other third-party payors; and
- the willingness of patients to pay out-of-pocket in the absence of third-party coverage or reimbursement.

If PAX-101 or any other product candidates we may develop or acquire is approved, but does not achieve an adequate level of adoption by physicians, health care payors and patients, we may not generate sufficient revenue and we may not be able to achieve or sustain profitability. Our efforts to educate the medical community and third-party payors on the benefits of such product candidate may require significant resources and may never be successful.

In addition, even if we obtain regulatory approvals, the timing or scope of any approvals may prevent or reduce our ability to commercialize such product candidate successfully. For example, if the approval process takes too long, we may miss market opportunities and give other companies the ability to develop competing products or establish market dominance. Any regulatory approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render such product candidate not commercially viable. For example, regulatory authorities may approve such product candidate for fewer or more limited indications than we request, may not approve the price we intend to charge for such product candidate, may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve such product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that indication. Further, the FDA may place conditions on approvals including potential requirements or risk management plans and the requirement for a REMS to assure the safe use of the drug. If the FDA concludes a REMS is needed, the sponsor of the NDA must submit a proposed REMS; and the FDA will not approve the NDA without an approved REMS. REMS could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. Any of these limitations on approval or marketing could restrict the commercial promotion, distribution, prescription or dispensing of such product candidate. Moreover, product approvals may be withdrawn for non-compliance with regulatory standards or if problems occur following the initial marketing of the product. Any of the foregoing scenarios could materially harm the commercial success of such product candidate.

We currently have no sales and marketing organization. If we are unable to establish satisfactory sales and marketing capabilities, we may not successfully commercialize any of our product candidates, if regulatory approval is obtained.

At present, we have no sales or marketing personnel. In order to commercialize products that are approved for commercial sales, we must either develop a sales and marketing infrastructure or collaborate with third parties that have such commercial infrastructure. If we elect to develop our own sales and marketing organization, we do not intend to begin to hire sales and marketing personnel until our product candidates are in Phase 3 clinical trials or closer to NDA submission, and we do not intend to establish our own sales organization in the United States until shortly prior to FDA approval of PAX-101 or any of our other product candidates for neurologic indications. For HAT we do not intend to establish a sales organization as we do not intend to sell PAX-101 for HAT in any market.

We may not be able to establish a direct sales force in a cost-effective manner or realize a positive return on this investment. In addition, we will have to compete with established and well-funded pharmaceutical and biotechnology companies to recruit, hire, train and retain sales and marketing personnel. Factors that may inhibit our efforts to commercialize PAX-101 or any of our other product candidates in the United States without strategic partners or licensees include:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to or persuade adequate numbers of physicians to prescribe our future products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we are not successful in recruiting sales and marketing personnel or in building a sales and marketing infrastructure, or if we do not successfully enter into appropriate collaboration arrangements, we will have difficulty successfully commercializing PAX-101 or any other product candidates we may develop or acquire, which would adversely affect our business, operating results and financial condition. Outside the United States, we may commercialize our product candidates by entering into collaboration agreements with pharmaceutical partners. We may not be able to enter into such agreements on terms acceptable to us or at all. In addition, even if we enter into such relationships, we may have limited or no control over the sales, marketing and distribution activities of these third parties. Our future revenues may depend heavily on the success of the efforts of these third parties.

Even if we obtain marketing approval for PAX-101 or any other product candidates that we may develop or acquire, we will be subject to ongoing obligations and continued regulatory review, which may result in significant additional expense. Additionally, our product candidates could be subject to labeling and other restrictions and withdrawal from the market and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our future products.

Even if we obtain United States regulatory approval of PAX-101 or any other product candidates that we may develop or acquire, the FDA may still impose significant restrictions on its indicated uses or marketing or the conditions of approval, or impose ongoing requirements for potentially costly and time-consuming post-approval studies, and post-market surveillance to monitor safety and efficacy. Our future products will also be subject to ongoing regulatory requirements governing the manufacturing, labeling, packaging, storage, distribution, safety surveillance, advertising, promotion, recordkeeping and reporting of adverse events and other post-market information. These requirements include registration with the FDA, as well as continued compliance with current Good Clinical Practices regulations, or cGCPs, for any clinical trials that we conduct post-approval. In addition, manufacturers of drug products and their facilities are subject to continuous review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMPs, requirements relating to quality control, quality assurance and corresponding maintenance of records and documents.

With respect to sales and marketing activities by us or any future partner, advertising and promotional materials must comply with FDA rules in addition to other applicable federal, state and local laws in the United States and similar legal requirements in other countries. In the United States, the distribution of product samples to physicians must comply with the requirements of the U.S. Prescription Drug Marketing Act. Application holders must obtain FDA approval for product and manufacturing changes, depending on the nature of the change. We may also be subject, directly or indirectly through our customers and partners, to various fraud and abuse laws, including, without limitation, the U.S. Anti-Kickback Statute, U.S. False Claims Act, and similar state laws, which impact, among other things, our proposed sales, marketing, and scientific/educational grant programs. If we participate in the U.S. Medicaid Drug Rebate Program, the Federal Supply Schedule of the U.S. Department of Veterans Affairs, or other government drug programs, we will be subject to complex laws and regulations regarding reporting and payment obligations. All of these activities are also potentially subject to U.S. federal and state consumer protection and unfair competition laws. Similar requirements exist in many of these areas in other countries.

In addition, our product labeling, advertising and promotion would be subject to regulatory requirements and continuing regulatory review. The FDA strictly regulates the promotional claims that may be made about prescription products. In particular, a product may not be promoted for uses that are not approved by the FDA as reflected in the product's approved labeling. If we receive marketing approval for our product candidates, physicians may nevertheless legally prescribe our products to their patients in a manner that is inconsistent with the approved label. If we are found to have promoted such off-label uses, we may become subject to significant liability and government fines. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant sanctions, including revocation of its marketing approval. The federal government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees of permanent injunctions under which specified promotional conduct is changed or curtailed.

If we or a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, problems with the facility where the product is manufactured, or we or our manufacturers fail to comply with applicable regulatory requirements or GMP, we may be subject to the following administrative or judicial sanctions:

- restrictions on the marketing or manufacturing of the product, withdrawal of the product from the market, or voluntary or mandatory product recalls;
- issuance of warning letters or untitled letters;
- clinical holds;

- injunctions or the imposition of civil or criminal penalties or monetary fines;
- suspension or withdrawal of regulatory approval;
- suspension of any ongoing clinical trials;
- refusal to approve pending applications or supplements to approved applications filed by us, or suspension or revocation of product license approvals;
- suspension or imposition of restrictions on operations, including costly new manufacturing requirements; or
- product seizure, detention, or refusal to permit the import or export of product.

The occurrence of any event or penalty described above may inhibit our ability to commercialize PAX-101 or any of our other product candidates and generate revenue. Adverse regulatory action, whether pre- or post-approval, can also potentially lead to product liability claims and increase our product liability exposure.

Recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our drug candidates and affect the prices we may obtain.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval for our drug candidates, restrict or regulate post-approval activities and affect our ability to profitably sell our drug candidates. Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We do not know whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our drug candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

In the United States, under the Medicare Modernization Act ("MMA"), Medicare Part D provides coverage to the elderly and disabled for outpatient prescription drugs by approving and subsidizing prescription drug plans offered by private insurers. The MMA also authorizes Medicare Part D prescription drug plans to use formularies where they can limit the number of drugs that will be covered in any therapeutic class. The Part D plans use their formulary leverage to negotiate rebates and other price concessions from drug manufacturers. Also under the MMA, Medicare Part B provides coverage to the elderly and disabled for physician-administered drugs on the basis of the drug's average sales price, a price that is calculated according to regulatory requirements and that the manufacturer reports to Medicare quarterly.

Both Congress and the Centers for Medicare & Medicaid Services ("CMS"), the agency that administers the Medicare program, from time to time consider legislation, regulations, or other initiatives to reduce drug costs under Medicare Parts B and D. For example, under the 2010 Affordable Care Act, drug manufacturers are required to provide a 50% discount on prescriptions for branded drugs filled while the beneficiary is in the Medicare Part D coverage gap, also known as the "donut hole." There have been legislative proposals to repeal the "non-interference" provision of the MMA to allow CMS to leverage the Medicare market share to negotiate larger Part D rebates. Further cost reduction efforts could decrease the coverage and price that we receive for our drug candidates and could seriously harm our business. Private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates, and any reduction in reimbursement under the Medicare program may result in a similar reduction in payments from private payors.

The 2010 Affordable Care Act is intended to broaden access to health insurance and reduce or constrain the growth of healthcare spending. Further, the Affordable Care Act imposes a significant annual fee on companies that manufacture or import branded prescription drug products. It also increased the amount of the rebates drug manufacturers must pay to state Medicaid programs, required that Medicaid rebates be paid on managed Medicaid utilization, and increased the additional rebate on "line extensions" (such as extended release formulations) of solid oral dosage forms of branded products. The law also contains substantial provisions affecting fraud and abuse compliance and transparency, which may require us to modify our business practices with healthcare practitioners, and incur substantial costs to ensure compliance.

While we are unable to predict what legislation, if any, may potentially be enacted, to the extent that future changes affect how our product candidates could be paid for and/or reimbursed by the government and private payers, our business could be adversely affected.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. For example, the Budget Control Act of 2011 included, among other things, provisions that have led to 2% across-the-board reductions in Medicare payment amounts. Several states have adopted or are considering adopting laws that require pharmaceutical companies to provide notice prior to raising prices and to justify price increases. We expect that additional healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, and in turn could significantly reduce the projected value of certain development projects and reduce our profitability.

Our future growth depends, in part, on our ability to penetrate foreign markets, where we would be subject to additional regulatory burdens and other risks and uncertainties.

Our future profitability will depend, in part, on our ability to commercialize our product candidates in foreign markets for which we intend to rely on collaborations with third parties. If we commercialize PAX-101 or any other product candidates that we may develop in foreign markets, we would be subject to additional risks and uncertainties, including:

- our customers' ability to obtain market access and appropriate reimbursement for our product candidates in foreign markets;
- our inability to directly control commercial activities because we are relying on third parties;
- the burden of complying with complex and changing foreign regulatory, tax, accounting and legal requirements;
- different medical practices and customs in foreign countries affecting acceptance in the marketplace;
- import or export licensing requirements;
- longer accounts receivable collection times;
- longer lead times for shipping;
- language barriers for technical training;
- reduced protection of intellectual property rights in some foreign countries;
- foreign currency exchange rate fluctuations; and
- the interpretation of contractual provisions governed by foreign laws in the event of a contract dispute.

Foreign sales of our product candidates could also be adversely affected by the imposition of governmental controls, political and economic instability, trade restrictions and changes in tariffs, any of which may adversely affect our results of operations.

If we market our product candidates in a manner that violates healthcare fraud and abuse laws, or if we violate government price reporting laws, we may be subject to civil or criminal penalties.

The FDA enforces laws and regulations which require that the promotion of pharmaceutical products be consistent with the approved prescribing information. While physicians may prescribe an approved product for a so-called "off label" use, it is unlawful for a pharmaceutical company to promote its products in a manner that is inconsistent with its approved label and any company which engages in such conduct can subject that company to significant liability. Similarly, industry codes in the E.U. and other foreign jurisdictions prohibit companies from engaging in off-label promotion and regulatory agencies in various countries enforce violations of the code with civil penalties. While we intend to ensure that our promotional materials are consistent with our label, regulatory agencies may disagree with our assessment and may issue untitled letters, warning letters or may institute other civil or criminal enforcement proceedings. In addition to FDA restrictions on marketing of pharmaceutical products, several other types of state and federal healthcare fraud and abuse laws have been applied in recent years to restrict certain marketing practices in the pharmaceutical

industry. These laws include the U.S. Anti-Kickback Statute, U.S. False Claims Act and similar state laws. Because of the breadth of these laws and the narrowness of the safe harbors, it is possible that some of our business activities could be subject to challenge under one or more of these laws.

The U.S. Anti-Kickback Statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce, or in return for, purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted broadly to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers on the other. Although there are several statutory exemptions and regulatory safe harbors protecting certain common activities from prosecution, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exemption or safe harbor. Our practices may not, in all cases, meet all the criteria for safe harbor protection from anti-kickback liability. Moreover, recent health care reform legislation has strengthened these laws. For example, the Health Care Reform Law, among other things, amends the intent requirement of the U.S. Anti-Kickback Statute and criminal health care fraud statutes; a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. In addition, the Health Care Reform Law provides that the government may assert that a claim including items or services resulting from a violation of the U.S. Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the U.S. False Claims Act. Federal false claims laws prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government or knowingly making, or causing to be made, a false statement to get a false claim paid.

Over the past few years, several pharmaceutical and other healthcare companies have been prosecuted under these laws for a variety of alleged promotional and marketing activities, such as: allegedly providing free trips, free goods, sham consulting fees and grants and other monetary benefits to prescribers; reporting to pricing services inflated average wholesale prices that were then used by federal programs to set reimbursement rates; engaging in off-label promotion that caused claims to be submitted to Medicare or Medicaid for non-covered, off-label uses; and submitting inflated best price information to the Medicaid Rebate Program to reduce liability for Medicaid rebates. Most states also have statutes or regulations similar to the U.S. Anti-Kickback Statute and the U.S. False Claims Act, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor. Sanctions under these federal and state laws may include substantial civil monetary penalties, exclusion of a manufacturer's products from reimbursement under government programs, substantial criminal fines and imprisonment.

We have been and expect to be significantly dependent on our collaborative agreements for the development of our product candidates, which exposes us to the risk of reliance on the performance of third parties.

In conducting our research and development activities, we currently rely, and expect to continue to rely, on collaborative agreements with third parties such as manufacturers, contract research organizations, commercial partners, universities, governmental agencies and not-for-profit organizations for both strategic and financial resources. The loss of, or failure to perform by us or our partners under any applicable agreements or arrangements, or our failure to secure additional agreements for our product candidates, would substantially disrupt or delay our research and development activities, including our in-process and anticipated clinical trials. Any such loss would likely increase our expenses and materially harm our business, financial condition and results of operation.

We expect that we will rely on third parties to conduct clinical trials for our product candidates. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize PAX-101 or any other product candidates that we may develop or acquire and our business could be substantially harmed.

We expect to enter into agreements with third-party CROs to conduct and manage our clinical programs. We rely heavily on these parties for execution of clinical studies for PAX-101 and our other product candidates and can control only certain and very limited aspects of their activities. Nevertheless, we would be responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards, and our reliance on CROs would not relieve us of our regulatory responsibilities. We and our CROs would be required to comply with cGCPs, which are regulations and guidelines enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area and comparable foreign regulatory authorities for any products in clinical development. The FDA enforces these cGCP regulations through periodic inspections of trial sponsors, principal investigators and trial sites. If we or our CROs fail to comply with applicable cGCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that, upon inspection, the FDA will determine that any of our clinical trials comply with cGCPs. In addition, our clinical trials must be conducted with products produced under cGMP regulations

and will require a large number of test subjects. Our failure or the failure of our CROs to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process and could also subject us to enforcement action up to and including civil and criminal penalties.

As a result, many important aspects of our drug development programs would be outside of our direct control. In addition, the CROs may not perform all of their obligations under their arrangements with us or in compliance with regulatory requirements. If the CROs do not perform clinical trials in a satisfactory manner, breach their obligations to us or fail to comply with regulatory requirements, the development and commercialization of PAX-101 or any other product candidates that we may develop or acquire may be delayed or our development program may be materially and irreversibly harmed. We cannot control the amount and timing of resources these CROs would devote to our program or our product candidates. If we are unable to rely on the clinical data collected by our CROs, we could be required to repeat, extend the duration of, or increase the size of our clinical trials, which could significantly delay commercialization and require significantly greater expenditures. If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs. As a result of the foregoing, our financial results and the commercial prospects for PAX-101 and our other product candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed.

Reimbursement decisions by third-party payors may have an adverse effect on pricing and market acceptance of PAX-101 or any other product candidates that we may develop or acquire. If there is not sufficient reimbursement for our future products, it is less likely that such products will be widely used.

Market acceptance and sales of PAX-101 or any other product candidates for which we obtain regulatory approval will depend on reimbursement policies and may be affected by future healthcare reform measures in both the United States and foreign jurisdictions. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which products they will cover and establish payment levels. In addition, government authorities and these third-party payors are increasingly attempting to contain health care costs by demanding price discounts or rebates and limiting both the types and variety of products that they will cover and the amounts that they will pay for these products. In addition, we might need to conduct post-marketing studies in order to demonstrate the cost-effectiveness of any future products to such payors' satisfaction. Such studies might require us to commit a significant amount of management time and financial and other resources.

Reimbursement rates may vary according to the use of the product and the clinical setting in which it is used, may be based on payments allowed for lower-cost products that are already reimbursed, may be incorporated into existing payments for other products or services, and may reflect budgetary constraints and/or imperfections in Medicare or Medicaid data used to calculate these rates. Net prices for products may be reduced by mandatory discounts or rebates required by government health care programs. Such legislation, or similar regulatory changes or relaxation of laws that restrict imports of products from other countries, could reduce the net price we receive for any future marketed products. As a result, our future products might not ultimately be considered cost-effective.

We cannot be certain that reimbursement will be available for PAX-101 or any other product candidates that we develop or acquire. Also, we cannot be certain that reimbursement policies will not reduce the demand for, or the price paid for, any future products. If reimbursement is not available or is available on a limited basis, we may not be able to successfully commercialize PAX-101 or any other product candidates that we develop or acquire.

Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and these third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that coverage and reimbursement will be available for any product that we commercialize and, if reimbursement is available, what the level of reimbursement will be. Reimbursement may impact the demand for, or the price of, any product for which we obtain marketing approval. Obtaining reimbursement for our products may be particularly difficult because of the higher prices often associated with products administered under the supervision of a physician. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any product candidate that we successfully develop.

There may be significant delays in obtaining reimbursement for approved products, and coverage may be more limited than the purposes for which the product is approved by the FDA or regulatory authorities in other countries. Moreover, eligibility for reimbursement does not imply that any product will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim payments for new products, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Payment rates may vary according to the use of the product and the clinical setting in which it is used, may be based on payments allowed for lower cost products that are already reimbursed and may be incorporated into existing payments for other services. Net prices for products may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of products from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for new products that we develop or acquire could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

While we believe we may be eligible to receive a tropical disease priority review voucher ("PRV") for the use of PAX-101 for the treatment of HAT, there is a risk that we will not receive such PRV, which would require us to find alternative sources of funding for our later stage clinical programs.

We may be eligible to receive a tropical disease PRV for PAX-101, as Human African Trypanosomiasis ("HAT") is defined as a disease qualifying for a tropical disease PRV under Section 524 of the Federal Food, Drug and Cosmetic Act (the "FD&C Act"). The FDA is authorized to award a tropical disease PRV to sponsors of applications for certain products for the prevention or treatment of certain tropical diseases, upon FDA approval of the sponsor's marketing application. A tropical disease PRV may be used by the sponsor that obtains the tropical disease PRV or may be transferred to another sponsor that may use it to obtain Priority Review for a different application. In order to be eligible for a tropical disease PRV, the application must: (i) be for a tropical disease as defined in Section 524 of the FD&C Act; (ii) be submitted under Section 505(b)(1) of the FD&C Act or Section 351 of the Public Health Service Act (the "PHSA"); (iii) be for a product that contains no active ingredient that has been approved in any other application submitted under Section 505(b)(1) of the FD&C Act or Section 351 of the PHSA; and (iv) qualify for Priority Review; (v) contain reports of one or more new clinical investigations (other than bioavailability studies) that are essential to the approval of the application and conducted or sponsored by the sponsor; and (vi) contain the applicant's attestation that such report(s) were not submitted as part of an application for marketing approval or licensure by a regulatory authority in India, Brazil, Thailand, or any country that is a member of the Pharmaceutical Inspection Convention or the Pharmaceutical Inspection Cooperation Scheme prior to September 27, 2007. A U.S. approval in HAT would potentially qualify us to earn a tropical disease PRV from the FDA, which we intend to monetize to raise funds to support the later stage development and commercialization of PAX-101 and PAX-102 in the treatment of ASD, FXAS, FXTAS, ME/CFS and LCS, the cost of which is estimated to be between $120 million and $140 million to gain FDA approval and commercially launch all indications in the United States, depending on the design of required clinical trial protocols. However, there can be no assurance that we will receive approval from the FDA for PAX-101, and even if PAX-101 is approved by the FDA, there is a risk that we will not receive a tropical disease PRV. Further, the PRV program has been subject to criticism, including by the FDA, and it is possible that even if we obtain approval for PAX-101 and qualify for a PRV, the program may no longer be in effect at the time of approval. In addition, although PRVs may be sold or transferred to third parties, there is no guarantee that we will be able to realize any value if we were to sell a PRV. If we are unable to obtain a PRV, or if we are unable to sell our PRV or if the amount we obtain from its sale is insufficient to fund our operations, we may be required to fund the later stage development and commercialization of PAX-101 and PAX-102 in the treatment of ASD, ME/CFS and LCS through sales of our equity or debt securities, through strategic collaborations with third parties or other similar transactions. None of these alternative arrangements may be available to us on commercially reasonable terms, or at all, and if we are unable to raise funding to further our clinical and commercial development, our business and stock price will be adversely impacted.

In addition, we will not be eligible to receive a PRV for PAX-101 for HAT until we submit our NDA for such indication, and we may experience delays in developing and maintaining an uninterrupted supply chain for suramin, which is necessary to support such submission. Without sufficient suramin supply we would not be eligible to receive a PRV and, if granted, monetize such PRV, which could result in the delays or abandonment of any potential development and commercialization of PAX-101 and PAX-102 in the treatment of HAT, ASD, ME/CFS and LCS.

If we do not obtain market exclusivity for our certain of our products, including orphan drug exclusivity, our business may be harmed.

We may seek exclusivity for certain of our product candidates, including PAX-101 and PAX-102. Regulatory authorities in some jurisdictions, including the United States, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan

Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States.

Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of market exclusivity, which precludes the FDA from approving another marketing application for the same drug for the same disease for seven years. Orphan drug exclusivity may be lost if the FDA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. Orphan drug designation must be requested before submitting an application for marketing approval.

A company that first obtains FDA approval for a designated orphan drug for the designated rare disease or condition receives orphan drug market exclusivity for that drug for the designated disease for a period of seven years in the United States. This orphan drug exclusivity prevents the FDA from approving another application to market a drug containing the same active moiety for the same orphan indication, except in very limited circumstances, including when the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care within the meaning of FDA regulations and guidance. In addition, a designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation.

Even though we received orphan drug designation for PAX-101 and may seek orphan drug designation for PAX-102, we may not be the first to obtain marketing approval for the orphan-designated indication due to the uncertainties associated with developing product candidates. If any of these other pharmaceutical companies obtains approval of an NDA before we are able to receive approval for one or more of our drug candidates with the same active moiety for the same indication, we would be barred from marketing that product in the United States during the seven-year orphan drug exclusivity period, unless we could demonstrate that such drug candidate is clinically superior to the approved products or satisfies one of the other limited exceptions to such orphan drug exclusivity.

In addition, even though we received orphan drug designation for PAX-101 and may seek orphan drug designation for PAX-102, that exclusivity may not effectively protect the product from competition because different drugs with different active moieties can be approved for the same condition or a drug with the same active moiety can be approved for a different indication. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process. In addition, even if we seek orphan drug designation for any of our product candidates or indications, we may never receive such designations or obtain orphan drug exclusivity.

Also, overcoming the orphan drug marketing exclusivity is difficult to establish, with limited precedent, and there can be no assurance that the FDA will agree with our position seeking to overcome such marking exclusivity and approve PAX-101 or PAX-102 for U.S. market access with orphan drug exclusivity. If we fail to receive such extensions or exclusive rights, our ability to prevent competitors from manufacturing, marketing and selling competing products will be materially impaired, and our results of operations and financial condition may be significantly adversely affected.

Any failure to comply with applicable data protection and privacy laws and regulations could lead to significant penalties against us, and adversely impact our operating results.

Under the Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, (collectively, "HIPAA"), the U.S. Department of Health and Human Services has issued regulations to protect the privacy and security of protected health information used or disclosed by covered entities including certain healthcare providers, health plans and healthcare clearinghouses. HIPAA also regulates standardization of data content, codes and formats used in healthcare transactions and standardization of identifiers for health plans and providers.

HIPAA and its regulations, including the final omnibus rule published on January 25, 2013, also imposes certain obligations on the business associates of covered entities that obtain protected health information in providing services to or on behalf of covered entities. In addition to federal privacy regulations, there are a number of state laws governing confidentiality and security of health information that are applicable to our business. In addition to possible federal administrative, civil and criminal penalties for HIPAA violations, state attorneys general are authorized to file civil actions for damages or injunctions in federal courts to enforce HIPAA and seek attorney's fees and costs associated with pursuing federal civil actions. Accordingly, state attorneys general have brought civil actions seeking injunctions and damages resulting from alleged violations of HIPAA's privacy and security rules. New laws and regulations governing privacy and security may be adopted in the future as well.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available under such laws, it is possible that some of our current or future business activities, including certain clinical research, sales and marketing practices and the provision of certain items and services to our customers, could be subject to challenge under one or more of such privacy and data security laws. The heightening compliance environment and the need to build and maintain robust and secure systems to comply with different privacy compliance and reporting requirements in multiple jurisdictions could increase the possibility that a healthcare company may fail to comply fully with one or more of these requirements. If our operations are found to be in violation of any of the privacy or data security laws or regulations described above that are applicable to us, or any other laws that apply to us, we may be subject to penalties, including potentially significant criminal, civil and administrative penalties, damages, fines, imprisonment, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and oversight if we become subject to a consent decree or similar agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. To the extent that any product candidates we may develop, once approved, are sold in a foreign country, we may be subject to similar foreign laws.

Risks Relating to Our Intellectual Property Rights

We depend on data licensed to us by third parties, and the loss of access to this data may terminate or delay the further development of our Human African Trypanosomiasis (HAT) NDA filing.

Our business relies on the license of data from the Ministry of Health, Republic of Malawi and Lwala Hospital (Soroti, Uganda). The loss of our key data may seriously impair our business and future viability, and could result in delays in developing, introducing or maintaining our product candidates and formulations until equivalent data, if available, is identified, licensed and integrated. In addition, any defects in the data we license could prevent the implementation or impair the functionality of our product candidates or formulation, delay new product or formulation introductions or injure our reputation.

It is difficult and costly to protect our intellectual property rights, and we cannot ensure the protection of these rights.

Our commercial success will depend, in part, on maintaining and obtaining patent protection for our technologies, products and processes, successfully defending these patents against third-party challenges and successfully enforcing these patents against third-party competitors. The patent positions of pharmaceutical companies can be highly uncertain and involve complex legal, scientific and factual questions for which important legal principles remain unresolved. Changes in either the patent laws or in interpretations of patent laws may diminish the value of our intellectual property. Accordingly, we cannot predict the breadth of claims that may be allowable, or whether any claims will be allowed in our pending applications or, the enforceability of our existing and future patents. As part of our intellectual property strategy, we intend to file U.S. nonprovisional, and foreign national and regional stage applications of this PCT application in due course. We also plan to file further patent applications covering our technology and products. We are not aware of any contested proceedings or third-party claims against our pending PCT international patent applications. We cannot predict the outcome of our patent applications related to suramin and its uses, as our pending patent application and future applications may never be approved by United States or foreign patent offices.

The degree of our current and future protection for our proprietary rights is uncertain, because legal means afford only limited protection and may not adequately protect our rights, permit us to gain or keep our competitive advantage, or provide us with any competitive advantage at all. For example, others have filed, and in the future are likely to file, patent applications covering products and technologies that are similar, identical or competitive to our development programs, or important to our business. Furthermore, because patent applications are generally not published until 18 months from their earlier priority date, there is always a moving window of uncertainty as to whether third parties currently have any pending patent applications of which we would not be aware. We cannot be certain that any patents or patent application owned by a third-party will not have priority over patents and patent applications filed by us, or that we will not be involved in interference, opposition or invalidity proceedings before United States or foreign patent offices. In addition, even if we are successful in protecting our proprietary rights, generic alternatives to our therapeutic products are, and will likely continue to be, available.

We also rely on trade secrets to protect technology, especially in cases when we believe patent protection is not appropriate or obtainable. However, trade secrets are difficult to protect. While we require employees, academic collaborators, consultants and other contractors to enter into confidentiality agreements, we may not be able to adequately protect our trade secrets or other proprietary or licensed information. Typically, research collaborators and scientific advisors have rights to publish data and information in which we may have rights. If we cannot maintain the confidentiality of our proprietary technology and other confidential information, our ability to receive patent protection and our ability to protect valuable information owned by us may be imperiled. Enforcing a claim that a third-

party entity illegally obtained and is using any of our trade secrets is expensive and time consuming, and the outcome is unpredictable. In addition, courts are sometimes less willing to protect trade secrets than patents. Moreover, our competitors may independently develop equivalent knowledge, methods and know-how.

If we fail to maintain or obtain additional patent protection or trade secret protection for PAX-101 or our technologies, third parties could use our proprietary information, which could impair our ability to compete in the market and adversely affect our ability to generate revenues and attain profitability. In addition, third parties' patent or trade secret protection could limit or impact our freedom to operate.

We may also rely on the trademarks we may develop to distinguish our products from the products of our competitors. We cannot guarantee that any trademark applications filed by us or our business partners will be approved. Third parties may also oppose such trademark applications, or otherwise challenge our use of the trademarks. In the event that the trademarks we use are successfully challenged, we could be forced to rebrand our products, which could result in loss of brand recognition, and could require us to devote resources to modifying advertising and marketing for our brands. Further, we cannot provide assurance that competitors will not infringe the trademarks we use, or that we will have adequate resources to enforce these trademarks.

PAX-101 and our other product candidates may infringe the intellectual property rights of others, which could increase our costs and delay or prevent our development and commercialization efforts.

Our success depends in part on avoiding infringement of the proprietary technologies of others. We respect the valid patent rights of third parties of which we are aware. The pharmaceutical industry has been characterized by frequent litigation regarding patent and other intellectual property rights. Identification of third-party patent rights that may be relevant to our proprietary technology is difficult because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. Additionally, because patent applications are maintained in secrecy until the application is published, we may be unaware of third-party patents that may be infringed by commercialization of PAX-101 or any of our other product candidates. There may be certain issued patents and patent applications claiming subject matter that we may be required to license in order to research, develop or commercialize PAX-101 or our other product candidates, and we do not know if such patents and patent applications would be available to license on commercially reasonable terms, or at all. Any claims of patent infringement asserted by third parties would be time-consuming and may:

- result in costly litigation;
- divert the time and attention of our technical personnel and management;
- prevent us from commercializing a product until the asserted patent expires or is held finally invalid or not infringed in a court of law;
- require us to cease or modify our use of the technology and/or develop non-infringing technology; or
- require us to enter into royalty or licensing agreements.

Furthermore, we cannot guarantee that we would be successful in defending against these claims of patent infringement. For example, if we were required to modify our use of the technology or develop an alternative non-infringing technology, we cannot be certain that we would be successful in making the modifications or developing the technology and whether it would be economically feasible or practical to do so. Also, it may not be possible to obtain royalty or licensing agreements on favorable terms or to obtain such agreements at all.

Although no third-party has asserted a claim of infringement against us, others may hold proprietary rights that could prevent our product candidates from being marketed. Any patent-related legal action against us claiming damages and seeking to enjoin commercial activities relating to our product candidates or our processes could subject us to potential liability for damages and require us to obtain a license to continue to manufacture or market PAX-101 or any other product candidates. We cannot predict whether we would prevail in any such actions or that any license required under any of these patents would be made available on commercially acceptable terms, if at all. In addition, we cannot be sure that we could redesign PAX-101 or any other product candidates or processes to avoid infringement, if necessary.

We are aware of PCT international patent application PCT/US2018/017674, titled "Methods for Autism Spectrum Disorder Pharmacotherapy", which lists Perfect Daylight Limited and The Regents of the University of California as Applicants, filed on February 9, 2018, published as WO 2018/148580 on August 16, 2018, and claiming priority to U.S. provisional patent application no. 62/457,120, filed on February 9, 2017. The patent application describes compositions of antipurinergic agents, such as suramin, and methods of use for treating cognitive developmental disorders and autism spectrum disorder. From publicly available databases, we are aware that a U.S. nonprovisional patent application of this PCT patent application, U.S. application Serial No. 16/537,397, was filed in the United States and was subsequently abandoned in favor of U.S. application Serial No. 18/323,375, filed Mary 24, 2023 and U.S. application Serial No. 18/414,171, filed January 16, 2024. The European equivalent of the application was granted as EP3579836 on December 15, 2021 and was validated in Belgium, France, Germany, Great Britain, and Switzerland. A Chinese application, CN201880024535.9, is also pending. Because patent applications of PCT/US2018/017674 are still pending at least in the United States and China, it is not certain if any patents will ultimately issue from these applications nor is it possible to predict the resultant claim scope of any such issued patent. We will continue to monitor the prosecution of these patent applications from publicly available documents.

We are also aware of PCT international patent application PCT/US2018/017200, titled "Antipurinergic Compounds and Uses thereof," which lists CSP Pharma, Inc. as Applicant, filed on February 7, 2018, published as WO 2018/148262 on August 16, 2018, and claiming priority to U.S. provisional patent application no. 62/456,438, filed on February 8, 2017. The patent application describes compositions and methods for treating neurodevelopmental disorders. The compositions contain an APT, such as suramin, and a carrier formulated for non-intravenous administration. The neurodevelopmental disorders include ASD. From publicly available databases, we are aware that a national phase application of this PCT patent application, U.S. application Serial No. 16/484,284 was filed in the United States. However, the US Patent Office issued a Notice of Abandonment on August 12, 2021 for applicant's failure to respond to the office action of January 14, 2021. No further child applications are listed as pending.

We are also aware of PCT international patent application PCT/US2017/041932, titled "Diagnostic and Methods of Treatment for Chronic Fatigue Syndrome and Autism Spectrum Disorders," which lists The Regents of the University of California as Applicant, filed on July 13, 2017, published as WO 2018/013811 on January 18, 2018, and claiming priority to U.S. provisional patent application nos. 62/464,369, filed on February 27, 2017 and 62/362,564, filed on July 14, 2016. The patent application describes biomarkers for diagnosing and predicting the development of chronic fatigue syndrome and methods of treating a mitochondrial disease or disorder, such as ASD, by administering an effective amount of an APT, such as suramin. Publicly available databases show no pending US or national or regional phase patent applications.

As discussed above, our success depends in part on avoiding infringement of the proprietary technologies of others. We are aware of the risks associated with the valid patent rights of third parties, however, identification of these third party proprietary technologies and patent rights is difficult because patent searching is imperfect. Also, because patent applications are maintained in secrecy until publication, we may be unaware of third-party patents that may be infringed by commercialization of PAX-101 or any of our other product candidates. Based on as yet unforeseen activities associated with the intellectual property of such third parties we may have to make modifications to our development plans, the filing of new patent applications and the prosecution of our patent portfolio, and our business.

A number of companies, including several major pharmaceutical companies, have conducted research on APTs and their effect on purinergic receptors and potential therapies which resulted in the filing of many patent applications related to this research. If we were to challenge the validity of these or any issued United States patent in court, we would need to overcome a statutory presumption of validity that attaches to every issued United States patent. This means that, in order to prevail, we would have to present clear and convincing evidence as to the invalidity of the patent's claims.

If we were to challenge the validity of these or any issued United States patent in an administrative trial before the Patent Trial and Appeal Board in the United States Patent and Trademark Office, we would have to prove that the claims are unpatentable by a preponderance of the evidence. Moreover, even if we were successful in such an invalidity challenge, the decision could be appealed by the other party to the Court of Appeals for the Federal Circuit, which could involve significant resources to litigate and we cannot predict whether we would prevail on appeal**.** There is no assurance that a jury and/or court would find in our favor on questions of infringement, validity or enforceability.

Accordingly, if patents exist or are in the future granted that conflict with our patents, and if we face an adverse determination in a judicial or administrative proceeding, or if we are unable to obtain necessary licenses, we could be prevented from developing and commercializing PAX-101 or another product candidate, which in turn would harm the viability of our company and our business, prospects, financial condition and operating results.

We may be subject to claims that we have wrongfully hired an employee from a competitor or that we or our employees have wrongfully used or disclosed alleged confidential information or trade secrets of their former employers.

As is commonplace in our industry, we employ individuals who were previously employed at other pharmaceutical companies, including our competitors or potential competitors. Although no claims against us are currently pending, we may be subject in the future to claims that our employees or prospective employees are subject to a continuing obligation to their former employers (such as non-competition or non- solicitation obligations) or claims that our employees or we have inadvertently or otherwise used or disclosed trade secrets or other proprietary information of their former employers. Litigation may be necessary to defend against these claims. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to management.

We may be subject to claims challenging the inventorship of our patents and other intellectual property.

Although we are not aware of any asserted third-party claims challenging inventorship of our patents or ownership of our intellectual property, we may in the future be subject to claims that former employees, strategic partners, commercial counterparties or other third parties associated with us or one of our predecessors in ownership of PAX-101 have an interest in our patents or other intellectual property as an inventor or co-inventor. While it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we cannot fully control the enforcement of these policies by third parties with which we contract, nor can we be certain that assignment agreements between us and our employees, between us and our counterparties, or between our counterparties and their employees or between our predecessors of ownership and their employees and counterparties, will effectively protect our interests as to any party who conceives or develops intellectual property that we regard as our own. Among other issues, the assignment of intellectual property rights may not be self-executing, the assignment agreements may be breached, or we may have disputes arise from conflicting obligations of consultants or others who are involved in developing our product candidates. As we approach potential commercialization of our product candidates, we will need to more closely analyze the facts that we believe might be used to assert an inventorship claim against us. Determinations like these involve complex sets of facts and the application of sometimes-unsettled patent law, resulting in inherent uncertainties regarding ownership rights.

If claims challenging inventorship are made against us, we may need to resort to litigation to resolve those claims. If we fail in defending against any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of valuable intellectual property rights or the right to assert those rights against third-parties marketing competing products. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

There are risks to our intellectual property based on our international business operations.

We may face risks to our technology and intellectual property as a result of our conducting business outside of the United States, including as a result of our license of clinical data from the Ministry of Health, Republic of Malawi and Lwala Hospital (Soroti, Uganda), as certain jurisdictions may not have comparable levels of protection of corporate proprietary information and assets such as intellectual property, trademarks, trade secrets, know-how and customer information and records. While these risks are common to many companies, conducting business in certain foreign jurisdictions, housing technology, data and intellectual property abroad, or licensing technology to or from foreign partners may have more significant exposure.

Risks Related to Our Common Stock

We have received deficiency letters from Nasdaq relating to non-compliance with Nasdaq's continued listing requirements. Our common stock could become subject to delisting from Nasdaq if we fail to regain compliance.

We were notified by the Nasdaq in December 2022 that we were not in compliance with the Minimum Market Value Requirement requiring us to maintain a market value of listed securities of a minimum of $35.0 million for a period of 30 consecutive business days, and Nasdaq granted us a period of 180 calendar days, or until June 5, 2023, to regain compliance with the Minimum Market Value Requirement. In June 2023, we received notice from the Nasdaq Staff stating that we have not regained compliance with the Minimum Market Value Requirement during the 180-day grace period and would be subject to delisting. After a hearing to appeal the Staff's delisting determination, we were granted an exception to maintain our listing on The Nasdaq Capital Market notwithstanding our failure to regain compliance with the Minimum Market Value Requirement. We were required to demonstrate compliance with the alternative criteria set forth in Nasdaq Listing Rule 5550(b)(1), which requires us to maintain stockholders' equity of at least $2.5 million by December 11, 2023. On December 20, 2023, Nasdaq notified us

that we had demonstrated compliance with this criteria and imposed a Discretionary Panel Monitor until December 20, 2024. The Discretional Panel Monitor provides that if we fail to maintain compliance with any continued listing requirement, Nasdaq will issue a Delist Determination Letter and schedule a new hearing with the Nasdaq Hearings Panel, and we would not be entitled to any grace periods for subsequent noncompliance with listing standards. We expect that we will need to raise additional capital on a regular basis to maintain compliance with the stockholders' equity standard, and such financing may be significantly dilutive to our existing shareholders, may not be available on favorable terms or at all and we may not be able to maintain our compliance with this standard in the future.

On January 12, 2024, we received a notification letter from Nasdaq advising us that for 31 consecutive trading days preceding January 11, 2024, the bid price of our common stock had closed below the $1.00 per share minimum required for continued listing on Nasdaq. As a result of the Nasdaq panel imposing the Panel Monitor on the Company until December 20, 2024, we are not eligible for a grace period.

We requested a hearing before the Panel which is expected to take place April 2024. This request stays any delisting action in connection with the notice and our common stock continues to be listed on Nasdaq until the Panel renders a decision subsequent to the hearing. At the hearing, we intend to present a plan to regain compliance with the Minimum Bid Price Requirement and may request that the Panel allow us additional time within which to regain compliance. While we expect to submit a comprehensive plan to regain compliance and a request for 180 days in which to implement the plan, there can be no assurance that the Panel will grant our request for the additional time requested.

If we are not granted the request or if we fail to satisfy any other continued listing requirements of Nasdaq, Nasdaq may take steps to delist our common stock. Such a delisting would likely have a negative effect on the price of our common stock and would impair your ability to sell or purchase our common stock when you wish to do so. In the event of a delisting, we can provide no assurance that any action taken by us to restore compliance with listing requirements would allow our common stock to become listed again, stabilize the market price or improve the liquidity of our common stock, prevent our common stock from dropping below the Nasdaq minimum bid price requirement or prevent future non-compliance with Nasdaq's listing requirements.

The prices of our shares may be volatile, which could subject us to securities class action litigation and prevent you from being able to sell your shares at or above the price at which you purchased them.

This price at which you purchased your shares may vary from the market price of our common stock thereafter. You may be unable to sell your shares of common stock at or above the price at which you purchased them. The market price for our common stock may be volatile and subject to wide fluctuations in response to factors including the following:

- actual or anticipated fluctuations in our quarterly or annual operating results;
- actual or anticipated changes in the pace of our corporate achievements or our growth rate relative to our competitors;
- failure to meet or exceed financial estimates and projections of the investment community or that we provide to the public;
- issuance of new or updated research or reports by securities analysts;
- share price and volume fluctuations attributable to inconsistent trading volume levels of our shares;
- additions or departures of key management or other personnel;
- disputes or other developments related to proprietary rights, including patents, litigation matters, and our ability to obtain patent protection for our technologies;
- announcement or expectation of additional debt or equity financing efforts;
- sales of our common stock by us, our insiders or our other stockholders;

- general economic, market or political conditions in the United States or elsewhere (including, without limitation, conditions arising out the COVID-19 pandemic). In particular, the market prices of early clinical-stage companies like ours have been highly volatile due to factors, including, but not limited to any delay or failure in a clinical trial for our product candidates or receive approval from the FDA and other regulatory agents;

- developments or disputes concerning our product's intellectual property rights;

- our or our competitors' technological innovations;

- fluctuations in the valuation of companies perceived by investors to be comparable to us;

- announcements by us or our competitors of significant contracts, acquisitions, strategic partnerships, joint ventures, capital commitments, new technologies or patents;

- failure to complete significant transactions or collaborate with vendors in manufacturing our product; and

- proposals for legislation that would place restrictions on the price of medical therapies.

In addition, trading in our common stock may be subject to abuse, volatility and shorting, which may have little to do with our operations or business prospects.

If equity research analysts do not publish research or reports about our business or if they issue unfavorable commentary or downgrade our common stock, the price of our common stock could decline.

The trading market for our common stock relies in part on the research and reports that equity research analysts publish about us and our business. We do not control these analysts. The price of our common stock could decline if one or more equity analysts downgrade our common stock or if analysts issue other unfavorable commentary or cease publishing reports about us or our business.

We are an "emerging growth company," and will be able take advantage of reduced disclosure requirements applicable to "emerging growth companies," which could make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the JOBS Act and, for as long as we continue to be an "emerging growth company," we intend to take advantage of certain exemptions from various reporting requirements applicable to other public companies but not to "emerging growth companies," including, but not limited to, not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an "emerging growth company" for up to five years, or until the earliest of (i) the last day of the first fiscal year in which our annual gross revenues exceed $1.235 billion, (ii) the date that we become a "large accelerated filer" as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended, which would occur if the market value of our common stock that is held by non-affiliates exceeds $700 million as of the last business day of our most recently completed second fiscal quarter, or (iii) the date on which we have issued more than $1 billion in non-convertible debt during the preceding three year period.

We intend to take advantage of these reporting exemptions described above until we are no longer an "emerging growth company." Under the JOBS Act, "emerging growth companies" can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have elected to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will not be subject to the same new or revised accounting standards as public companies that are not emerging growth companies. As a result of this election, our financial statements may not be comparable to those of companies that are not emerging growth companies.

We cannot predict if investors will find our common stock less attractive if we choose to rely on these exemptions. If some investors find our common stock less attractive as a result of any choices to reduce future disclosure, there may be a less active trading market for our common stock and the price of our common stock may be more volatile.

We incur significant costs and devote substantial management time as a result of operating as a public company, which we expect to increase after we are no longer an "emerging growth company.

As a public company, we incur significant legal, accounting and other expenses that we did not incur as a private company. For example, we are required to comply with certain of the requirements of the Sarbanes-Oxley Act and the Dodd-Frank Wall Street Reform and Consumer Protection Act, as amended, as well as rules and regulations subsequently implemented by the SEC, including the establishment and maintenance of effective disclosure and financial controls and changes in corporate governance practices.

Compliance with these requirements increases our legal and financial compliance costs and makes some activities more time consuming and costly. In addition, our management and other personnel need to divert attention from operational and other business matters to devote substantial time to these public company requirements. In particular, we expect to incur significant expenses and devote substantial management effort toward ensuring compliance with the requirements of Section 404 of the Sarbanes-Oxley Act. In addition, after we no longer qualify as an "emerging growth company," as defined under the JOBS ACT we expect to incur additional management time and cost to comply with the more stringent reporting requirements applicable to companies that are deemed accelerated filers or large accelerated filers, including complying with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act. We are just beginning the process of compiling the system and processing documentation needed to comply with such requirements. We may not be able to complete our evaluation, testing and any required remediation in a timely fashion. In that regard, we currently do not have an internal audit function, and we will need to hire or contract for additional accounting and financial staff with appropriate public company experience and technical accounting knowledge.

We cannot predict or estimate the amount of additional costs we may incur as a result of being a public company or the timing of such costs.

There may be limitations on the effectiveness of our internal controls, and a failure of our control systems to prevent error or fraud may materially harm our company.

Proper systems of internal controls over financial accounting and disclosure controls and procedures are critical to the operation of a public company. We are also at the early stages of establishing, and we may be unable to effectively establish such systems. This would leave us without the ability to reliably assimilate and compile financial information about our company and significantly impair our ability to prevent error and detect fraud, all of which would have a negative impact on our company from many perspectives.

Moreover, we do not expect that disclosure controls or internal control over financial reporting, even if established, will prevent all error and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Further, the design of a control system must reflect the fact that there are resource constraints and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, have been detected. Failure of our control systems to prevent error or fraud could materially adversely impact us.

We have identified material weaknesses in our internal control and if our remediation of such material weaknesses is not effective, or if we fail to develop and maintain an effective system of disclosure controls and internal control, our ability to produce timely and accurate financial statements or comply with applicable laws and regulations could be impaired.

In the course of preparing our financial statements for the years ended December 31, 2020 and December 31, 2023, we identified material weaknesses in our internal control relating to the evaluation of complex financial instruments, including earnings per share. A material weakness is a deficiency, or combination of deficiencies, in internal control, such that there is a reasonable possibility that a material misstatement of the annual or interim financial statements will not be prevented or detected on a timely basis.

The material weaknesses have not been remediated as of December 31, 2023. Our management has concluded that our control around the accounting for certain complex instruments issued by the Company and earnings per share was not effectively designed or maintained, and therefore not accounted for correctly.

We originally prepared an accounting position paper concluding that our Series Seed Preferred Stock should have been classified as mezzanine equity in accordance with ASC 480. Upon further analysis, it was determined that the Series Seed Preferred Stock should have been recorded as permanent equity because certain redemption provisions are within the Company's control. Therefore, management has concluded that our controls around the interpretation and accounting for our Series Seed Preferred Stock issued was not effectively designed or maintained. Additionally, the original earnings per share calculation did not correctly classify the shares associated with our SAFE investment and our Series Seed Preferred Stock as a separate class of participating securities. Upon further analysis we determined the controls over the calculation of earnings per share resulted in a material weakness.

To remediate the above material weaknesses, we have developed a remediation plan with assistance from our accounting advisors and have dedicated significant resources and efforts to the remediation and improvement of our internal control. While we have processes to identify and appropriately apply applicable accounting requirements, we plan to enhance our system of evaluating and implementing the complex accounting standards that apply to our financial statements. Our plans at this time include providing enhanced access to accounting literature, research materials and documents, and increased communication among our personnel and third-party professionals with whom we consult regarding complex accounting applications. The elements of our remediation plan can only be accomplished over time, and we can offer no assurance that these initiatives will ultimately have the intended effects. We do not believe that the remediation of these material weaknesses will result in significant incremental cost. However, another significant financial reporting failure or material weakness in internal control could result in substantial cost to remediate and could cause a loss of investor confidence and decline in the market price of our stock.

In addition to the material weaknesses identified above, we also did not design and maintain effective controls over information technology (IT) general controls for information systems that are relevant to the preparation of our financial statements, specifically, with respect to user provisioning and deprovisioning, user access review, passwords, privileged access, cybersecurity, system development lifecycle, and SOC report management review. These IT deficiencies did not result in a misstatement to our financial statements, however, the deficiencies, when aggregated, could impact the effectiveness of IT-dependent controls that could result in misstatements potentially impacting all financial statement accounts and disclosures that would not be prevented or detected. Accordingly, management has determined that these deficiencies in the aggregate constitute a material deficiency. To remediate the above material weaknesses, we have developed a remediation plan with assistance from our IT advisors and have dedicated significant resources and efforts to the remediation and improvement of our internal control.

We cannot assure you, however, that any actions we may take in the future will be sufficient to remediate the control deficiencies that led to our material weaknesses in our internal control or that they will prevent or avoid potential future material weaknesses. Our current controls and any new controls that we develop may become inadequate because of changes in conditions in our business. Further, weaknesses in our disclosure controls and internal control may be discovered in the future. Any failure to develop or maintain effective controls or any difficulties encountered in their implementation or improvement could harm our operating results or cause us to fail to meet our reporting obligations and may result in a restatement of our financial statements for prior periods.

Our independent registered public accounting firm is not required to formally attest to the effectiveness of our internal control until after we are no longer an "emerging growth company" as defined in the JOBS Act. At such time, our independent registered public accounting firm may issue a report that is adverse in the event it is not satisfied with the level at which our internal control is documented, designed, or operating. Any failure to implement and maintain effective internal control also could adversely affect the results of periodic management evaluations and annual independent registered public accounting firm attestation reports regarding the effectiveness of our internal control that we will eventually be required to include in our periodic reports that are filed with the Commission. Ineffective disclosure controls and procedures and internal control could also cause investors to lose confidence in our reported financial and other information, which would likely have a negative effect on the trading price of our common stock. In addition, if we are unable to continue to meet these requirements, we may not be able to remain listed on the Nasdaq Capital Market.

We have never declared or paid dividends on our common stock, and we do not currently intend to pay dividends on our common stock in the foreseeable future, and consequently, your ability to achieve a return on your investment will depend on appreciation in the price of our common stock.

We have never declared or paid cash dividends on our common stock and do not anticipate paying any cash dividends to holders of our common stock in the foreseeable future. Consequently, investors must rely on sales of their common stock after price appreciation,

which may never occur, as the only way to realize any future gains on their investments. There is no guarantee that shares of our common stock will appreciate in value or even maintain the price at which our stockholders have purchased their shares.

Our operations expose us to litigation, tax, environmental and other legal compliance risks.

We are subject to a variety of litigation, tax, environmental, health and safety and other legal compliance risks. These risks include, among other things, possible liability relating to product liability matters, intellectual property rights, contract-related claims, taxes, health and safety liabilities, environmental matters and compliance with U.S. and foreign laws, competition laws and laws governing improper business practices. We may be subject to claims from vendors, licensees, licensors and securityholders (including our current securityholders), including with respect to alleged breaches of agreements, material misstatements in our public filings and other reasons. We could be charged with wrongdoing as a result of such matters. We have not received any notice of any such claims and believe such claims would be without merit and would vigorously defend ourselves, however the risk of such claims is uncertain and there can be no assurance that our Company will not be liable for damages, the amount of which cannot be predicted. Further, in connection with any such claims, a court may grant other remedies that will have a material adverse effect on our Company's financial condition or results of operations, or that will result in changes to our liquidity or capitalization. Changes in laws or regulations could result in higher expenses and payments, and uncertainty relating to laws or regulations may also affect how we conduct our operations and structure our investments and could limit our ability to enforce our rights.

Anti-takeover provisions contained in our Certificate of Incorporation and our Bylaws, as well as provisions of Delaware law, could impair a takeover attempt.

Our Certificate of Incorporation, Bylaws and Delaware law contain provisions which could have the effect of rendering more difficult, delaying or preventing an acquisition deemed undesirable by our board of directors. These provisions include:

- classifying our board of directors into three classes;
- authorizing "blank check" preferred stock, which could be issued by our board of directors without stockholder approval and may contain voting, liquidation, dividend, and other rights superior to our common stock;
- limiting the liability of, and providing indemnification to, our directors and officers;
- limiting the ability of our stockholders to call and bring business before special meetings;
- requiring advance notice of stockholder proposals for business to be conducted at meetings of our stockholders and for nominations of candidates for election to our board of directors;
- controlling the procedures for the conduct and scheduling of board of directors and stockholder meetings; and
- providing our board of directors with the express power to postpone previously scheduled annual meetings and to cancel previously scheduled special meetings.

These provisions, alone or together, could delay or prevent hostile takeovers and changes in control or changes in our management.

As a Delaware corporation, we are also subject to provisions of Delaware law, including Section 203 of the Delaware General Corporation law, which prevents some stockholders holding more than 15% of our outstanding common stock from engaging in certain business combinations without approval of the holders of substantially all of our outstanding common stock.

Any provision of our Certificate of Incorporation, Bylaws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

Our Certificate of Incorporation designates the Court of Chancery of the State of Delaware as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or other employees.

Our Certificate requires that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will, to the fullest extent permitted by law, be the sole and exclusive forum for each of the following:

- any derivative action or proceeding brought on our behalf;
- any action asserting a claim for breach of any fiduciary duty owed by any director, officer or other employee of ours to the Company or our stockholders, creditors or other constituents;
- any action asserting a claim against us or any director or officer of ours arising pursuant to, or a claim against us or any of our directors or officers, with respect to the interpretation or application of any provision of, the DGCL, our Certificate of Incorporation or Bylaws; or
- any action asserting a claim governed by the internal affairs doctrine; provided, that, if and only if the Court of Chancery of the State of Delaware dismisses any of the foregoing actions for lack of subject matter jurisdiction, any such action or actions may be brought in another state court sitting in the State of Delaware

The exclusive forum provision is limited to the extent permitted by law, and it will not apply to claims arising under the Exchange Act or for any other federal securities laws which provide for exclusive federal jurisdiction, though it may apply to other state and federal law claims including actions arising under the Securities Act (although our stockholders will not be deemed to have waived our compliance with the federal securities laws and the rules and regulations thereunder).

However, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our Certificate of Incorporation provides that the Court of Chancery for the State of Delaware will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act. While the Delaware courts have determined that such choice of forum provisions are facially valid, there is uncertainty as to whether a court would enforce such a forum selection provision as written in connection with claims arising under the Securities Act. In addition, a stockholder may nevertheless seek to bring such a claim arising under the Securities Act against us, our directors, officers, or other employees in a venue other than the Court of Chancery for the State of Delaware. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our Certificate of Incorporation.

Although we believe this provision benefits us by providing increased consistency in the application of Delaware law in the types of lawsuits to which it applies, this provision may limit or discourage a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage such lawsuits against us and our directors, officers and other employees. Alternatively, if a court were to find the choice of forum provision contained in our Certificate of Incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could adversely affect our business and financial condition.

We note that there is uncertainty as to whether a court would enforce the provision and that investors cannot waive compliance with the federal securities laws and the rules and regulations thereunder. Although we believe this provision benefits us by providing increased consistency in the application of Delaware law in the types of lawsuits to which it applies, the provision may have the effect of discouraging lawsuits against our directors and officers.

General Company-Related Risks

Since certain of our directors and officers are employed by and/or consult for other companies, their other activities could compete for time on, or create conflicts of interest with, our activities.

Certain of our officers are not required to work exclusively for us. For example, David W. Hough, our Chief Medical Officer is the Chief Medical Officer of Freedom Biosciences and Stephen D. Sheldon, our Chief Financial Officer and Chief Operating Officer, is the Chief Executive Officer of Indochina Healthcare Co. Ltd. Pursuant to the agreements we have entered into with

these individuals, Messr. Hough is obligated to devote only 20 hours per week to activities related to our Company. Therefore, it is possible that a conflict of interest with regard to an officer's time may arise based on their other employment and/or business operations. As we progress, if the full-time services of these individuals are required and the current directors and officers cannot provide that level of commitment, we will need to identify suitable individuals who can dedicate such time to our Company. We can provide no assurance that we will be able to successfully identify and retain qualified candidates for these positions.

We will need to grow the size of our organization, and we may experience difficulties in managing this growth.

We have six full-time employees and one part-time employee. As our development and commercialization plans and strategies develop, we will need to expand the size of our employee base for managerial, operational, sales, marketing, financial and other resources. Future growth would impose significant added responsibilities on members of management, including the need to identify, recruit, maintain, motivate and integrate additional employees. In addition, our management may have to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. Our future financial performance and our ability to commercialize our drug candidates and our ability to compete effectively will depend, in part, on our ability to effectively manage our future growth.

If we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy. In addition, the loss of the services of certain key employees, including Howard J. Weisman, our Chief Executive Officer and Chairman of the Board, would adversely impact our business prospects.

Our ability to compete in the highly competitive pharmaceuticals industry depends in large part upon our ability to attract highly qualified managerial, scientific and medical personnel. In order to induce valuable employees to remain with us, we intend to provide employees with stock-based compensation that vests over time. The value to employees of stock-based compensation that vests over time will be significantly affected by movements in the price of our common stock that we will not be able to control and may at any time be insufficient to counteract more lucrative offers from other companies.

Our management team has expertise in many different aspects of drug development and commercialization. However, we will need to hire additional personnel as we further develop our drug candidates. Competition for skilled personnel in our market is intense and competition for experienced scientists may limit our ability to hire and retain highly qualified personnel on acceptable terms. Despite our efforts to retain valuable employees, members of our management, scientific and medical teams may terminate their employment with us on short notice. We have entered into employment agreements with our executive officers. However, these employment arrangements provide for at-will employment, which means that any of our employees could leave our employment at any time, with or without notice. The loss of the services of any of our executive officers or other key employees could potentially harm our business, operating results or financial condition. In particular, we believe that the loss of the services of Howard J. Weisman, our Chief Executive Officer, would have a material adverse effect on our business. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level, and senior managers as well as junior, mid-level, and senior scientific and medical personnel.

Other pharmaceutical companies with which we compete for qualified personnel have greater financial and other resources, different risk profiles, and a longer history in the industry than we do. They also may provide more diverse opportunities and better chances for career advancement. Some of these characteristics may be more appealing to high-quality candidates than what we have to offer. If we are unable to continue to attract and retain high-quality personnel, the rate and success at which we can develop and commercialize product candidates would be limited.

We face competition from other biotechnology and pharmaceutical companies and our operating results will suffer if we fail to compete effectively.

The biotechnology and pharmaceutical industries are intensely competitive and subject to rapid and significant technological change. We have competitors in a number of jurisdictions, many of which have substantially greater name recognition, commercial infrastructures and financial, technical and personnel resources than we have. Established competitors may invest heavily to quickly discover and develop novel compounds that could make PAX-101 or any other product candidates we may develop or acquire obsolete or uneconomical. Any new product that competes with an approved product may need to demonstrate compelling advantages in efficacy, cost, convenience, tolerability and safety to be commercially successful.

Other competitive factors, including generic competition, could force us to lower prices or could result in reduced sales. In addition, new products developed by others could emerge as competitors to PAX-101 or any of our other product candidates. If we are not able

to compete effectively against our current and future competitors, our business will not grow and our financial condition and operations will suffer.

We face competition from many different sources, including commercial pharmaceutical and biotechnology enterprises, academic institutions, government agencies and private and public research institutions. We face competition with respect to our current product candidates and we will face competition with respect to any product candidates that we may seek to develop or commercialize in the future. Our current and potential competitors in ASD include CureMark LLC, which is in Phase 3 studies for CM-AT for ASD, Yamo Pharmaceuticals, which is in Phase 2 studies for LI-79 for ASD, GW Pharmaceuticals, which is in Phase 2 studies for Cannabidivarin for ASD, Harmony Biosciences, which is in Phase 3 studies for Cannabidiol ("CBD") gel for ASD, QBioMed, which is developing a preclinical asset called QBM-001 for rare pediatric nonverbal autism and Kuzani Therapeutics, Inc., which has announced that it is in clinical development for the treatment of the core symptoms of ASD in children. There are two treatments that have been approved by FDA to treat the non-core symptom of irritability in ASD: Risperdal® (Risperidone) and Abilify® (Aripiprazole). Axial Therapeutics is in Phase 2 studies for AB-2004 for irritability in ASD. For ME/CFS, AIM ImmunoTech has an approval for rintatolimod in Argentina, and is in development for the drug in the US. For LCS, Tonix Pharmaceuticals is in Phase 2 studies for TNX-102 SL.

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our drug candidates.

We face a potential risk of product liability as a result of the clinical testing of our drug candidates and will face an even greater risk if we commercialize our drug candidates. For example, we may be sued if any product we develop or acquire or any materials that we use in our products allegedly causes injury or is found to be otherwise unsuitable during product testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability and a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our drug candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our drug candidates;
- injury to our reputation;
- withdrawal of clinical trial participants;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- the inability to commercialize our drug candidates; and
- a decline in the value of our stock.

Our inability to obtain and retain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop or acquire. We intend to obtain product liability insurance covering our clinical trials. Although we will maintain such insurance, any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. Our insurance policies also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts.

We may acquire businesses, assets or products, or form strategic alliances, in the future, and we may not realize the benefits of such acquisitions.

We may acquire additional businesses, assets or products, form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable to successfully integrate them with our existing operations and company culture. We may encounter numerous difficulties in developing, manufacturing and marketing any new businesses, assets or products we may acquire, and any delay in their integration may delay or prevent us from realizing their expected benefits or enhancing our business. We cannot assure you that, following any such acquisition, we will achieve the expected synergies to justify the transaction.

We rely significantly on information technology and any failure, inadequacy, interruption or security lapse of that technology, including any cybersecurity incidents, could harm our ability to operate our business effectively.

Despite the implementation of security measures, our internal computer systems and those of third parties with which we contract are vulnerable to damage from cyber-attacks, computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced a cyber-attack to date, there can be no assurance that we will not experience cyber-attacks in the future, suffer indirect consequences from cyber-attack on a third-party, or fail to anticipate, identify or offset such threats of potential cyber-attacks or security breaches in a timely manner. This is especially so considering the nature of cyber-attack techniques, which change frequently, can be difficult to detect for extended periods of time and often are not recognized until they succeed. System failures, accidents or security breaches could cause interruptions in our operations and could result in a material disruption of our product development and clinical activities and business operations, in addition to possibly requiring substantial expenditures of resources to remedy. The loss of product development or clinical trial data could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and our development programs and the development of our product candidates could be delayed.

Unstable market and economic conditions may have serious adverse consequences on our business and financial condition.

Our business, financial condition and results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. A severe or prolonged economic downturn could result in a variety of risks to our business, including our ability to raise additional capital when needed on acceptable terms, if at all. As widely reported, global credit and financial markets have experienced volatility and disruptions in the past several years and especially in 2020, 2021 and 2022 due to the impacts of the COVID-19 pandemic, and, more recently, the ongoing international conflicts, declines in consumer confidence, declines in economic growth, increases in unemployment rates and uncertainty about economic stability. There can be no assurances that further deterioration in credit and financial markets and confidence in economic conditions will not occur. For example, U.S. debt ceiling and budget deficit concerns have increased the possibility of additional credit-rating downgrades and economic slowdowns, or a recession in the United States. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

ITEM 1B. UNRESOLVED STAFF COMMENTS.

None.

ITEM 1C. CYBERSECURITY

Risk Management and Strategy

We continue to assess and improve the capabilities of our people, processes, and technologies in order to address our cybersecurity risks. Our cybersecurity risks, and the controls designed to mitigate those risks, are included as a party of our overall risk management governance which is reviewed yearly by our Board of Directors.

Risks from cybersecurity threats are regularly evaluated as a part of our broader risk management activities and as a component of our internal control system. Cybersecurity awareness, including specific topics related to social engineering and email frauds, are included as a part of our annual employee training program. We have processes and controls, in particular with respect to our financial and reporting technology, to prevent and minimize cybersecurity risks and attacks. We rely on industry-standard third-party software

and vendors for our core systems, including data storage, analysis and backup. As discussed under Item 9A – Disclosure Controls and Procedures, we are in the process of improving and enhancing our information technology policies and procedures for our information systems, including cybersecurity capabilities.

Governance

Our Board of Directors is responsible for overseeing our cyber security risk management and strategy. Our senior leadership, including our Chief Financial Officer and controller, regularly meets with and provides periodic briefings to our Board of Directors regarding our company performance and risk management, which include reports of any cybersecurity incidents and improvements and strategy of the Company's cybersecurity risk management. The Audit Committee of the Board of Directors will regularly review cybersecurity requirements and risks on a quarterly basis.

Notwithstanding the approach we take to cybersecurity, we may not be successful in preventing or mitigating a cybersecurity incident that could have a material adverse effect on us. See Item 1A. "Risk Factors" for a discussion of cybersecurity risks.

ITEM 2. PROPERTIES.

Our principal location is at 303 South Broadway, Suite 125 Tarrytown, NY 10591. Currently, our corporate and executive office space is provided by TardiMed pursuant to the Rent and Administrative Services Agreement. We intend to add new facilities or to expand our existing facilities as we add employees, and we believe that suitable additional or substitute space will be available as needed to accommodate any such expansion of our operations.

ITEM 3. LEGAL PROCEEDINGS.

We are not currently subject to any material legal proceedings. However, we may from time to time become a party to various legal proceedings arising in the ordinary course of our business.

ITEM 4. MINE SAFETY DISCLOSURES.

Not applicable.

PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES.

Market Information

Our common stock is listed on the Nasdaq Capital Market under the symbol "PXMD" and has been publicly traded since August 26, 2022. Prior to that date, there was no public market for our stock.

Holders of Record

As of March 11, 2024, there were 10 shareholders of record. The number of record holders does not include persons who held shares of our common stock in "street name" accounts through brokers, banks and other financial institutions.

Dividend Policy

We have not declared or paid any cash dividends on our common stock, and do not currently anticipate paying cash dividends in the foreseeable future. We currently expect to retain future earnings, if any, for the continued development of our business.

Securities Authorized for Issuance Under Equity Compensation Plans

Information about securities authorized for issuance under our equity compensation plans is incorporated herein by reference to Item 12 of Part III of this Annual Report on Form 10-K.

Recent Sales of Unregistered Securities

Other than those previously disclosed by us in our current reports on Form 8-K as filed with the SEC, there have been no unregistered sales of our equity securities during the period covered by this Annual Report.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers

None.

ITEM 6. [RESERVED]

ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS.

You should read the following discussion and analysis together with our consolidated financial statements and related notes included in "Item 8. Financial Statements and Supplementary Data." In this Annual Report on Form 10-K. The following discussion contains forward-looking statements that involve risks, and uncertainties. For a complete discussion of forward-looking statements, see the section above titled "Special Note Regarding Forward Looking Statements." Our actual results could differ materially from those expressed or implied in any forward-looking statements as a result of various factors, including, those set forth under the caption "Item A. Risk Factors."

Overview

We are a clinical stage biopharmaceutical company focusing on the development of anti-purinergic drug therapies ("APT") for the treatment of disorders with intractable neurologic symptoms, ranging from neurodevelopmental disorders, including autism spectrum disorder ("ASD"), to Myalgic Encephalomyelitis/Chronic Fatigue Syndrome ("ME/CFS"), a debilitating physical and cognitive disorder believed to be viral in origin and now with rising incidence globally due to the long term effects of SARS-CoV-2 ("COVID-19"). APTs have been shown to block the effects of excess production and extracellular receptor activity of adenosine triphosphate ("ATP"), which acts as both the main energy molecule in all living cells and a peripheral and central nervous system neurotransmitter via receptors that are found throughout the nervous system. Excess purinergic signaling can offset homeostasis and trigger immune responses that result in localized and systemic increases in inflammatory chemokines and cytokines, ultimately stimulating ATP production. APTs may also impact immunologic and inflammatory mechanisms that may be causing or exacerbating symptoms in these seemingly unrelated disorders, which may be caused in part by similar mechanisms of ATP overproduction.

One of our primary points of focus is currently the development and testing of our lead program, PAX-101, an intravenous formulation of suramin, in the treatment of ASD and the advancement of the clinical understanding of using that agent against other disorders such as fragile X syndrome (FXS), fragile X-associated tremor/ataxia syndrome (FXTAS), ME/CFS and Long COVID-19 Syndrome ("LCS"), a clinical diagnosis in individuals who have been previously infected with COVID-19.

In February 2021, we announced positive topline data from our Phase 2 dose-ranging clinical trial evaluating PAX-101 (commonly known as intravenous suramin) for the treatment of the core symptoms of ASD, as described in more detail below. We also intend to submit data to support a New Drug Application (an "NDA") for PAX-101 under the Tropical Disease Priority Voucher Program of the U.S. Food and Drug Administration (the "FDA") for the treatment of Human African Trypanosomiasis, a fatal parasitic infection commonly known as African sleeping sickness ("HAT"), leveraging suramin's historical use in treating HAT outside of the United States. We have exclusively licensed clinical data from certain academic or international government institutions to potentially accelerate PAX-101's development plans in the United States through this regulatory program and seek approval in the United States for the treatment of East African HAT (as defined below) as early as 2024.

We are also pursuing the development of next generation APT product development candidates for neurodevelopmental indications. These candidates include PAX-102, our proprietary intranasal formulation of suramin, as well as other new chemical entities that are more targeted and selective antagonists of particular purine receptor subtypes. We believe our lead drug candidate (suramin), if approved by the FDA, may be a significant advancement in the treatment of ASD and a potentially useful treatment for FXS, FXTAS, ME/CFS and LCS.

In July 2023, we announced positive topline results from the PAX-101 (intravenous suramin) Phase 3 African Sleeping Sickness Study, PAX-HAT-301. The conclusions of the study confirmed that the retrospective, non-randomized, externally controlled, interventional efficacy and safety study of suramin for the treatment of Stage 1 TBR HAT demonstrated better health outcomes when compared with a natural history control group of patients evaluated and treated from 1900-1910, prior to the availability of suramin in Africa. The adverse event profile of suramin observed in the study was consistent with what has been widely reported in published medical and clinical literature.

We have not generated any revenue to date and, through December 31, 2023, we had an accumulated deficit of approximately $52.0 million. To date, we have financed our operations through contributions from our prior members, proceeds from our initial public offering, the issuance of our convertible notes (including the 2023 Note, as described herein), the issuance of our Simple Agreement For Equity ("SAFE"), Series X preferred stock, par value $0.0001 per share ("Series X preferred stock"), the sale of shares of common stock to Lincoln Park pursuant to the Lincoln Park Purchase Agreement and our secondary public offering in November 2023. We expect our expenses to increase significantly in connection with our ongoing activities to develop, seek regulatory approval and commercialization of PAX-101 and our other product candidates. Furthermore, we expect to incur additional costs associated with operating as a public company. Accordingly, we will likely need substantial additional financing to support our continuing operations. We will seek to fund our operations through public or private equity or debt financings or other sources. Adequate additional financing may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy. We will need to generate significant revenues to achieve profitability, and we may never do so. Accordingly, there are material risks and uncertainties that raise substantial doubt about our ability to continue as a going concern.

2022 Notes

During the second and third quarters of 2022, we issued our senior secured convertible promissory notes (the "2022 Notes") with a principal balance totaling approximately $1.5 million. The 2022 Notes contained an original issue discount totaling approximately $0.2 million and we received net proceeds of approximately $1.2 million (net of financing fees of approximately $0.1 million). The 2022 Notes bore interest at 10% per annum and mature 12 months from the issuance date. The 2022 Notes were secured by all of our assets and personal property. The note holders had the right to convert all or any portion of the outstanding principal balance and accrued interest into shares of the our common stock, up to a beneficial ownership limitation of 9.99% of the number of shares of common stock outstanding at the time of conversion. The per-share conversion price was equal to $71.40 per share. In connection with the 2022 Notes, we issued warrants to purchase 11,479 shares of common stock. The warrants have an exercise price of $71.40 per share and expire five years from the issuance date.

On August 3, 2022, we entered into a conversion agreement with certain holders of the 2022 Notes, pursuant to which the holders agreed to convert $1.0 million of the principal balance at the consummation of our initial public offering at the conversion price of $71.40 per share. During the year ended December 31, 2022, the holders of the 2022 Notes converted the notes fair value of approximately $1.2 million to 15,406 shares of our common stock.

On August 3, 2022, we entered into a conversion agreement with an additional holder of the 2022 Notes, pursuant to which the holder agreed to convert $255,555 of the principal balance of the 2022 Notes at the consummation of our initial public offering into 2,555 shares of Series X preferred stock. During the fourth quarter 2022, the Company issued 251 additional shares of Series X preferred stock to the noteholder.

In February 2023, we paid down the remaining balance of the 2022 Notes (approximately $0.2 million) with a portion of the proceeds received from the 2023 Note.

Initial Public Offering

On August 30, 2022, we closed our initial public offering and received net proceeds of approximately $6.0 million, net of underwriter fees and commissions of approximately $0.8 million, and offering costs of approximately $1.4 million. We sold 90,909 shares of common stock at a price to the public of $89.25. In connection with the initial public offering, we issued 6,364 warrants to purchase shares of common stock with an exercise price of $116.88 per share.

Series X Preferred Stock

In August 2022, we authorized 500,000 shares of Series X preferred stock. The stated value of the Series X preferred stock is $100 per share. The holders of the Series X preferred stock have no voting rights and are not entitled to dividends. The Series X preferred stock is subject to a beneficial ownership limitation of 9.99% of the number of shares of common stock outstanding at the time of conversion.

In August 2022, we issued 3,200 shares of Series X Preferred Stock at a purchase price of $100 per share. We received net proceeds of approximately $0.3 million, net of issuance costs. On August 26, 2022, upon the consummation of our initial public offering, 61,689 shares of the Series X preferred stock were converted into 69,117 shares of our common stock. As of December 31, 2023, 45,567 shares of Series X preferred stock remain outstanding.

Lincoln Park Transaction

In November 2022, we entered into the Lincoln Park Purchase Agreement with Lincoln Park, which provides that, upon the terms and subject to the conditions and limitations set forth in the Lincoln Park Purchase Agreement, we have the right, but not the obligation, to sell to Lincoln Park up to $20 million worth of shares of common stock from time to time over the 30-month term of the Lincoln Park Purchase Agreement. The Lincoln Park Purchase Agreement contains an ownership limitation such that we will not issue, and Lincoln Park will not purchase, shares of our common stock if it would result in their beneficial ownership exceeding 9.99%. Lincoln Park has agreed under the Lincoln Park Purchase Agreement not to cause or engage in any manner whatsoever, any direct or indirect short selling or hedging of our common stock. During the fiscal year ended December 31, 2022, we did not sell any shares of common stock to Lincoln Park pursuant to the Lincoln Park Purchase Agreement, except for 11,705 commitment shares issued to Lincoln Park in November 2022, and in the year ended December 31, 2023 we issued approximately 0.2 million shares of common stock pursuant thereto, receiving net proceeds of approximately $5.6 million. We have an effective registration statement on Form S-1 (File No. 333-268882), registering 770,718 shares of common stock for resale by Lincoln Park from time to time under the Lincoln Park Purchase Agreement, of which 549,896 remain available.

2023 Note

In February 2023 we entered into a Securities Purchase Agreement with an investor and issued a convertible promissory note (the "2023 Note") with a principal balance of $3.7 million. We received proceeds of approximately $2.5 million, net of expenses and other costs. In connection with the 2023 Note, we issued a common stock warrant to purchase 47,059 shares of common stock, with an original exercise price of $55.25. The 2023 Note contained a price adjustment clause where the exercise and conversion price of the Note and Warrant is adjusted to the selling price of an offering if the Company's common stock are sold at a price below the conversion or exercise price, respectively. In November 2023, the Company entered into a public offering in which shares of its common stock were sold at $1.30 per share. As the price of the common stock sold as part of the public offering was below the exercise and conversion price of the Note and Warrant, the terms of the conversion and exercise price were reset to $1.30. As of December 31, 2023, none of these warrants have been exercised, and all of these warrants remain outstanding. In addition, we paid a $112,000 commitment fee to the investor to enter into the 2023 Note. A total of approximately 1.3 million shares of common stock were issued with a fair value of approximately $3.2 million upon conversion of the 2023 Note during the term of the 2023 Note and made a cash payment of approximately $0.5 million. The 2023 was paid off in full in November 2023.

Vox Nova Exclusive Pharmacy Distribution Agreement

On June 30, 2023, we entered into an exclusive specialty benefit manager agreement with Vox Nova, LLC ("Vox Nova") pursuant to which Vox Nova will act as the exclusive United States distributor for our lead pipeline asset, PAX-101 intravenous suramin. Vox Nova will provide certain distribution management, pharmacy benefit management, sales and supply monitoring services with respect to PAX-101, in the event PAX-101 receives FDA approval. The distribution agreement also provides for an exclusivity fee payable to us of up to $2.0 million, payable in installments based on various time and regulatory approval parameters. Vox Nova will pay $0.5 million of the exclusivity fee upfront in connection with the signing of the distribution agreement when the distribution right was transferred to Vox Nova. The remaining $1.5 million is due in four equal installments over a one-year period after PAX-101 is approved by the FDA, if approved, and made available for distribution.

Potential Priority Review Voucher Monetization

We may be eligible to receive a tropical disease Priority Review Voucher ("PRV") for PAX-101, as HAT is defined as a disease qualifying for a tropical disease PRV under Section 524 of the Federal Food, Drug and Cosmetic Act. We are exploring potential monetization opportunities in connection with receipt of a potential PRV. On October 17, 2023, we entered into an Asset Divestiture Engagement Agreement (the "BP Engagement Agreement") with Bourne Capital Partners, LLC ("BP") to engage BP as our exclusive advisor to assist us in exploring a potential sale of the rights to a PRV. However, there can be no assurance that we will receive approval from the FDA for PAX-101, and even if PAX-101 is approved by the FDA, there is a risk that we will not receive a tropical disease PRV. Further, the PRV program has been subject to criticism, including by the FDA, and it is possible that even if we obtain approval for PAX-101 and qualify for a PRV, the program may no longer be in effect at the time of approval. In addition, although PRVs may be sold or transferred to third parties, there is no guarantee that we will be able to realize any value if we were to sell a PRV.

Rediscovery Life Sciences AKI LLC Asset Purchase

On October 24, 2023, we entered into an Asset Purchase Agreement with Rediscovery Life Sciences AKI LLC ("RLS"). Pursuant to the Asset Purchase Agreement, we acquired substantially all of the assets of RLS, including but not limited to its intellectual property, equipment, and inventory. The total purchase price for the assets was $100,000, payable in cash at closing, along with a one-time payment of 1% of gross proceeds earned by us following any potential sale of a Priority Review Voucher Issued by the FDA in connection with the potential approval of an NDA for HAT. The acquisition was financed through our existing cash reserves

Financial Operations Overview

Revenue

To date, we have not generated any revenue. Our ability to generate product revenue, which we do not expect will occur in the near term, if ever, will depend on the successful development and eventual commercialization of our current, and any potential future, product candidates.

Research and Development Expenses

Research and development expenses consist of costs incurred for the development of PAX-101 and our other product candidates, which include:

- the cost of acquiring, developing and manufacturing pre-clinical trial materials;
- costs for consultants and contractors associated with chemistry, manufacturing and controls, or CMCs, pre-clinical activities and regulatory operations;
- expenses incurred under agreements with contract research organizations, or CROs, that conduct our pre-clinical trials; and
- employee-related expenses, including salaries for those employees involved in the research and development process.

Research and development costs are expensed as incurred. Costs for certain activities, such as preclinical studies and clinical trials, are generally recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and collaborators.

General and Administrative Expense

Our general and administrative expenses include costs associated with our executive, accounting, information technology and human resources functions. These expenses consist principally of payroll, employee benefits, travel, and professional services fees such as consulting, audit, tax and legal fees, and general corporate costs. We expense all general and administrative expenses as incurred.

We expect our general and administrative expenses to increase primarily as a result of costs related to us operating as a public company, such as additional legal, accounting, corporate governance, and investor relations expenses, and directors' and officers' insurance premiums.

Fair Value of Restricted Stock Units Granted

During the year ended December 31, 2022 the Company granted 127,015 RSUs with a fair value of approximately $18.1 million and are subject to service conditions.

During the year ended December 31, 2022, 17,148 RSU's (granted on January 1, 2022) were forfeited due to terminations of two of the Company's employees and two of its board members.

During the year ended December 31, 2023, we granted 53,654 RSUs with a fair value of approximately $1.5 million to certain officers, directors, and employees, and are subject to service conditions.

During the year ended December 31, 2023, we recorded stock-based compensation expense related to the RSUs of approximately $5.0 million. During the year ended December 31, 2022, we recorded stock-based compensation expense related to the RSUs of approximately $3.9 million. The unamortized stock-based compensation expense related to RSUs as of December 31, 2023 is approximately $1.5 million, which is expected to be recognized over a remaining weighted average vesting period of 0.92 years.

During the years ended December 31, 2023 and 2022, the Company recorded stock-based compensation expense related to canceled stock options of approximately $0.2 million and $0.5 million, respectively. The unamortized stock-based compensation expense related to canceled stock options (See Note 7), as of December 31, 2023 is less than $0.1 million.

Results of Operations

Comparison of the Year Ended December 31, 2023 to the Year Ended December 31, 2022

	Twelve Months Ended December 31,	
	2023	**2022**
Operating expenses		
General and administrative	$ 12,230,292	$ 8,816,940
Research and development	3,914,979	1,787,488
Total operating expenses	16,145,271	10,604,428
Loss from operations	(16,145,271)	(10,604,428)
Other expense, net	(2,144,454)	(4,197,339)
Net loss	**$ (18,289,725)**	**$ (14,801,767)**

Operating expenses

General and administrative

General and administrative expenses were approximately $12.2 million and $8.8 million for the years ended December 31, 2023 and 2022, respectively. The $3.4 million increase in general and administrative expenses for the year ended December 31, 2023, as compared to the prior period, was primarily due to an increase of $0.8 million for stock-based compensation, an increase of $1.2 million for legal and professional fees, an increase of $1.1 million in payroll and an increase of $0.3 million in other operating expenses.

Research and Development

Research and development expenses were approximately $3.9 million for the year ended December 31, 2023 and approximately $1.8 million for the year ended December 31, 2022. The $2.1 million increase in research and development expenses for the year ended December 31, 2023 as compared to the prior period was primarily attributable to higher costs incurred in connection with our both our research and development activities and include costs associated with clinical trials, consultants, clinical trial materials, regulatory filings, facilities, laboratory expenses and other supplies.

Of the $3.9 million in research and development expenses incurred during the year ended December 31, 2023, $2.4 million was associated with activities related to the HAT indication, and $1.5 million was associated with development activities related to the ASD indication.

Of the $1.8 million in research and development expenses incurred during the year ended December 31, 2022, $1.6 million was associated with activities related to the HAT indication, and $0.2 million was associated with activities related to the ASD indication. These activities included, but were not limited to, milestone payments in connection with the completed ASD trial.

The estimated aggregate costs expected to be incurred for the research and development activities relating to the filing of an NDA for HAT is approximately $2.5 to $2.8 million, which we expect to fund with the proceeds of our secondary public offering and future capital raising activities.

Other Expenses, net

Other expenses, net were approximately $2.1 million for the year ended December 31, 2023 and $4.2 million for the year ended December 31, 2022. The decrease of $2.1 million was comprised of approximately $1.8 million for change in the fair value of notes, approximately $0.2 million for other expenses, and less than $0.1 million for loss on extinguishment of debt.

Liquidity and Capital Resources

As of December 31, 2023, we had an accumulated deficit since inception of approximately $52.0 million. Since inception, we have not generated revenue from product sales and have incurred net losses and negative cash flows from our operations. From inception through December 31, 2023, we have funded our operations through contributions from TardiMed Sciences, LLC ("TardiMed"), the issuance of senior secured convertible promissory notes of approximately $4.2 million, proceeds from our initial public offering, net of fees, of approximately $6.0 million, our SAFE of $5.0 million, the 2023 Note of approximately $2.5 million, the sale of common stock to Lincoln Park Capital of approximately $5.6 million, and the proceeds from our Secondary Public Offering, net of fees, of approximately $6.1 million.

Convertible Notes

During the second and third quarters of 2022, we issued the 2022 Notes with a principal balance totaling approximately $1.5 million. The 2022 Notes contain an original issue discount totaling approximately $0.2 million and we received net proceeds of approximately $1.2 million (net of financing fees of approximately $0.1 million). During 2022 and the first quarter of 2023, all of the 2022 Notes were converted or repaid.

Equity Purchase Agreement with Lincoln Park Capital Fund, LLC

On November 17, 2022, we entered into the Lincoln Park Purchase Agreement which provided that, upon the terms and subject to the conditions and limitations set forth therein, we could sell to Lincoln Park, at its discretion, up to $20.0 million of shares of its common stock over the 30-month term of the purchase agreement. During the year ended December 31, 2022, we did not issue any shares of common stock under the Lincoln Park Purchase Agreement, except for 11,705 commitment shares issued to Lincoln Park, and in the year ended December 31, 2023 we issued approximately 0.2 million shares of common stock pursuant thereto, receiving net proceeds of approximately $5.6 million.

2023 Note

In February 2023 we entered into a Securities Purchase Agreement with an investor and issued a convertible promissory note (the "2023 Note") with a principal balance of $3.7 million. We received proceeds of approximately $2.5 million, net of expenses and other costs. In connection with the 2023 Note, we issued a common stock warrant to purchase 47,059 shares of common stock, with an original exercise price of $55.25. The 2023 Note contained a price adjustment clause where the exercise and conversion price of the Note and Warrant is adjusted to the selling price of an offering if the Company's common stock are sold at a price below the conversion or exercise price, respectively. In November 2023, the Company entered into a public offering in which shares of its common stock were sold at $1.30 per share. As the price of the common stock sold as part of the public offering was below the exercise and conversion price of the Note and Warrant, the terms of the conversion and exercise price were reset to $1.30. As of December 31, 2023, none of these warrants have been exercised, and all of these warrants remain outstanding. In addition, we paid a $112,000 commitment fee to the investor to enter into the 2023 Note. A total of approximately 1.3 million shares of common stock were issued with a fair value of approximately $3.2 million upon conversion of the 2023 Note during the term of the 2023 Note and made a cash payment of approximately $0.5 million. The 2023 was paid off in full in November 2023.

Operating activities

During the year ended December 31, 2023, net cash used in operating activities was $10.8 million, which primarily included our net loss of approximately $18.3 million, adjusted for non-cash expenses of approximately $8.2 million including stock-based compensation of approximately $5.2 million, change in fair value of the notes of approximately $1.8 million in connection with our 2023 and 2022 Notes. The net change in operating assets and liabilities was approximately $0.2 million, driven primarily by an increase in deferred revenue of approximately $0.5 million, and an increase in accounts payable of approximately $0.5 million, offset by an increase in prepaid and other current assets of $0.7 million.

During the year ended December 31, 2022, net cash used in operating activities was $6.1 million, which primarily included our net loss of approximately $14.8 million, adjusted for non-cash expenses of approximately $8.6 million including loss on conversion of our SAFE investment of $5.3 million, stock-based compensation of approximately $4.4 million, loss on issuance of debt of $0.4 million recorded in connection with the issuance of our 2022 Notes, the change in fair value of our 2022 Notes of $0.3 million and loss on extinguishment of debt of $0.2 million and other expenses of $0.1 million, offset by the change in fair value of our warrant liability of $1.9 million and the change in fair value of our SAFE investment of $0.2 million. The net change in operating assets and liabilities was nominal.

Investing Activities

There were no investing activities for the years ended December 31, 2023 and 2022.

Financing activities

During the year ended December 31, 2023, net cash provided by financing activities was approximately $13.6 million, consisting of proceeds received from the sale of common stock to Lincoln Park of approximately $5.6 million, the issuance of the 2023 Note of approximately $2.5 million, and the proceeds of our secondary public offering, net of transaction fees, of approximately $6.1 million.

During the year ended December 31, 2022, net cash provided by financing activities was approximately $7.5 million, consisting of proceeds received from our initial public offering of $6.0 million, the issuance of our 2022 Notes and warrants of $1.2 million, and $0.3 million from the issuance of our Series X preferred stock.

Funding requirements

As of December 31, 2023, we had a cash balance of approximately $4.7 million. Our financial statements have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business. The financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of this uncertainty.

We anticipate incurring additional losses for the foreseeable future and may never become profitable. We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of product candidates. Furthermore, we expect to continue to incur additional costs as a public company. Accordingly, we will likely need to obtain substantial additional funding. If we are unable to raise capital or otherwise obtain funding when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts. These factors raise substantial doubt about our ability to continue as a going concern.

We will seek to obtain additional capital through the sale of debt or equity financings or other arrangements such as, collaborations, strategic alliances and licensing arrangements to fund operations; however, there can be no assurance that we will be able to raise needed capital under acceptable terms, if at all. The sale of additional equity securities may dilute existing stockholders and may contain senior rights and preferences compared to currently outstanding shares of common and preferred stock. Debt securities issued or other debt financing incurred may contain covenants and limit our ability to pay dividends or make other distributions to stockholders. If we are unable to obtain such additional financing, future operations would need to be scaled back or discontinued.

Contractual obligations and commitments

As of December 31, 2023, there were no outstanding principal balance of any Notes.

Off-balance sheet arrangements

We do not have any relationships with unconsolidated entities or financial partnerships, including entities sometimes referred to as structured finance or special purpose entities that were established for the purpose of facilitating off-balance sheet arrangements or other contractually narrow or limited purposes. We do not engage in off-balance sheet financing arrangements. In addition, we do not engage in trading activities involving non-exchange traded contracts. We therefore believe that we are not materially exposed to any financing, liquidity, market or credit risk that could arise if we had engaged in these relationships.

Critical accounting policies and significant judgments and estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States of America ("U.S. GAAP"). The preparation of these financial statements requires us to make estimates, judgments and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities as of the date of the balance sheet and the reported amounts of expenses during the reporting period. In accordance with U.S. GAAP, we evaluate our estimates and judgments on an ongoing basis. The most significant estimates in the Company's financial statements relate to the valuation of warrants, common stock, valuation of the 2022 Notes and 2023 Note, and valuation of the SAFE liability. These estimates and assumptions are based on current facts, historical experience and various other factors believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. Actual results may differ materially and adversely from these estimates. To the extent there are material differences between the estimates and actual results, our future results of operations will be affected.

We define our critical accounting policies and estimates as those accounting principles that require us to make subjective estimates and judgments about matters that are uncertain and are likely to have a material impact on our financial condition and results of operations, as well as the specific manner in which we apply those principles. While our significant accounting policies are more fully described in Note 3 to our financial statements, we believe the following are the critical accounting policies used in the preparation of our financial statements that require significant estimates and judgments:

Stock-Based Compensation

We expense stock-based compensation to employees, non-employees and board members over the requisite service period based on the estimated grant-date fair value of the awards and actual forfeitures. We account for forfeitures as they occur. Stock-based awards with graded vesting schedules are recognized on a straight-line basis over the requisite service period for each separately vesting portion of the award.

The determination of the grant date fair value of options using an option pricing model is affected principally by our estimated fair value of shares of our common stock and requires management to make a number of other assumptions, including the expected term of the option, the expected volatility of the underlying shares, the risk-free interest rate and the expected dividend yield. The assumptions used in our Black-Scholes option-pricing model represent management's best estimates at the time of measurement. These estimates are complex, involve a number of variables, uncertainties and assumptions and the application of management's judgment, as they are inherently subjective. If any assumptions change, our stock-based compensation expense could be materially different in the future. These assumptions are estimated as follows:

- *Fair Value of Common Stock*. See the subsection titled "- Fair Value of Common Stock" below.

- *Expected Term*. The expected term represents the period that our options are expected to be outstanding. We calculated the expected term using the simplified method for options based on the average of each option's vesting term and the contractual period during which the option can be exercised, which is typically 10 years following the date of grant.

- *Expected Volatility*. The expected volatility was based on the historical share volatility of several comparable publicly traded companies over a period of time equal to the expected term of the options, as we did not have any trading history to use the volatility of our own common stock. The comparable companies were chosen based on their size, stage in life cycle and area of specialty. We will continue to apply this process until a sufficient amount of historical information regarding the volatility of our own stock price becomes available.

- *Risk-Free Interest Rate.* The risk-free interest rate was based on the yields of U.S. Treasury securities with maturities appropriate for the term of the award.

- *Expected Dividend Yield.* We have not paid dividends on our common stock nor do we expect to pay dividends in the foreseeable future. Therefore, we used an expected dividend yield of zero.

Fair Value of Common Stock

Prior to our initial public offering, there was no public market for our common stock. As such, the estimated fair value of our common stock and underlying stock options has been determined at each grant date by our board of directors, with input from management, based on the information known to us on the grant date.

Beginning in May 2020, we determined the estimated fair value of our common stock using the Hybrid Method, which incorporated the Option Pricing Model ("OPM") and the Probability Weighted Expected Return Method ("PWERM"), estimating the probability-weighted value across multiple scenarios by using the OPM to estimate the allocation of value within one or more of those scenarios. The Hybrid Method was utilized given there was transparency into one or more near- term potential exits but there existed uncertainty regarding what would occur if the near-term exit plans did not materialize. Under the PWERM, the values of the various equity interests were estimated based upon an analysis of future values for our company, assuming various potential future outcomes. Share value was based upon the probability-weighted present value of expected future investment returns, considering each of the possible future outcomes available to us, as well as the rights of each share class. The future outcomes modeled included an initial public offering, a dissolution or continued operation as a private company until a later exit date. To estimate our total equity value, a combination of the Backsolve Methodology ("backsolving" the implied enterprise value based on the price paid for each new preferred security sold), a discounted cash flow analysis and a guideline publicly traded company method was used for scenario options, based on the fact pattern that existed as of the particular valuation date. After deriving the indicated values of equity under the scenario options, the present value of the class specific equity allocations were calculated. After calculating the present values as applicable to the scenarios, the probability of each scenario occurring was multiplied by the indications of value under each scenario. The sum of the probability-weighted values for our common stock was then divided by our total common stock outstanding as of the relevant valuation date.

In addition to considering the results of these third-party valuation reports, our board of directors used assumptions based on various objective and subjective factors, combined with management judgment, to determine the fair value of our common stock as of the grant date, including:

- the prices at which we sold shares of preferred stock and the superior rights and preferences of the preferred stock relative to our common stock at the time of each grant;

- external market conditions affecting the life sciences research and development industry and trends within the industry;

- our stage of development and business strategy;

- our financial condition and operating results, including our levels of available capital resources and forecasted results;

- developments in our business;

- the progress of our research and development efforts;

- equity market conditions affecting comparable public companies; and

- general market conditions and the lack of marketability of our common stock.

Application of these approaches involves the use of estimates, judgment and assumptions that are highly complex and subjective, such as those regarding our expected future revenue, expenses and future cash flows, discount rates, market multiples, the selection of comparable companies and the probability of possible future events. Changes in any or all of these estimates and assumptions or the relationships between the assumptions impact our valuations as of each valuation date and may have a material impact on the valuation of our common stock.

Following our initial public offering, a public trading market for our common stock has been established and it will no longer be necessary for our board of directors to estimate the fair value of our common stock in connection with our accounting for granted stock options and other such awards we may grant, as the fair value of our common stock will be determined based on the closing price of our common stock as reported on the date of grant.

Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. Actual results could differ from those estimates.

Convertible Notes

In accordance with Accounting Standards Codification 825, *Financial Instruments* ("ASC 825"), we have elected the fair value option for recognition of its convertible notes. In accordance with ASC 825, we recognize these convertible notes at fair value with changes in fair value recognized in the statements of operations. The fair value option may be applied instrument by instrument, but it is irrevocable. As a result of applying the fair value option, direct costs and fees related to the convertible notes were recognized in general and administrative expense. Accrued interest for the notes has been included in the change in fair value of convertible notes in the statements of operations. The change in fair value measurement of the convertible notes is included in the Change in fair value of notes line item of the Company's statements of operations.

Accrued Outsourcing Costs

Substantial portions of our preclinical studies and clinical trials are performed by third-party laboratories, medical centers, CROs and other vendors. These CROs generally bill monthly or quarterly for services performed, or bill based upon milestone achievement. For preclinical studies, we accrue expenses based upon estimated percentage of work completed and the contract milestones remaining. Clinical trial costs are a significant component of research and development expenses and include costs associated with third-party contractors. We outsource a substantial portion of our clinical trial activities, utilizing external entities such as CROs, independent clinical investigators, and other third-party service providers to assist us with the execution of its clinical studies. For each clinical trial that we conduct, certain clinical trial costs are expensed immediately, while others are expensed over time based on the number of patients in the trial, the attrition rate at which patients leave the trial, and/or the period over which clinical investigators or CROs are expected to provide services. Our estimates depend on the timeliness and accuracy of the data provided by the CROs regarding the status of each program and total program spending. We periodically evaluate the estimates to determine if adjustments are necessary or appropriate based on information it receives.

Research and Development

Research and development expenses consist primarily of salaries, benefits and other related costs, including stock-based compensation, for personnel serving our development functions, and other internal operating expenses, the cost of clinical studies, and the cost of our drug candidate for clinical study. In addition, research and development expenses include payments to third parties for the development of our product candidates and the estimated fair value for the issuance of equity for the license rights to products in development (prior to marketing approval). Our expenses related to clinical trials are primarily related to activities at CROs that design, gain approval for and conduct clinical trials on our behalf. Such amounts are then recognized as an expense as the related goods are delivered or the services are performed.

Recent accounting pronouncements

See Note 3 to our financial statements for a description of recent accounting pronouncements applicable to our financial statements.

Emerging Growth Company and Smaller Reporting Company Status

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act ("JOBS Act"), and we may remain an emerging growth company for up to five years following the completion of our initial public offering. For so long as we remain an emerging growth company, we are permitted and intend to rely on certain exemptions from various public company reporting requirements, including not being required to have our internal control over financial reporting audited by our independent registered public accounting firm pursuant to Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive

compensation in our periodic reports and proxy statements, exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and any golden parachute payments not previously approved and an exemption from compliance with the requirements regarding the communication of critical audit matters in the auditor's report on financial statements.

Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies. We have elected to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will not be subject to the same new or revised accounting standards as public companies that are not emerging growth companies. As a result of this election, our financial statements may not be comparable to those of companies that are not emerging growth companies.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK.

We are a smaller reporting company as defined by Rule 12b-2 of the Exchange Act and are not required to provide the information otherwise required under this item.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA.

INDEX TO FINANCIAL STATEMENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Shareholders and Board of Directors of **Paxmedica, Inc.**

Opinion on the Financial Statements

We have audited the accompanying balance sheets of PaxMedica, Inc. (the "Company") as of December 31, 2023 and 2022, the related statements of operations, statements of stockholders' equity and cash flows for each of the two years in the period ended December 31, 2023, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2023 and 2022, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2023, in conformity with accounting principles generally accepted in the United States of America.

Explanatory Paragraph – Going Concern

The accompanying financial statements have been prepared assuming that the Company will continue as a going concern. As more fully described in Note 2, the Company has incurred significant losses and needs to raise additional funds to meet its obligations and sustain its operations. These conditions raise substantial doubt about the Company's ability to continue as a going concern. Management's plans in regard to these matters are also described in Note 2. The financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audit. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) ("PCAOB") and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audit we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audit included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audit also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audit provide a reasonable basis for our opinion.

/s/ Marcum LLP

Marcum LLP

We have served as the Company's auditor since 2020

Marcum LLP
New York, NY
March 11, 2024

PAXMEDICA, INC.
Balance Sheets

	December 31, 2023	December 31, 2022
ASSETS		
Current assets		
Cash	$ 4,710,642	$ 1,901,887
Accounts receivable	—	—
Prepaid administration	390,400	302,431
Prepaid contractors	579,363	—
Prepaid and other current assets	969,763	302,431
Total current assets	5,680,405	2,204,318
Total assets	**$ 5,680,405**	**$ 2,204,318**
LIABILITIES AND STOCKHOLDERS' EQUITY (DEFICIT)		
Current liabilities		
Accounts payable	$ 811,733	$ 721,955
Accounts payable - related party	18,500	20,000
Accrued expenses	177,118	518,613
Accrued expenses - Compensation	787,981	500,458
Note payable - fair value, current portion	—	173,543
Shares settled liability	—	160,949
Total current liabilities	1,795,332	2,095,518
Deferred revenue	500,000	—
Total liabilities	**2,295,332**	**2,095,518**
Commitments and contingencies (Note 9)		
Stockholders' Equity (Deficit)		
Preferred stock, par value $0.0001, 10,000,000 shares authorized:		
Series X preferred shares, 500,000 shares authorized as of December 31, 2023 and December 31, 2022; 45,567 shares issued and outstanding at December 31, 2023 and December 31, 2022	5	5
Common stock, par value $0.0001; 200,000,000 shares authorized as of December 31, 2023 and December 31, 2022; 7,401,242 and 707,976 shares issued and outstanding at December 31, 2023 and December 31, 2022, respectively	740	71
Additional paid-in capital	55,414,069	33,848,740
Accumulated deficit	(52,029,741)	(33,740,016)
Total stockholders' equity (deficit)	3,385,073	108,800
Total liabilities, and stockholders' equity (deficit)	**$ 5,680,405**	**$ 2,204,318**

The accompanying notes are an integral part of these financial statements.

PAXMEDICA, INC.
Statements of Operations
Years Ended December 31, 2023 and 2022

	Year Ended December 31,	
	2023	**2022**
Operating expenses		
General and administrative	$ 12,230,292	$ 8,816,940
Research and development	3,914,979	1,787,488
Total operating expenses	16,145,271	10,604,428
Loss from operations	(16,145,271)	(10,604,428)
Other income (expense):		
Interest expense	(9,165)	(5,237)
Loss on conversion of SAFE	—	(5,338,808)
Loss on issuance of debt	—	(391,246)
Loss on extinguishment of debt	(45,585)	(161,563)
Change in fair value of notes	(1,819,009)	(272,202)
Change in fair value of SAFE	—	163,025
Change in fair value warrant liability	—	1,873,192
Other expense	(270,695)	(64,500)
Total other expense	(2,144,454)	(4,197,339)
Net loss	**$ (18,289,725)**	**$ (14,801,767)**
Basic weighted average number of shares outstanding	1,662,795	514,216
Diluted weighted average number of shares outstanding	1,662,795	514,216
Basic net loss per share	$ (11.00)	$ (28.79)
Diluted net loss per share	$ (11.00)	$ (28.79)

The accompanying notes are an integral part of these financial statements.

PAXMEDICA, INC.
Statements of Stockholders' Equity (Deficit)
Year Ended December 31, 2023

	Series X Preferred Stock		Common Stock		Additional Paid-in	Accumulated	Total Stockholders'
	Shares	Amount	Shares	Amount	Capital	Deficit	Equity (Deficit)
Balance at January 1, 2023	**45,567**	**$ 5**	**707,976**	**$ 71**	**$ 33,848,740**	**$ (33,740,016)**	**$ 108,800**
Reclassification of shares settled liability to equity	—	—	1,824	—	160,949	—	160,949
Issuance of common stock in connection with convertible note	—	—	1,340,903	131	3,195,163	—	3,195,294
Issuance of common stock in connection with equity purchase agreement	—	—	5,279,501	532	11,945,404		11,945,936
Issuance of common stock warrants in connection with notes payable, net of fees	—	—	—	—	1,155,642	—	1,155,642
Delivery of common stock underlying restricted stock units, net of tax withholding	—	—	25,642	2	(122,612)	—	(122,610)
Warrant reset charge	—	—	—	—	25,836	—	25,836
Stock-based compensation	—	—	45,396	4	5,204,946	—	5,204,950
Net loss	—	—	—	—	—	(18,289,725)	(18,289,725)
Balance at December 31, 2023	**45,567**	**$ 5**	**7,401,242**	**$ 740**	**$ 55,414,069**	**$ (52,029,741)**	**$ 3,385,073**

	Preferred Stock		Series X Preferred Stock		Common Stock		Paid-in	Accumulated	Stockholders'
	Shares	Amount	Shares	Amount	Shares	Amount	Capital	Deficit	Equity (Deficit)
Balance at January 1, 2022	**2,696,439**	**$ 270**	**—**	**$ —**	**406,676**	**$ 41**	**$ 8,829,075**	**$ (18,938,249)**	**$ (10,108,864)**
Issuance of common stock for commitment fee in connection with equity purchase agreement	—	$ —	—	$ —	11,704	$ 1	$ (1)	$ —	$ 0
Issuance of common stock for consulting services	—	$ —	—	$ —	3,362	$ 0	$ 100,000	$ —	$ 100,000
Issuance of Series X preferred stock, net of fees	—	$ —	3,200	$ 1	—	$ —	$ 299,999	$ —	$ 300,000
Conversion of SAFE liability to Series X preferred stock	—	$ —	100,000	$ 10	—	$ —	$ 9,999,990	$ —	$ 10,000,000
Issuance of common stock and warrants, net of fees	—	$ —	—	$ —	90,909	$ 9	$ 6,023,004	$ —	$ 6,023,013
Issuance of common stock in connection with conversion of notes payable	—	$ —	—	$ —	14,006	$ 1	$ 1,159,499	$ —	$ 1,159,500
Issuance of Series X preferred stock in connection with conversion of notes payable	—	$ —	2,806	$ —	—	$ —	$ 327,993	$ —	$ 327,993
Conversion of Series Seed preferred stock to common stock	(2,696,439)	$ (270)	—	$ —	91,614	$ 9	$ 260	$ —	$ (0)
Conversion of Series X preferred stock to common stock	—	$ —	(61,689)	$ (6)	69,117	$ 7	$ (1)	$ —	$ (0)
Warrants exchanged for shares of common stock and Series X preferred stock	—	$ —	1,250	$ —	20,588	$ 2	$ 2,009,205	$ —	$ 2,009,207
Reclassification of warrants to equity	—	$ —	—	$ —	—	$ —	$ 912,580	$ —	$ 912,580
Costs incurred in connection with equity purchase agreement	—	$ —	—	$ —	—	$ —	$ (230,997)	$ —	$ (230,997)
Stock-based compensation	—	$ —	—	$ —	—	$ —	$ 4,418,134	$ —	$ 4,418,134
Net loss	—	$ —	—	$ —	—	$ —	$ —	$ (14,801,767)	$ (14,801,767)
Balance at December 31, 2022	**—**	**$ —**	**45,567**	**$ 5**	**707,976**	**$ 71**	**$ 33,848,740**	**$ (33,740,016)**	**$ 108,800**

The accompanying notes are an integral part of these financial statements.

PAXMEDICA, INC.
Statements of Cash Flows
Years Ended December 31, 2023 and 2022

	Year Ended December 31,	
	2023	**2022**
Cash flows from operating activities		
Net loss	$ (18,289,725)	$ (14,801,767)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation	5,204,950	4,418,134
Issuance of common stock for consulting services	—	100,000
Change in fair value of notes	1,819,009	272,202
Change in fair value of SAFE	—	(163,025)
Loss on conversion of SAFE	—	5,338,808
Loss on extinguishment of debt	45,585	161,563
Loss on issuance of debt	—	391,246
Warrant reset charge	25,836	—
Change in fair value warrant liability	—	(1,873,192)
Other	91,992	34,500
Changes in assets and liabilities:		
Prepaid and other current assets	(667,332)	—
other current assets	—	(302,431)
Deferred revenue	500,000	—
Accounts payable	456,225	94,914
Accounts payable - related party	(1,500)	19,250
Accrued expenses	46,456	238,615
Net cash used in operating activities	(10,768,504)	(6,071,183)
Cash flows from financing activities		
Proceeds from issuance of common stock and warrants, net of fees	—	6,023,013
Proceeds from issuance of convertible promissory notes and warrants	3,200,000	1,240,970
Proceeds from the issuance of common stock in connection with equity purchase agreement	11,945,936	—
Costs related to issuance of common stock and warrants	(503,695)	—
Proceeds from issuance of Series X preferred stock	—	300,000
Payment of costs in connection with equity purchase agreement	(195,997)	(35,000)
Repayment of convertible promissory notes	(746,375)	—
Settlement of shares withheld for payment of employee taxes	(122,610)	—
Net cash provided by financing activities	13,577,259	7,528,983
Net increase in cash	2,808,755	1,457,800
Cash, beginning of period	1,901,887	444,087
Cash, end of period	$ 4,710,642	$ 1,901,887
Non-cash financing activities:		
Series X preferred stock issued in connection with conversion of notes payable	$ —	$ 327,993
Common stock issued in connection with conversion of notes payable	$ 3,195,294	$ 1,159,500
Conversion of SAFE liability to Series X preferred stock	$ —	$ 10,000,000
Warrants exchanged for Shares of common stock and Series X preferred stock	$ —	$ 2,009,207
Reclassification of warrants to equity	$ —	$ 912,580
Reclassification of shares settled liability	$ 160,949	$ —
Unpaid costs for equity purchase agreement	—	195,997

The accompanying notes are an integral part of these financial statements.

PAXMEDICA, INC.
Notes To Financial Statements

Note 1. Organization and Description of Business Operations

PaxMedica, Inc. (Formerly Purinix Pharmaceuticals LLC) (the "Company") is a clinical stage biopharmaceutical company organized as a Delaware limited liability company on April 5, 2018 ("Inception") to focus on the development of drug candidates for the treatment of autism spectrum disorder (ASD), Fragile X syndrome tremor-ataxia (FXTAS) and Human African Trypanosomiasis (HAT).

Secondary Public Offering

On November 20, 2023, the Company entered into an underwriting agreement relating to the secondary public offering of its common stock, par value $0.0001 per share. The Company agreed to sell 5,384,615 shares of its common stock with the offering price to the public of $1.30 per share, pursuant to the Company's registration statement on Form S-1 (File No. 333-275416), as amended, under the Securities Act of 1933, that was filed by the Company under Rule 462(b) under the Securities Act. On November 22, 2023, the Company received net proceeds from its public offering of approximately $6.1 million, net of underwriter fees and commissions of approximately $0.6 million, and offering costs of approximately $0.3 million. In connection with its public offering the Company issued 5,384,615 warrants to purchase shares of the Company's common stock with an exercise price of $1.30 per share and issued 215,385 warrants to purchase shares of the Company's common stock with an exercise price of $1.625.

The 2023 Note contained a price adjustment clause where the exercise and conversion price of the 2023 Note and Warrant is adjusted to the selling price of an offering if the Company's common stock are sold at a price below the conversion or exercise price, respectively. In November 2023, the Company entered into a public offering in which shares of its common stock were sold at $1.30 per share. As the price of the common stock sold as part of the public offering was below the exercise and conversion price of the Note and Warrant, the terms of the conversion and exercise price were reset to $1.30, as further described below.

Note 2. Going Concern, Liquidity and Capital Resources

The Company has no product revenues, incurred operating losses since inception, and expects to continue to incur significant operating losses for the foreseeable future and may never become profitable. The Company had working capital of $3.9 million, an accumulated deficit of approximately $52.0 million at December 31, 2023, a net loss of approximately $18.3 million, and approximately $10.8 million of net cash used in operating activities for the year ended December 31, 2023.

Equity Purchase Agreement with Lincoln Park Capital Fund, LLC

On November 17, 2022, the Company entered into an equity purchase agreement (the "Purchase Agreement") with Lincoln Park Capital Fund, LLC ("LPC") which provided that, upon the terms and subject to the conditions and limitations set forth therein, the Company could sell to LPC, at its discretion, up to $20.0 million of shares of its common stock over the 30-month term of the Purchase Agreement (See Note 8). During the year ended December 31, 2022, the Company did not issue any shares of common stock in connection with the Purchase Agreement and for the year ended December 31, 2023, the Company issued approximately 0.2 million shares of common stock, receiving net proceeds of approximately $5.6 million.

Convertible Promissory Note

In February 2023 we entered into a Securities Purchase Agreement with an investor and issued a convertible promissory note (the "2023 Note") with a principal balance of $3.7 million. We received proceeds of approximately $2.5 million, net of expenses and other costs. In connection with the 2023 Note, we issued a common stock warrant to purchase 47,059 shares of common stock, with an original exercise price of $55.25. The 2023 Note contained a price adjustment clause, a down round feature (see Financial Instruments With Down Round Features policy discussion in Note 3) where the exercise and conversion price of the Note and Warrant is adjusted to the selling price of an offering if the Company's common stock are sold at a price below the conversion or exercise price, respectively. In November 2023, the Company entered into a public offering in which shares of its common stock were sold at $1.30 per share. As the price of the common stock sold as part of the public offering was below the exercise and conversion price of the Note and Warrant, the terms of the conversion and exercise price were reset to $1.30.

As the 2023 Note is presented at fair value pursuant to the Company's election of the fair value option (see Convertible Notes policy discussion in Note 3), the change in fair value resulting from the lower conversion price upon reset is captured as part of the fair value

measurement of the financial instrument in the fourth quarter of fiscal year 2023. See Note 4 for a reconciliation of convertible notes balance for the year ended December 31, 2023, including the change in fair value of the convertible notes during the year. The change in fair value of the warrants as a result of the reduced exercise price was recorded as a charge to operations and as an adjustment to additional paid in capital in the amount of $25,836. The Warrant remained equity classified. See Financial Instruments With Down Round Features policy discussion in Note 3 below for further information on the accounting for reset features.

As of December 31, 2023, none of these warrants have been exercised, and all of these warrants remain outstanding. In addition, we paid a $112,000 commitment fee to the investor to enter into the 2023 Note. A total of approximately 1.3 million shares of common stock were issued with a fair value of approximately $3.2 million upon conversion of the 2023 Note during the term of the 2023 Note and made a cash payment of approximately $0.5 million. The 2023 Note was paid off in full in November 2023.

2022 Notes

During the second and third quarters of 2022, the Company entered into senior secured convertible promissory notes (the "2022 Notes") with a principal balance totaling approximately $1.5 million. During the second and third quarters of 2022, the Company entered into senior secured convertible promissory notes (the "2022 Notes") with a principal balance totaling approximately $1.5 million. The 2022 Notes contain an original issue discount totaling $0.3 million and the Company received net proceeds of approximately $1.2 million. In February 2023, the Company paid down the remaining balance of its 2022 Notes (approximately $0.2 million) with a portion of the proceeds received from the 2023 Note. (See Note 6).

Going Concern

The accompanying financial statements have been prepared assuming the Company will continue to operate as a going concern, which contemplates the realization of assets and settlement of liabilities in the normal course of business, and do not include any adjustments to reflect the possible future effects on the recoverability and classification of assets or the amounts and classifications of liabilities that may result from uncertainty related to its ability to continue as a going concern.

The Company's future liquidity and capital funding requirements will depend on numerous factors, including:

- its ability to raise additional funds to finance its operations;
- the outcome, costs and timing of clinical trial results for the Company's current or future product candidates;
- the emergence and effect of competing or complementary products;
- its ability to maintain, expand and defend the scope of its intellectual property portfolio, including the amount and timing of any payments the Company may be required to make, or that it may receive, in connection with the licensing, filing, prosecution, defense and enforcement of any patents or other intellectual property rights;
- its ability to retain its current employees and the need and ability to hire additional management and scientific and medical personnel; and
- the terms and timing of any collaborative, licensing or other arrangements that it has or may establish.

The Company will likely need to raise substantial additional funds through one or more of the following: issuance of additional debt or equity, or the completion of a licensing transaction for one or more of the Company's pipeline assets. If the Company is unable to maintain sufficient financial resources, its business, financial condition and results of operations will be materially and adversely affected. This could affect future development and business activities and potential future clinical studies and/or other future ventures. Failure to obtain additional equity or debt financing will have a material, adverse impact on the Company's business operations. There can be no assurance that the Company will be able to obtain the needed financing on acceptable terms or at all. Additionally, equity or debt financings will likely have a dilutive effect on the holdings of the Company's existing stockholders. Accordingly, there are material

risks and uncertainties that raise substantial doubt about the Company's ability to continue as a going concern for the next twelve months from the issuance of these financial statements. The accompanying financial statements do not include any adjustments that result from the outcome of these uncertainties.

The COVID-19 global pandemic has been unprecedented and unpredictable, is likely to continue to result in significant national and global economic disruption, which may adversely affect our business. Based on the Company's current assessment, the Company does not expect any material impact on its long-term development timeline and its liquidity due to the worldwide spread of the COVID-19 virus. However, the Company is continuing to assess the effect on its operations by monitoring the spread of COVID-19 and the resulting global pandemic and the actions implemented to combat the virus throughout the world.

Note 3. Significant Accounting Policies

Basis of Presentation

The Company's financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP") as determined by the Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") and include all adjustments necessary for the fair presentation of its balance sheets, results of operations and cash flows for the period presented.

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. The most significant estimates in the Company's financial statements relate to the valuation of warrants, common stock, valuation of the 2022 Notes and 2023 Note, and valuation of the SAFE liability. These estimates and assumptions are based on current facts, historical experience and various other factors believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. Actual results may differ materially and adversely from these estimates. To the extent there are material differences between the estimates and actual results, the Company's future results of operations will be affected.

Cash, Cash Equivalents and Short-Term Investments

The Company considers all highly liquid investments with an original maturity of three months or less at the date of acquisition to be cash equivalents. As of December 31, 2023 and 2022, the Company had no cash equivalents or short-term investments.

Concentrations of Credit Risk

Financial instruments that potentially subject the Company to concentration of credit risk consist primarily of cash. The Company maintains its cash high credit quality financial institutions, which may at times, be in excess of federal insured limits. The Company believes it is not exposed to any significant losses due to credit risk on cash.

Risks and Uncertainties

The Company operates in a dynamic and highly competitive industry and is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, protection of proprietary technology, dependence on key personnel, contract manufacturer and contract research organizations, compliance with government regulations and the need to obtain additional financing to fund operations. Product candidates currently under development will require significant additional research and development efforts, including extensive preclinical studies and clinical trials and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure and extensive compliance and reporting. The Company believes that changes in any of the following areas could have a material adverse effect on the Company's future financial position, results of operations, or cash flows; ability to obtain future financing; advances and trends in new technologies and industry standards; results of clinical trials; regulatory approval

and market acceptance of the Company's products; development of sales channels; certain strategic relationships; litigation or claims against the Company based on intellectual property, patent, product, regulatory, or other factors; and the Company's ability to attract and retain employees necessary to support its growth.

Fair Value of Financial Instruments

The Company accounts for financial instruments under Financial Accounting Standards Board ("FASB") Accounting Standards Codification 820 ("ASC 820"), Fair Value Measurements. This statement defines fair value, establishes a framework for measuring fair value in generally accepted accounting principles, and expands disclosures about fair value measurements. To increase consistency and comparability in fair value measurements, ASC 820 establishes a fair value hierarchy that prioritizes the inputs to valuation techniques used to measure fair value into three levels as follows:

Level 1 - quoted prices (unadjusted) in active markets for identical assets or liabilities;

Level 2 - observable inputs other than Level 1, quoted prices for similar assets or liabilities in active markets, quoted prices for identical or similar assets and liabilities in markets that are not active, and model-derived prices whose inputs are observable or whose significant value drivers are observable; and

Level 3 - assets and liabilities whose significant value drivers are unobservable.

Observable inputs are based on market data obtained from independent sources, while unobservable inputs are based on the Company's market assumptions. Unobservable inputs require significant management judgment or estimation. In some cases, the inputs used to measure an asset or liability may fall into different levels of the fair value hierarchy. In those instances, the fair value measurement is required to be classified using the lowest level of input that is significant to the fair value measurement. Such determination requires significant management judgment. During the year ended December 31, 2022, the Company issued certain of the 2022 Notes and warrants in connection with the 2022 Notes. The 2022 Notes and warrants were classified as liabilities and measured at fair value on the issuance date, with changes in fair value recognized as other Income (expense) on the statements of operations and disclosed in the financial statements During the year ended December 31, 2021, the Company entered into its SAFE agreement and classified the SAFE as a liability measured at cost on the issuance date, with changes in fair value recognized as other income on the statement of operations. The carrying amounts of the Company's financial assets and liabilities, such as accounts payable, approximate fair value due to the short-term nature of these instruments.

Revenue Recognition

The Company recognizes revenue in accordance with ASC 606 – Revenue from Contracts with Customers. ASC 606 provides a five-step framework through which revenue is recognized when control of promised goods or services is transferred to a customer at an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. To determine revenue recognition for arrangements that the Company concludes are within the scope of the new revenue recognition standard, management performs the following five steps: (i) identifies the contract(s) with a customer; (ii) identifies the performance obligations in the contract; (iii) determines the transaction price, including whether there are any constraints on variable consideration; (iv) allocates the transaction price to the performance obligations; and (v) recognizes revenue when (or as) the Company satisfies a performance obligation. Upfront payments and fees are recorded as deferred revenue upon receipt or when due and most often require deferral of revenue recognition to a future period until the Company performs its obligations under the underlying arrangements.

On July 1, 2023, the Company entered into a Specialty Benefit Manager Agreement with Vox Nova, LLC pursuant to which the parties agree to an exclusive distribution arrangement in which Vox Nova is liable to the Company for a $2.0 million exclusivity fee in return for exclusive pharmacy distribution rights over PAX-101 for up to seven years. Vox Nova paid $0.5 million of the exclusivity fee upfront and will remit the remaining $1.5 million in four equal installments after PAX-101 is approved by the FDA and made available for distribution.

The Vox Nova Specialty Benefit Manager Agreement will be accounted for under ASC 606. In accordance with the provisions under ASC 606, the Company identified the material right related to exclusivity provision over contract term upon approval as the sole distinct performance obligations as of the effective date of the agreement, June 30, 2023. The Company determined that the revenue related to

the exclusivity fee was to be recognized ratably over the period subsequent to the FDA's approval of PAX-101. Pursuant to this, the Company will recognize the $0.5 million payment made by Vox Nova at the agreement inception as a deferred revenue liability and will begin recognizing the $0.5 million as revenue once PAX-101 has been approved by the FDA, and the Company has met all the requirements under ASC 606.

Convertible Notes

In accordance with Accounting Standards Codification 825, *Financial Instruments* ("ASC 825"), the Company has elected the fair value option for recognition of its convertible notes. In accordance with ASC 825, the Company recognizes these convertible notes at fair value with changes in fair value recognized in the statements of operations. The fair value option may be applied instrument by instrument, but it is irrevocable. As a result of applying the fair value option, direct costs and fees related to the convertible notes were recognized in general and administrative expense. Accrued interest for the convertible notes has been included in the change in fair value of convertible notes in the statements of operations.

Financial Instruments With Down Round Features

The Company adopted the provisions of ASU No. 2017-11, "Earnings per Share (Topic 260); Distinguishing Liabilities from Equity (Topic 480); Derivatives and Hedging (Topic 815): (Part I) Accounting for Certain Financial Instruments with Down Round Features ("ASU 2017-11") to account for the down round features of convertible notes and warrants issued with private placements effective as of January 1, 2020. In doing so, equity-linked financial instruments with a down round feature previously treated as derivative liabilities in the balance sheet and measured at fair value are henceforth treated as equity with no adjustment for changes in fair value at each reporting period unless the fair value option in ASC 825 is elected (see *Convertible Notes* policy discussion above). The impact of down round provisions are accounted for when the event occurs. Earnings per share ("EPS") data is adjusted for purposes of calculating diluted EPS to give effect of the feature when triggered (i.e. when the exercise price of the related equity-linked financial instrument is adjusted downward because of the down round feature) and effect of the trigger is also recognized within equity.

Warrant Liability

The Company accounts for certain common stock warrants outstanding as a liability at fair value and adjusts the instruments to fair value at each reporting period. This liability is subject to re-measurement at each balance sheet date until exercised, and any change in fair value is recognized in the Company's statements of operations. The fair value of the warrants issued by the Company have been estimated using the Monte Carlo simulation. As of December 31, 2022 and December 31, 2023, there are no warrant liabilities (See Note 4).

Research and Development

Research and development costs are expensed as incurred. Advance payments for goods and services that will be used in future research and development activities are expensed when the activity has been performed or when the goods have been received.

Accrued Outsourcing Costs

Substantial portions of the Company's preclinical studies and clinical trials are performed by third-party laboratories, medical centers, contract research organizations and other vendors (collectively "CROs"). These CROs generally bill monthly or quarterly for services performed, or bill based upon milestone achievement. For preclinical studies, the Company accrues expenses based upon estimated percentage of work completed and the contract milestones remaining. Clinical trial costs are a significant component of research and development expenses and include costs associated with third-party contractors. The Company outsources a substantial portion of its clinical trial activities, utilizing external entities such as CROs, independent clinical investigators, and other third-party service providers to assist the Company with the execution of its clinical studies. For each clinical trial that the Company conducts, certain clinical trial costs are expensed immediately, while others are expensed over time based on the number of patients in the trial, the attrition rate at which patients leave the trial, and/or the period over which clinical investigators or CROs are expected to provide services. The Company's estimates depend on the timeliness and accuracy of the data provided by the CROs regarding the status of each program and total program spending. The Company periodically evaluates the estimates to determine if adjustments are necessary or appropriate based on information it receives.

Stock-Based Compensation

The Company recognizes stock-based compensation expense to employees, non-employees and board members over the requisite service period based on the estimated grant-date fair value of the awards and actual forfeitures. The Company accounts for forfeitures as they occur. Stock-based awards with graded vesting schedules are recognized on a straight-line basis over the requisite service period for each separately vesting portion of the award. The Company estimates the fair value of stock option grants using the Black-Scholes option pricing model, and the assumptions used in calculating the fair value of stock-based awards represent management's best estimates and involve inherent uncertainties and the application of management's judgment. All stock-based compensation costs are recorded in general and administrative costs in the statements of operations.

Loss Per Share

Basic net loss per share ("EPS") of common stock is computed by dividing net loss by the weighted average number of shares of common stock outstanding during the period. Diluted EPS reflects the potential dilution that could occur if securities or other contracts to issue common stock were exercised or converted into common stock or resulted in the issuance of common stock that then shared in the earnings of the entity.

Since the Company has net losses, basic and diluted net loss per share is the same. Securities that could potentially dilute loss per share in the future were not included in the computation of diluted loss per share at December 31, 2023 and 2022, because their inclusion would be anti-dilutive are as follows:

	December 31,	
	2023	2022
Series X preferred stock	51,055	51,055
Unvested restricted stock units	86,745	110,453
Common stock warrants	5,681,623	26,204
Convertible notes	—	3,328
Shares settled liability	—	1,824
Total	**5,819,423**	**192,864**

Income Taxes

ASC 740 also provides guidance on derecognition, classification, interest and penalties, accounting in interim periods, disclosure and transition. Based on the Company's evaluation, it has been concluded that there are no significant uncertain tax positions requiring recognition in the Company's financial statements. The Company believes that its income tax positions and deductions would be sustained on audit and does not anticipate any adjustments that would result in material changes to its financial position.

Recent Accounting Pronouncements

In August 2020, the FASB issued ASU No. 2020-06, Debt - Debt with Conversion and Other Options (Subtopic 470-20) and Derivatives and Hedging - Contracts in Entity's Own Equity (Subtopic 815-40): Accounting for Convertible Instruments and Contracts in an Entity's Own Equity, which simplifies accounting for convertible instruments by removing major separation models required under current GAAP. The ASU removes certain settlement conditions that are required for equity contracts to qualify for the derivative scope exception and it also simplifies the diluted earnings per share calculation in certain areas. This ASU is effective for annual reporting periods beginning after December 15, 2021, including interim periods within those fiscal years. Early adoption is permitted, but no earlier than fiscal years beginning after December 15, 2020. This update permits the use of either the modified retrospective or fully retrospective method of transition. The Company adopted this standard on January 1, 2022, and the adoption did not have a material impact on the Company's financial statements or disclosures.

In May 2021, the FASB issued ASU 2021-04, Earnings Per Share (Topic 260), Debt-Modifications and Extinguishments (Subtopic 470-50), Compensation-Stock Compensation (Topic 718), and Derivatives and Hedging-Contracts in Entity's Own Equity (Subtopic 815-40). This ASU reduces diversity in an issuer's accounting for modifications or exchanges of freestanding equity-classified written

call options (for example, warrants) that remain equity classified after modification or exchange. This ASU provides guidance for a modification or an exchange of a freestanding equity-classified written call option that is not within the scope of another Topic. It specifically addresses: (1) how an entity should treat a modification of the terms or conditions or an exchange of a freestanding equity-classified written call option that remains equity classified after modification or exchange; (2) how an entity should measure the effect of a modification or an exchange of a freestanding equity-classified written call option that remains equity classified after modification or exchange; and (3) how an entity should recognize the effect of a modification or an exchange of a freestanding equity-classified written call option that remains equity classified after modification or exchange. This ASU will be effective for all entities for fiscal years beginning after December 15, 2021. An entity should apply the amendments prospectively to modifications or exchanges occurring on or after the effective date of the amendments. Early adoption is permitted, including adoption in an interim period. The Company adopted this standard on January 1, 2022, and the adoption did not have a material impact on the Company's financial statements or disclosures.

In June 2016, the FASB issued ASU 2016-13, Financial Instruments–Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments, and also issued subsequent amendments to the initial guidance: ASU 2018-19, ASU 2019-04, ASU 2019-05, ASU 2020-02 and ASU 2020-03 (collectively, "Topic 32"). Topic 326 significantly changes the impairment model for most financial assets and certain other instruments. Topic 326 will require immediate recognition of estimated credit losses expected to occur over the remaining life of many financial assets, which will generally result in earlier recognition of allowances for credit losses on loans and other financial instruments. The measurement will be based on relevant information, including historical experience, current conditions and reasonable and supportable forecasts that affect the collectability of the reported amount and requires disclosure requirements related to credit risks. ASU 2016-13 is effective for the Company's fiscal year beginning after December 15, 2022 and subsequent interim periods. The Company adopted ASC 2016-13 on January 1, 2023, and the adoption of this standard did not have a material impact on the Company's financial statements and disclosures.

Note 4. Fair Value Measurements

The following table classifies the Company's liabilities measured at fair value on a recurring basis into the fair value hierarchy as of December 31, 2022. As of December 31, 2023, the Company did not have any outstanding liabilities measured at fair value on a recurring basis.

	Fair value measured at December 31, 2022			
	Total carrying value at December 31, 2022	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Liabilities:				
Convertible notes	$ 173,543	$ —	$ —	$ 173,543

During the year ended December 31, 2023, there was a change of approximately $0.2 million in Level 3 liabilities measured at fair value, respectively. There were no liabilities measured at fair value on a recurring basis into the fair value hierarchy as of December 31, 2023.

The fair value of the convertible notes may change significantly as additional data is obtained, impacting the Company's assumptions used to estimate the fair value of the liabilities. In evaluating this information, considerable judgment is required to interpret the data used to develop the assumptions and estimates. The estimates of fair value may not be indicative of the amounts that could be realized in a current market exchange. Accordingly, the use of different market assumptions and/or different valuation techniques may have a material effect on the estimated fair value amounts, and such changes could materially impact the Company's results of operations in future periods.

The following table presents changes in Level 3 liabilities measured at fair value for the year ended December 31, 2023. Unobservable inputs were used to determine the fair value of positions that the Company has classified within the Level 3 category. Unrealized gains and losses associated with liabilities within the Level 3 category include changes in fair value that were attributable to unobservable (e.g., changes in unobservable long-dated volatilities) inputs.

	Convertible Notes
Balance at December 31, 2022	**$ 173,543**
Issuance of convertible notes	1,903,531
Issuance of common stock in connection with conversion of notes payable	(3,195,294)
Repayment of convertible notes	(746,374)
Loss on extinguishment of debt	45,585
Change in fair value	1,819,009
Balance at December 31, 2023	**$ —**

Convertible Notes

During the year ended December 31, 2023, the Company issued the 2023 Note. The fair value of the 2023 Note on September 30, 2023 were estimated using a Monte Carlo simulation to capture the path dependencies intrinsic to their terms. The significant unobservable inputs used in the fair value measurement of the Company's convertible notes are the common stock price, volatility, and risk-free interest rates. Significant changes in these inputs may result in significantly lower or higher fair value measurement. The Company elected the fair value option when recording its 2023 Note (See Note 6) and its convertible notes issued in 2022 (the "2022 Notes"). The notes were classified as liabilities and measured at fair value on the issuance date, with changes in fair value recognized as other income (expense) on the statements of operations and disclosed in the condensed financial statements. During the year ended December 31, 2023, the Company paid off the remaining balance of its 2022 Notes (approximately $0.2 million) with a portion of the proceeds received from the 2023 Note. (see Note 6)

A summary of significant unobservable inputs (Level 3 inputs) used in measuring the 2022 Notes before repayment and the 2023 Note upon the issuance date is as follows:

	February 6, 2023
Dividend yield	— %
Expected price volatility	30.0 - 53.7 %
Risk free interest rate	4.65 - 4.89 %
Expected term (in years)	0.5 - 1.4

On November 29, 2023, the Company satisfied in full the obligations under its outstanding secured convertible promissory note with Lind Global Fund II LP, through a final cash payment of $0.2 million. The satisfaction of the remaining open payment obligations under the Note terminates the Note in full and retires all of the Company's payment obligations thereunder. (See Note 6)

Warrants

On August 3, 2022, the Company entered into a warrant exchange agreement (the "Warrant Exchange Agreement") with a holder of certain warrants to purchase the Company's common stock. The warrants were issued in connection with the Company's 2020 Notes. Pursuant to the Warrant Exchange Agreement, the Company exchanged 2,596 warrants for 1,212 shares of common stock and 1,250 shares of Series X preferred stock at the consummation of the Company's initial public offering (See Note 8).

On August 26, 2022, upon the consummation of the Company's initial public offering, the remaining 28,195 liability classified warrants with a fair value of approximately $0.9 million were determined to be indexed to the Company's common stock, and therefore were reclassified to equity. As of December 31, 2022 and December 31, 2023, there are no liability classified warrants.

A summary of significant unobservable inputs (Level 3 inputs) used in measuring warrants during the year ended December 31, 2022 is as follows:

	December 31, 2022
Dividend yield	0 %
Expected price volatility	0.57% - 105.0 %
Risk free interest rate	.17% - 3.4 %
Expected term (in years)	0.1 - 5.0

Significant changes in the expected price volatility and expected term would result in significantly lower or higher fair value measurement of the warrants, respectively.

Note 5. Accrued Expenses

The Company's accrued expenses as of December 31, 2023 and 2022 consisted of the following:

	December 31, 2023	December 31, 2022
Employee and related expenses	$ 873,349	$ 510,297
Directors and officers insurance	—	269,753
Professional fees	91,750	162,021
Research and development	—	77,000
Total accrued expenses	**$ 965,099**	**$ 1,019,071**

Note 6. Convertible Promissory Notes

During the second and third quarters of 2022, the Company entered into its 2022 Notes with a principal balance totaling approximately $1.5 million. The 2022 Notes contain an original issue discount totaling approximately $0.2 million and the Company received net proceeds of approximately $1.2 million (net of financing fees of approximately $0.1 million). The 2022 Notes bore interest at 10% per annum and mature 12 months from the issuance date. The notes were secured by all assets and personal property of the Company. The note holders had the right to convert all or any portion of the outstanding principal balance and accrued interest into shares of the Company's common stock, up to a beneficial ownership limitation of 9.99% of the number of shares of common stock outstanding at the time of conversion. The per share conversion price was equal to the lessor of (i) $119.00 or (ii) 80% of the qualified offering price of the Company's common stock resulting from the listing for trading of its common stock on a qualified exchange. In connection with the notes, the Company issued common stock warrants to purchase 11,479 shares of the Company's common stock. The warrants had an exercise price of the lessor of (i) $119.00 or (ii) 80% of the qualified offering price and expire five years from the issuance date. As a result of the issuance of the common stock warrants, the exercise price of the Company's existing warrants was adjusted to an exercise of $51.00 per share.

On August 26, 2022, upon the consummation of the Company's initial public offering, the conversion price of the 2022 Notes and the exercise price of the warrants is calculated at 80% of $89.25 per share (the offering price) or $71.40 per share.

On August 3, 2022, the Company entered into a conversion agreement with certain holders of the 2022 Notes, pursuant to which the holders agreed to convert $1.0 million of the principal balance at the consummation of the Company's initial public offering at the conversion price of $71.40 per share. On August 26, 2022, the holders of the 2022 Notes converted the notes fair value of approximately $1.2 million to 14,006 shares of the Company's common stock. During the fourth quarter 2022, the Company recorded a shares settled liability with a fair value of approximately $0.1 million, which was calculated using the Company's closing price of its common stock on the note conversion date, in connection with 1,401 additional shares of common stock to be issued to the noteholders.

On August 3, 2022, the Company entered into a conversion agreement with an additional holder of the 2022 Notes, pursuant to which the holder agreed to convert $255,555 of the principal balance at the consummation of the Company's initial public offering into 2,555 shares of Series X preferred stock. On August 26, 2022, the note holder converted the notes fair value of $0.3 million to 2,555 shares of Series X preferred stock (See Note 8). During the fourth quarter 2022, the Company issued 251 additional shares of Series X preferred stock to the noteholder.

As of December 31, 2022, the outstanding principal balance of the 2022 Notes was approximately $0.2 million. In February 2023, the Company paid down the remaining balance of its 2022 Notes with a portion of the proceeds received from the 2023 Note.

For the year ended December 31, 2022, the Company recorded a loss on issuance of debt of $0.4 million, loss on extinguishment of debt of $0.2 million, and a fair value loss of $0.3 million which is included in the change in fair value of notes in the accompanying statements of operations. The Company recognized interest expense as a component of the change in fair value of the notes during the year ended December 31, 2022 (See Note 3).

The Company issued the 2023 Note in February 2023 with a principal balance of $3.7 million. In connection with the issuance, the Company received proceeds of approximately $3.2 million, net of a $0.5 million discount, incurred fees of approximately $0.5 million and used $0.2 million of proceeds pay down the remaining balance of the Company's 2022 Notes. In connection with the 2023 Note, the Company issued a common stock warrant to purchase 47,059 shares of the Company's common stock.

On November 29, 2023, the Company satisfied in full the obligations under its outstanding secured convertible promissory note with Lind Global Fund II LP, through a final cash payment of $0.2 million. The satisfaction of the remaining open payment obligations under the Note terminates the Note in full and retires all of the Company's payment obligations thereunder.

Note 7. Stock-based Compensation

Restricted Stock Units

Time-based Restricted Stock Units

The following is a summary of the time-based restricted stock units ("RSUs") during the year ended December 31, 2023:

	Number of Shares	Weighted Average Grant-Date Fair Value
Unvested as of December 31, 2022	108,982	$ 81.51
Granted	53,654	27.53
Vested	(77,362)	80.63
Unvested as of December 31, 2023	85,274	$ 48.12

During the year ended December 31, 2022 the Company granted 127,015 RSUs with a fair value of approximately $18.1 million and are subject to service conditions.

During the year ended December 31, 2022, 17,148 RSU's (granted on January 1, 2022) were forfeited due to terminations of two of the Company's employees and two of its board members.

During the year ended December 31, 2023, the Company granted 53,654 RSUs with a fair value of approximately $1.5 million to certain officers, directors, and employees, and are subject to service conditions.

During the year ended December 31, 2023, the Company recorded stock-based compensation expense related to the RSUs of approximately $5.0 million. During the year ended December 31, 2022, the Company recorded stock-based compensation expense related to the RSUs of approximately $3.9 million. The unamortized stock-based compensation expense related to RSUs as of December 31, 2023 is approximately $1.5 million, which is expected to be recognized over a remaining weighted average vesting period of 0.92 years.

During the years ended December 31, 2023 and 2022, the Company recorded stock-based compensation expense related to canceled stock options of approximately $0.2 million and $0.5 million, respectively. The unamortized stock-based compensation expense related to canceled stock options (as noted below), as of December 31, 2023 is less than $0.1 million.

Performance-based Restricted Stock Units

On October 11, 2022, the Company granted 1,471 performance-based restricted stock units with a fair value of approximately $60,000 for consulting services. The RSUs are subject to a performance condition, and will vest upon the Company signing a definitive agreement with a strategic partner.

Canceled Stock Options

On December 22, 2020, the Company granted 52,741 RSUs with a fair value of approximately $6.3 million to its officers and directors, in exchange for 46,324 vested and unvested stock options. The RSUs are subject to service conditions (vesting of 33.34% on May 1, 2021, with the remaining units vesting on each three-month anniversary, thereafter, fully vesting on May 1, 2023) and performance conditions in the form of a liquidity event. Vesting of the RSUs is subject to all grantees continuous service with the Company, and no vesting shall occur if the Company has not completed a Qualified Offering or a Change of Control on or before the vesting date. Pursuant to the guidance of ASC 718- "Compensation - Stock Compensation". The exchange of the options for the RSUs was accounted for as a probable (service only vesting) to improbable (performance and service with the performance criteria considered improbable at grant date since contingent vesting is upon a Qualified Offering or Change of Control) modification. As such, compensation cost for the original awards would be recognized if the awards would have vested pursuant to the original terms. Since the original awards were modified, the incremental cost would be measured as the result of modification; that is, the fair value of the options after the modification

to increase the exercise price to $152.66. This fair value would be compared to the fair value of the RSUs to determine the incremental compensation cost. Incremental compensation cost of approximately $1.9 million, related to the replacement awards would be recognized only if the modified vesting criteria are achieved. The Company did not satisfy the performance conditions to achieve vesting of the exchanged RSUs. As a result, the company did not recognize any incremental compensation cost associated with the exchanged RSUs. Compensation cost related to the exchanged stock options of $4.3 million will continue to be recognized over the original vesting criteria.

The Company previously granted options to purchase shares of the Company's common stock and during the year ended December 31, 2020 these options were exchanged (See above). No stock options were outstanding as of December 31, 2023 and December 31, 2022. Compensation cost related to the canceled stock options continues to be recognized over the original vesting criteria.

During the years ended December 31, 2023 and 2022, the Company recorded stock-based compensation expense related to these canceled stock options of approximately $0.2 million and $0.5 million, respectively. The unamortized stock-based compensation expense related to canceled stock options, as of December 31, 2023 is less than $0.1 million.

Note 8. Stockholders' Deficit

Amendment to Certificate of Incorporation

On August 30, 2022, the Company filed an amendment (the "Amendment") to its certificate of incorporation (the "Certificate") with the Secretary of State of the State of Delaware in connection with the completion of the Company's initial public offering. The Amendment amends the Company's Certificate to, among other things: (i) authorize 200,000,000 shares of common stock and (ii) authorize 10,000,000 shares of preferred stock, 500,000 of which are designated as Series X Preferred Stock. In connection with the initial public offering, the Board waived any lock-up restrictions contained in the Series X Certificate of Designations.

Common Stock

Year Ended December 31, 2023

On November 17, 2022, the Company entered into an equity purchase agreement (the "Purchase Agreement") with LPC which provided that, upon the terms and subject to the conditions and limitations set forth therein, the Company could sell to LPC, at its discretion, up to $20.0 million of shares of its common stock over the 30-month term of the Purchase Agreement. In the year ended December 31, 2023, the Company issued approximately 0.2 million shares of common stock, receiving net proceeds of approximately $5.6 million.

In the year ended December 31, 2023, the Company issued 78,245 shares of its common stock in connection with the vesting of RSUs issued to members of the Company's board of directors and employees. The Company withheld 7,207 of these shares at a fair value of approximately $0.1 million, to cover the withholding taxes related to the settlement of these vested restricted stock units.

In the year ended December 31, 2023, the Company issued 1,824 shares of its common stock in settlement of a shares settled liability at December 31, 2022 of approximately $161,000.

On November 22, 2023, the Company issued 5,384,615 in common stock and pre-funded warrants associated with it secondary public offering. The Company received net proceeds from its secondary public offering of approximately $6.1 million, net of underwriter fees and commissions of approximately $0.6 million, and offering costs of approximately $0.3 million. In connection with its public offering the Company issued 5,384,615 warrants to purchase shares of the Company's common stock with an exercise price of $1.30 per share and issued 215,385 warrants to purchase shares of the Company's common stock with an exercise price of $1.625.

Year Ended December 31, 2022

On August 5, 2022, the Company entered into exchange agreements with the holders of the Company's Series Seed preferred stock, par value $0.0001 per share. The Company and the holders exchanged all shares of outstanding Series Seed preferred stock into 91,614 shares of common stock immediately prior to the effectiveness of its registration statement filed in connection with the Company's initial public offering.

On August 9, 2022, the Company entered into an underwriting agreement relating to the public offering of its common stock, par value $0.0001 per share. The Company agreed to sell 90,909 shares of its common stock to the underwriters, at a purchase price per share of $82.11 (the offering price to the public of $89.25 per share minus the underwriters' discount), pursuant to the Company's registration statement on Form S-1 (File No. 333-239676), as amended, under the Securities Act of 1933, that was filed by the Company under Rule 462(b) under the Securities Act. On August 30, 2022, the Company received net proceeds from its public offering of approximately $6.0 million, net of underwriter fees and commissions of approximately $0.8 million, and offering costs of approximately $1.4 million. In connection with its public offering the Company issued 6,364 warrants to purchase shares of the Company's common stock with an exercise price of $116.88 per share.

On November 17, 2022, the Company entered into the Purchase Agreement with LPC, which provided that, upon the terms and subject to the conditions and limitations set forth therein, the Company could sell to LPC, at its discretion, up to $20.0 million of shares of its common stock over the 30-month term of the Purchase Agreement. Upon execution of the Purchase Agreement, the Company issued 11,705 shares of its common stock to LPC as commitment shares in accordance with the closing conditions contained within the Purchase Agreement. The commitment shares were valued using the closing price of the Company's common stock on the effective date of the Purchase Agreement resulting in an aggregate fair value of $0.3 million. The Company paid a $35,000 upfront fee to LPC and $195,997 in legal fees related to the issuance of the Purchase Agreement which are included in additional paid-in capital on the accompanying balance sheet. During the year ended December 31, 2022, the Company did not issue any shares of common stock in connection with the Purchase Agreement.

During the year ended December 31, 2022, the Company recorded a shares settled liability with a fair value of approximately $0.2 million in connection with 1,401 shares of common stock to be issued in connection with the conversion of notes payable (See Note 7), and 413 shares of common stock to be issued for consulting services. The fair value of the shares settled liability was determined using the Company's closing price of its common stock on the note conversion date, and the date of the consulting agreement.

During the year ended December 31, 2022 the Company issued 3,362 shares of its common stock with a fair value of $0.1 million in connection with consulting services.

As of December 31, 2022, the Company had 707,976 shares of common stock issued and outstanding.

Series X Preferred Stock

On August 1, 2022, the Company authorized 500,000 shares of Series X preferred stock, par value 0.0001 per share. The stated value of the Series X preferred stock is $100 per share. The holders of the Series X preferred stock have no voting rights and are not entitled to dividends. The Series X preferred stock is convertible into shares of the Company's common stock and is subject to a beneficial ownership limitation of 9.99% of the number of shares of common stock outstanding at the time of conversion.

On August 2, 2022, the Company issued 3,200 shares of Series X preferred stock in a private placement, at a purchase price of $100 per share, and received net proceeds of approximately $0.3 million, after deducting expenses (the "Series X Private Placement"). The Series X Private Placement constituted a qualified offering under the terms of the SAFE and the $5.0 million outstanding under the SAFE automatically converted into 100,000 shares of Series X preferred stock (See Note 6).

During the year ended December 31, 2022 the Company issued 2,806 shares of its Series X preferred stock in connection with the conversion of certain 2022 Notes (See Note 7). The 2,806 shares of Series X preferred stock are convertible into shares of common stock at the initial offering price of $89.25 per share, subject to the beneficial ownership limitation.

On the August 26, 2022, in connection with its Warrant Exchange Agreement (See Note 3), the Company exchanged 44,118 warrants for 20,589 shares of its common stock and 1,250 shares of its Series X preferred stock.

On August 26, 2022, upon the consummation of the Company's initial public offering, 61,689 shares of the Series X preferred stock were converted into 69,118 shares of the Company's common stock. As of December 31, 2022, 45,567 shares of Series X preferred stock remain outstanding.

Note 9. Income Taxes

Loss before income tax expense for the years ended December 31, 2023 and 2022, respectively, consisted of the following: (in thousands)

Domestic and foreign income before provision for income tax	**2023**	**2022**
Domestic	$ (18,290)	$ (14,802)
Foreign	—	—
Total	**$ (18,290)**	**$ (14,802)**

The Company had no income tax expense due to operating losses incurred for the years ended December 31, 2023 and 2022.

As of December 31, 2023, the Company had Federal and State NOL carryforwards of approximately $22.2 million and 14.6 million , respectively.

The Federal NOL carryforwards do not expire but the State NOL carryforwards expire if not utilized prior to 2043. The Company had Federal research and development tax credit carryforwards of approximately $29.0 thousand as of December 31, 2023, which expire at various dates through 2042.

As required by ASC 740, management of the Company has evaluated the evidence bearing upon the realizability of its deferred tax assets. Based on the weight of available evidence, both positive and negative, management has determined that it is more likely than not that the Company will not realize the benefits of these assets. Accordingly, the Company recorded a valuation allowance of $7.2 million at December 31, 2023. The valuation allowance increased by $2.4 million during the year ended December 31, 2023, primarily as a result of the increase in net operating loss carryforwards generated in the current year.

Utilization of the U.S. federal and state net operating loss and research and development credit carryforwards may be subject to a substantial annual limitation under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, due to ownership changes that have occurred previously or that could occur in the future. These ownership changes may limit the amount of net operating loss and research and development credit carryforwards that can be utilized annually to offset future taxable income and tax liabilities, respectively. The Company has not completed a study to assess whether a change of ownership has occurred, or whether there have been multiple ownership changes since its formation, due to the significant cost and complexity associated with such a study. Any limitation may result in expiration of a portion of the net operating loss carryforwards or research and development credit carryforwards begore utilization. Further, until a study is completed by the Company and any limitation is known, no amounts are being presented as an uncertain tax position.

The principal components of the Company's deferred tax assets and liabilities consist of the following at December 31, 2023 and 2022 (in thousands)

Tax effected significant temporary differences	**For the years ended December 31, 2023**	**2022**
Net operating loss carryforwards	$ 5,413	$ 3,687
Research & development credits	29	29
Equity based compensation	474	667
Capitalized research	1,106	447
Accrued expenses	186	—
Total deferred tax assets	7,208	4,830
less valuation allowance	(7,208)	(4,830)
Net deferred tax asset	**$ —**	**$ —**

A reconciliation of the statutory income tax rates and the Company's effective tax rate for the year ended December 31, 2023 and December 31, 2022 are as follows:

Rate Reconciliation	For the years ended December 31, 2023		2022	
Income (loss) before taxes	$ (18,290)		$ (14,802)	
Tax expense (benefit) at the statutory rate	(3,841)	21.00%	(3,108)	21.00%
State income taxes, net of federal benefit	(34)	0.20%	(737)	5.00%
Stock Compensation	800	(4.50)%	261	(1.80)%
Debt Conversion	392	(2.10)%	1,155	(7.80)%
Permanent differences	39	(0.20)%	105	(0.70)%
Non-deductible executive compensation	75	(0.40)%	—	0.00%
Difference and changes in tax rates	207	(1.10)%	—	0.00%
RTP and Other	(16)	0.10%	94	(0.70)%
Warrant Liability	—	0.00%	(393)	2.70%
Change in valuation allowance	2,378	(13.00)%	2,623	(17.70)%
Tax on Income	**$ —**	**0.00%**	**$ —**	**0.00%**

As of December 31, 2023 and 2022, the Company had no uncertain tax positions. The Company's policy is to recognize interest and penalties that would be assessed in relation to the settlement value of unrecognized tax benefits as a component of income tax expense. The Company did not accrue either interest or penalties for the years ended December 31, 2023 and 2022.

The Company files income tax returns in the United States federal tax jurisdiction and various state jurisdictions, including New York and New Jersey. The Company did not have any foreign operations during the year ended December 31, 2023. The statute of limitations for assessment by the Internal Revenue Service and state tax authorities is open since inception, and in addition, carryforward tax attributes generated since inception may be adjusted upon examination to the extent utilized in a future period. The Company is not aware of any tax examinations currently taking place.

Note 10. Commitments and Contingencies

Litigation

As of December 31, 2023 and 2022, there was no litigation against the Company. The Company may be involved in legal proceedings, claims and assessments arising from the ordinary course of business. Such matters are subject to many uncertainties, and outcomes are not predictable with assurance.

Note 11. Related Party Transactions

TardiMed Sciences - Tardimed is a startup venture investment and operating firm in the life sciences space. The former Chairman of the Board of the Company is also a Managing Member of TardiMed. The former Chief Operating Officer is an employee of TardiMed. As of December 31, 2023 TardiMed holds 431,574 shares of the Company's common stock which represents 5.85% of the total voting shares outstanding.

Expenses - During the years ended December 31, 2023 and 2022, the Company expensed $240,000 for management fees owed to Tardimed. In addition, during the year ended December 31, 2023, the company entered into a Consulting Services agreement with Tardimed for regulatory support to gain registration of PAX-101 and expensed $82,500 in fees owed to Tardimed for the Rent and Administrative Services agreement.

Accounts payable – As of December 31, related party payables totaled $18,500, owed to members of our board of directors.

Prepaids – As of December 31, 2023, the Company paid $0.1 million for services to be performed by PoloMar, a related party due to their ownership by Tardimed.

Note 12. Subsequent Events

The Company has evaluated all subsequent events that occurred after the balance sheet date through the date when the financial statements were issued. Except as described below, the Company has concluded that no subsequent event has occurred that require disclosure within these financial statements.

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE.

We have not had any disagreements with our accountants or auditors that would need to be disclosed pursuant to Item 304 of Regulation S-K promulgated under the Securities Act of 1933.

ITEM 9A. DISCLOSURE CONTROLS AND PROCEDURES.

Evaluation of Disclosure Controls and Procedures

Disclosure controls are procedures that are designed with the objective of ensuring that information required to be disclosed in our reports filed under the Exchange Act, such as this Report, is recorded, processed, summarized, and reported within the time period specified in the SEC's rules and forms. Disclosure controls are also designed with the objective of ensuring that such information is accumulated and communicated to our management, including the chief executive officer and chief financial officer, as appropriate to allow timely decisions regarding required disclosure. Management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Exchange Act Rules 13a-15(f) and 15d-15(f).

Our management evaluated, with the participation of our principal executive officer and principal financial and accounting officer (our "Certifying Officers"), the effectiveness of our disclosure controls and procedures as of December 31, 2023, pursuant to Rule 13a-15(b) under the Exchange Act. Based upon that evaluation, our Certifying Officers concluded that, as of December 31, 2023, our disclosure controls and procedures were not effective, because of material weaknesses in our internal controls.

A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of the Company's annual or interim financial statements will not be prevented or detected on a timely basis.

Material Weaknesses in Internal Controls

The Company's management has concluded that our control around the accounting for certain complex features of financial instruments and earnings per share was not effectively designed or maintained, and therefore not accounted for correctly. As a result, our management performed additional analysis as deemed necessary to ensure that our financial statements were prepared in accordance with accounting principles generally accepted in the United States of America. Management understands that the accounting standards applicable to our financial statements are complex and has since the inception of the Company benefited from the support of experienced third-party professionals with whom management has regularly consulted with respect to accounting issues. Management intends to continue to further consult with such professionals in connection with accounting matters.

In addition to the material weaknesses identified above, we also did not design and maintain effective controls over information technology (IT) general controls for information systems that are relevant to the preparation of our financial statements, specifically, with respect to user provisioning and deprovisioning, user access review, passwords, privileged access, cybersecurity, system development lifecycle, and SOC report management review. These IT deficiencies did not result in a misstatement to our financial statements, however, the deficiencies, when aggregated, could impact the effectiveness of IT-dependent controls that could result in misstatements potentially impacting all financial statement accounts and disclosures that would not be prevented or detected. Accordingly, management has determined that these deficiencies in the aggregate constitute a material deficiency. To remediate the above material weaknesses, we have developed a remediation plan with assistance from our IT advisors and have dedicated significant resources and efforts to the remediation and improvement of our internal control over financial reporting.

Management's Annual Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate controls over financial reporting as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act. As required by Rule 13a-15(c) promulgated under the Exchange Act, our management, with the participation of our Certifying Officers, evaluated the effectiveness of our internal control over financial reporting as of December 31, 2023. Management's assessment was based on criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission in Internal Control - Integrated Framework (2013 Framework). Based on management's assessment, management has concluded that our internal control over financial reporting was not effective as of December 31, 2023 due to the material weakness in our internal control over financial reporting as described above.

Changes in Internal Control over Financial Reporting

Other than with respect to the remediation efforts described in the remediation plan for the material weakness above, there was no change in our internal control over financial reporting that occurred during the fiscal quarter ended December 31, 2023 covered by this report on Form 10-K that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION.

During the three months ended December 31, 2023, none of our directors or officers adopted, modified or terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement," as each term is defined in Item 408(a) of Regulation S-K.

ITEM 9C. DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS.

Not applicable.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE.

Management

The information required by this item will be included in our Proxy Statement to be filed with the SEC and is incorporated herein by reference.

Code of Business Conduct and Ethics

We have adopted a written code of business conduct and ethics that applies to our employees, officers and directors. A current copy of the code will be posted on the Corporate Governance section of our website, which will be located at www.paxmedica.com. We intend to disclose future amendments to certain provisions of our code of business conduct and ethics, or waivers of such provisions applicable to any principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions, and our directors, on our website identified above or in filings with the SEC.

ITEM 11. EXECUTIVE COMPENSATION.

The information required by this item will be included in our Proxy Statement to be filed with the SEC and is incorporated herein by reference.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS.

The information required by this item will be included in our Proxy Statement to be filed with the SEC and is incorporated herein by reference.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS.

The information required by this item will be included in our Proxy Statement to be filed with the SEC and is incorporated herein by reference.

ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES.

The information required by this item will be included in our Proxy Statement to be filed with the SEC and is incorporated herein by reference.

PART IV

ITEM 15. EXHIBITS, FINANCIAL STATEMENT SCHEDULES.

The information required by this item will be included in our Proxy Statement to be filed with the SEC and is incorporated herein by reference.

(b)

Exhibits.

Exhibit No.	Exhibit Description
3.1	Certificate of Incorporation of PaxMedica, Inc., as amended (incorporated by reference to Exhibit 3.1 to Amendment No. 10 to Form S-1 filed on August 8, 2022).
3.2	Amendment to Certificate of Incorporation of PaxMedica, Inc. (incorporated by reference to Exhibit 3.1 to the Current Report on Form 8-K filed on August 30, 2022).
3.3	Certificate of Designations, Preferences and Rights of Series X Convertible Preferred Stock of PaxMedica, Inc. (incorporated by reference to Exhibit 3.3 to Amendment No. 10 to Form S-1 filed on August 8, 2022).
3.4	Amended and Restated Bylaws of PaxMedica, Inc. (incorporated by reference to Exhibit 3.2 to the Current Report on Form 8-K filed on August 30, 2022).
3.5	Certificate of Amendment to the Certificate of Incorporation of PaxMedica, Inc. (incorporated by reference to Exhibit 3.1 on the Current Report on Form 8-K filed on October 30, 2023).
4.1*	Description of the Registrant's Securities.
4.2	Specimen Certificate representing shares of common stock of PaxMedica, Inc. (incorporated by reference to Exhibit 4.1 to Amendment No. 10 to the Registration Statement on Form S-1 filed on August 8, 2022).
4.3	Form of 2022 Warrant (incorporated by reference to Exhibit 4.2 to Amendment No. 10 to the Registration Statement on Form S-1 filed on August 8, 2022).
4.4	Form of Representative Warrant (incorporated by reference to Exhibit 4.3 to Amendment No. 10 to the Registration Statement on Form S-1 filed on August 8, 2022).
4.5	Form of 2020 Warrant (incorporated by reference to Exhibit 4.4 to Amendment No. 10 to the Registration Stateme nt on Form S-1 filed on August 8, 2022).
4.6	Warrant, dated February 6, 2023, issued by PaxMedica, Inc. to Lind Global Fund II LP (incorporated by reference to Exhibit 4.2 to the Current Report on Form 8-K filed on February 7, 2023).
4.7	Warrant, dated February 6, 2023, issued by PaxMedica, Inc. to Lind Global Fund II LP (incorporated by reference to Exhibit 4.2 to the Current Report on Form 8-K filed on February 7, 2023).
4.8	Form of Pre-Funded Warrant (incorporated by reference to Exhibit 4.8 to the Registration Statement on Form S-1 filed on November 9, 2023).
4.9	Form of Placement Agent Warrant (incorporated by reference to Exhibit 4.9 to the Registration Statement on Form S-1 filed on November 9, 2023).
10.1	PaxMedica, Inc. Amended and Restated 2020 Omnibus Equity Incentive Plan (incorporated by reference to Exhibit 10.2 to Amendment No. 10 to the Registration Statement on Form S-1 filed on August 8, 2022).†
10.2	Form of Indemnification Agreement entered into by PaxMedica, Inc. with its Officers and Directors (incorporated by reference to Exhibit 10.1 to Amendment No. 10 to the Registration Statement on Form S-1 file on August 8, 2022)†
10.3	Form of Nonqualified Stock Option Award under the Amended and Restated 2020 Omnibus Equity Incentive Plan (incorporated by reference to Exhibit 10.3 to Amendment No. 10 to the Registration Statement on Form S-1 filed on August 8, 2022).†

10.4	Form of Incentive Stock Option Award under the Amended and Restated 2020 Omnibus Equity Incentive Plan (incorporated by reference to Exhibit 10.4 to Amendment No. 10 to the Registration Statement on Form S-1 filed on August 8, 2022).†
10.5	Form of Restricted Stock Unit Grant Agreement under the Amended and Restated 2020 Omnibus Equity Incentive Plan (incorporated by reference to Exhibit 10.13 to Amendment No. 10 to the Registration Statement on Form S-1 filed on August 8, 2022).†
10.6	Form of 2022 Convertible Promissory Note (incorporated by reference to Exhibit 10.15 to Amendment No. 10 to the Registration Statement on Form S-1 filed on August 8, 2022).
10.7	Form of 2022 Convertible Promissory Note Securities Purchase Agreement (incorporated by reference to Exhibit 10.16 to Amendment No. 10 to the Registration Statement on Form S-1 filed on August 8, 2022).
10.8	Purchase Agreement, dated as of November 17, 2022, by and between the Company and Lincoln Park (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed on November 21, 2022).
10.9	Registration Rights Agreement, dated as of November 17, 2022, by and between the Company and Lincoln Park (incorporated by reference to Exhibit 10.2 to the Current Report on Form 8-K filed on November 21, 2022).
10.10	Employment Agreement, dated as of November 19, 2022, between the Company and Stephen D. Sheldon (incorporated by reference to Exhibit 10.3 to the Current Report on Form 8-K filed on November 21, 2022). †
10.11	Employment Agreement, dated as of January 1, 2023, between the Company and Howard J. Weisman (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed on January 6, 2023). †
10.12	Securities Purchase Agreement, dated as of February 2, 2023, between PaxMedica, Inc. and Lind Global Fund II LP (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed on February 7, 2023).
10.13	Security Agreement, dated as of February 6, 2023, between PaxMedica, Inc. and Lind Global Fund II LP (incorporated by reference to Exhibit 10.2 to the Current Report on Form 8-K filed on February 7, 2023).
10.14	Letter Agreement between PaxMedica, Inc. and Zachary Rome, dated June 25, 2020 (incorporated by reference to Exhibit 10.6 to Amendment No. 10 to the Registration Statement on Form S-1 filed on August 8, 2022).†
10.15	Letter Agreement between PaxMedica, Inc. and Michael Derby, dated June 25, 2020 (incorporated by reference to Exhibit 10.7 to Amendment No. 10 to the Registration Statement on Form S-1 filed on August 8, 2022).†
10.16	Rent and Administrative Services Agreement between PaxMedica, Inc. and TardiMed LLC, dated July 1, 2020 (incorporated by reference to Exhibit 10.9 to Amendment No. 10 to the Registration Statement on Form S-1 filed on August 8, 2022).
10.17	Patient Records License Agreement between Purinix Pharmaceuticals LLC and Lwala Hospital, dated November 9, 2018 (incorporated by reference to Exhibit 10.10 to Amendment No. 10 to the Registration Statement on Form S-1 filed on August 8, 2022).‡
10.18	Patient Records License Agreement between Purinix Pharmaceuticals LLC and Ministry of Health, Republic of Malawi, dated October 10, 2018 (incorporated by reference to Exhibit 10.11 to Amendment No. 10 to the Registration Statement on Form S-1 filed on August 8, 2022).‡
10.19	Master Services Agreement between Purinix Pharmaceuticals LLC and CRO Consulting (Pty) Limited, dated May 25, 2018 (incorporated by reference to Exhibit 10.12 to Amendment No. 10 to the Registration Statement on Form S-1 filed on August 8, 2022).‡
10.20	Amendment to Rent and Administrative Services Agreement between PaxMedica, Inc. and TardiMed LLC, dated November 1, 2020 (incorporated by reference to Exhibit 10.14 to Amendment No. 10 to the Registration Statement on Form S-1 filed on August 8, 2022).

10.21	Letter Agreement between PaxMedica, Inc. and Howard J. Weisman, dated March 4, 2020 (incorporated by reference to Exhibit 10.5 to Amendment No. 10 to the Registration Statement on Form S-1 filed on August 8, 2022).†
10.22	Specialty Benefit Manager Agreement, effective as of June 30, 2023, by and between PaxMedica, Inc. and Vox Nova,LLC (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed on August 9, 2023).
10.23	Employment Agreement, dated as of August 16, 2023, between the Company and David W. Hough (incorporated by reference to Exhibit 10.23 to the Registration Statement on Form S-1 filed on November 9, 2023).
10.24	Form of Securities Purchase Agreement (incorporated by reference to Exhibit 10.25 to the Registration Statement on Form S-1 filed on November 9, 2023).
10.25*	Research Collaboration Agreement, dated as of June 7, 2023, by and between the Company and PoloMar Health
23.1*	Consent of Independent Registered Public Accounting Firm
24.1*	Power of Attorney (included on signature page).
31.1*	Certification of Chief Executive Officer (Principal Executive Officer) Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Chief Financial Officer (Principal Financial and Accounting Officer) Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1*	Certification of Chief Executive Officer (Principal Executive Officer) Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2*	Certification of Chief Financial Officer (Principal Financial and Accounting Officer) Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
97*	Form of Clawback Policy
101.INS	Inline XBRL Instance Document.
101.SCH	Inline XBRL Taxonomy Extension Schema Document.
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document.
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document.
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document.
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document.
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101).

* Filed herewith

† Indicates management contract or compensatory plan.

‡ Certain portions of this exhibit have been omitted because the omitted information is (i) not material and (ii) would likely cause competitive harm to the Company if publicly disclosed.

ITEM 16. FORM 10-K SUMMARY.

None.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this Annual Report on Form 10-K to be signed on its behalf by the undersigned, thereunto duly authorized.

Date: March 11, 2024

PAXMEDICA, INC.

By: /s/ Howard J. Weisman
Howard J. Weisman
Chairman of the Board and Chief Executive Officer
(Principal Executive Officer)

Date: March 11, 2024

By: /s/ Stephen Sheldon
Stephen Sheldon
Chief Financial Officer and Chief Operating Officer
(Principal Financial Officer and
Principal Accounting Officer)

POWER OF ATTORNEY

KNOW ALL BY THESE PRESENTS, that the undersigned officers and/or directors of the Registrant, by virtue of their signatures to this report, appearing below, hereby constitute and appoint Howard J. Weisman and Stephen Sheldon, or any one of them, with full power of substitution, as attorneys-in-fact in their names, places and steads to execute any and all amendments to this report in the capacities set forth opposite their names and hereby ratify all that said attorneys-in-fact do by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the Registrant and in the capacities and on the dates indicated.

Signature	Title	Date
/s/ Howard J. Weisman Howard J. Weisman	Chairman of the Board and Chief Executive Officer (*Principal Executive Officer*)	March 11, 2024
/s/ Stephen D. Sheldon Stephen D. Sheldon	Chief Financial Officer & Chief Operating Officer (Principal Financial and Accounting Officer)	March 11, 2024
/s/ Zachary Rome Zachary Rome	Director	March 11, 2024
/s/ Karen LaRochelle Karen LaRochelle	Director	March 11, 2024
/s/ John F. Coelho John F. Coelho	Director	March 11, 2024
/s/ Charles J. Casamento Charles J. Casamento	Director	March 11, 2024