

Poolbeg Pharma plc

Exclusive Option Agreement to Acquire Orphan Drug Candidate for Behçet's Disease

Novel therapeutic with Fast Track and Orphan Designation

30 April 2024 - Poolbeg Pharma (AIM: POLB, OTCQB: POLBF, 'Poolbeg' or the 'Company'), a biopharmaceutical company focussed on the development and commercialisation of innovative medicines targeting diseases with a high unmet medical need, announces that it has entered into an exclusive 12-month option agreement with Silk Road Therapeutics Inc, for a nominal fee, to acquire a novel topical muco-adherent formulation of Pentoxifylline (tPTX) for the treatment of oral ulcers in patient's suffering from Behçet's Disease.

Key Highlights

- 12-month option agreement with Silk Road Therapeutics to acquire novel topical muco-adherent formulation of Pentoxifylline (tPTX), a treatment for oral ulcers in patient's suffering from Behçet's Disease which currently has no cure
- Phase 2 trial successfully completed, demonstrating superiority over standard of care
- Secured Orphan Drug Designation and Fast Track Designation from the FDA
- Positioned for a potential 505(b)(2) approval pathway in the U.S.
- Poolbeg will continue due diligence throughout the term of the exclusive option agreement which will include engagement with Silk Road Therapeutics to ascertain the clinical approval pathway

There is a clear unmet medical need for an effective treatment for this rare disease, which has no cure. Behçet's Disease causes inflammation of blood vessels and tissues, resulting in debilitating symptoms, the most common being oral ulcers which impact essential functions like eating, drinking and speaking. ^[1] Patients also suffer from reduced psychosocial quality of life ^[2]. Current standard of care is inadequate with safety concerns and there is a clear need for alternative treatment options.

The topical formulation of PTX, a small molecule with broad anti-inflammatory activity, has successfully demonstrated accelerated oral ulcer healing and decreased pain compared to standard of care in patients with Behçet's Disease in a Phase 2 clinical trial.

The drug is positioned for a potential 505(b)(2) approval pathway in the U.S. which can result in a much less expensive and much faster route to approval and commercialisation. tPTX has successfully secured FDA Fast Track Designation, designed to expedite development and review of certain medicines. It has also received Orphan Drug Designation from the FDA, providing seven years of market exclusivity upon marketing authorisation.

Jeremy Skillington, PhD, Chief Executive Officer of Poolbeg Pharma, said: "The foundation of any pharma company is a strong pipeline of assets, and we believe that topical PTX could fit well into our pipeline alongside our other assets as we increase our focus on rare and orphan diseases.

"We are delighted to enter this exclusive option agreement with Silk Road Therapeutics and Dr Timothy Coté, whose extensive experience, including as Director of the FDA Office of Orphan Products Development, adds invaluable insights into the regulatory pathway and ultimate approval and commercialisation of the drug.

"tPTX has the potential to transform the lives of patients suffering from Behçet's Disease, who currently lack effective and safe long term treatment options. Under this exclusive option agreement, we will advance our due diligence process and engage with Silk Road Therapeutics to further understand the clinical pathway to approval, and we look forward to updating the market in due course."

Timothy R. Coté, M.D., M.P.H., Chief Executive Officer of Silk Road Therapeutics, said: "Poolbeg Pharma shares our commitment to advancing healthcare innovation to support rare disease patients. With Fast Track Designation and Orphan Drug Designation secured, topical PTX has the potential to swiftly reach patients and offer a potentially transformative solution for those suffering from Behçet's Disease."

Investor presentation

Poolbeg Pharma CEO, Jeremy Skillington, PhD, and CBO David Allmond, along with Dr Timothy Coté, CEO of Silk Road Therapeutics, will provide a live presentation via the Investor Meet Company platform on **Tuesday 30 April 2024 at 6pm BST**.

The presentation is open to analysts and investors, those who already follow Poolbeg on the Investor Meet Company platform will automatically be invited. Investors can sign up to Investor Meet for free and add Poolbeg Pharma plc to their company dashboard via: <https://www.investormeetcompany.com/poolbeg-pharma-plc/register-investor>

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About Behçet's Disease

Behçet's Disease is a rare condition that results in inflammation of the blood vessels and tissues. Inflammation across multiple parts of the body leads to diverse symptoms, the most prevalent being recurrent oral ulcers, in addition to genital ulcers and inflammation of the eye and skin lesions. It is not yet known what triggers the disease or causes it to persist. However, it is clear there is a significant immune component with some suggestions that exaggerated responses to pathogens and increased cytokine and chemokine production may play key roles in the disease.^[3]

About Poolbeg Pharma

Poolbeg Pharma plc is committed to the development and commercialisation of innovative medicines targeting diseases with a high unmet medical need, with a growing emphasis on rare and orphan diseases. Its model focusses upon developing its exciting clinical assets and commercialising approved and marketed drugs to support the growth of the Company and the development of its robust pipeline of innovative products, thereby driving significant value creation.

Poolbeg is led by an experienced leadership team with a history of delivering significant shareholder value. The team has been strengthened by the appointment of three former members of the Amryt Pharma plc leadership team, with the intention of repeating Amryt's success and generating near term revenues.

Poolbeg's clinical programmes target large addressable markets including cancer immunotherapy-induced CRS, infectious disease, and metabolic conditions such as obesity with the development of an oral GLP-1R agonist. It uses a cost-effective development philosophy to generate high quality human data to support partnering and further development. Its AI-led infectious disease programmes analyse unique data from human challenge trials to identify clinically relevant drug targets and treatments, leading to faster development and greater commercial appeal.

For more information, please go to www.poolbegpharma.com or follow us on [Twitter](#) and [LinkedIn](#) @PoolbegPharma.

Forward-Looking Statements

This announcement may contain forward-looking statements and the words "expect", "anticipate", "intends", "plan", "estimate", "aim", "forecast", "project" and similar expressions (or their negative) identify certain of these forward-looking statements. The forward-looking statements in this announcement are based on numerous assumptions and Poolbeg's present and future business strategies and the environment in which Poolbeg expects to operate in the future. Forward-looking statements involve inherent known and unknown risks, uncertainties and contingencies because they relate to events and depend on circumstances that may or may not occur in the future and may cause the actual results, performance or achievements to be materially different from those expressed or implied by such forward-looking statements. These statements are not guarantees of future performance or the ability to identify and consummate investments. Many of these risks and uncertainties relate to factors that are beyond Poolbeg's ability to control or estimate precisely, such as future market conditions, currency fluctuations, the behaviour of other market participants, the outcome of clinical trials, the actions of regulators and other factors such as Poolbeg's ability to obtain financing, changes in the political, social and regulatory framework in which Poolbeg operates or in economic, technological or consumer trends or conditions. Past performance should not be taken as an indication or guarantee of future results, and no representation or warranty, express or implied, is made regarding future performance. No person is under any obligation to update or keep current the information contained in this announcement or to provide the recipient of it with access to any additional relevant information.

[1] Hatemi G, Yurttas B, Kutlubay Z, Cote T, Derkunt S, Yazici Y, Yazici H. Pentoxifylline Gel for Oral Ulcers in Patients with Behçet's Syndrome [abstract]. Arthritis Rheumatol. 2019; 71 (suppl 10). <https://acrabstracts.org/abstract/pentoxifylline-gel-for-oral-ulcers-in-patients-with-behcets-syndrome/>. Accessed April 17, 2024.

[2] Ali S, Nagieb CS, Fayed HL. Effect of Behcet's disease-associated oral ulcers on oral health related quality of life. Spec Care Dentist. 2023 Jul-Aug;43(4):435-442. doi:

10.1111/scd.12782. Epub 2022 Sep 20. PMID: 36127751.

[3] behcetsuk.org

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