RNS Number : 9473V
Faron Pharmaceuticals Oy
11 July 2024

Faron Pharmaceuticals Ltd.

("Faron" or the "Company")

Inside Information: Faron Announces Positive FDA Feedback

Company announcement, Inside Information, 11 July 2024 at 7:00 a.m. BST / 9:00 a.m. EEST

Key highlights

- Faron had a formal meeting with the FDA to discuss the registrational clinical development plan for *bexmarilimab* in the treatment myelodysplastic syndrome (MDS).

- The FDA acknowledged the difficulties of running a randomized study with a comparator in the relapsed / refractory setting (r/r) and instead proposed that Faron conduct a confirmatory Phase III study in frontline high-risk MDS (HR MDS), that would not require a separate Phase III in r/r MDS.
- This FDA guidance is part of Project Frontrunner, an initiative intended to bring promising new cancer treatments as early as possible to a broader patient population.
- The Phase III suggested by the FDA targets a significantly larger patient population with potential for faster approval earlier than anticipated, speeding up and increasing our sales forecast for *bexmarilimab*.

TURKU, Finland - Faron Pharmaceuticals Ltd. (AIM: FARN, First North: FARON), a clinical-stage biopharmaceutical company pursuing a CLEVER-1 receptor targeting approach to reprogramming myeloid cells to activate anti-tumor immunity in hematological and solid tumor microenvironments, today provides information on the result of its formal Type D Scientific Advice Meeting with the USA Food and Drug Administration (the FDA) regarding the registrational study plan for its drug candidate *bexmarilimab* in relapsed and refractory high risk MDS (r/r MDS).

Given the previously reported promising results of treating r/r MDS using a combination of *bexmarilimab* + azacitidine to overcome primary or developed resistance to azacitidine, Faron had proposed to move into a randomized registrational Phase III study for the treatment of r/r MDS using *bexmarilimab* + azacitidine against the investigator's choice of a hypomethylating agent (HMA). Instead, given the encouraging efficacy already seen in both frontline and r/r HR MDS and the well-established safety profile of *bexmarilimab*, the FDA proposed that after the ongoing Phase II BEXMAB study in r/r MDS, Faron should move directly into a registrational blinded randomized frontline HR MDS study investigating *bexmarilimab* + azacitidine against placebo + azacitidine. The FDA noted that given the relatively modest efficacy of single agent azacitidine and the current response rates with *bexmarilimab* that the size of such a frontline study may not have to be substantially larger than the proposed study in the r/r setting.

Further, the FDA suggested such a frontline study could be seen in the context of FDA's Project Frontrunner.Project FrontRunner is an FDA Oncology Center of Excellence (OCE) initiative to encourage drug sponsors to develop and seek approval of promising new cancer drugs for advanced diseases in an earlier clinical setting, rather than the usual approach to develop and seek approval of a new drug for treatment of patients who have received numerous prior lines of therapies or have exhausted available treatment options. The FDA guidelines give different possible approval strategies to sponsors, including the conduct of a frontline trial supporting an accelerated approval in the r/r settings. Project FrontRunner | FDA

Subject to continued positive results, the FDA's feedback means that a separate Phase III in r/r MDS would not be required and Faron's ongoing BEXMAB Phase II study could be the registrational trial for patients with r/r MDS, given that the benefit of *bexmarilimab* + azacitidine against azacitidine alone will be confirmed in an interim read-out of the response rate from a Phase III in frontline HR MDS study. Accelerated approval for frontline HR MDS would come from the response rate of this single Phase III study and the full approval from the survival read-out of the same study.

"Faron is now adjusting its development plan accordingly", says Dr. Juho Jalkanen, Chief Executive Officer of Faron. "This is very positive feedback and exceeds our expectations. The FDA's proposal significantly reduces development costs and timelines to bring *bexmarilimab* therapy to all HR MDS patients. This feedback underlines that the FDA sees the high unmet need in HR MDS, a condition for which new treatment options are urgently needed. The FDA's proposal has provided Faron with clear guidance on the path to approval that will confirm the highly encouraging results *bexmarilimab* has already obtained in overcoming resistance to azacitidine. We are extremely grateful for this feedback and will work hard to deliver on this recommendation."

"The suggested Phase III targets a significantly bigger patient population sooner than anticipated, speeding up and increasing our sales forecast for *bexmarilimab*. This does not significantly impact our ongoing activities and cash runway, as the Phase II in r/r MDS continues as planned. In addition, we will enroll more frontline HR MDS patients into the Phase I part of BEXMAB to better understand the effect size, which will enable us to successfully power and design the proposed frontline Phase III study. We believe we can offset this additional clinical investment through other savings, so that it will not have a significant impact on our cash runway. The only deviation from the original plan is that instead of a Phase III in r/r MDS, we will start preparations for a Phase III in frontline HR MDS, which is a remarkable achievement.", continues Dr. Jalkanen.

Faron will be hosting a virtual webinar to discuss the FDA feedback and updated clinical development plans July 15th, at 15.00 EEST / 13.00 BST.

To register for the event visit: https://faron.videosync.fi/fda-feedback-update or contact the IR team for more information at investor.relations@faron.com.

For the purposes of MAR and UK MAR, the person responsible for arranging for the release of this announcement on behalf of Faron is Juho Jalkanen, Chief Executive Officer.

For more information please contact:

Investor Contact
Faron Pharmaceuticals
E-mail: investor.relations@faron.com

Media Contact
ICR Consilium
Mary-Jane Elliott, David Daley, Lindsey Neville
Phone: +44 (0)20 3709 5700
E-mail: faron@consilium-comms.com

Cairn Financial Advisers LLP, Nomad
Sandy Jamieson, Jo Turner
Phone: +44 (0) 207 213 0880

Peel Hunt LLP, Broker
Christopher Golden, James Steel
Phone: +44 (0) 20 7418 8900

Sisu Partners Oy, Certified Adviser on Nasdaq First North
Juha Karttunen
Phone: +358 (0)40 555 4727
Jukka Järvelä
Phone: +358 (0)50 553 8990

About BEXMAB
The BEXMAB study is an open-label Phase 1/2 clinical trial investigating *bexmarilimab* in combination with standard of care (SoC) in the aggressive hematological malignancies of acute myeloid leukemia (AML) and myelodysplastic syndrome (MDS). The primary objective is to determine the safety and tolerability of bexmarilimab in combination with SoC (azacitidine) treatment. Directly targeting Clever-1 could limit the replication capacity of cancer cells, increase antigen presentation, ignite an immune response, and allow current treatments to be more effective. Clever-1 is highly expressed in both AML and MDS and associated with therapy resistance, limited T cell activation and poor outcomes.

About Bexmarilimab
Bexmarilimab is Faron's wholly owned, investigational immunotherapy designed to overcome resistance to existing treatments and optimize clinical outcomes, by targeting myeloid cell function and igniting the immune system. *Bexmarilimab* binds to Clever-1, an immunosuppressive receptor found on macrophages leading to tumor growth and metastases (i.e. helps cancer evade the immune system). By targeting the Clever-1 receptor on macrophages, *bexmarilimab* alters the tumor microenvironment, reprogramming macrophages from an immunosuppressive (M2) state to an immunostimulatory (M1) one, upregulating interferon production and priming the immune system to attack tumors and sensitizing cancer cells to standard of care.

About Faron Pharmaceuticals Ltd.
Faron (AIM: FARN, First North: FARON) is a global, clinical-stage biopharmaceutical company, focused on tackling cancers via novel immunotherapies. Its mission is to bring the promise of immunotherapy to a broader population by uncovering novel ways to control and harness the power of the immune system. The Company's lead asset is *bexmarilimab*, a novel anti-Clever-1 humanized antibody, with the potential to remove immunosuppression of cancers through reprogramming myeloid cell function. *Bexmarilimab* is being investigated in Phase I/II clinical trials as a potential therapy for patients with hematological cancers in combination with other standard treatments. Further information is available at www.faron.com.

Forward-Looking Statements
Certain statements in this announcement are, or may be deemed to be, forward-looking statements. Forward looking statements are identified by their use of terms and phrases such as ''believe'', ''could'', "should", "expect", "hope", "seek", ''envisage'', ''estimate'', ''intend'', ''may'', ''plan'', ''potentially'', ''will'' or the negative of those, variations or comparable expressions, including references to assumptions. These forward-looking statements are not based on historical facts but rather on the Directors' current expectations and assumptions regarding the Company's future growth, results of operations, performance, future capital and other expenditures (including the amount, nature and sources of funding thereof), competitive advantages, business prospects and opportunities. Such forward-looking statements reflect the Directors' current beliefs and assumptions and are based on information currently available to the Directors.

A number of factors could cause actual results to differ materially from the results and expectations discussed in the forward-looking statements, many of which are beyond the control of the Company. In addition, other factors which could cause actual results to differ materially include the ability of the Company to successfully license its programs within the anticipated timeframe or at all, risks associated with vulnerability to general economic and business conditions, competition, environmental and other regulatory changes, actions by governmental authorities, the availability of capital markets or other sources of funding, reliance on key personnel, uninsured and underinsured losses and other factors. Although any forward-looking statements contained in this announcement are based upon what the Directors believe to be reasonable assumptions, the Company cannot assure investors that actual results will be consistent with such forward-looking statements. Accordingly, readers are cautioned not to place undue reliance on forward-looking statements. Subject to any continuing obligations under applicable law or any relevant AIM Rule requirements, in providing this information the Company does not undertake any obligation to publicly update or revise any of the forward-looking statements or to advise of any change in events, conditions or circumstances on which any such statement is based.

END

MSCSFEFASELSEEW