PILA PHARMA AB

Norra Vallgatan 72
211 22 Malmö
Sweden

pilapharma.com

Malmö 15 July 2022

Pila Pharma AB is granted Orphan Drug Designation status in the USA

Pila Pharma AB (publ) announces that the US Food and Drug Administration, FDA, has granted Orphan Drug Designation (ODD) of the development candidate XEN-D0501 for treatment of erythromelalgia, a rare disease that causes episodes of burning pain and redness due to neurogenic inflammation.

Pila Pharma's candidate molecule XEN-D0501 is otherwise under development for the treatment of type 2 diabetes. Preparations are ongoing according to plan for clinical trials in phase 2b. In addition, there are several indications where XEN-D0501 can have a good effect, including erythromelalgia, as well as other painful conditions.

The FDA's grant of ODD status means that Pila Pharma can develop XEN-D0501 as a new drug to treat erythromelalgia. In order to register and obtain marketing authorization clinical efficacy needs to be demonstrated. This will open the door to the potential for a 7-year orphan-drug/market exclusivity for XEN-D0501 in the USA.

The early results of development of XEN-D0501 can be credited from the diabetes project and the development of XEN-D0501 within this new indication can thus go directly to phase 2/3 clinical trials.

Pila Pharma now evaluates the most cost-effective way to clinically develop XEN-D0501 also in erythromelalgia to reach market sooner than later to the benefit of erythromelalgia patients and the Pila Pharma shareholders.

Dr. Hans Quiding has been hired as Project Director to lead the work with new projects including the OOD analgesia project. Working for Astra Zeneca for more than 35 years, Hans has been involved in developing two different analgesic products all the way through clinical development to registration and marketing (Citodon® and Ardinex®) and, later, became responsible for the clinical development extensions of Alvedon®.
He has also worked with potential treatments of erythromelalgia.

“The response we have now received from the FDA shows how great the potential is in the molecule XEN-D0501. Further, I’m really pleased that Hans who is such a great capacity within erythromelalgia specifically and analgesia in general, has chosen to join our team and I really look forward to work with him on developing a pipeline around TRPV1”, comments CEO Dorte X. Gram

For more information:
Dorte X. Gram, CEO
M: +46 (0)73 903 6969
E: dxg@pilapharma.com

This information is such information that PILA PHARMA AB is obliged to publish in accordance with the EU Market Abuse Regulation. The information was submitted for publication on 15 July 2022 at 16:00 CET.

Pila Pharma's share ticker PILA, is subject to trade on Nasdaq First North Growth Market, Sweden with Aqurat Fondkommission AB as Certified Adviser.
Contact: M: info@aqurat.se, T:+46 (0)8 684 05 800

About Pila Pharma AB (Publ)
Pila Pharma is a Swedish biotech company in the diabetes segment based in Malmö, Sweden. The aim of the company is to develop TRPV1 antagonists as novel treatments. The company currently develop XEN-D0501, as a new oral antidiabetic agent. The company owns both use patents for treating diabetes and obesity with TRPV1 antagonists, and the intellectual property rights for the mid stage clinical development candidate XEN-D0501. The company was listed at Nasdaq First North GM in Stockholm, Sweden on 15 July 2021 to finance the further development of XEN-D0501.

About XEN-D0501 and TRPV1 antagonists
XEN-D0501 is a selective, synthetic potent small molecule TRPV1 antagonist that was in-licensed in 2016 and, previously, in developed by Bayer Healthcare, Germany and Xention/Ario Pharma, UK. The TRPV1 target (also called the "chili-receptor") and TRPV1 antagonists that down-regulate neurogenic inflammation, has demonstrated applications across pain and inflammatory diseases and potentially plays a role in diabetes as well. Prior to in-licensing, XEN-D0501 had been found to have a good safety profile in other (non-diabetic) patient groups. Pila Pharma has to date completed two phase 2a clinical trials (PP-CT01 and PP-CT02), that both demonstrated that XEN-D0501 is well tolerated by type 2 diabetic patients. Further, PP-CT02, demonstrated that XEN-D0501 (administered as 4 mg BID for 28 days) – with statistical significance versus placebo – enhance the endogenous insulin response to oral glucose.
The company has recently completed the manufacture of new API needed for the conduct of 13-week preclinical safety studies, that are needed to further progress XEN-D0501 into a clinical 13-week phase 2b trial in patients with type 2 diabetes. The preclinical studies have recently been initiated and are ongoing. Considerations for best clinical development of XEN-D0501 in erythromelalgia in addition to diabetes are on-going.

About Diabetes
Diabetes is a world-wide pandemic with a staggering prevalence of 537 million diabetics corresponding to approximately 8-10% of the population. Approximately 90 % of all diabetics suffer from type 2 diabetes, whilst approximately 10% suffers from type 1 diabetes. The disease can lead to cardiovascular disease resulting in reduction of quality of life for the patient, increased risk of death and high health care expenses. Despite recent therapeutic advances, large and growing unmet needs exist both from an efficacy, safety, accessibility, and affordability perspective.

About Erytromelalgia
Erythromelalgia is a rare disease where neurogenic inflammation plays a role in the development of symptoms. The disease can cause near-constant or episodic erythema, pain (ranging from mild tingling to severe burning sensations), and redness to extremities. It most commonly affects the feet but may also occur in the hands, face, or other parts of the body with both nerves and blood vessels involved. Symptoms are frequently managed through avoidance of pain triggers. The disorder can be extremely debilitating, with a significant negative impact on quality of life and with potential to impact mortality rates in the young and suicide rates in adults.