PILA PHARMA AB

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Pila Pharma builds pipeline - applies for Orphan Drug Designation in the USA

Pila Pharma AB announces that the company has submitted a request for an Orphan Drug Designation to the U.S. Food and Drug Administration (FDA) for the company's development candidate XEN-D0501. The aim is to develop a novel treatment of a rare disease associated with pain due to neurogenic inflammation.

Pila Pharma's development candidate XEN-D0501 is under development for the treatment of type 2 diabetes. Preparations are ongoing according to plan for future clinical trials in phase 2b. The company's drug candidate, XEN-D0501, inhibits the activity of the body's TRPV1 receptors. TRPV1 has several functions in the body and among other things, the discovery of its role in pain has been awarded the 2021 Nobel Prize in Medicine.

Provided that the Orphan Drug Designation is designated by the FDA, Pila Pharma intends to seek funding to initiate clinical phase 2/3 studies to test the safety and efficacy of XEN-D0501 in the rare disease with the aim of registering a product with orphan drug status.

Pila Pharma CEO Dorte X. Gram comments: "I'm really pleased, that we can now publically share what we have been working on "under cover" for the last months! This submission marks a new phase in Pila Pharma's history with the active exploration of alternative indications in which XEN-D0501 or its analogues can be useful. We have had our eyes on several other indications for a while, including this specific rare disease, but it is only now that we have the expertise, resources and collaboration agreements that allow us to start new projects. We have previously said that XEN-D0501 has the potential to be useful as a treatment for other diseases where neurogenic inflammation plays a role, and we will continue on that path to eventually develop a pipeline with a focus on TRP as a platform technology."

Pila Pharma has developed this application in collaboration with Worldwide Clinical Trials, a contract research organization (CRO) with expertise in neuroscience and clinical development.

An approval of the request for Orphan Drug Designation would mean that Pila Pharma would receive market exclusivity including other benefits for XEN-D0501 for the treatment of that specific disease in the USA for seven years following registration.

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About PILA PHARMA (Publ)
PILA PHARMA is a Swedish biotech company in the diabetes segment based in Malmö. The aim of the company is to develop a novel and superior tablet based treatment for type 2 diabetes. 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 is listed at Nasdaq First North GM in Stockholm in July 2021 to finance the further development of XEN-D0501. Currently, new API for further 3 months preclinical safety studies is being manufactured to permit the company to progress XEN-D0501 to a pivotal 3 month phase 2b trial in patients with diabetes, scheduled to start in first part of 2023.

About XEN-D0501 and TRPV1 antagonists
XEN-D0501 is a highly selective and very potent small molecule TRPV1 antagonist, previously in development by Bayer Healthcare and Xention/Ario Pharma. The TRPV1 target (also called the “chili-receptor”) has demonstrated applications across pain and inflammatory diseases and potentially plays a role in diabetes as well. XEN-D0501 was acquired by PILA PHARMA in March 2016, due to its very good safety and tolerability profile as compared to other clinical TRPV1-antagonist development candidates. TRPV1 antagonists as a drug-class has previously been associated with severe adverse events as fever (hyperthermia) but this has not been the case for XEN-D0501 in 8 clinical trials conducted so far. The maximal tolerable dose in non-diabetic individuals has previously been determined to be 4 milligrams twice daily, a dose level with good safety but no effect in non-diabetic patients with either overactive bladder disease or chronic cough. In November 2018, PILA PHARMA reported the completion of its first clinical trial (PP-CT01), demonstrating a good safety profile of XEN-D0501 at single doses up to 8 milligrams when administered to people with type 2 diabetes. The most recent study results were announced in September 2020. The study (PP-CT02) demonstrated that multiple doses of XEN-D0501 (4 milligrams twice daily for 28 days) were likewise well-tolerated by people with type 2 diabetes and also – with statistical significance versus placebo - that XEN-D0501 enhances the endogenous insulin response to oral glucose, thus demonstrating proof of principle.

About Diabetes
Diabetes is a worldwide 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 Worldwide Clinical Trials
Worldwide Clinical Trials is a global, midsize contract research organization (CRO) that provides top-performing preclinical and Phase I-IV clinical development services to the biotechnology and pharmaceutical industries, with operations across 60 countries including the United States. Major therapeutic areas of focus include cardiovascular, metabolic, neuroscience, oncology, and rare diseases.

PilaPharma´s share, ticker PILA, is subject to trade on Nasdaq First North Growth Market with Aqurat Fondkommission AB as Certified Adviser. info@aqurat.se.
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