PILA PHARMA Prepares Proof-of-Concept Study in Rare Pain Disorder

PILA PHARMA announced on Monday that the company is preparing a proof-of-concept study in a rare pain disorder.

On Monday, PILA PHARMA announced that the company has begun preparations for an application to initiate a clinical proof-of-concept trial in the rare pain disorder erythromelalgia. The objective is to test whether its drug candidate XEN-D0501 (an oral TRPV1 inhibitor) can alleviate patients' pain. There are currently no approved treatments.

Next Steps and the Value of the Disease Area

The next step is the preparation of the clinical application for a smaller proof-of-concept study in erythromelalgia, followed later by a Phase 2/3 registration study. PILA PHARMA has already received regulatory tailwinds, as the company was granted orphan drug designation by the FDA in 2022 for erythromelalgia. This designation typically provides incentives, shorter development timelines and reduced costs, as well as seven years of market exclusivity following potential approval. The company has previously stated that, contingent on positive proof-of-concept data, a similar designation is expected to be granted by the EMA (the European Medicines Agency), providing ten years of market exclusivity in the EU.

PILA PHARMA expects that it will take approximately three years to complete the studies up to registration, subject to securing the necessary funding along the way. If the results are positive, the product would be eligible for seven years of market exclusivity in the United States following approval. Rare diseases can generally command higher pricing due to the low number of patients. The exact number of patients affected is not known with certainty.

Erythromelalgia is an extremely debilitating and painful condition characterized by burning pain and redness in the extremities, with a lack of meaningful treatment options. A successful product would therefore have the potential to help many patients. The Swedish research firm Analyst Group estimated in its most recent report that erythromelalgia could potentially achieve annual sales exceeding SEK 3 billion. With an approximate three-year timeline to market, the company could become profitable relatively quickly and would not be dependent on capital increases.

The Science Behind the Project

PILA PHARMA is working to inhibit the activity of the TRPV1 receptor, which plays a key role in regulating pain and inflammation. This receptor has previously been studied extensively in the development of new, non-addictive pain treatments as alternatives to opioids. There is therefore a strong scientific consensus and rationale that inhibition of TRPV1 can reduce the perception of pain—further supporting PILA PHARMA's decision to advance development in this disease area. With successful studies, PILA PHARMA may also expand its research into other painful conditions and potentially open the door to broader use of the drug candidate XEN-D0501 in pain

management. Persistence Research estimates that the market for opioid painkillers (which are highly addictive) amounted to approximately USD 45 billion in 2025 and is expected to grow to USD 62 billion by 2032. This could therefore represent an attractive additional area with significant opportunities.

Founder and Chief Scientific Officer of PILA PHARMA, Dorte X. Gram, stated:

> I am very pleased that we can now actively begin preparations to investigate the efficacy of our lead candidate in erythromelalgia. Patients often contact me because they have heard about PILA's ambitions to develop an effective treatment that can ease their daily suffering. Based on my conversations with them, they currently have no meaningful treatment options. If XEN-D0501 proves effective in relieving pain without excessive safety concerns, it would truly be a game changer for them and enable them to resume a normal life without the almost constant pain. At PILA, we are driven by a desire to heal, and it would be deeply meaningful if we could help reduce the suffering of people living with erythromelalgia.

PILA PHARMA's scientific expert in pain management, Hans Quiding, added:

> It would be fantastic to be able to bring relief to people living with erythromelalgia, who currently lack approved treatment options. I have worked extensively with TRPV1 inhibitors in pain management for many years at AstraZeneca, and I am very hopeful that this molecule, with its unique safety profile, could be of great value to EM patients. A small tablet is clearly preferable, as it can be swallowed quickly and easily during an attack. Moreover, with systemic absorption, the drug is rapidly distributed to the feet as well as other affected areas of the body. The tablet could potentially also be used for preventive treatment, not only when patients experience attacks, but possibly to prevent them from occurring in the first place. That would be fantastic.

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