The EMA and FDA Grant Orphan Drug Designation to Pharnext’s PXT-3003 for the Treatment of Charcot-Marie-Tooth Disease Type 1A

PARIS, June 23, 2014 – Pharnext SAS today announced that the European Medicines Agency (EMA) and the U.S. Food and Drug Administration (FDA) have granted orphan drug designation to PXT-3003 for the treatment of Charcot-Marie-Tooth disease type 1A (CMT 1A).

Highlights

- CMT 1A is a rare genetic neuromuscular disorder (prevalence rate up to 1 in 5,000 both in Europe and the U.S.) leading to progressive muscle atrophy in the extremities, resulting in the loss of normal use of feet, legs, hands and arms and associated with serious disabilities.1,2
- To date, no curative or symptomatic medications have been approved and CMT 1A treatment consists of supportive care such as orthotics, leg braces, physical and occupational therapy or surgery.3
- PXT-3003 is a novel oral fixed low dose combination of baclofen, naltrexone and sorbitol.
- PXT-3003 showed positive results in a Phase 2 study which enrolled 80 adult patients with CMT 1A. Pharnext is now preparing a Phase 3 trial to be conducted in both Europe and the U.S.
- The EMA and FDA granted the orphan drug designation to PXT-3003 for the treatment of CMT 1A following the submission of pre-clinical data and the Phase 2 clinical trial results. The Committee for Orphan Medicinal Product (COMP) at EMA stated that “the intention to treat CMT 1A with PXT-3003 was considered justified based on pre-clinical in vivo data obtained in a valid model of the condition as well as preliminary clinical data”.
- Orphan drug designation allows PXT-3003 marketing exclusivity for ten years in Europe and seven years in the U.S. if approved for the CMT 1A indication.
- Orphan drug designation benefits may also include fee reductions, assistance in study design from the FDA and EMA, potential for expedited drug development and eligibility for drug grants.

Quotes

Daniel Cohen, M.D., Ph.D., chairman and chief executive officer of Pharnext, said, “FDA and EMA orphan drug designations for PXT-3003 recognize the value this novel oral therapeutic could bring to CMT 1A patients. This is an important milestone that strengthens our ultimate commitment to develop and bring rapidly to market effective novel therapies based on low-dose combinations of repositioned drugs. This also provides additional confidence in our other programs currently in development.”
Catherine Scart-Gres, M.D., chief medical officer, said, “We are very pleased to receive orphan drug designation for PXT-3003. If approved, PXT-3003 could be a significant therapeutic advance for patients suffering from CMT 1A which impairs quality of life and can lead to severe disabilities. PXT-3003 has shown promise in a Phase 2 study and we are now actively preparing the next clinical trial.”

About Orphan Drug Designation

The EMA may grant orphan drug designation to provide regulatory and financial incentives for companies to develop and market therapies that treat a life-threatening or chronically debilitating condition affecting no more than five in 10,000 persons in the European Union (EU) and where no satisfactory treatment is available.

The FDA may grant orphan drug designation to facilitate drug development for drugs that target conditions affecting fewer than 200,000 patients in the U.S. while providing a significant therapeutic advantage over existing therapies.

About Pharnext

Pharnext is an advanced clinical stage biopharmaceutical company discovering and developing new therapeutics that target multiple key disease pathways for orphan and common neurological diseases including Charcot-Marie-Tooth disease, Alzheimer’s disease, amyotrophic lateral sclerosis and Parkinson’s disease. These new therapeutics consist of low dose combinations of repositioned drugs selected using Pharnext’s proprietary network pharmacology approach. The company’s first two lead programs are PXT-3003 for the treatment of Charcot Marie Tooth type 1A (Phase 2 completed) and PXT-864 for Alzheimer’s disease (Phase 2 ongoing) and other neurologic indications. Pharnext is supported by BPI France (French Public Investment Bank) and AFM (French Muscular Dystrophy Association).

For further information, please visit www.pharnext.com

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References

