The FDA grants Dimebon orphan drug status

Pfizer Inc. and Medivation Inc. have announced that the Food and Drug Administration (FDA) has granted Dimebon orphan drug status as a potential treatment for Huntington's Disease. The Orphan Drug Act, which was passed in 1983, provides financial and other incentives to pharmaceutical companies to develop treatments for diseases which affect less than 200,000 people per year.

The goal is to make drug development for orphan diseases more attractive than the smaller market would suggest. Patent protection is extended to seven years if the drug is approved, tax incentives are provided to conduct clinical trials, research grants may be available and the FDA's application user fee is waived. In addition, and there is a recognition that Phase III trials will involve smaller numbers of participants than for more common diseases.

A Phase III trial of the investigational drug dimebon (latrepirdine)* has been initiated for patients with Huntington disease. The international safety and efficacy trial, known as HORIZON, is designed to evaluate the potential benefits of dimebon on cognition (thinking and memory) in patients with Huntington disease.

"Based on the promising results of our Phase II trial of dimebon in Huntington disease, we are pleased to advance dimebon into late-stage clinical development," said Lynn Seely, M.D., chief medical officer for Medivation. "Huntington disease is a fatal genetic disease for which no medications are currently approved by the FDA to treat the cognitive impairment associated with the condition."

*Latrepirdine is the proposed generic (nonproprietary) name for dimebon.

Design of the HORIZON Study

The double-blind, placebo-controlled Phase 3 trial will enroll approximately 350 patients with Huntington disease at approximately 50 sites in North America, Europe and Australia. Patients will be randomized to receive either dimebon (latrepirdine) 20 mg three times daily or placebo for six months.

The primary endpoints of the trial are the Mini Mental State Examination (MMSE), which measures cognition, and the Clinician's Interview-Based Impression of Change, plus caregiver input (CIBIC-plus), which measures global function. The trial will include only patients who have cognitive impairment, as subjectively assessed by an investigator and objectively by MMSE score.

Secondary endpoints include the Neuropsychiatric Inventory (NPI), which measures behavior; the Alzheimer's Disease Cooperative Study - Activities of Daily Living (ADCS-ADL), which measures self-care and daily function; and the Unified Huntington Disease Rating Scale (UHDRS'99) Total Motor Score, which measures motor impairment; and safety.

The trial is being conducted in collaboration with the Huntington Study Group (HSG) and the European Huntington's Disease Network (EHDN). The HSG is a non-profit group of experienced clinical trial investigators from medical centers in the United States and abroad dedicated to clinical research of Huntington disease. The EHDN is a non-profit network of professionals providing an infrastructure for large scale Huntington disease clinical trials throughout Europe.

For more information about the HORIZON study, please visit www.horizontrial.com or call 800-487-7671. A list of participating sites can be found here: http://www.huntington-study-group.org/Portals/0/HORIZONSiteList.pdf. It will be updated regularly as more sites are up and running.

About Dimebon

Dimebon (latrepirdine) is an investigational drug in Phase 3 development for the treatment of Alzheimer's disease (AD) and Huntington disease (HD). In preclinical studies, dimebon has been shown to protect brain cells from damage and enhance brain cell survival, potentially by stabilizing and improving mitochondrial function. The dimebon mechanism is distinct from currently available AD and HD medications.

About the Pfizer/Medivation Dimebon Collaboration

Medivation and Pfizer have a global collaboration to develop and commercialize dimebon for the treatment of Alzheimer's disease and Huntington disease. Under the terms of the agreement, the companies are working in partnership to seek FDA approval for dimebon and bring it to market in the United States. In addition, following FDA approval, Medivation will co-promote dimebon to specialty physicians in the U.S. Pfizer has responsibility for development, regulatory and commercialization outside of the U.S.

References:

Pfizer/Medivation press release
FDA website

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