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Novartis receives FDA Fast Track designation for branaplam (LMI070) for the treatment of Huntington's Disease

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Novartis today announced that the US Food and Drug Administration (FDA) has granted Fast Track designation for branaplam (LMI070) for the treatment of Huntington's disease (HD). Fast Track designation facilitates the development and expedites the review of drugs to treat serious conditions and fill unmet medical needs[1]. Branaplam is being developed as a potential first in class orally administered disease modifying therapy for HD.

- HD is a devastating, fatal, familial disease for which there are no disease modifying therapies that delay onset or slow progression. A multi-faceted neurodegenerative disease, it causes a progressive decline in behavioral, cognitive and motor functioning. As a rare disease with rising prevalence, it is estimated that there are approximately 70k diagnosed patients with HD in the US and EU[2]. All carriers of the mutated Huntington gene will develop the disease.
- Existing pharmacologic treatments are very limited and only address individual symptoms which have no effect on the course of the disease or life expectancy. There is an urgent and unmet medical need for therapies that delay the onset or slow the progression of HD. HD invariably leads to total incapacity of daily function, institutionalization and ultimately to death within 15-20 years of onset[3].
- Branaplam has the potential to address this unmet medical need. An investigational oral disease modifying treatment, branaplam is an mRNA splicing modulator that targets the underlying pathophysiology in HD by modifying HTT mRNA throughout the brain and the body, resulting in lower levels of HTT protein. Informed by preclinical and early phase clinical studies[4], a Phase IIb study (VIBRANT-HD), in people (adults) with early-stage manifest HD is underway and currently enrolling[5].

Branaplam is among a number of investigational programs in the Novartis neuroscience pipeline, where together with key partners we seek to accelerate medical breakthroughs in neurological conditions with high unmet need, including multiple sclerosis, pediatric neurology, neurodegeneration and neuropsychiatry.

1. U.S. Food and Drug Administration (FDA). Fast Track. Available from: https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/fast-track/
2. Nopoulos PC. Dialogues Clin Neurosci. 2016 Mar;18(1):91-8
3. Walker FO. Lancet. 2007 Jan 20;369(9557):218-28
4. Data on File
5. Clinical Trials.gov. A Dose Range Finding Study With Open-Label Extension to Evaluate the Safety of Oral LMI070/Branaplam in Early Manifest Huntington's Disease

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1. https://prod1.novartis.com/news/novartis-receives-fda-fast-track-designation-branaplam-lmi070-treatment-huntingtons-disease
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