

INVESTOR & MEDIA UPDATE

Novartis receives US Food and Drug Administration (FDA) Orphan Drug Designation for branaplam (LMI070) in Huntington's disease (HD)

- *Huntington's disease is a rare, inherited neurodegenerative disease that leads to progressive disability and death*
- *There are no approved disease modifying therapies that delay disease onset or slow progression of the disease*
- *Branaplam (LMI070) is an orally administered, small molecule RNA splicing modulator that could potentially reduce the levels of mutant huntingtin protein*

Basel, October 21, 2020 — Novartis today announced that the US Food and Drug Administration (FDA) has granted Orphan Drug Designation for branaplam (LMI070) in Huntington's disease (HD). An Orphan Drug Designation grants special status to a drug that treats a rare disease or condition, and provides companies certain benefits to encourage the continued development of medicines that bring novel solutions to patients with these severe diseases.

In preclinical models, branaplam has been shown to reduce levels of mutant huntingtin protein. In addition, during the investigation of branaplam in spinal muscular atrophy (SMA), it was also observed to reduce huntingtin messenger RNA (mRNA) in SMA patients. A decrease of huntingtin mRNA is expected to result in reduction of huntingtin protein levels, the underlying cause of HD. Based on these findings, Novartis intends to start a development program for branaplam to determine if it has the potential to be a transformative treatment for people living with this devastating condition.

Current treatment options for HD are limited to symptomatic treatments and there are no approved disease modifying therapies that delay disease onset or slow progression of the disease.

Branaplam is currently under investigation for the treatment of spinal muscular atrophy (SMA). SMA is a rare, progressive genetic disease, characterized by loss of motor neurons that are responsible for muscle function. Branaplam is dosed once weekly for the treatment of SMA, and the same dosing regimen may also be a possibility for HD.

Novartis plans to start the Phase IIb trial for branaplam in HD patients in 2021.

About branaplam

Branaplam (LMI070) is a once-weekly, orally administered, small molecule RNA splicing modulator that is currently under investigation for the treatment of spinal muscular atrophy (SMA). SMA is a rare, progressive genetic disease, characterized by loss of motor neurons

that are responsible for muscle function. Novartis has more than 5 years' clinical experience with branaplam in our ongoing SMA development program.

About Huntington's disease

Huntington's disease (HD) is a rare, inherited neurodegenerative disease that leads to progressive disability and death. Everyone has the huntingtin (HTT) gene, but only those who have a mutated form of the gene will develop the disease.

HD is characterized by progressive worsening in motor, cognitive and psychiatric symptoms. These symptoms usually appear between the ages of 30 to 50, and worsen over a 15-20-year period. Approximately 70,000 people across Europe and the US have been clinically diagnosed with HD.

Current treatment options for HD are limited to symptomatic treatments and there are no approved disease modifying therapies that delay disease onset or slow progression of the disease.

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About Novartis

Novartis is reimagining medicine to improve and extend people's lives. As a leading global medicines company, we use innovative science and digital technologies to create transformative treatments in areas of great medical need. In our quest to find new medicines, we consistently rank among the world's top companies investing in research and development. Novartis products reach nearly 800 million people globally and we are finding

innovative ways to expand access to our latest treatments. About 109,000 people of more than 140 nationalities work at Novartis around the world. Find out more at <https://www.novartis.com>.

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