

Asceneuron Receives Regulatory Approval for Phase I Healthy Volunteer Study of Oral Tau Inhibitor

First-in-human trial of novel inhibitor targeting accumulation of toxic neurofibrillary tau tangles to fight neurodegenerative diseases including dementia

Lausanne, Switzerland, April 6th, 2017 - Asceneuron SA, an emerging leader in the development of innovative small molecules for neurodegenerative diseases, announced today the regulatory approval of its clinical trial application to initiate a first clinical study of ASN120290 (formerly known as ASN-561), belonging to a chemically novel group of O-GlcNAcase enzyme inhibitors. Based on preclinical studies, ASN120290 has the potential to become a new treatment for dementia.

The objective of the randomized, double-blind, placebo-controlled, phase I study is to assess the safety, tolerability, pharmacokinetics, pharmacodynamics and food effect of single and multiple doses of orally administered ASN120290. Within this phase 1 study, Asceneuron will assess a blood-based biomarker to support optimal dose selection of ASN120290 for future trials. Upon successful completion of phase 1, a phase 2 proof-of-concept trial in the orphan tauopathy disease progressive supranuclear palsy (PSP) is planned for 2018 in elderly patients. PSP is a rare neurological condition that causes serious problems with walking, balance, speech, swallowing and vision as a result of the accumulation of aggregates of the tau protein in the brain. Three to six people per 100,000 will develop PSP and there is currently no cure.

The therapeutic potential of ASN120290 has been demonstrated in preclinical studies in which it has been shown to substantially reduce the build-up of toxic aggregates of the tau protein into neurofibrillary tangles. Neurofibrillary tangles are widely recognized as a key driver of neurodegeneration and clinical symptoms in the majority of dementia cases, including Alzheimer's disease.

Dirk Beher, chief executive officer and a co-founder of Asceneuron, commented: "This is a significant milestone for Asceneuron and marks our transition to a clinical stage company. It is also a major achievement for our scientific team as ASN120290 is our first in-house developed molecule reaching the clinic and was designed to easily enter the brain. New approaches to treat dementia are urgently required and preventing toxic tau tangle formation with our O-GlcNAcase inhibitor represents a new mechanism of action."

J. Michael Ryan, chief medical officer of Asceneuron, added: "Neurodegenerative diseases are a growing public health concern, with high unmet medical need and no approved treatments for slowing disease progression. Receiving regulatory approval to progress ASN120290 into phase 1 moves us closer to our goal of bringing innovative, orally-administered, tau-focused therapies to patients suffering from neurodegenerative diseases."

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About Asceneuron

Asceneuron is an emerging, clinical stage biotech company excelling in the development of orally bioavailable therapeutics for debilitating neurodegenerative disorders with high unmet medical needs such as orphan tauopathies, Alzheimer's and Parkinson's diseases. The lead product, an O-GlcNAcase inhibitor that in preclinical studies has been demonstrated to modulate tau pathology, has received regulatory approval to start the first human clinical testing. The O-GlcNAcase inhibitor is being developed for the orphan tauopathy progressive supranuclear palsy (PSP). Asceneuron is a privately held company financed by a strong syndicate of investors consisting of Sofinnova Partners, Merck Ventures, SR One, Johnson & Johnson Innovation – JJDC, Inc. (JJDC) and Kurma Partners. For more information, please visit www.asceneuron.com.