Asceneuron's tau modifier ASN120290 receives Orphan Drug Designation for progressive supranuclear palsy from the FDA

Advancement of novel inhibitor targeting accumulation of toxic neurofibrillary tau tangles

Lausanne, Switzerland, July 18, 2018 - Asceneuron SA, an emerging leader in the development of innovative small molecules for the treatment of neurodegenerative diseases, announced today that the US Food and Drug Administration (FDA) has granted Orphan Drug Designation to ASN120290 for the treatment of progressive supranuclear palsy (PSP), a rapidly progressing rare neurodegenerative disorder. ASN120290 was discovered at Asceneuron and is a selective inhibitor of the O-GlcNAcase enzyme. Based on its unique mechanism of action, the molecule has the potential to become a first in class treatment for PSP and other tau-related dementias.

ASN120290 has recently completed a randomized, double-blind, placebo-controlled phase I study to assess its safety and tolerability of single and multiple doses in healthy young and elderly volunteers. Data from that study will be presented at the upcoming *Alzheimer's Association International Conference* (AAIC*)* to be held in Chicago July 22-26, 2018.

The therapeutic potential of ASN120290 has been demonstrated in preclinical studies with a substantial reduction in the build-up of toxic aggregates of tau 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.

PSP is a rare neurological condition that causes severe problems with walking, balance, speech, swallowing and vision as a result of the accumulation of aggregates of the tau protein in the brain. It is estimated that three to six people per 100,000 will develop PSP and there is currently no cure for the disease.

Dirk Beher, chief executive officer and a founder of Asceneuron, commented: "PSP is a rare neurological condition for which there is currently no treatment available. ASN120290 is an orally bioavailable molecule that has the potential of treating the root cause of the neurodegeneration. The granting of Orphan Drug Designation for ASN120290 by the FDA is an important milestone for the team and the company. It strengthens our commitment to serving this important unmet medical need and bringing this molecule to patients."

Orphan Drug status is intended to advance drug development for rare diseases. The FDA provides Orphan Drug Designation to drugs and biologics that demonstrate promise for the diagnosis and/or treatment of rare diseases or conditions that affect fewer than 200,000 people in the U.S. The designation can provide development and commercial incentives for designated compounds and medicines, including eligibility for a seven-year period of market exclusivity in the U.S. after product approval, FDA assistance in clinical trial design and an exemption from FDA user fees.

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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 need, such as orphan tauopathies, Alzheimer's and Parkinson's diseases. The lead program ASN120290, an O-GlcNAcase inhibitor, is being developed for the orphan tauopathy progressive supranuclear palsy (PSP). Asceneuron has completed a randomized, double-blind, placebo-controlled phase I study to assess the safety and tolerability of single and multiple doses of orally administered ASN120290. Asceneuron is a privately held company financed by a strong syndicate of investors consisting of Sofinnova Partners, M Ventures, SR One, Johnson & Johnson Innovation – JJDC, Inc. (JJDC) and Kurma Partners. For more information, please visit www.asceneuron.com.

About Progressive Supranuclear Palsy (PSP)

Progressive Supranuclear Palsy, also known as Steele-Richardson-Olszewski syndrome, is a rapidly progressing neurodegenerative disorder. PSP is often misdiagnosed because it is relatively rare and certain symptoms are similar to Parkinson's disease. However, PSP is much more common than previously believed. Its prevalence is about three to six people per 100,000 individuals. Symptoms generally appear in the 60s-70s, but can affect people from the age of 40 onwards. There are currently no treatments available to cure this disease.