Asceneuron initiates neuroimaging trial for tau modifier ASN120290

Major milestone in development of novel treatment for progressive supranuclear palsy and other tau-related dementias

Lausanne, Switzerland, November 20, 2018 - Asceneuron SA, an emerging leader in the development of innovative small molecules for the treatment of neurodegenerative diseases, announced today it has commenced a new clinical trial of its lead asset, ASN120290. The study aims to quantify target engagement of ASN120290 in the human brain using positron emission tomography (PET) to help guide dose selection for a planned clinical efficacy trial in PSP. ASN120290 has the potential to become a first-in-class treatment for progressive supranuclear palsy (PSP), a rapidly progressing rare neurodegenerative disorder, and other tau-related dementias.

ASN120290 is a selective inhibitor of the O-GlcNAcase enzyme and was recently granted Orphan Drug Designation by the US Food and Drug Administration (FDA) for the treatment of PSP. Its therapeutic potential has been demonstrated in preclinical studies with a profound reduction in the accumulation of toxic aggregates of the tau protein into neurofibrillary tangles. Neurofibrillary tangles are now widely recognized as the main cause of neurodegeneration and clinical symptoms in the majority of dementia cases, including Alzheimer's disease (AD).

Dirk Beher, Chief Executive Officer and Founder of Asceneuron, commented:
"The application of PET imaging to demonstrate that a drug molecule reaches its intended therapeutic target in the brains of living human beings has become best practice in CNS drug discovery. PET imaging provides tremendous value to accelerate clinical development programs. The PET imaging data will be critical for dose selection in subsequent studies with ASN120290. We are excited about this new clinical trial with ASN120290 which demonstrates our continuing commitment to bring urgently needed treatments to patients with PSP and other tau-related neurodegenerative diseases."

PET imaging is a non-invasive method to quantify the binding of ASN120290 to the O-GlcNAcase enzyme in the living human brain. In this study, a specific enzyme inhibitor-derived imaging agent (PET tracer) will be administered either alone or after a pre-dose of ASN120290 in healthy volunteers. Once the PET tracer is introduced into the bloodstream, it crosses the blood-brain barrier and binds directly to the O-GlcNAcase enzyme inside brain cells. When ASN120290 is given prior to the tracer, it binds to the same enzyme in the brain thereby blocking the binding of the subsequently administered PET tracer. This decrease of PET tracer binding can be quantified, thereby allowing to calculate O-GlcNAcase enzyme occupancy by ASN120290.

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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 ASN120290

Asceneuron's lead program ASN120290, an O-GlcNAcase inhibitor, is being developed for the orphan tauopathy progressive supranuclear palsy (PSP) and was recently granted Orphan Drug Designation by the US FDA for the treatment of PSP. 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 were presented at the *Alzheimer's Association International Conference* (AAIC*)* in Chicago July 22-26, 2018.

About Progressive Supranuclear Palsy (PSP)

PSP, 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.