UCB News

UCB presents UCB0107 anti-Tau immunotherapy Phase I study results at World Movement Disorders Conference®

- **Results in healthy volunteers suggest UCB0107 well tolerated with an acceptable safety profile[1]**
- **Study further supports progression of UCB0107 clinical development programme in patients with tauopathies, such as progressive supranuclear palsy (PSP)**

Brussels, Belgium, Wednesday September 25th, 2019, 07:00 (CEST): New data from a Phase I study,[1] conducted in healthy volunteers, presented at the 2019 International Congress of Parkinson's Disease and Movement Disorders®, Nice, France, suggest UCB0107, an anti-Tau immunotherapy treatment currently being investigated by UCB as a potential treatment for patients with tauopathies such as progressive supranuclear palsy (PSP), is well tolerated with an acceptable safety profile.

Tau is a microtubule-associated protein expressed in the central nervous system, which supports with the assembly and stabilization of neuronal microtubules.[2] In tauopathies, Tau becomes pathogenic, forming tangles, which cause cell damage and ultimately neuronal death.[2,3,4] It is hypothesised that the spread of Tau protein from neuron to neuron underpins disease progression in tauopathies[5] providing the rationale for antibody therapies.

Previous preclinical studies[3,4] have already shown that the choice of epitope is an important efficacy determinant for therapeutic anti-Tau antibodies. This first in human, randomised, subject blind, investigator blind, placebo-controlled, single-ascending-dose study has been selected as a late breaking highlight by the MDS congress organisers, reflecting the importance of these data.

The aim of the research was to evaluate the safety, tolerability and pharmacokinetics of UCB0107 in healthy volunteers.

"We're learning more and more about the role of Tau deposits and the extent to which they are closely linked to symptoms of neurodegeneration such as movement disorders, memory loss and dementia" explained Colin Ewen, UCB Development Lead, UCB0107. *"Development of a medicine with a positive safety and tolerability profile, which could tackle the build-up of Tau, would offer hope to many millions of people impacted by neurodegenerative diseases".*

The primary endpoint used in the study was incidence of adverse events. Data relating to other safety assessments (neurological examination, MRI, ECG, clinical chemistry, haematology, coagulation, urinalysis and vital signs) were also collected. Additional secondary endpoints measured serum and cerebrospinal (CSF) PK parameters.
52 healthy male adults were randomized and assigned to one of seven dose cohorts to receive UCB0107 or placebo by iv infusion. All participants completed the study.

Over the course of the study, the most common treatment-emergent adverse event (TEAE) in the total UCB0107 and placebo groups was headache (15.8% and 35.7%, respectively). One severe TEAE of leg varicose ulceration was reported in one participant with a history of varicose veins and varicose-vein stripping. No serious or drug-related TEAEs were reported. There were no clinically relevant changes in other safety results.

"The UCB development process combines insights from patients and caregivers, collaborations with leading experts from around the world, and cutting-edge scientific methods to address important unmet medical challenges. This approach provides a uniquely holistic view about the unmet needs facing patients and the potential for an effective anti-Tau antibody in the treatment of neurodegenerative diseases", explained Charl van Zyl, Head of Neurology Patient Value Unit & Executive Vice President, UCB. *"These first in human safety and tolerability results support our belief in the potential for UCB0107 to provide value for people living with neurodegenerative disorders who currently have very limited or no treatment options. They validate our decision to continue the clinical development of this new asset."*

Results from this study, combined with results from pre-clinical research[3,4] support progression of UCB0107 clinical development in patients with tauopathies such as PSP.

Preparations are underway to initiate an adequate and well controlled study in Q2 2020.

About UCB0107

UCB0107 is a recombinant, humanised, full-length IgG4 monoclonal antibody, targeting a central Tau epitope, which is being developed to block/reduce the spread of Tau pathology.

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About UCB

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Moreover, sales may be impacted by international and domestic trends toward managed care and health care cost containment and the reimbursement policies imposed by third-party payers as well as legislation affecting biopharmaceutical pricing and reimbursement.

References:

1. A randomised, placebo-controlled, first-in-human study with a central Tau epitope antibody – UCB0107. [Late breaking abstract 3]. Poster presented at the International Congress of Parkinson's Disease and Movement Disorders® 22–26 September 2019, Nice, France
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