UCB News

# Findings from minzasolmin proof-of-concept ORCHESTRA study shape next steps in UCB Parkinson's research program

- UCB announces ORCHESTRA[1] minzasolmin study did not meet primary or secondary clinical endpoints
- UCB reinforces commitment to Parkinson's research, investigating multiple and distinct treatment approaches with different mechanisms of actions, to improve symptom management and slow or halt the progression of disease

**Brussels, Belgium – 16 December 2024 – 7PM CET –** UCB today announced the ORCHESTRA proof-of-concept study of minzasolmin - an investigational, oral small molecule, alpha-synuclein misfolding inhibitor - developed in partnership with Novartis for early Parkinson's disease, did not meet its primary and secondary clinical endpoints.

UCB has a portfolio of early phase pre-clinical and clinical programs evaluating multiple and distinct potential new treatment approaches in Parkinson's disease. The misfolding and aggregation of alpha-synuclein is a key pathological driver of Parkinson's disease[2], therefore, in addition to minzasolmin, which focused on inhibiting intracellular alpha-synuclein misfolding, UCB is progressing UCB7583, currently under investigation for preventing extracellular alpha-synuclein spread. Going beyond alpha-synuclein pathology, UCB is progressing glovadalen (UCB0022), an orally available, brain-penetrant, small molecule, designed to enhance the potency of dopamine 'when and where needed' to activate the dopamine D1 receptor and thereby improve symptom control.[3,4]

Alistair Henry, Chief Scientific Officer, UCB commented: "Our team is grateful to the patients, families and healthcare professionals involved in the minzasolmin development program. With a strong and proven heritage in Parkinson's disease, UCB continues its commitment to a science and patient driven strategy to address both its causes and symptoms. By leveraging the latest biological evidence, UCB's strategy includes investigating mechanisms to inhibit the misfolding and propagation of alpha-synuclein - processes believed to underlie the spread of neurodegeneration. Alongside this, we are advancing research into innovative therapies for symptom control, recognizing the diverse needs of each patient throughout their disease trajectory."

Following the evaluation of the ORCHESTRA study results, UCB has initiated measures to terminate the extension phase of this program. This decision is based on the absence of evidence of clinical benefit in both primary and secondary endpoints. Disease biomarker data, in which there was a preliminary signal, is still being analyzed.[5] The safety profile of minzasolmin was consistent with previous knowledge and no new safety concern has been identified.[5] The findings from this study have been submitted to an upcoming scientific meeting and will be submitted for publication in a peer-reviewed journal.



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ORCHESTRA/PD0053 (NCT04658186) was a large Phase 2a, randomised, placebo-controlled, 18-month study evaluating the efficacy, safety, tolerability and pharmacokinetics of oral minzasolmin in people living with early-stage Parkinson's. The purpose of the study was to assess the safety and tolerability of minzasolmin and to demonstrate its superiority over placebo with regards to clinical symptoms of disease progression over 12 to 18 months in participants diagnosed with early-stage Parkinson's disease. The primary endpoint was the progression in the Movement Disorder Society-Unified PD Rating Scale (MDS-UPDRS) Parts I–III sum score. More than 450 patients were enrolled in the study worldwide. The incidence of treatment emergent adverse events was comparable across all three treatment groups (180mg/day, 360mg/day, or placebo) in the study, and no new safety risks were identified.[5]

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**About UCB**
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**References:**


1. https://www.ucb.com/innovation/clinical-studies/clinical-studies-index/UCB0599. Accessed December 2024.
2. Bendor JT, Logan TP, Edwards RH. The function of α-synuclein. Neuron. 2013 Sep 18;79(6):1044-66.
3. https://www.ucb.com/innovation/clinical-studies/clinical-studies-index/UCB7853. Accessed December 2024.
4. https://www.ucb.com/innovation/clinical-studies/clinical-studies-index/UCB0022. Accessed December 2024.
5. UCB Data on File. December 2024.




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