



UCB's zilucoplan shows no relevant effect in immune-mediated necrotizing myopathy (IMNM)

- In phase 2, zilucoplan did not show a meaningful effect in immune-mediated necrotizing myopathy (IMNM)
- No unexpected safety findings and no relevant safety differences between zilucoplan and placebo were identified
- Phase 3 headline results for generalized myasthenia gravis (gMG) are unchanged expected in Q4 2021

Brussels (Belgium) 22 April 2021 – 20:00 (CEST) – Based on the initial results of a Phase 2 study investigating *zilucoplan* in immune-mediated necrotizing myopathy (IMNM), UCB today announced that UCB has decided to not move forward with its IMNM development program.

The phase 2 study with *zilucoplan*, a peptide inhibitor of complement component 5 (C5), in immune-mediated necrotizing myopathy (IMNM) did not show a meaningful effect of *zilucoplan* for people living with IMNM. No unexpected safety findings and no relevant safety differences between zilucoplan and placebo were identified.

“We are very thankful to the patients, their caregivers and the investigators for their study participation and their efforts”, said Prof. Dr. Iris Loew-Friedrich, Chief Medical Officer, UCB. *“While this result is disappointing, we are satisfied to have conducted a high-quality Phase 2 study which provides a clear read-out and direction on the path forward - despite the challenging environment during the pandemic, which had no material impact on results. This result in IMNM in no way affects our confidence in zilucoplan in the other indications we are pursuing given the uniqueness of each of the diseases and the way that complement inhibition may affect the specific immune pathology in each of them. We are looking forward to our upcoming Phase 3 headline results in the generalized myasthenia gravis (gMG) study, expected in Q4 2021.”*

In December 2019, Ra Pharma started the Phase 2 clinical trial of *zilucoplan* for the treatment of immune-mediated necrotizing myopathy (IMNM). Ra Pharma pioneered C5 inhibition in IMNM, an untested, new indication based on a sound scientific hypothesis and supportive non-clinical data, with the goal to provide a treatment to patients with a significant unmet need.

The results of this study indicate that complement activation is less relevant in the disease biology of IMNM than hypothesized. UCB anticipates presenting this data at an upcoming scientific meeting to further inform future IMNM research and to contribute towards better understanding about the disease pathogenesis.

The appropriate impairment assessment of the decision, to not move forward with the IMNM development program, will be performed for the Half Year Results 2021, due 29 July 2021. This decision does not impact the financial guidance for 2021.

About *Zilucoplan* - The phase 3 product candidate, *zilucoplan*, is a once-daily self-administered, subcutaneous peptide inhibitor of C5 and is currently being tested in phase 3 for the treatment of gMG with top-line results expected in Q4 2021. Further indications that are potentially addressable by *zilucoplan* include amyotrophic lateral sclerosis (ALS) and other tissue-based complement-mediated disorders with high unmet medical need. *Zilucoplan* was selected as one of the first drugs to be tested in a multi-center amyotrophic lateral sclerosis (ALS) platform study sponsored by the Sean M. Healey & AMG Center for ALS at Mass General. *Zilucoplan* is in clinical development and is not approved in any region of the world.

About immune-mediated necrotizing myopathy (IMNM) - IMNM is a rare, severe, inflammatory myopathy characterized by necrosis of skeletal muscle fibers. This manifests as proximal limb weakness. IMNM is a relatively newly defined inflammatory myopathy, a group of diseases that also includes polymyositis, dermatomyositis, inclusion body myositis, and other, less well-defined myopathies. The prevalence of IMNM is estimated at approximately 16,000 patients in the US, Europe, and Japan (Pinal-Fernandez et al, 2018; Smoyer-Tomic et al, 2012; Anquetil et al, 2019).

About Generalized Myasthenia Gravis (gMG) - Generalized myasthenia gravis is an unpredictable, chronic auto-immune condition in which auto-antibodies attack specific proteins in the neuro-muscular junction. This disrupts the way that nerves can communicate with muscles, resulting in muscle weakness and fatigue. Both men and women are impacted equally, and it can occur at any age and in any race. Myasthenia Gravis is a rare disease impacting almost 200,000 patients in the US, EU and Japan (Gilhus N, N Engl J Med 2016;375:2570-812015). Those living with gMG can experience a variety of symptoms, including drooping eyelids and double vision as well as severe muscular weakness that can result in life threatening weakness of muscles of respiration.

About UCB

UCB, Brussels, Belgium (www.ucb.com) is a global biopharmaceutical company focused on the discovery and development of innovative medicines and solutions to transform the lives of people living with severe diseases of the immune system or of the central nervous system. With more than 7 600 people in approximately 40 countries, UCB generated revenue of € 5.3 billion in 2020. UCB is listed on Euronext Brussels (symbol: UCB). Follow us on Twitter: @UCB_news

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Forward looking statements UCB

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There is no guarantee that new product candidates in the pipeline will progress to product approval or that new indications for existing products will be developed and approved. Products or potential products which are the subject of partnerships, joint ventures or licensing collaborations may be subject to differences between the partners. Also, UCB or others could discover safety, side effects or manufacturing problems with its products after they are marketed.

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.

The updated financial guidance above does not reflect any potential impacts from the evolving COVID-19 pandemic. The company is following the development diligently to assess the financial significance of this pandemic to UCB.