

## UCB announces positive Phase 3 results for rozanolixizumab in generalized myasthenia gravis

- *Positive topline results from UCB MycarinG study investigating the efficacy and safety of rozanolixizumab in patients with generalized myasthenia gravis (gMG)*
- *Study met primary and all secondary endpoints with statistical significance*
- *Rozanolixizumab was well tolerated with no new observed safety signals*
- *UCB plans regulatory submissions for rozanolixizumab in gMG from Q3 2022*

**Brussels, Belgium December 10th, 2021 – 07:00 CET: Regulated Information – Inside Information** –UCB, a global biopharmaceutical company, today announced positive topline results from the Phase 3 MycarinG study<sup>1</sup> evaluating rozanolixizumab, a subcutaneously (SC) infused monoclonal antibody targeting the neonatal Fc receptor (FcRn), versus placebo in adults with generalized myasthenia gravis (gMG).

The trial met its primary endpoint, demonstrating a statistically significant and clinically meaningful change from baseline in the Myasthenia Gravis-Activities of Daily Living (MG-ADL) total score at Day 43. All secondary endpoints were also met with statistical significance.

Overall rozanolixizumab was well tolerated and no new safety signals were identified.

The safety and efficacy of rozanolixizumab have not been established, and it is not approved for use in any indication by any regulatory authority worldwide. The final Phase 3 data from the study and additional details will be presented at a forthcoming medical meeting in 2022.

Based on these results, UCB anticipates regulatory filings in the U.S., European Union and Japan from Q3 2022.

*“Today’s encouraging findings from the MycarinG study show the potential of rozanolixizumab in the treatment of myasthenia gravis, and further reinforce the suggestion that FcRn inhibition may be a promising approach for this disease,”* explained Professor Vera Brill, MycarinG study Lead Investigator, Professor of Medicine (Neurology), University of Toronto, and Director of the Neuromuscular Section, Division of Neurology, University of Toronto and University Health Network, Toronto, Canada.

Alongside rozanolixizumab, UCB is also investigating whether its developmental medicine zilucoplan, a peptide inhibitor of complement component 5 (C5 inhibitor), could deliver patient value to people living with gMG. Preliminary results from the company’s RAISE study are expected in the coming weeks. The safety and efficacy of zilucoplan have not been established, and it is not approved for use in any indication by any regulatory authority worldwide.

*“For the many thousands of people living with myasthenia gravis around the world, current treatment options can be very limited,”* said Samantha Masterson, Chief Executive Officer of the Myasthenia Gravis Foundation of America (MGFA). *“Given that this disease causes a wide range of symptoms, some of which can require urgent intervention or hospitalization,*

*there is a critical need for new treatment options that could address the unmet needs of patients living with myasthenia gravis.”*

UCB’s portfolio approach of two medicines with different but potentially complimentary mechanisms of action creates a unique opportunity for UCB to deliver choice, flexibility and impact to patients and healthcare professionals, giving them options best suited to their individual needs.

*“We are enthusiastic about these positive and clinically meaningful results, which mark a critical step forward for rozanolixizumab and UCB’s commitment to delivering differentiated solutions for people living with rare diseases, such as myasthenia gravis,”* said Iris Loew-Friedrich, Executive Vice-President and Chief Medical Officer at UCB. *“In line with our ambition to deliver a portfolio of treatment options which could improve and simplify the treatment experience for patients and physicians, we are committed to bringing transformational outcomes and experiences to those in need. We wholeheartedly thank the MG community for their ongoing partnership and participation in this study.”*

### **About Generalized Myasthenia Gravis (gMG)**

gMG is a rare, chronic and unpredictable auto-immune disease in which pathogenic autoantibodies can inhibit synaptic transmission at the neuro-muscular junction by targeting specific proteins on the post-synaptic membrane. This disrupts the way that the nerves can communicate with muscles. gMG can occur at any age and in any race, although previous studies have shown that women are more often impacted than men.<sup>2</sup>

Myasthenia gravis is a rare disease impacting almost 200,000 patients in the U.S., EU and Japan.<sup>3,4</sup> People living with gMG can experience a variety of symptoms, including drooping eyelids, double vision and difficulty swallowing, chewing and talking, as well as severe muscular weakness that can result in life threatening weakness of the muscles of respiration.<sup>5-8</sup>

### **About the rozanolixizumab MycarinG study<sup>9</sup>**

The MycarinG study (NCT03971422) is a multi-center, Phase 3, randomized, double-blind, placebo-controlled study evaluating the efficacy and safety of rozanolixizumab in adult patients with gMG, with an open-label extension.

The primary endpoint for the MycarinG study is change in the Myasthenia Gravis-Activities of Daily Living Profile (MG-ADL) score, an eight-item patient-reported scale developed to assess MG symptoms and their effects on daily activities. Secondary endpoints include response rates, changes in the Myasthenia Gravis composite (MGC) score, the Quantitative MG (QMG) score, patient-reported outcomes and adverse events (AEs).

For more information about the trial, visit <https://clinicaltrials.gov/ct2/show/NCT03971422>.

### **About the zilucoplan RAISE study<sup>10</sup>**

The RAISE study (NCT04115293) is a multi-center, Phase 3, randomized, double-blind, placebo-controlled study to confirm the efficacy, safety, and tolerability of zilucoplan in patients with gMG. Patients will be randomized in a 1:1 ratio to receive daily subcutaneous (SC) doses of zilucoplan or placebo for 12 weeks.

The primary endpoint for RAISE study is change from baseline to Week 12 in the Myasthenia Gravis-Activities of Daily Living (MG-ADL) score. Secondary endpoints include change in the Quantitative Myasthenia Gravis (QMG) score, the Myasthenia Gravis

Composite (MGC) and the Myasthenia Gravis Quality of Life 15 revised (MG-QoL15r) from baseline to Week 12; the proportion requiring rescue therapy; the proportion with minimum symptom expression (MSE) (defined as MG-ADL of 0 or 1), the proportion with a  $\geq 3$ -point reduction in MG-ADL and the proportion with a  $\geq 5$ -point reduction in QMG, all measured at Week 12.

For more information about the trial visit <https://clinicaltrials.gov/ct2/show/NCT04115293>.

### **About Rozanolixizumab**

Rozanolixizumab is a SC administered, humanized monoclonal antibody that specifically binds, with high affinity, to human neonatal Fc receptor (FcRn). It has been designed to block the interaction of FcRn and Immunoglobulin G (IgG), accelerating the catabolism of antibodies and reducing the concentration of pathogenic IgG autoantibodies.<sup>11,12</sup>

Rozanolixizumab is under clinical development with the aim of improving the lives of people with pathogenic IgG-autoantibody-driven autoimmune diseases, including gMG, primary immune thrombocytopenia (ITP), myelin oligodendrocyte glycoprotein antibody-associated disease (MOG-AD) and auto immune encephalitis (AIE) by driving removal of pathogenic IgG autoantibodies.

The safety and efficacy of rozanolixizumab have not been established and it is not approved for use in any indication by any regulatory authority worldwide.

### **About Zilucoplan**

Zilucoplan is a once-daily self-administered SC peptide inhibitor of complement component 5 (C5 inhibitor) under clinical development by UCB in gMG. Topline results from the RAISE study, a multi-center, Phase 3, randomized, double-blind, placebo-controlled study to confirm the efficacy, safety, and tolerability of zilucoplan in subjects with gMG, are expected in H1 2022.

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 ALS platform study sponsored by the Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital, Boston, MA.

The safety and efficacy of zilucoplan have not been established and it is not currently approved for use in any indication by any regulatory authority worldwide.

### **About UCB in Rare Diseases**

At UCB, we don't just see patients or population sizes, we see people in need. Through decades of serving the neurology and immunology communities, we have improved lives with impactful medicines and by enhancing the social and emotional well-being of patients. As a continuation of our heritage, we are now expanding our efforts to tackle rare neurological and immunological diseases where current options offer little hope, including investigational treatments for primary ITP, gMG, MOG-AD and AIE.

## **About UCB**

UCB, Brussels, Belgium ([www.ucb.com](http://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

## **For further information, contact UCB:**

### **Brand Communications**

Jim Baxter  
Rare Disease Communications, UCB  
T+32.2.473.78.85.01 [jim.baxter@ucb.com](mailto:jim.baxter@ucb.com)

### **Corporate Communications**

Laurent Schots  
Media Relations, UCB  
T+32.2.559.92.64 [Laurent.schots@ucb.com](mailto:Laurent.schots@ucb.com)

### **Investor Relations**

Antje Witte  
Investor Relations, UCB  
T +32.2.559.94.14 [antje.witte@ucb.com](mailto:antje.witte@ucb.com)

## **Forward looking statements UCB**

This press release may contain forward-looking statements including, without limitation, statements containing the words “believes”, “anticipates”, “expects”, “intends”, “plans”, “seeks”, “estimates”, “may”, “will”, “continue” and similar expressions. These forward-looking statements are based on current plans, estimates and beliefs of management. All statements, other than statements of historical facts, are statements that could be deemed forward-looking statements, including estimates of revenues, operating margins, capital expenditures, cash, other financial information, expected legal, arbitration, political, regulatory or clinical results or practices and other such estimates and results. By their nature, such forward-looking statements are not guarantees of future performance and are subject to known and unknown risks, uncertainties and assumptions which might cause the actual results, financial condition, performance or achievements of UCB, or industry results, to differ materially from those that may be expressed or implied by such forward-looking statements contained in this press release. Important factors that could result in such differences include: the global spread and impact of COVID-19, changes in general economic, business and competitive conditions, the inability to obtain necessary regulatory approvals or to obtain them on acceptable terms or within expected timing, costs associated with research and development, changes in the prospects for products in the pipeline or under development by UCB, effects of future judicial decisions or governmental investigations, safety, quality, data integrity or manufacturing issues; potential or actual data security and data privacy breaches, or disruptions of our information technology systems, product liability claims, challenges to patent protection for products or product candidates, competition from other products including biosimilars, changes in laws or regulations, exchange rate fluctuations, changes or uncertainties in tax laws or the administration of such laws, and hiring and retention of its employees. There is no guarantee that new product candidates will be discovered or identified in the pipeline, will progress to product approval or

that new indications for existing products will be developed and approved. Movement from concept to commercial product is uncertain; preclinical results do not guarantee safety and efficacy of product candidates in humans. So far, the complexity of the human body cannot be reproduced in computer models, cell culture systems or animal models. The length of the timing to complete clinical trials and to get regulatory approval for product marketing has varied in the past and UCB expects similar unpredictability going forward. Products or potential products which are the subject of partnerships, joint ventures or licensing collaborations may be subject to differences disputes between the partners or may prove to be not as safe, effective or commercially successful as UCB may have believed at the start of such partnership. UCB' efforts to acquire other products or companies and to integrate the operations of such acquired companies may not be as successful as UCB may have believed at the moment of acquisition. Also, UCB or others could discover safety, side effects or manufacturing problems with its products and/or devices after they are marketed. The discovery of significant problems with a product similar to one of UCB's products that implicate an entire class of products may have a material adverse effect on sales of the entire class of affected products. Moreover, sales may be impacted by international and domestic trends toward managed care and health care cost containment, including pricing pressure, political and public scrutiny, customer and prescriber patterns or practices, and the reimbursement policies imposed by third-party payers as well as legislation affecting biopharmaceutical pricing and reimbursement activities and outcomes. Finally, a breakdown, cyberattack or information security breach could compromise the confidentiality, integrity and availability of UCB's data and systems.

Given these uncertainties, you should not place undue reliance on any of such forward-looking statements. There can be no guarantee that the investigational or approved products described in this press release will be submitted or approved for sale or for any additional indications or labelling in any market, or at any particular time, nor can there be any guarantee that such products will be or will continue to be commercially successful in the future.

UCB is providing this information, including forward-looking statements, only as of the date of this press release and it does not reflect any potential impact from the evolving COVID-19 pandemic, unless indicated otherwise. UCB is following the worldwide developments diligently to assess the financial significance of this pandemic to UCB. UCB expressly disclaims any duty to update any information contained in this press release, either to confirm the actual results or to report or reflect any change in its forward-looking statements with regard thereto or any change in events, conditions or circumstances on which any such statement is based, unless such statement is required pursuant to applicable laws and regulations.

Additionally, information contained in this document shall not constitute an offer to sell or the solicitation of an offer to buy any securities, nor shall there be any offer, solicitation or sale of securities in any jurisdiction in which such offer, solicitation or sale would be unlawful prior to the registration or qualification under the securities laws of such jurisdiction.

**Job code:** GL-N-RZ-MG-2100009

**Date of preparation:** 09 December 2021

<sup>1</sup> Data on file. UCB. December 2021.

<sup>2</sup> Myasthenia Gravis Foundation of America. Clinical Overview of MG.

<https://myasthenia.org/Professionals/Clinical-Overview-of-MG>. Accessed November 2021.

<sup>3</sup> Chen J, et al. *Incidence, mortality, and economic burden of myasthenia gravis in China: A nationwide population-based study*. *Lancet Reg Health West Pac.* 2020;5:10063.

<sup>4</sup> Gilhus N. *Myasthenia Gravis*. *N Engl J Med.* 2016;375:2570-2581.<sup>5</sup> Lisak, RP. *Best Practice Myasthenia gravis*. *BMJ Best Practice.* 2021. Last accessed December 2021

<sup>6</sup> Robertson NP, et al. *Myasthenia gravis: a population based epidemiological study in Cambridgeshire, England*. *J Neurol Neurosurg Psychiatry.* 1998;65:492-496.

<sup>7</sup> Kupersmith MJ et al. *Development of generalized disease at 2 years in patients with ocular myasthenia gravis*. *Arch Neurol.* 2003;60(2):243-248.

<sup>8</sup> Hansen JS, et al. *Mortality in myasthenia gravis: A nationwide population-based follow-up study in Denmark*. *Muscle Nerve.* 2016;53:73-77.

<sup>9</sup> Clinical Trials.gov 'A Study to Test Efficacy and Safety of Rozanolixizumab in Adult Patients With Generalized Myasthenia Gravis': <https://clinicaltrials.gov/ct2/show/NCT03971422>. Accessed November 2021.

<sup>10</sup> Clinical Trials.gov 'Safety, Tolerability, and Efficacy of Zilucoplan in Subjects With Generalized Myasthenia Gravis (RAISE)': <https://clinicaltrials.gov/ct2/show/NCT04115293>. Accessed November 2021.

<sup>11</sup> Kiessling P, et al. *The FcRn inhibitor rozanolixizumab reduces human serum IgG concentration: A randomized phase 1 study*. *Sci Transl Med.* 2017;9(414).

<sup>12</sup> Smith B, et al. *Generation and characterization of a high affinity anti-human FcRn antibody, rozanolixizumab, and the effects of different molecular formats on the reduction of plasma IgG concentration*. *MAbs.* 2018;10:1111-30.