UCB News

UCB Presents Latest Data from Generalized Myasthenia Gravis Portfolio at 9th Congress of the European Academy of Neurology (EAN) Meeting

- **Results presented across UCB's generalized myasthenia gravis (gMG) portfolio**
- **Expanded data from the MycarinG and RAISE studies showcase further insights on potential of rozanolixizumab and zilucoplan in generalized myasthenia gravis (gMG)**
- **Presentations follow recent U.S. FDA approval of rozanolixizumab for treatment of generalized myasthenia gravis (gMG) in adult patients who are anti-acetylcholine receptor (AChR) or anti-muscle-specific tyrosine kinase (MuSK) antibody positive and expand evidence base in gMG**

Brussels, Belgium, 30th June 2023 – 18:30 PM CEST– UCB, a global biopharmaceutical company, today announced it will present results from across its portfolio in generalized myasthenia gravis (gMG) at the European Academy of Neurology (EAN) Meeting July 1-4, 2023.

Additional results from the MycarinG and RAISE studies,[1,2] and their open-label extension studies will be presented, investigating UCB's rozanolixizumab, an SC-infused monoclonal antibody targeting the neonatal Fc receptor (FcRn)[3,4] and zilucoplan, a self-administered, subcutaneous (SC) peptide inhibitor of complement component 5 (C5 inhibitor) in adults with gMG.[5] These pivotal Phase 3 trials supported U.S., European, and Japanese regulatory filings of both rozanolixizumab and zilucoplan. Presentations will also focus on the importance of real-world data and its use in better understanding current patient experiences. In total, nine abstracts will be presented, including three oral presentations.

In June, rozanolixizumab was approved by the U.S. Food and Drug Administration (FDA), for the treatment of generalized myasthenia gravis (gMG) in adult patients who are anti-acetylcholine receptor (AchR) or anti-muscle-specific tyrosine kinase (MuSK) antibody positive,[6] having been granted Priority Review for its Biologic License Application (BLA). In the U.S., under the FDAs non-proprietary naming of biological products guidance, the nonproprietary name for this medicine is rozanolixizumab-noli. It is an FDA-approved treatment for both anti-AChR and anti-MuSK antibody-positive gMG, the two most common subtypes of gMG. Rozanolixizumab is currently only approved in the U.S., and is under review by the European Medicines Agency (EMA) and the Japanese Pharmaceuticals and Medical Devices Agency (PMDA) for the treatment of adults with gMG. Responses from regulatory agencies to these submissions are expected by H1 2024.

Zilucoplan is currently under review by the U.S. FDA, EMA and the Japanese PMDA for the treatment of adult gMG patients who are anti-acetylcholine receptor (AChR) antibody positive. Responses from these regulatory agencies are expected from H2 2023 onwards. 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.

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These data further inform UCB's innovative approach to evolving science into meaningful solutions that help improve outcomes and address unmet needs of people living with gMG.

Donatello Crocetta, Head of Global Rare Disease & Rare Medical, UCB, explained: "*The additional results from the Phase 3 MycarinG and RAISE studies demonstrate UCB's dedication to finding treatment options for patients with gMG and reinforce the depth and strength of our expanding rare disease pipeline and portfolio. Following on from our recent approval for rozanolixizumab in the U.S. for the treatment of adult patients who are anti-acetylcholine receptor or anti-muscle-specific tyrosine kinase antibody positive gMG, we're very excited to be collaborating with the gMG community to extend knowledge about new medicines which could, in the future, play an important role in the treatment of this rare neuromuscular disease*."

Data from UCB's extensive work to better understand the burden of gMG in different European countries will also be presented in 4 posters. Additionally, through an immersive VR experience co-created with the members of the gMG community, UCB will enable HCPs to gain a deeper understanding of the daily challenges experienced by people living with gMG.

"*Because of the high disease and treatment burden that gMG elicits along with the impact on a person's daily life, it is important for us to contribute and improve our wealth of knowledge of this debilitating rare disease - especially relevant following Myasthenia Gravis Awareness Month by focusing - on patients and digital innovations, with the aim to address the needs of people living with gMG*." said Manuela Maronati, Head of Europe, Rare Disease, UCB. "*As well as increasing our understanding of gMG, we hope to continue to work towards bringing new treatment options to patients. We are looking forward to hearing from the European Medicines Agency on their review of the Marketing Authorization Application for both rozanolixizumab and zilucoplan*."

UCB presentations during EAN 2023

Presenting author	**Abstract title**	**Presentation Details (Timings EST)**
Renato Mantegazza	Response to zilucoplan in the Phase 3 RAISE study in patients with generalized myasthenia gravis	Oral Presentation EPR-132 July 2, 2023 2:25 - 2:30 PM
Maria Isabel Leite	Long-term safety, efficacy and self-injection satisfaction with zilucoplan in myasthenia gravis: An interim analysis of RAISE-XT	Poster Presentation EPO-219 July 2, 2023 1:06 – 1:09 PM
John Vissing	Rozanolixizumab responder and minimal symptom expression rates in generalized MG: Pooled Phase 3 and extension studies	Poster Presentation EPO-412 July 3, 2023 1:33 – 1:36 PM
Sabrina Sacconi	Rozanolixizumab in muscle-specific kinase autoantibody-positive myasthenia gravis: Further analyses from MycarinG study	Poster Presentation EPO-391 July 3, 2023 1:09 – 1:12 PM
David Reyes Leiva	Long-term follow up of generalized Myasthenia Gravis patients during 1998-2020 in a Spanish referral Unit	Poster Presentation EPO-231

		July 2, 2023 1:42 – 1:45 PM
Sophie Barry	Is Virtual Reality an effective educational tool to enhance patient-physician dialogue? ENGAGE learning intervention.	Oral Presentation EPR-059 July 1, 2023 2:00 – 2:05 PM
John Vissing	Incidence, prevalence, and mortality of myasthenia gravis: A population-based study in Denmark, Finland, and Sweden	Oral Presentation EPR-298 July 3, 2023 2:10 – 2:15 PM
Mari Savolainen	Comorbidities among myasthenia gravis patients: A population-based observational study in Denmark, Finland, and Sweden	Poster Presentation EPO-011 July 1, 2023 1:18 – 1:21 PM
Malin Petersson	Correlation of disease activity and EQ-5D-3L-derived utility in myasthenia gravis patients in a Swedish national cohort	Poster Presentation EPO-239 July 2, 2023 1:21 – 1:24 PM

About Generalized Myasthenia Gravis (gMG)

gMG is a rare disease with a global prevalence of 100–350 cases per every 1 million people.[7] People living with gMG can experience a variety of symptoms, including severe muscular weakness that can result in life-threatening weakness of the muscles of respiration, double vision, drooping eyelids, and difficulty swallowing, chewing and talking.[8,9]

In gMG, pathogenic autoantibodies can impair synaptic transmission at the neuromuscular junction (NMJ) by targeting specific proteins on the post-synaptic membrane.[3,10] This disrupts the ability of the nerves to stimulate the muscle and results in a weaker contraction. gMG can occur in any race, gender or age.[11]

About rozanolixizumab

Rozanolixizumab is a subcutaneous 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.[12]

Outside of the U.S. rozanolixizumab is not approved for use in any indication by any other regulatory authority worldwide.

Important U.S. Safety Information for RYSTIGGO®

WARNINGS AND PRECAUTIONS

Infections: RYSTIGGO may increase the risk of infection. Delay RYSTIGGO administration in patients with an active infection until the infection is resolved. During treatment with RYSTIGGO, monitor for clinical signs and symptoms of infection. If serious infection occurs, administer appropriate treatment and consider withholding RYSTIGGO until the infection has resolved.

Immunization

Immunization with vaccines during RYSTIGGO treatment has not been studied. The safety of immunization with live or live-attenuated vaccines and the response to immunization with any vaccine are unknown. Because RYSTIGGO causes a reduction in IgG levels, vaccination with live-attenuated or live vaccines is not recommended during treatment with RYSTIGGO. Evaluate the need to administer age-appropriate vaccines according to immunization guidelines before initiation of a new treatment cycle with RYSTIGGO.

Aseptic Meningitis: Serious adverse reactions of aseptic meningitis (also called drug-induced aseptic meningitis) have been reported in patients treated with RYSTIGGO. If symptoms consistent with aseptic meningitis develop, diagnostic workup and treatment should be initiated according to the standard of care.

Hypersensitivity Reactions: Hypersensitivity reactions, including angioedema and rash, were observed in patients treated with RYSTIGGO. Management of hypersensitivity reactions depends on the type and severity of the reaction. Monitor patients during treatment with RYSTIGGO and for 15 minutes after for clinical signs and symptoms of hypersensitivity reactions. If a reaction occurs, institute appropriate measures if needed.

ADVERSE REACTIONS

In a placebo-controlled study, the most common adverse reactions (reported in at least 10% of RYSTIGGO-treated patients) were headache, infections, diarrhea, pyrexia, hypersensitivity reactions, and nausea. Serious infections were reported in 4% of patients treated with RYSTIGGO. Three fatal cases of pneumonia were identified, caused by COVID-19 infection in two patients and an unknown pathogen in one patient. Six cases of infections led to discontinuation of RYSTIGGO.

Full U.S. Prescribing Information is available at https://www.ucb-usa.com/RYSTIGGO-prescribing-information.pdf

About zilucoplan

Zilucoplan is a once-daily subcutaneous (SC), self-administered peptide inhibitor of complement component 5 (C5 inhibitor) under clinical development by UCB in gMG. As a C5 inhibitor, zilucoplan inhibits complement-mediated damage to the neuromuscular junction through its targeted dual mechanism of action.[13] In 2019, the U.S. FDA granted orphan drug designation to zilucoplan for the treatment of myasthenia gravis.[14] Orphan designation was granted in July 2022 by the European Commission to zilucoplan for the treatment of myasthenia gravis.[15] 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.

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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 approximately 8 600 people in approximately 40 countries, the company generated revenue of € 5.8 billion in 2021. UCB is listed on Euronext Brussels (symbol: UCB). Follow us on Twitter: @UCB_news

Forward-looking statements
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's 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. 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Inspired by **patients.**
Driven by **science.**

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