UCB News

RYSTIGGO®▼(rozanolixizumab), for generalized myasthenia gravis (gMG), receives EU approval for two new administration methods

- RYSTIGGO® can now be self-administered, or administered by a caregiver, with an infusion pump or manual push with a syringe, after receiving training from a healthcare professional.[1]
- Self-administered subcutaneous treatment may offer many advantages, including high patient satisfaction, sense of control, and increased independence as patients do not need to attend regular clinic visits.[2]
- Approval underscores UCB's commitment to providing people living with generalized myasthenia gravis, and their caregivers, with treatments that suit their individual needs and preferences.

Brussels (Belgium) 31 January, 2025 – 07:00 AM (CET) – UCB, a global biopharmaceutical company, today announced the CHMP (Committee for Medicinal Products for Human Use) has issued a positive opinion for the self-administration of RYSTIGGO® (rozanolixizumab) via an infusion (syringe pump) or a new manual push syringe method, after training from a healthcare professional.

In the EU, rozanolixizumab is indicated as an add-on to standard therapy 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.[1]

"For people living with gMG, unpredictable symptoms can have a significant impact on daily life, leading to patients feeling vulnerable and lacking control. Subcutaneous self-administration may help address these challenges, enhancing patient autonomy and satisfaction by reducing the need for frequent clinic visits," said Donatello Crocetta, Chief Medical Officer and Head of Global Medical Affairs, UCB. "We welcome the EU approval for self-administration of rozanolixizumab in Europe, marking another significant step forward in our ongoing commitment to improving the lives of people living with gMG."

This approval is based on several studies, including a Phase 3, open-label, crossover study to evaluate the ability of patients with generalized myasthenia gravis (gMG) to successfully self-administer rozanolixizumab after training in the self-administration technique using the syringe driver and manual push methods.[3] The manual push method allows administration at a flow rate that is comfortable for the patient to accommodate individual preferences.[1] In clinical trials, infusion times by manual push for rozanolixizumab ranged from 1 to 30 minutes with a median infusion time of just 5 minutes per patient. This range of infusion times may serve as a guide when training the patient or caregiver.[1]

Self-administration of rozanolixizumab is also being reviewed by the Japanese Pharmaceuticals and Medical Devices Agency (PMDA), reflecting UCB's global efforts to address the diverse, unmet needs of the gMG community.

Currently, rozanolixizumab is administered by the patient's clinician as a subcutaneous infusion once weekly for six weeks using infusion pump/syringe pumps, and the recommended total weekly dose of rozanolixizumab is based on the patient's body weight.[1]

About rozanolixizumab

Rozanolixizumab 140 mg/ml solution for injection* is a subcutaneously administered, humanized IgG4 monoclonal antibody that specifically binds with high affinity to human neonatal Fc receptor (FcRn). It has been

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designed to block the interaction of FcRn and Immunoglobulin G (IgG), accelerating the catabolism of antibodies and reducing the concentration of pathogenic IgG autoantibodies.[4]

In January 2024, the European Commission (EC) granted a marketing authorization for RYSTIGGO® (rozanolixizumab) as an add-on to standard therapy 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.[1]

* Each ml of solution for injection contains 140 mg of rozanolixizumab. One vial of 2 ml contains 280 mg of rozanolixizumab. One vial of 3 ml contains 420 mg of rozanolixizumab. One vial of 4 ml contains 560 mg of rozanolixizumab. One vial of 6 ml contains 840 mg of rozanolixizumab.

This positive opinion is a type II variation to the terms of the marketing authorisation.

About generalized myasthenia gravis (gMG)
gMG is a rare autoimmune neuromuscular junction disease with a global prevalence of 100–350 cases per 1 million people.[5] People living with gMG can experience a variety of symptoms, including severe muscular weakness that can result in double vision, drooping eyelids, difficulty with swallowing, chewing and talking, as well as life-threatening weakness of the muscles of respiration.[6,7]

In MG, pathogenic autoantibodies can impair synaptic transmission at the neuromuscular junction (NMJ) by targeting specific proteins on the post-synaptic membrane.[8] This disrupts the ability of the nerves to stimulate the muscle and results in a weaker contraction.[9] gMG can occur in any race, gender or age.[6]

About the MG0020 study[3]
MG0020 was a Phase 3, open-label, randomized, two-period, two-sequence crossover study to evaluate the ability of study participants with generalized myasthenia gravis (gMG) to successfully self-administer rozanolixizumab after training in the self-administration technique using the syringe driver and manual push methods. For more information about the trial, visit https://clinicaltrials.gov/study/NCT05681715.

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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 9,000 people in approximately 40 countries, the company generated revenue of € 5.3 billion in 2023. UCB is listed on Euronext Brussels (symbol: UCB). Follow us on Twitter: @UCB_news.

Important Safety Information about RYSTIGGO®▼ (rozanolixizumab) in the EU/EEA[1]*
▼ *This medicinal product is subject to additional monitoring. This will allow quick identification of new safety information. Healthcare professionals are asked to report any suspected adverse reactions.*

The most commonly reported adverse reactions were headache (48.4%), diarrhoea (25.0%) and pyrexia (12.5%). The adverse reactions from clinical studies in gMG are as follows: Very common (≥1/10) headache, diarrhoea, and pyrexia; Common (≥1/100 to <1/10) upper respiratory tract infections, including cases of nasopharyngitis, rash, angioedema, arthralgia, and injection site reactions; Not known, aseptic meningitis (from spontaneous post-marketing reporting). In MG0003, headache was the most common reaction reported in 31 (48.4%) and 13 (19.4%) of the patients treated with rozanolixizumab and placebo, respectively. All headaches, except 1 (1.6%) severe headache, were either mild (28.1% [n=18]) or moderate (18.8% [n=12]) and there was no increase in incidences of headache with repeated cyclic treatment.

Rozanolixizumab is contraindicated in patients with hypersensitivity to the active substance or to any of the excipients.

Treatment with rozanolixizumab in patients with impending or manifest myasthenic crisis has not been studied. Aseptic meningitis (drug induced aseptic meningitis) has been reported following rozanolixizumab treatment. If symptoms consistent with aseptic meningitis (headache, pyrexia, neck stiffness, nausea, vomiting) occur, diagnostic workup and treatment should be initiated as per standard of care.

As rozanolixizumab causes transient reduction in IgG levels the risk of infections may increase. Treatment with rozanolixizumab should not be initiated in patients with a clinically important active infection until the infection resolves or is adequately treated. During treatment with rozanolixizumab, clinical signs and symptoms of infections should be monitored. If a clinically important active infection occurs, withholding rozanolixizumab until the infection has resolved should be considered.

Infusion reactions such as rash or angioedema may occur. In the clinical trial, these were mild to moderate. Patients should be monitored during treatment with rozanolixizumab and for 15 minutes after the administration is complete for clinical signs and symptoms of hypersensitivity reactions. If a hypersensitivity reaction occurs during administration, rozanolixizumab infusion should be discontinued and appropriate measures should be initiated if needed. Once resolved, administration may be resumed.

Immunisation with vaccines during rozanolixizumab therapy has not been studied. The safety of immunisation with live or live-attenuated vaccines and the response to immunisation with vaccines are unknown. All vaccines should be administered according to immunisation guidelines and at least 4 weeks before initiation of treatment. For patients that are on treatment, vaccination with live or live-attenuated vaccines is not recommended. For all other vaccines, they should take place at least 2 weeks after the last infusion of a treatment cycle and 4 weeks before initiating the next cycle.

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This medicinal product contains 29 mg of proline in each ml. The use in patients suffering from hyperprolinaemia should be restricted to cases where no alternative treatment is available. This medicinal product contains 0.3 mg of polysorbate 80 in each ml. Polysorbates may cause allergic reactions.

Please consult the full prescribing information in relation to other side effects, full safety and prescribing information. https://www.ema.europa.eu/

*EU is an abbreviation for the European Union. EEA is an abbreviation for the European Economic Area.

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

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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.

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