UCB News

# RYSTIGGO® (rozanolixizumab) receives approval in Japan for two new administration methods as UCB begins ONWARD program for patient support

- RYSTIGGO - for the treatment of generalized myasthenia gravis (gMG) in adult patients (only for patients who are not adequately responsive to steroids or nonsteroidal immunosuppressants) - can now be self-administered at home using an infusion pump or manual push with a syringe in Japan, after receiving training from a healthcare professional[1]
- UCB also introduces a home delivery service in Japan through its global ONWARD[2] program to further facilitate patient convenience and accessibility
- Self-administration can offer better patient satisfaction, a sense of control, and increased independence, minimizing the need for regular clinic visits[3]

**Brussels (Belgium) 9 May, 2025 – 07:00 AM (CET)** – UCB, a global biopharmaceutical company, today announced that it has received approval from the Pharmaceuticals and Medical Devices Agency (PMDA) in Japan for at-home self-administration with infusion pump or a new manual push syringe method for RYSTIGGO® (rozanolixizumab). With this approval, people living with generalized Myasthenia Gravis (gMG) can now self-administer RYSTIGGO at home using either a manual push or syringe pump method, following training from a healthcare professional.

Complementing this approval, UCB Japan will launch a home delivery service for RYSTIGGO, including portable syringe pumps, as part of the ONWARD program. ONWARD is a global UCB patient support program designed to assist individuals living with rare diseases like gMG, offering features such as symptom tracking, support by dedicated Care Coordinators for each patient, and 24/7 online tools.[2]

"For people living with gMG, unpredictable symptoms can significantly impact daily life, leading to feelings of vulnerability and loss of control. Subcutaneous self-administration can help address these challenges, providing better control over treatment schedules and enhancing autonomy and satisfaction," said Donatello Crocetta, Chief Medical Officer and Head of Global Medical Affairs. "We are delighted to achieve this approval in Japan and the roll out of the ONWARD home delivery service, which together provide personalized support and reduce the treatment burden for those affected by gMG."

RYSTIGGO was approved for the treatment of generalized myasthenia gravis gMG in adult patients (only for patients who are not adequately responsive to steroids or nonsteroidal immunosuppressants) in Japan in September 2023.[1]

**About ONWARD™**

ONWARD is a personalized support experience built to help people through every step of their prescribed UCB treatment. A key component of ONWARD program is being assigned your own dedicated Care Coordinator who provides tailored support based on an individual's unique needs. The program is not intended for diagnosis, treatment, or equivalent medical acts. Registration information

is strictly managed by our subcontractors, and UCB does not have access to personally identifiable information.

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*Refer to the RYSTIGGO® Japan PMDA. Prescribing Information for adverse reactions and full prescribing information.*

## About RYSTIGGO®▼ (rozanolixizumab) in the EU

▼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

RYSTIGGO binds specifically to the fetal Fc receptor (FcRn) with high affinity, inhibiting the interaction between FcRn and immunoglobulin G (IgG), promoting the catabolism of antibodies, and reducing the blood concentration of IgG, including pathogenic IgG autoantibodies.[4,5] In the Phase 3 global joint trial MycarinG trial, which included Japanese adult patients with gMG, RYSTIGGO showed a statistically significant reduction from baseline in the MG-ADL total score at 43 days compared to the placebo group ($p<0.001$).[6] It was designated as an orphan drug by the Ministry of Health, Labour and Welfare in November 2020.[1]

## About Generalized Myasthenia Gravis (gMG)

gMG is a rare, chronic autoimmune disease characterized by dysfunction and damage at the neuromuscular junction. It is associated with complement, immune cells, and pathogenic IgG autoantibodies. gMG patients have various symptoms such as ptosis, diplopia, dysphagia, difficulty chewing, difficulty speaking, and severe muscle weakness that can lead to life-threatening respiratory muscle weakness.[7,8] The prevalence of MG is estimated to be 100 to 350 per million people worldwide, with 29,210 patients in Japan*.[7,9]



GL-RZ-2500026 Date of preparation: April 2025 

* This Japanese study sent survey sheets to the randomly selected medical departments (n=7,545). The study first asked for the number of MG patients who visited medical departments from January 1, 2017, to December 31, 2017. Then, the study sent the second survey sheet to the medical departments that answered the first survey to obtain the clinical information of patients who received MG diagnosis between January 1, 2015, and December 31, 2017.

## RYSTIGGO®▼ (rozanolixizumab) EU/EEA* Important Safety Information[10]

*▼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 contra-indicated 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



GL-RZ-2500026 Date of preparation: April 2025 

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.

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/*

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

**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 9 000 people in approximately 40 countries, the company generated revenue of € 6.1 billion in 2024. 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



GL-RZ-2500026 Date of preparation: April 2025 

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. 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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GL-RZ-2500026 Date of preparation: April 2025 

**References:**


1. RYSTIGGO® Japan PMDA PI. 2025. https://www.pmda.go.jp/PmdaSearch/iyakuDetail/ResultDataSetPDF/820110_6399432A1028_1_04. Accessed April 2025.
2. ONWARD. UCB ONWARD™. 2025. https://www.ucbonward.com. Accessed April 2025.
3. Menon D, Sarpong E, Bril V. Practical Aspects of Transitioning from Intravenous to Subcutaneous Immunoglobulin Therapy in Neuromuscular Disorders Can J Neurol Sci. 2022;49(2):161–67.
4. Kiessling P, et al. The FcRn inhibitor rozanolixizumab reduces human serum IgG concentration: A randomized phase 1 study. Sci Transl Med. 2017;9(414):eaan1208.
5. Overview of MG. Myasthenia Gravis Foundation of America. 2025. https://myasthenia.org/understanding-mg/overview-mg. Accessed April 2025.
6. Bril V, et al. Safety and efficacy of rozanolixizumab in patients with generalized myasthenia gravis (MycarinG): a randomised, double-blind, placebo controlled, adaptive phase 3 study. Lancet Neurol. 2023;22(5):383-94.
7. Punga AR, et al. Epidemiology, diagnostics, and biomarkers of autoimmune neuromuscular junction disorders. Lancet Neurol. 2022;21(2):176-88.
8. Myasthenia Gravis Fact Sheet. National Institute of Neurological Disorders and Stroke. 2022. https://www.ninds.nih.gov/myasthenia-gravis-fact-sheet. Accessed April 2025.
9. Yoshikawa H, et al. Two-step nationwide epidemiological survey of myasthenia gravis in Japan 2018. PLoS One. 2022;17(9):e0274161.
10. RYSTIGGO® EU SmPC. https://www.ema.europa.eu/en/documents/product-information/rystiggo-epar-product-information_en.pdf. Accessed April 2025.




GL-RZ-2500026 Date of preparation: April 2025 