
UCB News

# UCB Accelerates Anti-FcRn *Rozanolixizumab* in Myasthenia Gravis into Confirmatory Development Phase

- Positive outcomes in proof-of-concept study with subcutaneous *rozanolixizumab* in patients with myasthenia gravis (MG): clinically meaningful improvement in multiple disease-related endpoints
- Strong 68% mean reduction of Serum IgG and IgG Autoantibodies observed
- Safety profile in-line with subcutaneous dosing in phase 1 and the safety profile observed in the proof-of-concept immune thrombocytopenia (ITP) study
- Proof-of-concept has been achieved in myasthenia gravis
- Confirmatory development study with *rozanolixizumab* in patients with myasthenia gravis to start in H2 2019

**Brussels, Belgium – 18 October 2018, 7:00 AM CEST –** UCB today announced positive results from a phase 2 study (MG0002; NCT03052751) with a novel, subcutaneous FcRn (neonatal Fc receptor) monoclonal antibody, *rozanolixizumab*, in patients with myasthenia gravis (MG), achieving proof-of-concept. Based on these results UCB intends to accelerate the development of *rozanolixizumab* with a confirmatory study in MG starting in the second half of 2019.

Professor Vera Bril, MD, University of Toronto, coordinating investigator for the MG0002 study said: “I am very excited about these positive results with subcutaneous *rozanolixizumab*. Today, there is a clear need for safe, effective, non-invasive, non-burdensome therapies for patients with generalized MG, who continue to face serious, potentially life-threatening symptoms associated with their disease.”

Dr Dhaval Patel, Executive Vice President and Head of NewMedicines at UCB said: “First I would like to express my sincere thanks to the patients, investigators, care partners and all those who contributed to this important study. The results strengthen our conviction that reducing pathogenic autoantibodies with the most advanced subcutaneous anti-FcRn therapy in clinical development may offer an innovative approach to improve outcomes and treatment experience for patients with myasthenia gravis. In addition, the results give rise to the expectation of potential therapeutic benefit in other IgG autoantibody-mediated conditions.”

Full data from MG0002 show that subcutaneous infusions of *rozanolixizumab* were safe and well tolerated and resulted in clinical improvement over the entire duration of the study. Clinical benefits were observed across several pre-specified disease-related endpoints, including Quantitative Myasthenia Gravis (QMG) score, Myasthenia Gravis Composite (MGC) responder rate and Myasthenia Gravis-Activities of Daily living (MG-ADL) score.

Building on the potential clinical utility of *rozanolixizumab* in other neurological conditions driven by pathogenic immunoglobulin G (IgG) autoantibodies, UCB will initiate a phase 2 study in patients with chronic inflammatory demyelinating polyneuropathy (CIDP) in the first quarter 2019. UCB is also

advancing development in immune thrombocytopenia (ITP), with results from an on-going dose ranging study expected at the end of 2018.

**Study Design:** MG0002 (NCT03052751) was a Phase 2 randomized, placebo-controlled, proof-of-concept trial that enrolled 43 MG patients from North America and Europe with generalized muscle weakness and a total QMG of at least 11. MG0002 compared three once/week subcutaneous infusions of placebo (N=22) and 7 mg/Kg *rozanolixizumab* (N=21) on days 1, 8, 15 and were compared during a four-week period (dosing period 1).

After dosing period 1, participants were re-randomized to receive either 7 mg/Kg or 4 mg/Kg *rozanolixizumab* on days 29, 36, and 43 (dosing period 2) with continued observation until day 99. Conventional therapies were allowed and included corticosteroids and/or immunomodulatory agents and/or Cholinesterase inhibitors. The protocol included no specific headache prophylaxis and mandated withdrawal of patients with severe headache. Pre-specified analyses of safety and efficacy across both dosing periods looked at the data following six subcutaneous infusions of *rozanolixizumab*.

**Study Results:** At the end of dosing period 1: The baseline-corrected delta in QMG between *rozanolixizumab* and placebo was -0.7 (p = 0.221), the baseline-corrected delta in MGC score was -1.8 (p = 0.089) and the baseline-corrected delta in MG-ADL score was -1.4 (p = 0.036). In a post-hoc analysis, the absolute change from baseline in the MG-ADL score, an established registration endpoint, was -2.0 for *rozanolixizumab* compared to -0.18 for placebo (p = 0.008).

The QMG responder rate was 38.1% compared to 22.7% for placebo (p=0.223), the MGC responder rate was 47.6% compared to 27.3% for placebo (p=0.144), and the MG-ADL responder rate was 47.6% compared to 13.6% for placebo (p=0.017). Response was defined as a reduction of 3 or more points from baseline for all scores.

During dosing period 2, additional clinically meaningful reductions of all scores were observed. Pre-specified analyses across the two dosing periods (i.e. following six subcutaneous infusions of *rozanolixizumab)* showed clinically meaningful patient benefit consistently across several disease-specific endpoints, including QMG, MGC and MG-ADL. Participants on active treatment showed a marked reduction of total IgG levels and IgG autoantibody levels. Serum IgG concentrations reduced by 56% after two weeks of *rozanolixizumab* treatment. Total IgG and anti-acetylcholine receptor (anti-AChR) antibodies decreased by 68% from baseline during dosing period 2 in participants receiving *rozanolixizumab* 7 mg/Kg in both dosing periods.

**Safety profile:** Safety and tolerability of *rozanolixizumab* were confirmed and in-line with subcutaneous dosing in the phase 1 program and the safety profile observed in the proof of concept ITP study. There was an expected greater frequency of headache (57.1%) compared to placebo (13.6%) during dosing period 1. Per protocol, three *rozanolixizumab*-treated participants with headache were withdrawn from the study. All headaches were manageable and resolved with standard therapies. The incidence of infections between *rozanolixizumab* and placebo was similar.

The full data will be presented at a medical congress in the near future and submitted for publication in a peer-reviewed journal.

*Rozanolixizumab* is a novel, subcutaneous anti-FcRn monoclonal antibody in clinical development at UCB and not approved in any region of the world.

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## For further information:


### UCB Corporate Communications

France Nivelle,
Global Communications, UCB

T +32.2.559.9178,
france.nivelle@ucb.com

Laurent Schots
Global Communications, UCB

T +32.2.559.92.64
laurent.schots@ucb.com

### UCB Investor Relations

Antje Witte,
Investor Relations, UCB

T +32.2.559.94.14,
antje.witte@ucb.com

Neil Wallace,
Investor Relations, UCB

T +32.2.386.2869,
neil.wallace@ucb.com


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