Researchers report on rozanolixizumab, zilucoplan phase 3 myasthenia gravis study

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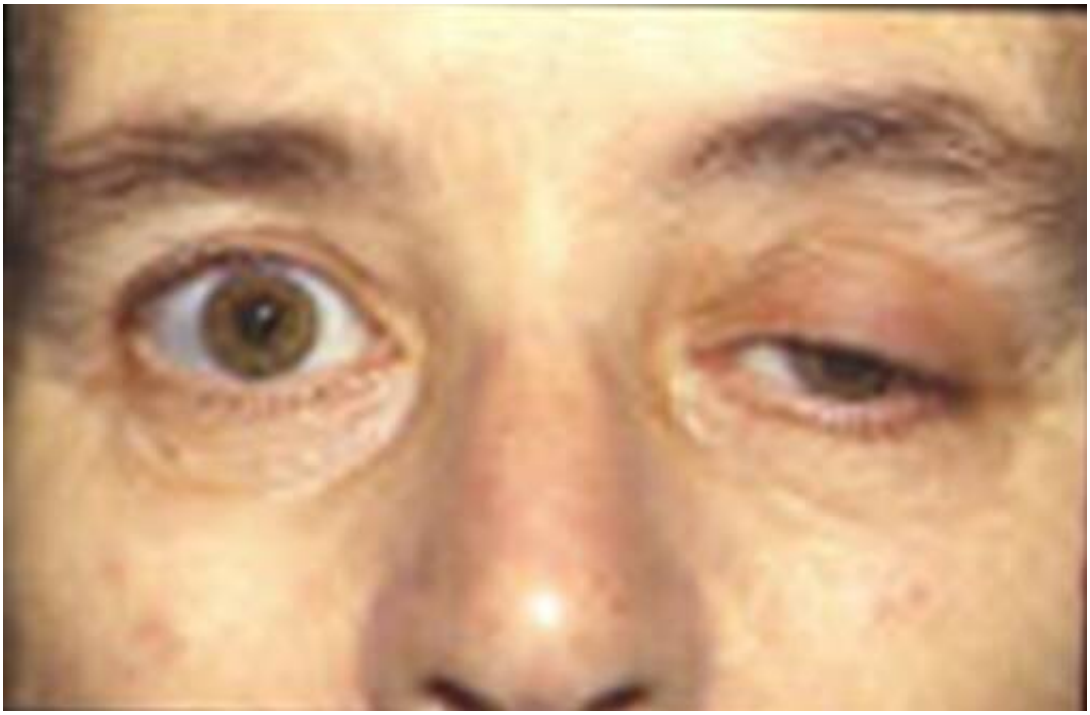

Patient with Myasthenia Gravis. Credit: Cumulus at Dutch Wikipedia, CC BY-SA 3.0 , via Wikimedia Commons

The Lancet Neurology has published data from the phase 3 MycarinG study evaluating the efficacy and safety of rozanolixizumab in adult patients with acetylcholine receptor autoantibody-positive (AChR-Ab+) or muscle-specific tyrosine kinase autoantibody-positive (MuSK-Ab+) generalized myasthenia gravis and the phase 3 RAISE study evaluating

the efficacy and safety of zilucoplan in adult patients with mild to severe AChR-Ab+ gMG.

The RAISE trial was led by James Howard, MD, professor of neurology at the UNC School of Medicine.

Generalized myasthenia gravis is a rare, chronic, and unpredictable auto-immune disease characterized by dysfunction and damage at the neuromuscular junction. Several factors are understood to be drivers of disease pathology.

People living with the condition can experience a variety of symptoms, including drooping eyelids, double vision, and difficulty in swallowing, chewing and talking, as well as severe muscle weakness that can result in life-threatening weakness of the muscles of respiration. It is a rare disease with a global prevalence of 100–350 cases per every 1 million people.

UCB, a global biopharmaceutical company, has been investigating both therapies as part of a broad offering to treat adult patients living with gMG throughout their treatment journey. Each drug has an individual mechanism of action targeting the underlying disease pathology that causes gMG.

The safety and efficacy of rozanolixizumab and zilucoplan have not been established and neither treatment is approved for use in any indication by any regulatory authority. In the RAISE study, according to UCB, zilucoplan demonstrated rapid efficacy, with consistent, sustained, clinically meaningful and statistically significant improvements versus placebo from baseline to week 12 in both patient and clinician-reported endpoints,

Zilucoplan was generally well tolerated with a favorable safety profile,

according to the paper in *The Lancet Neurology*.

"In the RAISE study, zilucoplan showed rapid and clinically meaningful improvements in myasthenia gravis-specific efficacy outcomes, had a favorable safety profile, and was generally well tolerated, with no major safety findings," Howard said. "These results suggest that, if approved in the future, zilucoplan could present a potential new treatment option for a broad population of adult patients with AChR-Ab+ generalized myasthenia gravis."

More information: James F Howard et al, Safety and efficacy of zilucoplan in patients with generalised myasthenia gravis (RAISE): a randomised, double-blind, placebo-controlled, phase 3 study, *The Lancet Neurology* (2023). DOI: 10.1016/S1474-4422(23)00080-7