

Experimental drug found to improve symptoms of generalized myasthenia gravis

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James F. Howard, Jr., MD. Credit: University of North Carolina at Chapel Hill School of Medicine

Argenx, a Belgian drug maker, recently announced a successful phase 3 ADAPT trial of its experimental drug, efgartigimod. The company plans to seek U.S. approval of the drug by the end of year as a potential new treatment for patients with generalized myasthenia gravis (gMG), a rare

and chronic autoimmune disease where communication between nerves and muscles is disrupted, causing debilitating muscle weakness.

Efgartigimod is an [antibody fragment](#) that binds to the neonatal Fc receptor resulting in a reduction of disease-causing immunoglobulin G antibodies.

"The efgartigimod data showed rapid and robust responses in people with gMG, as well as a favorable tolerability profile," said James F. Howard Jr., MD, professor of neurology and principal investigator for the ADAPT trial. "Patients with this devastating disease can experience chronic and potentially life-threatening muscle weakness that has a major impact on their quality of life, and more treatment options are needed. These data are very encouraging as they show efgartigimod has the potential to make a meaningful impact on daily living activities, and we are hopeful they will lead to a new treatment being available for the gMG community."

"With the ADAPT trial, we set out to evaluate efgartigimod's ability to redefine the treatment paradigm for people living with gMG. The data showed that efgartigimod drove fast and deep responses, including in a proportion of patients who achieved minimal or no symptoms after treatment. In addition, we saw responses that lasted beyond eight or 12 weeks, supporting our plans to offer individualized dosing schedules that are purpose-fit to the variability in [disease](#) course that gMG patients experience," commented Wim Parys, M.D., Chief Medical Officer of Argenx. "Based on these data, we intend to submit a BLA for efgartigimod to the FDA before the end of the year, taking us one step closer to potentially making efgartigimod available to patients in 2021. ADAPT is the first pivotal trial of efgartigimod and these data further our confidence in its broad opportunity in other severe, IgG-mediated [autoimmune diseases](#)."

Highlights of topline ADAPT data

- 67.7% of acetylcholine receptor-antibody positive (AChR-Ab+) patients treated with efgartigimod achieved the primary endpoint compared with 29.7% on placebo (p

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