argenx Announces Positive Topline Phase 3 ADAPT Trial Results

News
May 26, 2020

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Breda, the Netherlands / Ghent, Belgium

- Trial met primary endpoint (p <0.0001)
- Well-tolerated; safety profile comparable to placebo
- Biologics License Application on track to be submitted to U.S. Food and Drug Administration by end of 2020
- Conference call scheduled for today, May 26, 2020 at 8:30 a.m. EDT (2:30 p.m. CEST)

argenx (Euronext & Nasdaq: ARGX), a global immunology company committed to improving the lives of people suffering from severe autoimmune diseases and cancer, today announced positive topline data from the pivotal ADAPT trial of efgartigimod. ADAPT met its primary endpoint defined as percentage of responders on the Myasthenia Gravis Activities of Daily Living (MG-ADL) score among acetylcholine receptor-antibody positive (AChR-Ab+) generalized myasthenia gravis (gMG) patients. Responders are defined as having at least a two-point improvement on the MG-ADL score for at least

four consecutive weeks. Based on these results, argenx plans to submit a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) by the end of 2020.

Highlights of topline ADAPT data

- 67.7% of AChR-Ab+ patients treated with efgartigimod achieved the primary endpoint compared with 29.7% on placebo ($p<0.0001$).

- 63.1% of AChR-Ab+ patients responded to efgartigimod compared with 14.1% on placebo on the Quantitative Myasthenia Gravis (QMG) score ($p<0.0001$); responder defined as having at least a three-point improvement on the QMG score for at least four consecutive weeks.

- 40.0% of efgartigimod-treated AChR-Ab+ patients achieved minimal symptom expression defined as MG-ADL scores of 0 (symptom free) or 1, compared to 11.1% treated with placebo.

- Efgartigimod was well-tolerated with a safety profile that was comparable to placebo.

“The efgartigimod data showed rapid and robust responses in people with gMG, as well as a favorable tolerability profile,” said James F. Howard Jr., M.D., Professor of Neurology (Neuromuscular Disease), Medicine and Allied Health, Department of Neurology, The University of North Carolina at Chapel Hill School of Medicine 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 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. All of us at argenx want to thank the patients and healthcare providers who participated in the ADAPT trial. 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.”

Additional ADAPT results, including secondary endpoints and prespecified analyses

- In the ADAPT trial, the secondary endpoints listed below also demonstrated statistically significant differences in the efgartigimod arm for AChR-Ab+ patients, unless otherwise noted, compared to placebo:
    - MG-ADL responders in the overall population, including both AChR-Ab+ and AChR-antibody negative patients ($p<0.0001$).
    - Time on trial in clinically meaningful improvement (MG-ADL improvement ≥2) ($p=0.0001$).
    - Fast onset of response on MG-ADL score (onset observed in first two weeks) ($p=0.0004$).
    - Time to qualify for retreatment endpoint did not meet statistical significance.
- In AChR-Ab+ patients who met the primary endpoint, the majority showed a sustained response, including 88.6% who achieved a response for at least six weeks, 56.8% for at least eight weeks and 34.1% for at least 12 weeks.
- Of AChR-Ab+ patients who received a second treatment cycle, 70.6% were MG-ADL responders compared to 25.6% of placebo patients.
- 90% of patients enrolled in the ADAPT trial continued to the ADAPT-Plus open-label extension study.
- Percentage of efgartigimod responders on the MG-ADL score in the AChR-antibody negative patient population was consistent with the AChR-Ab+ patient population, but a greater placebo response was observed in this cohort.

Detailed data from the ADAPT trial will be submitted for presentation at a future medical meeting.

Phase 3 ADAPT Trial Design

The Phase 3 ADAPT trial was a randomized, double-blind, placebo-controlled, multi-center, global trial evaluating the safety and efficacy of efgartigimod in patients with gMG. A total of 167 adult patients with gMG in North America, Europe and Japan enrolled in the trial and were treated. Enrolled patients had a confirmed gMG diagnosis and an MG-ADL total score of five or greater. Patients were on a stable dose of at least

one gMG treatment prior to randomization, including acetylcholinesterase inhibitors, corticosteroids or nonsteroidal immunosuppressive drugs, and were required to remain on that stable dose throughout the primary trial. Patients were eligible to enroll in ADAPT regardless of antibody status, including patients with AChR antibodies (AChR-Ab+) and patients where AChR antibodies were not detected.

Patients were randomized in a 1:1 ratio to receive efgartigimod or placebo for a total of 26 weeks as part of the primary trial. ADAPT was designed to enable an individualized treatment approach with an initial treatment cycle followed by a variable number of subsequent treatment cycles. Treatment cycles consist of four infusions of efgartigimod (10mg/kg IV) or placebo at weekly intervals. Retreatment with additional treatment cycles was initiated according to clinical response. The primary endpoint was the number of AChR-Ab+ patients who achieved a response on the MG-ADL score defined by at least a two-point improvement for four or more consecutive weeks.

After the 26-week primary ADAPT trial, patients were eligible to roll-over into an open-label extension, ADAPT Plus.

About Efgartigimod

Efgartigimod is a first-in-class antibody fragment designed to reduce disease-causing immunoglobulin G (IgG) antibodies and block the IgG recycling process. Efgartigimod binds to the neonatal Fc receptor (FcRn), which is widely expressed throughout the body and plays a central role in rescuing IgG antibodies from degradation. Blocking FcRn reduces IgG antibody levels representing a logical potential therapeutic approach for several autoimmune diseases known to be driven by disease-causing IgG antibodies, including: myasthenia gravis (MG), a chronic disease that causes muscle weakness; pemphigus vulgaris (PV), a chronic disease characterized by severe blistering of the skin; immune thrombocytopenia (ITP), a chronic bruising and bleeding disease; and chronic inflammatory demyelinating polyneuropathy (CIDP), a neurological disease leading to impaired motor function.

About Myasthenia Gravis (MG)

MG is a rare and chronic autoimmune disease where IgG antibodies disrupt communication between nerves and muscles, causing debilitating and potentially life-threatening muscle weakness. More than 85% of people with MG progress to generalized MG (gMG) within 18 months, where muscles throughout the body may be affected, resulting in extreme fatigue and difficulties with facial expression, speech, swallowing and mobility. In more life-threatening cases, MG can affect the muscles responsible for breathing. Patients with confirmed AChR antibodies account for 80-90% of the total gMG population. There are approximately 65,000 people in the United States and 20,000 people in Japan living with the disease.