Zai Lab Announces Acceptance by China's NMPA of the BLA for Efgartigimod for Patients with Generalized Myasthenia Gravis

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Efgartigimod alfa injection is the first accepted BLA submission of an FcRn antagonist for gMG patients in China

SHANGHAI and SAN FRANCISCO and CAMBRIDGE, Mass., July 13, 2022 (GLOBE NEWSWIRE) -- Zai Lab Limited (NASDAQ: ZLAB; HKEX: 9688), a patient-focused, innovative, commercial-stage, global biopharmaceutical company, today announced that China's National Medical Products Administration (NMPA) has accepted the Biologics License Application (BLA) for efgartigimod alfa injection, a first-in-class neonatal Fc receptor (FcRn) antagonist, for the treatment of adult patients with generalized myasthenia gravis (gMG).

"We are pleased to have the NMPA's acceptance of the BLA filing for efgartigimod alfa injection for intravenous use. This important milestone brings us closer to delivering a truly novel treatment for gMG patients who face many challenges living with this complex and difficult-to-control autoimmune disease," said Dr. Harald Reinhart, President and Head of Global Development, Neuroscience, Autoimmune & Infectious Diseases, Zai Lab. "As a company focused on developing innovative medicines for diseases in need of more effective treatment options, we look forward to collaborating with the NMPA during the review process in a joint effort to address unmet needs of gMG patients in Greater China."

"For the estimated 200,000 people in China living with MG[1], there is a lack of innovative treatment options, representing significant unmet needs in clinical practice. First, current treatment options may fail to address refractory gMG or those likely to progress with life-threatening conditions. Second, long-term use of glucocorticoids or other immunosuppressants may lead to various intolerable side effects. Third, rescue therapies such as intravenous immune globulin (IVIg) or plasma exchange (PLEX) are limited in supply or require patients to go to specialty medical facilities. Therefore, there is an urgent need for a novel, safe, effective therapy to improve the treatment outcome and quality of life for patients," said Dr. Chongbo Zhao, M.D., Ph.D., Deputy Director of Department of Neurology, Huashan Hospital Affiliated to Fudan University, Director of Working Group of Huashan Rare Disease Center. "We are pleased to see efgartigimod accepted for review as it is a drug that directly targets the pathophysiologic core of the disease by reducing disease-causing autoantibodies. In clinical studies, efgartigimod has shown that it can provide fast, deep, and durable clinical response with a good safety profile. We appreciate Zai Lab's investment and continued support of gMG patients in China."

In December 2021 and January 2022, efgartigimod for intravenous use (VYVGART®) was approved by the U.S. Food and Drug Administration (FDA) and Japan's Ministry of Health, Labour and Welfare (MHLW), respectively.

Efgartigimod is the first-and-only approved FcRn blocker in the U.S. as VYVGART (efgartigimod alfa-fcab) for the treatment of adult gMG patients who are anti-acetylcholine receptor (AChR) antibody positive and in Japan for those who do not have sufficient response to steroids or non-steroidal immunosuppressive therapies (ISTs). These approvals were based on the comprehensive PK/PD, efficacy, and safety data from the pivotal Phase 3 ADAPT trial[2].

About efgartigimod alfa injection (VYVGART®)

Efgartigimod alfa injection (brand name VYVGART®) is an antibody fragment designed to reduce disease-causing IgG antibodies and block the IgG recycling process. Efgartigimod binds to 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.

It is the first and only approved FcRn blocker in the United States and in Japan for the treatment of adults with gMG. Efgartigimod is being studied in adults with ITP and other IgG autoantibody-mediated diseases.

Zai Lab has an exclusive license agreement with argenx to develop and commercialize efgartigimod in Greater China.

About the Phase 3 ADAPT Study

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 were enrolled in the trial and treated. Patients were eligible to enroll in ADAPT regardless of antibody status, including patients with AChR antibodies (AChR-Ab+) and patients in whom AChR antibodies were not detected. Patients were randomized in a 1:1 ratio to receive efgartigimod or placebo for a total of 26 weeks. ADAPT was designed to enable an individualized treatment approach with an initial treatment cycle followed by a variable number of subsequent treatment cycles. 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.

About Myasthenia Gravis in China

Myasthenia gravis (MG) is a rare chronic autoimmune disease, characterized by debilitating and potentially life-threatening muscle weakness. There are approximately 200,000 people in China living with the disease[1]. More than 85% of people with MG progress to gMG within 18 months; in this generalized form of the disease, skeletal muscles throughout the body may be affected, resulting in weakness and early fatigue. Difficulties with double vision, facial expression, speech, swallowing, and ambulation are frequent and difficult to manage for patients and treating physicians. In more life-threatening cases, gMG can affect the muscles responsible for breathing, which can be fatal. Acetylcholinesterase (AChE) inhibitors, steroids, immunosuppressants, and IVIg are the mainstay of treatment in China. However, there is a lack of high-level evidence-based recommendations for the treatment of MG, representing significant unmet needs.

[1] *Nationwide population-based epidemiological study of myasthenia gravis in Taiwan, 2010.*

[2] *Safety, efficacy, and tolerability of efgartigimod in patients with generalized myasthenia gravis (ADAPT): a multicenter, randomized, placebo-controlled, phase 3 trial, The Lancet Neurology, 2021.*

About Zai Lab

Zai Lab Limited (NASDAQ: ZLAB; HKEX: 9688) is an innovative, research-based, commercial-stage biopharmaceutical company based in China and the U.S. focused on bringing transformative medicines for oncology, autoimmune disorders, infectious diseases, and neurological disorders to patients in China and around the world. Our goal is to leverage our competencies and resources to positively impact human health worldwide.

For additional information about Zai Lab, including information on our products, business activities and partnerships, research, or other events or developments, please visit www.zailaboratory.com or follow us at www.twitter.com/ZaiLab_Global.

Zai Lab Forward-Looking Statements

This press release contains forward-looking statements about clinical trials, data readouts and presentations, our clinical development programs, including our research and development program for the treatment of myasthenia gravis in China; the potential of our commercial business and pipeline programs, and the risk and uncertainties associated with drug development and commercialization. These forward-looking statements include, without limitation, statements containing words such as “aim,” “anticipate,” “believe,” “could,” “estimate,” “expect,” “forecast,” “goal,” “intend,” “may,” “plan,” “possible,” “potential,” “will,” “would” and other similar expressions. Such statements constitute forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements are not statements of historical fact nor are they guarantees or assurances of future performance. Forward-looking statements are based on our expectations and assumptions as of the date of this press release and are subject to inherent uncertainties, risks, and changes in circumstances that may differ materially from those contemplated by the forward-looking statements. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including but not limited to (1) our ability to successfully commercialize and generate revenue from our approved products, (2) our ability to obtain funding for our operations and business initiatives, (3) the results of our clinical and pre-clinical development of our product candidates, (4) the content and timing of decisions made by the relevant regulatory authorities regarding regulatory approvals of our product candidates, (5) the effects of the novel coronavirus (COVID-19) pandemic on our business and general economic, regulatory and political conditions, (6) risks related to doing business in China, and (7) other factors identified in our most recent annual and quarterly reports and in other reports we have filed with the U.S. Securities and Exchange Commission. We anticipate that subsequent events and developments will cause our expectations and assumptions to change, and we undertake no obligation to update or revise any forward-looking statements, whether as a result of new information, future events, or otherwise, except as may be required by law. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this press release.

Our SEC filings can be found on our website at www.zailaboratory.com and the SEC’s website at www.sec.gov.

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