Zai Lab Announces Acceptance of Supplemental Biologics License Application with Priority Review for Efgartigimod Alfa Injection (Subcutaneous Injection) in Chronic Inflammatory Demyelinating Polyneuropathy (CIDP) in China

May 14, 2024

sBLA submission based on positive results from the ADHERE trial, the first positive global neonatal FC receptor (FcRn) pivotal study for CIDP

There are currently no approved therapies available in China for this serious autoimmune disease

Milestone underscores Zai Lab's operational capabilities and deep expertise developing and commercializing innovative treatments in China across a broad range of diseases

SHANGHAI & CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 14, 2024-- Zai Lab Limited (NASDAQ: ZLAB; HKEX: 9688) today announced that the Center for Drug Evaluation (CDE) of the China National Medical Products Administration (NMPA) has accepted the supplemental Biologics License Application (sBLA) for efgartigimod alfa injection (subcutaneous injection) (efgartigimod SC) for the treatment of chronic inflammatory demyelinating polyneuropathy (CIDP). The CDE granted priority review on May 11, 2024 and Breakthrough Therapy Designation for efgartigimod SC for the treatment of patients with CIDP on September 18, 2023.

"The sBLA acceptance with priority review designation brings us one step closer to providing a treatment option for patients with CIDP in China, a serious disease that affects approximately 50,000 diagnosed patients, with only a small fraction of patients able to achieve remission on corticosteroids and intravenous immunoglobulin (IVIg) treatment, the current standard of care. Achieving this milestone helps demonstrate our capabilities and commitment to develop and deliver meaningful and differentiated therapies to patients in China with our partner argenx," said Dr. Harald Reinhart, President and Head of Global Development, Neuroscience, Autoimmune & Infectious Diseases, Zai Lab. "Our collaboration with argenx is generating a robust pipeline of indications with the potential to improve care for many patients who live with autoimmune diseases, including CIDP."

The sBLA application is based on the ADHERE (NCT04281472) study, a multicenter, randomized, double-blind, placebo-controlled trial evaluating efgartigimod SC for the treatment of CIDP. Zai Lab enrolled patients into the ADHERE trial in Greater China and treatment response in these participants was consistent with global study outcomes. Subgroup analysis of Chinese participants demonstrated a 69% reduction in relapse rates with efgartigimod SC compared to placebo. In addition, 78% of Chinese participants treated in the open-label portion of the study demonstrated evidence of clinical improvement (ECI), further confirming the role IgG autoantibodies play in the underlying biology of CIDP. The favorable safety and tolerability profile of efgartigimod SC weekly dosing (up to 1 year) in the Chinese patient cohort was consistent with global trial participants.

In September 2023, Zai Lab launched VYVGART® (efgartigimod alfa injection) for generalized myasthenia gravis (gMG) in mainland China, with VYVGART becoming the first and only approved FcRn antagonist for these gMG patients.

In July 2023, Zai Lab announced that the CDE has accepted the BLA for efgartigimod SC for gMG in China.

About CIDP in China

There are an estimated 50,000 patients diagnosed with CIDP in mainland China.[1] Current treatment options are primarily corticosteroids and intravenous immunoglobulin (IVIg), with plasma exchange (PLEX) generally reserved for refractory patients. There is limited access to PLEX or IVIg in many parts of the world, including China. Because most patients require treatment for an extended period, there remains a significant unmet need for alternative treatment options that are effective, well-tolerated, and convenient for patients with CIDP in China.

[1] *Chronic inflammatory demyelinating polyneuropathy and diabetes, 2020.*

About ADHERE Trial Design

The ADHERE trial, sponsored by argenx, was a multicenter, randomized, double-blind, placebo-controlled trial evaluating efgartigimod SC for the treatment of CIDP. ADHERE enrolled 322 adult patients with CIDP who were treatment-naïve (not on active treatment for ≥6 months) or being treated with immunoglobulin therapy or corticosteroids. Zai Lab enrolled patients in the ADHERE trial in Greater China (mainland China, Hong Kong, Taiwan and Macau). The trial consisted of an open-label Stage A followed by a randomized, placebo-controlled Stage B. In order to enter Stage A and receive efgartigimod SC the diagnosis of CIDP was confirmed by an independent panel of experts. Patients entered a run-in stage, where any ongoing CIDP treatment was stopped and they had to demonstrate active disease, with clinically meaningful worsening on at least one CIDP clinical assessment tool, including INCAT, I-RODS, or mean grip strength. Treatment naïve patients were able to skip the run-in period with proof of recent worsening. To advance to Stage B, patients needed to demonstrate ECI to efgartigimod SC. ECI was achieved through improvement of INCAT score, or improvement on I-RODS or mean grip strength if those scales had demonstrated worsening during the run-in period. In Stage B, patients were randomized to either efgartigimod SC or placebo for up to 48 weeks. The primary endpoint was based on the hazard ratio for the time to first adjusted INCAT deterioration (i.e. relapse). After Stage B, all patients had the option to roll-over to an open-label extension study to receive efgartigimod SC.

About VYVGART® Hytrulo

VYVGART Hytrulo is a subcutaneous combination of efgartigimod alfa, a human IgG1 antibody fragment marketed for intravenous use as VYVGART®, and recombinant human hyaluronidase PH20 (rHuPH20), Halozyme's ENHANZE ® drug delivery technology to facilitate subcutaneous injection delivery of biologics. In binding to the neonatal Fc receptor (FcRn), VYVGART Hytrulo results in the reduction of circulating IgG. It is the first-and-only approved FcRn blocker administered by subcutaneous injection. VYVGART Hytrulo is the proprietary name in the U.S. for subcutaneous efgartigimod alfa and recombinant human hyaluronidase PH20. It may be marketed under different proprietary names following approval in other regions.

Zai Lab has an exclusive license agreement with argenx to develop and commercialize efgartigimod in Greater China (mainland China, Hong Kong, Macau, and Taiwan) for numerous autoimmune indications where there is significant unmet patient need.

About Zai Lab

Zai Lab (NASDAQ: ZLAB; HKEX: 9688) is an innovative, research-based, commercial-stage biopharmaceutical company based in China and the United States. We are focused on discovering, developing, and commercializing innovative products that address medical conditions with significant unmet needs in the areas of oncology, autoimmune disorders, infectious disease and neuroscience. Our goal is to leverage our competencies and resources to positively impact human health in China and worldwide.

For additional information about Zai Lab, 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 future expectations, plans, and prospects for Zai Lab, including, without limitation, statements regarding the prospects of and plans for development and commercialization of efgartigimod in Greater China, the safety and efficacy of efgartigimod, and the potential treatment of patients with chronic inflammatory demyelinating polyneuropathy. These forward-looking statements may contain 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 or 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 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) risks related to doing business in China, and (6) 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 (SEC). 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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