Zai Lab Obtains Breakthrough Therapy Designation for Efgartigimod Alfa Injection (Subcutaneous Injection) in Patients with Chronic Inflammatory Demyelinating Polyneuropathy in China

September 18, 2023

SHANGHAI, China and CAMBRIDGE, Mass., Sept. 18, 2023 (GLOBE NEWSWIRE) -- Zai Lab Limited (NASDAQ: ZLAB; HKEX: 9688) today announced that the Center for Drug Evaluation (CDE) of the National Medical Products Administration (NMPA) granted Breakthrough Therapy Designation for efgartigimod alfa injection (subcutaneous injection) (efgartigimod SC) for the treatment of patients with chronic inflammatory demyelinating polyneuropathy (CIDP). The Breakthrough Therapy Designation for efgartigimod SC was supported by data from both global and Chinese patients enrolled in the ADHERE study.

“In granting Breakthrough Therapy Designation, we are pleased to see that the CDE recognizes the promise of efgartigimod SC as an innovative treatment option for CIDP patients,” said Dr. Harald Reinhart, President and Head of Global Development, Neuroscience, Autoimmune & Infectious Diseases, Zai Lab. “We have seen how efgartigimod SC can meaningfully improve and stabilize disease symptoms in these patients. Existing treatment options are quite limited, and problematic given the general reliance on long-term steroid or chronic immunoglobulin therapy. In the ADHERE study, significant efficacy was demonstrated with a favorable safety profile. We are excited about the therapeutic potential of efgartigimod SC in CIDP, and we look forward to working with regulatory authorities in China to bring this important medicine to patients in need as soon as possible.”

The Breakthrough Therapy Designation review policy is designed to facilitate the development and expeditious review of novel medicines that are intended for the prevention or treatment of serious, life-threatening diseases or diseases that severely impact the quality of life for which there is no existing treatment, or where sufficient evidence indicates advantages of the novel drug over currently available treatment options. Drugs granted Breakthrough Therapy Designation receive priority communications and guidance from the CDE to promote and expedite the drug review process.

In July 2023, Zai Lab and argenx reported positive topline results from the ADHERE study evaluating efgartigimod SC in adults with CIDP.

- Primary endpoint met (p=0.000039); efgartigimod SC demonstrated 61% reduction (HR: 0.39 95% CI: 0.25; 0.61) in the risk of relapse versus placebo.
- 67% of global study participants in open-label Stage A demonstrated evidence of clinical improvement (ECI) indicating that IgG autoantibodies play a significant role in the underlying biology of CIDP.
- Safety and tolerability profile consistent with confirmed safety profile of VYVGART.

In the subgroup analysis for ADHERE trial participants in China, the results were consistent with global outcomes.

- The subgroup analysis of the mainland Chinese participants demonstrated an impressive reduction in relapse risk by 69% with efgartigimod SC compared to placebo.
- The mainland Chinese participants showed a similar level of response compared to the global population, with 78% of the mainland Chinese participants treated with open-label efgartigimod SC demonstrating confirmed ECI.
- In the mainland Chinese subgroup, efgartigimod SC demonstrated a safety profile consistent with that observed in the global population.

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 CIDP in China

The prevalence of CIDP in China is estimated at 50,000 patients.[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. As most patients require treatment for an extended period of time there remains a significant unmet need for alternate treatment options that are effective, well-tolerated, and convenient for patients with CIDP in China.

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

About Efgartigimod SC

Efgartigimod SC is a subcutaneous combination of efgartigimod alfa injection, 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), egartigimod SC results in the reduction of circulating IgG. It is the first-and-only approved FcRn blocker administered by subcutaneous injection.

Efgartigimod alfa injection (subcutaneous injection) is the proposed China International Nonproprietary Name for subcutaneous efgartigimod alfa injection and recombinant human hyaluronidase PH20. It is marketed in the United States as VYVGART Hytrulo and may be marketed under different names following approval in other regions.

Zai Lab has an exclusive license agreement with argenx to develop and commercialize efgartigimod in mainland China, Hong Kong, Macau, and Taiwan (Greater China).

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.

For more information, please contact:

Investor Relations:
Christine Chiou / Lina Zhang
+1 (917) 886-6929 / +86 136 8257 6943
christine.chiou1@zailaboratory.com / lina.zhang@zailaboratory.com

Media:
Jennifer Chang / Xiaoyu Chen
+1 (917) 446-3140 / +86 185 0015 5011
jennifer.chang@zailaboratory.com / xiaoyu.chen@zailaboratory.com

Zai Lab Limited

Source: Zai Lab Limited