Biogen Announces First Patient Dosed in Pivotal Study of Litifilimab in Cutaneous Lupus Erythematosus, an Autoimmune Disease Affecting Skin

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- Global Phase 2/3 AMETHYST study will evaluate the efficacy and safety of litifilimab (BIIB059), as compared to placebo in cutaneous lupus erythematosus (CLE)
- Initiation of the pivotal AMETHYST study is supported by positive results from the Phase 2 LILAC study, recently published in *The New England Journal of Medicine*[1,2]
- CLE is a chronic autoimmune skin disease, that can occur with or without affecting other parts of the body; no targeted therapies are currently approved for CLE

Cambridge, Mass. – October 10, 2022 – Biogen Inc. (Nasdaq: BIIB) announced that the first patient has been dosed in the global clinical study, AMETHYST. The Phase 2/3 study will evaluate the clinical efficacy and assess the safety of litifilimab (also known as BIIB059), a first in-class, humanized IgG1 monoclonal antibody (mAb) targeting blood dendritic cell antigen 2 (BDCA2), as compared to placebo, in participants with cutaneous lupus erythematosus (CLE). AMETHYST is expected to be conducted at approximately 238 sites worldwide and aims to enroll 474 adults with CLE.

“CLE is more severe and frequent among African American and Hispanic/Latino patients, compared to white patients, which is why for AMETHYST we have set enrollment targets with the objective of appropriate representation for these traditionally underrepresented groups,” said Priya Singhal, M.D., M.P.H., Head of Global Safety and Regulatory Sciences and Interim Head of R&D at Biogen. “We are excited to advance litifilimab into what will be one of the largest clinical studies in CLE, where there are currently insufficient treatment options that address the needs of people living with this disease.”

AMETHYST is a two-part, Phase 2/3, multicenter, double-blind, placebo controlled, randomized study to evaluate the efficacy and safety of litifilimab compared to placebo. The Phase 2 and Phase 3 parts of the study will each be 52 weeks in duration. Participants will be randomized to receive subcutaneous treatment with litifilimab or placebo every four weeks for 20 weeks with an additional loading dose at Week 2. All participants will receive litifilimab during the 28-week extended treatment period from Weeks 24 to 48. The primary endpoint will assess the effect of litifilimab on skin disease activity, compared to placebo.

More information on the AMETHYST study (NCT05531565) is available at clinicaltrials.gov and BiogenTrialLink.

About Litifilimab (BIIB059)
Litifilimab (known as BIIB059), discovered and developed in-house by Biogen scientists, is a humanized IgG1 monoclonal antibody (mAb) targeting BDCA2 and is being investigated for the potential treatment of systemic lupus erythematosus (SLE) and cutaneous lupus erythematosus (CLE). BDCA2 is a receptor that is predominantly expressed on a subset of human immune cells called Plasmacytoid Dendritic Cells (pDCs). Binding of litifilimab to BDCA2 has been shown to reduce production of pro-inflammatory molecules by pDCs, including type-I interferon (IFN-I) as well as other cytokines and chemokines.[3,4] These pro-inflammatory mediators are thought to play a major role in the pathogenesis of systemic and cutaneous lupus.

About Cutaneous Lupus Erythematosus (CLE)
CLE, a type of lupus, is a chronic autoimmune skin disease that can occur with or without systemic manifestations; people with CLE frequently experience symptoms including rash, pain, pruritis (itch) and photosensitivity as well as skin damage that may worsen over time and can include irreversible scarring, alopecia and dyspigmentation that can be disfiguring and substantially impact quality of life.[5-8]

Although anyone can develop lupus, an estimated 90 percent of people living with lupus are women; most begin to see symptoms between the ages of 15-40.[7] The disease disproportionately impacts diverse ethno-racial groups, including African American, Asian, American Indian/Alaskan Native and Hispanic/Latino communities.[10-13] There is currently no cure for lupus.

Decades of study by Biogen on pathways at the intersection of neurology and immunology provide the company with expertise in specialized immunology. Biogen is advancing two lupus therapies in Phase 3 trials. Dapirolizumab pegol is being developed in collaboration with UCB for systemic lupus erythematosus (SLE). Litifilimab (BIIB059), was fully developed in-house at Biogen and is now in Phase 3 for SLE and in Phase 2/3 for CLE.

About Biogen

As pioneers in neuroscience, Biogen discovers, develops, and delivers worldwide innovative therapies for people living with serious neurological diseases as well as related therapeutic adjacencies. One of the world’s first global biotechnology companies, Biogen was founded in 1978 by Charles Weissmann, Heinz Schaller, Sir Kenneth Murray, and Nobel Prize winners Walter Gilbert and Phillip Sharp. Today, Biogen has a leading portfolio of medicines to treat multiple sclerosis, has introduced the first approved treatment for spinal muscular atrophy, and developed the first and only approved treatment to address a defining pathology of Alzheimer’s disease. Biogen is also commercializing biosimilars and focusing on advancing one of the industry’s most diversified pipelines in neuroscience that will transform the standard of care for patients in several areas of high unmet need.

In 2020, Biogen launched a bold 20-year, $250 million initiative to address the deeply interrelated issues of climate, health, and equity. Healthy Climate, Healthy Lives™ aims to eliminate fossil fuels across the company’s operations, build collaborations with renowned institutions to advance the science to improve human health outcomes, and support underserved communities.

We routinely post information that may be important to investors on our website at www.biogen.com. Follow us on social media - Twitter, LinkedIn, Facebook, YouTube.

Biogen Safe Harbor

This news release contains forward-looking statements, including statements made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, about the potential benefits, safety and efficacy of BIIB059; the results of the Phase 2 LILAC study; the results and design of the AMETHYST study; the identification and treatment of lupus, SLE and CLE; our research and development program for the treatment of lupus, SLE and CLE; the clinical development program for BIIB059 risks and uncertainties associated with drug development and commercialization; and the potential of our pipeline programs, including BIIB059 and dapirolizumab pegol. These statements may be identified by words such as "aim," "anticipate," "believe," "could," "estimate," "expect," "forecast," "goal," "intend," "may," "plan," "possible," "potential," "will," "would" and other words and terms of similar meaning. Drug development and commercialization involve a high degree of risk, and only a small number of research and development programs result in commercialization of a product. Results in early-stage clinical trials may not be indicative of full results or results from later stage or larger scale clinical trials and do not ensure regulatory approval. You should not place undue reliance on these statements or the scientific data presented.

These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including without limitation risks that we may not fully enroll the AMETHYST study or it will take longer than expected; unexpected concerns that may arise from additional data, analysis or results obtained during the AMETHYST study; the occurrence of adverse safety events; risks of unexpected costs or delays; the risks of other unexpected hurdles; failure to protect and enforce our data, intellectual property and other proprietary rights and uncertainties relating to intellectual property claims and challenges; regulatory authorities may require additional information or further studies; product liability claims; third party collaboration risks; and the direct and indirect impacts of the ongoing COVID-19 pandemic on our business, results of operations and financial condition. The foregoing sets forth many, but not all, of the factors that could cause actual results to differ from our expectations in any forward-looking statement. Investors should consider this cautionary statement as well as the risk factors identified in our most recent annual or quarterly report and in other reports we have filed with the U.S. Securities and Exchange Commission. These statements are based on our current beliefs and expectations and speak only as of the date of this news release. We do not undertake any obligation to publicly update any forward-looking statements, whether as a result of new information, future developments or otherwise.

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MEDIA CONTACT:

Biogen

Dan Haro

+ 1 857 259 9880

public.affairs@biogen.com

INVESTOR CONTACT:

Biogen

Mike Hencke

+1 781 464 2442

IR@biogen.com