Biogen Announces First Patient Dosed in Phase 3 Systemic Lupus Erythematosus Study

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- *Global Phase 3 TOPAZ-1 study will evaluate the efficacy and safety of BIIB059, as compared to placebo, in active systemic lupus erythematosus (SLE), a debilitating autoimmune disease which has limited treatment options*
- *Positive Phase 2 LILAC study efficacy results support the further evaluation of BIIB059 in SLE*

CAMBRIDGE, Mass., June 17, 2021 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) today announced that the first patient has been dosed in the global clinical study, TOPAZ-1. The Phase 3 study will evaluate the clinical efficacy and assess the safety of BIIB059, a first in-class, humanized IgG1 monoclonal antibody (mAb) targeting blood dendritic cell antigen 2 (BDCA2), as compared to placebo, in participants with active systemic lupus erythematosus (SLE). TOPAZ-1 is expected to be conducted at approximately 135 sites worldwide and aims to enroll 540 adults with active SLE.

“A chronic autoimmune condition such as lupus, which overwhelmingly affects women, has a tremendous impact on my patients’ daily lives, including their physical, mental and social wellbeing,” said Richard Furie, M.D., Chief of the Division of Rheumatology at Northwell Health and Professor at Zucker School of Medicine at Hofstra/Northwell. “There remains a significant need for efficacious and generally safe treatment options for lupus patients. Based on the positive results observed in the Phase 2 LILAC study, we are excited to continue to evaluate the potential of BIIB059 in TOPAZ-1.”

TOPAZ-1 is a 52-week, multicenter, randomized double-blind, placebo-controlled Phase 3 study to evaluate the efficacy and safety of BIIB059 compared with placebo. Participants will be randomized to receive subcutaneous treatment with BIIB059 at one of two doses or placebo every four weeks with an additional dose at Week 2, in addition to their existing lupus therapy.

“We look forward to working with the lupus community as we advance the clinical development of BIIB059 with the hope of bringing a meaningful new treatment option to people living with systemic and cutaneous lupus,” said Nathalie Franchimont, M.D., Ph.D., Head of the Multiple Sclerosis and Immunology Development Unit at Biogen. “Additionally, we are reinforcing Biogen’s commitment to the inclusion of underrepresented groups in our clinical trials. We have set enrollment targets that reflect the prevalence of SLE in African-American and Hispanic/Latinx communities with the aim to achieve appropriate representation in the TOPAZ-1 study.”

The primary objective of TOPAZ-1 is to demonstrate reduction in disease activity as measured with the primary endpoint, proportion of participants who achieve an SLE Responder Index-4 (SRI-4) response at Week 52. SRI is a composite index using validated indices to measure global and organ-/system-specific disease activity. Key secondary endpoints will evaluate the effect of BIIB059 on additional efficacy parameters including proportion of patients achieving SRI-4 response at Week 24, oral corticosteroid use, organ-specific disease activity (joint and/or skin) and flare rates. Safety will be evaluated throughout the study duration.

The initiation of the TOPAZ-1 study is based on the results from the Phase 2 LILAC study. In LILAC, BIIB059 met its primary endpoint, demonstrating statistically significant reduction of disease activity in patients with SLE. The majority of adverse events in the LILAC study were mild or moderate.

More information on the TOPAZ-1 study (NCT04895241) is available at clinicaltrials.gov.

About BIIB059
BIIB059, discovered and developed exclusively by Biogen, is a humanized IgG1 monoclonal antibody (mAb) targeting blood dendritic cell antigen 2 (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 exclusively expressed on a subset of human immune cells called Plasmacytoid Dendritic Cells (pDCs), and it has been shown to reduce inflammatory cytokine production from pDCs, including type-I IFN (IFN-I). Inflammatory mediators are thought to play a major role in the pathogenesis of lupus.

About Systemic Lupus Erythematosus (SLE)
SLE is a chronic autoimmune disease that affects multiple organ systems, with periods of illness or flares alternating with periods of remission. SLE can present itself in several ways including rash, arthritis, anemia, thrombocytopenia, serositis, nephritis, seizures or psychosis. SLE is associated with a greater risk of death from causes such as infection and cardiovascular disease. There are an estimated four million people worldwide impacted by SLE.[i]

Although anyone can develop lupus, an estimated ninety percent of people living with lupus are women; most begin to see symptoms between the ages of 15-40.[ii] The disease disproportionately impacts certain ethno-racial groups, including African American, Asian, American Indian/Alaskan Native and Hispanic/Latinx communities.[iii,iv,v,vi] There is currently no cure for lupus.

Biogen is advancing two investigational lupus assets in Phase 3 studies: BIIB059, an anti-BDCA2, and dapirolizumab pegol, an anti-CD40L being developed in collaboration with UCB, which began a Phase 3 trial in 2020.

About Biogen
At Biogen, our mission is clear: we are pioneers in neuroscience. Biogen discovers, develops and delivers worldwide innovative therapies for people living with serious neurological and neurodegenerative 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, Kenneth Murray and Nobel Prize winners Walter Gilbert and Phillip Sharp. Today Biogen has the leading portfolio of medicines to treat multiple sclerosis, has introduced the first approved treatment for spinal muscular atrophy, commercializes biosimilars of advanced biologics and is focused on advancing research programs in multiple sclerosis and neuroimmunology, Alzheimer’s disease and dementia, neuromuscular disorders, movement disorders, ophthalmology, neuropsychiatry, immunology, acute neurology and neuropathic pain.

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 Statement
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 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; the design and enrollment of the TOPAZ-1 study; 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 TOPAZ-1 study or it will take longer than expected; unexpected concerns that may arise from additional data, analysis or results obtained during the TOPAZ-1 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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[i] Data on file. Estimated by Biogen epidemiology 2015

[ii] Pons-Estel GJ, Ugarte-Gil MF, Alarcón GS. Epidemiology of systemic lupus erythematosus. *Expert Rev Clin Immunol*. 2017 Aug;13(8):799-814.

[iii] Izmirly PM, Parton H, Wang L, et al. Prevalence of Systemic Lupus Erythematosus in the United States: Estimates From a Meta-Analysis of the Centers for Disease Control and Prevention National Lupus Registries. *Arthritis Rheumatol*. 2021;73(6):991-996.

[iv] Lim SS, Helmick CG, Bao G, et al. Racial Disparities in Mortality Associated with Systemic Lupus Erythematosus - Fulton and DeKalb Counties, Georgia, 2002-2016. *MMWR Morb Mortal Wkly Rep*. 2019;68(18):419-422.

[v] Rees F, Doherty M, Grainge MJ, Lanyon P, Zhang W. The worldwide incidence and prevalence of systemic lupus erythematosus: a systematic review of epidemiological studies. *Rheumatology (Oxford)*. 2017;56(11):1945-1961.

[vi] Drenkard C, Lim SS. Update on lupus epidemiology: advancing health disparities research through the study of minority populations. *Curr Opin Rheumatol*. 2019;31(6):689-696.