Biogen Reports Top-Line Results from Phase 2b Study of Natalizumab in Acute Ischemic Stroke

February 7, 2018

The primary and secondary efficacy endpoints were not met in the ACTION 2 study

Further development of natalizumab in acute ischemic stroke will not be pursued

Biogen remains committed to advancing treatments for stroke and other acute neurological conditions

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Biogen (Nasdaq: BIIB) announced today that in the Phase 2b dose-ranging ACTION 2 study in individuals with acute ischemic stroke (AIS), natalizumab did not demonstrate improvement in clinical outcomes compared to placebo. Both doses of natalizumab were generally well-tolerated and no new or important safety signals were observed. The results of the Phase 2b ACTION 2 study do not impact the benefit-risk profile of natalizumab in approved indications, including multiple sclerosis.

“As pioneers in neuroscience, Biogen remains committed to developing treatments for people with acute neurological conditions including stroke,” said Michael Ehlers, executive vice president, Research & Development at Biogen. “While we are disappointed with the ACTION 2 study results, we have furthered our knowledge of the disease and will continue to pursue innovative approaches in this area, including BIIB093 (intravenous glibenclamide) for prevention and treatment of edema in large hemispheric infarction, one of the most severe types of stroke.”

In the middle of 2018, Biogen plans to initiate a global Phase 3 study of BIIB093 in individuals with large hemispheric infarction, where brain swelling (cerebral edema) often leads to high morbidity and mortality.

Detailed Phase 2b ACTION 2 study findings will be made available in a future scientific forum.

About the natalizumab Phase 2 Development Program in AIS
ACTION 2 was a Phase 2b multicenter, double-blind, placebo-controlled, randomized, dose-ranging study with a 90 day follow up to evaluate the safety and efficacy of natalizumab primarily in patients with moderate severity acute ischemic stroke (AIS). The study investigated natalizumab vs placebo in approximately 270 individuals who had a clinical diagnosis of AIS with last known normal (LKN) ≤ 24 hours prior to treatment initiation. The study evaluated a 300 mg dose and a 600 mg dose versus placebo, both either within 9 hours of LKN or between 9-24 hours after LKN.

The primary objective of ACTION 2 was to assess the effects of natalizumab compared to placebo on clinical measures of independence and activities of daily living. The primary endpoint was a composite global measure of functional disability based on a score of 0 to 1 on a modified Rankin scale (mRS) and a score of ≥ 95 on the Barthel Index (BI) at Day 90. The mRS measures independence with specific tasks pre- and post-stroke. BI is a scale that consists of 10 items that measure activities of daily living and mobility.

Natalizumab was previously evaluated in AIS in the Phase 2a ACTION study. In this study, although natalizumab did not significantly decrease the primary endpoint of infarct volume at Day 5, secondary and exploratory endpoints suggested natalizumab treatment improved clinical outcomes compared with placebo which warranted further evaluation.

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. Founded in 1978 as one of the world’s first global biotechnology companies by Charles Weissman, 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 and only approved treatment for spinal muscular atrophy; and is focused on advancing neuroscience research programs in Alzheimer’s disease and dementia, multiple sclerosis and neuroimmunology, movement disorders, neuromuscular disorders, pain, ophthalmology, neuropsychiatry, and acute neurology. Biogen also manufactures and commercializes biosimilars of advanced biologics.

We routinely post information that may be important to investors on our website at www.biogen.com. To learn more, please visit www.biogen.com and follow us on social media – Twitter, LinkedIn, Facebook, YouTube.

Biogen Safe Harbor
This press release contains forward-looking statements, including statements made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, relating to potential clinical effects of natalizumab, the potential benefits, safety, and efficacy of natalizumab, risks and uncertainties associated with drug development and commercialization, the results of certain real-world data, the timing and scope of future clinical trials, including for BIIB093, the timing and status of Biogen’s current and future regulatory filings, and the potential of Biogen’s commercial business and pipeline programs, including natalizumab and/or BIIB093. These forward-looking statements may be accompanied by words such as “aim,” “anticipate,” “believe,” “could,” “estimate,” “except,” “forecast,” “intend,” “may,” “plan,” “potential,” “possible,” “will,” 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 unexpected concerns that may arise from additional data, analysis or results obtained during clinical trials; regulatory authorities may require additional information or further studies, or may fail or refuse to approve or may delay approval of Biogen’s drug candidates or expansion of product labeling, including, as applicable, natalizumab and/or BIIB093; risks of unexpected costs or delays; uncertainty of success in the development and potential commercialization of natalizumab and/or BIIB093, which may be impacted by, among other things, failure to protect and enforce Biogen’s data, intellectual property and other proprietary rights and uncertainties relating to intellectual property claims and challenges; Biogen may encounter other unexpected hurdles which may be impacted by, among other things, the occurrence of adverse safety events or failure to obtain regulatory

approvals in certain jurisdictions; product liability claims; or third party collaboration risks. The foregoing sets forth many, but not all, of the factors that could cause actual results to differ from Biogen's expectations in any forward-looking statement. Investors should consider this cautionary statement, as well as the risk factors identified in Biogen's most recent annual or quarterly report and in other reports Biogen has filed with the Securities and Exchange Commission. These statements are based on Biogen's current beliefs and expectations and speak only as of the date of this press release. Biogen does 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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