Biogen Announces Positive Topline Results from Phase 2 CONVEY Study in Small Fiber Neuropathy

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Vixotrigine, a non-opioid investigational pain drug, has the potential to address significant unmet medical needs of individuals living with chronic painful neuropathy

CAMBRIDGE, Mass., Sept. 16, 2021 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) today announced positive topline results from its Phase 2 CONVEY study of vixotrigine (BIIB074), a non-opioid investigational oral pain drug being evaluated for the treatment of small fiber neuropathy (SFN).

The CONVEY study 200 mg twice daily arm met its primary endpoint of change from baseline to week 12 of the double-blind period in mean average daily pain (ADP) score. In this study, all participants who enrolled received the higher dose (350 mg twice daily) in an open-label portion which preceded the double-blind portion of the study. While the 350 mg twice daily arm did not meet the primary endpoint, it met statistical significance in the Patient Global Impression of Change (PGIC) at week 12, an important self-reported measure of a patient's overall improvement since with the start of the study. The totality of data from the vixotrigine program will inform potential doses for study in future Phase 3 clinical trials. There is a significant unmet need for non-opioid treatments for people suffering from chronic neuropathic pain.

Small fiber neuropathy is often characterized by severe pain that typically begins in the feet or hands. Painful symptoms are described as burning, shooting and/or prickling. Pain can be caused by stimuli that does not normally provoke pain (allodynia) and potentially painful stimuli are increased in intensity (hyperalgesia). Symptoms tend to be worse at night and during periods of rest and can lead to a significant impact on overall quality of life.

"We are encouraged by the overall results of the CONVEY study, especially given the significant unmet medical need for additional agents to treat chronic painful neuropathy," said Katherine Dawson, M.D., Senior Vice President, and Head of the Therapeutics Development Unit at Biogen. "We are grateful to all the participants, investigators and study staff who contributed to this study and allowed us to evaluate vixotrigine as a non-opioid treatment option for people living with chronic neuropathic pain due to small fiber neuropathy."

CONVEY Topline Study Results
CONVEY was a Phase 2 placebo-controlled, double-blind, enriched enrollment, randomized withdrawal study that evaluated the efficacy and safety of vixotrigine in treating pain experienced by participants with confirmed idiopathic or diabetes mellitus-associated small fiber neuropathy. Statistical testing to compare each vixotrigine dose with placebo was pre-defined at the 10% significance level without multiplicity adjustment.

Vixotrigine 200 mg twice daily resulted in a statistically significant reduction in the mean average daily pain (ADP) score versus placebo at week 12 (p=0.0501). Treatment effect was noted in participants with diabetes mellitus based on a subgroup analysis but was not evident in the smaller subgroup of patients with idiopathic SFN. The 200 mg dose also resulted in statistically significant improvement versus placebo on the mean worst daily pain score at week 12 (p=0.0455). Numeric advantage of 200 mg over placebo was observed in additional secondary endpoints, including the proportion of participants with a 2-point or greater improvement in the average daily pain score and the proportion of participants with ≥30% reduction in ADP at week 12, but these did not meet statistical significance.

Vixotrigine 350 mg twice daily did not meet the primary endpoint of mean change in ADP at week 12. However, treatment with 350 mg vixotrigine resulted in a statistically significant increase in the proportion of participants who reported they were "very much improved" or "much improved" when compared to baseline, using the Patient Global Impression of Change (PGIC) questionnaire (p=0.0580). In addition, numeric advantage of 350 mg over placebo was observed in some secondary endpoints, including the proportion of participants with a 2-point or greater improvement in the average daily pain score and the proportion of participants with ≥30% reduction in ADP at week 12, but these did not meet statistical significance.

Both doses of vixotrigine were generally well tolerated and the safety profile was consistent with previous studies of vixotrigine with no evidence of abuse potential. In the open-label period, common AEs (incidence ≥ 2.5%) were dizziness, headache, vertigo, and nausea. 5.3% of subjects discontinued the open-label part of the study due to adverse events; across the entire study the majority of the AEs were mild or moderate in severity.

Biogen will further evaluate the CONVEY data and plans to complete a Phase 1 clinical study to inform potential next steps in the development of vixotrigine. In addition, detailed results from the CONVEY study will be made available in a future scientific forum.

About vixotrigine (BIIB074)
Vixotrigine (BIIB074) is an investigational peripherally and centrally acting, orally administered, voltage- and use-dependent voltage-gated sodium channel blocker. Sodium channels are important for nerve impulse conduction, including within pain-sensitive neurons which respond to tissue damage and within the pain pathway in the spinal cord and brain.

About the CONVEY Study (NCT03339336)
CONVEY was a Phase 2 placebo-controlled, double-blind, enriched enrollment randomized withdrawal study that enrolled 265 patients to evaluate the efficacy and safety of vixotrigine (BIIB074) in treating pain experienced by participants with confirmed small fiber neuropathy that is idiopathic or associated with diabetes mellitus. After a 4-week open-label run-in period, 123 responders to vixotrigine were randomized to receive either 200 mg or 350 mg vixotrigine or placebo twice-daily for 12 weeks in the double-blind portion of the study.

The primary objective of the study is based on change from baseline to week 12 of the double-blind period in the mean average daily pain score on an 11-point numeric rating scale. The secondary objectives of this study are to evaluate the effect on worst pain, neuropathic pain quality, sleep interference due to pain, patient global impression, use of rescue medication, and SFN symptoms in participants treated with vixotrigine; to investigate the safety and tolerability of vixotrigine in participants with SFN; and to characterize the pharmacokinetics of vixotrigine in participants with SFN. For more information about the CONVEY study, visit https://clinicaltrials.gov/ct2/show/NCT03339336.

About small fiber neuropathy (SFN)
Small fiber neuropathy (SFN) is a type of peripheral neuropathy characterized by degeneration of small-diameter sensory fibers, including those responsible for pain. SFN can be idiopathic or associated with conditions such as diabetes, immune-mediated diseases, infections, or toxic substances and may also involve multiple sensory and autonomic symptoms. Diabetes and impaired glucose tolerance are the most common causes

of SFN. For a significant portion of patients, SFN is idiopathic as no cause can be identified. With no treatments indicated specifically for this type of neuropathic pain and with other pain medications, there is a high unmet medical need for new effective and safe therapies.

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, including statements about results from the Phase 2 CONVEY study of vixotrigine; the potential clinical effects of vixotrigine; the potential benefits, safety and efficacy of vixotrigine; the clinical development program for vixotrigine; the identification and treatment of pain; our research and development program for the treatment of pain; the potential of our commercial business and pipeline programs, including vixotrigine; and risks and uncertainties associated with drug development and commercialization. These forward-looking statements may be accompanied by words such as "aim," "anticipate," "believe," "could," "estimate," "expect," "forecast," "intend," "may," "plan," "potential," "possible," "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, uncertainty of success in the development and potential commercialization of vixotrigine; the risk that we may not fully enroll our clinical trials or enrollment will take longer than expected; unexpected concerns may arise from additional data, analysis or results obtained during our clinical trials; regulatory authorities may require additional information or further studies, or may fail or refuse to approve or may delay approval of our drug candidates, including vixotrigine; the occurrence of adverse safety events; the risks of unexpected hurdles, costs or delays; failure to protect and enforce our data, intellectual property and other proprietary rights and uncertainties relating to intellectual property claims and challenges; product liability claims; 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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