Chiesi Global Rare Diseases Announces First Patient Treated in Expanded Access Program for Pegunigalsidase Alfa for Proposed Treatment of Fabry Disease

- Program grants access to investigational therapy for Fabry disease patients in the United States who cannot be adequately treated with currently available FDA-approved drugs -

BOSTON, Dec. 23, 2020 (GLOBE NEWSWIRE) -- Chiesi Global Rare Diseases, a business unit of Chiesi Farmaceutici S.p.A., an international research-focused healthcare group (Chiesi Group), today announced the first patient has been treated in the Expanded Access Program (EAP) in the United States for pegunigalsidase alfa, currently in development for the treatment of Fabry disease. Treatment was initiated on December 21, 2020 by Ozlem Goker-Alpan, M.D., founder and Chief Executive Officer of the Lysosomal & Rare Disorders Research & Treatment Center (LDRTC) in Fairfax, Va, and Lauren Noll, Clinical Research Coordinator.

“The initiation of treatment in the first patient enrolled in our EAP is an important milestone for the Fabry disease community and another reflection of our commitment to supporting patients, caregivers and their healthcare providers as they make important decisions about their health and disease management,” said Giacomo Chiesi, Head of Chiesi Global Rare Diseases. “Through this EAP, many patients can have access to treatment with pegunigalsidase alfa as we continue to work diligently to advance this therapy through the final stages of the regulatory review process in the months ahead. We would like to thank the Fabry patient and medical community for the support they have provided to us in the setup of this program.”

The EAP is open to patients with a clinical diagnosis of Fabry disease who, in the opinion of the treating physician, have no comparable or satisfactory alternative treatment options with currently available FDA-approved therapies for Fabry disease. Other eligibility criteria apply. Patients participating in the EAP receive infusions of pegunigalsidase alfa every two weeks at 1mg/kg body weight. Information related to adverse events and other limited data will be collected from participants.

“We are focused on providing patients with the widest possible range of options for high quality care,” said Dr. Goker-Alpan. “This is an exciting time for the Fabry disease community. We are pleased to participate in this EAP and will continue to support all efforts to help more patients have access to the treatments they need.”

Pegunigalsidase alfa is an investigational product and currently not approved by the U.S. Food and Drug Administration (FDA). The effectiveness and safety of pegunigalsidase alfa have not been reviewed or approved by the FDA. Before FDA review and approval, no conclusions can be drawn on pegunigalsidase alfa’s efficacy and safety profile. When seeking expanded access, treating physicians should consider all possible risks of treatment with pegunigalsidase alfa. Access must be compliant with all applicable federal and state laws and regulations. Investigators should not seek reimbursement for product provided to patients who participate in a government-funded insurance program.

In November 2020, the FDA extended the Prescription Drug User Fee Act (PDUFA) date to April 27, 2021 for review of the Biologics License Application (BLA) seeking accelerated approval of pegunigalsidase alfa for the proposed treatment of adult patients with Fabry disease. Concurrently with the EAP, pegunigalsidase alfa is being evaluated in patients with Fabry disease under an open Investigational New Drug application in the Phase III BALANCE, BRIGHT and BRIDGE clinical trials and other related open label studies.

The Expanded Access Program is listed on ClinicalTrials.gov Identifier: NCT04552691 (https://clinicaltrials.gov/ct2/show/NCT04552691). Additional information on Chiesi’s Expanded Access policy is available at https://www.chiesiusa.com/sustainability/expanded-access-programs/. Treating physicians must submit requests on behalf of their patients for consideration via the EAP request portal at https://chiesi.versaic.com.

About Fabry Disease

Fabry disease is an X-linked inherited disease that results from deficient activity of the lysosomal α–Galactosidase–A enzyme resulting in progressive accumulation of abnormal deposits of a fatty substance called globotriaosylceramide (Gb3) in blood vessel walls throughout a person's body. Fabry disease occurs in one person per 40,000 to 60,000. Fabry patients inherit a deficiency of the α–Galactosidase–A enzyme, which is normally responsible for the breakdown of Gb3. The abnormal storage of Gb3 increases with time and, accordingly, Gb3 accumulates, primarily in the blood and in the blood vessel walls. The ultimate consequences of Gb3 deposition range from episodes of pain and impaired peripheral sensation to end-organ failure – particularly of the kidneys, but also of the heart and the cerebrovascular system.

About Pegunigalsidase Alfa

Pegunigalsidase alfa is an investigational, plant cell culture-expressed, and chemically modified stabilized version of the recombinant α-Galactosidase-A enzyme. Protein sub-units are covalently bound via chemical cross-linking using short PEG moieties.

About Chiesi Global Rare Diseases

Chiesi Global Rare Diseases is a business unit of the Chiesi Group established in February 2020 and focused on research and development of treatments for rare and ultra-rare disorders. The Global Rare Diseases unit works in collaboration with Chiesi Group to harness the full resources and capabilities of our global network to bring innovative new treatment options to people living with rare diseases, many of whom have limited or no treatments available. The unit is also a dedicated partner with global leaders in patient advocacy, research and patient care. For more information visit www.chiesiglobalrarediseases.com.

About Chiesi Group

Based in Parma, Italy, Chiesi Farmaceutici is an international research-focused healthcare group with 85 years of experience in the pharmaceutical industry and a global presence in 29 countries. Chiesi researches, develops, and markets innovative drugs in the respiratory therapeutics, specialist medicine, and rare disease areas. Its R&D organization is headquartered in Parma (Italy), and is integrated with R&D groups in France, the USA, the UK, and Sweden to advance Chiesi's pre-clinical, clinical, and registration programs. Chiesi employs nearly 6,000 people. Chiesi Group is a certified Benefit corporation. For more information, please visit www.chiesi.com.

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