Chiesi Global Rare Diseases and Protalix BioTherapeutics Announce Launch of Expanded Access Program in the United States for Pegunigalsidase Alfa for the Proposed Treatment of Fabry Disease

Program grants access to investigational treatment for Fabry disease patients that cannot be adequately treated with any FDA-approved drugs and provides guidance to treating physicians

BOSTON and CARMIEL, Israel, Oct. 02, 2020 (GLOBE NEWSWIRE) -- Chiesi Global Rare Diseases, a business unit of Chiesi Farmaceutici S.p.A., an international research-focused healthcare group (Chiesi Group), and Protalix BioTherapeutics, Inc. (NYSE American:PLX) (TASE:PLX), a biopharmaceutical company focused on the development, production and commercialization of recombinant therapeutic proteins produced by its proprietary ProCellEx® plant cell-based protein expression system, today announced the launch of an Expanded Access Program (EAP) in the United States for pegunigalsidase alfa for the proposed treatment of Fabry disease. A biologics license application (BLA) for pegunigalsidase alfa is currently under review by the U.S. Food and Drug Administration (FDA). This EAP will run concurrently with Protalix's ongoing Phase III clinical program.

"The launch of this Expanded Access Program for pegunigalsidase alfa is another example of Chiesi's and Protalix's shared commitment to support patients whose condition cannot be adequately treated by currently available FDA-approved therapies for Fabry disease," said Marcel van Kuijck, Ph.D., Global Head of Medical Affairs at Chiesi Global Rare Diseases.

"We are excited that a broader group of physicians and patients beyond those in our Phase III program will have access to pegunigalsidase alfa, and that such support to Fabry patients in the U.S. is available prior to FDA's final review," added Raul Chertkoff, M.D., Vice President and Chief Medical Officer at Protalix.

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 will 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.

"The National Fabry Disease Foundation and the Fabry community are very excited about the launch of Chiesi's Expanded Access Program for pegunigalsidase alfa for the treatment of Fabry disease," said Jerry Walter, Founder and President, National Fabry Disease Foundation. "As the number of people diagnosed with Fabry disease continues to exceed predictions, access to treatment through Expanded Access Programs can play an important role in helping as many eligible patients as possible access the treatment they need."

Pegunigalsidase alfa is an investigational product and currently not approved by the 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 August 2020, the FDA accepted the pegunigalsidase alfa BLA and granted Priority Review designation for pegunigalsidase alfa for the proposed treatment of adult patients with Fabry disease. Concurrently with the EAP, pegunigalsidase alfa is being evaluated by Protalix BioTherapeutics 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.

About Protalix BioTherapeutics, Inc.

Protalix is a biopharmaceutical company focused on the development and commercialization of recombinant therapeutic proteins expressed through its proprietary plant cell-based expression system, ProCellEx®. Protalix was the first company to gain FDA approval of a protein produced through plant cell-based in suspension expression system. Protalix's unique expression system represents a new method for developing recombinant proteins in an industrial-scale manner.

Protalix's first product manufactured by ProCellEx, taliglucerase alfa, was approved for marketing by the FDA in May 2012 and, subsequently, by the regulatory authorities of other countries. Protalix has licensed to Pfizer Inc. the worldwide development and commercialization rights for taliglucerase alfa, excluding Brazil, where Protalix retains full rights.

Protalix's development pipeline consists of proprietary versions of recombinant therapeutic proteins that target established pharmaceutical markets, including the following product candidates: pegunigalsidase alfa, a modified version of the recombinant human α-Galactosidase-A protein for the proposed treatment of Fabry disease; OPRX-106, an orally-delivered anti-inflammatory treatment; alidornase alfa for the treatment of Cystic Fibrosis; and others. Protalix has partnered with Chiesi Global Rare Diseases, both in the United States and outside the United States, for the development and commercialization of pegunigalsidase alfa.

Protalix BioTherapeutics Forward-Looking Statements

To the extent that statements in this press release are not strictly historical, all such statements are forward-looking, and are made pursuant to the safe-harbor provisions of the Private Securities Litigation Reform Act of 1995. The terms "expect," "anticipate," "believe," "estimate," "project," "plan," "should" and "intend," and other words or phrases of similar import, are intended to identify forward-looking statements. These forward-looking statements are subject to known and unknown risks and uncertainties that may cause actual future experience and results to differ materially from the statements made. These statements are based on our current beliefs and expectations as to such future outcomes. Drug discovery and development involve a high degree of risk and the final results of a clinical trial may be different than the preliminary findings for the clinical trial. Factors that might cause material differences include, among others: that the FDA might not grant marketing approval for pegunigalsidase alfa by the PDUFA date or at all and, if approved, whether pegunigalsidase alfa will have significant limitations on its use or be commercially successful; risk that the FDA will request additional data or other conditions of the BLA filing for Accelerated Approval of PRX-102; failure or delay in the commencement or completion of our preclinical and clinical trials which may be caused by several factors, including: slower than expected rates of patient recruitment; unforeseen safety issues; determination of dosing issues; lack of effectiveness during clinical trials; inability to monitor patients adequately during or after treatment; and inability or unwillingness of medical investigators and institutional review boards to follow our clinical protocols; risks associated with the novel coronavirus disease (COVID-19) outbreak, which may adversely impact our business, preclinical studies and clinical trials; the risk that the results of the clinical trials of our product candidates will not support our claims of safety or efficacy, that our product candidates will not have the desired effects or will be associated with undesirable side effects or other unexpected characteristics; risks related to our ability to maintain and manage our relationship with any collaborator, distributor or partner; and other factors described in our filings with the U.S. Securities and Exchange Commission. The statements in this press release are valid only as of the date hereof and we disclaim any obligation to update this information, except as may be required by law.

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