Protalix BioTherapeutics and Chiesi Global Rare Diseases Announce U.S. Food and Drug Administration Acceptance of Biologics License Application (BLA) for Pegunigalsidase Alfa for the Proposed Treatment of Fabry Disease and Grants Priority Review

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CARMIEL, Israel, Aug. 11, 2020 /PRNewswire/ -- 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, or the Company, together with its development and commercialization partner Chiesi Global Rare Diseases, a unit of Chiesi, an international research-focused healthcare group, today announced that the U.S. Food and Drug Administration (FDA) has accepted the Biologics License Application (BLA) and granted Priority Review designation for pegunigalsidase alfa for the proposed treatment of adult patients with Fabry disease. The BLA was submitted via the FDA's Accelerated Approval pathway. Pegunigalsidase alfa is the Company's purposefully designed, long-acting recombinant, PEGylated, cross-linked α-galactosidase-A investigational product candidate. The FDA set an action date of January 27, 2021, under the Prescription Drug User Fee Act (PDUFA). The FDA also indicated in the BLA filing communication letter that it is not currently planning to hold an advisory committee meeting to discuss the application.

Priority Review is granted to therapies that the FDA determines have the potential to provide significant improvements in the treatment, diagnosis or prevention of serious conditions. This designation shortens the FDA review period following the acceptance of the BLA to six months compared to 10 months under standard review. Pegunigalsidase alfa was granted Fast Track designation by the FDA in January 2018.

The BLA submission includes a comprehensive set of preclinical, clinical and manufacturing data compiled from the Company's completed Phase I/II clinical trial of pegunigalsidase alfa, including the related extension study succeeding the Phase I/II clinical trial, interim clinical data from the Phase III BRIDGE switch-over study and safety data from the Company's on-going clinical studies of PRX–102 in patients receiving 1 mg/kg every other week.

"The FDA's acceptance of the BLA and grant of priority review for PRX-102 are significant achievements for Protalix and Chiesi, and represent a crucial step forward as we look to establish a new treatment option to the Fabry patient community," said Dror Bashan, Protalix's President and Chief Executive Officer. "Based on the encouraging results for PRX-102 we have seen to date, we are eager to continue discussions with the FDA and to continue our other development efforts for PRX-102, as marketing approval of PRX-102 is our top priority."

"PRX-102 represents an important advance in research with the potential to deliver significant advantages to patients with Fabry disease," said Giacomo Chiesi, Head of Global Rare Diseases. "We are very encouraged by the strong interest in this therapy among both patients and clinicians and we look forward to the prospect of making it available to patients around the world who can benefit from treatment."

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 (Gb_3) 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 Gb_3. The abnormal storage of Gb_3 increases with time and, accordingly, Gb_3 accumulates, primarily in the blood and in the blood vessel walls. The ultimate consequences of Gb_3 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 (PRX–102) 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, resulting in a molecule with unique pharmacokinetic parameters. In clinical studies, PRX–102 has been observed to have a circulatory half-life of approximately 80 hours. The Company designed PRX–102 to potentially address the continued unmet clinical need in Fabry patients.

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 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 U.S. Food and Drug Administration (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; PRX–115, a plant cell-expressed recombinant PEGylated Uricase for the treatment of gout; and others. Protalix has partnered with Chiesi Farmaceutici S.p.A., both in the United States and outside the United States, for the development and commercialization of pegunigalsidase alfa.

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 PRX–102 by the PDUFA date or at all and, if approved, whether PRX–102 will may have significant limitations on its use or be commercially successful; failure or delay in the commencement or completion of our preclinical and clinical trials which may be caused by several factors, including: risks that the FDA will request additional data or other conditions of our submission of any application for Accelerated Approval of PRX–102; 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 Chiesi Farmaceutici and any other collaborator, distributor or partner; risks related to the amount of our future revenues and expenditures; the risk that despite the FDA's grant of Fast Track designation for PRX-102, we may not experience a faster development process, review or approval compared to applications considered for approval under conventional FDA procedures; risks related to the FDA's ability to withdraw the Fast Track designation at any time; our dependence on performance by third party providers of services and supplies, including without limitation, clinical trial services; delays in the approval or potential rejection of any applications we file with the FDA or other health regulatory authorities, and other risks relating to the review process; the inherent risks and uncertainties in developing drug platforms and products of the type we are developing; the impact of development of competing therapies and/or technologies by other companies and institutions; potential product liability risks, and risks of securing adequate levels of product liability and other necessary insurance coverage; 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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