Protalix BioTherapeutics and Chiesi Global Rare Diseases Announce U.S. Food and Drug Administration Acceptance of a Resubmitted Biologics License Application for Pegunigalsidase Alfa for the Proposed Treatment of Fabry Disease

December 5, 2022

CARMIEL, Israel and BOSTON, Dec. 5, 2022 /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, and Chiesi Global Rare Diseases, a business unit of the Chiesi Group established to deliver innovative therapies and solutions for people affected by rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has accepted the resubmitted Biologics License Application (BLA) for pegunigalsidase alfa (PRX-102) for the proposed treatment of adult patients with Fabry disease. Pegunigalsidase alfa is a purposefully-designed, long-acting recombinant, PEGylated, cross-linked α-galactosidase-A investigational product candidate. The FDA indicated in the BLA filing communication letter that the resubmitted BLA was considered a complete, class 2 response and set an action date of May 9, 2023, under the Prescription Drug User Fee Act (PDUFA).

As announced last month, on November 9, 2022, a BLA was resubmitted to the FDA for PRX–102 for the treatment of adult patients with Fabry disease. The BLA resubmission includes a comprehensive set of clinical and manufacturing data. The data were compiled from studies that involved more than 140 Fabry disease patients with up to five years of follow up, including all three completed studies in the PRX-102 Phase III clinical program including the BALANCE study, the BRIDGE study and the BRIGHT study, as well as the Phase I/II clinical trial of PRX–102. The Phase I/II data includes data compiled from the related extension study succeeding the Phase I/II study. The BLA resubmission also includes safety data compiled from the ongoing Phase III extension studies of PRX–102. If approved, Protalix will be eligible to receive a milestone payment from Chiesi.

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 PRX–102

PRX–102 (pegunigalsidase alfa) is an investigational, plant cell culture-expressed, and chemically modified stabilized version of the recombinant α–Galactosidase–A enzyme. It is a novel, PEGylated enzyme replacement therapy (ERT) under development for the treatment of Fabry disease. 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 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 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 stabilized version of the recombinant human α Galactosidase A protein for the treatment of Fabry disease; alidornase alfa or PRX–110, for the treatment of various human respiratory diseases or conditions; PRX–115, a plant cell-expressed recombinant PEGylated uricase for the treatment of severe gout; PRX–119, a plant cell-expressed long action DNase I for the treatment of NETs-related diseases; 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.

About Chiesi Global Rare Diseases

Chiesi Global Rare Diseases is a business unit of the Chiesi Group established to deliver innovative therapies and solutions for people affected by rare diseases. As a family business, Chiesi Group strives to create a world where it is common to have a therapy for all diseases and acts as a force for good, for society and the planet. The goal of the Global Rare Diseases unit is to ensure equal access so as many people as possible can experience their most fulfilling life. The unit collaborates with the rare disease community around the globe to bring voice to underserved people in the health care system. For more information visit www.chiesirarediseases.com.

About Chiesi Group

Chiesi is an international, research-focused biopharmaceuticals group that develops and markets innovative therapeutic solutions in respiratory health, rare diseases, and specialty care. The company's mission is to improve people's quality of life and act responsibly towards both the community and the environment.

By changing its legal status to a Benefit Corporation in Italy, the US, and France, Chiesi's commitment to create shared value for society as a whole is legally binding and central to company-wide decision-making. Since 2019 Chiesi is certified B Corp, meaning that its sustainability efforts are measured and assessed by the most ambitious global standards. The company aims at becoming net-zero by 2035.

With over 85 years of experience, Chiesi is headquartered in Parma (Italy), operates in 30 countries, and counts more than 6,000 employees. The Group's research and development centre in Parma works alongside 6 other important R&D hubs in France, the US, Canada, China, the UK, and Sweden.

For further information please visit www.chiesi.com.

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," "may," "plan," "will," "would," "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; risks related to the timing, progress and likelihood of final approval by the FDA and European Medicines Agency (EMA) of the resubmitted Biologics License Application (BLA) and of a Marketing Authorization Application, respectively; risks related to the commercial success of PRX-102, and of our other product and product candidates, if approved; the likelihood that the FDA, EMA or other applicable health regulatory authorities will approve an alternative dosing regimen; failure or delay in the commencement or completion of our preclinical studies 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 satisfactorily demonstrate non-inferiority to approved therapies; inability or unwillingness of medical investigators and institutional review boards to follow our clinical protocols; and inability to monitor patients adequately during or after treatment; delays in the approval or potential rejection of any applications we file with the FDA, EMA or other health regulatory authorities for our other product candidates, and other risks relating to the review process; risks associated with the novel coronavirus disease, or COVID–19, outbreak, which may adversely impact our business, preclinical studies and clinical trials; risks related to any transactions we may effect in the public or private equity markets to raise capital to finance future research and development activities, general and administrative expenses and working capital; the risk that the results of the clinical trials of our product candidates will not support the applicable claims of safety or efficacy, or 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 our collaborators, distributors or partners; risks related to the amount and sufficiency of our cash, cash equivalents and short-term deposits; risks relating to our ability to make scheduled payments of the principal of, to pay interest on or to refinance our outstanding notes or any other indebtedness; our dependence on performance by third party providers of services and supplies, including without limitation, clinical trial services; delays in our preparation and filing of applications for regulatory approval; 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.

Investor Contact

Chuck Padala, Managing Director
LifeSci Advisors
646-627-8390
chuck@lifesciadvisors.com

Logo - https://mma.prnewswire.com/media/999479/Protalix_Biotherapeutics_Logo.jpg
Logo - https://mma.prnewswire.com/media/1656864/Chiesi.jpg

View original content:https://www.prnewswire.com/news-releases/protalix-biotherapeutics-and-chiesi-global-rare-diseases-announce-us-food-and-drug-administration-acceptance-of-a-resubmitted-biologics-license-application-for-pegunigalsidase-alfa-for-the-proposed-treatment-of-fabry-disease-301694436.html

SOURCE Protalix BioTherapeutics, Inc.; Chiesi