Protalix BioTherapeutics' pegunigalsidase alfa Receives Fast Track Designation from the U.S. Food and Drug Administration

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Fast Track designation highlights high unmet medical need in the treatment of Fabry disease

CARMIEL, Israel, Jan. 31, 2018 (GLOBE NEWSWIRE) --

Protalix BioTherapeutics, Inc. (NYSE American:PLX) (TASE:PLX), announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to pegunigalsidase alfa, or PRX-102, the Company's plant cell-expressed recombinant, pegylated, cross-linked α-galactosidase-A candidate for the treatment of Fabry disease. The FDA's Fast Track designation is a process designed to facilitate the development and expedite the review of drugs and vaccines for serious conditions that fill an unmet medical need.

"We are very pleased that the FDA has recognized the potential for pegunigalsidase alfa to fill an unmet need for Fabry patients," said Moshe Manor, Protalix's President and Chief Executive Officer. The data generated in our clinical trials of pegunigalsidase alfa thus far, as well as nonclinical data, as presented to the FDA with Protalix's application for Fast Track designation, demonstrate that pegunigalsidase alfa has the potential to address an unmet medical need for Fabry patients, such as the prevention of renal failure, improved survivability and a positive impact on quality of life. "We believe that Fast Track designation will help facilitate our development program for pegunigalsidase alfa and may shorten the timelines to an anticipated approval, which will greatly benefit Fabry patients."

According to the FDA, Fast Track designation is designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need, the purpose being to make important new drugs available to patients earlier. A drug that receives Fast Track designation from the FDA is eligible for some or all of the following:

- More frequent meetings with the FDA to discuss the drug's development plan and ensure collection of appropriate data needed to support drug approval;
- More frequent written communication from the FDA about such things as the design of the proposed clinical trials;
- Eligibility for the FDA's Accelerated Approval and Priority Review, if relevant criteria are met; and
- Eligibility for Rolling Review, which allows a drug company to submit completed sections of its Biologic License Application (BLA) or New Drug Application (NDA) for review by the FDA, rather than waiting until every section of the BLA or NDA is completed before the entire application can be reviewed. BLA or NDA review usually does not begin until the drug company has submitted the entire application to the FDA.

Fabry disease is a serious, life-threatening condition. It is a disease or condition associated with morbidity that has a substantial impact on survival, day-to-day function, and the likelihood that the disease, if left untreated, will progress from a less severe condition to a more serious one. Fabry disease is an X-linked multisystem lysosomal storage disorder caused by the absence or reduction of α-galactosidase-A (α-Gal-A) activity, which is a lysosomal enzyme that catalyzes the hydrolysis of globotriaosylceramide (Gb3) from oligosaccharides, glycoproteins and glycolipids. The absence or reduction of this enzymatic activity leads to the progressive accumulation of glycolipids, especially Gb3, in capillary endothelial cells, podocytes, tubular cells, glomerular endothelial cells, mesangial cells, interstitial cells, cardiomyocytes, fibroblasts, and neurons. The accumulation of glycosphingolipids (e.g., Gb3) leads to chronic pain, skin lesions, cardiac, deficiencies, and, in particular, renal involvement. End-stage renal failure and cardiomyopathy often lead to early death in Fabry patients. Fabry disease causes substantial reduction in life-expectancy, by an average of 15 years in female patients and 20 years in male patients, compared to the general population.

Pegunigalsidase alfa is currently being studied globally in three phase III clinical trials. Enrollment in each of the trials continues to progress and estimated timelines for top-line data announcements will be announced upon completion of enrollment for each individual trial.

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's unique expression system presents a proprietary method for developing recombinant proteins in a cost-effective, industrial-scale manner. Protalix's first product manufactured by ProCellEx, taliglucerase alfa, was approved for marketing by the U.S. Food and Drug Administration (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 includes the following product candidates: pegunigalsidase alfa, a modified version of the recombinant human alpha-GAL-A protein for the treatment of Fabry disease; OPRX-106, an orally-delivered anti-inflammatory treatment; alidornase alfa for the treatment of Cystic Fibrosis; and others. Protalix has entered into an ex-United States partnership with Chiesi Farmaceutici S.p.A. for the development and commercialization of pegunigalsidase alfa. Protalix maintains full rights to pegunigalsidase alfa in the United States.

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. Factors that might cause material differences include, among others: the risk that despite the FDA's grant of Fast Track designation for pegunigalsidase alfa for the treatment of Fabry disease, 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; 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; 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 and sufficiency of our cash and cash equivalents; risks related to the amount of our future revenues, operations and expenditures; inability or unwillingness of medical investigators and institutional review boards to follow our clinical protocols; and lack of sufficient funding to finance clinical trials; the risk that the results of the clinical trials of our product candidates will not support our claims of superiority, 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 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; 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; our ability to identify suitable product candidates and to complete preclinical studies of such product candidates; 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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