Protalix BioTherapeutics Doses First Patient in Global Phase III Clinical Trial of PRX-102 for the Treatment of Fabry Disease

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Six Sites Activated Across the United States and Europe with additional patients currently in the screening process for potential inclusion in trial

CARMIEL, Israel, Oct. 25, 2016 (GLOBE NEWSWIRE) -- Protalix BioTherapeutics, Inc. (NYSE MKT:PLX) (TASE:PLX), announced today that the first patient has been dosed in its global phase III clinical trial of PRX-102 for the treatment of Fabry disease. There are currently six of the leading Fabry centers activated and participating in the trial, with up to an additional 21 centers, currently in different initiation processes, that are expected to be opened before year end.

“The phase III Fabry trial is extremely important to the Company and remains a major focus for our team. We are pleased to announce the first patient has been infused, a milestone we have been working towards,” said Mr. Moshe Manor, Protalix’s President and Chief Executive Officer. “Interest from the physician and patient community in the trial is high, and a number of patients have already gone through the screening process. The trial has certain stringent inclusion criteria in place to maximize the likelihood of demonstrating superiority. Once all the anticipated trial sites are up and running, which we anticipate completing around year end, we will provide guidance regarding expected enrollment completion.”

The phase III efficacy and safety clinical trial, which we refer to as the BALANCE Study, is a 24-month multi-center, randomized, double-blind, active control study of PRX-102 for the treatment of Fabry disease in Fabry patients with impaired renal function. The trial is designed to enroll 78 patients previously treated with Fabrazyme® (agalsidase beta) with a stable dose for at least six months. Enrolled patients will be randomized to continue treatment with 1 mg/kg of either Fabrazyme or PRX-102, at a 2:1 ratio of PRX-102 to Fabrazyme, respectively. Patients are to be treated via intravenous (IV) infusions every two weeks.

The sites are recruiting adult symptomatic Fabry patients with plasma and/or leucocyte alpha galactosidase activity (by activity assay) less than 30% mean normal levels. All patients must have had treatment with a dose of 1 mg/kg agalsidase beta per infusion every two weeks for at least one year. In addition, to be included in the trial, patients need to have certain eGFR values and a meaningful decline in annualize eGFR slope.

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: PRX-102, a modified version of the recombinant human alpha-GAL-A protein for the treatment of Fabry disease; PRX-106, an orally-delivered anti-inflammatory treatment; PRX-110 for the treatment of Cystic Fibrosis; and others.

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 “anticipate,” “believe,” “estimate,” “expect,” “plan” 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: 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; 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 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; 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; 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 release are valid only as of the date hereof and we disclaim any obligation to update this information.

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