Protalix BioTherapeutics Initiates PRX-102 Global Phase III Clinical Trial of Fabry Disease to Support United States and European Filings

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24-Month Superiority Trial vs. Fabrazyme® for a United States Filing

At 12 Months, Interim Analysis of the Superiority Trial Data will be Performed to Test for Non-Inferiority Compared to Fabrazyme for an EMA Filing

Additional 12-Month Switch-Over Study from Replagal® to be Initiated to Provide Supportive Data for United States and EMA Filings

CARMIEL, Israel, June 06, 2016 (GLOBE NEWSWIRE) -- Protalix BioTherapeutics, Inc. (NYSE MKT:PLX) (TASE:PLX), announced today that based on guidance and feedback from the U.S. Food and Drug Administration (FDA), the Company initiated its phase III clinical trial of PRX-102 for the treatment of Fabry disease. Guidance from the European Medicines Agency (EMA) has been incorporated into the development plan to support an EMA filing as well.

"We are excited to initiate our global phase III clinical trial. The Institutional Review Board (IRB) process has been completed, including study protocol approval, and the first leading, United States-based clinical center for the first study has opened," said Moshe Manor, Protalix's President and Chief Executive Officer. "Patient enrollment is expected to commence shortly in centers throughout the United States and Europe."

The phase III efficacy and safety clinical trial, or, the BALANCE Study, is a 24-month multi-center, randomized, double-blind, active control study of PRX-102 in Fabry patients with impaired renal function. Seventy-eight patients previously treated with Fabrazyme® (agalsidase beta) for approximately one year, and with a stable dose for at least six months, will be enrolled and then randomized to continue treatment with 1 mg/kg with 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 primary efficacy parameter is the comparison of the mean annualized change in estimated glomerular filtration rate (eGFR) between treatment groups. The secondary endpoints include Left Ventricular Mass Index (g/m^2) by MRI, Plasma Lyso-Gb3, Plasma Gb3, Urine Lyso-Gb3, Exercise Tolerance (Stress Test), Short Form Brief Pain Inventory and Mainz Severity Score Index and Quality of Life. In addition, immunogenicity and safety parameters will be measured.

At 12 months, the Company intends to conduct an interim analysis to test for non-inferiority to support an anticipated regulatory filing with the EMA. Patients enrolled in the study will continue to be treated for a total of 24 months, at which point the data will be analyzed to test for superiority to support an FDA filing. To demonstrate superiority, during the SPA process, the FDA indicated that a 30% improvement in the rate of decline in eGFR relative to the active comparator arm is acceptable.

"The key endpoints for evaluation in the BALANCE Study are the same endpoints we analyzed in our phase I/II clinical trial of PRX-102. These endpoints demonstrated strong positive results in the phase I/II study with an annualized rate of eGFR change of only -0.32. According to a published report, an annualized rate of eGFR change of -1.89 was observed in a study of the effect of Fabrazyme in a similar patient population. Based on the above and given PRX-102's significantly longer half-life compared to other approved treatments, and its increased stability resulting from the molecule being covalently bound, we believe PRX-102 will achieve superiority," commented Einat Brill Almon, Ph.D., Protalix's Senior Vice President of Product Development.

The Company also intends to initiate the BRIDGE Study, a single-arm, switch-over study from Replagal®, to provide supportive data for anticipated filings in the United States and the European Union. This trial is designed to enroll 22 Fabry patients, currently treated with Replagal (alpha-galactosidase), who are to be switched to 1 mg/kg of PRX-102 for 12 months. The primary endpoint of the BRIDGE Study is safety; additional exploratory efficacy parameters include Left Ventricular Mass Index (g/m^2) by MRI, Plasma Lyso-Gb3, Plasma Gb3, Urine Lyso-Gb3, Exercise Tolerance (Stress Test), Short Form Brief Pain Inventory, Mainz Severity Score Index and Quality of Life.

During the Company's interaction with the FDA, including the SPA process, the Company received and implemented the FDA's recommendations for the BALANCE study design. In the interest of time, and based on the FDA's written guidance that an SPA is not a requirement for proceeding with the Company's clinical development program, the Company initiated the BALANCE Study without completing the full SPA process. Written guidance from the FDA has stated that the BALANCE Study is adequate to support a filing.

Additionally, through the SPA process, it became clear to the Company that a trial evaluating gastro-intestinal symptoms, which the Company had previously contemplated as part of the PRX-102 clinical development program, is not necessary for the Company's planned regulatory filings.

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(R). 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 pegylated version of a recombinant human alpha-GAL-A protein for the treatment of Fabry disease; PRX-106, an orally-delivered anti-inflammatory treatment; PRX-110, a chemically modified DNase I 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 analysis of data from the clinical trials of our product candidates will not achieve desired results; 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; risks related to the acceptance and use of our product candidates, if approved, by physicians, patients and third-party payors; the risk of significant delays in the commercial introduction of our product candidates, if and when approved; 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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