Protalix BioTherapeutics Reports Positive Phase I/II Interim Clinical Data on the 1mg/kg Cohort of PRX-102 for Fabry Disease

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Positive efficacy data across all disease parameters

Positive safety data with low level of antibody formation

End of Phase II meeting with FDA scheduled before year end

CARMIEL, Israel, Sept. 9, 2015 (GLOBE NEWSWIRE) -- Protalix BioTherapeutics, Inc. (NYSE MKT:PLX) (TASE:PLX), announced today positive interim data from the Company's phase I/II clinical trial of 1mg/kg of PRX-102 for the treatment of Fabry disease. PRX-102 is a recombinant plant cell expressed, chemically modified version of the human alpha-Galactosidase-A enzyme.

"We are very pleased with the positive results from the 1mg cohort for PRX-102," commented Mr. Moshe Manor, Protalix's President and Chief Executive Officer. "The efficacy results for the 1mg/kg cohort appear even more robust than those previously announced for the 0.2mg cohort, while maintaining a favorable safety profile with a very low level of antibody formation."

The phase I/II clinical trial of PRX-102 for the treatment of Fabry disease is an open-label, dose-ranging study treating up to 18 naïve male and female adult patients. The three dose cohorts include dosage groups of 0.2 mg/kg, 1mg/kg and 2mg/kg with intravenous infusions of PRX-102 every two weeks, with a six-month efficacy follow up period.

This interim analysis includes 6 patients enrolled in the 1mg/kg dose group at six months of treatment. The interim safety analysis includes 18 patients enrolled in three dose cohorts of 0.2mg/kg, 1mg/kg and 2mg/kg.

Interim Efficacy Results

Based on an analysis of kidney biopsies with randomized blinded scoring (n=4), PRX-102 demonstrated a reduction in renal peritubular capillary Gb3 of 86% using a quantitative Barisoni Lipid Inclusion Scoring System (BLISS).

Reductions of plasma Lyso-Gb3 and plasma Gb3 concentrations were also observed. Males (n=4) demonstrated a -67.5 ng/mL and a -5.3 µg/mL change, Females (n=2) demonstrated a -9.2 ng/mL mean change in Lyso-Gb3 and a -0.23 µg/mL mean change in plasma Gb3, respectively.

Furthermore, all patients had stable cardiac function after only six months, as measured by left ventricular mass (LVM), left ventricular mass index (LVMI) and ejection fraction (EF). Stable kidney function was also observed, as measured by estimated glomerular filtration rate (eGFR) and urine protein.

Safety Results

The safety analysis for adverse events represents a total of 15 patient years. PRX-102 was well tolerated, with the majority of adverse events being mild and moderate. Only one of the patients evaluated for safety experienced hypersensitivity, and only three patients, or approximately 19%, developed antibodies.

"The data presented from the 1mg cohort of PRX-102 continues to be very encouraging," said Dr. Derralynn Hughes of the Lysosomal Storage Disease Unit, Institute of Immunity and Transplantation, Royal Free London NHS Foundation Trust, London, UK, and a principal investigator in the PRX-102 clinical trial. "Significant improvement in Gb3 levels with stability in renal function and cardiac parameters were observed after a relatively short period of time with low antibody formation."

Raphael Schiffmann, M.D., M.H.Sc., an investigator with the Institute of Metabolic Disease, Baylor Research Institute, Dallas, TX, stated, "The data presented from the 1mg/kg cohort of PRX-102 continues to be very promising with a significant potential for improvement over the currently approved enzyme replacement therapies for Fabry disease. As PRX-102 has a different chemical structure with a different PK profile, which is probably the cause for the very low formation of antibodies, it has the potential for reduced immunogenicity as demonstrated in this interim report. Renal disease represents one of the main causes of morbidity and mortality among Fabry patients, and may not be adequately controlled by current standard of care. As a principal investigator in the ongoing clinical study, I am very encouraged when eGFR levels are kept stable."

Enrollment in the phase I/II clinical trial of PRX-102 was completed in early February 2015. All patients that completed the trial opted to continue to receive PRX-102 in an open-label extension study. The Company expects to report longer term data of the first 0.2mg/kg cohort by the end of this month and to report full top-line results from all dosing cohorts in the fourth quarter of 2015. The Company scheduled an End of Phase II meeting with the Food and Drug Administration before year-end to discuss the design of the pivotal phase III trial, which the Company expects to start in early 2016.

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 Israel's Ministry of Health, by the Brazilian National Health Surveillance Agency (ANVISA) and by the regulatory authorities of other countries. Marketing applications for taliglucerase alfa have been filed in additional territories as well. Protalix has partnered with Pfizer Inc. for the worldwide development and commercialization of taliglucerase alfa,

excluding Israel and 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 antiTNF; PRX-110 for the treatment of Cystic Fibrosis; and others.

About Baylor Research Institute

Established in 1984 in Dallas, Texas, Baylor Research Institute (BRI) promotes and supports research to bring innovative treatments from the laboratory workbench to the patient bedside. To achieve this bench-to-bedside concept, BRI focuses on basic science, clinical trials, healthcare effectiveness and quality of care research. Today, BRI is conducting more than 930 active research protocols with 400 research investigators, spanning more than 22 medical specialties, and has research and development projects in areas ranging from human immunology and orphan metabolic diseases to diabetes, cardio-vascular disease and many other unmet medical needs.

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: unforeseen safety issues; determination of dosing issues; lack of effectiveness during clinical trials; slower than expected rates of patient recruitment; 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.

CONTACT: Investor Contact
Marcy Nanus
The Trout Group, LLC
646-378-2927
mnanus@troutgroup.com

Protalix BioTherapeutics, Inc.