Protalix BioTherapeutics Presents Positive Preliminary Data from the BRIDGE Study of pegunigalsidase alfa for the Treatment of Fabry Disease at the 1st Canadian Symposium on Lysosomal Diseases 2018

October 5, 2018

~Preliminary Results Indicate Significant Improvement in Kidney Function in Patients Switched from agalsidase alfa (Replagal®) to pegunigalsidase alfa (PRX-102)~

A deterioration trend in patients' kidney function on agalsidase alfa (Replagal®) was reversed to an improvement trend when switched to pegunigalsidase alfa (PRX-102)

Presentation with full data to be held at the 1st Canadian Symposium on Lysosomal Diseases 2018

CARMIEL, Israel, Oct. 05, 2018 (GLOBE NEWSWIRE) -- Protalix BioTherapeutics, Inc. (NYSE American:PLX) (TASE:PLX), a biopharmaceutical company focused on the development and commercialization of recombinant therapeutic proteins expressed through its proprietary plant cell-based expression system, ProCellEx®, today announced that positive preliminary data from the BRIDGE study of pegunigalsidase alfa (PRX-102) for the treatment of Fabry disease will be presented today at the 1st Canadian Symposium on Lysosomal Diseases 2018. The symposium will take place October 5-6, 2018 at the OLT Gouverneur Hotel in Sherbrooke, Quebec. An oral presentation titled, "Pegunigalsidase Alfa-a Novel Enzyme Replacement Therapy for the Treatment of Fabry Disease: Preliminary Results from the Phase III Bridge Study," will be presented by Dr. Michael L. West, Professor, Division of Nephrology, Department of Medicine, Dalhousie University, Halifax, Nova Scotia, today, Friday, October 5 at 10:15 am ET. The slides from Dr. West's presentation will be available on the Company's website after the presentation.

The BRIDGE study is an open label switch over study evaluating the safety and efficacy of pegunigalsidase alfa, 1 mg/kg infused every two weeks, in up to 22 Fabry patients currently treated with agalsidase alfa (Replagal®) for at least 2 years and on a stable dose for at least 6 months. Patients are screened and evaluated over 3 months while continuing on agalsidase alfa. Following the screening period, each patient was enrolled and switched from Replagal treatment to receive intravenous (IV) infusions of PRX-102 1 mg/kg every two weeks for 12 months. Patients can receive PRX-102 infusions at a home care setup based on the infusion tolerability. The Company anticipates completing patient enrollment in the BRIDGE trial in the fourth quarter of 2018.

As previously announced by the Company, preliminary data from the first sixteen patients enrolled in the trial demonstrated an improvement in kidney function, in both male and female Fabry patients, when switched from agalsidase alfa to pegunigalsidase alfa. Based on available historical serum creatinine and study 3 month screening period values for approximately 2 years while treated with agalsidase alfa before switching to pegunigalsidase alfa treatment, the annualized estimated glomerular filtration rate (eGFR) slope for patients on Replagal was (negative) – -6.8ml/min/1.73m2. The mean eGFR slope for the same patients following six months of treatment with pegunigalsidase alfa was changed to be (positive) – +3.7ml/min /1.73m2. These results are statistically significant with a p-value of 0.015. Baseline characteristics of these patients were: mean estimated glomerular filtration rate (eGFR) 75.40 and 86.03 mL/min/1.73m2 for males and females, with annualized eGFR slope of -8.0 and -5.1 mL/min/1.73m2/year, respectively. This improvement in kidney function (e.g., eGFR) over time may potentially result in the delay or prevention of kidney failure in these populations.

The enzyme has been well tolerated in the study, with all adverse events being transient in nature without sequelae. Most of the patients who are eligible for home care therapy per country regulation are being treated under a home care arrangement in which certain of the scheduled infusions are performed at the patients' home, and the first patient who concluded the study has opted to enroll in a long-term extension study and continues to be treated with PRX-102.

"These results are very encouraging and show that pegunigalsidase alfa (PRX-102) has the potential to provide a better enzyme replacement therapy for Fabry patients. I am excited to be a part of this trial, and I look forward to continuing to work with Protalix as pegunigalsidase alfa progresses through its potential approval and availability for the treatment of Fabry patients," said Dr. Michael West, Professor, Division of Nephrology, Department of Medicine, Dalhousie University, Halifax, Nova Scotia.

In addition, in vitro analysis of PRX-102 in both human plasma and in lysosomal-like conditions shows significantly longer stability of enzyme activity compared to both commercially-available enzyme replacement therapies (ERTs). In lysosomal-like conditions, approximately 84% of PRX-102's activity was shown to have been preserved after 10 days compared to approximately 1% remaining enzyme activity in each of the commercially available ERTs. These results were statistically significant with a p-value of 0.01 (Kizhner T., et al (2015) Molecular Genetics and Metabolism 114: 259–267).

PRX-102 is the Company's plant cell-expressed recombinant, PEGylated, cross-linked α-galactosidase-A for Fabry disease. In pre-clinical and clinical studies, PRX-102 demonstrated higher stability in plasma, a longer half-life and higher exposure in Fabry patients, and a reduction in Gb3 in kidney biopsies in treatment naïve 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'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 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.

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 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: 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 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 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 ultimate purchase by Fundação Oswaldo Cruz of alfataliglicerase pursuant to the stated purchase intentions of the Brazilian Ministry of Health of the stated amounts, if at all; risks related to the successful conclusion of our negotiations with the Brazilian Ministry of Health regarding the purchase of alfataliglicerase generally; risks related to our commercialization efforts for alfataliglicerase in Brazil; risks relating to the compliance by Fundação Oswaldo Cruz with its purchase obligations and related milestones under our supply and technology transfer agreement; 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; 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; 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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