Protalix BioTherapeutics Announces Poster Presentation on Baseline Characteristics for Fabry Disease Patients Screened in the Phase III BALANCE Study of Pegunigalsidase Alfa at the 55th ERA-EDTA Congress

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Remaining Effective Enzyme in Blood Samples was more than Double for Pegunigalsidase Alfa (PRX-102) Compared to Fabrazyme® as a Result of Higher Inhibition of Neutralizing Antibodies Measured in Fabrazyme

CARMIEL, Israel, May 25, 2018 (GLOBE NEWSWIRE) -- GlobeNewswire /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 a poster presentation on the characteristics of Fabry disease patients screened in the phase III BALANCE clinical trial of pegunigalsidase alfa, a PEGylated alfa galactosidase alfa enzyme, or PRX-102, for the treatment of Fabry disease was presented at the 55thERA-EDTA Congress (European Renal Association – European Dialysis and Transplant Association).

Dr. David Warnock, Director of the Division of Nephrology and Professor of Medicine and Physiology at the University of Alabama at Birmingham, presented a poster titled “Progression of nephropathy in Fabry patients receiving enzyme replacement therapy (ERT); relation to anti-drug antibodies (ADA) status and proteinuria.” The poster highlights an analysis of key baseline characteristics including, presence of anti-drug antibodies (ADA) toward agalsidase beta (Fabrazyme®), cross recognition of those ADAs to pegunigalsidase alfa, annualized eGFR slope and proteinuria at screening for the first 37 patients (27 males, 10 females) screened for the BALANCE study as of January 2018.

The results demonstrate that 15 out of 27 screened male patients were ADA positive and, of those, all 15 had neutralizing antibodies to Fabrazyme and 14 out of the 15 also had neutralizing activity toward pegunigalsidase alfa as part as ex-vivo immunogenicity testing. However, titers measured in ADA against Fabrazyme were higher by an average of more than double than those measured against pegunigalsidase alfa. In addition, Fabrazyme was inhibited by an average of 83.6% leaving only 16.4% of effective enzyme, whereby pegunigalsidase alfa was inhibited by 61.6%, leaving 38.4%, more than double, the effective enzyme. No ADAs were present in the 10 female patients screened.

In addition, the results generated in a recent analysis of 60 patients screened for the BALANCE study to date, including the 37 patients analyzed for the poster presentation, have been substantially in-line with the above analysis implying that the remaining effective enzyme in blood samples was more than double for pegunigalsidase alfa compared to Fabrazyme.

Based on an analysis of patients’ history, the average annualized eGFR slope for the 15 male patients that tested positive for ADA was -7.9 (SD=6.0) mL/min/1·73m2/year demonstrating that the Fabry disease patients screened for the BALANCE study progressively lost kidney function, despite being treated with Fabrazyme for 1 to 12 years. Regarding proteinuria, the average urine protein creatinine ratio was 767 (SD=546) mg/gr for male patients.

“There is an unmet medical need in patients with Fabry disease who continue to show progressive loss of renal function despite years of treatment with agalsidase beta,” commented Dr. Warnock. “The progressive loss of kidney function and proteinuria seen in patients suffering from Fabry disease may be the result of neutralizing antibodies. Given pegunigalsidase alfa is less inhibited by preexisting neutralizing antibodies than Fabrazyme, coupled with its significantly longer half-life, there is the potential for pegunigalsidase alfa to control proteinuria and/or stabilize renal function in patients who have not had an optimal clinical response to agalsidase beta.”

The BALANCE study is a head-to-head blinded comparison study of pegunigalsidase alfa to agalsidase beta (Fabrazyme®) with 1 mg/kg every two weeks, with change in eGFR as the primary end-point. In addition, the BALANCE study also measures a wide range of clinical end points including pain, quality of life parameters, biomarkers and others. The study is enrolling Fabry patients who continue to progressively lose kidney function, despite receiving standard enzyme replacement therapy.

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: risks that data generated in ex vivo studies will not be repeated in clinical trials; 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 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; risks related to our ability to maintain and manage our relationship with Chiesi Farmaceutici and any other collaborator, distributor or partner; 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; 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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