Protalix BioTherapeutics Announces New Preclinical Results Demonstrating a Positive Effect of pegunigalsidase alfa (PRX-102) on Small-fiber Neuropathy in Fabry Disease Models Compared to Commercially Available Enzyme Replacement Therapies

April 18, 2017

CARMIEL, Israel, April 18, 2017 (GLOBE NEWSWIRE) -- Protalix BioTherapeutics, Inc. (NYSE MKT:PLX) (TASE:PLX), announced today new, promising results from a preclinical trial conducted in collaboration with Prof. Raphael Schiffmann, Director, Institute of Metabolic Disease at the Baylor Research Institute, Dallas, Texas. It was demonstrated that treatment of Fabry mice with pegunigalsidase alfa (PRX-102) slows the progression of small fiber neuropathy when compared to treatment of Fabry mice with currently approved enzyme replacement therapies, and when compared to untreated Fabry mice.

The preclinical study evaluated 1mg/kg pegunigalsidase alfa compared to Fabrazyme® and Replagal® at the approved clinical doses (1, 0.2 mg/kg, respectively) in Fabry mice, with untreated Fabry mice and wild type (healthy) mice as controls. Bi-weekly intravenous infusions were administered for 3 months.

Fabry mice treated with pegunigalsidase alfa showed a 53% reduction ($p<0.05$) in the number of Iba1 spots, a marker for inflammation of the peripheral sensory nerves system, compared to untreated Fabry controls. Levels of Iba1 in the pegunigalsidase alfa-treated mice reached the levels of the healthy mice. In contrast, there was no difference in the Iba1 marker in Fabry mice treated with Replagal® or Fabrazyme®, compared to untreated Fabry mice.

“Significantly decreased Iba1 marker in treated Fabry mice suggests that repeated infusions of the drug from an asymptomatic stage prevents the activation and/or proliferation of resident DRG macrophages and alleviates damage to the peripheral sensory nerves,” commented Dr. Yoseph Shaaltiel, Protalix’s Executive Vice President, Research and Development. “This change was not observed in Fabry mice treated with Fabrazyme® or Replagal®, an additional positive differentiation of pegunigalsidase alfa from currently approved therapies addressing Fabry disease neuropathy.”

Additionally, the sensitivity of the nervous system was assessed using the well-established hot plate test, which measures the effect of the enzyme treatment on response time. Pegunigalsidase alfa demonstrated effectiveness in attenuation of small fiber neuropathy progression. “This may show greater efficacy compared to the present enzyme preparations,” commented Dr. Shaaltiel.

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, 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 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; risks related to the amount and sufficiency of our cash and cash equivalents; risks related to the successful conclusion of our negotiations with the Brazilian Ministry of Health regarding the purchase of alfataliglicerase, and our commercialization efforts for alfataliglicerase in Brazil generally; risks relating to our ability to make scheduled payments of the principal of, to pay interest on or to refinance our 2018 convertible notes or any other indebtedness; risks relating to the compliance by Fundação Oswaldo Cruz with its purchase obligations and related milestones under our supply and technology transfer agreement; 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 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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