Protalix BioTherapeutics Presents Positive Results from the Phase I/II Open-Label Extension Trial for PRX-102 at the New Horizons for Fabry Disease Conference

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Continued to Demonstrate Improvement across all Key Fabry Disease Parameters at 24 Months

Low Incidence of Treatment Induced Anti-Drug Antibodies which Reversed Entirely Following 12 Months of Treatment without Recurrence

CARMIEL, Israel, Nov. 27, 2017 (GLOBE NEWSWIRE) -- Protalix BioTherapeutics, Inc. (NYSE American:PLX) (TASE:PLX), announced today that positive results from the Company's Phase I/II open label extension trial of pegunigalsidase alfa, or PRX-102, were presented by Prof. Raphael Schiffmann, Director, Institute of Metabolic Disease at the Baylor Research Institute, Dallas, Texas, at the New Horizons in Fabry Disease Conference. PRX-102 is a recombinant, plant cell expressed, pegylated, modified version of the human alpha-Galactosidase-A enzyme being developed for the treatment of Fabry disease.

Sixteen male and female adult patients were enrolled in the phase I/II clinical trial across three dosing cohorts (0.2 mg/kg, 1mg/kg and 2mg/kg) and received intravenous infusions of PRX-102 every two weeks. The two-year data described in this press release includes data from 11 patients enrolled and treated in the long-term open-label extension trial. Patients who did not continue in the extension trial included female patients who became or planned to become pregnant, and therefore were unable to continue in accordance with the study protocol, and patients that relocated to a location where treatment was not available under the clinical study.

"The long-term results at 24 months indicate that PRX-102 continues to demonstrate a remarkable improvement and stability across all key Fabry disease parameters. PRX-102 was also well tolerated, with a very low incidence of treatment induced anti-drug antibodies that were reversible with only a transient and reversible effect on pharmacokinetics," commented Prof. Raphael Schiffmann from the Institute of Metabolic Disease, Baylor Research Institute, Dallas, Texas, USA. "These long term results further support that PRX-102 has the potential to be a significant differentiated therapy when compared to currently approved enzyme replacement therapies, and carries an important hope for all Fabry patients."

Regarding efficacy, the following data was recorded at 24 months:

- Lyso Gb3 levels decreased approximately 90% from baseline;
- Renal function remained stable with mean eGRF levels of 108.02 and 107.20 at baseline and 24 months, respectively;
- An improvement across all the gastrointestinal symptoms evaluated, including severity and frequency of abdominal pain and frequency of diarrhea, were noted;
- Cardiac parameters, including LVM, LVMI and EF, remained stable with no cardiac fibrosis development detected;
- In conclusion, an improvement of over 40% in disease severity was shown as measured by the Mainz Severity Score Index (MSSI), a score compiling the different elements of the disease severity including neurological, renal and cardiovascular parameters; and
- An improvement was noted in each of the individual parameters of the MSSI.

Regarding safety, the following data was recorded:

- The majority of adverse events were mild to moderate in severity;
- During the first 12 months of treatment, only three of 16 patients (less than 19%) formed anti-drug antibodies (ADA), of which two of these patients (less than 13%) had neutralizing antibodies;
- Importantly, however, the ADAs turned negative for all three of these patients following 12 months of treatment; and
- The ADA positivity effect had no observed impact on the safety, efficacy or continuous biomarker reduction of PRX-102.

"PRX-102 is currently being evaluated globally in three phase III clinical trials," said Mr. Moshe Manor, Protalix's President and Chief Executive Officer. "The need for better treatments to address Fabry disease remains great, and Protalix, together with our European partner, Chiesi Farmaceutici S.p.A., look forward to introducing an anticipated new therapy to the market."

A copy of Professor Schiffmann's presentation will be posted in the Presentations page of the Investors tab of the Company's corporate website.

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 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 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 amount of our future revenues, operations and expenditures; 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 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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