Protalix BioTherapeutics Announces Additional Positive Data from its Phase I/II Clinical Trial for PRX-102 for the Treatment of Fabry Disease

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CARMIEL, Israel, Aug. 10, 2016 (GLOBE NEWSWIRE) -- Protalix BioTherapeutics, Inc. (NYSE MKT:PLX) (TASE:PLX), announced today additional positive data from its phase I/II clinical trial of PRX-102 for the treatment of Fabry disease. PRX-102 is a recombinant, plant cell expressed, pegylated, modified version of the human alpha-Galactosidase-A enzyme.

"We are pleased to announce additional positive results from our phase I/II Fabry clinical trial," said Moshe Manor, Protalix's President and Chief Executive Officer. "The efficacy results seen to date continue to be very strong with improvements or stabilization in efficacy demonstrated across all disease parameters giving us confidence in our phase III head-to-head study comparing PRX-102 versus Fabrazyme®. The safety data is remarkable with all adverse events being mild to moderate and transient by nature and with antibodies decreasing or being eliminated for the very few patients who tested positive. We believe the product profile for PRX-102 has the potential to be highly differentiated from currently available enzyme replacement therapies for Fabry disease and could impact significantly the lives of patients suffering from this rare, genetic disease."

The phase I/II clinical trial is an open-label, dose-ranging study designed to treat up to 18 naïve male and female adult patients. The three dose cohorts include 0.2 mg/kg, 1mg/kg and 2mg/kg with intravenous infusions of PRX-102 every two weeks.

The efficacy and safety analysis of the phase I/II clinical trial includes data from all 16 enrolled patients (9 male and 7 female) who completed the 12-month treatment period and, of more importance, a subset analysis of 10 patients who met the classic Fabry disease criteria (9 male and 1 female). The classic Fabry patient population is the most common Fabry patient population studied and reported on in the scientific and medical literature, and is the population to be evaluated in the Company's phase III pivotal trial. Classic Fabry disease is characterized by having less than 30% residual enzyme activity, and typical manifestations involve neurological, skin, ophthalmic and Fabry-specific biomarkers.

Efficacy Results

Improvements or stabilization in efficacy were demonstrated across all disease parameters. Reductions of plasma Lyso-Gb3 ranged from 66.7 to 22.6 ng/ml in all patients and from 102.0 to 33.1 ng/ml in classic patients. Stable kidney function was also observed, as measured by estimated glomerular filtration rate (eGFR), with change from mean eGFR value of 110.78 at base line to 110.23 after 12 months for all patients, and from mean eGFR value of 117.37 to 117.36 for classic patients. All patients had stable cardiac function as measured by left ventricular mass (LVM) and left ventricular mass index (LVMI). The detailed results, as a percentage of change from base line, for both all patients and for the classic Fabry patient subset are presented in the following chart.

% change ±SE	eGFR	LVM	LVMI	Gb3	lyso
All Patients (n=16)	-0.5 ±2.1	-0.0 ±2.5	0.4 ±2.6	-22.2 ± 6.1	-48.9 ± 5.7
Classic Patients (n=10)	-0.1±2.2	-2.6 ±3.4	-3.1 ±3.1	-33.3 ± 7.6	-57.6 ± 6.8

The eGRF data in the foregoing table was measured using the Chronic Kidney Disease Epidemiology Collaboration analysis (CKD-EPI), which is the analysis the Company is using in its phase III pivotal trial of PRX-102, based on discussions with the U.S. Food and Drug Administration. The eGFR slope for all patients (n=16) using CKD-EPI was -2.9 (BL-77.7-156.3). The eGFR slope for classic Fabry patients (n=10) was -1.8 (BL 82.4-156.3). According to a published report, an annualized rate of eGFR change of -3.8 (BL 49-170) was observed in a study of the effect of Fabrazyme on the classic Fabry patient population using CKD-EPI analysis with similar base line of eGFR. The Company's previously reported interim results for eGFR were analyzed using the MDRD equation, which is an older method and not commonly used in the clinical setting. Using MDRD, the eGFR slope is -3.25 for all patients (previously reported as -0.32), and -1.70 for classic patients.

PRX-102 also demonstrated, using the well-established BPI index, a substantial improvement in all pain parameters for all patients in the study, and an even more pronounced improvement in classic Fabry patients, as indicated below:

	Worse Pain		Average Pain		Pain Interference	
All Patients	-29	%	-26	%	-39	%
Classic Fabry Patients	-38	%	-41	%	-44	%

Safety Results

The safety analysis for adverse events represents a total of 26.2 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. Only three patients developed antibodies and, after competing 12 months of treatment, one of those three patients has now tested negative for antibodies further supporting the potential of an immune tolerance phenomenon associated with the PRX-102 enzyme. Accordingly, the previously reported 19% incidence of treatment-induced anti-drug antibodies is now only 13%. Moreover, titers of the other two positive patients that developed antibodies have continued to decline over time.

Currently, all 16 patients enrolled in the trial continue to receive 1 mg/kg of PRX-102 in an open label extension trial. The Company is recruiting patients for the phase III pivotal trial in centers recently opened in the United States.

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; OPRX-106, an orally-delivered anti-inflammatory treatment; PRX-110 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: 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.

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