Protalix BioTherapeutics Presents Additional Positive Phase I/II Interim Clinical Data on PRX-102 for Fabry Disease at the WORLD Symposium

February 12, 2015

New Positive Clinical Data on Cardiac and Kidney Functions

Detailed Positive Clinical Data on All Disease Parameters

CARMIEL, Israel, Feb. 12, 2015 (GLOBE NEWSWIRE) -- Protalix BioTherapeutics, Inc. (NYSE MKT:PLX) (TASE:PLX), announced today that additional positive interim data from the Company's phase I/II dose-ranging clinical trial of PRX-102 for the treatment of Fabry disease will be presented on February 12, 2015 at 10:45 AM ET at the Lysosomal Disease Network WORLD Symposium in Orlando, FL. PRX-102 is a recombinant plant cell expressed, chemically modified version of the human alpha-Galactosidase-A enzyme. A slide presentation featuring these data will be posted at the time of the presentation on the Company's web site at: http://phx.corporate-ir.net/phoenix.zhtml?c=101161&p=irol-presentations.

"The overall efficacy data generated for PRX-102 is remarkable, especially given the robustness of the different endpoints tested and considering that the data comes from the lowest dosing cohort of 0.2mg/kg," commented Dr. Ozlem Goker-Alpan, Director of the Lysosomal Disorders Unit, O&O Alpan LLC, Springfield, Virginia, USA, and principal investigator in the clinical trial. "It is very impressive to see stability data with favorable trends in cardiac and renal disease parameters after only six months of treatment. In addition, the safety profile is very favorable, with what appears to be a relatively low rate of antibody formation and adverse events."

Dr. Ozlem will be presenting interim results from the global, open-label, phase I/II dose-ranging trial. In the trial, 18 naïve male and female patients (11 male and 7 female) were enrolled across three dosing cohorts of 0.2 mg/kg, 1mg/kg and 2mg/kg for which intravenous infusions were administered every two weeks, with a six-month initial efficacy follow-up period.

Pharmacokinetics

PRX-102 has a significantly longer circulatory half-life (T½) of approximately 60 hours, and a substantially higher area under the curve (AUC) of approximately 70,000 ng/mL*hour for the 0.2mg/kg dose, when compared to currently marketed enzyme replacement therapies. These enhanced pharmacokinetic benefits are believed to be the result of the chemical modifications made to PRX-102, including cross-linking and covalent bonding to make the enzyme a more stable homo-dimer. The table below details the full pharmacokinetic profile of PRX-102.

Dose (mg/kg)	T½ (hr)	Tmax (hr)	Cmax (ng/mL)	AUC $_{0-\infty}$ (ng/mL*hr)
0.2	58.63 ±16.08	4.40 ±0.56	1858 ±531	69996 ±25904
1	73.76 ±10.89	5.16 ±1.38	10228 ±1812	373975 ±35490

The interim efficacy analysis includes 6 patients enrolled in the 0.2mg/kg dose group at six months of treatment (for Gb3 in renal peritubular capillaries n=5). The interim safety analysis includes 12 patients; 6 patients enrolled in the 0.2mg/kg dose group and 6 patients enrolled in the 1mg/kg dose group.

Clinical Data on Cardiac and Kidney Functions

The leading causes for death of Fabry patients include cardiovascular disease and renal failures. All patients that participated in the trial exhibited stable cardiac and kidney function with favorable trends after only six months of treatment, as measured by mean left ventricular mass (LVM), left ventricular mass index (LVMI), ejection fraction (EF), estimated Glomerular filtration rate (eGFR) and urine protein.

The table below sets forth the mean absolute values, at baseline and after six months of treatment, and including percentage changes, which were scored in a randomized blinded manner.

Timeframe	LVM (gr)	LVMI (gr/m2)	EF (%)	eGRF (mL/min/1.73m^2)	Urine Protein (mg/g creatinine)
Baseline	98.0	55.1	55.1	109.1	186.3
6 Months	94.4	52.7	55.8	115.8	167.8

Detailed Clinical Data on All Other Disease Parameters

Based on an analysis of kidney biopsies with randomized blinded scoring, PRX-102 demonstrated a major reduction from baseline in renal peritubular capillary Gb3 using both the quantitative Barisoni Lipid Inclusion Scoring System (BLISS) and the semi quantitative method. Using the BLISS method, a reduction in the rate of 82.2% for males, 65.4% for females and 75.5% for males and females combined were observed. Absolute change from baseline was -4.5, -1.2 and -3.2, respectively. Applying the semi quantitative scoring method, commonly used by approved enzyme replacement

therapies, PRX-102 demonstrated a reduction of 69.6% in abnormal capillary score.

Patient Data for BLISS Score Analysis

Patient	Absolute Change from Baseline	Percentage Change from Baseline
F101 (F)	-2.0	-76.9
F102 (F)	-0.4	-52.9
F103 (M)	-3.0	-91.7
F104 (M)	-5.3	-86.2
F106 (M)	-5.3	-69.5

Using the well-accepted Brief Pain Inventory scale, a 100% reduction in pain at its worst, a 60.0% reduction in mean severity, and 78.8% reduction on mean interference (which includes walking, working, sleeping, enjoyment of life and others) were observed. In addition to a 100% reduction in worst pain, all patients also reported a 100% reduction in mean interference, with the exception of one patient who experienced a 33.3% reduction.

Reductions of plasma Lyso-Gb3 and plasma Gb3 concentrations were also observed. Females (n=2) demonstrated a -2.4 ng/mL mean change in Lyso-Gb3 and a -0.4 µg/mL mean change in plasma Gb3. Males (n=4) demonstrated a -96.2 ng/mL and a -1.3 µg/mL change, respectively. All patients demonstrated a reduction in absolute Lyso-Gb3 concentration and all patients demonstrated a reduction in Gb3 except for one patient.

A meaningful reduction in the total score of Mainz Severity Score Index (MSSI), which looks at general, neurological, cardiovascular and renal parameters, was also demonstrated, with a reduction in all parameters included in MSSI.

The safety analysis for adverse events represents a total of 6.7 patient years (n=12). PRX-102 was well tolerated, with the majority of events being mild and moderate. Only 1 of the 12 patients evaluated for safety experienced hypersensitivity and discontinued per protocol. For this patient, anti PRX-102 IgG was negative and anti PRX-102 IgE was positive at baseline.

Six patients receiving the 0.2mg/kg dose and 2 patients receiving the 1mg/kg dose were evaluated for anti-drug antibody formation after six and three months of treatment, respectively. Of these 8 patients, only 2 patients, both in the 0.2 mg/kg dose cohort, developed anti-drug antibodies. All adverse events experienced by these patients were deemed by the investigators to be unrelated to the drug.

Enrollment in the phase I/II clinical trial of PRX-102 was completed in early February. All patients that completed the trial opted to continue to receive PRX-102 in an open-label extension study. The Company expects to report interim results from the 1mg/kg cohort in the third quarter of 2015, and full top-line results from all dosing cohorts in the fourth quarter of 2015.

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, by Israel's Ministry of Health in September 2012, by the Brazilian National Health Surveillance Agency (ANVISA) in March 2013, by the Mexican Federal Commission for the Protection against Sanitary Risk (COFEPRIS) in April 2013, by the Australian Therapeutic Goods Administration(TGA) in May 2014 and by the regulatory authorities of other countries. Marketing applications for taliglucerase alfa have been filed in additional territories as well. Protalix has partnered with Pfizer Inc. for the worldwide development and commercialization of taliglucerase alfa, excluding Israel and 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-112, an orally-delivered glucocerebrosidase enzyme that is produced and encapsulated within carrot cells, also for the treatment of Gaucher disease; PRX-106, an orally-delivered treatment for the treatment of Inflammatory Bowel Disease; 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.

CONTACT: Investor Contact

Marcy Nanus
The Trout Group, LLC
646-378-2927
mnanus@troutgroup.com

Protalix BioTherapeutics, Inc.