Protalix BioTherapeutics Announces Oral GCD Data to be Presented at WORLD Symposium 2014

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CARMIEL, Israel, Feb. 12, 2014 (GLOBE NEWSWIRE) -- Protalix BioTherapeutics, Inc. (NYSE MKT:PLX) (TASE:PLX) announced today that phase I clinical trial data for oral GCD (PRX-112) for the treatment of Gaucher disease will be presented by Professor Ari Zimran at the 10th Annual Meeting of the Lysosomal Disease Network: WORLD Symposium 2014 being held February 10-13 in San Diego, CA. Oral GCD is the Company's orally-delivered proprietary formulation of the plant cell-expressed enzyme, glucocerebrosidase (GCD), and contains the same active substance as taliglucerase alfa (ELELYSO™), the Company's approved enzyme replacement therapy.

"The data from the phase I clinical trial suggests that oral delivery of GCD has the potential to dramatically change the way Gaucher patients are treated—not only improving patients' quality of life by eliminating the need for bi-weekly intravenous infusions, but potentially also having clinical benefit due to the steady maintenance of enzyme levels in patients' circulation," commented Professor Ari Zimran, Associate Professor and Director of the Gaucher Clinic at the Shaare Zedek Medical Center in Jerusalem, Israel, and Principal Investigator.

The results demonstrate that Oral GCD was well tolerated across all three doses tested. No patient discontinued the study prematurely, there were no drug related serious adverse events (SAE) reported and no treatment-induced antibodies were detected in any of the patients participating in the trial. All adverse reactions were transient in nature, mild and moderate and the patients that experienced the adverse reactions recovered without further events. One patient experienced nausea related to treatment and two patients experienced mild dizziness and dizziness, which was possibly related to treatment.

Pharmacokinetic (PK) studies revealed that active GCD enzyme was detected in the patients' blood circulation, measured in the well-established assay in leucocytes of Gaucher patients, following oral administration of Oral GCD. C max analysis showed an average increase of over 100% in enzymatic activity from base line, with an increase ranging from approximately 50% to 350% among the different, individual patients in the study. In general, the PK profile of Oral GCD has a pattern of continuous enzyme presence over approximately 30 hours from administration. Thus, with a daily oral administration of Oral GCD, the Company expects to achieve a steady state level of active GCD enzyme in the blood circulation of patients similar to the physiological state in healthy individuals.

Platelet levels in 3 out of 8 thrombocytopenic Gaucher patients tested showed meaningful, rapid and unexpected improvement in platelet count after short-term treatment with Oral GCD. The data demonstrates platelet count increases ranging from 27% to 78% from base line.

David Aviezer, Ph.D., the Company's President and Chief Executive Officer added, "The biopharmaceutical industry is facing significant challenges regarding the delivery of therapeutic proteins orally through the digestive track. The plant cell wall serves as a protective agent against the gastric environment and forms a natural capsule for recombinant proteins. We are excited by the clinical proof of concept demonstrated in this study and look forward to continuing to develop our highly versatile plant cell expression platform for the oral delivery of different proteins. We are now looking to expand the use of our platform for the oral administration of our anti TNF fusion protein, as well as monoclonal antibodies."

The Company is currently in discussions with the U.S. Food and Drug Administration regarding the next stage of development for Oral GCD, with the aim of starting a next phase clinical trial this year.

Posters describing the data regarding Oral GCD, the PRX 102 Fabry program and taliglucerase alfa glycosilation profile will be available at the conference and will be posted on the Company's website under the Resources\Medical Presentations tab.

About the Oral GCD Phase I Clinical Trial

The phase I clinical trial was an exploratory, open-label study to evaluate the safety and pharmacokinetics of Oral prGCD (PRX-112) in 16 adult Gaucher patients. Patients received one of three doses of a plant cell suspension as a single oral administration during the first segment of the trial, and as three consecutive daily administrations during the second segment of the trial. Pharmacokinetic data was retrieved following the last dose in each segment. The primary objective of the trial was to measure the safety of Oral GCD in Gaucher patients. Additional objectives included an evaluation of Oral GCD's pharmacokinetic profile and exploratory endpoints.

About Protalix

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 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; pr-antiTNF, a similar plant cell version of etanercept (Enbrel®) for the treatment of certain immune and inflammatory diseases, such as rheumatoid arthritis, Crohn's disease, colitis, psoriasis and other autoimmune and inflammatory disorders; PRX-110 for the treatment of Cystic Fibrosis; PRX-107 for the treatment of emphysema due to hereditary alpha1-antitrypsin deficiency; 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. 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 studies 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 the clinical trials; the risk that the results of our clinical trials will not support the applicable claims of safety or efficacy, that our product candidates will not have the desired effects or will include 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; 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 other factors described in our filings with the U.S. Securities and Exchange Commission. These forward-looking statements are based on current information that may change and you are cautioned not to place undue reliance on these forward-looking statements. The statements in this release are valid only as of the date hereof and we disclaim any obligation to update this information. All forward-looking statements are qualified in their entirety by this cautionary statement.

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