Protalix BioTherapeutics Announces Positive Phase I Clinical Trial Results for Oral GCD in Gaucher Disease Patients

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CARMIEL, Israel, Oct. 14, 2013 (GLOBE NEWSWIRE) -- Protalix BioTherapeutics, Inc. (NYSE MKT:PLX) (TASE:PLX), announced today initial positive results from its phase I clinical trial of oral glucocerebrosidase (GCD), or Oral GCD (PRX-112), in patients with Gaucher disease. In the trial, Oral GCD was well-tolerated, and active enzyme was detected in patients' blood circulation. Oral GCD is an orally available form of the plant cell-expressed enzyme, glucocerebrosidase or GCD, which is the same active substance as the Company's approved enzyme replacement therapy, ELELYSO™. Oral GCD is an active form of human glucocerebrosidase which is naturally encapsulated within the carrot cells in which it is produced.

The phase I clinical trial is an open label safety and pharmacokinetic study designed to assess the delivery of prGCD after oral administration of Oral GCD in Gaucher patients. Patients received one of three doses of re-suspended carrot cells in a single oral administration during the first segment of the trial and three consecutive daily administrations during the second segment of the trial. The study is being conducted in two sites; at Shaare Zedek Medical Center, Jerusalem, Israel, and at the Rambam Medical Center, Haifa, Israel.

Overall, oral GCD was found to be safe and well tolerated in all 12 patients across all of the three doses tested. There were no serious adverse reactions reported and no patient discontinued the study prematurely. Presence of an enzyme was detected in patients' blood circulation and the enzyme demonstrated biological activity. In addition, some of the patients who suffered from thrombocytopenia and had low platelet counts demonstrated a meaningful improvement in platelet count. Accordingly, the trial has been extended to enroll and evaluate additional Gaucher patients with low platelet counts. The amended study is expected to conclude during the fourth quarter of 2013. Additional data from the phase I study will be presented at the 10th Annual WORLD Symposium, February 10-13, 2014 in San Diego, CA.

"Oral administration of proteins has been a long time challenge for the biopharmaceutical industry due to the early degradation of proteins in the digestive track. The results of this phase I trial provide compelling proof of concept data for the oral delivery of enzymes using plant cells as a delivery platform," commented Prof. Ari Zimran, Associate Professor and Director of the Gaucher Clinic at Shaare Zedek and Principal Investigator. "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 infusions, but perhaps also having clinical benefit due to the steady maintenance of enzyme levels in patients' blood circulation."

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 also 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-105, a pegylated recombinant human acetylcholinesterase in development for several therapeutic and prophylactic indications, a biodefense program and an organophosphate-based pesticide treatment program; 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 diseases such as rheumatoid arthritis, juvenile idiopathic arthritis, ankylosing spondylitis, psoriatic arthritis and plaque psoriasis; 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.

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

Media Contact
Kari Watson
MacDougall Biomedical Communications
781-235-3060
kwatson@macbiocom.com

Protalix BioTherapeutics, Inc.