Protalix BioTherapeutics Announces Positive Results from Phase II Clinical Trial of alidornase alfa (AIR DNase™) for the Treatment of Cystic Fibrosis

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Positive Results in a Number of Clinically Relevant Parameters Suggest Improved Lung Function with alidornase alfa

CARMIEL, Israel, April 12, 2017 (GLOBE NEWSWIRE) -- Protalix BioTherapeutics, Inc. (NYSE MKT:PLX) (TASE:PLX), announced today positive results from the Company's phase II clinical trial of alidornase alfa for the treatment of Cystic Fibrosis (CF). Sixteen patients were enrolled in the study, all of whom completed the study. alidornase alfa is a plant cell-expressed, chemically-modified recombinant DNase enzyme resistant to inhibition by actin, which the Company has specifically designed to enhance the enzyme's efficacy in CF patients.

The phase II trial is a 28-day switchover study to evaluate the safety and efficacy of alidornase alfa in CF patients previously treated with Pulmozyme® (currently the only commercially available DNase therapy). Participation in the trial was preceded by a two-week washout period from Pulmozyme® before treatment with alidornase alfa via inhalation.

The primary efficacy results show that treatment with alidornase alfa resulted in clinically meaningful lung function improvement, as demonstrated by a mean absolute increase in the percent predicted forced expiratory volume in one second (ppFEV1) of 3.4 points from baseline. Moreover, a mean absolute increase in ppFEV1 of 2.8 points was also observed in patients participating in the trial when compared to measurements taken from patients at initiation before the switch from Pulmozyme® to alidornase alfa.

A commercially available small molecule CFTR modulator for the treatment of CF has reported a mean absolute increase in ppFEV1 of 2.5 from baseline in its registration clinical study. This score was achieved while 74% of the patients participating in the trial of the CFTR modulator were also treated with the modulator on top of Pulmozyme®. While this marketed CFTR addresses a certain mutation applicable to less than 50% of CF patients, alidornase alfa is being developed to treat all CF patients.

Sputa available DNA samples were analyzed for approximately half of the patients. A mean reduction of over 70% in DNA content from baseline was observed, and a mean reduction of over 90% from baseline was observed for sputa visco-elasticity. Correlation between improvement in sputa parameters and pulmonary function was observed.

In addition, an *in vitro* study of alidornase alfa demonstrated a significant inhibition of Pseudomonas Aeruginosa, with alidornase alfa treated colonies reduced by over 50%, compared to baseline. Pseudomonas, strains of bacteria that are widely found in the environment, are a major cause of lung infections in CF patients. Chronic pulmonary infection is a leading cause of morbidity and mortality in CF patients, despite the aggressive use of antibiotics, and Pseudomonas is the most prevalent organism in the airway colonization of CF patients.

PK analysis performed indicated alidornase alfa is not absorbed into a patient's circulatory system, suggesting higher levels of alidornase alfa remains available in the patient's lungs. This provides further support for the potential that alidornase alfa may offer additional efficacy to CF patients.

The above-mentioned material decrease in visco-elasticity and DNA presence in CF patients' sputa, coupled with the significant inhibition of Pseudomonas and higher levels of alidornase alfa available in the patients' lungs, provides further supportive evidence of improved lung function after treatment with alidornase alfa, as demonstrated by the increase in FEV1.

alidornase alfa was well tolerated with no serious adverse events reported, and all adverse events that occurred during the study were mild and transient in nature.

"The efficacy and safety results of alidornase alfa are very encouraging as they demonstrate data that are clinically relevant which brings new hope to CF patients living with this devastating disease," said Professor Eitan Kerem, Chairman of Pediatrics, Head of The Cystic Fibrosis Center, Hadassah University Hospital, and a Principal Investigator in the clinical trial. "I look forward to taking part in future clinical studies of alidornase alfa as I believe it has the potential to become a gold standard treatment for all CF patients."

"We are very excited with the clinical data showing a significant, clinically meaningful improvement in efficacy, and potentially offering new alternatives to all CF patients," commented Moshe Manor, Protalix's President and Chief Executive Officer. "We look forward to exploring different paths for advancing the clinical development of alidornase alfa."

Data from the study was accepted as an oral presentation at the 40th European Cystic Fibrosis Conference to be held in June 2017.

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; PRX-106, an orally delivered anti-inflammatory treatment; PRX-110, a chemically modified DNase I 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: 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 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 related to the amount and sufficiency of our cash and cash equivalents; risks related to the successful conclusion of our negotiations with the Brazilian Ministry of Health regarding the purchase of alfataliglicerase, and our commercialization efforts for alfataliglicerase in Brazil generally; risks relating to our ability to make scheduled payments of the principal of, to pay interest on or to refinance our 2018 convertible notes or any other indebtedness; risks relating to the compliance by Fundação Oswaldo Cruz with its purchase obligations and related milestones under our supply and technology transfer agreement; 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 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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