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

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CARMIEL, Israel, Jan. 03, 2017 (GLOBE NEWSWIRE) -- Protalix BioTherapeutics, Inc. (NYSE MKT:PLX) (TASE:PLX), announced today positive interim results from the Company's phase II clinical trial of alidornase alfa for the treatment of Cystic Fibrosis (CF) for the first 13 CF patients enrolled in the study. Fifteen patients have been enrolled in, and are expected to complete, 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 switch-over study to evaluate the safety and efficacy of alidornase alfa in CF patients previously treated with Pulmozyme®. Participation in the trial is preceded by a two-week washout period from Pulmozyme® before treatment with alidornase alfa via inhalation.

The initial primary efficacy result shows that alidornase alfa improves lung function as demonstrated by a mean absolute increase in the percent predicted forced expiratory volume in one second (ppFEV1) of 4.1 points from baseline. 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 Pulmozyme® on top of the modulator. 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 approximately 60% in DNA content from baseline was observed, and a mean reduction of approximately 90% from baseline was observed for sputa visco-elasticity. This data provides further supportive evidence of improved lung function after treatment with alidornase alfa, as demonstrated by the increase in ppFEV1.

No serious adverse events were reported, and all adverse events that occurred during the study were mild and transient in nature.

"We are enthusiastic about the data generated in this trial as we were able to see meaningful improvements in efficacy in a way that have not been reported for a long time in the challenging CF space," commented Moshe Manor, Protalix's President and Chief Executive Officer. "We are looking forward to reporting full results from the study before the end of the first quarter of 2017."

"The preliminary efficacy results of alidornase alfa are very encouraging, even when compared to past trials of approved drugs for the treatment of CF. Although the study was performed on a small number of patients, the data is very encouraging since it shows clinically meaningful results," said Professor Eitan Kerem, Chairman of Pediatrics, Head of The Cystic Fibrosis Center, Hadassah University Hospital. "I look forward to following the results of upcoming trials of alidornase alfa. If the data continues to be as positive, clearly alidornase alfa will be a key treatment for all CF patients."

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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