Protalix BioTherapeutics Announces Positive Results from Phase II Clinical Trial of OPRX-106 for the Treatment of Ulcerative Colitis

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Clinical Responses Observed in 67% of Patients and Clinical Remission Observed in 28% of Patients

No Anti-Drug Antibodies Detected

CARMIEL, Israel, March 13, 2018 (GLOBE NEWSWIRE) -- Protalix BioTherapeutics, Inc. (NYSE American:PLX) (TASE:PLX), announced today, positive results from the Company's phase II clinical trial of OPRX-106 for the treatment of ulcerative colitis. OPRX-106 is a plant cell-expressed recombinant human tumor necrosis factor receptor II fused to an IgG1 Fc domain (TNFRII-Fc), in development for oral administration. When administered orally and while passing through the digestive tract, the plant cells function as a natural delivery vehicle, having the unique attribute of a cellulose cell wall which makes them resistant to degradation compared to proteins produced via mammalian cell expression.

The phase II clinical trial is a randomized, open label, 2-arm study of OPRX-106. A total of 24 patients with active mild to moderate ulcerative colitis were enrolled in the study. Patients were randomized to receive 2 mg or 8 mg of OPRX-106, administered orally, once daily, for 8 weeks. The average base line Mayo score was 7.1 for the 18 patients who completed the study with 89% of those patients having a Mayo score of between 6 and 9, which meets the criteria of moderate disease activity.

The key efficacy endpoints of the study were met:

- 67% of patients experienced a clinical response; and
- 28% of patients experienced a clinical remission.

Clinical response at week 8 is defined as a decrease in the Mayo score of at least 3 points and either a decrease in the sub-score for rectal bleeding of at least 1 point from baseline, or rectal bleeding sub-score of 0 or 1. Clinical remission at week 8 is defined as clinically symptom free, a Mayo score ≤ 2, with no individual sub-score exceeding 1 point after treatment.

In addition, other key efficacy endpoints were also achieved:

- 72% of patients showed an improvement in rectal bleeding scores;
- 72% of patients demonstrated an improvement in fecal calprotectin; and
- 61% of patients showed improved Geboes score (a histopathological scoring for the assessment of disease activity in ulcerative colitis).

The positive trend in efficacy was consistent in substantially all patients. This trend is demonstrated by 89% of the patients having showed an improvement in Mayo score, with an average decrease in Mayo score of 45%, or 3 points, or from 7.1 at baseline to 4.1, at week 8. In addition, the vast majority of patients also showed an improvement in at least one of the other efficacy parameters.

No anti-drug antibodies were detected. OPRX-106 was safe and well tolerated with only mild to moderate adverse events, which were transient in nature. Headaches were the most common adverse event reported.

"We are very excited by these results," commented Moshe Manor, Protalix's President and Chief Executive Officer. "They demonstrate efficacy and a lack of immunogenicity together with a favorable safety profile, which could potentially overcome one of the most challenging drawbacks of current ulcerative colitis therapies administered via injection and infusion."

"The data is very encouraging, suggesting that OPRX-106 could potentially address a large unmet medical need in the treatment of ulcerative colitis. OPRX-106 is delivered orally and is biologically active in the gut without triggering the formation of anti-drug antibodies. OPRX-106 has the potential to address the partial loss of response seen in anti-TNF alpha treatment, which is driven by the high presence of neutralizing antibodies," said Professor Yaron Ilan, Chairman of the Department of Medicine, and an expert in Gastroenterology, at The Hadassah Hebrew University Medical Center in Jerusalem. "In addition, by being delivered orally, OPRX-106 could potentially avoid certain side effects, such as malignancies and opportunistic infections, which currently appear in the black box warning of the prescribing information for commercially-available anti-TNF alpha biologics."

The Company intends to release full results at a medical conference later in the year.

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: pegunigalsidase alfa, a modified version of the

recombinant human alpha-GAL-A protein for the treatment of Fabry disease; OPRX-106, an orally-delivered anti-inflammatory treatment; alidornase alfa for the treatment of Cystic Fibrosis; and others. Protalix has entered into an ex-United States partnership with Chiesi Farmaceutici S.p.A. for the development and commercialization of pegunigalsidase alfa. Protalix maintains full rights to pegunigalsidase alfa in the United States.

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 superiority, 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 amount of our future revenues, operations and expenditures; risks relating to our ability to make scheduled payments of the principal of, to pay interest on or to refinance our outstanding notes or any other indebtedness; our dependence on performance by third party providers of services and supplies, including without limitation, clinical trial services; 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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