



Protalix BioTherapeutics to Conduct Phase III Clinical Trial for PRX-102 for the Treatment of Fabry Disease Following a Successful End-of-Phase II Meeting With FDA

November 16, 2015

Clear Path for Biologics License Application (BLA) Submission

One Short-Term Safety and Efficacy Study Required to Support Full Approval

In Parallel, Protalix to Conduct Phase III Head-to-Head Superiority Trial Comparing PRX-102 Versus Fabrazyme®

CARMIEL, Israel, Nov. 16, 2015 (GLOBE NEWSWIRE) -- Protalix BioTherapeutics, Inc. (NYSE MKT:PLX) (TASE:PLX), announced today that it recently held an End-of-Phase II meeting with the U.S. Food and Drug Administration (FDA) to discuss the Company's proposed BLA plan for PRX-102 for the treatment of Fabry disease. Official FDA meeting minutes indicate the FDA's acceptance of the Company's path forward for a phase III clinical trial to support a full BLA approval.

The phase III clinical trial will be a randomized, multi-center, placebo-controlled, safety and efficacy study in treatment-naïve Fabry patients evaluating the 1 mg/kg dose of PRX-102. The Company anticipates a small sample size of patients will be needed to achieve statistical significance with a study duration of approximately six months. The primary endpoint will be Gastrointestinal Symptoms, with key secondary endpoints including renal function.

In the official FDA meeting minutes, the FDA noted that the Company reported interim analysis results from its phase I/II clinical trial of PRX-102 that preliminarily show a favorable trend in the severity and frequency of abdominal pain and frequency of diarrhea after six months of treatment with PRX-102. According to the FDA, during a recent ERT (enzyme replacement therapy) shortage, patients who reduced or discontinued ERT dosing developed worsening of GI signs and symptoms within a few weeks to months.

In addition to the phase III clinical trial described above, the Company and the FDA also agreed to a phase III head-to-head superiority trial comparing PRX-102 versus Fabrazyme, which the Company plans to commence in early 2016. The primary endpoint for this head-to-head trial will be an improvement in eGFR. The trial will enroll patients who are currently treated with Fabrazyme; such patients will be treated with 1mg/kg of PRX-102 for a two-year period. Interim results from this head-to-head trial will also provide supportive safety data for the BLA submission.

"We are very pleased with the outcome of the FDA meeting and appreciate the valuable guidance the agency has provided us regarding the phase III clinical program required to support a full BLA approval," said Moshe Manor, Protalix's President and Chief Executive Officer. "We are in a strong financial position and believe we are well capitalized to run both phase III trials through an anticipated BLA filing."

Guidance from the official FDA minutes, suggests no additional non-clinical studies are required to support a BLA for PRX-102. The Company plans to submit a request for a Special Protocol Assessment (SPA) to the FDA later this year, and to commence both Phase III trials in early 2016. The Company will provide more detailed information on the design of the Phase III clinical trials after completion of the SPA process.

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(R). 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-112, an orally-delivered glucocerebrosidase enzyme that is produced and encapsulated within carrot cells for the treatment of Gaucher disease; PRX-106, an orally-delivered anti-inflammatory treatment; PRX-110 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; 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 release are valid only as of the date hereof and we disclaim any obligation to update this information.

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