Protalix BioTherapeutics Receives Positive Opinion for Orphan Designation for PRX-102 for the Treatment of Fabry Disease in the European Union

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CARMIEL, Israel, Nov. 13, 2017 (GLOBE NEWSWIRE) -- Protalix BioTherapeutics, Inc. (NYSE American:PLX) (TASE:PLX), a biopharmaceutical company focused on the development and commercialization of recombinant therapeutic proteins expressed through its proprietary plant cell-based expression system, ProCellEx®, announced today that the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA) issued a positive opinion on the Orphan Medicinal Product Application for pegunigalsidase alfa, or PRX-102, for the treatment of Fabry disease. To be eligible for orphan medicinal product designation, the Company had to establish medically plausible evidence that PRX-102 will provide a significant benefit over existing approved therapies in the European Union for the treatment of Fabry disease. In its official opinion letter, the COMP explained that Protalix has established that although satisfactory methods of treatment of Fabry disease have been authorized in the European Union, PRX-102 will be of significant benefit to those affected by Fabry disease. The COMP cited clinical and non-clinical justifications provided by Protalix to establish the significant benefit of pegunigalsidase alfa, noting that the COMP considered them to constitute a clinically relevant advantage.

pegunigalsidase alfa is the Company's PEGylated, chemically-modified version of the recombinant alpha-Galactosidase-A protein, for the treatment of Fabry disease. pegunigalsidase alfa is currently being evaluated globally in three phase III clinical trials.

To be designated an Orphan Medicinal Product, the sponsor must establish that no satisfactory method of diagnosis, prevention or treatment of the condition can be authorized in the European Union or, if such a method exists, the medicinal product must be of significant benefit to those affected by the condition. In addition, the sponsor must establish that the medicinal product in development is intended for the treatment, prevention or diagnosis of a disease that is life-threatening or chronically debilitating. The prevalence of the condition must not be more than five in 10,000 persons.

“We are delighted to receive the positive opinion for orphan drug designation for PRX-102 for the treatment of Fabry disease. The action from the EMA on this decision mirrors our own strategy for developing PRX-102 as a potential better therapeutic alternative for people living with Fabry disease, a patient population in need of new treatment options,” said Mr. Moshe Manor, Protalix’s President and Chief Executive Officer. “This is an important step in the continued advancement of our Fabry program, and furthers our mission to develop impactful, novel and clinically differentiated therapies.”

Orphan Medicinal Product designation potentially qualifies the sponsor for 10 years of marketing exclusivity upon approval, as well as certain fee reductions. The sponsor will also be eligible for access to a centralized authorization procedure, including applications for marketing authorization and inspections, and for protocol assistance.

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 “expect,” “anticipate,” “believe,” “estimate,” “project,” “plan,” “should” 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: risks related to the ultimate purchase by Fundação Oswaldo Cruz of alfataliglicerase pursuant to the stated purchase intentions of the Brazilian Ministry of Health of the stated amounts, if at all; risks related to the successful conclusion of our negotiations with the Brazilian Ministry of Health regarding the purchase of alfataliglicerase generally; risks related to our commercialization efforts for alfataliglicerase in Brazil; risks relating to the compliance by Fundação Oswaldo Cruz with its purchase obligations and related milestones under our supply and technology transfer agreement; risks related to our ability to maintain and manage our relationship with Chiesi Farmaceutici and any other collaborator, distributor or partner; 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; 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 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; 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; our ability to identify suitable product candidates and to complete preclinical studies of such product candidates; 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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