Protalix BioTherapeutics Provides Update on Complete Response Letter for Pegunigalsidase Alfa from the FDA

April 28, 2021

CARMIEL, Israel, April 28, 2021 /PRNewswire/ -- Protalix BioTherapeutics, Inc. (NYSE American: PLX) (TASE:PLX), a biopharmaceutical company focused on the development, production and commercialization of recombinant therapeutic proteins produced by its proprietary ProCellEx® plant cell-based protein expression system, today provided an update regarding the Complete Response Letter (CRL) received from the U.S. Food and Drug Administration (FDA) regarding the Biologics License Application (BLA) seeking accelerated approval of pegunigalsidase alfa (PRX–102) for the proposed treatment of adult patients with Fabry disease. Receipt of the CRL was announced earlier today.

The CRL did not report any concerns relating to the potential safety or efficacy of PRX–102 in the submitted data package.

In the CRL, the FDA noted that an inspection of Protalix's manufacturing facility in Carmiel, Israel, including the FDA's subsequent assessment of any related findings, is required before the FDA can approve the BLA. Due to travel restrictions, the FDA was unable to conduct the required inspection during the review cycle. The FDA explained that it will continue to monitor the public health situation as well as travel restrictions, and is actively working to define an approach for scheduling outstanding inspections. With respect to the third-party facility in Europe at which fill and finish processes are performed for PRX-102, due to COVID–19, the FDA reviewed records under Section 704(a)(4) of the Federal Food, Drug, and Cosmetic Act in lieu of a pre-licensing inspection. In the CRL, the FDA stated that it will communicate remaining issues to the facility in order to seek prompt resolution of any pending items.

In addition to the foregoing, the FDA noted that Fabrazyme® was recently converted to full approval which must be addressed in the context of any potential resubmission seeking accelerated approval of PRX-102. Protalix intends to work collaboratively with the agency to identify the most expeditious pathway to approval, including accelerated approval. Protalix remains confident that it will be able to work with the FDA to resolve these issues and provide a new, alternative drug to Fabry patients. Protalix intends to request a Type-A meeting with the FDA, and will provide further updates on next steps after the meeting.

About Pegunigalsidase Alfa (PRX–102)

Pegunigalsidase alfa (PRX–102) is an investigational, plant cell culture-expressed, and chemically modified stabilized version of the recombinant α-Galactosidase-A enzyme. Protein sub-units are covalently bound via chemical cross-linking using short PEG moieties, resulting in a molecule with unique pharmacokinetic parameters. In clinical studies, PRX–102 has been observed to have a circulatory half-life of approximately 80 hours. Protalix designed PRX–102 to potentially address the continued unmet clinical need in Fabry 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 was the first company to gain FDA approval of a protein produced through plant cell-based in suspension expression system. Protalix's unique expression system represents a new method for developing recombinant proteins in an industrial-scale manner.

Protalix's first product manufactured by ProCellEx, taliglucerase alfa, was approved by the 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 consists of proprietary versions of recombinant therapeutic proteins that target established pharmaceutical markets, including the following product candidates: pegunigalsidase alfa, a modified stabilized version of the recombinant human α–Galactosidase–A protein for the treatment of Fabry disease; alidornase alfa or PRX-110, for the treatment of various human respiratory diseases or conditions; PRX–115, a plant cell-expressed recombinant PEGylated uricase for the treatment of refractory gout; PRX–119, a plant cell-expressed long action DNase I for the treatment of NETs-related diseases; and others. Protalix has partnered with Chiesi Farmaceutici S.p.A., both in the United States and outside the United States, for the development and commercialization of pegunigalsidase alfa, and with SarcoMed USA, Inc. for the worldwide development and commercialization of PRX–110 for use in the treatment of any human respiratory disease or condition including, but not limited to, sarcoidosis, pulmonary fibrosis, and other related diseases via inhaled delivery.

Forward-Looking Statements Disclaimer

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," "may," "plan," "will," "would," "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 and the final results of a clinical trial may be different than the preliminary findings for the clinical trial. Factors that might cause material differences include, among others: Risks related to the timing and progress of the preparation of an updated BLA addressing the complete response letter; Risks related to the timing, progress and likelihood of final approval by the FDA of a resubmitted BLA for PRX–102 and, if approved, whether the use of PRX-102 will be commercially successful; failure or delay in the commencement or completion of our preclinical studies 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 or unwillingness of medical investigators and institutional review boards to follow our clinical protocols; and inability to monitor patients adequately during or after treatment; risks associated with the novel coronavirus disease, or COVID-19, outbreak, which may adversely impact our business, preclinical studies and clinical trials; risks related to any transactions we may effect in the public or private equity markets to raise capital to finance future research and development activities, general and administrative expenses and working capital; the risk that the results of the clinical trials of our product candidates will not support the applicable claims of safety or efficacy, or 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 our ability to maintain and manage our relationship with our collaborators, distributors or partners; risks related to the amount and sufficiency of our cash and cash equivalents; 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, EMA 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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