



Chiesi Global Rare Diseases and Protalix BioTherapeutics Receive Positive CHMP Opinion for an Additional Dosing Regimen of Every Four Weeks for Elfabrio® (pegunigalsidase alfa) in the EU

Descrizione

COMUNICATO STAMPA - CONTENUTO PROMOZIONALE

Committee for Medicinal Products for Human Use (CHMP) issues a positive opinion following re-examination, which will be reviewed by the European Commission (EC), with a decision anticipated by March 2026

If approved by the EC, this dosing regimen would reduce the burden to eligible patients, their families and the broader healthcare system due to the current requirement to visit infusion centres every two weeks for treatment

PARMA, Italy and CARMIEL, Israel, Jan. 30, 2026 (GLOBE NEWSWIRE) - Chiesi Global Rare Diseases, a business unit of the Chiesi Group established to deliver innovative therapies and solutions for people living with rare diseases, and Protalix BioTherapeutics, Inc. (NYSE American: PLX), a biopharmaceutical company focused on the discovery, development, production and commercialization of innovative therapeutics for rare diseases with significant unmet needs, today announced an update on pegunigalsidase alfa. The Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has issued a positive opinion recommending approval of the 2mg/kg every-4-weeks (E4W) dosing regimen for pegunigalsidase alfa in Fabry disease adult patients stable with an ERT treatment. This positive opinion follows the CHMP's re-examination of the company's application for the additional dosing regimen.

"It is our privilege to provide the Fabry community with a well-tolerated and effective option, and we are thrilled that the CHMP positive opinion supporting an every-four-week dosing regimen brings us one step forward to further reducing treatment burden in this condition," said Giacomo Chiesi, Executive Vice President, Chiesi Global Rare Diseases. "We're focused on evolving treatments based on real-world needs, so that people have not just the right care, but care that fits naturally into their lives."

By extending the time between infusions, our aim is that people living with this condition can focus on what truly matters, living their lives.â•

â•Expanding the range of treatment options is critical to better meet the needs of people with Fabry disease,â• said Prof. AleÅ Linhart, DrSc, FESC. â•Beyond managing the condition effectively, this extended administration protocol acknowledges the importance of meeting patient preferences in order to reduce disruptions to their daily lives.â•

â•The CHMPâ•s positive opinion is another testament to Protalixâ• s commitment to advancing treatments for people living with Fabry disease and, together with Chiesi, we are grateful to all of the patients and investigators and their staff members who participated in our clinical trial programs,â• said Dror Bashan, President and Chief Executive Officer of Protalix. â•The CHMPâ•s positive opinion is a powerful validation for Protalixâ• s innovative pipeline and our proprietary ProCellExÂ® manufacturing platform, built on years of rigorous research and clinical progress.â•

â•The CHMP positive opinion on the every-four-week regimen recognizes the importance of reducing treatment burden for people living with Fabry and their families,â• said Mary Pavlou, President, Fabry International Network (FIN). â•Extending infusion intervals allows therapy to better fit into everyday life, supporting work, study, and family commitments. This step reflects care that adapts to real life and respects the priorities of those who live with the disease.â•

The CHMP opinion is based on results from an open-label, switch-over study, BRIGHT (formally PB-102-F50), designed to assess the safety, efficacy, and pharmacokinetics (PK) of the new dosing regimen of pegunigalsidase alfa 2 mg/kg E4W for 52 weeks,¹ and its ongoing open-label extension study CLI-06657AA1-03 (formerly PB-102-F51, with a median exposure of almost 6 years).² Further support is provided by an updated Population Pharmacokinetics (PopPK) model and exposureâ•response analysis, which leverage data from multiple clinical studies.

Protalix will be eligible to receive a regulatory milestone payment of \$25 million from Chiesi if the E4W dosing regimen is approved by the EC.

About Fabry DiseaseFabry disease is a rare, inherited lysosomal storage disorder caused by mutations in the GLA gene, which leads to a deficiency of the enzyme alpha-galactosidase A. This deficiency results in an accumulation of a fatty substance called globotriaosylceramide (GL-3) in the bodyâ•s cells, affecting the heart, kidneys, skin, nervous system, and other organs.³ Fabry disease can cause a range of serious signs and symptoms, including fatigue, chronic pain, gastrointestinal issues, decreased ability to sweat, progressive kidney failure, heart complications, and increased risk of stroke.⁴

The condition affects both males and females and can present from childhood through adulthood, often with delayed diagnosis or misdiagnosis. While Fabry disease is rare, early detection and access to appropriate treatment â• such as enzyme replacement therapy or pharmacological chaperones â• are critical in managing symptoms and slowing disease progression.³

About Chiesi GroupChiesi is a research-oriented international biopharmaceutical group that develops and markets innovative therapeutic solutions in respiratory health, rare diseases, and specialty care.

The company's mission is to improve people's quality of life and act responsibly towards both the community and the environment.

By changing its legal status to a Benefit Corporation in Italy, the US, France and Colombia, Chiesi's commitment to creating shared value for society as a whole is legally binding and central to company-wide decision-making. As a certified B Corp since 2019, Chiesi is part of a global community of businesses that meet high standards of social and environmental impact. The company aims to reach Net-Zero greenhouse gases (GHG) emissions by 2035.

With 90 years of experience, Chiesi is headquartered in Parma (Italy), with 31 affiliates worldwide, and counts more than 7,500 employees. The Group's research and development center in Parma works alongside 6 other important R&D hubs in France, the US, Canada, China, the UK, and Sweden.

For more information visit www.chiesi.com.

About Chiesi Global Rare Diseases Chiesi Global Rare Diseases is a business unit of the Chiesi Group established to deliver innovative therapies and solutions for people living with rare diseases. As a family business, Chiesi Group strives to create a world where it is common to have therapy for all diseases and acts as a force for good, for society and the planet. The goal of the Global Rare Diseases unit is to ensure equal access so as many people as possible can experience their most fulfilling life. The unit collaborates with the rare disease community around the globe to bring voice to underserved people in the health care system.

For more information visit www.chiesirarediseases.com.

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About Protalix BioTherapeutics, Inc. Protalix is a biopharmaceutical company focused on the discovery, development, production and commercialization of innovative therapeutics for rare diseases. Protalix has researched, developed and currently manufactures two enzyme replacement therapies that are currently available in multiple markets. These therapies are recombinant therapeutic proteins expressed through Protalix's proprietary plant cell-based expression system, ProCellEx®. ProCellEx is a unique plant cell-based system that enables Protalix to produce recombinant proteins in an industrial-scale manner with no exposure to mammalian cells. Protalix is the first company to gain U.S. Food and Drug Administration (FDA) approval of a protein produced through plant cell-based in suspension expression system. Protalix has licensed to Pfizer Inc. the worldwide development and commercialization rights to taliglucerase alfa, Elelyso®, for the treatment of Gaucher disease, excluding in Brazil, where Protalix retains full rights. Protalix has partnered with Chiesi Farmaceutici S.p.A. for the global development and commercialization of pegunigalsidase alfa, which was approved by both the FDA and the EMA in May 2023. Protalix's development pipeline includes, among others, two proprietary versions of recombinant therapeutic proteins that target established pharmaceutical markets: PRX115, a plant cell-expressed recombinant PEGylated uricase for the treatment of uncontrolled gout; and PRX119, a plant cell-expressed long acting DNase I for the treatment of NETs-related diseases; To learn more, please visit www.protalix.com.

Protalix BioTherapeutics, Inc. 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, can, continue, could, intend, may, plan, potential, predict, project, should, will, would 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: the risk that the EC will not approve the CHMP's positive opinion recommending approval of the 2mg/kg every-4-weeks (E4W) dosing regimen for pegunigalsidase alfa in adults with Fabry disease; risks related to the commercialization of pegunigalsidase alfa; risks relating to pegunigalsidase alfa market acceptance, competition, reimbursement and regulatory actions, including as a result of the boxed warning contained in the FDA approval received for the product; delays in the approval or potential rejection of any applications filed with the FDA, EMA or other health regulatory authorities for Protalix's product candidates, and other risks relating to the review process; the risk that the results of clinical trials will not support the applicable claims of safety or efficacy; ; risks relating to changes to published interim, topline or preliminary data from clinical trials; 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 risks relating to changes in healthcare laws, rules and regulations in the United States or elsewhere; 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 Protalix disclaims any obligation to update this information, except as may be required by law.

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