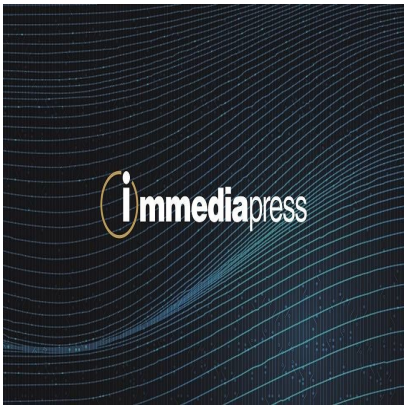

Chiesi Global Rare Diseases and Protalix BioTherapeutics Receive Positive CHMP Opinion for an Additional Dosing Regimen of Every Four Weeks for ElfabrioÂ® (pegunigalsidase alfa) in the EU

Descrizione

COMUNICATO STAMPA â?? 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

This dosing regimen for Elfabrio is not approved in the

U.S.
In
the
U.S.,
the
approved dosing
regimen
remains
1
mg/kg
every
2
weeks.
Please
consult
with
your
healthcare
provider

PARMA, Italy and CARMIEL, Israel, Jan. 30, 2026 (GLOBE NEWSWIRE) â?? 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 ElfabrioÂ® (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 Elfabrio in Fabry disease adult patients stable with an ERT (Enzyme Replacement Therapy) treatment. This positive opinion follows the CHMPâ??s re-examination of the companyâ??s application for the additional dosing regimen.

â??It is our privilege to provide the Fabry community with a safe 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,â?• said Giacomo Chiesi, Executive Vice President, Chiesi Global Rare Diseases. â??Weâ??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 of 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, and its ongoing open-label extension study CLI-06657AA1-03 (formerly PB-102-F51, with a median exposure of almost 6 years). 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.

This dosing regimen for Elfabrio is not approved in the U.S. In the U.S., the approved dosing regimen remains 1 mg/kg every 2 weeks. Please consult with

your
healthcare
provider.

About ELFABRIOELFABRIO (pegunigalsidase alfa-iwxj), a PEGylated enzyme replacement therapy (ERT) to treat Fabry disease, is a plant cell culture-expressed, and chemically modified stabilized recombinant version of the Î±-â??Galactosidase-â??A enzyme. Protein sub-units are covalently bound via chemical cross-linking using short PEG moieties, resulting in a molecule with stable pharmacokinetic parameters. In clinical studies, ELFABRIO has been observed to have an initial half-life of 78.9 Â± 10.3 hours.

Important Safety Information Indication

ElfabrioÂ® (pegunigalsidase alfa-iwxj) is indicated for the treatment of adults with confirmed Fabry disease.

Important Safety Information

Prior to Elfabrio administration, consider pretreating with antihistamines, antipyretics, and/or corticosteroids. Inform patients and caregivers of the signs and symptoms of hypersensitivity reactions and infusion-associated reactions (IARs), and instruct them to seek medical care immediately if such symptoms occur.

In clinical trials, 20 (14%) Elfabrio-treated patients experienced hypersensitivity reactions. Four Elfabrio-treated patients (3%) experienced anaphylaxis reactions that occurred within 5 to 40 minutes of the start of the initial infusion. The signs and symptoms of hypersensitivity reactions and anaphylaxis included headache, nausea, vomiting, throat tightness, facial and oral edema, truncal rash, tachycardia, hypotension, rigors, urticaria, intense pruritus, moderate upper airway obstructions, macroglossia, and mild lip edema.

In clinical trials, 41 (29%) Elfabrio-treated patients experienced one or more infusion-associated reactions, including hypersensitivity, nausea, chills, pruritus, rash, chest pain, dizziness, vomiting, asthenia, pain, sneezing, dyspnea, nasal congestion, throat irritation, abdominal pain, erythema, diarrhea, burning sensation, neuralgia, headache, paresthesia, tremor, agitation, increased body temperature, flushing, bradycardia, myalgia, hypertension, and hypotension.

A case of membranoproliferative glomerulonephritis with immune depositions in the kidney was reported during clinical trials. Monitor serum creatinine and urinary protein-to-creatinine ratio. If glomerulonephritis is suspected, discontinue treatment until a diagnostic evaluation can be conducted.

When switching to Elfabrio from a prior enzyme replacement therapy, the risk of hypersensitivity reactions and infusion-associated reactions may be increased in certain patients with pre-existing anti-drug antibodies (ADAs). Consider monitoring IgG and IgE ADAs and clinical or pharmacodynamic response (eg, plasma lyso-Gb3 levels). The most common adverse reactions (â?¥15%) were infusion-associated reactions, nasopharyngitis, headache, diarrhea, fatigue, nausea, back pain, pain in

extremity, and sinusitis.

Please see FullPrescribing Information for Elfabrio including Boxed Warning, for ElfabrioÂ® (pegunigalsidase alfa).â?•

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.1 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.2

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.1

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 companyâ??s mission is to improve peopleâ??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, Chiesiâ??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 Groupâ??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 DiseasesChiesi 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 Protalixâ??s proprietary plant cell-based expression system, ProCellExÂ®. 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, ElelysoÂ®, 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 Elfabrio, which was approved by both the FDA and the EMA in May 2023. Protalixâ??s development pipeline includes, among others, two proprietary versions of recombinant therapeutic proteins that target established pharmaceutical markets: PRXâ??115, a plant cell-expressed recombinant PEGylated uricase for the treatment of uncontrolled gout; and PRXâ??119, 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 StatementsTo 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 â??anticipate,â?• â??believe,â?• â??estimate,â?• â??expect,â?• â??can,â?• â??continue,â?• â??could,â?• â??intend,â?• â??may,â?• â??plan,â?• â??potential,â?• â??predict,â?• â??project,â?• â??should,â?• â??will,â?• â??wouldâ?• 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 CHMPâ??s positive opinion recommending approval of the 2mg/kg every-4-weeks (E4W) dosing regimen for Elfabrio in adults with Fabry disease; risks related to the commercialization of Elfabrio; risks relating to Elfabrio 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 Protalixâ??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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Categoria

1. Comunicati

Tag

1. ImmediaPress

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Gennaio 30, 2026
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redazione