PRESS RELEASE
For Medical & Trade Media

Chiesi Receives MHRA Approval for Additional Dosing Regimen of Elfabrio®▼ (pegunigalsidase alfa) 2mg/kg administered every-four-weeks in adults stable with an enzyme replacement therapy

- The UK Medicines and Healthcare products Regulatory Agency (MHRA) has approved an additional dosing regimen of 2mg/kg administered every-four-weeks for pegunigalsidase alfa in adults stable with an enzyme replacement therapy (ERT).
- Pegunigalsidase alfa is a long-term ERT for adult patients with Fabry disease – a rare disease affecting approximately 1 in 40,000 people in the UK.[1,2]
- This adjustment in dosing regimen would reduce the burden and frequency with which eligible UK adult Fabry patients require infusions, from once-every-two-weeks to once-every-four-weeks.

Manchester, UK – 14 May 2026 – Chiesi UK and Ireland has today announced that the MHRA has approved an additional 2mg/kg body weight every-four-week dosing regimen for pegunigalsidase alfa for Fabry adults stable with an ERT. This follows a recent positive European Medicines Agency (EMA) decision in March 2026.

Pegunigalsidase alfa is a long-term enzyme replacement therapy (ERT) for adult patients with a confirmed diagnosis of Fabry disease (deficiency of alpha-galactosidase) - a rare disease affecting approximately 1 in 40,000 people in the UK.[1,2] It was originally approved by the MHRA in August 2023, with a dosing regimen of 1mg/kg body weight administered every-two-weeks. The approval for the additional dosing regimen means that it would halve the number of infusions which eligible UK adult Fabry patients require, from approximately twenty-six per year to thirteen.

Derralynn Hughes, Professor of Experimental Haematology, Director of Research and Innovation, and Co-Clinical Director of the NCL Cancer Alliance said: *"For many people living with Fabry disease, regular fortnightly infusions can have a significant impact on day-to-day life, affecting work, family routines, and overall quality of life. The patient community consistently highlights the need for treatment approaches that help reduce this burden where clinically appropriate, and we look forward to being able to offer infusions every-four-weeks to those who are stable on their current ERT, with appropriate monitoring."*

The MHRA approval is informed by results from an open-label, switch-over study, BRIGHT (formally PB-102-F50), designed to assess the adverse event profile, efficacy, and pharmacokinetics (PK) of the additional dosing regimen of pegunigalsidase alfa 2 mg/kg body weight every-four-weeks for 52 weeks,[3] and its ongoing open-label extension study CLI-06657AA1-03 (formerly PB-102-F51).[4]

Bob Stevens, Group Chief Executive Officer of the MPS Society and Chief Executive Officer and Chair of Rare Disease Research Partners, said: *"People living with Fabry*

disease plan their lives around treatment. The possibility of attending infusions once every four weeks instead of two would offer greater flexibility and may help ease some of the practical challenges that come with long-term treatment. It is encouraging to see continued efforts to deliver options that may better reflect the needs of the Fabry community."

David Garzón, Senior Director, Rare Diseases, UK and Ireland said*: "We are pleased to receive MHRA approval for the additional dosing option for pegunigalsidase alfa in adults stable with an ERT. This is a positive step forward in improving quality of life for patients and their families. Our sincere thanks to the community who have collaborated throughout the regulatory approval process and continue to stand side by side with us to benefit patient care."*

For patients switched to pegunigalsidase alfa 2 mg/kg body weight once-every-four-weeks, regular monitoring (e.g. after 3, 6, 12, 18 and 24 months) should be performed. Monitoring should include at least the evaluation of lyso-Gb3, renal (eGFR, proteinuria), cardiac (LVMi, NT-proBNP, troponin or ECG), and biochemical parameters. A change in any individual parameter should be interpreted in the context of the patient's overall clinical status, and clinically relevant deterioration should prompt re-evaluation of the treatment regimen.[5] During the study, safety and tolerability profiles of the E4W dosing regimen were consistent with previous studies, with no severe or serious treatment-related TEAEs and all infusion-related reactions being mild or moderate in severity.

Eligible people in the UK may be prescribed the new dosing regimen of pegunigalsidase alfa from the date of approval if determined appropriate by their healthcare professional.

About Fabry Disease

Fabry 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 body's cells, affecting the heart, kidneys, skin, nervous system, and other organs.[6] 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.[7]

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 — such as enzyme replacement therapy or pharmacological chaperone therapy — are critical for long term disease management.[6]

About Pegunigalsidase alfa

Pegunigalsidase alfa, a PEGylated enzyme replacement therapy (ERT) to treat adults with Fabry disease, is a plant cell culture-expressed, and chemically modified stabilised recombinant version of the α-Galactosidase-A enzyme. Protein sub-units are covalently bound via chemical cross-linking using short polyethylene glycol (PEG)

moieties, resulting in a molecule with stable pharmacokinetic parameters. Pegunigalsidase alfa has been observed to have plasma half-life ranging from 53 – 134 hour.[1] Clinical studies have not shown that differences in pharmacological characteristics, including half-life, result in clinically meaningful differences in efficacy or clinical outcomes.

About Chiesi Group

Chiesi 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, and France, Chiesi's commitment to create shared value for society as a whole is legally binding and central to company-wide decision-making. As a certified B Corp since 2019, we're 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 over 85 years of experience, Chiesi is headquartered in Parma (Italy), with 31 affiliates worldwide, and counts more than 7,000 employees. The Group's research and development centre in Parma works alongside 6 other important R&D hubs in France, the US, Canada, China, the UK, and Sweden.

For further information please visit www.chiesi.uk.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.

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