PRESS RELEASE

# Chiesi Global Rare Diseases Announces FDA Approval of Lamzede® (velmanase alfa-tycv)

- *First and only enzyme replacement therapy for the treatment of non-central nervous system manifestations of alpha-mannosidosis in adult and paediatric patients;*
- *Ultra-rare progressive disease which presents with wide range of symptoms.*

BOSTON, February 20, 2023 – Chiesi Global Rare Diseases, a business unit of Chiesi Farmaceutici S.p.A., an international research-focused healthcare Group (Chiesi Group), announced today that the U.S. Food and Drug Administration (FDA) has approved Lamzede® (velmanase alfa-tycv) for the treatment of non-central nervous system manifestations of alpha-mannosidosis (AM) in adult and pediatric patients. AM is an ultra-rare, progressive lysosomal storage disorder caused by deficiency in the enzyme alpha-mannosidase.[1,2]

"*Today's approval of velmanase alfa represents a major milestone for people living with alpha-mannosidosis. Velmanase alfa is the first and only enzyme replacement therapy approved for alpha-mannosidosis in the United States, an achievement based on years of clinical development, as well as the dedication of our employees, clinicians, patients and their families*," said Giacomo Chiesi, Head of Chiesi Global Rare Diseases. "Alpha-mannosidosis presents with a variety of symptoms including impaired hearing, speech and mobility that progress from childhood into adulthood and is often under-recognised. Velmanase alfa is designed to provide an exogenous source of the α-mannosidase enzyme, and we look forward to the opportunity to offer a potential treatment option for alpha-mannosidosis patients residing in the United States."

The prevalence of AM is approximately one in every 500,000 newborns.[1] AM results in the body's cells being unable to properly break down certain groups of complex sugars.[1] The buildup of sugars can affect many of the body's organs and systems.[1] Effects of the disease vary significantly from person to person and progress over time.[2] Symptoms may change as a patient gets older and can include recurrent chest and ear infections, hearing loss, distinctive facial features, muscle weakness, skeletal and joint abnormalities, visual abnormalities, and cognitive abnormalities.[1,2]

Velmanase alfa is a recombinant form of human α-mannosidase intended to provide or supplement natural α-mannosidase, an enzyme that is involved in the degradation of mannose–rich oligosaccharides to prevent their accumulation in various tissues in the body.

"*We are thrilled to witness this milestone for the alpha-mannosidosis community*," said Mark Stark, treasurer at the International Society for Mannosidosis & Related Disorders (ISMRD) and father of a son living with AM. "*I have watched first-hand how alpha-mannosidosis can progress and impact daily life with my son and this approval gives hope to patients, caregivers and families impacted by this devastating disease. ISMRD is excited to continue to collaborate with Chiesi and we are thankful for their efforts to develop velmanase alfa to give patients in the U.S. a much-needed treatment option*."

## About velmanase alfa and alpha mannosidosis

Velmanase alfa is a once-weekly enzyme replacement therapy used for the treatment of non-neurological manifestations in patients with mild to moderate alpha-mannosidosis. Velmanase alfa received European Marketing Authorisation in March 2018 and is the only pharmacological licensed treatment for patients with alpha mannosidosis. For the Summary of Product Characteristics for velmanase alfa, see https://www.medicines.org.uk/emc/product/12836.

## 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 affected by rare diseases. As a family business, Chiesi Group strives to create a world where it is common to have a 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.

## About Chiesi Group

Chiesi is an international, research-focused biopharmaceuticals 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 at becoming net-zero by 2035.
With over 85 years of experience, Chiesi is headquartered in Parma (Italy), operates in 30 countries, and counts more than 6,000 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.com

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## References

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[2] Adam J, Malone R, Lloyd S, Lee J, Hendriksz CJ, Ramaswami U. Disease progression of alpha-mannosidosis and impact on patients and carers – A UK natural history survey. *Mol. Genet. Metab. Rep.* 2019;20(100480).