

UK-LAM-2200039: November 2022

## Chiesi Limited responds to NICE evaluation consultation document (ECD) for Lamzede▼<sup>®</sup> (velmanase alfa) for patients with alpha mannosidosis

Manchester, UK, 1 November 2022 – The National Institute for Health and Care Excellence (NICE) has opened a consultation on its initial recommendation for Lamzede<sup>®</sup> (velmanase alfa) for patients with alpha mannosidosis on the NHS in England and Wales.

Chiesi Limited will continue to work closely with NICE to ensure this treatment becomes available to eligible patients with alpha mannosidosis in England and Wales as soon as possible, as currently there are no available funded treatments for patients with alpha mannosidosis in England and Wales.

Velmanase alfa is a once-weekly enzyme replacement therapy for patients with mild to moderate alpha mannosidosis, an extremely rare inherited condition which affects around 25 patients in England and Wales.<sup>1</sup>

The Scottish Medicines Consortium (SMC) accepted velmanase alfa for patients with alpha mannosidosis within its ultra-orphan pathway in September 2022.<sup>2</sup>

### 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.<sup>3</sup> For the Summary of Product Characteristics for velmanase alfa, see <https://www.medicines.org.uk/emc/product/12836>.

Alpha mannosidosis is a lysosomal storage disorder caused by inheriting a faulty copy of the MAN2B1 gene from both parents, resulting in impaired production of the enzyme alpha-mannosidase.<sup>1</sup> Alpha mannosidosis is an extremely rare disorder with an estimated prevalence of 1 in 500,000-1,000,000, equating to 25 patients in England.<sup>1</sup> Alpha mannosidosis may cause a broad range of progressive symptoms including: difficulty controlling movement, breathing problems, deafness, speech difficulties, infections, bone abnormalities, muscle pain and weakness, and cognitive impairment.<sup>4</sup> Patients with alpha mannosidosis experience increasing problems with mobility and motor function as their disease progresses, which can result in wheelchair dependence. This has a considerable impact on a patient's ability to perform daily activities and also affects their carers who support their daily living.<sup>4</sup>

### 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. Since 2019 Chiesi is certified B Corp, meaning that its sustainability efforts are measured and assessed by the most ambitious global standards. 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 more information, please visit [www.chiesi.uk.com](http://www.chiesi.uk.com).

### Certified B Corps®

B Corps® are companies that meet the highest standards of verified social and environmental performance, public transparency, and legal accountability to balance profit and purpose. B Corps form a community of leaders and drive a global movement of people using business as a force for good: their economic activity, in addition to generating profit, creates a measured and certified positive impact on people, society and the environment. Certified B Corps achieve a verified score on the B Impact Assessment - a rigorous, material measurement of a company's impact on its workers, customers, community, and environment - and make their B Impact Report transparent on [bcorporation.net](http://bcorporation.net). B Corps are present in 155 sectors and in more than 80 countries. The B Corp movement has a single goal: to redefine a new business paradigm that is adequate to our times, concrete and replicable. Through the B Impact Assessment, measure all their impacts as thoroughly as their profits. The combination of third-party validation, public transparency, and legal accountability help Certified B Corps build trust and value. The Certification is administered by the non-profit B Lab. For more information, please visit [www.bcorporation.net](http://www.bcorporation.net).

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<sup>1</sup> NICE. Evaluation consultation document. Velmanase alfa for treating alpha mannosidosis. Available at <https://www.nice.org.uk/guidance/gid-hst10010/documents/evaluation-consultation-document-2> Accessed October 2022.

<sup>2</sup> Scottish Medicines Consortium. Medicine: velmanase alfa (brand name: Lamzede®) for alpha mannosidosis. Available at <https://www.scottishmedicines.org.uk/media/7089/ae-velmanase-alfa-lamzede-smc2466.pdf> Accessed October 2022.

<sup>3</sup> Chiesi Farmaceutici S.p.A. Lamzede 10 mg powder for solution for infusion. SmPC. 2022. Available at <https://www.medicines.org.uk/emc/product/12836> Accessed October 2022.

<sup>4</sup> SMC, Scottish Medicines Consortium. SMC2466: UMAR report velmanase alfa 2022. 5 August 2022.