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****PRESS RELEASE****

Chiesi Group receives positive opinion from CHMP for Lamzede® (velmanase alfa), the first therapy for Alpha-Mannosidosis

- Lamzede® (velmanase alfa) is the first pharmacological therapy for the treatment of Alpha-Mannosidosis (AM), an ultra-rare, disabling, genetic disorder[1]
- AM is a lysosomal storage disorder caused by the deficiency in the activity of the enzyme alpha-mannosidase, involved in the cellular breakdown of glycoproteins. This deficiency leads to the progressive toxic accumulation of undigested sugars in cells and tissues, resulting in progressive physical and intellectual disabilities, recurrent infections, and skeletal abnormalities.
- Velmanase alfa is administered by weekly intravenous infusions and is designed to replace the missing or malfunctioning enzyme in patients with AM.

Parma (Italy), 29 January 2018 – Chiesi Group (Chiesi), an international research-focussed Healthcare company, announced today that the European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) has issued a **positive opinion** recommending the marketing authorisation of **velmanase alfa**, under the brand name **Lamzede®**, providing, for the first time, a treatment for Alpha-Mannosidosis (AM), an ultra-rare progressive and debilitating disease[1].

Velmanase alfa is administered via weekly intravenous infusions to replace the missing or malfunctioning enzyme causing the disease. The positive CHMP opinion has been issued under "exceptional circumstances" according to the EU legislation, which aim to enable treatment of extremely rare disorders for which traditional large-scale clinical studies are not feasible. **Velmanase alfa** has been investigated in 33 patients, both children and adults.

AM is an ultra-rare, genetic lysosomal storage disorder caused by the absence or malfunction of alpha-mannosidase, an enzyme involved in the cellular breakdown of glycoproteins. The deficiency in alpha-mannosidase activity is caused by genetic mutations in the MAN2B1 gene and leads to the progressive toxic accumulation of undigested oligosaccharides in the cells of many tissues and organs. The most frequent clinical phenotype of AM encompasses a broad range of manifestations, including facial coarsening, intellectual disability, progressive motor function disturbances and physical disability, hearing impairment, impaired speech, immunodeficiency and recurrent infections, psychiatric symptoms, and skeletal abnormalities. The long-term prognosis is generally poor, with reduced life expectancy[1].

Paolo Chiesi, **Chiesi Group Vice President and Head of R&D**, said, "*Today's positive opinion is an important milestone for bringing this therapeutic option to patients affected by Alpha-Mannosidosis across Europe. I would like to thank all the patients, their families, and the healthcare professionals who participated in the clinical studies, and all Chiesi and Zymenex employees who worked with passion to make velmanase alpha a clinical reality. Chiesi Group is strongly committed to developing and commercialising innovative therapeutic options for patients affected by rare or ultra-rare disorders, as also demonstrated by our recent partnership agreements with Horizon Pharma plc and Protalix Biotherapeutics*."

Velmanase alfa was initially developed by Zymenex A/S, which was acquired by Chiesi in 2013. With this acquisition, Chiesi has established an integrated biotech research group based in Hillerod (Denmark) and Lidingö (Sweden) focussed on the development of protein-based therapeutics. The initial development of velmanase alpha was also supported by a Europe-wide research consortium and was the first investigational treatment to win an EU research grant for early clinical studies within the Framework VII (project Alpha-Mann, FP7-HEALTH-2010-261331).

The CHMP positive opinion is one of the final steps before the European Commission grants marketing authorisation. The granting of the marketing authorisation is expected in the second quarter of 2018.

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About Lamzede® (Velmanase alfa)

The active substance of Lamzede is velmanase alfa, a recombinant form of human alpha-mannosidase (ATC code: A16AB15). Lamzede is an enzyme replacement therapy intended to provide or supplement natural alpha-mannosidase, an enzyme that helps with the degradation of mannose–rich oligosaccharides and thus prevents their accumulation in various tissues in the body. Velmanase Alfa was designated as an orphan medicinal product on 26 January 2005.

The expected full indication is: “Enzyme replacement therapy for the treatment of non-neurological manifestations in patients with mild to moderate alpha-mannosidosis.” It is proposed that velmanase alfa be prescribed by physicians experienced in the management of patients with alpha-mannosidosis, or in the administration of other enzyme replacement therapies for lysosomal storage disorders.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

About Chiesi Group
Based in Parma, Italy, Chiesi Farmaceutici is an international research-focused Healthcare Group, with over 80 years of experience in the pharmaceutical industry, present in 26 countries. Chiesi researches, develops and markets innovative drugs in the respiratory therapeutics, specialist medicine and rare disease areas. Its R&D organization is headquartered in Parma (Italy), and integrated with 6 other key R&D groups in France, the USA, the UK, Sweden, and Denmark to advance Chiesi's pre-clinical, clinical and registration programmes. Chiesi employs more than 5,000 people. More info at www.chiesi.com

References

1. Malm D, Nilssen Ø. Alpha-mannosidosis *Orphanet J Rare Dis*. 2008;3:21

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For more information

Valentina Biagini
Chiesi Group
Senior Group Communication Manager
Tel : +39 348 7693 623;
Email: v.biagini@chiesi.com

Jess Wilkinson
M&F Health
Senior Account Executive
Tel: +44 7492 1778
Email: jessica.wilkinson@mandfhealth.com