

Chiesi Global Rare Diseases Announces Multiple Presentations at SSIEM Annual Symposium 2023

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- *Total of six poster presentations analyze the effects of treatment with enzyme replacement therapies in Fabry disease and alpha-mannosidosis;*
- *Company sponsoring symposium on advances in monitoring and management in Fabry disease.*

BOSTON (USA), September 1st, 2023 – Chiesi Global Rare Diseases, a business unit of the Chiesi Group established to deliver innovative therapies and solutions for people affected by rare diseases, today announced multiple presentations at the Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Symposium 2023 being held August 29 - September 1, 2023, in Jerusalem, Israel.

*“There continues to be a general lack of awareness and a limited appreciation of the numerous unique challenges that people living with rare lysosomal storage diseases face every day,” said **Giacomo Chiesi, Head of Chiesi Global Rare Diseases.** “As a family business, and as a certified benefit corporation, we are focused on the long-term support of these patients and will continue to challenge the status quo by building awareness of unmet medical needs and embracing drug development risks. As we continue to significantly invest in innovation and seek opportunities to improve health outcomes, we are pleased to showcase multiple data presentations in Fabry disease and alpha-mannosidosis at the SSIEM symposium.”*

Four poster presentations at the SSIEM symposium feature analyses of the safety and efficacy of pegunigalsidase alfa, the company’s PEGylated enzyme replacement therapy (ERT), in the

treatment of Fabry disease.

In a poster, titled, "Pooled safety profile of pegunigalsidase alfa: an analysis of data from 142 patients in the pegunigalsidase alfa clinical program," John Bernat, M.D., Ph.D., University of Iowa Hospitals and Clinics, USA, is presenting an analysis of treatment-emergent adverse events (TEAEs), infusion-related reactions (IRRs) during the infusion or within two hours, and premedication use in four clinical trials and three long-term extension studies. This pooled analysis showed that pegunigalsidase alfa had an overall favorable safety profile with most (95.6%) TEAEs being of mild or moderate severity.

David Warnock, M.D., The University of Alabama at Birmingham, USA, is presenting a poster, titled, "Pooled analysis of the effect of pegunigalsidase alfa on renal function: Data from 113 patients in the pegunigalsidase alfa clinical trial program," that includes data from patients with more than 18 months of treatment across multiple trials. The results of this pooled analysis suggest that long-term pegunigalsidase alfa treatment provides continued renal function benefits in patients with Fabry disease.

In other Fabry disease posters, Dr. Bernat presents a post hoc analysis that found COVID-19 mRNA vaccines do not appear to increase the risk of IRRs or anti-drug antibodies in patients treated with pegunigalsidase alfa, and Ozlem Goker-Alpan, M.D., Lysosomal and Rare Disorders Research and Treatment Center, USA, describes the tolerability and safety of pegunigalsidase alfa in seven patients treated in an Expanded Access Program.

The company is also sponsoring a symposium on August 29 from 12:45-1:45pm IDT, "Creating a new roadmap: Advances in Fabry Disease Monitoring and Management," that is chaired by Dominique Germain, M.D., Ph.D., University of Versailles, Division of Medical Genetics, France. During the event, Derralynn Hughes, M.A., D.Phil., Royal Free NHS Foundation Trust and University College London, UK, is presenting an update on monitoring modalities, and Dawn Laney, M.S., Emory University School of Medicine, USA, is discussing how to incorporate the patient experience to guide monitoring and management.

In addition, two poster presentations at the SSIEM congress feature analyses of the impact of treatment with velmanase alfa in alpha-mannosidosis.

Benedicte Heron, M.D., Department of Pediatric Neurology, Reference Centre for Lysosomal Diseases, Armand Trousseau-La Roche Guyon University Hospital, France, is presenting a poster, titled, "Long-term efficacy of velmanase alfa treatment in patients with alpha mannosidosis: updated integrated analysis of data from phase I/II, III, and follow-up clinical trials," which concludes that the effect of the ERT on key efficacy outcomes appeared to be maintained over a long-term period (up to 12 years) in pediatric and adult patients (N=33 patients, observation time 0.9 to 11.9 years).

Angela Messina, Ph.D., Consiglio Nazionale delle Ricerche (CNR), Italy, is presenting a poster, titled, "IgG glycosylation in patients with alpha-mannosidosis and the impact of enzyme replacement therapy." The results of this study suggest that IgG sialylation/galactosylation could offer a potential new pharmacodynamic biomarker for evaluating treatment benefits in patients with alpha-mannosidosis.

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 to reach Net-Zero greenhouse gases (GHG) emissions by 2035.

With over 85 years of experience, Chiesi is headquartered in Parma (Italy), operates in 31 countries, and counts more than 6,500 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.

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