

Chiesi Global Rare Diseases Releases Report on the Burden of Care for Rare Diseases and Its Economic Impact on European Citizens

- New research from Chiesi analyses the economic burden for 23 rare diseases across five therapeutic areas in three EU Member States and will be launched during a panel at the World Orphan Drug Congress (WODC) in Barcelona, Spain¹.
- The panel hosted by Chiesi is titled, 'Reducing the burden of rare diseases: how data can inspire innovations and inform policies in the European Union' and features panelists from across the spectrum of rare disease-related care and research.
- Up to 36 million people in the European Union (EU) live with a rare disease², and the majority (up to 95%) of rare diseases having no current treatments available³.

Parma (Italy), October 30th, 2023 – Chiesi Global Rare Diseases, a business unit of the Chiesi Group established to deliver innovative therapies and solutions for people living with rare diseases, will be presenting *Rare Disease Burden of Care and the Economic Impact on Citizens*⁴, a comprehensive report that thoroughly examines the escalating care challenges and economic impact on European citizens living with rare diseases, supported by extensive research and data at the WODC.

For 23 rare diseases across five therapeutic areas, this report assesses the economic burden of each disease across Germany, France, and Italy. Direct, indirect, and mortality-related costs were considered and benchmarked against high prevalence diseases. Using scenario analysis, the report compares the burden when treatment is available and what the cost would have been if there were not effective treatments available for those diseases.

The data showcase how the availability of treatments creates positive value and alleviates financial strain on families and the healthcare system, and the importance of providing a path for increasing access to therapeutics. This provides a strong case for providing health equity principles in fostering innovation and collaboration in Europe to help ease the substantial challenges and cost burden experienced by rare disease patients, caregivers, and their families.

Comprehensive plans and meaningful societal support should be provided to patients with rare diseases and their families. We encourage further exploration and hope that the next European Partnership for Rare Diseases, financed by the EU and its member states, will focus on the acute gap in research and treatments further, and the totality of its impact.

Key findings from this report include:

- **The cost burden on rare disease families is significant**
The report found that rare diseases incur a significantly higher average cost burden of €107,000 per patient per year (PPPY)⁴, approximately 13 times greater than the benchmark for the average burden of high-prevalence diseases at an average of

¹ Data on file.

² European Commission. Rare diseases. Available from: https://health.ec.europa.eu/non-communicable-diseases/expert-group-public-health/rare-diseases_en (Accessed 04 October 2023).

³ Global Genes: Rare disease facts. Available from: <https://globalgenes.org/rare-disease-facts/> (Accessed 04 October 2023).

⁴ Data on file.

€7,000 per patient per year⁵. In addition, data showed that indirect costs average 29% of the total burden of the rare diseases when treatment is available, rising to an average of 45% when no treatment is available⁴. Significantly, most of these indirect costs (e.g., caregiver burden, home changes, cost of secondary treatments, traveling and accommodation) are borne by families.

- **The availability of treatments creates positive value and alleviates financial strain on families and the healthcare system**

In the absence of treatment options, the report found that the burden would increase by 28% per rare disease patient per year across the 23 diseases studied.

"This report seeks to spotlight the tangible challenges experienced by rare disease patients and their caregivers in real life. We hope it opens the door to a more granular way to think of rare diseases that takes into account positive and negative societal externalities of rare diseases and how to account for them in future policies and societal commitments," said Enrico Piccinini, Head of Europe & Emerging Markets Rare Diseases at Chiesi. *"By launching this report at the World Orphan Drug Congress, we intend to kickstart a constructive discussion on how to effectively address and reduce this burden."*

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About Chiesi Global Rare Diseases

Chiesi Global Rare Diseases is a business unit of the Chiesi Group established in February 2020 and focused on research and development of treatments for rare and ultra-rare disorders. The Global Rare Diseases unit works in collaboration with Chiesi Group to harness the full resources and capabilities of our global network to bring innovative new treatment options to people living with rare diseases, many of whom have limited or no treatments available. The unit is also a dedicated partner with global leaders in patient advocacy, research, and patient care.

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.

About the report:

Chiesi Global Rare Diseases, with support from IQVIA, set out to quantify the societal impact relative to the significant unmet needs of rare disease patients, in Europe. This paper reports on the direct, indirect, and mortality-related costs for 23 rare diseases across five therapeutic areas in three EU Member States. Understanding the landscape for rare disease innovation in the European Union, along with cost drivers and

⁵ Chronic diseases, who will pay the bill? Available from: <https://meersens.com/chronic-diseases-who-will-pay-the-bill/?lang=en#BLOC2> (Accessed 04 October 2023).

economic impact stemming from treatment or lack thereof, is of critical importance particularly because citizens impacted by rare diseases face significant challenges, especially in managing the cost of care.

About the event:

The panel hosted by Chiesi, *'Reducing the burden of rare diseases: how data can inspire innovations and inform policies in the European Union'*, brings together experts from across the rare disease sector to discuss how the findings and data from the Pendulum Paper can be utilized to reimagine how we treat and address patients living with rare diseases. This panel is a pre-session event kicking-off the World Orphan Drug Congress focused on addressing the various treatments, experiences and understanding we share about rare diseases globally.

Panelists include Enrico Piccinini, Head of Europe & Emerging Markets Rare Diseases, Chiesi; Prof. Maurizio Scarpa, European Reference Network (ERN); Dimitrios Athanasiou, EURORDIS; Pedro Andreu, Principal, IQVIA; Michele Pistollato, Principal, Charles River Associates (CRA); Pierantonio Russo, Corporate Chief Medical Officer, Eversana; Petra Wilson, Senior Advisor, FTI Consulting (moderator)

The World Orphan Drug Congress is an award-winning event with an exhibition that has grown to become the largest and most established orphan drugs & rare diseases meeting of its kind across the globe.

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