PRESS RELEASE

Procysbi® (gastro-resistant mercaptamine) recommended for use in NHS Scotland for the treatment of nephropathic cystinosis[1]

- Procysbi® *(gastro-resistant mercaptamine)* has been accepted for use within NHS Scotland for the treatment of proven nephropathic cystinosis, providing a new treatment option for this ultra-rare condition.[1]
- Nephropathic cystinosis is typically diagnosed in infants and children, affecting around 1 in 100,000 – 200,000 people globally.[2,3]
- While *gastro-resistant mercaptamine* is now available to eligible patients in Scotland and Wales – the treatment remains unavailable in England via the NHS.

Manchester, UK – 10 November 2025 – Chiesi UK and Ireland today welcome the Scottish Medicines Consortium's (SMC) decision to recommend *gastro-resistant mercaptamine* (a cystine-depleting agent) for use within NHS Scotland for the treatment of people living with nephropathic cystinosis.[1] This recommendation follows a detailed appraisal process, where clinicians and patient organisations highlighted the significant burden of currently available treatment and the potential for *gastro-resistant mercaptamine* to address significant unmet needs in the day-to-day management of the condition.

Care for people with nephropathic cystinosis is often complex, time-consuming and demanding for both patients and their families. Until now, treatment options have been limited, requiring multiple daily doses. This can be particularly challenging for children and teenagers, disrupting sleep, schooling and social life, placing additional strain on families and caregivers.

Ben Reynolds, Consultant Paediatric Nephrologist, Royal Hospital for Children, Glasgow, said: *"The availability of mercaptamine bitartrate in Scotland represents an important step forward in expanding treatment choice for people living with nephropathic cystinosis. It gives clinicians greater flexibility to individualise care, while decisions should continue to be guided by each patient's clinical profile and circumstance."*

Nephropathic cystinosis is a rare, progressive, life-limiting condition that is usually diagnosed in infants and children, affecting around 1 in 100,000 – 200,000 people globally.[1,3] The disease is caused by the accumulation of cystine – an amino acid – within cells, leading to tissue and organ damage, particularly in the kidneys.[4]

David Garzón, Senior Director, Rare Diseases at Chiesi UK and Ireland, added: *"We welcome this positive development for patients in Scotland, however we recognise that work must continue to ensure equitable access across the UK. Delayed-release mercaptamine bitartrate has been available to patients in Wales since 2021 and in Ireland for several years, yet in England the process remains stalled. As a result, England remains the only nation in the UK where eligible patients cannot routinely*

access this medicine, highlighting a growing postcode lottery in care for people living with cystinosis."

Gastro-resistant mercaptamine has been approved across the UK and Europe including availability in France, Italy, Germany and Ireland. It has been available via the NHS in Wales since 2021. The treatment remains unavailable in England, where routine access is still pending. Funding for NHS use in Scotland is expected within 60 days of publication of SMC advice.

Comments from the Patient Community

Alex Hutchison, Scottish Trustee of Cystinosis Foundation UK, said: *"This approval marks a huge moment for people living with cystinosis in Scotland. Having the choice to move from a gruelling six-hourly medication schedule to a more manageable twelve-hourly routine will have a significant impact on quality of life, as well as potentially bringing about clinical improvements for patients. For families, young people, and adults navigating the relentless demands of this rare condition, this decision is an important step forward. We're proud to have worked both with and for the cystinosis community in advocating for this change."*

Laura Smith Van Carroll, Head of Insight & Advocacy at Metabolic Support UK, said: *"After many years of hard work and perseverance, we are thrilled to see a positive reimbursement decision for gastro-resistant mercaptamine in Scotland. This means so much to the cystinosis community, who have waited far too long for fair access to a treatment that can truly make a difference to daily life. Cystinosis may be rare, but its impact is immense - the current six-hourly treatment disrupts sleep, family life and wellbeing every single day. Gastro-resistant mercaptamine offers the chance for people living with cystinosis, and their carers, to reclaim normality and rest, without compromising on treatment. We are incredibly proud of the community's voice, through our surveys and advocacy, which helped bring this evidence to light and made sure their experiences were heard."*

"This is positive news for people living with this rare disease and their families." explained Alison Railton, Director of Policy and Public Affairs at Kidney Research UK. *"Having access to this treatment could make a meaningful difference, particularly for children and their parents. The twice-daily dosing means families no longer need to wake during the night, supporting a more consistent night's sleep. We believe everyone living with cystinosis should have access to the most effective treatments available to prevent them from developing kidney disease and eventually needing dialysis or a kidney transplant."*

About Procysbi® (gastro-resistant mercaptamine)

Procysbi (*gastro-resistant mercaptamine)* is a beaded, enteric-coated, delayed-release form of cysteamine, in which the microspheronised beads are further encapsulated in hard gelatin to allow oral administration every 12 hours.[5] The treatment is administered twice-daily, aiming to reduce treatment burden for patients and carers.[5]

Gastro-resistant mercaptamine is available in either 25 mg or 75 mg gastro-resistant hard capsules of cysteamine.[6] Therapy should be initiated as early as possible following diagnosis and continued throughout life.[7]

About Chiesi Group

Chiesi is a research-oriented international biopharmaceutical 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, Chiesi is 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), with 31 affiliates worldwide, and counts more than 7,000 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.uk.com.

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