PROCYSBI® [mercaptamine bitartrate (cysteamine) gastro-resistant hard capsules] recommended for routine commissioning by NHS England for eligible patients with nephropathic cystinosis

- NHS England has recommended mer*captamine bitartrate (cysteamine) gastro-resistant hard capsules*, also referred to as *"delayed-release mercaptamine bitartrate"* for routine commissioning for eligible patients aged one year and above with nephropathic cystinosis.[1]
- Nephropathic cystinosis affects around 1 in 100,000 to 200,000 live births, with approximately 200 people living with the condition in the UK and two to three new cases diagnosed each year.[1,2]
- The treatment provides an additional cystine-depleting therapy option that can be taken twice-daily, compared to four-times-daily dosing every six hours for the currently commissioned immediate-release formulation.[1]

Manchester, UK – 5th August 2026 – Chiesi UK and Ireland welcome NHS England's decision to recommend *delayed-release mercaptamine bitartrate* (a cystine-depleting agent) for routine commissioning for eligible patients living with nephropathic cystinosis. The decision means eligible patients aged one year and above who meet the defined clinical criteria* can access an additional treatment option through NHS England specialised services, expanding choice for people living with this ultra-rare condition.

Care for people with nephropathic cystinosis is often complex, time-consuming and demanding for both patients and their families.[2,3] With treatment options previously limited, requiring multiple daily doses, this treatment option with a twice-daily dosing schedule, can potentially help reduce disruption to daily routines for patients and carers, improve adherence, delay complications and improve patients' quality of life.[1]

"We welcome NHS England's decision to routinely commission delayed-release mercaptamine bitartrate for eligible people with nephropathic cystinosis," said David Game, Consultant Nephrologist and Clinical Lead for the Delayed-Release Mercaptamine Policy Proposal. *"Nephropathic cystinosis is a lifelong condition, and this decision marks an important milestone for the cystinosis community in England. By providing eligible patients and their clinicians with access to an additional treatment option, it supports treatment decisions based on individual patient needs and reflects the collaborative efforts of clinicians, patients, families and patient organisations throughout the policy process."*

Nephropathic cystinosis is an ultra-rare, progressive condition affecting around 200 people in the UK.[1] The disease is caused by the accumulation of cystine, an amino acid, within cells throughout the body.[1] This can lead to tissue and organ damage,

particularly in the eyes and kidneys.[1,4] Around 150 patients are estimated to be eligible for *delayed-release mercaptamine bitartrate* under NHS England's new commissioning policy.[1]

David Garzón, Senior Director, Rare Diseases at Chiesi UK and Ireland, added: *"This is an important milestone for the nephropathic cystinosis community in England, providing an additional treatment option for these patients with a twice-daily dosing regimen, that can help alleviate some of the treatment burden for patients and their families. Following earlier reimbursement decisions in Northern Ireland, Wales and Scotland, this decision marks an important step towards more equitable access across the UK, reflecting the collaborative efforts of clinicians, patient organisations, patients and families."*

Delayed-release mercaptamine bitartrate is approved across the UK and Europe, including in France, Italy, Germany and Ireland. Following NHS England's decision to routinely commission the treatment for eligible patients 1 year and above with nephropathic cystinosis, the medicine is now routinely available across all UK nations for patients meeting local access criteria.

Comments from the Patient Community

Will Newman, Chairperson of Cystinosis Foundation UK, said: *"We are delighted to see this decision, which represents an important step forward for the nephropathic cystinosis community in England. The approval means patients and carers can now explore whether this formulation better suits their individual circumstances, lifestyles, and tolerability needs."*

Laura Smith Van Carroll, Head of Insight & Advocacy at Metabolic Support UK, said: *"We are delighted that people living with cystinosis in England will now have access to Procysbi, bringing equitable treatment choice across the UK after many years of availability in Wales, followed by Scotland and more recently Northern Ireland. For many families, this approval has the potential to reduce the burden of frequent dosing, allowing uninterrupted sleep, and giving people greater choice in how they manage this lifelong condition. While we welcome this decision, we hope future CPAG processes will provide clearer timelines and secure funding alongside approval, helping people with rare conditions access new treatments more quickly and with greater certainty."*

About Procysbi® (*delayed-release mercaptamine bitartrate*)

Procysbi (*delayed-release mercaptamine bitartrate)* 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.[4] The treatment is administered twice-daily.[5]

Delayed-release mercaptamine bitartrate is available in either 25 mg or 75 mg gastro-resistant hard capsules of cysteamine.[5] Therapy should be initiated as early as possible following diagnosis and continued throughout life.[6]

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, France and Colombia, Chiesi's commitment to creating 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 verified standards for social and environmental business practices. The company aims to reach Net-Zero greenhouse gases (GHG) emissions by 2035.

With 90 years of experience, Chiesi is headquartered in Parma (Italy), with 31 affiliates worldwide, and counts more than 7,900 employees. The Group's research and development centre in Parma works alongside six 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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* Inclusion criteria can be found in the guidance here: Report template - NHSI website.

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