People and ideas for innovation in healthcare

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PRESS RELEASE

Chiesi's Procysbi® (mercaptamine bitartrate gastro-resistant hard capsules) is approved for use in Ireland for patients with nephropathic cystinosis

Manchester, UK, 1 July 2019 – Chiesi Limited has announced today that Procysbi (mercaptamine bitartrate gastro-resistant hard capsules) has been approved for use, in patients with proven nephropathic cystinosis, by the Health Service Executive (HSE) for reimbursement in Ireland.[1]

Nephropathic cystinosis is an ultra-rare, irreversible, progressive, life-long disorder,[2,3,4,5,6] leading to multiple complications throughout a patient's lifetime and presenting a heavy burden,[5,7] causing a reduced quality of life and emotional strain on them and their families.[8] It also negatively impacts on the patient's ability to perform everyday functions due to numerous disease-related health and social issues.[8] It can lead to end-stage renal disease and many non-renal complications such as crystal deposition in the conjunctiva.[9] Seldom do patients reach their late 30s without a major, life-altering medical complication caused by the condition.[7] In Ireland, it is estimated that up to 20 patients are affected.[10]

Early and continuous treatment with a cystine-depletion therapy (CDT) represents the mainstay of therapy in patients with nephropathic cystinosis,[11,12,13] and whilst a kidney transplant treats end-stage renal failure, CDT must still be taken for life.[14,15] Procysbi is a delayed-release formulation of mercaptamine, a CDT, with microgranule technology allowing for drug delivery to the small intestine, extended release and 12-hour dosing, which reduces interruptions for the patient during the day and night (compared to the 6-hourly dosing with immediate-release (IR) mercaptamine formulation).[16,17,18] The HSE's decision to reimburse Procysbi in Ireland follows a dialogue with Chiesi, and is part of the company's efforts to expand patient access to an impactful treatment for an ultra-rare condition of high unmet need.

Tom Delahoyde, Managing Director of Chiesi UK and Ireland said, "*Today's HSE decision is a positive step forward for patients with this debilitating rare disease in Ireland. This is an encouraging example of how industry, healthcare regulators and patient organisations can collaborate for the benefit of the patient. Chiesi is committed to improving the quality of life for these patients and their families and we will continue to work with the HSE to make this medication available to the patients who need it.*"

For the Summary of Product Characteristics for Procysbi, please visit www.medicines.org.uk/emc/product/2079/smpc.

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Notes to Editors

About cystinosis
Cystinosis is a life-long, multi-systemic, lysosomal storage disorder that causes progressive damage throughout the body.[2,4,5,9] It is a progressive dysfunction of multiple organs caused by the accumulation of cystine protein in the tissues, leading, for example, to end-stage renal failure, diabetes, hypothyroidism, myopathy, and central nervous system deterioration.[2] Without optimal management, cystinosis reduces quality of life and shortens life expectancy.[4] Approximately one in every 240,000 people in Ireland has cystinosis (up to 20 patients).[10,19]

Until now, the only available treatment in Ireland has been immediate release (IR)- mercaptamine, the most challenging impact of which was a six-hourly dosing schedule around the clock. Patients must wake in the middle of the night to be fully compliant with IR-mercaptamine;[16] this causes disrupted sleep which affects their quality of life and reduces compliance with therapy.[2,14] Non-adherence to IR- mercaptamine is also very common, especially in adolescents and adults.[4]

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About Procysbi (mercaptamine bitartrate, also known as cysteamine)

Procysbi is a delayed-release formulation of cysteamine with microgranule technology allowing for drug delivery to the small intestine.[16] The delayed-release preparation of cysteamine is based on a proprietary technology that delivers cysteamine bitartrate in individually enteric-coated, pH-sensitive microgranules, all encased in a hard gelatin capsule.[16,18,20] Procysbi is available in either 25 mg or 75 mg gastro-resistant hard capsules of cysteamine.[20] Therapy should be initiated as early as possible following diagnosis and continued throughout life.[12]

In a pivotal Phase III trial,[16,20] Procysbi showed non-inferiority to IR- mercaptamine in the per protocol analysis. Per protocol analysis included 38 subjects, following exclusion of three patients who had a three-day average white blood cell (WBC) level > 2 nmol hemi-cystine/mg protein during one of the IR- mercaptamine treatment periods and were, therefore, considered not well controlled. A total of 119 adverse events (AEs) and serious AEs were reported, of which 45% were gastrointestinal (GI). Of these, 70% were considered to be related to the drug. More GI AEs were related to Procysbi than to IR- mercaptamine (44% vs 15% for Procysbi and IR-mercaptamine, respectively). This may be associated with proton pump inhibitors (PPIs) being stopped abruptly for patients during the Procysbi treatment (72% of the patients using PPIs during IR- mercaptamine period stopped them when starting Procysbi) – these participants were previously taking a stable dose of IR-mercaptamine. No unexpected serious AEs associated with Procysbi or IR- mercaptamine were seen during the study.

About Chiesi Limited

Chiesi Limited is the UK and Ireland affiliate of Chiesi Farmaceutici S.p.A. It is headquartered in Manchester and employs over 250 employees. Chiesi Farmaceutici is an international research-focussed Healthcare Group based in Parma, Italy, and present in 26 countries. Chiesi researches, develops and markets innovative drugs in the respiratory, specialist medicine and rare disease areas. For more information visit www.chiesi.uk.com.

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Media contacts:

Donna Caslin
Senior PR and Communications Manager
Tel: +44 161 488 5555
Email: d.caslin@chiesi.com

Jess Wilkinson
M&F Health Communications
Tel: +44 20 7492 1778
Email: jessica.wilkinson@mandfhealth.com

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