PRESS RELEASE

NICE Recommends Raxone® (idebenone) in Leber Hereditary Optic Neuropathy

- The National Institute for Health and Care Excellence (NICE) has issued Final Draft Guidance (FDG) recommending idebenone within its marketing authorisation as an option for treating visual impairment in Leber Hereditary Optic Neuropathy (LHON) in people aged 12 years and over[1]
- LHON is a rare condition affecting approximately 1 in 31,000 people in England, causing rapid vision loss in both eyes and permanent blindness[2,3]
- According to NICE documentation, up to 471 people may be eligible for treatment with idebenone following publication of the final NICE guidance[2]

Manchester, UK – 7th August 2025 – Chiesi UK and Ireland today welcome a decision by the National Institute for Health and Care Excellence (NICE) to recommend *idebenone* within its marketing authorisation as an option for treating visual impairment in Leber Hereditary Optic Neuropathy (LHON) in people aged 12 years and over.[1]

"LHON causes devastating visual loss and it is a life-changing diagnosis for the affected individual and their family. England is now in line with the rest of the United Kingdom with idebenone now available through the NHS. This will come as a great relief to the LHON community in this country bringing hope to those who have experienced significant visual loss from this mitochondrial genetic disorder," said Professor Patrick Yu-Wai-Man, PhD, FRCPath, FRCOphth, Professor of Ophthalmology and Honorary Consultant Neuro-ophthalmologist at the University of Cambridge, Moorfields Eye Hospital, and the UCL Institute of Ophthalmology, United Kingdom.

LHON affects approximately 1 in 31,000 individuals in England (with approximately 20-25 new cases per year) - for those affected, vision loss often occurs suddenly and without warning.[2,3,4]

This decision from NICE makes *idebenone* the first and only licensed treatment available for vision impairment in adolescents and adults with LHON through the NHS, bringing England in line with Scotland, where *idebenone* has been available since May 2017, and with Wales and Northern Ireland, where access was established in 2021.[1]

Katie Waller, Head of Patient Programmes at The Lily Foundation and Registered Paediatric Nurse said: "*This is fantastic news for our community, and we're proud to have been a key stakeholder throughout the NICE submission process. While there still isn't a cure for LHON, making therapies available that may lead to vision improvement gives hope. We need options in LHON that can give people the chance to regain independence, confidence and a better quality of life.*"

"LHON is a truly devastating condition. Its impacts extend far beyond vision loss to the overall quality of life for patients. The effects of LHON reach deeply into the lives of patients, their families, caregivers, as well as individuals who carry the LHON genes. Patients often experience loss of independence, with every day tasks often becoming exceptionally challenging or even unmanageable. As a result patients

must rely on assistive technologies and support from caregivers and social services. LHON can also severely affect mental health, overall well-being and it creates a cascade of challenges related to education, employment, stigma and social participation. The LHON Society is delighted that idebenone has been recommended for use - this is a critical step towards full access to idebenone for patients, that may alleviate some of the impacts of LHON", added a LHON Society spokesperson.

Although vision loss can occur at any age, LHON predominantly affects young men between the ages of 15 and 35, with men carrying the predisposing genetic mutation and being 4-5 times more likely to be affected.[5] The condition poses a significant burden on quality of life, and in approximately 25% of cases, the onset of vision loss occurs in both eyes within a very short timeframe, leading to lifelong disability.[6]

David Garzón, Senior Director, Rare Diseases at Chiesi UK and Ireland said*: "We are proud that NICE has recognised the significant unmet need for people living with this rare and debilitating disease. Our heartfelt thanks go to the clinicians, patients and organisations who stood with us in advocating for the LHON community throughout this appraisal process. It has been a long journey - it is very challenging to assess rare disease therapies via the NICE standard technology appraisal route and, while we understand the uncertainties raised within the process, we must collectively acknowledge that this is the reality of rare diseases and find a way to bring innovation to patients faster."*

To find out more about LHON, please visit this Chiesi website: lhonaware.co.uk.

About Leber Hereditary Optic Neuropathy (LHON)

Leber Hereditary Optic Neuropathy (LHON) is a rare, inherited mitochondrial disorder that leads to sudden and severe loss of central vision. It typically affects young adults, particularly males, and is caused by mutations in mitochondrial DNA that impair the function of retinal ganglion cells.[3]

Vision loss usually begins in one eye and progresses to the other within weeks or months.[3] In 25% of the cases, vision loss occurs simultaneously in both eyes within a very short timeframe.[6] LHON is a rare condition affecting approximately 1 in 31,000 people in England.[2] Beyond vision impairment, the condition often has a profound impact on independence, education, employment and quality of life.

About Raxone® (idebenone)

Idebenone is the first and only licensed treatment for visual impairment in adolescents and adults with Leber Hereditary Optic Neuropathy (LHON).[1] Prior to the availability of idebenone in England, treatment options for people with LHON have been limited to best supportive care (BSC).[7]

In clinical and real-world studies, idebenone, which is taken orally, was shown to be generally well tolerated. The most common adverse reactions reported in clinical studies were mild to moderate diarrhoea (usually not requiring the discontinuation of treatment), nasopharyngitis, cough and back pain.[8]

Patient responses to treatment with idebenone vary but it can have a marked improvement in their visual acuity. Improvements in visual acuity can mean increased independence for patients with LHON.[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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