Chiesi Global Rare Diseases Announces European Commission Approval of LOJUXTA® (lomitapide) ▼ Capsules for Paediatric Use in Homozygous Familial Hypercholesterolaemia (HoFH)

European Commission approval expands the indication of lomitapide in the European Union (EU) to include children 5 years of age and older with HoFH, an ultra-rare genetic disorder affecting LDL-cholesterol levels.

PARMA, Italy – June 5, 2026 – Chiesi Global Rare Diseases, a business unit of the Chiesi Group established to deliver innovative therapies and solutions for people living with rare diseases, today announced that the European Commission (EC) has approved lomitapide capsules for use in children 5 years of age and older with Homozygous Familial Hypercholesterolaemia (HoFH), for use alongside diet and other lipid-lowering treatments, including LDL-apheresis where available.

The EC decision follows the positive opinion from the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) recommending the expanded indication. Lomitapide has been approved for use in the European Union (EU) for adult patients with HoFH since 2013.

"This European Commission approval marks an important milestone for children living with HoFH and their families," said Mitch Goldman, MD, PhD, SVP Research & Development, Chiesi Global Rare Diseases. "HoFH is a condition that can begin causing irreversible cardiovascular damage from the earliest years of life, and the unmet need remains for younger patients despite existing treatment options. By expanding the indication of lomitapide to children aged 5 years and older, we are offering families and their physicians a meaningful treatment option that is backed by robust clinical evidence. This approval reflects our deepest commitment: ensuring that having a rare disease never means being overlooked at any stage of life, across generations."

The EC approval is based on evidence from a Phase 3, open-label, single-arm, multicentre study evaluating lomitapide in 43 pediatric participants aged 5 to 17 years with HoFH. The APH-19 study achieved its primary endpoint, demonstrating a mean 53.5% reduction in LDL-C from baseline at week 24 ($p<0.0001$). Significant reductions (all $p<0.0001$) were also achieved in non-HDL-C, total cholesterol, VLDL-C, apolipoprotein B, and triglycerides at week 24 (secondary

outcomes). Adverse events were mostly mild, and gastrointestinal and hepatic in nature. Adverse events of special interest were reported for five (12%) patients (gastrointestinal in two patients and hepatic in three). One serious treatment-emergent adverse event was reported (also classed as an adverse event of special interest): an increase in hepatic enzymes, resulting in two dose interruptions, two dose reductions, and a repeated dose escalation. Overall, no new adverse event signals were identified, and the results were consistent with the known profile of lomitapide.[1]

About HoFH

Homozygous familial hypercholesterolaemia (HoFH) is a rare condition causing very high levels of cholesterol in the body since birth.[2] In HoFH, the arteries become clogged, with people affected often having more than 10 times the target, or acceptable level of so called bad cholesterol in their blood (LDL-C), leading to severe heart disease, premature atherosclerosis and cardiovascular events.[2] Early diagnosis and management in HoFH are critical, however, many patients remain undiagnosed and undertreated.[2,3]

About lomitapide

Lomitapide is a prescription-only medicine used along with a low-fat diet, exercise and other low-density lipoprotein (LDL) lowering medicines to reduce LDL-C in adults and children 5 years of age and older with a type of high cholesterol called homozygous familial hypercholesterolemia (HoFH).[4] Genetic confirmation of HoFH should be obtained whenever possible. Other forms of primary hyperlipoproteinemia and secondary causes of hypercholesterolaemia (e.g., nephrotic syndrome, hypothyroidism) must be excluded.

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

high standards of social and environmental impact. 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 7,500 employees. The Group's research and development center in Parma works alongside 6 other important R&D hubs in France, the US, Canada, China, the UK, and Sweden.

About Chiesi Global Rare Diseases

Chiesi Global Rare Diseases is a business unit of the Chiesi Group established to deliver innovative therapies and solutions for people living with rare diseases. As a family business, Chiesi Group strives to create a world where it is common to have therapy for all diseases and acts as a force for good, for society and the planet. The goal of the Global Rare Diseases unit is to ensure equal access so as many people as possible can experience their most fulfilling life. The unit collaborates with the rare disease community around the globe to bring voice to underserved people in the health care system.

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