

# Chiesi Global Rare Diseases receives positive CHMP opinion for LOJUXTA® (lomitapide) ? Capsules for pediatric use in Homozygous Familial Hypercholesterolemia (HoFH)

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*Committee for Medicinal Products for Human Use (CHMP) issued a positive opinion, with European Commission (EC) decision anticipated by June 1, 2026*

*If approved, this would expand the indication of lomitapide to include children 5 years of age and older with HoFH*

PARMA, Italy – March 30, 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 Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has issued a positive opinion recommending the approval of lomitapide capsules for pediatric use in homozygous familial hypercholesterolemia (HoFH) for children 5 years of age and older. Lomitapide has been approved in the European Union (EU) for adult patients with HoFH for use alongside diet and other lipid-lowering treatments, including LDL-apheresis, since 2013.

In alignment with Chiesi's commitment to supporting underserved patient communities globally, lomitapide is reimbursed in 16 countries and was approved by the Saudi Food and Drug Authority (SFDA) for pediatric use in HoFH in ages 5 years and above. Lomitapide is also available in other countries through named patient programs.

HoFH, an ultra-rare genetic disorder, impacts an estimated 1 in 250,000 to 1 in 360,000 individuals worldwide, including children who are born with extremely elevated levels of low-density lipoprotein cholesterol (LDL-C).<sup>1,2</sup> Without early intervention, those living with this condition face a significantly increased risk of severe atherosclerosis and premature death.<sup>3,4</sup>

"Children living with HoFH deserve treatment options that meet their needs from the very start," said **Mitch Goldman, MD, PhD, SVP Research & Development, Chiesi Global Rare Diseases**. "The CHMP's positive opinion brings us one step closer to offering lomitapide to those 5 years old and over. By expanding access to a therapy that has already benefited adult patients, we aim to help families navigate this condition with confidence and continuity of care."

The positive opinion 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.<sup>5</sup>

### **About HoFH**

Homozygous familial hypercholesterolemia (HoFH) is a serious, rare genetic disease that impairs the function of the receptor responsible for removing low-density lipoprotein cholesterol (LDL-C) ("bad" cholesterol) from the body. A loss of low-density lipoprotein (LDL) receptor function results in extreme elevation of blood cholesterol levels. Individuals with HoFH often develop premature and progressive atherosclerosis, a narrowing or blocking of the arteries.

### **About lomitapide**

Lomitapide is a prescription-only medicine and is indicated as an adjunct to a low-fat diet and other lipid-lowering medicinal products with or without low density lipoprotein (LDL) apheresis in adult patients with homozygous familial hypercholesterolaemia (HoFH). 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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