INTENDED FOR USE WITH MEDICAL MEDIA IN THE UK ONLY

NHS England Rolls Out Evkeeza® ▼(evinacumab) for Eligible Adults and Adolescents Aged 12 Years and Older with Homozygous Familial Hypercholesterolaemia (HoFH)

London, 20 December 2024 — Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialisation of novel therapies for rare and ultrarare genetic diseases, today announced that NHS England has implemented the commissioning of Evkeeza (evinacumab) following the National Institute for Health and Care Excellence (NICE) final guidance in September. The use of Evkeeza in eligible people aged 12 years and older will be routinely commissioned by NHS England in line with the NICE TA and will be available in seven hospital trusts in England. In addition, prior approval forms are in place to enable access for children aged 5 to 11 years, via the NHS England Commissioning Medicines for Children policy.

Dr. Jaimini Cegla, clinical lead of the Lipid and Cardiovascular Risk Service, Hammersmith Hospital said, "We are very pleased that NHS England has endorsed and implemented NICE recommendations for the use of evinacumab within the NHS. Our patients with HoFH often have early onset heart disease, in many cases in their teens, and many of the usual medicines we use to treat cholesterol have limited effectiveness in this condition. Evinacumab, which is effective at lowering LDL-C in HoFH when combined with other lipid-lowering therapies, is a much needed and very welcome addition to help us treat eligible patients as best we can."

Eligible people living with HoFH in England, Wales and Northern Ireland will now have access to Evkeeza, the first approved and commercialised monoclonal antibody inhibiting the angiopoietin-like 3 protein (ANGPTL3).[1] The seven commissioning providers in England include Bristol Teaching Hospital NHS Trust, Guy's & St Thomas' NHS Foundation Trust, Imperial College Healthcare NHS Trust, Manchester University NHS Foundation Trust, Newcastle University Foundation Trust, Sheffield Teaching Hospitals and University Hospital Birmingham.

"NICE's recognition of the clinical and economic benefits of Evkeeza including that it is a 'cost-effective use of NHS resources' paved the way for the NHS decision," said David Nestor, Vice President and General Manager for the UK, Ireland and Nordics at Ultragenyx. "We are grateful to the physician and patient communities for their support as we worked to secure access for patients living with HoFH in England, Wales and Northern Ireland."

"HEART UK is absolutely delighted that patients living with HoFH now have access to this treatment," stated Jules Payne, Chief Executive Officer of HEART UK.

About Homozygous Familial Hypercholesterolemia (HoFH)
HoFH is a devastating form of inherited hypercholesterolaemia, affecting 1 in 300,000 people globally and approximately 1,600 people in the European Union. HoFH occurs when two copies of the familial hypercholesterolaemia (FH)-causing genes are inherited, one from each parent, resulting in dangerously high levels (>400 mg/dL/>10 mmol/L) of LDL-C, or bad cholesterol. Patients with HoFH are at risk for premature atherosclerotic disease and cardiac events at an early age.[2]

About Evkeeza (evinacumab)
Evkeeza is approved by the UK MHRA as an adjunct to diet and other low-density lipoprotein-cholesterol (LDL-C) lowering therapies for the treatment of adult and paediatric patients aged 5 years and older with homozygous familial hypercholesterolaemia (HoFH).

Evinacumab, the active substance in Evkeeza, binds to a protein in the body called ANGPTL3 and blocks its effects. ANGPTL3 is involved in controlling cholesterol levels and blocking its effect reduces the level of cholesterol in the blood. Evkeeza is administered as an intravenous infusion.

Regeneron Pharmaceuticals, Inc. discovered and developed Evkeeza and commercialises the product in HoFH in the U.S. under the generic name evinacumab-dgnb, with dgnb as the suffix designated in accordance with Nonproprietary Naming of Biological Products Guidance for Industry issued by the FDA. Ultragenyx is responsible for commercialization efforts for Evkeeza in HoFH in countries outside of the U.S.

IMPORTANT SAFETY INFORMATION FOR EVKEEZA (evinacumab)
The most common side effects (>10%) include symptoms of the common cold, such as runny nose (nasopharyngitis) and for children aged 5 to 11 years feeling tired (fatigue). Evkeeza can cause serious allergic reactions.

Please see full Product Information, including Summary of Product Characteristics and Package Leaflet: Information for the patient.

About Ultragenyx Pharmaceutical Inc.
Ultragenyx is a biopharmaceutical company committed to bringing novel products to patients for the treatment of serious rare and ultrarare genetic diseases. The company has built a diverse portfolio of approved therapies and product candidates aimed at addressing diseases

with high unmet medical need and clear biology for treatment, for which there are typically no approved therapies treating the underlying disease.

For more information on Ultragenyx, please visit https://www.ultragenyx.eu/uk/.

References

1. Cuchel M *et al*. Eur. Heart J. 2023: 44 :2277 2291, https://doi.org/10.1093/eurheartj/ehad197
2. Cuchel M *et al*. Eur. Heart J. 2014: 35: 2146-2157, https://doi.org/10.1093/eurheartj/ehu274

Ultragenyx Forward-Looking Statements and Use of Digital Media

Except for the historical information contained herein, the matters set forth in this press release, including statements related to Ultragenyx's expectations and projections regarding its future operating results and financial performance, business plans and objectives, including its expectations regarding the market opportunities for Evkeeza are forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, collaboration with third parties, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainty of clinical drug development and unpredictability and lengthy process for obtaining regulatory approvals, risks related to adverse side effects, risks related to reliance on third party partners to conduct certain activities on the company's behalf, the potential for any license or collaboration agreement, including the company's collaboration agreement with Regeneron to be terminated, smaller than anticipated market opportunities for the company's products and product candidates, manufacturing risks, competition from other therapies or products, market acceptance of the company's products, risks related to international expansion of the company's business, uncertainties related to insurance coverage and reimbursement status of newly approved products, and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations, the company's future operating results and financial performance and the availability or commercial potential of Ultragenyx's products and drug candidates. Ultragenyx undertakes no obligation to update or revise any forward-looking statements.

For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of Ultragenyx in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with

the Securities and Exchange Commission (SEC) on November 6, 2024, and its subsequent periodic reports filed with the SEC.

In addition to its SEC filings, press releases and public conference calls, Ultragenyx uses its investor relations website and social media outlets to publish important information about the company, including information that may be deemed material to investors, and to comply with its disclosure obligations under Regulation FD. Financial and other information about Ultragenyx is routinely posted and is accessible on Ultragenyx's Investor Relations website (https://ir.ultragenyx.com/) and LinkedIn website (https://www.linkedin.com/company/ultragenyx-pharmaceutical-inc-/).

Contacts

Investors
Joshua Higa
+1-415-475-6370
ir@ultragenyx.com

Media
Joey Fleury
+1-925-784-5829
media@ultragenyx.com