Ultragenyx Receives Positive Recommendation from NICE in the U.K. for Evkeeza® ▼ (evinacumab) for Adults and Adolescents Aged 12 Years and Older with Homozygous Familial Hypercholesterolemia (HoFH)

January 4, 2024

NOVATO, Calif., January 04, 2024 -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE) today announced that the National Institute for Health and Care Excellence (NICE) has issued a final draft guidance recommending Evkeeza® (evinacumab) to NHS England. Evkeeza is recommended within its marketing authorization as an adjunct to diet and other low-density lipoprotein-cholesterol (LDL-C) lowering therapies for the treatment of adults and adolescent patients aged 12 years and older with homozygous familial hypercholesterolemia (HoFH). Evkeeza is the first angiopoietin-like 3 (ANGPTL3) inhibitor treatment indicated for this rare and debilitating condition.

"We are very pleased that NICE has recommended 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," stated Dr. Jaimini Cegla, clinical lead of the Lipid and Cardiovascular Risk Service, Hammersmith Hospital. "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."

NICE deemed that, despite uncertainties in the cost-effectiveness evidence comparing evinacumab with lomitapide in adults with HoFH, overall there are cost savings with evinacumab compared with lomitapide. The NICE committee acknowledged that clinical trial evidence shows that Evkeeza, combined with other lipid-lowering therapies (LLTs), can lower LDL-C levels when statins and other LLTs have not reduced them enough.

NICE also acknowledged that adolescent patients with HoFH on LLTs with or without lipoprotein apheresis have a high unmet need because LLTs have limited effectiveness in this patient population, lipoprotein apheresis can be traumatic and time consuming, and there is potential for an inequality of access if made available to adults and not adolescents. NICE considered that Evkeeza addresses an unmet need for new treatments to lower LDL-C, and that Evkeeza may improve adherence to treatment because it is administered once monthly (every 4 weeks) as an infusion.

"We are delighted that patients with HoFH will have another tool to help manage their condition, especially as it is liscensed from age 12," stated Jules Payne, CEO of Heart UK.

"This recognition from NICE on the clinical and economic benefits of Evkeeza is a meaningful milestone for people living with HoFH in England and Wales," said Erik Harris, chief commercial officer at Ultragenyx. "This highlights our commitment to working expeditiously on innovative ways to make Evkeeza broadly available to the HoFH community."

About Homozygous Familial Hypercholesterolemia (HoFH)

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

About Evkeeza® (evinacumab)

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 delivered via an infusion every 4 weeks.

Evkeeza is approved by the Medicines and Healthcare products Regulatory Agency (MHRA) as an adjunct to diet and other low-density lipoprotein-cholesterol (LDL-C) lowering therapies for the treatment of adult and adolescent patients aged 12 years and older with homozygous familial hypercholesterolemia (HoFH). The effects of Evkeeza on cardiovascular morbidity and mortality have not been determined. Regeneron Pharmaceuticals, Inc. discovered and developed Evkeeza and commercializes 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.

Evkeeza will now be reimbursed and commercially available to prescribe for appropriate patients with HoFH in the U.S., England and Wales, Canada, Italy and Germany.

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.

The company is led by a management team experienced in the development and commercialization of rare disease therapeutics. Ultragenyx's strategy is predicated upon time- and cost-efficient drug development, with the goal of delivering safe and effective therapies to patients with the utmost urgency.

For more information on Ultragenyx, please visit the company's website at: www.ultragenyx.com.

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, anticipated cost or expense reductions, the timing,

progress and plans for its clinical programs and clinical studies, future regulatory interactions, and the components and timing of regulatory submissions, 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 the Company's clinical development programs, commercial success of its products and product candidates, continued 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, 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 Ultragenyx's collaboration agreement with Regeneron to commercialize Evkeeza outside of the United States, to be terminated; uncertainty and potential delays related to clinical drug development; uncertainties and unpredictability of obtaining regulatory approval for the Company's product candidates and the scope of such potential regulatory approval; smaller than anticipated market opportunities for the Company's products and product candidates; fluctuations in buying or distribution patterns by distributors and specialty pharmacies; competition to the Company's products and product candidates; potential undesirable or serious side effects from the Company's products or product candidates; the Company's ability to effectively manage the expansion of its commercial organization; market acceptance of the Company's current or future products; uncertainties related to insurance coverage and reimbursement status of newly approved products; manufacturing risks and supply chain disruptions; competition from other therapies or 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, the timing of clinical trial activities and reporting results from same, and the availability or commercial potential of Ultragenyx's products and product 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 3, 2023, 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-/).

References

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Tromp et al. Lancet 2022; 399: 719–28 Published Online January 28, 2022 https://doi.org/10.1016/ S0140-6736(21)02001-8

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