ProQR Therapeutics and Laboratoires Théa Announce Agreement for Théa to Acquire ProQR's Sepofarsen and Ultevursen Ophthalmic Assets

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Agreement provides ProQR with initial payment of €12.5M and up to €135M in further payments, as well as potential additional earn outs based on commercial sales in the US and EU

Divestment of sepofarsen and ultevursen supports ProQR's strategic focus on the Axiomer® RNA editing technology platform and continued advancement of pipeline programs, AX-0810 and AX-1412, focused on genetic diseases originating in the liver

LEIDEN, Netherlands, CAMBRIDGE, Mass., and CLERMONT FERRAND, France, Aug. 01, 2023 (GLOBE NEWSWIRE) -- ProQR Therapeutics N.V. (Nasdaq: PRQR) (ProQR), a company dedicated to changing lives through transformative RNA therapies, and Laboratoires Théa, the leading independent eye care group in Europe ("Théa"), today announced an agreement in which ProQR will divest its late stage ophthalmic assets, sepofarsen and ultevursen, to Théa.

Under the terms of the agreement, ProQR will receive an initial payment of €12.5M and will also be eligible for up to €135M in further development, regulatory, and commercial payments, as well as additional earn outs up to high teens percentage based on commercial sales in the US and EU.

"Théa's proven expertise in the research, development, and commercialization of eye care products makes them the ideal company to continue the development of sepofarsen and ultevursen for patients with rare genetic eye diseases," said Daniel A. de Boer, Founder and Chief

of therapies for patients with high unmet need."

"For nearly 30 years, Théa has been committed to bringing the most modern and diverse range of innovative ophthalmic products to the market for the benefits of eye care practitioners and patients. We are very excited to continue the development of sepofarsen and ultevursen for patients," said Jean-Frédéric Chibret, President of the Théa group. "These two programs can deliver hope for patients suffering from retinal diseases that lead to blindness. We look forward to returning these assets into the clinic."

Within Théa, a fully dedicated team specializing in inherited retinal disorders and a new organization are currently being set up to manage these two projects. More information on the next steps for these programs will be available in the coming weeks from Théa.

ProQR's financial advisor is Lazard, with Allen & Overy acting as legal advisor. Théa's legal advisor is Dentons. The transaction is expected to close in the third quarter of 2023, subject to the satisfaction of certain closing conditions.

About ProQR

ProQR Therapeutics is dedicated to changing lives through the creation of transformative RNA therapies. ProQR is pioneering a next-generation RNA technology called Axiomer®, which uses a cell's own editing machinery called ADAR to make specific single nucleotide edits in RNA to reverse a mutation or modulate protein expression and could potentially yield a new class of medicines for both rare and prevalent diseases with unmet need. Based on our unique proprietary RNA repair platform technologies we are growing our pipeline with patients and loved ones in mind.

Learn more about ProQR at **www.proqr.com**.

About Théa

Théa is the leading independent European pharmaceutical company specialized in the research, development, and commercialization of eye care products. Based in Clermont-Ferrand, France, this family-owned and run company comprises more than 1700 collaborators and has expanded

Learn more about Théa at https://www.laboratoires-thea.com.

About Sepofarsen

Sepofarsen (QR-110) is an investigational RNA therapy designed to restore vision in Leber congenital amaurosis 10 due to the c.2991+1655A>G mutation (p.Cys998X) in the CEP290 gene. The mutation leads to aberrant splicing of the mRNA and non-functional CEP290 protein. Sepofarsen is designed to enable normal splicing, resulting in restoration of normal (wild type) CEP290 mRNA and subsequent production of functional CEP290 protein. Sepofarsen is intended to be administered through intravitreal injections in the eye and has been granted orphan drug designation in the United States and the European Union and received fast-track designation and rare pediatric disease designation from the FDA as well as access to the PRIME scheme by the EMA.

About Ultevursen

Ultevursen (formerly QR-421a) is a first-in-class investigational RNA therapy designed to address the underlying cause of vision loss in Usher syndrome type 2a and non-syndromic retinitis pigmentosa due to mutations in exon 13 of the *USH2A* gene. QR-421a is designed to restore functional usherin protein by using an exon skipping approach with the aim to stop or reverse vision loss in patients. Ultevursen is intended to be administered through intravitreal injections in the eye and has been granted orphan drug designation in the US and the European Union and received fast-track and rare pediatric disease designations from the FDA.

About Axiomer®

ProQR is pioneering a next-generation RNA base editing technology called Axiomer®, which could potentially yield a new class of medicines for diverse types of diseases. Axiomer® “Editing Oligonucleotides”, or EONs, mediate single nucleotide changes to RNA in a highly specific and targeted way using molecular machinery that is present in human cells called ADAR (Adenosine Deaminase Acting on RNA). Axiomer® EONs are designed to recruit and direct endogenously expressed ADARs to change an Adenosine (A) to an Inosine (I) in the RNA – an Inosine is translated as a Guanosine (G) – correcting an RNA with a disease-causing mutation back to a normal (wild type) RNA, modulating protein expression, or altering a protein so that it will have a new function that helps prevent or treat disease.

which are often indicated by terms such as "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "look forward to", "may," "plan," "potential," "predict," "project," "should," "will," "would" and similar expressions. Such forward-looking statements include, but are not limited to, statements regarding this divestment, the potential payments and earnouts arising out of the divestment, the expected timing for the closing of the divestment, the further development of sepofarsen and ultevursen, as well as the potential of our technologies and product candidates. Forward-looking statements are based on management's beliefs and assumptions and on information available to management only as of the date of this press release. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, the risks, uncertainties and other factors in our filings made with the Securities and Exchange Commission, including certain sections of our annual report filed on Form 20-F. These risks and uncertainties include, among others, the clinical development activities to be performed by Théa and the condition of successful market access for sepofarsen and ultevursen; the cost, timing and results of preclinical studies and other development activities by us and our collaborative partners whose operations and activities may be slowed or halted shortage and pressure on supply and logistics on the global market; our reliance on contract manufacturers or suppliers to supply materials for research and development and the risk of supply interruption or delays from suppliers or contract manufacturers; the ability to secure, maintain and realize the intended benefits of collaborations with partners, including the collaboration with Eli Lilly and Company; the possible impairment of, inability to obtain, and costs to obtain intellectual property rights; possible safety or efficacy concerns that could emerge as new data are generated in research and development; and general business, operational, financial and accounting risks, and risks related to litigation and disputes with third parties. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and we assume no obligation to update these forward-looking statements, even if new information becomes available in the future, except as required by law.

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