Lilly and ProQR to Expand RNA Editing Collaboration

December 22, 2022

New agreement supports the discovery and development of additional assets directed toward high conviction targets utilizing ProQR's Axiomer technology

INDIANAPOLIS; LEIDEN, Netherlands; and CAMBRIDGE, Mass., Dec. 22, 2022 (GLOBE NEWSWIRE) -- Eli Lilly and Company (NYSE: LLY) and ProQR Therapeutics N.V. (Nasdaq: PRQR), today announced the expansion of their licensing and collaboration agreement focused on the discovery, development and commercialization of new genetic medicines.

The collaboration, originally announced in September 2021, applied ProQR's proprietary Axiomer® RNA editing platform to target disorders of the liver and nervous system. Through the course of work to date, advances in the platform have significantly increased editing efficiency and refined biodistribution in both the liver and nervous system, opening up new potential applications to not only correct known mutations, but also introduce protective variants in specific transcripts. Through this expanded collaboration, Lilly and ProQR will explore further applications of the Axiomer platform to unlock new innovative treatments for people living with diseases with high unmet medical need.

"Discovering and developing the medicines of tomorrow takes time, sustained innovation and most importantly, collaboration," said Andrew C. Adams, Ph.D., Lilly senior vice president of genetic medicine and co-director of the Institute for Genetic Medicine. "We have been impressed with the progress to date with our partners at ProQR and have conviction that RNA editing can be an important alternative to other more permanent therapies."

"Our original collaboration with Lilly, which leverages our Axiomer RNA editing technology platform, continues to progress well and we are pleased to be expanding our partnership to include additional targets, along with an option for Lilly to opt in for more," said Daniel A. de Boer, founder and CEO of ProQR. "Lilly is a leader in RNA therapeutics, and our expanded partnership is another validation of our leadership in ADAR-mediated RNA editing, our robust IP estate, and the potential of our broadly applicable Axiomer platform technology. We look forward to making an impact on the lives of patients together with Lilly."

Under the terms of the expanded agreement, Lilly will gain access to additional targets in the central nervous system and peripheral nervous system with ProQR's Axiomer platform. ProQR will receive $75 million consisting of an upfront payment, as well as an equity investment. Lilly will have the ability to exercise an option to further expand the partnership for a consideration of $50 million. In addition, Lilly can elect to provide ProQR with access to the company's proprietary delivery technology for its wholly owned pipeline.

Based on its original September 2021 agreement and the expanded agreement announced today with Lilly, in total, ProQR is eligible to receive up to approximately $3.75 billion in research, development and commercialization milestones, as well as tiered royalties of up to mid-single digit percentage on product sales.

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.

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 Lilly

Lilly unites caring with discovery to create medicines that make life better for people around the world. We've been pioneering life-changing discoveries for nearly 150 years, and today our medicines help more than 47 million people across the globe. Harnessing the power of biotechnology, chemistry and genetic medicine, our scientists are urgently advancing new discoveries to solve some of the world's most significant health challenges, redefining diabetes care, treating obesity and curtailing its most devastating long-term effects, advancing the fight against Alzheimer's disease, providing solutions to some of the most debilitating immune system disorders, and transforming the most difficult-to-treat cancers into manageable diseases. With each step toward a healthier world, we're motivated by one thing: making life better for millions more people. That includes delivering innovative clinical trials that reflect the diversity of our world and working to ensure our medicines are accessible and affordable. To learn more, visit **Lilly.com** and **Lilly.com/newsroom** or follow us on **Facebook**, **Instagram** and **LinkedIn**. C-LLY

Forward Looking Statements for ProQR

This press release contains forward-looking statements. All statements other than statements of historical fact are forward-looking statements, 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 the collaboration with Lilly and the intended benefits thereof, including the upfront payment, equity investment, and milestone and royalty payments from commercial product sales, if any, from the products covered by the collaboration, 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 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 Lilly; 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.

Forward Looking Statements for Lilly

This press release contains forward-looking statements (as that term is defined in the Private Securities Litigation Reform Act of 1995) about the benefits of a collaboration between Lilly and ProQR, Lilly's research and development strategy, and potential payments to ProQR in connection with the collaboration and reflects Lilly's current beliefs and expectations. However, as with any such undertaking, there are substantial risks and uncertainties in the process of drug research, development and commercialization. Among other things, there can be no guarantee that Lilly will realize the expected benefits of the collaboration, that the collaboration will yield commercially successful products or that Lilly will execute its strategy as expected. For a further discussion of these and other risks and uncertainties that could cause actual results to differ from Lilly's expectations, please see Lilly's Form 10-K and Form 10-Q filings with the U.S. Securities and Exchange Commission. Lilly undertakes no duty to update forward-looking statements to reflect events after the date of this release.

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Source: ProQR Therapeutics N.V.