Press Release january 2nd, 2019
ProQR Receives Fast Track Designation from FDA for QR-421a for Usher Syndrome Type 2

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LEIDEN, Netherlands & CAMBRIDGE, Mass., Jan. 02, 2019 (GLOBE NEWSWIRE) -- ProQR Therapeutics N.V. (Nasdaq:PRQR), a company dedicated to changing lives through the creation of transformative RNA medicines for the treatment of severe genetic rare diseases, today announced that it received Fast Track designation from the Food and Drug Administration (FDA) for QR-421a. QR-421a is a first-in-class investigational RNA-based oligonucleotide designed to address the underlying cause of the vision loss associated with Usher syndrome type 2 and non-syndromic retinitis pigmentosa (RP) due to mutations in exon 13 of the USH2A gene.

Fast Track designation is granted by FDA to drugs that are under development for serious conditions and have the potential to fulfill an unmet medical need. It was established with the intention to bring promising drugs to patients sooner by facilitating the development with more frequent FDA interactions and expediting the review process.

“We are very pleased with the Fast Track designation the FDA granted us for QR-421a. Patients with Usher syndrome, the leading cause of combined deafness and blindness, currently have no available therapies for their vision loss and this designation emphasizes the high unmet need in this disease,” said Daniel de Boer, Chief Executive Officer of ProQR. “We are also looking forward to begin enrollment in the Phase 1/2 STELLAR clinical trial in the coming months with preliminary data expected in mid-2019.”

About QR-421a

QR-421a is a first-in-class investigational RNA-based oligonucleotide designed to address the underlying cause of vision loss in Usher syndrome type 2 and non-syndromic retinitis pigmentosa (RP) due to mutations in exon 13 of the USH2A gene. Mutations in this exon can cause loss of functional usherin protein that causes the disease. QR-421a is designed to exclude the genetic defect from the RNA in the eye, such that it leads to the expression of a shortened but functional usherin protein, thereby modifying the underlying disease. QR-421a has received orphan drug designation in the United States and the European Union.

About Usher Syndrome

Usher syndrome is the leading cause of combined deafness and blindness. Patients with this syndrome generally progress to a stage in which they have very limited central vision and moderate to severe deafness. Usher syndrome type 2 is one of the most common forms of Usher syndrome and is caused by mutations in

the USH2A gene. To date, there are no approved treatments or products in clinical development that treat the vision loss associated with Usher syndrome type 2.

About ProQR

ProQR Therapeutics is dedicated to changing lives through the creation of transformative RNA medicines for the treatment of severe genetic rare diseases such as Leber's congenital amaurosis 10, Usher syndrome type 2 and dystrophic epidermolysis bullosa. Based on our unique proprietary RNA repair platform technologies we are growing our pipeline with patients and loved ones in mind. *Since 2012*

FORWARD-LOOKING STATEMENTS

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. 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. These forward-looking statements include, but are not limited to, statements regarding QR-421a and its clinical development and therapeutic potential, including commencement of the STELLAR trial, trial design and timing of results from this trial. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with our clinical development activities, including that positive results observed in our prior and ongoing studies may not be replicated in later trials or guarantee approval of any product candidate by regulatory authorities, regulatory review or approval process, manufacturing processes and facilities, regulatory oversight, product commercialization, intellectual property claims, and 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. 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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