4D Molecular Therapeutics Announces FDA Fast Track Designation Granted to 4D-125 for the Treatment of X-linked Retinitis Pigmentosa

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EMERYVILLE, Calif., Jan. 10, 2022 (GLOBE NEWSWIRE) -- 4D Molecular Therapeutics (NASDAQ: FDMT), a clinical-stage gene therapy company harnessing the power of directed evolution for targeted gene therapies, announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation for 4D-125 for treatment of patients with inherited retinal dystrophies due to defects in the *RPGR* gene, including X-linked Retinitis Pigmentosa (XLRP). 4D-125 is a targeted and evolved R100-based product candidate, which was invented at 4DMT for efficient intravitreal delivery, and is designed to deliver a functional copy of the *RPGR* gene to photoreceptors in the retina.

“Patients living with XLRP currently have no approved treatments, and they suffer from progressive vision loss and blindness that reduces their quality of life and independence,” said Robert Kim, M.D., Senior Vice President and Ophthalmology Therapeutic Area Head of 4DMT. “Fast Track Designation is a landmark event for the program and underscores the potential of 4D-125 to address a significant unmet need for those living with XLRP.”

About Fast Track Designation

The FDA's Fast Track process is designed to accelerate the development and review of treatments for serious and life-threatening diseases where no treatment exists or where the treatment in discovery may provide advantages over what is currently available. A drug candidate that receives Fast Track designation is eligible for more frequent communication with the FDA throughout the drug development process and a rolling and/or priority review of its marketing application if relevant criteria are met.

About 4D-125 and XLRP

4D-125 is 4DMT’s targeted and evolved R100-based product candidate for XLRP and is designed to deliver a functional copy of the *RPGR* gene to photoreceptors in the retina. 4DMT is currently enrolling patients in an on-going Phase 1/2 clinical trial. The study employed a standard 3+3 dose-escalation design, followed by dose expansion. In dose-escalation, patients were enrolled in one of two dose cohorts: 3E11 vg/eye and 1E12 vg/eye. The dose expansion phase of the study is enrolling patients at the 1E12 vg/eye dose. The primary objectives of this trial are to evaluate the safety and maximum tolerated dose of 4D-125. Secondary endpoints include assessments of clinical activity, including both visual function and anatomical endpoints.

XLRP is a rare inherited X-linked recessive genetic disorder that causes progressive vision loss and blindness in boys and young men. There are currently no approved therapies for XLRP. Seventy percent of cases are caused by mutations in the retinitis pigmentosa GTPase regulator (“*RPGR*”) gene. The estimated worldwide prevalence of XLRP due to *RPGR* variants is approximately one in 25,600 people, which represents approximately 24,000 patients in the United States, and France, Germany, Italy, Spain and the United Kingdom (together, EU-5). It is characterized by dysfunction and degeneration of photoreceptors in the retina. Symptoms of XLRP are initially characterized by night blindness, followed by loss of peripheral visual field, decreasing visual acuity and eventually blindness.

About 4DMT

4DMT is a clinical-stage company harnessing the power of directed evolution for targeted gene therapies. 4DMT seeks to unlock the full potential of gene therapy using its platform, Therapeutic Vector Evolution, which combines the power of directed evolution with approximately one billion synthetic capsid sequences to invent evolved vectors for use in targeted gene therapy products. The company is initially focused in three therapeutic areas: ophthalmology, cardiology and pulmonology. The 4DMT targeted and evolved vectors are invented with the goal of being delivered through clinically routine, well-tolerated and minimally invasive routes of administration, transducing diseased cells in target tissues efficiently, having reduced immunogenicity and, where relevant, having resistance to pre-existing antibodies. 4DMT is currently advancing five product candidates in development: 4D-310 for Fabry disease, 4D-125 for XLRP, 4D-150 for wet AMD, 4D-110 for choroideremia and 4D-710 for cystic fibrosis.

4D-310, 4D-150, 4D-125 and 4D-110 are in clinical trials and have not yet been approved for marketing by the US FDA or any other regulatory authority. No representation is made as to the safety or effectiveness of 4D-310, 4D-150, 4D-125 or 4D-110 for the therapeutic use for which they are being studied. 4D Molecular Therapeutics™, 4DMT™, Therapeutic Vector Evolution™, and the 4DMT logo are trademarks of 4DM.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, implied and express statements regarding plans and timelines for the clinical development of 4D-310, 4D-150, 4D-125, 4D-710 and 4D-110, including the therapeutic potential and clinical benefits thereof; expectations regarding current and future interactions with the FDA; and 4D Molecular Therapeutics' strategy, business plans and focus. The words "may," “might,” "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," “expect,” "estimate," “seek,” "predict," “future,” "project," "potential," "continue," "target" and similar words or expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, risks associated with: the impact of COVID-19 on countries or regions in which we have operations or do business, as well as on the timing and anticipated results of our clinical trials, strategy and future operations; the delay of any current or planned clinical trials for the development of 4D Molecular Therapeutics' drug candidates, the risk that the results of our clinical trials may not be predictive of future results in connection with future clinical trials; 4D Molecular Therapeutics' ability to successfully demonstrate the safety and efficacy of its drug candidates; the timing and outcome of our planned interactions with regulatory authorities; and obtaining, maintaining and protecting our intellectual property. These and other risks and uncertainties are described in greater detail in the section entitled "Risk Factors" in 4D

Molecular Therapeutics' most recent Quarterly Report on Form 10-Q, as well as any subsequent filings with the Securities and Exchange Commission. In addition, any forward-looking statements represent 4D Molecular Therapeutics' views only as of today and should not be relied upon as representing its views as of any subsequent date. 4D Molecular Therapeutics explicitly disclaims any obligation to update any forward-looking statements. No representations or warranties (expressed or implied) are made about the accuracy of any such forward-looking statements.

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