Imara Announces IMR-687 Granted Fast Track Designation and Rare Pediatric Disease Designation for Treatment of Beta-Thalassemia

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BOSTON, July 30, 2020 (GLOBE NEWSWIRE) -- IMARA Inc. (Nasdaq: IMRA), a clinical-stage biopharmaceutical company dedicated to developing and commercializing novel therapeutics to treat patients suffering from rare inherited genetic disorders of hemoglobin, today announced the U.S. Food and Drug Administration (FDA) has granted Fast Track designation and Rare Pediatric Disease designation for its lead clinical asset, IMR-687, for the treatment of beta-thalassemia. The FDA previously granted Orphan Drug designation for IMR-687 for the treatment of patients with beta-thalassemia and Orphan Drug, Fast Track and Rare Pediatric Disease designations for the treatment of patients with sickle cell disease.

“We are pleased to receive from the FDA both Fast Track designation and Rare Pediatric Disease designation for IMR-687. Fast Track designation helps us create the opportunity to potentially accelerate the development of IMR-687 in beta-thalassemia. Rare Pediatric designation reflects the agency’s recognition that beta-thalassemia is a serious disease, with symptoms that manifest in childhood and progress over time into adulthood,” said Rahul Ballal, Ph.D., President and Chief Executive Officer of Imara. “We look forward to continuing to advance this novel investigational therapy on behalf of patients in the U.S. and globally living with beta-thalassemia.”

Imara recently initiated a Phase 2b clinical trial of IMR-687 in adult patients with beta-thalassemia and expects to dose the first patient in the near-term. Regulatory submissions are underway in 14 countries and screening has initiated for the Phase 2b clinical trial. Imara anticipates initiating pediatric clinical testing in beta-thalassemia after gathering sufficient clinical data in adult patients with beta-thalassemia.

About Fast Track Designation
The FDA’s Fast Track designation is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. Fast Track designation allows for early and frequent communication with the FDA throughout the entire drug development and review process. It also enables eligibility for Accelerated Approval and Priority Review, as well as a rolling review of a company’s New Drug Application, if relevant criteria are met.

About Rare Pediatric Disease Designation
Under the FDA’s Rare Pediatric Disease (RPD) program, a sponsor who receives marketing approval for a product with an RPD designation may be eligible for a voucher that can be redeemed to obtain priority review for any subsequent marketing application. The FDA defines a "rare pediatric disease" as a rare disease that affects fewer than 200,000 people in the United States and in which the serious or life-threatening manifestations primarily affect individuals from age zero to 18.

About IMR-687
IMR-687 is a highly selective and potent small molecule inhibitor of PDE9. PDE9 uniquely degrades cyclic guanosine monophosphate (cGMP), an active signaling molecule that plays a role in vascular biology. Lower levels of cGMP are often found in people with sickle cell disease and beta-thalassemia and are associated with impaired blood flow, increased inflammation, greater cell adhesion and reduced nitric oxide mediated vasodilation.

Blocking PDE9 acts to increase cGMP levels, which are associated with reactivation of fetal hemoglobin, or HbF, a natural hemoglobin produced during fetal development. Increased levels of HbF in red blood cells have been demonstrated to improve symptomology and lower disease burden in patients with sickle cell disease and patients with beta-thalassemia.

About Imara
Imara Inc. is a clinical-stage biotechnology company dedicated to developing and commercializing novel therapeutics to treat patients suffering from rare inherited genetic disorders of hemoglobin. Imara is currently advancing IMR-687, a highly selective, potent small molecule inhibitor of PDE9 that is an oral, once-a-day, potentially disease-modifying treatment for sickle cell disease and beta-thalassemia. IMR-687 is being designed to have a multimodal mechanism of action that acts on red blood cells, white blood cells, adhesion mediators and other cell types. For more information, please visit www.imaratx.com.

Cautionary Note Regarding Forward-Looking Statements

Statements in this press release about future expectations, plans and prospects, as well as any other statements regarding matters that are not historical facts, may constitute “forward-looking statements” within the meaning of The Private Securities Litigation Reform Act of 1995. These statements include, but are not limited to, statements made by Dr. Ballal in this press release and statements relating to the (i) design and timing of the company’s clinical development program for IMR-687 in beta-thalassemia, including the recently initiated Phase 2b clinical trial and (ii) the potential advantages of the Fast Track designation and Rare Pediatric Disease designation by the FDA. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “will,” “would” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: the impact of extraordinary external events, such as the risks and uncertainties resulting from the impact of the COVID-19 pandemic on the Company’s business, operations, strategy, goals and anticipated milestones, including its ability to enroll, dose and readout data from its Phase 2b clinical trial of IMR-687 in beta-thalassemia; and other factors discussed in the “Risk Factors” section of the Company’s most recent Quarterly Report on Form 10-Q, which is on file with the Securities and Exchange Commission and in other filings that the Company makes with the Securities and Exchange Commission in the future. Any forward-looking statements contained in this press release speak only as of the date hereof, and the Company expressly disclaims any obligation to update any forward-looking statement, whether as a result of new information, future events or otherwise.

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