Autolus Therapeutics Receives FDA Orphan Drug Designation for AUTO3 for Treatment of Acute Lymphoblastic Leukemia

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LONDON, April 23, 2019 (GLOBE NEWSWIRE) -- Autolus Therapeutics plc (Nasdaq: AUTL), a clinical-stage biopharmaceutical company developing next-generation programmed T cell therapies for the treatment of cancer, announced that the United States Food and Drug Administration (FDA) has granted orphan drug designation to autologous enriched T-cells genetically modified with a retroviral vector to express two chimeric antigen receptors targeting CD19 and CD22 (AUTO3) for the treatment of acute lymphoblastic leukemia (ALL).

According to the National Institute of Health's National Cancer Institute, in the United States, there will be an estimated 5,930 new cases of ALL and an estimated 1,500 related deaths in 2019. Patients are predominantly children; approximately 60% of cases occur at age < 20 years. ALL occurs when the bone marrow makes too many immature lymphocytes, which are a type of white blood cell. Despite a high rate of response to induction chemotherapy, only 30–40% of adult patients with ALL will achieve long-term remission. Similarly, pediatric patients typically respond well to first-line treatment (combination chemotherapy) but 10 to 20% of total patients relapse with chemotherapy-resistant disease, leading to a significant unmet need in pediatric patients with high-risk relapsed or refractory ALL.

"We are pleased to receive orphan drug designation for AUTO3 for acute lymphoblastic leukemia," said Dr. Christian Itin, chairman and chief executive officer of Autolus. "Earlier this year, we presented encouraging updated data from the AMELIA phase 1/2 trial of AUTO3 in pediatric ALL patients. We believe that AUTO3 has the potential to be a best in class therapy in pediatric ALL by addressing antigen escape, a common cause of relapse in these patients. AUTO3 may also provide an improved safety profile over currently marketed CAR T therapies with low levels of severe CRS and neurotoxicity observed in clinical studies."

Orphan drug designation is granted by the FDA Office of Orphan Products Development to drugs and biologics which are intended for the treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the U.S. Under the Orphan Drug Act, the FDA may provide grant funding toward clinical trial costs, tax advantages, FDA user-fee benefits, and seven years of market exclusivity in the United States following marketing approval by the FDA. For more information about orphan designation, please visit the FDA website at www.fda.gov.

About AUTO3
AUTO3 is a programmed T cell therapy containing two independent chimeric antigen receptors targeting CD19 and CD22 that have each been independently optimized for single target activity. By simultaneously targeting two B cell antigens, AUTO3 is designed to minimize relapse due to single antigen loss in patients with B cell malignancies. AUTO3 is currently being tested in pediatric ALL in the AMELIA clinical trial and in diffuse large B cell lymphoma in the ALEXANDER clinical trial.

For more information about the AMELIA trial, visit www.ClinicalTrials.gov (NCT03289455). For more information about the ALEXANDER trial, visit www.ClinicalTrials.gov (NCT03287817).

About Autolus Therapeutics plc
Autolus is a clinical-stage biopharmaceutical company developing next-generation, programmed T cell therapies for the treatment of cancer. Using a broad suite of proprietary and modular T cell programming technologies, the company is engineering precisely targeted, controlled and highly active T cell therapies that are designed to better recognize cancer cells, break down their defense mechanisms and eliminate these cells. Autolus has a pipeline of product candidates in development for the treatment of hematological malignancies and solid tumors. For more information please visit www.autolus.com.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Forward-looking statements are statements that are not historical facts, and in some cases can be identified by terms such as "may," "will," "could," "expects," "plans," "anticipates," and "believes." These statements include, but are not limited to, statements regarding the company's product candidates and research programs. Any forward-looking statements are based on management's current views and assumptions and involve risks and uncertainties that could cause actual results, performance or events to differ materially from those expressed or implied in such statements. For a discussion of other risks and uncertainties, and other important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the section titled "Risk Factors" in the company's Annual Report on Form 20-F filed on November 23, 2018 as well as discussions of potential risks, uncertainties, and other important factors in the company's future filings with the Securities and Exchange Commission from time to time. All information in this press release is as of the date of the release, and the company undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events, or otherwise, except as required by law.

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