



Autolus Therapeutics Receives FDA Orphan Drug Designation for AUTO1 for Treatment of Acute Lymphoblastic Leukemia

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LONDON, Nov. 05, 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 AUTO1 orphan drug designation for treatment of acute lymphoblastic leukemia (ALL) patients.

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 AUTO1 for acute lymphoblastic leukemia," said Dr. Christian Itin, chairman and chief executive officer of Autolus. "From the data reported in our ongoing studies, we have seen strong remission rates and excellent CAR T cell expansion and persistence without inducing high-grade CRS, a serious adverse event affecting a significant number of patients on currently available CAR T treatments. We look forward to presenting data on AUTO1 at ASH at the end of the year."

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 AUTO1

AUTO1 is a CD19 CAR T cell investigational therapy designed to overcome the limitations in safety - while maintaining similar levels of efficacy - compared to current CD19 CAR T cell therapies. Designed to have a fast target binding off-rate to minimize excessive activation of the programmed T cells, AUTO1 may reduce toxicity and be less prone to T cell exhaustion, which could enhance persistence and improve the T cells' abilities to engage in serial killing of target cancer cells. In 2018, Autolus signed a license agreement under which Autolus acquired global rights from UCL Business plc (UCLB), the technology-transfer company of UCL, to develop and commercialize AUTO1 for the treatment of B cell malignancies. AUTO1 is currently being evaluated in two Phase 1 studies, one in pediatric ALL and one in adult ALL.

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 Statement

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 Autolus' financial condition and results of operations, as well as statements regarding the anticipated development of Autolus' product candidates, including its intentions regarding the timing for providing further updates on the development of its product candidates, and the sufficiency of its cash resources. 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 Autolus' Annual Report on Form 20-F filed on November 23, 2018 as well as discussions of potential risks, uncertainties, and other important factors in Autolus' 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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