Autolus Therapeutics Announces Updated Results from Ongoing CARPALL Trial of Pediatric Acute Lymphoblastic Leukemia Presented at the EHA 1st European CAR T Cell Meeting in Paris

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Updated results confirm AUTO1 data from ASH 2017
No severe cytokine release syndrome (Grade 3-5)
86% molecular complete response rate after a single dose of AUTO1

LONDON, Feb. 19, 2019 /PRNewswire/ -- Autolus Therapeutics plc (Nasdaq: AUTL), a clinical-stage biopharmaceutical company developing next-generation programmed T cell therapies for the treatment of cancer, today announced that Professor Persis Amrolia, Consultant in Bone Marrow Transplant at Great Ormond Street Hospital (GOSH) and NIHR Research Professor of Transplantation Immunology at University College London (UCL) Great Ormond Street Institute of Child Health, presented updated data from the ongoing Phase 1 CARPALL trial of AUTO1 in a poster presentation at the European Hematology Association 1st European CAR T Cell Meeting held in Paris, France, February 14-16, 2019.

Enrolled patients had a median age of 9 years with a median of 4 lines of prior treatment. Seventeen patients were enrolled, and 14 patients received an infusion of CAR T cells. Ten of 14 patients had relapsed post allogeneic stem cell transplant. Eight patients were treated in second relapse, 5 in >second relapse and 3 had relapsed after prior blinatumomab or inotuzumab therapy. Two patients had ongoing CNS disease at enrollment.

Updated safety results
This data set confirms that AUTO 1 induces no severe cytokine release syndrome (CRS) (Grade 3-5). Nine patients experienced Grade 1 CRS, and 4 patients experienced Grade 2 CRS. No patients required tociluzumab or steroids. As previously reported, one patient experienced Grade 4 neurotoxicity; there were no other reports of severe neurotoxicity (Grade 3-5). The mean cumulative exposure to AUTO1 CAR T cells in the first 28 days as assessed by AUC was 1,721,355 copies/µg DNA. Eleven patients experienced cytopenia that was not resolved by day 28 or recurring after day 28: 3 patients Grades 1-3 and 8 patients Grade 4. Two patients developed significant infections, and 1 patient died from sepsis while in molecular complete response (CR).

Updated efficacy results
With a single dose of CAR T cells at 1 million cells/kg dose, 12/14 (86%) achieved molecular CR. Five patients relapsed with CD19 negative disease. Event free survival (EFS) based on morphological relapse was 67% (CI 34-86%) and 46% (CI 16-72%) and overall survival (OS) was 84% (CI 50-96%) and 63% (CI 27-85%) at 6 and 12 months, respectively.

CAR T cell expansion was observed in all responding patients (N=12), with CAR T cells comprising up to 84% of circulating T cells at the point of maximal expansion. The median persistence of CAR T was 215 days.

The median duration of remission in responding patients was 7.3 months with a median follow-up of 14 months. Five of 14 patients (37%) remain in CR with ongoing persistence of CAR T cells and associated B cell aplasia.

"AUTO1 combines a high molecular CR rate with excellent persistence and a good safety profile in pediatric acute B cell leukemia patients," said Professor Amrolia.

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.

For information about the CARPALL trial, visit **https://clinicaltrials.gov/ct2/show/NCT02443831**

About Pediatric Acute Lymphoblastic Leukemia (ALL)
According to the American Cancer Society, ALL is the most common cancer diagnosed in children, with approximately 3,400 new cases diagnosed in the United States each year. Pediatric ALL occurs when the bone marrow makes too many immature lymphocytes, which are a type of white blood cell. The current standard of care for pediatric ALL patients is combination chemotherapy. Although pediatric patients typically respond well to first-line treatment, 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.

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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