



## **Autolus Announces Publication in Nature Medicine of Data Supporting the Development of AUTO1 for Treatment of Patients with ALL**

*Findings from Phase I CARPALL Trial Demonstrate that Autolus' Novel CAR T Therapy, AUTO1, Induces Enhanced Anti-tumor Response Without Severe Cytokine Release Syndrome*

*86% molecular complete response rate after a single dose of AUTO1*

LONDON, September 3, 2019 – [Autolus Therapeutics plc](#) (Nasdaq: AUTL), a clinical-stage biopharmaceutical company developing next-generation programmed T cell therapies, today announced that the journal *Nature Medicine* has published both pre-clinical results and clinical data from the ongoing Phase I CARPALL trial of AUTO1, demonstrating the potential of the company's novel CAR T therapy targeting CD19 in development for the treatment of pediatric acute lymphoblastic leukemia (ALL). The paper reports that AUTO1, or CAT Chimeric Antigen Receptor T cells (CAT CAR T), utilizes a binder with a fast off rate and showed both increased proliferation/cytotoxicity in vitro and enhanced proliferative capacity and anti-tumor activity when compared to FMC63 CAR T therapies in vivo. In the Phase 1 clinical trial, 86% (n=14) of recurrent/refractory pediatric ALL patients achieved molecular complete remission after a single dose, with a median duration of remission of 7.4 months and no severe cytokine release syndrome (CRS;  $\geq$  grade 3 or 4), in this relapsed and/or refractory patient population.

"The safety profile emerging from this pediatric study is encouraging. AUTO1 was well-tolerated and we did not see severe cytokine release syndrome or neurotoxicity seen in other ALL programs," said Sara Ghorashian, PhD, Molecular and Cellular Immunology Section, UCL Great Ormond Street Institute of Child Health and a co-author of the paper. "It is very promising to see these strong remission rates and excellent CAR T cell expansion and persistence, which give us hope that AUTO1 could improve outcomes for these patients."

"The publication in *Nature Medicine* is a nice validation of our AUTO1 pre-clinical and Phase 1 clinical data," said Dr. Christian Itin, chairman and chief executive officer of Autolus. "AUTO1 CAR- T cells are designed to effectively engage leukemic cells while avoiding excessive immune stimulation. This profile results in an improved safety profile compared to current treatments, while achieving a high level of clinical activity. We are currently testing the activity of AUTO1 in adult patients who typically are even more susceptible to severe immunological adverse events than pediatric patients."

### **About the Studies**

The *Nature Medicine* publication includes a discussion of both pre-clinical and clinical studies. The pre-clinical assessment included evaluation of in vitro responses of AUTO1 versus FMC63

CAR T cells (cytotoxicity, proliferation and cytokine production) as well as a comparison of their anti-tumor efficacy within a xenogeneic model of ALL.

CARPALL (NCT02443831) is a multi-center, non-randomized, open label Phase I clinical trial of heavily pre-treated patients under age 24 with high risk and relapsed CD19+ ALL. 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.

This data set confirms that AUTO 1 induces no severe CRS (Grade 3-5). Nine patients experienced Grade 1 CRS, and 4 patients experienced Grade 2 CRS. No patients required tocilizumab or steroids. As previously reported, one patient experienced Grade 4 neurotoxicity; there were no other reports of severe neurotoxicity (Grade 3-5). 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).

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 7.4 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.

### **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 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, go to: <https://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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