Autolus Therapeutics announces data from AUTO1/22 trial in pediatric Acute Lymphoblastic Leukemia published in the journal Blood

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- *Data support utility of AUTO1/22 approach in preventing antigen evasion in pediatric B-ALL*
- *Complete responses observed in patients with CD19 negative disease*
- *No antigen negative relapse seen in responding patients*

LONDON, Sept. 05, 2023 (GLOBE NEWSWIRE) -- Autolus Therapeutics plc (Nasdaq: AUTL), a clinical-stage biopharmaceutical company developing next-generation programmed T cell therapies, today announces a publication on the AUTO1/22 Phase 1 study (CARPALL) in Pediatric B-cell Acute Lymphoblastic Leukemia in the journal *Blood*,[1] previously presented this year at the European Society for Blood and Marrow Transplantation in April.

CD19 negative relapse is a major cause of treatment failure after CD19 CAR T cell therapy for pediatric B-ALL. To address this, AUTO1/22 is designed to target both CD19 and CD22 using the fast-off rate CD19 CAR from obecabtagene autoleucel (obe-cel) combined with a novel CD22 CAR capable of effective signaling in response to low antigen density[2].

Twelve patients with advanced pediatric B-ALL were treated. Patients on study were high risk, with 4 patients who had failed prior CD19 CAR therapy, 3 patients with a CD19-negative disease component, 3 patients with non-CNS extramedullary disease and 6 patients who had received prior blinatumomab.

AUTO1/22 maintained the safety profile of obe-cel alone, with no cases of severe cytokine release syndrome.

AUTO1/22 induced MRD (minimal residual disease)-negative CR in 83% (10 of 12) patients. This includes 2 (of 3) patients who had CD19 negative disease, demonstrating the efficacy of the CD22 CAR. Two patients failed to respond: one with CD19+/CD22+ disease and another with progression of double CD19-/CD22- disease, present as a minor leukemic cell population pre-infusion.

Of 10 responding patients, 5 had emergence of MRD (2) or frank relapse (3) with CD19 and CD22 expressing disease associated with loss of CAR T-cell persistence. Importantly, there were no cases of relapse due to antigen-negative escape, with a median follow-up of 8.7 months. Overall survival was 75% at 6 and 12 months. Six and 12-month event free survival (EFS) were 75% and 60% respectively.

“CAR T-cell therapies which co-target CD19 and CD22 are needed to prevent antigen-negative escape in pediatric B-ALL. However, co-targeting has proved challenging in the field due to the different natures of these antigens and complications of receptor co-expression,” said **Dr Martin Pule, Chief Scientific Officer of Autolus**. “AUTO1/22 uses a co-transduction approach to allow full and independent expression of CD19 and CD22 receptors and was able to induce remission in 2 out of 3 children with CD19 negative disease. Importantly, we have not observed antigen negative relapse. AUTO1/22 specifically, and the co-transduction approach more broadly, may have utility in preventing antigen negative relapse in B-ALL and other B cell lymphoma or leukemias.”

1. Ghorashian et al, Blood; doi.org/10.1182/blood.2023020621

2. Kokalaki et al, Mol Therapy Vol. 31 No 7 July 2023; doi: 10.1016/j.ymthe.2023.03.020

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.

About AUTO1/22
AUTO1/22 is a novel dual targeting CAR T cell-based therapy candidate based on obe-cel. It is designed to combine the enhanced safety, robust expansion and persistence seen with the fast off rate CD19 CAR from obe-cel with a high sensitivity CD22 CAR to reduce antigen negative relapses. This product candidate is currently in a Phase 1 clinical trial for patients with r/r pediatric ALL. [NCT02443831]

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 continued development of Autolus' AUTO1/22 program; the status of clinical trials (including, without limitation, expectations regarding the data that is being presented, the expected timing of data releases and development, as well as completion of clinical trials) and development timelines for the Company's product candidates. 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. These risks and uncertainties include, but are not limited to, the risks that Autolus' preclinical or clinical programs do not advance or result in approved products on a timely or cost effective basis or at all; the results of early clinical trials are not always being predictive of future results; the cost, timing, and results of clinical trials; that many product candidates do not become approved drugs on a timely or cost effective basis or at all; the ability to enroll

patients in clinical trials; possible safety and efficacy concerns; and the impact of the ongoing COVID-19 pandemic on Autolus' business. For a discussion of other risks and uncertainties, and other important factors, any of which could cause Autolus' 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 with the Securities and Exchange Commission on March 7, 2023, as well as discussions of potential risks, uncertainties, and other important factors in Autolus' subsequent filings with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and Autolus 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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