



## **Autolus Therapeutics Announces New Data Showcasing Clinical Progress of Programmed T Cell Therapy Pipeline in Blood Cancers**

December 7, 2019 at 2:00 PM EST

***AUTO1 shows 87% MRD negative complete response in adult patients with r/r ALL, with no severe cytokine release syndrome***

***Data presented at 61<sup>st</sup> American Society of Hematology Annual Meeting form basis for advancement of AUTO1 into pivotal clinical trial in adult ALL***

***Investor call to be held December 9 at 8:30 am ET / 1:30 pm GMT to review data***

LONDON, Dec. 07, 2019 (GLOBE NEWSWIRE) -- Autolus Therapeutics plc (Nasdaq: AUTL) announced today new data highlighting progress on its next-generation programmed T cell therapies to treat patients with acute lymphoblastic leukemia (ALL) and adults with relapsed/refractory diffuse large B cell lymphoma (DLBCL). The data were presented in oral presentations at the 61<sup>st</sup> American Society of Hematology (ASH) Annual Meeting and Exposition in Orlando, FL. Additional data on pediatric patients with ALL will be presented on December 8.

"The data on AUTO1 presented at this year's ASH meeting demonstrate the favorable safety profile and high level of clinical activity of AUTO1 in both adults and pediatric patients with ALL, and we look forward to initiation of the pivotal program in adult ALL in the first half of 2020," said Dr. Christian Iltin, chairman and chief executive officer of Autolus.

### **Acute Lymphoblastic Leukemia Data Presented**

**Title: AUTO1 – A novel fast off CD19CAR delivers durable remissions and prolonged CAR T cell persistence with low CRS or neurotoxicity in adult ALL (Abstract # 226)**

Updated results for ALLCAR19, the Phase 1 trial evaluating AUTO1 in adults with recurrent/refractory ALL, were presented by Dr. Claire Roddie MB, PhD, FRCPath, honorary senior lecturer, Cancer Institute, University College London (UCL), in an oral presentation. The trial is designed to assess the primary endpoints of safety ( $\geq$  Grade 3 toxicity) and feasibility of product generation, as well as other secondary endpoints, including efficacy. The trial enrolled patients with a high tumor burden (44% had  $\geq$  50% BM blasts), who were considered high-risk for experiencing cytokine release syndrome (CRS). Product was manufactured for 19 patients; product for 13 of those patients was manufactured using a semi-automated closed process, which will be used for commercial supply.

As of the data cut-off date of November 25, 16 patients had received at least one dose of AUTO1. AUTO1 was well tolerated, with no patients experiencing  $\geq$  Grade 3 CRS, and 3 of 16 patients (19%), who had high leukemia burden, experiencing Grade 3 neurotoxicity that resolved swiftly with steroids.

Of 15 patients evaluable for efficacy, 13 (87%) achieved MRD negative CR at 1 month and all patients had ongoing CAR T cell persistence at last follow up. CD19-negative relapse occurred in 22% (2 of 15) patients. In the patients dosed with AUTO1 manufactured in the closed process, 9 of 9 (100%) achieved MRD negative CR at 1 month and 6 months event free survival, and overall survival in this cohort was 100%.

"Adult ALL patients, who face a median survival of less than one year after their ALL recurs or relapses, have a significant need for a CAR T cell therapy that is highly active, safe and is a standalone therapy not requiring a stem cell transplant," said Dr. Hagop M. Kantarjian, Chair of the Department of Leukemia at The University of Texas MD Anderson Cancer Center.

"The novel CD 19 CAR-T therapy, AUTO1, is potentially transformative as a standalone curative option for patients with r/r ALL, especially in adults, given its favorable safety profile," said Dr. Max Topp associate professor of Internal Medicine, Hematology and Oncology at the University of Wuerzburg.

**Title: Therapy of pediatric B-ALL with a lower affinity CD19 CAR leads to enhanced expansion and prolonged CAR T cell persistence in patients with low bone marrow tumor burden, and is associated with a favorable toxicity profile (Abstract # 225)**

Dr. Sara Ghorashian, honorary senior lecturer, Great Ormond Street Institute of Child Health, University College London, presented updated data from the phase 1 CARPALL study of AUTO1 in pediatric ALL patients with low bone marrow tumor burden. The trial is intended to assess the primary endpoints of safety and proportion of patients in molecular complete remission at 1 month. The study recruited a total of 25 patients and stratified them into 2 cohorts. Fourteen patients were treated in cohort 1, which utilized a manual manufacturing process; product was unable to be generated in 3 patients. Median follow-up was 23 months in cohort 1. Seven patients were treated in cohort 2, which utilized the semi-automated closed manufacturing process, which will be used for commercial supply. The aim of cohort 2 was to demonstrate feasibility of manufacture at scale. Product was generated for 100% of patients. Median follow-up was 7 months in cohort 2.

AUTO1 was well-tolerated overall, with no patients experiencing  $\geq$  Grade 3 CRS and 1 of 21 (5%) experiencing Grade 4 neurotoxicity, which was considered unrelated to CAR T therapy.

Nineteen of 21 treated patients (90%) achieved molecular complete remission at 1 month post infusion. Consistent with pre-clinical data, CAR T cell expansion was excellent and detectable by flow in a number of patients up to 36 months. Persistence was noted in 15 of 21 patients at last follow-up, up to 36 months. In cohort 2, 100% of patients achieved molecular complete remission at 1 month post infusion.

In the 14 patients in cohort 1, the overall survival at 6 months was 86% and at 12 months was 71%; event free survival (EFS) at 6 months was 71% and at 12 months was 54%. The patients in cohort 2 are not yet evaluable for these parameters. Overall, 8 patients relapsed; 5 of 8 evaluable relapses were due to loss of CD19 antigen on the tumor cells.

#### **Title: Clonal dynamics of early responder and long-term surviving CAR-T cells in humans (Abstract # 52)**

Dr. Luca Biasco, senior research associate at University College London, presented a detailed analysis of CAR T products, and insertion site analysis from the CARPALL phase 1 patients. This analysis revealed highly polyclonal engraftment, even at very late time-points. Dr. Biasco hypothesized that the propensity for high level polyclonal long-term engraftment was due to favorable phenotype of the CAR T product and the binding kinetic of the receptor.

#### **Diffuse Large B-cell Lymphoma Data Presented**

#### **Title: Phase 1/2 study of AUTO3, the first bicistronic chimeric antigen receptor (CAR) targeting CD19 and CD22 followed by an anti-PD1 in patients with relapsed/refractory (r/r) Diffuse Large B Cell Lymphoma (DLBCL): Results of cohort 1 and 2 of the ALEXANDER study (Abstract # 246)**

Dr. Kirit Ardeshtna, consultant hematologist, Department of Hematology, University College London Hospital NHS Foundation Trust, presented updated data from the ALEXANDER Phase 1/2 study of AUTO3, the first bicistronic CAR T targeting CD19 and CD22 followed by an anti-PD1, in diffuse large B cell lymphoma (DLBCL). 16 patients were treated, and fourteen patients were evaluable at one month. AUTO3 was well-tolerated, with no patients experiencing  $\geq$  Grade 3 CRS with primary treatment, and 1 of 14 experiencing Grade 3 neurotoxicity that resolved swiftly with steroids. Five of 14 had a complete response, with 4 of 5 complete responses ongoing, the longest at 18 months.

"DLBCL is an aggressive and rapidly progressing cancer, and early response is critical to ensuring positive outcomes for these patients. These early data show the promise of AUTO3 in DLBCL, and we expect to advance AUTO3 to a decision point in relapsed/refractory DLBCL by the middle of next year," said Dr. Christian Itin, chairman and chief executive officer of Autolus. "In addition, we look forward to presenting the data from the AMELIA trial of AUTO3 in pediatric ALL during poster sessions on Sunday, December 8, 6:00 – 8:00 PM ET."

#### **Investor call to review data on Monday, December 9**

Autolus management will host an investor conference call on Monday, December 9, at 8:30 a.m. EDT/ 1:30pm GMT, to review the data presented at ASH.

To listen to the webcast and view the accompanying slide presentation, please go to: <https://www.autolus.com/investor-relations/news-and-events/events>.

The call may also be accessed by dialing (866) 679-5407 for U.S. and Canada callers or (409) 217-8320 for international callers. Please reference conference ID 9796038. After the conference call, a replay will be available for one week. To access the replay, please dial (855) 859-2056 for U.S. and Canada callers or (404) 537-3406 for international callers. Please reference conference ID 9796038.

#### **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 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.

#### **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](http://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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