Autolus Therapeutics announces publication of obe-cel (AUTO1) Phase 1 ALLCAR19 data in adults with relapsed/ refractory B-ALL in Journal of Clinical Oncology

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JCO publication highlights durable responses and low toxicity following treatment with obe-cel

LONDON, Sept. 01, 2021 (GLOBE NEWSWIRE) -- Autolus Therapeutics plc (Nasdaq: AUTL), a clinical-stage biopharmaceutical company developing next-generation programmed T cell therapies, today announced publication of the obecabtagene autoleucel (obe-cel) Phase 1 ALLCAR19 data in Journal of Clinical Oncology[1]. Obe-cel is a fast off-rate CD19 CAR-T therapy, designed to reduce toxicity and improve engraftment[1,2]. ALLCAR19 is a clinical study in collaboration with Autolus' academic partner, UCL [NCT02935257].

"Currently there are no CD19 CAR T therapies approved for use in adult B-ALL and there exists a significant unmet need for r/r B-ALL patients," said Dr. Claire Roddie, Consultant Hematologist, UCL Cancer Institute and University College London Hospital. "We are very encouraged by the clinical profile of obe-cel, which demonstrates high sustained responses rates with remarkably little immunotoxicity despite high disease burden in many patients."

"We designed obe-cel, a unique rapid binding off rate CD19 CAR-T therapy, to specifically tackle the challenges of existing CD19 CAR T therapies, namely toxicity and lack of durable responses, by reducing the magnitude of T cell activation from each target cell interaction," said Dr. Martin Pulé, chief scientific officer of Autolus. "We have seen our design features play out in the clinical setting and these data further support our decision to progress obe-cel into the ongoing pivotal FELIX study [NCT04404660]."

Obe-cel demonstrated an excellent safety profile, with no patients experiencing high grade (≥ grade 3) cytokine release syndrome (CRS), despite the majority having a high disease burden prior to lymphodepletion[1]. In 8/20 patients developing grade 2 CRS, 7 patients received tocilizumab. No patients on study received corticosteroids for management of CRS[1]. Three of 20 patients experienced grade 3 immune effector cell-associated neurotoxicity syndrome (ICANS) and all resolved within 24-72 hours to grade 1 or less with corticosteroids[1].

CAR T cell concentration reached very high levels at peak and persistence in peripheral blood was evident in 15/20 (75%) patients at a median of 166.5 days, with 4/20 (20%) patients having follow-up duration over 2 years, and 3/4 of these with ongoing CAR persistence at data cut off[1]. Interestingly, B-cell aplasia was ongoing in 15/20 patients at last observation[1].

Of the 20 patients treated in the ALLCAR19 study, 85% patients achieved minimal residual disease (MRD) negative complete response (CR) at month 1[1]. Duration of response remains highly encouraging. With a data cut-off date of May 17, 2021 and as presented at the European Hematology Association (EHA) Virtual Congress in June 2021, event free survival (EFS) at 12 months and 24 months was 50.2%, with median EFS not reached across all patients treated[3].

Overall, obe-cel's profile of durability and favorable toxicity is consistently observed across the B cell malignancies tested so far, with data on a total of 50 patients reported to date, including patients with adult ALL[1] in ALLCAR19 [NCT02935257], pediatric ALL[2] [NCT02443831] and indolent B-cell lymphomas[3] [NCT02935257]. Alongside the FELIX study, a potential pivotal study ongoing in adult ALL, we continue to explore the profile of obe-cel in patients with other B-cell lymphomas through additional cohorts in the ALLCAR19 study and in patients with primary CNS lymphomas (PCNSL) through the CAROUSEL study [NCT04443829].

Citations (and hyperlinks)

1. Roddie et al. "Durable responses and low toxicity after fast off-rate CD19 CAR-T therapy in adults with relapsed/ refractory B-ALL." DOI: 10.1200/JCO.21.00917 Journal of Clinical Oncology - published online before print August 31, 2021
2. Ghorasian et al. "Enhanced CAR T cell expansion and prolonged persistence in pediatric patients with ALL treated with a low-affinity CD19 CAR." Nature Medicine volume 25, pages1408–1414(2019) (Sept 25, 2019)
3. Roddie et al. "Early Safety and Efficacy Findings of AUTO1 (CAT19), a Fast-Off Rate CD19 CAR, in Relapsed/Refractory Indolent B Cell Lymphomas." EHA annual meeting, June 11 2021, abstract EP788. EHA Investor slide presentation

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 Obe-cel (obecabtagene autoleucel)
Obe-cel is a CD19 CAR T cell investigational therapy designed to overcome the limitations in clinical activity and safety 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, Obe-cel may reduce

toxicity and be less prone to T cell exhaustion, which could enhance persistence and improve the ability of the programmed T cells to engage in serial killing of target cancer cells. In collaboration with our academic partner, UCL, Obe-cel is currently being evaluated in a Phase 1 clinical trial in adult ALL and B-NHL. The company has also progressed Obe-cel to the FELIX study, a potential pivotal study.

About Obe-cel FELIX study
The FELIX Phase 1b/2 clinical trial is enrolling adult patients with relapsed / refractory ALL. The trial has a short Phase 1b component prior to proceeding to a single arm Phase 2 clinical trial. The primary endpoint is overall response rate, and the key secondary endpoints include duration of response, MRD negative CR rate and safety. The trial will enroll approximately 100 patients across 30 of the leading academic and non-academic centers in the United States, United Kingdom and Europe. [NCT04404660]

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 future clinical development, efficacy, safety and therapeutic potential of obe-cel, including progress, expectations as to the reporting of data, conduct and timing and potential future clinical activity and milestones; expectations regarding the initiation, design and reporting of data from clinical trials. 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 4, 2021, 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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