Autolus Therapeutics Announces Positive CHMP Opinion for Obecabtagene Autoleucel for Adult Patients (age 26 and older) with Relapsed or Refractory B-Cell Precursor Acute Lymphoblastic Leukemia (R/R B-ALL)

May 23, 2025 at 8:30 AM EDT

- *Positive CHMP opinion based on FELIX clinical trial of obecabtagene autoleucel (obe-cel) in adult patients with r/r B-ALL, demonstrating high and durable response rates and low toxicity*
- *Opinion follows FDA approval and MHRA conditional marketing authorisation*
- *European Commission (EC) decision on conditional marketing authorization application (MAA) expected within approximately two months*

LONDON, May 23, 2025 (GLOBE NEWSWIRE) -- Autolus Therapeutics plc (Nasdaq: AUTL), an early commercial-stage biopharmaceutical company developing, manufacturing and delivering next-generation programmed T cell therapies and candidates, announces today that the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) has recommended European Commission (EC) approval of obecabtagene autoleucel (obe-cel) for the treatment of adult patients, 26+, with relapsed or refractory B-cell precursor acute lymphoblastic leukemia (r/r B-ALL).

"This positive CHMP opinion is a welcome advancement for physicians and patients in Europe, faced with treating r/r adult B-ALL patients with a poor prognosis," **said Dr. Claire Roddie, MD, PhD, FRCPath, Lead Investigator of the FELIX study and Associate Professor of Haematology at the University College London (UCL) Cancer Institute**. "Obe-cel's combination of favorable tolerability and potential long-term outcomes could offer an important new treatment option for patients in the EU."

The CHMP recommendation was based on the results of the FELIX study, an open-label, multi centre, single arm study in adult patients with relapsed or refractory B-cell acute lymphoblastic leukaemia. The results were published in the New England Journal of Medicine in November 2024[1]. In the pivotal cohort of patients, (cohort IIA (n=94)), the Complete Response/Complete Response with Incomplete Haematological Recovery (CR/CRi) for patients who received at least one infusion of obecabtagene autoleucel was 76.6%. Median response duration for all infused patients was 21.2 months. Median event-free survival (EFS) was 11.9 months and the estimated 6- and 12-month event-free survival rates were 65.4% and 49.5%, respectively.

The most common non-laboratory Grade 3 or higher adverse reactions were infections-pathogen unspecified (32%), febrile neutropenia (24%) and bacterial infectious disorders (11%). Cytokine release syndrome developed in 87 of the 127 patients (68.5%), with events of grade 3 or higher in three patients (2.4%). Immune effector cell-associated neurotoxicity syndrome developed in 29 of the 127 patients (22.8%), with grade 3 or higher occurring in nine patients (7%).

"This positive opinion from the CHMP highlights the significant unmet need and importance of effective treatment options for adult r/r B-ALL," **said Dr. Christian Itin, Chief Executive Officer of Autolus**. "With FDA approval received in November 2024 and an MHRA conditional marketing authorization received in April 2025, we are on our way to bringing this therapy to patients in need globally."

Obe-cel is an autologous CD19 CAR T cell therapy with a proprietary CD19 CAR, invented by a team led by Dr. Martin Pule, at University College London, along with collaborators at Great Ormond Street Hospital and University College London Hospital. The CAR is designed to have a fast "off-rate" which mimics physiological T-cell receptor interactions [2].

ALL is an aggressive type of blood cancer that can also involve the lymph nodes, spleen, liver, central nervous system and other organs. In Europe, there are approximately 6,000[2] new cases of ALL diagnosed every year. In frontline treatment for adult B-ALL, up to 50% of patients will ultimately relapse[3]. Survival rates remain very poor in adult patients with r/r ALL, with median overall survival of eight months with conventional treatments[4], and the standard-of-care treatment can trigger severe toxicities[5].

The positive CHMP opinion is a scientific recommendation for marketing authorization, serving as a basis for the EC's final decision on Autolus' MAA for obe-cel for adult r/r B-ALL patients. The EC is expected to make a decision following CHMP recommendation, and the decision will apply to all 27 European Union Member States, Iceland, Norway and Liechtenstein. Obe-cel is currently approved by the U.S. Food and Drug Administration (FDA) (Nov 8, 2024), and authorized by the U.K. Medicines and Healthcare products Regulatory Agency (MHRA) (April 25, 2025).

References

1. *Roddie C, et al "Obecabtagene autoleucel in B-cell acute lymphoblastic leukemia" N Engl J Med 2024; DOI: 10.1056/NEJMoa2406526*
2. https://www.sciencedirect.com/science/article/pii/S0006497120310946?via%3Dihub
3. Cancer Research UK - https://www.cancerresearchuk.org/health-professional/cancer-statistics/statistics-by-cancer-type/leukaemia-all/incidence
4. Aureli A, Marziani B, Venditti A, Sconocchia T, Sconocchia G. Acute lymphoblastic leukemia immunotherapy treatment:

now, next, and beyond. Cancers (Basel). 2023;15:3346.
5. Dhakal P, Kaur J, Gundabolu K, Bhatt VR. Immunotherapeutic options for management of relapsed or refractory B-cell acute lymphoblastic leukemia: how to select newly approved agents? Leuk Lymphoma. 2020;61:7-17.

About Autolus Therapeutics plc
Autolus Therapeutics plc (Nasdaq: AUTL) is an early commercial-stage biopharmaceutical company developing, manufacturing and delivering next-generation T cell therapies and candidates for the treatment of cancer and autoimmune disease. Using a broad suite of proprietary and modular T cell programming technologies, Autolus is engineering precisely targeted and controlled T cell therapies that are designed to better recognize target cells, break down their defense mechanisms and eliminate these cells. Autolus has an FDA approved and MHRA licensed product, obe-cel, and a pipeline of product candidates in development for the treatment of hematological malignancies, solid tumors and autoimmune diseases. For more information, please visit www.autolus.com.

About obe-cel FELIX clinical trial
Autolus' Phase 1b/2 clinical trial of obe-cel enrolled adult patients with r/r B-precursor ALL. The trial had a Phase 1b component prior to proceeding to the single arm, Phase 2 clinical trial. The primary endpoint in the pivotal cohort was overall response rate, and the secondary endpoints included duration of response, MRD negative complete remission rate and safety. The trial enrolled over 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 risks and uncertainties include, but are not limited to the impact of worsening macroeconomic conditions on Autolus' business, financial position, strategy and anticipated milestones, including Autolus' ability to conduct ongoing and planned clinical trials; Autolus' ability to obtain a clinical supply of current or future product candidates or commercial supply of obe-cel or any future approved products; Autolus' ability to obtain and maintain regulatory approval of its product candidates, including obe-cel and potential expansions into additional indications; Autolus' ability and plans in continuing to establish and expand a commercial infrastructure and to successfully launch, market and sell obe-cel and any future approved products; Autolus' ability to obtain marketing approval for obe-cel in additional geographies in the future; the delay of any current or planned clinical trials, whether due to patient enrollment delays or otherwise; Autolus' ability to successfully demonstrate the safety and efficacy of its product candidates and gain approval of its product candidates, including obe-cel and potential expansions into additional indications; Autolus' ability and plans in continuing to establish and expand a commercial infrastructure and to successfully launch, market and sell obe-cel and any future approved products; Autolus' ability to obtain marketing approval for obe-cel in additional geographies in the future; the delay of any current or planned clinical trials, whether due to patient enrollment delays or otherwise; Autolus' ability to successfully demonstrate the safety and efficacy of its product candidates and gain approval of its product candidates on a timely basis, if at all; competition with respect to market opportunities; and possible safety and efficacy concerns. 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 10-K filed with the Securities and Exchange Commission, or the SEC, on March 20, 2025 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. You should, therefore, not rely on these forward-looking statements as representing Autolus' views as of any date subsequent to the date of this press release.

Contact:

Amanda Cray
+1 617-967-0207
a.cray@autolus.com

Olivia Manser
+44 (0) 7780 471 568
o.manser@autolus.com