Autolus Therapeutics Announces License of AUCATZYL® (obecabtagene autoleucel) Issued by UK MHRA for Adult Patients (≥ 18 years) with Relapsed or Refractory B-Cell Precursor Acute Lymphoblastic Leukemia (R/R B-ALL)(1)

April 25, 2025 at 12:07 PM EDT

- *UK Medicines and Healthcare products Regulatory Agency (MHRA) authorisation based on FELIX clinical trial of obecabtagene autoleucel in adult patients with r/r B-ALL[1]*
- *AUCATZYL® licensed from MHRA under 'conditional marketing authorisation', meaning that the MHRA will review new efficacy and safety information at least once every year[1]*

Investors, US and UK National, Medical and Industry media only

LONDON, April 25, 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 UK Medicines and Healthcare products Regulatory Agency (MHRA) has granted conditional marketing authorisation for AUCATZYL® (obecabtagene autoleucel) for the treatment of adult patients with relapsed or refractory B-cell precursor acute lymphoblastic leukemia (r/r B-ALL).[1]

"Having treated a number of patients with AUCATZYL as part of the FELIX clinical trial, I am delighted that we have moved closer to eligible relapsed/refractory B-ALL patients being able to access AUCATZYL," **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**. "We now look forward to NICE completing its assessment of the medicine to potentially make it an option for eligible patients on the NHS."

"AUCATZYL was designed to address an unmet need for eligible adult r/r B-ALL patients and it is satisfying that is has been licensed in the country where it was created," **said Dr. Martin Pule, Chief Scientific Officer and Founder of Autolus.**

"Continuing our momentum, this MHRA license is a significant milestone for Autolus as a company. With our scientific expertise, operations and manufacturing based in the UK, this is an important achievement for our company," **said Dr. Christian Itin, Chief Executive Officer of Autolus**. "We want to thank all the patients and investigators at the UK trial centres for their contributions towards this license, as well as the foundational work by our partners at UCL and our internal team."

Obecabtagene autoleucel 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].

The MHRA authorisation of AUCATZYL 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 of which were published in the New England Journal of Medicine in November 2024[2]. Of the 153 r/r B-ALL patients enrolled in the FELIX study, 127 (83.0%) received at least one obecabtagene autoleucel infusion and were evaluable. 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%).

For further information regarding obecabtagene autoleucel, the Summary of Product Characteristics (SPC), including a full list of side effects and adverse reactions, is available here.

Autolus submitted obecabtagene autoleucel for appraisal by the National Institute for Health and Care Excellence (NICE)[3] in Q4 2024 and is working with NICE and the NHS to potentially achieve access for eligible patients in England. NICE provides guidance to the NHS in England on the clinical and cost-effectiveness of medicines, treatments, and technologies based on a rigorous process of evidence review and consultation with professionals and patients.

ALL is an aggressive type of blood cancer that can also involve the lymph nodes, spleen, liver, central nervous system and other organs. In the UK there are approximately 765[4] new cases of ALL diagnosed every year. In frontline treatment for adult B-ALL, up to 50% of patients will ultimately relapse[5]. Survival rates remain very poor in adult patients with r/r ALL, with median overall survival of eight months with conventional treatments[6], and the standard-of-care treatment can trigger severe toxicities[7].

References

1. Obecabtagene autoleucel Summary of Product Characteristics

2. *Roddie C, et al "Obecabtagene autoleucel in B-cell acute lymphoblastic leukemia" N Engl J Med 2024; DOI: 10.1056/NEJMoa2406526*
3. https://www.nice.org.uk/guidance/indevelopment/gid-ta11496
4. Cancer Research UK - https://www.cancerresearchuk.org/health-professional/cancer-statistics/statistics-by-cancer-type/leukaemia-all/incidence
5. https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7894150/
6. Aureli A, Marziani B, Venditti A, Sconocchia T, Sconocchia G. Acute lymphoblastic leukemia immunotherapy treatment: now, next, and beyond. Cancers (Basel). 2023;15:3346.
7. 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, AUCATZYL, 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 obecabtagene autoleucel FELIX clinical trial
Autolus' Phase 1b/2 clinical trial of obecabtagene autoleucel 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].

About AUCATZYL® (obecabtagene autoleucel, AUTO1)
AUCATZYL is a B-lymphocyte antigen CD19 (CD19) chimeric antigen receptor (CAR) T cell therapy. AUCATZYL is designed with a fast target binding off-rate to minimize excessive activation of the programmed T cells. AUCATZYL was approved by the FDA for the treatment of adult patients with relapsed or refractory B-cell precursor acute lymphoblastic leukemia on November 8, 2024, and licensed by the MHRA under conditional marketing authorisation on April 25 2025. In the EU a regulatory submission to the EMA was accepted in April 2024.
Please see full Summary of Product Characteristics.

About Conditional Marketing Authorisation
Conditional marketing authorisations (CMAs) are for medicines that fulfill a significant unmet medical need such as being for serious and life-threatening diseases, where no satisfactory treatment methods are available or where the medicine offers a major therapeutic advantage. A CMA is granted where comprehensive clinical data is not yet complete, but it is judged that such data will become available soon. CMAs are valid for one year and renewable annually with ongoing regulatory review of data.

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 Autolus' development and commercialization of its products and 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; 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 7780 471 568
o.manser@autolus.com

UK-AUC-0026 I APRIL 2025