



Autolus Therapeutics Highlights Advancing Autoimmune Pipeline at R&D Investor Event

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- *Company outlined potential for value creation driven by obe-cel across multiple B cell driven malignancies and autoimmune diseases, including acute lymphoblastic leukemia (ALL), lupus nephritis (LN) and multiple sclerosis (MS)*
- *Preliminary data in initial six patient cohort treated in Phase 1 trial in systemic lupus erythematosus (SLE) support progressing obe-cel into a planned Phase 2 pivotal study in lupus nephritis; first patient expected to be dosed in Phase 2 trial by year-end 2025*
- *Company plans to advance obe-cel in progressive forms of multiple sclerosis (MS); first patient expected to be dosed in Phase 1 trial by year-end 2025*

LONDON, April 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, today presented updates on its development pipeline and plans for expansion in autoimmune diseases at an R&D investor event.

The Company presented development plans to expand the obe-cel opportunity into autoimmune disease, including a potential registrational path for obe-cel in LN. They were joined by key opinion leaders David Isenberg, MD, FRCP, FAMS, Emeritus Professor of the University College of London (UCL) Centre for Ageing, Rheumatology and Regenerative Medicine, and Mark Freedman, HBSc, MSc, MD, CSPQ, FAAN, FRCPC, Professor of Medicine (Neurology) at the University of Ottawa and Director of the Multiple Sclerosis Research Unit. Drs. Isenberg and Freedman shared insights on the current clinical landscape, B cell depletion approaches and the significant unmet medical need in LN and MS.

"We believe that obe-cel could be a 'pipeline in a product' with the potential to deliver improved outcomes for patients and significant value creating opportunities for Autolus. We are building on obe-cel's recent approval in the U.S. for the treatment of adult patients with relapsed or refractory B cell precursor ALL," **said Dr. Christian Itin, Chief Executive Officer of Autolus.** "We have extensive clinical trial experience over several years, and data consistently show that obe-cel delivers a deep reset in the B cell compartment.^{1,2,3,4} Supported by our proprietary manufacturing, supply chain and commercial infrastructure, we have a strong foundation and are well-positioned to unlock the full value of this asset."

"Lupus patients who have failed treatment with B cell targeting agents and calcineurin inhibitors require new therapeutic options," **said David Isenberg, MD, FRCP, FAMS, Emeritus Professor of Rheumatology at University College London.** "Although a limited dataset, the CARLYSLE trial has shown encouraging clinical data in this advanced and relapsed patient population with lupus nephritis. Further evaluation to confirm these promising findings, including the favorable safety profile, are urgently warranted."

R&D Event Updates

- *Phase 1 data support plans to move into pivotal Phase 2 trial in SLE*
 - Preliminary data in six patients from the Phase 1 dose confirmation clinical trial (CARLYSLE) in refractory systemic lupus erythematosus (SLE) patients support progressing into a planned Phase 2 pivotal study. Three patients had complete renal response, all by month three. Complement normalized in all patients by month one. Rash, alopecia and mucosal ulcers resolved by month three and arthritis resolved by month one in all patients. Data show high peak expansion and deep B cell aplasia consistent with known obe-cel characteristics in oncology indications. No dose limiting toxicities (DLTs) or immune effector cell-associated neurotoxicity syndrome (ICANS) were observed in the study to date. Grade one cytokine release syndrome (CRS) was observed in three out of six patients. Transient hypertension, including grade three, occurred in five out of six patients, in one case with transient worsening of kidney function. Three patients had pre-existing hypertension at baseline.
 - The Company has aligned with U.S. Food and Drug Administration (FDA) on the Phase 2 trial design and potential registrational path to approval and anticipates dosing the first patient in a Phase 2 trial before the end of the year.
 - Full data with longer term follow-up from the Phase 1 CARLYSLE clinical trial is targeted for presentation at a medical conference in the second half of 2025.
- *Expanding the obe-cel opportunity in autoimmune; Phase 1 trial in progressive MS*
 - Autolus plans to advance obe-cel into clinical development in progressive MS. The Company expects to dose its first patient in a Phase 1 dose escalation study by year-end 2025.
- *Early-stage pipeline programs and collaborations support longer-term growth*
 - Autolus' translational programs with UCL continue to fuel its early-stage pipeline, providing a cost-efficient path to

development.

- **AUCATZYL® US launch progressing on track**
 - Autolus is executing on AUCATZYL's launch in r/r adult B-ALL with 38 centers fully activated as of April 22, 2025. AUCATZYL first quarter sales will be reported at the Company's Q1 2025 financial results on May 8, 2025.
 - Obe-cel is under regulatory review in both the EU and the U.K., and the Company expects to receive notification of approval status from the Medicines and Healthcare products Regulatory Agency (MHRA) in Q2 2025 and European Medicines Agency (EMA) in H2 2025.

Summary of Anticipated News Flow:

ALL - Notifications from UK MHRA regarding approval in r/r adult ALL	Q2 2025
ALL – Notification from EU EMA regarding approval in r/r adult ALL	H2 2025
ALL - Initial data from PY01 trial in pediatric ALL	H2 2025
SLE - Phase 1 CARLYSLE trial presentation at medical conference	H2 2025
LN – Expect to dose first patient in Phase 2 trial	By year-end 2025
MS – Expect to dose first patient in Phase 1 trial in progressive MS	By year-end 2025

R&D Event Webcast Details:

A replay of the webcast of the R&D investor event is available on the "Events" page in the "Investor Relations & Media" section of the Company's website at <https://www.autolus.com/investor-relations-media/events/>.

Q1 2025 Financial Results Conference Call:

Management will host a conference call and webcast at 8:30 am EDT/13:30 pm BST on May 8, 2025, to discuss the Company's first quarter financial results and provide a general business update. Conference call participants should pre-register using this [link](#) to receive the dial-in numbers and a personal PIN, which are required to access the conference call. A simultaneous audio webcast and replay will be accessible on the [events section](#) of Autolus' website.

References:

1. Roddie C, et al "Obecabtagene autoleucel in B cell acute lymphoblastic leukemia" N Engl J Med 2024; DOI: 10.1056/NEJMoa2406526
2. Ghorashian, S., Kramer, A.M., Onuoha, S. et al. Enhanced CAR T cell expansion and prolonged persistence in pediatric patients with ALL treated with a low-affinity CD19 CAR. Nat Med 25, 1408–1414 (2019). <https://doi.org/10.1038/s41591-019-0549-5>
3. Roddie C, et al. J Clin Oncol 2023;41:16_suppl, 7000
4. Roddie et al, ASH 2023, Poster 2114

About Autolus Therapeutics plc

Autolus Therapeutics plc (Nasdaq: AUTL) is an early commercial biopharmaceutical company developing, manufacturing and delivering next-generation T cell therapies 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, controlled and highly active 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 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 AUCATZYL® (obecabtagene autoleucel, obe-cel, AUTO1)

AUCATZYL is a B-lymphocyte antigen CD19 (CD19) chimeric antigen receptor (CAR) T cell therapy designed to overcome the limitations in clinical activity and safety compared to current CD19 CAR T cell therapies. 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. In the EU, a regulatory submission to the EMA for AUCATZYL was accepted in April 2024, and in the UK, an MAA was submitted to MHRA for AUCATZYL in July 2024.

INDICATION

AUCATZYL® is a CD19-directed genetically modified autologous T cell immunotherapy indicated for the treatment of adult patients with relapsed or refractory B cell precursor acute lymphoblastic leukemia (ALL).

IMPORTANT SAFETY INFORMATION

WARNING: CYTOKINE RELEASE SYNDROME, NEUROLOGIC TOXICITIES, and SECONDARY HEMATOLOGICAL MALIGNANCIES

- **Cytokine Release Syndrome (CRS) occurred in patients receiving AUCATZYL. Do not administer AUCATZYL to patients with active infection or inflammatory disorders. Prior to administering AUCATZYL, ensure that healthcare providers have immediate access to medications and resuscitative equipment to manage CRS.**
- **Immune Effector Cell-Associated Neurotoxicity Syndrome (ICANS), including fatal and life-threatening reactions, occurred in patients receiving AUCATZYL, including concurrently with CRS or after CRS resolution. Monitor for neurologic signs and**

symptoms after treatment with AUCATZYL. Prior to administering AUCATZYL, ensure that healthcare providers have immediate access to medications and resuscitative equipment to manage neurologic toxicities. Provide supportive care and/or corticosteroids, as needed.

- **T cell malignancies have occurred following treatment of hematologic malignancies with BCMA- and CD19-directed genetically modified autologous T cell immunotherapies.**

WARNINGS AND PRECAUTIONS

Cytokine Release Syndrome (CRS)

Cytokine Release Syndrome (CRS) occurred following treatment with AUCATZYL. CRS was reported in 75% (75/100) of patients including Grade 3 CRS in 3% of patients. The median time to onset of CRS was 8 days following the first infusion (range: 1 to 23 days) with a median duration of 5 days (range: 1 to 21 days). The most common manifestations of CRS included fever (100%), hypotension (35%), and hypoxia (19%).

Prior to administering AUCATZYL, ensure that healthcare providers have immediate access to medications and resuscitative equipment to manage CRS. During and following treatment with AUCATZYL, closely monitor patients for signs and symptoms of CRS daily for at least 14 days at the healthcare facility following the first infusion. Continue to monitor patients for CRS for at least 4 weeks following each infusion with AUCATZYL. Counsel patients to seek immediate medical attention should signs or symptoms of CRS occur at any time. At the first sign of CRS, immediately evaluate the patient for hospitalization and institute treatment with supportive care based on severity and consider further management per current practice guidelines.

Neurologic Toxicities

Neurologic toxicities including Immune Effector Cell-associated Neurotoxicity Syndrome (ICANS), which were fatal or life-threatening, occurred following treatment with AUCATZYL. Neurologic toxicities were reported in 64% (64/100) of patients, including Grade ≥ 3 in 12% of patients. The median time to onset of neurologic toxicities was 10 days (range: 1 to 246 days) with a median duration of 13 days (range: 1 to 904 days). Among patients with neurologic toxicities, the most common symptoms ($> 5\%$) included ICANS (38%), headache (34%), encephalopathy (33%), dizziness (22%), tremor (13%), anxiety (9%), insomnia (9%), and delirium (8%).

Immune Effector Cell-associated Neurotoxicity Syndrome (ICANS)

ICANS events occurred in 24% (24/100) of patients, including Grade ≥ 3 in 7% (7/100) of patients. Of the 24 patients who experienced ICANS, 33% (8/24) experienced an onset after the first infusion, but prior to the second infusion of AUCATZYL.

The median time to onset for ICANS events after the first infusion was 8 days (range: 1 to 10 days) and 6.5 days (range: 2 to 22 days) after the second infusion, with a median duration of 8.5 days (range: 1 to 53 days).

Eighty-eight percent (21/24) of patients received treatment for ICANS. All treated patients received high-dose corticosteroids and 42% (10/24) of patients received anti-epileptics prophylactically. Prior to administering AUCATZYL, ensure that healthcare providers have immediate access to medications and resuscitative equipment to manage ICANS.

Counsel patients to seek medical attention should signs or symptoms of neurologic toxicity/ ICANS occur. At the first sign of Neurologic Toxicity /ICANS, immediately evaluate patients for hospitalization and institute treatment with supportive care based on severity and consider further management per current practice guidelines.

Effect on Ability to Drive and Use Machines

Due to the potential for neurologic events, including altered mental status or seizures, patients receiving AUCATZYL are at risk for altered or decreased consciousness or coordination in the eight weeks following AUCATZYL infusion or until resolution of the neurological event by the treating physician. Advise patients to refrain from driving and engaging in hazardous occupations or activities, such as operating heavy or potentially dangerous machinery, during this initial period.

Prolonged Cytopenias

Patients may exhibit cytopenias including anemia, neutropenia, and thrombocytopenia for several weeks after treatment with lymphodepleting chemotherapy and AUCATZYL. In patients who were responders to AUCATZYL, Grade ≥ 3 cytopenias that persisted beyond Day 30 following AUCATZYL infusion were observed in 71% (29/41) of patients and included neutropenia (66%, 27/41) and thrombocytopenia (54%, 22/41). Grade 3 or higher cytopenias that persisted beyond Day 60 following AUCATZYL infusion was observed in 27% (11/41) of patients and included neutropenia (17%, 7/41) and thrombocytopenia (15%, 6/41). Monitor blood counts after AUCATZYL infusion.

Infections

Severe, including life-threatening and fatal infections occurred in patients after AUCATZYL infusion. Non-COVID-19 infections of all grades occurred in 67% (67/100) of patients. Grade 3 or higher non-COVID-19 infections occurred in 41% (41/100) of patients. AUCATZYL should not be administered to patients with clinically significant active systemic infections. Monitor patients for signs and symptoms of infection before and after AUCATZYL infusion and treat appropriately. Administer prophylactic antimicrobials according to local guidelines. Grade 3 or higher febrile neutropenia was observed in 26% (26/100) of patients after AUCATZYL infusion and may be concurrent with CRS. In the event of febrile neutropenia, evaluate for infection and manage with broad-spectrum antibiotics, fluids, and other supportive care as medically indicated. Viral reactivation, potentially severe or life-threatening, can occur in patients treated with drugs directed against B cells. There is no experience with manufacturing AUCATZYL for patients with a positive test for human immunodeficiency virus (HIV) or with active hepatitis B virus (HBV) or active hepatitis C virus (HCV). Perform screening for HBV, HCV and HIV in accordance with clinical guidelines before collection of cells for manufacturing.

Hypogammaglobulinemia

Hypogammaglobulinemia and B cell aplasia can occur in patients after AUCATZYL infusion. Hypogammaglobulinemia was reported in 10% (10/100) of patients treated with AUCATZYL including Grade 3 events in 2 patients (2%). Immunoglobulin levels should be monitored after treatment with AUCATZYL and managed per institutional guidelines including infection precautions, antibiotic or antiviral prophylaxis, and immunoglobulin replacement. The safety of immunization with live viral vaccines during or following treatment with AUCATZYL has not been studied. Vaccination with live viral vaccines is not recommended for at least 6 weeks prior to the start of lymphodepleting chemotherapy treatment, during AUCATZYL treatment, and until immune recovery following treatment with AUCATZYL.

Hemophagocytic Lymphohistiocytosis/Macrophage Activation Syndrome (HLH/MAS)

HLH/MAS including fatal and life-threatening reactions occurred after treatment with AUCATZYL. HLH/MAS was reported in 2% (2/100) of patients and included Grade 3 and Grade 4 events with a time of onset at Day 22 and Day 41, respectively. One patient experienced a concurrent ICANS events after AUCATZYL infusion and died due to sepsis with ongoing HLH/MAS that had not resolved. Administer treatment for HLH/MAS according to institutional standards.

Hypersensitivity Reactions

Serious hypersensitivity reactions, including anaphylaxis, may occur due to dimethyl sulfoxide (DMSO), an excipient used in AUCATZYL. Observe patients for hypersensitivity reactions during and after AUCATZYL infusion. Secondary Malignancies Patients treated with AUCATZYL may develop secondary malignancies. T cell malignancies have occurred following treatment of hematologic malignancies with BCMA- and CD19-directed genetically modified autologous T cell immunotherapies. Mature T cell malignancies, including CAR-positive tumors, may present as soon as weeks following infusion, and may include fatal outcomes. Monitor lifelong for secondary malignancies. In the event that a secondary malignancy occurs, contact Autolus at 1-855-288-5227 for reporting and to obtain instructions on the collection of patient samples for testing.

Adverse Reactions

The safety of AUCATZYL was evaluated in the FELIX study in which 100 patients with relapsed or refractory B cell acute lymphoblastic leukemia (B-ALL) received AUCATZYL at a median dose of 410×10^6 CD19 CAR-positive viable T cells (range: 10 to 480×10^6 CD19 CAR-positive viable T cells with 90% of patients receiving the recommended dose of 410×10^6 +/- 25%).

The most common serious adverse reactions of any Grade (incidence \geq 2%) included infections-pathogen unspecified, febrile neutropenia, ICANS, CRS, fever, bacterial infectious disorders, encephalopathy, fungal infections, hemorrhage, respiratory failure, hypotension, ascites, HLH/MAS, thrombosis and hypoxia. Nine patients (9%) experienced fatal adverse reactions which included infections (sepsis, pneumonia, peritonitis), ascites, pulmonary embolism, acute respiratory distress syndrome, HLH/MAS and ICANS. Of the 9 patients, five patients who died from infections had pre-existing and ongoing neutropenia prior to receiving bridging therapy, lymphodepletion chemotherapy treatment and/or AUCATZYL.

Please see full [Prescribing Information](#), including **BOXED WARNING** and Medication Guide.

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 therapeutic potential and expected clinical benefits of AUCATZYL/obe-cel (obecabtagene autoleucel) for adult patients with r/r B-ALL; Autolus' ability to generate revenues from AUCATZYL, which is dependent upon maintaining significant market acceptance among physicians, patients and healthcare payors; Autolus' ability to obtain and maintain regulatory approval for obe-cel for adult r/r B-ALL in additional territories and the timing thereof; expectations regarding the commercialization and marketing of AUCATZYL for adult r/r B-ALL, including expanding into additional territories and the related timing of reaching patients in such territories; the development of additional product candidates, including statements regarding the initiation, timing, progress and the results of clinical studies or trials and related preparatory work, the period during which the results of the trials will become available and our research and development programs; commercialization, marketing and manufacturing capabilities and strategy for AUCATZYL; the timing or likelihood of regulatory filings and approvals for product candidates, along with regulatory developments in the US, EU, the UK and other foreign countries; size and growth potential of the markets for AUCATZYL and product candidates, if approved; plans to collaborate, or statements regarding our current collaborations with BioNTech and others; and estimates regarding expenses, future revenue, capital requirements and needs for additional financing. 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 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 AUCATZYL or any future approved products; Autolus' ability to obtain and maintain regulatory approval of its product candidates, including AUCATZYL and potential expansions into additional indications; Autolus' ability and plans in continuing to establish and expand a commercial infrastructure in the US and to successfully launch, market and sell AUCATZYL and any future approved products; Autolus' ability to successfully expand the approved indications for AUCATZYL or obtain marketing approval for AUCATZYL 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; the risk 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; 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.

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