Autolus Therapeutics Presents Initial Clinical Data in Pediatric r/r B-ALL Patients and Other Oncology Data at the American Society of Hematology (ASH) Annual Meeting 2025

December 8, 2025 at 8:35 AM EST

- *Obe-cel demonstrates high remission rates in pediatric patients with high-risk r/r B-ALL with overall response rate (ORR) of 95.5%; low rates of high-grade cytokine release syndrome (CRS) and immune effector cell-associated neurotoxicity syndrome (ICANS) observed, consistent with obe-cel's adult safety profile*
- *FELIX study data analyses highlight product cell phenotype and level of CAR T persistence at three months as potential predictors of long-term remission*
- *Real-world data independently collected by the ROCCA consortium during the US commercial launch of obe-cel were presented and show a high response rate and low levels of high-grade CRS and ICANS consistent with the clinical trial experience in the FELIX study*

LONDON and GAITHERSBURG, Md., Dec. 08, 2025 (GLOBE NEWSWIRE) -- Autolus Therapeutics plc (Nasdaq: AUTL), an early commercial-stage biopharmaceutical company developing, manufacturing and delivering next-generation programmed T cell therapies, announces presentation of preliminary data from the CATULUS Phase 1 trial of obe-cel in pediatric relapsed or refractory (r/r) B-ALL patients, as well as further insights from the registrational FELIX study in adult r/r B-ALL, at the American Society of Hematology (ASH) Annual Meeting.

Dr. Matthias Will, Chief Development Officer of Autolus, said: "Pediatric patients with r/r B-ALL have a poor prognosis, particularly those who relapse early. We were pleased to share the first data from the Phase 1 CATULUS trial showing obe-cel can produce high remission rates in this pediatric patient population, including in patients with high-risk relapse and patients with primary CNS relapse. Consistent with our experience in the adult population, data show low rates of severe CRS and ICANS. We are now advancing into the Phase 2 portion of the study in line with our commitment to address the significant unmet need for new treatment options for pediatric patients with r/r ALL."

Dr. Will continued, "Insights from post-hoc analyses from our FELIX pivotal trial in r/r adult B-ALL explored various factors that may help to predict long-term patient outcomes. Specifically, investigators showed that detection of obe-cel in the blood three months post-treatment may be a predictor for long-term outcomes. They also identified characteristics of the product's cell phenotype as additional factors for treatment outcomes."

He concluded, "In addition to Autolus' presentations, we were highly encouraged by data from the real-world experience of the ROCCA consortium evaluating CAR T therapy for r/r adult ALL patients. These real world data mirror obe-cel's safety profile observed in the pivotal FELIX trial with low single digits rates of CRS and ICANS as one of the differentiating characteristics of the therapy."

Abstract 740 – Poster presentation
Title: Treatment of pediatric patients (pts) with relapsed/refractory B-cell acute lymphoblastic leukemia (R/R B-ALL) with obecabtagene autoleucel (obe-cel), a CD19-directed chimeric antigen receptor (CAR) T-cell therapy: preliminary findings from the Phase Ib/II CATULUS trial
Session Name: Acute Lymphoblastic Leukemias: Therapies Excluding Allogeneic Transplantation: Poster II
Session Date and Time: December 7, 2025; 6:00 – 8:00pm ET
Session Room: Orange County Convention Center; West Halls B3-B4
Publication Number: 3337
Presenting Author: Sara Ghorashian, Consultant Haematologist at Great Ormand Street Hospital for Children (GOSH) and Honorary Associate Professor at UCL

Summary: CATULUS is a single-arm, open-label, multi-center study enrolling high-risk patients under age 18 with r/r B-ALL that is primary refractory, in high-risk first relapse, or in second or later relapse. The safety profile of obe-cel in pediatric patients was consistent with that previously reported in adults, with low rates of high-grade CRS and ICANS (both 8.7%). The ORR was high at 95.5% (n=21), with 90.9% (n=20) achieving complete response (CR). Twenty patients were in ongoing remission at data cut-off with a median follow-up of 8.8 months. These preliminary findings support further exploration of obe-cel in pediatric R/R B-ALL and planning for the Phase II expansion is underway.

Abstract 4060 – Poster presentation
Title: Chimeric antigen receptor (CAR) T-cell persistence at Month 3 predicts clinical outcomes in adult patients with relapsed/refractory B-cell acute lymphoblastic leukemia (R/R B-ALL) treated with obecabtagene autoleucel (obe-cel)
Session Name: Acute Lymphoblastic Leukemias: Therapies Excluding Allogeneic Transplantation: Poster III
Session Date and Time: December 8, 2025; 6:00 – 8:00pm ET
Session Room: Orange County Convention Center; West Halls B3-B4
Publication Number: 5118
Presenting Author: Claire Roddie, MD, PhD, FRCPath, Associate Professor Haematology and Honorary Consultant Haematologist, Cancer Institute, University College London (UCL)

Summary: Of 99 patients who achieved remission (CR/CRi) in the FELIX study, 79 (79.8%) had ongoing remission at month three following obe-cel infusion and comprised the subgroup of interest for the analyses. At month three post infusion, 60/79 patients (75.9%) had ongoing CAR T-cell persistence, while 19/79 patients (24.1%) had loss of persistence. In patients who remained in remission beyond month three, including those with

deep MRD-negative remission and no post obe-cel SCT, ongoing CAR T-cell persistence at month three, measured by droplet digital PCR (ddPCR), was associated with longer event-free survival (EFS) and overall survival (OS) compared with loss of persistence. These results suggest that persistence status at month three may be a marker for predicting long-term outcomes following obe-cel treatment in patients with r/r B-ALL.

Abstract 4031 – Poster presentation
Title: Evaluation of commercially available chimeric antigen receptor (CAR) detection reagents for monitoring of CAR T-cell (CAR T) expansion and persistence in patients (pts) treated with obecabtagene autoleucel (obe-cel)
Session Name: Cellular Immunotherapies: Early Phase Clinical Trials and Toxicities: Poster I
Session Date and Time: December 6, 2025; 5:30 – 7:30pm ET
Session Room: Orange County Convention Center; West Halls B3-B4
Publication Number: 2367
Presenting Author: Rehan Hussain, Translational Medicine Senior Scientist

Summary: Measuring obe-cel expansion and persistence using flow cytometry (FC) is feasible with commercially available antibodies that directly target regions of the CAR construct, such as the G4S linker. These reagents show high correlation with anti-idiotype antibodies and provide a reliable method for tracking CAR expression in patients. Use of the G4S binder enabled tracking of CAR T expansion kinetics and phenotypic profiles in patients with different disease burdens. Reagents based on the CD19 protein, commonly used in other CAR T therapies, are unsuitable for obe-cel due to the unique features of the CAT19 binder, which limits effective detection.

Abstract 4429 – Oral presentation
Title: Impact of chimeric antigen receptor (CAR) product cell phenotypes on clinical outcomes following treatment with obecabtagene autoleucel (obe-cel)
Session Name: Acute Lymphoblastic Leukemias: Biomarkers, Molecular Markers, and Measurable Residual Disease in Diagnosis and Prognosis: Prognostic Genetic and Therapeutic Response Factors in Adult and Pediatric B-ALL
Session Date and Time: December 6, 2025; 10:00 – 10:15am ET
Session Room: Orange County Convention Center; W224CDGH
Publication Number: 33
Presenting Author: Benjamin Simpson, Ph.D., Bioinformatics & Data Management Principal Scientist, Autolus Therapeutics

Summary: Clinical data show the potential for obe-cel to produce long-term outcomes. This analysis details certain product features potentially affecting clinical outcomes, including how drug product phenotypes correlate with treatment outcomes following infusion with obe-cel. A higher percentage of central memory cells (Tcm) in the drug product samples was an independent predictor of positive clinical outcomes, including overall survival (OS), following obe-cel infusion. While the T-cell phenotype composition in the leukapheresis product (LP) was weakly correlated with that in the drug product, CD25+ HLADR+ CD4+ cells in the LP independently predicted less favorable clinical outcomes. However, other factors (e.g. tumor characteristics) are also likely to affect outcomes; therefore, further investigations are needed to better understand and predict favorable clinical outcomes, and to potentially guide studies of additional cell manipulations during CAR T-cell manufacturing.

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 a marketed therapy, 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)
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 in November 2024, and was granted conditional marketing authorization by MHRA in the UK and EMA in the EU in 2025.

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 *[see Dosage and Administration (2.2,2.3), Warnings and Precautions (5.1)]*.**

- **Immune Effector Cell-Associated Neurotoxicity Syndrome (ICANS), including fatal or 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 *[see Dosage and Administration (2.2,2.3), Warnings and Precautions (5.2)]*.**

- **T cell malignancies have occurred following treatment of hematologic malignancies with BCMA- and CD19-directed genetically modified autologous T cell immunotherapies *[see Warnings and Precautions (5.8)].***

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 (range: 1 to 23 days) with a median duration of 5 days (range: 1 to 21 days). Sixty-eight percent of patients (51/75) experienced CRS after the first infusion, but prior to the second infusion of AUCATZYL with a median time to onset of 6 days (range: 1 to 10 days). Among patients with CRS, the most common manifestations of CRS included fever (100%), hypotension (35%) and hypoxia (19%). The primary treatment for CRS was tocilizumab (73%; 55/75), with patients also receiving corticosteroids (21%; 16/75).

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 7 days following each infusion. Continue to monitor patients for CRS for at least 2 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). Fifty-five percent of patients (35/64) experienced neurologic toxicities after the first infusion but prior to the second infusion of AUCATZYL with a median time to onset of 6 days (range: 1 to 11 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.

During and following AUCATZYL administration, closely monitor patients for signs and symptoms of Neurologic Toxicity/ICANS. Following treatment with AUCATZYL, monitor patients daily for at least 7 days. Continue to monitor patients for at least 2 weeks following treatment with AUCATZYL. Avoid driving for at least 2 weeks after each infusion. 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.

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 ≥ 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 obecabtagene autoleucel (obe-cel); the period during which the results of clinical studies or trials will become available; 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. 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; 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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