



Autolus Therapeutics Receives EMA Orphan Medical Product Designation for obe-cel for Treatment of Acute Lymphoblastic Leukemia

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LONDON, March 31, 2022 (GLOBE NEWSWIRE) -- Autolus Therapeutics plc (Nasdaq: AUTL), a clinical-stage biopharmaceutical company developing next-generation programmed T cell therapies, today announced that the European Medicines Agency (EMA) has granted obe-cel, Autolus' leading CAR T clinical candidate, Orphan Medical Product Designation for treatment of acute lymphoblastic leukemia (ALL) patients.

"This regulatory designation is an important milestone toward addressing the urgent, unmet need in ALL patients, and follows the Orphan Drug Designation previously granted in 2019 by the U.S. Food and Drug Administration (FDA) for obe-cel," said Dr. Christian Itin, Chief Executive Officer of Autolus. "Recruitment is ongoing in the Phase 2 portion of the pivotal study of obe-cel and we look forward to announcing first Phase 2 data this year."

Orphan Medical Product Designation is granted by the EMA Committee for Orphan Medicinal Products to medicines intended for the treatment, diagnosis or prevention of a disease that is life-threatening or chronically debilitating that affects fewer than 5 in 10,000 people in the EU under the Orphan Regulation (Regulation (EC) No 141/2000). To attain Orphan Designation, it must be justified that a medicinal product could potentially be of significant benefit compared to the existing authorized medicinal products for those affected by the condition. Benefits of the designation include fee reductions for centralized activities including; applications for marketing authorization, inspections and scientific advice. A key benefit is ten years of market exclusivity in the EU following marketing approval by the EMA.

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 (AUTO1)

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 Autolus' academic partner, UCL, obe-cel is currently being evaluated in a Phase 1 clinical trials for B-NHL. Autolus has progressed obe-cel to the FELIX trial, a potential pivotal trial for adult ALL.

About obe-cel FELIX clinical trial

Autolus' Phase 1b/2 clinical trial of obe-cel is enrolling adult patients with relapsed / refractory B-precursor ALL. The trial had a Phase 1b component prior to proceeding to the single arm, Phase 2 clinical trial. The primary endpoint is overall response rate, and the secondary endpoints include duration of response, MRD negative CR rate and safety. The trial is designed to 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 Autolus' development of the obe-cel program; the future clinical development, efficacy, safety and therapeutic potential of its product candidates, 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; expectations regarding regulatory approval process for any product candidates; the collaboration between Autolus and Blackstone; the discovery, development and potential commercialization of potential product candidates including obe-cel using Autolus' technology and under the collaboration agreement; the therapeutic potential for Autolus in next generation product developments of obe-cel in B-cell malignancies; the potential and timing to receive milestone payments and pay royalties under the strategic collaboration; and the Company's anticipated cash runway. 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 10, 2022, 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.

Contact:

Olivia Manser
+44 (0) 7780 471568
o.manser@autolus.com

Julia Wilson
+44 (0) 7818 430877
j.wilson@autolus.com

Susan A. Noonan
S.A. Noonan Communications
+1-917-513-5303
susan@sanoonan.com