Autolus Therapeutics Reports Third Quarter 2023 Financial Results and Business Updates

November 2, 2023 at 7:12 AM EDT

- *Obe-cel, a potentially transformational treatment for relapsed/refractory (r/r) adult B-cell Acute Lymphoblastic Leukemia (ALL), on track for a Biologics License Application (BLA) submission to the US Food & Drug Administration (FDA) by end of 2023*
- *Updated clinical data at the American Society of Hematology (ASH) meeting in December 2023: Long-term follow-up and sub-group analysis from the FELIX trial of obe-cel and initial data from a study of AUTO8 in multiple myeloma*
- *Continued progress with commercial manufacturing facility, The Nucleus, with process performance qualification production phase complete and data package on track to support BLA submission*
- *Obe-cel Phase 1 study in refractory systemic lupus erythematosus (SLE) expected to initiate in early 2024*
- *Conference call to be held today at 09:00 am EDT/1:00 pm GMT: Conference call participants should pre-register using the link at the bottom of this press release*

LONDON, Nov. 02, 2023 (GLOBE NEWSWIRE) -- Autolus Therapeutics plc (Nasdaq: AUTL), a clinical-stage biopharmaceutical company developing next-generation programmed T cell therapies, today announced its operational and financial results for the quarter ended September 30, 2023.

“The coming months will be an exciting and instrumental time for Autolus as we plan for our first BLA submission for obe-cel with the US FDA by year end and we continue with our preparatory activities for a planned commercial launch in 2024, pending the necessary regulatory approvals,” **said Dr. Christian Itin, Chief Executive Officer of Autolus.** “The data we have observed to date in the FELIX study highlights the potential of obe-cel to deliver a high complete remission rate combined with low levels of immunotoxicity and excellent CAR T expansion and persistence in adult patients with relapsed/refractory ALL. We look forward to building on the positive data when we present longer term follow up and additional subgroup analysis data at ASH in December.”

“Alongside this, our commercial manufacturing facility, The Nucleus, completed process performance qualification activities in Q4 to support the BLA submission, and we believe we are in a strong position operationally to deliver product and meet the global demand for adult ALL treatment.”

“Obe-cel’s excellent risk/benefit profile combined with its ability to make deep cuts into the CD19+ cell compartment, as illustrated by MRD-negative complete remissions in adult and pediatric ALL patients, is a strong foundation for the development of obe-cel in autoimmune disease. The clinical profile together with our commercial product delivery base, attractive cost of goods, and commercial infrastructure should help to drive an accelerated and differentiated expansion of the obe-cel opportunity. We are excited at the growing body of evidence that CAR T can address a significant unmet need in autoimmune disease, and we look forward to starting a Phase I study of obe-cel in refractory systemic lupus erythematosus (SLE) patients in early 2024.”

Key obecabtagene autoleucel (obe-cel) updates and anticipated milestones:

- *Obe-cel in relapsed / refractory (r/r) adult ALL – The FELIX Study*
  - Longer term follow-up data and subgroup analysis data will be presented at ASH in December 2023, as well as at medical conferences in H1 2024.
  - BLA submission is on track to the FDA by the end of 2023 and a submission of a marketing authorization application to the European Medicines Agency (EMA) in the first half of 2024.
- *Obe-cel in B-cell mediated autoimmune diseases*
  - Phase 1 study in refractory SLE patients is on track to start in early 2024, with initial clinical data expected in late 2024.

Pipeline clinical trials, in collaboration with University College London (UCL), updates and anticipated milestones

- *AUTO1/22 in pediatric B-ALL patients – Phase 1 CARPALL Study*
  - The data presented at European Society for Blood and Marrow Transplantation (EBMT) in April 2023 on the AUTO1/22 Phase 1 CARPALL study was published in Blood, in August 2023, entitled *‘CD19/CD22 targeting with co-transduced CAR T-cells to prevent antigen negative relapse after CAR T-cell therapy of B-ALL’.*
- *AUTO8 in Multiple Myeloma – Phase 1 MCARTY Study*

- AUTO8 is a next-generation product candidate for multiple myeloma, which comprises two CARs for the multiple myeloma targets, BCMA and CD19. In collaboration with UCL, the Company initiated a study in 2022. Patients continue to be enrolled and initial data will be presented at ASH in December 2023.

- *AUTO6NG in Neuroblastoma – Phase 1 MAGNETO Study*
  - AUTO6NG contains a CAR that targets GD2 alongside additional programming modules to enhance the activity and persistence. UCL has received MHRA approval for the conduct of a Phase 1 clinical study in children with r/r neuroblastoma. The study is on track to be initiated in Q4 2023.

Key Operational Updates during Q3 2023

- The Company's new 70,000 square foot commercial manufacturing facility, The Nucleus, in Stevenage, UK has completed process performance qualification and is on track to support the BLA submission for obe-cel. The Company estimates capacity of approximately 2,000 batches per annum, which is anticipated to be sufficient to meet US and EU adult ALL demand.

- Previously announced leadership appointments of Rob Dolski as Chief Financial Officer, effective August 7, 2023, as well as Dr. Veronica Hersberger as Senior Vice President, Medical Affairs and Miranda Neville as Senior Vice President Program Management.

Scientific Publications:

- Publication of a paper in *Nature Medicine* titled: '*Transcriptional hallmarks of persisting CD19 CAR T-cells in children with leukemia'* - Ghorashian, S. et al, Nature Medicine; 2023. doi: 10.1038/s41591-023-02415-3. Link to paper.

- Publication of a paper in *Blood* titled: '*CD19/CD22 targeting with co-transduced CAR T-cells to prevent antigen negative relapse after CAR T-cell therapy of B-ALL'* - Ghorashian et al, Blood; doi.org/10.1182/blood.2023020621. Link to paper.

Expected News Flow

Obe-cel BLA submission to FDA	By end 2023
Obe-cel FELIX data at ASH	December 2023
AUTO8 update (MCARTY) at ASH	December 2023
AUTO6NG Phase 1 study start (MAGNETO)	By end 2023
Obe-cel in autoimmune disease – refractory SLE Phase 1 study start	Early 2024
Obe-cel filing for a Marketing Authorization Application to EMA	First half 2024

Financial Results for the Third Quarter Ended September 30, 2023

Cash and cash equivalents at September 30, 2023, totaled $256.4 million, as compared to $382.4 million at December 31, 2022.

Total operating expenses, net for the three months ended September 30, 2023, were $47.8 million, as compared to $43.5 million, for the same period in 2022.

Research and development expenses decreased by $0.4 million to $37.2 million for the three months ended September 30, 2023 compared to the same period in 2022. This change was primarily due to decreases in clinical and manufacturing costs related to the Company's obe-cel clinical program partially offset by increases in operating costs related to the Company's new commercial manufacturing facility and in salaries and related costs driven by increased headcount.

General and administrative expenses increased by $2.4 million to $10.6 million for the three months ended September 30, 2023 compared to the same period in 2022. This increase was primarily due to salaries and other employment-related costs driven by an increase in general and administrative headcount supporting the overall growth of the business, primarily relating to pre-commercialization activities.

Net loss attributable to ordinary shareholders was $45.8 million for the three months ended September 30, 2023, compared to $42.8 million for the same period in 2022. The basic and diluted net loss per ordinary share for the three months ended September 30, 2023, totaled $(0.26) compared to a basic and diluted net loss per ordinary share of $(0.47) for the three months ended September 30, 2022.

Autolus estimates that its current cash and cash equivalents on hand and anticipated future milestone payment from Blackstone will extend the Company's cash runway into 2025.

Unaudited Financial Results for the Third Quarter Ended September 30, 2023
Selected Condensed Consolidated Balance Sheet Data
(In thousands)

	September 30, 2023	December 31, 2022
Assets		

Cash and cash equivalents	$ 256,415	$ 382,436
Total current assets	**$ 308,382**	**$ 425,771**
Total assets	**$ 406,098**	**$ 490,274**
Liabilities and shareholders' equity		
Total current liabilities	**$ 37,540**	**$ 46,366**
Total liabilities	**$ 225,580**	**$ 191,600**
Total shareholders' equity	**$ 180,518**	**$ 298,674**
Total liabilities and shareholders' equity	**$ 406,098**	**$ 490,274**

Selected Condensed Consolidated Statements of Operations and Comprehensive Loss Data
(In thousands, except share and per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Grant income	—	—	—	$ 166
License revenues	$ 406	$ 2,369	$ 1,698	$ 2,369
Operating expenses:				
Research and development	(37,237)	(37,632)	(105,323)	(109,806)
General and administrative	(10,611)	(8,231)	(31,017)	(24,487)
Loss on disposal of property and equipment	—	—	(3,791)	—
Impairment of operating lease right-of-use assets and related property and equipment	(382)	—	(382)	—
Total operating expenses, net	**(47,824)**	**(43,494)**	**(138,815)**	**(131,758)**
Total other expense, net	(2,965)	(5,425)	(4,777)	(9,380)
Net loss before income tax	**(50,789)**	**(48,919)**	**(143,592)**	**(141,138)**
Income tax benefit	4,940	6,152	12,380	19,250
Net loss attributable to ordinary shareholders	**(45,849)**	**(42,767)**	**(131,212)**	**(121,888)**
Other comprehensive (loss) income				
Foreign currency exchange translation adjustment	(5,837)	(14,054)	5,104	(38,994)
Total comprehensive loss	**$ (51,686)**	**$ (56,821)**	**$ (126,108)**	**$ (160,882)**
Basic and diluted net loss per ordinary share	$ (0.26)	$ (0.47)	$ (0.75)	$ (1.34)
Weighted-average basic and diluted ordinary shares	173,984,101	91,240,801	173,890,666	91,028,562

Conference Call
Management will host a conference call and webcast at 9:00 am EDT/1:00 pm GMT to discuss the company's 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.

About Autolus Therapeutics plc
Autolus is a clinical-stage biopharmaceutical company developing next-generation, programmed T cell therapies for the treatment of cancer and autoimmune disease. 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 target 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, solid tumors and autoimmune diseases. 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. Obe-cel is designed with a fast target binding off-rate to minimize excessive activation of the programmed T cells. Clinical trials of obe-cel have demonstrated that this "fast off-rate" profile reduces toxicity and T cell exhaustion, resulting in improved persistence and leading to high levels of durable remissions in r/r Adult ALL patients. The results of the FELIX trial, a pivotal trial for adult ALL, are being prepared for regulatory submissions with the FDA and EMA. In collaboration with Autolus' academic partner, UCL, obe-cel is currently being evaluated in a Phase 1 clinical trials for B-NHL.

About obe-cel FELIX clinical trial
Autolus' Phase Ib/II clinical trial of obe-cel enrolled adult patients with relapsed / refractory B-precursor ALL. The trial had a Phase Ib component prior to proceeding to the single arm, Phase II 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 enrolled over 100 patients across 30 of the leading academic and non-academic centers in the United States, United Kingdom and Europe. [NCT04404660]

About AUTO1/22
AUTO1/22 is a novel dual targeting CAR T cell-based therapy candidate based on obe-cel. It is designed to combine the enhanced safety, robust

expansion and persistence seen with the fast off rate CD19 CAR from obe-cel with a high sensitivity CD22 CAR to reduce antigen negative relapses. This product candidate is currently in a Phase I clinical trial for patients with r/r pediatric ALL. [NCT02443831]

About AUTO6NG
AUTO6NG is a next generation programmed T cell product candidate in pre-clinical development. AUTO6NG builds on preliminary proof of concept data from AUTO6, a CAR targeting GD2-expression cancer cell currently in clinical development for the treatment of neuroblastoma. AUTO6NG incorporates additional cell programming modules to overcome immune suppressive defense mechanisms in the tumor microenvironment, in addition to endowing the CAR T cells with extended persistence capacity. AUTO6NG is currently in pre-clinical development for the potential treatment of both neuroblastoma and other GD2-expressing solid tumors.

About AUTO8
AUTO8 is a next-generation product candidate for multiple myeloma which comprises two independent CARs for the multiple myeloma targets, BCMA and CD19. We have developed an optimized BCMA CAR which is designed for improved killing of target cell that express BCMA at low levels. This has been combined with fast off rate CD19 CAR from obe-cel. We believe that the design of AUTO8 has the potential to induce deep and durable responses and extend the durability of effect over other BCMA CARs currently in development. This product candidate is currently in a Phase I clinical trial for patients with r/r multiple myeloma. [NCT04795882]

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 development of obe-cel and Autolus' other 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 research and development activities; the timing of availability or disclosure of data from those clinical trials and the timing of planned regulatory submissions; the potential for obe-cel or Autolus' other product candidates to receive regulatory approval from the FDA or equivalent foreign regulatory agencies, and whether, if approved, obe-cel or Autolus' other product candidates will be successfully distributed, marketed and commercialized; the size and growth potential of the markets for obe-cel or Autolus' other product candidates, if approved, and the rate and degree of market acceptance of obe-cel or Autolus' other product candidates, including reimbursement that may be received from payors; potential therapeutic effects of obe-cel and Autolus' other product candidates; anticipated clinical data presentations; Autolus' commercialization, marketing and manufacturing capabilities and strategy; the commercial potential of obe-cel; the progress and estimated completion of the Company's commercial manufacturing facility including anticipated capacity; the Company's ability to achieve milestones and receive associated milestone payments pursuant to the terms of its collaboration agreements, including pursuant to the Blackstone collaboration and the Company's expected 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; any of Autolus' or its partners' product candidates may fail in development, may not receive required regulatory approvals, or may be delayed to a point where they are not commercially viable; Autolus may not achieve milestones or receive additional payments under its collaborations; regulatory agencies may impose additional requirements or delay the initiation of clinical trials; the impact of new or changing laws and regulations; market conditions; and the impact of the global health crises or geopolitical conditions on Autolus' business, research and clinical development plans and timelines and results of operations, including impact on its clinical trial sites, collaborators, and contractors who act for or on Autolus' behalf; Autolus' assumptions and estimates regarding its financial condition, future financial performance and estimated cash runway may be incorrect; and inability to maintain or enter into new partnerships or strategic collaborations. 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 7, 2023, 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.

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