Autolus Therapeutics Reports Second Quarter 2023 Financial Results and Operational Progress

August 3, 2023 at 7:01 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*
- *Pivotal FELIX data at ASCO showed 76% of patients treated with obe-cel achieved a response (CR/CRi) with very low levels of high-grade CRS and ICANS*
- *Commercial manufacturing facility, The Nucleus, on track to commence Good Manufacturing Practice (GMP) operations in H2 2023 with capacity of approximately 2,000 batches per annum*
- *Obe-cel Phase 1 study in refractory systemic lupus erythematosus (SLE) to start in early 2024*
- *Rob Dolski appointed as Chief Financial Officer (CFO)*
- *Conference call to be held today at 8:30 am EDT/1:30 pm BST: Conference call participants should pre-register using the link at the bottom of this press release*

LONDON, Aug. 03, 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 June 30, 2023.

“We had a successful ASCO conference this quarter, with topline data presentation for obe-cel from the FELIX study highlighting low levels of immunotoxicity combined with a high complete remission rate and excellent CAR T expansion and persistence in adult patients with relapsed/refractory ALL. The attractive clinical profile driven by the unique CAR design combined with robust and reliable manufacturing have been the foundation for the successful outcome of the FELIX study,” **commented Dr. Christian Itin, Chief Executive Officer of Autolus**. “The Company is now focused on delivering a BLA filing to the US FDA by year end and the initial preparatory activities for a commercial launch in 2024, pending the necessary regulatory approvals.”

“We have also advanced our preparations for a US commercial launch, selecting Cardinal Health as our distribution partner and by putting into place the team that will initiate onboarding of treatment centers in the second half of this year. With our commercial manufacturing facility, The Nucleus, on track to commence Good Manufacturing Practice (GMP) operations in the second half of 2023, we are in a strong position to operationally deliver product and adequately meet the global demand for adult ALL treatment.”

“Looking beyond ALL and building on the pioneering work by Georg Schett and Andreas Mackensen at the University of Erlangen, we believe obe-cel's excellent safety profile and high level of activity in ALL and NHL patients, combined with our operational infrastructure, forms an attractive basis for development of obe-cel in autoimmune disease with a start of first clinical trial in early 2024.”

Key obe-cel Updates:

- *Obecabtagene autoleucel (obe-cel) in relapsed / refractory (r/r) adult ALL – The FELIX Study*
  - Pivotal Phase 2 data presented at ASCO and EHA confirmed attractive product profile with potential best-in-class tolerability and very low levels of high-grade CRS and ICANS. Longer term follow up data and subgroup analysis data to be presented at ASH in late 2023, as well as at medical conferences in H1 2024. BLA submission for obe-cel on-track to be submitted to the FDA at the end of 2023.

Obe-cel trials in collaboration with University College London

- *Obe-cel in r/r adult ALL patients – Phase 1 ALLCAR19 Study*
  - Long term follow-up data were presented at the Tandem Meetings: Transplantation & Cellular Therapy Meetings of the American Society for Transplantation and Cellular Therapy (ASTCT) and the Center for International Blood & Marrow Transplant Research (CIBMTR). The data demonstrated that 35% of adult ALL patients remained in complete remission at a median follow up of 36 months without the need for additional anti-leukemia therapy.
- *Obe-cel in r/r B-NHL and CLL patients – Phase 1 ALLCAR19 Extension Study*
  - Data presented at the ASH meeting in December 2022 demonstrated the potentially best-in-class profile of obe-cel supported by the data observed in B-cell non-Hodgkin lymphoma (NHL), with continued high levels of clinical

activity paired with an encouraging tolerability profile across diffuse large B-cell lymphoma (DLBCL), mantle cell lymphoma (MCL), follicular lymphoma (FL) and chronic lymphocytic leukemia (CLL). Patients continue to be enrolled into the study and the Company expects to publish the full results in a peer-reviewed journal.

- *Obe-cel in Primary CNS Lymphoma patients – Phase 1 CAROUSEL Study*
  - Data presented at the EHA meeting in June 2022 demonstrated first activity in primary CNS lymphoma. The study is fully enrolled, and the Company expects to publish the full results in a peer-reviewed journal.

- *AUTO1/22 in pediatric B-ALL patients – Phase 1 CARPALL Study*
  - Data presented at the European Society for Blood and Marrow Transplantation (EBMT) Annual Meeting in April 2023 by the Company's UCL collaborators, showed favorable safety profile and good efficacy in a heavily pre-treated cohort of patients. Importantly, there were no observed antigen negative relapses observed as of the data cut-off date, indicating that the combining of our optimized CD22 CAR design with the CD19 CAR used in obe-cel may be effective in preventing antigen-loss driven relapse in pediatric B-ALL. The preclinical data supporting this program was published in *Molecular Therapy* in March 2023, entitled '*Dual targeting of CD19 and CD22 against B-ALL using a novel high sensitivity aCD22 CAR*.'

Early-stage pipeline – leveraging academic collaborations / opportunity for non-dilutive funding

- *AUTO4 in Peripheral T Cell Lymphoma patients – Phase 1/2 LibrA T1 Study*
  - Data presented at the International Conference on Malignant Lymphoma (ICML) in an oral presentation titled '*First in Human Study of AUTO4, a TRBC1-Targetting CAR T Cell Therapy in Relapsed/Refractory TRBC1-Positive Peripheral T-Cell Lymphoma*' demonstrated safety with no dose limiting toxicities and remarkable durability in 2 out of 4 responding patients at the highest dose level tested, with ongoing complete metabolic responses (CMR) in two r/r PTCL patients at 15 and 18 months.

- *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 Q1 2022. Patients continue to be enrolled and initial data is expected by the end of 2023.

- *AUTO6NG in Neuroblastoma*
  - AUTO6NG contains a CAR that targets GD2 alongside additional programming modules to enhance the activity and persistence. In collaboration with UCL, the Company is planning on initiating a clinical trial of AUTO6NG in 2023.

Key Operational Updates during Q2 2023

- The Company's new 70,000 square foot commercial manufacturing facility, The Nucleus, in Stevenage, UK has continued to progress on track. Key equipment installation and validation were completed by Autolus in Q1 2023, enabling operational engineering trials to commence in Q2 2023. Activities are on track for the commencement of further BLA enabling GMP operations in the second half of 2023. The facility has been designed to manufacture and test approximately 2,000 batches per year with expansion opportunities.
- Autolus is on schedule to complete the development work and report generation for the Chemistry Manufacturing and Controls (CMC) package planned to be submitted to the FDA. All work including process qualification activities in The Nucleus is on track for submission of a BLA by the end of 2023.
- Autolus has selected Cardinal Health to provide core distribution capabilities required for US commercialization of CAR T-cell therapies. Under the proposed agreement, Cardinal Health 3PL Services will establish essential capabilities for Autolus to commercialize a CAR T-cell therapy in the US, including a depot model that allows Autolus to maintain custody and physically position product closer to treatment sites during the finalization of product release, with the goal of shortening vein-to-delivery times. In addition, Cardinal Health will help provide seamless order-to-cash capabilities.
- Autolus hosted a Virtual Capital Markets Day in April 2023, where members of the Executive Management Team and Key Opinion Leaders presented on the obe-cel commercial opportunity and positioning. A replay of the event is available on the Autolus website.
- Appointment of Dr. Robert Iannone, Executive Vice President, Global Head of Research & Development of Jazz Pharmaceuticals plc, as a Non-Executive Director to Autolus' Board of Directors, effective June 15, 2023.

Scientific Publications:

- Publication of a paper in *Molecular Therapy Nucleic Acids*, titled *'Novel Fas-TNFR chimeras that prevent Fas ligand-mediated kill and signal synergistically to enhance CAR T-cell efficacy'*. The paper outlined how Fas-CD40 chimera can render T cell therapies resistant to FasL-mediated cell death and improve their effectiveness against solid tumors. Link to paper.
- Publication of a paper in *Cancer Immunology Research* entitled *'Enhancing CAR T cell therapy using Fab-Based*

Constitutively Heterodimeric Cytokine Receptors' highlighting that for CAR T cells to be effective, they must engraft in the patient, expand to sufficient numbers and persist at the site of disease. Link to paper.

Post Period Update:

- Post period the Company announced the following appointments:
    - Rob Dolski as Chief Financial Officer, effective August 7, 2023. Rob is succeeding Dr. Lucinda Crabtree. Most recently Rob completed the successful sale of Checkmate Pharmaceuticals to Regeneron and had prior leadership and operational roles at Moderna, Human Genome Sciences and Amgen.
    - Dr. Veronica Hersberger as Senior Vice President, Medical Affairs. Most recently Veronica served as Chief Medical Officer of TargImmune Therapeutics AG. Prior to that she was Global Product Leader for the cancer therapies Calquence and Lumoxiti at AstraZeneca and led Medical Affairs for the Hematology Franchise at Roche where she was also involved in the development of a range of oncology programs.
    - Miranda Neville was promoted to Senior Vice President Program Management. Miranda successfully ran the commercial manufacturing facility project resulting in the Nucleus site and took over the obe-cel program lead in 2023. Prior to joining Autolus, Miranda, amongst other roles, led program management for the Benlysta program at Human Genome Sciences in systemic lupus erythematosus (SLE) through a successful BLA process, resulting in regulatory approval.

Financial Results for the Second Quarter Ended June 30, 2023

Cash and cash equivalents and restricted cash at June 30, 2023, totaled $307.8 million, as compared to cash and cash equivalents and restricted cash of $382.8 million at December 31, 2022.

Total operating expenses, net for the three months ended June 30, 2023, were $47.9 million, as compared to net total operating expenses, net of $46.5 million, for the same period in 2022.

Research and development expenses decreased by $1.5 million to $36.7 million for the three months ended June 30, 2023 from $38.2 million for the three months ended June 30, 2022 primarily due to:

- a decrease of $5.9 million in clinical costs and manufacturing costs primarily relating to our obe-cel clinical product candidate,
- a decrease of $0.6 million in legal fees and professional consulting fees in relation to our research and development activities,
- a decrease of $0.5 million in depreciation and amortization related to property, plant and equipment and intangible assets,
- a decrease of $0.1 million in material transportation costs, offset by
- an increase of $3.0 million in salaries and other employment related costs including share-based compensation expense, which was mainly driven by an increase in the number of employees engaged in research and development activities,
- an increase of $1.7 million in facilities costs related to our new manufacturing facility, The Nucleus, in Stevenage, United Kingdom as well as increases in costs related to maintaining our current leased properties, and
- an increase of $0.9 million related to information technology infrastructure and support for information systems related to the conduct of clinical trials and manufacturing operations.

General and administrative expenses increased by $2.8 million to $11.1 million for the three months ended June 30, 2023 from $8.3 million for the three months ended June 30, 2022 primarily due to:

- an increase of $1.5 million in commercial readiness costs due to increased commercial readiness activities being undertaken,
- an increase of $1.2 million in salaries and other employment related costs including share-based compensation expenses, which was mainly driven by an increase in the number of employees engaged in general and administrative activities,
- an increase of $0.2 million related to information technology infrastructure and support for information systems related to the conduct of corporate and commercial operations,
- an increase of $0.1 million in depreciation and amortization related to property and equipment and intangible assets, offset by
- a decrease of $0.2 million primarily related to a reduction in directors' and officers' liability insurance premiums, legal and professional fees.

Other income (expense), net increased to an income of $0.5 million for the three months ended June 30, 2023 from an expense of $1.3 million for the

three months ended June 30, 2022. The increase of $1.8 million is primarily due to the strengthening of the Pound Sterling exchange rate relative to the U.S. dollar for the three months ended June 30, 2023 as compared to the three months ended June 30, 2022.

Interest income increased to $3.4 million for the three months ended June 30, 2023, as compared to $0.1 million for the three months ended June 30, 2022. The increase in interest income of $3.3 million primarily relates to increased account balances and yield associated with our cash and cash equivalents during the three months ended June 30, 2023 as compared to the three months ended June 30, 2022.

Interest expense increased to $5.0 million for the three months ended June 30, 2023 as compared to $1.8 million for the three months ended June 30, 2022. Interest expense is primarily related to the liability for future royalties and sales milestones, net associated with our strategic collaboration agreement with Blackstone.

Income tax benefit decreased by $4.0 million to $3.5 million for the three months ended June 30, 2023 from $7.5 million for the three months ended June 30, 2022 due to a decrease in qualifying research and development expenditures and the reduction in effective tax rate related to the U.K. research and development tax credit regime under the scheme for SMEs.

Net loss attributable to ordinary shareholders was $45.6 million for the three months ended June 30, 2023, compared to $42.1 million for the same period in 2022. The basic and diluted net loss per ordinary share for the three months ended June 30, 2023, totaled $(0.26) compared to a basic and diluted net loss per ordinary share of $(0.46) for the three months ended June 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 runway into 2025.

Unaudited Financial Results for the Second Quarter Ended June 30, 2023

Condensed Consolidated Balance Sheet

(In thousands, except share and per share amounts)

	June 30 2023	December 31 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 307,500	$ 382,436
Restricted cash	332	325
Prepaid expenses and other current assets	47,533	43,010
Total current assets	**355,365**	**425,771**
Non-current assets:		
Property and equipment, net	36,857	35,209
Prepaid expenses and other non-current assets	295	2,176
Operating lease right-of-use assets, net	54,251	23,210
Long-term deposits	1,864	1,832
Deferred tax asset	2,360	2,076
Total assets	**$ 450,992**	**$ 490,274**
Liabilities and shareholders' equity		
Current liabilities:		
Accounts payable	$ 3,878	$ 531
Accrued expenses and other liabilities	30,954	40,797
Operating lease liabilities, current	6,231	5,038
Total current liabilities	**41,063**	**46,366**
Non-current liabilities:		
Operating lease liabilities, non-current	44,707	19,218
Liability related to future royalties and sales milestones, net	135,764	125,900
Other long term payables	122	116
Total liabilities	**221,656**	**191,600**
Shareholders' equity:		
Ordinary shares, $0.000042 par value; 290,909,783 authorized as of June 30, 2023 and December 31, 2022; 173,680,872 and 173,074,510 shares issued and outstanding at June, 2023 and December 31, 2022	8	8
Deferred shares, £0.00001 par value; 34,425 shares authorized, issued and outstanding at June 30, 2023 and December 31, 2022	—	—
Deferred B shares, £0.00099 par value; 88,893,548 shares authorized, issued and outstanding at June 30, 2023 and December 31, 2022	118	118
Deferred C shares, £0.000008 par value; 1 share authorized, issued and outstanding at June 30, 2023 and December 31, 2022	—	—

Additional paid-in capital	1,012,709	1,007,625
Accumulated other comprehensive loss	(27,957)	(38,898)
Accumulated deficit	(755,542)	(670,179)
Total shareholders' equity	**229,336**	**298,674**
Total liabilities and shareholders' equity	**$ 450,992**	**$ 490,274**

Condensed Consolidated Statements of Operations and Comprehensive Loss

(In thousands, except share and per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	**2022**	**2023**	**2022**
Grant income	$ —	$ —	$ —	$ 166
License revenue	—	—	1,292	—
Operating expenses:				
Research and development	(36,742)	(38,212)	(68,086)	(72,175)
General and administrative	(11,122)	(8,269)	(20,406)	(16,256)
Loss on disposal of property and equipment	(23)	—	(3,791)	—
Total operating expenses, net	**(47,887)**	**(46,481)**	**(90,991)**	**(88,265)**
Other income (expenses), net	482	(1,331)	1,264	(471)
Interest income	3,403	89	6,849	117
Interest expense	(5,020)	(1,810)	(9,925)	(3,599)
Total other expense, net	**(1,135)**	**(3,052)**	**(1,812)**	**(3,953)**
Net loss before income tax	**(49,022)**	**(49,533)**	**(92,803)**	**(92,218)**
Income tax benefit	3,470	7,474	7,440	13,098
Net loss attributable to ordinary shareholders	**(45,552)**	**(42,059)**	**(85,363)**	**(79,120)**
Other comprehensive income (loss):				
Foreign currency exchange translation adjustment	5,300	(17,485)	10,941	(24,941)
Total comprehensive loss	**$ (40,252)**	**$ (59,544)**	**$ (74,422)**	**$ (104,061)**
Basic and diluted net loss per ordinary share	$ (0.26)	$ (0.46)	$ (0.49)	$ (0.87)
Weighted-average basic and diluted ordinary shares	173,860,491	90,931,964	173,843,249	90,923,119

Conference Call

Management will host a conference call and webcast at 8:30 am EDT/1:30 pm BST 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. 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 pivotal trial for adult ALL.

About obe-cel FELIX clinical trial

Autolus' Phase 1b/2 clinical trial of obe-cel enrolled 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 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 1 clinical trial for patients with r/r pediatric ALL. [NCT02443831]

About AUTO4
AUTO4 is a programmed T cell product candidate in clinical development for T cell lymphoma, a setting where there are currently no approved programmed T cell therapies. AUTO4 is specifically designed to target TRBC1 derived cancers, which account for approximately 40% of T cell lymphomas, and is a complement to the AUTO5 T cell product candidate, which is in pre-clinical development.

About AUTO5
AUTO5 is a programmed T cell product candidate in pre-clinical development for T cell lymphoma, a setting where there are currently no approved programmed T cell therapies. AUTO5 is specifically designed to target TRBC2 derived cancers, which account for approximately 60% of T cell lymphomas, and is a complement to the AUTO4 T cell product candidate currently in clinical development.

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 our 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.

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 Autolus' product candidates, the status of clinical trials (including, without limitation, expectations regarding the data that is being presented, the expected timing of data releases and development, as well as completion of clinical trials) and development timelines for the Company's 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; possible safety and efficacy concerns; and the impact of COVID-19 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 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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