



## **Autolus announces license agreement with UCL Business PLC for clinical-stage product candidate in development for the treatment of B-cell malignancies**

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- A CD19 CAR with novel targeting properties designed to reduce cytokine release syndrome –

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Autolus Limited, a clinical-stage biopharmaceutical company developing next-generation programmed T cell therapies, today announced the execution of a license agreement under which Autolus has acquired global rights from UCL Business plc (UCLB), the technology-transfer company of University College London (UCL), to develop and commercialize a novel CD19 chimeric antigen receptor (CAR) T cell therapy with novel targeting properties for the treatment of B cell malignancies.

The product candidate, which we have designated as AUTO1, is an investigational therapy in which a patient's T cells are genetically modified to express a novel CD19-specific CAR designed to reduce side effects related to cytokine release syndrome (CRS). CD19 is a protein expressed by B-cell lymphomas and leukaemias. CD19 CAR T cells have proven effective in treating leukaemia and lymphoma, with efficacy dependent on engraftment and expansion of CAR T cells. However, rapid activation and expansion of CAR T cells can result in CRS, which in some cases can be life-threatening, particularly for elderly patients and patients with higher tumour burden that have poor tolerance for toxicity. Furthermore, excessive activation of CAR T cells can lead to cell exhaustion and limit their persistence.

AUTO1 is currently the subject of two Phase 1 studies, one in paediatric acute lymphoblastic leukaemia (ALL) and the other in adult ALL\*. AUTO1 has been designed to recognise CD19 with a fast-off binding kinetic, which allows CAR T cells to efficiently recognize cancer cells, inject cytotoxic proteins to initiate the natural self-destruction process present in all human cells and then rapidly dissociate from them in order to engage the next cancer cell - a process also known as serial killing. We believe that avoiding prolonged residence on targeted cells may minimize excessive activation of CAR- T cells and reduce toxicity and CAR T cell exhaustion. In a UCL Phase 1 clinical study (CARPALL) in paediatric ALL patients evaluating the properties of AUTO1, investigators observed levels of efficacy similar to those in other reported studies, without observing grade 3 or 4 CRS and without the need to administer immunosuppressive drugs. Data from the CARPALL study were presented at the 2017 Annual Meeting of the American Society of Hematology\*\*.

Dr Martin Pule, Chief Scientific Officer of Autolus Limited and Senior Lecturer in Haematology at UCL, commented:

“Current CARs in the clinic are designed with high affinity binders that can engage the CD19 target for an extended period of time. This can lead to excessive T cell activation and cytokine release, as well as exhaustion of the T cell. We developed a CD19 CAR that is designed to bind to its target with a fast on-rate but then releases quickly, which is more similar to naturally occurring T cell activity. The initial clinical data supports the premise that this kinetic profile reduces toxicity and increases CAR T cell engraftment.”

Dr Christian Itin, Chief Executive Officer of Autolus Limited, added:

“This licensing arrangement represents an exciting opportunity for Autolus as we continue to expand our broad pipeline of clinical-stage T cell programs, with clinical trials currently ongoing for five programmes in six indications. With AUTO1, we are collaborating with UCLB in an ongoing trial in adult ALL patients and also expect to leverage the improved safety profile of the CD19 binder in future generations of our programmed T cell therapies for the treatment of patients with B cell malignancies.”

Cengiz Tarhan, Managing Director of UCLB, said:

“The development of this product candidate represents the culmination of several years of research led by Martin Pule and his collaborators, drawing on funding from multiple government and charitable sources. UCLB is delighted to be able to partner with Autolus to support the continued development of this promising approach.”

\* Paediatric ALL “CARPALL Study”: <https://clinicaltrials.gov/ct2/show/NCT02443831> and adult ALL “ALLCAR19 Study”: <https://clinicaltrials.gov/ct2/show/NCT02935257>

\*\*Abstract: [http://www.bloodjournal.org/content/130/Suppl\\_1/80...](http://www.bloodjournal.org/content/130/Suppl_1/80...)

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## **About Autolus**

Autolus is a private, 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 recognise cancer cells, break down their defence mechanisms and attack and kill these cells. Autolus has a pipeline of product candidates in development for the treatment of haematological malignancies and solid tumours.

## **About AUTO1**

AUTO1 is a CD19 CAR T cell therapy with a binder that is designed to have an equivalent on-rate and faster off-rate compared to other CD19 CARs. We believe this feature of AUTO1 may indicate binding properties more similar to naturally occurring T cell activity than those of current CD19 CARs, which may lead to an improved safety profile, while maintaining similar levels of efficacy. This is particularly important in ALL, a rapidly progressing haematological B cell malignancy characterised by a relatively high tumour burden. In clinical studies with conventional CD19 CARs, many patients, particularly adults, have poor tolerance for toxicity which has contributed to serious side effects being reported, including CRS. AUTO1 is currently being evaluated in two Phase 1 studies, one in adult ALL and one in paediatric ALL.

## **About Acute Lymphoblastic Leukaemia (ALL)**

ALL is a cancer of the bone marrow and blood in which the body makes abnormal white blood cells (lymphocytes). The disease progresses quickly and is the most common childhood cancer in the United States and Europe but it also affects adults. The National Cancer Institute (NCI) estimates that approximately 3,400 patients aged 20 and younger are diagnosed with ALL each year in the United States alone. ALL can be of either T- or B-cell origin, with B-cell the most common. ALL typically worsens rapidly if it is not treated and can be fatal within a few months; therefore, it is critical for patients to start treatment soon after diagnosis. Although the majority of B-cell ALL patients respond well to conventional treatment, the prognosis for patients who relapse is poor and there is a significant unmet need for treatments for these patients.

## **About UCL Business PLC (UCLB)**

UCLB is a leading technology transfer company that supports and commercialises research and innovations arising from UCL, one of the UK's top research-led universities. UCLB has a successful track record and a strong reputation for identifying and protecting promising new technologies and innovations developed by UCL academics. UCLB has a strong track record in commercialising medical technologies and provides technology transfer services to UCL's associated hospitals; University College London Hospitals, Moorfields Eye Hospital, Great Ormond Street Hospital for Children and the Royal Free London Hospital. It invests directly in development projects to maximise the potential of the research and manages the commercialisation process of technologies from laboratory to market. For further information, please visit: [www.uclb.com](http://www.uclb.com) Twitter: [@UCL\\_Business](https://twitter.com/UCL_Business)

## **The Funders**

The development of the AUTO1 program has been supported by multiple government and charitable sources, including the National Institute for Health Research Invention for Innovation (i4i) Program, and Research Professorship, the Great Ormond Street Childrens' Charity, Children with Cancer UK, the J P Moulton Charitable Foundation, European Union Framework 7 Program, the National Institute for Health Research University College London Hospitals Biomedical Research Centre, and the National Institute for Health Research Great Ormond Street Hospital Biomedical Research Centre.