



5 January 2021

Hemogenyx Pharmaceuticals plc

("Hemogenyx Pharmaceuticals" or the "Company")

CAR-T Master Translational Agreement with University of Pennsylvania

Hemogenyx Pharmaceuticals plc (LSE: HEMO), the biopharmaceutical group developing new therapies and treatments for blood diseases, is pleased to announce that it has entered into a Master Translational Research Services Agreement ("Agreement") with the University of Pennsylvania ("Penn"). The goal of the Agreement is to advance the Chimeric Antigen Receptor ("CAR") T-cells ("HEMO-CAR-T") developed by the Company toward and through clinical trials.

Under the Agreement, the Company will retain Penn to conduct translational research activities in support of the research being performed under the existing Sponsored Research Agreement ("SRA"), which was announced on [August 11, 2020](#). As with the research being performed under the SRA, the research and development activities contemplated by the Agreement (the "R&D Activities") will involve Saar I. Gill, MD, PhD, an assistant professor of Medicine, a hematologist-oncologist physician scientist and scientific co-director of the Cell Therapy and Transplantation program in the Perelman School of Medicine at the University of Pennsylvania. Dr. Gill's laboratory is part of the Center for Cellular Immunotherapies ("CCI") at Penn. The Agreement governs the R&D Activities of various organizations within Penn and coordinates such R&D Activities with the work of the Company. The intended outcome of the complex of activities under the Agreement is the clinical proof of concept for HEMO-CAR-T, including its variations such as SAFE-HEMO-CAR-T, for the treatment of acute myeloid leukemia ("AML").

The R&D Activities are intended to include:

- i. vector manufacturing for the delivery of HEMO-CAR-T to the patient's T-cells;
- ii. an investigational new drug ("IND") filing for permission to conduct clinical trials; and
- iii. clinical manufacturing of patient-specific HEMO-CAR programmed T-cells

Dr. Vladislav Sandler, CEO & Co-Founder of Hemogenyx Pharmaceuticals, commented: *"This is the next incredibly important step on a direct path to clinical proof of concept for one of our leading product candidates. We are very pleased to be collaborating with Penn, which was the first institution to develop CAR-T technology into an approved treatment for leukemias, and which has already saved so many lives."*

We are confident that this collaboration will further accelerate the development of our CAR-T product candidate, which we believe will have a significant and positive impact in the treatment of acute myeloid leukemia, for which there is currently no real effective treatment."

About AML and CAR-T Therapy

AML, the most common type of acute leukemia in adults, has poor survival rates (a five-year survival rate of less than 30% in adults) and is currently treated using chemotherapy, rather than the potentially more benign and effective form of therapy being developed by Hemogenyx Pharmaceuticals. The successful development of the new therapy for AML would have a major impact on treatment and survival rates for the disease.

CAR-T therapy is a treatment in which a patient's own T-cells, a type of immune cell, are modified to recognize and kill the patient's cancer cells. The procedure involves: isolating T-cells from the patient; modifying the isolated T-cells in a laboratory using a CAR gene construct (which allows the cells to recognize the patient's cancer); amplifying (growing to large numbers) the newly modified cells; and re-introducing the cells back into the patient.

About the Center for Cellular Immunotherapies

CCI, under the directorship of Carl H. June, MD, Richard W. Vague Professor in Immunotherapy in the department of Pathology and Laboratory Medicine at Penn, is focused on coordinated interdisciplinary approaches for the discovery and development of core platform technologies for personalized cell and gene-based therapies in cancer, autoimmune disease, infectious disease, and organ and bone marrow transplantation. CCI interacts with a coalition of investigators in nearly all departments and centers in the Perelman School of Medicine, driving the clinical translation of novel and investigational immune-based therapies. CCI's mission is to accelerate and synergize efforts that quickly transition fundamental immunobiology research into the clinic.

As mentioned above, CCI and the team of Dr. June have conducted numerous clinical trials with CAR T-cells in patients with HIV infection and diverse forms of cancer. The CD19 CAR T therapy invented in the June Laboratory was awarded "Breakthrough Therapy" status by the FDA for acute lymphoblastic leukemia ("ALL") in children and adults in 2014 and lymphoma for adults in 2018. This technology has been developed for widespread use by Novartis culminating with the FDA approval of the first CAR T-cell therapy Kymriah® (tisagenlecleucel) for the treatment of ALL in 2017.

About Dr. Saar Gill and the Gill Laboratory

Saar I. Gill, MD, PhD, obtained his medical degree from the University of Melbourne in Australia in 1999. After internal medicine residency at St Vincent's Hospital in Melbourne and a hematology fellowship at the Peter MacCallum Cancer Centre, he completed post-doctoral training at the

laboratory of Robert Negrin, MD at Stanford University. In 2011, Dr. Gill moved to the University of Pennsylvania to study with Carl June, MD and David Porter, MD. Dr Gill runs a highly translational research lab dedicated to innovative cellular therapy approaches for the treatment of cancer, and his chief research interest and focus of his clinical practice is acute myeloid leukemia.

Market Abuse Regulation (MAR) Disclosure

Certain information contained in this announcement would have been deemed inside information for the purposes of Article 7 of Regulation No 596/2014 until the release of this announcement.

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About Hemogenyx Pharmaceuticals plc

Hemogenyx Pharmaceuticals is a publicly traded company (LSE: HEMO) headquartered in London, with its US operating subsidiaries, Hemogenyx LLC and Immugenyx LLC, located in New York City at its state-of-the-art research facility.

The Company is a pre-clinical stage biopharmaceutical group developing new medicines and treatments to treat blood and autoimmune disease and to bring the curative power of bone marrow transplantation to a greater number of patients suffering from otherwise incurable life-threatening diseases. Hemogenyx Pharmaceuticals is developing several distinct and complementary product candidates, as well as a platform technology that it uses as an engine for novel product development.

For more than 50 years, bone marrow transplantation has been used to save the lives of patients suffering from blood diseases. The risks of toxicity and death that are associated with bone marrow transplantation, however, have meant that the procedure is restricted to use only as a last resort. The Company's technology has the potential to enable many more patients suffering from devastating blood diseases such as leukemia and lymphoma, as well as severe autoimmune diseases such as multiple sclerosis, aplastic anemia and systemic lupus erythematosus (Lupus), to benefit from bone marrow transplantation.