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Hemogenyx Pharmaceuticals plc

("Hemogenyx Pharmaceuticals" or the "Company")

SAFE-HEMO-CAR-T Effective against AML in vitro

Hemogenyx Pharmaceuticals plc (LSE: HEMO), the biopharmaceutical group developing new therapies and treatments for blood diseases, is pleased to announce the following update on its activities.

As previously announced, Hemogenyx Pharmaceuticals' CDX bi-specific antibody has the potential to treat Acute Myeloid Leukemia ("AML") directly as well as to provide a benign conditioning regimen for blood stem cell replacement therapy. The Company has now carried out extensive work developing treatments for AML and has to date obtained encouraging results.

As announced on 20 February 2020, the Company has constructed and successfully tested *in vivo* Chimeric Antigen Receptor ("CAR") programmed T cells ("HEMO-CAR-T") for the potential treatment of AML. HEMO-CAR was constructed using the Company's proprietary humanized monoclonal antibody against a target on the surface of AML cells.

It was also announced that the Company was engaging in additional engineering of HEMO-CAR-T cells to increase their safety and versatility. The Company has now introduced and successfully *in vitro* tested a safety switch within the HEMO-CAR. The aim of this safety switch is to modulate the activity of HEMO-CAR-T cells and to turn them into a "controllable drug" – SAFE-HEMO-CAR-T. The purpose of these efforts is to dramatically improve the safety and potential versatility of HEMO-CAR-T cells for the treatment of AML and/or conditioning of bone marrow transplants, as well as a number of additional potential indications.

Following the successful completion of these *in vitro* tests, *in vivo* tests of the efficacy of SAFE-HEMO-CAR-T against AML are being conducted using a model of AML established on the background of Advanced peripheral blood Hematopoietic Chimera (ApbHC) – humanized mice developed by Immugenyx, LLC, a subsidiary of Hemogenyx Pharmaceuticals. If these *in vivo* tests are successful, the Company will discuss its findings with its partners under the Sponsored Research Agreement with the University of Pennsylvania, announced on 11 August 2020, with a view to considering the inclusion of SAFE-HEMO-CAR-T in the program of pre-clinical trials currently underway there.



Dr Vladislav Sandler, Chief Executive Officer, commented, "We are encouraged by this new data which demonstrates our continuing progress in the development of novel treatments for blood cancers such as AML. The development of SAFE-HEMO-CAR-T further expands the Company's pipeline and advances it into a cutting-edge area of cell-based immune therapy. We are excited to have developed another unique product candidate that should, if successful, provide a new and potentially effective treatment for blood cancers for which survival rates are currently very poor."

About AML and CAR-T

AML, the most common type of acute leukemia in adults, has poor survival rates (a five-year survival rate of less than 25% 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.

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 (EU) 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.