



3 March 2021

## **Hemogenyx Pharmaceuticals plc**

("Hemogenyx Pharmaceuticals" or the "Company")

### **HEMO-CAR-T Update**

Hemogenyx Pharmaceuticals plc (LSE: HEMO), the biopharmaceutical group developing new therapies and treatments for blood diseases, is pleased to issue an update on the development of its leading product candidate Chimeric Antigen Receptor ("CAR") T-cells ("HEMO-CAR-T").

As announced on [5 January, 2021](#) the company has entered into a Master Translational Research Services Agreement ("Agreement") with the University of Pennsylvania ("Penn"). The goal of the Agreement is to advance HEMO-CAR-T developed by the Company toward clinical trials.

Concurrently with a number of research and development activities contemplated by the Agreement (the "R&D Activities") that are being performed under the existing Sponsored Research Agreement ("SRA"), which was announced on [11 August, 2020](#), the Company has initiated the manufacturing phase of the Agreement.

The Company has initiated the process of engaging contract manufacturing organizations for product development and manufacturing of DNA plasmids, viral vectors and HEMO-CAR-T cells under Current Good Manufacturing Practices ("CGMPs") to support Phase I clinical trials and has contracted Randall Tlachac of Quality Systems LLC ("Quality Systems") to provide oversight and direct product development, manufacturing and quality control operations.

Mr Tlachac has extensive experience in the successful development of cell and gene-based therapies, having led the development of more than 30 products to Phase I/II clinical trial stage, and played a major role in the implementation of Good Tissue Practices regulations since their promulgation in 2004.

Quality Systems will be responsible for supporting the Company's chemistry, manufacturing, and controls ("CMC") efforts, including providing support for product development, operations, and quality, and for assisting the Company in the implementation of internal documentation systems, development of CMC sections of regulatory submissions, manufacturing supply agreements, Master Files and other tasks.

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 working with Quality Systems. We are confident that this 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 Quality Systems LLC**

With over 45 years of experience in quality, development, and manufacturing of pharmaceutical, biotherapeutic, and combination products, Quality Systems' expertise accelerates business development by compressing product development timelines, successfully bringing products to Phase I/II of the clinical trial process in the most cost-effective manner while assuring conformance with regulations and requirements.

Randall Tlachac, the founder and president of Quality Systems, has played the principal role in the approval of 7 New Drug Applications ("NDAs"), and has extensive experience with development of a wide array of products: over 70 Investigational New Drug ("IND") applications including multi-specific antibodies, cell, tissue and gene therapy products, CAR therapies, therapeutic proteins, peptides, peptide conjugates, cationic antimicrobial peptides, small molecule pharmaceuticals nanoparticle formulations, and sterile injectable pharmaceuticals.

Mr Tlachac has provided commercial strategic and business development direction and established Quality Systems and Product Development Processes at more than 50 startup firms and has implemented effective strategies for implementing early phase Development, GMPs/CGMPs and Quality Management Systems.

Quality Systems currently serves established pharmaceutical firms as well as major research institutions in the areas of biotherapeutic, pharmaceutical drug and xenotransplantation product development, biopharmaceutical facility design and validation, as well as product strategy and operational plan support.

Randall Tlachac has authored several FDA Guidance documents and developed the current NDA/ANDA Stability Testing Guidance adopted by the FDA and ICH, as well as other approaches, procedures and qualification for CMC activities.

For more than 25 years, Quality Systems has provided effective services to more than 50 firms and 10 major academic institutions in North America, Asia and Europe.

In the past 10 years Quality Systems has played a direct role in securing funding in excess of \$80 MM for startup firms and research institutions.

### **About Viral Vector and HEMO-CAR-T Manufacturing**

To advance HEMO-CAR-T to the clinic, the Company will manufacture clinical grade HEMO-CAR-T cells. To accomplish this task the Company needs first to manufacture ("package") a viral vector that will deliver HEMO-CAR to patients' T cells. The packaging of the viral vector is conducted using several circular replicating DNA constructs (plasmids) that are delivered (transfected) into a specialized cell line that uses these constructs to make viral proteins. When made inside transfected cells, these viral proteins self-assemble into the viral vector (viral particles). HEMO-CAR-T cells are manufactured by delivering (viral transduction) the viral vector's cargo (HEMO-CAR) to the patient's T cells. Transduced T cells are expanded and made ready for injection into the donor-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 No 596/2014 until the release of this announcement.

## Enquiries:

### **Hemogenyx Pharmaceuticals plc**

Dr Vladislav Sandler, Chief Executive Officer & Co-Founder  
Peter Redmond, Director

<https://hemogenyx.com>

[headquarters@hemogenyx.com](mailto:headquarters@hemogenyx.com)

[peter.redmond@hemogenyx.com](mailto:peter.redmond@hemogenyx.com)

### **SP Angel Corporate Finance LLP**

Matthew Johnson, Vadim Alexandre, Adam Cowl

Tel: +44 (0)20 3470 0470

### **Peterhouse Capital Limited**

Lucy Williams, Duncan Vasey, Charles Goodfellow

Tel: +44 (0)20 7469 0930

## **About Hemogenyx Pharmaceuticals plc**

Hemogenyx Pharmaceuticals is a publicly traded company (LSE: HEMO) headquartered in London, with its US operating subsidiaries, Hemogenyx Pharmaceuticals 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.