AvenCell Therapeutics Announces EMA Approval of Clinical Trial Application (CTA) for AVC-201, a Novel Allogeneic CD123-Directed Switchable CAR-T Investigational Therapy for the Treatment of Relapsed/Refractory Acute Myeloid Leukemia and other CD123 Hematological Malignancies

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- *AVC-201 is the world's first CRISPR-engineered switchable allogeneic CAR-T designed to fully avoid rejection by both the innate and adaptive host immune system.*

CAMBRIDGE, Mass., July 24, 2023 (PRNEWSWIRE) -- AvenCell Therapeutics, Inc., a leading clinical-stage cell therapy company focused on advancing both switchable and allogeneic engineered CAR-T cell therapies, today announced that the European Medicines Agency (EMA) has approved the company's Clinical Trial Application (CTA) application for AVC-201 for the treatment of relapsed/refractory Acute Myeloid Leukemia (AML) and other selected hematologic malignancies positive for CD123 (NCT05949125). AVC-201 is a CRISPR-engineered allogeneic switchable CAR-T candidate designed to target and eliminate cells expressing receptor CD123, which is known to be overexpressed in nearly all acute myeloid leukemias, and several other hematological malignancies.

The phase 1 study, which includes up to 35 patients, will be conducted at multiple sites in Germany. The primary objective of the trial is to assess the safety profile of AVC-201 and to determine the maximum tolerated dose. Secondary measures will include efficacy, safety, and CAR-T persistence.

"We are excited to build on the early success and promising activity and safety of our ongoing switchable autologous clinical program in AML (AVC-101) by now including what we believe is the most scientifically-compelling allogeneic technology in the industry," said Andrew Schiermeier, AvenCell's President & CEO. "With the application of both platform technologies, we are the first

company in this space to completely separate the manufacture of cells from the ultimate patients and cancer indications they will be targeted to. This modular approach allows for unparalleled future flexibility and reduction in cycle times, massive scaling of supply, and meaningful reductions in cost of goods, all of which will dramatically advance the field of cell therapy for patients."

About AVC-201

AVC-201 is a CRISPR-edited Chimeric Antigen Receptor ("CAR")-T Cell therapy that embodies two discrete technology platforms. The first leverages AvenCell's "UniCAR" universal/switchable technology which is comprised of a two-component system. Engineered T Cells are transduced with a "universal" receptor that is completely biologically inert (expressing human La peptide), and are only activated when bound to a second biologic molecule ("targeting module") which directs the T cells to a cancer antigen of interest (in this case, CD123). The presence or absence of the targeting module in circulation allows for exquisite "on" and "off" control, respectively, of the therapeutic activity. The second technology platform consists of an in-licensed allogeneic cell engineering technology developed by Intellia Therapeutics which allows for unrelated donors to provide cells for patients. These cells are uniquely engineered via CRISPR/Cas9 to avoid GvHD and rejection via the host/patient immune system by either innate or adaptive mechanisms.

About AVC-201 Clinical Program

AvenCell's Phase I study (NCT05949125) is evaluating the safety, tolerability, pharmacokinetics and pharmacodynamics of AVC-201 in adults with relapsed or refractory AML and other CD123 positive hematological malignancies. The study is an open-label, single-ascending dose design used to identify two dose levels of AVC-201 that will be further evaluated in a subsequent Phase 2 study.

About Acute Myeloid Leukemia (AML)

AML typically develops from mutations in the DNA of early blood-forming cells, leading to the disruption of normal cell maturation and proliferation. This results in a buildup of immature cells in the bone marrow, crowding out healthy cells and impairing their ability to function properly. AML accounts for a

significant proportion of all leukemia cases. In the United States, it is estimated that approximately 21,000 new cases of AML will be diagnosed in 2023, while on a global scale, the incidence of AML is estimated to be around 2-8 cases per 100,000 people annually. Treatment for AML usually involves chemotherapy to destroy cancer cells, and more recently available targeted therapies directed against specific mutations (e.g. IDH, FLT3.) While a majority of AML cells express the receptor CD123, several previous attempts to target this receptor therapeutically have failed due to the difficulty in managing toxicity. Stem cell transplant continues to be considered the only curative option. The survival rate of AML can vary depending on several factors, including age, overall health, specific genetic mutations, response to treatment, and other individual characteristics. However, the overall five-year survival rate for AML is around 25-30%.

About AvenCell Therapeutics

AvenCell derives its name from the French word “avenir” to reflect the aim to be the FUTURE of cell therapy. AvenCell is building a truly transformative cell therapy company that targets difficult-to-treat cancers, with its lead programs focusing on acute myeloid leukemia (AML) and prostate cancer, and additional programs targeting other hematological malignancies and solid tumors. AvenCell was formed with the goal to create truly allogeneic cells that persist as long or longer than autologous therapies and develop a universal and switchable construct that allows complete control and target redirection of T cells after they are infused into a patient. Integration of these two platforms allows for complete separation of the manufacturing of cells from ultimate patient and cancer target, thus providing significant scalability potential at orders of magnitude more efficient than current approaches.

AvenCell Therapeutics, Inc. was launched in 2021 by Blackstone Life Sciences, Cellex Cell Professionals, and Intellia Therapeutics, and incorporated the clinical-stage biopharmaceutical company GEMoaB GmbH. AvenCell is headquartered in Cambridge, Massachusetts with additional R&D and manufacturing operations in Dresden, Germany.

For more information, visit www.avencell.com.

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Forward-Looking Statements

This press release contains "forward-looking statements" of AvenCell Therapeutics, Inc. ("AvenCell" or the "Company") within the meaning of the Private Securities Litigation Reform Act of 1995. These forward-looking statements include, but are not limited to, express or implied statements regarding AvenCell's beliefs and expectations regarding: the safety, efficacy, success and advancement of its clinical program for AVC-201 for the treatment of CD123 positive hematological malignancies pursuant to its clinical trial applications, including the initiation, enrollment, dosing and completion of clinical trials, such as planned enrollment for the Phase 1 portion of the study for AVC-201.

Forward-looking statements involve substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Risks and uncertainties include, among other things, the uncertainties inherent in research and development, including AvenCell's ability to initiate, progress or complete pre-clinical or clinical studies within currently anticipated timelines or at all, and the possibility of unfavorable results from ongoing or additional pre-clinical or clinical studies; uncertainties relating to regulatory applications and related filing and approval timelines; risks related to AvenCell's relationships and collaborations with third parties; risks related to the maintenance and protection of AvenCell's intellectual property; risks related to the potential successful development and commercialization of AvenCell's product candidates; and risks associated with AvenCell's understanding of cell therapy and its application to the treatment of cancers.

All statements other than statements of historical fact are statements that could be deemed forward-looking statements. The reader is cautioned that any such forward-looking statements are not guarantees of future performance and involve risks and uncertainties, and is cautioned not to place undue reliance on these forward-looking statements. All forward-looking statements are based on information currently available to AvenCell, and

AvenCell assumes no obligation and disclaims any intent to update any such forward-looking statements.

AvenCell Contacts:

Media:
Constanze Medack
constanze.medack@avencell.com