

PRESS RELEASE

# Cellectis Submits IND Application for UCART123, an Allogeneic Gene Edited CAR T-Cell Product Candidate, in AML and BPDCN

**January 3, 2017** – New York (N.Y.) – <u>Cellectis</u> (Alternext: ALCLS; Nasdaq: CLLS), a biopharmaceutical company focused on developing immunotherapies based on gene edited CAR T-cells (UCART), today announced the submission of an Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA) requesting approval to initiate Phase 1 clinical trials of UCART123 the Company's most advanced, wholly owned TALEN® gene edited product candidate in patients with acute myeloid leukemia (AML) and blastic plasmacytoid dendritic cell neoplasm (BPDCN).

Pending regulatory clearance, Cellectis plans to initiate Phase 1 clinical trials in the first half of 2017. This is the first IND filing for human clinical applications of a gene edited allogeneic "off-the-shelf" product candidate in the U.S.

UCART123 is a gene edited T-cell investigational drug that targets CD123, an antigen expressed at the surface of leukemic cells in AML, as well as on leukemic and other tumoral cells in BPDCN.

The UCART123 program was subject to a public hearing by the National Institutes of Health's Recombinant DNA Advisory Committee (RAC) in December 2016, where it received the unanimous approval of the RAC committee members.

AML is a devastating clonal hematopoietic stem cell neoplasm that is characterized by uncontrolled proliferation and accumulation of leukemic blasts in bone marrow, peripheral blood and, occasionally, in other tissues. These cells disrupt normal hematopoiesis and rapidly cause bone marrow failure and death. In the U.S. alone, there are an estimated 19,950 new AML cases per year, with 10,430 estimated deaths per year.

The clinical research at Weill Cornell will be led by principal investigator Dr. Gail J. Roboz, Director of the Clinical and Translational Leukemia Programs and Professor of Medicine.

BPDCN is a very rare and aggressive hematological malignancy that is derived from plasmacytoid dendritic cell precursors. BPDCN is a disease of bone marrow and blood cells but also often affects skin and lymph nodes.

The UCART123 clinical program at MD Anderson will be led by Dr Naveen Pemmaraju, MD, Assistant Professor, and Professor Hagop Kantarjian, MD, Department Chair, Department of Leukemia, Division of Cancer Medicine.

The manufacturing process of Cellectis' allogeneic CAR T-cell product line, Universal CARTs or UCARTs, yields frozen, off-the-shelf, engineered CAR T-cells. UCARTs are meant to be readily available CAR T-cells for a large patient population. Their production can be industrialized and standardized with defined pharmaceutical release criteria.

"Following a Pre-IND meeting with the FDA in August 2016 and a NIH-RAC public hearing in December 2016, filing this IND is an important regulatory milestone for the Company. It represents many years of research and development by a dedicated team focused on developing highly innovative UCART products for the benefit of patients", stated Stephan Reynier, Chief Regulatory and Compliance Officer, Cellectis.

Chief Medical Officer, Dr. Loan Hoang-Sayag, commented: "UCART123 represents a unique therapeutic approach for patients with unmet medical needs such as relapsed or refractory AML, high risk AML and BPDCN and we are excited to move this experimental product into clinical development. We have designed robust Phase 1 clinical trials to better understand the potential of UCART123 to address the needs of different patient populations."

Information about ongoing clinical trials are publically available on dedicated websites such as:

www.clinicaltrials.gov in the U.S. www.clinicaltrialsregister.eu in Europe

## About Cellectis

Cellectis is a biopharmaceutical company focused on developing immunotherapies based on gene edited CAR T-cells (UCART). The company's mission is to develop a new generation of cancer therapies based on engineered T-cells. Cellectis capitalizes on its 17 years of expertise in genome engineering - based on its flagship TALEN® products and meganucleases and pioneering electroporation PulseAgile technology - to create a new generation of immunotherapies. CAR technologies are designed to target surface antigens expressed on cells. Using its life-science-focused, pioneering genome-engineering technologies, Cellectis' goal is to create innovative products in multiple fields and with various target markets. Cellectis is listed on the Nasdaq market (ticker: CLLS) and on the NYSE Alternext market (ticker: ALCLS). To find out more about us, visit our website: www.cellectis.com

Talking about gene editing? We do it. TALEN® is a registered trademark owned by the Cellectis Group.

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#### Disclaimer

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