Cellectis Files IND for UCART22 in Acute Lymphoblastic Leukemia (B-ALL)

This is the 3rd IND filing for a TALEN®-based Gene Edited Allogeneic UCART Product Candidate

May 2, 2018 – New York (N.Y.) – Cellectis (Euronext Growth: ALCLS - Nasdaq: CLLS), a clinical-stage biopharmaceutical company focused on developing immunotherapies based on gene-edited allogeneic CAR T-cells (UCART), announced today that the Company has submitted an Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA) requesting approval to initiate a Phase 1 clinical trial for UCART22, Cellectis' second wholly controlled TALEN® gene-edited product candidate, for the treatment of B-cell acute lymphoblastic leukemia (B-ALL) in adult patients.

Pending regulatory clearance, Cellectis plans to initiate a Phase I clinical trial in the third quarter of 2018. The clinical research will be led by Dr. Nitin Jain, Assistant Professor, and Prof. Hagop Kantarjian, Chairman in the Department of Leukemia and University Chair in Cancer Medicine at The University of Texas MD Anderson Cancer Center in Houston.

“This IND application for UCART22 is an important regulatory milestone for the Company,” said Stephan Reynier, Chief Regulatory and Compliance Officer, Cellectis. “The first ever FDA approval for a CAR T-cell therapy, directed against CD19, for pediatric and young patients with R/R B-ALL occurred in 2017. However, further CART approaches are needed, as some limitations of the CD19-CART treatment appear to be due to the expansion of CD19-negative leukemia clones.¹ The UCART22 product candidate will be evaluated in relapsed or refractory CD22 B-ALL, including relapses after CD19 CAR-T administration.”

Acute lymphoblastic leukemia (ALL) is a rapidly progressing form of leukemia that is characterized by the presence of a large number of immature white blood cells in the blood and bone marrow. In 2016, an estimated 6,590 new cases were diagnosed in the U.S., with over 1,400 deaths due to ALL.² Approximately 85 percent of ALL cases involve precursor B-cells (B-ALL).

UCART22 is an allogeneic, off-the-shelf gene-edited T-cell product candidate designed for the treatment of B-ALL. Like CD19, CD22 is a cell surface antigen expressed from the pre B-cell stage of development through mature B-cells and CD22 expression occurs in more than 90 percent of patients with B-ALL.³

¹ Ruella and Maus, 2016
² American Cancer Society
³ Shah et al., 2015
“Given the high unmet medical need for patients who suffer from B-ALL, filing the IND is the first vital step to potentially creating a treatment to be manufactured on an industrial scale, allowing these patients to get the help that they need much faster,” added Prof. Stéphane Depil, Senior Vice President Research & Development and Chief Medical Officer. “We are dedicated to making this a reality as soon as possible and look forward to hitting the ground running with the clinical trial once we obtain regulatory clearance.”

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

Information about ongoing clinical trials is publicly available on dedicated websites such as:

- [www.clinicaltrials.gov](http://www.clinicaltrials.gov) in the U.S.
- [www.clinicaltrialsregister.eu](http://www.clinicaltrialsregister.eu) in Europe

**About Cellectis**

Cellectis is a clinical-stage biopharmaceutical company focused on developing a new generation of cancer immunotherapies based on gene-edited T-cells (UCART). By capitalizing on its 18 years of expertise in gene editing – built on its flagship TALEN® technology and pioneering electroporation system PulseAgile – Cellectis uses the power of the immune system to target and eradicate cancer 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 Euronext Growth (ticker: ALCLS). To find out more about us, visit our website: [www.cellectis.com](http://www.cellectis.com)

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

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achievements expressed or implied by the forward-looking statements. Further information on the risk factors that may affect company business and financial performance is included in Cellectis’ Annual Report on Form 20-F and the financial report (including the management report) for the year ended December 31, 2017 and subsequent filings Cellectis makes with the Securities Exchange Commission from time to time. Except as required by law, we assume no obligation to update these forward-looking statements publicly, or to update the reasons why actual results could differ materially from those anticipated in the forward-looking statements, even if new information becomes available in the future.

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