PRESS RELEASE

# Cellectis Presents Data on Two TALEN®-based Gene Therapy Preclinical Programs for Patients with Sickle Cell Disease and Mucopolysaccharidosis type I at ESGCT 2022

**New York, NY – October 11, 2022** - Cellectis (the "Company") (Euronext Growth: ALCLS - NASDAQ: CLLS), a clinical-stage biotechnology company using its pioneering gene-editing platform to develop life-saving cell and gene therapies, announced today that the Company will present both an oral and poster at the European Society of Gene and Cell Therapy's (ESGCT) 29th Congress, to be held in Edinburgh from October 11-14, 2022.

Arianna Moiani, Ph.D., Senior Scientist & Team Leader Innovation Gene Therapy, will give an oral presentation on encouraging pre-clinical data that leverages TALEN® gene editing technology to develop a hematopoietic stem and progenitor cell (HSPCs)-based gene therapy to treat sickle cell disease.

Eduardo Seclen, Ph.D., Senior Scientist & Team Leader, Gene Editing, will present a poster illustrating a TALEN®-based gene editing approach that reprograms HSPCs to secrete alpha-L-iduronidase (IDUA), a therapeutic enzyme missing in Mucopolysaccharidosis type I (MPS-I).

"The pre-clinical data presented at ESGCT further demonstrate our ability to leverage TALEN® gene editing technology to potentially address genetic diseases, namely, sickle cell disease and lysosomal storage diseases. By correcting a faulty mutation or inserting a corrected gene at the HSPC level, we aim to provide a lifelong supply of healthy cells in a single intervention," said Philippe Duchateau, Ph.D., Chief Scientific Officer at Cellectis. "These new milestones bring us one step closer to our goal: providing a cure to patients that have failed to respond to standard therapy."

## Presentation details

### Pre-clinical data presentation on a non-viral DNA delivery associated with TALEN® gene editing that leads to highly efficient correction of sickle cell mutation in long-term repopulating hematopoietic stem cells

Sickle cell disease stems from a single point mutation in the *HBB* gene which results in sickle hemoglobin.

Cellectis leveraged its TALEN® technology to develop a gene editing process that leads to highly efficient *HBB* gene correction via homology directed repair, while mitigating potential risks associated to *HBB* gene knock-out.

Overall, these results show that non-viral DNA delivery associated with TALEN® gene editing reduces the toxicity usually observed with viral DNA delivery and allows high levels of *HBB* gene correction in long-term repopulating hematopoietic stem cells.

The oral presentation titled “Non-viral DNA delivery associated to TALEN® gene editing leads to highly efficient correction of sickle cell mutation in long-term repopulating hematopoietic stem cells”, will be made on Thursday, October 13th, 8:30AM-10:45AM BST by Arianna Moiani, Ph.D., Senior Scientist & Team Leader Innovation Gene Therapy. The presentation can be found on the Cellectis website on the day of the presentation.

## Presentation details

### Pre-clinical data presentation on TALEN®-mediated engineering of HSPC that enables systemic delivery of IDUA

Mucopolysaccharidosis type I (MPS-I) is caused by deficiencies in the alpha-L-iduronidase (IDUA) gene and it is associated with severe morbidity representing a significant unmet medical need.

Cellectis established a TALEN®-based *ex vivo* gene editing protocol to insert an IDUA-expression cassette into a specific locus of HSPC.

Editing rates *in vivo* were 6-9% sixteen weeks after injection, depending on the tissue analyzed (blood, spleen, bone marrow). Lastly, 8.3% of human cells were edited in the brain compartment.

Cellectis established a safe TALEN®-based gene editing protocol procuring IDUA-edited HSPCs able to engraft, differentiate into multiple lineages and reach multiple tissues, including the brain.

The poster presentation titled “TALEN®-mediated engineering of HSPC enables systemic delivery of IDUA”, will be made on Thursday, October 13th, 5:30PM - 7:15PM BST by Eduardo Seclen, Ph.D., Senior Scientist & Team Leader, Gene Editing, and can be found on Cellectis website.

## About Cellectis

Cellectis is a clinical-stage biotechnology company using its pioneering gene-editing platform to develop life-saving cell and gene therapies. Cellectis utilizes an allogeneic approach for CAR-T immunotherapies in oncology, pioneering the concept of off-the-shelf and ready-to-use gene-edited CAR T-cells to treat cancer patients, and a platform to make therapeutic gene editing in hemopoietic stem cells for various diseases. As a clinical-stage biopharmaceutical company with over 22 years of experience and expertise in gene editing, Cellectis is developing life-changing product candidates utilizing TALEN®, its gene editing technology, and PulseAgile, its pioneering electroporation system to harness the power of the immune system in order to treat diseases with unmet medical needs. Cellectis’ headquarters are in Paris, France, with locations in New York, New York and Raleigh, North Carolina. Cellectis is listed on the Nasdaq Global Market (ticker: CLLS) and on Euronext Growth (ticker: ALCLS).

For more information, visit www.cellectis.com. Follow Cellectis on social media: @cellectis, LinkedIn and YouTube.

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**Forward-looking Statements**

This press release contains "forward-looking" statements within the meaning of applicable securities laws, including the Private Securities Litigation Reform Act of 1995. Forward-looking statements may be identified by words such as "anticipate," "believe," "intend", "expect," "plan," "scheduled," "could," "may" and "will," or the negative of these and similar expressions. These forward-looking statements, which are based on our management's current expectations and assumptions and on information currently available to management. Forward-looking statements include statements about the potential of our preclinical programs and product candidates. These forward-looking statements are made in light of information currently available to us and are subject to numerous risks and uncertainties, including with respect to the numerous risks associated with biopharmaceutical product candidate development. With respect to our cash runway, our operating plans, including product development plans, may change as a result of various factors, including factors currently unknown to us. Furthermore, many other important factors, including those described in our Annual Report on Form 20-F and the financial report (including the management report) for the year ended December 31, 2021 and subsequent filings Cellectis makes with the Securities Exchange Commission from time to time, as well as other known and unknown risks and uncertainties may adversely affect such forward-looking statements and cause our actual results, performance or achievements to be materially different from those expressed or implied by the forward-looking statements. 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.