



PRESS RELEASE

## **American Society of Hematology (ASH) Abstract Shows Initial Anti-Leukemic Activity of UCART22 in BALLI-01 Phase 1 Study in R/R Adult B-ALL**

- Cellectis' Proprietary Program UCART22 was Safely Administered in BALLI-01 Phase 1 Study with No Dose-Limiting Toxicity or Evidence of Graft-Vs-Host Disease
- 2 out of 3 Patients at DL1 Achieved CR/CRi and 1 out of 2 Patients at DL2 Achieved a Significant Reduction in Bone Marrow Blasts
- Abstract Selected for Oral Presentation at ASH Based on Data Cutoff in July 2020 Represents Limited Data Set of 5 Evaluable Patients at DL1 and DL2 with FC Lymphodepletion Regimen
- BALLI-01 Currently Enrolling at DL2 with Addition of Alemtuzumab to the FC Lymphodepletion Regimen; Next Data Update Expected in 2021
- Additional ASH "Trials In Progress" Abstract For AMELI-01 Study with UCART123 In Adult Patients With R/R AML Selected for Poster Presentation
- AMELI-01 Currently Enrolling Additional Cohorts with and without Addition of Alemtuzumab to the FC Lymphodepletion Regimen; DL1 and DL2 of UCART123 Cells with FC Lymphodepletion Regimen Have Cleared Safety Without Dose Limiting Toxicity

**November 4<sup>th</sup>, 2020 – 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 the release of two abstracts at the American Society of Hematology (ASH) Annual Meeting, one oral presentation of initial data for its BALLI-01 clinical trial and one Trials in Progress poster presentation of its AMELI-01 clinical trial. This will be the first publicly released data from Cellectis' Phase 1 dose-escalation study of UCART22 product candidate in adult patients with Relapsed/Refractory CD22+ B-ALL.

“We are pleased with the encouraging preliminary results from patients administered UCART22 cells in our lower dose cohorts with fludarabine and cyclophosphamide lymphodepletion regimen, and are eagerly awaiting additional data from our currently enrolling cohorts that include alemtuzumab in the lymphodepletion regimen,” said Carrie Brownstein, MD, Chief Medical Officer, Cellectis. “We strongly believe in the potential of our innovative product candidates and are looking forward to presenting more data in the near future.”

### **BALLI-01 investigating UCART22 product candidate in R/R B-ALL**

BALLI-01 is a Phase 1 open-label dose-escalation study designed to assess the safety, the maximum tolerated dose (MTD), and preliminary anti-leukemia activity of UCART22 in patients with R/R B-ALL. Additional endpoints include characterization of the expansion, trafficking, and persistence of UCART22 cells.

As of July 2020, seven patients were enrolled. One patient failed screening and one patient was discontinued prior to the administration of UCART22 cells due to an adverse event related to the lymphodepletion.

The abstract includes preliminary data from the first five patients who received escalating doses of UCART22 cells after fludarabine/cyclophosphamide (FC) lymphodepletion. Enrolled patients were predominantly male [n=4], younger (median age 24 [range 22-52]), and heavily pretreated with a median of 3 prior lines of therapy [range 2-4]. The median baseline bone marrow blasts percentage prior to lymphodepletion was 35% [5-78.4%].

Adverse events were mainly mild to moderate in intensity and manageable. Four patients experienced treatment-related, treatment-emergent, adverse events which primarily consisted of abnormalities in liver function tests (i.e. increased alkaline phosphatase, increased bilirubin, and transaminitis), hypotension, fever, and other constitutional symptoms. Cytokine release syndrome was reported in three patients (one patient with Grade 1 and two patients with Grade 2). Two patients experienced serious treatment-emergent adverse events: one patient had Grade 3 febrile neutropenia and Grade 3 hepatic hematoma; one patient had Grade 4 bleeding and Grade 5 sepsis in the context of progressive disease. Importantly, no patients experienced treatment-related serious TEAE, GvHD, ICANS, protocol-defined Dose Limiting Toxicity nor AESI<sup>1</sup>.

Two of three patients in Dose Level 1 achieved an objective response, one patient with best response of CR, and a second patient with CR with incomplete hematologic recovery (CRi). One patient in Dose Level 2 with refractory disease did achieve a noteworthy reduction in bone marrow blasts [40% (Day -1) to 13% (Day 28)] after treatment with UCART22 product candidate.

Host lymphocyte reconstitution was observed in all patients within the DLT period (range Day 17-Day 28). Correlative analysis of UCART cell expansion and persistence is ongoing.

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<sup>1</sup> TEAE: treatment-emergent adverse event; GvHD: Graft versus Host Disease; ICANS: immune effector cell-associated neurotoxicity syndrome; DLT: Dose Limiting Toxicity; AESI: adverse event of special interest

UCART22 demonstrated preliminary signs of activity at low dose levels with fludarabine/cyclophosphamide (FC) lymphodepletion regimen, without unexpected nor significant treatment-related toxicities. CRS was observed in three patients and was mild to moderate and manageable. No patients reported DLT, GvHD nor ICANS<sup>1</sup>. One patient achieved a CR and another a CRi. Host immune recovery was observed early, supporting the addition of alemtuzumab to the FC lymphodepletion regimen which is expected to result in a deeper and more sustained T-cell depletion and thereby promote expansion and persistence of UCART22 cells. Enrollment into the Dose Level 2 cohorts with alemtuzumab is ongoing.

### **Oral Abstract Session:**

#### **Abstract: #163**

**Title:** Preliminary results of BALLI-01: A Phase I study of UCART22 (allogeneic engineered T-cells expressing anti-CD22 Chimeric Antigen Receptor) in adult patients with relapsed or refractory (R/R) CD22+ B-cell Acute Lymphoblastic Leukemia (B-ALL).

**Presenter:** Jain Nitin, MD, The University of Texas MD Anderson Cancer Center, Department of Leukemia, Houston, TX

**Session Name:** 614. Acute Lymphoblastic Leukemia: Therapy, excluding Transplantation: Chimeric Antigen Receptor T Cell Therapy

**Session Release Date & Time:** Saturday, December 5, 2020 at 1:00 PM Pacific Time

### **AMELI-01 investigating UCART123 product candidate in R/R AML**

This abstract is a Trials in Progress presentation. AMELI-01 is a Phase 1, multi-center clinical trial of Cellectis' UCART123 product candidate that employs a modified toxicity probability interval (mTPI) design to evaluate the safety, tolerability and preliminary anti-leukemia activity of UCART123 cells in patients with R/R AML. Additional objectives include the determination of the maximum tolerated dose or suitable lower dose for expansion; characterization of the expansion, trafficking and persistence of UCART123 cells; assessment of cytokine, chemokine and *C-reactive protein* expression after UCART123 cell infusion; and assessment of immune cell depletion, reconstitution and immune response.

Dose escalation will include up to 28 patients. The dose expansion portion follows a Simon 2-stage design and will enroll up to an additional 37 patients. Eligible patients must be ≤ 65 years of age with R/R AML, adequate organ and bone marrow function, a confirmed donor for potential back-up stem cell transplantation, and no > Grade 1 toxicity from prior treatment. Patients with acute promyelocytic leukemia, prior gene or cellular therapy, > 1 allogeneic stem cell transplants, or those with a clinically relevant central nervous system disorder (including CNS leukemia) are not eligible. Patients receive a lymphodepletion regimen of either fludarabine and cyclophosphamide (FC) or fludarabine cyclophosphamide plus alemtuzumab (FCA) starting on Day-5, followed by an infusion of UCART123 cells at one of 5 dose levels on Day 0. Patients are evaluated for the presence of dose-limiting toxicities during a 28-day observation period, which extends to 42 days in the setting of an aplastic marrow and/or persistent clinically significant cytopenia without residual AML. Dose Levels 1 and 2 with FC lymphodepletion regimen have cleared safety without dose limiting toxicity, and enrollment at the next dose levels including cohorts with fludarabine and cyclophosphamide (FC) or fludarabine, cyclophosphamide plus alemtuzumab (FCA) is proceeding.

## Poster Abstract Session:

### **Abstract:** #1039

**Title:** AMELI-01: Phase I, open label dose-escalation and dose-expansion study to evaluate the safety, expansion, persistence and clinical activity of UCART123 (allogeneic engineered T-cells expressing anti-CD123 chimeric antigen receptor), administered in patients with Relapsed/Refractory Acute Myeloid Leukemia

**Presenter:** Gail J. Roboz, MD, Professor of Medicine at Weill Cornell Medicine and New York-Presbyterian, New York, NY

**Session Name:** 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Poster I

**Session Release Date & Time:** Date: Saturday, December 5, 2020

The Virtual Poster Hall will be open for attendees to browse a different set of posters each day. The Poster Hall hours are as follows:

Saturday, December 5: 7:00 AM - 3:30 PM Pacific Time

Sunday, December 6: 7:00 AM - 3:30 PM Pacific Time

Monday, December 7: 7:00 AM - 3:00 PM Pacific Time

Collectis is the sponsor of these Phase 1 clinical trials which are designed to assess the safety and tolerability at increasing dose levels of UCART22 and UCART123 in patients with R/R B-Cell Acute Lymphoblastic Leukemia (B-ALL) and R/R Acute Myeloid Leukemia (AML), respectively.

### **About UCART22**

UCART22 is one of Collectis' wholly owned, allogeneic, off-the-shelf gene-edited T-cell product candidates, designed for the treatment of relapsed and refractory B-cell acute lymphoblastic leukemia (R/R B-ALL). Like CD19, CD22 is a cell surface antigen expressed from the pre-B-cell stage of development through mature B-cells. CD22 expression occurs in more than 90% of patients with B-ALL.

### **About UCART123**

Our wholly controlled product candidate, UCART123, is a gene-edited T-cell investigational drug that targets CD123, an antigen expressed at the surface of leukemic cells in AML.

### **About Collectis**

Collectis is developing the first of its kind 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. As a clinical-stage biopharmaceutical company with over 20 years of expertise in gene editing, Collectis is developing life-changing product candidates utilizing TALEN<sup>®</sup>, its gene editing technology, and PulseAgile, its pioneering electroporation system to harness the power of the immune system in order to target and eradicate cancer cells.

As part of its commitment to a cure, Collectis remains dedicated to its goal of providing life-saving UCART product candidates to address unmet needs for multiple cancers including acute myeloid leukemia (AML), B-cell acute lymphoblastic leukemia (B-ALL) and multiple myeloma (MM).

Collectis headquarters are in Paris, France, with additional locations in New York, New

York and Raleigh, North Carolina. Collectis is listed on the Nasdaq Global Market (ticker: CLLS) and on Euronext Growth (ticker: ALCLS). For more information, visit [www.collectis.com](http://www.collectis.com).

Follow Collectis on social media: [@collectis](#), [LinkedIn](#) and [YouTube](#).

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**Disclaimer**

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 “at this time,” “anticipate,” “believe,” “expect,” “on track,” “plan,” “scheduled,” 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, include statements about the timing and progress of clinical trials (including with respect to patient enrollment and follow-up), the timing of our presentation of data, the adequacy of our supply of clinical vials, the timing of construction and operational capabilities at our planned manufacturing facilities, and the sufficiency of cash to fund operations. 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 duration and severity of the COVID-19 pandemic and governmental and regulatory measures implemented in response to the evolving situation. 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, 2019 and subsequent filings Collectis 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.

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