



## PRESS RELEASE

### **FDA Lifts Clinical Hold on Collectis Phase 1 Clinical Trials with UCART123 in AML and BPDCN**

**November 6, 2017 – New York (N.Y.)** – Collectis (Alternext: ALCLS - Nasdaq: CLLS) a clinical-stage biopharmaceutical company focused on developing immunotherapies based on gene-edited allogeneic CAR T-cells (UCART), today announced that the U.S. Food and Drug Administration (FDA) has lifted the clinical hold, previously announced on September 4, 2017, on Phase 1 trials of Collectis' UCART123 product candidate in acute myeloid leukemia (AML) and blastic plasmacytoid dendritic cell neoplasm (BPDCN).

Collectis agreed with the FDA to the following main revisions to be implemented in Phase 1 UCART123 protocols to lift the hold:

- Decrease of the cohort dose level to  $6.25 \times 10^4$  UCART123 cells/kg;
- Decrease of the cyclophosphamide dose of the lympho-depleting regimen to 750 mg/m<sup>2</sup>/day over three days with a maximum daily dose of 1.33 grams of cyclophosphamide;
- Inclusion of specific criteria at Day 0, the day of UCART123 infusion, such as no new uncontrolled infection after receipt of lymphodepletion, afebrile, off all but replacement dose of corticosteroids, no organ dysfunction since eligibility screening;
- Provision to ensure that the next three patients to be treated in each protocol will be under the age of 65;
- Provision to ensure that the enrollment will be staggered across the UCART123 protocols AML123 and ABC123: at least 28 days should elapse between the enrollments of two patients across the two studies.

Collectis is currently working with the investigators and clinical sites to obtain IRB's approval on the revised protocols and resume patient enrollment.

#### **About UCART123**

Our first 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, as well as on leukemic and other tumoral cells in BPDCN. Collectis received in February 2017 an Investigational New Drug (IND) approval from the U.S. Food and Drug Administration (FDA) to conduct Phase 1 clinical trials with UCART123 in patients with acute myeloid leukemia (AML) and blastic plasmacytoid dendritic cell neoplasm (BPDCN). This marks the first allogeneic, "off-the-shelf" gene-edited CAR T-cell product candidate that the FDA has approved for clinical trials.

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 is led by principal investigator Dr. Gail J. Roboz, Professor of Medicine at Weill Cornell Medicine and Director of the Clinical and Translational Leukemia Programs at Weill Cornell Medicine and NewYork-Presbyterian.

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 is led by Dr Naveen Pemmaraju, MD, Associate Professor, Dr Marina Konopleva, Professor, and Professor Hagop Kantarjian, MD, Department Chair, Department of Leukemia, Division of Cancer Medicine.

Learn more about the ongoing clinical trials at [www.clinicaltrials.gov](http://www.clinicaltrials.gov)

### **About Collectis**

Collectis 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 17 years of expertise in gene editing – built on its flagship TALEN® technology and pioneering electroporation system PulseAgile – Collectis uses the power of the immune system to target and eradicate cancer cells.

Using its life-science-focused, pioneering genome engineering technologies, Collectis' goal is to create innovative products in multiple fields and with various target markets. Collectis 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.collectis.com](http://www.collectis.com)

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

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## **Disclaimer**

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