



## PRESS RELEASE

### **FDA Grants Collectis IND Approval to Proceed with the Clinical Development of UCART123, the First Gene Edited Off-the-Shelf CAR T-Cell Product Candidate developed in the U.S.**

#### *Collectis' UCART123 Product Candidate Targets AML and BPDCN*

**February 6, 2017** – New York (N.Y.) – Collectis (Alternext: ALCLS; Nasdaq: CLLS), a biopharmaceutical company focused on developing immunotherapies based on gene edited CAR T-cells (UCART), has received an Investigational New Drug (IND) approval from the U.S. Food and Drug Administration (FDA) to conduct Phase 1 clinical trials with 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). This marks the first allogeneic, "off-the-shelf" gene-edited CAR T-cell product candidate that the FDA has approved for clinical trials. Collectis intends to initiate Phase 1 trials in the first half of 2017.

UCART123 is a gene-edited T-cell investigational drug that targets CD123, an antigen expressed at the surface of leukemic cells in AML, tumoral cells in BPDCN. The clinical research for AML will be led, at Weill Cornell, by principal investigator Dr. Gail J. Roboz, Director of the Clinical and Translational Leukemia Programs and Professor of Medicine. The UCART123 clinical program for BPDCN will be led, at the MD Anderson Cancer Center, by Dr. Naveen Pemmaraju, MD, Assistant Professor, and Professor Hagop Kantarjian, MD, Department Chair, Department of Leukemia, Division of Cancer Medicine.

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.

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 FDA's approval of Collectis' UCART123 – the first "off-the-shelf" CAR T-cell product candidate to enter clinical trials in the U.S. – is a major milestone not only for the Company but also for the medical community, global biotech and pharmaceutical industries at large," said Dr. Loan Hoang-Sayag, Collectis Chief Medical Officer. "Collectis' allogeneic UCART products have the potential to create an important shift with regard to availability, and cost-effectiveness, to make these therapies widely accessible to patient population across the world."

"After the National Institutes of Health's Recombinant DNA Advisory Committee (RAC)'s unanimous approval of two Phase 1 study protocols for Collectis' UCART123 in

December 2016, the FDA's approval of Collectis' IND is a new major regulatory milestone achieved, for having UCART123 proceed into clinical development and reaching cancer patients in need," added Stephan Reynier, Chief Regulatory and Compliance Officer, Collectis.

Information about ongoing clinical trials are publically 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 Collectis**

Collectis 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. Collectis capitalizes on its 17 years of expertise in genome engineering - based on its flagship TALEN® products and meganucleases as well as its 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, 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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required by law, we assume no obligation to update these forward-looking statements publicly, or to update the reasons 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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