



PRESS RELEASE

## Collectis Reports Clinical Hold of UCART123 Studies

**September 4, 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), announced today having received notice from the U.S. Food and Drug Administration (FDA) that a clinical hold was placed on both UCART123 ongoing Phase 1 studies, respectively in acute myeloid leukemia (AML) and in blastic plasmacytoid dendritic cell neoplasm (BPDCN).

Collectis is working closely with the investigators and the FDA in order to resume the trials with an amended protocol including a lowered dosing of UCART123.

The clinical hold was initiated after Collectis reported one fatality in the BPDCN clinical trial (ABC study). This was the first patient treated in the BPDCN study, a 78-year-old male treated with one prior therapy, who presented with relapsed/refractory BPDCN with 30% blasts in his bone marrow and cutaneous lesions (biopsy-proven BPDCN) at baseline prior to conditioning regimen. He received 30mg/m<sup>2</sup>/day fludarabine for 4 days and 1g/m<sup>2</sup>/day cyclophosphamide for 3 days, as a preconditioning regimen. On August 16, 2017 (Day 0), he received 6.25x10<sup>5</sup> UCART123 cells per kilogram, the first dose level explored in the protocol, without complication. At Day 5, the patient experienced a grade 2 Cytokine Release Syndrome (CRS)<sup>1</sup>, and a grade 3 lung infection, which quickly improved after a first dose of tocilizumab and institution of anti-infective therapy (broad spectrum intravenous antibiotics). He then experienced at Day 8 a grade 5 CRS, together with a grade 4 Capillary Leak Syndrome<sup>2</sup>. Despite a treatment in keeping with CRS management including administration of corticosteroids and tocilizumab x 2 as well as intensive care unit support, the patient died on Day 9.

The first patient treated in the AML study was a 58-year-old woman, with 84% blasts in her bone marrow at baseline prior to conditioning regimen. On June 27, 2017 (Day 0), the patient received the same preconditioning regimen and the same dose of UCART123 as the BPDCN patient, without complication. She experienced an initial grade 2 CRS at Day 8, worsening to a grade 3 at Day 9 and resolving at Day 11 with

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<sup>1</sup> Cytokine release syndrome is an immediate complication occurring with the use of anti-T-cell antibody infusions. Severe cases are known as cytokine storms. CRS is characterized clinically by hypothermia or fever, rigors, hypotension, rash, dyspnea and occasionally bronchospasm, nausea and diarrhea. These side effects develop soon after the administration of the agent and can last for several hours. Severe and even fatal reactions associated with pulmonary edema and hepatitis have been described.

<sup>2</sup> Capillary leak syndrome is characterized by the escape of blood plasma through capillary walls, from the blood circulatory system to surrounding tissues, muscle compartments, organs or body cavities.

treatment management in intensive care unit. She also experienced a grade 4 Capillary Leak Syndrome at Day 9, resolved at Day 12.

No GvHD<sup>3</sup> was reported for any of these patients.

The DSMB (Data Safety Monitoring Board) met on August 28 and recommended lowering the dose to  $6.25 \times 10^4$  UCART123 cells per kilogram in both studies and capping cyclophosphamide to a total dose of 4g over 3 days.

### **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**

This press release contains “forward-looking” statements that are based on our management’s current expectations and assumptions and on information currently available to management. Forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. The risks and uncertainties include, but are not limited to, the risk that the preliminary results from our product candidates will not continue or be repeated, the risk of not maintaining regulatory approval to pursue UCART123 clinical trials, the risk of not obtaining regulatory approvals to commence clinical studies on UCART123 in other countries or on other UCART product candidates, the risk that any one or more of our product candidates will not be successfully developed and commercialized. Further information on the risks factors that may affect company business and financial performance, is included in filings Collectis makes with the Security Exchange Commission from time to

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<sup>3</sup> GvHD: Graft versus Host Disease

time and its financial reports. Except as 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.