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**First patient dosed in first-in-human Phase I - IIa clinical study with Cellestia's proprietary, protein-protein interaction inhibitor CB-103 targeting the NOTCH oncogenic signalling pathway in cancer.**

Cellestia Biotech AG, Basel, Switzerland, January 4<sup>th</sup> 2018.

Cellestia announces today the successful first dosing of cancer patients has started in December 2017 with the lead compound CB-103 in the first-in-human Phase I - IIa clinical study. CB-103 is a proprietary small molecule oncology drug candidate for treatment of solid tumors, lymphomas and leukemias, in which the cancer is driven by oncogenic activation of the NOTCH signalling pathway. It features a new mode of action, focused on the use of a highly potent, selective inhibitor of NOTCH related gene transcription.

Cellestia Biotech AG is a clinical-stage biopharmaceutical company developing first-in-class Protein-Protein Interaction (PPI) inhibitors targeting oncogenic transcription factors. The company is developing an integrated diagnostic program for patient selection alongside the clinical development of CB-103.

This study is a combined Phase I - IIa clinical trial of CB-103 to establish safety and efficacy in a range of cancer indications, treating patients with advanced or metastasized solid tumors, lymphomas and multiple myeloma. The dose-finding Phase I part of the study will be conducted in Europe and will assess safety, tolerability, pharmacokinetics, pharmacodynamics and will establish the recommended dose for further development. The dose-confirmation Phase IIa with expansions in several cancer indications aims to confirm CB-103's safety profile as well as assessing its clinical efficacy in a range of selected indications. It will be conducted across several EU countries and will also include clinical sites in the US following IND filing, which is anticipated in 2018.

"We are very pleased having reached this important milestone for Cellestia. The start of this trial at very experienced clinical sites in Spain, Switzerland and The Netherlands makes an innovative new anti-cancer drug available to patients, who at present have no access to targeted therapy for the treatment of tumors driven by genetic lesions of the NOTCH pathway," commented Dr. Dirk Weber, Chief Medical Officer at Cellestia.

Michael Bauer, Chief Executive Officer at Cellestia, added: "Cellestia's lead compound CB-103 is the first anti-cancer drug that can control NOTCH-driven cancers regardless of the molecular mechanism of NOTCH pathway activation, which is significant due to the wide range of genetic aberrations that can lead to such oncogenic activation. Controlling this selectively at the level of gene transcription is a major breakthrough in targeting this complex cause of cancer. Equally important is the biomarker diagnostic program, which is also well established and is already integrated in this Phase 1 trial."

## MEDIA RELEASE

### About NOTCH signalling pathway

*NOTCH signalling plays a key role in many cellular processes during development. NOTCH is a developmental pathway characterized by cell to cell communication and activation via ligand-receptor interaction. The pathway is known to play critical roles during embryonic development as well as for the regulation of self-renewing tissues. Oncogenic activation of NOTCH signalling leads to deregulation of the self-renewal process resulting in sustained proliferation, evasion of cell death, loss of differentiation capacity, invasion and metastasis, and resistance to chemotherapy, all of which are hallmarks of cancer. Over-activation of the NOTCH signalling pathway can lead to initiation, progression and maintenance of cancer development. Aberrant activation of NOTCH can also induce metastasis, evade apoptosis and is well established to cause resistance against chemotherapy, radiotherapy or other targeted therapies.*

### About CB-103

*Oncogenic activation of the NOTCH signalling pathway leads to expression of different target genes, such as c-MYC, HES, D type Cyclins, etc., ultimately driving the disease. A wide range of molecular mechanisms can lead to NOTCH signalling mediated oncogenic activation, making it a difficult target.*

*CB-103 is a new mode of action, highly potent small molecule protein-protein interaction inhibitor, which is selectively blocking gene transcription of oncogenic target genes triggered by NOTCH pathway activation. Blocking the NOTCH signalling pathway activation in its most downstream part at gene transcription level, by disrupting the NOTCH transcription complex, CB-103 effectively controls the disease as demonstrated in a range of in vitro and in vivo experiments. CB-103 is being developed for oral administration. The compound has excellent drug-like properties, is rapidly absorbed and well distributes into tissue. CB-103 has demonstrated excellent tolerability and efficacy in vitro, in vivo and ex-vivo in blood from leukemia patients, demonstrating clear disease control combined with excellent safety profile. Most importantly, CB-103 does not show the typical toxicities related with gamma secretase inhibitors or receptor/ligand targeting antibodies, due to the distinctly different new Mode of Action.*

### About Cellestia Biotech AG

*Cellestia was founded in 2014 as a spin-off from Ecole Polytechnique Fédérale de Lausanne, EPFL. The lead development compound of Cellestia is CB-103, a novel, first-in-class oral pan-NOTCH inhibitor indicated for treatment of patients with NOTCH-dependent leukemias, lymphomas and solid tumors. Cellestia holds a worldwide exclusive license on the intellectual property rights for CB-103 and related series of analogues, for development and commercialization. The company pursues an integrated approach combining drug and diagnostic development for patient selection.*

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