
Cellestia enters clinical development for its lead compound CB-103, a new mode of action anticancer drug targeting NOTCH positive cancers, with receiving full approval of its first-in-human Phase I – IIa clinical study.

Cellestia Biotech AG, Basel, Switzerland, August 14, 2017.

Cellestia announced today the full approval of clinical trial application for CB-103 Phase I-IIa first-in-human clinical trial in patients with advanced solid cancers and haematological malignancies. CB-103 is a first-in-class oral pan-NOTCH inhibitor with a novel mode of action selectively blocking gene transcription related to NOTCH pathway activation. The trial has been approved by Competent Authority and Ethics Committee in Spain.

Cellestia Biotech AG is a privately owned pharmaceutical company with strategic focus on targeted small molecule anti-cancer drugs modulating the NOTCH pathway by a novel mode of action. Cellestia has developed a discovery platform for compounds targeting the NOTCH signalling pathway selectively at the level of the NOTCH transcription complex. The most advanced program, CB-103, is a novel, oral pan-NOTCH inhibitor with unique new mode of action, indicated for treatment of NOTCH dependent leukemias, lymphomas and solid tumors. CB-103 has achieved proof of concept in various xenograft and patient-derived tumor models of NOTCH driven human cancers.

This Phase I - IIa study of CB-103 is a first-in-human, open-label study investigating the safety, tolerability, pharmacokinetics, pharmacodynamics, biomarker profile and preliminary efficacy of the pan-NOTCH inhibitor CB-103 in adult patients with advanced or metastatic solid tumors and haematological malignancies. Primary objective of the Phase I part is to determine the maximum tolerated dose (MTD) or recommended Phase II dose (RP2D). Purpose of the Phase IIa Part is to confirm safety, and determine single agent preliminary efficacy in a range of different NOTCH-driven cancer indications.

Comprehensive biomarker profiles will be investigated throughout the study, in the Phase IIa part, only patients confirmed for NOTCH positive cancers will be enrolled.

Dirk Weber, Chief Medical Officer of Cellestia stated: "Patients with advanced solid tumours and haematological malignancies which are functionally driven by activated NOTCH signalling generally have a poor survival prognosis. There are no approved NOTCH targeting agents on the market for this well-defined patient group. With CB-103, Cellestia is addressing a highly unmet medical need and we are delighted making a new therapeutic option available to patients." Michael Bauer, Chief Executive Officer of Cellestia added: "Reaching clinical development stage is a major milestone for Cellestia. Integrating development of our new mode of action therapeutic with a dedicated biomarker program for patients' selection represents the start of a new era in treatment of NOTCH positive cancers."

About CB-103

CB-103 is a small molecule protein-protein interaction inhibitor selectively blocking gene transcription of the NOTCH pathway. It inhibits the NOTCH signalling pathway activation in its most downstream part, binding to the NOTCH transcription complex. CB-103 is being developed for oral administration. CB-103 has excellent drug-like properties, it is rapidly absorbed, and rapidly distributes into tissue. CB-103 has demonstrated excellent tolerability and efficacy in vitro, in vivo and ex-vivo in blood from leukaemic patients, demonstrating clear disease control combined with excellent safety profile.

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 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 close analogues, for development and commercialization.

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