Cellestia Biotech receives Orphan Drug Designation from the European Commission for CB-103 for the treatment of Acute Lymphoblastic Leukaemia

Basel, Switzerland – 16 December 2021– Cellestia Biotech AG (Cellestia) is a fully integrated R&D company specialised in the discovery and development of first-in-class Gene Transcription Factor inhibitors (GTFi) and establishing new standards of care in currently un-treatable human diseases. Today Cellestia announced that the European Commission (EC) has granted Orphan Drug Designation for its lead clinical product CB-103 for the treatment of Acute Lymphoblastic Leukaemia (ALL).
CB-103 is an oral, first-in-class NOTCH-ICD/CSL gene transcription factor inhibitor that potently down-regulates the expression of key oncogenes such as cMyc, Cyclin D1, HES and many other cancer-promoting target genes. CB-103 is being tested in an international Phase 1/2a multicentre, open-label, dose-escalation study with expansion arms in adult patients with locally advanced or metastatic solid tumours and haematological malignancies characterised by alterations of the Notch signalling pathway. In this trial, CB-103 has demonstrated potent disease control, strong target engagement and an outstanding safety profile. The study is open for enrolment in Europe, USA and Asia.

“Receiving Orphan Drug Designation from the European Commission represents an official recognition from EMA that CB-103 looks promising in bringing significant benefit to patients affected by ALL. The survival prospect for patients with NOTCH positive T-cell ALL refractory to available therapies is dismal. With CB-103 treatment, such a patient has recently achieved a complete response and was able to undergo stem cell transplantation” said Dr. Florian D. Vogl, CMO of Cellestia. “The Orphan Drug Designation is an important milestone in our efforts to make CB-103 available to patients suffering from rare diseases who have limited or no other treatment options.”

Dr. Michael Bauer, CEO and co-founder of Cellestia stated: “Cellestia´s recent clinical data confirm that CB-103 can deliver a successful therapy for patients suffering from multi-drug resistant cancers in indications where no standard of care is available. Advancing the development of CB-103 for treatment of multi-drug resistant advanced stage cancers, Cellestia is integrating drug development with a sophisticated biomarker programme for patient selection and on-therapy, personalised biomarker driven treatment choices. In delivering individualised therapies for patients with advanced, multi-pathway driven cancers, we are setting new standards in clinical development.”

About Orphan Drug Designation
The EC grants orphan drug designation to drugs and biologics intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition that is rare (affecting not more than five in 10,000 people in the European Union). Companies who obtain orphan drug designation benefit from special incentives, including protocol assistance and possible regulatory fee reductions, and market exclusivity for 10 years once the medicine is on the market.

About ALL
Acute lymphoblastic leukaemia (ALL) is a heterogeneous group of lymphoid disorders resulting from clonal proliferation of immature lymphocytes of B-cell (80%) or T-cell (20%) lineages in the blood, bone marrow, and other lymphoid organs. ALL occurs in a bimodal age distribution, with approximately 60% of diagnosed cases occurring in patients less than 20 years old and over 25% of diagnosed cases occurring in adult patients over 45 years old. The expected number of new cases of ALL in the EU is approximately 5,370 people per year.

About Cellestia Biotech

Cellestia is one of the leading companies active in discovery and development of first-in-class gene transcription factor inhibitors. This innovative approach has successfully led to a pipeline of proprietary drug candidates. The lead molecule CB-103 is a first-in-class inhibitor of the NOTCH-ICD/CSL transcription factor complex. CB-103 is very well tolerated as single agent and combination therapy, with confirmed clinical efficacy in the ongoing clinical trial. It is the first drug that can control NOTCH-ICD/CSL signalling effectively and safely, with clinical proof of concept established in several oncology indications and GvHD. 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 personalised medicine development for patient selection and individualised therapies.

Media Inquiries

Dr. Michael Bauer, CEO
+41 61 633 29 80
michael.bauer@cellestia.com

Gaudenz von Capeller, CFO
+41 79 798 64 43
gaudenz.voncapeller@cellestia.com