CELLERON THERAPEUTICS

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Celleron Therapeutics announces EMA COMP Orphan Drug designation for CXD101 in peripheral T-cell lymphoma

Oxford, UK, 6 December 2017 - Celleron Therapeutics, the UK-based company developing personalised medicine for cancer patients, has today announced the grant of EU Orphan Drug Designation for Celleron's lead compound CXD101 for peripheral T-cell lymphoma.

On 8 November, 2017 the European Commission implemented the decision of the European Medicines Agency (EMA) Committee for Orphan Medicinal Medicinal Products (COMP) of 5 October 2017 to grant Orphan Drug Designation for CXD101, the medicinal product containing N-(2-aminophenyl)-4-(1-[(1,3-dimethyl-1H-pyrazol-4-yl)methyl]piperidin)benzamide.

CXD101 is a next generation epigenetic immune-regulator representing a class of drug that kills cancer cells by blocking certain vital functions involved in gene expression, and reactivates the patient's immune system so that cancer cells can no longer evade immune recognition.

The COMP considered that the requirements under Article 3(1)(a) of Regulation (EC) No 141/2000 on orphan medicinal products are fulfilled on the basis that Celleron's application had established that:

- the intention to treat peripheral T-cell lymphoma with CXD101 was justified, based on preliminary clinical observations in patients affected by the condition, who responded to treatment with the proposed product;
- the condition is life-threatening and chronically debilitating due to poor response to therapy and high rate of relapses. Clinical presentation and course varying from an indolent clinical behaviour for years in milder subtypes, to fulminant disease in aggressive sub-types.
- the condition was estimated to be affecting less than 1 in 10,000 persons in the European Union, at the time the application was made.

A major challenge in the development of cancer drugs is that cancer patients will respond differently to treatment with any particular therapy. Clinical trials with Celleron's CXD101 drug are investigating not only the efficacy of the new drug but also studying a novel biomarker test, known as a companion diagnostic, to predict which patients will respond best to treatment with the drug. This approach avoids the problem of treating patients who have little chance of benefiting from the treatment so that alternative options may be considered in a timely manner.

Professor Nick La Thangue, Founder and Chief Executive of Celleron Therapeutics, and Professor of Cancer Biology in the Department of Oncology at Oxford University, commented:

"This orphan designation will be very helpful in supporting the further development of CXD101 in peripheral T-cell lymphoma. Work is underway to pursue this challenge in cooperation with Celleron's development partners. CXD101 is a potent, orally administered drug representing a new class of agents with dual mode of action that not only act directly on cancer cells but also stimulate the patient's immune system to fight the tumour. We are seeing extremely encouraging results and we look forward driving the clinical trials forward as fast as possible, in a number of defined cancers, using our personalised treatment approach".

Dr John Whittaker, Celleron's Chief Operating Officer commented:

"I am delighted to see the achievement of this positive step in Celleron's strategy to progress its proprietary targeted therapeutic, CXD101, a product which opens up new and exciting opportunities for treating aggressive types of cancer".

About Celleron Therapeutics

Celleron Therapeutics, based on the Oxford Science Park, UK, is a drug development company focussed on precision medicine for cancer. It is a spin-out of Oxford University and has secured a number of exclusive licence agreements with pharmaceutical companies, including Astra Zeneca. Celleron's precision medicine approach is supported by a companion diagnostic biomarker platform, which allows new drugs to be tailored to responsive tumours. Celleron has two Phase 2 clinical assets: CXD101 is a novel dual mechanism HDAC inhibitor which has unique immuno-modulatory effects in tumour cells, and CXD201 represents a new type of topoisomerase inhibitor.