

Press release

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AML Therapy:

Nature Medicine publishes phase 1 study with encouraging results for cusatuzumab

Today in Nature Medicine: Results from a phase 1 study conducted by the Department of Oncology, Inselspital, Bern University Hospital and Bern University show positive results for a novel therapy for acute myeloid leukemia (AML). The new approach is based on the monoclonal anti-CD70 antibody cusatuzumab designed to block the CD27/CD70 pathway and directly target the leukemia stem cells. The study will be published today (29.06.2020) in Nature Medicine.

AML has an annual average incidence of about 3 patients per 100,000 of the population with a marked increase to well over 20 in 70 to 85-year-olds. People in this age group tend to be more fragile. Therefore, intensive chemotherapies can no longer be carried out. The previous standard therapy had a modest response rate and remained unchanged for decades. Based on the positive results of the phase 1 study published today, the research partners will pursue in-depth investigation with the hope to offer a new therapy option to AML-patients.

Breakthrough

Interim results of the phase 1 study were presented at the American Society for Haematology (ASH) in Orlando, FL in December 2019. Today the study is published in Nature Medicine. In the phase 1 study, a response rate of 100% was demonstrated. At the same time, a promising tolerability profile was observed. Likewise, in a four-stage toxicity test, no relevant toxicity was detected even at the highest dose. The tolerability profile is of great importance in the 70 years-plus age group. Study leader, Prof. Ochsenbein, Department Chairman and Chief Physician states: *“Until now we had insufficient resources to combat AML in older patients. We have reason to hope that with the monoclonal anti-CD70 antibody cusatuzumab we will eventually have an effective remedy in our hands.”*

Cusatuzumab – Mode of action

AML is a disease of the hematopoietic system in which the leukemia stem cells play a decisive role by multiplying and forming differentiated leukemia cells (blasts). The CD27/CD70 pathway plays a central role in the proliferation and maintenance of the leukemia stem cells in AML. Cusatuzumab

intervenes in this process in two ways: on the one hand, the proliferation of leukemia stem cells is stopped. This is done through the antibody binding to CD70, which leads to an interruption of the signalling pathway CD27/CD70. Furthermore, leukemia stem cells that express CD70 on the surface are directly attacked. According to the current state of research, the monoclonal anti-CD70 antibody cusatuzumab is able to inhibit the new formation of leukemia stem cells actively with minimal side effects. It thus intervenes in a decisive step in the chain of action of AML and will be studied further in the quest of a new treatment option for a priori fragile AML patients over 70 years old.

A special feature of this developmental work is the close cooperation with partners from the private sector and the many years of experience and focus of the Department of Oncology at the Inselspital, Bern University Hospital on research into the CD27/CD70 signalling pathway in leukemia. Prof Dr Adrian Ochsenbein and PD Dr Carsten Riether have been researching the CD27/CD70 signalling pathway for over 20 years. The Bern Oncology Department has become an international reference centre for AML immunotherapy.

Outlook

Cusatuzumab research will now focus on phase 2 studies and aims to verify the data of the phase 1 study in a larger patient population. Carsten Riether, first author of the study is confident: *“We were very pleased with the positive patient response in the phase 1 study. We’ll now test the efficacy of cusatuzumab together with our partners in different centres worldwide and hope to have a good drug for the treatment of AML on hand as soon as possible”.*

Experts:

- Prof Dr med. Adrian Ochsenbein, Department Chairman and Chief Physician, University Department of Medical Oncology, Inselspital, Bern University Hospital
- PD Dr sc. nat. Carsten Riether, Head of Research, University Department of Medical Oncology and Department of Biomedical Research, Inselspital, Bern and Bern University.

Study:

- Targeting CD70 with cusatuzumab eliminates acute myeloid leukemia stem cells in patients treated with hypomethylating agents. DOI: 10.1038/s41591-020-0910-8
- Link: <https://www.nature.com/articles/s41591-020-0910-8> (from June 29, 5pm)

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Insel Gruppe

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