# Research prize for the gene therapy of rare diseases

**The Eva Luise and Horst Köhler Foundation for People with Rare Diseases has awarded the 2011 Eva Luise Köhler Research Prize, which includes prize money of 50,000 euros, to an interdisciplinary team of researchers: Professor Dr. Christoph Klein, Department of Paediatric Haematology and Oncology at the Hannover Medical School (MHH), Professor Dr. Christopher Baum, Department of Experimental Haematology (MHH), Professor Dr. Christoph von Kalle, National Centre for Tumour Diseases (NCT) in Heidelberg and Dr. Kaan Boztug from the Medical University of Vienna who worked in the Department of Paediatric Haematology and Oncology (MHH) until January 2011.**

"We are very pleased to be awarded the renowned Eva Luise and Horst Köhler Foundation prize. We will use the prize money to establish an interdisciplinary platform for the gene therapy of rare diseases," said Professor Christoph Klein. The awardees have carried out pioneering work in the field of rare diseases in Germany. The team, led by Professor Christoph Klein, has developed a gene therapy approach to treating children with Wiskott-Aldrich syndrome (WAS), a rare recessive disease caused by a mutation in the WAS gene located on the short arm of the X chromosome. The disease is characterised by severe infections, bleeding, autoimmune disorders and is also known to lead to leukaemia and lymphomas in affected children.

The interdisciplinary team of researchers has developed a gene therapy which involves the removal of haematopoietic stem cells from patients. These autologous stem cells are then purified and coupled to gene shuttles that transport a healthy copy of the disease-causing gene into the patient's cells. The genetically corrected cells can then differentiate into healthy blood cells. Since WAS is primarily a disorder of the blood-forming tissues, autologous haematopoietic stem cell transplants now make it possible to cure an otherwise fatal condition.

"Gene therapy usually places less strain on the patient than the transplantation of donated foreign haematopoietic stem cells, which is frequently associated with complications due to incompatibility responses of the immune system to foreign cells," said Dr. Kaan Boztug. However, the new gene therapy method is also associated with risks. One of the ten patients treated with autologous stem cells developed leukaemia, which was nevertheless successfully treated with chemotherapy. In cooperation with the team led by Professor Christof von Kalle from the National Centre for Tumour Diseases (NCT) in Heidelberg, who also works at the German Cancer Research Center (DKFZ) in Heidelberg, the physicians are concentrating on the insertion sites of the gene shuttles on the chromosomes with the goal of reducing the risk of undesired side effects. "These investigations will help us to further reduce the risks in future applications and increase the efficiency of WAS gene therapy," said Professor von Kalle.

Professor Christopher Baum's group of researchers at the MHH is developing new, safer gene shuttles in order to reduce the risk of undesired side effects without however compromising the therapeutic effect of the clinical therapy. The researchers will use the Eva Luise Köhler Research Prize funds to establish a modern interdisciplinary platform for the treatment of children suffering from genetic blood and immune system diseases.

**Further information:**
Hannover Medical School
Stefan Zorn
Head of Public Relations
Carl-Neuberg-Straße 1
30625 Hannover
Tel.: +49 511 532-6772
Fax: +49 511 532-3852
E-mail: zorn.stefan(at)mh-hannover.de

National Centre for Tumour Diseases (NCT)
Alenka Tschischka
Press and Public Relations
Im Neuenheimer Feld 460

69120 Heidelberg
Tel.: +49 6221 42-2254
Fax: +49 6221 42-2968
E-mail: Alenka.Tschischka(at)nct-heidelberg.de

---

**Press release**

05-Mar-2011
Source: NCT Heidelberg, 01.03.2011