UNTERSTÜTZT DURCH DAS

NETZWERK SELTENE ERKRANKUNGEN BADEN-WÜRTTEMBERG KOMPETENZZENTRUM

International Symposium on Gene Therapy versus Haploidentical Stem Cell Transplantation

Concepts and Limitations

Organized on the occasion of the **40th anniversary**
of the University Medical Centre Ulm
pediatric stem cell transplantation program

May 31 – June 1, 2014

Stadthaus Ulm, Germany

► for your **Registration**, please fill in this form and send it via

FAX: +49-(0)4736102536 or
E-Mail: digel.f@t-online.de

► and please transfer the **Registration fee** of **€ 50,-** to

Di-Text Frank Digel
Kreissparkasse Köln
Kto. 0197001323, **BLZ** 37050299
IBAN DE 15370502990197001323
BIC COKSDE33

before **May 15th 2014**

First Name _____

Last Name_____

Institution_____

Address_____

Email_____

Phone_____

FAX_____

For **Hotel reservations** please contact the tourist Information Ulm/ Neu-Ulm. You can book your room online via the following link:
www.tourismus.ulm.de/tourismus/en/index.php

The **"Stadthaus Ulm"** is right next to the Ulm Cathedral in the centre of the City.

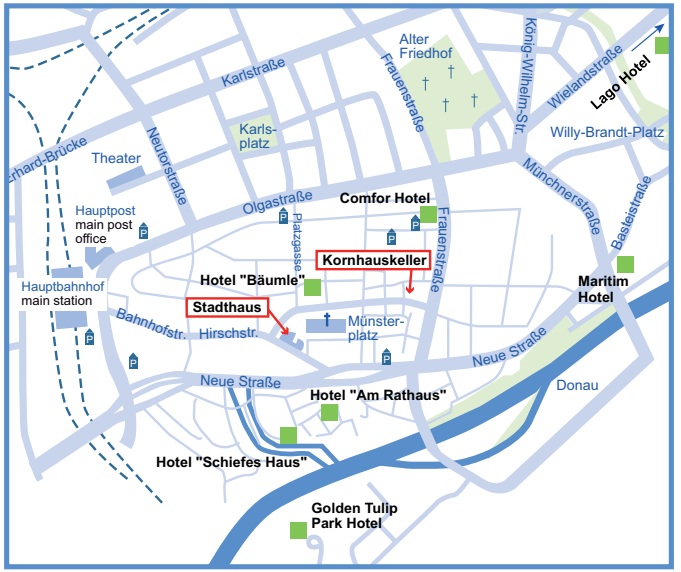
Hotels in walking distance are:

- Maritim Hotel Ulm, Basteistraße 40
- Golden Tulip Park Hotel Neu-Ulm, Silcherstraße 40
- Hotel "Schiefes Haus" Ulm, Schwörhausgasse 6
- Comfor Hotel, Frauenstraße 51
- Hotel am Rathaus, Kronengasse 8-10

easily reached by public transport:

- Lago Hotel, Friedrichsaustrasse 50

We kindly invite you to join us for dinner in the **Kornhauskeller** on Saturday evening (Hafengasse 19)



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Dear Collegues,

understanding of Primary Immnuodeficiencies has advanced almost exponentially over the last decades. Simultaneously, effective treatment strategies have been developed to a point, where an increasing proportion of patients has a realistic chance of complete cure of their otherwise fatal disease. Bone marrow and hematopoietic stem cell transplantation from an allogeneic donor have been central to this advance, and more recently transplantation of genetically modified autologous hematopoietic cells has been added with exciting results.
The Children's Hospital of Ulm University, looking back at 40 years of experience in the management of PID and in stem cell transplantation, wants to seize this opportunity to bring together a range of scientists with expertise in the field of allogeneic transplantation and in gene therapy for PID. With this symposium, which is organized by members of the Department of Pediatrics and the Institute of Transfusion Medicine of the University Medical Center Ulm, we want to offer a platform to present and discuss recent advances, to identify current limitations, and to define future directions in the application of both HLA-nonidentical stem cell transplantation and of gene therapy in the treatment of PID. Based on presentations by leading experts, the meeting should offer a chance for extensive discussions and exchange of ideas. We encourage all participants to take an active part in these discussions, and we hope, above all, that the meeting will help to further improve the management of our patients with these rare and complex disorders.

The Organizing Commitee
Klaus-Michael Debatin, Manfred Hönig,
Catharina Schütz, Ansgar Schulz, Klaus Schwarz

University Medical Center Ulm

Saturday, May 31st 2014

9:00 **Welcome**
Klaus-Michael Debatin, Ulm

Haploidentical SCT in primary immunodeficiencies
Richard O'Reilly, New York

Haploidentical SCT for X-SCID
Manfred Hönig, Ulm

Haploidentical SCT for B-negative SCID
Catharina Schütz, Ulm

Haploidentical SCT for ADA-deficiency
Bobby Gaspar, London

Haploidentical SCT for Wiskott-Aldrich Syndrome and other PIDs
Andy Gennery, Newcastle upon Tyne

Break

Current approaches to enhance immune reconstitution
Andrea Velardi, Perugia

New approaches in graft manipulation
Rupert Handgretinger, Tübingen

Concepts to enhance thymic maturation
Naomi Taylor, Montpellier

13:00 *Lunch*

Gene therapy for X-SCID
Alain Fischer, Paris

Gene therapy for ADA-deficiency
Alessandro Aiuti, Milano

Gene therapy for SCID - The American perspective
Donald Kohn, Los Angeles

Gene therapy for WAS
Christoph Klein, München

Lentivirus mediated gene therapy in WAS
Alessandro Aiuti, Milano

Gene therapy for CGD
Manuel Grez / Janine Reichenbach, Frankfurt / Zürich

Break

Regulatory aspects of genetically modified cells
Matthias Renner, Langen

The integration profiles of gene therapy vectors
Christof von Kalle, Heidelberg

Gene therapy vectors: past, present, future
Christopher Baum / Axel Schambach, Hannover

Do we need a virus for gene therapy? – A bifocal perspective on genome editing
Toni Cathomen, Freiburg

20:00 *Dinner at the Kornhauskeller*
Hafengasse 19

Sunday, June 1st 2014

9:00 **Stem cell regulation and development of gene therapy for Diamond Blackfan Anemia**
Stefan Karlsson, Lund

c-kit antibody for myeloid ablation
Judith Shizuru, Stanford

Break

Panel Discussion

Gene therapy and mismatched SCT: Potentials and limitations
Introduction by Alain Fischer
Future directions by Richard O´Reilly

12:30 *End of meeting*