

LMU

KLINIKUM

DER UNIVERSITÄT MÜNCHEN

DR. VON HAUNER CHILDREN'S HOSPITAL

3RD INTERNATIONAL KLAUS BETKE SYMPOSIUM

Clinical and immunological aspects of inflammatory
bowel diseases in children

February 28 - March 1, 2017
(public program)

SCIENTIFIC PROGRAM



SPEAKERS

Antoine Chabloz

Institute for Molecular Biology, Vienna, Austria

Judy Cho

Icahn School of Medicine at Mount Sinai, New York, USA

Anne Griffiths

The Hospital for Sick Children (SickKids), Toronto, Canada

Christoph Klein

Dr. von Hauner Children's Hospital, LMU, Munich, Germany

Sibylle Koletzko

Dr. von Hauner Children's Hospital, LMU, Munich, Germany

Daniel Kotlarz

Dr. von Hauner Children's Hospital, LMU, Munich, Germany

Dermot McGovern

Cedars-Sinai Medical Center, L.A., USA

Aleixo Muise

The Hospital for Sick Children (SickKids), Toronto, Canada

Edward Nieuwenhuis

Wilhelmina Children's Hospital, University Medical Center Utrecht, Utrecht, The Netherlands

Fiona Powrie

University of Oxford, Oxford, UK

David Relman

Stanford University, Stanford, USA

Tobias Schwerd

Dr. von Hauner Children's Hospital, LMU, Munich, Germany

Scott Snapper

Boston Children's Hospital, Boston, USA

Dan Turner

Shaare Zedek Medical Center, Jerusalem, Israel

WELCOME ADDRESS

Dear guests, colleagues and friends,

on behalf of all members of the Dr. von Hauner Children's Hospital I welcome you to the third International Klaus Betke Symposium!

Four years ago, we initiated this interdisciplinary conference to build bridges between clinical medicine and science and to bring physicians and scientists together. Our first and second meeting focused on red blood cells and neutrophil granulocytes, respectively. This year's symposium highlights clinical and immunological aspects of inflammatory bowel diseases in children. Together with Dr. Scott Snapper at Boston Children's Hospital and Dr. Aleixo Muise at SickKids in Toronto we launched a global initiative to shed light on the genetic etiology of very early onset inflammatory bowel diseases and to develop novel therapies for these rare diseases. This "VEO-IBD Consortium" is generously supported by the Helmsley Charitable Trust and I am particularly thankful that we can combine our annual meeting of this initiative with the Klaus Betke Symposium. Building alliances is key, together we are strong.

Children with rare diseases are disadvantaged in many respects. Our meeting coincides with the 10th International Rare Disease Day on February 28. Clinicians and scientists, patient organizations and philanthropic foundations work together to increase public awareness for patients with rare diseases. The Rare Disease Day slogan is 'With research, possibilities are limitless' – let us join forces in the best interest of children with rare diseases, in Munich, in Germany, and around the globe!

thank all speakers and the audience for their contributions as well as all supporters, in particular the Helmsley Charitable Trust and the Else-Kröner-Fresenius-Stiftung for their generous help!

I wish you all a pleasant and inspiring symposium!



Prof. Dr. Christoph Klein



PROGRAM

Tue, February 28, 2017

- 2.00 p.m.** Welcome address – **Christoph Klein**
- 2.25 p.m.** Symposium of the Else Kröner Fresenius Clinical Research School Munich “Rare diseases of the immune system”
- Short talks of the fellows:
- Alexandra Puchwein-Schwepcke**
Florian Gothe
Fabian Hauck/Tilmann Schober
Maximilian Witzel
Yoko Mizoguchi
Sebastian Hesse
Richard Klaus
- 3.15 p.m.** COFFEE BREAK & POSTER SESSION
- 4.00 p.m.** Musical introduction
- 4.10 p.m.** Award ceremony: Dr. August von Hauner Award to **Anne Griffiths** (University of Toronto)
- Laudatory speech – **Sibylle Koletzko**
- Talk by **Anne Griffiths**
- Discussion on VEO-IBD and the “Orphans of Medicine” with Anne Griffiths, Sibylle Koletzko, Hans Clevers, Scott Snapper, Garabet Yeretsian
- 5.00 p.m.**
- 5.45 p.m.** Final remarks
- 6.00 p.m.** Musical farewell
- RECEPTION

Wed, March 1, 2017

9.00 a.m. Registration

SESSION 1

9.30 a.m. Welcome address – **Christoph Klein, Aleixo Muise, Scott Snapper**

9.40 a.m. **Judy Cho**
“Genetics and personalized therapy of IBD”

10.05 a.m. **Sibylle Koletzko**
“Nutrition in pediatric IBD: impact on disease and therapeutic option”

10.30 a.m. **Daniel Kotlarz**
“VEO-IBD – a model to study genetic factors controlling intestinal homeostasis”

10.55 a.m. COFFEE BREAK

SESSION 2

11.25 a.m. **Edward Nieuwenhuis**
“N.N.”

11.50 a.m. **Aleixo Muise**
“Novel players in VEO-IBD”

12.15 noon **Scott Snapper**
“Bridging genetics to molecular functions to therapy”

12.40 noon LUNCH & POSTER SESSION

PROGRAM

SESSION 3

1.50 p.m.

Antoine Chabloz

"Engineered Bacteria as a platform for IBD therapy"

2.15 p.m.

Fiona Powrie

"Gut reactions: Immune pathways in the intestine in health and diseases"

2.40 p.m.

David Relman

"The human microbiome: stability during health and disease"

3.05 p.m.

COFFEE BREAK

3.35 p.m.

Short Talks Selected from Abstracts

SESSION 4

4.20 p.m.

Tobias Schwerd

"Bacterial handling defects in early onset IBD"

4.45 p.m.

Dermot McGovern

"Genotype x Phenotype in IBD"

5.10 p.m.

Dan Turner

"Specifications of treatments in early onset IBD"

5.35 p.m.

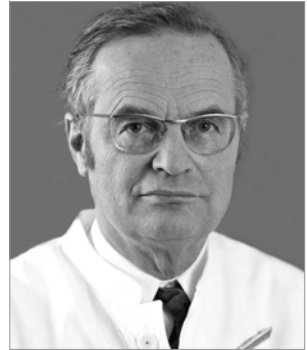
Summary, Final Remarks, Farewell

6.30 p.m.

DINNER at Schlosswirtschaft Schwaige

PROFESSOR DR. KLAUS BETKE

Klaus Betke, 1914 born in Munich, received his Dr. med. at the University of Berlin in 1940 for his thesis titled, "Assoziationen bei genuiner und symptomatischer Epilepsie." In 1953, he habilitated at the Albert-Ludwigs-University Freiburg with his work titled "Human haemoglobin in the fetal and mature forms." In 1962, he was appointed to the Chair of Pediatrics in Tübingen. Betke then succeeded Alfred Wiskott (1898-1978) at the Ludwig-Maximilians-University Munich. From 1967 until 1983, he held the position of Director and Professor of the Children's Hospital, Children's Clinic, and Pediatric Surgery at the Dr. von Hauner Children's Hospital of LMU Munich.



*Prof. Dr. Dr. h.c. Klaus Betke
(* 30.10.1914; † 26.06.2011)*

Betke was highly esteemed in the field of pediatrics in Germany. In 1966 he became an honorary member of the Gynecology and Pediatrics section of the German Academy of Sciences Leopoldina. From 1968 until 1971, Betke was a member of the Science Council. In 1981, he was awarded the Bavarian Order for Science and Art. He was a member of both the Bavarian Academy of Sciences and Academy of Finland.

Betke has been a long-time member of the German Society of Pediatrics and an honorary member since 1991. In 1983, the German Society of Pediatrics awarded him the Otto Heubner Prize, the most important award of the German Society of Pediatrics and Adolescent Medicine (Deutsche Gesellschaft für Kinder- und Jugendmedizin). He has also received the Bavarian Order of Merit. Additionally, he is an honorary member of the University of Regensburg and has received honorary doctorates from the medical faculty of the Ruhr University in Bochum.

Klaus Betke was a passionate pediatrician. He brought forward the development of the pediatric sub-disciplines and thus formed the field of pediatrics in Germany sustainably.

DR. AUGUST VON HAUNER MEDAL TO PROFESSOR ANNE GRIFFITHS



Prof. Anne Griffiths

Prof. Anne Griffiths, MD, receives the Dr. August von Hauner Medal for her outstanding work as a paediatrician and researcher in the field of paediatric inflammatory bowel disease.

Anne Griffiths studied medicine and graduated from the University of Toronto. She trained in Paediatrics and Paediatric Gastroenterology at The Hospital for Sick Children, and in Clinical Epidemiology at the University of Toronto. She has a broad interest in science and translational research and her work has inspired numerous colleagues around the world. She is considered one of the foremost international leaders in patient-based research related to inflammatory bowel disease

(IBD). Early in her career she was one of the pediatric leaders in the NIH (NIDDK) and International Paediatric and Adult IBD Genetics Consortia studying IBD genetic susceptibility. Her efforts, in part, have led to a number of high impact publications describing the IBD genetic risk in both pediatric and adult IBD patients. Under her leadership at The Hospital for Sick Children, the IBD program has become one of the first Pediatric centers to integrate clinical care and research, thereby making SickKids one of the top IBD centres in the world. She has significantly contributed to clinical trial design and outcome assessment in paediatric IBD, spearheading the development of a disease-specific measure of health-related quality of life (IMPACT questionnaire), and of the Paediatric Ulcerative Colitis Activity Index (PUCAI) that are both used in all pediatric IBD clinical trials today. Anne Griffiths is now the Principal Investigator of the Canadian Children Inflammatory Bowel Disease Network (CID-sCaNN), a pan-Canadian Network focused on understanding the pathogenesis and optimizing treatment of children with IBD. Possibly her most important contribution to the field has been her role in the training and mentoring of numerous Canadian and international pediatric gastroenterologist. Her trainees have become world leaders in the pediatric IBD and most contribute their success to their mentor Anne Griffiths.

In order to remember **Dr. August von Hauner**, founder of Dr. von Hauner Children's Hospital built in 1846, the Dr. August von Hauner Medal is awarded biennially to an outstanding personality who strongly influenced the field of pediatrics by working as a „physician-scientist“.



10TH INTERNATIONAL RARE DISEASE DAY

28th February 2017 – The 10th Rare Disease Day

Children with rare diseases are the „orphans of medicine“ – their fate barely enters public consciousness. They are neither in the focus of translational research nor the pharmaceutical industry. However, rare diseases are actually not that rare when looking at them as a whole: In Germany alone, about 4 million people suffer from a rare disease. Around 7,000 rare diseases are known today and this number is growing constantly. Many of the rare diseases remain incurable to date and often there are no effective options for treatment. As a consequence, still today about 2,000 children die from rare diseases in Germany every year.

To raise awareness for rare diseases world-wide, the international Rare Disease Day was launched in 2008 by networks of patient organisations: EURORDIS and the Council of National Alliances. Since then, it takes place on the last day of February every year. Succeeding in its vision to reach out to the general public as well as policy makers, public authorities, industry representatives, researchers and health professionals, the Rare Disease Day has grown dramatically since its creation. The number of participating countries has increased year-on-year, with thousands of events taking place on all continents for Rare Disease Day 2016. In total, events have taken place in over 100 countries since 2008.

This year's Rare Disease Day slogan is ‚With research, possibilities are limitless‘. Only through enhanced research activities and international cooperation we can change the fortune of patients with rare diseases. Defining the molecular mechanisms of diseases is key to improving care, developing novel treatment options, and curative therapies. Therefore, patient-oriented research for rare diseases is the mission of the research initiatives and international alliances presented at the 3rd International Klaus Betke Symposium. We want to take this opportunity to raise awareness, discuss recent developments, and expand networks to the benefit of all patients suffering from rare diseases.



ELSE KRÖNER FRESENIUS CLINICAL RESEARCH SCHOOL MUNICH



Else Kröner Fresenius Clinical
Research School München

ELSE KRÖNER-FRESENIUS-STIFTUNG
Forschung fördern. Menschen helfen.

The **Else Kröner Fresenius Clinical Research School Munich** “Rare diseases of the immune system – from pathophysiology to new therapies” was initiated in 2014 and is an excellence program for junior physician-scientists. The fellows are granted a leave of absence from their clinical duties for 6–18 months to pursue a research project in the field of rare diseases of the immune system.

The main focus of the program is on monogenic diseases of the immune system: reaching from classical primary immune defect diseases to diseases with organ-specific inflammation and specific susceptibility for defined microbial pathogens. Besides the research groups of Prof. Christoph Klein and Prof. Johannes Hübner at the Dr. von Hauner Children’s Hospital, also groups of the Gene Center of the Ludwig-Maximilians-Universität München (Prof. Veit Hornung and Prof. Karl-Peter Hopfner), the Max-Planck-Institute for Biochemistry (Prof. Julia von Blume) and the Department of Pharmacology of the University Hospital of the LMU (Prof. Stefan Endres and PD Dr. Sebastian Kobold) participate in the program aiming at fostering translational research in and beyond Munich. The program is funded by the Else Kröner-Fresenius-Stiftung that is dedicated to advancing medical research and medical-humanitarian projects.

During the first three years of the program eight fellows were supported. They will present their work – some of which has already been published in top-tier journals – in short talks and on posters on 28th of February.



*Back: Florian Gothe, Gabriel Zorn, Sebastian Hesse,
Alexandra Puchwein-Schwepcke, Christoph Klein
Front: Yoko Mizuguchi, Tilman Schober, Maximilian Witzel
Missing: Richard Klaus*

<http://www.klinikum.uni-muenchen.de/Else-Kroener-Fresenius-Research-School>
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VEO-IBD Consortium



Inflammatory Bowel Diseases (IBD) comprise a heterogeneous group of disorders, phenotypically classified as Crohn's disease, ulcerative colitis, and IBD unclassified. Although the exact causative factors for IBD are often unknown, experimental studies and genetic evidence indicate that it is likely caused by a combination of intertwined factors: immune dysfunctions, environmental factors, imbalances of the microbial flora, and genetic susceptibility. Children with Very Early Onset Inflammatory Bowel Disease (VEO-IBD) (onset < 6 years) develop a disease that is significantly different from forms observed in adults. These children often fail to respond to conventional therapies or surgery.

To study the molecular basis of VEO-IBD and develop individualized treatments, Dr. Aleixo Muise (The Hospital for Sick Children, Toronto), Dr. Scott Snapper (The Boston Children's Hospital, Boston) and Dr. Christoph Klein (Dr. von Hauner Children's Hospital, Munich) have established an international and interdisciplinary **VEO-IBD Consortium** that is funded by The Leona H. and Harry B. Helmsley Charitable Trust. The consortium is supported by 10 founding investigators who are world experts in the fields of gastroenterology, immunology, genetics, bioinformatics, microbiomics, and intestinal stem cell biology. Moreover, the VEO-IBD Consortium expanded its network and collaborates with over 100 clinical and academic centers worldwide. Thus, the consortium provides a unique platform to analyze molecular causes of VEO-IBD in a systems biology approach and develop innovative tailored therapies. The clinical and immunological aspects of the disease are the main focus of the 3rd International Klaus Betke Symposium. In the context of the symposium, members of the VEO-IBD Consortium will present state-of-the-art clinical and research data.

For more information, please visit the VEO-IBD Consortium's website <http://veoibd.org/>

We thank our supporters for their contribution!



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