



# 4TH ANNUAL DRUG DISCOVERY AND DEVELOPMENT SYMPOSIUM FOR PULMONARY HYPERTENSION

July 10-11, 2017  
Hilton Berlin.  
Mohrenstrasse 30  
Berlin, Germany

## **THE PATHWAY TO BREAKTHROUGH THERAPIES**

This meeting will focus on promising therapies in clinical development, new targets, and changes in clinical trial designs that can lead to successful clinical trials and faster drug approval. The faculty includes world renowned academic experts and members of the EMA.



**PVRI**  
Pulmonary Vascular  
Research Institute

INSIGHTS FROM  
LEADERS IN  
ACADEMIA,  
INDUSTRY, AND  
REGULATORY

PROMISING TARGETS  
READY FOR CLINICAL  
DEVELOPMENT

INNOVATIVE CLINICAL  
TRIAL DESIGNS FOR  
FASTER APPROVAL

PULMONARY  
VASCULAR DISEASES

RIGHT HEART  
FAILURE

SPONSORED BY THE  
PULMONARY  
VASCULAR RESEARCH  
INSTITUTE

PARTICIPATION FROM  
THE EUROPEAN  
MEDICINES AGENCY

**4th ANNUAL DRUG DISCOVERY AND DEVELOPMENT SYMPOSIUM  
FOR PULMONARY HYPERTENSION**

**HILTON Berlin**

**Mohrenstrasse 30, Berlin, Germany**

**July 10-11, 2017**

The Drug Discovery and Development for Pulmonary Hypertension Symposium is held annually each summer to bring leading scientists in the fields of pulmonary vascular disease, right heart failure, and clinical trial designs together with the pharmaceutical industry and regulatory authorities to help identify the most promising treatments for future development. For each meeting we assemble a faculty with exceptional achievements and expertise, and choose topics in areas that will influence the development of therapies immediately. Each topic will be critically reviewed by an expert panel, and open dialogue will allow your voice to be heard. Attendees will experience how novel therapies can best be developed towards successful clinical trials from the input of the world's leading authorities.

This symposium serves as a unique forum for stakeholders interested in treatment of pulmonary vascular diseases for the sharing of cutting-edge science with the input of international thought leaders. For researchers involved in the treatment of pulmonary hypertension this meeting should stimulate new ideas and approaches. For the pharmaceutical and biotechnology industries that have an interest in pulmonary vascular diseases, right heart failure or orphan diseases, it is a meeting that should not be missed. The meeting by design allows for opportunities for interaction among colleagues during meeting and at the evening reception.

**PROGRAM CHAIRPERSONS:**

**Ardeschir Ghofrani M.D.**

**John H. Newman, M.D.**

**Stuart Rich, M.D**

## Meeting Agenda

**Day 1: Monday, July 10<sup>th</sup> 2017**

**Session #1: 8:30 to 12:30**

### **New Drugs for Pulmonary Hypertension Entering Clinical Trials**

**Moderator:** John Newman

**Expert Panel:** Marion Delcroix, Nazzareno Galie

- **8:30 – 9:30 A Study of Ubenimex in Patients With Pulmonary Arterial Hypertension (LIBERTY)**
  - inhibitor of leukotriene A<sub>4</sub> hydrolase, (anti-inflammatory/antiproliferative)
  - ClinicalTrials.gov NCT02664558
  - Phase 2 (active)
  - Sponsor: Eiger BioPharmaceuticals
  - **Presenter: Norbert Voelkel, VU University Medical Center**
  
- **9:30 – 10:30 Tocilizumab in the Treatment of Pulmonary Arterial Hypertension (TRANSFORM-UK)**
  - monoclonal antibody against the interleukin-6 receptor (IL-6R)
  - ClinicalTrials.gov NCT02676947
  - Phase 2 (open label)
  - Sponsor: Papworth Hospital NHS Foundation Trust and Roche Pharma AG
  - **Presenter: Mark Toshner, University of Cambridge**
  
- **10:30 – 11:30 ABI-009 for Severe Pulmonary Arterial Hypertension (WHO FC III and IV)**
  - mTOR Inhibitor (inhibits the mechanistic target of rapamycin)
  - ClinicalTrials.gov NCT02587325
  - Sponsor: Aadi, LLC, and University of Pittsburgh
  - **Presenter: Elena Goncharova, University of Pittsburgh**
  
- **11:30 – 12:30 UPDATE: Tyrosine kinase inhibitors as a treatment of pulmonary hypertension. Lessons learned and future challenges.**
  - **Presenter: Ralph Schermuly, University of Giessen**
  
- **12:30 – 13:30 Lunch**

## Session #2: 1:30 – 5:30

### Novel Drugs for Pulmonary Vascular Diseases Ready for Clinical Trials

**Moderator:** Ardeschir Ghofrani

**Expert Panel:** Jean-Luc Vachieri, Laura Price

- **13:30 – 14:30 KAR5585**
  - inhibitor of tryptophan hydroxylase 1 (TPH1)
  - ClinicalTrials.gov NCT02746237
  - Phase 1 completed
  - Sponsor: Karos Pharmaceuticals
  - **Presenter: Margaret MacLean, University of Glasgow**
  
- **14:30 – 15:30 MFC1040**
  - MIF inhibitor that blocks vascular cell proliferation and perivascular inflammation
  - Orally available, small molecule antagonist of MIF
  - Sponsor: MIFCARE
  - **Presenter: Marc Humbert, Université Paris-Sud**
  
- **15:30 – 16:30 CXA-10 (10-nitro-oleic-acid)**
  - modulator of Nrf2 and NF- $\kappa$ B,
  - Phase 1 completed, phase 2 planned
  - Sponsor: Complexa Inc
  - **Presenter: Tanja Rudolph, University of Cologne**
  
- **16:30 – 17:30 Inhaled sodium nitrite in pulmonary hypertension associated with heart failure with preserved ejection fraction**
  - ClinicalTrials.gov NCT01431313
  - Phase 2 trial completed
  - Sponsor: Mast Therapeutics and NIH
  - **Presenter: Marc Simon, University of Pittsburgh**
  
- **17:30 -19:00 Cocktail Reception**

## Day 2: Tuesday July 11<sup>th</sup>, 2017

### Session #3: 8:30 – 12:30

#### Issues and Challenges in Clinical Trials for Pulmonary Vascular Diseases

**Moderator:** Stuart Rich

**Expert Panel:** Gerald Simonneau, Joanna Pepke-Zaba

- **8:30 – 9:30** Precision medicine approach to rare disease therapies. The European Medicines Agency view.
  - **Presenter:** Krishna Prasad, Member, Cardiovascular Working Party, The European Medicines Agency
  
- **9:30 – 10:30** UPDATE: Strategies to alter miRNA expression that can be employed in clinical trials for pulmonary vascular disease and right ventricular failure.
  - **Presenter:** Stefanie Dimmeler, University of Frankfurt
  
- **10:30 – 11:30** Peripheral balloon angioplasty in the treatment of CTEPH: Review of the clinical experience.
  - **Presenter:** Christoph Wiedenroth, Kerckhoff-Klinik, Bad Nauheim
  
- **11:30 – 12:30** UPDATE: Implantable drug delivery pumps for chronic intravenous therapies for pulmonary vascular disease and right ventricular failure.
  - **Presenter:** Manuel Richter, University of Giessen
  
- **12:30 – 12:45** INTRODUCTION: The PVRI/Pharma Taskforce
  - **Presenter:** Paul Corris, President of the PVRI

12:45 pm

Adjourn

## Invited Faculty

**Paul A Corris, MD**

Professor of Thoracic Medicine  
Institute of Cellular Medicine,  
University of Newcastle, UK

**Marion Delcroix, MD**

Department of Clinical and Experimental Medicine  
Service Pneumology  
University Hospitals, Leuven, Belgium

**Stefanie Dimmeler, PhD**

Head of Molecular Cardiology Institute of Cardiovascular Regeneration  
Goethe-University Frankfurt, Germany

**Nazzareno Galie, MD.**

Head, Pulmonary Hypertension Center  
Institute of Cardiology, University of Bologna, Italy

**Hossein-Ardeschir Ghofrani, MD.**

Professor for Pulmonary Vascular Research, Head of Pulmonary  
Hypertension Division, Medical Clinics II/V, Department of Internal  
Medicine, University Hospitals Giessen and Marburg GmbH (UKGM),  
Justus-Liebig University, Giessen, Germany

**Elena Goncharova, PhD**

Director, VMI Cell Processing Core and Animal Hypoxia Core  
Associate Professor of Medicine,  
Division of Pulmonary, Allergy and Critical Care Medicine  
University of Pittsburgh School of Medicine

**Marc Humbert, MD. PhD.**

Professor of Respiratory Medicine  
Vice Chair National Reference Centre for Pulmonary Hypertension  
Université Paris-Sud

**Margaret MacLean, PhD**

Professor of Pulmonary Pharmacology  
Research Institute of Cardiovascular and Medical Sciences  
University of Glasgow, UK

**John H. Newman, MD.**

Professor of Medicine  
Elsa S. Hanigan Chair in Pulmonary Medicine,  
Vanderbilt University School of Medicine, Nashville USA

**Joanna Pepke-Zaba, PhD, FRCP**

Director of National Pulmonary Vascular Diseases Unit  
Papworth Hospital NHS Foundation Trust  
University of Cambridge, UK

**Krishna Prasad MD, MRCP, FRCP**

Member, Cardiovascular Working Party, European Medicines Agency  
Medicines and Healthcare Products Regulatory Agency,  
UK Member of Pharmacogenomics

**Laura Price, BSc PhD MBChB MCRP**

Consultant Respiratory Physician  
National Pulmonary Hypertension Service  
Royal Brompton Hospital  
Imperial College London, UK

**Stuart Rich, MD.**

Professor of Medicine  
Northwestern University Feinberg School of Medicine  
Director, Pulmonary Vascular Disease Program  
Bluhm Cardiovascular Institute  
Northwestern Memorial Hospital, Chicago USA

**Manuel Richter, MD**

Department of Pneumology, Kerckhoff Heart, Rheuma and Thoracic Center,  
Bad Nauheim, University of Giessen, Germany

**Tanja K. Rudolph, MD**

Department of Cardiology  
University Heart Center Cologne  
University of Cologne, Germany

**Ralph Schermuly, PhD.**

Extraordinary Professor  
Pathophysiology and Experimental Pulmonology,  
Justus-Liebig University of Giessen, Germany

**Marc A. Simon, MD, MS**

Associate Professor of Medicine, Bioengineering, and Clinical Translational Science  
Head of Translational Research, UPMC Comprehensive Pulmonary Hypertension Program  
UPMC Heart & Vascular Institute  
University of Pittsburgh, Pittsburgh, USA

**Gerald Simonneau, MD**

National Reference Center for Pulmonary Hypertension  
Service de Pneumologie et Réanimation  
Hôpital Bicetre  
Université Paris-Sud 11, France

**Mark Toshner, MD**

Consultant Respiratory Physician, Papworth Hospital  
Pulmonary Vascular Diseases Unit, Papworth Hospital,  
University of Cambridge, UK

**Christoph Wiedenroth, MD**

Kerckhoff Heart and Lung Center  
German Centre for Cardiovascular Research  
Bad Nauheim, Germany

**Jean-Luc Vachiery, MD**

Director of the Pulmonary Vascular Diseases and Heart Failure Clinic.  
Associate Professor in Cardiology  
Erasmus University Hospital in Brussels, Belgium

**Norbert Voelkel, MD**

Affiliate Professor  
VU University Medical Center, Amsterdam.