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.

SPONSORED BY THE PULMONARY VASCULAR RESEARCH INSTITUTE
PARTICIPATION FROM THE EUROPEAN MEDICINES AGENCY

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
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:**

Ardeshir Ghofrani M.D.

John H. Newman, M.D.

Stuart Rich, M.D.
Meeting Agenda

Day 1: Monday, July 10th 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 A₄ 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 Vachiery, 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-κB,
  - 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 11th, 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

  - **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
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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
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Jean-Luc Vachiery, MD
Director of the Pulmonary Vascular Diseases and Heart Failure Clinic.
Associate Professor in Cardiology
Erasme University Hospital in Brussels, Belgium

Norbert Voelkel, MD
Affiliate Professor
VU University Medical Center, Amsterdam.