WELCOME TO
BETHESDA

8th Annual Drug Discovery & Development Symposium

Date // 10-11 July 2023

Hotel // Marriott • Bethesda
Welcome to Bethesda...
10-11 July 2023

Welcome to our 8th Annual Drug Discovery & Development Symposium for Pulmonary Hypertension.

It is our great pleasure to welcome you back to our first face-to-face Drug Discovery and Development Symposium since the Covid lockdown.

The Symposium brings together leading scientists, clinicians, pharma colleagues and regulators in the field of pulmonary vascular disease and clinical trial design. It is a unique forum for sharing cutting-edge science, and as ever, we have assembled a faculty of exceptional expertise and achievement, and chosen topics that we hope may influence the development of therapies and improve patients’ lives.

As always, our aim is to foster genuine collaboration and creativity, so we hope that you will enjoy the opportunities to network with old friends and new during the next two days.

We are greatly indebted to our organisers Mark Toshner, Sylvia Nikkho, Peter Fernandes and Raymond Benza who have put together an outstanding programme for us. Together, these four PH experts also make up the Leadership Team that oversees our Innovative Drug Development Initiative workstreams, and on behalf of our members, we would like to thank them for their tireless work in this role.

We would also like to thank the wonderful line-up of speakers and panellists for their enthusiastic acceptance of our invitation, and for agreeing to share their skills, knowledge and experience.

And finally thank you to you, our attendees, for what we know will be some invaluable scientific contributions.

We hope you enjoy the event and look forward to a productive and inspiring meeting!

Karen Osborn
PVRI CEO
London, UK

Professor Kurt Stenmark
PVRI President 2023/24
University of Colorado Anschutz Medical Campus School of Medicine, USA
PVRI IDDI Drug Discovery Meeting
Bethesda Marriott Hotel, USA
10-11 July 2023

The decision to reintroduce the PVRI's Drug Discovery Meeting, following recommendations from our collaborating colleagues from Pharma, affords me the opportunity, as Chairman of the PVRI, to pen a few words in appreciation of Stuart Rich, who has recently resigned from the Board of Directors.

It was Stuart’s vision that led to the PVRI developing this meeting in the first place, aiming to bring academics, colleagues from Pharma, and regulatory bodies together to discuss and debate important mutually relevant issues pertaining to novel drug development. The meeting rapidly gained traction within the PH community, and it is a real pleasure that we will meet once again in Bethesda. I anticipate a vibrant, effective, and enjoyable meeting, typified by robust scientific debate.

The PVRI Board will miss Stuart’s wise counsel and experience, but his resignation affords us the opportunity to continue efforts to increase diversity in our Board membership, so important for a Global Organisation like PVRI.

We wish him well in his new endeavours and thank him most sincerely for his service.

Professor Paul Corris
PVRI Chairman, Emeritus Professor of Thoracic Medicine,
Faculty of Medical Sciences, Newcastle University, Visiting Academic Fellow & Teaching associate, Green Templeton College University of Oxford, Chairman PVRI, UK
Introducing Our Co-Chairs...

**Raymond Benza**

Dr. Benza is the Network Director for Advanced Heart failure and Pulmonary Vascular Diseases and the Associate Chief Academic Officer at the Catholic Health Network, St Francis Hospital and Heart Center in New York. He is a fellow of the American College of Physicians, the American Heart Association Council on Clinical Cardiology, the American College of Chest Physicians, and the American College of Cardiology. He is a diplomate of the American Board of Internal Medicine with certification in the subspecialties of cardiovascular diseases and advanced heart failure and transplantation. Dr. Benza currently holds a position on the Governance Committee at the International Society of Heart and Lung Transplantation and is the former treasurer of the society. He served on President Barack Obama’s Advisory Committee on Organ Transplantation (ACOT) in the Health Resources and Services Administration for 4 years and was knighted in 2015 by His Royal Highness Vittorio Emanuele of Italy for his philanthropic work in patients with pulmonary arterial hypertension.

**Peter Fernandes**

Peter Fernandes serves as the Chief Executive Officer of Bellerophon Therapeutics, in addition to also heading up the Regulatory, Safety, and Quality departments that he has held since joining Bellerophon in 2015. Prior to joining Bellerophon, Peter was Vice President of Global Regulatory Affairs at Ikaria Inc., from 2012 to 2015. Previously, he led Regulatory Affairs and Quality Assurance for OptiNose, Inc., was Vice President of US Drug Regulatory Affairs Respiratory and US DRA Respiratory Franchise Head for Novartis Pharmaceuticals. He has also served as the Head of the US Development Site and Vice President of Regulatory Affairs and Quality Assurance at Altana Pharma, a subsidiary of Nycomed Inc., and led the US Respiratory and GI Drug Regulatory Affairs group at Boehringer Ingelheim. Peter also serves as Co-Chair of the Pulmonary Vascular Research Institute (PVRI) Innovative Drug Development Initiative that is instrumental in developing novel regulatory endpoints and clinical trial design for Pulmonary Hypertension (PH) in collaboration with Academia, Industry, and Regulators. Peter has an M. Pharm. from the Grant Medical College and a B. Pharm, from the K.M. K College of Pharmacy, both at the University of Bombay in India.
Mark Toshner
Dr Toshner is an Associate Professor at the University of Cambridge where he is the Director of the Heart & Lung Institute NIHR Clinical Research Facility.
He is the Executive Director of the UniPHy UK clinical trials network and is funded by the BHF, NIHR, and MRC.
Dr Toshner co-chairs the Pulmonary Vascular Research Institute Innovative Drug Discovery Initiative Global Alliance and has an interest in novel experimental trial designs in cardiorespiratory disease.
He is an honorary consultant in the Pulmonary Vascular Diseases Unit at Royal Papworth Hospital where he is also the Director of Pulmonary Vascular Research.

Sylvia Nikkho
Dr. Sylvia Nikkho a Board-certified Specialist in Internal Medicine with experience covering a broad range of disease areas.
She has been leading as director and global clinical lead of the clinical drug discovery & development of pulmonary & cardiovascular drugs, drug-device combination products, gene- and cell therapy as well as contrast media over 26 years at Schering and Bayer AG.
The highlights of her pharmaceutical career were the successful development of treatments for orphan, life-threatening pulmonary vascular diseases such as Ventavis® (inhaled iloprost) and Adempas (oral riociguat) from the initial idea through the approval in various countries and regions.
Dr. Nikkho has been appointed as a Senior Science Fellow in recognition of her scientific expertise and engagement at Bayer AG recently.
She serves as Co-Chair of the Pulmonary Vascular Research Institute (PVRI) Innovative Drug Development Initiative with main interest in developing novel clinical endpoints, biomarkers, and clinical trial design for adults as well as children in collaboration with Academia, Industry and Regulators.
# Day 1 Session 1

## Welcome

- **Kurt Stenmark** PVRI PRESIDENT 2023

### 08:25-08:30

**Digital Health Technology (DHT) advancements & implementation at the FDA, Foundation for National Institutes of Health (FNIH) & pharma to optimise clinical trial designs & endpoints in PH**

- **Chairs:** Christine Garnett & Peter Fernandes
- **Panellists:** Veronica Franco & Harry Rowland

**FDA Center for Devices & Radiological Health (CDRH) DHT Center of excellence: an overview**

- **Speakers:** Troy Tazbaz & Matt Diamond

**FDA Center for Drug Evaluation & Research (CDER) Division of Cardiology & Nephrology (DCN) DHT initiatives: adults & paediatrics**

- **Speaker:** Christine Garnett

### 09:10-09:30

**Panel & delegate discussion**

### 09:30-09:45

**FNIH, pharma & academia pioneering of digital endpoints**

**FNIH Initiative to further the Clinical Outcome Assessment (COA) development of DHT endpoints**

- **Speakers:** Josh Fessel & Jessilyn Dunn

**Taking the plunge with actigraphy: USA & China (lessons learnt)**

- **Speaker:** Peter Fernandes

**Application of the CardioMEMS in the artisan trial**

- **Speaker:** Yan Liu

### 10:15-10:40

**Break**

### 11:00-11:15

**Novel Trial Designs**

**The decentralised trial design: overcoming the hidden obstacles**

**FIT-PH/ UK cohort study - deciphering the rosetta stone of digital endpoints**

- **Speakers:** Mark Toshner, Alex Rothman & Martin Wilkins

### 11:15-11:40

**Panel & delegate discussion**

### 11:40-11:55

**Stratified & adaptive trial designs**

- **Speaker:** Michael Harhay
<table>
<thead>
<tr>
<th>Event</th>
<th>Time</th>
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<tr>
<td>What can Bayesian adaptive designs offer for trials in rare disease?</td>
<td>11:55-12:10</td>
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<tr>
<td>Speaker: Sofía Villar</td>
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<tr>
<td>Putting the Physician in the corner: the Patient-Centred trial</td>
<td>12:10-12:25</td>
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<tr>
<td>Speaker: Joanna Pepke-Zaba</td>
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<tr>
<td>Panel &amp; delegate discussion</td>
<td>12:25-13:00</td>
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**Lunch**                                                                                     | 13:00-14:00 |

**Day 1 Session 2**                                                                            | 14:00-17:00 |

**Lessons learnt from clinical trials in PH groups 2 & 3: What are the similarities & differences in pathogenesis & pathophysiology?**
- Chairs: Paul Corris & Steven Nathan
- Panellists: Jordan Pomeroy & Sylvia Nikkho

**Pulmonary Hypertension Group 2**                                                             | 14:00-17:00 |

**Pathophysiology & pathogenesis**                                                             | 14:00-14:20 |
- Speaker: Raymond Benza

**In adults**                                                                                   | 14:20-14:40 |
- Speaker: Raymond Benza

**In paediatrics**                                                                              | 14:40-15:00 |
- Speaker: Rachel Hopper

**Panel discussion & debate**                                                                    | 15:00-15:30 |

**Pulmonary Hypertension Group 3**                                                              | 14:00-17:00 |

**Pathophysiology & pathogenesis**                                                             | 15:30-15:50 |
- Speaker: Paul Hassoun

**In adults**                                                                                   | 15:50-16:10 |
- Speaker: Steven Nathan

**In paediatrics**                                                                              | 16:10-16:30 |
- Speaker: Steven Abman

**Panel discussion & debate**                                                                    | 16:30-17:00 |

**Drinks reception**                                                                            | 17:00-19:00 |
Day 2 Session 1  08:30-13:30

Novel drug & device therapies
• Chairs: Anna Hemnes & Raymond Benza
• Panellists: Stephanie Hopley, Carol Satler & Hunter Gillies

Novel compliance enhancing device therapy: Initial data from 28 implanted patients in WHO groups 1, 2 & 3 in PH 08:30-08:40
• Speaker: John Scandurra

Targeting Histone Deacetylase (HDAC) inhibition for pulmonary hypertension 08:40-08:55
• Speaker: Björn Dahlöf

Panel & delegate discussion 08:55-09:05

TGF-β ligands for pulmonary arterial hypertension 09:05-09:20
• Speaker: Dena Grayson
Discussion 09:20-09:25

Targeting the zinc transporter, ZIP12, with a monoclonal antibody 09:25-09:40
• Speaker: Lan Zhao
Discussion 09:40-09:45

Panel & delegate discussion 09:45-10:00

Mechanism of action in inhaled therapies: Why do they work & how do we design the trials? 10:00-10:45
• Speaker: Nicholas Hill

Break 10:45-11:10

Disruptive technologies in novel drug discovery

Using large data aggregates 11:10-11:30
• Speaker: Steven Kawut

Molecular clustering 11:30-11:50
• Speaker: Allan Lawrie

Imaging & the lung 11:50-12:10
• Speakers: Alex Dusek & Benjamin Lavon

Panel & delegate discussion 12:10-12:30

Roundtable: Practical solutions to challenges in drug development 12:30-13:30
• Chairs: Mark Toshner, Sylvia Nikkho, Raymond Benza & Peter Fernandes
Highlight 10 challenges in drug development, vote on the top 3 & solve them

END OF MEETING
OVER TO YOU

PVRI2024 Annual Congress London
31 Jan - 3 Feb 2024
Steven Abman  
- Professor of Pediatrics  
- University of Colorado School of Medicine & Children’s Hospital Colorado, USA

Raymond Benza  
- Professor & Network Director  
- Advanced Heart Failure & Pulmonary Vascular Diseases & Associate Chief Academic Officer  
- Catholic Health Network, St Francis Hospital & Heart Center Long Island New York, USA

Paul Corris  
- Emeritus Professor of Thoracic Medicine  
- Faculty of Medical Sciences, Newcastle University  
- Visiting Academic Fellow & Teaching associate, Green Templeton College University of Oxford, Chairman PVRI, UK

Björn Dahlöf  
- CMO, CSO, Head of Clinical Development  
- Cereno Scientific & Gothenburg University, SWEDEN

Matthew Diamond  
- CMO, Digital Health Center of Excellence, Center for Devices & Radiological Health (CDRH) US Food & Drug Administration (FDA), USA

Jessica Dunn  
- Assistant Professor, Duke University, USA

Alex Dusek  
- CCO, Polarean Imaging, USA

Peter Fernandes  
- CEO Bellerophon Therapeutics, USA

Josh Fessel  
- Senior Clinical Advisor, Division of Clinical Innovation, Office of the Director, National Center for Advancing Translational Sciences, USA

Veronica Franco  
- Professor of Medicine, Section of Pulmonary Vascular Disease, Advanced Heart Failure & Transplantation, The Ohio State University, USA

Christine Garnett  
- Clinical Reviewer, Division of Cardiology & Nephrology, US Food & Drug Administration (FDA), USA

Hunter Gillies  
- CMO, Aerovate Therapeutics, USA

Dena Grayson  
- SVP of Clinical Development  
- Keros Therapeutics, Inc., USA

Michael Harhay  
- University of Pennsylvania, USA

Ben Lavon  
- CSO, Fluidda, USA

Paul Hassoun  
- Director, Pulmonary Hypertension Program, Professor of Medicine, Johns Hopkins University School of Medicine, USA

Anna Hemnes  
- Division of Allergy, Pulmonary & Critical Care Medicine, Vanderbilt University Medical Center, USA

Nicholas Hill  
- Chief, Division of Pulmonary, Critical Care & Sleep Medicine  
- Professor of Medicine, Tufts University School of Medicine, USA

Stephanie Hopley  
- Program Director, Apollo Therapeutics, USA

Rachel Hopper  
- Clinical Associate Professor of Pediatrics (Division of Cardiology), Stanford University School of Medicine & Lucile Packard Children’s Hospital, USA

Steven Kawut  
- Perelman School of Medicine, the University of Pennsylvania, USA
Allan Lawrie  
Imperial College London, UK

Yan Liu  
United Therapeutics, USA

Steven Nathan  
H.O. Advanced Lung Disease & Transplant Program Inova Fairfax Hospital, USA

Sylvia Nikkho  
Senior Science Fellow, Global Clinical Lead, Bayer AG, Germany

Joanna Pepke-Zaba  
Consultant Respiratory Physician, Senior Honorary Fellow, University of Cambridge, Royal Papworth Hospital NHS Foundation Trust, UK

Jordan Pomeroy  
Medical Officer, Division of Cardiology & Nephrology, CDER, FDA, USA

Alex Rothman  
Wellcome Trust Clinical Research Career Development Fellow & Honorary Consultant Cardiologist, The University of Sheffield & Sheffield Teaching Hospitals NHS Foundation Trust, UK

Harry Rowland  
CEO, Co-founder, Endotronic, USA

Carol Satler  
President & CMO, Respira Therapeutics, USA

John Scandurra  
CSO, Aria CV, Inc, USA

Kurt Stenmark  
PVRI President, University of Colorado Anschutz Medical Campus School of Medicine, USA

Troy Tazbaz  
Director of Digital Health Center of Excellence, USA Food & Drug Administration (FDA), USA

Mark Toshner  
University Lecturer, University of Cambridge School of Clinical Medicine, Director of Translational Biomedical Research MPhil, Honorary Consultant Papworth Hospital NHS Trust, UK

Sofía Villar  
MRC Investigator - Programme Leader Track MRC Biostatistics Unit, University of Cambridge, UK

Martin Wilkins  
Imperial College London, UK

Lan Zhao  
Professor of Experimental Medicine, National Heart & Lung Institute, Academic Director of Biological Imaging Centre Faculty of Medicine, Imperial College London, UK

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The official journal of the PVRI, *Pulmonary Circulation* is a leading forum for communication in the fields of pulmonary circulation and pulmonary vascular disease.

With a focus on translational science, the journal connects researchers and physicians in all parts of the world. An Open Access journal, articles are freely available to read immediately on publication. The journal’s goal is to advance our understanding of physiology and disease mechanisms pertaining to the pulmonary circulation and right heart, and to become the journal of choice for researchers in this field of science.

The Editors welcome article submissions including clinical and basic research in humans and animals, randomised controlled trials, intervention studies, and outcome studies. Articles published in *Pulmonary Circulation* will make a major impact not only on future research but also on improvement of health outcomes in our field.

**Why publish in *Pulmonary Circulation*?**

- Fast turnaround times.
- 20-day target from submission to first decision.
- Immediate dissemination of articles to 6,000 PVRI members and affiliates, the global population of leading scientists and physicians studying the pulmonary circulation and pulmonary vascular disease.
- Article-level metrics for real-time updates on when your article has been read, cited or shared.
- Articles published Open Access allowing immediate access to all readers.
- Impact Factor 2.886.

Editors in Chief:
- Jason X.-J. Yuan MD, University of California San Diego, USA
- Anna R. Hemnes MD, Vanderbilt University Medical Center, USA

Deputy Editors:
- Kurt R. Stenmark MD, University of Colorado Denver, USA
- Irene Lang MD, University of Vienna, Austria

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