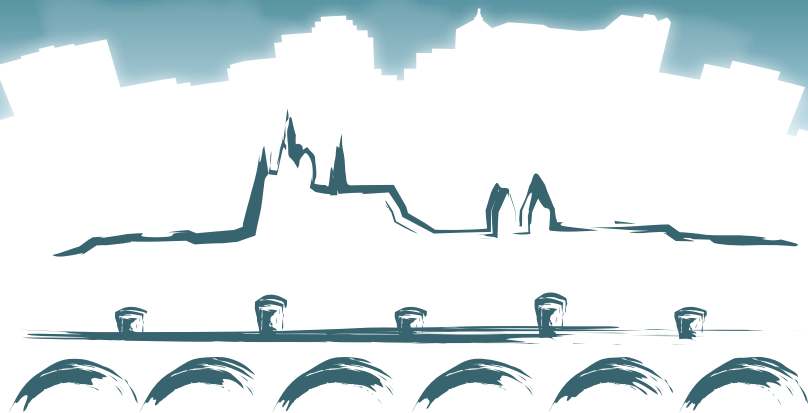


„SOMETHING NEW IS ON THE HORIZON“



# NEW HORIZONS IN FABRY DISEASE

INTERNATIONAL CONFERENCE ON ADVANCES  
IN THE TREATMENT OF FABRY DISEASE

PRAGUE, CZECH REPUBLIC  
MAY 16–17, 2025

HELD UNDER THE AUSPICES OF  
THE CZECH MEDICAL ACADEMY



ENDORSED BY

International  
Cardiomyopathy  
Network | ICON



[WWW.HORIZONS-FABRY.COM](http://WWW.HORIZONS-FABRY.COM)

Organized by GALÉN-SYMPOSIUM  
under the auspices of the Czech Medical Academy



### Endorsed by

The International Cardiomyopathy Network (ICoN)



### Scientific and Organizing Committee

Professor Aleš Linhart, MD DSc., FESC  
(General University Hospital and First Faculty of Medicine,  
Charles University Prague, Czech Republic)

Professor Dominique P. Germain, MD PhD  
(French Referral Centre for Fabry Disease, University of Versailles, France)

Professor Perry M. Elliott, FESC  
(Institute of Cardiovascular Science, University College London,  
London, United Kingdom)

### General information

#### Conference Venue

hotel Diplomat Prague Vienna House by Wyndham  
Evropská 15, Prague, Czech Republic

#### Conference Dates

Friday May 16<sup>th</sup> & Saturday May 17<sup>th</sup>, 2025

#### Organizing agency

GALÉN-SYMPOSIUM s.r.o.  
Břežanská 10, 100 00 - Prague 10  
Czech Republic  
phone: +420 222 518 535  
www.gsymposium.com

## SCIENTIFIC PROGRAMME

### Friday May 16<sup>th</sup>, 2025

9:00 – 9:05	<b>Welcome address</b>
9:05 – 10:00	<b>SESSION 1</b> <b>Fabry disease in the context of cardiomyopathies</b> Perry M. Elliott: <i>Differential diagnosis of a hypertrophied heart</i> Pablo Garcia-Pavia: <i>Cardiac amyloidosis – differential diagnosis and treatments</i> Iacopo Olivotto: <i>Hypertrophic cardiomyopathy management in the era of myosin inhibitors</i>
10:20 – 11:40	<b>SESSION 2</b> <b>Fabry cardiomyopathy – advances in diagnosis and management</b> James Moon: <i>Contributions of cardiac MRI to the understanding of Fabry disease</i> Elena Biagini: <i>Novel insights from imaging studies</i> Josef Marek: <i>Obstructive cardiomyopathy in Fabry disease</i> Mehdi Namdar: <i>ECG in Fabry cardiomyopathy – a forgotten method?</i>
12:00 – 13:00	<b>INDUSTRY SYMPOSIA</b> <b>Industry Symposium 1</b> <b>Industry Symposium 2</b>
13:00 – 14:00	LUNCH
14:00 – 15:20	<b>SESSION 3</b> <b>Fabry disease and the kidney</b> Camilla Tøndel: <i>The role of renal biopsy in the understanding of pathophysiology and treatment effects</i> Christoph Wanner: <i>Can we improve outcomes by SGLT2 inhibitors RAAS blockers?</i>

David Warnock:  
*Monitoring kidney disease – is serum creatinine and eGFR just enough?*

Sima Canaan-Kühl:  
*End-stage kidney disease – what next? How do patients do on Haemodialysis and after kidney transplantation. Does the immune response to ERT differ?*

Alberto Ortiz:  
*The role of podocytes and treatment effects*

**14:40 – 17:00** **SESSION 4**

**Advancements in understanding mechanisms of the disease – beyond the storage**

Fabian Braun:  
*The role of alpha-synuclein in the pathogenesis of Fabry Nephropathy*

Guido Iaccarino:  
*The role of mitochondrial dysfunction and muscular abnormalities in Fabry disease*

Moran Dvela Levitt:  
*Endoplasmic reticulum and its role in cellular function*

Martina Živná:  
*Agalopathy – the role of ER stress in pathophysiology of Fabry disease*

**17:00 – 18:00** **SESSION 5**

**Setting-up a multidisciplinary team of a Fabry center**

Federico Pieruzzi:  
*Paediatric Fabry disease – the transitional care model issue*

Bojan Vujkovic:  
*Organization of a multidisciplinary Fabry center team*

Gabriela Dostálová:  
*“Once upon a time in Prague... there was a...?”*

**18:00 – 18:05** **DAY 1 ADJOURN**

Aleš Linhart

**Saturday May 17<sup>th</sup>, 2025**

**8:40 – 10:00** **SESSION 6**

**Current and novel therapies – where do we stand?**

Antonio Pisani:  
*Switch from agalsidase beta to migalastat – registry data*

Daniel Bichet:  
*Migalastat – analysis of registry data, amenability testing, clinical and biomarker monitoring*

Dominique Germain:  
*Substrate reduction therapies – where do we stand?*

Raphael Schiffmann:  
*Update on gene therapies in Fabry disease*

Aleš Linhart:  
*Enzyme-replacement therapies – long term data*

**10:20 – 11:40** **SESSION 7**

**Monitoring of the disease and treatments impacts**

Uma Ramaswami:  
*Monitoring PROs in Fabry disease*

Malte Lenders:  
*Measurements of migalastat levels and antibody responses*

Maud Janssens:  
*Antibody response to enzyme replacement therapies*

Albina Nowak:  
*Lyso Gb3 and novel biomarkers*

**12:00 – 13:00** **INDUSTRY SYMPOSIA**

**Industry Symposium 3**

**Industry Symposium 4**

**13:00 – 14:00** **LUNCH**

**14:00 – 14:50** **SPLIT-OUT ABSTRACT SESSIONS**

15:00 – 16:20

## **SESSION 8**

### **Advancements in understanding mechanisms of the disease and treatment effects**

**João Paulo Oliveira:**

*Peroxidasin (PXDN) expression in kidney tissue of patients carrying GLA gene variants, with or without Fabry*

**Maurizio Pieroni:**

*The role of inflammation in pathogenesis of cardiac involvement*

**Alessandro Burlina:**

*Cerebrovascular events in Fabry patients - clinical course and outcomes*

**Juan Manuel Politei:**

*Gastrointestinal complications in Fabry patients*

16:40 – 18:00

## **SESSION 9**

### **Genetics, late onset variants, disease management and outcomes**

**Gheona Altarescu:**

*Novel insights in Fabry genetics*

**Stanislav Kmoch:**

*How to read genetic test results conflicting with clinical evidence*

**Olga Azevedo:**

*Fabry disease due to p.F113L mutation*

**Ana Jovanovic:**

*Fabry disease due to p.N215S mutation*

18:00 – 18:10

## **YOUNG INVESTIGATORS AWARDS**

18:10 – 18:15

## **DAY 2 ADJOURN**

Dominique P. Germain

## **Registration**

online at [www.horizons-fabry.com/registration](http://www.horizons-fabry.com/registration)

### Registration fees

REGULAR registration

POSTER 1<sup>st</sup> Author under the age of 35

Standard  
(until 12. 5. 2025)

500 €

150 €

Late / On-site

600 €

180 €

### The Registration fee includes:

- access to all sessions,
- access to the industry exhibition,
- programme booklet,
- all conference meals (coffee breaks and lunches).

## **Accommodation**

Vienna House by Wyndham Diplomat Prague

Evropská 15 | Prague | Czech Republic

A special accommodation price has been arranged for the Conference participants directly at the Conference Venue.

To book the accommodation at this price please use the exclusive FabryNH - reservation link available at [www.horizons-fabry.com/accommodation](http://www.horizons-fabry.com/accommodation)

The link will remain open until March 31, 2025.

For any special inquiries regarding the accommodation contact the hotel directly.