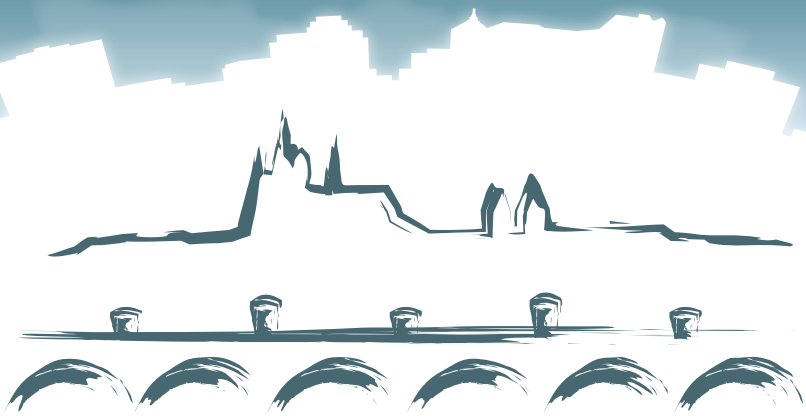
"SOMETHING NEW IS ON THE HORIZON"



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

ČESKÁ LÉKAŘSKÁ AKADEMIE

ENDORSED BY

International CardiOmyopathy Network | ICON

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

Endorsed by

The Czech Society of Cardiology
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 16th & Saturday May 17th,2025

Organizing agency

GALÉN-SYMPOSION s.r.o.
Březanská 10, 100 00 - Prague 10
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SCIENTIFIC PROGRAMME

Friday May 16th, 2025

9:00 – 9:05 **Welcome address**

9:05 – 10:00 **SESSION 1**

Fabry disease in the context of cardiomyopathies

Perry M. Elliott:
Differential diagnosis of a hypertrophied heart

Pablo Garcia-Pavia:
Cardiac amyloidosis – differential diagnosis and treatments

Iacopo Olivotto:
Hypertrophic cardiomyopathy management in the era of myosin inhibitors

10:20 – 11:40 **SESSION 2**

Fabry cardiomyopathy – advances in diagnosis and management

James Moon:
Contributions of cardiac MRI to the understanding of Fabry disease

Elena Biagini:
Novel insights from imaging studies

Josef Marek:
Obstructive cardiomyopathy in Fabry disease

Mehdi Namdar:
ECG in Fabry cardiomyopathy – a forgotten method?

12:00 – 13:00 **INDUSTRY SYMPOSIA**

12:00 – 12:30 **Symposium supported by SANOFI**

Practical recommendations for patients under ERT
presented by Andrea Matucci

12:30 – 13:00 **Symposium supported by TAKEDA**

Two Decades of Experience from the Fabry Outcome Survey

Fabian Braun:
Welcome and overview of objectives

Uma Ramaswami:
Background and structure of the FOS registry

Uma Ramaswami:
Effects of long-term agalsidase treatment on renal function

Uma Ramaswami:
Effects of long-term agalsidase treatment on cardiac structure

Uma Ramaswami:
Effects of long-term agalsidase treatment on morbidity and mortality

Fabian Braun:
Closing remarks and conclusion

13:00 – 14:00 LUNCH

14:00 – 15:20 **SESSION 3**

Fabry disease and the kidney

Camilla Tøndel:
The role of renal biopsy in the understanding of pathophysiology and treatment effects

Christoph Wanner:
Can we improve outcomes by SGLT2 inhibitors RAAS blockers?

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

15:40 – 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 Vujkovac:
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

19:00 – 22:00 Dinner with expert discussion & networking

Saturday May 17th, 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 – 12:30 **INDUSTRY SYMPOSIA**

12:00 – 12:30 **Symposium supported by CHIESI • Global Rare Diseases**

From silent damage to prompt intervention: enhancing monitoring in Fabry disease
chaired by Robert Hopkin

Robert Hopkin:
Welcome and introduction

Christine Kurschat:
Silent kidney progression in Fabry disease: the role of monitoring and the prompt intervention

Christine Kurschat:
Main Pegunigalsidase alfa renal data

Q&A

12:30 – 13:00 **Symposium supported by AMICUS Therapeutics**

Advancing Patient-Centered Care in Fabry Disease: Real-World Insights and the Impact of Migalastat Therapy

Robert Hopkin:
Patient experience with Fabry disease and Migalastat

Uma Ramaswami:
UK experience of managing patients with Fabry disease with Migalastat

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

POSTERS

1) Cardiovascular Involvement in Fabry Disease: Association Between Basilar Dolichoectasia and Cardiomyopathy
Esteban Calabrese, Guillermo Rodriguez Botta (ARG)

2) Establishment of a patient-specific endothelial cell model to study immune response and endoplasmic reticulum stress in Fabry disease
Elisa Rudolph, Malte Lenders, Eva Brand (DEU)

3) Identification of new interaction partners and pathways of α-galactosidase A to analyse the pathogenesis of Fabry disease
Elise Raphaela Menke, Malte Lenders, Eva Brand (DEU)

4) Exploring the journey of patients living with Fabry disease in Poland
Agnieszka Brzezinska, Anna Moskal, Chris Wingrove, Jayne Gershkowitz, Adrian Goretzki, Bernadeta Prandzioch-Goretzki, Aleksandra Gladys, Michal Nowicki (GBR)

5) Fabry disease in Argentina: Clinical, biochemical and molecular correlation in all reported GLA variants
Juan Politei, Romina Ceci, Domingo Procopio, LUCAS SILVESTROFF, Rita Valdez, Paula Rozenfeld (ARG)

6) Phase III, Open-label, Switch-over Trial of the Efficacy and Safety of Agalsidase Beta Biosidus (Agalzyme®) in Fabry Disease Patients Previously Stabilized with Fabrazyme®
Norberto Antongiovanni, Gustavo Cabrera, Alberto A. Fernández, Norberto Guelbert, Juan M. Muraro, Juan M. Politei, Carlos Cusumano, Fernando Gómez Pizarro, Guillermo Guelbert, Sergio Lucca, Erika Nieto, Fernando Perretta, Nicolás M. Antognoni, Sabrina Coppola, Lucía Giménez, Gabriel D. Robbesaul, Gabino Rolandelli, Hugo Sotelo, Viridiana Berstein (ARG)

7) Worse pregnancy and delivery outcomes in children with a pathogenic GLA variant - a post-hoc analysis of the PROFABIA study
Christopher Paschen, Natalja Haninger-Vacariu, Constantin Gatterer, Senta Graf, Markus Ponleitner, Paulus Rommer, Raute Sunder-Plassmann, Gere Sunder-Plassmann, Alice Schmidt (AUT)

8) Maternal and Postnatal Outcomes Study (MOS) in patients with Fabry disease receiving pegunigalsidase alfa – study design
Joana Lopes de Almeida , Eleonora Riccio, Benedetta Borella, Giovanni Piotti (ITA)

9) Exploring genetic factors of Fabry nephropathy progression via whole-exome sequencing
Tina Levstek, Bojan Vujkovac, Albina Nowak, João-Paulo Oliveira, Gabriela Dostálová, Aleš Linhart, Markéta Šafaříková, Gheona Altarescu, Katarina Trebušak Podkrajšek (SVN)

10) Electrocardiographic Features Distinguishing Fabry Disease from Sarcomeric Hypertrophic Cardiomyopathy
Roberto Giugliani, Fabiano de Oliveira Poswar (BRA)

11) Preliminary Experience of Switching Agalsidase Beta to Pegunigalsidase Alfa in Six Patients in Finland
Jukka Saarinen, Päivi Pietilä-Effati, Susanna Kouhi, Minna Kylmälä, Ilkka Kantola (FIN)

12) Quality of life of migalastat-treated adolescents with Fabry disease: results from the ASPIRE study and open-label extension
Robert J. Hopkin, Amarilis Sanchez-Valle, Esperanza E. Font-Montgomery, Ozlem Goker-Alpan, Damara Ortiz, Chester B. Whitley, William R. Wilcox, Hai Jiang, Lee Ann Lawson, Jennie Vosk, Haichen Yang, Uma Ramaswami (USA)

13) Clinical assessment of disease severity in patients with Fabry disease treated with pegunigalsidase alfa: an integrated analysis
Bojan Vujkovac, Derralynn Hughes, Aleš Linhart, Eric L. Wallace, William R. Wilcox, Chester B. Whitley, Irene Koulinska, Giovanni Piotti, Raul Chertkoff, Sari Alon, Anat Sakov, Antonio Pisani, John A. Bernat (SVN)

14) Long-term safety and efficacy of migalastat in adolescent patients with Fabry disease: results from the ASPIRE study and open-label extension
Uma Ramaswami, Esperanza Font-Montgomery, Ozlem Goker-Alpan, Damara Ortiz, Amarilis Sanchez-Valle, Chester B. Whitley, William R. Wilcox, Hai Jiang, Lee Ann Lawson, Jennie Vosk, Haichen Yang, Robert J. Hopkin (USA)

15) Extending the interval between pegunigalsidase alfa infusions in patients with Fabry disease: five-year interim results from the ongoing BRIGHT51 study
Antonio Pisani, John A. Bernat, Myrl Holida, Stephen Waldek, William R. Wilcox, Nicola Longo, Ozlem Goker-Alpan, Eric Wallace, Patrick Deegan, Camilla Tøndel, François Eyskens, Ulla Feldt-Rasmussen, Derralynn Hughes, Ankit Mehta, Khan Nedd, David G. Warnock, Giovanni Piotti, Meng Wang, Einat Almon-Brill, Sari Alon, Raul Chertkoff, Ales Linhart (ITA)

16) Treatment satisfaction in patients with Fabry disease: patient-reported outcomes from the followME Fabry Pathfinders registry and the SATIS-Fab study
Ulla Feldt-Rasmussen, Aleš Linhart, Biliana O. Veleva-Rotse, Joseph D. Giuliano, Hai Jiang, Vipul Jain, Caroline Martinez, Olivier Lidove, on behalf of the followME Fabry Pathfinders registry and the SATIS-Fab study investigators (CZE)

17) **Additional value of standard ECG to echo staging in prediction on cardiovascular risk in Anderson Fabry disease**
Serena Serratore, Maria Alessandra Schiavo, Raffaello Ditaranto, Vanda Parisi, Rosa Lillo, Annamaria Del Franco, Emanuele Monda, Pier Paolo Bocchino, Nevio Taglieri, Claudia Raineri, Chiara Leuzzi, Andrea Barison, Ines Monte, Cinzia Zuchi, Daniele Torella, Francesco Cappelli, Maurizio Pieroni, Francesca Graziani, Iacopo Olivotto, Giuseppe Limongelli, Elena Biagini (ITA)

18) **Evaluating the relationship between infusion-related reactions and antidrug antibody status: results from 109 patients with Fabry disease treated with pegunigalsidase alfa**
Robert J. Hopkin, Derralynn Hughes, John A. Bernat, Aleš Linhart, Nicola Longo, Camilla Tøndel, Bojan Vujkovac, Antonio Pisani, Jasmine Knoll, Irene Koulinska, Giovanni Piotti, Raul Chertkoff, Sari Alon, Anat Sakov, Eric L. Wallace (USA)

19) **Echocardiographic Analysis: Factors Affecting Mitral Regurgitation in Fabry Disease**
Alena Vecerova, Gabriela Dostalova, Beata Soltesova, Ales Linhart (CZE)

20) **Uncovering the Fabry Disease treatment experience: a survey of US patients receiving enzyme replacement therapy (ERT)**
Robert J. Hopkin, Angel Rosas, Irene Koulinska, Lucas Oliveira (USA)

21) **Real-World Patient Experiences of Enzyme Replacement Therapy (ERT) in Adults with Fabry Disease: A UK Market Research Study**
Derralynn Hughes, Gisela Wilcox, Alison Muir, Tom Kenny, Kamran Iqbal, John Ndikum (GBR)

22) **Novel insights on blood pressure variability impacts on cardiovascular manifestations of Fabry Disease**
Jan Pudil, Josef Marek, Gabriela Dostalova, Kristyna Bayerova, Beata Soltesova, Ales Linhart (CZE)

23) **The Price of Not Diagnosing Fabry Disease in Puerto Montt, Chile**
Benjamin Solar, Juan Politei, Felipe Mendez (CHL)

24) **Novel Variant in GLA and Classic Fabry Disease: Defining an Aggressive Phenotype in Southern Chile**
Benjamin Solar, Juan Politei (CHL)

25) **Trying to Reducing the Burden of Fabry Disease: The Most Southern Chilean Experience**
Benjamin Solar, Joaquin Cerda, Ivonne Zamorano, Nicole Rivera, Felipe Mendez (CHL)

NOTES

Registration

online at **www.horizons-fabry.com/registration**

Registration fees	On-site
REGULAR registration	600 €
POSTER 1st Author under the age of 35	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

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GOLD PARTNERS:

Amicus Therapeutics®

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EXHIBITOR:

Bristol Myers Squibb™

ORGANIZING AGENCY:

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