# **Fabry** masterclass

# **Scientific Coordinator**

# Prof. Olga Azevedo

Director of the Reference Centre on Lysosomal Storage Disorders (LSD) in Hospital Senhora da Oliveira Guimarães, Portugal

# **SCIENTIFIC RATIONALE**

This international masterclass provides a unique opportunity for healthcare professionals to immerse themselves in an intensive, high-level educational experience focused on Fabry disease. Hosted in a renowned Center of Excellence, the program is designed to promote best practices and foster in-depth exchange between participants and leading experts in the field.

Over three days, the course combines theoretical knowledge with practical exposure: from foundational insights on diagnosis and treatment strategies, including enzyme replacement therapy and ADA monitoring, to hands-on sessions involving patient consultations and case discussions. A strong emphasis is placed on multidisciplinary care and emerging therapies, empowering clinicians to refine their skills in managing the complex clinical presentations of Fabry disease.

Tailored for physicians and healthcare providers with a focus on metabolic disorders, this masterclass offers an unparalleled setting for clinical learning, peer networking, and collaborative dialogue to enhance patient care on a global level.

# **Registration Information**

Official language: English

**Continuing Medical Education:** UEMS/EACCME and Italian CME credits will be requested Educational grants are available for Nephrologist, Internal Medicine and Cardiologists from all over the World belonging to private or public institutions involved in the diagnostic process of rare diseases.

Please send an email to: info@fclassevents.com specifying your affiliation, role and CV.

Applications will be reviewed by the Scientific Board and you will be informed of the outcome of your application after few days.

#### Masterclass venue

Hospital Senhora da Oliveira – Guimarães, Portugal



SEGRETERIA ORGANIZZATIVA First Class srl - Meetings and Conferences Via Vittoria Colonna, 40 - 20149 Milano Phone: +39 02 30066329 Email: info@fclassevents.com Guimarães
1 - 3 October
2025

# Fabry masterclass

Scientific Coordinator **Prof. Olga Azevedo** 





for rare or low prevalence complex diseases

# Network Hereditary

Hereditary Metabolic Disorders (MetabERN)

#### Member

Hospital Senhora da Oliveira, Guimarães, EPE — Portugal





# **Preliminary Program**

### Wednesday, 1 October 2025

12:30 - 13:30	Arrival and Lunch
13:30 - 13:45	Welcome to the Reference Center of LSD of Guimarães Olga Azevedo
13:45 - 14:15	Agenda and Introduction Olga Azevedo
	FD in the Portuguese region of Guimarães
14:15 - 14:45	From the discovery of a founder effect to the characterization of the late-onset phenotype due to the F113L variant Olga Azevedo
	Multidisciplinary management of FD – Part I
14:45 - 15:05	Diagnosis and staging of Fabry cardiomyopathy Filipa Cordeiro
15:05 - 15:30	Management of dysrhythmias and recommendations for ICD implantation Sílvia Ribeiro
15:30 - 15:50	Management of heart failure and other cardiac manifestations in FD Tamara Pereira
15:50 - 16:10	Multidisciplinary panel discussion Olga Azevedo, Filipa Cordeiro, Sílvia Ribeiro, Tamara Pereira
16:10 - 16:30	Coffee-break
	Multidisciplinary management of FD – Part II
16:30 - 17:00	Stroke, brain white matter lesions and Parkinsonism: From pathophysiology to management Miguel Gago
17:00 - 17:20	Acroparesthesias and neuropathic pain: Differential diagnosis and treatment Joana Magalhães
17:20 - 17:50	Fabry nephropathy: Diagnostic and therapeutic approach in the clinical practice Carlos Soares
17:50 - 18:10	Genetic counselling and reproductive options for Fabry patients  Maria Lopes Almeida
18:10 - 18:30	Multidisciplinary panel discussion Olga Azevedo, Miguel Gago, Joana Magalhães, Carlos Soares, Maria Lopes Almeida, Maria Antónia Costa, Catarina Lombo, Olga Pereira
18:30	Closing of Day 1 Olga Azevedo
19:30	Dinner

# Thursday, 2 October 2025

#### FD pathophysiology

09:00 - 09:30 Evolving concepts on the pathophysiology mechanisms of FD Olga Azevedo

#### **FD Screening**

09:30 - 09:50 Screening of high-risk populations
Márcia Torres

09:50 - 10:20 Newborn screening: Challenges and risks Laura Vilarinho

10:20 - 10:40 How to clarify the clinical significance of a VUS?

Maria Lopes Almeida

10:40 - 11:00 Discussion

Olga Azevedo, Márcia Torres, Laura Vilarinho, Maria Lopes Almeida

11:00 - 11:30 Coffee-break

#### Genetic conundrums in FD

11:30 - 12:00 What is the clinical significance of D313Y, R118C and A143T variants?

Olga Azevedo

#### **FD Biomarkers**

12:00 - 12:20 Lyso-GB3: Usefulness and limitations Paulo Gaspar

12:20 - 12:40 Novel biomarkers on the horizon Patrício Aguiar

12:40 - 13:00 Discussion

Olga Azevedo, Paulo Gaspar, Patrício Aguiar

13:00 - 14:00 Lunch

#### Treatment challenges in FD

- 14:00 14:30 Current therapeutic options: Efficacy and limitations Olga Azevedo
- 14:30 14:50 Anti-drug antibodies: Clinical impact, Monitoring and therapeutic implications
  João Gonçalves
- 14:50 15:20 When to start treatment in females and late-onset patients? Filipa Cordeiro
- 15:20 15:50 Discussion

Olga Azevedo, João Gonçalves, Filipa Cordeiro

15:50 - 16:20 Coffee-break

#### Challenging clinical cases - Session I

16:20 - 16:50 Clinical case 1 Sofia Correia

16:50 - 17:20 Clinical case 2 Tamara Pereira

> 17:30 Closing of Day 2 Olga Azevedo



# Friday, 3 October 2025

#### Cardiac imaging in FD

09:00 - 09:30 Present and future role of cardiac MRI in FD Catarina Ferreira

#### Challenging clinical cases - Session II

09:30 - 10:00 Clinical case 3 Miguel Gago

10:00 - 10:30 Clinical case 4 Luisa Pinheiro

10:30 - 11:00 Coffee-break

#### **Emerging therapies for FD**

11:00 - 11:30 Emerging therapies for FD Olga Azevedo

11:30 - 11:45 Satisfaction questionnaire

11:45 - 12:00 Closing remarks Olga Azevedo

12:00 Lunch and Departures

