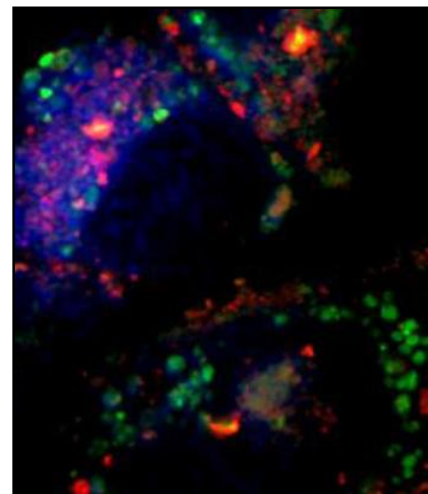


## Organizers:



## Sponsors:



**November 19, 2018**

**Main Conference Room  
Pavelló Docent**

**Campus Vall d'Hebron, Barcelona**

**Vall d'Hebron Barcelona Hospital Campus**

Passeig Vall d'Hebron, 119-129 – 08035 Barcelona

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# VALL D'HEBRON SEMINAR **LYSOSOMAL DISORDERS: FOCUS ON FABRY DISEASE**

## Overview

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Genetic deficiencies of lysosomal components, most commonly enzymes, are known as "lysosomal storage disorders" (LSDs) and lead to lysosomal dysfunction, broadly affecting peripheral organs and the central nervous system, debilitating patients and frequently causing fatality. This seminar will update clinicians and researchers on most recent advances in diagnosis, pathophysiology and management of Fabry disease as well as in emerging therapies in the treatment of LSD.

## Registration and accessibility

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Participation on the event is free.

Online registration ([www.vhir.org/activities](http://www.vhir.org/activities)) is required.

Simultaneous translation into Spanish or Catalan will be available upon request.

### 8:30 Opening session:

- Vicenç Martínez-Ibáñez, CEO Hospital Universitari Vall d'Hebron, Barcelona
- Joan Comella, Director Vall d'Hebron Institut de Recerca (VHIR)
- Roser Francisco, Secretary of the Advisory Commission on Rare Diseases, CatSalut
- Ramon Martínez Máñez, Director of CIBER-BBN
- Mireia del Toro, Coordinator of CSUR and ERN MetabERN, Hospital Universitari Vall d'Hebron

### 9:00 Clinical management of Fabry disease. Chair: M. del Toro & G. Pintos-Morell

**Miguel A. Torralba** (*Hospital Clínico, Zaragoza*): Pathophysiology of Fabry disease: role of biomarkers and genetic variants

**Alberto Ortiz** (*Fundación Jiménez Díaz, Madrid*): Overview of consensus treatment and precision medicine approach in Fabry disease

**Olga Azevedo** (*Hospital Senhora da Oliveira, Guimarães, Portugal*): Insights into cardiovascular involvement in Fabry disease

**Michael West** (*Dalhousie University, Halifax, Canada*): What have we learned after 10 years of Canadian Fabry Disease Initiative (CFDI)?

### 11:00 Coffee Break

### 11:30 New therapeutic strategies for lysosomal disorders. Chair: N. Ventosa & S. Schwartz Jr.

**Ibane Abasolo** (*CIBBIM-Nanomedicine*): Nanomedicine in lysosomal disorders. Project Smart4Fabry

**Roberto Giugliani** (*Porto Alegre, Brazil*): Fusion proteins and other strategies to cross the Blood-Brain-Barrier (BBB)

**Michael West**. (*Dalhousie University, Halifax, Canada*). Gene therapy clinical trial for Fabry disease

**Fàtima Bosch**. (*CBATEG-UAB*): Gene therapy approaches for Sanfilippo A and other lysosomal disorders

### 13:30 General Discussion

### 14:00 Lunch