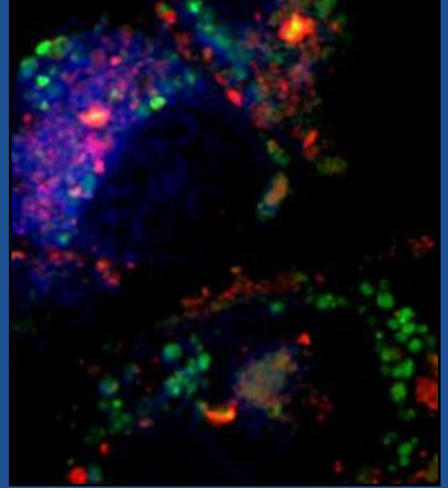
# Vall d'Hebron Seminar: LYSOSOMAL DISORDERS, A FOCUS IN FABRY DISEASE

**November 19, 2018**

**Main Conference Room**
**Pabelló Docent**
**Campus Vall d'Hebron, Barcelona**

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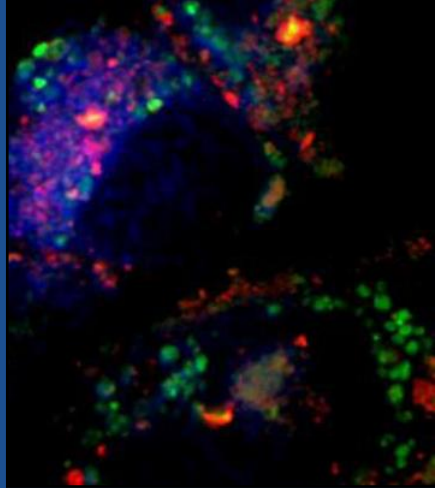

## Scientific Program

**8:30 Opening session: Vall d'Hebron Hospital CEO & Director of Research Institute VHIR**

**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 precission medicine approach in Fabry disease

**José Rodríguez Palomares** (*Hospital Universitari Vall d'Hebron, Barcelona*): 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**

## Overview

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

Participation on the event is free. Online registration is required due to limited seat availability

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

## Organizers

## Sponsors: