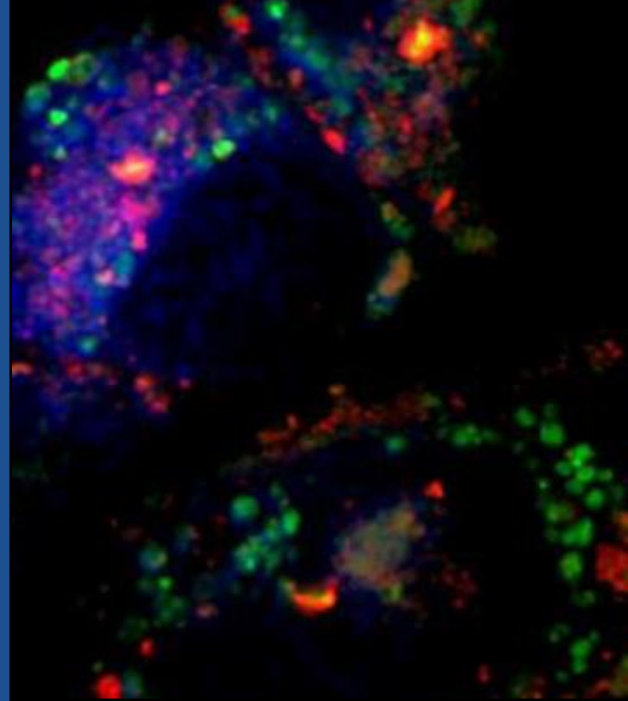


Vall d'Hebron Seminar

Lysosomal disorders: focus on Fabry disease

November 19, 2018

Main Conference Room
Pavelló Docent
Vall d'Hebron Barcelona Hospital Campus



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 precision medicine approach in Fabry disease

James C. Moon (*Barts Heart Centre, London, United Kingdom*): Insights into early and advanced cardiomyopathy in Fabry disease from MRI

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

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

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

13:30 General Discussion

Open forum for patients and health care providers

15:00 Present situation of the treatment for lysosomal diseases. Chair: J. Torrent-Farnell Personal experiences. Patients, families and physicians get together

18:00 Summary, take-home messages, and farewell. G. Pintos