

21ST

INTERNATIONAL SYMPOSIUM
OF THE PORTUGUESE SOCIETY
FOR METABOLIC DISORDERS

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SATELLITE MEETING

RAISING AWARENESS IN INNOVATIVE THERAPIES IN IMD

SATURDAY, 29TH MARCH

- 09:30 Secretariat opening
10:00 Satellite Meeting Opening
Welcome Address on behalf of the SPDM
10:10 RNAi - revolutionary new treatment approach for acute hepatic porphyrias: Givosiran (Alynlyam)
Daniel Costa Gomes, Lisbon, PT
10:40 Cerliponase alfa: state of the art in CLN2 (BioMarin)
Sofia Quintas, Lisbon, PT
11:10 **Coffee Break**
11:40 Challenges in the diagnosis of LAL-D (Alexion)
Patrício Aguiar, Lisbon, PT
12:10 Avalglucosidase alfa: what is the impact on the Pompe disease patients' quality of life? (Sanofi)
Miguel Santos, Lisbon, PT
Anabela Bandeira, Porto, PT
12:40 **Discussion and final remarks**



**SANA
METROPOLITAN
HOTEL, LISBON**



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PATROCÍNIOS

ALEXION
AstraZeneca Rare Disease

Alynlyam

BiOMARIN

sanofi