Rare Disease Workshop

Preclinics + Clinics + Patients

PROGRAMME

14:00	**14:15**	**Welcome and introduction** Dr Laura Padilla - *Health & Biomedicine division. Leitat*
14:15	**14:45**	**Developing new therapies for Batten Disease** Dr Sara Mole - *Laboratory for Molecular Cell Biology Group Leader, MRC. UCL Professor of Molecular Cell Biology (London)*
14:45	**15:15**	**Targeted nanoliposomes for the treatment of Fabry disease** Dr Ibane Abasolo - *CIBBIM-Nanomedicine, Drug Delivery and Targeting Vall d'Hebron Research Institute (Barcelona)*
15:15	**15:45**	**Development of MIN-102 for the treatment of X-linked Adrenoleukodystrophy and Friedreich's Ataxia** Dr Marc Martinell - *CEO, Minoryx Therapeutics*
15:45	**16:00**	Coffee break
16:00	**16:30**	**The role of patients in the process of drug designation and approval. Videoconference** Mr. Julián Isla - *Member of Committee for Orphan Medicinal Products (COMP). Chairman and Co-Founder Foundation twenty-nine. Chief Scientific Officer Dravet Syndrome European Federation*
16:45	**17:15**	Dr Mireia del Toro - *Neuropediatrics and Congenital Metabolic Diseases Unit Coordinator, Vall d'Hebron Barcelona Hospital Campus*
17:15	**17:45**	**Study of Rett syndrome from the perspective of synaptic metabolism: search for new therapeutic options** Dr. Alfonso de Oyarzábal Sanz. - *Synaptic Metabolism Lab. Hospital Sant Joan de Déu*
17:15	**17:30h**	Closing remarks

This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No. 666918