

Challenges and barriers for gene therapies in rare neurological disorders: preparing the next generation of clinicians and researchers

Hospital Universitari Vall d'Hebron, June 29th-30th Barcelona.

Day 1

Welcome (12:50-13:00). Organizing committee (J.Barquinero, F. Munell and D. Gómez-Andrés) and representatives from Campus Vall d'Hebron.

Vectors for gene therapy Moderation: J. Barquinero, VHIR (Barcelona)

Topic	Speaker	Time
Capsid and promoter variation to enhance expression	M. Cabanes-Creus; Children Medical Research Institute (Sidney)	13:00
Immune response to vectors	G. Logan; Children Medical Research Institute (Sidney)	13:30
Non-AAV vectors	R. Hernandez; CIMA, Univ. de Navarra (Pamplona)	14:00
Non viral vector design	R. Schiffers; UMC Utrecht (Utrecht)	14:30
Targeting neural cell types with AAV	A. Bosch; Universitat Autònoma de Barcelona (Barcelona)	15:00
Durability of gene therapy	X. Angela; Estuary Biotherapeutics (Philadelphia)	15:30
Joint discussion		16 :00

Coffee break

Beyond gene replacement : complementary technologies Moderation: D. Gómez-Andrés, Hospital Universitari Vall d'Hebron (Barcelona)

Topic	Speaker	Time
Gene complementation	G. Gonzalez-Asequinolaza ; CIMA, Univ. de Navarra (Pamplona)	16:40
Gene editing	M.Güell ; U. Pompeu Fabra (Barcelona)	17:10
The delivery hurdle in the field of Nucleic Acids Therapeutics	V. Arechavala-Gomez; Biocruces Bizkaia (Bilbao)	17:40
tRNA-based therapies	Z. Ignatova; Hamburg Universität (Hamburg)	18:10
Gene therapy for brain circuit disorders	A. Almacellas-Barbanoj; University College of London (London)	18:40
Other technologies	M. Pallarés; Vall d'Hebron Institut de Recerca (Barcelona)	19:10
Joint discussion		19:40

Dinner (venue to be confirmed)



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Day 2.
Barriers and challenges for transferring efficient gene therapy into the clinical field

Moderation: A. Macaya Hospital Universitari Vall d'Hebron (Barcelona)

Topic	Speaker	Time
Lessons from preclinical models	A. Buj-Bello; Inserm, UMPC (Évry)	08:30
Safety of the integration	E. Montini ; San Raffaele Telethon Institute for Gene Therapy (Milan)	09:00
Hiding the virus	S.Borros; Institut Químic de Sarrià (Barcelona)	09:30
Joint discussion		10:00

Coffee break
Gene therapies from different points of view

Moderation: M. del Toro Hospital Universitari Vall d'Hebron (Barcelona)

Topic	Speaker	Time
The perspective of the regulator: quality	D. Rogers; European Medicine Agency (Amsterdam)	11:00
The perspective of the regulator: demonstration of efficacy	E. H. Martinez-Lapiscina; European Medicine Agency (Amsterdam)	11:20
The perspective of the payer	J. Fontanet; CatSalut (Barcelona)	11:40
The perspective of the industry : manufacturing and logistics	C. Castro González de Vega; Roche (Basel)	12:00
The perspective of the patient	M. Montoliou; Spanish Duchenne Parent Project (Barcelona)	12:20
Joint discussion		12:40

Lunch (venue to be confirmed)
Gene therapies in action

Moderation: L. Costa-Comellas. Hospital Universitari Vall d'Hebron (Barcelona)

Topic	Speaker	Time
The neurologist's point of view	Francina Munell; H.U. Vall d'Hebron (Barcelona)	15:00
The clinical developer's point of view	S. Galo; Pfizer (Germany)	15:30
Hemolytic uremic syndrome	G. Ariceta; H.U. Vall d'Hebron (Barcelona)	16:00
Liver problems	J. Quintero; H.U. Vall d'Hebron (Barcelona)	16:30
Joint Discussion		17:00

Case studies from participants and closing remarks (17.30-18.30)


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