

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

Hospital Universitari Vall d'Hebron, June 29<sup>th</sup>-30<sup>th</sup> 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-Aseguinolaza ; 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)



« This project has received funding from the European Union's Horizon 2020 research and innovation programme under the EJP RD COFUND-EJP N° 825575"»

**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, Genethon (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. Montolio; 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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