Bambino Gesù
OSPEDALE PEDIATRICO

European Reference Networks

un evento organizzato da | BioMedia
La condivisione del sapere

WORKSHOP

OUTCOME MEASURES FOR GENE THERAPY

Rome, December 3rd, 2025

Ospedale Pediatrico Bambin Gesù
Sede di Roma Gianicolo

Workshop on gene therapy outcomes in IRDs
Rome, December 3rd, 2025

Presidents of the Meeting S. Rizzo H. Dolfuss

09.00-9.15 **Introductory Remarks on Rare Eye Diseases**
M. Raponi – L. Buzzonetti
New therapeutic approaches in IRDs and role of ERN-EYE
S. Rizzo – H. Dolfuss

09.15-12.30 **Morning session** *(Chair – G. Iarossi, H. Dolfuss)*
1) Gene therapy in some specific IRDs: where are we now?
2) Analysis of IRD clinical trial experience; what was missing?

9.15-9.30 **Diagnostic approaches and endpoints for IRD Clinical Trials**
A.P. Salvetti

9.30-9.45 **RPGR**
A.P. Salvetti

9.45-10.15 **X-linked Retinoschisis**
P. Sieving

10.15-10.45 **RPE65/ USH1B**
F. Testa

10.45-11.15 **CEP290/USH2A**
B. Leroy

11.15-11.35 **RP11**
J Prener Holtan

11.35-1.00 **Gene-agnostic approaches**
I. Audo

Discussion

Lunch break

13.30-16.00 **Afternoon session** *(Chair – B. Falsini, G. Iarossi)*

3) Clinical vision testing (electrophysiology, psychophysics, Multimodal imaging); how to select the most appropriate parameters to evaluate therapy efficacy

13.30-13.50 **Electrophysiology**
P. Sieving, B. Falsini, G. Iarossi

13.50-14.10 **Psychophysics**
A. Amato, R. Mastromartino

14.10-14.30 **Multimodal imaging**
A.P. Salvetti, A. Coppè, G. Gambini

14.45-15.15 **ERN-EYE act4red initiative**
H. Dolfuss – B. Leroy

15.15-15.45 **Patient and regulatory agency perspectives**
E. Borgonovi, S. Bologna

Discussion

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Faculty

M. Raponi – *Rome*

L. Buzzonetti – *Rome*

S. Rizzo – *Rome*

H. Dolfuss – *Strasbourg*

F. Testa – *Neaples*

P. Sieving – *Sacramento*

A.P. Salvetti – *Milan*

J Prener Holtan– *Oslo*

B. Leroy – *Ghent*

B. Falsini – *Rome*

G. Iarossi – *Rome*

A. Amato – *Rome*

R. Mastromartino – *Rome*

A. Coppè – *Rome*

G. Gambini – *Rome*

E. Borgonovi – *Milan*

S. Bologna – *Rome*

I. Audo – *Paris*

Sede evento

Aula Salviati
Ospedale Pediatrico Bambino Gesù
Piazza di Sant'Onofrio, 4 – 00165 Roma RM

Iscrizione

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