

RE(ACT)[®]

INTERNATIONAL CONGRESS ON RESEARCH
OF RARE AND ORPHAN DISEASES

Full Program

MONDAY, MARCH 5th

E-RARE Monitoring meeting, by invitation only

TUESDAY, MARCH 6th

2018  E-RARE, Membership meeting (by invitation only), all day 9 to 17

RE(ACT) CONGRESS 2018:

WEDNESDAY, MARCH 7th

Special morning sessions 9 to 12 open to all:

Parallel session 1: Undiagnosed Diseases Network International (UDNI) – Chair Domenica Taruscio

Panellists:

- Helene Cederroth, SE
- [William Gahl, USA](#)
- [Steve Groft, USA](#)
- Domenica Taruscio, IT

Parallel session 2: European Reference Networks (ERNs), where we stand? – Chair Luca Sangiorgi

Pannelist:

- Enrique Terol, BE; DG Sante
- Maurizio Scarpa, DE; European Reference Networks
- [Holm Grassner, DE](#); EURO-NMD European Reference Networks
- Luca Sangiorgi, IT; BOND European Reference Networks
- Daria Julkowska, FR; E-RARE and Europe Joint Program

A. Session, Afternoon 14 to 17 “Opportunities in rare diseases around the world”

- Maurizio Scarpa, DE: European References Networks (ERNs)
- Hugh Dawkins, AU: IRDiRC and RD Connect
- Daria Julkowska, FR: E-RARE and European Joint Programme
- [Olivier Menzel, CH](#): RE(ACT) Community
- BBMRI, UDNI, Solve RD

POSTER SESSION A 17 to 18

Public Opening Ceremony 18 to 20

Welcome message

Professor [Silvio Garattini, IT](#), pioneer in rare disease research, professor in chemotherapy and pharmacology and director of the Mario Negri Institute for Pharmacological Research.

Professor [Harvey F. Lodish, USA](#), professor at the Massachusetts Institute of Technology (MIT), Founding Member of the Whitehead Institute for Biomedical Research and lead author of the textbook Molecular Cell Biology. “*Academic Entrepreneurs, New Technologies, and Building Companies to Treat Rare Diseases*”

THURSDAY, MARCH 8th

B. Session, Morning 9 to 12  “NGS and undiagnosed rare diseases”

- [Xavier Estivill, ES](#)
- [Marco Gattorno, IT](#) “*Impact on NGS in daily practice in autoinflammatory diseases*”
- [Nicolas Lévy, FR](#)
- Vincenzo Nigro, IT
- [Lisenka Vissers, NL](#) “*The circle of NGS innovation: from research to diagnostics and back*”

Lunch 12 to 13

POSTER SESSION B & C 13 to 14

C. Session, Afternoon, 14 to 17 “Pathophysiology”

- [Gregor Andelfinger, CA](#)
- [Kristina Hettne, NL](#)
- [Roman Chrast, SE](#) “*The role of endoplasmic reticulum-mitochondria crosstalk in axonal maintenance*”

POSTER SESSION B & C 17 to 19

FRIDAY, MARCH 9th**D. Session, Morning 9 to 12³⁰ “Gene and cell therapy”**

- [Peter Lenting, FR](#) “*Small Antibody Fragments as Alternative Tools in Haemophilia Care*”
- [Luigi Naldini, IT](#)
- [Josef M. Penninger, AT](#) “*Haploid stem cells – from discovery to a library of repairable mutations for reproducible research*”
- [Tatiana Petrova, CH](#) “*Dissecting the biology of lymphedema-distichiasis*”

Lunch 12 to 13

POSTER SESSION D,E & F 13 to 14

E. Session, Afternoon 13 to 17 “Neurological diseases”

- [Donald W. Cleveland, USA](#) “*Gene silencing therapy for human neurodegenerative disease*”
- [Antonio Federico, IT](#)
- [Jesus Requena, ES](#) “*In search of a therapy for Creutzfeldt-Jakob disease: identification of chemical chaperones that stabilize the prion protein PrP*”
- [Edward Wild, UK](#)

POSTER SESSION D,E & F 17 to 19

Social event: Delegates' dinner/party

SATURDAY, MARCH 10th¹¹**F. Session, 9 to 14, public session “Patients and research” and Rare Disease Day Emilia Romagna**

Program updated November 14, 2017