

IRDIRC MAY 2016 UPDATE

IRDIRC at the ECRD



The Eight [European Conference on Rare Diseases and Orphan Products](#) (ECRD 2016) was held in Edinburgh, United Kingdom, from May 26 to May 28. ECRD 2016 brought together over 80

speakers and more than 700 participants, covering six themes:

from the latest research and developments in new treatments, to innovations in healthcare, social care and support at the European, national and regional levels. This edition was linked by the common theme "Game Changers," and was aimed at changing the the future of rare diseases together. Numerous sessions aimed to contribute to these changes: in the domain of research; diagnosis; drug development, authorization and access; care provision; social policy; and global society.

Several IRDiRC Scientific and Executive Committee members contributed actively to the meeting and participated through session presentations and panel discussions, highlighting the contributions IRDiRC has made over the last couple of years. In particular, the session dedicated to "boosting rare diseases in a global collaborative research environment" gave a comprehensive overview of IRDiRC's development so far, and showed glimpses of what is yet to come. The recommendations resulting from IRDiRC Task Forces: Patient-Centered Outcome Measures and Small Population Clinical Trials were also presented.

IRDIRC new documents

- Executive Committee – [report](#) of the 19th Executive Committee meeting – March 15, 2016, Lyon, France
- Joint meeting – [report](#) of the 2nd Joint Executive and Scientific Committee meeting – March 14, 2016, Lyon, France
- Diagnostic Scientific Committee – [report](#) of the 10th Diagnostic Scientific Committee meeting – March 14, 2016, Lyon, France
- Interdisciplinary Scientific Committee – [report](#) of the 13th Interdisciplinary Scientific Committee meeting – March 14, 2016, Lyon, France
- Therapies Scientific Committee – [report](#) of the 9th Therapies Scientific Committee meeting – March 14, 2016, Lyon, France

Upcoming IRDiRC teleconferences and meetings

- June 1, 2016 – Operating Committee – Teleconference call
- June 9, 2016 – Participant Unique Identifiers Task Force Leadership – Teleconference call
- June 28, 2016 – Data-Mining and Repurposing Task Force – Teleconference call
- July 6, 2016 – Operating Committee – Teleconference call
- September 22-23, 2016 – Executive Committee – Face-to-face meeting, Catania, Italy

Rare disease research published on the website

- [NGO](#) Committee for Rare Diseases – United Nations, New York
- EMA report on integrating [patients' views](#) in clinical studies of anticancer medicines
- [Cooperation](#) between European Medicines Agency and Health Technology Assessment bodies creates synergies
- [Investing](#) in health care research systems for rare disease therapies: why and how
- [Randomized controlled trials](#): are they the gold standard?

Research highlights from IRDiRC members

- New recommendations to improve [social](#) care for rare diseases
- Shire's campaign to support International [mucopolysaccharidosis \(MPS\) awareness day](#) takes off
- GSK, Fondazione Telethon and Ospedale San Raffaele announce publication of pivotal safety and efficacy of gene therapy for children with [ADA-SCID](#)
- BioMarin announces EMA grants accelerated assessment for [Cerliponase Alfa](#), experimental treatment for a form of batten disease
- [Perspective](#): Precision medicine may move us beyond the use of race in prescribing drugs
- NIH' Biomedical [Data Translator](#) program

IRDIRC-related calls

The French Foundation for rare diseases has launched a call entitled "[Preclinical Research in rare diseases: translational steps in large animals](#)." The objective of this call is to support pilot scientific projects at the interface between the *in vivo* proofs of principle, usually in rodents, and the development of a clinical application in human patients, aiming to improve human well being. Deadline for pre-proposals: June 28, 2016

AFM-Téléthon has launched its [annual medical and a scientific call for proposals](#). The international scientific call for proposals, open to both French and foreign groups, aims to support research which will increase understanding of the neuromuscular system and encourages the development of therapies for neuromuscular and rare genetic diseases. Application deadline: July 5, 2016

The NHS has launched a call on "[Biological response modifier drugs for rare autoimmune diseases](#)." The aim of the call is to investigate what the clinical effectiveness of biological response modifiers (biologics) used for a range of rare autoimmune diseases are, and if they are cost-effective. Application deadline: September 15, 2016

All calls can be found on the [IRDIRC-related calls](#) page.

Other News

Of course it is not new that the IRDiRC Scientific Secretariat has taken up tweeting about IRDiRC activities, but in May we reached the milestone of our first hundred followers. Tag or follow us with [@Irdirc](#).



If you are presenting in a meeting or a conference, and you would like to show some information about IRDiRC, standard slides are available on the IRDiRC private website; additional slides can be made available upon request. Please also email the Scientific Secretariat when and where you will be presenting, so we can keep track of "IRDIRC" presence at conferences.

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