

IRDIRC NOVEMBER 2015 UPDATE for Executive Committee, Scientific Committees and Working Groups



Productive meeting joint GA4GH-IRDIRC Task Force Machine Readable Consent

On November 9-10, 2015, the jointly organized [GA4GH-IRDIRC](#) Task Force on Machine Readable Consent (MRC) met in Paris for a workshop. A group of highly-dedicated specialists came together to develop computer-readable data access and use categories, which could eventually inform consent form language, through aligning a user's permissions against a common set of permitted data use types. The group discussed on consent, data access and discovery and the limitations of MRC.



The second day was focused on different 'user stories' of data providers and users, related to conditions on data access. The successful workshop ended with a session on pilot testing and the identification of next steps.

Events for your calendar

ECRD 2016



European Conference on Rare Diseases & Orphan Products

EURORDIS and DIA Europe, Middle East & Africa invite all stakeholders involved in rare disease diagnosis, treatment and care to join us for the 8th European Conference on Rare Diseases & Orphan Products (ECRD 2016). ECRD is the foremost meeting of the rare disease community and provides a unique platform across all rare diseases, across all European countries, and across all stakeholders. The ECRD brings together patients, caregivers and patients' representatives; academics, scientists and researchers; payers and regulators; healthcare professionals, industry, policy-makers and representatives of the EU Member States.

ECRD 2016, May 26-28, 2016, Edinburgh International Conference Centre (EICC)- Edinburgh, UK

More details and registration: <http://www.rare-diseases.eu/>

RE(ACT) Congress 2016

RE(ACT) announces its 3rd international congress on research of rare and orphan diseases. This congress, jointly organized by the Blackswan Foundation and E-Rare, and co-sponsored by IRDiRC, will explore issues and cutting-edge technologies that affect many adult and pediatric conditions. The Congress will bring together world leaders and young scientists from stem cell, cell biology, gene therapy,

human genetics and therapeutic applications to present state-of-the-art research, to discuss results and to exchange ideas.

RE(ACT) 2016, March 9-12, 2016, Crowne Plaza Barcelona – Fira Center, Barcelona, Spain

More details and registration: <http://www.react-congress.org/>

Upcoming IRDiRC workshops

- November 30, 2015 - Patient-Centered Outcome Measures Task Force - Workshop, Paris, France
- March 3, 2016 - Small-Population Clinical Trials Task Force - Workshop, London, UK

Upcoming IRDiRC teleconferences and meetings

- December 10, 2015 – Operating Committee – Teleconference call (every second Thursday the month, until March 2016)
- January 11, 2016 – Executive Committee – Teleconference call
- March 14, 2016, Morning – Individual Scientific Committee meetings – Lyon, Paris, France
- March 14, 2016, Afternoon – Joint Scientific and Executive Committees meeting – Lyon, Paris, France
- March 15, 2016 – Executive Committee meeting – Lyon, Paris, France

Rare disease research published on the website

- A Drug Approval Case Study by the [FDA](#) Center for Drug Evaluation and Research
- [EMA](#) Initiative for the Collection of High-Quality Data on Medicines through Patient Registries
- [ADAPT-SMART](#): a Platform for Coordinating Medicines Adaptive Pathways to Patients
- Creating a [Sustainable](#) Environment for Orphan Drug Development

Research highlights from IRDiRC members

- Centers for Mendelian Genomics uncovering the [genomic basis](#) of hundreds of rare conditions
- [Telethon 2015](#) is just around the corner
- [Lysogene](#) receives Orphan Drug and Rare Pediatric Disease Designations from the FDA
- [NCATS](#) and Harvard Collaborate to Advance Global Rare Diseases Data Repository
- [WuXi](#) Nextcode one of Boston Children's Hospital CLARITY Undiagnosed Challenge winners



Research News

Visit of the Japan Health Sciences Foundation to the IRDiRC Scientific Secretariat

On November 2, 2016, a delegation of the Japan Health Sciences Foundation ([JHSF](#)) visited the IRDiRC Scientific Secretariat in Paris, to learn about the activities of IRDiRC. JHSF is a public corporation established for the purpose of contributing

to Japan's health and welfare by supporting research and development of drugs, medical equipment, diagnostics, and other medical products. The JSHF delegation, which consisted of representatives from the pharmaceutical industry, the prefectural governments and scientific institutes, visits several public institutes and companies both in Europe and the US to learn more about medical technologies and drugs for the treatment of both intractable and rare diseases. A report of these visits will be published online at the JHSF website in March 2016.

IRDiRC-related calls

The NIH has launched a call to support clinical trial readiness for [rare neurological and neuromuscular](#) diseases. It is expected that this call will fill gaps in the design of upcoming clinical trials in rare neurological or neuromuscular diseases.

E-Rare-3 has launched a call for proposals for clinical research for [new therapeutic uses of already existing molecules in rare diseases](#). This call attempts to promote the clinical and pre-clinical proof of concept studies for the potential application of repurposed drugs.

The NIH has a funding call to support [multi-site clinical trials](#). The purpose of this call is to provide a vehicle for submitting grant applications for investigator-initiated multi-site randomized controlled clinical trials.

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



Other News

The annual IRDiRC [State-of-Play](#) of Research is received very positively and has been cited on various sources, among which are 13 tweets!

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