IRDiRC JANUARY 2016 UPDATE

IRDiRC's Draft Document on "Small Population Clinical Trials Initiatives in the field of Rare Diseases" is open for comments

The IRDiRC's Small Population Clinical Trials Task Force and the Scientific Secretariat have made a draft document available, which is currently open for comments and suggestion for the community at large. Clinical research and trials in rare diseases face evident obstacles: very or exceptionally low disease prevalence, small and heterogeneous patient populations, difficulty in recruiting such patients, disease severity, lack of or limited knowledge of disease natural history and high attrition rates during research and development processes. The traditional randomized controlled study designs are difficult to conduct in small populations because of the difficulty to create homogeneous groups and to assess changes adequately between variable groups. Controlled rigorous designs that allow within-patient comparisons and treat all subjects would assess therapies more accurately, if feasible. Therefore, the field needs to develop cost-effective, novel, rigorous controlled study designs and relevant analyses to assess treatment efficacy in heterogeneous small populations.

This area has been identified by IRDiRC's Scientific Committees as a focal point; consequently a Task Force with several nominated experts in the field has been set up. In this Task Force, several international initiatives are represented, as well as industrial actors and agencies for the evaluation of medicinal products. Together, the goal of this Task Force is to advance discussions on ways to optimize and improve commonly adopted approaches and to reach agreement between the different stakeholders on appropriate small population studies.

IRDiRC new documents

• Interdisciplinary Scientific Committee- report of the teleconference meeting held on January 6, 2016
• Small Population Clinical Trials background document

Upcoming IRDiRC teleconferences and meetings

• February 8, 2016 – Interdisciplinary Scientific Committee – Teleconference call
• February 11, 2016 – Operating Committee – Teleconference call (every second Thursday of the month, until March 2016)
• February 17, 2016 – Small Population Clinical Trials Task Force – Teleconference Call
• March 3, 2016 - Small Population Clinical Trials Task Force - Workshop, London, UK
• March 14, 2016, Morning – Individual Scientific Committee meetings – Lyon, France
• March 14, 2016, Afternoon – Joint Scientific and Executive Committees meeting – Lyon, France
• March 15, 2016 – Executive Committee meeting – Lyon, France

Rare disease research published on the website

• The EMA releases guidance on conducting post-authorisation efficacy studies
• The Exomiser: a tool that goes one step further for variant identification
• Wide disparity of clinical genetics services and EU rare disease research funding across Europe

• FDA guidance document on communication between pharmas and FDA during drug development
• Health Technology Assessment in rare diseases: a dynamic process

Research highlights from IRDiRC members

• Future of ENCODE: Looking deeper into genome function
• NIH genome sequencing program targets the genomic bases of common, rare disease
• NORD establishes Rare Disease Patient/Caregiver Speakers Bureau
• Nominations for the 2016 Rare Impact Awards are Open!

Research News

Recommendations of IRDiRC's Therapies Scientific Committee

The members of the International Rare Disease Research Consortium's (IRDiRC) Therapies Scientific Committee (TSC) have discussed and agreed on a specific set of recommendations to guide policies and funding strategies so as to reach its goal of 200 new therapies by 2020, based on IRDiRC Polices & Guidelines which were adopted in April 2013. They focus on the improvement of guidelines for the clinical development of orphan drugs; the alignment of scientific and regulatory guidance and the enhancement of the continuous data collection and assessment all along the life cycle of therapy.

It is expected that the implementation of IRDiRC's policy and funding recommendations as a whole will significantly increase their overall impact. These recommendations address a specific number of bottlenecks associated with biomedical research which will further foster the collaboration of rare disease therapy development. While respecting the individuality of each regulatory and health technology assessment agency, the recommendations encourages collaboration among the different agencies on a number of fields.

The recommendations of the TSC are structured around three axes: the first revolves around recommendations for IRDiRC funding oçrganizations, which are subdivided in strategic recommendations, criteria for research funding, priorities for research funding and priorities for gap analysis funding. A second axis of recommendations is related to regulatory processes and the final one towards the metrics of progress.

IRDiRC-related calls

E-Rare-3 has launched a call for proposals for "clinical research for new therapeutic uses of already existing molecules (repurposing) in rare diseases." The objective of this call is to promote the clinical and pre-clinical proof of concept studies for the potential application of medicinal products.

The European Commission (EC) has launched a call in the framework of Horizon2020 for "New therapies for rare diseases." Support will be provided to clinical trials on substances where orphan designation has been given by the EC, with protocal assistance by the EMA, and a clear patient recruitment strategy.

The European Commission (EC) has also launched a call for "Diagnostic characterization for rare diseases. "The aim of this research should be to apply genomics and/or other –omics and/or other high-throughput approaches for the molecular characterisation of rare diseases.

The French Foundation for Rare Diseases has launched a call for "High-throughput screening of molecules with therapeutic potential," to support research projects using high-throughput screening for the discovery of active molecules with therapeutic potential.

All calls can be found on the IRDiRC-related calls page.

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