IRDiRC MARCH 2017 UPDATE

Open for public consultation: recommendations from the Data Mining and Repurposing Task Force

The members of IRDiRC's Task Force on Data Mining and Repurposing have drafted recommendations resulted from the Task Force's workshop in November 2016. This workshop's discussion included perspectives from different experts and stakeholders in rare disease drug development, including researchers, commercial development partners, regulators, and patient organizations. These consensus recommendations reflect the shared view that strategic investments in research will accelerate the development of new therapies for rare disease patients.

Recommendations were made concerning data mining and repurposing research infrastructure needs, in particular on improving the capture and sharing of patient data, the better integration of existing research data, increasing experimental and clinical testing capacity, and nurturing rare disease research and development expertise. Recommendations were also made on increasing repurposing and data-mining opportunities, emphasizing on the identification of more repurposing candidates, and on expanding strategic business models for repurposing development.

The Task Force is now seeking wider feedback on their recommendations. Anyone with an interest or expertise in data mining and repurposing is invited to comment on the document until April 21st, 2017. Feedback should be send to the IRDiRC Scientific Secretariat (contact@irdirc.org).

IRDiRC new documents

• Diagnostic Scientific Committee – report of the12th Diagnostic Scientific Committee meeting – February 6, 2017, Paris, France
• Interdisciplinary Scientific Committee – report of the16th Interdisciplinary Scientific Committee meeting – February 6, 2017, Paris, France
• Therapies Scientific Committee – report of the 10th Therapies Scientific Committee meeting – February 6, 2017, Paris, France
• Interdisciplinary Scientific Committee – report of the15th Interdisciplinary Scientific Committee meeting – January 13, 2017 - Teleconference

Upcoming IRDiRC teleconferences and meetings

• April 5, 2017 – Operating Committee – Teleconference
• November 13-14, 2017 – Consortium Assembly – Face-to-face meeting in Tokyo, Japan

Research highlights from IRDiRC members

• YposKesi welcomes Alain Lamproye, an expert in industrial bioproduction, as its Chief Executive Officer
• Lysogene receives Orphan Drug Designation from EMA and FDA for LYS-GM101
• (Update) Initiative on Rare and Undiagnosed Diseases (IRUD) in Japan
• Shire launches “Rare Count” campaign to personalize the global impact of rare diseases

• NIH scientists identify rare disorder causing blindness, deafness, albinism and fragile bones
• Reddit "ask me anything" recap: the Undiagnosed Diseases Network
• NICHD scientists identify in mice a potential mechanism of lysosomal storage disorders
• Stars of rare disease community recognized at EURORDIS Awards Ceremony
• NORD unveils “do your share for rare,” year-long campaign for rare disease public awareness
• The 30th AFM-Telethon edition (France) reaches a final collection of 92,740,769 euros

Research highlights published on the website

• European Reference Networks: a new concept came to reality
• Report of the inauguration of the NGO committee for rare diseases
• A 10 year report on Conditional marketing authorisation by the European Medicines Agency

Research highlights

EU Malta Presidency Conference and Parallel Session on Rare Diseases

On March 20-21, 2017, a conference was held for the EU Malta Presidency. In this conference, a session took place on integration of healthcare and research on rare diseases. The ultimate goal of this session was to establish a long-awaited single pipeline covering research, tools and clinics leading to optimization and exploitation of results (and means), faster drug discovery and improved patients’ care for rare diseases.

TSC Chair Diego Ardigò presented on behalf of IRDiRC, in a part of the session that was dedicated to presenting different research collaboration initiatives in the current rare diseases landscape. Other initatives that were presented were RD-ACTION, Orphanet, BBMRI-ERIC, E-RARE, European Commission Directorate General Joint Research Center and the National Center for Rare Diseases of Instituto Superiore di Sanità (ISS), Italy.

At the second day of the conference saw a session dedicated to development and access to medicines. This session was dedicated to promote structured European collaboration on rare diseases and also to promote structured European collaboration on joint procurement and access to medicines.

IRDiRC's new members

IRDiRC welcomes the Loulou Foundation and Recursion Pharmaceuticals as new members

The Loulou Foundation, a private, non-profit UK foundation dedicated to advancing research into the understanding and development of therapeutics for CDKL5 deficiency disorder, has joined IRDiRC as new funding agency. Next to funding research, the Loulou Foundation places a high priority on partnering with the biopharma industry.

Recursion Pharmaceuticals, Inc, is a US-based company, that has set themselves the challenging goal of discovering 100 rare diseases treatments for rare diseases in the next 7 years. This goal is aligned with IRDiRCs objectives for therapeutic development. Christoper Gibson, CEO and Co-Founder of Recusion, recently presented at the 3rd IRDiRC Conference, in which he outlined Recursion's pipeline for drug development.

IRDiRC-related calls

The French Foundation for Rare Diseases has launched a call entitled 'Human and Social Sciences & Rare Diseases.' This call for proposals is intended to get a better understanding of individual consequences, family and social specifically related to the scarcity of the disease and increase knowledge

on the specific impact of these diseases in terms of disability and of quality of life. Applications need to be connected to a French healthcare institution. Application deadline: April 27, 2017

Other news

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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