IRDiRC HIGHLIGHTS

New Task Forces & Working Groups

Enabling and Enhancing Telehealth for Rare Diseases Across the Globe

IRDiRC is currently assembling a team of experts to populate this Task Force and is specifically looking for members with expertise/experience in one or more of the following areas:

- Telehealth providers across a wide variety of specialties, disorders, and geographic locations
- Professional organizations that support career development for clinicians
- Primary and specialty care providers
- Policy makers
- Healthcare systems
- Healthcare insurance companies

Able to commit to monthly teleconferences, a yearly meeting, regular committee activities, and email correspondence

**The deadline for application is 30 November.** If you are interested in taking part in this activity, please send your CV, biosketch, and letter of motivation (one paragraph each) to the Scientific Secretariat.

Working Group Opening: MedTech for Rare Diseases

The **Therapies Scientific Committee (TSC)**, the **Interdisciplinary Scientific Committee (ISC)** and the **University of Twente** are jointly establishing a Working Group to explore the role and value of medical devices in rare diseases. The Working Group aims to create a better understanding and enhanced awareness of device developer’s needs, the standardized outcomes to define user needs for devices, and
to offer a groundwork for developing solutions to improve the (regulatory) landscape of MedTech use for rare disease patients.

Interested candidates can submit their application (CV, biosketch and letter of motivation, one paragraph each) to the Scientific Secretariat before 14 December.

1000 new rare diseases treatments by 2027, identifying and bringing forward strategic actions

The IRDiRC: 1000 new rare diseases treatments by 2027, identifying and bringing forward strategic actions has been published in the Rare Disease and Orphan Drugs Journal. The manuscript describes the recent key steps undertaken by the IRDiRC Therapies Scientific Committee (TSC) to support the future approval of 1000 new therapies for rare diseases.

NEWS FROM IRDiRC MEMBERS

Chan Zuckerberg Initiative launches Request for Information (RFI) on Patient Registry Data Interoperability

The Chan Zuckerberg Initiative (CZI) has launched a Request for Information (RFI): Patient Registry Data Interoperability as part of the Rare As One project, aimed at mapping data interoperability challenges and support ideas that will make it easier for patient communities to share data with researchers, and for researchers to fully leverage patient voices in research. Application Deadline: November 30th
European Joint Programme on Rare Diseases Joint Transnational Call 2022: Pre-announcement of the funding opportunity for research projects

The European Joint Programme on Rare Diseases just pre-announced the Joint Transnational Call 2022, a funding opportunity for research projects on the development of new analytic tools and pathways to accelerate diagnosis and facilitate diagnostic monitoring of rare diseases. The aim of the funding opportunity is to enable scientists in different countries to build an effective collaboration on a common interdisciplinary research project based on complementarities and sharing of expertise, with expected impact to use the results in the future for benefit of patients.

Topic: Development of new analytic tools and pathways to accelerate diagnosis and facilitate diagnostic monitoring of rare diseases

The call will open on December 14th.

Leave No Patient Behind - A Global Black Bone Disease Registry by the AKU Society

The AKU Society just launched a crowdfunding campaign to raise funds for a global Black Bone Disease registry in order to gather information about AKU patients around the world in one single place.

This will allow the AKU Society to make the case for access to the life-changing drug Nitisinone in the many countries where it is not yet available. It will also help them prepare plans for gene therapy and other new treatment studies.