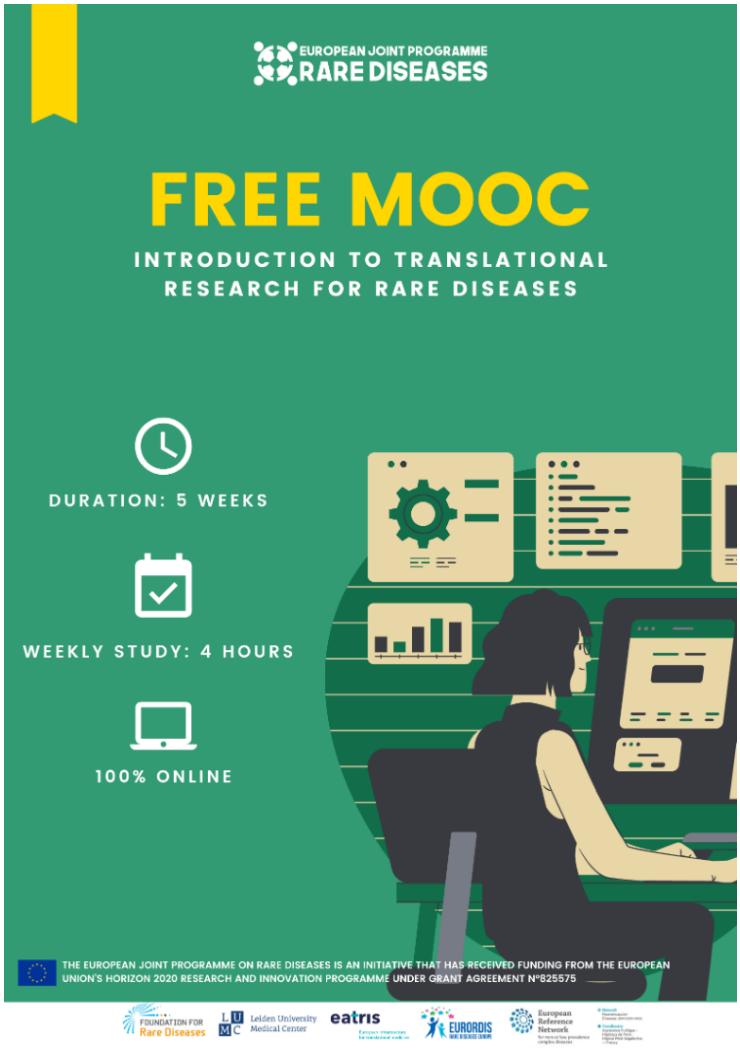
To consult the online version, click here

What's new?

September 2022

HIGHLIGHTS

EJP RD launches a new free MOOC "Introduction to translational research for Rare Diseases"



On this five-week course from the European Joint Programme on Rare Diseases entitled ***"Introduction to translational research for Rare Diseases"***, you'll explore the **therapeutic development process from the unique perspective of rare diseases.**

You will follow the journey from discovery to approved treatment, learn how therapeutic targets and drug candidates are identified and validated, and examine the special regulatory frameworks.

This course is designed for **researchers and students in medicine and health-related research fields, as well as healthcare professionals wanting to further their knowledge of translational research in rare diseases.**

It is also suitable for **biotech and start-up developers and Patients Advocacy Organisation representatives** who want to increase their understanding of current practices in therapeutic developments for rare diseases.

More information

International Conference on Clinical Research Networks for Rare Diseases

The International Rare Diseases Research Consortium (IRDiRC) and the European Joint Programme on Rare Diseases (EJP RD) are jointly organizing a **two-day conference on clinical research networks (CRNs) for rare diseases**, which will take place on **December 1st and 2nd**. The objective of this conference is to gather experts from different continents to increase mutual knowledge on CRNs structure, activities and identify pathways to stimulate collaboration and interoperability of these networks.

To register for online participation, please complete this registration form.

More information

SAVE THE DATE: RE(ACT) Congress & IRDiRC Conference

Standard registration deadline: December 31st

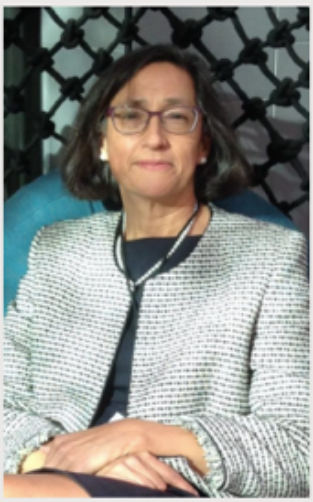
The joint event **"RE(ACT) Congress and IRDiRC Conference 2023"** aims to bring together **scientific leaders and experts and young scientists from various breakthrough scientific fields to present cutting-edge research, exchange ideas, and discuss rare diseases research policies**. Patients and patient organizations committed to research will also be in attendance to share their experiences and perspectives.

This event represents an exciting program with outstanding speakers and an **in-person event with multiple ways of networking!**

We look forward to welcoming you to **Berlin, Germany** from

March 15th - 18th, 2023.

Below some of the speakers you will get the opportunity to hear from:



ALARCÓN RIQUELME Marta

My background in Medicine, Immunology, and Genetics make a unique combination and provide me with the tools I need to carry out this project successfully. For over 20 years of my research career, I have focused on the identification of the genetic basis of SLE as a first building block toward understanding how such genes […]

BROOKS Philip John (P.J.)

Philip John (P.J.) Brooks is a Program Director in the NCATS Office of Rare Diseases Research (ORDR). Dr. Brooks received his Ph.D. in neurobiology from the University of North Carolina at Chapel Hill. After completing a postdoctoral fellowship at the Rockefeller University, Brooks became an investigator in the intramural program of the National Institute on [...]

WONG-RIEGER Durhane

DURHANE WONG-RIEGER, PHD is Chair of Rare Disease International, Vice-Chair of Asia Pacific Rare Disease International, member of the Editorial Board of The Patient-Patient Centred Outcomes Research, member of the Global Commission to End the Diagnosis Odyssey for Rare Diseases and member of Health Technology Assessment International Patient /Citizen Involvement Interest Group. In Canada, [...]

BUCKINX Tim

Tim Buckinx is the founder and CEO of epihunter. Tim has a professional background in global digital strategy leadership and is the father of a son with ring chromosome 20 syndrome, a type of rare refractory epilepsy. In 2015, his son, at that moment 10 years old, asked Tim « Papa, you work in digital, can't [...]

And many more speakers from all over the world!

Registration and programme

European Partnership Stakeholder Forum - One-year review of European Partnership Initiatives in Horizon Europe

On **15 and 16 November 2022** the European Commission will launch its first **European Partnership Stakeholder Forum**. The Forum focuses on the **review of the first year of partnerships and, in particular, how they can contribute to the twin green and digital transitions, as well as increasing Europe's resilience.**

EJP RD Coordinator Daria Julkowska will participate to the Panel discussion/ High-level round-table ***"How do European Partnerships contribute to EU political priorities".***

ERA-LEARN and the European Commission are co-organising this

Forum as a physical event with the possibility to follow it in streaming in silent mode.

Registration for the event is now possible. Given the limited capacity of the venue, please register as soon as possible. We can only confirm a limited number of registrations.

Registration & more

OPEN FUNDING OPPORTUNITIES

ERN Research Training Workshops Call
Deadline extended: October 15th

The **ERN Research Training Workshops funding opportunity** is now open for applications until **October 15th**. The goal of the workshops is to **train researchers and clinicians affiliated with ERN Full Members or Affiliated Partners in relevant topics on research in rare diseases**.

The workshops will be delivered as two-day events. The **costs for the workshop organization will be covered up to a limit of €25,000.**

Organizer's profile: The applicant submitting workshop topics must fulfil one of the following conditions:

- Affiliated to any EJP RD beneficiary institution
- Affiliated to an ERN Full Member
- Affiliated to an ERN Affiliated Partner institution at the time when the application is submitted, as well as during the period of the execution of the workshop.

More information

ERN Research Mobility Fellowship Call
Now open until November 13th

EJP RD has announced the opening of the call for **Research Mobility Fellowships** which aims to support PhD students, postdocs and medical doctors in training to undertake scientific visits fostering specialist research training outside their countries of residence.

Deadline: November 13th

The exchange can be carried out **(1) within the same ERN (Full Members and Affiliated Partners), (2) between different**

ERNs (Full Members and Affiliated Partners), or (3) between ERN Full Members / Affiliated Partners and non-ERN institutions.

Either home or host (secondment) institution must be a Full Member or Affiliated Partner of an ERN at the time when the application is submitted, as well as during the proposed period of the training stay.

Successful applicants should acquire new competences and knowledge related to their research on rare diseases, with a defined research plan and demonstrable benefit to the ERN of the home and/or host institution.

The research mobility fellowships are meant to cover stays of **4 weeks to 6 months duration**.

More information

Networking Support Scheme

Last collection date: December 1st

The Networking Support Scheme is back. **The aim of the NSS call is to encourage knowledge-sharing between health care professionals, researchers and patients on rare diseases and rare cancers, as well as to enable or increase the participation of usually underrepresented countries in Europe in new and existing research networks.** Eligible applicants are health care professionals, researchers, and patient advocacy organisations from the following countries involved in the EJP RD: Armenia, Austria, Belgium, Bulgaria, Croatia, Czech Republic, Denmark, Estonia, Finland, France, Germany, Georgia, Greece, Hungary, Ireland, Israel, Italy, Latvia, Lithuania, Luxembourg, Malta, Norway, Poland, Portugal, Romania, Serbia, Slovakia, Slovenia, Spain, Sweden, Switzerland, the Netherlands, Turkey, United Kingdom. There is no limit on the number of participants per event; however, the maximum budget that can be requested is **€30,000 per networking event.**

The next collection date is **December 1st at 14:00 (CET). This will be the last collection date.**

More information

RARE DISEASES TRAINING AND EDUCATION

Biobanking in rare diseases: the Poland experience

Registration deadline: November 10th

The upcoming EJP RD training entitled **Biobanking in rare diseases: the Poland experience** is now open for registration. The 2-day training will be held on **November 28-29th online**. The training is dedicated to scientists of biomedical sector who want to learn about biobanking standards in rare disease research.

Registration is open until November 10th.

Registration and more

The Statistical Evaluation of Surrogate Endpoints in Clinical Trials

Registration deadline: November 14th

As part of the training activities proposed by the EJP RD, a **2h30 training course titled "The Statistical Evaluation of Surrogate Endpoints in Clinical Trials" is being organised. The fully online event will take place from November 18th.**

The aim of the lecture is:

- To get an overview of the methodological developments in surrogate endpoint evaluation over the last 30 years
- To understand practical use
- To understand promise and limitations
- To understand how they can be useful in the context of rare diseases

This training is at an advanced level course. While an overview will be given, without all technical details, a variety of statistical modelling and inference concepts will be used.

Registration is currently open until November 14th.

Many speakers are in the line-up such as Prof. Geert Molenberghs.

Check out all the speakers' biosketches and register!

Registration and more

Update on ”MOOC on Diagnosing Rare Diseases: from the Clinic to Research and back

New facilitation window

In the context of EJP RD’s ERN Workshops, a face-to-face workshop entitled “**Epidermolysis bullosa: from genes to translation into therapies**” is being organized by Prof. Dr. Cristina Has of the University Hospital Freiburg.

The in-person event will take place on **23-24 March 2023** in CIBSS – Centre for Integrative Biological Signalling Studies in Freiburg, Germany.

Registration is open until December 15th.

Registration and more

Update on ”MOOC on Diagnosing Rare Diseases: from the Clinic to Research and back

New facilitation window

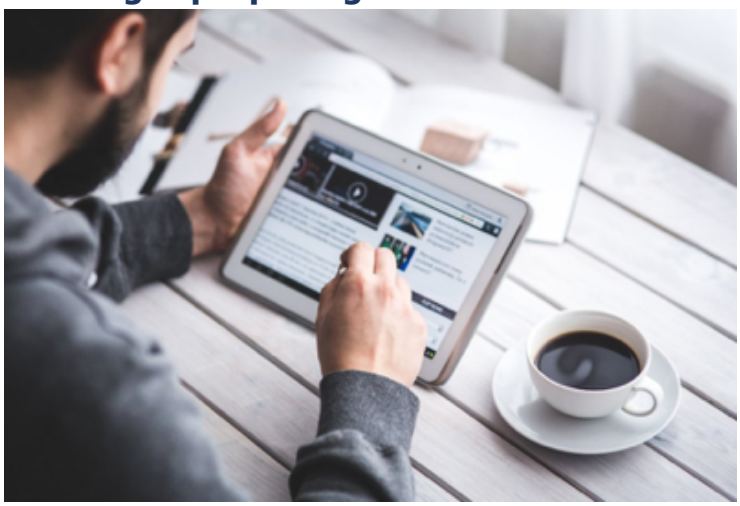
Mentors and educators, experts in the field, are available online to answer your questions and to support you during your journey in this EJP RD MOOC until **December 3rd, 2022**.

Take the opportunity to interact with them!

More information

OTHER RELATED NEWS

Article: Identifying obstacles hindering the conduct of academic-sponsored trials for drug repurposing on rare-diseases: an analysis of six use cases



An article entitled ***Identifying obstacles hindering the conduct of academic-sponsored trials for drug repurposing on rare-diseases: an analysis of six use cases*** has been published on September 15th. This paper, in which EJP RD members participated in, **identifies hurdles in the set-up of six multinational clinical trials for drug repurposing, as use cases.**

Read the article

Registration open for the Third International Summit on Human Genome Editing

The registration for the **Third International Summit on Human Genome Editing is now open**. The event will take place on **6-8 March 2023** at the Francis Crick Institute, London UK.

Related news: Dr. Daria Julkowska, Coordinator of the EJP RD, is a member of the planning committee. More information here

Registration

IRDiRC INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

NEWS FROM THE INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM (IRDIRC)

Orphanet Journal of Rare Diseases series looking for authors on MedTech

As one of the outcomes of the **MedTech for Rare Diseases Working Group** led by IRDiRC members Anneliene Jonker and Marc Dooms, the Orphanet Journal of Rare Diseases has decided to focus a thematic issue on the questions regarding the research and development of orphan devices and medical technologies that are being developed for Rare Diseases patients.

More information

FDA Rare Neurodegenerative Disease Grant Program

In addition to the Orphan Products Grants Program that the Office of Orphan Products Development (OOPD) currently administers, a new grant program was established this year by the Accelerating Access to Critical Therapies for Amyotrophic Lateral Sclerosis Act (ACT for ALS) named the **FDA Rare Neurodegenerative Disease Grant Program.** This new program will be administered by OOPD **to promote medical product development for rare neurodegenerative diseases such as ALS.**

FDA U.S. FOOD & DRUG ADMINISTRATION

More information

CAREERS

[Job opportunities] are available at EJP RD member institutions:

- **EJP RD Coordination Team** is looking for an **Office Manager (bilingual French-English)**
- **EURO-NMD** is looking for a **Senior Project Manager**
- **EURORDIS** is looking for an **Events and Nominations Intern,** an **Operations Junior Manager, 2 Research Executives** and a **Research Assistant**
- **Izmir Biomedicine and Genome Center (IBG)** is looking for an **ERA Chair and Head of the Unit for Rare Diseases at IBG (project RareBoost #952346, H2020- WIDESPREAD-06-2020)**

EJP RD has received funding from the

European Union's Horizon 2020 research and innovation programme under GA N°825575