What's new
this month?

August–September 2021

EJP RD HIGHLIGHTS

UPCOMING EVENT

The EJP RD General Assembly and Consortium Meeting (online)

Ongoing: 14-16 September 2021

The **Third EJP RD General Assembly (GA) and Consortium meeting** is currently taking place online (**September 14 to 16, 2021**).

The GA meeting is the perfect occasion to **bring together all EJP RD members to have an open discussion on the work done so far and define the new road map for the next year(s)**.

This year, the program includes sessions focusing on **trans-pillar activities and on joint activities between EJP RD and external collaborators**. In addition, plenary sessions are providing more original inputs that would be used to finalise the new EJP RD road map for 2022 and beyond, with a plenary session dedicated to the Rare Diseases Partnership that is planned to be launched under Horizon Europe.

Search for #EJPRDGeneralAssembly2021 on Twitter for updates!

FUNDING OPPORTUNITY

ERN Research Training Workshops Funding Opportunity

Now Open until 4 October

The **ERN Research Training Workshops funding opportunity is now open** until **October 4th**.

The goal of the workshops is to train researchers and clinicians affiliated to ERN Full Members or Affiliated Partners in relevant topics on research in rare diseases. Training themes may include innovative research methodologies, diagnostic research methodologies, interdisciplinary treatment approaches, such as gene therapy and transplantation, etc.

The workshops will be delivered as two-day events. **Costs for workshop organisation will be covered up to a limit of €25,000** (venue, administrative, audio-visual and IT facilities essential for the workshop, catering, travel and accommodation expenses of workshops participants and invited speakers, if envisaged).

The workshops selected for funding will be attended only by individuals affiliated to ERN institutions. The applicant submitting workshop topics must fulfil one of the following conditions:

• Affiliated to any EJP RD beneficiary institution, or

• Affiliated to an ERN Full Member, or

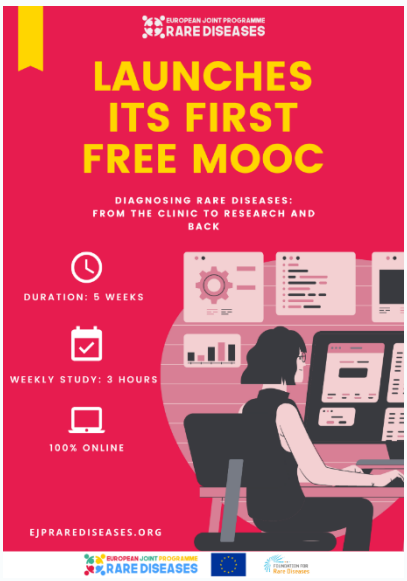
• Affiliated to an ERN Affiliated Partner institution (at the time when the application is submitted, as well as during the period of the execution of the workshop)

More information

MOOC

Diagnosing Rare Diseases: From the Clinic to Research and back

Second run from October 4th!



The second run of the **MOOC (Massive Open Online Course) "Diagnosing Rare Diseases: From the Clinic to Research and back"** co-developed by EJP-RD, ERN Ithaca, ERN Genturis and the Foundation for Rare Diseases will start on **October 4th**. Registrations are free and open **here**.

1800 learners from **117 countries** (40% outside Europe) **joined the first session**.

This first run received a **91.5% positive feedback** and learners contributed with 1836 comments.

We specifically encourage medical and biomedical science students to register and follow the MOOC.

More information

SEPTEMBER 20TH

Resource Webinar: European Genome-Phenome Archive (EGA)

As part of the **EJP RD Resource Webinar series**, a webinar dedicated to the **European Genome-Phenome Archive (EGA)** will be held on **September 20th**. Participants will be given an overview of the EGA. This will include a background to the EGA, data types held, how to search and apply for data access, and how to download data.

More information

OCTOBER 14TH

Resource Webinar: European Paediatric Translational Research Infrastructure (EPTRI)

As part of the **EJP RD Resource Webinar series**, a webinar on **European Paediatric Translational Research Infrastructure (EPTRI)** will be held on **October 14th**.

The webinar will introduce the audience to the processes of **obtaining age-tailored drugs through dedicated services in paediatric medicines discovery, biomarkers, developmental pharmacology, formulations, medical devices,** and any other advancements and innovations for children health, which are available thanks to the **participation of 113 centres of excellence** in research involved in the EPTRI project.

More information

OCTOBER 25TH - 27TH

Training: Pluripotent stem cells for rare disease research: banking, data, application

REGISTRATION DEADLINE: September 27th

As part of the training activities proposed by the EJP RD, **Pluripotent stem cells for rare disease research: banking, data, application** is a **3-day online training programme** organised by EJP-RD partners Fraunhofer Institute for Biomedical Engineering (Berlin, Germany) and Fondazione Telethon (Milan, Italy) , aimed at biomedical researchers, medical professionals, and biobank managers who want to learn about the **application of human pluripotent stem cell (hPSC) lines in rare disease research**.

The fully online event will take place from **October 25th – 27th** and is composed of **four learning modules.**

Online registration for the event is open until **September 27th** at the link **here**.

More information

EJP RD FUNDING OPPORTUNITIES

Next collection date: December 2nd

Networking Support Scheme (NSS) Funding Opportunity
Next collection date: December 2nd, 2021

UPDATE: The NSS has been expanded to include online and hybrid networking events that can now be funded in addition to face-to-face events. A hybrid networking event consists of a group of participants networking face-to-face at a specific location together with other participants networking online.

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 2nd at 14:00 (CET).

Selected past networking events are available **here**.

To get more information and to apply, click below.

More information

All EJP RD open funding opportunities here

EJP RD IN EVENTS

During the month of September, EJP RD is being presented at the following events:

- During the **National Press Foundation workshop** titled *Covering Rare Diseases* **(September 13th-14th) at which Dr. Daria Julkowska presented the approach to RDs in Europe and spoke about EJP RD to the panel of journalist participants from all over the world.**
- During the **RWE4Decisions webinar** entitled ***Delivering a Health Data Strategy for the European Reference Networks (ERNs)*** (**September 29th**). See the Other News section for more details.

NEWS FROM THE INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM (IRDIRC)

IRDiRC at National Press Foundation Workshop on Rare Diseases

IRDiRC members Lucia Monaco (Consortium Assembly Chair), Daria Julkowska, Durhane Wong-Rieger, Gareth Baynam, Samuel Agyei Wiafe, Anne Pariser, and Ritu Jain **discussed IRDiRC at a global scale** during a **two-day online workshop** entitled "Covering Rare Diseases" on **September 13th – 14th** and organised by the **National Press Foundation**.

More information

Publication: Essential List of Medicinal Products for Rare Diseases

IRDiRC is pleased to announce that the **recommendations from the IRDiRC Rare Disease Treatment Access Working Group** have been accepted for publication in the **Orphanet Journal of Rare Diseases**. The manuscript is titled **"Essential List of Medicinal Products for Rare Diseases – Recommendations from the IRDiRC Rare Disease Treatment Access Working Group"** and is authored by William A. Gahl, Durhane Wong-Rieger, Virginie Hivert, Rachel Yang, Galliano Zanello, and Stephen Groft.


Orphanet Journal of Rare Diseases

RESEARCH Open Access

Essential list of medicinal products for rare diseases: recommendations from the IRDiRC Rare Disease Treatment Access Working Group

BMC

More information

Leadership and Membership Changes

IRDiRC congratulates **Samuel Agyei Wiafe** for his election as **Vice-Chair** of the **Patient Advocates Constituent Committee (PACC)**.

More information

OTHER NEWS

CeBIL Annual Symposium 2021 – Orphan Drug Innovation: Needs and Priorities

The Centre for Advanced Studies in Biomedical Innovation Law (CeBIL) at the University of Copenhagen and the University of Cambridge's Centre for Law, Medicine and Life Sciences (LML) are jointly organising the **2021 Annual Symposium** (**fully online**) on the theme of **Orphan Drug Innovation: Needs and Priorities** on **September 17th** from 15.00 – 17.30 CET.

The symposium aims to **identify and explore the most pressing priorities in orphan drug innovation, and whether and how social science research, particularly legal research, can help meet these needs**.

More information

Webinar: Leaving the darkness – tackling rare bone diseases

Economist Events and Ipsen are organising a webinar entitled **"Leaving the darkness: Tackling rare bone diseases"** on **September 22nd** from 17.00 – 18.00 CET.

The webinar will discuss the **complex context of rare bone diseases and how care can be re-oriented to ensure the patient's priorities are put first**. The speakers will also address the **barriers to ensuring equitable access for patients suffering from rare bone diseases** and the **opportunities provided by new technologies to improve diagnosis, care, and education**.

More information

ISPOR Webinar: Health Preference Research in the Context of Gene Therapy in Rare Diseases

The Professional Society for Health Economics and Outcomes Research (ISPOR) is organising a webinar entitled "Health Preference Research in the Context of Gene Therapy in Rare Diseases" that will be limited to **1000 registered participants on a first-come, first-served basis**.

The webinar will take place on **September 27th** from 16.00 – 17.00 CET.

This webinar will present the **value of health preference research to study the promise of gene therapy in rare diseases, through three short presentations in which the speakers will present their respective studies on gene therapy in Duchenne muscular dystrophy, spinal muscular atrophy (SMA), and hemophilia.**

More information

RWE4Decisions Webinar: Delivering a Health Data Strategy for the European Reference Networks (ERNs)

RWE4Decisions, a multi-stakeholder initiative commissioned by the Belgian National Institute of Health, is organising a webinar entitled **"Delivering a Health Data Strategy for the European Reference Networks (ERNs): Developing a Shared Vision with All Stakeholders"** to **discuss what a co-created Health Data Strategy for the ERNs could look like**.

The webinar will take place on **September 29th** from 16.00 – 17.30 CET.

During the webinar, experts from ERNs will discuss the need to build a long-term vision and a holistic data strategy for ERNs.

More information

EFPIA Workshop: Accelerating Adoption of Complex Clinical Trials in Europe and Beyond

The **European Federation of Pharmaceutical Industries and Associations (EFPIA)** is hosting a multi-stakeholder virtual workshop titled "Accelerating Adoption of Complex Clinical Trials in Europe and Beyond" aimed at **developing shared solutions for the use of complex clinical trials to optimise drug development in Europe**.

The online event will take place over two days: **October 5th - 6th**. The **hands-on workshop format** features use of **case examples and interactive sessions to facilitate co-creation of solutions** and active contribution by participants.

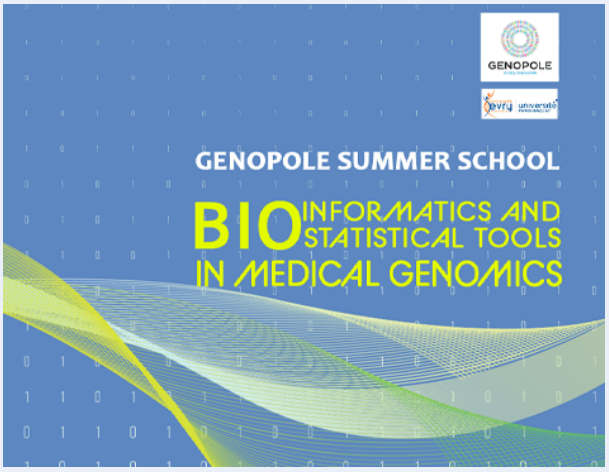
More information

Better Medicines for Children Conference: Global Paediatric Drug Development Challenges & Solutions

The **European Forum for Good Clinical Practice (EFGCP)** and the **Drug Information Association (DIA)** are organising the **Better Medicines for Children Conference 2021** (**fully virtual**) on the theme of "Global Paediatric Drug Development Challenges and Solutions – The Path Forward" from **October 18th – 19th** from 11.45 – 18.00 CET. The conference is an **annual flagship event providing a unique opportunity to share best practice updates and a truly global outreach**, bringing together all the stakeholders involved, namely regulators, health technology assessment (HTA) bodies and payer representatives, industry, academia and patient representatives.

More information

Genopole Summer School 2021: Bioinformatics and Biostatistical Tools in Medical Genomics



Genopole, the oldest and largest biocluster in France, is organising its **Summer School 2021** on **statistical methodologies and bioinformatics tools used in genomics and metagenomics in the context of a comprehensive approach to pathological mechanisms**. The Summer School, which is **limited to 20 participants**, will take place in the **Greater Paris region (Seine-Port)** over four days from **October 18th – 21st**. The programme includes lectures by guest speakers as well as hands-on sessions with experts in the field.

More information

National Organization for Rare Disorders (NORD) Rare Breakthrough Summit 2021

The **National Organization for Rare Diseases (NORD)** is organising its annual **NORD Summit 2021**, one of the largest multi-stakeholder events in rare disease, bringing together rare disease community stakeholders, including rare disease experts and leaders from patient advocacy groups, government, industry, and academia **to discuss the most current and critical topics related to rare diseases and orphan products**. The **fully virtual** summit will take place over two days from **October 18th – 19th**.

More information

CAREERS

Job opportunities are available at EJP RD member institutions:

- Department of Genetics, AP-HP (Paris University Hospital Trust) is looking for a **Pedagogical Engineer/Moodle Developer**
- Faculty of Medicine, Heinrich Heine University (HHU) Dusseldorf is looking for a **Postdoctoral Research Scientist**
- Duchenne Data Foundation is looking for a **Software Engineer**
- Institute of Human Genetics, University Hospital Heidelberg is offering a **Post-Doctoral Position**

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