

# What's new this month?

June 2021

## EJP RD HIGHLIGHTS

### RESOURCE FOCUS

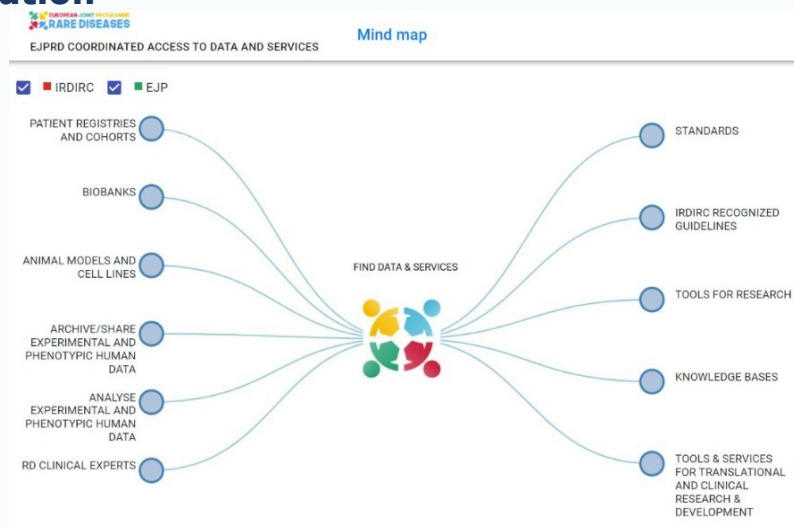
## Resource Finder Demonstration

One of the aims of EJP RD is to **increase the**

**discoverability of resources** that can be useful to the rare disease community.

To achieve this goal, EJP RD has developed the **Resource Finder**, a **new tool to easily find important rare disease resources based on different categories**. It is based

on a simple interface that provides information on IRDiRC-recognised and EJP RD-funded resources and tools.



[More information](#)

JUNE 21ST

## Resource Webinar: Human Pluripotent Stem Cell Registry (hPSCreg)



As part of the [EJP RD Resource Webinar series](#), the next webinar will be dedicated to [hPSCreg](#) and will be held on **June 21st**. The **human pluripotent stem cell registry (hPSCreg)** maintains resources to track the usage of human embryonic and induced pluripotent stem cell lines from research to clinical applications. Participants will be given an overview of hPSCreg from different

stakeholder perspectives (basic and translational researchers, industry, and funders) and will learn about what data is available for each registered line and the benefits of registering lines in hPSCreg.

[More information](#)

FORTHCOMING

## ERN Workshop: Clinical Epidemiological Research—The Basics and Beyond

**Registration deadline: June 18th**

In the context of the EJP RD [ERN Research Training Workshop](#) funding opportunity,

a **face-to-face workshop on clinical epidemiological research for ERNs** titled **Clinical Research: The Basics & Beyond**, consisting of presentations by experts in the field of epidemiology and a feedback session on the research projects of participants to **enable clinical researchers to plan, perform and analyse large datasets** by taking into account characteristics of research in rare diseases, is being organised by **Dr. Olaf Dekkers** of the Leiden University Medical Center.

The in-person event will take place over two days on **30th September–1st October 2021** at the **Hotel Golden Tulip Leiden Centre, Schipholweg 3, 2316 XB Leiden, the Netherlands**.

Registration closes on **June 18th**, and those selected to participate from among the applicants will be informed by **July 9th** of their selection.



[More information](#)

NOMINATIONS OPEN!

## Nominations now open for the EURORDIS Black Pearl Awards 2022!



**EURORDIS** is pleased to announce that **nominations** for the **EURORDIS Black Pearl Awards 2022** are now open. The Awards are presented to patient advocates and organisations, policy makers, scientists, companies, and media in recognition of their exceptional achievements and outstanding commitment to the rare

disease cause. Just like every year, **you can nominate any individual or organisation from anywhere in the world** (including yourself and/or your own organisation) whose hard work and contribution to the community, in your opinion, deserves to be honoured. The awardees will be selected by the EURORDIS Board of Directors in **October 2021** and presented with their awards at the official Awards ceremony in **February 2022** to mark the occasion of **Rare Disease Day**.

[More information](#)

## EJP RD FUNDING OPPORTUNITIES

Next collection date: September 2nd

### Networking Support Scheme (NSS) Funding Opportunity

**Next collection date: September 2nd, 2021**

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 September 2nd at 14:00 (CET)**. As a result of an assessment of the NSS, new documents will be available **mid June**. One of the changes is that from **June 3rd** the **NSS will be expanded: online and hybrid networking events 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. 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 May, EJP RD was presented at the following events:

- During an **Alexion Rare Conversations** webinar titled *Research In Rare Diseases: Specificities and Needs* (**May 5th**). Read more about this event [here](#).
- During a series of meetings organised by **Orphanet** on the *rare disease data ecosystem* (**May 7th**).
- During a *rare disease stakeholders event* (**May 11th**) organised by the **Federal Office of Public Health (FOPH), Switzerland** in the context of Swiss involvement in rare disease activities at the EU level.
- During the **European Rare Disease Research Coordination and Support Action (ERICA) Kick-Off Meeting** (**May 27th**). Read about the event [here](#).

## NEWS FROM THE INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM (IRDiRC)

### Nomination Call: Therapies Scientific Committee

**Deadline: June 15th**

To best represent the worldwide rare diseases community in its full width and complement the current membership, IRDiRC invites applications for **two new members from Africa, Asia, Australia or South America** for its **Therapies Scientific Committee (TSC)**, a multi-stakeholder, multi-disciplinary group of experts in medical research and therapy development in rare diseases. Interested candidates are requested to apply **before June 15th**.

Apply before **June 15th**

### IRDiRC Nomination Call

Therapies Scientific  
Committee (TSC)

TSC has two openings for  
new members who are  
from the following  
regions: Africa, Asia,  
Australasia or South  
America.

IRDiRC  
INTERNATIONAL  
RARE DISEASES RESEARCH  
CONSORTIUM

[More information](#)

## Leadership and Membership Changes

IRDiRC warmly welcomes **two new members**, [InnoSkel](#) and the [Chan Zuckerberg Initiative](#).

**Samantha Parker**, earlier representing Lysogene, will now represent InnoSkel in her capacity as Chief Patient Access Officer. IRDiRC welcomes **Ralph Laufer**, Chief Scientific Officer, as the new representative of Lysogene.

IRDiRC congratulates **Anneliene Jonker** for her election as **Vice-Chair** of the **Therapies Scientific Committee (TSC)**.

[More information](#)

### OTHER NEWS

## NIH Virtual Roundtable on Gene-Targeted Therapies: Early Diagnosis and Equitable Delivery

[NCATS of the National Institutes of Health \(NIH\)](#) is hosting a virtual NIH roundtable meeting on the theme of Gene-Targeted Therapies: Early Diagnosis and Equitable Delivery. The virtual roundtable will take place on **June 3rd, 10th and 17th from 18.00 – 22.30 CET**.

This event will discuss **challenges and barriers to equitable delivery of gene-targeted therapies** to individuals living with rare diseases in a timely manner and identify possible solutions for these issues.

[More information](#)

## ProDGNE Kick-Off Webinar



The [ProDGNE consortium](#), funded as part of the [EJP RD's Joint Transnational Call 2020 "Pre-Clinical Research to Develop Effective Therapies for Rare Diseases"](#), announces its **ProDGNE Kick-Off Webinar**. The webinar will take place on **June 3rd from 18.00 – 19.00 CET**.

The webinar will present ProDGNE, a **3-year transnational pre-clinical research project** that brings together patients

and researchers from Europe and Canada to **develop an innovative therapeutic compound to treat GNE Myopathy (GNEM)**.

[More information](#)

# Findacure's Drug Repurposing for Rare Diseases Conference 2021

**Findacure** is organising its annual Drug Repurposing for Rare Diseases Conference 2021, a fully online event bringing together **patient groups, researchers, medical professionals and industry representatives** to **showcase innovative repurposing projects** and encourage conversation between all members of the rare disease community. The **online conference** will take place over two days on **June 15th and 16th**.

[More information](#)

## PedCRIN: Fostering International Paediatric Clinical Research

**Paediatric Clinical Research Infrastructure Network**

**(PedCRIN)** announces its PedCRIN Final Event on "Fostering International Paediatric Clinical Research", a 3.5-hour interactive session focused on the **challenges of paediatric multinational clinical trials**.

The webinar will take place on **June 16th from 09.30 – 13.00 CET**.



[More information](#)

## European Commission launches public consultation on the Orphan and Paediatric Regulations

As part of its **review of the Orphan and Paediatric Regulations** announced in 2020, the European Commission (EC) has launched a **public consultation** for all relevant stakeholder, who are invited to share their views and experiences on the main obstacles they are facing concerning treatments for rare diseases and children with a view to **making the current legislation future-proof**.

The public consultation is currently accepting inputs as a questionnaire and will close on **July 30th**.

[More information](#)

## EBiSC Bank for iPSC Lines



The **European Bank for induced pluripotent Stem Cells (EBiSC)** is a **non-profit iPSC repository** based in Germany and the UK that has collected, safeguarded and distributed **>900 iPSC lines** since 2014. As EBiSC aims to accelerate health research by making iPSC resources widely and publicly available for all researchers, they are assessing how

they can **support other iPSC-based research projects**, in addition to **continuing collaborations with iPSC researchers worldwide**. Click on the link to know more.

[More information](#)

## CAREERS

**Job opportunities** are available at EJP RD and its member institutions:

- The EJP RD Coordination Team is looking for a **Junior Project Manager**
- Centro Nacional de Análisis Genómico (CNAG-CRG) is looking for a **Communications and Data Helpdesk Coordinator**
- Institute of Human Genetics, University Hospital Heidelberg is offering a **post-doc position**
- ERN-EYE, Hôpitaux Universitaires de Strasbourg, France is looking for a **Scientific Project Manager**



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