

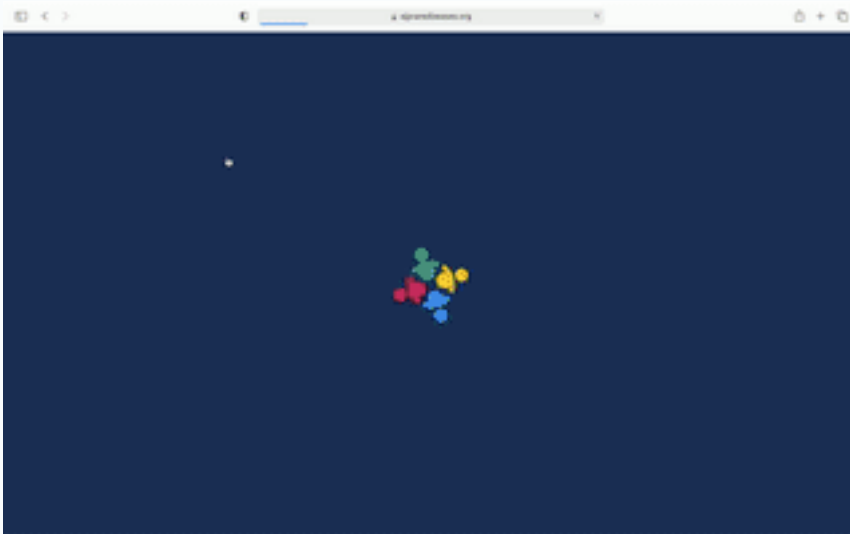
What is new this month?

April 2021

EJP RD HIGHLIGHTS

The EJP RD website is getting a new look!

30 MARCH 2021



After months of work and reflexion about how to make the EJP RD website more user-friendly and how to help visitors understand the programme and EJP RD activities, **we are glad to inform you that the new website is**

here! <https://www.ejprarediseases.org/>

Visit the new website

APRIL 26TH

Course: Diagnosing Rare Diseases: from the Clinic to Research and back

This course is designed **for individuals with a keen interest in diagnostic research and rare diseases**. While primarily designed for **medical students and PhD/post-doc students in biomedical sciences**, it will also be of **interest to Patients Advocacy Organisations' representatives, Healthcare professionals or paramedics** who want to further their knowledge of rare diseases diagnosis.

The preparation of this course has been a joint effort from several EJP RD Work Package 16 partners, in particular [ERN ITHACA](#) (represented by Prof Laurence Faivre), [ERN GENTURIS](#) (represented by Dr Chrystelle Colas) and [FFRD](#) (Roseline Favresse). Dedicated support and enlightened advice was also provided all along the development by [EURORDIS](#) (Dr Virginie Bros-Facer)

[Join course](#)

APRIL 26TH

Resource Webinar: Cellosaurus

In the context of [EJP RD Resource Webinar series](#), the next webinar will be dedicated to **Cellosaurus** on April 26th. Cellosaurus is cell line knowledge resource. The webinar will emphasise its use in the context of RD.

To register to the webinar, please click below.

[Registration](#)

OPEN UNTIL APRIL 26TH

Apply to The ERN Research Mobility Fellowship **until April 26th**

The ERN **Research Mobility Fellowships is still open. The funding opportunity aims to support PhD students, Postdocs and medical doctors in training to undertake scientific visits fostering specialist research training outside their countries of residence.**

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

Click below for more information and to apply.

[More information & Application](#)

EJP RD FUNDING OPPORTUNITIES

MARCH

Networking Support Scheme (NSS) Funding Opportunity Survey: Your View On Networking

What are your wishes and needs for networking? And how have they changed due to the COVID-19 pandemic? We are eager to hear your opinion! Click the button below and help us to improve through a survey the EJP RD Networking Support Scheme funding opportunity dedicated to networking.

To learn more about the **Networking Support Scheme funding opportunity**, click [here](#).

Complete the survey

All EJP RD open funding opportunities here



EJP RD IN EVENTS

During the month of March EJP RD was presented at the following events:

- **Supporting the innovation and exploitation activities of transnational research projects on rare diseases – an EJP RD and EC Webinar (March 25th):** The webinar provided an overview of the process and challenge of translating knowledge into patient benefit, and raised awareness of the tools, services and expert support available to translational researchers in the RD field through the European Joint Programme on Rare Diseases.
- **9th Presidency Conference: The why, what and how of tackling the Implementation Gap for healthcare in the EU (March 8th)** where you can join us at the panel discussion of Session III: *The why, what and how of promoting innovation to tackle rare diseases*. Other stakeholders such as healthcare professionals, decision makers, patient organisations, and European umbrella organizations representing interest groups and associations actively engaged in the field of Personalised Medicine will also have interactions that will create a cross-sectoral, highly relevant and dynamic discussion forum.
- **1st International Conference on Rare Diseases (March 1st)** which aspired to bring together all stakeholders in the rare disease community – patient representatives, policy makers, clinicians, researchers, industry representatives, payers and regulators to exchange invaluable knowledge with the aim to enhance dialogue and promote policy changes to

help build a better pathway from Diagnosis to Access for Rare Diseases. EJP RD was presented at the session 4 "Advance Research For Rare Diseases". **You can find the speakers presentations [here](#) and the Conference Press Release [here](#)**

NEWS FROM THE INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM (IRDIRC)

Leadership and Membership Changes

Dr. Daniel O'Connor, MHRA, UK, was elected new Chair of the [Therapies Scientific Committee \(TSC\)](#) for a 3-years mandate.

IRDiRC thanks **Dr. Diego Ardigò** and Dr. Virginie Hivert for serving as Chair and Vice Chair of the [Therapies Scientific Committee](#). Their commitment was essential to the work developed by the TSC in the last six years.

Dr. Diego Ardigò is replacing **Dr. Andrea Chiesi** as representative of Chiesi in the [Companies Constituent Committee](#).

IRDiRC welcomes [Congenica](#) as new member of the [Companies Constituent Committee](#) and the [Consortium Assembly](#). Congenica will be represented by **Dr. Christina Waters**.

Medics4RareDiseases online interactive e-learning platform

[Medics4RareDiseases](#) (M4RD) has launched its online interactive e-learning platform dedicated to teaching medics the fundamentals of rare disease and help them manage both their undiagnosed and diagnosed patients.

Click below to access M4RD courses.

M4RD courses

OTHER NEWS

Congenital Anomalies of Kidney and Urinary Tract disorders (CAKUT) Pathway Curation Workshop

Bioinformatics pathway modelling experts and biomedical/clinical researchers are working together to create improved pathway models of **Congenital Anomalies of Kidney and Urinary Tract disorders (CAKUT)**. **The first online pathway curation workshop was held on February 4th to create an expert curated** pathway (organizers: Friederike Ehrhart, Franz Schäfer, Chris Evelo, Lauren Dupuis). For this workshop, CAKUT experts were invited through the network of EJP RD member Franz Schäfer (12 Experts from Canada, France, Germany, Israel, UK, USA and the Netherlands joined the workshop). After a short welcome and introduction in "how to read and make pathway models in WikiPathways"

by BiGCaT member Lauren Dupuis, the experts took over, supported by pathway modelling experts from BiGCaT. **After the session two pathways were removed, two new pathways were created, and another one was modified.** The follow up is still ongoing. The ability to show that modeling specialists collaborate best with domain experts to create pathways for data analysis witnesses of the success of the meeting.

This workshop is prime example of how experts can be involved in getting the molecular basis. Similar events could be organized with any enthusiastic group that has similar knowledge.

RDCA-DAP Professional Webinar Series: Value of Integrated Data and Analytics in Rare Disease Drug Development

If you are an **industry, academic researchers and research-based nonprofit organization leaders interested in understanding how rare-disease data can be applied to accelerate drug development for rare diseases, [the Rare Disease Cures Accelerator–Data and Analytics Platform](#)** is launching a **new webinar series** with the goal of **sharing with the community examples of the use of rare disease person-level data in drug development and regulatory decision making.** The series will feature 1-hour webinars highlighting analyses that have been done in individual disease areas, how they informed drug development, and how similar approaches could be applied to common drug development issues encountered in rare diseases. After each presentation time will be allowed for discussion with a panel of quantitative and regulatory experts around why the solutions presented were informative, lessons learned and how similar approaches could be applied to related problems. **The webinars will be held on the third Wednesday of each month at 5 PM CET**

For more information on the webinar schedule and to register, please click below.

[More information](#)

VISION-DMD White Paper: Returning Individual Clinical Trial Results Back To Patients

[VISION-DMD](#) published a white paper on **how to return individual clinical trial results back to patients.** It focuses on **addressing the ethical and technical challenges, using the vamorolone clinical trial experience.**

Click below to read the paper.

[Read the paper](#)

Three-Year Post-Doctoral Open Position

A **post-doctoral fellow position is immediately available** in the Retrovirus-Host Interactions and Innate Immunity to Gene Transfer Lab, led by Anna Kajaste-Rudnitski at the San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), Milan, Italy. Click below to get more information on the job opportunity.

[More information](#)

CAREERS

Job opportunities are available at EJP RD member institutions

- ERN-EYE, Hôpitaux Universitaires de Strasbourg,
France is looking for a **Scientific Project Manager**



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