To consult the online version, click here

EUROPEAN JOINT PROGRAMME RARE DISEASES

What is new
this month?

March 2021

EJP RD HIGHLIGHTS

The EJP RD Rare Disease Day Video is online!

28 FEBRUARY 2021

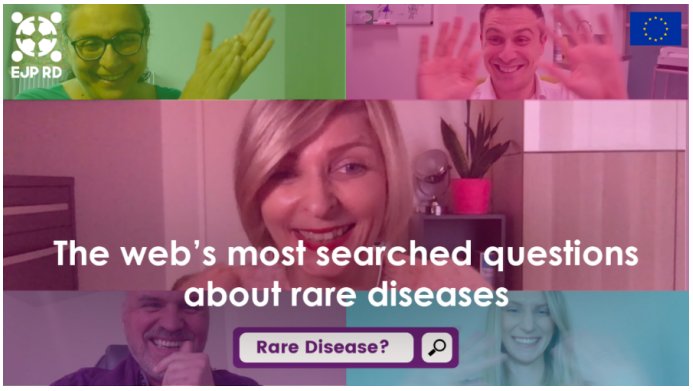
Happy Rare Disease Day!

Watch EJP RD contributors answer the web's most search questions about rare diseases in a new video that you can find HERE (subtitles available).

Like every year, EJP RD is proud to contribute to spreading awareness on Rare Disease Day.

Don't forget to **share the video and subscribe to our Youtube channel and to our Twitter to be kept updated on our funding opportunities, trainings, services and news!**

Watch also last year's Rare Disease Day video **here**.



Watch the Video

EURORDIS 10th Blackpearl Award & Rare2030 Final Conference

FEBRUARY 23-24th, 2021

EURORDIS held the **Rare2030 Final Conference**

(23rd of February) and the **10th annual Black Pearl Award (24th of February), to which EJP RD was glad to be an official outreach partner**. The Rare2030 Final Conference gathered over 700+ participants. The **eight final recommendations** cover **diagnosis, treatment, care, research, data and European and national infrastructures sets out the roadmap for the next decade of rare disease policies**. Big round of applause for **EJP RD members Biruté Tumienė & Victoria Hedley who were awarded with the European Leadership** at the EURORDIS Black Pearl Awards 2021 alongside with Enrique Terol for their contribution to a stronger European cooperation in the #raredisease field through the ERNs.

They follow last year's awardee, EJP RD Coordinator Daria Julkowska.

Click HERE to sign up to Rare2030's newsletter to find out about their next steps.

Click HERE to see all the EURORDIS Black Pearl Awardees 2021.

IRDiRC: Two New Task Forces (last day to apply!)

If you are interested in becoming an **IRDiRC** Task Force member, two new task forces are opening:

- **"Shared Molecular Etiologies Underlying Multiple Rare Diseases"** aiming to address and document the existing challenges in adapting the basket trial approach used in molecularly targeted oncology clinical trials to drugs targeting shared molecular etiologies underlying multiple rare diseases.
- **"Integrating New Technologies for the Diagnosis of Rare Diseases"** aiming to identify the most clinically beneficial combination(s) of metabolomic and genomic tests coupled with artificial intelligence methodologies, which would then be prioritized for development of diagnostic standards.

The call for candidates will remain **open until today March 1st, 2021**.

Please send your CV to the Scientific Secretariat of IRDiRC at scisec-irdirc(a)ejprarediseases.org. For more information, click below.

Shared Molecular Etiologies Underlying Multiple Rare Diseases

Integrating New Technologies for the Diagnosis of Rare Diseases

EJP RD FUNDING OPPORTUNITIES

The ERN Research Training Workshops Call

OPEN UNTIL MARCH 7TH

If you are interested in **organizing a workshop, the ERN Research Training Workshops funding opportunity is open.**

COVID-19 outbreak:

For the applications received in the Research Workshop Calls in 2020, the workshops may be organized between 4 and 18 months after receiving a positive response to the submitted application.

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.** Moreover, the workshops will be aiming to provide a cross-ERN added value.

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** (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. Participants will be selected by the coordinator of the ERN managing institution and the workshop organizer based on pre-defined criteria.

Organizer's profile:

The applicant submitting workshop topics must fulfil one of the following conditions:

- Affiliated to any EJP RD beneficiary institution. The list of EJP RD beneficiary organizations can be found here
- Affiliated to an ERN Full Member. The list of full ERN members per country and per network can be found here
- 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.

To get more information and to apply, click the button below.

More information

OPENING SOON

The ERN Research Mobility Fellowship - opening on the 15th of March

We are glad to pre-announce the **upcoming opening of the call for Research Mobility Fellowships on the 15th of March**, 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.

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.

More information

NEXT COLLECTION DATES: MARCH 2ND & JUNE 1ST

Networking Support Scheme (NSS) Call

The aim of the NSS call is to encourage the sharing of knowledge of 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 in existing research networks. Events can be organised between 6 and 18 months after the application date. Online events are also possible.

Eligible applicants are health care professionals, researchers and patient advocacy organizations 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 to the number of participants to the event, however the maximum budget that can be requested is **€ 30,000 for a networking event**.

The collection date is March 2nd at 14:00 (CET). The next collection date will be June 1st. To get more information and to apply, click below.

More information

EJP RD IN EVENTS

If you want to hear a bit about EJP RD, join us at the following virtual events:

- **March 1st, 2021: 1st International Conference on Rare Diseases** which aspires 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. Join us at the session 4 "Advance Research For Rare Diseases" at 15:30 (GMT+2).
- **March 8th, 2021: 9th Presidency Conference: The why, what and how of tackling the Implementation Gap for healthcare in the EU**, 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.

OTHER NEWS

1st International Conference on Rare Diseases "Building a Pathway from Diagnosis to Access"

The 1st International Conference on Rare Diseases "Building a Pathway from Diagnosis to Access" will be held online on the 1st and 2nd of March. As the EU shapes its future regulations, strategies and access policies, this conference will serve as an opportunity to press the 'pause button' and take the time to co-create policy options today that can lead to a better patient journey in the future.

More Information

ANGELINI FOR FUTURE 2021 – Call for proposals

Angelini and INCIPIT, sharing the scope to promote high quality research on drugs, are now launching **the first edition of call for proposals – Angelini for Future 2021- to support independent research projects proposed by European investigators working in public or private non-profit healthcare organizations**. In this joint collaboration, Angelini allocates 300 K€ to fund **up to two research projects** either in late preclinical phase or in early clinical phase and INCIPIT provides the expertise to prepare the call, to manage the evaluation process of the research projects and to perform monitoring of the scientific results.

The aim of this initiative is **to support research investigating drug repurposing in rare diseases** or in conditions with high-unmet medical needs in CNS, targeting the paediatric population. Although there are over 7,000 rare diseases, only around 400 have licensed treatments. Drug repurposing is a good option for these diseases, which are affecting small populations, and has the potential to deliver new treatments with drugs already available but not yet specifically studied in these patients.

Budget: Total amount up to 300k€ will be allocated by Angelini Pharma to fund up to two projects.

Pre-proposal deadline: 31 March 2021

To get more information including the call text, the application form and more guidelines for the proposal, click below

More Information

FDA Rare Disease Day Virtual Public Meeting

The Food and Drug Administration will be holding a virtual public meeting on **Friday, March 5, 2021**, from 9:00 a.m. to 4:00 p.m. EST to highlight strategies to support rare disease product development. Please visit the public meeting page to register for the event.

To learn more about other activities relevant to rare diseases and listen to stories from the rare disease community and FDA staff working on rare diseases, please visit this **webpage**.

More information

EMA's draft toolbox guidance on scientific elements and regulatory tools

The European Medicines Agency (EMA) has published for public consultation a draft toolbox guidance on scientific elements and regulatory tools to support quality data packages for PRIME marketing authorisation applications.

The EMA launched the PRIME scheme to enhance support for the development of medicines that target an unmet medical need. This voluntary scheme is based on enhanced interaction and early dialogue with developers of promising medicines, to optimise development plans and speed up evaluation so these medicines can reach patients earlier. To be accepted for PRIME, a medicine has to show its potential to benefit patients with unmet medical needs based on early clinical data.

Experience to date has shown that applicants face challenges to complete quality and manufacturing development and data requirements during development of medicines for early access. This document provides guidance, in a 'toolbox approach', by summarising scientific elements and

regulatory tools, available in the existing EU regulatory framework, that can be applied to support the development and completion of Module 3 quality data packages in the preparation of marketing authorisation applications of designated PRIME medicinal products.

The scope of this document is on medicinal products that have received PRIME designation by the Committee for Medicinal Products for Human Use and includes medicinal products containing chemical, biological and/or biotechnologically derived substances and Advanced Therapy Medicinal Products.

More information

CAREERS

Job opportunities are available at EJP RD member institutions

- The EJP RD Coordination Team is looking for:
  - A Junior Project Manager
  - A Project Assistant
- The Fondation Maladies Rares is looking for their new **Research Administration Manager**
- The VASCERN coordination team is looking for their new **Project Manager**
- The Banque Nationale de Données Maladies Rares (national bank of rare diseases data) is looking for an **Interoperability Data Steward**
- ERN-EYE, Hôpitaux Universitaires de Strasbourg, France is looking for:
  - a **Medical Fellow**
  - a **Scientific Project Manager**
  - a **REDgistry Project Manager**
  - a **Medical Writer**

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