



External Newsletter n° 7: March 2020

Welcome to the EJP RD NEWSLETTER

Every month, latest news of the biggest European rare diseases initiative, bringing together funders, researchers, patients and clinicians!

Edito

In this edition we would like to do update you about measures taken by the EJP RD and other organizations to reorganize the activities under the covid-19 outbreak, since several events have been adjusted to the new situation. Specific means/tools are being put in place to facilitate your participation in different events.

We will also do a flashback to the intense Rare Disease Awareness Month EJP RD had! You will find some reminders about past and current EJP RD initiatives and some relevant highlights from rare diseases community.

Furthermore, in March EJP RD joined the [World Birth Defect Day](#) - 3 March - and the [International Women Day](#) - 8 March - social media campaigns raising awareness of these important scientific and social matters.

COVID-19 OUTBREAK

Due to the current covid-19 virus outbreak pandemic, our lives and activities have been changed or are changing right now. We are facing a new situation on a global scale and we are re-organizing our work, many events have been canceled, postponed or re-organised in online format in order to keep everybody safe and collaborate all together to overcome this crisis.

- The [RE\(ACT\) Congress - IRDiRC Conference](#) has been **postponed to 13 - 16 January 2021**, please save the date!
- The [EJP RD International Training Course on strategies to foster solutions of undiagnosed rare disease cases](#) will be **organized online**
- The [Eurordis European Conference on Rare Diseases & Orphan Products](#) (ECRD) will also **take place online**
- The [2nd TransplantChild Workshop on Translational Research](#) has been **postponed**

We will keep you posted about the situations.

EJP RD FLASHBACK ON THE RARE DISEASE MONTH



The **EJP RD** has celebrated the **Rare Disease Day (RDD)** by launching its first official [video](#), have you seen it? EJP RD has also a new [YouTube channel](#), more videos will come!

Furthermore, our contribution to the RDD was strengthened by a dedicated motto:

"We are #ejprd and we stand #together4rarediseases to celebrate the community on #rarediseaseday"

The 3 hashtags have been identified and used for the Twitter campaign, in particular the #together4rarediseases has been used 250 times. From now on it will be used in every **EJP RD twitter campaign in support of the rare disease community**. Thank you all for your help and support on this social media campaign.

Congratulations to Dr. Daria Julkowska, the EJP RD Coordinator, for having been the **EURORDIS Black Pearl awardee for the European Rare Disease Leadership**, the ceremony took place in Brussel on 18 February in live streaming. On the same day, Dr. Daria Julkowska and Prof. Alberto Pereira, Coordinator European Reference Network for Rare Endocrine Conditions (Endo-ERN) were among the invited speakers to the **"Reframe Rare" policy event at the EU Parliament** to discuss areas of action at EU level that can contribute to the integration of people living with a rare disease into society and improve their lives. And always on 29 February, the **podcast with DNA Radio** has been relaunched to mention the important work EJP RD is doing on rare diseases at European and international scale.

EJP RD HELPDESK

Don't forget the [EJP RD Helpdesk](#) is available for any inquiries and services provided by the EJP RD including Clinical Trials Support Office and Innovation Management Advice.



EJP RD TRAINING WORKSHOPS & CALLS

EJP RD TRAINING WORKSHOP



EJP RD TRAINING WORKSHOP
Biomedical Research Training Workshop Week.
Implementing Biomedical Research Projects: The Complete Workflow
from Concept, ELSI and Privacy Considerations to High-Quality Biobanking

<https://www.ejprd.europa.eu/en/index.php/biomedical-research-training-workshop/biomedical-research-training-workshop/>

- Use-cases will be used to address the key issues in biomedical research involving human subjects, human biological samples and associated medical data
- 2 modules on ethical, legal and regulatory contents and on procurement of samples and data in a workflow in a clinical context. The two modules can be registered separately.
- Up to 4 travel fellowships are available.
- participants from EU-13 countries and patient representatives are encouraged to apply
- The course and the registration are free of charge

Registration is OPEN, deadline is 2 April 2020.
11 – 15 May 2020. Medical University of Graz, Graz, Austria

Implementing Biomedical Research Projects: The Complete Workflow from Concept, ELSI and Privacy Considerations to High-Quality Biobanking

Registration for this [course](#) is now open, deadline is 2 April. The course is foreseen to take place in Graz, Austria on 11-15 May. The applicants will be informed in case of changes or moving the course to online version. This workshop **is aimed at biomedical researchers, medical professionals and biobank managers** who want to organize biomedical research projects on human biological samples. In two modules there will be several use-cases to address the key issues in biomedical research involving human subjects, human biological samples and associated medical data. There are #4 travel fellowships available, participants from lower income countries (EU-13, Turkey) and patient representatives are encouraged to apply. The workshop and registration are free of charge. Mitigation plan is already in place.

EJP RD OPEN CALLS



EJP RD RARE DISEASES RESEARCH (RDR) CHALLENGES CALL

- The call will be published soon
- New deadline: 15 April
- A € 1M budget to establish consortia on a B2B basis

#4 CHALLENGES:

- Development of a rare disease tool for measuring rare disease patient burden in public life
- Addressing the impact of rare diseases on the health of the population
- Characterization of rare diseases (RDR) study: Challenges in rare disease testing
- The impact of rare diseases on the quality of life of patients and their families

Rare Diseases Research (RDR) Challenge Call

The Rare Diseases Research Challenge Networking Event that took place on 3rd March in Paris has been a success despite the Covid-19 outbreak. The innovative [RDR Challenges Call](#) will be implemented to facilitate and fund collaboration between industry, academia, SMEs, and patient organizations to solve specific research challenges in rare diseases. **The call will be launched soon for a total budget of 1.5 Mio€** and it is still possible to establish consortia via a **B2B**



EJP RD INTERNAL CALL FOR PROPOSALS

CLINICAL TRIALS METHODOLOGY DEMONSTRATION PROJECTS


MAIN FEATURES:

- Aims to show the usability and capability of the innovative statistical methodologies for clinical trials in rare diseases
- Only teams from EJP RD beneficiary institutions are allowed
- 50% funding ratio from clinical trial data which is completed should be used

Deadline for the 1st stage (Letter of Interest): 31 March 2020
The duration of the projects can be up to 2 years

Clinical Trials Methodology Demonstration Projects Call

The [Clinical Trials Methodology Demonstration Projects Call](#) is still open for submission of the 1-pager letters of interest. Due to Covid-19 outbreak the deadline has been **postponed to 31 March 2020**. It aims to show the usability and capability of the innovative statistical methodologies for clinical trials in rare diseases, which have not been demonstrated on existing data for specific rare disease clinical trials yet. Please note that this call is



EJP RD NETWORKING SUPPORT SCHEME (NSS) CALL

- Knowledge sharing of knowledge on rare diseases and rare cancers
- Increase the participation of EJP RD countries
- Digital resources on health care professionals, researchers, and patient advocacy organizations from the EJP RD countries
- Network of experts in rare diseases
- Network of 45 digital resources from at least 4 different countries
- A maximum of 4.5 partners per institution is eligible

The Call to the NSS is open on a continuous basis.
First selection date for applications is June 1, 2020 at 1 pm CEST

Networking Support Scheme (NSS) Call

The [NSS call](#) provides funds (**up to 30 K€**) **for the organization of networking events**. It aims to encourage sharing of knowledge on rare diseases and rare cancers of health care professionals, researchers and patients. The participation of usually underrepresented countries in Europe in new and in existing research networks on rare disease(s) or rare cancer(s) is encouraged. **The call is open all year long and the next**

tool.

open for teams from ALL institutions that are beneficiaries of the EJP RD project including 24 ERNs.

collection date for evaluation is June 2, 2020 at 2 pm CEST.

OTHER CALLS & RELATED MEETING



Fund Generet Call

The [Fund Generet Call](#) supports research on rare diseases to better understand causes and mechanisms underpinning rare diseases and/or to turn fundamental discoveries into better prevention, diagnosis or treatment of patients. This annual award of **1.000.000 €** aims to catalyze the research agenda on rare diseases in **Belgium**. The amount will be paid in 2 phases of 500.000 euros. Deadline is 20 April 2020.



European
Reference
Networks

Preceptorship program on Treatable Lysosomal Storage Diseases (LSDs). 29 June - 1 July 2020. Salzburg, Austria.

As part of the capacity building and training activities, MetabERN in collaboration with the Clinical Research Center in Salzburg and the Brains for Brain Foundation, will organize this [course](#) aimed at hosting about 24 physicians from different countries who will share experiences and updates on the recent developments in Mucopolysaccharidoses (MPS). The meeting will also provide a comprehensive global forum for experts and participants to exchange ideas and present results on all aspects of MPSs. Participant fee and travel expenses (hotel room, train and flight economy class up to 500 Euro roundtrip) are covered and will be booked by CRCS GmbH. For registration and more info click [here](#).



European
Reference
Networks

3rd Neuromuscular Translation Summer School. 6 - 10 July 2020. Leiden, Netherlands.

The EURO-NMD and TREAT-NMD building on the past two summer schools, organize this [residential course](#) that will furnish attendees such as researchers and clinicians with a well-rounded knowledge of the translational pathway. Attendees will also benefit from the very latest knowledge available in the neuromuscular community as this course is delivered by key stakeholder in the field. To ensure a high-quality learning environment class sizes are kept deliberately small. It is recommended to apply promptly to ensure the maximum chance of successfully securing a place at their next Summer School. Registration is still open.

RELATED NEWS

In this section you will find the World Economic Forum white-paper on rare diseases, the joint statement of ICPeMed and ERA PeMed on personalized medicine in Europe, and some highlights from RDD actions from all over the world. Also, ERNs initiatives such as meetings and the updated list of forthcoming webinars are reported.

Global Data Access for Solving Rare Disease

The World Economic Forum (WEF) published the white paper on rare diseases [“Global Data Access for Solving Rare Disease”](#) that reviews the “known knowns and known unknowns” of a federated data system solution to the unmet needs of people living with rare diseases. This will help collaborating nations to understand whether federated data systems are **a best-fit solution to the global challenges inherent in rare-disease diagnosis and treatment plans.**

“We support a global approach to data sharing to improve the diagnosis and treatment of rare diseases,” said Yann Le Cam the Chief Executive Officer of EURORDIS - Rare Diseases Europe and Member of the Council of Rare Diseases International. “Our recent Rare Barometer survey on data sharing of 2,000+ rare disease patients, family members and carers shows that, nearly 100% of respondents are supportive of data-sharing initiatives to foster research and improve healthcare and diagnosis. In parallel, 80% of respondents want full or near to full control over the data they share. They want to decide who has access to their data, and how and why those people are using that data.”

#ERAPerMed

Towards the Implementation of Personalised Medicine in Europe - The Advantage of a European Partnership

In a joint statement [“Towards the Implementation of Personalised Medicine in Europe - The Advantage of a European Partnership”](#), ICPeMed and ERA PerMed underlined the need for establishing a European Partnership for Personalised Medicine. Already a lot has been achieved by large number of international, pan-European, national as well as regional activities and initiatives. But to further develop and foster promising personalised medicine approaches, there is a need of an even closer collaboration and effort which could be achieved by a European Partnership for Personalised Medicine under Horizon Europe.



"We can diagnose more than 4,000 rare diseases but there's still a long way to go"

IRDiRC has celebrated the Rare Disease Day with the [interview of its chair - Dr. Lucia Monaco](#), to the Horizon Magazine. Dr. Monaco on the article titled *'We can diagnose more than 4,000 rare diseases but there's still a long way to go'*, made clear that in RD no one will be left behind and IRDiRC is committed to it in order to **reduce the “diagnostic odyssey”** patients face and by taking care of the **“rare among the rare”** within the **indigenous populations in the developing countries.**

"You are not alone as we all are



one!”

« [You are not alone as we all are one!](#)” with this motto the Indian Organisation for Rare Diseases (IORD) celebrated the Rare Disease Day by organizing a ‘*Raising the Awareness: Prevention of Rare Diseases*’ conference in Hyderabad, India, where different stakeholders in the RD field took part. While inaugurating the IORD’s conference, Eatela Rajender, Minister of Medical & Health and Family Welfare, reiterated the government’s committed support to the cause of RD prevention by making available **prenatal diagnostic testing for early diagnosis**. IORD CEO and President Dr. Ramaiah Muthyala reminded that “the government can actively promote the manufacturing of orphan drugs for RD patients by providing **incentives to pharmaceutical companies**” and **accelerating drug repurposing**.



Jazz4Rare 2020

[Jazz4Rare 2020](#), a unique jazz concert took place on Rare Disease Day at the Emigration Museum in Gdynia, Poland. The concert was organized by **Foundation of Borys the Hero**, Tata Studio and Emigration Museum in Gdynia to **raise awareness of RD through the power of music**. The event gathered almost 300 people. As the organizers explained the choice of jazz music and location were carefully thought since rare diseases, jazz, and the emigration museum share the same **journey to the unknown**.

ERNs RELATED INFO



Webinars & Meetings

A list of upcoming [webinars](#) organized by the following ERNs is available: ERN-RND - ERN-EuroNMD - European Academy of Neurology (EAN), ERN-EpiCARE, ERKNet, EuroBloodNet

Info regarding ERKNet and EURO-NMD [annual meetings](#) scheduled in 2020 can be found here.

CAREER

Two [opening positions](#) are available at EJP RD member institutions:

- HELPDESK ASSISTANT, Institution of Myology. France
- TECHNOLOGY TRANSFER ASSOCIATE, Fondazione Telethon. Italy

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EJP RD is coordinated by the National Institute for Health and Medical Research (INSERM)



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