



European Joint Programme on Rare Diseases (EJP RD)

Call for Proposals 2020

Recommendations for full proposal submission and practical information

We would like to advise you to read the Guidelines, the Call text and the recommendations below carefully before completing the online and full proposal forms.

Important dates

16 th June 2020	Full proposal submission deadline
28 th July 2020	Deadline for rebuttals (~1 week)
November 2020	Notification of funding decision

Recommendations for full proposal

Writing of the full proposal

- Clarity:
 - o The lead hypothesis for the presented proposal which should not be a fishing expedition or data only gathering exercise,
 - o The aims should be addressed by the work plan,
 - o All work packages should fall within the scope of the call; applicants should remove any sections that do not relate to the objectives outlined in the call text,
 - o The abstract should reflect the content of the proposal. This abstract should be well written, as it will be published on the EJP RD website if the project is funded.

- Content of the Description
 - o The full proposal should show preliminary data that justify and support the proposed studies. It is not sufficient to state that the data exists, data must be included.
 - o The full proposal should give a strong rationale for the physiological relevance of the cell and/or animal models if they are used.
 - o Consortia should bring out the translational potential of the full proposal.



- Respect of the relevant European and international standards is mandatory (e.g. the EC Regulation (EC 2016/679) on the protection of natural persons with regard to the processing of personal data and on the free movement of such data; e.g. the ARRIVE Guidelines for animal research (See the Call text and the instructions in the full proposal form).
- The likely impact of the results to be gained in the project should be established and compelling.
- Existing clinical trials for the same target/pathways or the disease pathology should be cited in the reference section or in a table for easy reference by the reviewers. This adds considerably to the validation of the target.

Consortia composition

- Organisation
 - Coordination and organisation of the work performed by the different partners. Proposals in which there is clear synergy and the whole is greater than the sum of their parts are usually the most competitive.
- Additional expertise
 - Consortia should possess the expertise necessary to carry out the work plan. This may include, as required for the proposal, additional experts on medicinal chemistry PK/PD, pharmaceuticals, clinical pharmacology, toxicology, bioinformatics and data science, statistics and regulatory scientists, etc.
 - If industry partners or industrial connections are established, their actual involvement/participation should be clearly described.
- Patients and patient representatives
 - The consortia should clearly present the role and responsibilities of patient advocacy organisations (PAOs), how they will operate, at what levels and stages of the research, including detailed justifications of allocated resources. Consider using tools such as the INVOLVE cost calculator.
 - PAOs should be involved in establishment of patient registries and natural history development to ensure that appropriate clinical outcomes can be considered in preclinical studies.
 - If a project doesn't involve patients or PAOs, this absence should be clearly explained (i.e. why it is not relevant), to demonstrate that researchers have considered patient involvement but did not deem it relevant in the proposed study.
 - Patient advisory boards should be established where necessary to discuss: the research idea; possible outcomes; patient reported outcome measures; informed consent, acceptability of the research and its design to integrate the needs and priorities of patients within the studies and within the proposal; possible patient intervention in the project; review of the data collected.
 - Sources of where to find patient representatives and PAOs willing to be involved in research are: Orphanet, EURORDIS, European Reference Networks (ERNs) and EUPATI. Regarding the ERNs, all have integrated patient representatives (ePAG representatives) within their governing boards, disease-specific committees and transversal working groups.



Be specific about

- a. Methods and feasibility of data collection *
- b. Harmonization of information and structured data across project partners *
- c. Biostatistical and bioinformatics methodologies **
- d. Translational and Innovation management activities ***
- e. Contingency plans

Be specific about, which methods will be used to

- a. Repurposing or development of new therapies
- b. Development of predictive and pharmacodynamic (PD) biomarkers, including appropriate analytical methods
- c. Validation of therapies/biomarkers in a preclinical setting
- d. Mitigation of off-target effects based on preclinical data, if possible
- e. Determination of dosing routes and strategies using allometric scaling

Provide transparent justifications on precision or robustness of the expected results of the project.

Make good use of European Research Infrastructure if needed/necessary

- a. [EU-Openscreen](#) for chemistry services, high-capacity screening platforms etc...
- b. [BBMRI](#) for biobanking services
- c. [EATRIS](#) for translational services

** Data management and FAIR principles*

As the data management plan is mandatory in EJP RD Calls, we would like to give you some additional information. The full proposal should address the different aspects of "FAIR" data explicitly (see also <https://www.go-fair.org/fair-principles/> and <https://www.go-fair.org/technology/>):

Findable: How information on the data collected and the place where they are stored will be promoted (publications, consortium website), and when they be findable (requirement: ultimately 1 year after the EJP RD funding period of the project).

Accessible: Is there information on how to access the data in a technical and organisational, administrative way? What is the technical process? Is there a Data Use and Access Committee to review requests for data use from an external group? Is there a limit to the time allowed for this committee to provide the answers? When will secured deposition of data in (what type of) databases occur?

Interoperable: Describe the metadata, what information on the data will be provided? Are the experimental conditions described, are there quality measures of the data provided?

Reproducible/reusable: Which analysis scripts (software specific statistical code to allow for analysis replication) will be used to produce the main results of the project? Will the code eventually be made public (e.g. on GitHub, see <https://guides.github.com/activities/hello-world/>) and when?

IMPORTANT: For secured and FAIR deposition of data, several Infrastructures (like ELIXIR) or projects (like RD-Connect) are mentioned in the call text.

**** Recommendations for (bios-statistical and bio-informatics) methodology**

- The full proposal should provide a full description of biostatistical and bioinformatics methodologies for each proposed experiment type, and if necessary, for each work package.
- Sample sizes should be justified and where necessary power calculations should be presented.
- Be specific about which project partners are competent to conduct and take responsibility for statistics and bioinformatics. Investigators in charge of the statistical or bioinformatics analyses should be among the PIs listed in the project, and if this is not the case this should be justified (e.g. when these needs are minor components of the proposal).

***** Innovation support from partners in European Joint Programme on Rare Diseases**

Applicants are strongly recommended to make use of the [mentoring service](#), provided by EJP RD WP19, during the development of the full proposal. By using the service, provided free of charge and under full confidentiality, applicants will receive project-specific feedback from a panel of drug development, biomarker validation, methodological and regulatory experts tasked with optimising the translational feasibility of the study.

Practical information

Please read carefully the following important information. Your pre-proposal has been re-opened in PT Outline to allow for the submission of your full proposal. **Please upload not only your new full proposal form, but also review and adjust your previous input in all of the online PT Outline sections.** Changes have been made to **all** sections, so please read everything carefully and adjust your answers if necessary. Please pay extra attention to the explanation of the budget for the project coordinator *and* project partners.

- The call text and guidelines for applicants contain instructions for the full proposal submission and can be downloaded from the EJP RD website (<https://www.ejprarediseases.org/index.php/documents-jtc2020/>)
- The information given in the pre-proposal is binding. Thus, any changes between the pre- and full proposals, e.g. consortium composition or project objectives, must be communicated to the Joint Call Secretariat (EJP RD JTC 2020 Call secretariat) with detailed justification and will only be allowed by the Call Steering Committee (CSC) under exceptional circumstances.
- The composition of the consortium can only be changed in the context of the “widening concept” applied by EJP RD to encourage the inclusion of partners from underrepresented and undersubscribed countries. In the present call, consortia may include an additional partner from **Hungary, Lithuania, Luxembourg, Slovakia, Turkey, and the Tuscany Region of Italy**. (see item 4.2, page 6 in the document “Guidelines”). This widening of the consortium is NOT mandatory. Please note that:
 - The new eligible partner should bring an added value and expertise to the project.
 - The rule governing the maximum number of eligible partners within a consortium still applies (see table below).
 - The limit of two eligible partners per country per consortium still applies unless stated otherwise in the guidelines for applicants.



- The new partner will be funded by his/her own national funding agency, and must ensure their eligibility with their regional/national funding agency before submission.
- **If even one of the consortium partners is not eligible, then the full proposal will be rejected.**
- A single pdf document (digitally converted from Word, not a scanned file) containing all the information solicited in the full proposal template, and the adjustments for the full proposal in all PT Outline sections, must be uploaded in the electronic submission system not later than **June 16th 2020 at 14:00 CEST**.
- Only proposals using the full proposal template will be accepted.
- **Incomplete full proposals, proposals using a different format or exceeding length limitations of any sections will be rejected without further review.**
- In the literature reference list, you must use Vancouver Style (see: International Committee of Medical Journal Editors. Uniform Requirements for Manuscripts submitted to Biomedical Journals. NEJM 1997;336:309-15), and include PUBMED IDs.
- In the investigators' CV, provide also active links for each of the cited references.
- Please note that project coordinators will be provided with the opportunity to read and respond to the assessments of external reviewers to the full proposal (for details see section 6.3. Rebuttal stage in the "Call text"). You will have up to one week (final week of July 2020) for this optional response to the reviewers' comments (max 1 page).

Total number of partners requesting funding (including the project coordinator)
4
5
6
7 (only possible with inclusion of a partner as an early career researcher, or representative from underrepresented/undersubscribed countries)
8 (only possible with inclusion of two partners as early career researchers, or representatives from underrepresented/undersubscribed countries)

National Contact Persons for Hungary, Lithuania, Luxembourg, Slovakia, Turkey, and the Tuscany Region of Italy:

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For any questions please contact your regional/national funding agencies or the joint call secretariat: EJPRDcall@anr.fr

Contact points and further information on the EJP RD and this call is available at the EJP RD website: <http://www.ejprarediseases.org/>.