**European Joint Programme on Rare Diseases**

**(EJP RD)**

**Internal Call for Proposals**

**"** **Innovative Statistical Methodologies to Improve Rare Diseases Clinical Trials in Limited Populations”**

**Proposal application form**

**All fields must be completed using "Arial font, size 11" characters, single-spaced, margins of 1.27 cm.**

**Please note that incomplete full-proposals, proposals using a different format or exceeding length limitations of any section will be rejected without further review.**

All the information requested in this document must be compiled into one **single PDF-document** and **sent to the call secretariat** of the EJP RD by **March 03, 2021** at 5 pm CET, on the following email address

innovation.callsec@ejprarediseases.org

**All headings and all subpoints in section “Project description” need to be addressed and clearly indicated**

**Basic project data**

**Project title**

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| **Acronym (max. 20 characters)** |  |

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| --- | --- | --- |
| **Project duration** |  | **Months (max. 26 months)** |

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| --- | --- | --- |
| **Total requested funding (maximum 400,000 €)** |  | € |

**Keywords and medical domain**: please identify between three and seven keywords that represent the scientific content (medical domain, disease area, etc.), the methodological approach(es), tools.

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**Innovative statistical approaches dedicated to limited populations**: please identify which promising area(s) will be addressed in the application.

Develop a disease progression model from a natural history cohort or other observational studies.

Develop and validate a disease specific clinically meaningful outcome with special interest in PCOMs, or composite endpoints.

Develop a design and analysis procedure for a pharmacometric model and/or bridging study.

Develop a randomization-based model as an alternative analysis strategy and explore the level of evidence.

Other.

If the applicants are presenting a proposal covering **other areas** in the context of limited population clinical studies where methodological challenges remain, please describe them below (max. 200 characters including spaces)

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**Lay summary**: please give a comprehensive and readable summary of the primary aims and methods of the project. Please note that if your proposal is selected for funding this abstract could be used for communication purposes by the EJP RD (max. 1600 characters including spaces)

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**Consortium coordinator:**

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| **Family Name, first Name** |  |
| **Institution/Department** |  |
| **PIC Number** |  |
| **Department** |  |
| **Position** |  |
| **Address** |  |
| **Zip code, City Country** |  |
| **Phone + Fax** |  |
| **E-mail address** |  |
| **Type of entity** | Academia, Clinical or Public Health |
| **Type of entity (public/private-for-profit/private-non-for-profit)** |  |

**Project Partners (Principal Investigators):**

1. Additional research partners asking for funding (the total number of partners, including the coordinator indicated above should not exceed 6):

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| --- | --- | --- | --- | --- | --- |
| **No.** | **Zip code, City, Country** | **Project Partner (principal investigator)** | **Institution, Department, full affiliations (PIC number, address, phone + fax)** | **Email address** | **Type of entity Academia, Clinical or Public Health** |
| 1 |  |  |  |  |  |
| 2 |  |  |  |  |  |
| 3 |  |  |  |  |  |
| 4 |  |  |  |  |  |
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1. Contact information of the associated partners (not receiving funding)

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| **No.** | **Zip code, City, Country** | **Responsible person** | **Organisation, full affiliations (PIC Number, address, phone + fax)** | **Email address** | **Type of entity (academia, clinical/public health sector, private companies, patient advocacy organizations)** |
| 1 |  |  |  |  |  |
| 2 |  |  |  |  |  |
| n |  |  |  |  |  |

1. Contact information of the company owning the submitted data, if applicable

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| --- | --- | --- | --- | --- |
| **No.** | **Zip code, City, Country** | **Responsible person** | **Company name, full affiliations (PIC Number, address, phone + fax)** | **Email address** |
| 1 |  |  |  |  |
| 2 |  |  |  |  |
| n |  |  |  |  |

1. Contact information of the patient organisation(s) and patient representative(s) involved in the initial clinical trial study, if applicable

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| **No.** | **Zip code, City, Country** | **Responsible person** | **Organisation, full affiliations (PIC Number, address, phone + fax)** | **Email address** | **Type of entity (public / private-non-for-profit)** |
| 1 |  |  |  |  |  |
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1. Confirmation letter that the consent and/or authorization for data re-use is granted by the private owner, if applicable. The letter should be signed and dated by the data owner.

**Project description**

**1. Description of the project** (max. 6 pages)

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| State of the art (max. 2 pages): Detailed description of the methodological gap. This can be one of the topics listed in section “2. Aims of the call” or can be an additional topic. The gap description needs to summarise the state of the art of the selected methodology and its previous application to the selected clinical area as well as the methodological issues that have not previously been studied. The applicants should justify and explain their motivation to choose the specific unmet need, and present available material (e.g. “unable to investigate a research question in a specific RD due to non-existing methodology”), then present the available material (part of registry data, part of clinical trial data), explain the specific research question, how the existing methodology fails, and provide details and sources to prove the population size, registry details. |  |
| Description of methodological concepts that will be utilized to tackle the identified gap and build the innovative methodology (max. 1 page). |  |
| Research Project (max. 3 pages): The proposed work as well as the aims should be related to the unmet medical and patient related needs that is addressed to show the potential health impact.   * Aims * Relationship of the aims to the unmet medical need, patient need and methodological gap * Research questions * Methods * Expected results * Pathway to health impact * Methodological risks of the project |  |

**2. Description of the disease area** (max. 0.5 pages)

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**3. Impact** (max 1.5 pages)

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| Potential of the expected results on the future clinical, public health and/or other socio-economic health relevant applications, including patients’ needs. |  |
| Transferability: In case the application of the innovative statistical methodology is limited to a specific RD, the applicants are encouraged to show it values for other RD areas. Quality of the strategy for exploitation and/or dissemination of project results. |  |
| Added value of the proposed transnational collaboration. |  |

**4. Quality and efficiency of the implementation** (max 2 pages)

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| Coherence and effectiveness of the work plan (including Gantt chart, deliverables and milestones), appropriateness of the allocation of tasks, resources and time-frame to respective partners. |  |
| Feasibility of the project (adequateness of resources, access to patients or to patient’s data and /or material). |  |
| Complementarity of the participants within the consortium in the case multiple EJP RD partners perform a joint application. |  |
| Appropriateness of the management structures and procedures, including risk management, contingency plans and innovation management. |  |
| Quality of the proposed Data Management Strategy (how clinical data will be handled during and after the project; how data will be stored and processed; which methodology for protection of data will be applied, including transfers to non-EU countries; identification of Data Protection Officer). |  |

**5. Ethical standards**

**Ethical and legal issues**

*Please provide a short description of ethics and legal aspects in your proposal. For this, the following questions stemming from the H2020 Ethics self-assessment should be answered. If your answer is “Yes” please provide* ***additional information*** *listed in the H2020 Guidance “How to complete your ethics self-assessment” (see column “Information to be provided” of ethics issues checklist of each section; the guidance can be found at*

*http://ec.europa.eu/research/participants/data/ref/h2020/grants\_manual/hi/ethics/h2020\_hi\_ethics-self-assess\_en.pdf). Additionally, please mention* ***related tasks, responsible partners and documents to be provided for each question****. Please note that at this stage you do not need to submit supporting documents; you should only mention which documents are necessary to perform your research and whether they are already available or when will they become available.*

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| **PERSONAL DATA** | | | YES/NO | If yes, indicate page of description in proposal |
| **Does this research involve personal data collection and/or processing?** | | |  |  |
| **If YES:** | | - Does it involve the collection and/or processing of sensitive personal data *(e.g. health, sexual lifestyle, ethnicity, political opinion, religious or philosophical conviction)*? |  |  |
| - Does it involve processing of genetic information? |  |  |
| - Does it involve tracking or observation of participants? |  |  |
| **Does this research involve further processing of previously collected personal data (secondary use)?** | | |  |  |
| **Does your research involve publicly available data?** | | |  |  |
| **Is it planned to export personal data from the EU to non-EU countries?**  *(Specify the type of personal data and countries involved)* | | |  |  |
| **Is it planned to import personal data from non-EU countries into the EU?**  *(Specify the type of personal data and countries involved)* | | |  |  |
| **THIRD COUNTRIES** | | | YES/NO | Page |
| **In case non-EU countries are involved, do the research related activities undertaken in these countries raise potential ethics issues?**  *Specify the countries involved:* | | |  |  |
| **Is it planned to use local resources (e.g. animal and/or human tissue samples, genetic material, live animals, human remains, materials of historical value, endangered fauna or flora samples, etc.)?** | | |  |  |
| **Is it planned to import any material – including personal data – from non-EU countries into the EU?** | | |  |  |
| **If Yes**: | *Specify material and countries involved* | |  |  |
| **Is it planned to export any material – including personal data –from the EU to non-EU countries?** | | |  |  |
| **If Yes**: | *Specify material and countries involved* | |  |  |
| **In case this research involves** [**low and/or lower-middle income countries**](http://data.worldbank.org/about/country-classifications/country-and-lending-groups)**, are any benefit-sharing actions planned?** | | |  |  |
| **Could the situation in the country put the individuals taking part in the research at risk?** | | |  |  |
| **Section 10: MISUSE** | | | YES/NO | Page |
| **Does this research have the potential for misuse of research results?** | | |  |  |
| **Section 11: OTHER ETHICS ISSUES** | | | YES/NO | Page |
| **Are there any other ethics issues that should be taken into consideration?**  *Please specify:* | | |  |  |

**6. Data management Strategy: Describe management of clinical patient data required for the project** (max 2 pages)

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| Include information on the handling of the clinical data during and after the end of the project |  |
| What data will be transferred, stored, processed and/or re-used? |  |
| Which methodology and standard will be applied for the protection of the data? |  |
| How data will be curated and preserved (including after the end of the project)? |  |

**7. Added value of the proposed transnational collaboration** (max. 1 pages)

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**8. Budget plan of the project: sum of months 1-26.**

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| **Double click on the budget table to open it in Excel. Cells in orange are automatically calculated** |
| 1 Please detail number of person months (PM), qualification (Cl: Clinician, Si: scientist, e.g. postdoc; PhD: PhD-student; N: non-scientist, e.g. technician; Ot: other) and € requested. Please use one cell per person to provide this information. |
| 2 Travel expenses should include the participation of the coordinators and/or national partner leaders at an intermediate status symposium to present the results of their projects (organized by the Joint Call Secretariat). |
| 3 e.g. provisions, licensing fees |
| 4 Indirect costs: 25% of the eligible direct costs (excluding subcontracting). |
| 5 Each partners is obliged to provide at least 30% of in kind contribution to the total cost of the project. In case if the maximum funding of 400 000 € is requested, the total cost of the project (eligible direct costs, including in kind contribution) should be of at least 572 000 €.  The in kind contribution is usually accounted through the costs of permanent personnel working on the project. The verification of the justification if in kind conrtribution of different partners will be made during yearly financial reporting of the EJP RD. Please note that the reimbursement of 70% is always dependent on the total (100%) costs of the project and thus if a partner is not able to justify the needed minimum of 30% contribution, the budget will be descreased accordingly. In case of doubts on how to complete this table please contact the Call Secretariat. |

**9. Brief CVs for each participating partner** (with a list of up to five relevant publications within the last five years demonstrating the competence to carry out the research project. (max. 1 page per partner)

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**10. Signature of the coordinator**

**Coordinator:**

Name, Institution

Place, Date, Signature