The Patients as Partners in European Research Programmes on Rare Diseases

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Preparatory process

A future European Partnership on Rare Diseases proposed for Horizon Europe Work Programme 2023-24 (Member States (MS) asked in 2019: high consensus)

Publication of the "Concept Paper" (well developed) in February 2022 on EC website:
https://ec.europa.eu/info/files/european-partnership-rare-diseases_en

Next step: the development of the **Scientific Research & Innovation Agenda (SRIA)**

EJP RD

Initial members identification _ SRIA TF

Active experts involved in the Concept paper development representing (**but not limited to**):

- **Various fields of activity**
  - (preclinical, translational and clinical research; drug development and diagnostics innovation; biostatistics; data science; regulatory science; research funding);
- **Different types of stakeholders**
  - (research organisation/institutions; hospitals/university hospitals; EU research infrastructure**; patients' organisations**; foundations; funding bodies; regulatory & health technology assessment bodies, Member States representatives, European Commission);
- **Relevant programmes, initiatives and networks**
  - (**EJP RD**; Solve-RD**; ERNs**; Innovative Health Initiative; European Health Data space; DARWIN EU; CSA STARS; C-PATH).

European Joint Programme on Rare Diseases

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Call for Proposals 2023

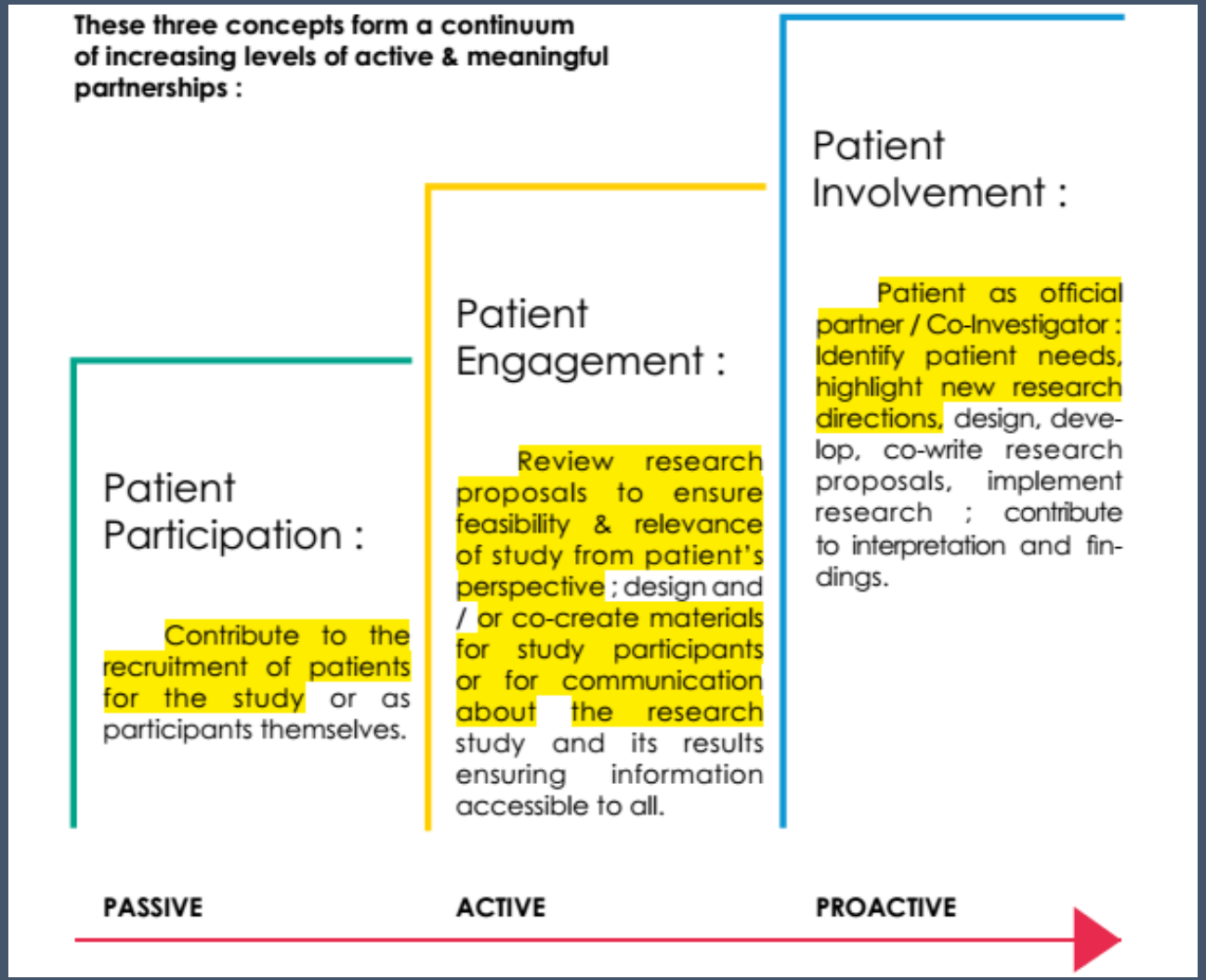
"Natural History Studies addressing unmet needs in Rare Diseases"

Submission deadline for pre-proposals:
February 15th, 2023; 2 p.m. (CET)

Pre-proposal application form

Within the Joint Translational Calls (JTC) of the European Joint Programme on Rare Diseases (EJPRD), patient organisations are eligible to apply as fundable partners of a consortium submitting a research proposal.

**** The EJP RD's Resource Finder provides scientific partners with a vast number of existing research data and services grouped into categories and represented as 11 `nodes´ in the mindmap.*



1 BENEFITS FOR PATIENT PARTNERSHIPS

STRONGER FUNDING APPLICATIONS

Applications written by/with patients clearly illustrate patient benefits, study importance to all evaluation panel members

EXPANDED OUTREACH & IMPROVED COMMUNICATION

Patients can assist in the creation of communications, translating information into accessible language help with a better understanding of patient needs

GREATER RELEVANCE

Involving patients ensures that the research results translate into concrete benefits and address patients needs.

MOTIVATION & FOCUS

Hearing directly from people living with a RD can provide researchers with meaning and context

NEW IDEAS

Talking to groups of patients, particularly in the early stages of research, can identify novel challenges and ideas.

GREATER IMPACT

Patient partners are excellent advocates to generate public interest and impact, raise awareness of the research needs for the benefits of rare disease patients,

BUILDING REACH

Patients and/or patient representatives can facilitate the creation of research consortia by bringing partners together.

2

AVOIDING COMMON PITFALLS

Patient engagement / involvement is not applicable or relevant to the proposed project. "

" We could not find a relevant organisation / a relevant organisation does not exist/the disease is too rare."

There is rarely an example in which patients cannot be involved at all e.g. they can be involved in the dissemination activities at the very minimum. It is also important to think carefully about whether such dissemination activities are the only activities in which patients can be meaningfully engaged.

" Patient organisations will recruit patients as donors for the biobank . "

Not enough explanation is given as to how this will be achieved. Who ? How ? When ? Was the patient organisation involved in developing the recruitment strategy ?

" Patients organisations have been involved in the design of the study "

It is important to explain how this has been achieved and what has improved in the design of the study as a result of the patient involvement.
Any specific roles and responsibilities need to be discussed and agreed between the researchers and the patient organisations) and to be detailed before submitting the proposal.

3. EXAMPLES OF TYPES OF PARTNERSHIPS

EVALUATING IMPACT

- Collaborate with researchers to evaluate the research process.
- Evaluate the impact to the involvement on the research.
- Patients / public reflect on their role / what they learned

IMPLEMENTING

- Increase likelihood of results being implemented due to patient support / lobbying.
- Assessment of value.
- Analysis of benefit/ risk.

IDENTIFYING & PRIORITISING

- Patients / stakeholders identify relevant research topics through consultation.

DISSEMINATING

- Advise on avenues for dissemination.
- Jointly present research findings.
- Contribution to publications.
- Draft lay summaries of results.
- Collaborate in publishing results e.g. via charities / patient groups.

RARE INVISIBLE DISEASES AND SCHOOLING OF CHILDREN : ENHANCING THE SCHOOL INCLUSION OF CHILDREN WITH 3 DIFFERENT RARE DISEASES

PATIENT INVOLVEMENT

Study design :

The 3 Patient Organisations partners participate in the implementation of experimental scenarios to assess teachers' training.

Project activity :

The 3 Patient Organisations participate in the set up of training and information materials.

PATIENT ENGAGEMENT

Communication :

The 3 Patient Organisations partners design & carry out awareness raising campaigns with their members through various communication activities.

The Patient Organisations partners also support dissemination of the project results to social and economic experts.

- The aim of the NSS call is to **encourage knowledge-sharing between 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 existing research networks.
- Eligible applicants are **health care professionals, researchers, and patient advocacy organisations 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 on the number of participants per event; however, the maximum budget that can be requested is **€30,000** per networking event.

EJP RD

Filling the format

1.a. Project title:

1.b. Project acronym:

The application is:

- ☐ a new proposal
- ☐ a resubmission from a previous EJP RD call JTC 2019, JTC 2020, JTC 2021, JTC2022
- ☐ a proposal asking for an extension of a previously funded E-Rare or EJP RD project

If so, please state the acronym of the project:

2. Consortium coordinator:

Last Name, First Name	
ID (ORDIC or otherwise)	
Institution/Department	
PIC number of the institution (EC Participant Identification Code)	
Position	
Address	
Zip code, City Country	
Phone + Fax	
E-mail address	
Type of entity (Academia, Clinical or Public Health or SME)	
Type of entity (public/private-for-profit/private-non-for-profit)	
Early Career Researcher (yes/no)	

3. Project Partners:

3a. Research partners asking for funding:

No	Zip code, City, Country	Research Partner (principal investigator)	ID (ORDIC or otherwise)	Institution, Department, full affiliations (address, phone + fax)	PIC number of the institution (EC Participant Identification Code)	Email address	Early Career Researcher (yes/no)	Type of entity Academia, Clinical or Public Health, SME and Industry	Type of entity (public/private-for-profit/private-non-for-profit)
Coo									
P2									
P3									
P4									
P5									
P6									
P7		(7th partner is an early career researcher, or from usually underrepresented countries)							
P8		(8th partner is an early career researcher, or from usually underrepresented countries)							

3b. Patient advocacy organisation asking funding from their national/regional funding agency

No.	Zip code, City, Country	Responsible person	Organisation, full affiliations (address, phone + fax)	Email address	Type of entity (public / private-non-for-profit)
1					
2					
xx					

Each Patient Advocacy Organisation from a participating country, requesting funding at its national/regional funding agency (if eligible) should complete and sign the letter in Annex 1 and send it by email to the contact person of this funding agency before

3c. Collaborators (not funded): (PAOS not asking for funding may be collaborators)

No.	Zip code, City, Country	Principal investigator	Institution, Department, full affiliations (address, phone + fax)	Email address	Early Career Researcher (yes/no)	Type of entity Academia, Clinical or Public Health, SME or Industry	Type of entity (public / private-for-profit / private-non-for-profit)
1							
2							
xx							

4. Duration of the project (max. 36 months)		**Months**
5. Total requested funding in application		**€**

6. Keywords
Please identify between three and seven keywords that represent the scientific content (medical domain, disease, etc.), approach (es), tools (animal models, OMICS, etc.) methodology

1	
2	
3	
4	
5	
6	
7	

7. Lay summary
(max. 1600 characters including spaces) *Please note that if your proposal is selected for full proposal submission, this abstract may be communicated to researchers from underrepresented or undersubscribed countries as part of the widening process (see section 5.2 of Guidelines for Applicants for details).*

8. Description of the project

Description of the working plan including:

- *Need for research rationale: description of the unmet need that is addressed by the proposed work, rationale of the rare diseases chosen.
- *Present state of the art, recent insight from literature.
- **Preliminary results obtained by the consortium members**

- **Hypothesis and Objectives.** Main and secondary hypothesis. Main hypothesis (es) for the proposed research plan and sample size calculation (if applicable).

- **Workplan & methodology** *(highlighting feasibility)*

- Research strategy
- Methodologies - justification and presentation
- Enrollment: study location(s), total number of corresponding patients followed by partners and collaborators of the project.
- Statistical power: appropriate statistical methods description, name and affiliation of the responsible biostatistics' expert.

Workplan

This project is divided into N work packages (WPs),

WP1: Natural history of RD (RD),
WP2: Medical Treatment (MT),
WP3: Surgical Treatment (ST),
WP4: Patient experience (PE)
WP5: Management, dissemination, and exploitation (MD).
The time plan is depicted in the Gantt chart (Point 9):

Work package description, one by one

Work package number **WP1**
Work package title Natural history of RD (RD)
Objectives: are developed in tasks within each WP

- Age of Onset and development in life
- International scales to measure severity
- How the disease was treated before.

Delivery of a technical report containing the mean age, scales internationally accepted, trends in management.

For clinical trials, prospective, retrospective, observational, interventional

Inclusion/exclusion criteria	
Main outcomes to be analysed	
Anonymisation/pseudonymisation of data and statistical details	
Number of participants calculation (if applicable): description, justification, expected response rate, duration in months	

- **Impact**
- Results: description of expected results and their implementation
- Impact : description of the potential impact of the expected results on the addressed unmet need
- Benefits: description of individual and collective benefits that could be expected
- **Added values of the consortium**
- Competence, experience and complementarity of all the participants, benefit of transnational collaboration
- **PAOs engagement/involvement**
  - role of PAOs and patient representatives within the consortium (active and meaningful participation)

9. Diagram of the work plan Gantt's diagram

Lead	Month				12				24				36
Partner 1	Task 1.1			M1.1									
	Task 1.2									M1.2			
	Task 1.3												D1.3
	Task 1.4												D1.4
Partner 2	Task 2.1				D2.1								
	Task 2.2				M2.1					D2.2			
	Task 2.3									D2.3			
	Task 2.4												D2.4
Partner 3	Task 3.1								M3.1				
	Task 3.2									M3.2			
	Task 3.3												D3.3
Partner 4	Task 4.1				D4.1								
	Task 4.2								M4.1				M4.2/D4.2
Partner 1	Task 5.1		D5.1		D5.2								
	Task 5.2								D5.3/M5.1				
	Task 5.3				D5.4								D5.4

10. In addition,
two more sections can be added to the pre-proposal (*optional*):

- a page of results-related diagrams, figures, etc. to support the work plan description
- a list of literature references

BUDGET

	Project coordinator[4]	Partner 2	Partner 3	Partner 4	Partner 5	Partner 6	Partner 7[5]	Partner 8[5]
Name (principal investigator)								
Country								
Funding organization								
Personnel €								
Consumables €								
Equipment €								
Travel €[1]								
Other direct costs €[2]								
Overheads €[3]								
Total requested budget €								

Brief CV

Name, First Name
Country
Phone, email
URL for Website

PROFESSIONAL EXPERIENCE & EDUCATION

Dates	Institution	Function

RESEARCH FOCUS (4 lines max

SCIENTIFIC PRODUCTIONS (grants, awards….)

VALORISATION (patents, spin-offs, tools/databases development….)

FIVE SELECTED PUBLICATIONS (five most relevant within last five years)
Number of publications and citations :

CHECKLIST FOR SELF-EVALUATION OF APPLICANTS. GUIDANCE FOR RESEARCHERS, FUNDERS AND PATIENT REPRESENTATIVES

- Have discussions between researchers and patient representatives before identifying the research objectives, and writing the proposal
- Have you described how the patients/patient representatives were selected ?
- Has the input of patients/patient representatives been integrated in the development of the proposed research project ? Describe changes and improvements as a result of this input ?
- Clear roles and responsibilities must be assigned to the patients / patient representatives in the project
- Have the available resources of respective partners been maximised to the Benefit of the research project (e.g. registries, know-how, networks, communication channels) ?
- Have the approaches through which the patients / patient representatives will be engaged / involved / participate in the project been described (e.g. focus groups, interviews, surveys etc.) ?
- Has a process been included to ensure two-way communication between the partners throughout the life of the project ?
- Are patient representatives included in the governance of the research project e.g. as steering committee member, leader or co-leader of a work package ?
- Are follow up reports (e.g. including feedback from patients / patient representatives) planned within the deliverables of the project to assess the actual Patient Partnership once the project has started ?
- Are there other specifi cdeliverables relating to the Patient Partnership activities described (e.g. publication of guidelines, analysis of a focus group and/or a survey data, development of a video etc...) ?
- Has a budget been allocated, and costs estimated and justified in line with the proposed specific activities for the Patient Partnership ?
- Has the monitoring of the Patient Partnership been integrated within the consortium management plan ?
- Have you planned to include the impact of your Patient Partnership on your study in your publications ?

THANK YOU

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EUROPEAN JOINT PROGRAMME RARE DISEASES

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