EJP Rare Diseases Joint Transnational Call "Natural History Studies addressing unmet needs in Rare Diseases" (2023)

Guidance Notes

Guidance Notes

Key Dates & Times	
Application Open	12 December 2023
Application Closing Date	15 February 2023 @13:00

Applications must be completed and submitted through the electronic proposal system (https://ptoutline.eu/app/ejprd23), and this system will close automatically at the stated deadline and timeline listed above.

This document provides additional guidance to researchers based in Ireland applying to this call as part of a transnational consortium. A summary of the call is presented herein along with eligibility criteria for Irish applicants requesting HRB funding.

This document must be read in conjunction with the call documents provided on the main EJP Rare Diseases call webpage, and the HRB FAQ for this call on the HRB call website.

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1 Introduction

The Health Research Board (HRB) Strategy (2021-2025)[1] sets out a lead role of the HRB to invest in research that delivers value for health, the health system, society, and the economy, as well as to foster and enhance European and international coordination, collaboration and engagement.

The specificities of rare diseases – limited number of patients per disease, scarcity of relevant knowledge and expertise, and fragmentation of research – single them out as a distinctive domain of very high European added-value. Rare diseases are therefore a prime example of a research area that necessitates collaboration/coordination on a transnational scale.

There are at least 7,000 distinct rare diseases, the great majority being of genetic origin. Although individually rare, taken together rare diseases affect at least 26–30 million people in Europe. Moreover, they represent a major issue in healthcare; a large number of these diseases have an early or very early onset and/or lead to a significant decrease in life expectancy. Moreover, most of them cause chronic illnesses with a large impact on quality of life and the health care system.

Therefore, research on rare diseases is needed to provide knowledge for prevention, diagnosis, better care and everyday life improvement for patients. Yet, research is hampered by lack of resources at several levels: (1) Few scientists work on any given specific disease; (2) There are few patients per disease and they are scattered over large geographic areas, causing difficulties in assembling the necessary cohorts; (3) Existing databases and bio-material collections are usually local, small, and not accessible or standardized; (4) The complex clinical phenotypes of these diseases require interdisciplinary cooperation to improve research and treatment.

The **European Joint Programme on Rare Diseases (EJP RD)** has successfully implemented four Joint Transnational Calls since 2019 to further help in coordinating the research efforts of European, Associated and non-European countries in the field of rare diseases and implement the objectives of the International Rare Disease Research Consortium (IRDiRC). For more information, visit the IRDiRC website.

2 Aim and Objectives

The aim of the call is to enable scientists in different countries to build an effective collaboration on a common interdisciplinary research project based on complementarities and sharing of expertise, with the expected impact being to use the results in the future for the benefit of patients.

The objective is to conduct efficient, innovative and high-quality natural history studies which will facilitate understanding of the disease's or group of disorders' progression throughout the lifespan of a patient. The goal of these studies is to collect and analyze comprehensive patient data to define targets for future therapies, taking into consideration innovation, safety, and efficacy.

3 Scope of Call

Projects shall focus on **a group of rare diseases or a single rare disease following the European definition**; i.e. a disease affecting not more than **five in 10,000 persons** in the European Community, EC associated states, and Canada. Applicants are encouraged to assemble groups of rare diseases

[1] https://www.hrb.ie/strategy-2025/

based on relevant criteria and commonalities if these leverages add value in sharing resources or expertise.

3.1 Topics list

Research proposals should cover **at least one** of the following areas**:**

- Estimation of disease prevalence;
- Identification of biomarkers/companions for the diagnosis/prognosis of a RD;
- Identification of biomarkers/indicators/predictors of a rare disease or group of disorders (e.g. having the same aetiology) onset/progression (including collection of genetic, physiological, environmental data or variables etc);
- Identification of relevant endpoints for future studies that include potential biomarkers, querying patient-reported outcomes (PROs) and quality-of-life measures;
- Identification of biomarkers/variables for therapeutic approaches (pharmacology, drug repurposing, gene therapy, RNA therapy, cell therapy, medical devices etc).

It is possible to use cellular and animal models for validation of the new diagnostic approaches in the subtopics listed above where relevant.

Furthermore, additional elements need to be considered in the application:

- Strategies and timelines for patient recruitment, retention, assessment, and analysis must be included. Data supporting the proposed recruitment numbers is mandatory. The study design and objectives should take into consideration what information would be needed regarding the rare disease population in order to pursue clinical trials or other healthcare related studies in the specific rare disease/group of rare diseases studied. There must always be clear research questions that are addressed in the study/registry. Clear plans for sustainability of the resources must be described. Consideration of common data elements as outlined in the recent publication “Set of Common Data Elements for RD Registration”;
- Integration of appropriate bioinformatics and statistical skills should constitute, whenever justified, an integral part of the proposal, and the relevant personnel should be clearly specified;
- Proposals are expected to consider how sex and/or gender might shape research activities. Applicants are encouraged to visit European Commission, Directorate-General for Research and Innovation, Horizon Europe, gender equality, and CIHR's Sex, Gender and Health Research resource page for more information on key considerations for the appropriate integration of sex and gender in their proposal. See also HRB’s guidance in the Appendices below;
- Provision of harmonized/standardized data, collected using innovative means (AI, simulations, modelling…) is highly encouraged. Modelling and simulation allow for organisation of diverse data sets, optimisation of the possible future product based on individual physiology and genetics. Innovative methods for data collection as well as data dissemination that can serve as a model for future studies are highly encouraged;
- The new research data resulting from the project should be permissible according to the Findable, Accessible, Interoperable and Reusable (FAIR) for humans and machines, conforming to FAIR principles, and deposited and shared, according to the national/regional rules of the

countries involved. It is strongly advised to make data accessible through RD-Connect or Elixir. It is strongly advised to make use of IRDiRC recognized resources and European and global resources for making data, tools, workflows, data models, (e.g. see the EJP RD resource map, and the ELIXIR Research Data Management Toolkit). To make research data FAIR, **a data management strategy for the proposed full project is mandatory in the full proposal stage**. It should contain a timeline, budget, and designated consortium partners to comprise a FAIR data steward team. If FAIR expertise is not present in the project consortium, then a FAIR partner can be added later, possibly identified via the EJP RD helpdesk. The FAIR expert group guides the FAIR publication process, including alignment with the EJP RD Virtual Platform specifications, and helps identify goals for exploiting FAIR data. Research including Indigenous people should also adhere to the CARE (Collective benefits, Authority to control, Responsibility, Ethics) Principles for Indigenous Date Governance. HRB will also ask for a data management plan (DMP) at national level after granting of the project;

- Insights into defining genotype/phenotype correlations, identifying appropriate subpopulations or stratification of patients for a trial are of importance to characterise newly discovered disease, a new subgroup of patients etc.;
- Inclusion of patient and caregiver perspectives from the RD community is strongly encouraged. Patients living with a RD or a family member who cares for them, have experiences and knowledge that can contribute to generating data about the natural progression of the disease. Patients should be involved in all steps of planning and implementation of the study;
- For the small group of well-characterized rare diseases with approved treatments or improved standard of care, prospective studies can define the altered disease progression under the current medical setting. Thereby, studies collecting data regarding adverse events and providing reference/data for development of a more effective or safer treatment can be considered for complementing the natural history study.

Applicants should refer to HRB's Appendices below for further guidance and resources on the above.

3.2 Excluded approaches and topics

The following approaches and topics will be excluded from the scope of the JTC2023:

- Interventional clinical trials to prove efficacy of drugs, treatments, surgical procedures, medical procedures. This also includes studies comparing efficacy, e.g. two surgical techniques or therapies. Projects whose main objective is the implementation of a clinical phase IV pharmacovigilance study cannot be funded either. Note that HRB will not fund clinical trials at any phase.
- Studies on the exclusive testing of the safety of medical devices and drugs.
- Development of new therapies as covered in EJP RD JTC 2020.
- Development of new analytic tools and pathways to accelerate diagnosis and facilitate diagnostic monitoring of rare diseases as covered in EJP RD JTC 2022.
- Projects focusing only on rare neurodegenerative diseases which are within the main focus of the Joint Programming Initiative on Neurodegenerative Disease Research (JPND). These are:

Alzheimer's disease and other dementias; Parkinson's disease (PD) and PD-related disorders; Prion diseases; Motor Neuron Diseases; Huntington's disease; Spinal Muscular Atrophy and dominant forms of Spinocerebellar Ataxia. Interested researchers should refer to the relevant JPND calls. However, childhood dementias/neurodegenerative diseases are not excluded.

- Rare infectious diseases, rare cancers and rare adverse drug events in treatments of common diseases. Rare diseases with a predisposition to cancer are not excluded.

In addition to the exclusions above, Irish Partner(s) are not eligible for HRB funding for:

- Proposals seeking to evaluate a pilot or feasibility study.[2]
- Proposals seeking to evaluate a definitive intervention.[3]
- Proposals involving basic biomedical research.
- Research intended to create human embryos solely for the purposes of research or for the purposes of stem cell procurement, including by means of somatic cell nuclear transfer.

Where an application is outside the scope of the scheme, the application will be deemed ineligible and will not be accepted for review.

4 Funding Available, Duration and Start Date

The HRB plans to commit up to **€370,000** (inclusive of overheads) to the EJP Rare Diseases (EJP RD) awards. Additional funding of up to €130,000 will be made available for coordination activities (excludes equipment and consumables), bringing the total maximum funding to **€500,000 for coordinators**. Quality permitting, a minimum of one award will be funded. Awards will have a duration of 36 months.

The award will offer research related costs for:

a) Personnel

[2] **Feasibility studies:** For the purposes of this scheme, we adopt the concept of feasibility as described by Eldridge et al (2016). Eldridge describes 'feasibility' as an overarching concept, within which we distinguish between three distinct types of studies (1) randomised pilot studies (2) non-randomised pilot studies and (3) feasibility studies that are not pilot studies. This call is open to all types of stand-alone feasibility studies conducted in preparation for a future definitive trial of an intervention.

[3] **Definitive interventions: Intervention studies** of any appropriate design, including randomised controlled trials and non-randomised trials, are designed to assess the efficacy, effectiveness, cost and broad impact of a therapy or intervention. Interventions can be on individual human participants (patients or healthy volunteers), or alternatively could involve an intervention on an element of the health system, e.g. testing an intervention on healthcare setting, healthcare pathway, with the aim being to improve how healthcare is delivered. **Definitive interventions** should have potential for immediate use for decision makers in everyday clinical practice or policy, must have supporting feasibility information, and must have a basis in evidence that has been synthesised systematically.

i. Salary-related costs in line with the IUA most recent scale for funded personnel

ii. Stipends and fees (EU rate only)

iii. Early Career Researchers salary related costs for a maximum of 0.5 FTE protected time for research funded by HRB for up to three years

b) Small equipment costs (not expected to exceed €10k)

c) Direct running costs (including travel, or mobility costs)

d) FAIR data management costs: Data stewardship costs (e.g. service/fees from data steward, access to secondary data, costs of making data FAIR, etc). Please refer to the Appendix I for additional guidance on FAIR data management costings.

e) Dissemination and knowledge exchange activities (including dissemination-related travel)

f) Overheads contribution

Funding available is inclusive of overheads and pension contributions.

Note: The HRB's EJP RD award will not fund the salary and related costs of tenured academic staff within research institutions (including buy-out from teaching time etc.).

The budget requested and the award duration **must** reflect the scale and nature of the proposed research, and reviewers will thoroughly assess the level of funds and timeframe requested when reviewing the application.

5 Eligibility Criteria

Please also refer to Section 3 for excluded approaches and topics.

This call is not open for Host Institutions from Northern Ireland.

Where an applicant fails to meet the eligibility criteria, the application will be deemed ineligible and will not be accepted for review. The Joint Call Secretariat will contact the consortium in the event that this situation arises.

5.1 Consortium Composition

The use of the matchmaking tool is strongly encouraged to build multidisciplinary research projects: https://connect.eventtia.com/en/public/events/jtc2023matchmaking/registration/attendees.

- Only transnational projects will be funded.
- Each consortium submitting a proposal must involve four to six eligible principal investigator (PI) partners (referred to as partners below) from at least four different participating countries (see list in section 10 of the core call guidance). In specific cases this can be increased to eight partners, as follows:

  a) The inclusion of partners from participating countries usually underrepresented in projects (Hungary, Lithuania, Poland, Slovakia, Sweden and Türkiye).

  b) The inclusion of Early Career Researchers as full partners (see section 5.6).

- No more than two eligible partners from the same country can be present in each consortium; further national/regional limits may apply, see "Guidelines for Applicants". PAOs requesting funding do not count toward the total.
- A single PI will represent each project partner.

5.1.1 Coordinator

Each transnational proposal must nominate a project consortium coordinator from the project partner PIs. The coordinator must be an eligible project partner from an EJP RD JTC 2023 funding country/region. The project coordinator will represent the consortium externally, to the Joint Call Secretariat (JCS) and to the Call Steering Committee (CSC), and will be responsible for its internal scientific management (such as controlling, reporting, and intellectual property rights issues). This workload should be considered in the estimation of the budget of the coordinator – the HRB will make available additional funds of up to €130,000 for Irish coordinators for this purpose.

5.1.2 Lead Applicants based in Ireland

The following will apply to partners seeking HRB funding – i.e., Lead Applicants based in Ireland.[4]

The **Lead Applicant** will serve as the primary point of contact for the HRB during the review process and on the award, if successful. The Lead Applicant will be responsible for the scientific and technical direction of the Irish research programme. They have primary fiduciary responsibility and accountability for carrying out the research within the funding limits awarded and in accordance with the terms and conditions of the HRB.

Early Career Researchers (ECRs) are encouraged to join consortia as full research partners. ECRs based in Ireland should refer to the eligibility criteria in section 5.1.3 rather than the below.

The Irish Lead Applicant **must**:

Hold a post (permanent or a contract that covers the duration of the award) in a HRB recognised Host Institution in the Republic of Ireland (the "Host Institution") as an independent investigator. For clinicians, an adjunct position in a HRB recognised Host Institution is acceptable.

OR

Be an individual who will be recognised by the Host Institution upon receipt of an award as an independent investigator who will have a dedicated office and research space for the duration of award, for which they will be fully responsible. The Irish Lead Applicant does not necessarily need to be employed by the Host Institution at the time of the application submission.

They **must** show evidence of achievement as an independent researcher in their chosen research field by:

Demonstrating a record of research output, with at least three publications of original research in peer reviewed journals. Where appropriate, they should also provide evidence of other outputs (e.g., published book chapters, reports to government, research data and datasets, research materials, databases, audio/video products, national and/or international reports, patents, models and

[4] In view of the overwhelming evidence that both active and passive smoking of tobacco are injurious to health, the HRB is unwilling to fund applications from individuals applying for, holding, or employed under a research grant from the tobacco industry.

protocols, software production, evidence of influence on health policy and practice, outreach and/or knowledge exchange activities, media coverage or other relevant activities) and/or any other relevant outputs that have resulted in a significant impact in their field.

Demonstrating record of independence by showing that they have secured at least one peer-reviewed research grant for a research project/s, as either the Lead Applicant or a Co-Applicant. Funding received for travel to seminars/conferences and/or small personal bursaries will not be considered in this regard.

Show evidence that they possess the capability and authority to manage and supervise the research team.

5.1.3 Early Career Researchers as Lead Applicants based in Ireland

ECRs eligible for this scheme are **postdoctoral researchers** from different disciplines who are engaged in health-related research activities typically in **academic or other research institutions.**

The early career researchers are those who have already consolidated their research knowledge, skills, methodologies and capabilities through a period of mentored postdoctoral research and who are currently progressing towards becoming independent researchers.

Early career researcher Lead Applicants must be able to demonstrate they have the skills, knowledge and supports necessary to direct the proposed research and to carry the research through to completion by showing:

- Appropriate evidence of expertise matching the nature and context of the project;
- A track record of contribution to scientific knowledge demonstrated by relevant research outputs that can prove the Lead Applicant is ready to transition to research independence;
- Some experience, capability and authority to supervise researchers (e.g. early stage researchers, research assistants, other health and care practitioners);
- A track record in independently peer-reviewed grant funding. This may include being Lead Applicant on personal awards and/or fellowships and/or being listed as co-applicant and/or collaborator on any other type of research grant.

Qualification:

The ECR Lead Applicant must have:

- a PhD or
- have been granted PhD equivalence by the HRB (are proven to have at least four years of active research experience post-primary degree).

Note: **PhD equivalence** must be granted by the HRB before the call submission date and will not be considered after application submission. Contact HRB in relation to this approval process. PhD equivalence can be granted only to individuals who are not undertaking a PhD at the time of submission. Individuals currently studying for a PhD are ineligible to apply to this funding call. This includes individuals who have research experience prior to starting their PhD.

Note: Active research experience will be considered when assessing eligibility by the HRB and competitiveness of the track record of the Lead Applicants by reviewers. Career breaks, flexible

working arrangements, changes in discipline and sector (e.g. industry, health organisation/agency) will be taken into account when assessing the research experience and scientific contribution to knowledge.

Career stage

The ECR Lead Applicants must have at least four years and up to seven years active post PhD (or equivalent) research experience.

For the purposes of this call the official date of a PhD is defined as the year that the dissertation was successfully defended. ECRs who defended their thesis in 2018 or before are eligible to apply unless they have gaps (e.g. career breaks, flexible working arrangements) in their curriculum vitae.

Employment history

The scheme is open to individuals who have the support of a HRB approved Host Institution in the republic of Ireland.

The ECR Lead Applicant:

- must hold a fixed term post-doctoral or other research based positions that covers the duration of the award **or**
- is an individual who will be recognised by the Host Institution upon receipt of the EJP RD award as a post-doctoral researcher as defined above

  AND

- is not requesting their own salary **or**
- is requesting salary related costs for a maximum of 0.5 FTE

5.1.4 Collaborators

In order to be considered as an eligible partner, a group must contribute substantially to at least one of the project's work packages. If the only role of a group is to provide patient access, data or samples for the study, they will not be considered as partners of the consortium, but can be included otherwise, via cooperation agreements or subcontracting.

Consortia may include collaborators that secure their own funding. Collaborators cannot be work package leaders, and their contribution to the consortium must be described (where relevant a CV can be included in the proposal).

If necessary to implement the action, consortia may also include sub-contractors, according to country/regional regulations. Sub-contractors may cover only a limited part of the action, and their contribution to the consortium must be described.

Collaborators and sub-contractors do not count toward the limit of 8 partners requesting research funding (nor is there a limitation of subcontractors per country, as long as their participation is justified and if subcontracting is possible according to national/regional funding rules).

5.1.5 Patient Advocacy Organisations and Patient Involvement

Consortia are strongly advised to include patient representatives and patient advocacy organisations (PAOs). **Please see Appendices below for HRB guidance.**

6 Host Institution

A HRB Host Institution is a research performing organisation that is approved by the HRB for the purpose of receiving and administering HRB grant funding and is responsible for compliance with all general and specific terms and conditions of awards. HRB Host Institution status is a requirement to submit an application under all HRB award schemes. The **Host Institution for the award** is normally that of the **Irish Lead Applicant** but it may be another organisation/institution designated by the research team, where it is clearly justified. In order to be eligible to apply for funding, an Institution must be an approved HRB Host Institution no later than two calendar months before the closing date of a call. A list of currently approved HRB Host Institutions and information on the application process for research performing organisations to be approved as HRB Host Institutions can be found on the HRB website[5].

Host Institution Letters of Support must be provided for **(1) all Lead Applicants in a contract position and (2) Co-Applicants in a contract position who are seeking their own salary.** The formal letter on headed notepaper, dated and signed by the Head of School/Research Centre/Hospital must include the following information; [*Host Institution - insert name*] which is the host institution of [*applicant - insert name*] confirms that [*applicant - insert name*]: (i) holds an employment contract which extends until [*insert date*] or will be recognized by the host institution upon receipt of the HRB [scheme] award as a contract researcher; (ii) has an independent office and research space/facilities for which they is fully responsible for at least the duration of the award, and (iii) has the capability and authority to mentor and supervise the research team. Electronic signatures are acceptable for letters that are uploaded on the HRB GEMS system.

7 Application, Review Process and Assessment Criteria

7.1 Application

There will be a two-stage application procedure for joined applications. One joint proposal document (in English) shall be prepared by the partners and must be submitted by the Coordinator in electronic format no later than 13:00 GMT on 12 December 2022 via the electronic proposal system, https://ptoutline.eu/app/ejprd23. **No other means of submission will be accepted.**

Further information on how to submit proposals electronically (including Guidelines for Applicants and submission templates) is available at the EJP RD website.

If you need additional information, please contact the Joint Call Secretariat (JCS). Please refer also to HRB Grant Policies.

Irish Lead Applicants will be required to provide additional information to the HRB at the time of submission of full proposals (i.e., stage 2). This will include justification for their requested budget,

[5] http://www.hrb.ie/funding/funding-schemes/before-you-apply/all-grant-policies/hrb-policy-on-approval-of-host-institutions/

and clarification on deliverables assigned to the partner from Ireland. A template requesting the information required from applicants from Ireland will be provided by the HRB.

7.2 Review Process

7.2.1 Pre-proposal

The JCS and CSC will check all pre-proposals to ensure that they meet the call's formal criteria. **Please note that proposals not meeting the formal criteria or the national/regional eligibility criteria and requirements will be declined without further review.**

Pre-proposals passing the eligibility check will be evaluated by two SEC members. The SEC members will then meet to establish a ranking of the pre-proposals. This ranking will be used by the CSC to decide which pre-proposals will be accepted for full proposal submission.

7.2.2 Full proposal

The JCS will check the full proposals to ensure that they meet the call's formal criteria. Each eligible proposal will be allocated to at least two external scientific reviewers. Following a rebuttal stage, the JCS will send full proposals, reviews and rebuttals to the SEC, who will meet to discuss each proposal and assign final scores, make a classification of the proposals, and rank proposals recommended for funding.

Full proposals recommended for funding by the SEC will be remotely evaluated by independent experts in ethics. Only those proposals approved by both the scientific and ethical evaluations (complying with all central Horizon 2020 and regional/national ethical requirements), will be funded.

7.2.3 Funding decision

Based on the ranking list established by the SEC and on available funding, the CSC will suggest the projects to be funded to the national/regional funding organisations. Final decisions will be made by the national/regional funding organisations and will be subject to budgetary considerations.

If necessary, the CSC will determine a priority order for proposals which have been awarded the same score within a ranked list. This will be based on (in descending order): availability of national/regional funding; maximization of the use of national/regional funding; proposals with participation of underrepresented or undersubscribed countries; proposals that address diseases not otherwise covered by more highly ranked proposals; proposals with meaningful engagement/involvement of PAOs; gender balance within the consortium's partners in order to reduce any inequalities.

The JCS will notify all project coordinators of the final funding decision and disseminate the SEC consensus report.

7.3 Assessment Criteria

Evaluation scores will be awarded according to specific evaluation criteria that are in line with Horizon 2020 rules – excellence, impact and quality & efficiency of implementation – using a common evaluation form. Each criterion will be scored out of five, for a maximum overall score of 15 points. The threshold for an individual criterion is three, with an overall threshold of 12 points.

8 Timeframe

Date	
12 December 2022	Call Opening
15 December 2022	Information webinar for potential applicants (pre-proposal)
15 February 2023 @13:00	Call Closing (pre-proposal)
End of April 2023	Invitation to full proposal
2 May 2023	Information webinar for applicants (full proposal)
14 June 2023	Call Closing (full proposal)
26 July 2023	Deadline for rebuttals
December 2023	Funding decision

9 Contacts

For further information on the EJP RD call for Natural History Studies addressing unmet needs in Rare Diseases contact:

For general information, please contact the Joint Call Secretariat (JCS):

French National Agency for Research (ANR, France)
Florence Guillot, Camille de Almeida and Ingrid Pfeifer
E-mail: EJPRDcall@anr.fr

For country-specific information for Irish Partners, please contact the HRB, Ireland:

Dr Siobhán Hackett
Email: eujointprogrammes@hrb.ie

Appendix I: HRB Funding Policies and Procedures

Access and support from research infrastructures

Applications availing of the advice, research design, data management services and/or other forms of support from a Clinical Research Facility/Centre (CRF/CRC), other infrastructure (e.g., Centre for Applied Medical Imaging, CAMI, Centre for Support and Training in Analysis and Research (CSTAR), HRB-TNRN, Cancer Groups, National clinical trials office (NCTO), CTNs or a biobank) are required to provide additional information detailing the scope and nature of the engagement (this includes national and international facilities, Units, and networks where justified).

An Infrastructure Agreement form will be requested as part of the application addressing the nature/scope of the service or collaboration, the rationale behind the choice of facility/centre/network and any costs associated with the project (including those provided as in-kind contributions). Applications proposing research with patients which do not detail advice and/or support from a CRF/CRC/CTU will be asked to justify why they have not done so.

Public, Patient and Carer Involvement (PPI) in Research

The HRB promotes the active involvement of members of the public, patients and carers in the research that we fund[6]. Public and patient involvement in research means that the public and patients are involved in planning and doing research from start to finish and help tell the public about the results of research. PPI, as defined here, is distinct from and additional to activities which raise awareness, share knowledge, and create a dialogue with the public, and it is also distinct from recruitment of patients/members of the public/carers as participants in research.

PPI represents an active partnership between members of the public, patients and carers and researchers in the research process. This can include, for example, involvement in the selection of research topics, assisting in the design, advising throughout or at specific decision points of the research project or in carrying out the research.

PPI contributors should be actively involved and part of decision making. Involving members of the public in research can improve quality and relevance of research. It can:

- Provide a different perspective - even if you are an expert in your field, your knowledge and experience will be different to the experience of someone who is using the service or living with a health condition.
- Help to ensure that the research uses outcomes that are important to the public.
- Identify a wider set of research topics than if health or social care professionals had worked alone.
- Make the language and content of information such as questionnaires and information leaflets clear and accessible.

[6] https://www.hrb.ie/funding/funding-schemes/public-and-patient-involvement-in-research/

- Help to ensure that the methods proposed for the study are acceptable and sensitive to the situations of potential research participants.
- Help you increase participation in your research by making it more acceptable to potential participants.

In addition to improving relevance and quality of research, it ensures that research is influenced by broader principles of citizenship, accountability, and transparency. PPI is an ethos as well as a practice. It should be context-specific and should aim to ensure that all voices are heard. Where members of the public, patients or carers are involved, they must be compensated for their time and contributions.

In the application, you are asked to describe any public involvement in your research throughout the various stages of identifying and prioritising the research question, the research design, conduct, analysis, and dissemination. We recognise that the nature and extent of active public involvement is likely to vary depending on the context of each study or award. PPI contributors should be named as Co-applicants where justified by their level of involvement.

For guidance and support on PPI in your research please consult with the PPI Ignite Network Ireland or your Host Institution. The PPI Ignite Network Ireland has offices located in the following seven Host Institutions: DCU, NUIG, RCSI, TCD, UCC, UCD, UL.

FAIR Data Management and Stewardship

Data management/stewardship plans (DMP) are nowadays widely accepted as part of good research practice. The HRB support open research[7] and open publishing directly through the HRB Open Research platform[8]. The HRB is driving the making of research data **FAIR** (**F**indable, **A**ccessible, **I**nteroperable and **R**e-usable) in order to benefit science by increasing the re-use of data and by promoting transparency and accountability.

FAIR data principles[9] provide a guideline for those wishing to enhance the re-usability of their data holdings: these principles put specific emphasis on enhancing the ability of machines to automatically find and use the data, in addition to supporting its re-use by individuals. For researchers, the move to FAIR and open data, where applicable, means researchers should consider data management issues and find suitable data repositories at the research planning stage. Applicants will have to provide information about their plans for data management and data sharing at application stage.

In line with the HRB's policy on management and sharing of research data[10], all successful applicants are required to submit a completed data management plan (DMP) to the HRB on or before three months after the award start date, and a final updated version of the DMP with the last annual report.

[7] http://www.hrb.ie/funding/policies-and-principles/open-research/

[8] https://hrbopenresearch.org/

[9] https://www.nature.com/articles/sdata201618

[10] https://www.hrb.ie/fileadmin/user_upload/HRB_Policy_on_sharing_of_research_data.pdf

The DMP will need to be submitted alongside a certification of completion from the designated representative(s) within the Host Institution.

Applicants will have to provide an outline of their plans for data management and data sharing in the application inclusive of the costs associated to the plan.

The timing for completion and submission of the DMPs must be also included among the objectives and deliverables of the programme.

Additional guidance to on FAIR Data Management Costs

People	Staff time per hour for data collection, data anonymisation, etc
	Staff time per hour for data management/stewardship support, training, etc
Storage and computation	Cloud storage, domain hosting charge
Data access	Secondary data access, costs for preparing data for sharing (e.g. anonymisation)
Deposition and reuse	Costs for depositing research data and metadata in an open access data repository
	Defining semantic models, making data linkable, choosing the licence, defining metadata for dataset, deploying/publishing
Others	Please provide explanations.
Notes	*The HRB is currently not covering the cost of long-term preservation of data*
	This list is not exhaustive and aims to provide examples only of eligible costs

General Data Protection Regulation

By submitting an application to the EJP RD JTC2023, applicants consent to the use, processing and retention of their data for the purposes of:

- processing and evaluating the application where processing shall be lawful only if and to the extent that processing is necessary for the performance of a task carried out in the public interest or in the exercise of official authority vested in the controller.
- administering any subsequent funding award.
- managing the Funding Party's relationship with them.
- analysing and evaluating the call.
- reporting to the European Commission/ Research Executive Agency (REA) on the Co-funded call.
- providing aggregate data to national and European surveys and analyses.
- complying with audits that may be initiated by the Funding Parties and the European Commission (or its agencies).

The members of the EJP RD consortium may share an applicant's data with third parties (some of which may be based outside the European Economic Area) in relation to the above activities including evaluators, auditors and the European Commission (or its agencies).

The members of the EJP RD consortia may link the data that applicants provide in the application with national, bibliographic or external research funding data which is available through public subscription-based databases (e.g. Scopus, Web of Science, etc.) or other national / open datasets. The members of the EJP RD consortia may also link the data that applicants provide in their application with future data that applicants provide as part of the ongoing management and reporting on a call award which may be awarded to them.

Data on Funding Parties including contact details of FC members and National Contact Points/Regional Contact Points are kept for the purpose of the call communication. The information will be published with prior consent of the respective management bodies.

Use of personal data by HRB

By participating in this call, you agree to the use of the information you provide (regarding all applicant team members) for HRB to consider your application, contact you about your application, and if you are successful, to manage your grant throughout its lifetime in accordance with HRB general T&C for research awards.

This will include contacting you with regard to monitoring of progress through written reporting and other means e.g., interim review. We will publish some basic information on successful awards including PI, Host Institution, amount awarded and lay summary on our website and may highlight individual awards or researchers in more detail (with specific consent). We will also use the information you have provided to generate general statistics around our current funding portfolio, and to evaluate our funding mechanisms and investment. After your grant has ended, we will continue to keep your information on file (in accordance with HRB policies) to allow us to evaluate the outcomes, outputs and impacts of HRB investment in your research.

Please note that we will also use information associated with *unsuccessful* applications for a number of the purposes outlined above such as generating general statistics around our current funding portfolio, and to evaluate our funding mechanisms and investment e.g., demographics of applicants, research areas of applicants. Similarly, we will use the information provided about people employed on awards to help evaluate our career support and capacity building initiatives.

The Health Research Regulations

Following the implementation of GDPR, a regulation for health research known as the Health Research Regulations 2018 (S.I. 314) has been implemented, with further amendments made in 2019 (S.I. 188) and 2021 (S.I. 18)[11]. These regulations outline the mandatory suitable and specific measures for the processing of personal data for the purposes of health research. They further set out that explicit consent is a mandatory safeguard that must be obtained from individuals when using their

[11] http://www.irishstatutebook.ie/eli/2021/si/18/made/en/pdf

personal data for health research. Where it is not feasible to obtain explicit consent, an application for a consent declaration can be made to the Health Research Consent Declaration Committee[12].

Research on Research

The HRB is developing its approach to research on research (RoR) with the aim of enhancing the evidence base for HRB research funding practices. We may also collaborate with researchers on request regarding specific RoR questions. Should your application become of interest to such a study, the HRB will seek your consent for the use of your information.

Privacy Policy and Retention Policy

To understand why we collect the information we collect and what we do with that information, please see our Privacy[13] and Retention Policies[14].

Appendix II: Resources/Useful Links

REPORTING

EQUATOR Network Library for health research reporting: an international initiative that seeks to improve reliability and value of health research literature by promoting transparent and accurate reporting of research studies

https://www.equator-network.org/library/

Registry of Research Data Repositories

http://www.re3data.org/

Zenodo Data Repository (OpenAIR)

https://zenodo.org/about

https://zenodo.org/

EVIDENCE SYNTHESIS

Evidence Synthesis Ireland: aims to build evidence synthesis knowledge, awareness and capacity among the public, health care institutions and policymakers, clinicians, and researchers on the Island of Ireland.

https://evidencesynthesisireland.ie/

[12] https://hrcdc.ie/

[13] https://www.hrb.ie/about/legal/privacy-policy/

[14] https://www.hrb.ie/fileadmin/user_upload/HRB_Document_retention_policy..docx

The Cochrane Library: online collection of databases in medicine and other healthcare specialties which summarise and interpret the results of medical research.

www.thecochranelibrary.com

The Campbell Collaboration: promotes positive social and economic change through the production and use of systematic reviews and other evidence synthesis for evidence-based policy and practice.

https://www.campbellcollaboration.org/

The Campbell Collaboration UK & Ireland: hub at Queens University Belfast.

https://www.qub.ac.uk/research-centres/CampbellUKIreland/

EQUATOR Network Library for health research reporting: an international initiative that seeks to improve reliability and value of health research literature by promoting transparent and accurate reporting of research studies.

http://www.equator-network.org/resource-centre/library-of-health-research-reporting/

BIOBANKING

Council of Europe Recommendation of the Committee of Ministers to member States on research on biological materials of human origin (2016)

https://search.coe.int/cm/Pages/result_details.aspx?ObjectId=090000168064e8ff

BBMRI-ERIC is a European research infrastructure for biobanking

https://www.bbmri-eric.eu/

OECD Guidelines on Human Biobanks and Genetic Research Databases

http://www.oecd.org/science/biotech/44054609.pdf

ISBER Best Practices for Repositories

https://www.isber.org/page/BPR

Molecular Medicine Ireland Biobanking Guidelines

http://www.molecularmedicineireland.ie/resources/biobanking-guidelines/

NCI Best Practices for Biospecimen Resources (2016 version)

https://biospecimens.cancer.gov/bestpractices/2016-NCIBestPractices.pdf

PUBLIC, PATIENT AND CARER INVOLVEMENT IN RESEARCH & RESEARCH PRIORITIES

The National PPI Ignite Network https://ppinetwork.ie/

NIHR PPI resources

https://www.nihr.ac.uk/documents/ppi-patient-and-public-involvement-resources-for-applicants-to-nihr-research-programmes/23437

Patient-Centred Outcomes Research Institute (PCORI)

http://www.pcori.org

Public Involvement Impact Assessment Framework: Provides tools for successful involvement of members of the public in research projects and for assessment of impacts.

http://piiaf.org.uk/

NIHR Payment guidance for researchers and professionals

https://www.nihr.ac.uk/documents/payment-guidance-for-researchers-and-professionals/27392

European Patient Forum Value + Handbook: For Project Co-ordinators, Leaders and Promoters on Meaningful Patient Involvement.

http://www.eu-patient.eu/globalassets/projects/valueplus/doc_epf_handbook.pdf

The James Lind Alliance Priority Setting Partnerships: Research priorities in disease areas set jointly by patients, clinicians, and researchers.

http://www.jla.nihr.ac.uk/

Campus Engage: Supporting Irish HEIs to embed civic engagement in their work. Includes resources, how-to-guides, and case studies for engaged research.

http://www.campusengage.ie/what-we-do/publications/

UK Standards for Public Involvement: The six UK Standards for Public Involvement provide clear, concise statements of effective public involvement against which improvement can be assessed.

https://sites.google.com/nihr.ac.uk/pi-standards/home

USE OF ANIMALS IN RESEARCH

Experimental Design Assistant (EDA) (online tool for design of animal experiments)

https://www.nc3rs.org.uk/experimental-design-assistant-eda

ARRIVE (Animal Research: Reporting of In Vivo Experiments) guidelines

https://www.nc3rs.org.uk/arrive-guidelines

SYRCLE (Systematic review of animal studies, register 2014-2017)

https://www.radboudumc.nl/en/research/departments/health-evidence/systematic-review-center-for-laboratory-animal-experimentation

PROSPERO (Register for systematic reviews including animal studies 2018)

https://www.crd.york.ac.uk/PROSPERO/

GENDER AND/OR SEX ISSUES IN RESEARCH

Examples of case studies in Health & Medicine where gender/sex in research matters

http://genderedinnovations.stanford.edu/case-studies-medicine.html

Gender Toolkit in EU-funded research for examples and guidance

http://www.yellowwindow.be/genderinresearch/downloads/YW2009_GenderToolKit_Module1.pdf

Sex/Gender Influences in Health and Disease

https://orwh.od.nih.gov/sex-gender/sexgender-influences-health-and-disease

Methods and Techniques for Integrating Sex into Research

https://orwh.od.nih.gov/sex-gender/methods-techniques-integrating-sex-research

NIH Policy on Sex as a Biological Variable

https://orwh.od.nih.gov/sex-gender/nih-policy-sex-biological-variable

DATA MANAGEMENT AND SHARNG AND FAIR PRINCIPLES

Digital Curation Centre: How to develop a data management and sharing plan and examples DMPs.

http://www.dcc.ac.uk/resources/data-management-plans/guidance-examples

FAIR data principles FORCE 11

https://www.force11.org/fairprinciples

UK Concordat on Open Research Data (July 2016)

https://www.ukri.org/wp-content/uploads/2020/10/UKRI-020920-ConcordatonOpenResearchData.pdf

Guidelines on FAIR data management plans in Horizon 2020

http://ec.europa.eu/research/participants/data/ref/h2020/grants_manual/hi/oa_pilot/h2020-hi-oa-data-mgt_en.pdf

FAIR at the Dutch centre for Life sciences

https://www.dtls.nl/fair-data/

Registry of Research Data Repositories

http://www.re3data.org/

RESEARCH DATA MANAGEMENT PLANS

Data Stewardship Wizard created by ELIXIR CZ and NL

https://dmp.fairdata.solutions/

DMPonline of the Digital Curation Centre (DCC), UK

https://dmponline.dcc.ac.uk/

DMPTool of University of California Curation Center of the California Digital Library (CDL), USA

https://dmptool.org/

RDMO Research Data Management Organiser of the German Research Foundation, Germany

https://rdmorganiser.github.io/en/

Guidelines on FAIR data management plans in Horizon 2020

http://ec.europa.eu/research/participants/data/ref/h2020/grants_manual/hi/oa_pilot/h2020-hi-oa-data-mgt_en.pdf

KNOWLEDGE TRANSLATION RESOURCES

Health Service Executive Research & Development Main Page

https://hseresearch.ie/research-dissemination-and-translation/

Health Service Executive Research & Development: Knowledge Translation, Dissemination, and Impact A Practical Guide for Researchers

https://hseresearch.ie/wp-content/uploads/2021/04/Tools-and-templates-.pdf

Integrated Knowledge Translation (iKT) NUI Galway

https://www.nuigalway.ie/hbcrg/ikt/

The Canadian Institutes of Health Research: Guide to Knowledge Translation Planning

https://cihr-irsc.gc.ca/e/45321.html

Training Institute for Dissemination and Implementation Research in Health: Open Access Course

https://cancercontrol.cancer.gov/is/training-education/TIDIRC-open-access

IMPLEMENTATION SCIENCE RESOURCES

Centre for Effective Services

https://www.effectiveservices.org/resources/implementation

UCC Implementation Science Training Institute

https://www.ucc.ie/en/cpd/options/medhealth/cpd1778uccimplementationsciencetraininginstitute/

European Implementation Collaborative

https://implementation.eu/resources/

CO-CREATION RESOURCES

ACCOMPLISSH Guide to impact planning

https://www.accomplissh.eu/publications-and-deliverables

Working together to co-create knowledge: A unique co-creation tool – Carnegie UK Trust

https://www.carnegieuktrust.org.uk/publications/working-together-to-co-create-knowledge-a-unique-co-creation-tool/

INFORMATION ON PERSISTENT IDENTIFIERS

DOI: List of current DOI registration agencies provided by the International DOI Foundation

http://www.doi.org/registration_agencies.html

Handle: Assigning, managing and resolving persistent identifiers for digital objects and other Internet resources provided by the Corporation for National Research Initiatives (CNRI)

http://www.handle.net/

PURL: Persistent Identifiers developed by the Online Computer Library Center (OCLC). Since 2016 hosted by the Internet Archive

https://archive.org/services/purl/

URN: List of all registered namespaces provided by the Internet Assigned Numbers Authority (IANA)

https://www.iana.org/assignments/urn-namespaces/urn-namespaces.xml

DATA REPOSITORIES

Registry of Research Data Repositories

http://www.re3data.org/

Data centers accredited by the German Data forum according to uniform and transparent standards (Germany)

https://www.ratswd.de/forschungsdaten/fdz

Zenodo Data Repository (OpenAIR)

https://zenodo.org/

FAIR/OTHER USEFUL LINKS

Main FAIR Principles

https://www.go-fair.org/fair-principles/

UK Concordat on Open Research Data (July 2016)

http://www.rcuk.ac.uk/documents/documents/concordatopenresearchdata-pdf/

Tool that helps to select and apply a license to a resource, provided by Creative Commons

https://creativecommons.org/choose/