

Rare Diseases Research and Innovation Catalyst Award (RDCAT)


HRB Information Session

23rd February 2023

Caitriona Creely, PhD

Programme Manager: Data Infrastructures and
Personalised Medicine

| Time | Agenda item |
|-------------|---|
| 2.00 | <i>Arrival and Coffee – Welcome from HRB</i> |
| 2.15 | <p>Dr Caitriona Creely, HRB</p> <ul style="list-style-type: none"> • Background to the proposed Rare Disease Catalyst Award call • Planned European Partnership in Rare Diseases |
| 2:30 | <p>Professor Eileen Treacy, Clinical Lead, National Rare Diseases Office</p> <p>European Reference Networks: Research Opportunities</p> |
| 2:40 | <p>Professor Rachel Crowley, Consultant Endocrinologist / UCD Clinical Professor</p> <p>The Rare Disease Clinical Trial Network (RD-CTN)</p> |
| 2:50 | <p>Dr Caitriona Creely, HRB</p> <ul style="list-style-type: none"> • Principles of the Rare Disease R&I Catalyst Award |
| 3:10 | Feedback session on proposed call principles |
| 3:30 | <i>End of meeting</i> |



Background to the proposed Rare Disease R&I Catalyst Award call



Background to the proposed HRB call

- Planned large scale European Research Collaboration: EU Partnership on Rare Diseases expected to begin in 2024
- Continuity and **expansion** of the work of EJP Rare (HRB and NRDO involved)
- If funded, Partnership will have budget of over €200m budget (at least €100m each from EC and from Member States)
- Ireland (through DOH & DFHERIS) has committed to participation in the Partnership
- HRB is participating in discussions to shape the Partnership, supporting input of stakeholders from Ireland
- Partnership opportunity to build on prior national and charity investments to advance research in rare diseases and increase connectivity with European (and global) efforts

HRB Investments to date

- 2005 **Research Fellowships in Rare Diseases** (with then MRCG, HRCI) €600k
- Since 2006 **HRCI/HRB Joint Funding Scheme**, with over €7m in Rare disease research funded – including possibility to fund outside of Ireland where no research capacity in Ireland
- **Joint Transnational Calls** under the European Joint Programme for RD: Two projects funded thus far €600k: current applications under peer review
- 2021 Funded **Clinical Trial Network in RD** €1m (Leads R. Crowley, C. McCarthy)

Planned European Partnership in Rare Diseases

Motivation for European Partnerships

- Horizon Europe will support the next-generation of European Partnerships to deliver on global challenges through concerted R&I effort with the Member States, private sector, foundations and other stakeholders.
- Horizon Europe expects partnerships to take a “systemic approach in the achievement of the objectives”, including “to ensure coordination with other relevant R&I initiatives”.
- European Partnerships
 - provide mechanisms to link R&I closely to policy needs
 - develop close synergies with national and regional programmes
 - bring together a broad range of innovation actors to work towards a common goal
 - turn research into socio-economic results

EUROPEAN JOINT PROGRAMME ON RARE DISEASES (2019-2023)

- EJP RD Five year programme Jan 2019 to Dec 2023 (+ 6 month extension)
- Budget > €100 million, €55 million of EU contribution
- Research and innovation pipeline for rapid translation of research results into clinical applications and uptake in healthcare for the benefit of patients
- Research funders, universities, research institutes, research infrastructures, hospitals from ERNs and patient organisations from 35 countries (including 27 EU Member States, 7 Associated Countries, UK and Canada)
- Joint Transnational Calls for rare diseases research projects: JTC 2019, JTC 2020, JTC 2021, JTC 2022 launched
- Virtual platform for rare diseases research data, information and tools
- Capacity building, training, facilitation of partnerships, validation of new methods for clinical trials

Process to establish a Partnership

- 2019: High consensus of Member States requesting a future European Partnership on Rare Diseases was proposed for Horizon Europe Work Programme 2023-24
- Autumn 2021: “Drafting group” composed of a few MS drafted a first proposal for a Partnership in RD
- Dec 2021: Consultation workshop held with EJP RD support (5 IE experts involved)
- Feb 2022: Publication of the “Concept Paper” on EC website
- Summer 2022 -> the development of the Strategic Research & Innovation Agenda (SRIA)

SRIA development

- The Strategic Research and Innovation Agenda is a partnership's strategy document, which identifies the partnership's targeted impact, foreseen portfolio of activities, measurable expected outcomes and resources within a defined timeframe.
- A SRIA should be able to translate the vision of the partnership in a long-term systemic approach to define the logic, rationales and principles of its operations also involving dealing with emerging uncertainties.
- SRIA "Taskforce" established to develop & propose a SRIA draft – to be subject of consultation(s) by EC group & a public consultation
- Began work late summer 2022

Feb 2022 Concept Paper: Vision

- To leave no one behind, deliver a RD ecosystem that builds on the successes of previous programmes by:
 - supporting robust patient need-led research,
 - developing new therapeutics, diagnostic methods and pathways,
 - spearheading the digital transformational change connecting the dots between care, patient data and research while
 - ensuring individual control over the use of personal data and strong alignment of strategies in RD research across countries and regions.
 - Finally structuring goal-oriented public-private collaborations targeted at interventions all along the R&D value chain ensure that the journey from knowledge to patient impact is expedited, thereby optimizing EU innovation potential in RD.

Feb 2022 Concept Paper: Mission (by 2031)

- Bring to bear the high value supporting services from across Europe under one roof so that every high-quality **RD research project** will benefit from cross-disciplinary expertise, goal-oriented study planning and efficient execution.
- Enable every consenting **patient living with a rare disease** to be **findable and enrolled** in a suitable clinical study that is necessary for generating advances in diagnosis, understanding of diseases, having regulatory-compliant data sets and developing treatments.
- Make Europe a global leader in rare disease research through providing a suitable **infrastructural and regulatory environment** as well as significant **increase in investment** to spur innovation, leading to job creation and optimizing EU competitiveness in R&D and healthcare innovation.

OVERARCHING ACTIVITIES

Coordination

Strategy

Governance

COMPETITIVE RESEARCH FUNDING AND SUPPORT

Joint Transnational Calls

Networking

« In house » funding of
CRN research projects

EUROPEAN CLINICAL RESEARCH NETWORK FOR RARE DISEASES

RD Clinical Research Infrastructure

- Data exploitation hub
- Diagnostic research support
- COA/PCOMs support
- Biostatistical guidance
- Clinical trial support
- other ad-hoc support services

TRANSVERSAL SUPPORT SERVICES

Data integration

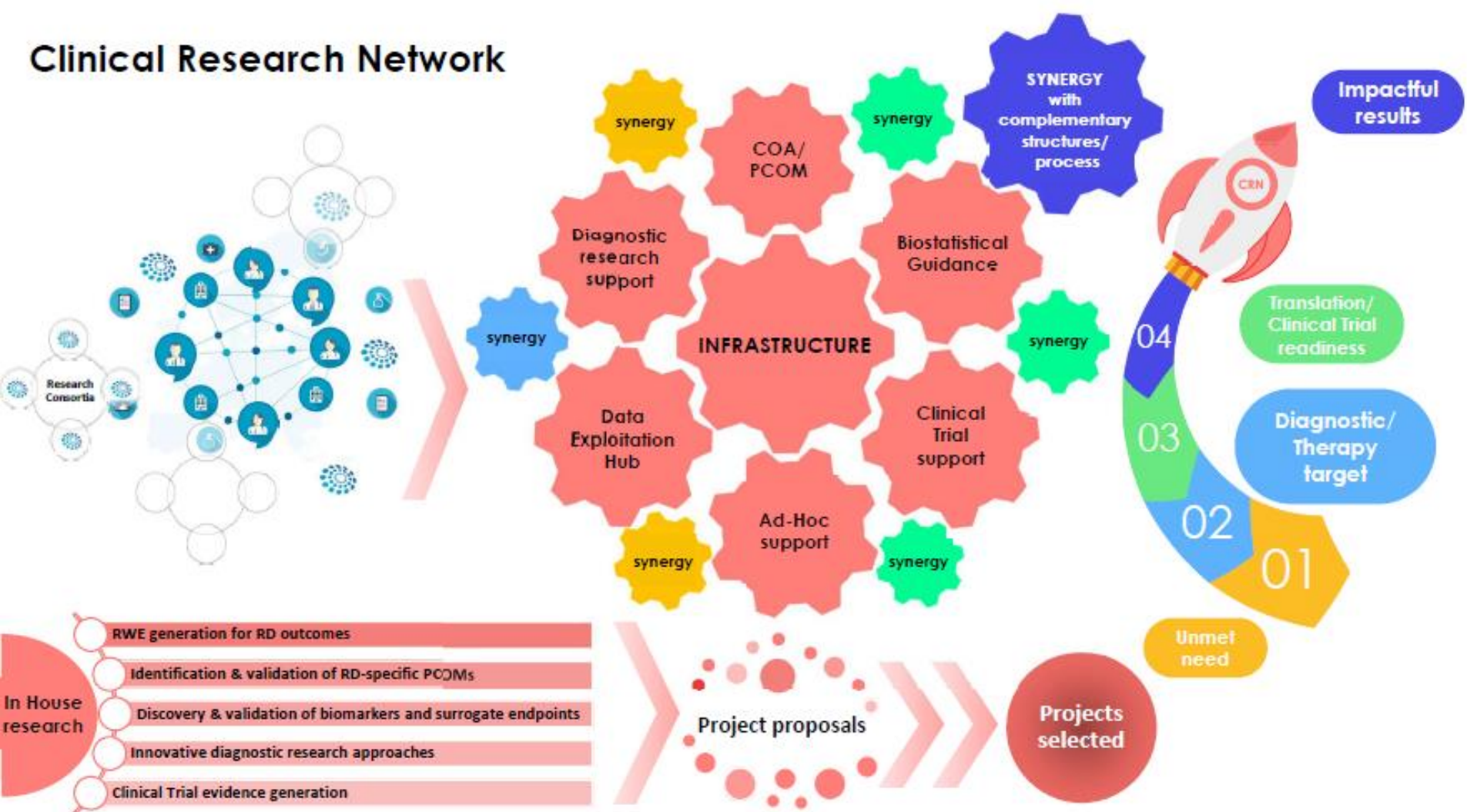
Mentoring service

Acceleration hub

ELSI support services

Capacity building

Clinical Research Network



Steps towards Partnership in RD



Key timepoints

- SRIA available for public consultation 20 March - 28 April
- Identification of partners (beneficiaries) to join the proposal 20 Feb – 30 April: Ireland will have to nominate partners (likely via DOH)
- Description of Work (what, who, how and how much) Feb – Sep
- Facilitating group of “under-represented” MS to seek common areas for focus in the Partnership – budget for this?
- Will circulate further details of process of engagement as this becomes clear



Principles of the HRB Rare Disease Research and Innovation Catalyst Award

Purpose of the call

- Position Ireland to engage in ERN research activities and benefit from the Planned European Partnership in Rare Diseases through coordinated approach
- Catalyse and strengthen the research and innovation capability across the five main clinical sites in Ireland hosting ERNs (Beaumont Hospital, Children's Health Ireland, Mater Hospital, St. Vincent's University Hospital, and Tallaght University Hospital)
- Maximise the likelihood that the ERN clinical leads can engage in research and innovation activities, including but not limited to clinical trials
- Prepare ERNs and other stakeholders in Ireland to engage in the transversal activities associated with the Partnership in RD
- Stimulate and formalise further co-investment in rare disease research

Proposed investment

- **Up to €3M Euro** over three years to catalyse R&I activity in Ireland, and drive significant engagement with the planned new RD Partnership
- Stimulus funding to create a more supportive environment for rare disease R&I, with a broad-based portfolio (e.g. patient-oriented, clinical, population health, health services)
- Single national award, made to a recognised HRB Host Institution (**single HI**)
- Support for **ERNs** with sites in Ireland, according to prioritised needs
- Co-investment (cash or in-kind required)
- The HRB research-focused investment should complement national/EU investments to support clinical activities associated with the ERNs
- HRB funding should be additional – it will not replace existing funding

HRB expectations

- Applicant team to act collaboratively towards common goals
- Enhance and extend research collaborations across the island of Ireland, across Europe (and beyond)
- Ensure relevant Rare Disease research stakeholders involved
- Ensure PPI embedded throughout supported activities
- Complement and increase engagement in the recently funded Rare Diseases Clinical Trials Network
- Supporting training of the investigator community (complementary to EJPRD/ICAT etc)

Expected outputs & outcomes

- Strategic approach to building R&I research agenda for RD in Ireland
- Increased numbers of early-career researchers completing exchanges, training activities, COST actions etc
- Coordinated, quality managed, approach to populating and maintaining database(s) of patients (families, carers) interested in research/PPI opportunities
- Increased number of competitive proposals being submitted by Ireland to e.g.:
 - European Partnership on Rare Diseases, Joint Transnational Calls (RD, PM, etc)
 - HRCI/HRB joint funding scheme, HRB Project grants, Clinical trials and SDAP etc
 - Innovative Health Initiative II, SFI, Wellcome, charity funded research
- Greater accessibility to research studies for patients and healthy volunteers
- Greater number of participants from Ireland on ePAGs
- Increased number of researchers generating preliminary data on which to base concrete research proposals

Who should apply

- Applicant teams with necessary breadth and depth of expertise
- **Lead Applicant**, expected to be active in rare disease research, and with experience of clinical service delivery in Ireland
- **Applicant team**: co-applicants need defined roles in delivery of the award
- **PPI team members** expected
- Mechanism for inclusion of ERN Clinical Leads and patient voice in governance structures
- Strategic Advisory Board suggested to provide outside perspective and aid with horizon scanning
- **Industry partners** or associations may join as collaborators

Host Institution

- Single application, submitted by a single HRB Host Institution
- Host Institution ideally has experience of supporting multi-institutional collaborative initiatives
- Up to 30% overheads can be applied
- The apportioning of overheads should be agreed between the partner institutions during the preparation of the application. The HRB expects this to be formalised for successful applications.

Cash/in-kind contributions

- Cash or in-kind expected
- Contributions intended to be additive – evidence that we are not replacing existing support
- Contributions should support integration of research activity into healthcare system
- In the region of 0.5 FTE contribution to RDCatalyst Coordinator cost would be expected
- HI/Hospital/Associated charity contributions counted
- Money drawn-down from Partnership on RD can be counted

Scope of activities

- Provide targeted research support for sites in Ireland as they transition to active members of the ERNs, with the aim of increased capacity for pre-clinical, clinical, population health and health services research, and to promote CT readiness
- Added-value, strategic and focused investment
- Certain posts may be valuable*, for example:
 - RDCatalyst Research Coordinator (across Ireland, and connectivity with external initiatives)
 - RDCatalyst Research Data Coordinator (complement work done to date by CTN-RD)
 - Training and outreach coordinator (strong liaison with EJP RD)
 - PPI liaison and support, including for activities of ePAG (e.g. PPI liaison officer)
 - Research fellows to provide research support to the ERN leads/co-leads

Out of scope

- Building work, fit-out of buildings, or major pieces of equipment
- Establishment of new registries without a sustainability plan – these can be funded through other mechanisms and may count as co-investment as long as they are established aligned with ERN standards, and intended to be interoperable
- Project funding
- Direct costs of biobanking
- Applications from individuals applying for, holding, or employed under a research grant from the tobacco industry

| Stage | Indicative dates |
|---|------------------|
| Approval of call by HRB Board | Late March |
| Invitation to submit application | April 2023 |
| Closing date | June 2023 |
| Review period | July-Sep |
| International Review Panel meeting | Early Sep 2023 |
| HRB Board approval of Panel recommendations | Late Sep 2023 |
| Pre-contract (including approving any conditions of funding) and contract negotiation | Oct/Nov 2023 |
| Awards commence from | Dec 2023 |

Feedback session

Next steps

- Reflecting on discussion today, further feedback to reach HRB by COB Wednesday March 1st
- Feedback to be collated and submitted by a nominated person to Christina Rushe crushe@hrb.ie
- HRB will consider feedback when finalizing details of the Rare Disease Research and Innovation Catalyst Award
- HRB Board approval will be sought (March 2023)
- Call will be published April 2023, subject to HRB Board approval

Thank you