UK Rare Disease Research Platform
Webinar
07 September 2022

Chair.
Professor Brian Walker

Panel:
Kath Bainbridge (DHSC),
Richard Evans (MRC), Joanna Robinson (MRC).
1 Welcome
Format, ‘housekeeping’ and aims

2 Background
Why we are establishing the UK Rare Disease Research Platform

3 Platform overview
Aims and planned structure of the Platform

4 Call for Nodes
Remit & scope, costing, panel criteria & timelines

5 Question and Answer session
Ask questions via ‘Q&A’ function
Any questions not answered ‘live’ – future FAQs
Context: Rare Disease Research (RDR)

- 2021 UK Rare Disease Framework
  - Follows on from the 2013 UK Strategy for Rare Diseases
  - Underpinning theme: ‘Pioneering research’. MRC, DHSC & NIHR are working on a landscape map and portfolio

- Relevant to a range of funders…
  - including MRC/UKRI and NIHR but also research charities and industry.
  - Draft portfolio shows that rare disease research is not always labelled as such

- …and of global interest
  - EU Joint Programme in Rare Diseases (EJPRD)
  - IRDiRC
The Challenge & Opportunity

- UK has real strengths in RDR, but these are distributed
- Strategic Steering Group: opportunity through coordination
- Create ‘critical mass’ linking to key stakeholders (including patients, industry, policy and beyond)
Aims of the UK RDR Platform

**Coordinate** broad benefits:
- Greater coordination and cohesion of UK RDR
- Visible accessible entry point for stakeholders and new partners
- Strengthen links and maximise value with existing networks and major investments

**Enable** focused advances
- address blockers of progress across pipeline, pilot work, de-risk next steps
- increase competitive response mode and strategic bids
Platform Structure

A. One central administrative HUB
Single point of contact, web presence, annual Network meeting/workshops, supports nodes & governance

B. ~10 domain-specific NODES (3 shown, lozenges)
Based on a specified, tractable opportunity, including:
• cross-disease clusters/groupings
• development or application of common tools, technology, methods or disease management
• cross-cutting issues

C. Each node delivers 2-3 PROJECTS (circles)
Specific deliverables identified as valuable for the UK RDR community.

D. Network activities (open to all)
delivered by Hub, Nodes and other partners.
# Call for Nodes – Remit and Scope

<table>
<thead>
<tr>
<th>In scope</th>
<th>Out of scope</th>
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<tbody>
<tr>
<td><strong>Domain</strong></td>
<td><strong>Single disease or focus on common disease stratification</strong></td>
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<tr>
<td>Tractable opportunity in RDR, e.g.:</td>
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<tr>
<td>• cross-disease clusters/groupings</td>
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<td>• development or application of common tools, technology, methods or disease management</td>
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<td>• cross-cutting issues</td>
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<td><strong>Team</strong></td>
<td><strong>Duplication of existing networks/investments</strong></td>
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<td>RDR researchers spanning disciplines &amp; institutions to address domain</td>
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<td><strong>Networking &amp; Coordination</strong></td>
<td><strong>Bids lacking appropriate networking and coordination</strong></td>
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<td>Across relevant researchers, existing investments, patients &amp; wider stakeholders</td>
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<td><strong>Projects</strong></td>
<td><strong>Projects suitable for response mode</strong></td>
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<td>Up to 3 projects (each 1-2 years, max 3) to de-risk future funding &amp; remove obstacles</td>
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<td>Animal model development</td>
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Call for Nodes – Funding

- £12m: 10 Nodes (this call) and coordinating Hub over 5 years
- Each Node: 0.8-1.0m (up to 1.25m max) (80% FEC)

Eligible costs:
- Salary PI/CoI (time directing Node)
- Project management (up to 0.5FTE)
- Coordination (conferences, engagement...)
- Projects: Up to 3, duration 1-2 years

Ineligible costs:
- salary costs for advisory group members
- equipment
- relocation costs
- publication costs
- PhD stipends
What the panel will look for…

• compelling tractable opportunity with impact in the identified ‘domain’

• suitability and inclusivity of the proposed project team and planned partners/stakeholder engagement

• coordination activities that will enable the community and deliver benefits including:
  • effective networking & strategic coordination
  • sustained impact (by bringing together an enduring community)
  • underpinning competitive funding applications
  • enabling access to infrastructure

• projects that are deliverable, with an acceptable level of risk and plans to co-develop the work.

Panel will ensure thematic spread, national diversity and non-duplication across node applications.
Call timeline and process

Today

• select competitive ideas
• shape proposals for cohesive platform

WEBINAR, AND LINKEDIN GROUP OPEN 7TH SEPT

OUTLINE DEADLINE 8TH NOV

INVITE TO FULL PROPOSAL DEC 2022

Connect and shape full proposals

NETWORKING WORKSHOP 11TH JAN

FULL PROPOSAL DEADLINE MID-FEB 2023

Select Nodes

PANEL MEETING MAR 2023

AWARDS START JUL 2023

Call timeline and process
Q&A

Call page:  Establish an integrated UK Rare Disease Research Platform: nodes – UKRI

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LinkedIn: https://www.linkedin.com/groups/12708006/