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UK Rare Disease Research Platform Webinar

07 September 2022

Chair.

Professor Brian Walker

Panel:

Kath Bainbridge (DHSC),

Richard Evans (MRC), Joanna Robinson (MRC).

Agenda

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

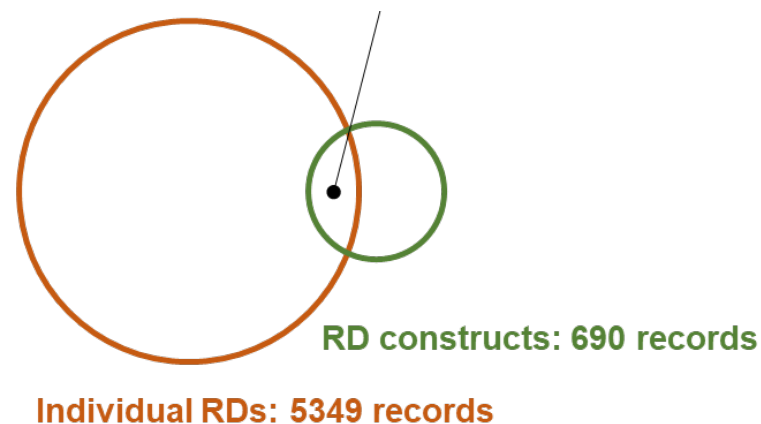
5 Question and Answer session

Ask questions via 'Q&A' function

Any questions not answered 'live' – future FAQs

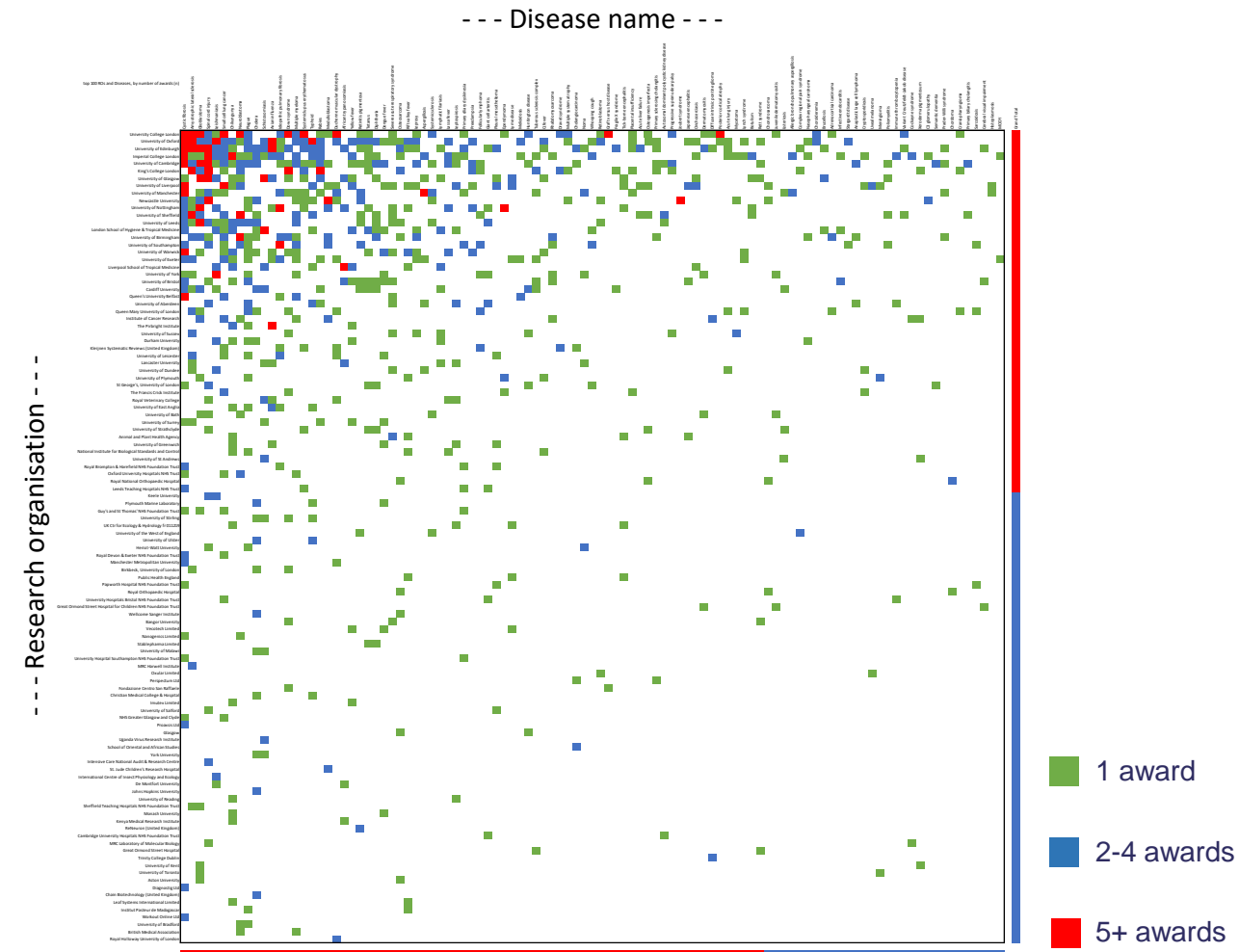
Context: Rare Disease Research (RDR)

- 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



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Platform Structure

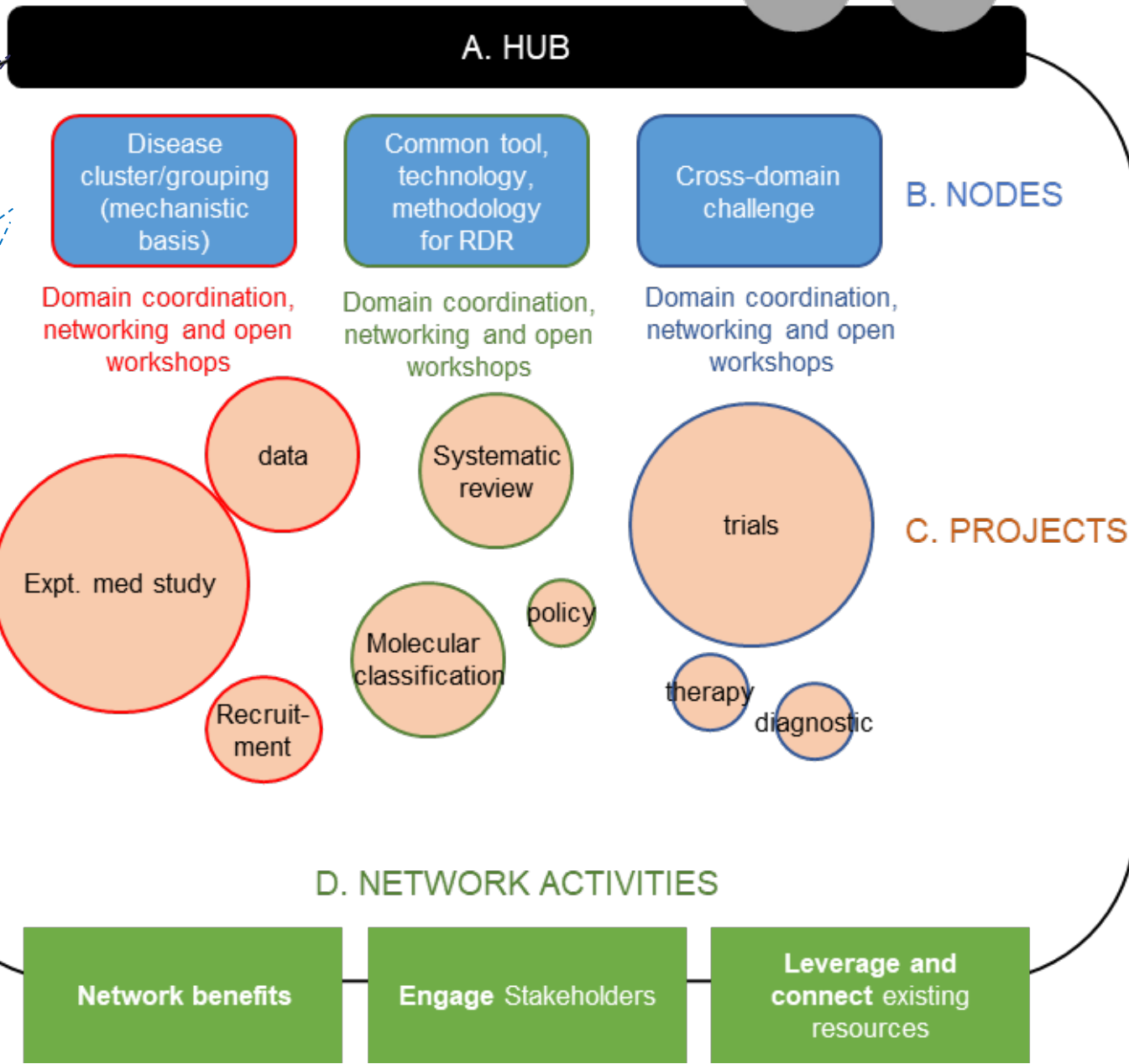
A. One central administrative HUB
Single point of contact, web presence, annual Network meeting/workshops, supports nodes & governance

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

	In scope	Out of scope
Domain	Tractable opportunity in RDR, e.g.: <ul style="list-style-type: none">• cross-disease clusters/groupings• development or application of common tools, technology, methods or disease management• cross-cutting issues	Single disease or focus on common disease stratification
Team	RDR researchers spanning disciplines & institutions to address domain	Duplication of existing networks/investments
Networking & Coordination	Across relevant researchers, existing investments, patients & wider stakeholders	Bids lacking appropriate networking and coordination
Projects	Up to 3 projects (each 1-2 years, max 3) to de-risk future funding & remove obstacles	Projects suitable for response mode Animal model development



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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/Col (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



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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.

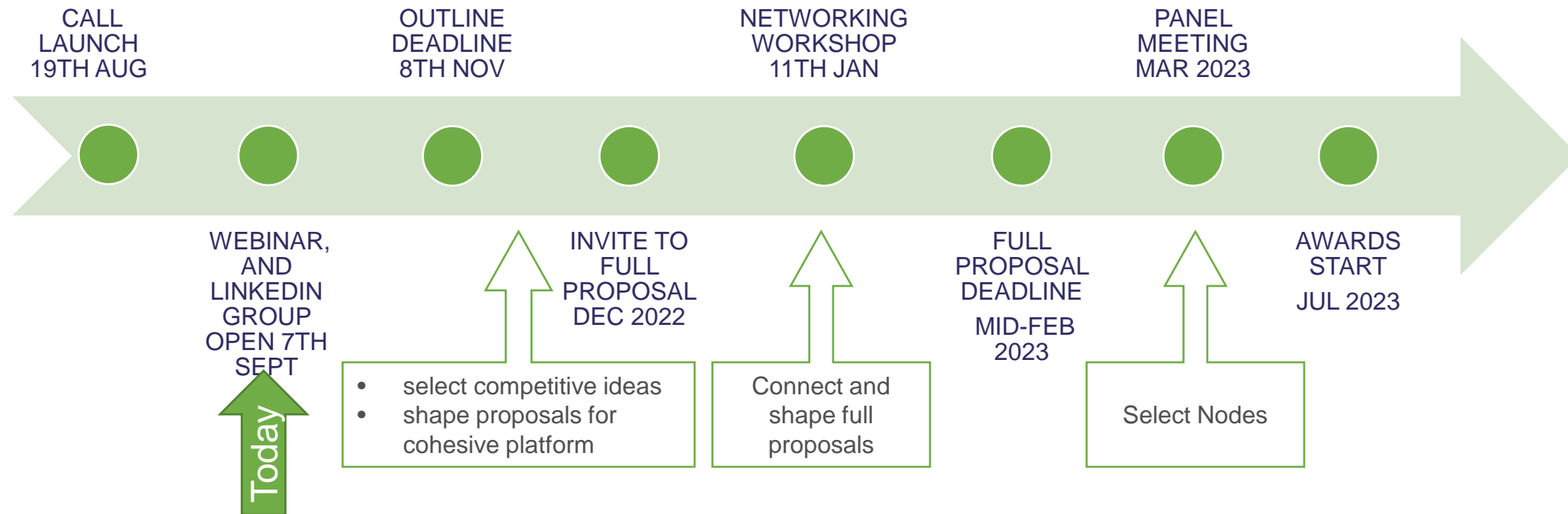


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Call timeline and process





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Q&A

Call page: [Establish an integrated UK Rare Disease Research Platform: nodes – UKRI](#)

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