Rare Disease Research Platform FAQ

FAQ questions on:

Remit

1. **What is a domain – what constitutes a tractable challenge?**
   We envisage domains being built around aspects that are common across multiple rare diseases, such as involving the same organ or sharing a mechanism. Our Steering Group advised that rare disease research was not one group of interests, but that an overarching platform could link up domains, based on some common feature, to solve distinct challenges. Compelling applications are likely to address challenges in rare disease research that that could result in scientific and patient impact, if addressed in a concerted fashion.

2. **Activity X would not normally be funded through NIHR but would be through MRC – is it eligible?**
   Standard MRC criteria apply and we will support activities that are aligned with the principles set out in the call text – this call does not aim to replace response mode funding, but focussed on coordination and tackling key barriers to rare disease research across different rare diseases

3. **How is prevalence defined to establish that a disease is rare?**
   In general, we use the prevalence definition of less than 1 in 2000 people, as used for example in the UK Rare Disease Framework. This is usually a lifetime prevalence and can vary with geography. In practice however, this is not an absolute cut-off, and the call will consider research that is broadly applicable to rare diseases and the challenges that are specific to this group of diseases. Applications to the call should not focus on a single rare disease.

4. **Can disease X be shown as a disease exemplar?**
   Rare disease of all cause and any cause are within remit. We will not fund nodes focused on only one disease and projects in Nodes should not be project grants suitable to response mode funding. However, a disease could be an exemplar with other related diseases that may benefit as long as cross-cutting impact is clear. A compelling application is likely to be one that applies to a number of rare diseases, perhaps of different types, and may address a challenge that applies across rare diseases.

5. **Are rare cancers/fungal infections…area X in remit?**
   The definition we have used is a disease with a prevalence of less than one in two thousand, for example those included in the Ophanet database; however, it shouldn't be an arbitrary cut off so if your disease is rare and needs consideration as a rare disease

then it is probably within scope if positioned in the overall landscape of rare diseases. Depending on the level of interest in the call, we would not expect to fund multiple Nodes on any one group of rare diseases. A compelling application is likely to be one that applies to a number of rare diseases, perhaps of different types, and may address a challenge that applies across rare diseases. Nodes will propose 2-3 projects, and these could be focused on similar or different groups of diseases.

6. **Do all diseases within a node need to be rare or would research in rare disease(s) which is/ may be applicable to more common diseases be eligible?**

   The focus of the proposal should be on rare diseases, based on prevalence, but links to more common diseases when this is of clinical or scientific relevance are not excluded. Research on the stratification of common diseases into less common subtypes is likely to be more suitable for support via other schemes and is not the focus for this call.

7. **What is the balance of discovery versus care delivery research to be supported.**

   The framework is deliberately trying to enhance the UKs capability for care delivery. This call is about enabling the research that underpin this enhancement. We are not focussing the call at a specific aspect of the translational pathway, with the partnership with NIHR enabling us to cover a wide breadth of activity. The UK rare diseases framework highlights one of the four priorities as being around coordination of care, and another one around access to specialist care. Applications to address key research blockers in those areas would therefore be welcome.

Who can/should be involved in the bid?

8. **Who needs to be involved to make a Node bid competitive?**

   We envisage funding only one Node in a given area/domain, so will be looking for the right team for the domain. The Panel will consider suitability and breadth of the proposed project team as well as appropriateness of partners, proposed links (for example to existing investments and infrastructure) and proposed stakeholder engagement, including patient and public involvement and engagement.

   Applications from new groupings of researchers from multiple disciplines, institutions and research interests are strongly encouraged.

   Applications from existing networks are permitted only where there are clear plans to engage new partners across the UK and enable new activities to address tractable opportunities that would otherwise not be possible.

   All nodes must remain open to new members, partnerships and collaborations if funded.

9. **How can I find others interested in a similar domain to consider a joint bid?**

   A LinkedIn Group has been set-up to enable new connections or identify common interests: https://www.linkedin.com/groups/12708006/. This group is an optional tool to support the formation of Nodes throughout the funding opportunity process. Membership of this group is not a prerequisite to submitting an application. Your activity in this group will not influence decision-making on your application. The MRC does not expect to be involved in any subsequent discussions that arise from introductions in this group.

For successful outlines that are invited to make full submissions we will publish details of the principal investigator (node leader), the proposal title and summary of the outline application. The aim is to provide a refined set of information to ensure the wider community has a view of the developments. This will provide an opportunity to approach developing bids in areas of interest.

10. Is this a UK-wide call that can include co-investigators from across the UK?

Yes, standard UKRI eligibility applies. Co-investigators must be based in the UK , from a UK research organization and meet the standard MRC eligibility criteria as per the **MRC guidance for applicants**.

11. Can International co-investigators be involved?

Researchers who are not based at UK institutions are not eligible to apply as principal or co-investigator (PI/CoI) for this opportunity but can be part of the wider membership of the node. Standard funding rules apply (e.g. see section 3.3 MRC Guidance for Applicants). The costs for work undertaken at an international organisation are admissible and should be discussed with the MRC programme manager before submission of the full application.

12. Can wider stakeholders (industry and patient stakeholders) be located outside of the UK?

Yes, if this wider membership is beneficial to the node.

13. What linkage should there be to existing Rare Disease Platforms and relevant investments (e.g. NIHR BioResource, Genomics England)?

We are aiming to coordinate and complement existing activities, without duplication and encouraging links to relevant existing resources. The Hub will be responsible for ensuring links to wider existing resources to provide cohesion.

14. What sort of linkages/synergies with the UK Mouse Genetics Network would be considered appropriate?

We don’t expect generating of new animal models to be in scope for this call. But we encourage linkage to existing investment and assets and these could be part of the wider network of a proposed node where they provide additional value.

15. Can applicants for this call hold existing substantive awards from the funders (e.g. MRC Fellowship, MRC Unit Programme Leader, NIHR Professorship)?

Yes, if you are eligible to apply for MRC funding you are eligible to apply for this call. However, applicants must abide by the terms and conditions of pre-existing awards (such as time commitments to those awards). In addition, the Node application should not duplicate any work or salaries that are covered by other funding, as for any funding bid.

16. Are there restrictions on the number of bids being led by an institution?

While there are no formal restrictions on the number of bids per institution, we are seeking to establish a platform that spans the UK. We would encourage the best bids to be led by the best placed partner within the proposed team.

17. Can industry partners be involved and what agreements need to be in place?

We welcome industry involvement where it helps to address the identified domain or challenge.

Wider partners should be able to engage with the Nodes and Platform at different times and levels, and to come and go during the lifetime of the investment; and in many cases this may not need formal agreement. If a node or project requires an industry partner from the start, then MRC has a standard industry collaboration framework (ICF) which should be followed.

18. Are you expecting patient level involvement at nodes, or will this be at the broader hub level?

Both engagement points are likely to be relevant. The Hub will have a supportive function across Nodes, and will help the Platform and Nodes to engage with patients, but we would also really encourage direct patient engagement to be embedded in individual Nodes when this is appropriate or necessary.

19. Will the 'no funding' rule for advisory group members extend to patient representatives?

Salary costs for members of external advisory groups cannot be included. However, reasonable reimbursement of costs to enable all relevant participation, including by patient representatives, is eligible for funding and should be included as necessary.

Funding and scale

20. How will the £12m investment over 5 years enable research in the longer term?

There is significant support for research in rare diseases via standard response mode mechanisms at a number of funders. This initiative seeks to provide the coordination across rare diseases and provide funding for small focussed high risk projects to address barriers to rare disease research. We envisage that this will enable and strength the submission of competitive bids to response modes schemes, and also enable the community to develop the case for strategic investment where there is a need and when funding opportunities arise.

21. What is the overall scale of a Node and the scale of funding available for projects within it?

The maximum cost per Node that can be requested is up to £1.25 million, and we typically fund 80% of the full economic cost. Funding requests should usually be in the range of

£800,000 to £1 million, but up to £1.25 million may be considered when specifically justified, for example by the inclusion of projects with higher than typical science costs. Eligible costs are outlined in the call text and in MRC standard Guidance to Applicants. Requested funds should support a range of varied activities, dependent on the identified need, and should allow the identified challenges to be addressed in creative and innovative ways. Each node should support both 1) enabling science projects and 2) networking/coordination.

22. Is the scale of investment per Node (£0.8m-1.25m) sufficient to achieve the ambitions and support up to 3 high risk paradigm shifting projects?

The intention is to support short-term focused projects that will enable you to make the next step and then prepare and submit competitive response mode bids that will deliver more substantive research at the scale needed.

23. Do projects need to be distributed across the 5 year period or could they be delivered early in the life-time of a node?

The timing of projects should be based on the needs identified, and projects do not need to be delayed or deliberately spread over time. Projects may however benefit from being refined or adapted once the Platform is in existence, perhaps by co-production with new partners who engage with the Platform, so we encourage including flexibility in timelines so that this can happen.

24. How will funding be distributed for multi-institution bids?

Funding would be distributed via the designated lead institution

25. What budget is available for the Hub in the Platform?

A budget of ca. £100k- 150k per year is envisaged for the Hub, and we expect to commission this via tender in a separate procurement exercise.

26. Are costs for training in scope?

PhD stipends are out of scope and we are not seeking to duplicate provisions such as fellowships – however, alignment of early career researchers who may be inspired to work on the tractable challenge of the node with support from other sources would be encouraged.

Process and selection questions

27. If Node outlines overlap significantly, will you suggest that they merge?

The outline expert panel may guide bids to combine or complement to ensure optimal coverage of bids. We also encourage applicants to seek to reach out to forge partnerships ahead of the outline, e.g. via the provided LinkedIn page. As stated in the call guidance, we also reserve the right to invite applications in areas where there is a gap in coverage. High

level details of successful outline applications will be published to enable additional partners to join prior to a full application. Nodes and their associated activities are expected to be open to new partnerships and links throughout the funding period.

28. How will you ensure balance of rare diseases in the final platform and will there be a preference for understudied diseases?

The panel for the outline stage will seek to identify proposals that have the potential to form a coherent Platform with the best coverage and balance that is achievable. All rare disease research is in scope for the call although clinical need, patient impact and scientific/translational potential will be important considerations. The ability to bridge silos or address neglected clinical or research areas that are understudied or where there is high need, could be an advantage. We would not expect to fund more than one node in a given area, and the outline panel may provide feedback to shape proposals, e.g., if there is overlap or areas of synergy.

29. Are there plans to select Nodes based on geography?

We are seeking to establish a platform that will encompass the major aspects of rare diseases, although these aspects can be defined in different ways. We would expect most node applications to be from multiple locations, institutions and/or disciplines in order to bring together the right expertise to tackle an important, novel challenge. We envisage a platform that spans much of the UK and the funding panels will ensure both thematic and geographical, cross-UK, coverage.

30. How important do the panel see the sustainability of any 'node' beyond the period of this funding?

Sustainability will be part of the assessment criteria of full bids and are part of the added value of any proposal, depending on the need that is being addressed.

31. What level of detail is expected in the outline application?

Details of exact headings to use in the outline case for support are included in the call page under ‘How to Apply’. The document should be up to 3 pages and should give a high-level overview and enough detail for the outline stage panel to assess suitability against the aims of the call.

32. Can I change or add CoIs to my application after the outline phase?

Yes, we envisage teams changing with feedback from the panel and further networking that will be part of the process towards a full application

33. When will we know if we have been invited to submit a full proposal?

We envisage informing successful outline applicants in mid December, with a virtual workshop and further guidance on 11 January

Other

34. What will happen to the Platform after 5 years?

We envisage that the Platform will seek to establish sustainable partnerships, that will remain viable and well positioned to coordinate the domain without targeted funding by attracting response mode funding. Some limited continuation of funding for coordination activity may be considered, but sustainability will be part of the assessment criteria of full bids.

35. What will the governance look like for the Platform?

A Platform Executive Board will bring together all Node Directors as well as nominated funder rep, external science experts and representatives of major investments to discuss direction of the platform and co-develop activities. While meetings will be convened by the Hub, the Hub will not have a direction setting role but provide support to the Platform and Nodes. Major changes would require referral back to the Funders, who will be advised by a Platform Monitoring Group (drawing on the original Panel to establish the Platform). A governance diagram will be published.

36. How will the Hub be selected?

The Hub will be commissioned separately with a tender to be issued soon – this will be selected to be separate and independent of the selected Nodes. It will provide an administrative, coordination and supportive role rather than a direction-setting role.

37. How do you envisage working with charities and wider stakeholders in this area?

We see the platform coordinating efforts as well as tackling key cross-cutting challenges. We envisage the Hub supporting this linkage and enabling connections to be made where they are currently difficult and critically enhancing visibility/making it easier to find the right connections.

If your question has not been answered in this document please feel free to email it to rarediseaseresearch@mrc.ukri.org.