Translational Rare Disease Centres Funding call - Frequently Asked Questions

Contents
Scope / remit ................................................................................................................................. 1
Definitions ...................................................................................................................................... 2
Applicants & Partners .................................................................................................................... 2
Hub ............................................................................................................................................... 3
Relationship to the MRC / NIHR rare disease call ....................................................................... 3
Budget .......................................................................................................................................... 3
Expression of Interest (EoI) Form & submission ............................................................................ 4
Timings .......................................................................................................................................... 4
Contact us ...................................................................................................................................... 4

Scope / remit
1. How many Centres will you be funding in total?
Response: We envisage 4 or 5 Centres in addition to the coordinating Hub.

2. Are there any themes of particular interest to LifeArc?
Response: What we would like to see is how you think you can make a step-change in the translational rare disease research landscape. LifeArc has several Translational Challenge focus areas, but for this call any area of unmet need in rare disease translation would be in scope.

3. Can the Centre bids be both modality-based, and disease area-based?
Response: Yes, provided they are of appropriate scale and address a major translational challenge. Please contact us at trdcentres@lifearc.org if you would like to discuss your proposal before submitting an expression of interest (EoI) application.

4. Would you like to see projects that span full length of drug discovery to clinic, or can we focus on a particular stretch?
Response: It is fine to focus on one stretch of the development pathway if that is your area of expertise. We would want to see where the projects fit in the development pathway – so what has already happened and the route to patient benefit.

5. Can clinical trials be included in Centre bids?
Response: Yes – clinical trials can be included in Centre bids, as can the work leading up to a trial. Remember that bids need to address a major challenge, beyond the scope of a single therapeutic development project.

6. Does translational include pre-clinical disease models?
Response: Yes, if they are relevant to the critical path to translation.
7. You highlighted that basic underpinning research is out of scope. Could you expand on the specifics of that please. What about disease models (animal/cell)? Biomarker discovery? Functional testing of genetic variants? Etc. Could these be included as part of a bid?
Response: See above re. disease models (Qu. 6). Novel biomarkers & their validation – yes, these are in scope.

8. Will you allow animal work, of any type (i.e. pre-clinical trials of a therapy)?
Response: Yes, we will support translational animal work.

Definitions
9. What is LifeArc’s definition of a rare disease?
Response: We are not narrowing in on either the US or EU definitions. For us, a rare disease is one that is uncommon and has a particular need for investment, as others are not investing in it. We do not consider any disease ‘too rare’.

10. What is LifeArc’s definition of translation?
Response: We are looking at the whole landscape holistically, so policy change, capability infrastructure building and other underpinning enablers would be in scope, along with critical path translation of therapeutics and diagnostics, as part of an overarching, larger scale aim. We have left the scope of the call deliberately broad so that we hear from you what you consider to be the important challenges, and solutions to them.

11. How are ‘well resourced’ research topics / areas defined?
Response: We are not providing a definition for this. Instead, we would like to know your arguments for why your area of interest is under-resourced and what the unmet need is.

Applicants & Partners
12. Can academic institutions be involved in multiple applications?
Response: Yes. However, each Principal Investigator can only lead on one application.

13. Please could you define an organisation? Is this multi-University or multi-department within a University.
Response: The lead partner needs to be a UK academic institution. While we aren’t imposing rigid criteria, we prefer consortia to be built across different organisations, for reach into the community as a whole.

14. How many partner organisations do you expect in a single bid?
Response: At least two. We are not being prescriptive. Please consider the expertise needed and how all partners will work together.

15. Do the collaborating organisations need to be academic institutions?
Response: No, collaborating partners can be drawn from any relevant stakeholder groups. However, all bids must be led by a UK academic institution.

16. Do all partners in the consortia need to be based in the UK?
Response: No, the partners can be based overseas, but the lead applicant and Institution need to be based in the UK.

17. Are the centres expected to be multisite?
Response: Centres can be virtual, with only a single physical site, but all consortia need to involve two or more different partner organisations.
18. Is it expected that collaborators must be from different regions? Or collaboration between a University and an NHS trust in the same region be considered sufficient?
Response: We will consider multiple different models.

19. Does the leadership of the Centre have to be by a senior academic Prof level or could it be by a less senior person supported by senior academic Prof and strict governance?
Response: We fully support applications from lead applicants who demonstrate the appropriate skill set to manage a consortia, irrespective of their seniority.

Hub
20. Can a centre also be a co-ordinating hub or are they mutually exclusive?
Response: This call is focussed on the Centres. However, if you are interested in being part of the coordination hub rather than, or in addition to, a Centre, please do contact us.

21. It’s mentioned about capacity development being part of the Hub - can you say more about that aspect?
Response: In the scoping work we’ve done to date, we have identified a number of areas that require capacity development to enable building the translational research ecosystem in the UK. One example is a lack of skilled translational project managers. We are open to Centre bids that include an element of capacity building and training.

Relationship to the MRC / NIHR rare disease call
22. Would the expectation be that all bids have strong links with groups funded under the recent MRC/NIHR rare disease node call?
Response: We timed this call intentionally so that the MRC/NIHR node call outcomes were known. Links to groups who have been awarded the MRC/NIHR node funding are expected in some bids, but are not a requirement for applications to this call. We expect anyone who has been successful in an MRC/NIHR node call application acknowledges this in their Expression of Interest application.

Budget
23. What proportion of the 'deliverables' and the total £ of the Centre award do you envisage will focus on direct treatments, and should these treatments be 'novel'?
Response: The treatments should be novel. The proportion of your bid that is allocated to developing therapeutics is up to you.

24. Can PhD studentships can be included?
Response, post-webinar update: This has been asked by several groups and we are open to considering PhD studentships if these are under resourced, with appropriate justification.

25. Will you support any outsourcing to CROs?
Response: Yes, where justified.

26. Will potential NHS costs, for example staff/equipment, be covered?
Response: We are very supportive of clinical and allied healthcare professionals being involved in research and will support their inclusion in these proposals. See separate response to Equipment costs (Qu. 27).

27. Can equipment costs be included?
Response: Equipment can be included, provided that it is fully justified. We are not expecting to fund large items of equipment that take up a significant proportion of the budget.
Expression of Interest (EoI) Form & submission

28. Are there word limits on the EoI form?
Response: We have given a word limit on the Abstract summary only (200 words). However, please do keep your overall applications as succinct as possible.

29. How should completed EoI forms be submitted?
Response: Please send your completed EoI form and any supporting documents as one pdf by email to trdcentres@lifearc.org by the end of the day on Friday 26 May 2023.

Timings

30. Is the start date negotiable?
Response: We want the Centres to start as soon as possible, which we anticipate being in the first half of 2024.

31. Is 5 years a maximum project duration as some rare diseases in the UK may not have enough patients to recruit within 5 years to reach the sample size?
Response: The Centre awards are for five years. We do not expect the Centre bids to be focussed on a single clinical trial, although clinical studies and trials infrastructure can be included. We would like to see your plans for continuation after the 5-year period as part of the sustainability plans, e.g. what follow-on funding you could apply for, or partners who might be interested in taking a candidate drug / device / diagnostic into further development.

Contact us
If you still have questions, or have follow-on questions after the webinar please contact us on trdcentres@lifearc.org. Use the same email address, or the contact form on the Website to request a phone call with the team to discuss your application.