# Frequently Asked Questions (FAQs) for RDCRN RFAs

**1. Can more than one application be submitted from the same institution? Can one institution lead in multiple Rare Diseases Clinical Research Consortia (RDCRCs) for different rare disease?**

*Yes, more than one application per organization/Institution may be submitted as long as the rare diseases represented in the applications are distinctly different.*

**2. What is the definition of a rare disease?**
*According to the definition we follow –a rare disease is generally considered to have a prevalence of fewer than 200,000 affected individuals in the United States. Rare diseases and terms are listed on our website http://rarediseases.info.nih.gov/RareDiseaseList.aspx?PageID=1*

**3. Can we just propose one rare disease for the RDCRC?**

*No. This RFA requires that you propose clinical studies on at least three related rare diseases.*

**4. Is the RFA open to those RDCRCs previously funded to re-apply for additional funding?**

*Yes. It is an open competition. The RFA is open to both existing consortia and new consortia.*

**5. I had applied last time. Can I reapply?**

*Yes. Submit a new application. Resubmissions are not allowed (do not refer to previous comments received on your summary statement).*

**6. How should I do the grouping of rare diseases to be proposed in my RDCRC application?**

*The applicant must describe the group of (at least 3) related rare diseases to be included and the rationale for this grouping, as well as the relevant expertise available in the proposed RDCRC.*

*The focus of each RDCRC can be on particular defects, e.g., lysosomal storage diseases, amino acid metabolism defects; particular organ systems, e.g., primary immune deficiencies, mental retardation syndromes; or other groupings. Since rare diseases are diverse, the nature of clinical research that is feasible varies.*

**7. Is it okay to submit a RDCRC application on any rare disease?**

*The RFA allows RDCRC applications for all rare diseases research relevant to the mission of the NIH ICs listed on the RFA. Prospective applicants are urged to consult with the program staff of the NIH early in the preparation of the application Please see the Areas of Research Interest for participating NIH ICs at http://grants.nih.gov/grants/guide/contacts/rfa-od-08-*

**8. Is submission of an application from a foreign institution (non-USA institution) allowed?**

*Foreign institutions may not submit applications, but collaborating co-investigators and clinical research sites at foreign institutions are allowed as components of an RDCRC application. Consortium agreements with foreign institutions can be proposed as long as the appropriate federal-wide assurances for the protection of human subjects are in place (see http://www.hhs.gov/ohrp/) and the activities at the foreign site(s) do not exceed 49 percent of the direct costs of the overall budget.*

**9. Is there a limit (maximum/minimum) on numbers of centers for the RDCRC?**

*We have not included a limit on minimum or maximum number of centers in the RFA. In the current RDCRN clinical projects involve multi-site studies. There are on average 7 performance sites per consortium (ranging from approximately 4-11).*

**10. For RDCRC what is the cap on budget request? Is it $1.25M Total Cost for five years?**

*The budget request should be limited to $1.25 million in total cost (i.e., direct cost plus Facilities and Administrative (F&A) costs) per year. An applicant must request a project period for five years. See RFA for details.*

**11. Are Multiple PIs allowed?**

T*his RFA does not use "Multiple-PI" mechanism. There should be only one PI for the proposed RDCRC cooperative agreement application. However, a Co-PI must be identified to share responsibilities with the PI of the consortium.*

**12. Can the Co-PI be from a different institution from the PI's institution?**

Yes, as long as *the Co-PI can assist the RDCRC Director (PI of the application) with the day-to-day administrative details, program coordination, and planning and evaluation of the program. The Co-PI would be in charge to perform required functions of the RDCRC Director in the absence of the RDCRC Director***.**

**13. Are all components mentioned in the RFA required?**

Yes. *An RDCRC must include the following:*

1. *Clinical Research Projects for Observational/Longitudinal Studies and/or Clinical trials At least two projects are required, one of which must be a longitudinal study*
2. *Pilot/Demonstration Projects (At least one project is required)*
3. *Training (career development) Component*
4. *Website resource for education and research in rare diseases*
5. *RDCRC Administrative Unit*
6. *Collaboration with Patient Advocacy Group*

**14. A Web site is a required component of the consortium application. Are we supposed to work with the NIH Office of Rare Diseases before we apply or after? What web resources should be included?**

*After funding, an RDCRC will work cooperatively with DMCC to develop the web site resource and provide content related to the RDCRC specific rare diseases. You will not work with the Office of Rare Diseases for RDCRC web resources.*

*In your RDCRC application describe resources to be included in your RDCRC web site for education and research for your proposed rare diseases (you should include links or materials for lay public, patients, basic and clinical researchers, and clinicians). Some examples include: contacts for animal models**;** availability of tissue, serum, specimens, DNA, etc; antibodies and research reagents; genetic resources; registries; education materials; and/or diagnostic flow charts.*

**15. Where can we find information on currently active RDCRCs Web sites and RDCRN?**

*Here is the link to the current RDCRN http://rarediseasesnetwork.epi.usf.edu/. Please check individual RDCRCs to get an idea.*

**16. Who will support the web sites for the RDCRCS?**

*The Data Management Coordinating Center (DMCC) will work with all RDCRCs to develop and support the web-sites for communicating disease information .*

**17. May a not-for-profit disease advocacy organization be the lead entity submitting an application, and thus be eligible for an award?**

*The Advocacy Group is eligible to apply for an RDCRC with an appropriately trained PI, research collaborating institutions, and related rare diseases as required by the RFA. Historically, we do not know how they have fared in other RFA/FOA reviews.*

**18. Is individual DMCC required for each RDCRC's? Will each RDCRC also propose for its own DMCC?**

*No. Each RDCRC will use the Data Management Coordinating Center (DMCC) supported by the Rare Diseases Clinical Research Network (RDCRN). The RDCRN will consist of all funded RDCRCs and one DMCC. The individual RDCRC will not be responsible for creating/developing a data center for their longitudinal studies and pilot projects. The DMCC will serve as their resource.*

**19. If an applicant proposes a clinical study in the RDCRC application, should the design (and statistics) will have already been worked out by the RDCRC team? OR, is the RDCRC supposed to work with the DMCC before submitting an application and propose a design already "approved" by the DMCC?**

As mentioned in the RDCRC RFA, for any proposed clinical study in an RDCRC application, the design and statistics will have to be worked out by RDCRC team and statistician.

**20. We have already developed a database, data collection mechanisms and a relationship with biostatisticians at our coordinating center. Can we continue to use our own database coordinating center and transmit files to the DMCC?**

*To be a part of Rare Diseases Clinical Research Network (RDCRN) you need to use the DMCC. All RDCRCs need to work in coordination with the DMCC. The interaction of the RDCRC will be more than simple transfer of the files from a data base to DMCC. RDCRC and DMCC will need to work together from the beginning to assure compatibility etc. The data collection process by DMCC doesn't preclude sending the data to your own group.*

**21. Can the RFA funds be used to support a tissue repository of affected and unaffected populations for future biomarker studies? Or only those biologic analyses proposed as a clinical or pilot project?**

*Funding for the sole purposes of creation of tissue repositories is not part of this RFA. However, tissues can be collected as part of a proposed study (longitudinal study or a pilot study) with in the five years of grant period,.*

**22. What are the rules for interaction with cooperative group trials? We would like to propose addition of biomarker and response assessment studies on top of a clinical trial which will be conducted through a cooperative group.**

*Interactions with cooperative group trials are appropriate.*

**23. Can 2 Institutes/Centers (ICs) from NIH provide scientific oversight?**

*One IC will take the lead for each RDCRC application. The ICs will decide who will take the lead. However, because of overlapping interests, more than one IC can have involvement with a RDCRC. Contact the program staff from the participating ICs. To contact a Program staff from a participating NIH IC, see http://grants.nih.gov/grants/guide/contacts/rfa-od-08-001_contacts.htm*

**24. Would a proposal for a clinical trial aimed at prevention of a group of related rare diseases as one of the clinical studies be responsive to this RFA?**

*Yes.*

**25. Could an NIH intramural program participate in a RDCRC and receive funds from the RFA? Are there special instructions to allow an application which includes intramural scientists?**

*NIH intramural scientists can not use RFA set aside money, but can be part of the Network as collaborators of any RDCRC.*

*No special instructions were included in RDCRC RFA to allow an application from an intramural program. Though the intramural scientists can not use funds from the RFA, they can participate fully in the protocol and provide whatever additional resources to the project. If one of the institution has an investigator or other staff member listed on their personnel for the RDCRC application, that individual can spend some time at the NIH intramural site, as part of their training (if part of a training component) or as part of a specific aim to accomplish a task for the RDCRC. We have had collaborations like this in the current RDCRN that have benefited both a project and training of new investigators in rare diseases clinical research.*

**26. Required components of an RDCRC cooperative agreement application include a minimum of two clinical research projects (at least one of them must be a longitudinal study). Does this imply that more than one clinical study is required OR would multiple CLINICAL SITES qualify?**

*Multiple clinical sites do not qualify as multiple clinical projects. Yes, a minimum of two clinical projects are required.*

**27. For the training component, is the requirement to set aside $50K per year to support at least two trainees over the 5 year funding period?**

*That is correct. At least two trainees over 5 years must be supported and trained. The RDCRC should spend $250,000 over 5 years for training.*

**28. For a training (career development) component, can a clinical fellow be supported?**

*Yes. The purpose of training component is to provide training to new investigators in clinical research of rare diseases. Please see the RFA for details.*

**29. At least one pilot/demonstration project is required--not sure what this means?**

*An applicant must propose a plan for (at least one) pilot project (short term project for a period of no more than two years). See RFA for details.*

**30. We are planning a newborn screening trial for the Pilot/Demonstration project. Does this single project have to run the life of the cooperative agreement award or can we propose other pilots once the newborn screening pilot is complete? Can we have more than one pilot/demonstration study?**

*As mentioned in the RFA, the pilot projects are for 2 years or less. A single project can not run the life of the RDCRC. You will need to propose other pilots projects.*

**31. What is the difference in support for pilot and clinical projects?**

*For Clinical Research Projects for Observational/Longitudinal Studies or Clinical Trials (full research projects), the plan has to be proposed for five years. In contrast, pilot projects are for 2 years or less. See RFA for details.*

**32. If there is overlap between the rare diseases studied by two funded RDCRCs, is there a mechanism whereby registry data can be shared between RDCRCs?**

*If there is overlap between the rare diseases studied by two funded Rare Diseases Clinical Research Consortia (RDCRCs), patient registry data can be shared between RDCRCs.*

**33. Can we initially focus on one disease and later on other diseases?**

*The RDCRC RFA requires that you focus on a group of at least 3 diseases, but you may focus your initial efforts on 1 disease. Do include a timeline in the application to describe when clinical studies on other diseases will be initiated.*

**34. For longitudinal/observational studies,** will **a long term follow-up study on rare diseases and a prospective study that would focus more on biologic markers etc as well as characterizing genotype/phenotype would be appropriate for RDCRC?**

Yes.

**35. The PI of the RDCRC who must commit 2 month/year (~17%time) to the cooperative agreement award does not have to be a Project Leader of any project within the RDCRC. But, I'm assuming that a Project Leader of a clinical project must commit 10% time to the project/grant, correct?**

*That is correct. The PI of the RDCRC cooperative agreement must commit 2 month/year. If he/she is also a leader of a clinical project-- then he/she must commit an additional 10% of the time for that project.*

**36. I have a budget related question for the participating sites. Whom should I contact?**

*Contact the relevant Program contact and Grants Management contact listed in http://grants.nih.gov/grants/guide/contacts/rfa-od-08-001_contacts.htm*

**37. Whom should I contact to discuss the scope of my proposed application and its responsiveness to the RFA?**

*Contact the relevant Program contact listed in http://grants.nih.gov/grants/guide/contacts/rfa-od-08-001_contacts.htm. General questions about the RFA can be answered by the Office of Rare Diseases (ord@od.nih.gov).*