

Frequently Asked Questions for ORDR Challenge Grants

What is a rare disease?

A rare disease is defined as a disease or disorder having a prevalence of fewer than 200,000 persons living with the condition in the US at this time. When responding with an application to any of the Challenge Grant topic areas of the Office of Rare Diseases Research (ORDR), please provide your best prevalence estimate for the disease(s) under study and references to the extent possible.

If you have a question about a specific rare disease, please contact Henrietta Hyatt-Knorr at hh70f@nih.gov.

What is the intent of 15-OD (ORDR)-101*, Pilot projects for prevention, early detection and treatment of rare diseases?

The intent of this topic included in Challenge Grant RFA is to design research projects to provide preliminary results to demonstrate feasibility of novel approaches to rare diseases. Some examples of approaches are: identification of molecular targets for rare diseases; development of models (vertebrate, invertebrate, computational); development of micro arrays and tissue micro arrays which are applicable to screening or detection of rare diseases; development of tools for drug discovery (e.g. development of assays for screening compounds); and clinical trials. Clinical trials can be proposed, but it is limited to pilot studies (Phase I, Phase I/II) which can be completed within two years.

If you have further questions about the pilot project topic area, please contact Dr. Rashmi Gopal-Srivastava at gopalr@mail.nih.gov.

What is the intent of 03-OD (ORDR)-101*, Validating biomarkers for functional outcomes in rare diseases?

The intent of this topic area is to establish validated biomarkers to clinical functional outcomes that could then be used in clinical trials for rare diseases. Validated biomarkers are needed for efficient clinical trials and to enhance pharmaceutical interest in therapeutic development. The biomarker can be any measure (as examples: molecular, physiologic, computational, imaging, etc.) used as a sign/surrogate that a given therapy would express the functional outcome. Proposals can include both discovery and validation, but the validation would need to be part of the proposal to be responsive. The expert consultative group mentioned in the original description of this topic area is not a feature of the initiative.

If you have further questions about biomarkers for rare diseases, please contact Dr. Giovanna Spinella at spinellg@od.nih.gov.

What is the intent of 07-OD (ORDR)-101*, Library of standardized patient registry questions?

The intent of this initiative is to develop standardized questions and data elements that can be used for developing new rare diseases patient registries and for revising existing ones. The library would contain standardized questions that can be used across many different rare disease registries. The Library questions would maintain consistent terminology and vocabulary across the questions to be utilized by rare disease patient registries to facilitate the most reliable and consistent information. The library should have questions directed to the physician/researcher as well as to the individual with the rare disease (patient). The level of the questions should be appropriate for all and in more than one language. A standardized library of data elements will enable cross-indication analyses of patient populations, facilitate collaboration and generate more meaningful results for the individuals with a rare disease (patients) and the physicians/researchers. In addition, a library of standardized registry questions should speed the development and deployment of patient registries and allow registries to exchange and aggregate patient registry data.

The applicants are expected to collaborate with rare disease registries to develop specific questions that may be relevant and unique for a single specific registry. Applicants should use a format (e.g., XML-based applications) that will allow sharing electronic information between different registries. Applicants should demonstrate collaborations with some rare disease communities and propose plans to incorporate this library into some registries in the second year of funding. Applicants responding to this topic area should put a statement of willingness to collaborate with the investigators who receive the award of the rare diseases patient registry topic area: 07-OD (ORDR)-102. Questions developed for the library should be responsive to the federal regulations for the protection of human subjects (45 CFR part 46), as well as to HIPAA and GINA

If you have further questions about registry question libraries, please contact Dr. Yaffa Rubinstein at rubinsty@od.nih.gov.

What is the intent of 07-OD (ORDR)-102*, Rare disease genetic patient registry?

The intent of this topic area is to develop a genetic registry infrastructure or platform that can support any rare genetic disease. The intent is to create the capacity for many rare diseases to utilize the platform and not to develop disease-specific registries. Disease specific experts and advocacy organizations will be able to design the rare disease specific items for their registry utilizing the infrastructure/platform support. The platform could hold as many rare disease registries as are willing to participate. This platform/infrastructure would be a center, or a hub, with different modules, one of which would direct clinicians and patients to the specific rare disease registry of interest. Patients could search for suitable clinical trials, and clinicians/investigators could look for suitable patient populations for specific clinical trials. Although, in the long run, it would be desirable to link the platform to biorepositories, the intent of this initiative is not to support existing biorepositories for rare diseases, nor to support the establishment of new ones. In addition to platform development, applicants should demonstrate collaborations with some rare diseases community as reasonable for the funds available for this initiative and propose plans to initiate some registries in the second year of funding. Applicants

should have excellent IT support and comply with HIPAA, IRB, GINA and all other federal regulations as stipulated in the Code of Federal Regulations (CFR) protection of human subjects, part 46.

If you have further questions about genetic registries for rare diseases, please contact Dr. Yaffa Rubinstein at rubinsty@od.nih.gov.

What is the intent of 15-OD (ORDR)-102*, Collaborative translational research platform for rare diseases?

The intent of this topic area is to foster the development of the infrastructure necessary to support collaborative teams of researchers working at disparate institutions so that they can plug into the developed platform and work collaboratively. While we won't rule out funding a specific group looking to work collaboratively within the confines of a developed infrastructure, the emphasis is on the infrastructure and not on the collaborative group.

If you have further questions about collaborative research platforms, please contact Dr. David Eckstein at eckstein@od.nih.gov.

Will a New PI or an Early Stage Investigator applying for a challenge grant award lose his/her status as a New PI?

New PIs and Early Stage Investigators (ESIs) are encouraged to apply for Recovery Act Challenge Grants in Health and Science Research. Because the awards made under this program are substantial competing NIH research grants, **recipients will not be considered New PIs or ESIs when they apply for NIH research grants in the future.** More information can be found at http://grants.nih.gov/grants/new_investigators/index.htm.

Will a New PI or an Early Stage Investigator listed as a Co-PI in a challenge grant award lose his/her status as a New PI?

No. NIH does not use the term co-Principal Investigator, so using this designation in your application will not affect your status as a New PI or Early Stage Investigator. More information can be found at http://grants.nih.gov/grants/new_investigators/index.htm.

Will the Office of Rare Diseases Research be the funding office for my research application?

The NIH Office of Rare Diseases Research does not have grant-making authority. All applications sent in response to any of the OD-ORDR Challenge Grant topic areas will be assigned to the appropriate NIH institute. Grants management and program oversight will be handled by that institute.