

**Lysosomal Disease Network (LDN) Request for Proposals (RFP)
(for studies aiming to participate in the competitive re-application to the
National Institutes of Health “Rare Diseases Clinical Research Network”
program)**

Key Dates:

*All correspondence and draft applications are to be submitted by email to
info@lysosomaldiseasetwork.org*

January 1, 2018: **Letter of Intent, with Outline** (a brief outline addressing proposed elements of the standard NIH PHS 398 form)

January 15, 2018: [Executive Committee](#) audit completion date; **Response to Investigators**

January 30, 2018: **Completed OUTLINE of Application** to LDN office

February 1, 2018: Completed outlines of applications to LDN Reviewers

February 9, 2018: “Pitch Meeting”: Each applicant’s face-to-face oral presentation to the LDN [Expert Advisory Committee](#) at Manchester Hyatt, San Diego, CA, USA:

6 Minutes presentation and 4 minutes Q&A

8:00 AM – 2:00 PM

3:00 PM – 5:00 PM – Discussion (closed session)

May 15, 2018: Final proposal in NIH format due at LDN Office by email to:
info@lysosomaldiseasetwork.org

**Final applications will use standard NIH PHS 398 forms available for download at:
<https://grants.nih.gov/grants/funding/phs398/phs398.html>**

July 31, 2018: Final LDN application [components in place](#)

September 15, 2018: LDN application submission to NIH

Section I. Funding Opportunity Description

Funding is for participation in the Lysosomal Disease Network, requesting new and revised proposals to be funded by the NIH Rare Diseases Clinical Research Network (“RDCRN4”) consortium cooperative agreement grant.

Section II. Award Information

Funding Instrument:

This is a continuation of the Lysosomal Disease Network U54 consortium cooperative

agreement grant, supporting rare disease research and administered by the Rare Diseases Clinical Research Network (RDCRN).

Application types:

New and continuing longitudinal studies, new pilot studies for lysosomal disease, with an emphasis on patient advocate group collaboration.

Anticipated funding period:

September 1st, 2019 – August 31st, 2024

Section III. Eligibility Information

Eligible Applicants:

Investigators working at a clinical care and research center with a lysosomal disease population and able to receive federal funds.

Cost Sharing or Matching

Applicants are required to provide information about additional funds used to support this work such as NIH, industry, institutional, foundation, and patient advocate group support.

Section IV. Application and Submission Information

Address to Request Application Information

Please email application questions and documents to: info@lysosomaldiseasetwork.org

Although this preliminary application to the LDN is intended to qualify the research project to join the Lysosomal Disease Network, the organization must follow standard NIH formatting as outlined below:

Requirements:

- 1) Title of proposal
- 2) P.I. – address and site
- 3) Other sites, subcontracts
- 4) Research content (3-4 pages)
 - a. Specific Aims
 - b. Research Plan
 - i. Significance
 - ii. Approach
 - c. Literature cited (no page limit)
 - d. Human subjects (provide evidence that human subjects are available)
- 5) Other intended sources of funding:
 - a. Industry grants/matching funds – description of plan for integration into LDN, description of pharma products relating to research, and to continued funding of current related projects
 - b. NIH
 - c. Patient advocate groups/foundations - description of Coalition of Patient Advocate Groups (CPAG) proposal

- 6) Status of IRB review and approval of applicant's proposed research project: please state whether or not you are continuing an existing IRB approval
- 7) Indication of institutional support
- 8) Letters of support

Section V. Application Review Information

Criteria for selection

Application must specify:

- 1) Clinical significance: how will this change the lives of patients?
- 2) Confirmed cross-collaboration with LDN other sites in the USA (and outside of the United States).
- 3) Institutional support.
- 4) Other support (co-funding from patient advocate groups, foundations, industry, other NIH grants, etc.) in addition to LDN.
- 5) Resources and staff appropriate for longitudinal study.
- 6) Comprehensive research plan.

Review and Selection Process

- 1) January 1, 2018: Initial proposal due date.
- 2) January 5th, 2018: Completed application outlines to study section.
- 3) Preliminary scoring grid generated.
- 4) February 9th: Presentation at Pitch Meeting (February 9, 2018, San Diego, CA, USA) - 6 minutes presentation and 4 minutes Q&A, during LDN Meeting.
- 5) Expert Advisory Committee discussion and review of applications.
- 6) Final scores determined.
- 7) List of final applicants posted.
- 8) Announcement of selection and Award Dates

Section VI. Post-Selection NIH Application Assembly

- 1) May 15th, 2018: Final proposal in NIH format due at LDN Office.
- 2) July 31st, 2018: Final application components in place.

Applications format will use standard NIH PHS 398 forms available for download at:
<https://grants.nih.gov/grants/funding/phs398/phs398.html>

Section VII. Review Criteria

Applications will be reviewed on a modified version of the standard NIH Study Section review criteria (modified to be consistent with the goals of the NIH Rare Diseases Clinical Research Network (RDCRN)):

1. Significance.

Does the project address an important problem or a critical barrier to progress in the field? If the aims of the project are achieved, how will scientific knowledge, technical capability, and/or clinical practice be improved? How will successful completion of the aims change the concepts, methods, technologies, treatments, services, or preventative interventions that drive this field?

2. Investigator(s).

Are the PD/PIs, collaborators, and other researchers well suited to the project? If Early Stage Investigators or those in the early stages of independent careers, do they have appropriate experience and training? If established, have they demonstrated an ongoing record of accomplishments that have advanced their field(s)? If the project is collaborative or multi-PD/PI, do the investigators have complementary and integrated expertise; are their leadership approach, governance and organizational structure appropriate for the project?

3. Innovation.

Does the application challenge and seek to shift current research or clinical practice paradigms by utilizing novel theoretical concepts, approaches or methodologies, instrumentation, or interventions? Are the concepts, approaches or methodologies, instrumentation, or interventions novel to one field of research or novel in a broad sense? Is a refinement, improvement, or new application of theoretical concepts, approaches or methodologies, instrumentation, or interventions proposed?

4. Approach.

Is the overall strategy, methodology, and analyses well-reasoned and appropriate to accomplish the specific aims of the project? Are potential problems, alternative strategies, and benchmarks for success presented? If the project is in the early stages of development, will the strategy establish feasibility and will particularly risky aspects be managed?

If the project involves human subjects and/or NIH-defined clinical research, are the plans to address 1) the protection of human subjects from research risks, and 2) the inclusion (or exclusion) of individuals on the basis of sex/gender, race, and ethnicity, as well as the inclusion (exclusion) of children, justified in terms of the scientific goals and research strategy proposed?

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If the project involves clinical research, are the plans for 1) protection of human subjects from research risks, and 2) inclusion of minorities and members of both sexes/genders, as well as the inclusion of children, justified in terms of the scientific goals and research strategy proposed?

Does the application provide sufficient evidence that the project can stimulate the interests of students so that they consider a career in the biomedical or behavioral sciences?

5. Environment.

Will the scientific environment in which the work will be done contribute to the probability of success? Are the institutional support, equipment and other physical resources available to the investigators adequate for the project proposed? Will the project benefit from unique features of the scientific environment, subject populations, or collaborative arrangements?

Are the institutional support, equipment and other physical resources available to the investigators adequate for the project proposed? Does the application demonstrate the likely availability of well-qualified students to participate in the research project? Does the application provide sufficient evidence that students have in the past or are likely to pursue careers in the biomedical or behavioral sciences?

Protections for Human Subjects. For research that involves human subjects but does not involve one of the six categories of research that are exempt under 45 CFR Part 46, the committee will evaluate the justification for involvement of human subjects and the proposed protections from research risk relating to their participation according to the following five review criteria: 1) risk to subjects, 2) adequacy of protection against risks, 3) potential benefits to the subjects and others, 4) importance of the knowledge to be gained, and 5) data and safety monitoring for clinical trials. For research that involves human subjects and meets the criteria for one or more of the six categories of research that are exempt under 45 CFR Part 46, the committee will evaluate: 1) the justification for the exemption, 2) human subjects involvement and characteristics, and 3) sources of materials. For additional information, see the Guidelines for the Review of Human Subjects.

Inclusion of Women, Minorities, and Children. When the proposed project involves human subjects and/or NIH-defined clinical research, the committee will evaluate the proposed plans for the inclusion (or exclusion) of individuals on the basis of sex/gender, race, and ethnicity, as well as the inclusion (or exclusion) of children to determine if it is justified in terms of the scientific goals and research strategy proposed. For additional information, see the Guidelines for the Review of Inclusion in Clinical Research.

Vertebrate Animals. The committee will evaluate the involvement of live vertebrate animals as part of the scientific assessment according to the following five points: 1) proposed use of the animals, and species, strains, ages, sex, and numbers to be used; 2) justifications for the use of animals and for the appropriateness of the species and numbers proposed; 3) adequacy of veterinary care; 4) procedures for limiting discomfort, distress, pain and injury to that which is unavoidable in the conduct of scientifically sound research including the use of analgesic, anesthetic, and tranquilizing drugs and/or comfortable restraining devices; and 5) methods of euthanasia and reason for selection if not consistent with the AVMA Guidelines on Euthanasia. For additional information on review of the Vertebrate Animals section, please refer to the Worksheet for Review of the Vertebrate Animal Section.

Biohazards. Reviewers will assess whether materials or procedures proposed are potentially hazardous to research personnel and/or the environment, and if needed, determine whether adequate protection is proposed.

Resubmission. For Resubmissions, the committee will evaluate the application as now presented, taking into consideration the responses to comments from the previous scientific review group and changes made to the project.

Renewal. For Renewals, the committee will consider the progress made in the last funding period.

Applications from Foreign Organizations. Reviewers will assess whether the project presents special opportunities for furthering research programs through the use of unusual talent, resources, populations, or environmental conditions that exist in other countries and either are not readily available in the United States or augment existing U.S. resources.

Select Agent Research. Reviewers will assess the information provided in this section of the application, including 1) the Select Agent(s) to be used in the proposed research, 2) the registration status of all entities where Select Agent(s) will be used, 3) the procedures that will be used to monitor possession use and transfer of Select Agent(s), and 4) plans for appropriate biosafety, biocontainment, and security of the Select Agent(s).

Resource Sharing Plans. Reviewers will comment on whether the following Resource Sharing Plans, or the rationale for not sharing the following types of resources, are reasonable: 1) Data Sharing Plan; 2) Sharing Model Organisms; and 3) Genome Wide Association Studies (GWAS)/Genomic Data Sharing Plan.

Budget and Period of Support. Reviewers will consider whether the budget and the requested period of support are fully justified and reasonable in relation to the proposed research.