

## REQUEST FOR PROPOSALS

### The National Organization for Rare Disorders (NORD)'s Jayne Holtzer Rare Disease Research Grants Program

*Announces a research grant opportunity for one grant  
up to \$40,000 US for*

## Levy-Yeboa Syndrome (LYS), an *STXBP3*-associated disease

**DEADLINE FOR INITIAL APPLICATIONS: November 14, 2022 (11:59 pm PT)**

NORD, with fundraising by the Maxwell Family is accepting applications for one grant, up to \$40,000 US, for scientific and/or clinical research studies related to Levy-Yeboa syndrome (LYS).

Levy-Yeboa syndrome (LYS) is a multi-system disorder involving signs of musculoskeletal involvement such as low muscle tone and stiffening of the joints of the arms and legs (contractions), hearing loss (neuronal deafness), intense burn-like eruptions of the skin containing clear fluid (bullous eruptions) and dangerous gastrointestinal distress involving substantial loss of fluids (secretory diarrhea), among other issues (Levy J, 2003). Most, if not all, of these signs are apparent at, or within a few months of, birth. A recent report suggests that LYS may be part of the disease spectrum of very early onset inflammatory bowel disease, that are also associated with hearing loss and immune dysregulation. These particular cases have been found to have an autosomal recessive genetic condition caused by pathogenic variants (mutations) in the Syntaxin Binding Protein 3, *STXBP3* gene. (Levy J, 2003; Ouahed J, 2021).

#### References

- Levy J, C. W.-Y. (2003). Congenital myopathy, recurrent secretory diarrhea, bullous eruption of skin, microcephaly, and deafness: a new genetic syndrome? *Am J Med Genet A.*, 20-25.
- Ouahed J, K. J.-Y. (2021). Variants in *STXBP3* are Associated with Very Early Onset Inflammatory Bowel Disease, Bilateral Sensorineural Hearing Loss and Immune Dysregulation. *J Crohns Colitis*, 1908-19191.

#### Research Objectives

The NORD Rare Disease Research Grant Program was established in 1989 to encourage meritorious scientific and clinical studies designed to improve the diagnosis, understanding of underlying disease mechanisms, or therapy of specific rare diseases. studies related to Levy-Yeboa syndrome (LYS) will be considered. Grants will be awarded to qualified researchers to initiate small scientific research studies or clinical trials, the results of which could be used to obtain funding from the NIH, FDA, or other funding agencies, or to attract a corporate sponsor. Ideally, the proposed research should have the potential to lead to the ultimate development of a new or better therapy. Evaluation of proposals will include careful consideration of protocol design, objectiveness of parameters measured, and statistical evaluation proposed.

**NORD REQUEST FOR PROPOSALS: Levy-Yeboa syndrome (LYS)**

T 203-744-0100 F 203-263-9938 Email [research-programs@rarediseases.org](mailto:research-programs@rarediseases.org)

## **About NORD**

The National Organization for Rare Disorders (NORD) is the leading independent advocacy organization representing all patients and families affected by rare diseases. NORD is committed to the identification, treatment and cure of the more than 7,000 rare diseases, of which approximately 90 percent are still without an FDA-approved treatment or therapy. Rare diseases affect 25-30 million Americans. More than half of those affected are children. NORD began as a small group of patient advocates that formed a coalition to unify and mobilize support to pass the Orphan Drug Act of 1983. For more than 35 years, NORD has led the way in voicing the needs of the rare disease community, driving supportive policies and education, advancing medical research and providing patient and family services for those who need them most. NORD is made strong together with over 300 disease-specific member organizations and their communities and collaborates with many other organizations on specific causes of importance to the rare disease patient community.

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## APPLICATION PROCESS OVERVIEW

### INITIAL APPLICATIONS

- Requests for proposals will be released by NORD.
- Letters of intent will be due November 14, 2022.
- Letters of intent will be reviewed by NORD reviewers.
- Requests for full proposals will be issued via email in mid-January 2023.

### FULL PROPOSALS

- Full proposal invitations will be issued via email.
- Application requirements for full proposals will accompany these invitations.

### AWARDING OF GRANT

- Award announcements will be made via email and posted on NORD's website in May 2023.
- Funding will begin after all necessary documents (e.g., IRB forms, patient consent forms, signed grant agreements) have been received by NORD.

### FURTHER INFORMATION

- If the study involves human or animal subjects, copies of governance documents will be required from each site involved in the study before payment can be issued.
- Clinical drug trials must meet requirements established by the U.S. Food & Drug Administration (FDA).
- Duplicate/overlapping funds from any other private or public source are not to be used.
- All applications determined to have scientific merit will be considered, however before an award is issued compliance with international funding regulations must be confirmed when applicable.

## INITIAL APPLICATION

Interested applicants should submit a completed application and letter of intent electronically to [research-programs@rarediseases.org](mailto:research-programs@rarediseases.org) with "NORD Letter of Intent" as the subject line. Applicants can complete the requested information directly in the document provided below and/or merge any additional required documents into a single PDF file. Incomplete applications may not be considered. All applications must be received by **November 14, 2022 (11:59 pm PT)**.

| REQUIRED ELEMENTS CHECKLIST               | PAGE | ✓ |
|---|------|---|
| Application summary                       | 4    |   |
| Letter of Intent (Maximum Length 2 Pages) | 5    |   |
| Biographical sketch                       | 6    |   |
| List of co-investigators, if applicable   | 7    |   |
| Budget outline                            | 7    |   |
| OPTIONAL ELEMENTS                         | PAGE | ✓ |
| Reviewer information                      | 8    |   |
| Letter of support                         | 8    |   |

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## APPLICATION SUMMARY

Deadline for Initial Applications: November 14, 2022 (11:59 pm PT)

| PRINCIPAL INVESTIGATOR INFORMATION                                   |  |
|--|--|
| Name   |  |
| Position/Title   |  |
| Email  |  |
| Mailing Address  |  |
| Telephone  |  |
| PROPOSAL INFORMATION   |  |
| Project Title  |  |
| Project Term   | 2 YEARS  |
| Funding Amount Requested (\$ US)<br><i>not to exceed \$40,000 US</i> |  |
| Institution(s) where research will be conducted                      |  |
| City, State/Province, Country of Institution(s)                      |  |
| Will research involve human subjects?                                | <input type="checkbox"/> YES<br><input type="checkbox"/> NO  |
| Will research involve animals?                                       | <input type="checkbox"/> YES<br><input type="checkbox"/> NO  |
| How did you hear about this RFP?                                     | <input type="checkbox"/> NORD <input type="checkbox"/> Academic Organization<br><input type="checkbox"/> Rare Disease Organization <input type="checkbox"/> Referral from Colleague<br><input type="checkbox"/> Professional Organization <input type="checkbox"/> Other (please specify)          |
| Please be as specific as possible:                                   |  |
| Did you hear about this RFP via:                                     | <input type="checkbox"/> NORD Website <input type="checkbox"/> Email<br>Google <input type="checkbox"/> Medical/Research Publication<br>▪ Social Media <input type="checkbox"/> Rare Action Network<br>(Facebook, Twitter, LinkedIn, Instagram)<br><input type="checkbox"/> Other (please specify) |
| Please be as specific as possible:                                   |  |

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## LETTER OF INTENT

Please provide a Letter of Intent addressing the following elements of your research. Applicants may use the form below or attach a separate document to address the following details.

| <b>Letter of Intent (Not to exceed 2 pages)</b>  |  |
|--|--|
| Summary Statement  |  |
| Statement of Need <ul style="list-style-type: none"> <li>• What issue will the research address</li> <li>• Significance of the work</li> <li>• Why you have chosen this issue</li> <li>• Who will benefit from this research</li> <li>• Why this funding is essential</li> </ul> |  |
| Project Activity <ul style="list-style-type: none"> <li>• Overview of research activities</li> <li>• Why this approach is novel</li> <li>• How the research builds upon and differs from previous research (if applicable)</li> </ul>  |  |
| Credentials <ul style="list-style-type: none"> <li>• Why your institution/program is best equipped to do this research</li> </ul>  |  |
| Closing  |  |
| Principal Investigator Signature<br><b>REQUIRED</b>  |  |

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## BIOGRAPHICAL SKETCH

Please provide a biographical sketch and bibliography for the principal investigator. Applicant may use this form or the NIH Biosketch form. Please modify the form to include, when applicable, the following:

|   |                          |        |         |                |
|---|--------------------------|--------|---------|----------------|
| <b>Name</b><br>Position/Title   |                          |        |         |                |
| <b>Education/Training</b><br>Begin with baccalaureate or other initial professional education and include postdoctoral training.  | Institution and Location | Degree | Year(s) | Field of Study |
|   |                          |        |         |                |
| <b>Research and Professional Experience</b><br>Concluding with present position, list in chronological order previous employment, experience, and honors. Include present membership on any advisory committee. |                          |        |         |                |
| <b>Honors and Awards</b>  |                          |        |         |                |

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## CO-INVESTIGATOR(S)

|                         |  |
|-------------------------|--|
| Name of Co-Investigator |  |
| Position/Title          |  |
| Institution             |  |
| Email                   |  |

|                         |  |
|-------------------------|--|
| Name of Co-Investigator |  |
| Position/Title          |  |
| Institution             |  |
| Email                   |  |

|                         |  |
|-------------------------|--|
| Name of Co-Investigator |  |
| Position/Title          |  |
| Institution             |  |
| Email                   |  |

## BUDGET

Please provide a brief budget outline describing how the funding will be used. Do not include PI salary, overhead, or indirect costs. Funding can be used to cover expenses such as staff salary, technical assistance, supplies, and small equipment.

REVIEWER INFORMATION (OPTIONAL)

Please list up to five areas of scientific/medical expertise needed to review this application (optional). Do not list names of individuals.

- 1.
- 2.
- 3.
- 4.
- 5.

Please list below any individuals who should not review this application (optional).

| NAME | INSTITUTION | JUSTIFICATION |
|------|-------------|---------------|
|      |             |               |
|      |             |               |
|      |             |               |

LETTER OF SUPPORT (OPTIONAL)

Please include a letter of support for your research from an individual advocate (e.g., patient, care partner) or patient advocacy group in this disease space (optional, but encouraged).

To receive notification of future funding opportunities through NORD, sign up for NORD research news here: <https://rarediseases.org/communications-sign-up/>