



## RESEARCH GRANT POLICY

### 1. BACKGROUND

The National Organization for Rare Disorders (NORD), a 501(c)(3) organization, is a patient advocacy organization dedicated to individuals with rare diseases and the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services. NORD represents the estimated 25-30 million Americans with approximately 7,000 rare “orphan” diseases. Under the federal Orphan Drug Act of 1983, a rare disease is defined as a health condition that affects fewer than 200,000 people in the United States. Because each disease affects a small number of people, there is generally limited commercial interest in conducting research or developing new products to diagnose or treat these conditions. Moreover, with few funding sources available to support rare disease research, academic scientists may not have access to resources to support the conduct of novel rare disease research studies.

### 2. PURPOSE

NORD's Rare Disease Research Grant Program, which is focused on disease-specific funding, provides small seed grants to scientists and clinicians performing basic translational science, studying new treatments, or developing diagnostic tests for rare diseases. The goal of the small studies supported by NORD's research grants is to provide preliminary data that may one day lead to a treatment (drug, device, or medical food) for patients with rare disorders. Researchers can use the preliminary data funded by NORD's Rare Disease Research Grant Program to apply for larger multi-year government grants, or to attract a commercial sponsor who will manufacture the orphan product and get it approved for marketing by the U.S. Food & Drug Administration (FDA). NORD's research review guidelines follow those of the National Institutes of Health (NIH) and administrative costs are kept to a minimum.

### 3. DONATING FOR RARE DISEASE RESEARCH

Donors may target their donations to research on a specific disorder or support NORD's general research fund. NORD encourages potential donors interested in disease-specific research to conduct fundraising efforts so that sufficient funding is made available within a reasonable period of time to attract the highest quality research proposals. Medical research is costly, so the minimum amount of \$35,000 U.S. is required to fund one grant. When this target amount is reached, NORD begins the process of issuing a request for research proposals. When donors have given small donations restricted to research on specified diseases, several years of targeted fundraising efforts may be required in order to raise the fund balance to the minimum. Therefore, NORD's Board of Directors set forth the following guidelines for funds donated to a specific disease that do not reach the following amounts within the specified period of time:

- If the total of donated funds for research on a specific disease is less than \$1,000 U.S. after two years without significant donations, the fund will be transferred to NORD's general research fund.
- If the total of donated funds for research on a specific disease is more than \$1,000 U.S. but less than



\$5,000 U.S. after four years, the money will be transferred to research on related diseases. (e.g. neurological, metabolic, connective tissue diseases).

- If the total of donated funds for research on a specific disease is more than \$5,000 U.S. but less than \$10,000 U.S. after six years, the money will be transferred to research on related diseases.
- If the total of donated funds for research on a specific disease is more than \$10,000 U.S. but less than \$15,000 U.S. after eight years, the money will be transferred to research on related diseases.
- If the total of donated funds for research on a specific disease is less than \$25,000 U.S. in ten years, the money will be transferred to research on related diseases.

Exceptions can be made if NORD sees continued donor activity for a specific disease, indicating that there is a determined effort to reach the minimum \$35,000 U.S. goal in a reasonable period of time. In many instances, families will launch fundraising campaigns in their communities or via social media that raise several hundred dollars at a time. NORD encourages these activities and recognizes that it can take several years to reach the necessary sum required to issue a request for proposals (RFP). All research contributions are restricted by NORD and cannot be used for an alternate purpose without the donor's permission. Donations to NORD's research funds are tax deductible under IRS regulations. Interest cannot be accrued on gifts to research funds. [Donate to a research fund here.](#)

#### 4. COSTS

A minimum of \$35,000 U.S. is required before issuing an RFP. Out of each grant awarded between \$30,000 U.S. and \$50,000 U.S., NORD's grant management fee is only \$5,000 U.S. This administrative fee includes targeted outreach to qualified scientists and clinicians, marketing of available grant opportunities, content development for the RFP, management of applications, collaboration with NORD's Scientific and Medical Advisory Committee on the two-stage review of research proposals, consultation with NORD's legal team for contract negotiations, and oversight of the fund year over year. Sufficient funds to cover the administrative fee must be in place before an RFP is issued, but the administrative fee is not withdrawn until a grant has been accepted and awarded.

#### 5. GRANT CYCLES

NORD issues RFPs on a cyclical basis. Adequate funds must be in place before preparations to initiate an RFP begin. Therefore, to maximize the full benefits of international advertisements and wide circulation of the funding opportunity, donors should understand that it takes several months after an RFP is issued until the grant is awarded and actual research can begin. The following table depicts a representative cycle. For deadline information about open funding opportunities, please visit [Current Requests for Proposals.](#)

January - March	NORD prepares to issue RFPs.
April	NORD issues RFPs.
June	Deadline for preliminary proposals.



July - August	Peer review of preliminary proposals by Scientific and Medical Advisory Committee. Finalists invited to submit full proposals.
October	Deadline for full proposals from finalists.
November - December	Peer review of full proposals by Scientific and Medical Advisory Committee. Committee recommendations submitted to NORD Board of Directors for vote.
December	Awardees notified and announced.

**6. SELECTION OF GRANTEES**

Each initial request for proposals asks applicants to submit a brief description (abstract) of their proposed research project along with their professional qualifications and a draft budget. For each proposal, the principal investigator must have sufficient education and experience to demonstrate subject matter expertise. Strong preference is given to researchers holding a faculty-level appointment or senior scientific position at an academic or non-profit institution or foundation. Grants are available to researchers both within the United States and internationally.

Once all application materials have been submitted, NORD's Scientific and Medical Advisory Committee, composed of leading academic scientific experts, reviews the applications (peer review) and selects finalists. The finalists are invited to submit a full grant application describing their proposed project in detail. NORD's Scientific and Medical Advisory Committee then reviews the full grant applications and ranks them through a scoring system. NORD's grant review process is adapted from the peer review standards utilized by the National Institutes of Health. Scoring of grants is done individually by each medical expert and any reviewer with a conflict of interest is excused from voting on a specific proposal. Individual scores are added together to determine the final ranking of each proposal and the highest scoring grant applications are recommended for funding to NORD's Board of Directors, who approves the final grant awards. The grant selection process is unbiased and independent; awards are based solely on scientific merit. Only the highest-quality, highest-impact research projects will be funded. It is not possible for donors to signify how they want their donations spent (e.g. on a genetic test rather than development of a treatment), or which researcher should be funded.

After a grant is awarded, NORD monitors the progress of the research. Every six months, awarded investigators must submit financial and scientific progress reports, which are reviewed and approved by NORD's Scientific and Medical Advisory Committee. At the conclusion of a grant, any unused funds are returned to the corresponding disease-specific restricted research fund.

NORD is so grateful for the continued support and generous gifts that have enabled us to continue to promote critical foundational research for rare diseases, accelerating scientific discovery related to the identification, treatment, and cure of rare disorders. Please contact NORD's research team with any questions about this policy: [research@rarediseases.org](mailto:research@rarediseases.org)