

RESEARCH GRANT POLICY

The National Organization for Rare Disorders (NORD) is a non-profit voluntary health agency dedicated to the identification, treatment and cure of rare "orphan" diseases. NORD represents more than 25 million Americans with nearly 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 Americans. Because each disease affects a small number of people, there generally is little commercial interest in performing research or developing new products to diagnose or treat these diseases. Moreover, academic scientists believe it is easier for them to obtain government research grants for the study of more prevalent diseases, so they sometimes avoid applying for grants to study rare diseases.

PURPOSE

NORD's mission is to promote the diagnosis, treatment, and cure of rare disorders through programs of education, research, advocacy, and service to families and health professionals. NORD's *Research Grant Program*, which is focused on disease-specific grants, provides small "seed money" grants to academic scientists performing basic translational science or studying new treatments or 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 then use the preliminary data funded by NORD's 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 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.

DONATING FOR RARE DISEASE RESEARCH

Donors may target their donations to research on a **specific disorder**. NORD encourages potential donors interested in **disease-specific** research to conduct fundraising efforts so that a sufficient amount of money is made available, within a reasonable period of time, to attract the highest quality research proposals. Medical research is expensive so a minimum of \$35,000 is required to fund one grant. When this target amount is reached, NORD begins the process of issuing a request for research proposals.

In the past, when donors gave small sums restricted to research on specified diseases, the gifts languished unspent for several years because sums were too low to support medical research. Therefore, NORD's Board of Directors decided if the funds donated to a specific disease do not reach the following amounts within the specified period of time, the funds will be transferred to the following:

- If the total of donated funds for research on a specific disease is *less than \$1,000 after two years* without significant donations, the fund will be transferred to general research and related activities.
- If the total of donated funds for research on a specific disease is *more than \$1,000 but less than \$5,000 after four years*, the money will be transferred to research on related diseases. (e.g., neurological, metabolic, connective tissue diseases, etc.)
- If the total of donated funds for research on a specific disease is *more than \$5,000 but less than \$10,000 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 but less than \$15,000 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 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 goal, in a reasonable period of time. In many instances, families will launch fundraising campaigns in their communities 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 that triggers a request for research proposals. [Donate to a research fund here.](#)

COSTS

A minimum of \$35,000 is required to trigger a request for research proposals. Out of each grant awarded between \$30,000 and \$50,000, NORD uses only \$5,000 to cover the administrative expenses and direct costs of initiating a request for proposals (RFP). A search for researchers who have published journal articles about a specific disease in the last 2 - 5 years is conducted in order to reach the most expert scientists. NORD's research grant program is international. Our grants are available to researchers both within the United States and overseas.

Each research proposal is reviewed by NORD's Medical Advisory Committee, which recommends funding for the highest scored proposals. After the grant is awarded, NORD monitors the progress of the research, processing biannual reports to NORD's Medical Advisory Committee. Any restricted funds that are not spent

during a grant cycle are returned to the restricted research fund for that disease. Administrative fees are not taken until a grant has been awarded, regardless of the number of RFP's issued.

GRANT CYCLES

Requests for proposals (RFP's) are issued on a cyclical basis. Adequate funds must be in place before preparations to initiate a research grant 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 outline below depicts a typical cycle. For deadline information about open funding opportunities, please visit [Current Requests for Proposals](#).

October - January	NORD prepares to initiate Request For Proposals (RFP)
March	Issue RFP
April/May	Deadline for preliminary proposals. NORD Medical Advisory Committee narrows applicants and invites finalists to submit detailed proposals.
July/August	Deadline for full proposals from finalists. Peer review of final proposals.
September/October	Winners selected and awards announced.
November	Winners selected and awards announced.

SELECTION OF GRANTEES

The initial Request for Proposals asks scientists to submit a brief description (abstract) of their proposed research project along with their professional qualifications and a draft budget. Once that information is received, NORD's Medical Advisory Committee (composed of leading academic scientific experts) reviews all of the applications and selects finalists. The finalists are invited to submit a full grant application describing their proposed project in detail. NORD's Medical Advisory Committee then reviews the full grant applications (peer review) and ranks them through a scoring system. The highest scoring grant applications are recommended for funding to NORD's Board of Directors who votes on the final grant awards.

Depending upon the amount of money available for grants, one or more applications are chosen by the MAC for the grant award. Interim progress and budget reports are required every six months from the investigators, which are also reviewed and approved by NORD's Medical Advisory Committee. It is possible, however, that some researchers may require more or less time for completion of their research project.

NORD's grant review process is based on the same peer review system utilized by the National Institutes of Health (NIH). Scoring of grants is done individually by each medical expert, and scores are added together to determine the final ranking of each proposal. Any reviewer with a conflict of interest is excused from voting on a specific proposal. **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.** The grant selection process is unbiased and independent; awards are based solely on scientific merit. Only the best research projects will be funded.

By encouraging research applicants to compete against each other on an international basis, NORD's Research Grant Program funds only the best proposals that are most likely to result in research breakthroughs that can be applied to the diagnosis or treatment of people with rare disorders.

All research contributions are restricted by NORD and can be used for no other purpose without the donor's permission. Donations to NORD's Research Fund are tax deductible under IRS law. Interest cannot be accrued on gifts to the research Fund. Your gifts enable NORD to continue its vitally important research program aimed at promoting the diagnosis, new treatments, and cures for rare "orphan diseases."