



Request for Proposals

National Organization for Rare Disorders (NORD) with APS Type 1.Org

Announce

Research Seed Money Grant

For

APECED Syndrome/APS Type 1

Submission Deadline: May 9, 2008

NORD, in conjunction with the patient organization, APS Type 1.Org, is accepting applications for a two-year grant for the support of clinical research on APECED Syndrome/APS Type 1.

APS Type 1, also known as autoimmune polyglandular syndrome, type 1, is a very rare hereditary disorder that strikes children and adults younger than 35 years of age. It presents as a combination of hypoparathyroidism (below-normal functioning of the parathyroid glands) and adrenal gland insufficiency, accompanied by frequent fungal infections (candidiasis) of the mucous membranes and nails. There may also be T-cell abnormalities. The abnormally low hormone production caused by APS Type 1 can lead to life-threatening consequences. APS Type 1 appears to be caused by mutations of a single gene, called the AIRE (“autoimmune regulator”) gene, and is the result of autosomal recessive genetic transmission. Currently, treatment consists of careful monitoring and replacement of hormones.

Research Objectives

The purpose of the NORD Research Grant Program is to encourage meritorious clinical studies designed to improve the diagnosis or therapy of rare “orphan” diseases. Grants will be awarded to academic researchers to initiate small clinical trials, the results of which could be used to obtain funding from NIH, FDA or other funding agencies, or to attract a corporate sponsor. Procedures or proposed therapeutic trials may be new, based on recent biochemical or pharmacological evidence, or in preliminary states of clinical investigation. Evaluation of proposals will include careful consideration of protocol design, objectiveness of parameters measured, and statistical evaluation proposed. Protocols that will focus on early detection, diagnosis, or treatment (pharmacological, devices, surgery, or dietary) will be given priority.

APPLICATION PROCEDURE – Letter of Intent Deadline: May 9, 2008

- Complete pages 3 through 5 of this document;
- An initial letter of intent;
- A one-page abstract of the proposal;
- Brief budget outline;
- Cogent reasons why NORD funding is essential;
- A biographical sketch and bibliography (forms provided)
- **14 copies of above documents, in addition to the original, to be received by May 9, 2008**

FULL PROPOSALS

- Full proposal invitations will be announced, via e-mail, the week of June 25, 2008. Applications for full proposals will accompany these announcements.
- Full proposals must be received by NORD by August 25, 2008.

AWARDING OF GRANT

- Award announcements will be made on or about November 7, 2008, via e-mail.
- Funding will begin November, 2008, providing that all necessary documents (IRB forms, patient consent forms, signed application agreements) have been received by NORD.

FURTHER INFORMATION

- If the study involves human subjects, copies of the Informed Consent form and the Institutional Review Board committee report will be required from each site involved in the study, before payment can begin.
- Clinical drug trials must meet the requirements established by the Food and Drug Administration (FDA).
- If the study involves human gene therapy, a copy of the NIH Recombinant DNA Advisory Committee (RAC) or waiver of review will be required.

ABOUT NORD

The National Organization for Rare Disorders (NORD) is a unique federation of voluntary health organizations and individuals dedicated to helping people with rare “orphan” diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, service, and research. Besides advocating for increased government research funding and referring patients to clinical trials and genetic investigations, NORD funds clinical research grants on new treatments for rare diseases.

To receive notification of future funding opportunities through NORD, email complete mailing address to: research@rarediseases.org.



National Organization For Rare Disorders
 2008 Abstract Proposal
APECED Syndrome/APS Type 1

Name of Researcher	
Researcher's Email	
Researcher's Mailing Address	
City/State/Zip Country	
Researcher's Telephone	
Researcher's Fax	
Institution(s) Where Research Will Be Conducted	
City, State, Country of Institution(s)	
Title of Project	
Funding Amount Requested (Not to exceed \$45,620)	
Will Research Involve Human Subjects?	[] YES [] NO
How Did You Hear About This RFP?	<input type="checkbox"/> U.S. Postal Service Notice <input type="checkbox"/> Email Subscription Service <input type="checkbox"/> Medical Journal Ad <input type="checkbox"/> Website Posting
Please Be As Specific As Possible:	
Applicant Signature (required)	

Please provide the following information and mail 14 copies of this application, in addition to the original, with attachments to be received by May 9, 2008 to:
National Organization for Rare Disorders, (NORD)
Attn: Stefanie Putkowski, RN, Research Program Administrator,
PO Box 1968, 55 Kenosia Avenue Danbury, CT 06813-1968, USA

Yes/No

An initial letter of intent	
A one-page abstract of the proposal	
Brief budget outline. Do not include PI salary, overhead, or indirect costs. Funding can be used to cover expenses such as technical assistance, supplies, and small equipment.	
Cogent reasons why NORD funding is essential	
Biographical sketch and bibliography (Forms provided, see pages 4 & 5)	

BIOGRAPHICAL SKETCH

Not to exceed 2 pages

Applicant may use this form or the NIH Biosketch form. Please modify the form to include, when applicable, the following:

Name Position/Title									
Education/Training Begin with baccalaureate or other initial professional education and include postdoctoral training.	<table border="1"> <thead> <tr> <th data-bbox="509 602 834 636"><u>Institution and Location</u></th> <th data-bbox="834 602 997 636"><u>Degree</u></th> <th data-bbox="997 602 1159 636"><u>Year(s)</u></th> <th data-bbox="1159 602 1508 636"><u>Field of Study</u></th> </tr> </thead> <tbody> <tr> <td colspan="4" data-bbox="509 636 1508 1131" style="height: 200px;"></td> </tr> </tbody> </table>	<u>Institution and Location</u>	<u>Degree</u>	<u>Year(s)</u>	<u>Field of Study</u>				
<u>Institution and Location</u>	<u>Degree</u>	<u>Year(s)</u>	<u>Field of Study</u>						
Research and Professional Experience Concluding with present position, list in chronological order previous employment, experience, and honors. Include present membership on any advisory committee.									
Honors and Awards									
	Not to exceed 2 pages								

BIBLIOGRAPHY

Not to exceed 2 pages

Publications: List in chronological order the titles, all authors, and complete references to all publications in the last three years and to representative earlier publications pertinent to this application. If the list of publications exceeds two pages, select the most pertinent publications.

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