



## REQUEST FOR PROPOSALS (RFP)

The International Rett Syndrome Foundation, (IRSF), announces the availability of grants in conjunction with both its Basic and Translational Research programs. Details for both programs may be viewed below. **Please Note:** IRSF has changed the timeline of its Basic Research Program to be in synchronization with its fundraising cycle.

### Basic Research Program

IRSF announces the availability of **Regular Research grants** and **Post-doctoral Fellowships** for international biomedical research to promote the understanding of MeCP2 in the pathogenesis of the neurobehavioral phenotype of Rett syndrome (RTT). We seek to gain a better understanding of the underlying pathology of the disorder leading to an amelioration of the symptoms and a cure for RTT. IRSF seeks to encourage research that encompasses innovative therapeutic approaches and state of the art diagnostic techniques.

IRSF encourages applications within the following emphasis areas:

- Understanding the role of MeCP2 during normal brain development
- Characterizing the role of MeCP2, including MeCP2 target genes, in normal structure and function of the developing and adult nervous system
- Determination of the relationship between patterns of expression of MeCP2 and related proteins in the nervous system and the neurologic and behavioral phenotypes of patients with RTT and/or related animal models
- The investigation of neuronal abnormalities that result from MeCP2 dysfunction
- Understanding mechanisms and systems leading to aberrant behavior in RTT
- The development of assistive technologies to improve the quality of life of individuals affected by RTT
- Support for other advances in therapy such as physical therapy, communication and education

The two year **Regular Research Grants** are designed to assist investigators establish hypotheses relevant to Rett syndrome research and obtain future funding from other agencies. The maximum funding level for research grants is \$100,000 over two years.

The two-year **Post-doctoral Fellowships** are designed to assist post-doctoral researchers establish careers in fields relevant to Rett Syndrome research. The maximum funding level for Post-doctoral Fellowships is \$100,000 over two years. Post-doctoral Fellowship applicants are required to have a sponsoring mentor.

**Frequency:** Awards will be made in one cycle in 2011

**Timeline:**

**Letters of Intent due:** August 15, 2011

**Invitation of full applications by:** August 29, 2011

**Applications due:** October 10, 2011



## Translational Research Program

IRSF announces the availability of **HeART (Help Accelerate RTT Therapeutics) and ANGEL (Advanced Neurotherapeutic Grant of Excellence) grant awards** to promote the development and testing of therapeutics to treat and reverse Rett syndrome (RTT) and its symptoms. These award mechanisms seek to facilitate the development and testing of novel therapeutics targeting the underlying pathology of the disorder, thereby leading to an amelioration of symptoms or ultimately a pharmacological reversal of RTT. Through these awards, IRSF seeks to foster academic and public-private partnerships and further facilitate drug discovery, development and testing.

IRSF encourages novel research programs that broadly encompass the following areas of unmet need:

- Design, synthesis and testing of potential disease modifying therapeutics to treat or reverse RTT
- Testing of existing therapeutics both in vitro and in vivo to repurpose their use in RTT
- Development and/or validation of in vitro and in vivo models of RTT for therapeutic testing
- Development and/or validation of novel biomarkers for objective clinical trials outcome measures
- Testing of IND-ready therapeutics in pilot clinical trials

### HeART (Help Accelerate RTT Therapeutics) Grant Award

**Goal:** To provide seed funding for early stage drug discovery and development efforts.

**Funding:** The maximum funding level for **HeART grant awards** is **\$50,000** for one year.

HeART grant awards are provided for cell-based assay development, early-stage drug discovery and development, early stage medicinal chemistry efforts on high value candidate therapeutics, cell-based screening of candidate therapeutics and follow-on early-stage in vivo testing. Grant applications for exploratory studies towards development of biomarkers or objective clinical outcome measures will also be considered.

Applications require only a 3 page proposal narrative.

**Frequency:** Awards will be made in two cycles in 2011

#### Timelines:

##### Cycle 1

**Letter of Intent due:** March 28, 2011

**Invitation of full applications by:** April 11, 2011

**Applications due:** May 16, 2011

##### Cycle 2

**Letter of Intent due:** August 15, 2011

**Invitation of full applications by:** August 29, 2011

**Application due:** October 10, 2011



### **ANGEL (Advanced Neurotherapeutic Grant of Excellence) Grant Award**

**Goal:** To provide funding for the later stages of translational research.

**Funding:** The maximum funding level for **ANGEL grant awards** is **\$300,000** per year for up to two years.

Proposals must be comprised of very specific programs focused on pre-clinical drug discovery and development or clinical testing of therapeutic candidates. This mechanism requires development of a milestone based research plan, interim reporting on milestones and interaction with IRSF staff prior to and during the award period. The budget and duration of funding are variable and continued funding will be based on satisfactory progress on milestones. Applications are usually solicited, however, unsolicited proposals will be considered. Preliminary inquiries are required to determine IRSF's level of interest prior to submission of an Expression of Interest (EOI).

**Frequency:** Applications may be proposed on a rolling basis year round and solicited applications will be subject to approximately a 90 day period for review and response following submission of a full application.

HeART & ANGEL grant awards are provided for early and late stage translational research to treat and reverse RTT, such as; assay development, target identification and validation, screening and testing of candidate therapeutics, exploratory studies to develop biomarkers and objective clinical outcome measures, development and optimization of therapeutic candidates and their testing in pilot clinical trials.



## General Submission Guidelines

Considering the interdisciplinary nature of both Basic and Translational Research projects, collaborative studies among geneticists, molecular biologists, neuroscientists, child neurologists, medicinal chemists and pharmacologists engaged in drug discovery and other clinical or basic researchers are encouraged. Teams working together from different institutions as well as within pharmaceutical and biotechnology industries are strongly encouraged.

Grants are made for technical support, supplies, equipment and relevant travel. Indirect costs of up to 10% of direct costs may be included with the request amount. Proposals for less than the maximum amount are encouraged. Second year funding for Regular Research Grants, Post-doctoral Fellowships and ANGEL awards is non-competitive but is based on review of the first year's progress report.

## The IRSF Two-Step Grant Application Process

### 1) Submit a Letter of Intent or Expression of Interest

The investigator must submit a Letter of Intent (LOI) or Expression of Interest (EOI) through IRSF's website describing the project in a succinct manner. The LOI should include objectives and specific aims of the proposal, research design and methods, qualifications of the Principal Investigator, mentor (if applicable) and other principal investigators involved in the project. Preliminary data may be appended where appropriate. LOIs should not exceed two pages; EOIs should not exceed three pages.

### 2) After review of the LOI or EOI, IRSF will contact the investigator via email to invite or decline a full proposal. Successful applicants will be provided with specific instructions to login, download, complete and submit the electronic application form.

## Eligibility

IRSF accepts qualified, scientific proposals from Academic Institutions, existing, private, early-stage pharmaceutical and biotechnology industries--if a clear need for non-profit funding to support the project is demonstrated and justified and other Non-Profit organizations worldwide. Investigators with "Research Associate" as their title are eligible to apply for post-doctoral fellowships only. Exceptions may be considered on a case by case basis.

**Please Note:** IRSF requires all applicants to use our online submission process.

## Submit Letter of Intent or Expression of Interest

### For application submission inquiries, please contact:

Jim Keller, Grants & Research Manager - [jkeller@rettsyndrome.org](mailto:jkeller@rettsyndrome.org) | 917-267-4504

### For program related inquiries, please contact:

Antony Horton, PhD, Chief Scientific Officer – [thorton@rettsyndrome.org](mailto:thorton@rettsyndrome.org) | 917-267-4502