

## ORPHAN DISEASE CENTER MPS I PILOT GRANT PROGRAM

**Overview:** One-year grants up to \$150,000 (direct costs) will be available to develop novel treatments for MPS I. The proposed therapeutic strategies should address the unmet needs of CNS manifestations of MPS I in patients with the severe and attenuated forms of the disease. Approaches that also address the somatic manifestations of the disease including the eye, bone, connective-tissue, and heart, which are not effectively treated with enzyme replacement therapy or bone marrow transplantation are welcome.

The applicant must have a proven track record in the development of novel therapeutics that progress toward clinical applications. The applicant must demonstrate utility of the proposed therapeutic platform in MPS I animal models during the course of the grant. A second year of funding is possible but should not be incorporated into the initial application.

### Eligibility

All individuals holding a faculty-level appointment at an academic institution or a senior scientific position at a non-profit institution or foundation are eligible to respond to this RFA.

### Letter of Interest Instructions:

Please visit [our website](#) to submit your Letter of Interest (LOI), which can also be [found here](#). This one-page LOI is due no later than **Tuesday, September 6, 2022 by 8pm (EST)**.

### Full Application Instructions and Review Procedure

**NOTE:** Full Application is by invitation only after review of Pre-Application

Proposal Due Date: **Monday, October 17, 2022 no later than 12pm (EST)**

Full application documents are to be uploaded on our [website](#), by invitation only.

### FORMAT for documents:

*Font and Page Margins:* Use Arial typeface, a black font color, and a font size of 11 points. A symbol font may be used to insert Greek letters or special characters. Use 0.5 inch margins (top, bottom, left, and right) for all pages, including continuation pages. Print must be clear and legible; all text should be single-spaced.

*Header:* There should be a header at the top right on all pages of the PDF indicating the full name of the PI (e.g., **PI: Smith, John D.**).

For your convenience, a continuation page template is included at the end of the application document.

*File names:* ALL files to be uploaded should start with the LAST NAME of the PI followed by the brief name of the document. Examples: SMITH CV, SMITH Cover Page, SMITH Budget. **If files are not labeled properly, you will be asked to resubmit the PDFs before your application can be considered.**

### CONTENT to be uploaded:

☐ **Cover Page/Checklist/Institutional Signature Page [PDF].**

- ☐ **NIH-style Biosketch with Other Support of PI and key personnel** (5 pages max). **[PDF]**  
The PI must include accurate and complete information regarding all other sources of grant support (current and pending), including title, abstract, annual and total amount of grant, inclusive funding period, and percent effort.
- ☐ **Detailed Budget and Justification. [combined into one PDF]**  
Complete Excel budget sheet (to be provided). Describe justifications in a Word document. Award will be for one year. Proposed funding period: February 1, 2023– January 31, 2024.

Institutions may opt to take up to 10% IDCs from their award totals. Awarded amounts will not exceed \$165,000.

#### **Allowable direct costs**

- Salary for PI
- Salary/stipend and related benefits for graduate student/postdoctoral fellow/technical support
- Travel (up to \$1500)
- Laboratory supplies and other research expenses
- IDCs of 10% are included in the total award amount

#### **Unallowable costs**

- Consultant costs
- Tuition
- Professional membership dues
- Equipment >\$5,000
- General office supplies, institutional administrative charges (e.g., telephone, other electronic communication, IT network, etc.)
- Pre-award charges
- Any other expenses not directly related to the project

- ☐ **Research Plan** (5 pages max) and **Bibliography** (1 page max). **[combined into one PDF]**  
Include the following sections: Specific Aims, Background and Significance, Preliminary Studies/Data, Research Design and Methods. Research plan should address the following questions: 1) Do you require access to reagents, cell lines, animal models, IRB/ethical board approvals, and/or equipment necessary to complete work? If so, please describe your plan to gain access within the time-frame of this grant period. 2) Have you identified qualified personnel to complete this project within the grant period? If not, please provide your plan to do so. Text citations should use a numbered format. Include all author names in the reference list.
- ☐ **Appendix [combined into one PDF]**  
Limited to 5 pages of supplemental information pertaining to proposal or preliminary data only; a maximum of 3 relevant reprints are also acceptable. Include IRB and/or IACUC approval letters if relevant.

#### **Project Disclosures and No Cost Extensions (NCE):**

- NCEs will be granted at the discretion of the ODC.
- Awardees will be limited to 1 NCE request for their award.
- Maximum NCE time awarded will be 6 months.
- NCEs will be granted after a formal request through [this form](#) found on the ODC website prior to the NCE deadline with adequate justification.
- If granted a NCE, you are still required to submit an interim scientific report 6 months into the duration of the original award period, regardless of your new project end date.

- In your letter of interest, you will be required to certify that you have identified qualified personnel to complete this project within the grant period **PRIOR** to the start date of the award. If you have not, you will be required to provide your plan to engage said personnel. Only under extenuating circumstances will personnel issues be considered for NCE requests.
- In your letter of interest, you will also be required to state whether or not you require access to reagents, cell lines, animal models, IRB/ethical board approvals, and/or equipment necessary to complete your work. If so, you will be required to describe your plan to gain access within the time-frame of this grant period.

#### **Grant Review Procedure:**

- 1) Grants will be reviewed for scientific content and relevance to the goals of the RFA.
- 2) Full applications proceed through a two-step review process. The first step includes external review and rating with an assessment of the strengths and weaknesses of each application based on the defined review criteria described below. During the second step, funding recommendations are determined based on an assessment of the reviewer scores and written comments. Final decision of funding will be made by Center Leadership.
- 3) Proposal Content and Review Criteria: The following criteria will be utilized in proposal review.
  - **Project Proposal** – Is the proposed project of high scientific quality? Is the budget fully justified and reasonable in relation to the proposed project?
  - **Background** – Is the fundamental objective of the study and hypothesis to be addressed clearly defined?
  - **Scientific Approach** - Will the proposed specific aims answer the study hypothesis? Will the scientific approach effectively test and answer each specific aim? Are the study goals supported by existing data?
  - **Clinical Impact** - Is the answer to the study hypothesis important to our ability to treat MPS I? Will the proposed research lead to substantial advances and/or contribute to large leaps of understanding or knowledge that will contribute to an improved quality of life, improved cognition, and/or greater survival rate?
  - **Research Significance** - Does the study address an important question that is not likely to be addressed without this funding? Does the proposed study offer a unique opportunity to explore an important issue and/or employ a novel approach to this disease research? Will the study outcomes advance our knowledge of this disease and/or contribute to changes in the focus of future research questions or the way we conduct research on this issue?
  - **Investigator Qualifications** – One consideration is to attract new talent in to MPS I research. While it is important for the investigator to have access to the resources and environment necessary to complete the proposed work, this RFA is not limited to scientists currently working on MPS I. However, we encourage junior and senior investigators not previously working in this area to apply.

#### **Fund Disbursement:**

Funds will be issued through a cost reimbursement mechanism executed by purchase order from the University of Pennsylvania. Details of invoicing schedules and reporting requirements will be made available upon award.

For additional information, please contact Cara M. Weismann, PhD, Director, Program of Excellence in MPS Diseases, [weismann@upenn.edu](mailto:weismann@upenn.edu) or 215-498-8740.