

**ORPHAN DISEASE CENTER
WIEDEMANN-STEINER SYNDROME FOUNDATION
2025 GRANT PROGRAM**

The Wiedemann-Steiner Syndrome Foundation will provide 2-year grants to support research related to Wiedemann-Steiner Syndrome. At least 3 awards will be granted.

Background

Wiedemann-Steiner Syndrome (WSS) is a rare genetic disorder resulting from mutations in the MLL gene (also known as KMT2A) on the long arm of chromosome 11. The syndrome's genetic underpinnings were clarified in 2012 by a group of researchers led by Dr. Wendy Jones. The gene encodes a histone methyl transferase which helps modify the expression of many other genes. The condition is autosomal dominant, and in most cases, the mutation occurred de novo. From an unpublished survey of 76 WSS families, the symptom determined to be of "Very High Impact" by most families, was disruptive behaviors. This included aggressive behaviors, impulsivity, and behaviors outside of age-appropriate societal norms and the most important therapeutic goals were to reduce these behaviors and improve cognitive functioning.

The Orphan Disease Center, in collaboration with the WSS Foundation, is seeking grant applications that aim to further progress the understanding of the disease:

- Two \$90,000 grants to support the development and characterization, for the advancement toward therapeutic goal, of patient-derived cell models of WSS Syndrome (ex. iPSCs, neurons, reporter lines). These cells model will be deposited into a publicly accessible biobank.
- One \$90,000 grant to support a Natural History Study. Proposals for this grant should focus on understanding the progression of the condition over time, often to support product development, especially for rare diseases. These studies are observational and aim to identify factors that influence disease development and outcomes.

Eligibility

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

Letter of Interest Instructions

All applicants must first submit a Letter of Interest (LOI) to be reviewed for consideration of a full application submission. Please submit your LOI by August 8th, 2025.

Format for the 1-page LOI:

- Project Title
- PI and Co-PIs, and associated institutions or organizations
- Overall goal of the project

Translational Research Laboratories

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- Why application is responsive to the RFA: please note that the applicant will not be invited to submit a full application if relevance to the RFA is not clearly described in the LOI
- Brief background including relevant literature.
- Specific Aims provided as a brief list
- Specify the variant(s) and/or cell type(s) to be studied, a high-level project timeline and budget
- Requested resources should be in terms of up to 2 years of funding and total award amount – up to \$90,000 (including direct and indirect costs). It is strongly preferred that applicant institutions waive indirect costs allowing the total amount to go towards research, but indirect costs up to a maximum of 10% could be accepted.

LOI Due Date: LOI document is to be uploaded no later than 5pm (ET) on **August 8, 2025**, via [ONLINE FORM](#).

Applicants will be notified via email with a decision regarding their LOI, which, if successful, will invite the applicant to submit a full application.

Full Application Instructions and Review Procedure

NOTE: Full Application is by Invitation only after review of LOI

Full Proposal Due Date: **September 26, 2025, no later than 5pm (ET)**. Full application documents are to be uploaded on our [WEBSITE](#)

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]**. An application template will be provided.

☐ **Biosketch/CV, with key personnel supporting the project (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. PI should add a section listing key personnel working on the project.

☐ **Detailed Budget and Justification. [combined into one PDF]**

Complete Excel budget sheet (to be provided). Describe justifications in a Word document including all subcontracts to co-investigators.

Proposed funding period: January 1, 2026 – December 31, 2027

Awarded amounts will not exceed Award Total

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

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** (no limit) **[combined into one PDF]**

Include the following sections: Specific Aims, Detailed Background including relevant literature and Significance of intended work, Preliminary Studies/Data, Research Design and Methods. Research plan should address the following questions: 1) Do you require access to reagents, animal models, patient blood samples, 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 Wiedemann-Steiner Syndrome Foundation.
 - ☐ 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 prior to the NCE deadline with adequate justification.
 - ☐ If granted an 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.

Fund Disbursement:

Funds will be issued through a cost reimbursement mechanism executed by the Wiedemann-Steiner Syndrome Foundation. Details of invoicing schedules and reporting requirements will be made available upon award. For additional information, please contact Deborah Requesens at dreques@upenn.edu.