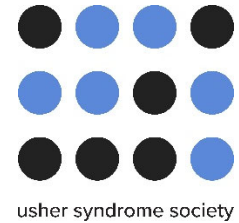


**Usher Syndrome Society
Request for Applications
Translational Research Grant
Application Instructions**



IMPORTANT DATES

Letter of Intent Due: August 27, 2021

Applications Due: October 15, 2021

Notification of Award: December 1, 2021

Funds Available: January 1, 2022

Please submit grant materials as a single PDF file to nancy@ushersyndromesociety.org

For questions regarding these instructions, please contact the USS nancy@ushersyndromesociety.org

Background: The Usher Syndrome Society (USS) is a non-profit organization that uses the arts, educational events, and collaboration to raise awareness and funding for Usher syndrome (USH) Research. USS funds research designed to develop treatments and cures for all forms of Usher syndrome. USS has secured commitments for \$500,000 to fund USH research. For more information see: <https://www.ushersyndromesociety.org/welcome.html>

To fulfill the goal of “timely and efficient translation”, USS Translational Research Grants are intended to support translational research on **Usher syndrome** within the domains of:

Preclinical Research, encompassing basic science research with a direct link to the clinical population (human genetics, patient tissue, iPSC, humanized animal models).

Mechanism-based Therapeutic Development, focused on novel disease modifying therapeutic modalities utilizing *in vitro* or *in vivo* preclinical models.

- 1. Purpose of grants:** Proposed research projects must be related to the mission of the USS. The USS focus is on the discovery pipeline including identification of therapeutic strategies and development of novel therapeutic agents. Particular emphasis is placed on collaborative efforts that extend across sensory modalities and platform technologies that may advance therapeutic development for multiple forms of USH. Proposals that address specific mutations, such as USH2A exon 20 mutations, are also encouraged.
- 2. Description of grants:**
 1. New research proposals: These grants are up to two years of support, contingent upon adequate progress. The maximum allowable budget (direct costs only, no indirect costs allowed) is \$100,000 per year. The number of awardees will be determined by the quality of the proposals, the total amount of the requested budgets of sufficiently meritorious proposals, and available funds. It is anticipated that 2-3 awards will be made. The second year of funding will be based on adequate progress and milestone achievements and will be reviewed on a non-competitive basis.
 2. Research projects that include well-documented collaborations that merge research

efforts across sensory modalities and across scientific disciplines will be prioritized. Further priority will be assigned to applications that may offer the greatest benefit for the largest patient population or targeted approaches that hold great promise for select mutations, such as those found in USH2A exon 20.

3. Principle investigators selected for funding will have collaborative consultations biyearly with USS scientific advisors for feedback and progress updates.
3. **Eligibility:** Applications will be accepted from research scientists at qualified U.S. and international research institutions. Collaborative projects between U.S. and international scientists are also acceptable. Applicant principle investigators must hold a tenure-track faculty position with an M.D., Ph.D., M.D.-Ph.D., or equivalent degree.
4. **LOI:** The initial Letter of Intent (LOI) will have a 1-page limit. The LOI should include USH gene(s) of interest, a hypothesis, specific aims and a brief description of the research design and methods. LOIs will be screened to determine if the proposed research project is consistent with the goals of the USS. Following screening, notifications will be sent to selected investigators inviting submission of a full application. **LOIs must be submitted to nancy@ushersyndromesociety.org by 5 pm on August 27, 2021.** An email confirmation of receipt will be returned to the applicant. **Invitations to submit a full application will be sent by Sept 3, 2021.**
5. **Full applications (invited following review of LOI):** Full applications must be submitted in single-spaced text, one-half inch margins, and 12-point Arial or Helvetica font. The primary applicant's name must appear in the upper right-hand corner of each page. **Proposal text is limited to five pages** (including figures, excluding references). Use standard PHS 398 forms for biosketch and other support.
6. **Application submission:** Submit applications electronically as a single PDF file to nancy@ushersyndromesociety.org by **5 pm** on **October 15, 2021.**
7. **Composition of research proposal:** Invited research proposals should include:
 1. Cover Letter
 2. Abstracts, scientific (250 words) and lay summary (100 words)
 3. Budget & Budget Justification (1 page limit)
 4. Biosketch(es) (include PI and co-investigators; NIH format)
 5. Other support page (list pending and current support only)
 6. Research Plan (5 page limit, not including references)
 - a. Hypothesis and Specific Aims
 - b. Background and Significance
 - c. Preliminary Results
 - d. Research Design and Methods
 - e. Statement of how proposal supports USS mission and research priorities
 7. Literature cited
 8. *Letters of support from collaborators or consultants (1 page limit/letter) *optional
8. **Grant review:** Applications will be reviewed and prioritized for funding by a multidisciplinary scientific advisory committee composed of established hearing/vision scientists. The committee will be chaired by Jeffrey R. Holt, Ph.D., Professor of Otolaryngology and Neurology at Boston Children's Hospital and Harvard Medical School.