



Cure SMA Request for Proposals for Spinal Muscular Atrophy Research Projects and Postdoctoral Fellowships

April 18, 2022

Cure SMA is pleased to announce that we are accepting grant applications for funding of research projects and postdoctoral fellowships, under a competitive review by our Scientific Advisory Board (SAB). [Please click here to view the SAB roster.](#) Please note that Cure SMA will not release a separate drug discovery RFP in the next twelve months.

Program Overview:

The deadline for submission is Friday, July 15, 2022. We anticipate awarding \$500,000 in total funding comprised of 3-4 grants. The 5-year historical acceptance rate for research proposals to Cure SMA is 28% with an average of 8 grants awarded per year.

PI Awarded Research Grants: Applications will be accepted for one or two years of funding with a maximum funding level per year of \$75,000 and a total award of \$150,000 (including 8% overhead) over the duration of the entire project.

Postdoctoral Fellowship: Applications will be accepted for one or two years of funding with a maximum funding level per year of \$50,000 and a total award of \$100,000 (including 8% overhead) over the duration of the entire fellowship. Please note that a specific postdoctoral fellow to complete the work must be identified at the time of submission.

Scientific Priorities: Cure SMA wants to encourage applications on novel research that will enhance our understanding of SMA disease pathology at the molecular, cellular, and biochemical level; generate key reagents and tools to facilitate drug development and clinical trials; and identify new therapeutic strategies for SMA, in particular combination approaches. Cure SMA welcomes high risk, high reward projects and values the ability to provide seed funding for these types of exploratory projects. Please note that high risk projects are generally not awarded maximum funding without compelling preliminary data.

We envision that studies funded by this Research RFP will fall into one the following key areas:

- Studies focused on the molecular and biochemical mechanisms regulating SMN expression or mediating SMN function. Results should lead to a better understanding of the requirements for SMN protein biologically. There is a particular interest in identifying genetic modifiers, upstream regulators of SMN expression / splicing / function, and downstream effectors of SMN functional activity, resulting in new drug targets for SMA.
- Studies resulting in greater understanding of the pathophysiology of SMA, using well-validated animal or cellular models of SMA. This includes focus on the tissue or timing requirements for SMN protein, the cellular autonomy of the disease in motor neurons

and other cells, understanding the basis of motor neuron selectively, peripheral versus central manifestations of SMA, and others.

- Studies focused on early proof-of concept assessment of novel therapeutic approaches for SMA in well-validated animal or cellular models of the disease or on progressing aspects of ongoing preclinical drug programs for SMA towards IND. Proposed SMN enhancing approaches should have advantage over current candidates or have the ability to be used in combination. *Particular interest exists in non-SMN based approaches with the potential for combination use with SMN up-regulation strategies. This includes approaches to be used in combination or alone to address the unmet needs of patients in symptomatic phases of disease.*
- Work focused on generating research or clinical trial tools for SMA, such as new animal models, phenotypic cellular assays, activity assays for SMN function, biomarkers or outcome measures for clinical trials.

Application Process: To apply for a research grant or postdoctoral fellowship, please visit <https://www.curesma.org/funding-opportunities/> to obtain the full RFP and application form. Please submit an electronic copy to jackie@curesma.org. Include the PI's surname and Research/Postdoc Fellowship in the subject line.

Review Criteria: All applications are reviewed by our Scientific Advisory Board (SAB), on both scientific quality and relevance to the Cure SMA research mission of accelerating the discovery of safe and effective treatments for SMA.

Timeline:

RFP Release Date:	April 18, 2022
Proposal Due Date:	July 15, 2022 (midnight ET)
Award Notification:	By December 1, 2022

For more information or if you have questions, please contact Jackie Glascock PhD, Cure SMA Director of Research Programs at jackie@curesma.org