



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

June 15, 2023

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.](#)

Program Overview:

The deadline for submission is Friday, September 1, 2023. We anticipate awarding \$750,000 in total funding comprised of 5-6 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 all projects, including those that are high risk, are generally not awarded maximum funding without compelling preliminary data.

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

- Studies focused on the molecular, biochemical, and genetic mechanisms regulating SMN expression or mediating SMN function. Results should lead to a better understanding of the requirements for SMN protein biologically. Proposals focused on identifying genetic modifiers, regulators of SMN expression / splicing / function, and effectors of SMN functional activity, are of great interest. Please note that proposals seeking to investigate genetic modifiers should feature strong rationale for the investigation of the selected modifier.
- Studies resulting in greater understanding of the pathophysiology of SMA, using well-

validated animal or cellular models of SMA. This includes focus on the spatial or temporal requirements for SMN protein, the cellular autonomy of the disease in motor neurons and other cells, further understanding of 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 novel research tools for SMA, such as new animal models, phenotypic cellular assays, activity assays for SMN function, or biomarkers. Proposals focused on generating novel methods should offer compelling rationale for their development and the potential of their use to help address key unanswered questions in the field and/or facilitate drug development.

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.

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:	June 15, 2023
Proposal Due Date:	September 1, 2023 (midnight ET)
Award Notification:	By December 1, 2023

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