



Muscular Dystrophy Association, Inc.
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MDA Venture Philanthropy Application Process

MDA Venture Philanthropy (MVP) makes targeted investments to support drug development for neuromuscular disease. Projects will be milestone-driven, contract-mediated and a return on investment will be negotiated.

Investment Profile: MVP invests in small pharmaceutical and biotech companies and in academics developing treatments and therapies for neuromuscular diseases. Companies may be public or private, in industry or in academia, and located in the U.S. or internationally. MVP funds academic investigators doing appropriate studies, but encourages corporate collaboration. MVP seeks to apply funding where it will achieve the greatest leverage to increase the number of effective drugs in development for neuromuscular diseases. As such, well-financed projects that will proceed without MVP investment may not be selected for funding.

Therapeutic Areas: MVP supports projects in any of the more than 40 neuromuscular diseases as outlined on mda.org.

Stage of Research: MVP funds projects from proof of principle in an animal model through Phase II trials. Projects may include toxicology, manufacturing and scale-up, pre-IND, Phase I or early Phase II trials.

Size and Timeframe of Investment: MVP will consider funding projects up to \$1M, depending on the stage of research. MVP is interested in partnering with applicants to accelerate research on projects with strong institutional support, and in general MVP funds should constitute less than 50% of the total project budget. The award period will be on average 2 years or less, but follow-on investment may be negotiated if all milestones are met and the project moves into later phases of development. MVP acknowledges that drug development is a long process, and that the horizon for drug approval may be up to ten years after MVP investment.

- Research and Development (up to \$300,000)
- Preclinical (up to \$500,000)
- Early Clinical (Phase I/II) (up to \$1M)

Application Process

Deadlines for letters of intent are March 1, June 1, September 1, and December 1.

At each stage of the process outlined below, the project may pass on to the next stage of evaluation, be rejected, or the company may be asked to supply additional data or reapply at a later date. Reapplication may be requested due to issues with the project or company, or lack of fit with the MVP portfolio at the given time.

1. Submit a letter of intent through [proposalCENTRAL](#). If you do not have an account, click on the orange rectangle on the Proposal Central home page to create an account. If you already have an account, log in using your user name and password. Complete the fields in proposalCENTRAL and for section 5 you will need to download and complete the Letter of Intent document as outlined in the Letter of Intent instructions using Adobe Acrobat. Once the document is completed you will need to upload it as the Letter of Intent in section 5.
2. MDA scientific staff will evaluate the basic information gathered to determine if the investment is a good fit for the MVP program.
3. Company will make a presentation to MDA scientific staff via a non-confidential webinar to provide further information. Staff will ask about the stage of the investment, the preliminary data and the company's intentions for moving forward.
4. Applicants successful through this stage will be invited to submit a full application package for formal review through Proposal Central. For a full application, a company will not only need to demonstrate the scientific soundness of the therapeutic candidate but also capabilities of the management and scientific staff, financial strength, and intellectual property protection. This information is evaluated (under CDA) by external reviewers and the MVP Advisory Committee.
5. Term sheet negotiation occurs followed by contract negotiation. The final contract is approved by members of MDA staff and board.

Materials for full application package:

General Submission Guidelines

- ❖ Applications and all supporting documents **MUST** be submitted in English.
- ❖ Request support in U.S. dollars (\$) only.
- ❖ Avoid abbreviations except for those in common use such as DNA, ATP, CK, and so forth.
- ❖ All documents for full application package are submitted through Proposal Central.

The material below should not be considered an exhaustive list of the documentation that MVP will require, but rather a guideline as to the type of information that will be requested depending on the specific nature of your project. The evaluation process will be an

iterative one, and as such new information may be requested throughout the process (both scientific data and information about the company).

Scientific Data Package Contents:

The data package will cover the scientific basis of the investment, and how therapy development is expected to continue into the next phase. Specifically, data packages should include the following:

For ALL applications the following are required

1. Lay abstract
2. Non-confidential summary
3. Impact statement: State how this project will promote major advancement in the understanding of neuromuscular disease, accelerate treatments and cures, or optimize patient care)
4. 10-12 page research plan that includes:
 - A brief summary of published results that support the concept (relevant papers may be attached as appendix materials).
 - Preliminary data that describes the feasibility or supports the concept of the study. You must describe the number of replicates, type of controls, as well as statistical analyses for each figure.
 - A description and timeline of studies proposed
 - Identification of milestones and go/no go points in the study.
5. Literature Cited
6. Brief list of key personnel working on the project. Include academic expert advisors where appropriate. Biosketches should be included.
7. Budget description and justification, including any subcontracts.
8. Current/pending funding pertinent to the project.
9. All facilities available for conducting the proposed research project. Identify by name and address any facilities that are not part of the sponsoring institution and describe the arrangements made for using those off-site facilities.
10. *If applicable*, TACT (or other agency) review and rebuttal.

For Preclinical Studies

- Sound preliminary data demonstrating functional data in an animal model.
- Preliminary data demonstrating activity against a validated target where available, and ideally demonstrating validation of target (published data may be attached in support).
- Preliminary safety and/or toxicology data where available (GLP or non-GLP)
- Preliminary data supporting efficacy and/or safety greater than competing therapies targeting the same pathway, where available.
- Large animal data may be preferred or required for some biologics. This data may be shown in preliminary data or included in proposed studies as necessary.

- A summary of the company's regulatory strategy for bringing the therapy to the clinic, including a description of any discussions that the company may have had with the FDA, and/or a plan for future discussions.
- A description of the company's future plans for development and route to market assuming success (this may include licensing/selling the compound as an exit strategy).

For Clinical Studies

- A summary of the IND-enabling data for the therapy, which should address the following points:
 - Preliminary data demonstrating solid proof-of-principle in appropriate animal models.
 - Description of results from toxicology studies, and justification of safety/benefit ratio.
 - Description of study design.
 - Data showing that endpoints and natural history data support study design and that power calculations have been done as appropriate.
- Any available preliminary data proving efficacy or safety greater than competing therapies targeting the same pathway.
- Summary of FDA discussions and FDA input into the protocols.
- Description of the route to market for the therapy assuming that the trial is successful.

Business Due Diligence:

In addition to scientific data, MVP will also evaluate financial, legal and management aspects of the company to assess the company's viability and ability to bring a potential therapy to market. Academic projects will not require the same level of documentation, but PI's should be aware that MVP will request information pertaining to additional funding for the project, the management of the project, the IP behind the project and any corporate collaborators

While additional documents may be requested at later stages of review, MVP asks that applicants submit the following information in their initial application:

1. Company Information
2. Financial
 - a. Key investors to date
 - b. Other current/pending funding to the company
 - c. Financial Statements for previous two years
3. Legal
 - a. Brief discussion of intellectual property position
 - b. Succinct analysis of competitive space and how proposed project is differentiated
4. Management/Operations:
 - a. Key management personnel with brief bio indicating relevant experience
 - b. Scientific Advisory Board

Contract requirements:

MVP contracts will include the following general terms, although each contract will be negotiated separately. A reduced version of the contract may be used when MVP funds academic projects, although academic projects will still include milestones.

1. Completion of milestones will be tied to payments for the next stage of development.
2. MVP will require proof of company commitment to the project in the form of matching funds, or equivalent.
3. Investments greater than \$1 million will have external steering committees consisting of scientific experts in the field agreed upon by the company and by MVP, who will evaluate data and milestones as the project progresses. For smaller investments, companies will submit short progress reports demonstrating achievement of milestones.
4. MVP will negotiate a return on its investment, in the form of a royalty share, equity or other outcome. This agreement will be set up in such a way that it does not inhibit future investment in the company, and the amount will be proportional to the percentage of the project supported by MVP.
5. MVP will negotiate some form of licensing agreement such that if the company ceases development of the therapy for the indication supported by MVP *for reasons other than scientific failure* (such as a change in strategic direction), MVP has access to the necessary IP and results from MVP-sponsored studies such that development of the therapy can be continued through other avenues.
6. MVP will be credited for its support in all acknowledgements.

Updated 7/13/17