INSTRUCTIONS FOR COMPLETION OF HUMAN CLINICAL TRIALS GRANT APPLICATION

Introduction

In preparing your grant application, please read and follow these instructions carefully. Incomplete or improperly prepared applications will not be reviewed. An application will be considered incomplete if: (1) it is not prepared and submitted according to instructions; or (2) the information it contains is not sufficient to permit an adequate review.

To facilitate proper review of your application please remain succinct and limit the sections of your application describing your "Preliminary Studies", "Background, Significance and Rationale" and "Trial Design" to a maximum total of fourteen (14) pages including FIGURES and LEGENDS. Applications must be submitted using 11pt font only. This section of the application is found under Section 12 and titled "Study Design." If it is already available, you should attach your Investigator Brochure and Research Protocol to the application as an appendix. If a full protocol is included as an appendix, you may state "protocol attached" in lieu of the clinical plan section of the main application.

All times referenced in the application process and through proposalCENTRAL are given in **Eastern Time**.

General Submission Guidelines

- Applications and all supporting documents MUST be submitted in English. This includes your IACUC Approvals, IRB Approvals and Biohazardous Use Certificates.
- Request support in U.S. dollars ($) only.
- Avoid abbreviations except for those in common use such as DNA, ATP, CK, and so forth.
- Appendix material should include your investigator brochure and/or trial protocol if they are available, along with preprints and manuscripts accepted for press but not yet published (these should be uploaded to the application along with the acceptance letter/correspondence). You are permitted one unpublished/unaccepted manuscript.
- A resubmission must be accompanied by a Resubmission Statement responding to the previous reviewers' concerns. The Resubmission Statement is limited to a maximum of one (1) page.

Principal Investigator

The Principal Investigator is the one person responsible for the scientific and technical direction of the project. An application may have only one Principal Investigator.

If co-investigator(s) and/or collaborator(s) are involved with the proposed project, a letter of support and a biosketch for each must be uploaded to the application.

SECTION 1

Trial Title

Whenever possible, the title should include the name of the neuromuscular disease or class of neuromuscular disease to which the trial is most related. (MDA reserves the right to edit grant titles for clarity.)

Resubmission

If you have marked the application as a resubmission, please put the date of the previous submission. In a case where it is the 2nd resubmission of a grant, please indicate the most recent submission prior to the current application.

At the bottom of this page, your previous application(s) should appear. Please click on the previous proposal for which you are resubmitting.

A resubmission application will be required to upload a "Resubmission Statement." The Resubmission Statement should address the previous reviewer's concerns and any changes you have made to the current application. The Resubmission Statement **must not exceed** one (1) page.

SECTION 2

Download Templates and Instructions

This section contains all templates that are required to complete or which might be necessary for the full submission of your application.

You must use the "Study Design" template provided in this section for submission. This part of your application is required, but limited to fourteen (14) pages in an 11 pt font. DO NOT change the margins which are 0.50 on all sides. If you are attaching an Investigator Brochure and Protocol, state that this is the case and leave the clinical plan section blank.

The Biosketch template provided should be used for the Principal Investigator. You will also need to upload a Biosketch for each Co-PI, Collaborator, Consultant and Post-Doctoral Associate who will be responsible for the execution of this project. Each Biosketch should clearly identify all papers in all fields published during the past three (3) years and a list of all grants held within the past three (3) years, specifying funding sources. Please identify films, tape recordings and monographs on which you may have collaborated. Each Biosketch is limited to a maximum of four (4) pages. A Biosketch is not needed for graduate students, technicians or coordinators.

The Resources Template is a required section of the application but is not limited to a maximum number of pages. Please use 11 pt Font and all margins must be 0.50. Please list all resources available for conducting the proposed trial. Include laboratory space, clinical

facilities, animal facilities, computer facilities, office space, clerical staff and major equipment available. 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.

The Milestone Schedule should be filled in with at least two milestones per year that will indicate that the project is progressing. If a planning period is proposed, the first milestone will be initiation of the trial at the end of the planning period. Subsequent milestones may include events such as recruitment of cohorts of patients, DSMB approval to move to higher doses, interim safety analyses, completion of the trial, analysis of data etc. Payment will be tied to completion of these milestones.

The Budget Justification is a required attachment and should be completed by fully justifying all expenses listed on the main Detailed Budget page.

References for Literature Cited template is a required section of the application. Please use 11 pt Font for this section and 0.50 margins. There are no page limits for this section. Make every attempt to be judicious in compiling a relevant reference list. It need not be exhaustive.

A Subcontract Detail Budget is provided in the event that you will have a subcontract listed on the Detailed Budget. Each subcontracting institution should have its own Sub Contract Detail Budget attached. Overhead is not permitted on the subcontract budget page. The overhead will be fully included on the main Detail Budget page to the Lead Institution. All overhead is limited to a maximum of ten (10) percent of the direct costs.

SECTION 3

Enable Other Users to Access this Proposal

This section is used specifically for providing access rights to other people whom you may wish to have access to your application. You may choose their access as “View” or “Edit.” If you give someone “Edit” ability they can upload documents or add attachments in your absence.

If you mark an individual as “Auto Notify” this means each time an email is sent to you through proposalCENTRAL, that person will automatically receive a copy of the email.

SECTION 4

Applicant/PI

This section of the application asks for the Principal Investigator’s information. The Principal Investigator must be the same person that submitted the Letter of Intent. All fields that are marked with asterisk (*) are required fields. If you already have a professional profile within proposalCENTRAL, these fields will be automatically populated and filled in. Please review them carefully to confirm the information is correct.

Conflict Of Interest Disclosure

Any potential conflict of interest the Principal Investigator(s) or collaborator(s) may have relating to the project must be revealed. Such conflict would include, but may not be limited to, having a proprietary interest that may be affected by the outcome of a research project. It is expected that MDA grantees will observe the highest ethical standards in the conduct of research. Please attach a one-page explanation if a conflict of interest exists.

SECTION 5

Institution and Contacts

This section contains the information of the "Lead Institution." This page defaults to the institution of the Principal Investigator. If the institution is incorrect, you may click on the "Change Institution" button and search for the correct institution. The asterisks (*) denote required fields. Please make sure that all information on this page is correct, including the IRS EIN number. The IRS EIN number should be 9 digits in the following format XX-XXXXXXX. Do NOT include letters or additional separators. You will need to upload a copy of your institution's W-9 form before submitting the application.

The W-9 Form should be available to you through your Grants and Contracts or Sponsored Programs Office.

NOTE: If you are applying from a non-U.S. institution/university and your institution/university does not have an EIN number, you will need to type in N/A in the space provided.

Your University/Institution may already have contacts listed under their profile. Contacts that are required on all grants are marked with an asterisk (*) and cannot be removed. These contacts are generally institutional officials, financial officers or grant and contract personnel. However, if you need to add one, you may do so by entering their email information in the space provided and clicking the "Add" button.

SECTION 6

Key Personnel

All personnel working, collaborating, over-seeing or coordinating on the project must be listed in this section. This should also include all Co-PIs, Collaborators and Sub-Contract PIs. You will need to insert their email address in the space provided and click "Add." Complete all required fields and click "Save" when completed. This person will now appear in the "Key Personnel" window.

SECTION 7

Lay Summary/Abstract and Impact Statement

Please provide a succinct and non-technical summary of your proposed project in non-scientific terms that would be understood by a general audience. Since this summary will be public information, do

not include any proprietary or confidential information in this section. Do not summarize past accomplishments or cite literature in this section.

The Lay Summary section is limited to 1,500 characters, including spaces. Information entered in this section must be text only. Scientific notations, special characters, special fonts and other rich-text formatting (i.e. bold, italics, underline) cannot be saved or displayed. Do NOT insert carriage returns at the end of each line. Type continuously until completed or starting a new paragraph.

If you “cut and paste” in this section, please double check that there are no additional carriage returns before submitting the application.

The Abstract section should be a succinct summary in scientific terms that would be understood by a technical audience. Since this summary may be public information, do not include any proprietary or confidential information.

In concise terms state: (1) the long term objectives; (2) the specific aims; (3) the primary methodology and principal organism, tissue, or preparation being used; (4) the relationship of the project to neuromuscular disease.

Do NOT summarize past accomplishments or cite literature in this section. Limit the Abstract summary to 3,000 characters or less, including spaces. Information entered in this field must be text only: scientific notations, special characters, special fonts, and other rich-text formatting (e.g. bold, italics, underline) cannot be saved or displayed. Do not insert carriage returns at the end of each line. Type continuously until completed or starting a new paragraph.

If you “cut and paste” in this section, please double check that there are no additional carriage returns before submitting the application.

For the impact statement, please state how this project will promote major advancement in the understanding of neuromuscular disease, accelerate treatments and cures or optimize patient care. This statement will play a major role in the review of your application and its importance to MDA’s mission. Please limit your statement to 1000 characters or less.

Research Category

At the bottom of this section you will see a listing of the main categories under the MDA umbrella of neuromuscular diseases. You may choose one category or multiple categories that apply. Under the general category you will find the disease specific categories. You can choose one primary disease and multiple secondary diseases. Please note that the one(s) you have chosen will appear to the right of the boxes.

Please choose these categories carefully as they will be used to help facilitate the selection of scientific peer reviewers.

SECTION 8

Detailed Budget

You will need to enter the Start Date and End Date of each Budget Period. To change to a new Budget Period, click on the buttons at the top of the page for “Period 1,” “Period 2,” or “Period 3.” You will need to complete each section of the Detailed Budget for each year of support for which you are

requesting funds, including the planning period if you choose to include it. These numbers should match those included in the milestone table.

The Budget Summary (Section 9) will auto complete itself once you have completed the Detailed Budget.

Personnel must be listed by name, role, and percentage effort devoted to project. The Principal Investigator's salary is permitted, equivalent to the ratio of effort up to 25% but not more than $15,000, plus a proportionate ratio of fringe benefits. Requested salaries are not to be used to replace salaries or partial salaries that are already assured by institutional or other funds. The Principle Investigator's specific role(s) in the proposed research must be described under "Proposed Budget Justification."

The "Institutional Base Salary" should be the Principle Investigator's total base salary and "Fringe Benefits" listed should be the proportionate percentage of the Principle Investigator's benefit cost.

Equipment, whether capital or not, must be listed in this section. Along with a full justification, identify the manufacturer and model number under budget justification section. Computer equipment is limited to $5,000 per grant.

Supplies required to carry out the proposed studies.

Travel to attend foreign or domestic scientific or medical meetings to present the results of MDA-supported research is permitted. Also, funds for travel required in conducting the specific aims of the research project may be requested. All travel must be fully justified in the proposed budget justification; however, should not exceed $1,000 in any given year and is restricted to personnel listed on the Budget in the Personnel Cost category. In exceptional circumstances the PI may justify why additional travel funds are required for this trial.

Site Visit Travel to support travel for the investigators between trial sites. This is limited to $5,000 per year and should only be used for travel of investigators between trial sites.

Subcontract(s) must be listed in this section of the budget. A separate "Sub Contract Detailed Budget" must be submitted for each subcontract. A template for the Sub Contract Detailed Budget is provided in the Supporting Attachments section (Section 12). The subcontract budgets may not include indirect costs as they are absorbed through the main budget "indirect costs." If fees for consultants are requested, their names and institutional affiliations must also be given.

Other expenses may include items such as reimbursement for patient's attendance to trial sites, publication costs, computer use fees, and equipment maintenance and office supplies. Office supplies may not exceed $600 in any given year. The need for each item must be justified in the budget justification section.

Indirect Costs are limited to a maximum of 10% of all direct costs.

Unauthorized Expenses. The following expenses are not permitted under MDA's research program:

- Salary or fringe benefits for collaborating Investigators, coInvestigators or consultants;
- Salaries, travel and/or housing related to sabbatical leaves;

- Salaries for administrative, secretarial and/or clerical staff;
- Life and Disability insurance fees;
- General Liability Insurance;
- Purchase or rental of office equipment; (i.e., furniture, filing cabinets, and copy machines);
- Expenses normally covered by the indirect cost of the Principal Investigator's institution;
- Fees for tuition, registration or other fees relating to academic studies;
- Fees for or related to obtaining visas or citizenship status;
- Membership dues, subscriptions, books or journals; and/or
- Expenses for or related to moving from one institution to another.

SECTION 9

Section 9 (Budget Summary) will automatically complete itself once you have completed the Detailed Budget section.

SECTION 10

Other Support

All sources of current and pending research support - including other MDA projects - must be identified in this section for the Principal Investigator only. This includes all sources – federal, non-federal, commercial or institutional. Prizes or gifts do not need to be included.

Please upload the current budget or proposed budget for all Supplemental or Alternate funding sources for this project.

SECTION 11

Organization Assurances

If your application requests support for research involving human subjects, tissues or materials, then this section must be completed. A copy of the IRB/FDA approval must be uploaded to your application. If your IRB or FDA approvals are "pending", please indicate this by clicking the "Pending" button. In cases where the IRB/FDA approvals are pending, you must upload a copy of the approval once you have obtained it from the appropriate governing board. An approval must be on file with MDA before funds may be forwarded for the project if funded.

MDA-funded projects must be in compliance with all policies, rules, and regulations governing clinical trials, including those of the federal regulatory agencies, the respective university and institution, and MDA. MDA must be advised about any amendments to the original research protocol (including the patient consent form) occurring prior to the commencement of or during the course of the research project.

If your application requests support for research involving experimental drugs or devices, this section must be completed. If your FDA approval is pending, please indicate this by clicking on the "Pending" button. An approval must be on file with MDA before funds may be forwarded for the project if funded.

If your application requests support for research involving vertebrate animals or materials derived there from, this section must be completed. If your Animal Care and Use Committee approval is pending, please indicate this by clicking the "Pending" button. An approval must be on file with MDA before funds may be forwarded for the project if funded.

Continue down the list of the assurances, marking them either "Yes" or "No." Click on the "Save button in the corner to save any changes you may have.

SECTION 12

Study Design and Supporting Attachments

PRELIMINARY STUDIES, BACKGROUND AND RATIONALE AND TRIAL DESIGN

You must use the "Study Design" template settings for the body of this section. You may have up to 14 pages in 11 pt font including figures and legends. Do not exceed fourteen (14) pages in an 11 pt font with 0.50 margins. Your application will not be forwarded for review if the page limit is exceeded.

Explain the questions that the trial sets out to answer, and the rationale for why these questions are important. This section should clearly provide the reader with succinct information on the research you are proposing, why it is important and how it will advance the neuromuscular disease research field.

Summarize the key results and major conclusions from published, in preparation and/or unpublished studies that specifically relate to your proposed project.

Describe the protocol that is planned for the trial, and explain the rationale for this design. Succinctly state the potential difficulties and limitations of the proposed procedures. Discuss how data will be analyzed and interpreted. Point out any procedures, situations, or materials that may be hazardous to personnel and the precautions to be taken for their protection. If the approved study protocol is available, this section should be left blank aside from a note that the protocol is attached. This should be attached as an appendix.

APPENDIX

Appendix material is limited to:

1. The Investigator Brochure for the trial
2. The Protocol for the trial

3. Manuscripts or pre-prints accepted for publication, but not yet published. Please provide the communication of acceptance along with the manuscript or preprint.

4. One unpublished or not yet accepted manuscript.

W-9 FORM

You will need to upload a W-9 Form for your University or Institution. If you are with a non-US University of Institution, please upload the blank form to fulfill the “required” attachments for the Validation process. You do not need to fill out the form if you are a non-US institution.

SECTION 13

PI Data Sheet

This part of your profile is not mandatory. MDA uses this strictly for association statistics and does not print or advertise this information.

SECTION 14

Validate

After you have validated the document you must click "SUBMIT" for the application to be submitted. Validating the document DOES NOT submit the application to MDA.

IMPORTANT NOTE

One copy of your completed application should be filed with the business office of the sponsoring institution to alert them to your pending request for MDA support. Hardcopies are not to be sent to MDA.