

GUIDELINES COVERING RESEARCH GRANTS

Advancement of Research for Myopathies (ARM)

PURPOSE & MISSION

ARM purpose and goal is to accelerate biomedical research aimed at developing treatments for Autosomal Recessive Vacuolar or Inclusion Body Myopathies associated with GNE mutations (IBM2-MIM:600737, Nonaka/DMRV-MIM:605820), and skeletal muscle regeneration. ARM's mission is to accomplish this goal in the most efficient manner possible, with special emphasis on considerations of results/duration/cost value of proposed research.

ONLY TWO GRANT REVIEW ROUNDS ARE HELD EACH YEAR. ONLY ONE "REQUEST FOR GRANT APPLICATION" IS PERMITTED PER GRANT REVIEW ROUND. You may obtain the latest grant guidelines online at <http://www.hibm.org/grantguideline>.

SPRING REVIEW

Request for Grant Application:
Submission of Application:
Pre-Application Deadline: December 15,
Application Deadline: January 15,
Award Start Date: July 1

FALL REVIEW

Request for Grant Application:
Submission of Application:
Pre-Application Deadline: June 15,
Application Deadline: July 15,
Award Start Date: January 1

CONTACT

Attn: Grants Manager
Advancement of Research for Myopathies (ARM)
Grants Manager - Research Department
PO Box 261926
Encino, CA 91426-1926
Tel: (800) 276-2000
Fax: (818) 337-7250
grants@hibm.org

Please do not use a private carrier such as FedEx or AirbornExpress because they will not deliver to a U.S. Post Office Box. Thank you.

GUIDELINES COVERING ARM RESEARCH GRANTS

Table of Contents

SECTION A - PROGRAMS AND APPLICATIONS

- I. TYPE AND PURPOSE OF RESEARCH GRANTS
- II. APPLICATION PROCEDURE
- III. DEADLINE DATES
- IV. APPLICATION REVIEW
- V. PATENT AND LICENSING POLICY INFORMATION

SECTION B - RESEARCH GRANT PROGRAM

- I. ELIGIBILITY
- II. DURATION OF GRANTS
- III. DELAY IN ACTIVATION
- IV. GRANT PAYMENT
- V. AUTHORIZED EXPENSES
- VI. UNAUTHORIZED EXPENSES
- VII. SUPPORT FROM OTHER SOURCES
 1. ALTERNATE FUNDING
 2. SUPPLEMENTAL FUNDING
- VIII. BUDGET REVISIONS
- IX. EXPENDITURES BEYOND GRANT EXPIRATION DATE
- X. CHANGE IN STATUS
 1. PROLONGED ABSENCE
 2. MOVE TO NEW INSTITUTION
 3. WITHDRAWAL FROM PROJECT
 4. CANCELLATION OF GRANT
 5. CURRICULUM VITAE

SECTION C - RESEARCH DEVELOPMENT GRANT PROGRAM

- I. ELIGIBILITY FOR RESEARCH DEVELOPMENT GRANTS
- II. DURATION OF GRANTS
- III. DELAY IN ACTIVATION
- IV. GRANT PAYMENT
- V. AUTHORIZED EXPENSES
- VI. UNAUTHORIZED EXPENSES
- VII. SUPPORT FROM OTHER SOURCES
 1. ALTERNATE FUNDING
 2. SUPPLEMENTAL FUNDING
- VIII. CHANGE IN STATUS
 1. MOVE TO NEW INSTITUTION
 2. CANCELLATION OF GRANT
 3. RELINQUISHMENT OF GRANT
- IX. TERMS OF AWARD

SECTION D - RESEARCH REPORTS AND PUBLICATIONS

- I. REPORT OF EXPENDITURES
- II. REPORT OF PROGRESS
- III. PUBLICATIONS AND NEWS RELEASES

SECTION E - HUMAN AND/OR ANIMAL SUBJECTS/TISSUES

- I. RESEARCH PROTOCOL
- II. FOOD AND DRUG ADMINISTRATION
- III. PATIENT CHARGES
- IV. ANIMAL RESEARCH
- V. CONFLICT OF INTEREST

SECTION "A": PROGRAMS AND APPLICATIONS

I. TYPE AND PURPOSE OF RESEARCH GRANTS

1. PRIMARY RESEARCH (Section "B"): To advance areas of scientific and medical knowledge that could improve understanding of the causes of disease or assist in developing strategies for treatment.
2. RESEARCH DEVELOPMENT (Section "C"): To expand the number of scientists conducting meritorious biomedical research in an attempt to significantly advance the rate of scientific discovery towards developing a treatment for IBM2 (HIBM). Interested scientists may come from among investigators who may be part of a team in the laboratory of a senior investigator under whose guidance the researcher will be given flexibility to work independently or as part of a collaborative effort.
3. SPECIAL RESEARCH: To encourage scientific meetings for researchers to exchange ideas and to establish collaborations.

II. APPLICATION PROCEDURE

Applications are not provided to institutions for general distribution. Grant applications are sent to qualified applicants only. An application may be submitted and accepted at ARM's sole discretion and is based on the nature of the research proposed and the qualifications of the applicant. In order to receive an application, a Pre-Application form must be completed and submitted to ARM for review.

III. DEADLINE DATES

The Pre-Application form is due December 15 for a grant to begin the following July 1, or June 15 for a grant to begin the following January 1. The completed application is due January 15 for a grant to begin the following July 1, or July 15 for a grant to begin the following January 1.

IV. APPLICATION REVIEW

To ensure support of meritorious biomedical research, applications are peer-reviewed to assess their scientific merit and to evaluate their relevance to ARM's goals. ARM's Board of Directors has the sole authority to award research grants.

V. PATENT AND LICENSING POLICY INFORMATION

Grants awarded through ARM's Research Program are subject to the ARM's Patent Policy. By accepting a grant offered through ARM's Research Program, the Principal Investigator, all personnel contributing to and working on the respective project, as well as the institution with which they are affiliated, agree to be bound by the terms and conditions of ARM's most recent policy on patents and licensing.

SECTION "B": PRIMARY RESEARCH GRANT PROGRAM

I. ELIGIBILITY

Those eligible to apply for an ARM Research Grants must:

1. Hold a Doctor of Medicine, Doctor of Philosophy, Doctor of Science or equivalent degree.
2. Have access to resources necessary to conduct the proposed research project;

Proposals from applicants outside the United States will be considered for projects of highest priority to ARM and when, in addition to the applicant's having met the requirements noted above, one or more of the following conditions exist:

1. The applicant's country of residence has inadequate sources of financial support for biomedical research on recessive Hereditary Inclusion Body Myopathy (MIM: 600737)
2. Collaboration with an ARM-supported U.S. investigator is required to conduct the research;
3. An invitation to submit an application has been extended by ARM.

II. DURATION OF GRANTS

Research grant awards are for one, two or three years. Payments for years two and three are contingent upon the availability of research funds, submission of respective progress reports satisfactory to ARM and confirmation that appropriate Institutional Review Board ("IRB") approval is current and on file at the institution.

III. DELAY IN ACTIVATION

The activation of a research grant by the Principal Investigator may not be delayed. A Principal Investigator who is unable to begin his or her grant on its designated start date must relinquish the award and reapply.

IV. GRANT PAYMENT

Checks are made payable to the grantee's institution and are issued quarterly. The institution's financial officer should establish an account from which research expenses may be paid under the terms of the approved award.

V. AUTHORIZED EXPENSES

When ARM deems them justified by the research, the expenses identified below are permitted under the ARM research grants program:

1. Technicians', research assistants' and post-doctoral fellows' salaries and fringe benefits at levels appropriate to the institution;
2. Laboratory and research consumables and supplies necessary to fulfill the project's specific aims
3. Travel expenses:
 - a. Directly related to the implementation of the research;
 - b. Expressly and solely for the purpose of reporting the results of ARM-supported research at suitable scientific or medical meetings;
 - c. Limited to \$1,000 maximum per year;
4. Costs associated with making the products of the research (i.e., cell lines, DNA, protein and other biological substances) available to others for research;

VI. UNAUTHORIZED EXPENSES

The following expenses are not permitted under the ARM research grants program:

1. Any indirect or other administrative costs.
2. Laboratory Equipment and Computer hardware (i.e., PC's, printers, monitors, etc.). ARM seeks to fund laboratories that are well equipped to perform the research proposed. Only under special circumstances, funding for research equipment may be considered by the Board of Directors of ARM. Unless otherwise stipulated at the time of the award, equipments purchased with ARM funds belong to and is considered the property of ARM.
3. Salary or fringe benefits for the Principal Investigator, collaborating Investigators or co-Investigators (except where the recipient of a Development Grant will be deemed the "Principal Investigator" on the ARM funded project);
4. Salaries, travel and/or housing related to sabbatical leaves;
5. Salaries for secretarial and/or clerical staff;
6. Costs associated with publication of the research;
7. Purchase or rental of office equipment; (i.e., typewriters, word processors, furniture, filing cabinets, and copy machines);
8. Expenses normally covered by the indirect cost of the Principal Investigator's institution;
9. Fees for tuition, registration or other fees relating to academic studies;
10. Membership dues, subscriptions, books or journals; and/or
11. Expenses for or related to moving from one institution to another.

VII. SUPPORT FROM OTHER SOURCES

1. **ALTERNATE FUNDING:** An Applicant may not apply for, use or accept ARM funds for a research project or part of a project already supported for the SAME PURPOSE either by ARM or by funds from another public or private source. Accordingly, full disclosure of all funds for research support available to the Principal Investigator from private, governmental and institutional sources, including ARM, is required. Such disclosure must be made in the research grant application. If funds from other sources become available to the applicant during the review or tenure of an ARM grant then, the Principal Investigator must so inform ARM's Research Department in writing. ARM will then make a decision about the allocation of its research award.
2. **SUPPLEMENTAL FUNDING:** Financial support for clearly different aspects of one project or parts of a project from separate funding sources is permitted under ARM grants. Such supplementary funding must

be disclosed, fully, to ARM as part of the research grant application or at the time such funding is received.

VIII. BUDGET REVISIONS

ARM requires the submission of a revised budget when the grant awarded is less than originally requested. The revised budget must reallocate the amount awarded for items requested in the original budget - except for any items specifically described in the award letter that must be deleted from the budget. A revised budget must be submitted to ARM's Research Department and to the business office of the institution within four (4) weeks of receipt of ARM's Notice of Award.

Subsequent to budget approval the Principal Investigator must submit a written request to ARM's Research Department for authorization to reallocate funds totaling more than fifteen percent (15%) of the current annual budget or to reappoint personnel or change equipment purchases. Such requests must include the amount of the reallocation and a detailed justification. Requests for budget revisions may be submitted up to four (4) weeks prior to the termination date of an award. ARM does not permit budget revisions exceeding twenty-five percent (25%) of the total approved annual budget. Reallocations are permitted only during the current funding year.

IX. EXPENDITURES BEYOND GRANT EXPIRATION DATE

Expenditures may not be committed against a grant after its expiration date except when authorized in writing by ARM's Research Department.

Under exceptional circumstances, at the termination of the grant unexpended funds may be used for a period of either three (3) or six (6) months beyond the grant's expiration date. The Principal Investigator must request in writing such an extension of the use of grant funds. The request must state the amount of unexpended funds, how those funds will be used during the extension period and provide a detailed justification satisfactory to ARM. Such a request must be made no later than four (4) weeks after the termination date of the award. If funds are not completely expended and a second or third year of support still exists, any carry-over of remaining funds to the next year of support is limited to 10% of the total award for that year. Such carry-over of funds must be requested in writing no later than four (4) weeks after the termination date of that year of support, and the request must specify how those funds will be used in the following year.

X. CHANGE IN STATUS

The continued use of grant funds following any major change in status of the Principal Investigator requires prior written authorization from ARM. As described below, such changes include but are not limited to prolonged absence, change in institution or withdrawal from the project.

1. **PROLONGED ABSENCE:** Continued use of funds by or reassignment of a project to another qualified investigator during a prolonged absence of the Principal Investigator (excluding institutionally authorized vacation) requires prior written ARM authorization. The Principal Investigator must write to the ARM Research Department requesting such authorization at least six (6) weeks before the starting date of the period of absence. The request must contain an explanation of the reasons for the absence and details about the arrangements made for conducting the research project during the absence. The letter must include the following:
 - a. Inclusive dates of absence;
 - b. Reason(s) for absence;
 - c. Name, address, telephone number, and curriculum vitae of the investigator who has agreed to be responsible for the scientific conduct of the research project;
 - d. Proposed method and frequency of communication between the Principal Investigator and the investigator-in-charge;
 - e. Signature of the investigator referred to in item "c" above confirming that he or she is familiar with all aspects of the project and accepts full responsibility for the conduct of the research during the absence of the Principal Investigator.

When a request for continued use of grant funds during a prolonged absence of the Principal Investigator is not authorized, the grant is terminated and all unexpended funds plus unexpended accrued interest, if any, must be returned to ARM accompanied by a Report of Expenditures within eight (8) weeks of the date of termination.

2. **MOVE TO NEW INSTITUTION:** Continued use of funds by a Principal Investigator who changes institutions requires prior written authorization from ARM. The Principal Investigator must write to the ARM Research Department requesting such authorization at least eight (8) weeks before the effective date of change in institution. The letter must include:
 - a. Effective date - month/day/year - of change in institution;
 - b. Titles and periods of support of all ARM grants affected by the change in institution;
 - c. Complete address of the new institution. The new mailing address of the Principal Investigator should also be included if it differs from that of the new institution;
 - d. Statement of the adequacy of the new institution's facilities for conducting the research projects identified in item "b" above.

When continuation of a grant and/or a transfer of funds to a new institution are authorized, a new application cover sheet signed by the Principal Investigator's new institution is required. Instructions for transfer of funds between institutions will be provided by ARM's Research Department. When a transfer is not authorized, the grant is terminated and all unexpended funds plus unexpended accrued interest, if any, must be returned to ARM accompanied by a Report of Expenditures within eight (8) weeks of the termination of that award.

3. **WITHDRAWAL FROM PROJECT:** When a Principal Investigator withdraws from a project, his/her grant terminates and all unexpended funds plus unexpended accrued interest, if any, must be returned to ARM accompanied by a Report of Expenditures within eight (8) weeks of the withdrawal from the project. Under exceptional circumstances, a grant may be continued under a new Principal Investigator at the same institution. In such cases the Principal Investigator must write ARM's Research Department requesting authorization for such a continuation at least eight (8) weeks before the effective date of withdrawal from the project. The following documentation must be provided:
 - a. Effective date - month/day/year - of the change in Principal Investigator;
 - b. Updated progress report on the project;
 - c. Name, address and curriculum vitae of the proposed new Principal Investigator

The proposed new Principal Investigator must, in a separate letter, indicate to ARM his/her familiarity with the specific aims of the project and agree to accept responsibility for all scientific and administrative aspects of the research grant and also provide a statement about the availability of equipment, personnel, etc., necessary to conduct the research.

4. **CANCELLATION OF GRANT:** If, for any reason, the recipient of a grant must relinquish the award, the Principal Investigator should promptly so notify ARM's Research Department in writing. The notification should state the effective date of cancellation of the grant. Unexpended grant funds plus unexpended accrued interest, if any, must be returned to ARM accompanied by a Report of Expenditures within eight (8) weeks of the cancellation date. ARM reserves the right to cancel a grant if circumstances render the individual on whose behalf the award was made unfit, unqualified and/or unable to perform under the terms and conditions of this Research Grants Policy. Such circumstances include, but are not limited to, abandonment of the project, loss of license, conviction of a crime, or withdrawal of insurance or other material institutional protections.

XI. CURRICULUM VITAE

Curriculum Vitae (CV) of all investigators, advisors, co-investigators and post-doctoral fellows who will be participating in the execution of the research project must be provided to ARM with the grant application. When a project is underway, ARM's Research Department must be informed immediately in writing of any change in personnel participating in the project, the reason(s) for such a change, and be provided the curriculum vitae of any additional or replacement personnel.

SECTION "C": RESEARCH DEVELOPMENT GRANT PROGRAM

I. ELIGIBILITY FOR RESEARCH DEVELOPMENT GRANTS

In addition to all the requirements stated under Section B, those eligible to apply for an ARM Research Development Grant must:

1. Have an acceptable research plan for accelerating the development of an effective treatment for recessive Hereditary Inclusion Body Myopathy; and
2. Have access to institutional resources necessary to conduct the proposed research project.

A Principal Investigator who is assigned his or her own laboratory during the term of a Research Development Grant would be ineligible to continue to receive ARM funding through this aspect of the ARM's research grants program.

Proposals from applicants outside the United States will be considered only for projects of highest priority to ARM and when, in addition to the applicant's having met the eligibility requirements noted above, one or more of the following conditions exist:

1. The applicant's country of residence has inadequate sources of financial support for biomedical research;
2. Collaboration with an ARM-supported U.S. investigator is required to conduct the research; or
3. An invitation to submit an application has been extended by ARM.

II. DURATION OF GRANTS

Awards are for one, two or three years at a maximum level of \$45,000 per year. Payments for years two and three are contingent upon the availability of research funds, submission of respective progress reports satisfactory to ARM and confirmation that appropriate Institutional Review Board ("IRB") approval is current and on file at the institution.

III. DELAY IN ACTIVATION

The activation of a research grant by the Principal Investigator may not be delayed. A Principal Investigator who is unable to begin his or her grant on its designated start date must relinquish the award and reapply.

IV. GRANT PAYMENT

Checks are made payable to the institution and are issued quarterly. The institution's financial officer should establish an account from which research expenses may be paid under the terms of the approved award. The amount authorized by ARM for institutional overhead may be disbursed as the institution deems appropriate providing that such institutional overhead relating to the Principal Investigator of the ARM-funded project is fully covered.

V. AUTHORIZED EXPENSES

The following expenses are, when ARM deems them justified by the research, permitted under the institutional overhead:

1. Salary and fringe benefits:
 - a. Development Grants are limited to salary for the Principal Investigator only;
2. Equipment and supply expenses necessary to fulfill the project's specific aims. Unless otherwise stipulated at the time of the award, equipment purchased solely with ARM funds belongs to and is considered the property of ARM. Development Grant equipment is limited to \$2,000 in any given year;
3. Travel expenses:
 - a. Directly related to the implementation of the research;
 - b. Expressly and solely for the purpose of reporting the results of ARM-supported research at suitable scientific or medical meetings;
 - c. Limited to \$1,000 maximum per year;
4. Costs associated with making the products of the research (i.e., cell lines, DNA, protein and other biological substances) available to others for research;

VI. UNAUTHORIZED EXPENSES

The following expenses are not permitted under ARM's research program:

1. Any indirect or other administrative costs.
2. Costs associated with publication of the research;
3. Salary or fringe benefits for anyone other than the Principal Investigator (limited to Development Grants only);
4. Salaries, travel and/or housing related to sabbatical leaves;
5. Purchase or rental of office equipment; (i.e., typewriters, word processors, furniture, filing cabinets, and copy machines);

6. Expenses normally covered by the indirect cost of the Principal Investigator 's institution;
7. Fees for tuition;
8. Membership dues, subscriptions, books or journals.
9. Expenses for or related to moving from one institution to another.

VII. SUPPORT FROM OTHER SOURCES

1. **ALTERNATE FUNDING:** A Principal Investigator may not apply for, use or accept ARM funds for a research project or part of a project already supported for the SAME PURPOSE either by ARM or by funds from another public or private source. Accordingly, full disclosure of all funds for research support available to the Principal Investigator from private, governmental and institutional sources, including ARM, is required. Such disclosure must be made in the research grant application. If funds from other sources become available to the Principal Investigator during the review or tenure of an ARM grant, then, the Principal Investigator must so inform ARM's Research Department in writing. ARM will then make a decision about the allocation of its research award.
2. **SUPPLEMENTAL FUNDING:** Financial support for clearly different aspects of one project or parts of a project from separate funding sources is permitted under ARM grants. Such supplementary funding must be disclosed, fully, to ARM as part of the research grant application or at the time such funding is received.

VIII. CHANGE IN STATUS

1. **MOVE TO NEW INSTITUTION:** Continued use of funds by a Principal Investigator who changes institutions requires prior written authorization from ARM. The Principal Investigator must write to the ARM Research Department requesting such authorization at least eight (8) weeks before the effective date of change in institution. The letter must include:
 - a. Effective date - month/day/year - of change in institution;
 - b. Complete address of the new institution. The new mailing address of the Principal Investigator should also be included if it differs from that of the new institution;
 - c. Statement of the adequacy of the new institution's facilities for conducting the research projects identified in item "b" above.
 - d. Statement from Principal Investigator's new mentor at the institution, accepting the Principal Investigator and the responsibility of the award.

When continuation of a grant and/or a transfer of funds to a new institution are authorized, a new application cover sheet signed by the Principal Investigator's new institution is required. Instructions for transfer of funds between institutions will be provided by ARM's Research Department. When a transfer is not authorized, the grant is terminated and all unexpended funds plus unexpended accrued interest, if any, must be returned to ARM accompanied by a Report of Expenditures within eight (8) weeks of the termination of that award.

2. **CANCELLATION OF GRANT:** If, for any reason, the recipient of a grant must relinquish the award, the Principal Investigator should promptly so notify ARM's Research Department in writing. The notification should state the effective date of cancellation of the grant. Unexpended grant funds plus unexpended accrued interest, if any, must be returned to ARM accompanied by a Report of Expenditures within eight (8) weeks of the cancellation date. ARM reserves the right to cancel a grant if circumstances render the individual on whose behalf the award was made unfit, unqualified and/or unable to perform under the terms and conditions of this Research Grants Policy. Such circumstances include, but are not limited to, abandonment of the project, loss of license, conviction of a crime, or withdrawal of insurance or other material institutional protections.
3. **RELINQUISHMENT OF GRANT:** A Development Grant recipient must relinquish his or her award upon receiving a tenure-track faculty position at an institution. ARM will allow a six-month transition period, beginning the first day of the month in which a recipient's status changed. Payment will continue during this transition period. The award will be pro-rated according to the time expended on the award.

IX. TERMS OF AWARD

All other provisions described in this policy guide applying to research grants specifically not covered in this Section "C" shall apply to Research Development Grants.

SECTION "D": RESEARCH REPORTS AND PUBLICATIONS

I. REPORT OF EXPENDITURES

A Report of Expenditures form will be mailed to the financial officer of the Principal Investigator's institution with a copy of the award letter. The financial officer of the institution must, within twelve weeks of the conclusion of each funding year of the grant, return the completed form to ARM with a check in the amount of all uncommitted and unexpended funds plus any unexpended accrued interest. When unexpended funds are not returned with the Report of Expenditures, the Report of Expenditures will be considered unacceptable and will be returned to the financial officer of the awarded institution. In such cases, ARM will expect the financial officer to remit payment in full within four (4) weeks.

II. REPORT OF PROGRESS

Brief 1-2 page research progress updates must be submitted quarterly. Formal scientific Progress Reports must be submitted semi-annually. A final report must be submitted no later than four (4) weeks following the grant termination date. ARM may require additional progress reports at any time during an award period as a condition of continuing the award.

III. PUBLICATIONS AND NEWS RELEASES

ARM expects timely public disclosure and/or publication of the results of all research projects it supports and requires that every such publication - whether in peer-reviewed journals, meeting abstract formats, or in review articles or similar publications - contain the following statement or its equivalent: "Supported by Advancement of Research for Myopathies (ARM) of California, USA."

Funds to support ARM's research program come primarily from donations from private citizens. It is essential to the growth and maintenance of ARM and its research program that these donors are kept fully informed of the research progress their contributions make possible. Individuals and families affected by IBM2 (HIBM) must also be kept fully informed of research progress. For these purposes ARM often issues press releases on newsworthy research developments and produces various publications for the public that report research activities. Such a press release or report may be issued on the occasion of the publication of an article in a professional journal or a presentation at a scientific or medical meeting.

To avoid misinterpretation of research results or the raising of false hopes about a possible treatment or cure for diseases covered under ARM programs, the ARM requires the cooperation of the Principal Investigator in providing ARM with advance prepublication copies of all articles and abstracts reporting the results of ARM-supported research which ARM shall keep confidential. ARM also requires the cooperation of its Principal Investigators in participating in interviews as ARM may deem necessary. This cooperation will enable ARM to prepare press releases or other reports ARM issues on the research it supports.

SECTION E: HUMAN AND/OR ANIMAL SUBJECTS/TISSUES

I. RESEARCH PROTOCOL

When human subjects, tissues and/or materials are to be used in a research project, it is the responsibility of the Principal Investigator and the institution to ensure that the institution has the following on file:

1. A complete copy of the research protocol approved by the Institution's Human Subjects Review Board and a copy of that Board's current approval notice;
2. A copy of the patient informed consent form(s) to be used.

A copy of the Board's current approval notice and a copy of the patient informed consent form must be submitted with the application and upon renewal. 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 ARM. Principle Investigators must advise ARM about any amendments to the original research protocol (including the participant consent form) occurring prior to the commencement of or during the course of the research project.

II. FOOD AND DRUG ADMINISTRATION

When experimental drugs and/or experimental medical devices are to be administered to patients, the materials, required in the "Research Protocol" section "E" of this document, are necessary. In addition, it is the responsibility of the Principal Investigator and the institution to ensure that the institution has the following on file:

1. A complete copy of the Investigational New Drug (IND) and/or Investigational Device Exemption (IDE) application approved by the Federal Food and Drug Administration (FDA) and a copy of the FDA's approval notice; and
2. Copies of all correspondence during the application and award periods between the FDA and the ARM Principal Investigator pertaining to the experimental drug(s) and/or device study.

III. PATIENT CHARGES

ARM requires that patients participating in experimental drug and/or device studies not be charged directly for any research procedures included under the project's approved protocol. Patients must be fully advised about their responsibility for ancillary costs relating to participation in a research project -- travel, lodging, food, etc.

IV. ANIMAL RESEARCH

ARM investigators should use animals and animal tissues for research purposes only when reasonable and practical alternatives do not exist. When attainment of the specific aims of a project requires the use of animals and/or animal tissues, a detailed justification must be included in the research grant application submitted to ARM. The justification shall include statements confirming that institutional guidelines:

1. Are at least as protective as those of the National Institutes of Health;
2. Conform to all applicable laws and regulations;
3. Meet prevailing community standards for responsible scientific research;
4. Apply throughout the project to ensure the humane treatment of any animals involved in the project.

It is the responsibility of the institution to ensure that no ARM funds will be released for research involving humans and/or animals until the required documentation described above is on file with the appropriate official at the institution as well as ARM.

V. CONFLICT OF INTEREST

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 ARM Principal Investigators will observe the highest ethical standards in the conduct of research.

Revised 1/2003- Terms of this policy are subject to revision or alteration at any time

PATENTS AND LICENSING POLICY
ADVANCEMENT OF RESEARCH FOR MYOPATHIES (ARM)

All ARM grants are subject to ARM's Policy on Patents and Licensing. By accepting an ARM award for a research project, the Principal Investigator or other personnel contributing to and working on the Project, as well as the Institution(s) with which they are affiliated, agree to be bound by the terms and conditions of ARM's Patents and Licensing Policy.

ADVANCEMENT OF RESEARCH FOR MYOPATHIES, INC., A NON-PROFIT ORGANIZATION (ARM) understands that patents and licensing agreements may be sought on inventions resulting from research by the grant recipient supported in whole or in part by funds furnished by ARM; that such inventions should be administered so that they are introduced into public use as soon as practicable; and that such result will be achieved through granting permission to patent and license such inventions. Accordingly, it adopts the following policy:

1. An invention (hereinafter "ARM invention") resulting from the support in whole or in part to the grant recipient of funds awarded by ARM shall be reported to ARM promptly in writing.
2. If the university or other research institution or an individual investigator(s) associated therewith ("Institution" or "Investigator") which is the recipient of financial support for the work leading to the ARM invention, has an established patent and licensing policy and procedure for procuring and administering inventions which are known to and accepted by ARM, or has an agreement with another organization, including agencies or departments of the U.S. Government, relating to the ARM invention due to joint support, ARM will defer to that policy or agreement on the following terms:
 - a. With respect to any such invention, the Institution or Investigator shall have the right to file a patent application, and if the Institution or Investigator decides not to file a patent application ARM shall be notified thereof within a reasonable time and thereupon ARM shall have the right to file a patent application. On any Institution- or Investigator-filed or ARM-filed patent application and on any patent obtained thereof or thereon, ARM shall have a nonexclusive, nontransferable, irrevocable, paid-up license to practice or have practiced for and on its behalf any such invention and to grant sublicenses there under. The patent application(s) and patent(s) obtained thereon shall embrace the United States and all countries outside of the United States. The inventions hereinabove contemplated shall include those made by employees or agents of the Institution or Investigator and third parties under the control of the Institution or Investigator.
 - b. The Institution or Investigator will notify ARM in writing of any decision not to continue the prosecution of a patent application, pay maintenance fees, or defend a reexamination or opposition proceeding on a patent, in any country, not less than thirty days before the expiration of response period required by the relevant patent office. The Institution or Investigator will convey to ARM, upon written request, title to any such patent application or patent.
 - c. The Institution or Investigator will make the invention available for commercial licensing upon reasonable terms and conditions.
 - d. From the monies, if any, received from licensing the invention, ARM and the Institution or Investigator and all other parties shall share on terms mutually agreed upon by the Institution or Investigator and ARM, such terms to be determined prior to any licensing or commercial exploitation of the invention with ARM's share being at least equal to the percent of total funding that ARM has financially supported the specific research project through grants and awards.
 - e. In the event that it obtains a patent, license arrangement or other commercial exploitation of an invention, the Institution or Investigator shall make periodic reports to ARM with respect to its utilization of the invention and account for any income received by it by reason of exploitation of the invention.
 - f. The Institution or Investigator or its licensee will use its best efforts to make ARM inventions available for the public benefit within a reasonable period of time. In circumstances of unreasonable delay, ARM shall have the right to require assignment of the patent or the invention to it; cancellation of any outstanding exclusive licenses granted relating to the invention and particularly under said patent; and the granting of any such licenses to a party designated by ARM on a nonexclusive royalty-free basis or on terms that are reasonable in the circumstances.
3. If the Institution or Investigator has no patent or licensing policy and procedure for administering inventions, ARM shall have the right to determine the disposition of the invention rights in any such case.