Myhre Syndrome Foundation

RESEARCH GRANT POLICIES

1. Protecting the rights and welfare of human research participants is a top priority of the Myhre Syndrome Foundation (MSF). Federal regulation and MSF policy require that all institutions maintain appropriate policies and procedures to protect human subjects. In addition, MSF encourages a research environment in which ethical and productive investigation is valued. If applicable, a copy of the informed consent form for any human subjects for the project, as well as proof of current project approval by an Institutional Review Board (IRB) (or a similar oversight group), must be provided to MSF before funding for a project will be released by MSF.

2. Similarly, it is an MSF policy that all projects must conform to regulations for the safe and humane treatment of animals. Specifically, MSF stipulates that animals can be used in MSF-funded research only when no other means of obtaining scientifically sound, valid, and useful results are available. Furthermore, only the minimum number of appropriate animals required to obtain and validate results should be used. In cases requiring the death of an animal, only the most appropriate and humane form of animal sacrifice consistent with the purpose of the research shall be employed. If applicable, a copy of proof of current project approval by the institution's Animal Use and Protection Committee (IACUC or a similar oversight group) must be provided to MSF before any funding for the project will be released by MSF.

3. MSF expects that any results and accomplishments from research it has funded will be made public (preferably through a high-quality, peer-reviewed journal article) to maximize progress toward improved understanding, treatments, and ultimately the discovery of a cure for Myhre syndrome.

4. MSF also expects that "Materials" from research funded by the organization will be made available to other researchers reasonably and quickly. "Materials" means reagents useful for scientific or preclinical investigation, animal and cellular models, and any other physical inventions useful in further scientific or preclinical work other than intellectual property that are at least partially funded by this award and unencumbered by either i) existing University intellectual property or contractual obligations as of the Project Start

Date and/or ii) subsequent University intellectual property or contractual obligations related to work that was not funded by MSF. MSF considers an optional one-year period of exclusivity for the creator laboratory to be reasonable, and distribution should be able to commence with the first publication. Furthermore, such Materials are expected to be made available to non-commercial laboratories at cost and to commercial enterprises for a reasonable licensing fee. Please see **Addendum A** for exceptions.

5. MSF recognizes that in the effort to develop therapies for Myhre Syndrome, the grant recipient may need to maintain confidentiality so that intellectual property rights can be preserved and patent applications filed appropriately. The grant recipient may enter into a for-profit enterprise to pursue and/or commercialize discoveries made in whole or in part with funding from MSF. For those situations where a for-profit enterprise is anticipated or the grant recipient is part of a for-profit company, please see **Addendum A.**

6. MSF will not challenge any decisions concerning patent applications that might be made by a grantee for inventions resulting from work performed under an MSF research grant. All inventions or discoveries made in whole or in part with funding from MSF must be reported to MSF as soon as possible. The grantee must notify MSF immediately of any decision to apply for a patent or other legal protection. This information will be kept confidential.

7. In any published research report funded in part or in whole by an MSF research grant, MSF must be cited as a source of funds, and MSF must be sent a copy of the published material or paper.

8. A yearly progress report on the research that resulted from the MSF grant is required and sent to MSF on the grant commencement date anniversary by the Principal Investigator. In addition, a layman's summary (which can be published by MSF as is or in edited form) must be submitted.

9. A final detailed accounting of the funds spent must be sent to MSF within 60 days of the end of the grant period. If the grant period is longer than one year, then annual financial reports must be filed with MSF on the anniversary of the grant commencement date. At the end of the grant period, any unused funds must be returned to MSF unless a letter explaining the situation and requesting an extension of the grant period (without

additional funds) is submitted at least 90 days ahead of the end of the grant period and approved by MSF.

10. Any change in a budget *category* equal to or greater than 10% of the total research grant amount must be requested by the Principal Investigator to MSF in writing and approved by MSF before being executed.

11. MSF must be notified immediately if there is to be any change of the Principal Investigator or the Institution with which he/she is associated. Furthermore, if the Principal Investigator is to be absent from the grantee Institution for more than 30 days, then MSF must be notified. MSF reserves the right to request a written status report on the progress of the research project and an accounting of the funds spent as of the request date and to determine the final dispensation of any research grant funds remaining in any such situations.

12. MSF reserves the right to suspend or cancel a grant at any time at its sole discretion for failure to abide by MSF policies governing research grants. Upon receipt of notice of project suspension or cancellation by the Institution, MSF's financial support of further work on the project will cease. At that point, the Principal Investigator must prepare and submit a project status report to MSF, while the grantee's institution must submit a complete accounting of funds expended to date. All unused funds must be returned to MSF immediately upon its request. A grantee may terminate a grant by sending notice in writing to MSF, providing MSF with a written accounting of funds expended to date and a project status report sufficient in detail for a third party to replicate and continue the research project, and returning all unused funds to MSF.

13. To properly evaluate the applications, each research grant application will be reviewed by two (2) members of the MSF PAB (Professional Advisory Board) who have not also submitted applications in that grant cycle and two (2) external reviewers with relevant knowledge and expertise to the submitted application. If two (2) eligible PAB members are unavailable to participate in the review, additional external reviewer(s) will fill the role. The applicant should furnish MSF with a list of three individuals who would be appropriate outside reviewers. These individuals must be qualified to review your application, have not worked with you during the last three years, and have no conflict of interest. In addition, the applicant should make known to MSF at the time of application submission if there are any MSF PAB members or other outside experts whose review of the application would constitute a conflict of interest.

14. Some information about research grants awarded by MSF will be made available to its constituents and to the general public. This information will include the title the project, the Principal Investigator, the grantee Institution, the grant award amount, and the abstract provided as part of the grant application. No privileged or confidential information or trade secrets previously identified as such to MSF will be divulged.

15. The nature of this arrangement is a funding agreement; no employment or agency relationship is hereby created.

16. The Principal Investigator and the grantee Institution indemnify and hold harmless MSF, its Board, officers, agents, advisors, and constituents from any claim, judgment, award, damage, settlement, liability, negligence or malpractice arising from research or investigation related to this MSF research grant.

Addendum A

INTELLECTUAL PROPERTY ISSUES WITH THE MSF RESEARCH GRANT PROGRAM

Because the development of pharmaceuticals to treat human disease often requires the participation of for-profit biotechnology or pharmaceutical companies, MSF recognizes that special considerations may need to be implemented. For example, to maintain intellectual property rights that allow commercialization, the need to publish may be delayed for some period of time. Often the researcher will need to patent discoveries to preserve commercial viability. In consideration of these special circumstances with a for-profit enterprise, or with an awardee that licenses Intellectual Property or Materials, that receives funding from MSF the following is to be understood:

Myhre Syndrome Foundation is dedicated to serving Myhre Syndrome families by providing a network of hope and support and collaborating with scientific and medical communities to encourage and fund promising research. As a 501(c}(3), charitable organization, MSF intends to do all it can to encourage the commercial development of safe and effective treatments for Myhre Syndrome. MSF understands that the cost for developing commercial products for extremely rare diseases that also meet regulatory requirements may exceed the revenues. The awardee of an MSF Research Grant that is part of a for-profit enterprise or a University that licenses Intellectual Property or Materials will:

- Adhere to MSF Research Grant Policies with the following clarification:

o That publication of study results shall be sufficient to comply with MSF policy that "Materials resulting from research funded by the organization will be made available to other researchers on a reasonable basis;"

o That it shall not be required, at the early stage of development, to make Materials resulting from research funded by the organization “available to non-commercial laboratories at cost and to commercial enterprises for a reasonable licensing fee.” After publication of study results describing Materials, then the usual requirements for allowing others to repeat the experiments of the published author will take precedence.

- Be expected to diligently pursue opportunities to license Materials and “Intellectual Property” that is funded, at least in part, by MSF. If the awardee does pursue licensing opportunities based on research funded, at least in part, by MSF, the awardee has an obligation to notify MSF as soon as practicable. If the awardee does not or chooses not to diligently pursue such licensing opportunities, the awardee will notify MSF such that MSF can pursue other options and will negotiate in good faith to permit use of the Intellectual Property by MSF or third parties identified by MSF.

- Agree that, if and when any Intellectual Property or Materials arise from work that was funded, at least in part by MSF, MSF shall:

o Share in any Net Revenue received from any Intellectual Property (the "IP Consideration"). “Net Revenue” means licensing fees, royalties, or any other income derived from Intellectual Property or Material (“Income”) less unreimbursed out of pocket expenses that are directly related to licensing or the filing prosecution, maintenance or enforcement of a patent for Intellectual Property or Material. In addition, the awardee shall not enter into any agreement that will derogate MSF's right to the IP Consideration and shall notify MSF promptly and in writing of any license, lease, sale, or other agreement concerning an invention developed under the MSF Research Grant Program. MSF's participation shall be calculated on a *pro rata* basis, determined by multiplying any Net Revenue by a fraction, the numerator of which is MSF's grant amount, and the denominator of which is the total direct cost for the Intellectual Property, including all Intellectual Property patent and licensing costs. Notwithstanding the forgoing, the awardee’s share pursuant to the forgoing calculation shall not be less than 50%.

o Share in any Net Revenue, less unreimbursed out of pocket expenses directly related to review of Material encumbrances (“Material Revenue”) received from licensing of a Material (the "Material Consideration"). MSF's portion shall be calculated on a *pro rata* basis, determined by multiplying any Material Revenue by a fraction, the numerator of which is MSF’s award amount, and the denominator of which is the total direct cost of the development of such Material, including patent and licensing costs. Notwithstanding the forgoing, the awardee’s share pursuant to the forgoing calculation shall not be less than 50%.