



Research Funding and Awarding

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The Society's research program is critical since it supports scientists in the early stages of their careers and funds projects that are considered either too speculative or too clinical for consideration by the National Institutes of Health (NIH). Members of the National MPS Society have done an extraordinary job over the years of raising millions of dollars to both support the Society and fund research. As of 2008, the Society will have funded over \$3 million in research.

This fact sheet explains the process that is utilized for the distribution of those funds, as well as the background on the decisions regarding how the funding is spent.

Sources of Funding

The Society's research funding has been designated or restricted for use on research (as opposed to unrestricted uses or for family support) by the type of fundraiser that was held or by the donor. Research funding sources include the National run/walk events, designated donations, family fundraisers, and foundation requests.

The National run/walk events proceeds are collected in a general research pool. The donors of the other sources have the ability to designate their funds for the general research pool or to further restrict their funding to research on certain syndromes. Though the National run/walk events are the primary source of funding for the research program, all of the sources are used together to make the Society's research program as successful as possible while respecting the wishes of the families that are working so hard to raise the funds.

Mechanism of the Research Program

The research funds raised by the Society are administered by the Society's Board of Directors, with heavy influence by the Scientific Advisory Board (SAB). The SAB is comprised of medical doctors, clinicians, and researchers that are knowledgeable about MPS and related diseases. The SAB provides the Society's board with general direction for the research program and assists in evaluating specific research grants for funding.

Annually, the board determines how much funding is available for the research program and solicits input from the SAB on how the funds can best be utilized. After considering the SAB's input, the board determines the areas of research focus and the number and amounts of individual research grants. These grants are often in the \$60,000 to \$100,000 range that extends over a two-year period with funding at the beginning of the first and second years. Other syndrome specific research organizations may be invited to partner with the Society in offering syndrome specific research grants to increase the size of the grants.

After advertising and receiving letters of intent, detailed proposals are requested for certain projects which are deemed competitive for funding. After the completion of a competitive evaluation process, individual projects are selected by the board for funding. Upon accepting the research funding, the researcher agrees to provide a written report of findings to the Society at the completion of the first year and upon final completion of the project.

The board's desire is to minimize the amount of time between collecting the funds and starting the research project so that cures and treatments are developed as soon as possible.

General versus Syndrome Specific Research

The SAB strongly believes that the general research category provides the maximum flexibility and effectiveness of the research dollars. This approach allows the focus on symptoms or issues that cross syndromes, such as bone and joint disease, central nervous system issues, etc. Discoveries from these grants have allowed researchers to secure larger grants from the NIH and have moved science closer to the development of effective therapies for these diseases. Some of the most innovative approaches and technologies that drive scientific fields forward by leaps and bounds can come from unexpected areas of research. Additionally, much of the research efforts on one syndrome equally benefit the other syndromes. Therefore, this approach allows the funding of the very best research grants (regardless of the syndrome) instead of just the best grant for a particular syndrome.

In addition, this approach allows the research dollars to be spent as soon as possible. When research dollars are designated for a particular syndrome, it may take two or more years for enough money to be collected to advertise a grant for that syndrome. Once advertised, there have been instances when there were no quality grant requests submitted for that syndrome which further delays the distribution of funds on any type of research.

Summary

The Society's board of directors (many of whom are also run/walk organizers) believe that the overall good of all syndromes is best served by maximizing the amount of general research funding. It allows:

- Funding of the very best research proposals,

- Maximum benefit to all syndromes,
- Most timely expenditure of the funds, and
- Most flexibility to leverage funds of other lysosomal storage disorder groups on joint research projects, such as the Lysosomal Storage Disease Research Consortium.

As a result, the proceeds of the National run/walk events are designated for general research projects with advice and direction from the SAB. In addition, this focus also allows all of the MPS families supporting a run in a particular location (regardless of syndrome) to work together for a common goal. However, the Society encourages and supports all fundraising efforts and accepts syndrome specific research funds from sources other than the National run/walk events to honor the donors' wishes.

The Society is extremely grateful to members who choose to host a fundraising event of any size or who choose to just make an additional donation in addition to their dues. These actions are appreciated by the Society's board of directors and its members.