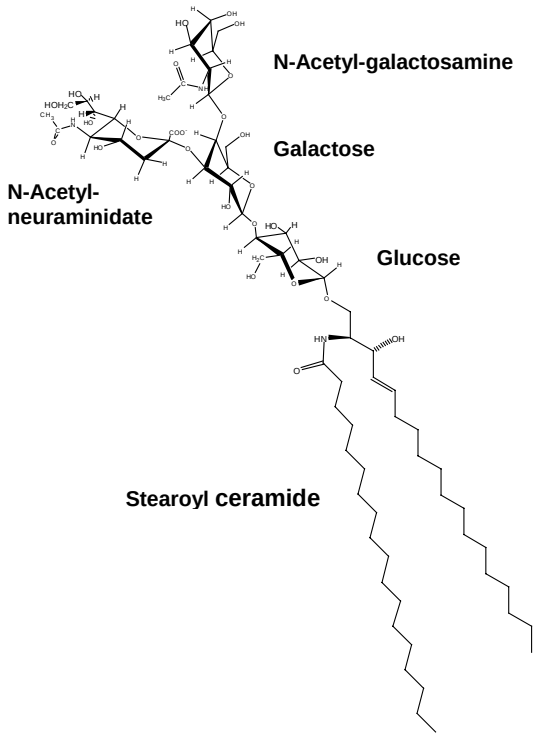
Foundation Mission

The New Hope Research Foundation is a non-profit organization dedicated to helping find a cure for GM2 gangliosidosis and other lysosomal storage diseases and providing hope to patients and their families currently facing the degenerative and debilitating aspects of these diseases. It is the mission of the foundation to coordinate, direct, and financially support activities intended to find an effective genetic cure for patients with GM2 gangliosidosis and other lysosomal storage diseases (LSDs) that affect the central nervous system. These activities include:

- **Research** on gene therapy and the means for infusing or delivering this therapy to correct lysosomal enzyme deficiencies of the central nervous system,
- **Clinical studies** which are intended to show safety and efficacy of gene therapy for the central nervous system aspects of GM2 gangliosidosis and related lysosomal storage diseases,
- **Regulatory applications** required to initiate investigations of clinical studies to allow patient access to proven therapies, and
- **Education and communication** for patients and families on the clinical manifestations and molecular basis of gangliosidosis, and related diseases, on gene therapy, and on the status of research and clinical studies.

LSD Impact on the CNS

Of the more than 40 known lysosomal enzyme deficiencies more than 60% have central nervous system (CNS) disease involvement. The inability of neuronal cells to regenerate makes LSDs with CNS involvement clinically significant and makes early treatment essential. The CNS involvement in LSDs remains the most devastating aspect of these diseases.



Tay Sachs, Sandhoff, and GM2 Activator Protein Deficiency diseases involve excessive lysosomal storage of GM2 gangliosides, such as shown above. Gangliosides were named based on the high concentration of these lipids found in the ganglion cells of the central nervous system.

Why Gene Therapy

There are four therapeutic concepts that are currently being explored to address the CNS aspects of LSDs:

- Enzyme Replacement Therapy (ERT)
- Substrate Reduction Therapy (SRT)
- Small molecule chaperone drugs
- Gene therapy

While there is research to support the possibility of treatment of the CNS aspects of LSDs with ERT, SRT, and chaperone drug therapy, perhaps the greatest opportunity for curing the GM2 Gangliosidosis is the use of gene therapy. The concept has already been demonstrated in multiple small animal models of lysosomal storage disease (MPSVII, MPSI, CLN2) and is currently undergoing human clinical investigation for Batten and Canavan diseases. The effectiveness of gene therapy in mouse models of Tay Sachs disease and Sandhoff disease have also already been shown with some success using viral vectors, such as adeno associated viral (AAV) vector.

While there have been many exciting advances in this area of gene therapy for the LSD's, there is considerable research remaining to bring this promising treatment to clinical trials. Identifying a gene vector and an effective means for delivering it to broad areas of the brain are two key research challenges that must be addressed to bring this therapy forward.

Research Focus

Call for Proposals

The New Hope Research Foundation is soliciting proposals for pre-clinical or clinical research projects that will be beneficial in achieving its mission and charter. Preference will initially be given to proposals directly related to finding cures for GM2 gangliosidosis, but as funding permits, will also be extended to related lysosomal storage diseases affecting the central nervous system. Proposals will be evaluated based on the potential to accelerate the advancement of a genetic cure. Funding would generally be in the range of $10,000 to $40,000 for one year, inclusive of indirect costs not to exceed 10%.

Funding will not be granted in cases where intellectual property will be exclusively retained by the individual or organization conducting the activities and where the intellectual property might later lead to restricting or delaying the therapy availability or might cause economic hardship to patients wishing to receive the therapy.

For more information on application guidelines, please contact the Foundation.

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