Cure Mucolipidosis

Business Plan

Cure
MUCOLIPIDOSIS

2021

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Introduction

Cure Mucolipidosis has a single focus – to find a cure for this rare and debilitating disease

Cure Mucolipidosis is a non-profit organization established as a 501 c 3 on 18th May 2021. The organization is located in Illinois and has a mission to work alongside the **Mucolipidosis Collaborative Research Network** (MCRN) that was established in 2019 to set a pipeline for the investigation of new modes of therapies for Mucolipidosis that will lead to a cure!

Cure Mucolipidosis covers the following group of Mucolipidosis.

- Mucolipidosis II alpha/beta
- Mucolipidosis II/III alpha/beta
- Mucolipidosis III alpha/beta
- Mucolipidosis III Gamma
- Sialidosis (Mucolipidosis I)

Mission

Cure Mucolipidosis is a global organisation who is committed to the identification and treatment of Mucolipidosis through education, advocacy, and research. Cure Mucolipidosis will form partnerships with Science, Medicine, and industry and will work towards finding a cure for people affected by Mucolipidosis globally.

Vision

- To serve as a resource for stakeholders in the work, identification, treatment, and continual developments towards a cure for Mucolipidosis.
- To promote and support a global multi-stakeholder collaboration for Mucolipidosis
- To identify patient numbers globally in preparation for clinical trials and drug company development for a therapy or cure.

Executive Summary

The mucolipidosis (ML) are a group of inherited metabolic diseases that affect the body's ability to carry out the normal processing of various substances within cells. In ML, abnormal amounts of carbohydrates and fatty materials (lipids) accumulate in cells, and these trapped substances damage the patient's cell function. This cell functional degeneration then causes symptoms that range from mild learning disabilities to severe intellectual impairment and skeletal deformities. Symptoms of ML can be congenital (present at birth) or begin in early childhood or adolescence. Early symptoms can include vision problems or developmental delays. Over time, many children with ML develop poor mental capacities, may develop myoclonic seizures, have difficulty reaching normal developmental milestones, and often die of the numerous systemic issues caused ML cellular damage.

The prevalence for Mucolipidosis II, II/III, III alpha/beta is: 1 in 100,000 to 400,000 individuals world-wide

The prevalence of Sialidosis (Mucolipidosis 1) is not at all clear.

Since the identification of the Mucolipidosis group of disorders, knowledge and understanding has moved to a point where therapies and a cure for these diseases is now possible.

In April 2021 we were approached to form Cure ML as a joint collaboration with the MCRN to assist with finding families, ensuring Natural History studies of the patient communities were completed and published, assisting with the commercial aspects of clinical trials, assisting families to participate in these trials, helping to raise funding for research and ensuring that progress is accelerated to deliver therapies and a cure to this ultra-orphan patient group.

We ensure no patient gets left behind and that ML has a fair chance of gaining access to small molecule therapies or a cure via correction gene therapy.

Cure Mucolipidosis Board of Directors

The table below shows the organizations lead team members and the function and experience of each member.

Jackie James, USA **President** 	Jackie is the mother of Anna who has Mucolipidosis III alpha/beta. Jackie began her journey into advocating for rare disease in 2012 when she joined the board of ISMRD. In 2015 she stepped up as the board president of ISMRD and spent several years working with the board advocating for Mucolipidosis and several other rare diseases. Jackie has spent the last 14 years managing her family business and brings her business skills to the table to work with the board and accomplish the mission and vision that defines Cure Mucolipidosis. Jackie is very much looking forward to seeing the science and research develop and ultimately see a cure/treatment for Mucolipidosis.

Jenny Noble, MNZM **New Zealand,** **Vice President** **Administration/Research** 	Jenny is the parent of two adults with Mucolipidosis III alpha/beta. Jenny began her career in the rare disease field as the Field Officer for Lysosomal Diseases New Zealand in 1999. In 2004 she joined the Board of Directors for ISMRD "The International Advocate for Glycoprotein Storage Diseases." She was instrumental in helping to set up The New Zealand Organisation for Rare Diseases in 1999 and played a large role in trying to gain access to therapies for New Zealand Lysosomal patients. In 2020 Jenny was awarded the New Zealand Order of Merit for her work in Research and Rare Diseases both in New Zealand and around the world. Jenny has played a pivotal role in trying to find therapies for Mucolipidosis over many years, and though not trained in health or science, she is one of the co-authors of **The Osteodystrophy of Mucolipidosis Type III and the Effects of Intravenous Pamidronate Treatment** published in the Journal of Inherited Metabolic Diseases Jenny is delighted to be involved in setting up Cure Mucolipidosis and has more than 30 years' experience as a patient advocate.

Kevin Gates, USA
Treasurer

Kevin lives in Southern California and is the proud father of two adult children. Spencer was born in 1997 and diagnosed with Mucolipidosis III in 1998. Sydney was born in 1999 and is unaffected. Both kids are currently attending college.
Kevin is a founding member of Cure ML. He is also a member of the National MPS Society USA and ISMRD. He hopes to find a cure for ML through fundraising, supporting research and advocacy.

Kevin continues to enjoy a 29 year plus career selling back-up power generators. He also enjoys surfing, reading, watching TV, home maintenance and fellowship through his Catholic church.

Truls Roll, Norway
Board Member, Fundraising

Truls lives in Bergen, Norway with his family; his wife Birthe and their 3 children Pernille ML III, Mia & Stine (who are both unaffected).

Pernille was born in 2009 and diagnosed with ML III in 2012. Since then, they have attended rare disease family/medical conferences in both USA and Europe. Truls and his family has earlier had board positions and worked with the Norwegian MPS society and ISMRD.

After many years in finance Truls now works with Human Resources as Head of the business area in an international consulting company. Truls is excited to join the Board of Directors to help further the mission of finding a cure to mucolipidosis.

Nadia Jack, New Zealand
Board Member,
Social Media

Nadia lives in Palmerston North, New Zealand with her husband, two daughters and their dogs. Her 8-year-old daughter was diagnosed with Mucolipidosis Type II/III in 2019.

Nadia is a strong advocate for her daughter and is passionate about discovering new treatments and a cure for Mucolipidosis. She has been on the Board of Trustees at her daughter's school for the past 3 years, as well as working part-time for a rural insurance company and running her own business in her spare time.

Her background in leadership, communication and earlier study in Health Science at university has helped her navigate the difficult path of patient advocacy along the way and to understand the importance of fostering successful relationships within the health, science and patient community.

Nadia brings a holistic view approach and is excited and honoured to be a part of this organization. She is looking forward to sharing resources, information and connecting with the research community and families affected by Mucolipidosis.

Susan Kester, USA
Board Member,
Family Support Officer

Susan M. Kester was born in Michigan and raised in Southwest Florida. Upon graduating high school, she began her business career as an administrative assistant for the local coroner. She brought her clerical abilities to several different attorney firms spanning more than 20 years.

Susan's love for children led to her and her husband adopting three. Their first child adopted was diagnosed with ML 3. In as much as most physicians were unaware of the disorder, Susan spent countless hours researching and contacting other afflicted families, ultimately serving three years on the Board of Directors for The Society for Mannosidosis and Related Diseases (ISMRD).

Daniel Peach, New Zealand **Board Member, Research** 	

Charlotte Gilbert, USA **Board Member Fundraising**	

Operations Plan

The day-to-day Operations of Cure Mucolipidosis will be run from New Zealand. Cure Mucolipidosis board of directors have set the following short term and long-term goals.

Immediate Operating Plan – 0 to 6 months				
Category	**Key Initiatives**	**Prioritization**	**Project Lead**	**Due Date/Completed**
Organisation Set up	Appoint Board of Directors	Immediate	Jackie James/ Jenny Noble	Completed
	Not for Profit registration	Immediate	Jackie James	Completed 18th May 2021
	Articles of Incorporation and Bylaws		Jackie James/ Kevin Gates	Completed
	Tax Exemption	Immediate	Jackie/Jenny/Susan Kester	Pending
	Bank Account	Immediate	Jackie James/ Jenny Noble	Completed
	Set Operations Budget	Immediate	Jackie James	Pending
		Immediate	Jackie James/ Kevin Gates/Jenny Noble	Completed
	Set up accounting system	Immediate	Kevin Gates/ Jackie James	Completed
Logo Mission / Vision Development	Logo Establishment	Immediate	Jenny Noble	Completed 5th June 2021
	Mission/Vision affirmation	Immediate	Board of Directors	Pending
Social Media Platform	Facebook, Twitter, Tik Tok, Instagram, Linked In	Immediate	Nadia Jack, Jenny Noble	Completed
	Set up Donations on Facebook	2 months	Nadia Jack	Pending
Website Development	Domain Name	Immediate	Jackie James	Completed
	Website Platform	Immediate	Jackie James	Completed
	Information development	Immediate	Jenny Noble	Completed
Database development	Family Contacts	Immediate	Susan Kester/Nadia Jack/Jenny Noble	Under development
	Patient Registry	Immediate	Susan Kester/ Jenny Noble	Under development
Communication	Newsletter introducing Cure Mucolipidosis	3 months	Jenny Noble	

Operating Plan – 6 months – 1 year				
Category	**Key Initiatives**	**Prioritization**	**Project Lead**	**Due Date/Completed**
Fundraising for Research	Develop Funding Plan Grant funding Natural History Study database	4months time 4 months' time 6 months	Truls Roll Jenny Noble Jenny Noble	Pending Pending Pending
Virtual Meetings	Scientific Meetings Family Q&A meetings	6 months' time 6 months' time	Jenny Noble Jenny Noble	Pending Pending
Marketing of Cure Mucolipidosis	Social Media, Press Releases, Newsletters, Website	Immediate	Nadia Jack/ Board	Pending

Marketing Plan

In order to reach our target audience and raise awareness about Cure Mucolipidosis we will need to deploy a variety of marketing activities. These initiatives include direct mail, phone calls, press releases, events, social media, grants etc. since this is where we can reach our target audience most effectively.

MARKETING INITIATIVE	GOAL	Action Plan	Due By
Direct Mail	Email campaign	• Identify families and obtain personal email addresses • Establish a mail chimp account • Establish a newsworthy two-sided coffee-time email format • Investigate translation services for non-English speaking members	
Advertising	Advertise with other Rare Disease groups in their magazines.	• Build contact list of Rare Disease groups • Prepare Press releases and advertise Cure ML's activities	
Digital marketing	Social Media, email marketing, Website	• Post all activities to Social Media platforms • Keep website up to date and constantly changing with information	
Video Marketing	YouTube, Tik Tok	• Develop YouTube and Tik Tok video's • Develop Rare Disease Day Videos	
Contest Marketing	Photo contest, Video Contest, Raise awareness	• Develop a Photo Contest to raise awareness • Establish the Protocols around voting • Establish the Prize for the winner	
GoFundMe	Set up fundraising awareness	• Develop the GoFundMe platform • Establish the Fundraising goals for this platform • Write the text for this	
Amazon Smile	Register with Amazon Smile to receive a % of every purchase made by our community	• Advertise on website Amazon Smile • Include this on all Marketing programmes	

Financial Plan

The establishment of Cure Mucolipidosis was not done lightly. Finding the funds to achieve all our goals is a monumental task but we have taken the first step. Our budget is in three parts.

1. ***Administration*** – there are no salaries paid to Cure Mucolipidosis Board Members or any staff. The work done is purely voluntary in nature. We do however have administration expenses some of which have already been met by Board Member donations. The Board has set a preliminary administration budget of $US11,228.90 for the first twelve months. Some of this is for one off costs but the majority will be on-going costs such as accounting, website, social media etc. These will be reviewed by the Board annually. There is no intention of allowing for salaries and initially there are no travel expenses.

2. ***Projects*** – the key projects are:
    a. the establishment of a patient database so that information on research and treatments can be disseminated accurately and quickly to patients.
    b. Hosting of the Natural History Database.
    c. Hosting Meeting costs for Researchers

3. ***Research Fundraising*** – this is broken down into the following areas:
    a. Supporting families for clinical trials – this involves eliminating any financial barriers that would impede families to attend clinical trials. Ordinarily the cost of attending a trial is borne by the Drug Companies running the trial, however these costs are often limited and do not cover the real cost of families with special needs having to travel significant distances and be away from supporting services.
    b. Raising monies to help support the MCRN to:
        i. Support the costs of maintaining the animal colonies for trials.
        ii. On-going research projects to find therapies and a cure for Mucolipidosis.

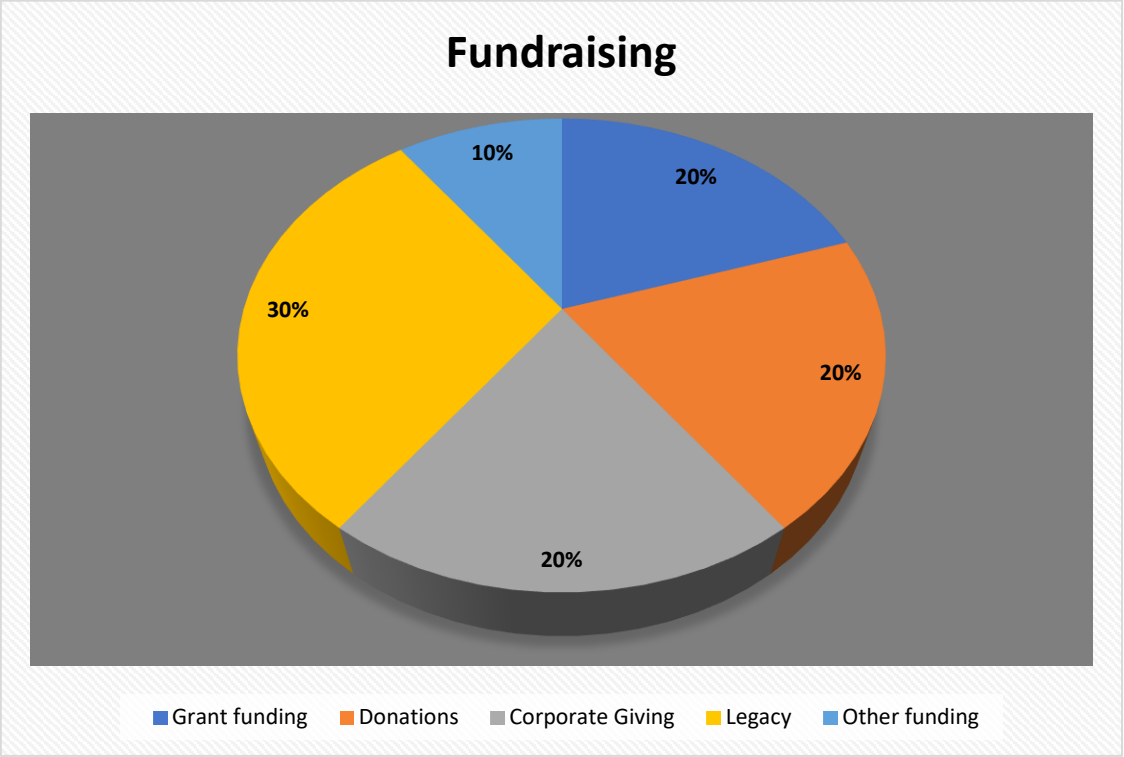
When a cure is developed and released, Cure Mucolipidosis will be in a position to eventually receive royalties from the implementation of a therapy from drug companies to become self-funding for the outreach to patients in other parts of the world that are not well known to our organisation at present. In keeping with our vision, we want to carry the positive outcomes to “third world” areas where services and support are limited for these patients so that we can intercede as early as possible in the outcomes and results for all Mucolipidosis patients worldwide.

Financially our initial objectives are small – please refer to the budget pages under Appendix 1, however research and treatments are expensive, and our success will be measured by our ability to fundraise for these projects. Our Board Members have already committed personal resources and funds to Cure Mucolipidosis as we believe in what we are doing – we hope you will to and join us in this!

Fundraising Plan

Cure Mucolipidosis will seek corporate grants, foundation grants, Industry sponsorship, Family giving through Facebook and other social media fundraisers, In kind grants and Legacy's. It is our intention to bring into our network people who have fundraising experience.

We think our percentage splits might look like this.



Social Media

Cure Mucolipidosis will use the following Social Media Platforms to raise Awareness, to bring affected patients together, announce research developments on these platforms to keep people informed of Research progress.

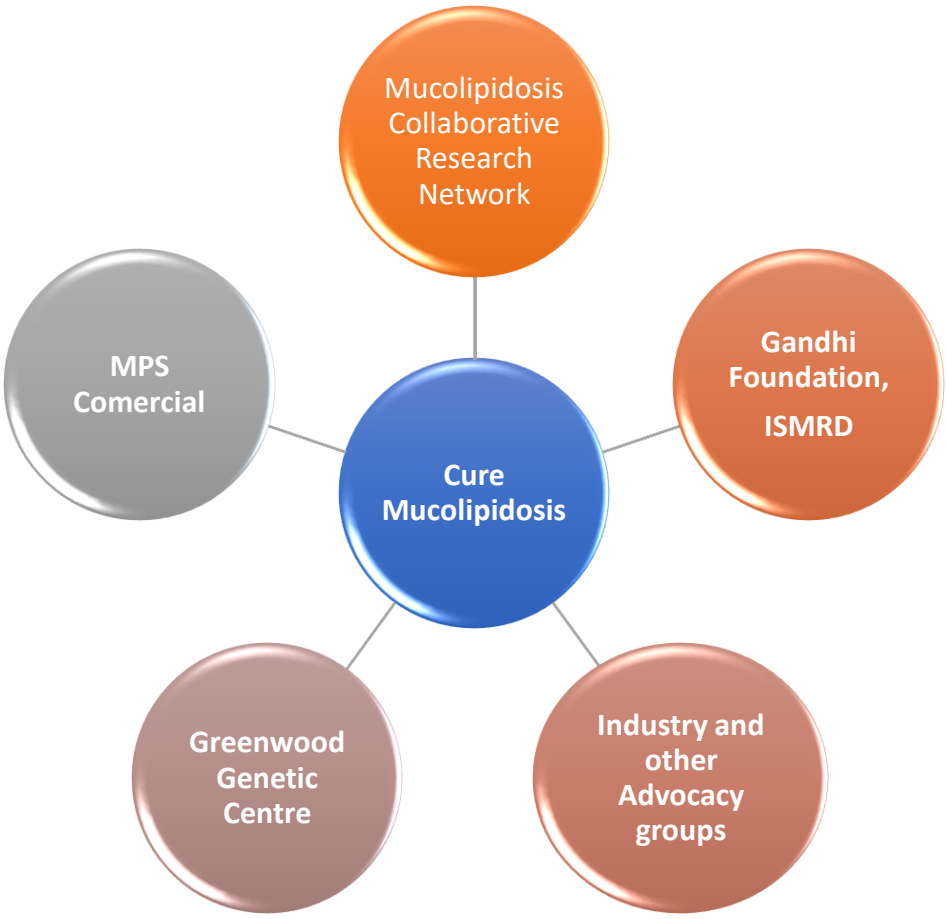
- Facebook
- Facebook Messenger
- Twitter
- Instagram
- Tik Tok
- Website
- GoFundMe.
- LinkedIn Profile
- Yash Gandhi Foundation

Business Objective	Social Media Goals	Complete by
Grow the Brand	Awareness Increase following on each social Media Channel by 25% in the first year.	
Turn customers into Advocates	Spread the word Raise funds for Research Engage the Rare Disease Community	

Research Collaboration Plan

Cure Mucolipidosis has been set up to support and work alongside the Mucolipidosis Collaborative Research Network (MCRN). Our joint goal is to find a cure for Mucolipidosis. The MCRN functions as a platform for professionals (clinicians and scientists) to share scientific results and openly discuss ideas for ML therapy. They do so through monthly video calls where scientific articles, unpublished results and new concepts are shared. This information will be shared with Cure Mucolipidosis in order to help identify suitable pharmaceutical companies, academic partners, and patient populations who might be able to assist in the development of these therapeutic concepts and subsequent clinical trials.

Cure Mucolipidosis will interface with the Greenwood Genetic Center through its interactions with Dr. Steet and Dr. Flanagan-Steet on the research front as well as Dr. Cathey on the clinical front. The GGC will also help promote the agenda of Cure Mucolipidosis through its increasing efforts in the treatment of genetic disorders.



Family Outreach

Cure Mucolipidosis will actively support families with these diseases and gather their information to be housed in a patient registry.

We will through our Social Media platforms and other communication modes keep families up to date on research.

Appendix 1

Cure Mucolipidosis Start up Budget 2021

Administration	Cost		Total	
Software:				
Accounting Package	$	2,000.00		
Website Development	$	2,000.00		
Logo Development	$	500.00		
Patient Database Annual Fee	$	1,000.00		
Technology				
Zoom Annual Subscription	$	149.90		
(Zoom is free for up to 40 mins)				
Website Annual Fees	$	500.00		
Meeting Platforms	$	1,000.00		
Social Media Costs	$	1,000.00		
Licensing in State of Illinois				
Articles of Incorporation	$	79.00		
ByLaws	$	1,000.00		
Accounting Fees				
Annual Accounting	$	2,000.00		
Total Administration			**$**	**11,228.90**
Projects				
Patient Database/Registry	$	5,000.00		
Natural History Database	$	40,000.00		
Meeting hosting costs	$	2,000.00		
Total Projects			**$**	**47,000.00**
Total Budget			**$**	**58,228.90**