

# MSD Research Strategy

As a rare condition, Multiple Sulfatase Deficiency has historically struggled to attract research funding from larger funding bodies. Our program aims to ensure that this devastating condition receives the research attention it requires and deserves.

## Our Research Strategy:

- Aims to maximise the impact of the funds we invest, as we identify the research areas that promise to make the greatest scientific advances and have the most relevance for our families.
- Defines three focus areas: halt disease progression, repair damage and improve quality of life. We believe that investment in these areas is essential to bring desperately needed treatments to market.
- Offers research grants with applications assessed via a robust review process to ensure we fund the most promising research that can be translated into the clinic in the soonest time frames.

## Research Focus Areas

Our Research Strategy will focus on 2 priority areas and our Research Funding Program will consider projects that address the following areas:

Halt Disease Progression	Repair Damage
<ul style="list-style-type: none"><li>• Gene therapy, enzyme replacement, cell therapy &amp; other emerging therapies to stop progression of Multiple Sulfatase Deficiency.</li><li>• Strategies to enhance the effectiveness of emerging therapies</li><li>• Halt disease progression early to optimise brain development</li></ul>	<ul style="list-style-type: none"><li>• Repair and reverse cell damage</li><li>• Broad application of regenerative treatments, for example repurposing approved drugs</li><li>• Opportunity to collaborate with researchers working on other neuro-degenerative diseases</li></ul>

Note future funding focus areas may include: prevention (including pre-conception), diagnosis and prognosis.

**Research Principles:** The following principles underpin our Research Strategy and apply to each focus area.

#### Fund the best research

Funding priority given to research most relevant to our objectives regardless of geography, discipline or researcher.

#### Focus on translational research

Drive research showing translational promise, to give patients the quickest access to emerging therapies via clinical trials and pilot studies

#### Encourage innovation and collaboration

Apply advances from other research areas; fund initiatives involving new cutting edge ideas and enabling international collaboration and excellence; and collaborate with other funding bodies to co-fund projects to extend our reach

We plan to invest annually across the following grant types:

- **Translational Research** Grants are funding for projects with significant potential to move through the translational pipeline towards treatment. This includes pre-clinical and clinical research to develop new treatments. We fund projects up to €100k/year for up to 2 years.
- **Incubator Grants** focus on small scale projects investigating bright new ideas with the potential to generate results that may attract larger scale funding. We fund projects up to a maximum of €100k. Smaller amounts with high potential and shorter timelines may be prioritised. Only where a clear commitment to apply to lever larger funding is apparent.

If an Incubator grant is being sought, we would strongly encourage the researcher to highlight in detail how they anticipate the receipt of seed funding is going to leverage greater funding and how any preliminary data obtained would lead to a bigger grant. We would also encourage identification of matched funding or other funding sources.

## How we fund research

Our funds are raised primarily through grassroots fundraising activities initiated by the foundation, our supporters and families.

We have a duty of care to ensure that our charitable funding is used to fund research that has the greatest scientific possibility of having an impact on the lives of children affected by Multiple Sulfatase Deficiency. Whenever possible, we will invest funds with the potential for not only a scientific return but also a commercial return, allowing us to optimise our limited resources and minimise risk associated with funding research. Any return to us will be reinvested to help further our goals.

## Review Process

MSD Action Foundation and United MSD Foundation have a competitive research grant application program. Expressions of interest will be reviewed by our Scientific Advisory Board (SAB). Our SAB, in consultation with our Boards of Directors, will shortlist projects subject to available funding and alignment with our objectives and priorities. Chief/Principal Investigators for short-listed projects may be invited to complete our Request for Proposal form (RFP). RFP's will subsequently be reviewed by our Scientific Advisory Board and may be subjected to external peer review, if deemed necessary.

Review by our Scientific Advisory Board will help us to ensure that the research is:

- Scientifically valid, relevant and significant
- Timely and achievable
- Not duplicating other work
- Using appropriate methodologies
- Carried out by researchers with the right skills and facilities
- Providing value for money.

Full Translational and Incubator Grant Applications (RFP's) will be assessed by the following criteria

Criteria	Description	Weight
Scientific Quality	Is the project clearly defined, well designed and feasible?	25% *
Significance and/or Innovation	Does the project have the potential to change the course of Multiple Sulfatase Deficiency?	25% *
Team Quality and Capability	Do the researchers have the required skills and experience to carry out the project?	20% *
Timely	Is there a fair timeline for the project?	10% *
Translational Potential	Does the project have the potential to translate to the clinic in an accelerated time frame?	20% *

These percentages may change without notice\*