Policy Plan MD Europe
2024 - 2028

Online



1. Greater brand awareness
2. More funding for research
3. Faster drug development

Awareness and proactively contributing to a solution

Why MD Foundation

About MD

MD (“Myotonic Dystrophy”) is a common, progressive and hereditary disease. Because it is hereditary, the disease by definition affects entire families: if one of the parents has the disease, on average 50% of the children will develop MD. The disease is also passed on in an increasingly severe form, which means that within a family it is the children who face the most serious problems.

MD affects both men and women. The disease causes a broad spectrum of symptoms: heart problems, cognitive impairment, cataracts, diarrhoea and muscle weakness. People die from the consequences of MD: heart failure, choking and a very fragile immune system resulting from the combination of all these problems.

Role of MD Foundation

MD Foundation was founded in 2019 out of astonishment that such a common and serious disease is so little known. On average it takes 10 years from the first symptoms until the correct diagnosis is made. Throughout that time, people with MD do not receive the right medical support and the disease can unknowingly be passed on to the next generation. MD is therefore unknown not only among the general public, but also among doctors. MD Foundation creates visibility by sharing the personal impact of MD with both the wider public and professionals.

MD Foundation was also founded because we want to contribute actively to finding a solution. Because it is clear which gene defect causes MD, many companies and universities are working in a targeted way on a first generation of medication. MD Foundation plays an initiating and supporting role in this: we are active in mapping the problems of MD more quickly and in accelerating existing initiatives. We have initiated a first international project that is directly linked to faster availability of medication.

MD Foundation wants to use hopeful developments to motivate people with MD, their families and friends to become actively involved in these positive developments. MD Foundation complements patient associations through its proactive approach and its focus on MD. Inspired by, among others, the Myotonic Dystrophy Foundation in the United States, we are looking for like-minded partners in Europe, as no such organisation exists in many countries.

Objectives 2024 - 2028

Mobilising those affected: more events and media campaigns

MD Foundation is supported by volunteers: families and friends of people with MD. That will remain the case. We focus on organising gatherings and advertising and media campaigns. Through our fundraising platform we give people with MD and their loved ones the opportunity to contribute to our mission themselves. We also initiate our own events to raise awareness and involve a wider group of people. In addition to our Thousand Euro Donors, we also want Supporters: people who likewise commit to us for the long term with a smaller amount.

A foundation that can initiate international projects every year

MD Foundation needs to professionalise its fundraising activities to bring our mission to wider attention in this area too. This will give us the capacity to act that matches our mission and its urgency.

MD Foundation wants to connect families across Europe so that we can support research and accelerate projects with greater impact. Raising awareness has both a national component (such as organising gatherings) and an international one (such as showing which scientific breakthroughs have been made and which clinical trials are already in the development pipeline).

Further scaling up and professionalisation

MD Foundation has a large number of initiatives that will be developed further. This means these initiatives need to grow beyond the family-and-friends stage, which in many cases requires investment: organised coaches to the SED Happening, paid recruitment around the MiDsummer Run and more professional content to carry our mission to a wider audience.

MD needs structural attention, given the severity and scale of the disease as well as the many positive developments. To achieve this, we need a stronger financial base to increase our capacity. In addition to our current volunteer professionals, we will therefore also hire staff so that our mission receives full-time attention, particularly in fundraising, awareness and events.

For structural attention

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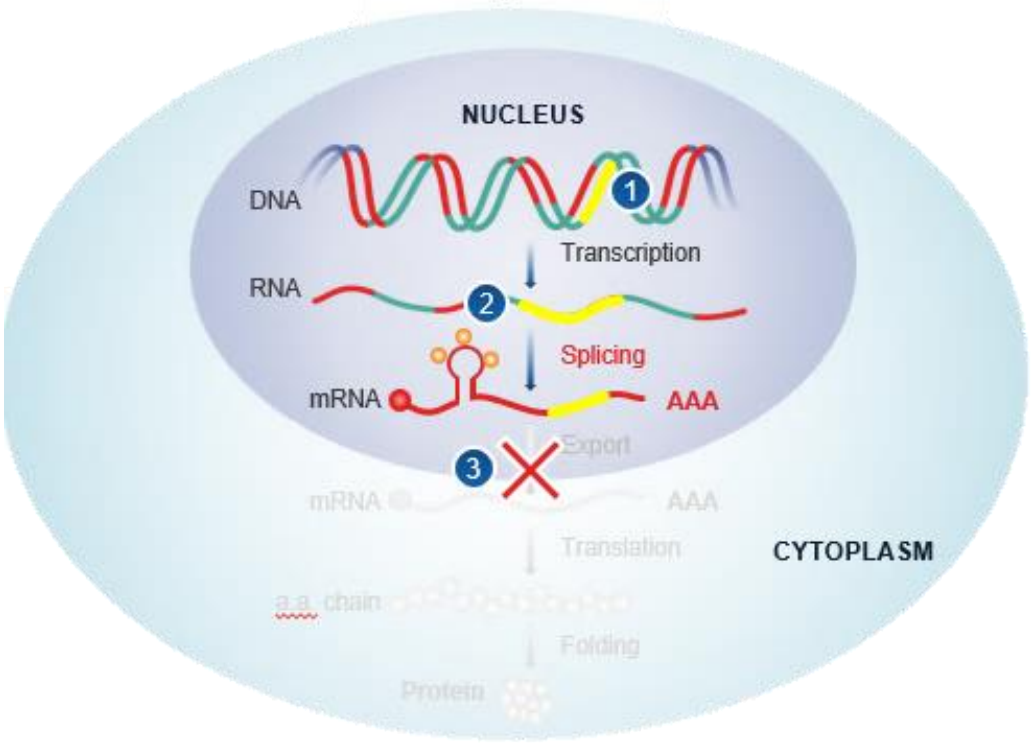
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A Appendix

1

MD

The most common fatal muscle disease



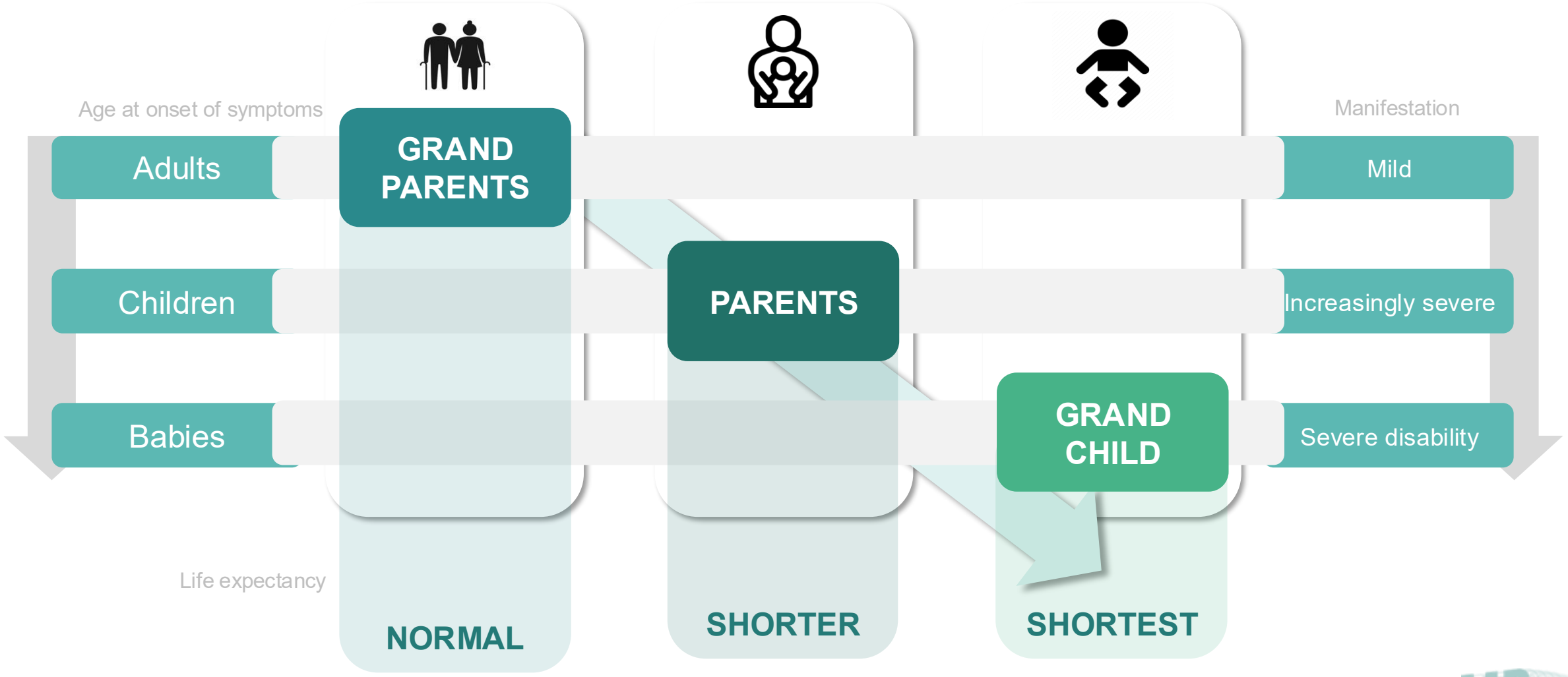
The technical explanation

1. MD is caused by an expansion of the **DMPK gene**

2. This creates a ‘hairpin’ during splicing **that causes proteins to get stuck**

3. The absence of these proteins **leads to the various symptoms of MD**

The disease becomes more severe with each generation



WHAT DOES MD DO?

The disease affects **both organs and muscles** and manifests differently in every patient

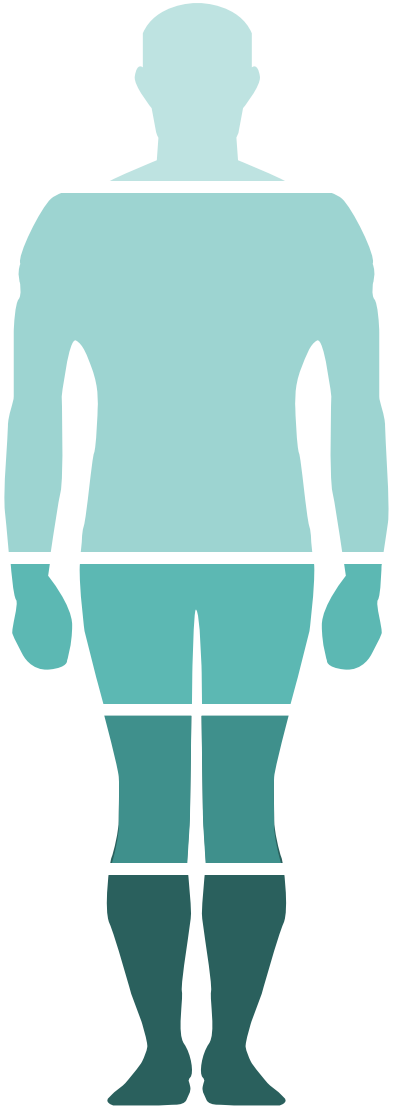
Cognitive functions
- Excessive fatigue
- Intellectual disabilities
- Concentration problems

Vision
- Cataracts
- Drooping or fully closed eyelids

Muscles
- Weakness and pain due to cramping
- Heart problems

Endocrine system
- Low thyroid hormone
- Diabetes

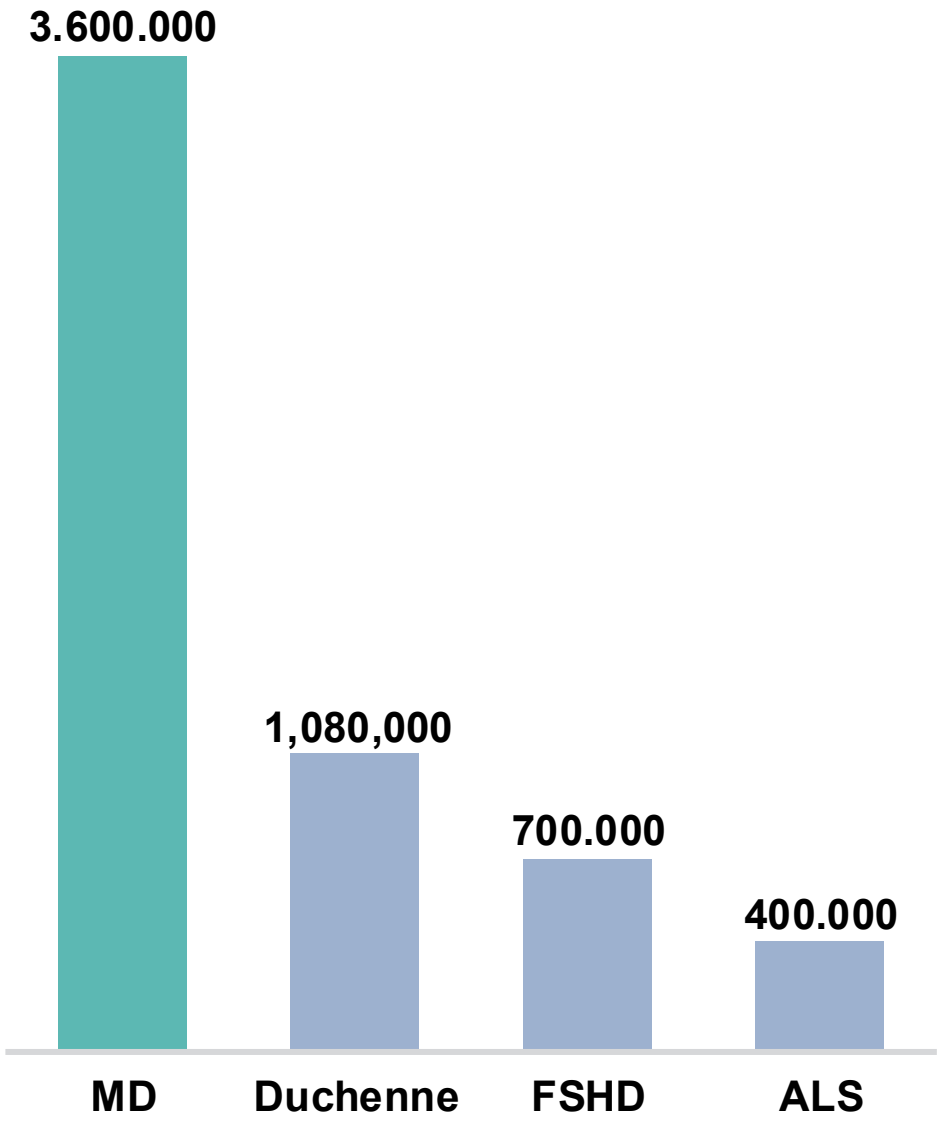
Respiratory system
- Sleep apnoea
- Increased risk of pneumonia
- Difficulty breathing



No medicine or treatment for a common disease…

… but potential solutions are in sight

Number of patients worldwide with the most common muscle diseases



Medication is in development

MD Drug Development Pipeline Top 20 - May 2024

PRE- CLINICAL	PHASE 1	PHASE 2	PHASE 3	DRUG TARGET/ MECHANISM	Type
AMO				Glycogen synthase kinase 3 beta (GSK3ß)	Small Molecule
LUPIN NEUROSCIENCES				Sodium Channels	Small Molecule
AVIDITY				DMPK	Antibody Conjugated Oligonucleotide
HARMONY BIOSCIENCES				Histamine-3 receptor (H3R)	Small Molecule
Dyne				DMPK	Antibody Fragment Conjugated Antisense Oligonucleotide
ARTHEx biotech				MBNL and DMPK via miR-23b	Antisense microRNA Oligonucleotide
VERTEX				DMPK	Peptide Conjugated Oligonucleotide
PepGen				DMPK	Peptide Conjugated Antisense Oligonucleotide
arrowhead				r(CUG) of DMPK	Investigational RNA interference (RNAi) Therapeutic
EXPANSION THERAPEUTICS				r(CUG) of DMPK	Small Molecule
Juvena				--	Stem Cell-Secreted Proteins
Rgenta				r(CUG) of DMPK	Small Molecule
sanofi				DMPK	miRNA Technology in Adeno-Associated Virus
astellas				DMPK	Adeno-Associated Viral Antisense
GRITTGENE THERAPEUTICS				--	--
ENZERNA				r(CUG) of DMPK	Artificial Site Specific RNA Endonucleases (ASREs)
DESIGN THERAPEUTICS				r(CUG) of DMPK	Gene Targeting Chimera Small Molecule
dewpoint$_x$				DMPK	Biomolecular Condensates
KATE THERAPEUTICS				--	--

Source: www.myotonic.org/

Different types of medication are at different stages of development. A first medicine is expected to be approved within the next five years.

MD is one of the most variable diseases, which makes it unlikely that a single medicine will be the solution for everyone.

MD Foundation's involvement is therefore not focused on developing a single medicine, but on the structural development of a range of medications.

2 MD Foundation

Mission

Greater awareness and actively contributing to a solution

Development since founding

2019

Founding

- Founded after a diagnosis in the immediate family of the first chair
- The board is made up of medical and non-medical professionals

2019-2023

Start

- Started raising awareness: commercials, website, fundraising page, our own documentary and various interviews with personal MD stories
- Initiating the first research project
- Expansion from the Netherlands to Belgium

2024-2029

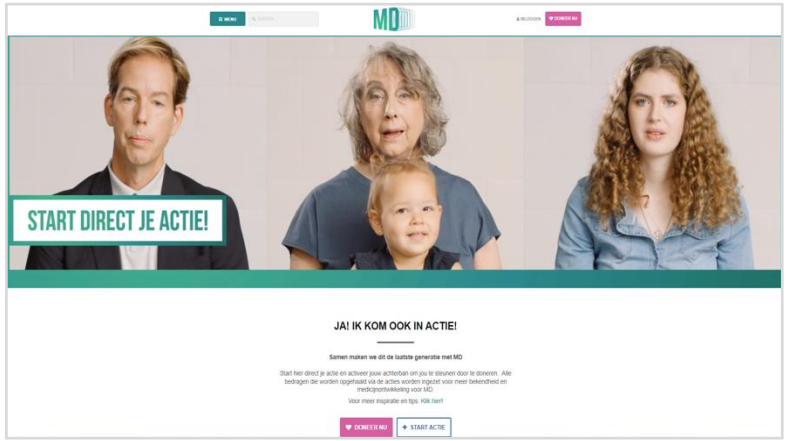
Structural organisation

- Funding our first own study
- Setting up a professional organisation for fundraising and day-to-day operations
- Network in multiple countries

What we have achieved: our first steps

Raising visibility & mobilising

- Platform where companies and individuals can start their own fundraisers, such as running a marathon
- Already 33k raised in 5 months



- Organising events such as:
- ‘Komen eten’ dinner in Belgium (25k raised in 2023)
- MD festival in Belgium
- The MD Summer Run in NL (2024)



International expansion and joint projects

- Foundation converted into MD Europe following expansion to Belgium and initial contacts in Italy & Spain, among others
- Partnerships with companies and universities such as [XYZ]

The added value of MD Foundation

01

Mobilise friends and family

- Medical technology makes drug development realistic
- Everyone can contribute to a solution
- Making the impact of the disease visible to mobilise people

Not: peer support (as most patient associations offer)



02

Specialise in MD with an international perspective

- A common disease without its own fundraising foundation in Europe (vs. ALS, Duchenne, SMA etc.)
- The MD field is complex and international, and therefore benefits greatly from specialisation

General fundraising organisations are our partners (not competitors)



03

Active commitment to accelerating drug development

- Active across the entire chain, from scientific research to the clinical phase
- Awareness is important because of widespread misdiagnosis

Not only scientific research, as there are more obstacles to making medication available



The connecting, initiating MD organisation in the EU

National

- Usually a general muscle disease foundation
- Patient association focused on peer support and sharing information about existing care options
- CureDM UK is a volunteer organisation focused on children

- MD Foundation specialises in MD
- MD Foundation takes the initiative, with a focus on awareness and finding a solution

Europe

- EuroDyMa is an umbrella MD patient association, focused on communication between patients, researchers and pharma

- MD Foundation takes the initiative, with a focus on awareness and finding a solution

Worldwide

- The Myotonic Dystrophy Foundation (MDF) is an American foundation specialised in MD, with both a patient association (“Care”) and a branch focused on finding a solution (“Cure”)
- MDF has a professional staff with a network of volunteers

- MD Foundation brings proactive families across Europe together

The current organisation consists of volunteers

Board

Marketing and fundraising teams

Professional focus

Communications Team

Goal: Develop and execute a long-term comms strategy, led by people with **experience** at large (international) companies

FCBAMSTERDAM

Nestlé

PLUS

Volunteers & Events Team

Goal: Mobilise and facilitate the foundation's supporters by organising events and rolling out ideas at scale.

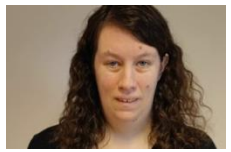
Venture Team

Goal: Actively accelerate research by organising and funding studies, led by people with **experience** in biotechnology

SAOLA

Orthros Medical

Patient ambassadors



Bianca Huisman



Remco Roovers

Non-patient ambassadors (Dutch celebrities etc.)

An example of the impact we are already making today

Project ANIQ

THE PROBLEM	Our impact
Medicines for children cannot be approved because there is insufficient data on how the disease progresses in children	Discussions between our Venture Team and researchers at Radboud made it clear that the lack of data on children will soon become a major obstacle to the approval of medication. Moreover, such knowledge will also help improve care for children with MD, the patient cohort with the greatest potential health gain. MD Foundation has initiated a project involving several pharma companies and research groups. The study will be led by Stanford University, Maastricht UMC+ and Radboudumc. With EUR 500k this project can be launched, triggering a multiple of that amount in contributions from pharma. The start-up phase covers protocol development including preparatory studies, testing potential endpoints and setting up data management.

€450k

€160k raised — **€450k**

Partners

Sources of funding

1. Long-term donors	- Renewal of existing donors (300-2,000 per year) - New format: “Membership” or “Supporters” at EUR [25-100 per year] - Legacies (in the longer term)
2. Fundraisers (Kentaa platform)	- Roll-out to Belgium -
3. Events	- MD-SED Happening - MiDsummer Run - Information meetings (“Amsterdam” meeting) - Participation in existing events
4. Major donors	- Structured approach to grant-making foundations - Lotteries
5. Sponsorship	- Pharma partners -

3 What are our goals?

And what does it take?

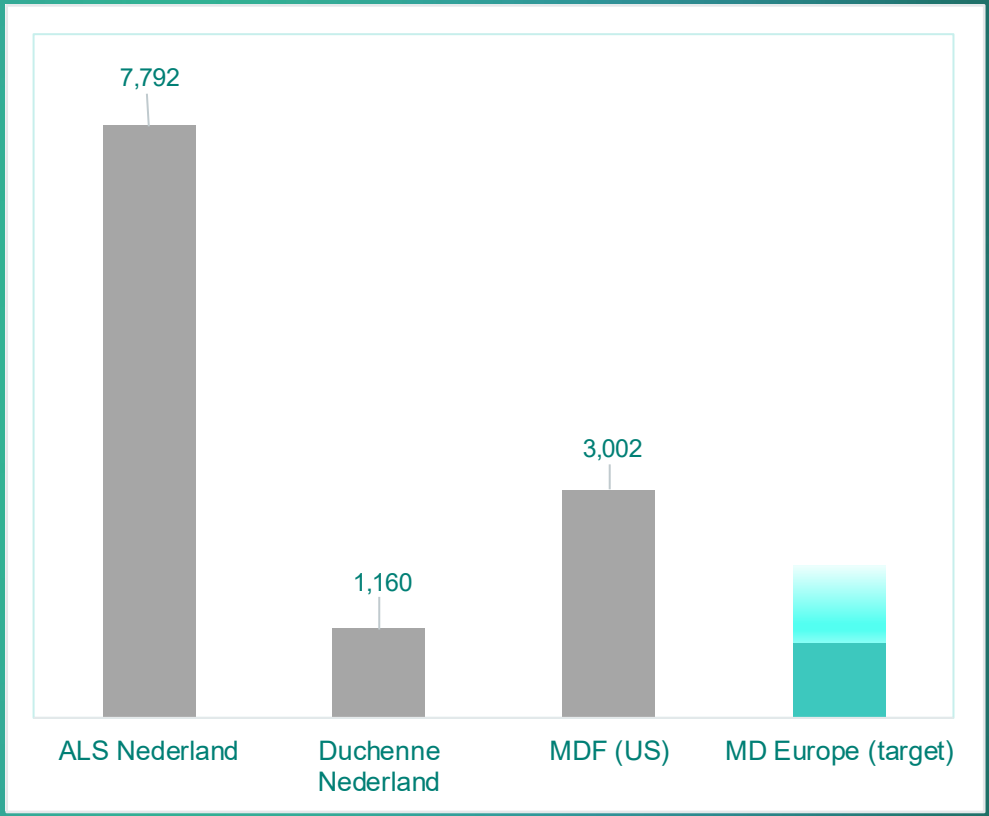
Objective

Structural attention for our mission

1. Greater brand awareness
2. More funding for research
3. Faster drug development

A budget more in line with comparable diseases

- **There is no foundation for MD in Europe with a meaningful budget. This makes it much harder to start research projects or to draw structural attention to the disease**
- **Foundations with significant impact all work with a mix of committed volunteers and paid staff**



Annual budget in EUR '000

ALS Nederland is the largest muscle disease fund in the Netherlands, with 14 employees (11.5 FTE). Most of its staff focus on fundraising and on initiating events that raise awareness and funds. The money is used to run campaigns, support patients and launch many new research programmes.

Duchenne Nederland employs 3.4 FTE and focuses on supporting the families of affected boys and stimulating scientific research towards a solution. Duchenne is a hereditary disease related to MD that occurs only in boys (prevalence 1 in 3,500 boys).

Myotonic Dystrophy Foundation (MDF US) is the American MD foundation. It organises patient meetings, an annual conference on MD and supports scientific research. MDF employs 11 staff (excl. interns).

Indicative budget of €1m

1 Greater brand awareness

€ 200k For public and medical campaigns

2 More funding for research

€ 250k For funding scientific research

€ 150k For professionalising and expanding the foundation

3 Faster drug development

€ 400k Projects such as ANIQ to accelerate drug development for children

4 Multi-year budget

Draft multi-year budget

Beleidsplan 2024 - 2028

In eur	2024	2025	2026	2027	2028	*Commentaar*
FTE (average)	0.3	1.7	3.0	4.0	5.0	
Gem FTE Salaris (bruto + lasten)	100,000	100,000	90,000	90,000	90,000	Duchenne en ALS Nederland: gemiddeld ca 80.000/FTE
Salarissen (all in)	30,000	170,000	270,000	360,000	450,000	Voor groot deel te alloceren aan doelstelling (Bekendheid en
1. Bekendheid / Communicatie	10,000	60,000	80,000	100,000	150,000	
2. en 3. Projecten	100,000	50,000	200,000	250,000	340,000	
Overige kosten	10,000	60,000	60,000	60,000	60,000	
(A) Uitgave	**140,000**	**280,000**	**550,000**	**770,000**	**1,000,000**	
LT Donateurs (bestaand)	25,470	25,470	35,470	45,470	55,470	
Nieuwe LT Donateurs (incl churn)	-	10,000	10,000	10,000	10,000	
LT donateurs	25,470	35,470	45,470	55,470	65,470	
Acties (Kentaa)	25,000	25,000	25,000	40,000	60,000	
Evenementen (netto na kosten)	28,000	40,000	60,000	100,000	150,000	
Major donors	25,000	100,000	150,000	250,000	350,000	
Sponsoring	5,000	10,000	10,000	10,000	10,000	
Overige	-	-	-	-	-	
(B) Inkomsten	**158,940**	**345,940**	**485,940**	**760,940**	**1,050,940**	
(A) Uitgave - (B) Inkomsten	18,940	65,940	(64,060)	(9,060)	50,940	
Ratio's						
Vaste lasten	40,000	230,000	330,000	420,000	510,000	Personeel plus overige kosten
Vaste inkomsten	25,470	35,470	45,470	55,470	65,470	LT donateurs >> Lange termijn Major donor?
Vaste inkomsten - Vaste lasten	(14,530)	(194,530)	(284,530)	(364,530)	(444,530)	
Go get als % van begroting	0%	0%	0%	0%	0%	
FTE als % van begroting	19%	49%	56%	47%	43%	ALS: 12%, Duchenne 30%
Inkomsten/FTE	529,800	203,494	161,980	190,235	210,188	Totaal: ALS 677k, Duchenne 341k. Excl initiatieve door verbonden stichtingen ALS 426k, Duchenne 144k
Inkomsten volgend jaar / FTE	1,153,133	285,847	253,647	262,735	na	

Notes to the multi-year budget

1. This is the budget for MD Europe including its local activities in the Netherlands, as there is no separate entity there. The assumption is that the other countries fund their local activities, as described on page [20], locally, as Steinert Everybody Different vzw does in Belgium.

2. Much of the planned income is one-off in nature, while we are entering into long-term commitments, particularly with paid staff. For the growth phase we are looking for major donors willing to support our growth for a number of years, to reduce uncertainty about the organisation’s funding.