

# Policy research and innovation

The Prinses Beatrix Spierfonds aims to ensure that everyone in the Netherlands living with a neuromuscular disorder has access to medicines or effective treatments. To achieve this, we focus on cutting-edge scientific research, innovative therapies, and strengthening care and support for all those affected by a neuromuscular disorder.

# Our strategy

For decades, the Spierfonds has invested in scientific research into neuromuscular disorders. With success: for many conditions, the underlying causes have now been identified, and work is progressing on targeted treatments. Thanks to new technologies and insights, developments are moving rapidly. For the first neuromuscular disorders, medicines are now available; for dozens more, hundreds of promising therapies are in development worldwide.

This acceleration offers great opportunities, but also brings profound changes to the research and healthcare landscape. The growing number of clinical trials requires sufficient expertise, capacity, and accessible patient populations. At the same time, translating research into real-world practice is often complex: promising therapies may face delays, for instance in approval, reimbursement, or implementation. And whether or not a medicine becomes available, appropriate care remains a lasting need for everyone living with a neuromuscular disorder. Medicines and rehabilitation go hand in hand; both are essential in the search for the best possible treatment.

The Spierfonds aims to respond proactively to current developments and be prepared for what the future demands. If scientific breakthroughs are to translate into real improvements in daily life, the entire chain – from development to access – must function seamlessly. We therefore seek collaboration with other foundations and organisations across the field, ensuring that knowledge, capacity and resources are used as effectively as possible.

With this ambition in mind, we have defined four themes and corresponding objectives for the policy period 2025-2028. These were formulated through a careful process involving people living with a neuromuscular disorder and their representatives, researchers, partners, and other stakeholders. By focusing on these objectives, we aim to increase our impact and deliver concrete progress for those who face the daily consequences of a neuromuscular disorder.

Therapy developmen	Trial participation	Rehabilitation	Access to new therapies
More new therapies are developed in Dutch centres of expertise.	More patients in the Netherlands have access to (inter)national clinical trials that test potential medicines.	More patients in the Netherlands have access to the best rehabilitation treatments to improve their quality of life in the short term.	People with a neurmuscular disorder have faster access to new therapies.



# Research programmes

The Spierfonds offers various funding instruments to stimulate medical breakthroughs in the field of neuromuscular disorders and to accelerate access to the latest treatments. We act as a funder, partner, catalyst, and connector. To ensure targeted use of available resources, we work with a phased approach. Not every theme will be prioritised equally each year; instead, focus will rotate to maximise impact across all themes over time.

Programme	Topic(s)	Format	Frequency
Medicine development	Innovative therapies	Open call	Annual
Medicine development	Rotating themes	Open call	Every 2 years
Trial readiness	TBA	Open call	Every 2 years
Rehabilitation	Personalised lifestyle	Consortium	2025-2029
Acceleration	Medicine development Trial readiness Rehabilitation Rapid access	Projects, coaching, co- funding	Open year-round

Table 1 Planned funding opportunities for the 2025–2028 policy period. These are subject to change. For current information on open calls, please consult the <u>grant portal</u> of the Spierfonds.

## Medicine Development Programme

For most neuromuscular disorders, no medicines are currently available. Through our long-term Medicine Development programme, we invest in developing treatments for neuromuscular disorders in the Netherlands. This programme consists of:

#### • Innovatieve Therapies call

For many neuromuscular disorders, targeted therapeutic approaches are now within reach. With the new annual 'Innovative Therapies' call, we aim to further and more effectively advance promising preclinical results towards clinical application. From 2026 onwards, the deadline will fall in the autumn.

#### • Rotating thematic calls

Not all neuromuscular disorders are ready for medicine development. Through biennial thematic calls, we therefore continue to support other essential research lines, such as disease mechanisms/causes, disease models, and diagnostics. The first call is planned for spring 2027.

#### **Trial Readiness Programme**

Worldwide, the number of potential medicines being developed for neuromuscular disorders is increasing rapidly. We want people with neuromuscular disorders in the Netherlands to benefit from these as soon as possible. To achieve this, robust registries, reliable outcome measures, and a strong infrastructure are essential. Therefore, a new trial readiness programme is planned for 2026. It is being developed in close collaboration with the field to ensure that the Spierfonds can contribute effectively to accelerating access to promising therapies.

#### Rehabilitation Programme

In 2025, the Spierfonds launched a new programme focused on lifestyle, aimed at reducing the burden of disease for people with a neuromuscular disorder. One of the motivations was a survey completed by more than 1,700 people with neuromuscular disorders and their relatives. The results revealed a clear need for greater attention to cross-disorder symptoms



and lifestyle factors such as fatigue, nutrition, and physical activity. The programme aligns with the wider movement in healthcare towards 'positive health', emphasising resilience and self-management rather than disease and limitations. It is carried out by a consortium of rehabilitation experts from Amsterdam UMC, Radboudumc and UMC Utrecht, in close partnership with patients and their representatives.

## **Accelerator Programme**

The Accelerator Programme (or Boost Programme) is a continuous funding stream supporting urgent and topical projects that can provide a boost within one of the four themes, where the Spierfonds can play a key accelerating or multiplying role. Examples include:

- Short, one-off projects with rapid impact;
- Large (inter)national projects in which the Spierfonds acts as co-funder;
- Seed funding for spin-off companies;
- Knowledge support/training/coaching.



# Talent Programme

To fulfil our ambitions in scientific research, investing in talent is essential. Dutch researchers are among the international leaders in the field of neuromuscular disorder research - a position the Spierfonds is proud of and intends to strengthen. To maintain this leading role, we invest in the next generation of research leaders through the following instruments:

Activity	Description	Frequency
Young Talent Symposium	Event for PhD candidates	Every 2 years
Spierfonds Network Event	Event for early-career researchers	Every 2 years
Spierfonds Impact Award	Annual prize for a young researcher	Annual
(Endowed) chairs	Chairs within university medical centres	Incidentally

Table 2: Planned components of the talent programme for the 2025–2028 policy period. These are subject to change.

## Events for early-career researchers

The Spierfonds supports research talent in the Netherlands to attract and retain young researchers within the field of neuromuscular disorders. We therefore organise two alternating annual events:

- Young Talent Symposium, a two-day symposium for PhD candidates.
- Network Event, a networking event for early-career researchers at a more advanced stage.

Both events are co-organised by young researchers themselves and offer an excellent opportunity to connect and deepen expertise within the field.

## Spierfonds Impact Award

The annual Spierfonds Impact Award is presented to a young researcher who has made a concrete and valuable contribution with impact for science and/or for people with a neuromuscular disorder. The prize is intended to reward and inspire early-career researchers. The submission deadline is in the autumn.

#### (Endowed) professorships

Where necessary, the Spierfonds funds (endowed) chairs in the field of neuromuscular disorders to ensure that the topic holds or gains a strategic position within university medical centres. In this way, we contribute to strengthening the research infrastructure. Together with the research community, we monitor developments and identify potential gaps.



# Research with societal impact

It is essential for the Spierfonds to invest in research that bridges science and society. Ultimately, everything we do serves one purpose: improving the lives of people with a neuromuscular disorder. We therefore want research outcomes not only to generate new knowledge, but also to be implemented – in practice, in policy, and in society. This requires a strong focus on relevance, active collaboration with patients and their representatives, and the translation of research results into practical and policy applications. These principles are integrated into all our funding instruments and in every phase of research.

# Knowledge utilisation

Research results only create real impact once they reach practice. The Spierfonds therefore attaches great importance to the implementation of research findings. We expect researchers to consider applicability and knowledge utilisation already when designing their project proposals. During assessment, we explicitly review their plans for implementation and dissemination. After project completion, we remain in dialogue with researchers to explore next steps for translating knowledge into concrete societal, clinical or policy applications.

#### Patient involvement

Research must align with real societal needs and priorities. By engaging with patients and their families, we ensure that the research focus reflects what truly matters. We therefore expect researchers to incorporate the perspective of patients (or patient representatives) in both the design and conduct of their research, even when this is not straightforward, such as in preclinical studies. Patient involvement is valuable at every stage of every research project.

Further information on patient participation can be found at:

- Participation matrix
- Spierziekten Nederland
- Kickstarter

#### Socially responsible licensing

Affordability is a key concern for the Spierfonds. We believe that the licensing of research results should adhere to the '<u>Ten Principles for Socially Responsible Licensing</u>' established by the NFU. By applying these principles, knowledge institutions, (academic) hospitals and companies can better fulfil their societal responsibility when licensing knowledge generated largely with public funds. This contributes to the accessibility of treatments and ensures that the societal value of publicly funded medicine research is preserved.

## Open Science en FAIR data

Sharing data, methods, and outcomes accelerates knowledge development and increases the reproducibility and accessibility of research. The Spierfonds therefore aims for 100% Open Access publication. Closely linked to this is the FAIR principle: Findable, Accessible, Interoperable and Reusable. We consider it a societal responsibility for researchers to contribute to Open Science and thus to greater transparency in scientific results. This ensures that knowledge is available to our community and to society as a whole, while also enabling researchers to build on existing work and accelerate the development of practical applications.



## Animal research policy

The Spierfonds would prefer a future in which animal research is no longer necessary. At the same time, we do not want to delay possible treatments for people with a neuromuscular disorder. To prevent unnecessary animal studies, we apply strict evaluation procedures. The majority of the projects we fund use alternatives, such as laboratory-grown human cells or computer models. We continue to actively promote the development and use of alternatives, such as the 3D-muscle-on-a-chip we helped develop, and through our joint programme on 'human measurement models' with the Dutch Health Foundations.

Read our joint statement on animal research and animal-free innovation by the Dutch Health Foundations (SGF).

#### Assessment of research

The Spierfonds funds scientific research that is relevant, of the highest quality, and has potential for continuation. To help us select the right projects, we rely on external experts, our User Committee, and our independent Scientific Advisory Board. All funded projects are assessed by one or more of these bodies. The assessment procedure differs between funding instruments.

#### External experts

External reviewers are PhD-qualified researchers from abroad with no conflict of interest. They assess projects based on scientific quality, impact, and the expertise of the applicant and project group.

#### **User Committee**

The User Committee consists of individuals who have a stake in the outcomes, such as people with a neuromuscular disorder, their families, and care professionals. They assess relevance, impact, and patient involvement.

#### Scientific Advisory Board

The Scientific Advisory Board consists of independent researchers from Dutch universities and university medical centres. The board evaluates relevance, feasibility and anticipated results, and reviews the quality of the external peer review. It also supports the Spierfonds in monitoring the progress of ongoing research projects.