# Spierfonds Bridging the Gap

Grant round 2024

The aim of the one-time grant round 'Bridging the Gap' is to provide a solution to urgent issues on the path to a treatment for people with a neuromuscular disorder. We do this by funding scientific research into the various phases of drug development. We are looking for projects that align with the current status of research of the neuromuscular disorder(s) in question. What is needed to make progress, and why is this necessary right now? The grant round is open to young research talents, ranging from (almost) postdoctoral researchers to emerging group leaders.



# Stop neuromuscular disorders!

In the ideal world of the Prinses Beatrix Spierfonds, all people with a neuromuscular disorder in the Netherlands have access to effective medications that stop the progression of the disease. We achieve this by funding targeted and innovative scientific research, which is the key to fundamental success. However, our focus extends beyond tomorrow; we also invest a portion of our energy and funds in good care, education, and advocacy. This ensures that people with a neuromuscular disorder can lead a fulfilling life and can count on understanding from society.

Creating breakthroughs is a matter of perseverance. Much has already been done, developments are progressing rapidly, and we are achieving success more quickly. However, there is still much work to be done. It is crucial to maintain focus, persevere, and persist, especially now. Only by doing so can we transform neuromuscular disorders from untreatable to treatable.

# Bridging the Gap

The Spierfonds believes that we can win the fight against neuromuscular disorders through scientific research. Therefore, we are fully committed to developing new medications, improving existing treatments, and providing rapid access to new drugs.

The goal of the grant round 'Bridging the Gap' is to provide a solution to urgent issues on the path to a treatment for people with a neuromuscular disorder.

We want to achieve this by funding scientific research into the various phases of drug development, from accurately describing the symptoms and finding the cause to developing and marketing a medication. **Urgency** is the key word in this process and plays a significant role in the evaluation procedure and the ultimate ranking of research proposals.

We are seeking projects that align with the current status of research into the neuromuscular disorder(s) in question. We ask researchers to consider the context of the disease: what is already happening, in the Netherlands and globally? What is needed to make progress, and why is this necessary right now?

#### What types of research are eligible?

Within this call, proposals can be submitted for one of the following themes:



#### **Improved diagnostics**

It is crucial that individuals with a neuromuscular disorder receive an accurate diagnosis. Only then can they be treated effectively. We fund research aimed at improving the diagnostics for neuromuscular disorder(s) that are challenging to diagnose or have not yet been clearly identified.



#### **Targets for therapy**

To find solutions, it's necessary to understand what goes wrong. Therefore, we finance research focused on identifying the cause or unravelling the disease mechanism. This is particularly urgent when, in the current status of research, there is insufficient knowledge to work on targeted therapies, and there are few or no drugs in development globally.



#### **Development and/or testing of treatments**

Once we understand what is going wrong, we can move from the laboratory to the patient. We provide funding for both clinical and preclinical research focused on developing and/or testing a therapy that targets the cause or disease mechanism. Drug repurposing is one of the subthemes here.



#### **Trial readiness**

We aim to prepare the Netherlands adequately for the arrival of clinical trials. In the execution of clinical trials, robust measurement tools are essential to determine the effect of new medications on patients. Therefore, we fund research on the natural course of the disease and outcome measures. This becomes particularly urgent when clinical trials are on the horizon.

#### What is important to the Spierfonds?

#### Patient participation

Patient participation can be valuable for any research project in any phase. Therefore, involvement of patients (and/or patient representatives) in the design of the project is mandatory. It is also required to include a recommendation letter from a relevant patient organization. Please consider a lead time of at least three weeks for this process.

#### • <u>Implementation</u>

We aim to make a difference for patients as quickly as possible. Therefore, we highly value the implementation of research results. In the application form, we inquire about the steps that will follow the proposed project. To ensure successful implementation in the clinic, it is mandatory for one of the members of the project group to be affiliated with an <u>expertise centre for neuromuscular disorders</u> recognized by the Dutch Ministry of Health, Welfare, and Sport.

#### Socially responsible licensing

Affordability is a crucial topic for the Spierfonds, which we are keen to discuss – if applicable. We align ourselves with the position of the SGF and believe that licensing research results should adhere to the 'Ten Principles for Socially Responsible Licensing' established by the NFU.

## • Open Science

The Spierfonds considers it a societal responsibility for researchers to contribute to Open Science and thereby enhance transparency of scientific research results. We strive towards 100% Open Access Publishing.

#### Collaboration

We encourage collaboration with patients, other departments, institutions, and companies. This way, knowledge, resources, creativity, and experience can reinforce each other, especially if it enhances the possibilities for implementing research results.

# Who can apply?

Applicants must meet the following criteria:

- The applicant is a postdoctoral researcher (or almost) or an emerging group leader, aspiring to a career in the field of neuromuscular disorders.
- The applicant is appointed as an assistant professor or in a more junior position.
- On the closing date of the grant round, the applicant must have obtained their PhD within the
  past ten years or is a PhD candidate and can reasonably demonstrate that they will complete
  their PhD in 2024. The <a href="https://www.nwo.extension.org/nwo

The applicant submits the application individually but is endorsed by a co-applicant: a senior researcher (an established scientific researcher in the field of neuromuscular disorders with their own research line or program) who endorses the importance and urgency of the project.

#### What is the budget?

For this grant round, a total amount of € 1,420,000 has been budgeted. An amount of € 100,000 - € 350,000 can be requested per grant application, for projects with a duration of a minimum of 1 year to a maximum of 4 years. We encourage researchers to request an amount that is appropriate for the project and their own position. The budget is composed of personnel costs (for own salary or hiring personnel), material costs, and a budget for career building.

#### Career building budget

With the career building budget, the Spierfonds wants to stimulate the professional development of the applicant. This budget can be used, for example, for experience abroad, setting up a new collaboration or organising a network meeting. This budget can only be used by the applicant, not by any appointed staff. The maximum reimbursement for career building is € 20,000, and this falls within the overall total budget.

#### How are applications assessed?

Applications are assessed by external reviewers on scientific quality, impact, urgency and quality of the project group, and by our User Committee on relevance, the route to societal impact, and consideration and involvement of patients. The assessments by the reviewers and the User Committee are submitted to the applicant, who is given the opportunity to respond to them in writing (rebuttal). Based on the assessments and the rebuttal, our Scientific Advisory Board makes a final assessment. In case of equal suitability, the Spierfonds may prioritise based on policy and interest.

More information can be found in the Scientific Research Assessment Procedure 2024.

#### What is the deadline for submission?

The deadline for submission is **Tuesday 26 March 2024 at 2pm**. Relevant documents and forms can be found on our <u>grant portal</u>. Applicants must send the fully completed and signed form via the grant portal before the deadline.

#### When will the results be announced?

Applicants will be notified of the decision in September 2024. The timetable is as follows:

Deadline applications	26 March at 2pm
Assessment by reviewers and User Committee	April – half of June
Rebuttal	17 June – 11 July
Meeting Scientific Advisory Board	August/September
Meeting Supervisory Board and Management Board	September
Notification of decision	September

#### Relevant documents when applying for a grant (see our grant portal)

- This manual
- The Grant application form 'Spierfonds Bridging the Gap' 2024
- The Scientific Research Assessment Procedure 2024
- The Regeling Subsidieverlening 2023-2024
- The Algemene Subsidievoorwaarden

#### Points to consider when filling out the application form

- Make sure that the application fits within the guidelines in this manual.
- Make sure that the application meets the conditions in Appendix I.
- Ensure that you consult with patients (and/or patient representatives) on impact and participation during the design of the application.
- Include a recommendation letter from a relevant patient organization.
- Ensure that the maximum number of words per section is not exceeded.
- Ensure that the public summary in section 5 is easily understandable and does not contain unnecessary technical terms. This section is crucial in the evaluation by the User Committee.
   Tip: ask a layperson (e.g., a patient or someone outside the research field) to read it and use the tips and tricks from the SGF Guide for Researchers (page 4).
- Provide clear answers in sections 9 to 11 and use easily understandable language. These sections are crucial in the evaluation by the User Committee.
- Clearly describe the methodology in section 14 and, especially in the case of animal and patient-related research, provide a power analysis; this is a crucial point in the evaluation. In animal-related research, justify why the animal model is necessary, why the specific model was chosen, and what the relevance of the animal model is to humans.

#### **Useful links**

<u>Spierziekten Nederland</u> - patient association that can provide input regarding patient participation. Kickstarter - a website on patient participation from PGO Support for researchers.

<u>Dutch Clinical Research Foundation</u> - information on clinical research, with tips on inclusion. <u>Toolkit Maatschappelijk Verantwoord Licentiëren</u> - a toolkit from VSNU and NFU on socially responsible licensing.

<u>PreclinicalTrials.eu</u> - international register with protocols for preclinical animal research. <u>SGF Handreiking voor onderzoekers</u> - tool for writing a public summary in lay language.

### **Contact**

If you have questions about this grant round, such as whether your research idea aligns with the objectives or about patient participation, we are happy to assist. You can contact the Research & Innovation department of the Prinses Beatrix Spierfonds at the general phone number 070 – 3 607 607 or via email at <a href="mailto:onderzoek@spierfonds.nl">onderzoek@spierfonds.nl</a>.

# APPENDIX I: TERMS AND CONDITIONS

In order for grant applications to be considered for evaluation in the 'Bridging the Gap' grant round of the Prinses Beatrix Spierfonds, they must adhere to the following requirements. In case of doubt, the Spierfonds reserves the right not to process a grant application. The final decision rests with the general director of the Spierfonds based on the advice of the chairperson of the Scientific Advisory Board. Applicants will be notified of this decision within six weeks after the submission deadline.

- The applicant must have obtained their PhD within the past ten years (or is a PhD candidate and can reasonably demonstrate that they will complete their PhD in 2024) and holds a position at the institution where the project (mostly) takes place, being a Dutch university, university medical centre, or KNAW institute.
- The applicant is the official contact person for the Spierfonds during the procedure and has submitted the application in Word format via the grant portal before the specified deadline.
- One of the members of the project group is employed at an <u>expertise centre for neuromuscular</u> <u>disorders</u> recognized by the Dutch Ministry of Health, Welfare, and Sport.
- The application relates to one or more of the neuromuscular disorders listed in Appendix II.
- The application includes a recommendation letter from a patient organization.
- The application is written in clear and understandable English and is accompanied by a comprehensible public summary in Dutch.
- The same project proposal may be submitted a maximum of two times. In the case of a
  resubmission, the changes made must be specified in the relevant section of the application
  form. If an application in the second submission was of sufficient quality but could not be funded
  due to a lack of financial resources, it may be submitted a third and final time. Prior to
  resubmitting the application, the applicant must contact the Spierfonds.
- If multiple research proposals are submitted from your department on the same disease topic in one grant round, an accompanying letter should explain why multiple different applications have been chosen and what the relationship between the applications is.
- If funding has been sought elsewhere for the research outlined in the project proposal, this must be reported to the Spierfonds with the results, if applicable.
- If the research pertains to a very rare condition (less than 40 patients in the Netherlands), the application must demonstrate that the results of the research project are more broadly applicable to the Spierfonds' target audience.
- The Spierfonds acknowledges the necessity of using laboratory animals for scientific research. As
  a socially responsible organization, the Spierfonds encourages alternatives to or reduction of
  animal testing, following the guidelines of the <u>Samenwerkende Gezondheidsfondsen</u>. The
  following conditions apply to research with animals:
  - Only research involving invertebrates and small rodents is eligible for funding. Research primarily based on the use of large mammal species is not subsidized by the Spierfonds.
  - The development and characterization of animal models is not funded by the Spierfonds, as these may deviate too far from the diseases covered by the objectives.
- Most of the research project must be conducted in the Netherlands. If part of the research
  project takes place abroad, the Spierfonds must receive a clear proposal for this in advance.
- A research project that has already started before the assessment by the Scientific Advisory Board has taken place is not eligible for funding.

- When drawing up the required budget, the researcher must adhere to the *Regeling Subsidieverlening* of the Spierfonds.
- When a research proposal is granted, the *Algemene Subsidievoorwaarden* of the Spierfonds apply.

# APPENDIX II: NEUROMUSCULAR DISORDERS

#### Motor neuron diseases

- Amyotrophic lateral sclerosis (ALS)
- Polio and post-polio syndrome (PPS)
- Primary lateral sclerosis (PLS)
- Progressive spinal muscle atrophy (PSMA)
- · Spinal-bulbar muscular atrophy (SBMA)
- Spinal muscular atrophy (SMA)

# Peripheral nerve diseases

- Charcot Marie Tooth (CMT / HMSN)
- · Chronic idiopathic axonal polyneuropathy (CIAP)
- · Hereditary neuropathy with pressure palsies (HNPP)
- Small fiber neuropathy

#### Inflammatory neuropathies

- Chronic inflammatory demyelinating polyneuropathy (CIDP)
- Guillain-Barré syndrome (GBS)
- MGUS polyneuropathy
- · Multifocal motor neuropathy (MMN)
- Neuralgic amyotrophy (NA)

# Neuromuscular junction diseases

- · Congenital myasthenic syndromes
- Lambert-Eaton myasthenic syndrome (LEMS)
- Myasthenia gravis (MG)

# Muscular dystrophies

- Becker muscular dystrophy (BMD)
- Duchenne muscular dystrophy (DMD)
- Emery-Dreifuss muscular dystrophy (EDMD)
- Facioscapulohumeral muscular dystrophy (FSHD)
- Limb-girdle muscular dystrophies (LGMD)
- Oculopharyngeal muscular dystrophy (OPMD)
- · Congenital muscular dystrophies (merosin-deficient, Ullrich, dystroglycanopathy, integrin-deficient, rigid spine)

# Myotonic disorders

- Myotonic dystrophy (DM)
- Non-dystrophic myotonias (Thomsen, Becker, Paramyotonia Congenita)
- Periodic paralysis (PP)

## Congenital myopathies

· Brody myopathy

- · Central core disease
- Myotubular myopathy/centronuclear myopathy
- Nemaline myopathy
- · Distal myopathies (Miyoshi, Nonaka, Welander, Markesbery, Laing)

#### Inflammatory myopathies

- Dermatomyositis
- Inclusion body myositis (IBM)
- · Polymyositis

#### Metabolic myopathies

- Glycogen storage diseases
- Lipid storage myopathies
- Mitochondrial myopathies
- Of the infectious diseases, only acute anterior poliomyelitis is eligible for funding.
- Not eligible for funding are research into diseases resulting from trauma, diabetes mellitus, cardiovascular abnormalities, cancer, drug use, or intoxications (alcohol); diseases that are manifestations of a mental illness or disorder; unexplained conditions without an organic substrate.
- For multisystem diseases, the grant application must demonstrate that it primarily presents a neuromuscular phenotype with muscle weakness at the forefront.