Innovative Therapies

Grant round 2025

Prinses Beatrix Spierfonds

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The Prinses Beatrix Spierfonds funds, guides, and stimulates scientific research into neuromuscular disorders. Because we know that scientific research is the only key to making all neuromuscular disorders treatable in the future. By collaborating with leading researchers and maximising the potential of our research, we achieve success. Furthermore, we stay alert to the opportunities that modern technologies and innovations provide.

Programme Drug Development

The Spierfonds believes that we can win the fight against neuromuscular disorders through scientific research. That is why the Spierfonds is launching a multi-year programme to accelerate the development of medicines for neuromuscular disorders in the Netherlands. Through various funding opportunities, we support innovative approaches, multidisciplinary collaboration, and the application of new technologies.

The 'Innovative Therapies' grant round

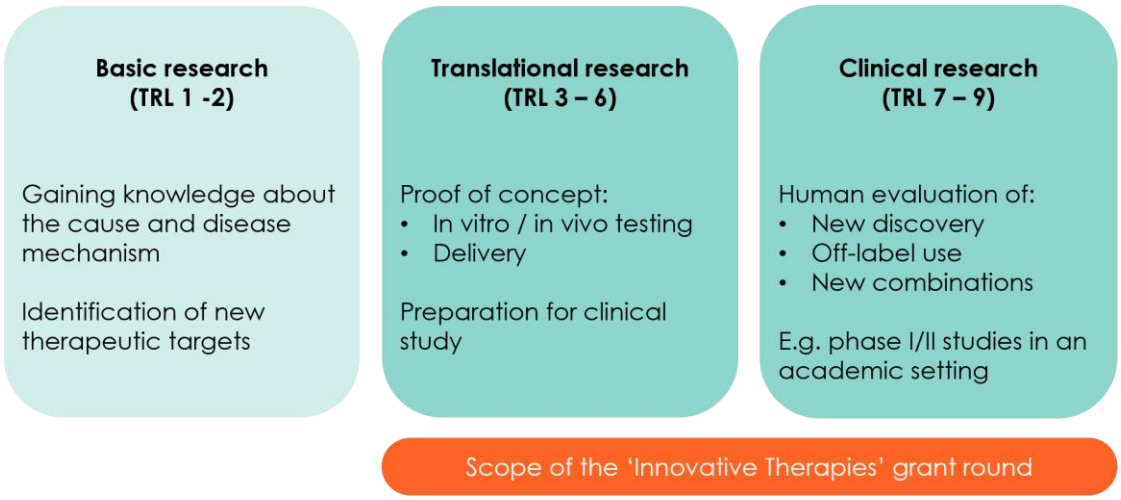
A key part of the Drug Development Programme is the new 'Innovative Therapies' grant round. The goal of this initiative is to advance promising research from preclinical studies more efficiently toward clinical application. We seek projects that contribute to the development of innovative therapies for neuromuscular disorders, targeting either the underlying cause or disease mechanisms.

Principles:

- **Academic research with a strong focus on application**
  To accelerate the translation of promising research results into real-world application, this grant round encourages a goal-oriented approach. Our aim is to enable researchers to take significant steps toward developing innovative therapies. Therefore, we support translational and clinical projects (starting at approximately Technology Readiness Level 3, see Figure 1) that not only expand scientific knowledge but also take concrete steps towards the next phase of development.
- **Equal opportunities for all neuromuscular disorders**
  The Spierfonds is committed to supporting research into all neuromuscular disorders, not just the most well-known or common conditions. This means that no neuromuscular disorder is excluded in advance from this grant round. If the research concerns an ultra-rare condition (fewer than forty patients in the Netherlands), the research results must have broader applicability for the Spierfonds' target group.
- **Collaboration between scientists, expertise centres, and relevant parties**
  Research conducted in isolation often delays the transition from laboratory findings to patient treatments. Therefore, we encourage multidisciplinary collaboration and knowledge sharing, both within and beyond academia. Project proposals are evaluated, among other factors, on the quality and complementarity of the research team. Experience in drug development is not a prerequisite, and junior researchers are welcome to apply, provided that the project group as a whole possesses the necessary expertise to successfully conduct the research and advance its results towards application.
- **Appropriate duration and realistic project planning**
  A well-thought-out project design increases the likelihood of success. We encourage researchers to choose a project duration that aligns with their intended goals and to carefully consider the required expertise and staffing. In some cases, such as sequential projects (where a second phase depends on the results of the first) or high-risk, high-gain studies, a shorter duration may help to evaluate interim outcomes and reduce the risk of

unachieved objectives. This could enhance the chances of obtaining funding. In the application form, we ask researchers to justify their choices, ensuring that the proposed duration and staffing are realistic and appropriate.

Figure 1: Phases of scientific research into drug development.



What research is eligible?

Projects applying for funding in this grant round must meet the following criteria:

- **Relevance:** The research project contributes to the development of an innovative therapy for neuromuscular disorders (including drug repurposing) that targets the underlying cause and/or disease mechanism.
- **Timeliness:** The project addresses a bottleneck or crucial step in drug development and provides clear added value within the international research field.
- **Feasibility**: The project includes realistic and well-substantiated steps towards the next development phase, with a concrete end goal.
- **Project group**: The project group has the right expertise and composition to successfully execute the project.
- **Patient participation:** Patients (or their representatives) are actively involved in the design and execution of the project.
- **Impact**: The research project makes a tangible contribution to drug development and bringing promising therapies closer to patients.

Evaluation process

Preliminary application review

To minimise the burden on applicants, this grant round includes a preliminary selection phase. Project proposals that meet the conditions outlined in Appendix I of this guide will be assessed in writing against the funding criteria mentioned above by representatives from the User Committee, the Scientific Advisory Board (SAB), and the Research & Innovation Department of the Spierfonds. Based on this assessment, up to eight preliminary applications will be selected for further development. These applicants will receive instructions from the Spierfonds on how to submit a full application.

Full application review

Full applications will be assessed by international reviewers and our User Committee. Based on this evaluation, selected applicants will be invited for an interview with the evaluation committee, which includes members of the SAB and patient representatives. During the interview, applicants will have the opportunity to present their proposal, respond to feedback, and address any additional questions from the committee. Following the interviews, the committee will issue a funding recommendation to the Supervisory Board and the Management Board of the Spierfonds.

For the full evaluation procedure, see Appendix II of this manual.

Deadline and budget

We invite all researchers to submit a preliminary application. The deadline is Thursday, **15 May 2025, at 14:00**. Applicants can request funding between €100,000 and €350,000 for projects lasting a minimum of 1 year and a maximum of 4 years. We aim for a funding success rate of at least 40% of full applications.

Timeline

The timeline is as follows:

Deadline pre-proposal	15 May 2025, 14:00
Invitation for full application	16 June 2025
Deadline full application	25 August 2025, 14:00
Interview	Friday, 14 November 2025
Decision	December 2025

Contact

Do you have questions about this grant round? For example, whether your research idea fits within the call? We are happy to assist. Please contact Marije Geilenkirchen via onderzoek@spierfonds.nl.

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Appendix 1: Conditions

Grant applications submitted for the 'Innovative Therapies' grant round of the Prinses Beatrix Spierfonds must meet the following requirements to be considered for evaluation. In case of doubt, the Spierfonds reserves the right not to process a grant application. The decision will be made based on advice from the chair of the Scientific Advisory Board. The applicant will be notified within six weeks after the submission deadline.

The applicant

- The applicant must have a PhD and hold (or will hold) a position at the institution where the project will mainly be conducted, i.e., a Dutch university, university medical centre, university of applied sciences, or KNAW institute.
- A researcher may submit only one application as lead applicant in this grant round.
- The applicant is the official point of contact for the Spierfonds during the evaluation procedure.
- One member of the project group must be employed at a recognized expert centre for neuromuscular diseases accredited by the Dutch Ministry of Health, Welfare, and Sport (VWS).

Objective

- The project must relate to one or more of the neuromuscular disorders listed in Appendix III.
- If the research concerns an exceedingly rare condition (fewer than 40 patients in the Netherlands), the application must demonstrate that the research results are broadly applicable to the Spierfonds' target audience.

The project

- A research project that has already started before the assessment by the Scientific Advisory Board has taken place is not eligible for funding.
- 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.
- 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 Samenwerkende Gezondheidsfondsen. 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.

The project proposal

- The (pre)application must be written in clear and understandable English. A full application must also include an accessible Dutch public summary.

- If multiple project proposals are submitted from the same department in the same grant round on the same subject, a cover letter must be submitted explaining why multiple applications have been chosen and how they relate to each other.
- If a project proposal is submitted from a department where the Spierfonds is already funding research in the same subject area, the new proposal must clearly explain how it relates to the ongoing research.
- If funding has been requested elsewhere for the research outlined in the project proposal, this must be reported to the Spierfonds, along with the outcome.

Regulations

- 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.

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Appendix II: Evaluation procedure 'Innovative Therapies' grant round

Evaluation of preliminary proposals

Research proposals that meet the conditions outlined in Appendix I will be assessed in writing against the funding criteria (see page 3) by representatives from our User Committee, the Scientific Advisory Board (SAB), and the Research & Innovation Department of the Spierfonds. Based on this assessment, a maximum of eight preliminary applications will be selected for further evaluation. These applicants will receive instructions from the Spierfonds on how to submit a full application.

Evaluation of full proposals

Full grant applications will be evaluated substantively by at least three external experts (reviewers) and at least two members of the User Committee. The grant applications will be assessed by the reviewers on four criteria:

- Scientific quality and feasibility
- Timeliness
- Impact
- Quality of the project team

The User Committee will assess from the patient perspective on three criteria:

- Relevance for (future) patients
- Route to societal impact
- Attention to and involvement of patients

Both reviewers and User Committee members will provide their motivated judgments in the designated evaluation form, anonymously.

Conditions for reviewers

Reviewers are non-interested parties to a grant application. The reviewer and the applicant may not have co-authored publications in the last three years. Reviewers are selected internationally, through suggestions from the applicants and online databases. A list of external reviewers from previous years who have provided well-reasoned assessments is also used.

Interviews

Based on the evaluation by the reviewers and the User Committee, a selection of applicants will be invited for an interview. An evaluation committee will be formed, including members of the SAB and patient representatives. Both the applicant and the committee will receive the evaluations from the reviewers and the User Committee in preparation for the interviews. During the interview, the applicant will present the proposed research, address the evaluations, and respond to additional questions from the committee. After the interviews, the evaluation committee will provide a funding recommendation to the Supervisory Board and the Management Board of the Spierfonds.

Funding / rejection

The Supervisory Board and the Management Board of the Spierfonds will decide on the funding and rejection of grant applications based on the funding proposal and the available budget. In case of equal suitability, the Spierfonds may resort to a lottery.

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Appendix III: 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)

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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.