Survey results for the research agenda on children with a neuromuscular disease

SPIERZIEKTEN
NEDERLAND

1. Introduction

One of the objectives of Spierziekten Nederland is to promote high-quality scientific research. Such research cannot be achieved without the input of children with a neuromuscular disease and their relatives (e.g., parents/caregivers).

Which research topics on neuromuscular diseases do children with a neuromuscular disease and their parents consider important? And what information and opportunities for meeting others do they need? Spierziekten Nederland explored these questions through a survey conducted in July, August, and September 2024. The survey was developed in collaboration with the Spieren voor Spieren teams at the university medical centers (UMCs) and the Spieren voor Spieren Youth Panel.

In total, 150 parents and children completed the survey, including approximately 60 children. The children's results have been incorporated into this document.

2. Results

Diagnosis

Diagnosis	Aantal
arthrogryposis multiplex congenita (AMC)	**3**
Becker	**1**
congenital muscle disease	**9**
Duchenne	**4**
FSHD	**1**
Guillain–Barré syndrome	**1**
CMT	**8**
HSP	**2**
myositis	**2**
myotonic dystrophy	**12**
SMA	**3**
Pompe disease	**1**
Other…	**9**

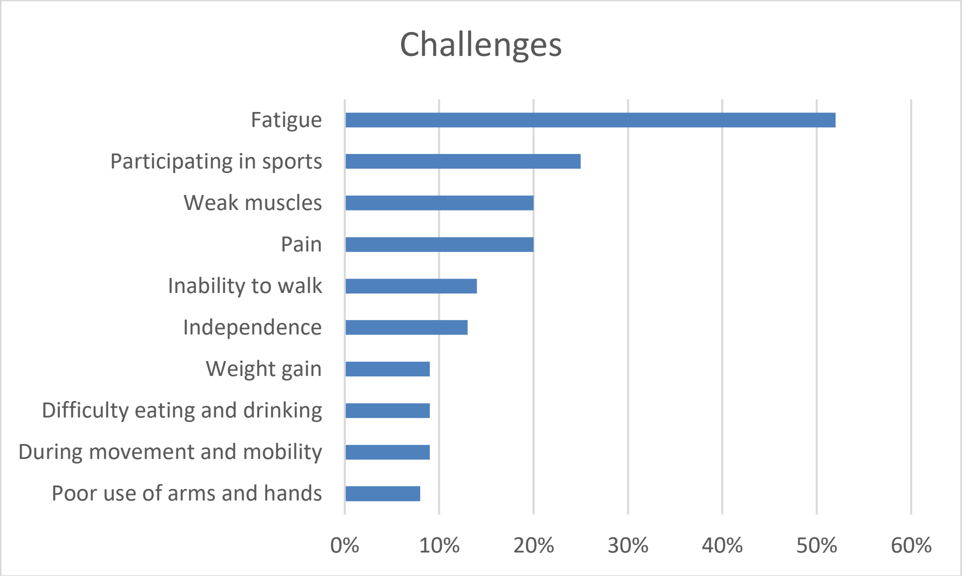
The age of the participants

Age	Total
< 10 years	12
10 years	5
11 years	4
12 years	9
13 years	4
14 years	4
15 years	5
16 years	5

17 years	4
18 years	4

2.1 Living with a muscle disease

These symptoms cause the greatest challenges in daily life for children with a neuromuscular disease. Participants were allowed to select a maximum of three items.



2.2 Research agenda quality of life - children

Which quality-of-life research topics do children consider most important?
We asked this question twice: once as an open-ended question and once as a closed-ended question with items that could be selected.

Top 10

- **Medication and treatment (most mentioned)**
- **Fatigue**
- **Sports and physical activity**
- **Participation**
- **Pain**
- **Assistive devices / aids**
- **Research into the cause of my disease**
- **Understanding from others / social support**
- **Accessibility**
- **Other (nutrition, learning difficulties, ventilation)**

	Subject	*Total*	*Percentage*
1	Being able to participate at school, in play, sports, and part-time work	**28**	**21%**
2	Fatigue	**21**	**11,5%**
3	Coping with having a neuromuscular disease	**17**	**10%**
4	Assistive devices (new devices)	**13**	**8,3%**
5	Mental well-being (feeling good)	**10**	**6,2%**
6	Pain	**7**	**6%**
7	Attention to the family	**7**	**5,2%**
8	Sports and physical activity	**10**	**5%**
9	Coping with disease progression	**8**	**4%**
10	Respiratory problems	**4**	**3,5%**

2.3 Research agenda quality of life - parents

Which quality-of-life research topics do parents consider most important?
We asked this question twice: once as an open-ended question and once as a closed-ended question with items that could be selected.

Top 10

- **Medication and treatment (most mentioned)**
- **Psychological treatment / mental well-being**
- **Physical activity / movement**
- **Fatigue**
- **Assistive devices / aids**
- **Gastrointestinal / urinary problems**
- **Pain**
- **Environmental accessibility**
- **Diagnosis and disease progression**
- **Other**

	Subject	Total	Percentage
1	Being able to participate at school, in play, sports, and part-time work	45	33,3%
2	Assistive devices (new devices)	20	12,2%
3	Fatigue	22	11,8%
4	Attention to the family	19	11,5%
5	Mental well-being (feeling good)	19	11,2%
6	Coping with having a neuromuscular disease	19	10,7%
7	Coping with disease progression	15	10,2%
8	Pain	11	8,7%
9	Use of arms and hands	7	5,3%
10	Respiratory problems	6	4,8%