Questionnaire about care for people with Duchenne Muscular Dystrophy

Patient-ID

Thank you for taking the time to fill out this questionnaire!

Quality of Life and the quality of care for people with DMD can be quite different between European countries or even within one country. In this questionnaire, we would like to find out more about the care you get. The questions ask about different areas of life and care:

A. Some questions about you

B. Your / your son's health

C. The clinic you / your son goes to

D. The treatment you / your son gets for DMD

How to complete the questionnaire

For most questions you should choose the one response which fits best for you, and mark it with a cross. In some cases more than one answer can be given, and these are clearly marked.

Example:

1. Who is filling out the questionnaire?	
The person with DMD on his own	☒
The parents of the person with DMD/other	☐
Both, the person with DMD and parents/other together	☐

If you want to correct your answer, please mark the correct one with a circle as in the following example:

1. Who is filling out the questionnaire?	
The person with DMD on his own	☒
The parents of the person with DMD/other	☒
Both, the person with DMD and parents/other together	☐

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A. Some questions about you

1. Who is filling out the questionnaire?	
The person with DMD on his own	☐
The parents of the person with DMD/other	☐
Both, the person with DMD and parents/other together	☐

2. What is the year and month of your / your son's birthday? (E.g. May 2002 should be written down as 05 2002)	
_____ mm	_____yyyy

3. What is your / your son's current occupation?	
Nursery	☐
Regular mainstream public or private school	☐
Special needs school	☐
Vocational training	☐
University	☐
Job	☐
None	☐

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4. What is the highest educational level of the following members of your family? If vocational training has been finished, this box can be ticked additionally.						
	Nursery	**Primary school**	**Secondary school**	**A-level**	**University, college, master, doctor etc**	**Finished vocational training**
1. Patient	☐	☐	☐	☐	☐	☐
2. Mother	☐	☐	☐	☐	☐	☐
3. Father	☐	☐	☐	☐	☐	☐

5. How do you/does your son usually get to kindergarten/school/university/job?	
Transport by a family member or friend	☐
Public transportation/school bus/special transfer	☐
No transport needed	☐

6. Where do you /does your son mostly live?	
With parents or with other relatives or friends	☐
In an institution	☐
On his own [with support as necessary]	☐
With a partner	☐

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7. In addition to family or friends, do you/ does your son have a personal assistant at home or nursery/school/job/university? If more than one response applies, please tick the one which applies most of the time.	
Yes, 24 hours per day	☐
Yes, for more than 6 but less than 24 hours a day	☐
Yes, for less than 6 hours per day	☐
Yes, but not every day	☐
No	☐

8. Is anyone in the family member of a patient advocacy group for Duchenne or neuromuscular diseases in general?	
Yes ☐	No ☐

9. What is your monthly net household income? This is the total income of all people living in your household after tax. We ask this question because we think that the household income might have an influence on quality of life. If you don't want to answer this question, you can leave it out.	
Less than 750 Euro	☐
750 - 1500 Euro	☐
1500 – 2250 Euro	☐
2250 – 3000 Euro	☐
More than 3000 Euro	☐

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B. Finding out a bit about your / your son's health

10. Prior to the diagnosis of DMD, how old were you/ was your son, when the family first expressed concerns about motor development to their doctor?	
_____ years_____months	
I don´t know.	☐

11. In the course of Duchenne Muscular Dystrophy, key stages can be defined. These stages are a simplification and not all descriptions might apply to you. However, they can be a general guide. **Which of these <u>best</u> describes the <u>current</u> stage of your / your son's DMD?**		
Presymptomatic	No symptoms	☐
Early-ambulatory	Abnormal gait, able to climb stairs	☐
Late-ambulatory	Walking more difficult, wheelchair intermittently used, losing abilities to get up from floor and climb stairs	☐
Early non-ambulatory	Lost walking ability but can still sit and stand, full time active use of non-powered wheelchair which can be used independently	☐
Non-ambulatory I	Active use of non-powered wheelchair not possible, armstrength increasingly limited, hands can be raised to mouth	☐
Non-ambulatory II	Electric wheelchair necessary. Hands cannot be raised to mouth, but hands can be used to hold a pen or use to move electric wheelchair.	☐
Non-ambulatory III	Electric wheelchair necessary. No useful function of hands	☐

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12. If you are/your son is no longer able to walk independently, at what age did you / he lose the ability to walk?	
_____years_____ months	
I am still able to walk independently.	☐
I don´t know.	☐

13. How do you / does your son sit?	
Without support	☐
Only with the support of a spinal brace, backrest or neck support	☐
Cannot tolerate a sitting position by any means	☐

14. Irrespective of who funded them, what types of assistive device, if any, do you / does your son have at the moment?			
Assistive device	**Yes**	**No, not needed**	**No, but I/we need it**
1. Manual wheelchair	☐	☐	☐
2. Electric (powered) wheelchair or electric vehicle for outdoor use	☐	☐	☐
3. Sitting support in wheelchair	☐	☐	☐
4. Transfer device (lift, slide sheet)	☐	☐	☐
5. Bathroom equipment (e.g.bath chair, grab bars)	☐	☐	☐
6.Hight adjustable bed and / or special mattress	☐	☐	☐
7. Enviromental control access(„top end equipment“ like infrared pointing, eye-gaze selection)	☐	☐	☐
8. Computer to compensate for functional loss (e.g. writing at school)	☐	☐	☐

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15. How many hours a day does the person with DMD use for his personal needs such as toileting, washing himself, getting dressed, being positioned and eating?	
less than 2 hours	☐
2- less than 4 hours	☐
4-less than 6 hours	☐
6 hours or more	☐

16. How many days a week do you / does your son spend outside home or take part in activities outside your home? (apart from winter month)	
1-2 days	☐
3-5 days	☐
6-7 days	☐
none	☐

17. Do you / does your son believe that you are / your son is viewed on equal terms by other citizens in your local community?	
Yes, always	☐
Most of the time	☐
Sometimes	☐
Seldom	☐
Never	☐

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C. Now we would like to find out more about the main clinic you / your son goes to.

18. Do you / does your son attend a clinic where the medical staff specialise in neuromuscular disorders? If yes, how often?	
Yes, at least once every 6 months	☐
Yes, at least once every year	☐
Yes, less than yearly	☐
No	☐

19. If you do not attend a specialized clinic, why is this?	
A specialized doctor / clinic is too far away	☐
A specialist is not needed	☐
I / we didn't know that this existed.	☐
Other reasons: _____	☐
Not applicable, I do attend a specialized clinic.	☐

20. What is the travel time from your home to your neuromuscular clinic?	
Less than 1 hour driving time	☐
1-3 hours driving time	☐
More than 3 hours driving time	☐
I don't know/ I don't go to a specialized clinic.	☐

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21. How was the diagnosis of DMD established in your case? You may mark more than one answer.	
Muscle biopsy	☐
Blood test for genetic testing	☐
Physical assessment	☐
Blood test for elevated Creatinekinase (CK)	☐
Positive family history of DMD	☐
I don't know.	☐

22. How old were you/ was your son, when DMD was confirmed either genetically or by muscle biopsy, NOT only based on symptoms or elevated CK in blood?	
_____years_____ months.	
I don't know / No biopsy or genetic testing was done.	☐

23. What kind of help for coping with the diagnosis was offered to you by your doctor at the time of diagnosis? You may mark more than one answer.	
Contact with psychologist	☐
Contact with social worker / care coordinator	☐
Referral to support group	☐
Taking part in courses for families with DMD	☐
Sufficient time to talk to doctor or a second appointment with the doctor	☐
Brochures/Websites	☐
Not what I/we needed	☐
Others: _____	☐

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Continue of question 23 → for coping with the diagnosis	
I did not need help for coping.	☐
I don't know.	☐

24. Information about DMD				
Has a medical professional ever talked to you/your son about	**Yes, sufficiently**	**Yes, but not enough**	**No, not at all**	**I don't remember**
1. Genetic counselling?	☐	☐	☐	☐
2. The course of the disease and the main problems that may arise?	☐	☐	☐	☐
3. Treatment with steroids in DMD?	☐	☐	☐	☐
4. Breathing problems in the course of DMD?	☐	☐	☐	☐
5. Cardiac problems in DMD?	☐	☐	☐	☐
6. Prevention of curved spine (scoliosis) and joint contractures in the course of DMD?	☐	☐	☐	☐
7. How to prevent excessive weight gain or loss?	☐	☐	☐	☐
8. Gastric tube placement in case in case of weight loss and difficulties with eating enough?	☐	☐	☐	☐
9. Adequate schooling or special learning needs of some children with DMD?	☐	☐	☐	☐
10. Patient organisations?	☐	☐	☐	☐

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25. Regular assessments				
How often do you/ does your son get…	**At least once every 6 months**	**At least once per year**	**Less than once per year**	**Never**
1. An ultrasound of your heart (= echocardiography)?	☐	☐	☐	☐
2. An assessment of lung function?	☐	☐	☐	☐
3. Inspection of the spine or spinal X-rays to check for curved spine (scoliosis)?	☐	☐	☐	☐
4. Assessment of functional abilities like walking, standing, sitting, use of hands	☐	☐	☐	☐

26. A marker of lung function is the FVC ("forced vital capacity"). It measures the amount of air which can be forcibly exhaled from the lungs after taking the deepest breath possible**. In what range is your current FVC? Please consider the most recent FVC, measured within the past 12 months.** It may be helpful to consult your latest medical records for this information. FVC is expressed as a percentage of what can be expected from a healthy person of the same age, sex and bodyweight/hight. Please also note the date of the examination.

FVC	**Below 20%**	**20-49%**	**50-79%**	**80% and more**	**No measurement within the past 12 months**	**I don't know**
	☐	☐	☐	☐	☐	☐

27. In the ultrasound, cardiac function is determined by measuring the ejection fraction of the left ventricle (LVEF). This is the percentage of the blood volume ejected by the left ventricle at each heart beat. **In what range is your current LVEF? Please consider the most recent LVEF, measured within the past 12 months.** For answering this question you might check the last medical note again.

LVED	**Below 40%**	**40-54%**	**55% and more**	**No measurement within the past 12 months**	**I don't know**
	☐	☐	☐	☐	☐

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D. The next questions deal with the treatment you / your son get for DMD

28. Treatment of DMD I					
	Yes, sufficiently	**Yes, but not enough**	**No, not at all**	**I don't remember**	**I don't need it**
1. Have you/has your son ever been instructed in doing stretching at home?	☐	☐	☐	☐	☐
2. Have you / has your son ever had an assessment to determine any special educational needs?	☐	☐	☐	☐	☐
3. Have you/ has your son ever received psychological support for coping with the diagnosis or in managing daily life?	☐	☐	☐	☐	☐
4. Have you ever been given advice concerning social issues (e.g. legal rights to assistance, job opportunities)?	☐	☐	☐	☐	☐
5. Do you feel involved in decision making in your / your son's current medical treatment?	☐	☐	☐	☐	☐

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29. Treatment of DMD II				
Do you/ does your son currently receive…	**Yes, 60 minutes or more weekly**	**Yes, less than 60 minutes weekly**	**No, have received it before**	**No, never received it**
6. physical training, stretching or other physical exercises by a qualified professional?	☐	☐	☐	☐
7. training in activities relating to daily life and/or advices for use of technical aids by a professional?	☐	☐	☐	☐
8. Speech therapy?	☐	☐	☐	☐

30. Are you / is your son on steroid treatment (brand name) for DMD?	
Yes	☐
No, I have never taken them as a treatment for Duchenne.	☐
No, I have stopped.	☐

31. If you have/ your son has never taken steroids, why is this?	
They are too expensive.	☐
They are not available in our region/country.	☐
They were not proposed by the doctor.	☐
We didn't want to take steroids.	☐
Not applicable, I am taking/have taken steroids.	☐

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32. If you have/ your son has stopped taking steroids, why is this?	
Stopped after loss of ambulation	☐
Stopped because of intolerable side effects	☐
Stopped for other reasons:_____	☐
Not applicable, I am taking steroids	☐

33. If you are/your son is or have/ has been on steroids, at what age in years did you / he start taking them?	
At _____years	
I don´t know	☐
Not applicable, I am not taking and have never taken steroids	☐

34. Has a cardiomyopathy been diagnosed?	
Yes	☐
No	☐
I don´t know	☐

35. Do you / does your son receive medication to prevent or treat cardiomyopathy?	
Yes	☐
No	☐
I don´t know	☐

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36. How many nights have you / has your son spent in hospital in the past two years?	
_____ nights	
None	☐
I don't know.	☐

37. How many of these nights (see question 36) were <u>unplanned</u> and due to each of the following causes? Please write down the number of <u>unplanned</u> nights spent in hospital for each possible reason. If you / your son hasn't spent a night for a specific reason, please write "0" in that row.	
Unplanned admissions:	
_____ nights due to acute respiratory problems (e.g. chest infection)	
_____ nights due to bone fractures	
_____ nights because of other reasons (unplanned)	
I don't know.	☐

38. How many of these nights (see question 36) were <u>planned</u> and due to each of the following causes? Please write down the number of <u>planned</u> nights spent in hospital for each possible reason. If you / your son hasn't spent a night for a specific reason, please write "0" in that row.	
Planned admissions:	
_____ nights for routine check-ups	
_____ nights because of planned surgery	
_____ nights because of other reasons (planned)	
I don't know.	☐

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39. Have you / has your son undergone spinal surgery because of scoliosis?	
Yes	☐
No	☐
No, but spinal surgery is planned	☐

40. What kind of breathing support do you/does your son get at the moment? You may mark more than one answer.	
None	☐
Lung volume recruitment techniques	☐
Manual assisted cough	☐
Mechanical assisted cough (e.g. cough assist, Pegasus or other devices)	☐
Non-invasive ventilation (with a mask) intermittently for some hours during sleep or daytime	☐
Non-invasive ventilation continuously (24 hours/day)	☐
Invasive ventilation (via tracheotomy) for some hours during sleep or daytime	☐
Invasive ventilation during (via tracheotomy) continuously (24 hours/day)	☐

41. How satisfied are you with your overall medical treatment?			
Very satisfied	**Rather satisfied**	**Rather not satisfied**	**Not satisfied at all**
☐	☐	☐	☐

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42. Do you miss any aspects of your treatment in this questionnaire? Here you can also provide any other comments or additional feedback you have about the care you receive.

Thank you for your help!