

Registration form for the TREAT-NMD DMD Patient Registry

To be included in the DMD Patient Registry, you need to fill in this registration form and also read and sign the patient information and informed consent form.

Before you start filling in this form, please read the instructions provided. The instructions provide explanations of a lot of the things that might sound a little complicated at first.

MANDATORY ITEMS

(If you don't answer all of these questions, we won't be able to include your details in the global registry.)

I am:

- The patient
 The patient's representative *(please tick as appropriate)*

1. Patient's personal details:

Sex: male / female *(delete as appropriate)*

First name(s): _____

Family name: _____

Date of birth: ___ / ___ / _____ (dd / mm / yyyy)

(UK) NHS number: _____

Address
(incl. postcode): _____

Email: _____

Phone: _____

2. If you are the patient's representative (parent/guardian), please provide your details:

Name: _____

Address: _____

Email: _____

Phone: _____

Relationship to
patient: _____

3. What is the patient's genetic test result?

Mutation name in *DMD* gene following HGVS rules (based on cDNA Ref Seq):

C. _____

- Deletion of exon(s): Have all exons been tested?
 - Yes
 - No
 - Unknown
- Duplication of exon(s): Have all exons been tested?
 - Yes
 - No
 - Unknown
- If the result of the genetic testing shows a deletion or duplication: Are the boundaries known?
 - Yes
 - No
 - Unknown
- If the result of the genetic testing shows a point mutation: Have all exons been sequenced?
 - Yes
 - No
 - Unknown
- If targeted mutation testing has been performed with the patient's DNA, have all exons been tested in a male relative who is also a DMD patient?
 - Yes
 - No
 - Unknown

4. What is the patient's diagnosis, according to your doctor?

- Duchenne Muscular Dystrophy
- Becker Muscular Dystrophy
- Intermediate Muscular Dystrophy
- Female Carrier
- I don't know

5. Current best motor function (please tick the most appropriate answer):

- Patient is currently able to walk (with or without help/support)
- Patient is currently not able to walk

6. If the patient is 3 years old or more, does he/she have to use a wheelchair?

- Patient uses a wheelchair permanently (started full-time use at age: _____ years)
- Patient uses a wheelchair part-time/intermittently (started at age: _____ years)
- Patient has never used a wheelchair
- Unknown

7. Is the patient taking steroids (glucocorticoids) for Muscular Dystrophy?

- Yes, he/she is currently taking steroids (glucocorticoids)
- No, not at the moment, but he/she previously took steroids
- No, he/she has never taken steroids
- Unknown

8. Has the patient had spinal surgery for scoliosis?

- Yes
- No
- Unknown

9. Is the patient currently taking any medication to treat or protect his/her heart?

- Yes, he/she is currently taking the following heart medication:
 - Atenolol (e.g., Tenormin, Selobloc)
 - Bisoprolol (e.g., Concor, Rivacor)
 - Celiprolol (e.g., Cardem, Celectol, Cordiax, Dilanorm, Selectol)
 - Metoprolol (e.g., Lopressor, Selokeen, Minax, Betaloc, Neobloc, Corvitol, Beloc)
 - Nebivolol (e.g., Hypoloc, Nebilet, Lobivon)
 - Oxprenolol (e.g., Trasicor, Coretal, Laracor)
 - Pindolol (e.g., Visken, Cardilate, Pectobloc)
 - Propranolol (e.g., Dociton, Inderal)
 - Bupranolol
 - Penbutolol (e.g., Levatol, Lobeta, Hostabloc)
 - Sotalol (e.g., Betapace, Darob, Rentibloc)
 - Carvedilol (e.g., Coreg, Eucardic, Dilatrend)
 - Captopril (e.g., Capoten, Adocor)
 - Enalapril (e.g., Renitec, Vasotec, Benalapril, Alapril, ACEpril)
 - Lisinopril (e.g., Prinivil, Tensopril, Zestril, Hipril, Acerbon, Acemin)
 - Ramipril (e.g., Altace, Delix, Hypren, Triatec)
 - Benazepril (e.g., Lotensin)
 - Perindopril (e.g., Coversyl, Aceon)
 - Fosinopril (e.g., Monopril, Dynacil)
 - Trandolapril (e.g., Mavik)
 - Hydrochlorothiazid (e.g., Disalunil, Esidrex)
 - Furosemid (e.g., Lasix, Diurapid)
 - Torasemid (e.g., Unat, Torem)
 - Spironolacton (e.g., Aldactone, Novo-Spiroton)
 - Other (please specify: _____)
- No, he/she is not taking any heart medication
- Unknown

10. Is the patient currently included in a clinical trial?

- Yes, he/she is currently included in one of the following clinical trials:
 - PTC Therapeutics: PTC 124 (Ataluren) trial plus extension
 - Prosensa: PRO51 Exon-Skipping trial, systemic administration plus extension
 - AVI: AVI4658 Exon-Skipping trial, systemic administration plus extension
 - Santhera: Idebenone trial

- He/she is currently not included in a clinical trial, but has previously participated in the following trial:
 - PTC Therapeutics: PTC 124 (Ataluren) trial
 - Prosensa: PRO51 Exon-Skipping trial, local or systemic administration
 - AVI: AVI4658 Exon-Skipping trial, local or systemic administration
- No, he/she has never participated in a clinical trial
- Unknown

HIGHLY ENCOURAGED ITEMS

(We can still include your details in the global registry even if you can't answer all of these questions, but please answer as many of them as you can.)

11. Is the patient currently able to sit without support?

- Yes
- No
- Unknown

12. Has cardiomyopathy (affection of the heart muscle) been diagnosed in the patient?

- Yes
- No
- Unknown

If the patient has had an ultrasound of the heart, please fill in the result as far as you know it:

LVEF (Left Ventricular Ejection Fraction) _____ %

Date of the examination: _____

13. Does the patient regularly use a non-invasive ventilation device?

- Yes, all day
- Yes, but only part-time (e.g. at night)
- No, never
- Unknown

14. Does the patient use invasive ventilation?

- Yes, all day
- Yes, part-time
- No
- Unknown

If the patient has had pulmonary function testing, please fill in the result if you know it:

FVC (Forced Vital Capacity) _____ % (predicted value)

Date of the test: _____

15. Has the patient ever had a muscle biopsy?

- Yes
- No
- Unknown

16. Has the patient signed up for any other DMD registry?

- Yes (if yes, please specify: _____)
- No
- Unknown

17. Does anybody else in the patient's family have the same kind of disease?

- Yes (if yes, please specify: _____)
- No
- Unknown