

**Muscular
Dystrophy UK**

Fighting muscle-wasting conditions



A guide for parents: **Duchenne muscular dystrophy**



About Muscular Dystrophy UK

Muscular Dystrophy UK is the charity bringing individuals, families and professionals together to beat muscle-wasting conditions.

- ▶ We support high-quality research to find effective treatments and cures, and lead the drive for faster access to emerging treatments for UK families.
- ▶ We ensure everyone has the specialist NHS care and support they need, with the right help at the right time, wherever they live.
- ▶ We provide a range of services and opportunities to help individuals and their families live as independently as possible.

About the authors

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Other publications

- ▶ Physiotherapy Management for Duchenne Muscular Dystrophy
- ▶ Inclusive Education for Children with Muscular Dystrophy and other Neuromuscular Conditions

You can order these for free from Muscular Dystrophy UK, or download them from the website.

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Contents

How do we deal with the diagnosis?	p5
How do we cope with the information?	p11
What does the diagnosis mean for our child's development?	p15
What about schools?	p19
What do we need to think about when caring for our child?	p25
What should we consider when planning our family's future?	p33
What about research?	p37
Useful resources.....	p42
How can we support the charity?	p47

How Muscular Dystrophy UK supports you

This booklet is for you, as parents and/or carers of a child recently diagnosed with Duchenne muscular dystrophy. The aim is to offer support and information to families at this difficult time. The booklet is in a question and answer format and includes the things most parents ask us.

We are here for you at the point of diagnosis and at every stage thereafter, and can:

- ▶ give you accurate and up-to-date information about Duchenne muscular dystrophy, and keep you updated about progress in research
- ▶ give you tips and advice about day-to-day life, written by people who know exactly what it's like to live with Duchenne muscular dystrophy
- ▶ put you in touch with other families living with Duchenne muscular dystrophy, who can share their experiences with you
- ▶ tell you about – and help you get – the services, equipment and support you're entitled to.



How do we deal with the diagnosis?

For many families, the diagnosis of Duchenne muscular dystrophy comes as a complete shock. Parents may have suspected something was wrong, however they rarely expect this diagnosis and seldom know anything about any muscle-wasting conditions. For others, there may be a known family history.

What happens now?

Depending on the age of your child at the time of diagnosis, there are a number of things you can do to assist him/her and there is more about this later in the booklet.

It is important to equip yourselves with information and identify local sources of support, and have a follow-up plan from the clinic where the diagnosis was made.

What is Duchenne muscular dystrophy?

Duchenne muscular dystrophy is a life-shortening muscle-wasting condition, caused by the lack of a vital muscle protein called dystrophin. The condition causes muscles to weaken and waste over time, leading to increasing and severe disability.

- ▶ Duchenne muscular dystrophy currently affects around 2,500 people in the UK and around 100 babies are born with the condition each year.
- ▶ Duchenne muscular dystrophy is caused by genetic mutations on the 'X' chromosome, meaning almost all of those affected are male. These mutations prevent the body from producing the vital protein, dystrophin, needed to build and repair muscle.

- ▶ Most children with Duchenne muscular dystrophy are diagnosed by the age of five. Most will use a wheelchair by the age of 12 and will face life-threatening health problems by their late teens as the muscles of the heart and lungs weaken. Few of those born with the condition currently live to see their 30th birthday, although with medical advances, life-expectancy is improving.
- ▶ The right specialist healthcare and equipment, including access to heart and respiratory experts and regular physiotherapy, can make a significant difference to both quality and length of life for people with Duchenne muscular dystrophy.
- ▶ While it is severely disabling, many of those living with Duchenne muscular dystrophy lead full and active lives. There are authors, designers, campaigners and Paralympians who have Duchenne muscular dystrophy.
- ▶ Promising potential treatments are in clinical trials, some of them at an advanced stage (see p37).

What shall we say to our child and when?

Coming to terms with the news that your child has Duchenne muscular dystrophy can be extraordinarily difficult. If the diagnosis is recent, you may find things generally feel overwhelming. You may be worried about what the future holds for your child and how you can help them to live as happy and independent a life as possible.

Many parents wish to hold off from telling their child about the diagnosis for as long as possible; they feel that by doing so they are protecting their child.

Contact the Muscular Dystrophy UK care and support team (**see contact details on p42**), who can support you with helpful information. They can send you a copy of *When your child has a muscle-wasting condition: A guide to talking with your child about their condition*. This booklet contains suggestions about why it might be useful to begin having these conversations early on with your child. You'll also find some practical advice on how you might go about doing so, addressing some worries and concerns you may have.

What shall we say to our other children?

Siblings are likely to have questions (and often worries) about their brother or sister too. It can be difficult to judge how much to tell siblings, particularly if there is a large age gap. Generally speaking, it is important to ensure the siblings do not know more about the condition than the affected child.

It is a big responsibility to expect a child to hold on to this sort of information.



“It is important to remember that siblings will also find it extremely tough to cope with their brother’s/sister’s condition. They may find it hard to separate their brother’s/sister’s personality from the condition and feel it is somehow their fault if they do not have it.

“Again, openly talking and listening to siblings’ concerns over a long period of time helps.

“Supporting siblings can be challenging, as parents may be accused of paying more attention to or loving the child with the condition more, but it is important to comfort them too.”

Manjula, whose son, Vivek, has Duchenne muscular dystrophy

What shall we say to other adults in the family?

Unless there is good reason not to, tell your relatives the truth. You will need their support and understanding in the years ahead.

As these conditions are usually genetic, other family members may like to be referred to their local clinical genetics department for advice. Be aware that other people may be frightened or upset by the news and may not react in the way you would expect.

You may feel that because of your ethnic or religious background, sharing information about the condition will raise particular issues. You can talk about these concerns, in confidence, with the team caring for your child and the relevant genetic service. They are used to dealing with such situations and will not pass on information about you or your child to other family members without your consent.

What if a female relative is planning to have children or is pregnant?

If a female relative is planning to have children, she can ask her GP to refer her to a genetic counsellor. If a female relative is pregnant, she can request an urgent referral. Consider making your child's relevant medical records available for the consultation. This is a common occurrence and staff are skilled at talking about the issues raised.

How shall we tell our friends?

What you say will very much depend on how close people are to you. If you have a partner, agree together on the people you will share information with and if you want to keep it confidential, ensure that these friends know it is given in confidence.

You may wish to give yourself time before sharing information outside your own close circle. Good friends are important but it can be difficult coping with other people's distress as well as your own.





How do we cope with the information?

How do we cope with the way we are feeling?

There is no right or wrong way to feel. You will probably feel different at different times. One minute (or day) you may feel able to cope and then much more fragile the next. Some parents have said they feel as if they are on a giant roller coaster, and the good days gradually start to outnumber the bad ones.

It is never easy coping with the unfamiliar. Most parents have a vision for their child and it is very challenging when this vision is changed by something beyond their control.

Some people find that talking to a counsellor can help. Ask your GP to arrange this if you think it would be useful. Asking for help is a sign of strength and we all need extra support sometimes.

How do we cope with our partner's feelings?

No two people are alike and you and your partner may cope with the situation differently. It is important to respect these differences.

It is hard to cope alone though, and it is often the case that one partner wants to talk while the other doesn't. Try to negotiate some agreed 'talking time' so that each partner's needs are at least partly met.

If one partner is out at work, the other may take on more of the responsibilities relating to the home and children, including attending clinics. Feeding back information from clinic appointments can be difficult. Understand that one partner may not have asked the same questions as the other. This difficulty can be partly overcome by

compiling a list of questions in advance but it really helps if both partners can attend appointments together. People's levels of acceptance about a situation vary. A trusted friend can be invaluable in offering support and professionals can also have a supportive role to play.

How do we cope with the feelings of others?

It can be very distressing to have a child diagnosed with Duchenne muscular dystrophy, and others will feel for you. When talking to them, remember they may not be ready for what you have to tell them, so try to prepare the ground a little. Speak to them at a time when you feel strong, and preferably in an environment where there are others to support you, so you're not having to cope with their emotions.

Avoid sharing the information when your children are around. You might think they are busy playing, but they often overhear more than you would expect.

People like to help so let them know how they can. Perhaps they can look after the child(ren) while you have some time to yourself, or simply just be there for you.

“As a mother, I found counselling very helpful. Being able to talk about my feelings to someone outside the family helped enormously through the toughest period when Vivek stopped walking.”

Manjula, whose son, Vivek, has Duchenne muscular dystrophy

Meeting other families living with Duchenne muscular dystrophy

Many people find it helpful to meet with other families who have a child affected by the same or a similar condition, but usually not straight away.

Perhaps someone at Muscular Dystrophy UK, or a neuromuscular care advisor or staff at the clinic your child attends, may be able to put you in touch with another family that has a child of a similar age to yours.

You may wish to contact them by email initially. This allows you to control the amount and timing of the contact, and you don't have to disclose personal details until you have established a relationship.

Most families with a child with Duchenne muscular dystrophy do meet other families at clinics, physiotherapy sessions or at events organised by Muscular Dystrophy UK and other support groups.

Other families can be a unique source of information as, unlike the professionals involved, they are experiencing (or have experienced) some of the same emotions as you are, and have faced similar practical challenges.

Muscular Dystrophy UK often brings together families who have children with Duchenne muscular dystrophy. Contact the care and support team at the charity's London office to find out more.



What does the diagnosis mean for our child's development?

What physical changes can we expect to see in our child?

Any physical changes in your child are likely to be subtle rather than sudden or dramatic. In very young children, as a result of normal development, you may initially see positive progress. However if your child is walking, it is common for him/her to tire more quickly when walking longer distances, to complain of pain in legs after exercise, to struggle with stairs or to fall more frequently. A child may struggle more at the end of the day, particularly if they have been busy with activities such as swimming, for example.

For some children, weakening of the arms or hands may occur. Writing for long periods, or taking clothing off or putting it on over the head may become more difficult.

If your child is not able to walk at all, changes may take place in their posture or joint position, owing to weakness in some muscle groups.

It is good to gently encourage your child to be as active as possible, but try not to deny that there is a difficulty. Your child needs to know that you understand that some activities aren't easy. Try to balance periods of activity with periods of quieter play to avoid exhaustion.

The Muscular Dystrophy UK care and support team (**see contact details on p42**) can send you a copy of *The diagnosis and management of Duchenne muscular dystrophy: A guide for families*. Discuss this with your care advisor too.

Will our child be in pain as a result of having Duchenne muscular dystrophy?

Most muscle-wasting conditions themselves do not cause pain, however some children may be troubled by muscle cramps or joint pains. Advice and regular monitoring by a physiotherapist will be beneficial for most children. They are likely to provide a programme of stretches and exercises to maintain a good range of movement in the joints, which will reduce discomfort. Cramps can be an indication that your child needs to rest. Gentle massage and warmth can help relieve cramps.

Will our child develop eyesight, hearing or speech difficulties?

Duchenne muscular dystrophy does not cause difficulties with eyesight or hearing. Weakness of facial muscles, however, which may affect speech, chewing and the ability to swallow, are late symptoms and are not usually a problem in childhood.



Will our child become incontinent?

This is very unlikely. Sensation usually remains normal and children will be aware of when they need to use the toilet. Of course, accidents can happen if a child delays going to the toilet and is a little slow to get there. If your child does have difficulties, tell your consultant, care advisor or GP, as they will suggest adjustments if necessary.

Constipation can be a problem in some children, particularly if they are not physically active. It can usually be managed by adjustments to the diet. Seek advice from your GP, your child's consultant or specialist nurse.

Will our child have learning difficulties?

Some children with Duchenne muscular dystrophy may also have learning and/or behavioural difficulties, which may come to light as the child gets older. Some children with Duchenne muscular dystrophy may have learning difficulties, such as attention deficit and hyperactivity disorders, obsessive compulsive disorders as well as dyslexia. These can arise from the effect Duchenne muscular dystrophy has on the brain, however, unlike muscle weakness these difficulties will not get worse over time. Family support is essential and specialists may be needed to address specific issues of learning and behaviour.

Ensure your child is properly assessed by an educational psychologist, and discuss whether or not your child needs an Education Health and Care Plan (EHCP) or, in Scotland, a Co-ordinated Support Plan.



What about schools?

Some children are already at school when they are diagnosed with Duchenne muscular dystrophy, while others may not yet be at nursery school age.

A diagnosis of Duchenne muscular dystrophy does not mean your child cannot attend a mainstream school. This will depend on a variety of factors, including your preference and the inclusiveness of the school. Most children with Duchenne muscular dystrophy are able to attend a local primary school along with their friends.

When selecting a school for your child, it is important to explain to the Head Teacher that your child has Duchenne muscular dystrophy. Knowing this will be helpful for the teaching staff, enabling them to plan appropriately for your child. Every school has a Special Educational Needs Co-ordinator (SENco), who will need to be involved to ensure your child's needs are met in school.

The clinic staff or therapists who work with your child could also talk to school staff about your child's condition and how it will affect them while at school. This may be the case for nursery schools too.

It is important you raise any concerns you have about access. The school you choose needs to be right for your child throughout their time there. Remember, their condition may change with time. Local Education Authorities (LEAs), or Education Authorities (EAs) in Scotland, can usually make minor adaptations to buildings. They are not legally required to make every school in the area fully accessible.



Staff at your preferred school should seek expert advice from the LEA/EA if they have any concerns.

The LEA/EA should assess any additional needs your child may have for learning or physical management. They may issue an Education Health and Care Plan (EHCP) or, in Scotland, a Co-ordinated Support Plan (a Statement of Special Educational Needs).

Most parents of young children don't want their child to be treated any differently from other children, and most schools try to respect these wishes. Allowances will sometimes have to be made, however, to ensure your child's wellbeing. Agree with teachers what these will be and review them on a regular basis.

Other children in the class may ask about your child's special needs. Discuss with teachers, and your child, what explanation should be given. You may want someone to explain to the class that your child has a medical condition that causes their muscles to weaken and waste, and makes certain activities difficult or impossible. Let the class know how they can be supportive by, for example, holding doors open. Only do this with your child's consent.

A few children with Duchenne muscular dystrophy may prefer to be in a special school environment, particularly if they have a significant learning difficulty. Classes tend to be smaller, and physiotherapy and other therapy services will probably be available on site. Some parents also find a special school environment more supportive.

Muscular Dystrophy UK publishes a range of factsheets on education as well as a comprehensive guide for schools entitled *Inclusive Education for Children with Muscular Dystrophy and other Neuromuscular Conditions*.

Will our child get upset or angry? How will he/she cope?

You may be surprised at how well young children cope with their diagnosis. However, as the condition progresses and changes occur, they will have to adapt. Change is never easy and children may express their anger or frustration by displaying challenging behaviours.

Try to keep life as 'normal' as possible, and don't treat your child with Duchenne muscular dystrophy differently from your other children. Have clear guidelines about what is acceptable behaviour and what is not.

It may be appropriate to give your child more information about the condition to help them make sense of the changes taking place. Try to involve your child in decision-making and give them the space to develop friendships. When you talk to your child, ensure you really listen, otherwise you could assume he/she shares the same anxieties you feel. They are more likely to be concerned with today, rather than tomorrow (or next year). Avoid being 'over-protective'; as any children do, he/she will need some adult-free time!

If your child is being teased or bullied, seek advice from school staff and work with them. With your child's permission, consider telling classmates about the situation. This is often the best way to gain support and respect. It can be very helpful to get a professional to do this, for example the Care Advisor or Clinical Nurse Specialist. Some children like to do this themselves, with their teacher supervising.

Some children may benefit from talking to a counsellor. Art or music therapy can also be helpful and fun. Be aware that relatives and friends may treat your child differently – this can create tensions within the family and between siblings, so be clear about your wishes in this area.





What about puberty and sexual issues?

Children with Duchenne muscular dystrophy experience puberty in the same way as others their age do. As they get older, they will require an increasing amount of physical care, and extra thought needs to be given to ways of maintaining privacy and dignity.

Your child will need opportunities to learn about sexual issues and form relationships with others, as any other child their age would. Being physically dependent on others should not prevent your child increasing their independence in thought and actions.



What do we need to think about when caring for our child?

A multi-disciplinary approach, with the input of specialists such as physiotherapists and occupational therapists, is the best way to manage Duchenne muscular dystrophy.

Having access to a multi-disciplinary team is vital to ensure someone with Duchenne muscular dystrophy receives a holistic approach to their care. This means that in a single visit to your specialist neuromuscular centre, you can get important input from each health professional involved in your care. This includes respiratory, cardiac and physiotherapy professionals who are able to provide better support when working within a multi-disciplinary team.

Watch our video about this approach:
www.musculardystrophyuk.org/mdt

Do we need to keep attending specialist appointments? Do we also need to attend local paediatric services?

Duchenne muscular dystrophy is a very rare condition. As most GPs may not see more than one or two patients with the condition in their entire career, it is helpful to see professionals who have experience of Duchenne muscular dystrophy. They usually work at specialist clinics and, as experts in this field, they will discuss with you the right issues at the right times and keep you up-to-date with any new developments, research and clinical trials. Specialist clinics also have established links with other services that may be of help to your child.

The relationship between a family affected by Duchenne muscular dystrophy and the specialist team that supports you, is an important one. A strong relationship, based on good communication, helps to create a supportive environment and if there's ever a difficulty, you and your child will be well-known to the team. The relationship is also a partnership, so feel free to question why an appointment is necessary, or ask for less frequent appointments if you feel this is appropriate. Different families appreciate different levels of support, and the team will be keen to get it right for you. Let them know your preferences.

Unless your specialist centre is very near to you and has a community remit, you will also need support from local paediatricians. They can help you to get local services such as physiotherapy, and they also contribute to a child's Education Health and Care Plan (EHCP) or, in Scotland, a Co-ordinated Support Plan.



Local paediatricians will play a role in your child's care should he/she become unwell or need to be admitted to a local hospital. They work with the specialist team, GP and school health services to champion your child's needs. Try, if possible, to space out the visits between the specialist centre and your local centre.

Why has our child been referred to a cardiologist?

As your child gets older, he/she will need additional support from doctors specialising in the care of joints and the spine (orthopaedic specialists), and breathing (respiratory specialists). A cardiologist will be involved in assessing the heart muscle function, and providing treatment if any weakness is found. In some cases, other specialists may need to be consulted.

How much physical activity and exercise should my child do?

Keep your child as active as possible and try to make the activities fun. There is no need for anything excessive; normal play is fine. Allow your child to join in with PE and games. They will judge for themselves if an activity is too challenging. Your child's local physiotherapist can visit the school to advise on appropriate activities, including PE.

If appropriate, encourage walking, however it is important to recognise that your child may tire more quickly than their friends, and you'll need to make allowances for this. Activities such as walking up hills, and/or climbing stairs, may be particularly difficult. If your child can manage them, swimming, cycling and horse-riding are excellent physical activities.

Liaise with your child's school about playground safety, and the amount of activity your child can manage. Review this regularly.

What if our child falls over and breaks a leg?

It would be unwise to restrict your child's activity because of fear of a broken bone, however the risks do need to be assessed. If your child does break a bone, ensure that staff at the hospital get in touch with staff at the specialist muscle clinic. Contact the relevant professionals yourself too. Where possible, try to avoid having limbs immobilised (particularly in plaster casts) for long periods of time, as once muscle strength is lost, it is not easy to regain.

Alert cards and neuromuscular care plans

Muscular Dystrophy UK has produced an alert card for people with Duchenne muscular dystrophy.

Designed to fit easily into a wallet, the card outlines information about the condition, as well as key recommendations and precautions for consideration during medical emergencies.

Working alongside a range of specialist health professionals, Muscular Dystrophy UK has also developed a care plan that you can complete along with your neuromuscular clinicians. This care plan can then be shown to any health professional to develop a tailored approach to your child's healthcare.

Order your alert card and care plan for your child by emailing **info@muscular dystrophyuk.org** or calling our **Freephone helpline** on **0800 652 6352**.

Should our child follow a special diet?

There is no evidence that a child with Duchenne muscular dystrophy requires a special diet. Some children do experience difficulty with feeding and gaining weight. If this is the case, your child is likely to be referred to a speech and language therapist (SALT) and/or a dietician.

If there are no feeding difficulties, aim to establish a healthy eating pattern, which maintains weight within a normal range. Excess weight makes it harder for a child with weak muscles to move. It also makes any moving and handling assistance more risky.

If your child becomes less physically active, they will require a lower calorie intake.

Muscular Dystrophy UK has a factsheet, entitled *Healthy eating for children with neuromuscular conditions*, which you may find helpful.

What are night splints?

As the name suggests, these splints are worn at night. When a joint cannot move through its full range, because of tightened muscles, this is known as a contracture. Night splints help to delay contractures because they hold the joint in a good position during the night. Splints are made from a variety of lightweight materials.

A child should, preferably, wear night splints throughout the night but some children find this uncomfortable. It may be necessary to find a compromise, for example, wearing the splints on week nights only, or for just half the night. Some children find it easier to wear just one splint at a time. If it becomes very difficult, try persuading your child to wear them in the evenings while watching television.

Night splints can be uncomfortable in hot weather and it may help if your child wears a cotton layer underneath them. Sometimes the orthotist (someone who specialises in assessing for and supplying orthoses such as supportive footwear or splints) will make air holes in the splint, which can help. It can also be difficult to turn over in bed, when wearing splints. If night splints are uncomfortable, or your child grows out of them, contact your physiotherapist or orthotist.

Should our child have physiotherapy?

The team at your child's muscle clinic will usually make a referral to the local physiotherapy service. If this does not happen, inform the muscle clinic staff or ask your GP for help.

Most children with muscle-wasting conditions benefit from regular physiotherapy reviews. A programme of regular exercises and stretching is likely to be suggested.

Try to make these fun and part of the daily routine.



Are steroids helpful? What about side-effects?

Long-term treatment with steroids will usually be considered before there is significant deterioration in muscle strength. For many children, such treatment can help them to carry on being able to walk for longer than would otherwise be the case. There are, however, significant side-effects, so it is important for parents and clinicians to carefully weigh up the advantages and disadvantages of steroid treatment for each child before deciding whether or not to embark on it. Muscular Dystrophy UK's factsheets on Duchenne muscular dystrophy give further helpful information.

Can alternative therapies help?

There is no evidence of alternative therapies proving helpful. Some non-invasive therapies, such as massage, may be pleasant and relaxing, but do seek guidance from your specialist before undertaking any. Muscular Dystrophy UK has published a factsheet – *Alternative therapies* – that you may find useful to read.

Speak to your GP if you're unsure about any of these therapies for your child.





What should we consider when planning for our family's future?

How can we afford all the things our child will need?

Having a child with a disability can put pressure on a family's finances. Be clear about what the statutory authorities should provide. Some essential equipment for using around the house, such as banister rails and bath aids, is provided free of charge by social services. Manual and powered wheelchairs are available through Wheelchair Services, which is part of the NHS. The LEA/EA can provide computers for use at school. Ask, and ask in good time, as the process of obtaining equipment can be slow.

The Joseph Patrick Trust (part of Muscular Dystrophy UK) and other charities can assist with grants towards the cost of equipment. The Family Fund also regularly helps low-income families who have a child with Duchenne muscular dystrophy.

With regard to benefits, seek advice from a welfare rights centre or Citizens Advice Bureau; doctors are not experts in benefit issues. Benefits for families on Income Support can be affected by accruing large amounts of Disability Living Allowance (DLA) savings. You may need supportive letters from medical professionals to support applications for benefits.

Muscular Dystrophy UK's information and advocacy team (**see contact details on p42**) can help you with these applications, if you are having any difficulties. They also have leaflets and factsheets explaining welfare benefits, and what you are entitled to.



Will we need to give up work to care for our child?

Many children with Duchenne muscular dystrophy are in families where their parents work. There are no rights or wrongs in this situation. Much depends on the nature of your work, the flexibility of your employer and how much support you have from friends, family and/or paid carers. Every family is different. If you enjoy your job, it may be right for you to continue working.

However, children with Duchenne muscular dystrophy often require more support from their parents than other children their age. They may need help with practical tasks and have a range of appointments to attend, which may increase in frequency as they get older. Appointments are generally during working hours, and it can be difficult to take time off from work to attend them.

Get up-to-date advice on benefits and tax credits. Don't assume you are not entitled to help because you are working or have income/savings at a particular level. Not all benefits are income-related. A parent who gives up work to care for a disabled child can be entitled to financial help.

Will we need to move? Will we need a bungalow?

Many families worry that they will have to move house. Seek expert advice and don't rush into any decisions. Children with Duchenne muscular dystrophy will eventually need their own bedroom and a suitably-equipped bathroom.

When they become wheelchair users, you will need to consider how they will get in and out of your property and how they will access communal rooms. Most children are diagnosed many years before these issues become pressing, so there is time to make plans accordingly.

Housing issues can take a long time to sort out. If major alterations are likely to be needed, council and housing association tenants should advise their landlords at an early stage of their child's diagnosis.

Tenants of private landlords should seek advice from an occupational therapist and the housing department, as it can be difficult to make adaptations to privately-rented properties.

An occupational therapist will help you by assessing your current and future housing needs. You can generally refer yourself to the occupational therapy department (usually based at the local social services).

Both owner/occupiers and tenants need to obtain advice on the funding of adaptations, as financial help cannot be given retrospectively. Muscular Dystrophy UK's care and support team can advise you. The charity's *Adaptations Manual* is a comprehensive guide, identifying the appropriate adaptations for your situation.



What about research?

Is research taking place that could lead to a treatment or cure for duchenne?

Muscular Dystrophy UK invests about £1.5m every year into research, and funds a number of research projects into Duchenne muscular dystrophy. We are part of a worldwide research effort to find effective treatments and ultimately cures for Duchenne muscular dystrophy.

We have laid the foundations for the first potential treatments for Duchenne muscular dystrophy, some of which are currently in clinical trials. Many scientists say research is at the most exciting stage they have known.

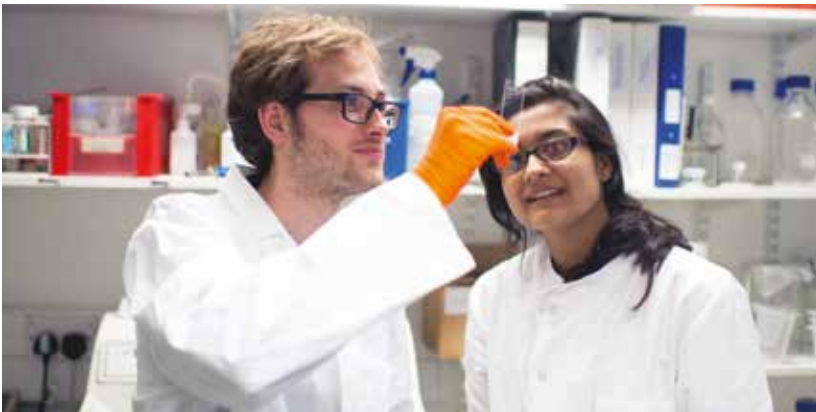
Since the charity was established in 1959, we've invested more than £55m in high-quality research into muscle-wasting conditions. With that solid basis, we have laid the foundations for the first potential treatments for some muscle-wasting conditions and contributed to some crucial scientific breakthroughs. Here are some examples.

- ▶ We funded research into exon skipping technology for the treatment of Duchenne muscular dystrophy, which is currently in clinical trial. We are currently funding research that explores how exon skipping technology might be used for other muscle-wasting conditions too.
- ▶ We supported utrophin modulation research for the treatment of all types of Duchenne and Becker muscular dystrophies for many years. Utrophin modulation-based therapies are at a very exciting stage of development, with the first utrophin up-regulating compound being tested in clinical trial.

- ▶ We played a crucial role in developing a gene therapy approach based on the use of harmless viruses called Adeno-Associated Virus (AAV) to deliver a miniature but still functional version of the dystrophin gene to the muscles for the potential treatment of Duchenne muscular dystrophy.

Other drugs are beginning to become available for Duchenne muscular dystrophy, including Translarna (ataluren), which is currently available in some European countries to slow down the progression of symptoms in children with Duchenne muscular dystrophy. The drug works for only a small group of children who carry a particular mutation in the dystrophin gene ('nonsense' mutation – where a single letter change in the DNA code results in a premature stop codon). Your clinician will be able to tell you whether or not your son could benefit from this medicine. Other drugs targeting specific mutations may be approved in the coming years.

Intense research is continuing, in trying to find treatments for Duchenne muscular dystrophy. Some medicines are currently being tested in clinical trials.



It can also be useful to ask for a copy of the genetic report (with the type and the location of the mutation in the dystrophin gene identified in your child). This will help in understanding which medicine and trials might be suitable for your child.

The Duchenne Muscular Dystrophy Registry provides updated information on ongoing clinical trials for Duchenne muscular dystrophy and can help identify which children are potentially eligible for specific clinical studies. Your clinicians will be able to tell you how to register your child on this Registry.





The North Star Adult Network, made up of neuromuscular expert consultants, allied health professionals, individuals living with Duchenne muscular dystrophy, and Muscular Dystrophy UK, is working together to improve the standards of care and support available to adults across the UK. This network mirrors the paediatric version – the North Star Project – which works to optimise the care of children with Duchenne muscular dystrophy.

You can find out more about these on our website **www.muscular dystrophyuk/research**, as well as through our research news and regular updates. Register with the charity at **info@muscular dystrophyuk.org** to ensure you get these research updates from us.

Research has also helped improve our understanding of how best to manage the condition, and this has had a positive impact on the clinical care of many children.

Your consultant can advise you further on this.



Useful resources

Muscular Dystrophy UK

The friendly staff in the **care and support team** at Muscular Dystrophy UK's London office are available to offer free information and emotional support. If they can't help you, they can signpost you to specialist services close to you, or to other people who can help.

61A Great Suffolk Street, London SE1 0BU

0800 652 6352 / info@musculardystrophyuk.org

(Monday to Friday 8.30am to 6pm)

www.musculardystrophyuk.org

Joseph Patrick Trust (JPT)

Muscular Dystrophy UK's welfare fund, the JPT provides grants towards the cost of specialist equipment for children and adults.

61A Great Suffolk Street, London SE1 0BU

020 7803 4814

JPTgrants@musculardystrophyuk.org

www.musculardystrophyuk.org/jpt

Duchenne Family Support Group (DFSG)

Families supporting families affected by Duchenne muscular dystrophy.

78 York Street, London W1H 1DP

Office: 0870 241 1857

Helpline: 0800 121 4518

(an answering service in place outside operational hours)

www.dfsg.org.uk

Family Fund

The Family Fund provides financial help, through one-off grants, for families raising a disabled or seriously ill child.

4 Alpha Court, Monks Cross Drive, York YO32 9WN

01904 621115

www.familyfund.org.uk

Carers UK

Carers UK give expert advice, information and support to carers.

20 Great Dover Street, London SE1 4LX

020 7378 4999

**The Carers UK Adviceline – 0808 808 7777 or email
advice@carersuk.org – Monday to Friday(10am – 4pm)**

www.carersuk.org

Disabled Living Foundation (DLF)

DLF is a national charity providing impartial advice, information and training on independent living.

**4th Floor Jessica House, 191 Wandsworth High Street,
London SW18 4LS**

**Helpline: 0300 999 0004 (helpline@dlf.org.uk) – Monday
to Friday (10am – 4pm)**

www.dlf.org.uk

Muscular Dystrophy UK

– here for everyone with Duchenne muscular dystrophy

We're committed to improving the quality of life of people living with Duchenne muscular dystrophy. We campaign to make sure treatments are available as soon as possible, and that everyone gets the healthcare, benefits and equipment they need and are entitled to.

If you'd like to get involved and make a positive difference, here's what you can do:

- ▶ campaign with us –
in your local area, in parliament, online
- ▶ meet other families at a Muscle Group near you
- ▶ when your child is a little older, he/she may want to join Trailblazers – our growing network of young disabled people who fight social injustices and campaign on issues that are important to them
- ▶ fundraise to support our work (**see p47**)
- ▶ help to raise awareness to ensure the fight to beat Duchenne muscular dystrophy gets the exposure and support it deserves. If you'd like to become a media volunteer, please contact our press team at press@musculardystrophyuk.org

Research

Visit our website to keep up-to-date with the latest news on research topics relating to Duchenne muscular dystrophy. You can also find out information about patient registries and clinical trials, including those relating to Duchenne muscular dystrophy.

Information resources

Muscular Dystrophy UK has lots of helpful resources for you and your child. All of our medical information resources are accredited by the Department of Health's Information Standard, which means they are clear, accurate, impartial, evidence-based and up-to-date:

Our *Duchenne muscular dystrophy factsheet* gives a detailed overview of the condition.

In case of an emergency: our Duchenne muscular dystrophy alert card outlines the vital and specific issues emergency healthcare professionals need to know.

Specialist care: our GP and physiotherapist training modules are designed for healthcare professionals who provide care to people living with muscle-wasting conditions like Duchenne muscular dystrophy.

The North Star Project for children: aims to optimise the care of young patients with Duchenne muscular dystrophy.

The North Star Adult Network: is working to improve the standards of care and support available to adults.

Diagnosis and management of Duchenne muscular dystrophy: this published research paper outlines care guidelines which are the result of an international consensus on the medical care of Duchenne muscular dystrophy.

To find out more, contact us at:
0800 652 6352 (Mon-Fri, 8.30am-6pm)
info@muscular dystrophyuk.org





How can we help beat Duchenne muscular dystrophy?

There are lots of ways to support Muscular Dystrophy UK's work to beat Duchenne muscular dystrophy. If you'd like to fundraise to accelerate the pace of research, or to support our care and information services, please get in touch with us.

You may want to start a Family Fund, which is a great way to fundraise for us as a family or group. We look after the admin and paperwork, and you can direct your funds to the areas that most interest you, for example, the Duchenne Research Breakthrough Fund. Please contact our Regional Development team to find out more.

You may have contacts through your work, family or friends that could support our work. For example, Muscular Dystrophy UK was Tesco's Charity of the Year. The partnership raised over £1 million, and all because the mother of a boy with Duchenne muscular dystrophy worked for the supermarket chain and invited her employers to support the charity.

We hope this booklet has addressed some of your queries and has helped to reassure you. Much more material is available from Muscular Dystrophy UK when you are ready to read it.

Muscular Dystrophy UK is the charity bringing individuals, families and professionals together to beat muscle-wasting conditions. We're providing a range of services and opportunities to help people live as independently as possible.

We're here for you at the point of diagnosis and at every stage thereafter, and can:

- ▶ **give you accurate and up-to-date information about your child's muscle-wasting condition, and let you know of progress in research**
- ▶ **give you tips and advice about day-to-day life, written by people who know exactly what it's like to live with a muscle-wasting condition**
- ▶ **put you in touch with other parents living with the same condition, who can tell you about their experiences**
- ▶ **tell you about the services, equipment and support you're entitled to.**

0800 652 6352
info@muscular dystrophyuk.org
@MDUK_News

www.muscular dystrophyuk.org

Muscular Dystrophy UK, 61A Great Suffolk Street, London SE1 0BU

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