What a Duchenne muscular dystrophy treatment would mean for my family

Duchenne families
February 2015
About Duchenne muscular dystrophy

Duchenne muscular dystrophy is a severe muscle-wasting condition caused by the lack of a muscle protein called dystrophin. Around 2,500 people in the UK are affected by Duchenne muscular dystrophy. The condition mainly, but not exclusively, affects boys and young men, with approximately 100 diagnosed every year. This extremely serious condition causes progressive muscle weakness leading to dramatic loss of muscle function. Average life expectancy is in the late twenties. However, thanks to improvements in care, some people with the condition are now living well into their thirties and even forties.

The search for a treatment

There are currently no effective treatments for Duchenne muscular dystrophy available in the UK. However, a number of promising potential treatments for the condition are in clinical trials.

The Muscular Dystrophy Campaign is urgently campaigning with families for fast access to these potential treatments. This includes pushing for streamlined clinical trials, a ring fenced fund for rare disease drugs and faster processes so that decisions on whether to approve a drug can be taken quickly.

The first of these emerging treatments is Translarna, which was licensed for use with eligible boys in Europe when a conditional approval was granted in August 2014. Translarna is licensed to treat boys whose Duchenne is caused by a nonsense mutation, who are aged five and over and who are still walking.

This was a landmark decision: Translarna is the first ever drug to treat an underlying genetic cause of Duchenne. Clinical trials indicate that it could help improve children’s walking ability and keep them on their feet for longer.

However, despite now being available in France, Spain, Italy and Germany, Translarna has not been approved for use in this country. NHS England have delayed their decision, and NICE is not yet due to begin an assessment of the drug.

Duchenne progresses rapidly, and those affected simply do not have the time to wait, as the families’ testimonies demonstrate with such heart-rending clarity.

#everydaycounts
Between January and February 2015, the Muscular Dystrophy Campaign asked Duchenne families to tell NICE and the Health Minister what a treatment for the condition would mean to them.

Here’s what they said:

**Laura Smith:** This is my son Callum. He would benefit from Translarna. It needs to be available. No more delays, no more waiting. #teamcallum

![Callum](image1.jpg)

**Catherine Rimmington Wilson:** This is my little boy, Johnny, who has DMD. Heartbreakingly he is not suitable for Translarna and we are just desperate for a new medicine to treat this awful condition. It is our dearest wish that a cure/better treatment be found soon and this terrible weight of anguish lifted from our shoulders...

![Johnny](image2.jpg)

**Heather Bilbrough:** This is our son Jack, aged 10 he has duchenne, translarna would mean everything! Everyday our lives are shadowed by dark clouds this drug is our ray of sunshine through those dark clouds! Our boys (and girls) need this like we all need air, it's vital!!

![Jack](image3.jpg)
Stephen O’Brien: My son Bobby (2) has DMD. He is my everything. I can't imagine losing him. He’s the bravest lad I know. His smile lights up a room. Please please please find a cure soon.

Shelley Simmonds: This is my 22 month old son Fraser. I don't want him to die - without a treatment he will. Please save my son, his life is in your hands!

Elaine Thompson: This is us and my son keir who has DMD. ..We had just completed the 5 k run or dye marathon in London docklands..as you can see it get messy ....I'm not sure if keir would benefit from translarna but it should be available to everyone that needs it...I too prey every day for a cure or for treatment to help this wicked disease. ..my heart breaks every second of every day watching him go through this please please please find some help somewhere xx

Shanine Large: This is my son Leighton, he has dmd, translarna would help him so much it should be available for these children! X
Koral Louise Clarke: This is Ieuan who is six he also has dmd please don't stop him from doing what he loves and give them the life they deserve. Stop wasting time these boys don't have. !!!!!!

Lisa Niblett: My two babies that both suffer from duchenne, I pray for a cure if not a treatment daily. I wish there was more we can do. It's bad enough to watch one go thru it let alone 2.

Annie Johnston: This is my son harry age 6 who has duchenne. He is our world and i couldn't imagine him not in my life he means everything to and his family. I pray everyday for a treatment to come out to save our little solider a better life which he deserves please please please help my son and all the other children suffering with this horrible illness

Nikki McMullan Baker: This is my baby brother he has Duchenne. It would mean the world to me if he could have some what of a normal life. Please we need the Translama!
Sarah Powell: This is Eloise at an RDA riding competition in which she won 3rd place! We were so proud of her 💖 Eloise is 10 and is still mobile most of the time. She is one of a minority of little girls affected by Duchenne and we hope and pray these medicines are made available to all the boys and girls that live with this cruel disease every day.

Alissa Ellis: This is our amazing Arthur (7) who has DMD. Everyday is a struggle for him and it is heartbreaking to witness. A treatment for him would literally change his world. We need some hope for the future because at the moment we are all helpless and running out of time. PLEASE PLEASE help fund treatments NOW!

Rob and Laura Newman: My son Stanley is 2, he doesn't have DMD, he has CMD, (Muscular Dystrophy from birth). He will never experience the harrowing loss of mobility because he's never had it. Stanley's CMD is also progressive. A treatment for Duchenne is essential & will lead to treatments for other muscle wasting conditions. Time is a luxury these boys don't have.

Emma Wilkinson: I don't think I can say anything that's not on here already by the hundreds of parents that have a child suffering this disgusting condition! We are all desperate for help and hope. This is my son Joel he is 3years old and has DMD. I would give anything to spare him and us the future we have in store. Translarna won't help Joel but the next treatment might! Please don't wait action is required immediately if our sons are to have any hope.
Rachel Mackenzie: It would mean the world to my husband and I and family as my son is only 3 and is the 4th one to have muscular dystrophy in our family the youngest is my nephew who two and he is the 5th person. It would mean everything to my nana as she blames her self for starting the gene. I would love this to be here xx

Louisa Hill: It would mean everything...A dream come true for all the families. #Action4Archie

Katrina Ruthven: This is my son Alexis age 6, he has DMD. We need a treatment for all our boys with Duchenne, not just those who could benefit from Translarna. It breaks my heart to think of what he will have to suffer unless a treatment is found.

Joanne Scott-Fernandes: This is my amazing son Luca who is 5 years old and has no idea what the future holds - I'm begging NHS UK to let him have Translarna so I don't have to break his heart by explaining to him how he's going to lose his ability to walk, run, dance, ride his bike.....and eventually this awful condition will stop his heart. Please do this for us and all the boys who have DMD. It not their fault, they didn't ask for this- they are completely innocent. They need your help now, not in months or years time.
Pedro Fernandes: This is my son Luca aged 5 he has DMD and would benefit from translarna. TIME IS LIFE.

Myra Naughton: These are my Three WONDERFUL BRAVE NEPHEWS we need a treatment, ideally a cure that will benefit EVERY child/young adult with DMD REGARDLESS of the genetic mutation. We Love them more that words can say esp their cousins. PLEASE PLEASE find a cure for them and all the other children with DMD

Clare Large: This is nathan who has dmd let's give Nathan and all sufferers a chance at a better life, time to do what's right and do it now because everyday we are losing boys to this cruel disease, we need action now!

Manoj Thakrar: Our 3 year old son, Shiv, was diagnosed with Duchenne Muscular Dystrophy in April 2014. Although Translarna will not benefit him, we as a community together must fight for our boys to get access to potential treatments as soon as they are approved. Time is NOT on our side. Every day that goes by, our boys become weaker. An effective Duchenne treatment would mean our boys can walk longer, move longer, breathe longer and LIVE LONGER! Let this not just be a dream but a reality! #smilewithshiv
Mandy Nyree Edwards: This is Daniel, he'll be six..
Every day the clock it ticks..
One more birthday one less year.. We wait for cures to 
appear.. Fundraise just another pound.. One year closer to the 
ground.. We saw his first steps and his first smile.. We dread the 
last and time goes by.. Please just listen.. Understand you aren't there to 
hold his hand.. You won't hear his last breath.. One more 
appointment.. One more test.. We simply do things for the best.. if 
you can help please can I suggest.. if not for Daniel then for the 
rest.. all our boys deserve to stand.. To walk until they are a man!!
Xx please help xx

Linda Pinkney: My gorgeous grandson Keir had DMD. I pray these treatments are available soon, 
while he is still mobile. Mr Freeman please try 
putting yourself in our shoes and having to watch 
our children deteriorate for want of a helping hand 
from the health department. Meet these wonderful 
children and their desperate families ASAP please.

Lesley Wegg: This is Ashley Wegg with his big Sister Melissa. 
Ashley is 7 and still walking. We, like many parents, have 
decided to give Ashley steroids with the hope that these will 
keep him mobile, but dread the possible side effects kicking in. 
Currently this is the only treatment available. Ashley sleeps in 
rigid plastic boots all night, every night to help his leg muscles. 
It breaks my heart every bed time to put these on him. We 
need the delays with potential new treatments and therapies to 
end NOW.
Our boys just do not have the time to wait for decisions that are 
being postponed.

Some boys that could be suitable for Translarna this month may not be in the future due to 
the monster that is Duchenne Muscular Dystrophy. Please understand that our Son's lives 
are at stake here.

Whilst this drug is not suitable for Ashley, allowing access will help pave the way for variants 
of this treatment. This drug is available in other countries, we need to catch up NHS 
England.
Sharon Morgan: This is John who will be 16 in March who also has DMD. John can't do a lot for himself no more and relies on us for everything. If this is a way of helping any child with this disgusting disease then why haven't you done it yet. I mean it's a life of hell for these children we don't know how long John has got and he will never get the help. Some days he's in so much pain but John laughs it off. It breaks my heart to know there is help out there so come on no more waiting, these children deserve to live a better life after all.

This is my son Lewis Powell who has Duchenne every day is a blessing to have him with us and well a treatment would be amazing and life changing for us and other family's of boys with Duchene and hopefully also all drugs trials can become more local to make it easier for family's travel.

Jaspal Mann: This is my son Kirath. He is 5 years old and would benefit from Translarna. It's the only hope we have. Please don't take this away NHS England.

Kirstie Hesketh: This is my family. My little super hero Finley aged 5 almost 6 has Duchenne. He was diagnosed on the 13th May 2014. We still do not know if a Translarna will help Finley but that is not the point. It will slow down the disease for some boys and give a glimmer of hope for the rest and that is vital. These boys do not have time to wait.
Lisa Hair: This is my son Aaron a week after a kidney transplant. We feel that we are so lucky to have a now happy and healthy son thanks to our NHS. Please please allow these parents to have that same feeling. These boys need your help now x

Gemma Jones: This is my son Andrew on his 3rd birthday two days ago. A treatment for duchenne would mean the world to us. Please help us to find a treatment. Thank you

Ruth le Gal: Translarna is not a cure for Duchenne, but it is a very effective treatment that could dramatically slow down the deterioration caused by the disease. In the same way, although it is not a cure, Insulin is a very effective treatment for Diabetes. It would be unthinkable to deny children with Diabetes access to insulin, and it would be unthinkable to deny children with Duchenne access to Translarna.

Janet Edwards: My nephew Dean who we lost to DMD at the age of 19, it is awful to think that 18 years later families are still losing their boys to this cruel disease - treatment must be made available!!!
**Georgina Olsen:** This is our little family. In March 2013 we had the diagnosis that Connor (now 5 years old) has Duchenne, we'd not heard of this vile, disgusting and deadly disease before. We had IVF treatment to get both our boys now this disease will take one. When we found out the diagnosis we were told there was nothing we could do apart from go home and love him. We could have driven off a cliff. Absolutely devestating to find out your child will totally waste away before your eyes and there is nothing you can do to stop it. He will loose every ability he has ever gained, even simple things we all take for granted like being unable to scratch his own nose, roll over in bed, everything!! Have you ever laid in bed unable to move? Try it!! Its really not good. We hate what outcome our life has instore for us all. It's not good enough. You can now do something about it, so please do it, save lives. Imagine if this was your child or grandchild.

**Karen Huyton:** Sadly our boy loss his brave fight to DMD on the 1st of june 2011 aged 25.our world truely fell apart.would love to see this happen...

**Batul Savliwala:** A prayer for these beautiful children and I pray they get the treatment they deserve. I lost my cousin in 2001 when we knew so little about DMD... So many years later we still losing so many awesome boys to DMD... Unfair... Please help...

**Stephanie Cornwell Carol:** This was my brother Bobby. He lost his fight with DMD in 2004. No child should have to live with the injustices of this disease. A treatment would mean a life for all of these beautiful children!
**Donna Mitchelson:** Our Logan is 10 and such a happy boy. There are no words to describe how families living with DMD have to deal with on a daily basis. The pain of watching your child lose the ability to do things he was once able to do. How we all hope for a treatment or a cure and that is what we cling to. It is the last thing we think of at night and the first thing we think of in the morning. We need people in power to see these children, by putting themselves in their shoes and give our families the chance of hope that these boys deserve.

**Michelle McDonough:** Its not just about the boys!! This is my daughter Tilly she is 7 years old and has duchenne it is considered a boys disease but there is more and more girls being diagnosed as manifesting carriers of dmd she has it just as bad as the boys and uses a wheelchair life for us at the moment is very dificult her mobility gets worse every day !! Whe where told 4 years ago when she was diagnosed that it would be mild she has shocked us and doctors by how sever it has actuly become in tilly ....please help us we need treatments and a cure!!! And not just for the boys but for all the children and adults with duchenne muscular dystrophy.

**Tracey Yeoman:** This is Aidan 16 my boy my life my everything, the strength he shows is unbelievable

**Hayley Courts:** This is my gorgeous son Ryan he will soon be 12 , hes head strong and determined ,theres not many things he wont get up to . A cure would be so amazing , even if it took the life limiting part away , we could work with being in a wheelchair . But to find a cure for it all would be everyones prayers answered. Dmd is so cruel for these children ,we teach them to talk ,crawl , walk and run ,and then its cruelly taken away from them .
Verity Bignold: A future

Liam Chadwick: This is my son Liam he's 10 years old he has Duchenne muscular dystrophy. A treatment which could help him in anyway would mean the world to our family, and I don't understand how any new treatments or potential cures wouldn't be rushed through to help the lives of Liam and other boys affected by this horrible condition.

Keasha Brooke Arbuckle Roberts: This is our son Dallin. He will be seven in March and he has Duchenne muscular dystrophy. A treatment would mean everything to our family. It would be the difference between life and death to us. It would give our son a higher quality of life and the ability to follow his dreams of being a police officer. It would mean that he could finally do the same things as his older sister and little brother. It would mean everything to every boy and girl who is affected by this disease. Our son is such a blessing to us and has opened our eyes to what life is really about and taught us that having a disabled child is not the worst thing possible. We just want him to have the opportunities as every other child.

Art Connolly: This is my seven year old son who has DMD, he dreams of being a footballer, even a treatment which would allow him to be mobile enough to kick the ball about would mean the world to him and us (his family). He has three sisters each of which could possibly be a carrier. Please help !!!
**Leanne Barry:** This is our beautiful first born son, Toby, he's 3 and we've just had a diagnosis of Duchenne's Muscular Dystrophy, we are devastated... Our happy, wonderful little boy's muscles are wasting away as the minutes of everyday go by and there's not a thing we can do except beg the people involved with the decision to give our little boy and others a chance... A chance to live a life like other boys with the ability to walk and a future which doesn't involve painful contractures and leg braces, and as a teenager having to go into a wheelchair and a respirator at night... So I BEG with all of my heart and soul to please please give our boys this gift, Toby is so happy and we never want to have to say good bye to him prematurely. From mummy, daddy, and Brandon (plus all his family and friends)

**Carol Bell:** This is my son lee he cant ask for himself now but he would really wish for help for these children.

**Florence Allen:** If it is even remotely possible to halt progression and improve functioning, no matter how clinically insignificant it may seem, then these kids and parents deserve the chance. Walk a mile in their shoes!

**Kerry Collins:** This is me and my son Harvey, he is 8 and a half, a cure for DMD would simply mean everything to me and to all my family, to his brothers, his grandparents and friends of the family alike, it is heartbreaking to see his daily struggles, a treatment would simply be a miracle which would take the weight of the world from our shoulders.

**Gary Kelly:** I really don't think I can post anything other than "everything". Having a disabled child is the worst thing imaginable. The lack of mainstream attention for the disease, in comparison to others is nearly as bad
Carol Bell: We the parents of children with DMD and everyday people raise to make money to donate to the cause to help find any sort of cure or help for these children so when they find it YOU the government should be helping us the parents give a much better life to our children and with this they will. I had 3 brothers with this condition and my lovely son who I miss dearly but I wish there was something that could of helped all of them now there is and this generation still has to fight to get it I think these children have fought enough with DMD without having to fight for something that can help them.

Susi Wong: Charlie is 7. To be told your beautiful child has such a devastating condition is the worst thing imaginable. Slowly you rebuild your life and your future with your child but as a very different person with a different perspective on life. You are stronger but the pain is never far away as you watch your son's peers grow stronger and more agile knowing the future is so uncertain for your son. Charlie is on a drug trial; each week he has to endure weekly injections and blood tests and a 270 mile round trip missing 20% of his education each week as well as other invasive tests. This is his only hope. However if the drug he is on passes the scrutiny for prescribing then the drug may require like Translarna for the NHS to approve it. There is too much delay and these drugs need to be given as soon as they are available. These trials take years as it is without any further delay. Please save our children.

Eilish Clohessy: This is Nathan! Nathan is 11 and just loves sport, he loves playing with his local club Cappry! Nathan is our pride and joy! We would dearly love anything that would prolong Nathan's quality of life! These boys and girls are very precious to their families and we must have hope and fight for potential treatments!! Nothing less will do! These children deserve the best chance possible!

Vikki Cunningham: This is my super amazing son Noah who is 11 years old. He has drive passion and loves life. Unfortunately unlike some well known diseases duchenne muscular dystrophy has a 100% death rate. Noah loves his lego and x box but unfortunately he can no longer push his lego bricks together or play x box for very long due to his arm and hand weakness. He stopped walking 2 years ago so it has been an huge struggle and life changing time to adjust to life as a wheelchair user full time. Although we are fighters, we are fighting a losing battle.
Denise Megran: This is Liam he’s 21 can still walk around in the house with difficulty but not outdoors! He has intermediate DMD was diagnosed at 6 years old but any treatment that would improve his daily living would be a great improvement!!

Hilary Gray: This is my grandson, Callum, with his sister Imogen. He has Duchennes and was diagnosed when just six years old (he is now 8).... The whole family just want Callum to be able to achieve all that he wishes in life (he has had to give up football already and cannot ride a bike like all his friends). Any help towards a cure or extension to his mobility is all that we wish for.

Steve Currier: It would save so much heart ach pain living in fear I have a son who has this evil illness I am living a nightmare already lost a daughter to lose him I would lose my life for a dad going though this u feel useless cant help I feel like I am drowning bn pulled down and cant get back I would swop places if I could heres to having faith placed in god hands

Alan John Pockley: This probably wouldn’t help me as i’m 30 but I would be glad the younger generation could have a better life with slower progression. This would be a huge step forward for this condition.

Linda Coulthurst: This is Christopher with his mum, my lovely nephew who sadly lost his fight for life 5 years ago. It was terrible watching him towards the end. Something needs to be done to help these other children. It’s to late sadly for Christopher but please help these other children.
**Kelly Symonds:** This is my young man Jordan. He is 16 years old and treatment for him would be amazing as he wants to be a games designer but his hands are failing terribly on him. He is an amazing young man as are all our children affected with dmd.

**Caroline Tress:** This Josh he is 9 he has not walked for 2 years and has just been diagnosed with Cardiomyopathy and his bowels are affected. He is a happy determined boy who loves his sport and going out, He doesn't let Duchenne stop him doing anything. A treatment would give Josh a chance to live along with all the others affected by this awful disease.

**Roy Syphas:** I think its safe to say we would all give our own lives for the chance of our boys to extend theirs. We are living life in a bubble of uncertainty, Un-certain about how long, what if, what next.. Alec is a very happy boy, always smiling. it would be a good day to see a cure for him and bring that smile back to my wifes face too.

**Rachel Kemp:** This is my son Regan he is 9 was diagnosed 6 years ago, as you can see he is able to stand!! Which we as a family find a blessing as he loves playing football even though he struggles to keep up with his friends, I would love him to be able to continue playing football, these kids need everything out there that can help this awful illness. HELP SUPPORT OUR KIDS!
**Brendan Casey:** I’m 21 of age with very little movement and was very hard at times when i couldn’t do things anymore i was devastated i don’t want the youngsters to go through what i did at a early age every child should not be denied this drug they deserve the best life possible and freedom for as long as they can god i wish i could walk around a park or something and a big move in the right direction for a cure

**Himanshu Sharma:** My Son, who is 6 and has DMD. There is a big pain in our life to watch our son to lose his walking ability. We hope this treatment to get approve fast and cure him. Treatment means life will give him a chance to get off from the pain. He wants to run, treatment will give him ability to run and play with his friends.. He has stopped playing, he has no charm to play with toys, friends etc... Treatment will give our family life back.

**Jacki Mills:** A treatment for Duchenne would mean my grandson Martin would be able to do what his sister, baby brother and cousin do without getting frustrated and upset.

**Monica Bullock:** My brother passed away at the age of 18 from DMD I am a carrier of this terrible disease and was told I would have a son with it. I am 39 and have decided not to have children because I do not want this disease to continue. So finding a cure would be amazing.

**Saeed Najmeh Amitis:** My nephew is 25 years old and he diagnosed with DMD when he was 9 years old. There are no words to describe how my sister, her husband, my mum and dad are dealing with this issue on a daily basis. The pain of watching your child lose the ability to do things he was once able to do. How we all hope for a treatment or a cure and that is what we cling to. It is the last thing we think of at night and the first thing we think of in the morning. We need people in power to see these children, by putting themselves in their shoes and give our families the chance of hope that these boys deserve. I always wants to have a wish only one wish in this world to come true and that would be a cure for DMD.

**Frances Ann Downey:** My young Son might have had a better quality of life. This needs to be addressed urgently.
Jannette Toomey: This is Matthew Barrett, aged 19. He went off his feet at the age of 10, and is desperate to help those younger than himself for whom this drug could prolong walking. Matthew has been involved in campaigning for a cure for DMD since he was 9.

Shannon Suhuy: My son would be able to run, jump, play sports, and be happy forever!!

Linda Dale Yaughn: My son is 19 and totally dependant on others for his every need that most take for granted. He lost his ability to stand and walk at 9 years. We have come very close to losing him a couple of times due to complications from just having a cold. This disease is horrible it affects the mobility as well as the lungs and heart. As of now the doctor's have told me that all we can do is keep him comfortable and as healthy as possible. There has got to be a cure. This is the worst pain I have ever known watching my son suffer. A cure would mean life. I pray that it will come in time to help my son. But if not at least to help the other boys diagnosed with this evil disease. This young is my heart and my world.

Samantha Turner: My beautiful little boy was diagnosed with DMD in September 2012, words cannot describe the utter devastation we felt upon receiving this diagnosis. The feeling of complete helplessness is something I carry around everyday. To know that there's nothing I can do to save him is impossible to bear. Please give hope to all the families dealing with this. Please allow our boys to have a chance.
Jane Devlin: We have a beautiful little grandson, Leo, who is 3 years old. The thought of him losing the ability to move, play and be the happy wonderful energetic little person he is at the moment is unbearable. For his parents the idea of him declining is even worse and we all have to make the most of every moment we have with him while he has all his movement - the future is unimaginable. Any hope of finding a DMD drug and it being allowed to be used has to be considered. There are so few boys with the diagnosis and surely they should be allowed to be helped.

Tricia de Lazlo: An effective treatment or cure for our beautiful son Sammy who is 6 and was diagnosed with DMD when he was 3 would mean the world to us and all the parents grandparents uncles aunts and friends of children and young adults with DMD. It breaks our hearts to think that all the things that Sammy is able to do like walking around and trying to keep up with his brother and friends will be taken away from him. How would the politicians feel if it was their son or daughter and any possible cure or treatment was blocked. We all need the help now before it is too late for these wonderful children.

Mark Sully: This is my son Nathan, aged 11... We live in Donegal, Ireland... Nathan was diagnosed with Duchenne Muscular Dystrophy in 2014... He is a huge sports fan, playing Soccer GAA and swims... He loves Cappry Rovers Sean MacCumhaills Chelsea and Donegal... His mobility is severely affected and he finds climbing stairs, running and walking difficult... Any advancement in finding a potential cure or treatment that can prolong his life would be a gods blessing... We live in hope for Nathan and all the people suffering from this condition... These boys and girls like Nathan deserve better and not to suffer in silence... We love our Nathan

Jessica Renne Lewis: A chance
Ann Jones: This is Lewis nearly 4

Dawn McCarthy: This is my son Ben. Any treatment for DMD will give my family Hope for the future.

Geri Seaton Karlin: My dear son Ryan loved to play hockey now all he an do is watch. DMD is the most devastating awful disease our child could ever go through it robs the body of everthing. Your muscles waste away we need exon skipping to move ahead with all the other trials to offer treatments to help these children and families.

Gaynor Amphlett: Harvey is my Grandson & a treatment for him is our prayer every day. He is a vibrant young boy being robbed of his mobility. It's not right for children to suffer this way and the chance of a treatment is all we live pray and fund raise for. PLEASE put yourself in our place and imagine what it would do to your family. If love could cure Harvey he would not be sick, but sometimes love is not enough. For the love of god please get a treatment available NOW before it's too late for Harvey and the other boys like him.
**Martin Moore:** Our son Ryan Moore age 4 was diagnosed with duchenne last year. We hope this is as hard as it gets but unfortunately this is not the case. It is quite unbelievable that translarna is still not available and I call on all bodies in the decision making process to please hurry things up. Although Ryan does not qualify for translarna we hope and pray that 1 day we will get to see a cure to help all DMD patients so they can live a full and happy life that they so rightly deserve.

**Naima Alikhan:** My beautiful daughter Hannah, 12 is a manifesting carrier of Duchenne with the right nonsense mutation for this treatment. She’s battled every day of her life to keep walking but I can see the condition taking hold and she’s losing her physical abilities. I’m heartbroken. Please, please help us before it’s too late.
About Duchenne muscular dystrophy:

Duchenne muscular dystrophy is a severe muscle-wasting condition caused by the lack of a muscle protein called dystrophin. Around 2,500 people in the UK are affected by Duchenne muscular dystrophy, with approximately 100 diagnosed every year. This extremely serious condition causes progressive muscle weakness leading to dramatic loss of muscle function. The natural history of Duchenne shows that patients typically lose ambulation in their early teens, will require respiratory support in their mid to late teens and most are likely to die of heart failure or respiratory compromise before the age of 30.

About the Muscular Dystrophy Campaign

The Muscular Dystrophy Campaign is the leading UK charity fighting muscular dystrophy and related neuromuscular conditions. The charity is dedicated to beating these conditions by finding treatments and cures and by improving the lives of everyone affected by them. Founded in 1959, the Muscular Dystrophy Campaign takes the lead in investing in world-class research to find treatments and cures. People also rely on the charity to provide to expert information, advocacy and community support, and to signpost them to effective specialist services.

The charity also campaigns and works with parliamentarians and clinicians across the UK to ensure all people living with neuromuscular conditions have equal access to high-quality health and social care services.