

Annual Report

2014-2015



www.dmdpathfinders.org.uk

Supporting teenagers and adults
living with
Duchenne Muscular Dystrophy

DMD Pathfinders Annual Report 2014 – 2015



Introduction

DMD Pathfinders is a user-led charity (reg. no. 1155884) and was set up in 2014 to promote choice and control and quality of life for teenagers and adults with Duchenne Muscular Dystrophy in the UK.

It campaigns for improved standards of health and social care and provides advice, guidance and support to teenagers and adults with Duchenne Muscular Dystrophy on issues such as independent living, housing, employment and welfare rights.

The aims of DMD Pathfinders are:

- I. To provide a voice for adults living with DMD.
- II. To provide a forum for adults living with DMD to share experiences, ideas and opinions in complete confidence.
- III. To provide information, advice and peer support to adults living with DMD.
- IV. To identify, promote, and develop best practice, innovative treatments and technologies for adults living with DMD.
- V. To campaign and influence treatments for adults living with DMD within health and local authorities, government, relevant professionals, disability organisations and charities.
- VI. To work with health and local authorities, government, disability organisations and charities in improving care, support and services for adults living with DMD.
- VII. To increase awareness of adults living with DMD.

About Duchenne Muscular Dystrophy

Duchenne is a fatal genetic muscle-wasting condition that affects 1 in 3500 births and currently there is no cure. There are an estimated 500 people in the UK aged over 18 with Duchenne and many more with Duchenne-like types of dystrophy. In later stages, Duchenne leads to almost total paralysis, reliance on a ventilator to breathe and a need for round-the-clock-care.

Due to advances in medical care in the last few decades we are now living well into our 20's,30's and 40's, with extensive health, care and social needs. We are an unforeseen generation, since we were not expected to still be alive and as a consequence organisations and agencies that were originally created to advise and support our parents/carers, have been slow to respond to our needs.

What have we done to achieve our core aims?

I. To provide a voice for adults living with DMD.



In October 2013 Trustees of DMD Pathfinders Jon Hastie and Mark Chapman presented at an International Respiratory Meeting in London to discuss standards of respiratory care for adults living with DMD.

Speakers and delegates included Dr Jes Rahbek from Muskelvindfunden in Denmark, Professor Anita Simonds from the Royal Brompton Hospital, Dr Nicholas Hart and Angela Reddy from Lane Fox Respiratory Unit, Guys and St Thomas' Hospital, Dr Mark Elliott from Leeds, Dr Martin Allen from Stoke and Professor Francesco Muntoni from Great Ormond St Hospital.

The discussion focused on the need for high quality respiratory care services and ventilation to ensure a good quality of life for adults living with Duchenne. This led directly to the idea to produce a Frequently Asked Questions booklet on Ventilation and Duchenne.

Members and Trustees of DMD Pathfinders have presented at International and National Conferences such as:



Action Duchenne National Meeting, Hereward College, May 2014

Mark Chapman attended Action Duchenne's National Meeting at Hereward College in Coventry where he contributed in their Takin' Charge sessions with Duchenne youngsters and parents. He also gave a very well received presentation on ventilation at the main meeting to parents, clinicians and professionals, prompting much interest and many questions.

206th European Neuromuscular Centre (ENMC) Conference, May 2014



Jon Hastie attended the 206th ENMC International Workshop in the Netherlands where the key focus was on care for adults with Duchenne.

This workshop provided a platform for experts in adult Duchenne care from across Europe and North America to share best practice and work together to update the existing consensus care considerations to include this adult group of patients.

The workshop heard from clinical experts on the issues faced by adults with Duchenne in areas of cardiac and respiratory management, nutrition and gastro-intestinal problems, as well as issues of pain and fatigue, bone health, upper body function, emotional and psychological health. The workshop also heard from organisations representing parents and adults such as the Dutch Parent Project, Parent Project Muscular Dystrophy and DMD Pathfinders, including two expert patients living with Duchenne (Jon Hastie and Peter Mikkelsen from Denmark) who shared personal and peer experiences. This

patient voice played an integral part in identifying gaps in existing care and helping to shape the basis of updated care considerations.

The workshop also identified considerable potential for further follow-up work in regards to care for this group of patients beyond these research needs. It was recognised as critical that this builds on existing engagement with patients to increase registry participation, peer support and advocacy provision. Engagement with regulators and commissioners across Europe and North America is also needed in order to respond comprehensively to the needs of this growing patient population.

Action Duchenne International Conference, London, November 2014



In collaboration with Action Duchenne and the clinicians mentioned above, we have produced a Frequently Asked Questions guide on Ventilation and Duchenne.

This was launched at the Acton Duchenne Conference 2014 in a workshop co-facilitated by Jon and Mark, also joined by Professor Anita Simonds from the Royal Brompton with her session on Respiratory Research which ended in her highly recommending the new frequently asked questions guide on Ventilation and Duchenne. The launch resulted in international interest in our publications and orders being taken by respiratory clinics to benefit adults and young people throughout the UK and overseas.

Jon and Mark then presented to parents and professionals interested to hear about DMD Pathfinders and how it is run by and for adults with Duchenne. They also spoke of the need for members and volunteers to help with fundraising and communications and people left with a copy of our new guide and another of our publications – ‘Life and Breath’ which is a fact sheet guide for those who rely on ventilation support and need to have a plan for emergency situations such as a power failure.

II. To provide a forum for adults living with DMD to share experiences, ideas and opinions in complete confidence.

DMD Pathfinders have provided an online private Facebook group for people living with DMD to raise issues of concern to them in confidence. It has over 300 members. Through this group, DMD Pathfinders have collated evidence of the extent of need within the Adult DMD Community.

The online group provides support to adults living with Duchenne to live productive, fulfilling and independent lives. The many questions and concerns from our members has highlighted the need for information and advice in key areas. This has led to three guides being produced this year.

DMD Pathfinders have invited guest bloggers to write advice and guidance on key areas of interest. This has included:

- How to get to University
- Mental Health and the value of peer support
- The benefits of disability sport
- The journey towards independent living

DMD Pathfinders have also contributed to research studies relevant to adults with DMD, for example studies carried out on end of life care (Abbot 2014).

III. To provide information, advice and peer support to adults living with DMD.



DMD Pathfinders Mithun Soul, Farhan Mian and Aaron Small have supported families and young people with DMD through Takin' Charge sessions.

They have talked about the different housing options that they have managed to secure for themselves, how and why this housing option works for them and what difference it makes to their lives.

DMD Pathfinders have made really important contribution to the work of Takin' Charge. It is planned that DMD Pathfinders will continue to contribute to workshops with families and young people with DMD providing support and advice on a range of issues."



Members and Trustees of DMD Pathfinders have been actively involved in the Action Duchenne Takin' Charge preparation for adulthood programme for young people with DMD 14 – 25.

This has included support as part of the Steering Committee of the project but also more recently, support in directly mentoring younger people with DMD.



IV To identify, promote, and develop best practice, innovative treatments and technologies for adults living with DMD.

DMD Pathfinders have produced three very well received guides for adults living with Duchenne.



Ventilation and Duchenne

A Frequently Asked Questions guide on Ventilation and Duchenne

dmdpathfinders-ventilation-faq-booklet



Life and Breath

A Guide for having a ventilation emergency plan in the event of power failure

dmdpathfinders/life-and-breath



Touch Type Swipe - An Assistive Technology Guide

dmdpathfinders/touch-tap-swipe

A guide for people with minimal body movement to choose and use touch phones and tablets. This is much needed accessible advice on assistive technology that can remove barriers to everyday communication. This is much needed accessible advice on assistive technology that can remove barriers to everyday communication.

V To campaign and influence treatments for adults living with DMD within health and local authorities, government, relevant professionals, disability organisations and charities.



Members and Trustees of DMD Pathfinders have increasingly been invited to participate in meetings with Local and National Government representatives in England and Scotland to lobby for improved Health and Social care provision for adults with DMD. We have been in attendance at various meetings at both the All Party Parliamentary Group at Westminster and the Cross Party Group at Holyrood.

“Give Us A Break’ Campaign



An important campaign was launched by Robert Watson a Trustee of DMD Pathfinders in Scotland for improved Respite Care for adults with DMD and other life-limiting physical disabilities. This was supported by other members of DMD Pathfinders. It involved firstly addressing the Scottish parliament's Public Petitions Committee, which was made possible thanks to the support of the well known Labour MSP Jackie Baillie. This

presentation made such an impression on the politicians who were present, that it prompted them to hold a Chamber debate on the issue which was attended by some of the most influential MSPs in the country. Robert gave several media interviews on the issue which led to TV appearances both on STV news and BBC Scotland news which really helped to raise the profile of the campaign. This led to a report being produced by Muscular Dystrophy UK on the respite issue, entitled 'Give us a Break'.

Respite Campaign links

MDUK 'Give Us A Break: muscular-dystrophy.org/Give-Us-A-Break.pdf

BBC Scotland news report: youtube.com/BBC/Give-Us-A-Break

STV News report: youtube.com/STV/Give-Us-A-Break

Scottish Parliament Chamber Debate: bbc.co.uk/parliament/debate



In October 2014 Robert was recognised for his campaigning efforts by Muscular Dystrophy UK by being presented with their 'Campaigner of the Year' award.

VI To work with health and local authorities, government, disability organisations and charities in improving care, support and services for adults living with DMD



Members of DMD Pathfinders have been actively supporting other Duchenne and Muscular Dystrophy charities in their campaign to get funding for treatments for DMD.

They have also raised the need to reconsider the focus of the research into a cure for Duchenne so that the needs of adults with the condition are also considered.

DMD Round Table Meeting, TREAT-NMD, Centre for Life, Newcastle, 14th May 2015



Jon Hastie & Mark Chapman represented DMD Pathfinders with 11 other Duchenne charities at the DMD Round Table Meeting organized by TREAT-NMD, held at the Centre for Life in Newcastle on 14th May 2015.

The Key aims of the meeting were to; provide an overview of TREAT-NMD activities relevant to DMD and ensure input from UK DMD organisations, understand the specific priorities of all UK organisations involved in DMD research and care, identify areas where the TREAT-NMD Alliance can foster collaboration to help move the DMD field forward.

We had the opportunity to highlight the relevant priorities for adults, that included; ensuring research is made applicable, improvement of available care and support, enabling our involvement in trials, and ensuring our voices are heard.

In addition to the particular priorities of each charity, several shared themes emerged, which were:

- Funding research into new therapies for DMD.
- Improving medical and social care for those living with DMD and their families.
- Engaging regulators.
- Promoting care for and giving a voice to adults with DMD.
- Awareness raising and lobbying around DMD.
- Ensuring equitable and swift access to treatment.
- Collaborating closely with other organisations.
- Ensuring full utilisation of registries to advance research.

National Workshop on Duchenne Muscular Dystrophy Clinical Trial Capacity, TREAT-NMD, Centre for Life, Newcastle, 10th July 2015



Mark Chapman represented DMD Pathfinders with 11 other UK & Ireland Patient Organisation Participants at the National Workshop on DMD Clinical Trial Capacity organised on behalf of TREAT-NMD, held at the Centre for Life in Newcastle on 14th May 2015.

The aims of the workshop were to identify barriers to clinical trial activity in the UK and form an action plan to improve clinical trial capacity for DMD in the UK. It also highlighted concerns from DMD patient organisation's about the lack of capacity for trials in DMD in the UK.

The workshop brought together 75 people from patient organisations, clinical staff from centres and representatives from the National Institute for Health Research (NIHR) and industry to; assess the current situation, develop a strategy to improve capacity, and better utilise resources.

It was encouraging to hear that current research is being conducted to focus on the development of validated outcome measures for the non-ambulant and adult DMD populations, such as arm and trunk function, contractures and quality of life.

There was also clear acknowledgement that more work has to be done on standards of care for adults in order to facilitate future trial readiness in this population.

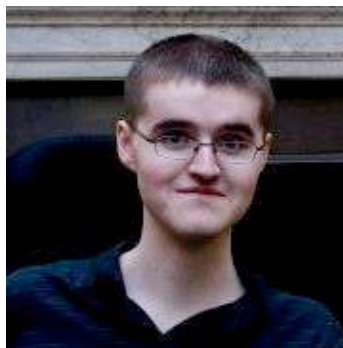
The meeting concluded that the UK must continue as one of the key countries for clinical trials in DMD, clearly demonstrated by the support of all parties to increase capacity and maintain and improve quality.

A five year objective plan with three phases of development, seeking to ensure that all patients with DMD, children and adults, have access to clinical research opportunities will be taken forward by a group of meeting participants.

Patient organisations plan to meet shortly to ensure funding is identified and efforts are coordinated to move the process forward. Detailed information about requirements for capacity building will also be systematically collected by MDUK.

VII To increase awareness of adults living with DMD.

All of the work of DMD Pathfinders on the facebook group, website, social media, attendance at conferences and lobbies has increased awareness of the needs of adults living with DMD. With improved medical care and the reality of more people with Duchenne living into adulthood, the role of a charity for adults with Duchenne run **by** adults with Duchenne is even more crucial. This is a fledgling organisation which has coped well with the challenges of being a new user led organization. The focus for future work is to continue to deliver on our core aims as well as to build the skills of people involved in the organization so as to be sustainable in the future.



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