# Table of Contents

Section 1  
Introduction and Definitions  

Section 2  
Responses  

Section 3  
Demographics  

Section 4  
Neuromuscular Care Centre  

Section 5  
Specific Mutation  

Section 6  
Action Duchenne DMD Patient Registry  

Section 7  
Clinical Trials – Overview  

Section 8  
Clinical Trials – Interest  

Section 9  
Clinical Trials – Discussion  

Section 10  
Clinical Trials – Willingess to Travel  

Section 11  
Clinical Trials – Expectations of Trial  

Section 12  
Clinical Trials – Declined Trials  

Section 13  
Clinical Trials – Tried to enrol and was unable to  

Section 14  
Clinical Trials – Barriers  

Section 15  
Clinical Trials – Feedback on how to improve the Clinical Trial Process  

Section 16  
Clinical Trials – Summary  

Section 17  
DMD Hub  

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"A questionnaire to understand Duchenne patient and caregiver perspectives on Duchenne clinical trials"
Patient and Caregiver perspectives on Duchenne Clinical Trials Survey Report

In the summer of 2017, Duchenne UK launched its first patient survey, to better understand patient views on accessing clinical trials and research. The survey was entitled, “A questionnaire to understand Duchenne patient and caregiver perspectives on Duchenne clinical trials”.

The survey is part of Duchenne UK’s community engagement work to better understand what patients and caregivers want from care, research and clinical trials.

The aim is to help the Duchenne community to better understand the current clinical trial recruitment process, and to see where we can make improvements to create a fair and equitable trial recruitment process. The survey also aligns with Duchenne UK’s broader mission and the DMD Hub to ensure that all patients with Duchenne have access to clinical trial opportunities.

The survey highlighted a number of challenges facing patients who want to access clinical trials:

- Of 180 respondents; 91% of patients surveyed said they DID want to take part in research.
- 50% of those who wanted to, couldn’t because they didn’t meet the inclusion criteria.
- 68% of patients had never been given an explanation of how to access research opportunities. Instead they use social media to find out about studies. Patients were most likely to get onto a trial through persistent emailing of clinicians and trial coordinators.
- 29% had actively tried to enrol on a trial but were not able to. The survey highlighted the sense of desperation felt by patients and caregivers who were unable to access research: “absolutely devastated”, hopeless and a feeling that we are missing out. Disappointed with an unfair system.
- More than 50% said they would travel more than 5 hours to access a clinical trial. However, the cost of participation and burden of travel were also highlighted as the main barriers to taking part in research.
- 71% of those surveyed were registered on Action Duchenne’s registry. 77% of those never or rarely updated their information and 90% did not receive reminders to update details.
This survey was designed by Alex Johnson from Duchenne UK and Sejal Thakrar from Smile with Shiv in consultation with our fellow Duchenne Charities, Action Duchenne, Alex’s Wish, Duchenne Now, DMD Pathfinders, The Duchenne Research Fund, Harrison’s Fund and Muscular Dystrophy UK, patients and caregivers from across the Duchenne community, Emma Heslop from the Duchenne Hub, Industry and healthcare professionals.

The results have been analysed and Sejal Thakrar has created a survey report.

The survey was open for three weeks between the 05th and 17th May 2017 for those who reside within United Kingdom (England, Scotland, Wales and Northern Ireland) and for those who were living with or were direct caregivers of children or adults with Duchenne.

The responses and participants of the survey are not published in the report and will remain anonymous, only total values and numbers are used to ensure anonymity.

For each question only, those who have responded have been analysed e.g. 200 respondents answered question on Neuromuscular care centre, therefore the percentage is based on the 200 and not the total 217 who participated on the survey to ensure data is not skewed and is representative.

Duchenne Muscular Dystrophy may be abbreviated to 'DMD' throughout the report.
A total of 229 responses were received and a total of 217 completed responses have been used. 12 were excluded for the following reasons:

1) 6 - From non-direct caregivers e.g. uncles, aunties and grandparents.
2) 4 - From carers who completed the survey on behalf of more than 1 child/adult with Duchenne e.g. they were caring for a child and an adult with Duchenne, these were excluded to ensure the data is interpreted correctly e.g. where a carer may be caring for adult of 18 years and over with Duchenne and a child of 11 with Duchenne the experiences, hope and expectations could differ for both.
3) 1 - Duplicated response.
4) 1 - Incomplete response; only first 2 questions answered.

Total number of responses
The chart below shows the respondents by country and profile of respondents.

78% (n=171) were from England, 8% (n=17) Scotland, 10% (n=21) Northern Ireland and 4% (n=7) Wales.

There were more ‘Caregivers of a child living with Duchenne (under the age of 13)’ – this group appeared to be more engaged and motivated to complete the survey, we acknowledge that this may not be a complete representation of the Duchenne community.

Note: Country is based on 217 responses
The age range of those living with Duchenne was between 1 and 47 years of age. 71% (n=154) were below the age of 13 years, 17% (n=38) between the ages of 13 and 18, 11% (n=23) were adults or carers of adults and 1% (n=2) was a parent who had lost a son to Duchenne.

**Age diagnosed with Duchenne**

![Graph showing age distribution]

216 respondents completed the question on the age of Duchenne Diagnosis. 55% (n=119) were 3 years or younger at the time of diagnosis with 3 being the most common age for diagnosis (n=49. The average age of diagnosis reported in the 2014 study ‘Improving recognition of Duchenne muscular dystrophy: a retrospective case note review’* was between 4.5 and 4.11 years of age for boys, the study excluded those diagnosed via post-natal screening and those with a family history. Although the published study did conclude that the age of diagnosis had improved, based on the results of this survey, we cannot conclude that the age of the diagnosis has decreased as the survey population may not be a complete representation of the DMD community. However, it is certainly positive that with the increase in awareness we see that over 50% of those who responded were diagnosed at the age of 3 or below.

The top 10 care centres are being attended by 72% of the respondents, with the other 28% consisting of 27 different individual care centres.

Great Ormond Street Hospital is ranked as number one in terms of number of respondents.

<table>
<thead>
<tr>
<th>Rank by Responses</th>
<th>Main Neuromuscular Care Centre</th>
<th>Nbr of Responses</th>
<th>% of Responses</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>LONDON (GOSH), Dubowitz Neuromuscular Centre, Great Ormond Street Hosp for Children NHS Trust</td>
<td>34</td>
<td>17%</td>
</tr>
<tr>
<td>2</td>
<td>NEWCASTLE, Institute of Genetic Medicine,</td>
<td>23</td>
<td>12%</td>
</tr>
<tr>
<td>3</td>
<td>BIRMINGHAM, Birmingham Heartlands Hospital</td>
<td>15</td>
<td>8%</td>
</tr>
<tr>
<td>4</td>
<td>LONDON (Evelina), Evelina Children’s Hospital</td>
<td>14</td>
<td>7%</td>
</tr>
<tr>
<td>5</td>
<td>BELFAST, Royal Belfast Hospital for Sick Children</td>
<td>13</td>
<td>7%</td>
</tr>
<tr>
<td>6</td>
<td>LIVERPOOL, Alder Hey Children’s hospital</td>
<td>13</td>
<td>7%</td>
</tr>
<tr>
<td>7</td>
<td>MANCHESTER, Royal Manchester Children’s Hospital</td>
<td>9</td>
<td>5%</td>
</tr>
<tr>
<td>8</td>
<td>LEEDS, Yorkshire Regional Muscle Clinic at LGI</td>
<td>9</td>
<td>5%</td>
</tr>
<tr>
<td>9</td>
<td>SHEFFIELD, Sheffield Children’s Hospital</td>
<td>7</td>
<td>4%</td>
</tr>
<tr>
<td>10</td>
<td>GLASGOW, Fraser of Allander Neurosciences Unit</td>
<td>7</td>
<td>4%</td>
</tr>
<tr>
<td>Other (27 Locations)</td>
<td></td>
<td>56</td>
<td>28%</td>
</tr>
<tr>
<td>Total Responses</td>
<td></td>
<td>200</td>
<td>100%</td>
</tr>
</tbody>
</table>

Note: Main Neuromuscular Centre, Shared Care, Based on 200 Responses

How often do you or the child in your care attend the Neuromuscular Care Centre?

65% (n=133) attend their main care centre every 6 months.
15% (n=31) attend more frequently than 6 months, 8 of these were on a clinical trial at the time they responded to the survey.
3% (n=6) say they “do not attend” - these are all adults.

Note: Frequency of visit based on 207 Responses
35 respondents indicated that they had shared care.

The table below shows shared care by main care centre, for example, if you look at Great Ormand Street Hospital a total of 34 respondents attend Great Ormond Street Hospital as their main care centre. Of the 34, 8 have indicated that they have shared care with another care centre; 24% of the respondents who go to Great Ormond Street Hospital have shared care.

For those whose main care centres are outside of England are the ones who also have shared care.

Within England; we see a higher % of shared care for those who attend Bristol, Manchester and Birmingham as the main care centre and for the 36 respondents who attend Newcastle or Liverpool none have shared care.

### Main Neuromuscular Care Centre and Shared Care

<table>
<thead>
<tr>
<th>Main Neuromuscular Care Centre</th>
<th>Nbr of Respondents who have shared care</th>
<th>Nbr of Respondents Attending Main Care</th>
<th>% of Shared care</th>
</tr>
</thead>
<tbody>
<tr>
<td>Tallaght Hospital Dublin Ireland Total</td>
<td>1</td>
<td>1</td>
<td>100%</td>
</tr>
<tr>
<td>Larbert Total</td>
<td>1</td>
<td>1</td>
<td>100%</td>
</tr>
<tr>
<td>SWANSEA, Morriston Hospital Total</td>
<td>3</td>
<td>5</td>
<td>60%</td>
</tr>
<tr>
<td>GLASGOW, Fraser of Allander Neurosciences Unit Total</td>
<td>4</td>
<td>7</td>
<td>57%</td>
</tr>
<tr>
<td>ABERDEEN, Royal Aberdeen Children’s Hospital Total</td>
<td>1</td>
<td>2</td>
<td>50%</td>
</tr>
<tr>
<td>Bristol Children’s Hospital Total</td>
<td>1</td>
<td>2</td>
<td>50%</td>
</tr>
<tr>
<td>MANCHESTER, Royal Manchester Children’s Hospital Total</td>
<td>4</td>
<td>9</td>
<td>44%</td>
</tr>
<tr>
<td>BIRMINGHAM, Birmingham Heartlands Hospital Total</td>
<td>2</td>
<td>7</td>
<td>29%</td>
</tr>
<tr>
<td>BRISTOL, Frenchay Hospital Total</td>
<td>1</td>
<td>4</td>
<td>25%</td>
</tr>
<tr>
<td>LONDON (GOSH), Dubowitz Neuromuscular Centre, Great Ormond Street Hosp for Children NHS Trust Total</td>
<td>8</td>
<td>34</td>
<td>24%</td>
</tr>
<tr>
<td>LEEDS, Yorkshire Regional Muscle Clinic at LGI Total</td>
<td>2</td>
<td>9</td>
<td>22%</td>
</tr>
<tr>
<td>PRESTON, Royal Preston Hospital Total</td>
<td>1</td>
<td>5</td>
<td>20%</td>
</tr>
<tr>
<td>BELFAST, Royal Belfast Hospital for Sick Children Total</td>
<td>2</td>
<td>13</td>
<td>15%</td>
</tr>
<tr>
<td>LONDON (Evelina), Evelina Children’s Hospital Total</td>
<td>2</td>
<td>14</td>
<td>14%</td>
</tr>
<tr>
<td>SHEFFIELD, Sheffield Children’s Hospital Total</td>
<td>2</td>
<td>15</td>
<td>13%</td>
</tr>
<tr>
<td>Grand Total</td>
<td>35</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

Note: Main Neuromuscular Centre, Shared Care, only those who have given main care centre included Based on 200 Responses
Looking further at shared care; Newcastle, the Institute of Genetic Medicine appears to be the preferred shared care centre.

**Shared Neuromuscular Care Centre**

A higher percentage of those who are or have been on a clinical trial have shared care. The question that remains and will need further investigation is — "Do these patients have shared care to access a clinical trial, or do they have shared care as a result of taking part in a clinical trial".
Do you know the specific mutation?

Of the 215 who responded to this question 90% are aware of their specific mutation.

**Note:** (1) Mutation known is based on 215 responses, (2) 'No, waiting results' removed from Mutation not know by age group chart

**Mutation not known by age group**

<table>
<thead>
<tr>
<th>Age Group</th>
<th>Number</th>
</tr>
</thead>
<tbody>
<tr>
<td>Less than 13</td>
<td>7</td>
</tr>
<tr>
<td>Between 13-18</td>
<td>4</td>
</tr>
<tr>
<td>18+</td>
<td>11</td>
</tr>
</tbody>
</table>

**Note:** (1) Mutation known is based on 215 responses, (2) 'No, waiting results' removed from Mutation not know by age group chart

50% of those who were not aware were adults (age 18+).
Of those who were not aware of their specific mutation, 12 did not have any intention of testing, the reasons are listed above.
The Action Duchenne DMD patient registry acts as a database for all patients who have been diagnosed with Duchenne or Becker Muscular Dystrophy and female carriers in the UK and is part of the TREAT-NMD global database, which is used to carry out clinical feasibility studies.

**Are you registered on the Action Duchenne DMD patient registry?**

71% (N=137) of 193 respondents who responded to this question are registered on the Action Duchenne DMD patient registry in the United Kingdom.

**At time of diagnosis were you informed of Action Duchenne DMD patient registry?**

58% (n=111) were informed of the Action Duchenne DMD patient registry at time of diagnosis.
45% were informed of the Action Duchenne DMD patient registry by their Consultant/Doctor at the time of diagnosis. This is the case for all countries with the exception of Northern Ireland.

**When did you register on the Action Duchenne DMD patient registry?**

133 responded to this question; 48% (n=65) registered on the Action Duchenne DMD patient registry within the first 3 months of diagnosis.
How often do you update the Action Duchenne DMD patient registry?

- Extremely frequently (Every 2-3 months): 1% (n=1)
- Very frequently (Every 6 months): 4% (n=5)
- Frequently (Every 12 months): 13% (n=18)
- Not very frequently (>12 months): 36% (n=50)
- When prompted (by a Doctor, Nurse, Charity etc): 4% (n=5)
- I gave details to registry when my son was first diagnosed: 1% (n=1)
- I gave details at the time of diagnosis 5 months ago: 1% (n=1)
- Never: 41% (n=56)

Note: How frequently do you update the AD Registry based on response 137 responses

137 responses to this question.

77% (n=106) Never update or update less frequently; most common reasons are “forgetting to”, “not aware that the registry needs updating” and “issues accessing (e.g. forgetting password)” and “lack of time to update”

18% (n=23) update frequently or very frequently; most common reason here is to ensure “opportunity for a suitable clinical trial is not missed”

Are you reminded to update details on Action Duchenne DMD patient registry?

- Yes: 90% (n=115)
- No: 10% (n=13)

112 of the 128 who responded to both questions above do not receive a reminder from either their consultant/doctor or the registry owner to update their details on the registry.
Are you aware of how the Action Duchenne DMD patient registry works to aid recruitment for clinical trials?

“Trial sites tend to contact patients that meet the criteria for a trial from their own databases. If the quota for a trial is not reached and more patients are required, they will send a formal request to the DMD Registry to assist recruitment. It is a point of call for researchers and pharmaceutical companies”

- Action Duchenne.

Of the 167 responses, just over half are aware of how the registry works to aid recruitment of Duchenne clinical trials.

Are you aware of how the Action Duchenne Registry works to aid recruitment?

Note: Based on those who answered above; 167 who responded yes or no
Has anyone ever explained the process of how you could enrol you or the child in your care onto a clinical trial?

By Interest in taking part in clinical trials

68% (n=123) have not had the process of how to enrol on a clinical trial explained to them.

163 responses were received for this question; 1/3 have taken part in a clinical trial. Of the group who have taken part in a trial 59% (n=32) are currently taking part in one.
Of those who have taken part in a clinical trial - 34% (n=18) have taken part in more than one different clinical trial; these include both therapeutic and non-therapeutic therapies. **Therapeutic trials** are ones which enrol patients and provide a specific treatment to the patients to **study** its impact on DMD for example Exon Skipping or Protein replacement. **Non-therapeutic trials** are ones which do not provide a treatment to patients, but instead **study** important factors which help advance the understanding of DMD and its impact for example Natural History.

**Same Trial vs Different Clinical Trial for those who have been part of more than 1 clinical trial**

22% (n=4) have taken part in the ‘same trial’ e.g. PTC phase 2b and PTC Open label.

78% (n=14) who have taken part in more than one ‘different clinical trial’ e.g. Natural History and FORDMD.
Of the 180 responses; only 9% (n=16) of those who responded do not want to or do not want their child to take part in trials.

Interested in taking part in clinical trials by age living with Duchenne

126 responded to this question. 50% of those who want to take part in trials are in fact older boys/men living with Duchenne and carers of younger boys who do not meet the criteria to take part; 21% (n=27) 13 years or over and 29% (n=37) between the age of 1 and 5.
127 responses; above is based only on those who want to or very much want to take part in a clinical trial.

The highest interest was in Protein replacement therapies and therapies that will target the underlying cause of Duchenne.

Top 5 interested therapies

1. **Protein replacement e.g. Summit,**
2. **Stem cell therapies e.g. Capricor**
3. **Gene Therapy e.g. Solid GT**
4. **Genome Editing e.g. CRISPR**
5. **Exon Skipping e.g. Sarepta**
There are some differences in preference of type of clinical trials for those who have previously taken part in a clinical trial compared to those who have not.

Those who have experience of clinical trials show a preference for Stem cell, Anti-inflammatory, Cardiac and Nutritional/supplement studies over those who have not taken part in a clinical trial.

Those who have never taken part in a clinical trial show a preference for Gene Therapy, Exon Skipping, Corticosteroids and Natural History studies.
Discussion of Clinical Trial opportunities is extremely important to both those living with Duchenne and those who are caregivers; these discussion are however more important at regular check up’s compared to at diagnosis.

**Does your or your child’s Doctor talk to you about potential upcoming trials at check-ups?**

*Only those who very much want to or want to take part in a clinical trial*

Consultants are discussing upcoming clinical trials with those who are interested in clinical trials, however we did see a higher % of these discussions taking place with those who are currently on a trial compared to those who are not.
Looking at how far people are willing to travel to access a clinical trial, over 50% of those who want to be part of a trial are prepared to travel more than 5 hours to take part in a trial. However, looking at section 14 on pg 33, ‘living too far away from trial sites’ is ranked high as a barrier when considering taking part in a clinical trial. The cost of participating and looking to take part in a suitable trial are also main barriers; in conclusion when considering traveling to a clinical trial site the type of clinical trial and cost of taking part will likely influence the willingness to travel to access a trial.
Did you have to go to a different hospital from where you or the child in your care receives routine care?

37% (n=20) have taken part or are part of a clinical trial where the trial site is different from where routine care takes place.

Note: based on 53 responses
One of the questions asked about the hopes and expectations of being part of a clinical trial. The top two identified were longer and better quality of life and finding a drug that would work resulting in a better future for those living with Duchenne.

Hopes and expectations for taking part in a clinical trial

Note: 1) Not taken part in a clinical trial = 92 responses 2) Have taken part in a clinical trial = 21 Responses
This section is looking specifically at the reasons why patients/carers have declined a Duchenne clinical trial.

**Have you or the child in your care been invited to take part in a clinical trial but declined?**

A total 139 responses; included those who are currently on a clinical trial and want to be part of a trial; 17% of those are interested in taking part in a clinical trial have declined trials.

**Note:** based on 139 responses and restricted to those who want to take part in a clinical trial or are currently on a trial.
Reasons for declining trials differ for those who have had experience of trials compared to those who have not had experience.

For those who have never participated in a trial the concerns are with the trial itself and the burden of taking part on a trial, for example, the financial burden, the emotional burden and the screening process.

For those who have previously taken part and have experience their concerns differ and are associated with the drug itself for example side effects and concerns of being part of the placebo arm.
The FOR DMD was the most declined clinical trial for those who completed the survey.

**Which trials have been declined?**

Note: based on 21 responses, some respondents have declined more than 1 trial
Have you or the child in your care ever tried to enrol into a clinical trial and been unable to?

29% (n=45) had actively tried to enrol on a trial but were not able to. 6% (n=9) would have liked to but did not as they knew they would not meet the inclusion criteria required to take part.

How many trials have you or the child in your care actively applied for or would have liked to have taken part in but have not been able to? June 2017

57% (n=22) have actively tried to enrol on one clinical trial and not been able to.
Social Media appeared to be the most important source when looking for information in relation to clinical trials; 15 respondents came to know about clinical trials which they tried to enrol.

**Describe how you attempted to enrol yourself or the child in your care onto the trials and the challenges you faced? (Open ended question)**

Persistently emailing the clinician team and trial co-ordinators was the method most used when trying to proactively enrol for a clinical trial. The main challenge faced was not fitting the inclusion criteria.
Reasons for not being accepted on a clinical trial

Main reasons for not being accepted on a clinical trial which had been proactively applied for;
1) Did not meet inclusion criteria
2) No spaces were left at time of enquiry.

Why was inclusion criteria not met?

68% (n=15) claimed that Age was the main reason inclusion criteria was not met; either 27% (n=6) too old or 41% (n=9) too young.
How did not being able to take part in a trial make the person with Duchenne feel?

An open ended question was asked to understand how the person living with duchenne felt when they could not take part in a trial, here are a range of responses that were received.
If you are a carer of a child with Duchenne, how did you feel about the child in your care not being able to take part in the trial?

An open ended question was asked to carers to understand how they felt when the child or adult they care for could not take part in the trial, here are the range of responses that were received.

"absolute devastation, somewhat resentful as a fund raiser that donates everything to research"

"Furious"

"Cross as I had not understood how to enrol for a trial and we missed out"

"Excluded"

"Useless. Helpless. Frustrated with the entry criteria"

"Helpless"


"Deflated"

"Gutted"

"Heartbroken"

"Very critical of the trial designs and tired of industry constant claim that no boys are available for trials in such a rare disease. Tired that no adaptive trials are being adopted"

"angry, frustrated, helpless, mad at the system for excluding us"

"Devastated ... it would be a little hope to hold on to."

"Isolate"
In terms of barriers, the top 3 are:

1) Caregivers or those living with Duchenne appear to be looking for a more suitable trial.
2) It appears that living too far away from trial sites acts as a barrier although previously we could see that over 50% were prepared to travel 5 or more hours to access a trial.
3) The cost it would incur to participate in a clinical trial was also a barrier.
Finally the respondents were asked to give feedback on how they feel the clinical trial process could be improved. This was categorised into the relevant sections below:

**Communication**

- Better communication from doctors/consultants/pharmaceuticals
- Better knowledge transferred to doctors
- Timely information; not before or after a trial has started or recruited
- Update on trials at each hospital visit
- Direct email sent to with trial status updates
- Charities to provide more information
- Better explanation of studies and their impact
- Updated list of current and upcoming trials
- Better awareness/communication from consultants on how clinical trials are recruited
- One clear message and update from all involved
- Families to share their experiences of being on trials

**Trial design/Outcome measures**

- 6mw test – abolish!
- Placebo - abolish or make very short and use natural history as the placebo
- Better outcome measures – ie upper body strength
- Speed up the process
- Clearer ethical considerations when extending trial e.g. Efficacy data available to make informed consent
- Consideration from drug companies for the unseen costs of clinical trials
- Childcare expenses for siblings, out of pocket expenses such as gifts, items to relieve boredom for child whilst in hospital
- Policies on ‘how far is too far’ when considering failed blood draws and cannulations
- Better access and availability of psychological support for child and family
- Ability rather than age for cut off points
- Children/adults on trials are treated well

**Accessibility**

- More Trial sites/Capacity
- Have centres in Scotland
- Trials held at local sites where people may be living in remote areas
- Accepting wider age range- adults and younger children
- More flexibility on non ambulant options
- Include those not taking steroids
SECTION 15 Feedback on how to improve the clinical trial process

**Action Duchenne Registry**
- Increase awareness of registry
- DMD registry to update and inform of all trials and contact families more often
- All to ensure registry is kept up to date

**Social Media**
- Families refrain from posting pictures of upset children on social media

**Fairer recruitment process**
- Should not be ‘Who you know’
- Why are some people offered places and others are not?
- Fairer in terms of location, should not be based on geographic location as people are prepared to travel to access sites
- Clear guidelines and protocol that should be followed by all parties involved
- Opportunity to meet co-ordinators/Clinical trial nurses at each appointment if requested
Below are the summary of the findings from this survey:

- **217 responses predominantly from England**
- **Average age of diagnosis is 3 years of age**
- **Do you know your mutation;**
  - 90% (n=163) know their/child’s specific mutation
  - 10% (n=22) do not know mutation of which 11 are adults, 7 under 13’s and 4 between 13-18
  - 12 respondents do not plan to test; don’t know how to and do not see what difference it will make
- **Action Duchenne Registry**
  - 29% (n=56) are not registered or are not sure if they are
  - 71% (n=137) are registered, half of those had registered within the first 3 months
  - 18% (n=23) update regularly (least once a year) to ensure they do not miss out on any clinical trials, others forget or are not aware of the value
  - Those registered do not appear to be reminded to update; either by registry or clinicians
  - AD registry accessed by pharma’s, influence on inclusion criteria? Need to remind to update regularly
- **Expectations of clinical trials;**
  - Longer and better quality of life
  - Finding a drug that would work and result in a better future for others living with Duchenne
- **Barriers for taking part in a clinical trial;**
  - Looking for a more suitable trial
  - Living too far away from trial sites
  - Cost it would incur to participate are the main barriers to taking part in clinical trials
- **Tried to enrol on a clinical trial and not able to**
  - N=45 have tried to enrol on a trial and have been unable to do so
  - N=9 would have liked to but knew they would not fit inclusion criteria; being too old or too young was the main reason
  - Found out about trials mainly via social media
  - Persistently emailing the clinician team and trial co-ordinators was used when trying to enrol for a clinical trial.
  - The challenges faced included oversubscription of trials and not fitting the inclusion criteria
  - Not fitting inclusion criteria; too old, too young
  - Over subscribed; patients are hearing about upcoming clinical trials too late
Duchenne UK is committed to addressing many of the concerns raised by patients in this survey, especially around the issues of communications and trial recruitment through the setting up of the DMD Hub.

The DMD Hub is a partnership between Duchenne UK and the centres of excellence Newcastle and Great Ormond Street. It’s aim is to address the lack of clinical trial capacity in the UK and is working with key stakeholders to build on existing expertise and create a network of clinical trial sites, which fits into Duchenne UK’s broader mission that all patients with Duchenne are given the opportunity to take part in research.

In particular the Hub will:

- **Improve communication**, working with Hub sites and patient organisations to produce and disseminate information sheets on clinical trial opportunities in a timely manner.

- **Provide fair and equitable access to clinical trial opportunities** by increasing the number of sites able to take on interventional trials, developing standard operating procurers for clinical trial recruitment and working with TREAT-NMD and the Action Duchenne patient registry.

- **Develop the DMD Hub website** which will be a key resource for industry, clinicians and patients. It will host an interactive map of the UK detailing clinical trial opportunities for patients, contain a repository for training material for sites and act as a one-stop shop for industry / sponsors interested in conducting trials in the UK.

Duchenne UK is delighted that Emma Heslop is in post as Hub Manager.

Thank you to all those who participated in helping to put together the survey and all those who took the time to complete the survey. Any questions in regards to the survey please contact

Naomi Litchfield at Duchenne UK - naomi@duchenneuk.org

Any questions in regards to the DMD Hub please contact

Emma Heslop - emma.heslop@newcastle.ac.uk