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Original article

Delivery of physiotherapy and occupational therapy standards of care for Duchenne muscular dystrophy: Key recommendations based on UK web-based survey

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ABSTRACT

Duchenne muscular dystrophy is a neuromuscular disorder which has benefited from the implementation of key management strategies embedded in International Standards of Care. This study was prompted by the need for more practical, implementable guidance for physiotherapists and occupational therapists than were presented in the international guidelines. Using two web-based surveys, we explored how therapy is currently being delivered in the UK at specialist neuromuscular clinics as well as in community settings, and how it could be improved. The surveys showed that a significant proportion of families report they are not accessing any physiotherapy in either neuromuscular centres (23 %) or in the community (33 %) and that occupational therapy was particularly limited in neuromuscular centres (lacking in 70 %) but also in the community (33 %). There was evidence that, although much is reported as positive about appointments, key gaps are evident. The feedback from families and therapists indicates that care could be better delivered by improving communication, alongside upskilling and education of therapists and families. It demonstrated the value of and real need for a guide for therapists for Duchenne muscular dystrophy as well as a simultaneous family guide to facilitate the improvements identified.

1. Introduction

Duchenne muscular dystrophy (DMD) is an inherited, X-linked recessive neuromuscular disorder due to variants in the *DMD* gene, encoding for the structural protein dystrophin [1]. Due to the absence of dystrophin in skeletal muscles [2], there is progressive muscle weakness, which leads to loss of ambulation (LOA) by age 10–14 years [3], and beyond this, continuing loss of function also affecting upper limbs, respiratory and cardiac muscles [2]. Dystrophin also plays an important

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Abbreviations

DMD	Duchenne muscular dystrophy
SoC	Standards of Care
NMC	Neuromuscular Centre
PT	Physiotherapist
OT	Occupational Therapist
TWG	Therapy Working Group
APCP	Association of Paediatric Chartered Physiotherapists

role in the brain although its function here is still being elucidated. People with DMD are more likely than average to experience a number of neurobehavioral and learning issues including autistic spectrum disorders, attention deficit hyperactivity disorder, anxiety, learning delay [4].

As a result of glucocorticoid treatment, ventilatory intervention, anticipatory cardiac intervention and multidisciplinary care, the rate of progression of individuals with DMD has slowed significantly over the past three decades along with improvements in life expectancy [5,6]. The publication of international standards of care (SoC) for DMD [7–9], first in in 2010 [10,11] and then updated in 2018, were an important step towards improving DMD patient care in all areas including therapy and rehabilitation. These guidelines were also reproduced as Family Guides, and are now translated into many languages, which are distributed to patients and health care providers via patient organisations, the TREAT-NMD network, and patient registries with the aim to further improve delivery of best practice.

People living with DMD have undoubtedly benefited from the publication and dissemination of these multi-disciplinary SoC [8]. They provide a framework for therapy care, including physiotherapy and occupational therapy assessment and management. Currently in the United Kingdom (UK), most people living with DMD are understood to be seen by a specialist neuromuscular team including specialist neuromuscular physiotherapy approximately twice a year, with hands on therapy support supplied locally where available. Precise and current information on this service delivery is not known. The current SoC, although suitable for a worldwide audience, lacks practicality and detail, particularly stage-specific advice, required for national health service delivery. Detailed advice can be valuable to therapists working in specialist and community settings who are aiming to manage people with DMD more effectively over their lifetime [12]. For adults living with DMD, a UK consensus document has been recently published which includes more detailed therapy guidelines [13]. This substantial piece of work was clearly required given the inequalities in care between adults and the paediatric population in the UK in particular [14,15]. A recent European survey showed significant service provision differences between countries, Germany had the highest provision of physiotherapy (90%), and the UK showed the lowest provision at 50%. However, most of the responders in the European survey reported receiving physiotherapy in the form of stretching or physical exercises from a qualified professional, with only 7.4 % reporting never having received this form of therapy. Adults were generally less likely than children to receive physiotherapy. However, the survey showed that adequate physiotherapy and occupational therapy is still not being delivered in the UK, in either the paediatric or the adult population. Further investigation is warranted which includes the perspective of therapists and families on the delivery of physiotherapy care to gain a more comprehensive picture of SoC delivery in the UK and how implementation could be improved.

The aims of this project are 1) report on how physiotherapy and occupational therapy is currently being delivered for people living with DMD in the UK in specialist neuromuscular clinics and in community settings with reference to previously published therapy international SoC [8] (box 1); 2) to seek input from individuals living with DMD, their families and therapists in all settings on how delivery of therapy SoC could be improved; 3) link the findings of this study to the launch of UK Therapy Guidelines for DMD in order to shape them in a way that addresses the needs identified.

2. Methods

Two online surveys were designed to capture current delivery of SoC in the UK and how delivery might be improved.

2.1. Survey part 1 – parents and individuals living with DMD

One survey was aimed at parents and individuals living with DMD (Family Survey) and consisted of 29 questions about frequency, location and content (what was assessed and what else was covered) at specialist neuromuscular appointments with therapists (physiotherapists and occupational therapists) as well as community-based therapy interactions. Opportunities were given for free text answers regarding how appointments and therapy services could be improved. Ethical approval was obtained from Newcastle University (Ref: 44080/2023, Lead Investigator: Professor Michela Guglieri). The survey was disseminated by Duchenne UK (DUK), leading UK Duchenne charity, on their website and via their newsletter and was available from March 15, 2024 to June 30, 2024. Flyers with QR codes were sent to all paediatric North Star Network Neuromuscular Centres to be distributed to families attending clinic. Participants accessed the survey via a QR code or web link. Consent was embedded within the online survey and participants were not enrolled as part of routine clinic appointments.

2.2. Survey part 2 - therapists

The second survey was directed to therapists (in specialist

Box 1
Key Therapy statements in International DMD standards of care [8].

International SoC state that ‘*Consistent and reproducible clinical assessments of neuromuscular function done by trained practitioners underpin the management of DMD’* and they should ‘*provide comprehensive multidisciplinary assessments, at least every* 6 months*’ including range of motion and monitor for scoliosis’ - and assess respiratory function*’.

Role of Therapy is to ‘*Assist in prevention of contracture or deformity, overexertion, and falls; promote energy conservation and appropriate exercise or activity; provide orthoses, equipment, and learning support*’ and ‘*continue all previous measures; provide mobility devices,* seating, *supported standing devices, and assistive technology; assist in pain and fracture prevention or management; advocate for funding, access, participation, and self-actualisation into adulthood*’.

Rehabilitation can be provided in outpatient clinic and school setting and should continue throughout your life. You should be *assessed by a rehabilitation specialist at t every 4 to* 6 months [8].

neuromuscular centres and in the community) and included 23 questions of a similar structure to the parental survey, also accessed via QR code or web link. It was designed so therapists were only asked to respond to questions based on their location (neuromuscular centre therapist or community therapist) and qualification (physiotherapists or occupational therapist). The therapy survey had audit approval through Newcastle NHS (Ref: 15159, submitted by Dr Meredith James) and ran from March 15, 2024 to June 30, 2024. It was distributed via DUK, the North Star Clinical Network and the Association of Paediatric Chartered Physiotherapists (APCP). Consent was embedded within the online survey.

Details of the question content of these two surveys can be requested from the authors.

2.3. Analysis

Frequency analysis of responses, as well as qualitative thematic analysis from open response questions was conducted for both surveys. Themes were compiled by hand (AGM) and reviewed by a second researcher (CT) with the focus being identification of key common themes and their frequency.

3. Results

3.1. Study cohort

The characteristics of the responders for the family as well as the therapist survey are presented in Tables 1 and 2. Table 3 summarises the access to therapy reported by families.

The results from the Family Survey and Therapy Survey are divided into Specialist neuromuscular and Community based results.

3.2. Specialist neuromuscular therapy - family and specialist neuromuscular therapist responses

For specific assessments conducted at specialist neuromuscular centres, there is some overlap between the role of PT and OT, although OTs are much less likely to be accessed this way. Table 4 (far left column) contains details of the most likely professional to conduct a particular role but significantly likely to be conducted by a PT.

Twelve families (41%) reported that appointments to see a specialist neuromuscular therapist were not frequent enough, 5 (17%) said they would like to see an OT and 8 (28%) that they want more time with the therapy team. Six (21%) did not think there was enough time to get advice or information and 4 (14%) reported they were not given advice. Additional comments from 3 (10%) included difficulty getting in touch with therapists outside their clinic appointment.

Of the 34 specialist NMC PTs, 33 (97%) reported that they see individuals every 6 months or more frequently. 31 (91%) review patients face to face in clinic, other options included home visits, 8 (23%), remotely by video or phone 12 (35%) or in another setting 7 (20%). Thirteen (38%) of physiotherapists were happy with the set up and frequency of appointments with issues relating to lack of space, 8 (23%), lack of time with patients, 11 (32%), not having the correct equipment, 7 (21%) (with particular reference to lack of hoists in clinic by four therapists), frequency of appointments not often enough, 4 (12%) and not having a specialist OT, 19 (56%). Only one specialist NMC OT responded to the survey and reported to see patients every 6 months face to face and raised issues with a lack of equipment and space.

Fig. 1 describes the circumstances that might trigger a specialist neuromuscular PT to see a patient more frequently. Appointments were reported to potentially become less frequent if local support was particularly good or if an individual was seen at another centre.

Table 1
Characteristics of family survey respondents.

Family Survey	N = 37 (%)
Parent or carer	36 (97)
Individual	1 (3)
Geographical location	
England	28 (76)
Wales	4 (11)
Northern Ireland	1 (3)
Scotland	4 (11)
Age range of individuals represented in survey	
Less than 5 years of age	5 (14)
5–9 years of age	14 (38)
10–14 years of age	11 (30)
15–17 years of age	3 (8)
18–29 years of age	3 (8)
30+ years of age	1 (3)
Functional level of individuals	
Early ambulatory phase (walking)	18 (49)
late ambulatory (still walking but with difficulty)	8 (22)
Early non-ambulatory (still stepping and standing but using a wheelchair for most things)	2 (5)
Late non-ambulatory (full time wheelchair user, no ventilatory support)	6 (16)
Late non-ambulatory with ventilatory support	2 (5)
Other – not disclosed	1 (2)
Highest educational level of a family member	
School qualification (GCSE or A Level)	8 (22)
Studied to degree level	15 (41)
Post-graduate qualification and	13 (35)
Preferred not to say	1 (3)
Ethnicity	
White British	25 (68)
White Irish	2 (5)
Other white background	2 (5)
Asian/Asian British: Indian	4 (11)
Asian/Asian British: Pakistan	1 (3)
Multiple background: White and black African	1 (3)
Jewish	1 (3)
Prefer not to say	1 (3)
First language	
English	29 (78)
Hindi	3 (8)
Polish	1 (3)
Russian	1 (3)
Other	3 (8)

3.3. Community based therapy - family and community therapist responses

In terms of the specific assessment within the community setting, there is some overlap between the role of PT and OT, although each professional may approach the same aspect of the assessment in a different context depending upon their training, and it would not be expected that every domain is assessed or managed at each visit. Table 5 (far left column) contains details of the most likely professional to conduct a particular role.

3.4. Overall therapy care

Both community and specialist neuromuscular teams review patients for reasons other than those listed in Tables 4 and 5. Specialist teams reported giving advice on weight and diet, transition, offering hands on treatment with signposting and advice on charity funding. Community teams give support to educational centres on training and school trips. They conduct some motor function assessment (North Star Ambulatory Assessment, NSAA) (although historically this has been the role of the specialist neuromuscular team), signpost sibling support and complete joint assessments with their specialist counterparts.

Table 2
Characteristics of therapy respondents.

Therapy Survey	N = 96 (%)
Physiotherapists based in a specialist neuromuscular centre	34 (35)
Community physiotherapists	54 (56)
Occupational Therapist based in a specialist neuromuscular centre	1 (1)
Community occupational therapists	7 (7)
Geographical location	
England	63 (66)
Wales	7 (7)
Northern Ireland	6 (6)
Scotland	20 (21)
Ethnicity	
White British	83 (87)
White Irish	4 (4)
Other white background	1 (1)
Asian/Asian British: Indian	1 (1)
Asian/Asian British: Pakistan	1 (1)
Black/Black British African	1 (1)
Black/Black British Caribbean	1 (1)
Arab	1 (1)
Jewish	1 (1)
Other	2 (2)
Age group therapist involved with	
Children only	82 (85)
Adults only	7 (7)
Both children and adults	7 (7)
Frequency of contact with individuals living with DMD	
At least every week	29 (30)
More than once a month but not every week	37 (39)
A few times a year	26 (27)
None currently, on caseload or needs basis	3 (3)

Table 3
Access and frequency of appointments with therapists from Family Survey.

Type of Therapy	Yes, seen by	When seen, frequency of appointments	N = X (%)
See a PT at specialist NMC	29 (78 %)	More often than every 6 months	5 (17)
Majority face to face in hospital clinic		Approximately every 6 months*	18 (62)
		Approximately once a year	6 (21)
See an OT at a specialist NMC	11 (30 %)	Usually at every appointment	8 (80)
Majority face to face in hospital clinic or in educational setting		Sometimes	1 (10)
See a community PT	25 (67 %)	Once a month	4 (16)
Majority: Face to face in home, school, hospital		Every 3 months	10 (40)
		Once or twice a year	8 (32)
		Blocks of treatment	2 (8)
		Only when requested	1 (4)
See a community OT	25 (67 %)	Once a month	2 (8)
Majority: Face to face in home, school, hospital		Every 3 months	5 (20)
		Once or twice a year	11 (44)
		Blocks of treatment	2 (8)
		Only when requested	7 (28)

NMC = Neuromuscular Centre, PT = Physiotherapist, OT = Occupational Therapist. *Recommended frequency in SoC.

3.5. *How satisfied are parents/carers and therapists with the care they receive or offer, what is important and how can therapy SoC be improved*

From the Family Survey, more than 80 % agree or strongly agree that it's important to them to know:

- what assessments are being done and why
- how current assessments compare to previous assessments
- that they receive advice on managing contractures and exercise

Table 4
Parental and Therapist report on what takes place at **specialist** neuromuscular therapy appointments – list based on SoC recommendations.

Role	What does the therapist do (assess and management) usually or sometimes at each appointment (where applicable)	Specialist neuromuscular PT [a]Usually/sometimes performs ([b]Not applicable)		Specialist neuromuscular OT Usually/sometimes performs Parent report %	
		Parent report% n = 29 [a]Usually/ sometimes	**Therapy Report %** n = 34 [a]Usually/ sometimes	**Parent report %** n = 10	**Therapy Report %** n = 1
Joint	Ask about changes	79/14	100/0	80/10	100
Joint	Checks range of motion	83/10	88/12	78/111 (10)	100
Joint	Checks arm function	83/10	88/6	44/44 (10)	100
Joint	Asks about exercise and activity	76/14	97/3	40/50	100/0
Joint	Asked about energy levels and fatigue	59/21	97/3	30/50	0/100
Joint	Asked/assesses about risk of falls	50/15 (10)	78/22 (3)	40/50	100/0
Joint	Checks or asked about specialist equipment	61/13 (15)	91/9	56/44 (10)	100/0
Joint	Check or asked about wheelchair	57/19 (25)	88/12	71/14 (30)	100/0
Joint	Get in and out of wheelchair (transfers)	33/22 (38)	91/6	50/17 (40)	100/0
Joint	Fits splint or plaster for tight joints	27/32 (24)	30/13 (32)	0/100	0/100
Joint	Extra help/ referrals	37/37 (7)	50/50	30/50	0/100
OT	Ask about home setting (including environmental adaptations)			71/29 (30)	100/0
OT	Checks hand function			67/11 (10)	100
OT	Learning and attention			40/30	0/100
OT	Emotional or mental health			30/30	100/0
OT	Participation			30/40	0/100
PT	Checks level of function	76/10	88/6		
PT	Gives or is given written advice (at or after an appointment)	36/44 (14)	48/48 (3)		
PT	Checks spine and posture	76/14	94/3 (3)		
PT	Asks about pain or cramps	72/14	88/12	40/50	100/0
PT	Ask about education or work setting (including environmental adaptations)		85/15	63/38 (20)	100/0
PT	Show stretches or exercises	41/31	44/56		
PT	Advice on exercise activity	45/28	68/32	30/60	100/0

(*continued on next page*)

Table 4 (*continued*)

Role	What does the therapist do (assess and management) usually or sometimes at each appointment (where applicable)	Specialist neuromuscular PT [a]Usually/sometimes performs ([b]Not applicable)		Specialist neuromuscular OT Usually/sometimes performs Parent report %	
		Parent report% n = 29 [a]Usually/ sometimes	**Therapy Report %** n = 34 [a]Usually/ sometimes	**Parent report %** n = 10	**Therapy Report %** n = 1
	or skills of independence				
PT	Advice on fatigue and pacing		68/32		
PT	Respiratory Assessment	36/32 (14)	31/19 (16)		
PT	Another team perform respiratory assessment		56/22 (21)		
PT	Advice equipment for breathing	31/69 (55)			

Joint = Often shared or blurred distinction on who conducts this part of care.
OT Usually OT led but may be conducted by PT.
PT = Usually PT lead but maybe be conducted by OT.

[a] Usually or sometimes as opposed to rarely or never (4 response options given in survey).

[b] Not applicable. If the therapist or parent stated it was not applicable to their role or to the young person with DMD these responses were deducted from the total and expressed as a percentage.

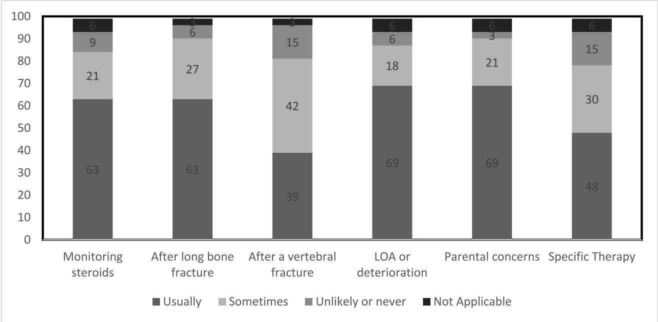

Fig. 1. Bar chart giving frequencies (as a percentage) of circumstances when a specialist neuromuscular therapist might see a patient more often
Monitoring steroids = starting or monitoring significant change in steroid regime/dose
LOA = Loss of ambulation
Parental concerns = additional concerns arising that require attention before next regular clinic appointment.

More than 70 % agree or strongly agree that it's important that:

- they receive information in writing
- the information is shared with other teams
- they see the same therapy staff
- Those staff understand the specific needs of those with DMD.

Fig. 2 and Table 6 summarise satisfaction with delivery of care from a family and therapist perspective as well as the importance placed on certain aspects of the care received or given.

Table 5
Family and Therapist report on what takes place at community appointments based on SoC recommendations and expert opinion.

Role	What does the therapist do (assess and management) usually or sometimes at each appointment (where applicable)	Community PT [a]Usually/ sometimes performs ([b]Not applicable)		Community OT Usually/ sometimes performs Parent report % (Therapy Report n = 7)	
		Parent report% n = 25 usually/ sometimes	**Therapy Report %** n = 54 usually/ sometimes	**Parent report%** n = 22 usually/ sometimes	**Therapy Report %** n = 7 susually/ sometimes
Joint	Ask about changes	68/24	91/9	70/17	100/0
Joint	Checks range of motion	64/24	63/33	36/32	29/14
Joint	Checks level of function	27/23 (12)	50/44	46/23	43/57
Joint	Asked/assesses about risk of falls	38/24 (16)	24/56 (7)	38/19 (5)	17/17/ (17)
Joint	Checks or asked about specialist equipment	56/16	72/28	64/23	83/17 (14)
Joint	Check or asked about wheelchair	42/26 (24)	59/37 (6)	58/16 (14)	43/57
Joint	Get in and out of wheelchair (transfers)	41/18 (32)	24/57	41/29 (23)	50/33 (14)
Joint	Changes the stretches or exercises	48/24	19/80	32/32	29/43
Joint	Fits splint or plaster for tight joints Usually PT - lower limb, OT - Upper limb	39/33 (25)	10/80 (9)		33/66 (14)
OT	Checks hand function			55/23	43/57
OT	Ask about home setting (including environmental adaptations)			59/27	43/43
OT	Ask about education or work setting (including environmental adaptations)			68/16 (14)	66/33
OT	Learning and attention			46/23	33/50
OT	Emotional or mental health			41/23	29/43
OT	Participation			45/27	71/29
PT	Checks spine and posture	44/36	54/41		
PT	Asks about pain or cramps	36/32	65/30		
PT	Asks about exercise and activity	60/28	74/22		
PT	Asked about energy levels and fatigue	48/36	63/33		
PT	Show stretches or exercises or activities	60/24	57/43	36/32	29/29

(*continued on next page*)

Table 5 (*continued*)

Role	What does the therapist do (assess and management) usually or sometimes at each appointment (where applicable)	Community PT [a]Usually/ sometimes performs ([b]Not applicable)		Community OT Usually/ sometimes performs Parent report % (Therapy Report n = 7)	
		Parent report% n = 25 usually/ sometimes	**Therapy Report %** n = 54 usually/ sometimes	**Parent report%** n = 22 usually/ sometimes	**Therapy Report %** n = 7 susually/ sometimes
PT	Advice on fatigue and pacing	52/27 (8)	26/63		
PT	Help with pain management	36/27 (12)	17/69		
PT	Offer/refer to hydrotherapy		9/66 (19)		
PT	Offer or refer to exercise classes		1/31 (35)		
PT	Offer chest physiotherapy/ respiratory advice	28/28 (56)	13/49 (17)		

Joint = Often shared or blurred distinction on who conducts this part of care.
OT Usually OT led is available but may be conducted by PT.
PT = Usually PT lead but maybe be conducted by OT.

[a] Usually or sometimes performs as opposed to rarely or never.

[b] Not applicable. If the therapist or family stated it was not applicable to their role or relevant to their child these responses were deducted from the total shown as a percentage.

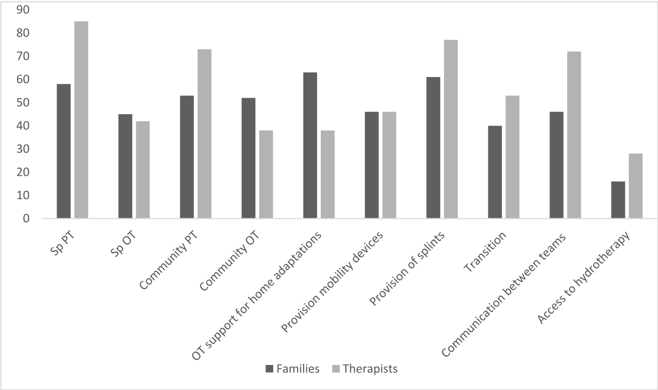

Fig. 2. Percentage of parents/carers either very satisfied or satisfied with care they receive, and percentage of therapists very satisfied or satisfied with the care they deliver (as opposed to neither satisfied or dissatisfied, dissatisfied or very dissatisfied).

3.6. Qualitative analysis results

Key themes identified from the Family Survey as well as the Therapist Survey included appointments, communication and training.

3.6.1. Appointments

Duration: 44% (15/34) of NM PTs reported that improvements to delivery of SoC would include longer appointment times with 32% (11/34) saying this would give the patient more time to ask questions and for the therapist to offer advice and give information. The OT reported that medical/MDT appointments could be improved by offering more time (perhaps with just one member of the MDT team) for issues such as career options or relationships.

Table 6
Percentage of therapy respondents who strongly agree or agree with the statement on 'how important it is to me that ….'

It is important to me that …	Specialist NMC PT (n = 34)	Specialist NMC OT[a] (n = 1)	Community PT (n = 54)	Community OT (n = 7)
I have training in all the outcome measures	97	100	76	72
I have time to talk to the patient about other issues	97	100	96	100
That I give advice on stretches and managing tight joints	100	100	98	57
That I give advice on exercise and activity (PT) or daily living (OT)	97	100	100	100
That information is written down	97	100	78	72
That information is shared with the community or specialist team	97	100	76	72
That the patient sees me at each visit	62	100	89	72
That I understand the learning and social needs of the patient	100	100	94	100

Important percentages included responses that either strongly agree or agree with the statement as opposed to neither agree or disagree, disagree or strongly disagree.

[a] Limited response given scarcity of NMC OTs.

Content of the appointments: The importance of therapy input (rather than just assessments) was recognised. There were comments from therapists expressing a desire to deliver treatment rather than just an assessment and advice (n = 3). Families mentioned that assessments should be relevant to the individual boy but not necessarily the primary focus of an appointment.

Therapists highlighted that it was important to provide emotional support for boys and families and train relevant educational staff and carers. Regarding community care, there was some evidence of frustration from families with how little therapy is offered. This may be due to misalignment of perception of care between families and therapists with therapists seeing their role in upskilling individuals who are involved with care every day and families expecting active hands-on treatment from a qualified therapist more often. Improving education and expectations of care would be of value here.

Timing: Some families would like their own appointment with the physiotherapist as opposed to a multidisciplinary appointment, and 3 mentioned they much prefer a morning appointment due to higher energy levels. Others (n = 2) prefer a later appointment to fit around siblings and the individual's morning routine.

> "The advice and follow up is the most valuable thing for the patient not the assessment itself". [Parent]

Multi-disciplinary appointments: Neuromuscular PTs commented it was important that families do not need to keep repeating information during one appointment or that assessments are duplicated. They saw this as a benefit of a coordinated MDT. Families said it was important that they are listened to, and concerns are addressed directly. This was reflected in the community setting where therapists identified the need for flexibility with appointments but also working to reduce burden by having joint appointments with other therapists including speech and language therapy. Families agreed that joint PT and OT appointments were helpful in the community. Families found the burden of having to chase equipment and support was exhausting

> “Physiotherapy is a daily routine. It's left to the parents. More professional active physio and hydrotherapy should be offered within the community or hospital, without question” [Parent]

> ‘We are aware of best practice, but it is impossible to provide this for all children and families all the time. Staff are incredibly stressed and stretched within NHS. I have the skills I just don't have time to offer best service and go home on time (ever).’ [Community Physiotherapist]

Communication: Feedback from all therapists and families highlighted that consistent information should be shared across all settings which includes parents/carers and teachers. This needs to work between specialist and community teams and between parents and educational establishments.

> “I feel there is not enough dialogue between the neuromuscular centre and community so sometimes there are exercises we are given that the other does not agree with. Very confusing.”[Parent]

> “When my child is seen in school, I get no update on how it went. I only know he's had physio if I ask his teacher.”[Parent]

> Community teams expressed some frustration with specialist centres.

> ‘Sometimes tertiary centres appear to want community therapy to embed their programmes and not add anything else, but communication by hospitals to community is poor and we have to find out information from parents most of the time’. [Parent]

> *It is* important *that we “have the support of the specialist neuromuscular team”* when providing care. [Community physiotherapist]

Not all feedback was negative, however, improving communication was a common theme (regularity, up to date, consistent) and both therapists and parents felt better communication would help ensure advice on changes or updated management information is supplied to everyone in a timely manner. In general, it was considered important that this information was written down and that the specialist NMC could offer a parent-friendly summary of the assessment results.

Community staff commented they would like more joint working with the specialist team and that a more structured guide to care would be helpful to improve communication. In addition, it was noted that parents need improved information on what OTs can offer as without this knowledge they don't know what to ask for.

Additional comments were included, relating to **gaps in service provision** set against recommendations in the international SoC document and the importance of the role of commissioning services in this situation. Geographical discrepancies in the delivery of SoC were also highlighted

> [it would be useful to] *“Highlight gaps in commissioning. For example, in our area there is no community respiratory support. Also,* [provide and highlight] *standards of care so when we refer for a powered chair or orthotics (which are outside of our service, so we have no influence) then* [we are] *more likely get an agreement to provide clinically appropriate orthotics/chair”. [Community physiotherapist]*

> ‘I think there is a discrepancy of services across the UK for people with DMD and really it should not matter where you live as to what input you get’ [Community Physiotherapist]

Other issues raised related to the **time it can take to deliver best practice** with regard to provision of an educational health care plan (EHCP) and equipment.

> ‘We have struggled with EHCP requests as, although we know the medical outcome and the increased difficulties the child will have in terms of accessing education without support etc, they have been rejected when the child is fully mobile meaning that you have to reapply when the young person is going or has gone off their feet (given this takes months to complete) and then there is a period of time (when it is most needed as there is change occurring) where additional support is not being provided.

> ‘The long waits of social services OTs also mean that relevant adaptations for families take much longer than it should and are often met with issues (e.g. don't include needs of other family members; family have to part fund)’

3.6.2. *Training*

The need of increased knowledge of DMD within the community team was highlighted by both parents/patients and community therapists. Among community therapists, 80% reported that they were either very interested or interested in the following training: assessments for DMD (79%), management of DMD (84%), respiratory care (70 %), orthotics (68%) and wheelchair requirements for DMD (66 %). In addition, therapists asked for input on exercise in DMD, transition, managing behavioural issues, end of life care and discussion. Training could be delivered face to face (29 %), remotely (34%), via online module (23 %) or via remote testing (14%).

4. Discussion

This study aimed to gather information about the current delivery of SoC in the UK regarding physiotherapy and occupational therapy from different perspectives, that of the family and from the therapists involved in the community and in the specialist NMC.

The study provides clear evidence that the current provision of therapy care both in specialist NMC and in the community does not meet the published SoC guidelines [8]. This is particularly evident in the information in Tables 4–6 where not all individuals are seen every 6 months, and specialist neuromuscular and community provision may be limited both in terms of content and frequency. This echoes earlier surveys where children living with DMD were reported as not receiving therapy as per SoC [15]. Both parents and therapists are dissatisfied with the frequency of appointments and intervention and a proportion of families report they are not accessing any physiotherapy in specialist centres (23 %) and/or in the community (33 %). Therapists who participated in this survey were also dissatisfied with delivery of care.

There are valuable suggestions from the two surveys for ways in which care could be improved around the themes of content of appointments and communication as well as education and provision of care. Implementing improvements is not always straightforward but a key step is establishing a detailed evidence-based guide to SoC delivery. This has been one of the roles of DMD Care UK project, a nationwide collaborative initiative between clinicians and patient organisation, to improve and harmonise best care for everyone living with DMD in the UK. Within the DMD Care UK project, a therapy working group (TWG) was established to develop UK-specific, practical therapy guidelines based on the international SoC. This TWG met on 11 occasions and subgroups were set up to write sections of the guide based on stages and topics outlined in the SoC (For example; early ambulant, late ambulant, early non ambulant etc; occupational therapy, wheelchairs etc). Over three years these were edited, updated based on emerging evidence as well as the results of the surveys described here, referenced and peer reviewed. Finally, they have been endorsed by the North Star Network and APCP Neuromuscular Group. This Therapy Guide works in conjunction with the published Adult Therapy Guide [13] and will be disseminated via the DMD Care UK website as well as linked to this publication (see conclusion) The guidelines have been developed as a online resource to allow continuous updates as new evidence comes to light, new therapies are approved and as other guidelines for specific, relevant areas of care (e.g. orthopaedics, spinal care) are developed and published as part of the DMD Care UK project.

4.1. Limitations of the study

The results of these surveys highlight differences between parent/patient and therapy experiences. This is in part because parents report a single user experience of services so the content of any care will depend on their stage and circumstances whereas therapy staff reported their experience with a heterogeneous cohort of individuals with DMD.

The study had a good response rate from therapists, however, the number of responses from parents and individuals affected by DMD was limited and the findings should be confirmed by a larger study. The survey was distributed through patient organisations and inevitably included a bias on the responders who are more likely to represent more engaged and informed families; the parent population surveyed also had a higher level of education compared to national average. A further study more effectively recruiting patients through clinics to ensure a more representative socioeconomic cohort would be useful. We are

Regular appointments are critical (6 monthly SP NEUROMUSCULAR and more frequent in community) and should be more frequent if necessary*.
Discourage ‘as requested appointments’ or ‘episodes of care’ where possible as this results in reactive management rather than proactive management.
*More frequently: New diagnosis, long bone fracture, vertebral fracture, steroid changes, parental concerns significant loss of function, specific therapy input

Assess and manage. Whole team should be aware of the link between assessment and management and therefore if things change how management might need to be updated.
Time spent with individual’s should be **driven by changes and issues**
Issues include: Function – upper and lower limbs), mobility including transfers and risk of falls, pain & cramps, contractures, orthotics, spine /posture, activity & exercise, fatigue, equipment, wheelchair, respiratory as well as other forms of support (emotional, learning, participation)

Content of Therapy Appointments
Families should be aware that **not all assessments or forms of management need to happen at every appointment**. This is particularly true in the community where issues often relate to a particular location. E.g. Home or place of education
Standard assessments should be used where possible for a specific issue. E.g. North Star Ambulatory Assessment (NSAA) to measure function and response to steroids or a specific postural assessment for evaluating specialist seating.
Advice should be given even if it is to reassure the family that they should continue with current management. This should include hands on advice and instruction.
Structure of appointments should help families avoid repeating the same verbal information too many times and making use of joint appointments to save time and improve communication. This may be a MDT format but provision should be made for discreet conversations where necessary

Communication.
Families want to know what is being assessed and why and may want to know the scores / results of any tests, but this should not be assumed.
Having reliable methods for families to contact the therapy team is critical, be it a telephone number which takes messages and / or specific or team emails.
Communication could be written advice, photos, videos on family phones, emails, phone calls. Do families know where the teams are based and how to contact them? Have you informed the family of changes.
Use joint working to improve communication and knowledge amongst teams
Use the opportunity when demonstrating therapy to communicate this to the wider team effectively (other family members, community, educational establishment)
The community would benefit from easier ways to share information between teams and families and update these quickly and efficiently.
Make it clearer to families the role of OT in all areas and how to access and source OT support.

Additional recommendations. Younger boys are best seen earlier in the day. Older children / young people often later appointments as getting up and out is a lengthy process
Having access to a hoist if needed when they attend clinic so assessments can be conducted properly.
Deliver a strong Transition Team to support the process of transition from child to adult health and social care

Resources and education
A more detailed guide to care could improve knowledge and care of those with DMD
Community therapists should be offered more opportunities for learning and peer support in specific therapies
Education of therapists should include the learning and behavioural issues

Service provision
Specialist therapy teams and community teams need support on comparing current delivery to SoC and using the information on inadequate provision to commission better services or access to equipment etc.
Business plan support could help specialist teams secure funding for therapy provision

Fig. 3. Key recommendations improving SoC delivery for Therapy for DMD in the UK.

aware the survey was only made available in English, and this is a further limitation although we did receive responses from those whose native language was not English.

Finally, whilst the number of therapist responses was good, a poor representation of specialist OT views is evident, although it reflects the known limited numbers of specialist neuromuscular OTs.

5. Conclusion

The results of the survey have clearly identified ways in which delivery of Therapy SoC could be improved (Fig. 3). Some of these include the upskilling and education of therapists and families. This has already been partly addressed by the recent publication of a DMD Care UK Therapy Guide for DMD endorsed by the Association of Paediatric Chartered Physiotherapists (APCP) alongside an accompanying Family Guide. Other areas for improvement may take longer to deliver as they involve supporting commissioning of services and require additional funding, both of which can involve lengthy processes. However, the established North Star Neuromuscular Network as well as DMD Care UK, can help in addressing these gaps.

While the work has been conducted in the UK with a focus on national health care service provision, the outcomes and recommendations could have a broader, international impact.

Declaration of interests

AGM has served on medical/scientific advisory boards for Regenxbio, Sarepta, Biogen and Roche; and has received fees for consulting and training services for Biogen, Roche, Novartis, Biohaven, PTC, Sarepta, Italfarmaco, Dyne, Pfizer, Summit, Catabasis, Capricor, Santhera, Vision, Lysogene, Modis, Amicus, Taysha, Antisense, Analysis Group, MDUK and DUK.

CM has received fees for consulting and training services for Dyne therapeutics, Avidity Biosciences, Pep Gen Inc, Arthex Biotech, and Lupin Neurosciences.

RR and HM have received fees for providing expertise to creating a therapy app for Novartis.

LP has received fees for serving on advisory panels for Roche and Italfarmaco and for providing training for PTC. She has received consulting fees from Novartis.

MDM has worked on a consultancy basis for various sponsors including PTC Therapeutics, Italfarmaco, Roche and Biogen. She is a trainer with ATOM International.

AP: Received fees for consulting and training services from Orphan Reach for Reneo, clinician consultation fee for DMD study on meaningful change for Roche.

HD & SW received fees for advisory boards for Roche.

JMS has received fees for consulting and training services for Sarepta, Vertex and Italfarmaco.

CT, AW, AT, DW, NB, MM, SB, EG, SN, AK, ST, AJ have no declarations of interest.

MKJ has served on scientific advisory boards for Sarepta, Roche, Pfizer, Sanofi, Amicus and Genethon with fees paid to Newcastle University. Meredith has received fees for consulting and training services for ATOM International including services for PTC, Sarepta, Italfarmaco, Dyne, Pfizer, Summit, Catabasis, Capricor, Santhera, Amicus, NSPharma, Antisense, Edgewise and BridgeBio.

MG reports currently or previously acting as CI/PI for clinical trials sponsored by Dyne, Pfizer, Italfarmaco, Edgewise, Roche, Santhera, ReveraGen, Dynacure; participating in advisory boards for Pfizer, NS Pharma, Dyne (consultancies through Newcastle University); to have act as study chair for a study sponsored by ReveraGen (no financial benefits); to have research collaborations with ReveraGen, Sarepta and Edgewise and receiving speaker honoraria from Sarepa, Italfarmaco, Novartis, Roche and Dyne.

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Appendix A. Supplementary data

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