



---

# All Party Parliamentary Group for Muscular Dystrophy

**Impact of NHS reforms on access to neuromuscular services**

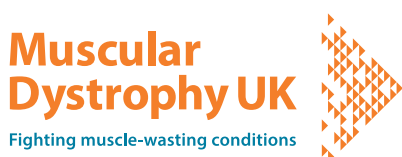
---

March 2015

## Contents

---

Muscular Dystrophy UK	02
Foreword	03
Executive summary	04
Recommendations	05
The All Party Parliamentary Group for Muscular Dystrophy	07
Acknowledgments	07
Methodology	07
Summary of evidence	08
Appendix	17
• Witnesses	17
• Terms of reference	17
• The importance of early diagnosis	18
• Developing neuromuscular networks across England	18
• Access to Translarna – a new treatment for Duchenne muscular dystrophy	18
Additional (written) evidence	19
References	19



## Muscular Dystrophy UK

---

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

We support high quality research to find effective treatments and cures; and lead the drive for faster access to emerging treatments for UK families.

We ensure everyone has the specialist NHS care and support they need, with the right help at the right time, wherever they live.

We provide a range of services and opportunities to help individuals and their families live as independently as possible.

We know we can beat muscle-wasting conditions more quickly by working together and hope you will join us.

## Foreword from Dave Anderson MP

---

There are over 60 different types of muscular dystrophy and related neuromuscular conditions, which lead to muscle-wasting and severe muscle weakness. People affected by these complex and rare muscle-wasting conditions require specialist multi-disciplinary care and support, which should be supported by a network of specialist centres and accessed as locally as possible.

All of these rare conditions are degenerative and this is something I myself understand, having lost my brother, sister, a nephew and two nieces to myotonic dystrophy.

In 2013, the Government introduced major reforms to the NHS in England. This has led to dramatic changes to the way NHS services for people with muscle-wasting conditions are commissioned, as well as significant changes to how new treatments for the conditions are funded and approved.

After these reforms were introduced, the All Party Parliamentary Group (APPG for Muscular Dystrophy) was contacted by patient groups, specialist clinicians and families affected by muscle-wasting conditions. We were concerned to hear that, far from simplifying the NHS, the reforms had resulted in complexity and confusion which was impacting on patient access to vital services and equipment.

For these reasons, the APPG for Muscular Dystrophy decided to conduct an inquiry to determine the impact of the reforms on access to services for people with muscle-wasting conditions. Over the course of the last six months we have taken evidence from NHS commissioners, expert healthcare professionals and patients and families from across England.

I thank everyone who has taken part in the inquiry for their invaluable contributions, which have helped to inform my thoughts and those of my parliamentary colleagues in the group.

One of the new responsibilities placed on NHS England in the NHS reforms was the assessment of new treatments. A new system, as well as criteria for this purpose, therefore had to be developed by an organisation that had very limited experience in this complex area, and would soon be taking major decisions on the funding and availability of new treatments.

After several months of progress through NHS England's approval and funding procedures, the assessment of a new treatment for Duchenne muscular dystrophy – Translarna – ground to a halt in December 2014. This was owing to the threat of a legal challenge over the criteria NHS England had adopted to assess new treatments. Translarna was not the only victim of this flawed new infrastructure: access to Vimizim, which can treat the devastating condition, Morquio, was also delayed, as was access to Everolimus to treat tuberous sclerosis.

This has delayed a decision on whether to fund and approve these drugs and to make them available to patients. It has caused understandable concern, upset and anger for families affected by Duchenne muscular dystrophy. This concern is shared by my parliamentary colleagues and me. Time is of the essence, as boys who could benefit from Translarna still need to be walking in order to be eligible for the treatment. I very much hope that the Department of Health and NHS England reach a decision to fund Translarna without further delays to the process.

Finally, I would like to refer back to the Walton Report, which this group produced in August 2009. I know the NHS checked assessments on compliance against the recommendations in that report, and I look forward to NHS England treating this report with the same consideration and importance.



Dave Anderson MP  
**Chair of the All Party Parliamentary Group for Muscular Dystrophy**

## Executive summary

---

The Health and Social Care Act 2012 delivered major structural reforms to the NHS in England, relating to the commissioning of services and potential treatments for muscle-wasting conditions.

Shortly after this, the Government published its UK Strategy for Rare Diseases, aimed at identifying and preventing rare diseases, supporting research, enabling early detection and diagnosis and co-ordinating specialist care.

It was against this backdrop that the APPG launched its inquiry on the impact of NHS reforms on access to services for people living with muscle-wasting conditions.

Following this extensive inquiry, we are concerned that the Government's reforms have in many cases not resulted in improved care. In some instances these reforms have made it even harder for patients to access support as a result of significant regional variation in the commissioning and funding of services.

Key findings include:

- lengthy delays in NHS England's assessment of new treatments for muscle-wasting conditions, resulting in delays to patients' access and agonising waits for families
- people going without crucial respiratory support, such as cough assist machines, because of a lack of clear guidance as to which bodies in the NHS fund these vital aspects of care
- Clinical Commissioning Groups in some areas of the country refusing to fund sessions of specialist neuromuscular physiotherapy, despite the clear clinical value of these services
- Clinical Commissioning Groups, now responsible for commissioning wheelchair services, are not providing sufficient funding for people to get the right wheelchair at the right time.
- leading consultants reporting their services are at breaking point, as a result of confusion and complexity at the heart of NHS commissioning

Urgent action is clearly required to improve specialist care and support for people living with muscle-wasting conditions, and to speed up access to treatments.

**The APPG for Muscular Dystrophy has identified areas for improvement within the new NHS structures. These include:**

- the re-introduction of a ring-fenced fund for rare disease drugs, which was abolished following NHS reforms
- a simple, streamlined mechanism within NHS England for assessing new drugs, which allows patients to access new treatments without having to face bureaucracy and delay
- a clear and binding instruction from the Secretary of State for Health and NHS England on which neuromuscular services and equipment are commissioned by Clinical Commissioning Groups, and which are commissioned nationally by NHS England, to aid collaborative commissioning
- a named neuromuscular lead commissioner in each of the four NHS regions of England with responsibility for co-ordinating commissioning and addressing difficulties and uncertainties that arise in the process
- improving the assessment, delivery and maintenance of wheelchairs and equipment to specifically meet patient needs, a responsibility which now lies with Clinical Commissioning Groups
- better co-ordination of the transition from paediatric to adult services.

The APPG for Muscular Dystrophy calls on the Government, NHS England and organisations identified in the recommendations to take fast and effective action to prioritise the improvements to specialist neuromuscular care and support and to put in place the mechanisms for fast access to emerging new treatments.

As muscle-wasting conditions are degenerative, time is of the essence. The APPG urges the Department of Health and NHS England to ensure that Translarna is available as soon as possible to those who are eligible and can benefit from the treatment.

# Recommendations

---

## 1. Access to new treatments

- a. The Health and Social Care Act 2012 placed a new responsibility on NHS England for the assessment of new treatments. Far from streamlining and simplifying the drug appraisals process, these reforms resulted in a complex system which had to be halted all together following the threat of a legal challenge in December 2014. This has significantly delayed access to a treatment for Duchenne muscular dystrophy, Translarna. **We call on NHS England to introduce a streamlined process for assessing new drugs, which allows patients to access new treatments without having to face bureaucracy and delay.**
- b. Delays to the assessment of Translarna have caused significant anxiety for families whose children could benefit from the treatment. **We recommend that NHS England issues a commitment to decide on the approval and funding for Translarna as soon as possible and at the latest by June 2015.**
- c. As patient registries are vital to developing clinical trial infrastructure in the UK, **we therefore call on the National Institute for Health Research to support the development of additional patient registries for muscle-wasting conditions through statutory funding, as well as national clinical trial networks for rare conditions.**
- d. The ring-fenced fund for rare disease drugs was abolished in 2012, following the reforms to the NHS. Since then, Translarna has emerged as a treatment for Duchenne muscular dystrophy and other potential treatments are on the horizon for this and other muscle-wasting conditions. **We recommend that the Government re-introduces a ring-fenced fund for rare disease drugs.**

## 2. Early diagnosis and co-ordinating access to services through neuromuscular networks

- a. The creation of Clinical Commissioning Groups has given GPs a central role in the commissioning of some services for people affected by muscle-wasting conditions. As a result, they need to have a secure knowledge of these rare and complex conditions. We recognise efforts by Muscular Dystrophy UK and the Royal College of General Practitioners to increase knowledge and understanding of muscle-wasting conditions among GPs through a new GP online training module. **We call on all Clinical Commissioning Groups to ensure protected learning on muscle-wasting conditions for GPs and to publicise the newly-developed GP online training module.**
- b. The neuromuscular network in the South West is a model of best practice to develop similar managed clinical networks across England. This network, known as an ‘Operational Delivery Network’, helps co-ordinate services for patients with muscle-wasting conditions and must be replicated across the country. **We call for NHS England and Trusts to bring together neuromuscular services in each region, to create new, funded networks in each area of England.**

## 3. Improving neuromuscular services in the context of the new NHS set-up

- a. NHS England’s neuromuscular service specifications are designed to outline the specialisms and support that a specialist neuromuscular service should contain. However, owing to regional interpretations of the guidance provided and a lack of clear guidelines on which aspects of the service should be commissioned locally and which nationally, significant local variations in care have developed. **We call for clear and binding instruction from the Secretary of State for Health and NHS England on which bodies in the NHS have responsibility for the funding and commissioning of these services. This will in turn aid collaborative commissioning.**
- b. Following the publication of the APPG’s 2009 Walton Report, a national neuromuscular lead commissioner was appointed in the NHS. In order to address some of the current confusion and uncertainty in the commissioning of neuromuscular services, we believe this model should be replicated regionally. **We therefore call on NHS England to appoint a neuromuscular lead commissioner in each of the four NHS regions in England. This commissioner can be the first point of contact when difficulties arise in the commissioning of neuromuscular services.**
- c. We welcome Muscular Dystrophy UK’s Right to Breathe report published in February 2015 highlighting the need for access to specialist respiratory support and equipment. We note the Minister for Public Health, Jane Ellison MP, recently confirmed that cough assist machines were the commissioning responsibility of Clinical Commissioning Groups. **We call on Clinical Commissioning Groups to commit to funding and providing cough assist machines for all people with muscle-wasting conditions where the need has been identified and recommended.**
- d. Muscle-wasting conditions are rare, complex and distinct, needing input from a range of specialists. While conditions such as cystic fibrosis are represented on one Clinical Reference Group, muscle-wasting conditions are

currently divided between paediatric neurosciences and adult neurosciences Clinical Reference Groups. **We call on NHS England to ensure that muscle-wasting conditions are represented on one Clinical Reference Group, to ensure a joined-up approach.**

- e. We were alarmed to learn during the inquiry that some Clinical Commissioning Groups were not funding specialist physiotherapy sessions for patients wishing to access services at the NeuroMuscular Centre (NMC) in Cheshire and NMC Midlands. **We call on all Clinical Commissioning Groups to recognise the value of specialist physiotherapy and allow all neuromuscular patients in their area to access these services.**
- f. The specific needs of people with muscle-wasting conditions must be met when assessing the most appropriate wheelchair and equipment provision. **We call on Clinical Commissioning Groups, who now have responsibility for this area, to conduct adequate assessments to reflect the complex needs of patients with muscle-wasting conditions and allocate protected funding for specialist wheelchair provision.**
- g. In 2013, the Government published the UK Strategy for Rare Diseases, aimed at identifying and preventing rare diseases, supporting research, enabling early detection and diagnosis and co-ordinating specialist care. NHS England must now work to turn this vision into a reality. **We call on the NHS to support a network of at least 10 specialist centres across the UK and support them to evolve into clinical and research centres of excellence. This would ensure that comprehensive and consistent care was delivered by multi-disciplinary teams across the country, and would also mean that Muscle Centres and clinics were able to deliver forthcoming treatments with the support of fully trained staff.**
- h. Difficulties in accessing hydrotherapy pools are a recurrent theme throughout the country. **We call on NHS England to work in partnership with Muscular Dystrophy UK to compile an audit of hydrotherapy pools in order to improve access to hydrotherapy for people with muscle-wasting conditions.**
- i. Transition from paediatric to adult services has been highlighted by NHS England commissioners as an area of significant service development required across the country. **We recommend that NHS England host working groups in each of the NHS England regions with neuromuscular health professionals, patients and families to implement recommendations for improving transition services.**
- j. It is vital that steps are taken to offer specific psychological support to people with muscle-wasting conditions across England. **We recommend that NHS England work with education and professional organisations to develop an increased workforce who can provide the required support.**
- k. We understand that the National Institute for Health and Care Excellence (NICE) is developing a Clinical Guideline for 'uncommon neuromuscular conditions'. **We call on NICE to publish a timeline for the production of these Clinical Guidelines.**

#### 4. Gaps in specialist care

- a. In some areas of the country, the NHS reforms have led to previously earmarked investment being lost. We find it unacceptable that five years on from NHS commitment to invest in a paediatric neuromuscular consultant post in the West Midlands, there are still delays in implementing this investment. **We call on NHS England to work with Heart of England NHS Foundation Trust and Birmingham Children's Hospital NHS Foundation Trust to ensure that this commitment is honoured and the post advertised and recruited as a matter of urgency.**
- b. Shortly after the NHS reforms, new commissioners told families that funding for the South East Coast neuromuscular care advisor role for children was to be discontinued. It is essential that neuromuscular care advisor provision for children and their families in the South East Coast region not be lost with the changes to the hosting of the care co-ordinator role in the region. We recommend that NHS England work with Evelina Children's Hospital to address this potential shortfall, with a commitment to long-term provision for these patients. **We also call on NHS Trusts in London to increase Care Advisor support, which currently lags far behind other regions of England.**
- c. Support for adults in Lancashire with muscle-wasting conditions needs to be addressed. **We recommend that NHS Hospital Trusts in Lancashire collaborate to ensure that neuromuscular care advisor support for adults is put in place as soon as possible.**
- d. One neuromuscular care advisor for the whole of the North East region – an estimated patient population of 3,000 – is clearly not enough to meet the needs of people with muscle-wasting conditions. **We call on Newcastle upon Tyne Hospitals NHS Foundation Trust to address urgently the need to increase this vital support.**

## The All Party Parliamentary Group for Muscular Dystrophy

---

The All Party Parliamentary Group (APPG) for Muscular Dystrophy, chaired by Dave Anderson MP, is a cross-party group of MPs and Peers which raises the profile in Parliament of muscle-wasting conditions. The Secretariat of the group is provided by the Muscular Dystrophy UK.

### Acknowledgments

Below are the Twenty Qualifying Members of the All Party Parliamentary Group, as well as Parliamentarians who received oral evidence submissions:

Dave Anderson MP	(Labour, Blaydon) (Chairman)
Stuart Andrew MP	(Conservative, Pudsey)
Sir Kevin Barron MP	(Labour, Rother Valley)
Clive Betts MP	(Labour, Sheffield South East)
Steve Brine MP	(Conservative, Winchester)
Russell Brown MP	(Labour, Dumfries and Galloway)
Mark Durkan MP	(Social Democratic and Labour Party, Foyle)
Graham Evans MP	(Conservative, Weaver Vale)
Tim Farron MP	(Liberal Democrat, Westmorland and Lonsdale)
Dr Hywel Francis MP	(Labour, Aberavon)
Pat Glass MP	(Labour, North West Durham)
Mary Glindon MP	(Labour, North Tyneside)
Baroness Howe of Idlicote	(Crossbench)
Barbara Keeley MP	(Labour, Worsley and Eccles South)
John Leech MP	(Liberal Democrat, Manchester Withington)
Stephen Lloyd MP	(Liberal Democrat, Eastbourne)
Paul Maynard MP	(Conservative, Blackpool North and Cleveleys)
Baroness Masham of Ilton	(Crossbench)
Jason McCartney MP	(Conservative, Colne Valley)
John McDonnell MP	(Labour, Hayes and Harlington)
Anne Marie Morris MP	(Conservative, Newton Abbot)
Greg Mulholland MP	(Liberal Democrat, Leeds North West)
Caroline Nokes MP	(Conservative, Romsey and Southampton North)
Alison Seabeck MP	(Labour, Plymouth Devonport)
Baroness Thomas of Winchester	(Liberal Democrat)
Lord Walton of Detchant	(Crossbench)

**The APPG for Muscular Dystrophy wishes to thank Muscular Dystrophy UK for its administrative support in the organising and staging of evidence sessions, the gathering of written evidence and the production of a draft of this report.**

### Methodology

---

Between October 2014 and February 2015, we held three oral evidence sessions of the APPG for Muscular Dystrophy. Appendix 1 provides information on those who submitted evidence at each session. We also received written evidence from individuals and organisations with a specific interest in the inquiry's work. We are grateful to all those who assisted in our work.

## Summary of evidence

---

### First evidence session – 28 October 2014

#### Focus on the Midlands and East of England

##### Witnesses:

- **Carolyn Young** – Programme of Care manager – Trauma, NHS England (Midlands & East)
- **Dr Owen Jones** – Senior Service Specialist, Specialised Commissioning (Leicestershire and Lincolnshire Area Team), NHS England
- **Mel McFeeters** – Clinical Services Specialist (Leicestershire and Lincolnshire Area Team), NHS England
- **Dr Tracey Willis** – Consultant Paediatric Neurologist, Robert Jones and Agnes Hunt Orthopaedic Hospital NHS Foundation Trust

Further questions and comments were provided during the session by patients, family members and other healthcare professionals.

### Commissioning and funding of specialist care for people with muscle-wasting conditions

We heard from commissioners about adapting to the new commissioning arrangements and addressing funding concerns. In this way, patients living with muscle-wasting conditions will be able to access the specialist care and support they require.

Carolyn Young highlighted to us the ongoing issue of the provision of equipment – a challenge which remains:

*One of the biggest challenges that we had in 2013 and is potentially still there, but we are working on it, is around not only equipment such as cough assist, but around wheelchairs, home adaptations, communication aids, and environmental control type issues.<sup>i</sup>*

Dr Owen Jones described the balance that needs to be struck between finalising funding arrangements and ensuring that patients have specialist equipment when they need it:

*We've tried to address certain issues where people have debated who is funding what for things like specialist respiratory care, elements of communication aids. We have taken a view that we will try to make a judgement and agree to allow people to have the right kit or treatment time and then sort out who is paying for it afterwards.<sup>ii</sup>*

We were concerned about the lack of clarity in relation to the funding and provision of cough assist machines, further highlighted by Muscular Dystrophy UK's Right to Breathe report published in February 2015. Mel McFeeters commented:

*There are different commissioning arrangements, so for adults through the CCGs and certainly for children respiratory cough assists have been in the prescribed services manual for children. So, we have had lots of discussions about that and ongoing conversations in regard to that equipment at the moment, so that we can make sure that it is a standardised, streamlined process rather than one thing for adults, one thing for children.<sup>iii</sup>*

Dr Tracey Willis commented:

*At the moment, we are not having a problem, as I said, with funding and certainly moving children through to the adult services at Oswestry is a seamless transition because we run the same clinic with the same staff. Our referrals to respiratory and cardiac are not a problem for us but we do have issues sometimes with some of the equipment.<sup>iv</sup>*

We noted with concern the provision of services away from the neuroscience centres, with Dr Jones commenting:

*The main challenge for us is according to the rules we are commissioning things through a neuroscience centre and I'd be the first to acknowledge that there are massive numbers of patients who are probably not accessing the same service who happen to live some distance from the neuroscience centre.<sup>v</sup>*

Carolyn Young outlined for us the measures which neurosciences centres have to take if they do not fulfil the requirements of the neuroscience service specification:

*Any centres that did not meet the core requirements have had to deliver an action plan with very clear details about how they are going to meet their requirements with a very clear timetable, and that timetable cannot be later than 31 March next year [2015] and they will be assessed against that timetable.<sup>vi</sup>*



We received concerning evidence about the lack of co-operation from many Clinical Commissioning Groups (CCGs) to allow patients to access services at NMC Midlands. Stuart Sullivan, Executive Director of NMC Midlands, wrote to us:

*I would therefore request, on behalf of patients, that support is provided to patients to challenge these CCGs to ensure that they adhere to their commissioning responsibilities and funding responsibilities in terms of this group of patients so that services are funded appropriately and remain available.<sup>vii</sup>*

## Developing neuromuscular networks

Following the success of co-ordinating services, which has been evidenced from progress achieved by the South West Neuromuscular Network, the APPG challenged commissioners to outline plans for developing a neuromuscular network approach across the rest of England.

Dr Jones suggested that the forthcoming increase in neuromuscular care advisor support could help to increase the development of a network in the East Midlands in conjunction with existing neuromuscular health professionals:

*There was historically only one care advisor in the East Midlands, so we can't fund a network separately but what we can do is try and provide funding for three individuals that will be based in different geographical areas in the East Midlands and I would see those forming the hub with the consultant and therapy colleagues of the network.<sup>viii</sup>*

Carolyn Young spoke about the increasing collaboration between the hospitals in the East of England and ensuring there is specialist care and support for people with muscle-wasting conditions across the region:

*We have very much an informal network which meets twice yearly with the specialised centre as the hub and referring clinicians meeting together to discuss some of the longer term issues. We do come together two or three times a year as well through the [Muscular Dystrophy UK] Bridging the Gap project, which brings all of the parties and some of our patient representatives together as well. So I think although it's not a formal network we are very much beginning to establish an informal clinical network where they begin to liaise together and clinicians work between hospitals now, so rather than patients having to come into the centre we are now able to deliver outreach services to some of the local hospitals which has got to be an improvement to people having to travel longer distances.<sup>ix</sup>*

## Gaps in specialist care

We were shocked to hear that five years on from £400,000 investment in specialist neuromuscular care in the West Midlands, the paediatric neuromuscular consultant post has not been filled. This post was a significant component of the funding approved by the NHS West Midlands Specialised Commissioning Group, representing the 17 Primary Care Trusts in the region and working with the Strategic Health Authority.

We learned that the NHS reforms had had a detrimental impact on the funding availability for this vital post and this issue has been highlighted with Ministers and officials from the Department of Health. Carolyn Young told us:

*When NHS England was established and Primary Care Trusts were no more, there were no financial legacy arrangements passed on, so none of that money that was agreed in 2010 was part of the agreement of what was passed forward into the new world. So, I suppose that is number one, but secondly NHS England doesn't fund clinical posts as such within hospitals.<sup>x</sup>*

Dr Willis expressed her disappointment at the continued delay to the introduction of the post:

*When I joined I thought, great, we can get another neurologist with a neuromuscular interest who can be on the ground in the hospital all the time, pick up the ward reviews and referrals for the diagnoses as well as doing a multidisciplinary clinic – it doesn't matter if they are not up there with all their neuromuscular. They have got me and got Dr Roper to train them up over time and gain that experience so I actually did find two eligible consultants who were about to finish. They were really interested. This is what they wanted to do. Their training was in neurology and neuro-disability and neuro-rehab, great, but now one of them has gone to Australia because the money fell through and one's taken a post up in Newcastle. I stopped looking because the money has gone.*

*But we are struggling – this is a tertiary hospital, which sees thousands of children every year through Intensive Therapy Unit (ITU), some are undiagnosed with neuromuscular conditions but they come to ITU with respiratory problems. I'm only there one day a week. If they come on a Monday and get transferred somewhere else on a Wednesday, I don't see them and they go undiagnosed. It's something that just shouldn't be happening in a tertiary hospital.<sup>xi</sup>*

Naima Ali-Khan from Coventry commented:

*I am the mother of two children with a rare form of muscular dystrophy, SEPN-1, also known as rigid spine myopathy. Sufyaan is 10 years old and Hannah is 11. Hannah is also a carrier of Duchenne muscular dystrophy, making her [condition] rarer still. We have spent all of their lives, battling this unforgiving condition. Their childhoods lost to poor health, failing to reach their developmental milestones, failing to thrive. Battling recurrent respiratory infections and collapsed lungs, constant physiotherapy to prevent contractures, maintain mobility and manage scoliosis are a way of life. I've lost count of the number of appointments we've attended at numerous hospitals with various healthcare professionals, all to try and grasp the condition, get a handle on it and best manage it until a treatment presents itself.<sup>xii</sup>*

Emma Gallagher, a respiratory physiotherapist in the West Midlands, highlighted her difficulties in securing cough assist machines for patients who need this vital piece of equipment:

*Our experience in ventilation is that achieving and obtaining a non-invasive ventilation machine is quite easy. We can do that quite well but problems, as have been highlighted, really exist when it comes to cough assist machines. There is, in my opinion, no clarity whatsoever on where I'm supposed to be asking for who is going to fund cough assists. They are declined because they are not accepted as an exceptional case because one patient is no different than another patient because they have the same conditions and problems. CCGs tell us to go to NHS England, NHS England tell us to go back to CCGs, and clinically I'm spending an awful lot of my time not with a patient but actually just with finding out where I'm supposed to be asking these requests. I have data – out of the 25 requests I've put in only three have been funded.<sup>xiii</sup>*

Emma Gallagher went on to tell us that complex support is required for patients:

*But it's more than the machine itself, it's the clinical time of the patient that is suffering from a chest infection and needing their settings changed, so in order to prevent that emergency admission we need to be able to go into the homes to alter the machine's settings at homes, or even just having a cough assist machine and clinician that can go into the home and just do some chest treatment for every day for two weeks in order to keep the patient out of the hospital. It's the outreach care that we want to offer and it's that we are having difficulty with.<sup>xiv</sup>*

Mel McFeeters informed us:

*I have personally asked for clarity nationally looking at the prescribed services manual about making sure that there isn't this discrepancy where it says for adults that the CCGs fund it and the children that it's NHS England. The non-invasive ventilators and the cough assist are just two bits of equipment so why that should be treated any differently to any other bit of equipment doesn't make any sense whatsoever so it should be the same straightforward commissioning process for those bits of equipment along with all the feeding machines or whatever else is required – it shouldn't be that they come from NHS England for one and the CCGs for the other. It should be agreed that there is one process for all of it. But we have fed that back directly through the regional programme of care lead for women and children and that has gone up through the national team to look at and hopefully will be reviewed in the next edition of the prescribed services manual whenever that comes through.<sup>xv</sup>*

We were alarmed by the shortfall in psychological support. Action needs to be taken across the country.

Mel McFeeters told us:

*Psychological support and psychological services generally: we do recognise that there are insufficient services at the moment for not just neuromuscular conditions but across the patch. We do not have sufficient [numbers of] psychologists to provide that service and there are very limited numbers and again there are workforce issues in trying to recruit to those positions, so it is a recognised area of deficit.<sup>xvi</sup>*

Margot Keats from Derby wrote to us about the importance of ongoing physiotherapy for people with muscle-wasting conditions:

*If physiotherapy is offered it is only up to six sessions, which completely misunderstands that a long term condition is just that, and the physiotherapy needs to be ongoing.<sup>xvii</sup>*

The need for ongoing physiotherapy was echoed by Sheila Hawkins from Leicester as she conveyed her experiences:

*Physiotherapy is only available for short courses (up to four sessions) as this is what is commissioned by the Clinical Commissioning Group. After the last course the physiotherapist discharged me back to the GP saying that she was not able to offer any support/treatment to patients with my condition.<sup>xviii</sup>*

Transition from paediatric to adult services is an issue that the APPG has been alerted to on a number of occasions during the inquiry. We received written evidence from Acorns Children's Hospice:

*For those known to Acorns, the major issues occur in and after transition to adult services including both poor local provision of high quality therapy services directed to the needs of Duchenne muscular dystrophy and significant problems accessing a fragmented provision of specialist medical care.<sup>xix</sup>*

We were encouraged to hear that outreach services had been developed in the East of England region, which Carolyn Young outlined for us:

*We have set up a clinic in Ipswich from Addenbrooke's Hospital, we have a new clinic in Norwich, we have a clinic in Bedfordshire that is now supported from Oxford. We are also looking at where we can develop services in Essex. I think for the East of England it is quite a difficult geography in that [it] included the East of England, for instance, the Watford population which is kind of inside the M25 who actually always tend to look into London for their services, so it's working with in London.<sup>xx</sup>*

However, there is clearly more neuromuscular service development needed to ensure that patients receive the required standard of care locally. We received evidence from Karen Duckmanton from Essex:

*I would say that access to specialist neuromuscular care has not improved in my local area since April 2013. In fact I have not been seen locally for over a year – mainly because my consultant retired and I have not been seen by any other doctor/consultant.*

*I have to travel to London (a journey of between two and three hours) to get access to specialist neuromuscular care and support. Here I have seen a major improvement, as at the National Hospital for Neurology and Neurosurgery they have opened in the last year, a new purpose-built ward, which has six beds and is fully accessible with among other things ceiling hoists. When I attended this ward last year, I was in for two days having a sleep study, but whilst in there they looked at all aspects of my care and support needs and would have been able to offer assistance if required. I feel that if this (or something similar) could be available more locally to me in Essex, this would be a vast improvement locally.<sup>xxi</sup>*

## Second evidence session – 2 December 2014

### Focus on the North

#### Witness:

- **Anthony Prudhoe** – Programme of Care Manager, North region, NHS England

Further questions and comments were provided during the session by patients, family members and other healthcare professionals.

### Delivery of services

We heard from Anthony Prudhoe about the need for collaboration within NHS England for integrating and co-ordinating services for people with muscle-wasting conditions:

*So the challenge for the NHS with this – and I do know that work has already started to do some of this – is to get Clinical Commissioning Groups (CCGs) to work with NHS England, and work with hospitals to actually start thinking about how these services need to be integrated and networked and actually delivered. There is some distance to go yet, it is a Five Year Forward view, but I actually think some of the messages are really positive in the context of what you are trying to achieve.<sup>xvii</sup>*

Anthony Prudhoe recognised that transition was still an issue which NHS England needed to address:

*I think there is an acknowledgement by NHS England that we do have some way to go to solve transition properly. One of the areas of work we are still developing is service specification for transition. It's been a gap for us and social care and local authorities for at least 25, 30 years. It is an area that has been improving but definitely needs to get better.<sup>xviii</sup>*

We expressed concern about the lack of progress on increasing care advisor support in the North East. Anthony Prudhoe informed us:

*Newcastle-upon-Tyne Hospitals have a block contract arrangement in place with them. This means that we give them a block of money for delivering a whole range of things within neurosciences service, and again, quite often the way in which health care services are funded means you actually pay on tariffs. You actually pay for what you get rather than giving hospitals a block contract. So when it comes to bringing in new staff or additional staff it is quite difficult sometimes [with] a block contract in place, because the hospital will say the block contract does not cover you asking for new people and new staff, and commissioners will say it does.*

*So, we've got these issues quite often going on and we do work it out eventually between ourselves, but when it comes to finance sometimes these things are difficult, and of course the NHS hasn't got any new pot of money that it can dip into. So it is constantly looking into the way it spends money and whether we can recycle some of that cash through block contracts to actually shift its priorities. So, we still haven't got that particular post in place. The colleagues are still working on that from the North East Area Team.<sup>xix</sup>*

Matthew Lanham, Chief Executive of the NeuroMuscular Centre (NMC) in Cheshire, reported to us the inconsistency of CCG funding for patients to be able to access services at the Centre:

*We are funded by most CCGs across the patch to a degree to provide physio and hydro services for patients that they refer. Interesting though that is, this is just a little funding issue that I would flag up. Some CCGs that fund us make common sense decisions that what we are doing is of value, which they endorse, and they see it has clinical value. They see that the patients aren't going to the hospital, and it is having benefit so it is worth the small investment from their point of view. Most other CCGs have two funding mechanisms that they think about. One is exceptionality and the other is full, commissioned, contracted service, and for the numbers of patients we are talking about neither of those is appropriate.*

*CCGs should make common sense decisions. We have got CCGs that say this patient isn't exceptional, which is ridiculous. We have got CCGs that say we should commission a whole service, which is not appropriate for the number of people with these conditions.<sup>xx</sup>*

Professor Kate Bushby explained how research and clinical expertise could be combined in a specialist centre. This combination of expertise is highly appreciated by patients and their families.

*There have been some very frustrating issues that have arisen since the NHS commissioning reforms in 2013.*

*Clinical Reference Groups (CRGs) were set up to drive forward specialised services in England. But there are too many CRGs in our disease group with the main groups including adult neurology, paediatric neurology, and formerly multi-system disorders meaning that responsibility is fragmented.*

*The CRGs developed a neuromuscular annex, for the care of patients of all ages, to the neurosciences service specification, but this was only included in the adult service specification. There is also continued confusion as to whether this annex is mandatory or not. In addition, compliance to delivering the service specification is self-reported, which means that what is reported is not always really what happens on the ground.*

*It appears that although there is a national service specification, there is still no consistency from area to area in how NHS England deal with specialised services. I suggest that people could share best practice and take responsibility for different diseases by region. For example, one area defines the pathway for neuromuscular disorders and others are bound to that across [all areas] with reciprocal arrangements for other diseases. This way work could be shared, and not duplicated, improving the use of resources in the long run.*

*There has also been a complete planning blight due to a commissioning “pause”, but in reality this has been ongoing since the re-organisation of the commissioning structures was first planned, which has led to a lack of investment and development for years. This is very demotivating for staff and does reflect also on patient experience.*

*Nationally commissioned highly specialised services have experienced a particular lack of clarity as to what is happening. The Multi-system Disorders CRG was suspended for months, meaning there was no cohesion of who to talk to about what. This CRG has now been disbanded and highly specialised services will now return to be commissioned nationally by NHS England, but there has been no formal feedback to the CRG members about this.*

*Finally, NHS England’s handling of the process of approving rare disease drugs like Translarna, which can treat some boys with Duchenne muscular dystrophy, has been shambolic. The process seems to be too complicated and protracted. One potential solution to this could be for the European Medicines Agency procedures, with reviews and questions and responses, to be made available rather than going through endless re-reviews of the same information.<sup>xxvi</sup>*

## Neuromuscular network commitment

We were pleased to hear that there was a commitment from hospitals trusts in the North West to form a neuromuscular network. We look forward to this network being formed as soon as possible so that patients living with muscle-wasting conditions can benefit from better co-ordination of services. Anthony Prudhoe told us:

*Certainly the Cheshire, Warrington, and Wirral Area Team, which is responsible for specialised services, has recommended that the network is put in place. It certainly has the support of Alder Hey and other parts of the system, because we do have a paediatric neurosciences network across the North West which has just started and it would be really great to actually move this network quickly so it becomes part of, not consumed by, this broader network. In terms of timeframe it shouldn’t take too long to actually pull this together because the commitment is definitely there to do it.<sup>xxvii</sup>*

## Knowledge and understanding of muscle-wasting conditions

We received evidence from Yvonne Petitjean, who told us:

*I do not expect general practitioners to be specialists but I would expect them to have a good general knowledge and understanding of a neuromuscular condition when they have a patient suffering with such. General ailments and many medications may have a detrimental impact on the neuromuscular condition and its symptoms. Therefore there is often an overlapping of the two. However, it appears to be an area of ‘pot luck’ when it comes to which GPs take an interest of a patient’s neuromuscular condition and how it affects them and their various needs. In the past I have had one GP who was interested to learn and completed his own research.*

*I feel passionate that all GPs are supported by the NHS by providing doctors more time and most importantly provide relevant regulated training to safely support any patient of theirs who suffers from a neuromuscular or rare condition.<sup>xxviii</sup>*

Anthony Prudhoe commented:

*We need to do more with GPs. If you haven't got GPs sorted out as part of that network, you aren't going to deliver a great network. So you have got to bring them in to the tent. You've got to make sure they are actually part of this system that we are doing here.*<sup>.xxix</sup>

### Third evidence session – 3 February 2015

#### Focus on the South and London

- **Mark Satchell** – Associate Director of Specialised Commissioning, NHS England (South)
- **Steven Duckworth** – Manager, South East Coast Strategic Clinical Networks (Cancer and Mental Health, Dementia & Neurological Conditions), NHS England
- **Dr Andria Merrison** – Consultant Neurologist with Specialist Interest in Neuromuscular Disease and Chair/Clinical Lead for the South West Neuromuscular Disease Operational Delivery Network

Further questions and comments were provided during the session by patients, family members and other healthcare professionals.

### Co-ordinating delivery of services

We heard about the importance of co-ordinating service and including the essential components of specialist multi-disciplinary care for people with these complex and rare muscle-wasting conditions.

Dr Andria Merrison informed us:

*Some have several specialists, as it were, in the centre whereas others are thin on the ground with specialists. And then an issue is not just the specialist services themselves, it's how they integrate with all the other services that are needed to care for somebody who's living with a neuromuscular condition. It's not just specialist neuromuscular centres; it's the respiratory support side, and cardiology care that's needed. So it's thinking about the whole thing holistically and making sure that as we develop our services, all the other things around us that are needed are developed alongside, otherwise we can't provide that support to our patients.*<sup>.xxx</sup>

We were interested to hear Mark Satchell telling us about the commissioning arrangements with CCGs and were encouraged to hear him highlighting patients' needs as a priority. He commented:

*The specialised commissioning part of NHS England is working to develop a strategy for specialised commissioning to ensure that the very highly specialised services and the services that need to be commissioned on a wide and larger footprint are given due attention within NHS England, and to start to set out an agenda that says there are services which can be provided on a more local basis and a smaller footprint which should be commissioned in collaboration with Clinical Commissioning Groups. I think partly within that the key is to try and work out where different services sit and what is best for patients and to enable the high standard of services to be provided.*<sup>.xxxi</sup>

However, we were concerned about the lack of knowledge and expertise within CCGs on not just neurological conditions, but also on the specific needs of patients with muscle-wasting conditions. Steven Duckworth told us about the challenges:

*The real difficulty [that] there is [is that] out of the 20 CCGs that I have a direct interface with, only one CCG has a commissioner that could hold their hand up and say 'I am a commissioner for neurological conditions', and that's one of the challenges.*<sup>.xxxi</sup>

### Gaps in specialist care

We were concerned to hear about the delays in securing the implementation of neuromuscular service developments in the region.

Mark Satchell outlined the need for development for transition services:

*For example, one of the areas which we find challenging, [and] this isn't just for this group of services, is the transition from paediatric services to adult. I think in the NHS, that's a challenging part because you go from having care which is almost wrapped around a child so that the child who needs health services gets them, in a package in their local hospital or the specialist centre they need to attend, and when they become adults suddenly they have to pull this together a little bit themselves and the health service isn't always brilliant at doing the signposting for them.*

*I'm sure we can all remember what it's like going from school to work, it's that same sort of shock to the system and the health*

*service needs to work better to make sure it's easier for young people to move from paediatric services to adult services.<sup>xxxiii</sup>*

Steven Duckworth commented on the development of business cases for neuromuscular care advisor support on the South East Coast for both children and adults:

*It's my understanding that there's currently a business case going through specialised commissioning for the South East Coast, commissioned with providers in London. So for the paediatric role it's the Evelina Children's Hospital and for the adult role it's King's College Hospital, and it's my understanding that there's a business case going through both these organisations now which are provider organisations to create these roles which will then go out into the South East Coast.*

<sup>xxxiv</sup>

Mark Satchell commented on service provision in the South East on a broader scale:

*One of the challenges in the South East is that a lot of specialist care is provided outside of the region, from London. That's a challenge in itself in the sense that the London providers clearly look at London and the wider footprint, but inevitably the further away it is the more difficult, and local commissioners will tend to look at the more immediate issues rather than some of the issues that other colleagues at NHS England are raising with them. This is something we'll be taking away and discussing with them.<sup>xxxv</sup>*

Janet Bloor spoke of the extreme difficulties that her son, Phillip, who has Duchenne muscular dystrophy, is experiencing in accessing hydrotherapy and physiotherapy:

*Now he's the grand old age of 21, I'm having to source all these different services and be told 'You don't get hydro', like I'm asking for the moon, like it's some crazy, wacky alternative therapy. It's a necessity, it's hydro and physio that stops my son needing extremely expensive back surgery.<sup>xxxvi</sup>*

We were alarmed to hear from Phillippa Farrant from Eastbourne about difficulties in access to hydrotherapy in the South East. She told us:

*I have a 23-year-old [son] with Duchenne [muscular dystrophy]. Following on from Janet's comment about the physio, Dan used to get hydrotherapy once a week via his specialist school down in Hastings. I've now been in touch with physios down there who are horrified as they have had funding withdrawn, so even paediatric lads are not getting hydrotherapy. That needs to be urgently addressed and put back in place.<sup>xxxvii</sup>*

We were concerned to hear about the problems in accessing specialist respiratory support, which Dr Merrison outlined for us:

*Because of the way things are set up around us, for example in the north part of our region, it's easy for adults to get access to cough assist machines and quite easy to get non-invasive ventilation [NIV]. But for children, it's quite difficult to get access to both these things: cough assists and NIV at home. And that relationship is quite difficult: I still think quite a lot of power sits with commissioners as they have the money, and as a network I feel we should be much more integrally involved in that process, because otherwise this money is handed over without a clear commitment as to what that network is trying to achieve.<sup>xxxviii</sup>*

Bev Toms, paediatric physiotherapist from Bristol, added:

*It's important to bear in the mind that it isn't just handing out a machine; there are assessments, you have to set it up and support the patient and the family in the community. There's a big physiotherapy role, but often the funding isn't there to provide that role. It varies, depending on which area you are in.<sup>xxxix</sup>*

We were alarmed to hear about the difficulties faced by patients to gain access to specialised wheelchair provision. Nicki Doran, specialist neuromuscular physiotherapist from Bristol, commented:

*There is also an urgent need for specialised commissioning to look at how they relate/share local commissioning arrangements for wheelchair services. The clinicians are constrained within a 'financial envelope' so the extra adaptations/additions that may be necessary for a neuromuscular patients are often not provided through the NHS. I have come across examples of where the extra [adaptation] (i.e. seat riser) has been sourced by wheelchair services but has to be paid for by the patient (privately or via charitable funds which may take some time). Usually these adaptations are essential for a person's day-to-day functioning and independence.*

*This is of course an issue for many long-term chronic conditions but the specific muscle weakness issues for neuromuscular patients usually do not have individual attention (identified by wheelchair therapists) but not with the*

*provision to match i.e. seat risers/ high-low function.<sup>xi</sup>*

We pursued the provision of specialist care and support based around Oxford and Southampton, to which Mark Satchell responded:

*The local NHS in Oxford are about to recruit for one of their [care advisor] posts. It's taken a long time to get adverts placed but the set-up across South Central is to run more with the two centres (Oxford and Southampton), effectively having a network arrangement with local hospitals and community services, but certainly in a less structured way than we have in the South West and South East. This is something that we should be challenging, but we've focused on looking to improve on the level and range of services that are provided outside of Southampton and Oxford, when we had reports criticising some of the services that were provided, so we put our efforts into addressing this initially rather than the network. The challenge for us over the next few years is whether that is good enough and the geography works or whether gaps are emerging and we have good outreach or network arrangements.<sup>xii</sup>*

One of the principles to develop access to services which the APPG has been continuously highlighting is the idea of improving standards of care and quality of life for people with muscle-wasting conditions, while also saving the NHS money. We were encouraged to hear Mark Satchell referring to NHS England's plans to reduce the number of unplanned emergency admissions to hospital:

*One of the initiatives commissioners across the South are taking this year is to look at how we can deliver improvements towards reducing non-elective admissions and the length of stay for patients that do need to go into hospital with these conditions.<sup>xiii</sup>*

## Neuromuscular networks and learning from best practice

The South West Neuromuscular Network, now called the South West Neuromuscular Operational Delivery Network, is an example of a best practice approach to co-ordinating specialist neuromuscular care. By engaging commissioners, expert health professionals and patient representatives, it demonstrates how specialised services should be delivered and can be protected and strengthened.

Dr Merrison outlined the origins of the South West Network, which helped inform us about the ideal practice which should be followed in other parts of the country:

*The Muscular Dystrophy Campaign [now Muscular Dystrophy UK] helped take this forward, as there was so much concern that the region was woefully behind compared to other areas of the country. So just setting up advice and support was a start, and then taking forward, and sticking to, a proper network concept, which I see as an entirely patient-centred and patient-integrated approach to care. It's a radically different approach to care: instead of us being locked into our own individual provider organisations, we are looking at trying to make sure care is provided across traditional boundaries in Health and Social Care and across traditional organisation boundaries. We've done that as a group of professionals, but also our governance structure encompasses all stakeholders, including people living with neuromuscular disease, coming with us every step of the way, reining us in at some points, and really making sure that that voice (the patient and carer voice) is the guiding voice. It's also allowed us to have flexibility, drawing on not just state-run services, but also charities and other organisations around us.*

*Geographically, this has allowed us flexibility in providing the right level of care, in the right place at the right time. It has also allowed us to use a few specialists across a wide area supporting large teams of people, not just people who are employed to specialist commissioners to form part of our network, but a wider group of professionals. That's based not just on relationships we have with people and how we have integrated with existing services: in other words, rather than marching up and saying 'I am the expert and I know how to run these services', we say 'you are running this fantastic service. How best can we work with you to improve care for the same group of people?' That ethos has definitely helped, and the whole concept of self-management for people living with the conditions: that's how professionals have been approaching this. It's been a great opportunity in that sense and we were very well supported by specialised commissioning to make a start.<sup>xiiii</sup>*

We heard about encouraging recent developments for a network covering London and the South East from Steven Duckworth:

*One of the things that I think will be addressed further down in the questions is how we in the South East Coast can use some of the examples from the South West in providing a network. There is I understand an advert that's gone out for an equivalent to Andria's position as Chair of a network, across London and the South East. Whether that changes the position of a business case going through a provider, or whether it's paid for directly by specialised commissioning I'd*



*hope that that network (which wouldn't just be providers, but commissioners, patients, public, etc.) can start mapping out some of those pathways as well, so even if those roles were just to fall out of particular London-based providers, that forum would look at ways of ensuring that provision of services is equitable across South East Coast.<sup>xiv</sup>*

He went on to say:

*Well I think with the creation of the network similar to the one in the South West, I'd hope that then you could start to map out some of the pathways that people need to get their care and work along that rather than just coming out of King's [College Hospital] into arbitrary areas. That network could actually start looking at the demand, look at the need and say 'is one co-ordinator enough?' and start making a compelling case to commissioners as part of that network that it isn't enough and more needs to be invested.<sup>xv</sup>*

We welcomed Dr Merrison's suggestion that named commissioners could help networks develop the services that are provided:

*I really think it would make a massive difference if you had a named commissioner who was available to us as organisations who was responsible for making sure that even when you have a structure and a framework as a clinical network, that all the relationships actually work. Because for an individual like myself as Chair of a Network, it's very difficult to influence individual providers, it's very difficult to influence change. The only way I can see how we could do that is to have a commissioner who is able to deal with that on a daily basis, or at least on a monthly basis, in an operational way.<sup>xvi</sup>*

## Appendix

---

### First evidence session – 28 October 2014

- **Carolyn Young** – Programme of Care manager – Trauma, NHS England (Midlands & East)
- **Dr Owen Jones** – Senior Service Specialist, Specialised Commissioning (Leicestershire and Lincolnshire Area Team), NHS England
- **Mel McFeeters** – Clinical Services Specialist (Leicestershire and Lincolnshire Area Team), NHS England
- **Dr Tracey Willis** – Consultant Paediatric Neurologist, Robert Jones and Agnes Hunt Orthopaedic Hospital NHS Foundation Trust

### Second evidence session – 2 December 2014

- **Anthony Prudhoe** – Programme of Care Manager, North region, NHS England

### Third evidence session – 3 February 2015

- **Mark Satchell** – Associate Director of Specialised Commissioning, NHS England (South)
- **Steven Duckworth** – Manager, South East Coast Strategic Clinical Networks (Cancer and Mental Health, Dementia & Neurological Conditions), NHS England
- **Dr Andria Merrison** – Consultant Neurologist with Specialist Interest in Neuromuscular Disease and Chair/Clinical Lead for the South West Neuromuscular Disease Operational Delivery Network

## Terms of Reference

The terms of reference for the inquiry are:

*“To determine the impact of NHS reforms which came into effect in April 2013 on the delivery of specialist care and support for people with rare conditions such as muscular dystrophy and related neuromuscular conditions.”*

## The importance of early diagnosis

Early diagnosis of muscle-wasting conditions not only leads to earlier intervention in managing the condition but also provides a potential opportunity to gain access to any emerging treatments that become available.

GPs play an important role in identifying people who may have muscle-wasting conditions, arranging appropriate referral and co-ordinating care. GPs should be confident in managing acute complications, such as respiratory infections, and in arranging appropriate follow-up where needed. GPs are also in an ideal position to talk to patients, their families and carers about their expectations and concerns and to address the potential need for psychological support.

The new e-learning module, developed by Muscular Dystrophy UK and the Royal College of General Practitioners, will give GPs greater understanding of muscle-wasting conditions. With at least 1,000 GPs expected to take this training module, it is likely to lead to earlier and more accurate diagnosis and effective intervention.

Neuromuscular care advisors and specialist neuromuscular nurses play a vital role in providing support during and after the diagnosis of a muscle-wasting condition. They can help by signposting the individual and family towards available services, and help co-ordinate and plan for the likely progression of the condition.

Through upskilling of local community teams, healthcare professionals are better informed about the whole neuromuscular pathway. They also know when to refer to specialist teams – leading to earlier diagnosis, effective intervention and fewer avoidable, unplanned admissions to hospitals.

## Developing neuromuscular networks across England

In the south west of England, following an NHS-led review of neuromuscular services in 2008, a team of specialists – including consultants, physiotherapists and neuromuscular care advisors – was employed as part of an innovative new Managed Clinical Neuromuscular Network.

The now 'Operational Delivery Network' is overseen by a group of NHS and patient representatives, health professionals and Muscular Dystrophy UK. The network helps co-ordinate service provision for patients with muscle-wasting conditions, and is an excellent example of how improvements and investment can be made if services are linked together. This, in turn, can lead to savings of £2.76m through preventing emergency admissions to hospital.

This should be used as best practice for further Neuromuscular Networks to be developed across England. It is important to bring together specialists who work across neurology and neuromuscular as a unique specialism, and then to share their skills and expertise with community teams.

A network also plays a vital role in developing 'hub and spoke' outreach models, whereby patients would receive the majority of their care closer to home while remaining, particularly for access to research, under the care of specialist tertiary services.

## Access to Translarna – a new treatment for Duchenne muscular dystrophy

Translarna is the first treatment to address an underlying genetic cause of Duchenne muscular dystrophy.

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

The number of people who have Duchenne muscular dystrophy resulting from a 'nonsense' (or 'stop') mutation is very small. It is expected that the number of patients in England that could be eligible for treatment is about 80-90, although not all of these are diagnosed cases.

Louisa Hill, whose son, Archie, aged 9, is eligible for Translarna, said:

*At the end of the day, parents like us believe Translarna is safe. I don't understand why it takes so long to make decisions about licensing these drugs. The EC have recommended it, so why does it take so long for the decision to be made here in the UK?*

*People making these decisions don't understand the impact on children of even a small change. It gives them more time to run and play football with their friends. It's really buying precious time. Archie will have to deal with very difficult mental and physical challenges as his condition progresses. Translarna could buy time for Archie just to be a kid."*

Translarna had been going through the stages of the NHS England commissioning policy process until the process was stopped in December 2014. It has already been made available in European countries, including Spain, Germany, France and Italy.

MPs and Peers from across the party political spectrum have continually been voicing the concerns and frustrations of families affected by Duchenne muscular dystrophy: delays will mean boys will lose ambulation while they wait and they will therefore no longer be eligible for the treatment.

A 90-day consultation is being conducted by NHS England on how they assess new treatments. The consultation concludes at the end of April 2015. The APPG understands that a Clinical Priorities Advisory Group meeting will be arranged in May before a decision on approval and funding of Translarna is made in June.

## Written and additional evidence

All Party Parliamentary Group for Muscular Dystrophy, *Access to high-cost drugs for rare diseases*, September 2013

All Party Parliamentary Group for Muscular Dystrophy, *Access to specialist neuromuscular care: the Walton Report*, August 2009

*Developing a more collaborative approach to the commissioning of specialised services: guidance document*, NHS England, March 2015

*Service Specification for Neurosciences: Specialised Neurology (Adult) D04/S/A*, NHS Commissioning Board, 2013

*Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management*, Bushby et al, 2009

*Diagnosis and management of Duchenne muscular dystrophy, part 2: implementation of multidisciplinary care*, Bushby et al, 2009

*Improving recognition of Duchenne muscular dystrophy: a retrospective case note review*, Henriette J A van Ruiten, Volker Straub, Kate Bushby, Michela Guglieri

*Right to breathe: access to respiratory care for people with muscle-wasting conditions*, Muscular Dystrophy UK, February 2015

*UK Strategy for Rare Diseases*, Department of Health, November 2013

*UK Strategy for Rare Diseases: NHS England Statement of Intent*, February 2014

*Unplanned admissions of neuromuscular patients: a collaborative audit*, Hana et al, June 2012

We thank everyone who has made contributions at the oral evidence sessions and submitted written evidence.

## References

- |       |                                   |         |                                   |
|-------|-----------------------------------|---------|-----------------------------------|
| i     | Evidence session, 28 October 2014 | xxiv    | Evidence session, 2 December 2014 |
| ii    | Evidence session, 28 October 2014 | xxv     | Evidence session, 2 December 2014 |
| iii   | Evidence session, 28 October 2014 | xxvi    | Evidence session, 2 December 2014 |
| iv    | Evidence session, 28 October 2014 | xxvii   | Evidence session, 2 December 2014 |
| v     | Evidence session, 28 October 2014 | xxviii  | Evidence session, 2 December 2014 |
| vi    | Evidence session, 28 October 2014 | xxix    | Evidence session, 2 December 2014 |
| vii   | Evidence session, 28 October 2014 | xxx     | Evidence session, 3 February 2015 |
| viii  | Evidence session, 28 October 2014 | xxxi    | Evidence session, 3 February 2015 |
| ix    | Evidence session, 28 October 2014 | xxxii   | Evidence session, 3 February 2015 |
| x     | Evidence session, 28 October 2014 | xxxiii  | Evidence session, 3 February 2015 |
| xi    | Evidence session, 28 October 2014 | xxxiv   | Evidence session, 3 February 2015 |
| xii   | Evidence session, 28 October 2014 | xxxv    | Evidence session, 3 February 2015 |
| xiii  | Evidence session, 28 October 2014 | xxxvi   | Evidence session, 3 February 2015 |
| xiv   | Evidence session, 28 October 2014 | xxxvii  | Evidence session, 3 February 2015 |
| xv    | Evidence session, 28 October 2014 | xxxviii | Evidence session, 3 February 2015 |
| xvi   | Evidence session, 28 October 2014 | xxxix   | Evidence session, 3 February 2015 |
| xvii  | Evidence session, 28 October 2014 | xl      | Evidence session, 3 February 2015 |
| xviii | Evidence session, 28 October 2014 | xli     | Evidence session, 3 February 2015 |
| xix   | Evidence session, 28 October 2014 | xliii   | Evidence session, 3 February 2015 |
| xx    | Evidence session, 28 October 2014 | xliv    | Evidence session, 3 February 2015 |
| xxi   | Evidence session, 28 October 2014 | xlvi    | Evidence session, 3 February 2015 |
| xxii  | Evidence session, 2 December 2014 |         |                                   |
| xxiii | Evidence session, 2 December 2014 |         |                                   |

---

# All Party Parliamentary Group for Muscular Dystrophy

**Impact of NHS reforms on access to neuromuscular services**

---

March 2015