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**Access to Specialist  
Neuromuscular Care:  
The Walton Report**

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**All Party Parliamentary Group  
for Muscular Dystrophy**

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## Access to Specialist Neuromuscular Care: The Walton Report

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### Foreword

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The publication of the evidence set out in the Walton Report highlights the urgent need for the NHS to address the failings identified by the expert witnesses – both individuals and organisations – who submitted their evidence to the Inquiry.

It was clear during each of the evidence sessions and in the written submissions that the steps that need to be taken to ensure that each child and each adult living with one of the conditions has access to specialised, multi-disciplinary health care are known and understood. It is also very clear that the health needs of this important group of patients and families are often overlooked and the standard of care provided by the NHS is often simply not acceptable. Indeed, the evidence shows regrettably that this is the situation in many parts of the country. I am personally passionate about this as I have spent a lifetime in a family that has been devastated by muscle disease. My brother and sister, who were affected by a form of muscular dystrophy, had lifelong disabilities but not very long lives.

The responsibility for addressing these problems lies with the Department of Health and the NHS. However, we found a lack of clarity and accountability within the NHS regarding who is held accountable between the Head of National Specialised Commissioning, the Strategic Health Authorities, the Specialised Commissioning Groups and the Primary Care Trusts in England and the comparable devolved organisations in Scotland, Northern Ireland and Wales.

I must thank all the experts – and I include patients and families as experts – who gave oral evidence to the Inquiry and made written submissions. I also wish to thank the Muscular Dystrophy Campaign for their hard work and efficiency in providing the Secretariat to the Inquiry. I also want to note and warmly thank my Parliamentary colleagues for their remarkable level of interest and concern in supporting the work of this Inquiry from December 2008 to July 2009.

Finally, the members of the APPG wish to place on the record their thanks and indeed admiration for the contribution made by Lord Walton of Detchant. He not only played a leading role in guiding the Inquiry through its work over seven months but he has been a leading figure in the world of muscular dystrophy and related conditions for more than 50 years. John Walton was one of the three founders of the Muscular Dystrophy Group, as the Campaign was then known, and served for many years as its Chairman and in recent years has continued to make a huge contribution as its Honorary Life President.

It was 50 years ago that John Walton had the commitment, vision and determination to help to found the charity and in this anniversary year it is as a sincere tribute to his lifetime's contribution and outstanding achievements in this field that the APPG decided unanimously to call our report 'The Walton Report'.

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

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## About Lord Walton of Detchant

Lord Walton of Detchant was a founder of the Muscular Dystrophy Campaign in 1959, its Chair from 1971-95 and continues to be an active and influential supporter as its Honorary Life President.

As Professor Walton, John had a long and distinguished medical career and his outstanding contribution to research and clinical development has been widely recognised through his holding of the offices of President of the General Medical Council, President of the British Medical Association, President of the Royal Society of Medicine, President of the Association of British Neurologists and President of the World Federation of Neurology.

Lord Walton was made an Honorary Freeman of Newcastle upon Tyne in 1980 and became a crossbencher, independent life peer in 1989. He has a well earned reputation as an extremely effective, skilled and influential member of the House of Lords who commands the utmost respect when he contributes to debates in Parliament.

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## 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 of muscular dystrophy and related neuromuscular conditions amongst Parliamentarians. The Secretariat of the group is provided by the Muscular Dystrophy Campaign.

### The Parliamentary Inquiry

The Inquiry was launched in December 2008 to carry out an in-depth investigation looking at access to specialist, multi-disciplinary care for people living with muscular dystrophy and related neuromuscular conditions. This arose from the concerns of the All Party Parliamentary Group that the existing specialist services in the four leading neuromuscular centres were vulnerable through their reliance on charitable funding and the lack of succession planning for the leading clinicians. It should be noted that the four centres evolved through the research and clinical interests of the leading consultants supported by charity investment made by the Muscular Dystrophy Campaign.

However, outside the specialist centres at Newcastle, Oswestry, Oxford and London, the picture is very different with considerable variations in services. There are a few neuromuscular consultants who are innovative and persistent in overcoming institutional barriers or indifference that stand in the way of improving services for their patients, while in other regions the picture is much bleaker, with a lack of adult services, weak transition support and very poor links with social care. Many patients in the poorer-served regions are faced with weak services, delays to see clinicians and poor community-based services.

The Parliamentary Inquiry has received both written and oral evidence that underlines the need for urgent action to improve and strengthen multi-disciplinary health care for this vulnerable group of patients living with rare and very rare conditions.

### Acknowledgements

The All Party Parliamentary Group (APPG) for Muscular Dystrophy wishes to thank the Muscular Dystrophy Campaign for its administrative support in the organising and staging of evidence sessions, gathering written evidence and producing this report.

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## The Parliamentarians who received oral evidence submissions were:

Dave Anderson MP (*Labour, Blaydon*) (Chairman)  
Russell Brown MP (*Labour, Dumfries and Galloway*)  
Lorely Burt MP (*Liberal Democrat, Solihull*)  
Geoffrey Cox MP (*Conservative, Torridge & West Devon*)  
Lord Dubs of Battersea (*Labour*)  
Baroness Finlay of Llandaff (*Crossbench*)  
Dr Hywel Francis MP (*Labour, Aberavon*)  
Dr Ian Gibson MP (*Labour, Norwich North*)  
Earl Howe (*Conservative*)  
Baroness Howe of Idlicote (*Crossbench*)  
Dan Rogerson MP (*Liberal Democrat, North Cornwall*)  
Alison Seabeck MP (*Labour, Plymouth Devonport*)  
Baroness Thomas of Winchester (*Liberal Democrat*)  
Mark Todd MP (*Labour, South Derbyshire*)  
Lord Walton of Detchant (*Crossbench*)  
Baroness Wilkins of Chesham Bois (*Labour*)  
Betty Williams MP (*Labour, Conwy*)

**A full note of the witnesses who gave oral evidence to the Inquiry is attached in the Appendix together with a note of the written evidence submissions.**

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## Recommendations

- 1. The Department of Health and the NHS (together with each of the National Health organisations in the devolved countries) should ensure that specialised paediatric and adult neuromuscular services are highlighted for commissioners based in both regional Specialised Commissioning Groups (SCGs) and in local Primary Care Trusts by giving specialised neuromuscular services their own place in the National Definition Set to reflect the urgent need to treat these services as a priority following years of under-investment and weak coordination. We call on SCGs to follow the lead of the South West SCG and West Midlands SCG and conduct an urgent in-depth review of neuromuscular services in the regions;**
- 2. The responsibility for ensuring that access to specialised, multi-disciplinary neuromuscular services is available for all patients should lie with a named individual postholder in the National Specialised Commissioning Group and with a named individual in each of the English regions and devolved Health administrations;**
- 3. The lack of Workforce Planning for specialist neuromuscular clinicians has been a recurrent theme throughout the Inquiry. This must be addressed as a priority as highlighted in the NHS Next Stage Review. While there are some excellent services provided at the four national muscle centres, they are vulnerable through their reliance largely on the research interests and drive of the individual leading clinician. These services must be protected and strategically developed with succession planning. We call for a summit meeting to be organised by NHS Workforce Planning with the leading neuromuscular clinicians, the Royal Colleges, the other professional bodies and the Muscular Dystrophy Campaign to develop and implement a Neuromuscular Workforce Plan by June 2010;**
- 4. We found it a matter of great concern that most individuals and families living with a neuromuscular condition do not have a named individual to provide advice and support at critical times during the disease process. There are currently only 13 Neuromuscular Care Coordinators. To provide adequate support to the 60,000 people living with these conditions a network of some 60 Care Coordinator posts is required. Furthermore, we heard many patients and families, together with the leading clinicians, praise the initiative and commitment of the Muscular Dystrophy Campaign in funding eight of these Care Coordinator posts in several parts of the country. Due to the reliance on charitable funding, these eight existing posts are extremely vulnerable. Investment in these posts is cost-effective as they have been shown to save consultants' time, reduce emergency admissions and re-admissions, reduce hospital stays and coordinate care locally. We call on the DH and the NHS to introduce a funded development plan (working with the Muscular Dystrophy Campaign) by the end of March 2010 to retain the existing posts and to ensure that a national network of Care Coordinators is established within 5 years;**

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5. Further, we are convinced that similar action is urgently required to ensure that vital support is accessible from professionals such as physiotherapists, speech and language therapists, occupational therapists and dieticians. Muscular dystrophy and related neuromuscular conditions are complex, multi-system disorders that can be managed effectively with specialist multi-disciplinary care, but too many patients are currently being failed by the NHS. We therefore call on all health commissioners and managers to assess the needs and gaps in professional support in their local area and produce a development plan to address service weaknesses by March 2010;
  6. It has become clear that the UK falls well behind some other countries internationally in the provision of care for patients with a neuromuscular condition. Survival rates in the UK for individuals with Duchenne muscular dystrophy, for example, lag behind those achieved elsewhere in Europe in countries such as Denmark and the Netherlands. In those countries, specialist multi-disciplinary care with excellent links to supported, independent living is the norm. We call on the Department of Health to commission an urgent comparative study to identify international best practice and ensure this is disseminated in this country to clinicians, commissioners and patients;
  7. Further, we call on NICE to put in place a Clinical Guideline for Duchenne muscular dystrophy, building on the existing Standards of Care developed by the TREAT-NMD European consortium; this will help to drive up standards and also give patients and families a clear understanding of the standards of care to which they are entitled;
  8. While the current, early stage (Phase I and Phase II) clinical trials are extremely encouraging, it is essential for patients' participation in future trials to ensure that our standards of care are harmonised with the best available elsewhere in Europe. Otherwise UK patients will be excluded from participation in trials and the outcome of the trials could be influenced or skewed. While the rate of progress now being made in taking research from the laboratory bench to the patient's bedside is most encouraging, we caution that eligibility for the current trials is only possible for that proportion of the patient population who have a particular genetic mutation responsible for many cases of Duchenne muscular dystrophy;
  9. We recognise that ensuring patients' participation in clinical trials is vitally important, but there are currently too few centres in the UK with the infrastructure to support trials for neuromuscular conditions. We underline our call for urgent action to improve the care and treatment provided for patients. It should be noted that this will also help to develop additional tertiary and secondary centres with the capacity to participate in clinical trials. There is clear interest in developing new treatments from a number of biotechnology and pharmaceutical companies whose investment is given some protection through, for example, the easing of guidelines regarding the size of clinical

trials and extended patent protection in Europe (and the USA) achieved by the relevant legislation;

10. We call on the Government and commissioners to take urgent action to make sure that a named transition co-ordinator is in place for each young person with a neuromuscular condition who is moving from paediatric to adult services. We heard evidence that transition services to provide support to young people when they move from paediatric to adult services are very weak in many parts of the country;
11. Further, a recurrent problem about which we heard from several expert witnesses relates to the paucity and weakness of adult neuromuscular services in many parts of the country. Even where there are reasonable or good paediatric services in place, the provision of adult services is generally much weaker and this is unacceptable. Where good multi-disciplinary services are well co-ordinated, they involve specialities such as genetics, a muscle pathology service, cardiology, respiratory and cardiac support and specialist physiotherapy. The successful development and management of specialist care leads to a growing population of adults who in earlier times would not have survived, and these people have a right to continuing, high quality support to ensure they enjoy the best possible quality of life, as in some other European countries. We call on health service commissioners, local authorities and the local government associations to ensure they develop effective plans to guarantee they meet the comprehensive health and social care needs of this growing number of adults;
12. While the responsibility for health policy and service delivery rests with the devolved administrations in Scotland, Wales and Northern Ireland, we were nevertheless alarmed to hear of the decline in services in Wales over the last 10 years and the real fear that there will be further decline in the next 2 years unless action is taken now. We also heard evidence that paediatric services in Northern Ireland have been weakened by the absence of a paediatric neuromuscular consultant for several months and the vulnerability of the post of Care Coordinator which is reliant on charitable funding. The evidence submitted regarding the vulnerability of specialist services in Scotland also concerns us. We therefore strongly suggest that the Health Ministers in the devolved countries should work closely with the professional bodies and the Muscular Dystrophy Campaign to address these and related issues;
13. Further, we call on the NHS National Specialised Commissioning Group and Health Commission Wales to ensure that arrangements are put in place urgently between Wales and England similar to those which work effectively between Scotland and England to enable patients to have smooth access to diagnostic services at the specialist tertiary centres in England;
14. We are very concerned by the volume of evidence we heard which set out unacceptable delays in the provision of wheelchairs, equipment and orthotics

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support necessary to ensure a good quality of life and to maintain the individual's independence and dignity. Further, we find it unacceptable that many individuals are forced into financial hardship or to appeal for charitable funds in order to purchase the vital equipment they require. We are aware that there have been several recent reviews of national wheelchair and equipment services but the issues have still to be addressed effectively. We therefore now call on the Department of Health to set up a Short-Life Review Group involving the Muscular Dystrophy Campaign and representatives of other patients' organisations to bring forward proposals by June 2010 to address the failings in the provision of these vital services;

15. We heard in many of our evidence sessions of both the value and benefits of hydrotherapy for many people with a neuromuscular condition but also the difficulties often encountered in accessing a local hydrotherapy pool. We therefore call on the Department for Communities and Local Government to undertake an urgent review of access to hydrotherapy pools across the country, involving the Local Government Association, the Muscular Dystrophy Campaign and other organisations. The review should be completed and recommendations brought forward within 12 months to ensure that all people living with a neuromuscular condition have the opportunity to use a fully accessible, local hydrotherapy pool, if they wish;
16. We call on the NHS to develop and implement a plan by December 2010 to gather data systematically regarding the numbers of children and adults with a particular neuromuscular condition and also to collect data regarding survival. These data are essential to plan services effectively and also to enable comparisons to be made both between UK regions and countries and also on a comparative, international basis.
17. While the focus of this Inquiry was on health care, we also heard evidence from several witnesses which highlighted stark failings in social care and a lack of the support to which people are entitled to enable them to lead full and meaningful lives, living as independently as possible. It is clear that many people are encountering significant problems in accessing support to take up and retain employment, to secure home adaptations in a reasonable timeframe, to use public transport that enables them to travel independently and with dignity and in accessing higher and further education. These are all extremely important areas which require urgent, coordinated action by the agencies concerned to address the issues drawn to our attention. Therefore, we call on the Department of Health, the Social Care Institute for Excellence and the local government associations to undertake a systematic review of social care support for people living with a neuromuscular condition by September 2010.

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## Executive Summary

The Inquiry into access to all aspects of specialist neuromuscular care was launched by the All Party Parliamentary Group (APPG) in December 2008 when leading neuromuscular consultants gave evidence setting out the staffing and relevant specialisms required to provide patients with a comprehensive, multi-disciplinary service.

In subsequent sessions, evidence was presented which highlighted the fact that neuromuscular services in many parts of the country currently fall well below a minimum acceptable level, and this compromises patient survival and well being. Indeed, the clinical audit data from the South West region which show the mean age of death at 19 years of age for patients with Duchenne Muscular Dystrophy compares starkly with published survival data showing the average of death for similar patients in the North East region has reached 30 years of age and is extending beyond this age. In any decent, civilised society these variances are unacceptable and we cite them here as evidence of service failures that must be addressed with the utmost urgency.

The evidence we received also shows that the UK lags behind some European countries in the provision of specialist services, support for independent living and survival into mid-adulthood for men with Duchenne Muscular Dystrophy. We strongly believe that second-rate care resulting in shorter lives and limited survival in this country is not acceptable. We have therefore brought forward a number of recommendations which call for prompt action to address the failings in services in this country.

It became clear during the course of the Inquiry that the responsibility for the development of specialist neuromuscular services lies at several levels within the NHS. This has resulted in a lack of accountability in terms of who is personally responsible to patients and families for addressing the service failings that lead to shorter lives and cause avoidable suffering and distress. We were advised in one of the later sessions that the Strategic Health Authority (SHA) has an oversight role to ensure that the local Primary Care Trusts and the Specialised Commissioning Group in each region carry out their duties effectively. We will write to each SHA on this point and also formally request them to respond to the 'Walton Report' as a whole.

The Inquiry took some very encouraging evidence from leading researchers in the neuromuscular field who highlighted the clinical trials of treatment that are at last under way. We fully accept that the research infrastructure must be developed in the UK to enable trials to be conducted at additional centres and thereby to allow more patients to participate in the trials. The goal should be to develop a national network of research and trial centres alongside specialist neuromuscular clinical services in each region.

We received expert evidence regarding the weak support available to young people making the transition from paediatric to adult services. Again, it is not the case that no-one knows how the problems can be addressed, as there is clearly some good practice, and effective services are provided now in several Trusts and PCT areas. Rather, it is more worrying to the members of the APPG that the actual problem that has to be addressed is

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the failure of service planners and commissioners to provide support to these vulnerable young people at this difficult period in their lives.

The members of the APPG were all impressed with the evidence provided by patients and family members during the course of the Inquiry. They were unfailingly helpful and direct in giving their evidence despite the often difficult personal circumstances they have to manage on a daily basis, particularly those who are living with advanced muscle disease. The members of the APPG all feel that we owe it to the patients and families to ensure that this Inquiry leads to effective change and improvements in health and social care, in access to clinical trials and in support for vital research.

We also heard evidence from a number of witnesses about the very effective work of the Muscular Dystrophy Campaign. We wish to endorse these views and give our own thanks to the Muscular Dystrophy Campaign for their tireless efforts to address these issues and for bringing them to our attention.

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## **What is muscular dystrophy?**

There are more than 60 different types of muscular dystrophy and related neuromuscular conditions. It is accepted that approximately 1,000 children and adults for every 1 million of the population are affected by muscle wasting neuromuscular diseases in the UK. It is therefore estimated that some 60,000 people are affected by a neuromuscular condition in the UK.

Many neuromuscular conditions are low-incidence, orphan conditions and indeed some are very rare and are regarded as ultra orphan. Neuromuscular conditions can be genetic or acquired and, with the exception of a couple of acquired conditions, there are no known effective treatments or cures. However, clinical trials in some forms of muscular dystrophy are now under way and it is hoped that these will lead in time to the introduction of new treatments that can slow down or arrest the progressive nature of these often devastating conditions. There is a pressing need to develop the clinical trials infrastructure with additional trial centres in the UK to enable more patients to participate in trials which in turn will help to hasten the development and introduction of new treatments.

## **Muscular Dystrophy Campaign**

The Muscular Dystrophy Campaign is the leading UK charity focusing on all forms of muscle disease. The charity is dedicated to improving the lives of all people with muscle disease. Founded in 1959, the Muscular Dystrophy Campaign funds vital research, provides and supports care services and also gives information, advice and direct help to individuals living with these conditions.

The charity also campaigns effectively to ensure all people living with muscular dystrophy or a related neuromuscular condition have access to high quality health and social care services and it works with Parliamentarians across the UK.

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## Section 1

### Evidence Summary

#### What constitutes specialist multi-disciplinary care?

Oral evidence was provided by:

*Professor Michael Hanna – Consultant Neurologist, MRC Centre for Neuromuscular Disease, National Hospital for Neurology and Neurosurgery and Institute of Neurology, London*

*Dr David Hilton-Jones – Consultant Neurologist, Clinical Director, Oxford MDC Muscle & Nerve Centre, John Radcliffe Hospital, Oxford*

*Dr Michelle Eagle – Consultant physiotherapist, Newcastle Muscle Centre*

*Dr Stephen Lynn – Project Manager, TREAT-NMD, Newcastle*

1. Listening to experts giving evidence, we were left with the clear impression that a patient with muscular dystrophy or related neuromuscular condition who has access to a specialist multi-disciplinary team for care, treatment and support will enjoy increased quality of life and potentially a prolonged life. We repeatedly heard about the big gaps in a broad range of the components of specialist neuromuscular services across the country. Professor Michael Hanna made the following analogy with cancer:

If you had a treatment that would prolong life by 10 years [for a type of cancer] that was available in Newcastle but not in the South West, it would be an absolute outrage.

**The Department of Health and the NHS (together with each of the National Health organisations in the devolved countries) should ensure that specialised paediatric and adult neuromuscular services are highlighted for commissioners based in both regional Specialised Commissioning Groups and in local Primary Care Trusts by giving specialised neuromuscular services their own place in the National Definition Set to reflect the urgent need to treat these services as a priority following years of under-investment and weak coordination. We call on SCGs to follow the lead of the South West SCG and West Midlands SCG and conduct an urgent in-depth review of neuromuscular services in the regions. (Recommendation 1)**

2. We heard alarming evidence that in many regions of the country, the diagnostic process for muscle disease is severely undermined by the lack of a team with specific neuromuscular expertise. Professor Hanna told us:

The diagnostic process is clinical assessment, muscle biopsy and genetic testing; in many regions of the country the combination of a specialist clinician with training in muscle disease and a muscle pathologist just doesn't exist.

3. We gathered from experts that the lack of a proper diagnosis has wide and long-lasting implications for an individual with such a condition and for the rest of the family.
4. We learned of the vulnerability of the specialist multi-disciplinary services and that it is the enthusiasm and dedication of individual experts around the country which builds up and maintains the specialist service. This is extremely disturbing when considering the long-term stability of those specialist services. Professor Hanna emphasised the vulnerability:

It is not part of the commissioning system to ensure an adult muscle specialist, proper muscle pathology service, paediatric muscle specialist and proper physiotherapy service. It is just by the luck of interested people.

5. Evidence also suggests that even with the enthusiasm of interested individuals, if the decision-makers with the funds do not view neuromuscular services as a priority, then services are at risk of severe weakening or collapse. Dr David Hilton-Jones informed us:

Even if someone has a specific interest, they will have to battle to get resources from the Trust to provide for patients with neuromuscular disease, because neuromuscular conditions are not subject to current government targets for improvement of health care.

**Further, we are convinced that similar action is urgently required to ensure that vital support is accessible from professionals such as physiotherapists, speech and language therapists, occupational therapists and dieticians. Muscular dystrophy and related neuromuscular conditions are complex, multi-system disorders that can be managed effectively with specialist multi-disciplinary care, but too many patients are currently being failed by the NHS. We therefore call on all health commissioners and managers to assess the needs and gaps in professional support in their local area and produce a development plan to address service weaknesses by March 2010. (Recommendation 5)**

6. Evidence we received depicts a worrying situation in relation to physiotherapy services across the country. Where there is physiotherapy provision, it often appears to be in blocks of treatment and not ongoing over a long time period. Dr Michelle Eagle emphasised the enormous difference physiotherapy can make to quality of life, during her evidence submission. Further, she highlighted the distances people have to travel to receive specialist physiotherapy:

Patients who come to Newcastle are increasingly flying in from Northern Ireland, Scotland, or the South of England because they can't get services and advice locally.

7. A clear message to us was that specific NICE guidelines would be of great help in improving access to specialist care.

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**Further, we call on NICE to put in place a Clinical Guideline for Duchenne muscular dystrophy, building on the existing Standards of Care developed by the TREAT-NMD European consortium; this will help to drive up standards and also give patients and families a clear understanding of the standards of care to which they are entitled. (Recommendation 7)**

8. The current lack of consistency of services in a broad range of aspects of specialist care was demonstrated by descriptions of the patchy nationwide hydrotherapy service and the shortage of neuromuscular nurse specialists.
9. Evidence received in both oral and written evidence also highlights the vital position of a Care Coordinator, who provides crucial support to individuals with muscle disease and their families. We have learned that for there to be long term stability in these posts, they need to be embedded in the NHS.

### Impact of research on specialist multi-disciplinary care

Oral evidence was provided by:

*Professor Kate Bushby – Newcastle Muscle Centre*

*Professor Francesco Muntoni – Dubowitz Neuromuscular Centre, Great Ormond Street Hospital, London*

*Professor Volker Straub – Newcastle Muscle Centre*

10. We heard evidence that the need to increase capacity for trials in the UK is an issue which needs to be urgently addressed. Centres in London, Newcastle and Oswestry are currently taking part in international trials. Since the UK is at the limits of its current research capacity, researchers in the neuromuscular field are seeking commitments to capacity building to ensure Britain's role in international trials. Professor Francesco Muntoni expressed his concerns to us:

Neuromuscular conditions are more in the focus of translational research and therapeutic delivery. There will be much more focus in the next 5-10 years. Capacity is a concern for all of us, both clinically and in terms of the delivery of complex therapies in the future.

11. Professor Kate Bushby explained how research and clinical expertise can be combined in a specialist centre:

If you have an area, e.g. Birmingham, where there are clinicians to look after both children and adults with neuromuscular diseases, and you had a push to build up a research team around them plus extra capacity for these clinical people to have academic time, you could see that building into a centre which is able to operate very effectively quite quickly and I think that model would work quite well.

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**We recognise that ensuring patients' participation in clinical trials is vitally important, but there are currently too few centres in the UK with the infrastructure to support trials for neuromuscular conditions. We underline our call for urgent action to improve the care and treatment provided for patients. It should be noted that this will also help to develop additional tertiary and secondary centres with the capacity to participate in clinical trials. There is clear interest in developing new treatments from a number of biotechnology and pharmaceutical companies whose investment is given some protection through, for example, the easing of guidelines regarding the size of clinical trials and extended patent protection in Europe (and the USA) achieved by the relevant legislation. (Recommendation 9)**

12. We were alarmed to discover that the standards of specialist care for people with muscular dystrophy and related neuromuscular conditions in the UK fall behind those in other European countries. Professor Volker Straub highlighted his concern to us:

When it comes to adult care in the UK, the lack of care for patients is sometimes shocking, in particular in terms of therapy. It is unimaginable that in other countries you wouldn't have access to regular physiotherapy for years – for the rest of your life. In the UK it is a real fight to just ask for some basic care like physiotherapy for adult patients.

13. We ascertained that basic care and treatment need to be in place for patients to be enrolled on trials. Professor Straub emphasised the importance of high standards of care for the purposes of trials:

You can only participate if you have ensured that patients enrolled receive the same basic care and treatment, otherwise this could influence the outcome of the trial. So we need to ensure that we have the highest standards of care across Europe, that it is harmonised, and that our patients aren't left behind.

**While the current, early stage (Phase I and Phase II) clinical trials are extremely encouraging, it is essential for patients' participation in future trials to ensure that our standards of care are harmonised with the best available elsewhere in Europe. Otherwise UK patients will be excluded from participation in trials and the outcome of the trials could be influenced or skewed. While the rate of progress now being made in taking research from the laboratory bench to the patient's bedside is most encouraging, we caution that eligibility for the current trials is only possible for that proportion of the patient population who have a particular genetic mutation responsible for many cases of Duchenne muscular dystrophy. (Recommendation 8)**

14. We were clearly told during evidence submissions that funding for research infrastructure is a crucial component of the research process. Long-term commitment by the EU member states for the Europe-wide TREAT-NMD network based in Newcastle is vital. It is important that member states and national health systems sustain and maintain projects like TREAT-NMD. Professor Straub drew our attention to the necessary political involvement:

Standards of care need to be updated, you need to have trials to define what kind of outcome measures you can use for clinical trials. This is where it is very important that there is support at a national level for what has been established within European projects and that you make sure that, in our case, neuromuscular diseases are on the research agenda of the European Commission as well. It is important to have a close dialogue between parliamentarians, patient organisations and clinicians.

15. We understand that better support for neuromuscular experts would greatly help in building up a regional network of specialists. Professor Bushby stressed the significance of this during her evidence:

There are a lot of dedicated clinicians who work in neuromuscular diseases who are stretched. They do a neuromuscular clinic but maybe don't have physiotherapy support and clinical evaluated support.

16. We heard interesting evidence that Denmark has an excellent care system for patients with muscular dystrophy with a particularly good social system which is much better than that in the UK. Professor Bushby elaborated on the system in Denmark for us:

Denmark has an excellent care system for patients with muscular dystrophy, and alongside their excellent medical care, their social care is fantastic. They all get a house/flat of their own when they are 18, complete with round-the-clock carers whom they choose, and who are young, interactive and fun.

**It has become clear that the UK falls well behind some other countries internationally in the provision of care for patients with a neuromuscular condition. Survival rates in the UK for individuals with Duchenne muscular dystrophy, for example, lag behind those achieved elsewhere in Europe in countries such as Denmark and the Netherlands. In those countries, specialist multi-disciplinary care with excellent links to supported, independent living is the norm. We call on the Department of Health to commission an urgent comparative study to identify international best practice and ensure this is disseminated in this country to clinicians, commissioners and patients. (Recommendation 6)**

## Section 2

### Evidence Summary

#### What are the experiences of patients?

Oral evidence was provided by:

*Phillippa Farrant – Eastbourne, mother of young man with Duchenne muscular dystrophy*

*Andy Findlay – Derby, living with Facioscapulohumeral muscular dystrophy (FSH)*

*Moira Findlay – Derby, wife and carer for Andy Findlay*

*Laura Merry – London, living with Congenital muscular dystrophy*

17. We have received detailed and very helpful evidence from a variety of patient perspectives in relation to their experiences of access to specialist care. There is a clear message from patients with muscular dystrophy and related neuromuscular conditions that long-term specialist multi-disciplinary care and support are essential.

18. Laura Merry, who has congenital muscular dystrophy, described to us:

I use a ventilator and a powered wheelchair. My condition has deteriorated and muscular dystrophy makes life more challenging.

19. Phillippa Farrant, whose son has Duchenne muscular dystrophy, highlighted the constant support that her son needs:

As their muscles weaken, they need more and more support. It takes over your whole life and also the parents' lives because they're the 24/7 carers. This is where all the problems are occurring. They need much more support from all the different disciplines – clinical and social services and a lot more co-ordinated care.

20. Andy Findlay, who has FSH muscular dystrophy, gave an alarming insight to us about the diagnostic process:

Up until I was 41, I knew I had got a problem, but didn't know what it was. Because of work, I needed to go to the doctor's. From there, I found out that I had FSH md. I was told, "Go away, deal with it, there is no cure." It was a bit of a shock.

21. We heard about the obstacles to receiving satisfactory and sufficient specialist care and support; these were outlined during oral evidence submissions. Phillippa Farrant commented on the situation she and her son face regarding physiotherapy, hydrotherapy and respiratory support:

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Physio has been offered since diagnosis. He receives hydrotherapy at school, but nothing at all for 6 weeks when he comes home for holidays – the water is so good for their muscles and movement. It's very patchy countrywide – some people get it, some don't. Respiratory is just beginning to be an issue. We'll be travelling to London for this since there is nothing locally.

22. We heard once again how much of a battle it is to receive ongoing physiotherapy, despite the significant impact it has on quality of life for people with muscle disease. Andy Findlay related his experience to us during his evidence submission:

I don't have regular physio, I have to fight for physio, we are made to feel like we are taking it away from someone else because we're not going to get better so what's the point. They never actually say that but that's the impression you get.

23. We received evidence in relation to difficulties and positive aspects of the transition from paediatric to adult services. Laura Merry emphasised the challenging nature of moving from paediatric to adult care:

I have been fortunate with my paediatric care but moving into the adult services has been challenging. There should be a young people's ward because of the different needs. I had to sort out physio, respiratory and back-up for uni.

24. Phillippa Farrant drew our attention to the issue that services and expertise have not caught up with the fact that boys with Duchenne are living longer:

The goal posts have changed for Duchenne, with boys now living into adult life whereas they weren't before. However, the people with expert knowledge are not there in adult services to support the children – around the country there are people not being seen, there is no-one to support them. Therefore, they are being given up on. The clinicians are not in place to help them. Therefore, all the good work done over the years in childhood is wasted because of the serious effect on quality of life and lack of support.

25. Patients have expressed their frustrations at the widespread problems in accessing hydrotherapy services. We are fully aware of the benefits of hydrotherapy, particularly in the winter months, and we received enlightening evidence, gaining an understanding of the nationwide picture of hydrotherapy services. Laura Merry highlighted the cost of private hydrotherapy during her evidence submission:

Now I have to pay for hydro once a week myself, £20 a session – approximately 45 minutes. It is therefore £40 when both and my sister and me have hydro. Both are essential – hydro helps my muscles and lungs a lot.

26. It was extremely alarming to learn of the prohibitive costs of using hydrotherapy pools and the inaccessibility and lack of coordination of hydrotherapy services at certain times of the year. We were shocked to hear of Phillippa Farrant's and her son Daniel's experiences:

Daniel gets hydro at school but not in the holidays because there is nowhere locally. There is a hydro pool at the local hospital, but we can't get funding for Daniel to use it. If a group of us would like to use it, the hospital would charge us £200 for a half hour session. There is a local school for disabled people with a hydro pool, but we can't get access to that, it's only there for the school pupils so that sits empty in the holidays as well. There are community nurses from the hospice who would be willing to take these children for a session, even if only for once a week.

**We heard in many of our evidence sessions of both the value and benefits of hydrotherapy for many people with a neuromuscular condition but also the difficulties often encountered in accessing a local hydrotherapy pool. We therefore call on the Department for Communities and Local Government to undertake an urgent review of access to hydrotherapy pools across the country, involving the Local Government Association, the Muscular Dystrophy Campaign and other organisations. The review should be completed and recommendations brought forward within 12 months to ensure that all people living with a neuromuscular condition have the opportunity to use a fully accessible, local hydrotherapy pool, if they wish. (Recommendation 15)**

27. We received written evidence from Martyn Blenkarn, whose personal experience emphasises the postcode lottery of quality of services in the UK:

No hydrotherapy available in the area – private arrangements can be made but no assistance from NHS physiotherapists can be obtained.

Compared with the previous PCT area (North Cumbria) which covered the area where I used to live, the service in my current PCT area (North Lancashire) is totally unsatisfactory – the quality of care from those who treat me directly is fantastic, but their hands are tied to provide what is really needed.

28. We are shocked by the situation that Ameena Berkowitz described in her written evidence in relation to accessing ongoing physiotherapy, and we are appalled at the attitude of the therapist, which caused her distress:

I have had a long (and ultimately unsuccessful) struggle to access suitable, ongoing physiotherapy and hydrotherapy. In the end I have chosen to access private care, as hospital physio services are simply not geared up for ongoing support. Unfortunately I do not have the type of equipment that would be available in a hospital gym, such as a standing frame and physio plinth.

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I had a long search to find a good private physio. At one point I was being seen in hospital outpatients and asked for a recommendation but was told by the therapist under whose care I was that I was unlikely to find someone to work with me as “physiotherapists do not like working with people who don’t get better”. This obviously made me very angry and depressed and I considered making a formal complaint, but did not have the energy.

**While the focus of this Inquiry was on health care, we also heard evidence from several witnesses which highlighted stark failings in social care and a lack of the support to which people are entitled to enable them to lead full and meaningful lives, living as independently as possible. It is clear that many people are encountering significant problems in accessing support to take up and retain employment, to secure home adaptations in a reasonable timeframe, to use public transport that enables them to travel independently and with dignity and in accessing higher and further education. These are all extremely important areas which require urgent, coordinated action by the agencies concerned to address the issues drawn to our attention. Therefore, we call on the Department of Health, the Social Care Institute for Excellence and the local government associations to undertake a systematic review of social care support for people living with a neuromuscular condition by September 2010. (Recommendation 17)**

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## Section 3

### Evidence Summary

#### Commissioning and funding

Oral evidence was provided by:

*Steve Collins – Deputy Director of NHS Specialised Commissioning*

*Deborah Evans – Chair of South West Specialised Commissioning Group and Chief Executive of NHS Bristol*

*Louise Tranmer – Director of South West Specialised Commissioning Group*

*Eamonn Kelly – Director of Commissioning, West Midlands Strategic Health Authority*

29. We learned from these witnesses of the structure of commissioning, both at national and regional level and in consequence we had serious concerns regarding the ability of specialised commissioning bodies to effect improvements to neuromuscular services nationwide and to ensure long term stability.

**The responsibility for ensuring that access to specialised, multi-disciplinary neuromuscular services is available for all patients should lie with a named individual in the National Specialised Commissioning Group and with a named individual in each of the English regions and devolved Health administrations. (Recommendation 2)**

30. After hearing the submissions from these witnesses, it seemed to us that the National Specialised Commissioning Group (NSCG), in its current form, is unable to exert direct influence on decision-making of regional Specialised Commissioning Groups (SCGs). Steve Collins outlined the remit of the NSCG to us:

The function of the group is to advise Ministers on the designation of the very top end of services – tertiary and quaternary – national specialised services, but also to pull together strategies and plans across the 10 regional SCGs. For the regional services, the SCGs are made up of PCTs and we don’t have power over the SCGs. They have a delegated responsibility from the PCTs. Of course the PCTs are ultimately the commissioners. The SCGs, as a collection of PCTs, have that decision-making responsibility.

31. We were alarmed by the specialised commissioners’ lack of knowledge and the understanding of the Specialised Services Definition Set that appears to exist in various disciplines of specialist care. We have made it clear that we strongly believe that neuromuscular disease should stand alone as a clearly identifiable service since the multi-disciplinary nature of specialised services for these conditions means that they can be developed within a number of settings including rehabilitation, genetics, paediatrics or neuroscience. The ineffectiveness of the National Definition Set is exemplified in the following explanation by Steve Collins during his evidence:

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The reality is that none of the 35 definitions are commissioned by all 10 SCGs and no SCG commissions all 35 of them, but where they are commissioned, they are helpful for SCGs to understand what they are commissioning and how.

- 32.** We want to see as much of the specialised care that directly benefits patients as widely available as possible, and most importantly, sustained. We welcome the short term improvements achieved by the campaigning work of the Muscular Dystrophy Campaign but we need something that is going to be there long term. Steve Collins was asked about whether the NSCG would work with the Muscular Dystrophy Campaign. Steve Collins responded:

I really would love to. The reports that the Muscular Dystrophy Campaign – Robert Meadowcroft, Director of Policy and Operations at the Muscular Dystrophy Campaign, and his team at the charity – has put out are absolutely fantastic and it is remarkable how they have turned this around in such short time with a small staff. I travel around the SCGs a lot and you absolutely have their attention. I was down at South Central last week and the first thing they asked me about when I walked through the door was this [the Muscular Dystrophy Campaign’s South Central report], and so I am absolutely delighted and privileged to work with Robert Meadowcroft.

- 33.** We were offered assurances by Steve Collins that the NSCG would play a role in assisting on key workforce planning issues:

Certainly if an issue like this where there is a very thin pool of people to draw from starts to emerge as a national issue, it is absolutely the role of the NSCG members i.e. [SCG Chairs] such as Deborah Evans and us as the team that support them, to try and identify that and try to find a way through, while working with Robert Meadowcroft and other experts.

- 34.** We recognise the part that the South West SCG has played in the investment in specialised neuromuscular services in the region through the November 2008 Neuromuscular Service Development Strategy. A commitment of £1million investment in neuromuscular services in the South West was also agreed in writing by Sir Ian Carruthers, Chief Executive of the South West SHA.

- 35.** We were keen to hear the benefits that patients would see in the region as a result of the investment and sought reassurances that improvements to specialist services are imminent for people living with muscle disease in the region. Louise Tranmer explained the possible structure of services to us:

We have got a big workshop with our clinical colleagues in July and we will take final decisions there but I would estimate that Bristol will have a significant role because of its role in the care of children with specialised needs, and also Plymouth. Southampton will play a role because it

provides specialist services to parts of Dorset, Bournemouth and Poole. Of course, because our geographical area is so large and a particular shape, we need to make sure that the services that we design reach out into the community as well as having centres of excellence.

- 36.** Louise Tranmer went into more detail in relation to the proposals for care coordination and physiotherapy when the new investment allocated translates into improved services in the region:

Part of our proposals include appointing Care Coordinators that will stay with you throughout your life, supporting you and your family, helping you to connect not just to specialised services but also to your local services and helping people to live their lives while they manage their condition, which I think is very important. It is one of things that the users and carers who were part of our development process said was most important to them.

Some of our resources are identified for additional physiotherapy posts that will be positioned around the South West, perhaps associated with our centres and then outreaching, as well as being involved in multi-disciplinary clinics where physio and OT and the clinicians are together. We’ve identified a need for specialist physiotherapy and it is in our strategy, but some patients have felt they have needed to seek treatment privately and of course we hope that they won’t need to do that in the future.

- 37.** We recognise the key issue of the funding mechanisms and the different options that the PCTs choose from when agreeing to commission services. Deborah Evans explained to us the source of the funding:

The PCTs have to commit the money for specialised services from their allocations from the Department of Health. So we have collectively a budget for all specialised services across the South West – about £400million this year.

- 38.** We heard evidence from Louise Tranmer regarding the weighted capitation system of funding:

The funding – for the neuromuscular strategy – the contributions were agreed on a weighted capitation basis according to population size and so on, using a fairly standard formula that we use in other situations. Sometimes we use different arrangements – it depends on the nature of the service.

- 39.** We were encouraged to hear that the tariff system should not be an obstacle to the pathway of treatment. We are concerned that there may be inflexibility by commissioners, but Eamonn Kelly emphasised during his evidence:

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The tariff system is simply a tool in the system and not an end in itself so we shouldn't allow any tariff to determine the pathway of treatment for any patient. In those circumstances if the tariff was providing a disincentive to develop the service, I would expect commissioners to be much more flexible and to look at payment outside of tariff, and there is permission to do that.

**40.** We warned that in the South West region, it might be difficult to find the right kind of consultants with expertise and knowledge of neuromuscular disease and therefore advised that it may be worth considering appointing someone of good quality without expertise in the field and then sending them to a specialist centre for several months to learn the important techniques in the neuromuscular field. Louise Tranmer acknowledged the suggestion and stated that this could be one of the ways in which they tackle these posts.

**41.** We were interested to establish whether the West Midlands would be going through a similar process to the South West. Eamonn Kelly responded during his evidence submission:

We have been asked by the Muscular Dystrophy Campaign to carry out a similar review and I am pleased to confirm the intention to do that. The SHA is very keen to provide some sponsorship and financial support to make that happen quite quickly. We, as part of that process, will make sure that we learn from colleagues in the South West and adopt what they have found, which is easily transferable across to the West Midlands. Many of the issues you have raised already with colleagues from the South West will of course will be relevant to the West Midlands.

One of the things that also struck me from the observations in the West Midlands is that many of the deficits in the services are local services – wheelchair services for example. These are not specialised services – these are services that are provided – I managed wheelchair services when I was a PCT Chief Exec. In 2002 we had the sort of waiting times that were described in the Muscular Dystrophy Campaign's report. We were ashamed of those and I thought that those had been removed everywhere, so if they haven't, we need to tackle that.

It is critically important that commissioners sit down with clinicians to understand the challenges that they are facing.

**42.** We were reassured that there is appreciation of the fact that work needs to be done to ensure that patients with muscle disease in the West Midlands receive the specialised care and support that they are entitled to. Eamonn Kelly reported to us:

All 17 PCTs in the West Midlands are signed up to developing and being able to offer personal health budgets for people with long term conditions, and end of life care as well, which is particularly relevant here.

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We have got to put our money where our mouth is now and get on with this and make it happen of course, but it touches all of the things that certainly as an SHA we feel that the service needs to be doing now. I think we have by and large made really good use of all the extra resource but the experience of this group of patients is evidence that personalising the service is still a big challenge for us and making sure that highly specialised services are where they need to be.

**43.** We established from Eamonn Kelly that the West Midlands PCTs are committed in terms of services for neuromuscular disease:

We've got a commitment from all PCTs to work together on this, which is the first stage. By the autumn, it will be clear about the extent to which that is translated into investment.

In the less specialised services where some of what you've found is very uncomfortable, I can't imagine that my PCT Chief Executives colleagues wouldn't feel exactly the same about this. One of the first questions I will be asking them is to have a look and be clear themselves about access on basic things like wheelchairs.

**44.** We were keen to hear the thoughts of those attending the specialised commissioning session. Philip Butcher, Chief Executive of the Muscular Dystrophy Campaign, asked the panel about accountability and longevity of neuromuscular services in the region. The clear message from Eamonn Kelly was:

I am absolutely clear that it is the responsibility of the SHA – it is our responsibility to ensure that PCTs put in place arrangements for commissioning services for the people of the West Midlands, including specialised services. It is our responsibility to supervise it.

**45.** At the heart of the Inquiry into access to specialist care are the people living with muscular dystrophy and related neuromuscular conditions. We were very moved to hear the contributions by Sharon and Terry Kitcher from Plymouth, whose son has Duchenne muscular dystrophy.

**46.** There can be no excuse for delays in improving health services and links with social care when every day is vital for those living with these often devastating conditions.

**47.** We hope that the panel of national and regional commissioners were left in no doubt as to the urgency of implementing improved specialised neuromuscular services.

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## Section 4

### Evidence Summary

#### Transition into adulthood

Oral evidence was provided by:

*David Abbott – Senior Research Fellow at the Norah Fry Research Centre, University of Bristol*

*Emily Ballard – Specialist Transitional Care Therapist at the Lane Fox Unit, Guy's and St Thomas' NHS Foundation Trust, London*

*Dr Nicholas Hart – Clinical Research Consultant and Honorary Senior Lecturer in Respiratory and Critical Care Medicine at the Lane Fox Unit, Guy's and St Thomas' NHS Foundation Trust, London*

*Stuart Reid – Solihull, living with Duchenne muscular dystrophy*

48. We were extremely alarmed to hear about the severe lack of essential long-term assistance in transition planning. David Abbott, who has been conducting research looking in detail at transition into adulthood for young men with Duchenne muscular dystrophy, reported to us:

It seemed shocking that 20 years on from [national policies setting out] basic duty and frameworks around transition that should kick in at around 14, more than half of families couldn't recall any structured transition planning, looking back or currently.

49. We heard first-hand evidence that adult services lack the organised structure that exists in paediatric services. Stuart Reid explained his perspective on the service that he has personally experienced and the problems at transition to adult services:

Paediatric services are more structured and people seem to know what they're doing more. I had a particularly good social worker when I was younger.

**Further, a recurrent problem about which we heard from several expert witnesses relates to the paucity and weakness of adult neuromuscular services in many parts of the country. Even where there are reasonable or good paediatric services in place, the provision of adult services is generally much weaker and this is unacceptable. Where good multi-disciplinary services are well co-ordinated, they involve specialities such as genetics, a muscle pathology service, cardiology, respiratory and cardiac support and specialist physiotherapy. The successful development and management of specialist care leads to a growing population of adults who in earlier times would not have survived, and these people have a right to continuing, high quality support to ensure they enjoy the best possible quality of life, as in some other European countries. We call on health service commissioners, local authorities and the local government associations to ensure they develop effective plans to guarantee they meet the comprehensive health and social care needs of this growing number of adults. (Recommendation 11)**

50. We received very helpful evidence regarding an explanation of transition services in the context of specialist health care. Dr Nicholas Hart gave us an insight into the overlap of different services within specialist multi-disciplinary care:

Transition is difficult to pinpoint as a clinician in terms of start and end – 14, 18? There is only one transition service – in neurology. In respiratory, I only come in from about the age of 12. A cardiologist may be seen but the cardiac complications are later on. People are slotting in. We are in a co-ordinating role and pull in the specialist services as needed. We are allowed to have this set-up because we have the support.

51. We gathered further evidence of the long distances that patients travel to access specialised neuromuscular services at specialist centres. Dr Nicholas Hart gave an overview of how far people come to the Lane Fox Unit:

We have referrals from Birmingham and Southampton. Although the service stretches down to the south coast, we take patients from many different parts of the country, partly driven by patient choice through GP referral.

52. We had clear evidence that young people are frequently frustrated by over-reliance on family support and care. Stuart Reid told us how he is often forced to rely on his parents for his care:

I need to get extra funding from the local health authorities. Mum or Dad sometimes has to care for me during the night once or twice a week. I don't want to have to rely on them. I want them to be able to get on with their lives. My care assistant was meant to come with me. Dad had to cancel business meetings to come with me today to help me.

53. After hearing about the specialist multi-disciplinary team that is in place at the Lane Fox Unit, this further highlights that young people greatly benefit when all aspects of specialist care and support are put in place. Good practice must be shared and replicated across the country.

**We call on the Government and commissioners to take urgent action to make sure that a named transition co-ordinator is in place for each young person with a neuromuscular condition who is moving from paediatric to adult services. We heard evidence that transition services to provide support to young people when they move from paediatric to adult services are very weak in many parts of the country. (Recommendation 10)**

54. We recognise the need for good links to be established and maintained between a specialist centre and the community, as exemplified by Emily Ballard during her evidence submission:

I have been doing a lot of education with community teams and sharing my skills so that hospice staff, community nurses and community physios

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feel confident with the new equipment for these patients and they understand the disease needs.

A community nurse with adults who has not dealt with the disease and doesn't know what to do – if we can support and educate them then they will be able to deal with them in the future rather than saying that the patient should just go to the specialist service and do everything there. There is a huge role for joint work with the community teams and that has been really successful so far.

- 55.** The joint working described here should be developed and implemented across the country. Patients prefer to access integrated services as close to their home as possible – provided they are comprehensive and good quality. This clearly results in financial savings compared with referrals to attend specialist centres some distance away.

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## Section 5

### Evidence Summary

#### Importance of the role of the Care Coordinator

Oral evidence was provided by:

*Jane Stein – Regional Care Advisor/Coordinator<sup>1</sup>, Oxford*

*Dr Laura McKeown – Regional Care Advisor/Coordinator, Northern Ireland*

- 56.** We have established that where there is care coordination by a professional, it is an extremely valuable addition for someone with muscular dystrophy and their wider family. Jane Stein outlined to us her extensive role as a Care Coordinator in Oxford:

I work linking home and clinic – there is both an adult and paediatric clinic in Oxford and I will liaise with consultants running those clinics as well as other staff so that they're aware of what's happening in a family's life and what the issues for that family might be beyond the medical. We have a big role liaising with local services and acting as an advocate because we need to work effectively with local services such as social services, education and health. People live in their local communities and that's where they need accessible services in everyday life and in points of crisis. So I am involved in individual work with OTs, social workers, care managers and schools. I am involved in running training events and clinical networks so that we can bring together people who don't have an opportunity to gain a wide range of experience but they still need a degree of expertise and understanding about the conditions and a network to plug into as other professionals may have answers to their queries.

- 57.** We are deeply concerned that due to capacity in terms of the number of patients on their caseload, Care Coordinators are often unable to build relationships with families before encountering an upsetting crisis management situation. Jane Stein expressed her frustrations during her evidence submission:

I would like to be able to visit families at home to build up a relationship before a problem develops. It is often now a case of crisis management – responding when a family says there is a difficulty.

- 58.** We were alarmed by the workload that Dr Laura McKeown has in her care coordinating role for the whole of Northern Ireland. She outlined her role to us during her evidence:

I have about 450 clients on my database. I provide advice and support on a range of issues – housing adaptation, equipment issues, benefits, counselling and support, particularly at time of diagnosis, and crisis

situations in people's lives, I signpost patients to services, making onward referrals to social work. I run information days, conferences, family weekends, which give service users the opportunity to get together and meet people with similar diagnosis. I provide advice and training to health care professionals – as you know, not all are experienced at dealing with people with neuromuscular conditions. I go to OT departments, and deliver training and guidance to them; I work on a one to one multi-disciplinary team basis, for example if a family has an issue with housing adaptations – I would become involved and give advice and guidance. I have a very wide-ranging remit.

59. Dr McKeown, as the only care coordinator for Northern Ireland, faced the same constraints as Jane Stein and explained the imbalance she feels that exists with her post:

There is only myself to fill this role, I feel very reactive in terms of problems, meeting people at clinic, helping deal with problems. It should be much more pro-active, there is an inequity in the service if people don't contact me or I don't meet them at clinic.

**We found it a matter of great concern that most individuals and families living with a neuromuscular condition do not have a named individual to provide advice and support at critical times during the disease process. There are currently only 13 Neuromuscular Care Coordinators. To provide adequate support to the 60,000 people living with these conditions a network of some 60 Care Coordinator posts is required. Furthermore, we heard many patients and families, together with the leading clinicians, praise the initiative and commitment of the Muscular Dystrophy Campaign in funding eight of these Care Coordinator posts in several parts of the country. Due to the reliance on charitable funding, these eight existing posts are extremely vulnerable. Investment in these posts is cost-effective as they have been shown to save consultants' time, reduce emergency admissions and re-admissions, reduce hospital stays and coordinate care locally. We call on the DH and the NHS to introduce a funded development plan (working with the Muscular Dystrophy Campaign) by the end of March 2010 to retain the existing posts and to ensure that a national network of Care Coordinators is established within 5 years. (Recommendation 4)**

## Section 6

### Evidence Summary

#### Professional development – specialist staff and workforce planning

Oral evidence was provided by:

*Professor Caroline Sewry – Professor of Muscle Pathology, Oswestry and London*

*Dr Tim Wreghitt – Royal College of Pathologists*

*Annie Aloysius – Specialist Speech and Language Therapist, Great Ormond Street Hospital and Hammersmith Hospital, London*

*Dr Linda Marks – Consultant in Rehabilitation Medicine, Royal National Orthopaedic Hospital in Stanmore, London*

*Gary Robjert – Head of Public Affairs, Chartered Society of Physiotherapy*

**The lack of Workforce Planning for specialist neuromuscular clinicians has been a recurrent theme throughout the Inquiry. This must be addressed as a priority as highlighted in the NHS Next Stage Review. While there are some excellent services provided at the four national muscle centres, they are vulnerable through their reliance largely on the research interests and drive of the individual leading clinician. These services must be protected and strategically developed with succession planning. We call for a summit meeting to be organised by NHS Workforce Planning with the leading neuromuscular clinicians, the Royal Colleges, the other professional bodies and the Muscular Dystrophy Campaign to develop and implement a Neuromuscular Workforce Plan by June 2010. (Recommendation 3)**

60. We established from the evidence submissions that recognition of neuromuscular disorders as specialist is crucial, with robust commissioning and service delivery for the whole field of diagnosis, treatment, management and care of patients with neuromuscular disease on a national basis. Professor Caroline Sewry, who will shortly be retiring herself, emphasised the importance of this:

I want to see the full recognition of neuromuscular disorders as specialist and to ensure we have the training to go with that and planning for succession.

61. We are deeply concerned that there is a widespread lack of planning across the multi-disciplinary care posts which results in services being made extremely vulnerable. We fear that specialist posts will be disbanded or broken up into less specialised disciplines. Professor Sewry highlighted her concerns and drew attention to the patchy service across the country and the changing field of neuromuscular services:

Many of us have been in the field for a long time and it is encouraging that the field is expanding and developing but we need new people to

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come into it. The planning is not there in any sphere and so it makes the whole service vulnerable. It is very patchy across the country. In some areas it is better than others. If you live near a specialist centre, then you will do much better than if you live a long way away from one.

- 62.** We received evidence from Dr Linda Marks, who expressed her frustrations at the significant shortage of clear commissioning guidelines for conditions where there are small numbers:

We are dealing with fairly rare conditions and therefore it is absolutely pivotal that we have clear commissioning guidelines for the small, vulnerable groups. We don't have that at the moment. There is a lack of joined-up thinking when you're talking about funding lifetime needs which may cross health, social and emotional and psychological boundaries.

- 63.** We are extremely alarmed at the lack of understanding some commissioners have of the ongoing commitment and care that are required for many patients with muscle disease in terms of their wheelchairs, as highlighted by Dr Marks:

One of the issues and battles is getting commissioners to realise that people who need wheelchairs don't just need one wheelchair for life but they need that wheelchair maintained and repaired and reassessed and a new prescription given.

- 64.** A national commissioning lead on succession planning would make it more likely that regional health authorities and commissioning bodies would make training for smaller prevalence specialties a priority. Dr Tim Wreghitt emphasised this to us during his evidence submission:

Mostly the SHAs, workforce commissions etc. don't see the need for training in these important but smaller specialties. So national commissioning is really important and with more patients, we do need to make sure that the diseases are properly diagnosed and training places for succession planning are provided.

- 65.** We are shocked that existing concentrated expertise is being threatened by the decommissioning of services and the break-up of specialist teams. The service that Dr Marks and her team provide to north London is encountering this scenario, as she explained to us:

Commissioners feel that [the service] can be run more 'cost-effectively' by doing it locally. So the expertise of my team is going to be dissolved and dissipated.

- 66.** It is alarming that this reduction in the expertise available is happening despite Specialised Wheelchair Services being recognised on the National Definition Set for Specialised Services.

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- 67.** It is a deep concern to us that there is a distinct lack of urgency and appreciation for need for long-term funding of highly specialised and crucial posts in the neuromuscular field, despite the insistence of key people that this is an issue that needs resolving. Professor Sewry told us:

There hasn't been any succession planning [for my post]. I have been saying for 10 years that we have needed to plan it but there is no dedicated funding for it.

- 68.** Annie Aloysius explained her specialised role in a neuromuscular centre and is worried about the future of her post:

I am the only dedicated Speech and Language Therapist attached to a neuromuscular centre, so the other centres rely on their child development service to provide that service. Over the years, a number of junior therapists have worked with me and gained some expertise but there is no dedicated junior post, so there is nobody waiting in the wings to take on my experience.

I think there are moves away from specialist services and more towards generic therapists, that there may be a speech therapist that specialises in paediatric dysphagia and feeding and swallowing but they wouldn't necessarily be encouraged to have one area specific expertise, such as muscular dystrophy.

- 69.** We heard of an alarming variety in the level of community services to follow up referrals from the specialist centre, as highlighted to us by Annie Aloysius:

In some places we can refer out to the community and there is a very good responsive service where the children are picked up and monitored but then there are other places where there may be nothing.

- 70.** We have concerns about the lack of recognition of the lifelong need for physiotherapy. Gary Robjent explained the situation in relation to specialist physiotherapy provision during his evidence submission:

There is a move away from the more specialist areas [in physiotherapy] ... things need to be addressed with increasing life expectancy of people with neuromuscular conditions.

- 71.** We are concerned about the growing body of evidence that specialist services are being weakened and diluted. Generic services will not meet the needs of children and adults with these rare and very rare conditions.

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## Section 7

### Evidence Summary

#### Situation in the devolved countries

Oral evidence was provided by

*Dr Louise Hartley – Consultant Paediatric Neurologist, Cardiff*

*Dr Mark Rogers – Consultant Clinical Geneticist, Cardiff*

*Rachel Salmon – Newborn Screening Specialist Nurse, Cardiff*

*Dr John McConville – Consultant Neurologist, Belfast*

*Dr Laura McKeown – Regional Care Advisor, Northern Ireland*

- 72.** Although health and social care services are devolved, we are deeply concerned about the vulnerability of vital multi-disciplinary specialist services in Wales and Northern Ireland and the disparity of service provision in the two countries. The written evidence submitted underlined these concerns in Wales, Northern Ireland and Scotland.
- 73.** We received evidence that Health Minister Edwina Hart gave a public commitment to families last year at a Neuromuscular Conference in Swansea that services for families with neuromuscular conditions in Wales would improve by October 2009. In fact, we have heard extensive evidence that services are in fact getting worse in Wales – not better.
- 74.** Further, Wales is the only UK country without any access to a Muscular Dystrophy Care Coordinator – an essential service needed by the 3,000 patients and families who are affected by a neuromuscular condition.
- 75.** We welcome the news that a paediatric neurologist with some experience of neuromuscular disorders has been appointed in Northern Ireland and will be in post in September 2009. We are also encouraged to hear that the new paediatric neurologist will receive further training.
- 76.** However, we are alarmed to hear about the gaps in service provision in Northern Ireland, as Dr John McConville described to us:

The problem has been recently that we haven't had a paediatric neurologist at all for the last 15 months. There has been a complete hiatus, and so the children have been left to the GP and community paediatricians.

Chronic neurological disability has not been targeted and has been de-prioritised, which means losing individuals like paediatric neurologists, and then it is not difficult to lose the orthopaedic surgeon who sees the children; multi-disciplinary teams all fall apart when people are not seen as necessary, and it shows the extreme vulnerability when a couple of key people go.

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**While the responsibility for health policy and service delivery rests with the devolved administrations in Scotland, Wales and Northern Ireland, we were nevertheless alarmed to hear of the decline in services in Wales over the last 10 years and the real fear that there will be further decline in the next 2 years unless action is taken now. We also heard evidence that paediatric services in Northern Ireland have been weakened by the absence of a paediatric neuromuscular consultant for several months and the vulnerability of the post of Care Coordinator which is reliant on charitable funding. The evidence submitted regarding the vulnerability of specialist services in Scotland also concerns us. We therefore strongly suggest that the Health Ministers in the devolved countries should work closely with the professional bodies and the Muscular Dystrophy Campaign to address these and related issues. (Recommendation 12)**

- 77.** The shortcomings in specialised services in Scotland were also highlighted in written evidence submissions. We received evidence from the Muscular Dystrophy Campaign that families in Scotland are being denied treatment and face major delays in the provision of essential equipment and services.
- 78.** We are extremely concerned to discover that despite health professionals' enthusiasm and dedication in the neuromuscular field, the services that they provide are extremely vulnerable. Dr Mark Rogers told us that his specialised clinical genetics service in Cardiff is under threat and expressed his concerns for neuromuscular services in Wales:

As a geneticist I have been told to stop running management clinics, seeing patients with muscle disease, because it is not seen as being part of the remit of genetics services and does not comfortably fit within their interpretation of the national definition. Also Dr Jane Fenton-May, one of my colleagues, will retire in April next year and as far as I'm concerned the service will completely implode because there will be one consultant doing part time work.
- 79.** We were shocked by the evidence regarding the complete lack of post-diagnosis care. Rachel Salmon provided damning evidence on what happens after diagnosis in her unique role of newborn screening specialist nurse:

You give a devastating diagnosis to these families and then there is no family support. This is negligence and a lack of duty of care to these families. Families are given a devastating diagnosis, and then there is no family care officer [care coordinator] in Wales unlike in Northern Ireland or Scotland.

I am also responsible for cystic fibrosis. When we pick that up, they are referred straightaway to a cystic fibrosis centre wherever it is within Wales, and they designate a CF nurse to go out to the family to provide a service of support, knowledge and specialist care. The same should apply to Duchenne families that we pick up.

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80. We express hope that care coordination in Northern Ireland will be embedded within the NHS, as outlined in Dr McConville's evidence to us:

The Department of Health have told us that they are supporting the embedding of a family care officer [care coordinator] or two posts within NHS structure, who we hope will coordinate multi-disciplinary management and provide this directly for specialist-led services, not necessarily in the short-term requiring a specialist neurologist to be a part of that.

81. We note with alarm the inflexibility of the cross-border arrangements between Wales and England. Dr Louise Hartley expressed her frustrations at the obstacles that have to be overcome on this issue:

We can't readily access specialist services, particularly diagnostic, and have to pay and argue for funding each time we have a child or adult with a rare disease. Wales had wanted to have its own in-house solutions and occasionally to pay for something outside, rather than joining in with the excellent service available in the UK and committing funding to that. We don't subscribe to NSCG funding; Wales doesn't want to be part of what England and Scotland does.

**Further, we call on the NHS National Specialised Commissioning Group and Health Commission Wales to ensure that arrangements are put in place urgently between Wales and England similar to those which work effectively between Scotland and England to enable patients to have smooth access to diagnostic services at the specialist tertiary centres in England. (Recommendation 13)**

82. We are shocked at the complete lack of succession planning and long-term funding for key posts in Wales. Dr Rogers made clear his disappointment at the lack of registrars trained and ready to take over in the event of a consultant's retirement:

There hasn't ever been any sustained succession plan, actually recognising that a given consultant with an interest will retire and on retirement needs to be replaced by someone from outside who has training, or someone who has had training within – registrars attached to the service who can then come on and deliver the service. At most registrars are attached for a couple of weeks or occasionally months, but they just come and do clinics.

83. We are alarmed by evidence received which informs us that the respiratory sleep study service in Wales has recently been withdrawn and that patients now have to travel out of Wales to access this service. Dr Hartley predicted during her evidence that this would happen and expressed her displeasure at the lack of succession planning in paediatric long-term ventilation:

Succession planning for long term ventilation – that is in complete crisis on the paediatric side – it did improve for a while. Just after I started there was a respiratory paediatrician appointed who had a very strong interest in

sleep medicine and long term ventilation but there was no commissioning for that service. She has gradually built it up over the last 4 years but completely uncommissioned and unfunded. It has fallen apart – Health Commission Wales have severely restricted the business case that she has put in to fund the service properly. As a result she has resigned and is leaving in about a month. There will be no sleep studies done at all in Wales. Everyone that needs a sleep study in a month or two will now have to go London or possibly Bristol, but probably London from now on.

84. We note with concern the poor quality of adult physiotherapy compared to paediatric physiotherapy in Northern Ireland, which mirrors the picture across many parts of the UK, as explained by Dr Laura McKeown to us during her evidence submission:

When it works it works quite well, but in most Trusts when the young adult turns 19, they are into adult services and it is very poor in terms of physio. If patients are fortunate enough to get physio, they will probably only get six sessions and that is it, and the therapists on the ground don't really have a good knowledge of neuromuscular conditions as well, and that can be an issue in terms of access and community services too.

85. We share Dr McConville's concern of the imbalance of the specialist centre care provision that boys with Duchenne muscular dystrophy receive and the lack of comprehensive local multi-disciplinary care:

A significant portion, particularly boys with Duchenne muscular dystrophy are travelling to Newcastle and London, about 15 or so, because of a lack of paediatric service. They are travelling over night and flying, and, of course, fitness to fly is an issue with respiratory failure. Although they get excellent central services there, I am always concerned how it delivers into comprehensive multi-disciplinary local services for these kids. They need to access their local services day to day – therapists, education authorities, transport – it's hard to coordinate and deliver remotely even though they get a good specialist service.

86. We were shocked by the written evidence we received from Tara McColgan from Derry in Northern Ireland, whose son Travis has Duchenne muscular dystrophy. Her evidence highlights our concerns of the crucial need for a long-term specialist multi-disciplinary team to be in place in Northern Ireland. Her overall impression of specialist services in her region was very poor:

Travis has not been seen by a specialist at the muscle clinic in Belfast since January 07, because they have not been able to find a suitable replacement.

Travis received more care as a baby than he does now – when he needs it most.

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## Section 8

### Evidence Summary

#### Wheelchairs, equipment and orthotics

Oral and written evidence was provided by:

*Paul Charlton – Orthotist with specialist interest in adult neurology*

*Nigel Shapcott – Head of the Rehabilitation Engineering Unit at Morrison Hospital, Swansea and Chair of the Posture and Mobility Group*

- 87.** We are deeply concerned about the postcode lottery that exists for receiving an acceptable service for wheelchairs, equipment and orthotics. The lack of flexibility, co-operation and funding in the commissioning set-up for these services is extremely worrying. Paul Charlton delivered this damning indictment to us during his evidence submission:

There is currently no coordinated or coherent strategy for the commissioning of orthotic services in England and, as far as I or any of my colleagues are aware, nor are there plans to do so.

It is recognised that an orthotist needs to work very closely with the clinician to get the best for the patient. What is equally important is my relationship with those making the orthotics. My job is to work with the clinicians to identify what is best for the patient but to also work with manufacturers for the making of the orthotics and necessary tweaks to make it in the best way. Splitting me from that relationship makes things difficult.

- 88.** We are shocked at the lack of knowledge disseminated to individuals in this crucial field of specialist services so that people with muscular dystrophy and neuromuscular conditions could receive the service that they are entitled to. Paul Charlton explained his lack of awareness of the Specialised Services National Definition Set 5 Document relating to orthotics:

I wasn't aware of that document so I have dug it out. Having read the document, it actually makes some very good recommendations. I believe the document is about advising commissioners as to how to best think about commissioning a service. If a lot of those things were adopted, it would be a very good start but I am unaware of anywhere in the UK where it has been adopted.

There is a real need for a specialist centre. The document that we've been referring to talks about the hub and spoke model which is very much what happens in the North East whereby there is a specialist centre where there

are experts who see all the clients on an occasional basis. They then go back to their localities and all the professionals involved there know the people in the centre who they can speak to. They can assess what is not working for a particular patient and get advice from or refer back to the central hub. It is an ideal scenario but it has happened by good fortune through the work of strong-minded clinicians rather than good planning.

- 89.** We welcome the fact that there are certain areas of the Specialised Services National Definition Set Document 5 relating to wheelchairs and equipment already in existence in Wales but Nigel Shapcott's description raises questions about the national coordination of the specialist service provision:

I've looked through it and there are some things we are doing in Wales. We have a consultant, a rehab consultant involved with our wheelchair clinic when we need to so we can run regular clinics. We don't do 24 hour postural management, we have talked about it a lot and there are spots where social services will provide it.

**We are very concerned by the volume of evidence we heard which set out unacceptable delays in the provision of wheelchairs, equipment and orthotics support necessary to ensure a good quality of life and to maintain the individual's independence and dignity. Further, we find it unacceptable that many individuals are forced into financial hardship or to appeal for charitable funds in order to purchase the vital equipment they require. We are aware that there have been several recent reviews of national wheelchair and equipment services but the issues have still to be addressed effectively. We therefore now call on the Department of Health to set up a Short-Life Review Group involving the Muscular Dystrophy Campaign and representatives of other patients' organisations to bring forward proposals by June 2010 to address the failings in the provision of these vital services. (Recommendation 14)**

- 90.** We have concerns about the pressure being put on specialist clinics in London and Newcastle after receiving evidence from Paul Charlton of the distances people will travel to access orthotic services:

Currently patients may have to travel over 100 miles for our specialist clinics in the Northern region, for example we have quite a few clients fairly regularly from Whitehaven in Cumbria. We have people flying in from Ireland, people from Sheffield. Where people hear of a good centre, they will do whatever they can to access it. Obviously, funding is difficult and they have to source their own funding. My colleague in London has had a similar experience. He looks after a very wide area but people are trying to access him from areas where there isn't the expertise locally.

- 91.** We have doubts about the long-term security of funding for wheelchair services in the UK. Nigel Shapcott outlined his fears of budgetary constraints to us during his evidence submission:

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We are extremely fortunate in Wales compared to many of the wheelchair services in England. We have a good budget which is supposedly ring-fenced but we are fighting that battle as we speak.

What has amazed me is the way we are so protective about budgets in education, social services and health. We can share those budgets if you battle, but you can only fight so many battles.

- 92.** We are extremely disappointed that there is a lack of stability in physiotherapy services and this has disturbing consequences for people with muscle disease. Nigel Shapcott emphasised this to us:

In some places there is good transition and you can continue with physiotherapy and community services, but in others it is cut off completely, so as a rehab engineer, you could be faced with a different situation of somebody deteriorating in terms of their physical condition because they are not getting therapies.

I have physios who come and work for me and I have to educate them. They are very valuable to us once they have gone through 2 years of looking at what we are doing but we really need accredited specialist courses.

- 93.** We were keen to hear about how wheelchairs, equipment and orthotic services should be run and what improvements could be made to ensure that this happens.

- 94.** Paul Charlton offered his views in his written evidence to us:

It is recognised that where these services work best, professionals work together over years to create a mutual understanding and responsiveness to these patients' needs.

In areas where the services have been established, they work very well and are well respected. In the first instance, I would suggest these practices are documented, compared and a best model sought which is then commissioned with an appropriate level of funding. Optimum funding for a hub and spoke model may be difficult in isolation. The spokes require other activity to make them financially viable and hence our current attempts to look at orthotics as a whole as well as just specialist services.

- 95.** Nigel Shapcott made these points and recommendations in his written evidence to us:

Join the three main services, health, social care and education; make computer systems between the three services usefully communicate so that staff have good access to information; develop joint budgeting; develop joint care pathways; develop pathways for defined appropriate equipment provision.

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Intelligent follow up – coordinate services so that the wheelchair and equipment can be checked at the same time as the client attends for other hospital visits.

- 96.** We strongly feel that urgent action is required to address the vital issues that have been highlighted to us in this crucial field. The absence or inadequacy of these services severely affects patients' well-being and quality of life.

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## Section 9

### Evidence Summary

#### (i) NHS supporting independent living

#### (ii) Jonathan Shaw MP, Minister for Disabled People, Department of Work and Pensions

(i) Oral evidence was provided by:

*Matthew Lanham – Executive Director, NeuroMuscular Centre, Cheshire*

*Ben Dale – Operations Manager, NeuroMuscular Centre, Cheshire*

**97.** We welcome the existence and success of the NeuroMuscular Centre (NMC) in Cheshire, which was established with charitable funding. We recognise its importance in providing physiotherapy treatment and employment opportunities for people with muscular dystrophy and related neuromuscular conditions to enable them to live more independently.

**98.** We acknowledge and appreciate the significant percentage of PCT funding for physiotherapy treatment at the NMC but we express deep concern at the amount of charitable funding that has to be set aside to make up for the shortfall of NHS funding for high quality treatment provision. Matthew Lanham provided us with some statistics:

We've been reasonably successful over the past few years in getting more and more funding from the PCTs for the treatments we provide. About 65% of the PCTs from where people come from fund their treatment.

**99.** We received evidence about the social enterprise at the NMC, which has been of great benefit to people with muscle disease in relation to employment opportunities. Ben Dale described the set-up to us:

Our National Award winning Design & Print Service – it's a social enterprise employing 15 people, 12 of whom have a neuromuscular condition. It's just a normal company providing design and print solutions. It started because it's something that some of the original group were into. One of them, now the Head of Design, he went to Manchester University, had a degree in Graphic Design then found opportunities very hard to come by in the private sector and was just at home for quite a while.

**100.** We share Matthew Lanham and Ben Dale's concerns regarding the lack of ongoing physiotherapy provided by the NHS for patients with muscular dystrophy and related neuromuscular conditions. Matthew Lanham informed us during his evidence:

Increasingly what's getting reported to us by young people who are joining us from mainstream school is that physiotherapy services for a child with muscular dystrophy in a mainstream school are very, very sporadic, very patchy, and often quite poor.

**101.** Ben Dale emphasised the vital importance of the ongoing physiotherapy service provided by the NMC:

As a consequence of our services, it's anecdotal I think, but still significant, 70% are in employment of those who use our physiotherapy service. Just the ongoing physio that we provide that is not provided within the NHS is having a huge impact on how people are able to live their lives.

**102.** We express the hope that the Department of Health will recognise the central role that physiotherapy plays in the specialist care that patients with muscle disease receive, and which ensures that independent living can be realised.

#### (ii) Jonathan Shaw MP, Minister for Disabled People, Department for Work and Pensions, also gave evidence to the Inquiry.

**103.** We welcome the Government's commitment to independent living for disabled people and we approve of the consultation of disabled people in the Independent Living Strategy. Jonathan Shaw MP informed us:

The Government is committed through its Independent Living Strategy to enable disabled people to have choice and control over the support that they need to live their daily lives. The Strategy was produced in partnership with disabled people to ensure that it is informed by their knowledge and experience of the barriers to independent living. Direct Payments are an important part of our Strategy as they will allow disabled people to design and purchase services to meet their specific circumstances and need. These payments help increase opportunities for independence, social inclusion, enhanced self esteem and they can result in better outcomes for both the service user and the carer.

**104.** We are concerned about the lack of knowledge of muscular dystrophy amongst Disability Employment Advisers (DEAs). Jonathan Shaw explained the steps that could be taken to increase knowledge:

I wouldn't want to give an absolute assurance that across the board we have got a good knowledge of muscular dystrophy – I would say that it is patchy. Where a JobCentrePlus worker has had an engagement with someone with muscle disease, particularly a DEA, they would develop that body of knowledge. What is important is we need to ensure the DEAs have broad generic knowledge of different conditions and disabilities and most importantly are active in seeking out information and know

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where it is readily available through a peer or web based information.

- 105.** We asked the Minister the question “Severely disabled children under the age of 3 are not currently receiving the help they need in terms of the mobility component of the disability living allowance. Can this limitation be removed and the needs of each child under 3 assessed simply on their merits?” Jonathan Shaw responded to us:

I agree with the principle. It would cost around £14-15 million. If a parent has a whole lot of apparatus and equipment around to support the child, we should be giving every help we can. At the moment we are not in a position to make that financial commitment. An additional £15 million is needed, and it is not straightforward how it would apply due to variance of equipment. We need to properly understand the group being targeted – a smaller group may be more affordable.

- 106.** We are greatly encouraged that there are intentions to give greater control to patients in the commissioning process. Jonathan Shaw outlined potential future commissioning arrangements:

The Health Bill currently going through Parliament allows for some pilots for individual budgets, and we are determined that we can try to align the health pilot for the budget under the ‘right to control’. We want to provide the individual with the choice. It could be imagined in the future that a group of disabled people would be the commissioners – pool their individual budgets, interview prospective service providers. So rather than being the passive recipients of what the council or health service say they can have, they are in control, and that seems a wholly desirable route to travel.

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## Section 10

### Evidence Summary

#### Weaknesses in NHS care for people with muscular dystrophy or a neuromuscular condition in each country and region within the UK

##### NHS South West Region:

- 107.** We were pleased to hear more about the commitment of the South West Specialised Commissioning Group to address the considerable deficits in their provision of specialist neuromuscular services. A 2007 report by the Muscular Dystrophy Campaign highlighted that an audit of 40 sequential Duchenne muscular dystrophy deaths over 10 years in the South West region showed a median age of death of 18 years; compared to a mean of age of death of almost 30 years in patients with Duchenne muscular dystrophy receiving home ventilation and specialist multi-disciplinary care reported by the Newcastle group in the most recent study by Eagle *et al* (2007).<sup>2</sup> We understand that through working with patients, clinicians and the Muscular Dystrophy Campaign, the local commissioners are implementing a **Neuromuscular Strategy** which will create a managed clinical network, bringing together new adult and paediatric specialist neuromuscular consultants, care coordinators, specialist physiotherapists and existing support services. We welcome this much-needed investment, and are hopeful that children and adults affected by muscular dystrophy and related neuromuscular conditions will soon be able to access to specialist multi-disciplinary care they need.

##### NHS South Central Region:

- 108.** We are very concerned to note from written evidence by the Muscular Dystrophy Campaign that the regional Specialised Commissioning Group are failing to commission services for specialised neuromuscular conditions. A 2009 report, **Building on the Foundations: The Need for a Neuromuscular Service Serving Patients in the NHS South Central Region**, published by the Muscular Dystrophy Campaign and co-authored by the leading neuromuscular clinicians in the area highlighted a number of serious concerns including: the vulnerability of current essential NHS services due to a reliance on charitable funding, the lack of capacity of the existing specialist centres, which are dependent on one lead consultant, and misdiagnosis of rare conditions such as Myasthenia Gravis. However, we are very disappointed to understand that the SCG have chosen to ignore the recommendations of this report, and have even told families with muscle disease that they “are not a priority”. We call on the local commissioners to revise their deplorable attitude on this matter and to carry out a thorough review of current neuromuscular services as a matter of urgency.

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### NHS West Midlands Region:

**109.** We welcome the work of the NHS in the West Midlands to address the concerns of local clinicians and families regarding the provision of specialist neuromuscular care, particularly on issues such as, misdiagnosis, respiratory care and poor wheelchair provision. We particularly welcome the dynamic style of leadership shown by Eamonn Kelly, Director of Commissioning at the Strategic Health Authority, who gave his personal commitment to overseeing improvements in neuromuscular services in the region. We look forward to the results of the West Midlands review of neuromuscular services in the hope that they can build on the excellent services provided by the existing specialist clinicians and ensure that everyone living with muscle disease in each part of the region has access to specialist multi-disciplinary neuromuscular care. This should be supported on a 'hub and spoke' model with local support which is enhanced through the neuromuscular network proposed by the Muscular Dystrophy Campaign backed by the leading clinicians in the region.

### NHS South East Coast Region:

**110.** We have further noted the concerns of families living in the NHS South East Coast area, particularly regarding the absence of local care to support the work of the specialist centres in London. Evidence from the 2009 report *Building on the Foundations; The Need for a Neuromuscular Service Serving Patients in the NHS South East Coast Region* highlighted the alarming fact that there are currently no neuromuscular care coordinators in the whole of the Kent, Surrey and Sussex region. Furthermore, widespread deficits in the provision of specialist physiotherapy, psychology and hydrotherapy were also reported. We understand that the regional Specialised Commissioning Group has met with the Muscular Dystrophy Campaign and have agreed to look into the issues of this patient group. However we would urge the South East Coast SCG to take forward proposals to improve neuromuscular services to start in April 2010, as recommended by the Muscular Dystrophy Campaign and endorsed by the leading clinicians serving the region.

### NHS North East Region:

**111.** The neuromuscular services in the North East have consistently been held up as the standard bearer for the UK as a whole throughout this Inquiry. We praise the work of the Newcastle Muscle Centre, which well deserves its international reputation for excellence in all aspects of research, diagnosis, care and support for children and adults with neuromuscular conditions. However, we note that there are some areas where the Muscle Centre's services need to be strengthened and, further, there is a vulnerability through the reliance on charitable funding. To secure its longer term future, the issue of succession planning must be addressed, and we would ask the Specialised Commissioning Group in the North East to take the necessary action to sustain the North East's position as the flagship region in the UK for neuromuscular

services. In particular we urge the Specialised Commissioning Group and Northern Specialised Commissioning Core Team (NORSCORE), its commissioning arm, to meet with the local PCTs together with the Muscular Dystrophy Campaign and leading clinicians to agree the steps needed to ensure that comprehensive, multi-disciplinary neuromuscular services are embedded in the North East.

### NHS North West Region:

**112.** We heard from clinicians in the North West region about the problems facing the multi-disciplinary North West Neuromuscular Network - founded and chaired by Dr Stefan Spinty, Consultant Paediatric Neurologist and lead neuromuscular clinician at Alder Hey Children's Hospital, Liverpool. Dr Spinty is currently managing this vital network single-handedly without any funding. We believe that the Network should at least be supported by a Network Coordinator. Greater support at transition from paediatric to adult services is also needed, given the evidence of services being removed or greatly reduced when people leave paediatric services. Existing transition services are extremely vulnerable and we were concerned to learn from clinicians that none of the transitional services in the region are funded nor appropriately staffed. Having met with commissioners from the North West Specialised Commissioning Group to discuss these concerns, local clinicians and representatives from the Muscular Dystrophy Campaign were disappointed to be told that there are competing priorities in the region, and that PCT budgets were under strain. We urge the North West SCG and the local PCTs to undertake a service review to address the weaknesses highlighted by clinicians, patients and the Muscular Dystrophy Campaign.

### NHS Yorkshire and the Humber Region:

**113.** We were deeply concerned to learn that the mean age of death for boys with Duchenne muscular dystrophy in the region is 17.2 years – which compares with 30 years in the North East. This figure was uncovered following a clinical audit of the 100 boys with Duchenne muscular dystrophy born between January 1980 and December 1999 in the region (of which there have been 34 deaths). There is also the particular challenge of providing care for the Asian community in the region. The incidence of neuromuscular conditions is twice as high in Bradford as in surrounding areas, with a very different spectrum of disease – a trend also seen in other neurological conditions. Adults in the region are also particularly poorly served - there is only one adult consultant neurologist to serve the entire region, which is having a serious impact on patients and their families. The region's only Care Advisor/Coordinator, based in Leeds, is partly funded by the charity and we are calling for this vital post to become fully embedded in the NHS from April 2010. A service review should also be undertaken in this region to address the issues highlighted by clinicians, patients and the Muscular Dystrophy Campaign.

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### NHS East of England Region:

**114.** Clinicians from the region expressed their urgent concern that there is no tertiary provider in the East of England offering a devoted neuromuscular service or clinic to children. One family in Suffolk are forced to travel from Suffolk to London, a journey of up to five hours each way, in order to receive specialist healthcare for their eight year old son. Many other families are travelling over two hundred miles to receive specialist diagnosis, assessment and treatment in London. There are also no neuromuscular care coordinators to provide information, care and support to families living in the region, and we believe that this is having a serious impact on patients in the region. As a first step in addressing these weaknesses, a service review should be conducted in this region to take action to resolve the problems highlighted by clinicians, patients and the Muscular Dystrophy Campaign.

### NHS London Region:

**115.** We are pleased to note that London enjoys the benefit of specialist tertiary referral centres at Great Ormond Street Hospital, and the National Hospital for Neurology and Neurosurgery, UCLH, and many migratory patients travel from other regions, and indeed other countries, to receive specialist support at these centres. However, we have been alarmed by problems encountered in the linking of these centres with the localities within London.

**116.** We are concerned about major gaps in the diagnosis procedure in London, and want to emphasise that even where muscle pathology in London is prioritised it is important to remember that diagnosis does not stop at the point of the initial assessment. There is only one Regional Care Advisor serving the entire London region – funded by the Muscular Dystrophy Campaign. Eight full-time Regional Care Advisors are needed to serve the 7,500 people in London living with a neuromuscular condition. We have observed major problems that exist in the transition of patients from paediatric to adult services. Patients require a wide range of different services and support requirements change rapidly; centres are currently not geared up to cope with transition services.

**117.** We are alarmed by the emerging evidence of a crisis affecting areas such as North East London. This is an area facing multiple deprivation and there is a real concern that the Asian community is experiencing a significantly higher incidence of neuromuscular conditions. This is the same phenomenon emerging in West Yorkshire in the NHS Yorkshire and the Humber Region.

**118.** The Muscular Dystrophy Campaign has met with the London Specialised Commissioning Group and has called on the SCG to work together with patients and clinicians to improve services. We echo this call, and look forward to hearing the response of the SCG to our report.

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### NHS East Midlands Region:

**119.** We heard evidence from clinicians which underlines the urgent need for the East Midlands Specialised Commissioning Group, together with PCTs in the region, to undertake a review of neuromuscular services. Such evidence included a lack of ongoing physiotherapy, the vulnerability of the neuromuscular care advisor post, as well as the lack of a dedicated psychology service for neuromuscular patients. We were disappointed to learn that local PCTs do not share this view. A meeting for PCTs was called by the Specialised Commissioning Group in July to discuss these issues, but this was cancelled due to fact that only one out of the region's nine PCTs agreed to attend. Without commitment from the PCTs in the region, it will be impossible to improve services for this vulnerable group of patients.

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## Appendix

### Witnesses

The Group would like to thank all the expert witnesses, both from a patient and professional perspective, who gave oral evidence and submitted written evidence describing and analysing a wide range of key issues. The APPG greatly appreciates the very useful and informative evidence which it can now take forward through the recommendations in this report.

Below is a list of the witnesses who provided oral evidence for the Inquiry:

#### 10 December 2008 – “The clinician perspective”

**Professor Michael Hanna** – Consultant Neurologist, MRC Centre for Neuromuscular Disease, National Hospital for Neurology and Neurosurgery and Institute of Neurology, London

**Dr David Hilton-Jones** – Consultant Neurologist, Clinical Director, Oxford MDC Nerve & Muscle Centre, John Radcliffe Hospital, Oxford

**Dr Michelle Eagle** – Consultant physiotherapist, Newcastle Muscle Centre

**Dr Stephen Lynn** – Project Manager, TREAT-NMD, Newcastle

#### 20 January 2009 – “Living with muscular dystrophy – the patient perspective”

**Phillippa Farrant** – Eastbourne, mother of young man with Duchenne muscular dystrophy

**Andy Findlay** – Derby, living with by Facioscapulohumeral muscular dystrophy (FSH)

**Moira Findlay** – Derby, wife and carer for Andy Findlay

**Laura Merry** – London, living with Congenital muscular dystrophy

#### 3 March 2009 – “Transition and Care”

**David Abbott** – Senior Research Fellow at the Norah Fry Research Centre, University of Bristol

**Emily Ballard** – Specialist Transitional Care Therapist at the Lane Fox Unit, Guy’s and St Thomas’ NHS Foundation Trust, London

**Dr Nicholas Hart** – Clinical Research Consultant and Honorary Senior Lecturer in Respiratory and Critical Care Medicine at the Lane Fox Unit, Guy’s and St Thomas’ NHS Foundation Trust, London

**Stuart Reid** – Solihull, living with Duchenne muscular dystrophy

**Jane Stein** – Regional Care Advisor/Coordinator, Oxford

#### 24 March 2009 – “Professional development – specialist staff and workforce planning”

**Professor Caroline Sewry** – Professor of Muscle Pathology, Oswestry and London

**Dr Tim Wreghitt** – Royal College of Pathologists

**Annie Aloysius** – Specialist Speech and Language Therapist, Great Ormond Street Hospital and Hammersmith Hospital, London

**Dr Linda Marks** – Consultant in Rehabilitation Medicine, Royal National Orthopaedic Hospital in Stanmore, London

**Gary Robjent** – Head of Public Affairs, Chartered Society of Physiotherapy

#### 28 April 2009 – “Research, new treatments and international perspective”

**Professor Kate Bushby** – Newcastle Muscle Centre

**Professor Francesco Muntoni** – Dubowitz Neuromuscular Centre, Great Ormond Street Hospital, London

**Professor Volker Straub** – Newcastle Muscle Centre

#### 19 May 2009 – “Wheelchairs, equipment and orthotics”

**Paul Charlton** – Orthotist with specialist interest in adult neurology

**Nigel Shapcott** – Head of the Rehabilitation Engineering Unit at Morriston Hospital, Swansea and Chair of the Posture and Mobility Group

#### 19 May 2009 – “Employment and independence”

**Matthew Lanham** – Executive Director, NeuroMuscular Centre, Cheshire

**Ben Dale** – Operations Manager, NeuroMuscular Centre, Cheshire

#### 9 June 2009 – “Focus on the devolved countries – Wales and Northern Ireland”

**Dr Louise Hartley** – Consultant Paediatric Neurologist, Cardiff

**Dr Mark Rogers** – Consultant Clinical Geneticist, Cardiff

**Rachel Salmon** – Newborn Screening Specialist Nurse, Cardiff

**Dr John McConville** – Consultant Neurologist, Belfast

**Dr Laura McKeown** – Regional Care Advisor/Coordinator, Northern Ireland

#### 23 June 2009 – “Meeting responsibilities – NHS Specialised Commissioning”

**Steve Collins** – Deputy Director of NHS Specialised Commissioning

**Deborah Evans** – Chair of South West Specialised Commissioning Group and Chief Executive of NHS Bristol

**Louise Tranmer** – Director of South West Specialised Commissioning Group

**Eamonn Kelly** – Director of Commissioning, West Midlands Strategic Health Authority

#### 30 June 2009 – Government role in promoting disability equality

**Jonathan Shaw MP** – Minister for Disabled People, Department for Work and Pensions

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## Background to the Inquiry

The APPG for Muscular Dystrophy National Inquiry into Access to Specialist Care, chaired by Dave Anderson MP, aims to improve services to ensure that all people living with muscle disease are able to access the right care, support and advice.

The Inquiry wanted to hear the views of those people who are affected by muscular dystrophy and those clinicians and health professionals who have a direct interest in the provision of services for people with muscular dystrophy. The Inquiry was also keen to hear from those people who are responsible for the commissioning of these services. Several high profile reports in the past two years by the Muscular Dystrophy Campaign have highlighted distinct differences in the quality of service provision for people with muscular dystrophy across the UK.

The importance of discussion of the key issues is exemplified by the current lack of NICE guidelines for muscular dystrophy and related neuromuscular conditions and the absence of these conditions on the National Definition Set.

## Terms of Reference

The formal Terms of Reference for the Inquiry are: **“To determine the current provision and quality of specialist services for people with muscular dystrophy and related neuromuscular conditions; to highlight areas of best practice and recommend possible solutions where improvements are necessary.”**

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## Written Evidence

**Building on the Foundations:** The Need for a Neuromuscular Service Serving Patients in the NHS East Midlands Region. *Muscular Dystrophy Campaign, June 2009*

**Specialist Services in the East Region of England Briefing.**  
*Muscular Dystrophy Campaign, January 2009*

**Building on the Foundations of Excellence in London:** The need to secure and strengthen neuromuscular services. *Muscular Dystrophy Campaign, August 2009*

**Building on the Foundations in Northern Ireland:** Improving Specialist Care, Support and Independence. *Muscular Dystrophy Campaign, February 2009*

**Building on the Foundations of Excellence in the North East:** The need to secure and strengthen neuromuscular services. *Muscular Dystrophy Campaign, May 2009*

**Building on the Foundations:** Strengthening Neuromuscular Services Serving Patients in the NHS North West Region. *Muscular Dystrophy Campaign, June 2009*

**Building on the Foundations in Scotland:** Improving Specialist Care, Support and Independence. *Muscular Dystrophy Campaign, September 2008*

**Building on the Foundations:** The Need for a Neuromuscular Service Serving Patients in the NHS South Central Region. *Muscular Dystrophy Campaign, May 2009*

**Building on the Foundations:** The Need for a Neuromuscular Service Serving Patients in the NHS South East Coast Region. *Muscular Dystrophy Campaign, May 2009*

**Building on the Foundations:** The Need for a Specialist Neuromuscular Service across Wales. *Muscular Dystrophy Campaign, February 2008*

**Building on the Foundations:** the need for a neuromuscular service across the West Midlands. *Muscular Dystrophy Campaign, April 2009*

**Building on the Foundations:** The Need for a Neuromuscular Service Serving Patients in the NHS Yorkshire and Humber Region. *Muscular Dystrophy Campaign, June 2009*

**Equipment shortfall:** How disabled children are being failed.  
*Muscular Dystrophy Campaign, March 2009*

**Building on the Foundations:** Focus on Physio. *Muscular Dystrophy Campaign, May 2008*

**Hydrotherapy.** *South West Muscle Group, January 2009*

**Building on the Foundations:** State of the Nation – The 2008 National Survey.  
*Muscular Dystrophy Campaign, September 2008*

**Protect research through recession.** *Muscular Dystrophy Campaign and Association of Medical Research Charities, May 2009*

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**National Specialised Commissioning Group Briefing Note.** *June 2009*

**Neuromuscular Service Development Strategy.** A development plan for services in the South West. *South West Specialised Commissioning Group, December 2008*

**Neuromuscular services in the South West.**  
*South West Specialised Commissioning Group, June 2009*

**Neuromuscular services in Wales – a clinician’s perspective.**  
*Dr Mark Rogers, April 2009*

**Muscular Dystrophy Campaign’s Evidence to the Welsh Affairs Committee Inquiry into the Provision of Cross-Border Public Services for Wales.** *April 2008*

**The Case for Regional Neuromuscular Care Advisors.**  
*Dr Sutha Yoganathan, May 2009*

**Helen Starbuck** – South West

**Tara McColgan** – Northern Ireland

**Martyn Blenkharn** – North West

**Nigel Shapcott** – Head of the Rehabilitation Engineering Unit at Morryston Hospital, Swansea and Chair of the Posture and Mobility Group

**Paul Charlton** – Orthotist with specialist interest in adult neurology

**Ameena Berkowitz** – London

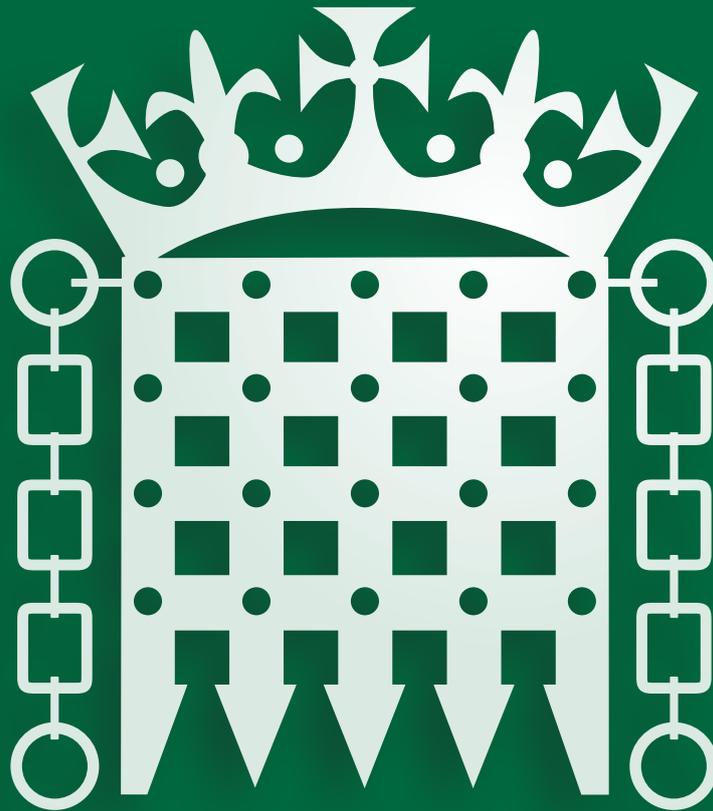
**Louise Brock** – East of England

**Clive Gilbert** – London

**Carole Clayton** – East Midlands

### Notes:

- 1 Eagle M, Bourke J, Bullock R, Gibson M, Straub V and Bushby K. (2007) ‘Managing Duchenne muscular dystrophy – The additive effect of spinal surgery and home nocturnal ventilation in improving survival’ *Neuromuscular Disorders* Volume 17, Issue 6, p.470-475.
- 2 Care Advisors may also be known as key workers or neuromuscular care coordinators. They carry out the role envisaged by Lord Darzi in his 2008 *High Quality Care for All* report.



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**Access to Specialist  
Neuromuscular Care:  
The Walton Report**

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**All Party Parliamentary Group  
for Muscular Dystrophy**

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