

**Muscular
Dystrophy
Campaign**



Building on the Foundations of Excellence in the North East: The need to secure and strengthen neuromuscular services

May 2009



**A report by the Muscular Dystrophy Campaign with
contributions from the leading neuromuscular clinicians in
the North East region.**

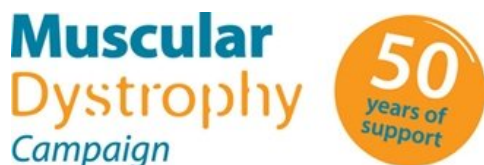
**A report by the Muscular Dystrophy Campaign
with contributions from and endorsed by the
leading neuromuscular clinicians serving the
NHS North East region**

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***“I am fully supportive of these proposals and the work
of the Muscular Dystrophy Campaign in the North
East”***

***David Anderson MP for Blaydon and Chair of the All Party Parliamentary Group
for Muscular Dystrophy***

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Building on the Foundations of Excellence in the North East: The need to secure and strengthen neuromuscular services May 2009

Executive Summary:

This report by the Muscular Dystrophy Campaign draws on the views and experience of leading specialists, patients and their families from across the North East Strategic Health Authority region to set out a number of serious concerns regarding the provision of specialist clinical services in the North East for patients with muscular dystrophy and related neuromuscular conditions.

The Newcastle Muscle Centre has a well deserved international reputation for excellence in all aspects of research, diagnosis, care and support for children and adults with neuromuscular conditions living not just in the North East but for those who attend from other parts of the UK and indeed from overseas countries. However, 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.

Further this report also highlights the innovative proposal to establish **md enterprises north east** as a vital new element in the managed clinical network in the North East. **md enterprises north east** is being designed with user involvement to provide *inter alia* specialist expertise, professional development and access to FE and employment opportunities. It will also be one of the delivery arms for specialist physiotherapy and other services in the region, as guided by its influential Project Board.

We are calling on the local PCTs, NORSCORE and the North East Specialised Commissioning Group (SCG) to follow the lead of the South West and West Midlands regions and carry out an urgent, in-depth review of current specialised service provision in the North East. This review would aim to bring forward proposals in autumn 2009 to secure and develop the comprehensive neuromuscular service for children and adults including transition services for young people. Now is the time to build on the reputation and success of the Newcastle Muscle Centre, support its developing innovative partnership **md enterprises north east**, and sustain the North East's position as the flagship region in the UK for neuromuscular services.

Action needed:

- 1. A short life working group should be established to carry out an in-depth review of current service provision and its vulnerability in the North East. This review would involve PCTs, NORSCORE, the SCG, clinicians and patient representatives and would bring forward proposals in Autumn 2009 to secure and develop the comprehensive, multidisciplinary service for children and adults, including transition services for young people.**

2. The excellent services provided from the Newcastle Muscle Centre should continue to be funded through the Northern Genetics Service, the Newcastle Hospitals Trust and, where appropriate, through research grant funding secured through Newcastle University. However, the vulnerability of some core aspects of the service including care advisor, physiotherapy time and some administrative support through the reliance on charitable funding should be addressed.
3. Further, two additional full-time equivalent (FTE) Care Advisors with expertise in muscular dystrophy and related neuromuscular conditions should be established and core funded by the NHS in the NHS to ensure that advice and support are provided to the remaining two-thirds of the 3,000 people living with these conditions who currently do not have access to the service.
4. Ongoing physiotherapy should be provided to all adults and children with a neuromuscular condition in each PCT. The paediatric physiotherapy service in the region is good but the needs of adults are not currently met effectively. To ensure that the needs of adults with a neuromuscular condition in each PCT area are met effectively, there needs to be enhanced specialist physiotherapy available through the Muscle Centre, and from April 2010, an extended pathway commissioned for specialist physiotherapy through md enterprises north east, as a specialist clinical service and training provider, working with front line clinicians.
5. The service review should embrace the proposals to develop the innovative md enterprises north east service to ensure that PCTs, NORSCORE and the SCG play a full role as partners in the strategic development of this multi-agency service development. In addition to providing specialist (physiotherapy sessions) therapies and support to adult patients, md enterprises north east will also provide work experience for (physios) a range of health and social care students in training and valuable professional development for community-based practitioners to enhance their role working in each PCT area.
6. There is an urgent need to strengthen the input to clinical care from speech and language therapy and dietetics to tackle eating and swallowing problems that can cause major problems if not addressed. Studies have reported problems with malnourishment for some older patients with Duchenne muscular dystrophy, for example, and these problems are avoidable with correct advice and treatment as conditions progress.¹
7. Psychological support is a vital element of a comprehensive service that should be provided for patients and families living with a neuromuscular condition in each PCT area across the North East. These are in the main genetic conditions and families need psychological support through various stages such as at diagnosis, through genetic counselling, when becoming wheelchair dependent, in the transition stage, before spinal surgery and when end of life issues need to be addressed.
8. The Newcastle muscle clinics have developed collaborative links with directorates across the Trust delivering cardiology, respiratory support and orthopaedic services to patients with neuromuscular conditions. These are currently delivered for the most part in separate clinics. There is an opportunity within this review to address the possibility of increased joint working and joint clinical services to be enhanced.

Our key findings include:

- 1. The Newcastle Muscle Centre is an established international and national centre of excellence in all aspects of research and clinical practice relating to neuromuscular conditions,² and provides the leadership for an effective and successful 'hub and spoke' model. The Regional Genetics Service funds its work in specialist genetics although the Centre currently relies on charitable funding for some areas of its work. Further, the Muscle Centre is heavily dependant on its leading expert clinical academic consultants who are entirely university funded (Professors Bushby, Straub and Lochmuller) which, with the reliance on charitable funding, gives the Muscle Centre a vulnerability which must be addressed.**
- 2. There is only one Care Advisor in the region – and this post is vulnerable as it is dependent on funding currently provided by the Muscular Dystrophy Campaign. A minimum of two additional FTE Care Advisors are needed to serve the 3,000 people in the area with a neuromuscular condition (with a workload of 1,000 patients each). These two new posts plus the existing, valued post should be core funded by the NHS, as has happened in other regions to ensure their continuity.**
- 3. A widespread deficit in provision of services by allied health professionals was also identified with very limited access in particular to ongoing physiotherapy for adult patients in particular – over a third of patients do not see a physiotherapist and half feel that they do not receive adequate physiotherapy support.³ Specialist physiotherapists are required to support outreach clinics and provide training and professional development for community physiotherapists.**
- 4. There is no dedicated psychology service despite its importance as part of multi-disciplinary care for this patient group with rare and very rare conditions, often genetic in origin and with no known cures and only limited treatments available.**
- 5. Input for nutritional support and specialised speech and language services is necessary to adhere to current standards of care for neuromuscular conditions.**
- 6. Greater support at transition from paediatric to adult services is needed given the evidence of services in the community being removed or greatly reduced when people leave paediatric services even though needs may well increase given the progressive nature of many conditions.**
- 7. There has been very encouraging progress in developing the innovative md enterprises north east service which will provide *inter alia* the delivery arm for specialist physiotherapy support for young people and adults from each PCT in the region. Further, it will offer professional development and work experience for community physios and physios in training. The proposed service review will ensure that the strategic development of md enterprises north east is planned within a comprehensive neuromuscular service serving the region. A costed proposal has already been prepared relating to funding for specialist therapies provided through md enterprises north east to start from April 2010. The SCG and NORSCORE, acting on behalf of local PCTs, should play a full role as partners in this multi-agency service development with Newcastle College, Newcastle Hospitals Trust (NuTH) and Newcastle University.**

1. Background:

There are over 200 forms of muscular dystrophy and related neuromuscular conditions. They are multi-system disorders, which require complex long-term surveillance and care. Without multi-disciplinary and holistic care most patients and their families experience a further reduction in quality of life.

2. Demographics:

There are some 2,500 people in the North East region affected by muscular dystrophy or a related neuromuscular condition (see appendix 1). There are a further 330 people in the North Cumbria area affected by muscular dystrophy or a related neuromuscular condition who have their specialised services commissioned through the North East Specialised Commissioning Group rather than the North West Specialised Commissioning Group (the commissioning structure in the North East is outlined in appendix 2).

Improved genetic counselling is likely to lead to a small reduction in the overall incidence of these conditions but improved survival will increase their prevalence in the adult population. The very welcome improvement in survival has led to additional needs at transition and for specialist, adult services.

3. Multi-disciplinary care for muscular dystrophy and related conditions:

People living with severe disabling and/or life limiting neuromuscular conditions need access to the appropriate interventions and support as their condition progresses. Essential, specialist services should be delivered by a range of professionals from primary, secondary and tertiary providers working within a managed clinical network. Coordinated and comprehensive multi-disciplinary specialist care should include a neuromuscular specialist consultant⁴ and Care Advisors are essential to provide vital specialist care, support and advice for each individual and family affected by one of these conditions. Specialist multi-disciplinary care can help the region fulfil the 'Seven No's' aim as set out in [Our Vision, Our Future](#).⁵

Importance of respiratory care:

Breathing disorders are recognised as the leading cause of mortality in neuromuscular disease⁶. However specialist treatment, including ventilation, has been shown to improve both quality and length of life.⁷

In a peer-reviewed study from 2007, the Newcastle Centre's Michelle Eagle *et al* highlighted that patients with Duchenne muscular dystrophy treated at the Newcastle Centre and receiving home ventilation had a mean age of death of almost 30 years.⁸ The survival rate has continued to improve.

In comparison, an audit of 40 sequential DMD deaths over 10 years in the South West region (where specialist adult services were not provided) showed a median age of death of 18 years, underlining the critical importance of specialist multi-disciplinary care.

Regular comprehensive check-ups are required with clinicians being instructed to go through a full checklist of signs and symptoms. A study published in 2002 highlighted that patients can become too accustomed to their chronic illness and therefore rarely raise complaints about respiratory distress spontaneously.⁹

Evidence published in 2003 showed that it is more cost-effective to manage respiratory issues through check ups and home ventilation than through unplanned critical hospital admissions.¹⁰

Cardiac care is also an essential part of multi-disciplinary care as a number of neuromuscular conditions affect the heart. Cardiac screenings are crucial even for conditions which may appear to cause less severe weakening of the muscles, as “the severity of cardiomyopathy may be out of proportion to that of skeletal muscle involvement.”¹¹ As an example of the frequency required for cardiac screenings, best practice guidelines for Duchenne muscular dystrophy recommend that they should take place before any surgery, every two years up to the age of 10 and annually after age 10.¹² Without screening, cardiomyopathy can progress almost entirely without symptoms until signs of heart failure emerge, when all cardiac reserve has been eroded.¹³ Cardiac screening should also be offered to women who are carriers of mutations in the dystrophin gene, who are at increased risk of cardiomyopathy, even if they are not experiencing symptoms.

Dependent on medical need, orthopaedic care may also be required. Spinal deformity, such as scoliosis, is common in many neuromuscular conditions, (90% of people affected by Duchenne muscular dystrophy, for example, are likely to develop a clinically significant scoliosis).¹⁴ Surgery to correct spinal deformity can prolong life and improve posture and comfort. It is imperative that the development of scoliosis is monitored by the specialist muscle clinic as success rates are likely to be highest and complication rates lowest if surgery is performed when the spine is still mobile at a Cobb angle of 20-40°.¹⁵ For such a major operative procedure a multidisciplinary approach involving the paediatrician/paediatric neurologists and orthopaedic surgeons is essential in the approach to surgery.¹⁶

Transition:

Increasing numbers of young people with complex conditions are reaching transition and living longer because of improvements in therapies and medical care. For young people living with muscle disease, the period between mid and late teens is crucial and the transition from paediatric and adolescent care into adult-oriented healthcare services must be as smooth as possible.¹⁷ In the North East, fewer than one in five of the respondents to the Muscular Dystrophy Campaign Patient Survey rated the transition process as ‘excellent’ or ‘good’.

Case study:

A young man from the North East recently moved from paediatric to adult services. He said: “I feel that my transition was a poor experience. It really felt that as soon as I hit 18 the interest in me dropped both in terms of the physio and services that were offered/available as well as the level of support in general.”

Genetic counselling and psychological services should also be offered, together with locality based dietetic, occupational therapy, physiotherapy and speech therapy provision which can both improve the quality of these patients’ lives and increase their life spans (see appendix 6). Boys with Duchenne muscular dystrophy who are still ambulant should be offered the opportunity to discuss treatment with steroids such as deflazacort which studies have shown can stabilise muscle strength and delay the loss of ambulation and may also delay the onset of breathing complications (see appendix 7). For a number of neuromuscular conditions, regular check-ups are required irrespective of symptomatology, because deterioration can advance rapidly over the course of months.¹⁸

4. Current level of essential, specialist provision in the North East:

- **Newcastle Muscle Centre**

The Newcastle Muscle Centre is a specialist, world class centre of excellence for the diagnosis, management of and research into inherited muscle disease. It is part of the Institute of Human Genetics, a research organisation within Newcastle University and is hosted at the International Centre for Life in Newcastle. Clinical services are delivered through Newcastle Upon Tyne Hospitals NHS Foundation Trust. The Muscle Centre receives part funding from the Muscular Dystrophy Campaign and runs clinics for over a thousand children and adults in Newcastle and at various locations across the North of England. The main base for the children's clinic is Newcastle General Hospital, and for adults the International Centre for Life.

Children's clinics are also held at North Tees and Hartlepool and a range of other locations throughout the region, in conjunction with local physiotherapists and other local staff. In addition to the clinics at the International Centre for Life, adult clinics are held in the James Cook University Hospital, in Middlesbrough, and the Central Clinic in Carlisle.

The Muscle Centre is led by Professor Kate Bushby, Professor Volker Straub and Professor Hanns Lochmuller who hold joint appointments between the University of Newcastle upon Tyne and the NHS. Their time is split between research and clinical commitments, both of which are mainly focused on muscle disease. In total there are three clinical academic professors (consultants) all fully funded through the University. There is also one 0.75FTE specialty doctor funded via the NHS through the National Commissioning Group. The clinicians at the Muscle Centre see it as a priority to get this post fully funded to meet current caseload effectively.

In addition to the clinicians based at the Muscle Centre, there are a number of clinicians supporting the muscle service elsewhere: Dr Ramesh, a consultant paediatric neurologist and Dr Strehle, a consultant paediatrician from North Tyneside who once a month supports the paediatric and adult clinics respectively. Dr Miller leads the inflammatory muscle disease clinics and supports the biopsy service from the Neurology department. With Dr Horvath he is also taking an increasing role in developing services for peripheral neuropathies

Clinics are multidisciplinary and attended by members of the team, which includes specialist physiotherapist Dr Michelle Eagle, specialist genetic counsellors Louise Hastings, Catherine Prem and Chris Harling, physiotherapy technical instructor Michelle McCallum and Edwina Perkins, Regional Care Advisor funded through the Muscular Dystrophy Campaign. Additional physiotherapy time is also supported by the MDC as is some administrative support. There is also a programme of nurse-led clinics for patients with myotonic dystrophy with cardiological backup. In addition, the Muscle Centre has developed collaborative links with colleagues in cardiology, respiratory support, orthopaedics, neuropathology and other specialties.

The Muscle Centre also receives funding through the National Commissioning Group as a national specialist centre for Limb Girdle Muscular Dystrophy, and sees referrals from across the country of people with a suspected diagnosis of Limb Girdle Muscular Dystrophy. Muscle or DNA samples are also sent for analysis at the centre.

The Muscle Centre is also coordinating a European Union Network of Excellence for the development of translational research in rare neuromuscular diseases -TREAT-NMD (see Appendix 4).

- **md enterprises north east:**

Working with staff from the Muscle Centre, **md enterprises north east**, a new social enterprise in Newcastle upon Tyne, will be a key element of the managed clinical network for specialist neuromuscular services in the north east, strategically and operationally dovetailed with but independent of NHS provision.

md enterprises north east will be the only provider in this region offering an integrated skills, employment and health support/rehabilitation pathway with and for people affected by muscle disease. The enterprise is being designed around excellence, innovation, involvement and sustainability. It is based on new and innovative knowledge and delivery networks, partnership governance and an enterprise culture.

md enterprises north east will contribute to improved quality of life, raise aspirations and increase life choices for people affected by muscle disease. It will offer community-based specialist therapies and support services, including physiotherapy, occupational therapy and psychology. It will extend current NHS provision and improve accessibility for those diagnosed with md as well as family members and informal carers. It will also offer training to further education level and will support people into higher education, work or to develop their own businesses.

As a source of specialist expertise, professional and patient, it will also offer continuing professional development and mentoring opportunities to people who are providing front-line services, and placement and project experience for students on a range of courses at local colleges and universities.

Based on learning from the Neuromuscular Centre in Cheshire (see below) **md enterprises north east** will demonstrate the positive impact of co-locating this range of services and opportunities in an independent but integrated enterprise. This is being formalised in partnership agreements between core organisations: MDC, Newcastle upon Tyne Hospitals, Newcastle University and Newcastle College, acknowledged for excellence and leadership in their particular fields.

As a development of the Neuromuscular Centre and the Muscular Dystrophy Campaign **md enterprises north east** is already establishing working links with the Muscle Team and other regional research clinicians. With them, it will operate as a translational health resource offering expertise and services across tertiary, secondary and primary care. This powerful partnership is positioning **md enterprises north east** as a key delivery agency for community based and personalised services for people affected by muscular dystrophy and related neuromuscular conditions. Negotiations are progressing with the Muscle Team on innovative ways to co-work on safe, high quality services, streaming research and best evidence into practice (DH (2008) National Service Review: High Quality Care for All).

md enterprises north east will evolve into a nationally recognised, locally delivered Neuromuscular Centre of Excellence, co-located with a regionally accessible Training and Enterprise Centre. It could ultimately be a partner in a translational 'muscle scene' development on Science Central, co-located with regional/national md research and clinical services.

The **md enterprises north east** Project Board brings together the partners, representatives of service users, and families and carers, and social enterprise expertise. It has approved the Phase 1 Project Plan which scopes these services and opportunities, for which funding is now being sought.

Best practice model The Neuromuscular Centre (NMC) based in Cheshire provides ongoing specialist physiotherapy, in addition to training and employment within a small social enterprise. There are currently 360 registered patients at the NMC who, following a detailed assessment, are provided with an individual treatment programme which targets their main problems and deals with the areas that cause them most concern. There is also an advice and information service. A number of physiotherapy treatments are offered at the NMC including:

Passive stretches	Hydrotherapy
Assisted standing	Mobilisation techniques
Active exercises	Acupuncture for pain relief
Core stability exercises	Intermittent compression
Walking re-education	Electrotherapy

The benefits of the specialist physiotherapy are clear: 100% of service users said NMC physiotherapy treatment helped them stay out of hospital and 80% of those who are able to walk said that the physiotherapy had helped them to stay on their feet and walking.

In addition to physiotherapy, the NMC provides training and employment through a small, user-led social enterprise which it hosts, NMC Design+Print. It has recently been awarded the Social Enterprise Coalition's Best Social Enterprise, Enterprising Solutions 2008 award.

See: www.nmcentre.com for further information

5. Issues to be addressed:

Greater NHS investment is required in specialist staff such as physiotherapists, psychologists and dieticians to ensure that a multidisciplinary approach to care is delivered. There is currently no dedicated dietetic or psychology support to the clinics. The clinicians have also identified the need to develop pain treatment for the number of adult neuromuscular patients who suffer from chronic pain.

Capacity at Muscle Centre also needs to be increased to meet the demand of patients traveling from elsewhere in the UK and across Europe, particularly the Republic of Ireland, for treatment.

In addition, a number of the existing services at the Muscle Centre are vulnerable – despite its recognition as an international centre of excellence - due to their reliance on charitable funding, and the lack of succession planning.

o Care Advisors

Care Advisors play an essential role in providing a multidisciplinary package of care to individuals with muscular dystrophy and related conditions. They successfully coordinate health and social care needs, provide support and information to families and ensure a seamless transition from child to adult services.

The region has only one Care Advisor, Edwina Perkins, who is based at the Newcastle Muscle Centre. Nine out of ten of the people who have access to the service describe it as 'excellent' or 'good'. However two additional FTE posts are needed to serve the estimated 3,000 people with muscular dystrophy and related conditions served by the NESCG. The three posts of Care Advisor should be embedded within the NHS to end the present reliance on charitable funds.

The Muscular Dystrophy Campaign Patient Survey underline the need for an increase in care advisors/coordinators with over half of patients in the North East reporting that they are not satisfied with the level of emotional support available to their families and to themselves. Furthermore, two thirds of patients are not satisfied with the amount and clarity of information available to them.

Further, the importance of a named care advisor/coordinator was demonstrated by it being one of the recommendations of Health Minister Lord Darzi in his final report High Quality Care for All which set out how the Government intends to provide this more personalised level of care for people with long-term conditions.¹⁹ In addition, this issue was also highlighted in the Parker *et al* study of Duchenne patients at the Lane Fox Unit (2005) which noted: "Most patients received full provision of disability allowances, but full access to social services provision was inadequate, and often depended on the input of the muscular dystrophy key worker."²⁰

It has been noted by the NHS in the South West that neuromuscular care advisors are an essential element in a redesigned neuromuscular service that is likely to recoup the minimal costs through "benefits realisation such as reduced hospital admissions, stays, re-admissions and GP intervention".²¹

Case study:

One patient living in Newcastle underlines the importance of the current care advisor/coordinator based at the Centre for Life: "I received benefits and services I wasn't aware of due to the help of the co-ordinator."

o **Physiotherapy:**

There is only one full-time NHS funded physiotherapist and one technician at the Newcastle Muscle Centre – at least one further full time clinic-based physiotherapist is required to meet current demand and increasing demands of national audit projects.

It is accepted that all patients with a neuromuscular condition will at some point during the course of their condition require access to specialist physiotherapy which should be supported at the local level in each PCT area by ongoing physiotherapy support.²² Physiotherapy is the physical treatment and management of a condition which enables people with neuromuscular conditions to reach their maximum physical potential by maintaining mobility, independence and improving quality of life.

For people with a muscular dystrophy or related neuromuscular condition, this should be provided by a specialist physiotherapist, who has skills in both neurological and musculoskeletal physiotherapy, experience in treating muscle conditions and the confidence to treat patients with rare disorders, with support from a local physiotherapy service.²³ Specialist physiotherapy can delay the progression of the condition, reduce pain and minimise emergency hospital admissions.

Case study:

A young man with Limb Girdle Muscular Dystrophy from the North East receives physiotherapy from his father only. He says: "I know from having holidays away that going just two weeks without physio sees a tightening of my hamstrings. The years of physio have certainly not improved the range of movement in my legs but they have unquestionably slowed the constrictive process and prolonged my ability to walk unaided."

Evidence of a deficit in the delivery of specialist physiotherapy is shown by a number of Freedom of Information requests carried out in April 2008 by the Muscular Dystrophy Campaign. Ten out of twelve PCTs and five out of seven NHS Trusts in the North East responded to the request – key findings include:

- Fewer than half of the NHS Trusts and only a third of PCTs in the North East provide ongoing physiotherapy for children or adults with muscular dystrophy and related neuromuscular conditions where required;
- 80% of PCTs in the North East do not have physiotherapists with specific training in muscular dystrophy and related neuromuscular conditions available to adults;
- Two thirds of PCTs in North East do not have physiotherapists with specific training in muscular dystrophy and related neuromuscular conditions available to children.

These findings correlate with the results of the Muscular Dystrophy Campaign Patient Survey in which 38% of respondents reported that they had no access to physiotherapy. More children than adults received physiotherapy, often due to provision at their special school. However, this provision is then removed when the child leaves school or moves from paediatrics to adult services (see Transition section below).

For adults, access to ongoing physiotherapy can be extremely limited. For example the Newcastle upon Tyne Foundation Hospital Trust has an average wait of 4 months for adult physiotherapy. South Tyneside PCT only offers physio for children and reports that adults should attend this service as there is no other provision for them.

Case studies:

- *One patient from Middlesbrough said: "I've been affected as an adult for 36 years and I've never had or been offered any physio, instead I was given leaflets with some exercises on it for me to do at my own at home."*
- *A young woman from the North East said: "The physiotherapy I receive is of high quality just not on a regular basis. Adult services for physiotherapy are appalling! My physiotherapist is the same one I had when under child services!"*
- *A woman from Middlesbrough with Spinal Muscular Atrophy said: "I am now nearly chair shaped so regular physiotherapy could have helped prevent this. My arms no longer go straight which has happened since I left school where I received regular physio."*

The provision of physiotherapy in short blocks of sessions is also problematic for patients and indicates a clinical focus on conditions – such as recovery from a fracture - in which quantifiable improvement can be measured, rather than the maintenance of chronic and progressive conditions. Northumberland Care Trust reports that it provides physiotherapy only in blocks of 6-12 weeks.

○ **Psychology service:**

Psychology support has been identified as an important aspect of multi-disciplinary care, and as a key part of rehabilitation services.²⁴

Children and adults with neuromuscular conditions, including Duchenne muscular dystrophy, myotonic dystrophy and congenital myotonic dystrophy, would particularly benefit from the input of a clinical psychologist to help families develop management strategies. Specific issues for patients with muscular dystrophy and related neuromuscular conditions include support at the time of diagnosis, chronic illness, loss of ambulation, transition to adulthood, times of crisis and bereavement.

There are no dedicated psychologists currently serving the Newcastle Muscle Centre, and as such there is a pressing need to develop clinical and educational psychology input and support for this patient group.

○ **Speech and Language Therapy and Dietetics**

There is a clear and pressing need to develop Speech and Language Therapy (SALT) and dietetics services for neuromuscular patients in the North East.

Chewing and swallowing difficulties are frequent among neuromuscular patients and can have substantial impact on quality of life.²⁵ This has been related to increased weakness of the masticatory muscles, malocclusion or other abnormalities of the oropharyngeal phase. As a consequence of this, patients may be at risk of food aspiration and aspiration pneumonias. Chewing difficulties become increasingly present with age, associated with a progressive increase in the duration of meals.

The leading clinicians in the field recommend a multidisciplinary team approach, including input from speech therapist, dietician, neurologist, and respiratory clinicians to evaluate and managing feeding problems. A systematic evaluation of weight gain, feeding abilities and respiratory function should be part of the routine medical examination in order to identify early signs of failure to thrive and the best options of management.

A speech and language therapist will take a feeding history and look at how the muscles of the tongue, lips and throat are working. In addition they will look at any other problems that may affect chewing, for example with teeth. The safety of swallowing and if there are any risks of food or drink going down the wrong way (aspiration) needs to be assessed to enable safe management. The doctors and speech therapist will evaluate if this needs to be assessed in more detail. A videofluoroscopy, which is an X-ray of swallowing, may be done to look closely at how food is chewed and swallowed.

○ **Wheelchair services**

Wheelchairs for children and adults with conditions like muscular dystrophy have been identified by the Department of Health as a specialised service and are therefore subject to collaborative commissioning arrangements at a regional rather than local level. They are covered within the Department of Health's Specialised Services Definition No 5: The Assessment and Provision of Equipment for People with Complex Physical Disabilities (all ages) and also within Specialised Services Definition No. 23 Specialised services for children.

However PCTs do not always collaborate to provide specialist wheelchair services. Consequently, children and adults affected by these rare and progressive conditions are competing for equipment with patients who have acute episodes, for example a leg fracture. This can cause lengthy waits for essential equipment.

In addition, the Muscular Dystrophy Campaign Patient Survey revealed that a number of people are not being properly assessed or offered appropriate equipment. A number of children and adults with neuromuscular conditions are considered to have profound disabilities where the assessment process requires greater knowledge and expertise than are often available in local wheelchair services.

In the North East region, Freedom of Information requests have shown that disabled children wait on average 11 weeks to receive a powered chair. None of the North East's PCTs provide wheelchairs for disabled children which incorporate the sit-to-stand feature that is essential to help maintain muscle strength and prevent pressure sores. All of the region's PCTs rely on charitable funding to provide powered wheelchairs with the riser function that allows children to raise their seat to eye level with their peers and aid their social skills and personal development.

The North East SCG has recognised in its Agreed Specialised Services Investments for 2008-09 document that extra powered wheelchairs at a cost of £206,000 are needed to meet current demand.²⁶ We believe that rehabilitation equipment should be an integral part of a review into neuromuscular services in the region.

Appendix 1:Demographics:**North East PCT populations***(Population data taken from PCT websites/annual reports)*

PCT	Resident population	Prevalence of neuromuscular conditions (including muscular dystrophy)	Prevalence of muscular dystrophy	2009-10 PCT Revenue Allocations (£ thousands)
County Durham	497,000	497	248.5	886,825
Darlington	103,000	103	51.5	166,081
Gateshead	196,200	196.2	98.1	357,224
Hartlepool	93,000	93	46.5	163,405
Middlesbrough	196,000	196	98	257,714
Newcastle	265,000	265	132.5	466,097
North Tyneside	206,000	206	103	345,791
Northumberland	318,000	318	159	498,897
Redcar and Cleveland	130,000	130	65	233,544
South Tyneside	154,500	154.5	77.25	279,272
Stockton on Tees	186,000	186	93	287,728
Sunderland	277,960	277.96	138.98	510,293
Totals	2,622,660	2,623	1,311	4,452,871

Appendix 2:

Current North East commissioning structure

Specialised care in the region is commissioned by the North East Specialist Commissioning Group - a joint sub-committee of the twelve PCTs in the region (County Durham, Darlington, Gateshead, Hartlepool, Middlesbrough, Newcastle, North Tyneside, Northumberland Care Trust, Redcar and Cleveland, Stockton on Tees, South Tyneside, Sunderland). It had a commissioning budget for 2007/08 of £124 million.²⁷ The NE SCG serves the North East region population of approximately 2.6 million people, as well as 330,000 people in the North Cumbria area, who have their specialised services commissioned by the North East SCG rather than the North West SCG. The total population served by the NE SCG is therefore almost 3 million people.

The NE SCG was set up in April 2007 replacing the Northern Specialised Commissioning Group which had also covered Cumbria (now in the North-West SCG area). The NE SCG is chaired by Chris Reed, Chief Executive, North of Tyne Primary Care Organisations.

The NESCG is supported by the Northern Specialised Commissioning Core Team (Norscore), an integrated management team hosted by North Tyneside PCT. Norscore is responsible for leading the day to day commissioning of most specialised services in the North East and also commissioning services on behalf of North Cumbria residents at the request of the North West SCG. It is responsible for planning, procurement, performance monitoring and performance management of specialised services. Norscore informs and supports the Specialised Services Directors Group (SSDG), a subgroup of the NESCG, chaired by the director of commissioning of the North of Tyne PCOs.

The SSDG, which is responsible for driving the detailed work of specialised commissioning, comprises the directors of commissioning of the PCOs, a director of public health, director of finance, a SHA representative, the mental health commissioning lead for the North East and a representative from the North West SCG. As the SSDG is a sub-group of the NESCG, it refers matters that require a higher level of debate as well as binding collective decision making issues to the NESCG.

There are 12 PCTs in the region, managed within 5 groups or integrated management teams:

- NHS South of Tyne and Wear covers Gateshead PCT, South Tyneside PCT and Sunderland TPCT.
- NHS North of Tyne covers North Tyneside PCT, Newcastle PCT and Northumberland Care Trust
- County Durham PCT and Darlington PCT work together to provide services.
- North of Tees: Hartlepool PCT and Stockton-on-Tees TPCT,
- South of Tees: Middlesbrough PCT and Redcar and Cleveland PCT

While each remains a statutory organisation in its own right, in each group the day-to-day management of the PCTs is been brought together under a single management team.

There are also seven NHS hospital trusts in the region.

North East Specialised Commissioning Group Membership 2007/08

The North East Specialised Commissioning Group (NESCAG) voting membership consists of the PCO chief executives. SHA directors are also invited to attend NESCAG meetings. The NESCAG has overall responsibility for the commissioning of specialised services on behalf of PCOs across the North East. The current members of the group are:

Name	PCT Representing
Chris Reed (Chair)	Newcastle, North Tyneside, Northumberland
Chris Willis	North Tees, Hartlepool
Karen Straughair	Sunderland, South Tyneside, Gateshead
Colin McLeod	Middlesbrough, Redcar & Cleveland
Yasmin Chaudhry	County Durham
Colin Morris	Darlington

Specialised Services Directors Group Membership 2007/08

The NESCAG is supported by the Specialised Services Directors Group which is responsible for driving the detailed work of specialised commissioning.

Name	PCT Representing
Mark Adams	North of Tyne PCOs
Cameron Ward	County Durham & Darlington PCOs
David Hambleton	South of Tyne PCOs
Mike Procter	Tees PCOs
Tricia Cresswell	Public Health Lead
Chris Macklin	Finance Lead
Rosemary Granger	Mental Health & Learning Disabilities Regional Team
Moira Davison	North of England Cancer Network
Rachel Chapman	Communications Lead
Mark Smith	North East Strategic Health Authority
Jon Develing	North West Specialised Commissioning Team
Mike Maunder	Northern Specialised Commissioning Core Team

Appendix 3:

North East Neurosciences Network

The Neurosciences Network, hosted by Middlesbrough PCT, was set up in March 2008 and is led by Lynne Barr. The Network meets bi-monthly and aims to work together with different organisations, voluntary sectors, and people with neurological conditions and their carers to improve services across the region.

The Network was created following a meeting of Tees Valley Health Scrutiny Joint Committee on November 21st 2007, when the Neurological Alliance presented a paper highlighting that little had progressed under the Long-term Conditions (LTC), National Service Framework specifically in respect of neurological conditions.

The Network's aims are as follows:

- To meet the quality requirements in the National Service Framework for Long Term Neurological Conditions
- Improve equity of access,
- Establish quality and consistency of services
- Support local service redesign & care closer to home
- Learn, share and work in Partnership

The Network is comprised of:

- **Lynne Barr**, Neurosciences Network Lead
- **Glenys Marriott**, Chair of Neurosciences Network
- **Laura Bailey**, Neurosciences Network Support Officer
North East Primary Care Trusts
- Local Authorities
- Employment Agencies
- NEPHO (North East Public Health Observatory)
- Independent Sector Neurological Alliance (TVDNY)
- Specialist NHS Neurological Services - e.g. Walkergate Park
- National Long Term Conditions Team

In January 2009 Lynne Barr presented the Network's two year commissioning proposals to the Directors of Commissioning (DOCs). They were:

- Community Neuro Rehab
- Support for the Network
- Service Improvements for Neuro Pathways.

Appendix 4:

Treat-NMD

The Newcastle Muscle Centre is also coordinating a European Union Network of Excellence for the development of translational research in rare neuromuscular diseases -TREAT-NMD.

TREAT-NMD is an international initiative bringing together some of the world's leading neuromuscular specialists in a pan-European 'network of excellence' aimed at improving treatment and finding cures for patients with neuromuscular disorders. Linking 21 partner organisations and over 300 doctors, researchers and other professionals throughout 11 European countries, the EU-funded network is enabling experts to work together to share good practice and improve global standards of care.

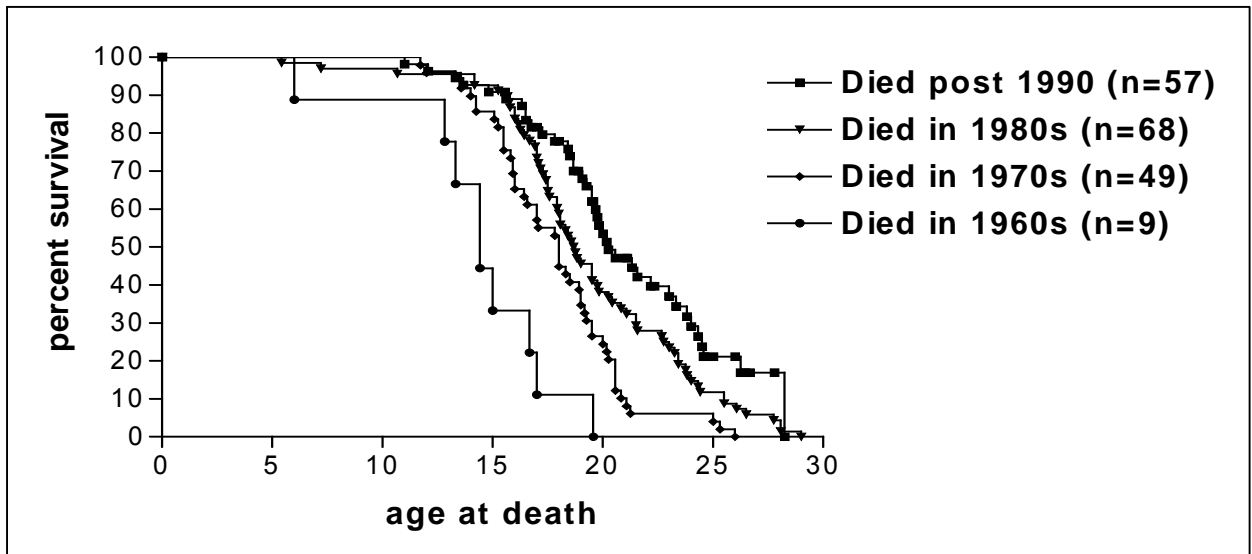
Recent advances in cutting-edge therapies mean that clinical trials are no longer in the realm of fantasy. But such advances are being delayed because of the fragmentation that currently exists in research and healthcare systems across the globe. Without validated outcome measures, consensus on standards of care and diagnosis, or uniform patient databases, setting up international trials of the most promising therapies is an uphill struggle. TREAT-NMD addresses all these areas of concern, with working groups of partners devoted to each of the major issues.

TREAT-NMD aims to:

- Develop common standards for diagnosis and care of neuromuscular disorders
- Create harmonised international patient registries and biobanks
- Establish a Clinical Trials Coordination Centre
- Work with industry to develop new and effective treatments for neuromuscular diseases
- Aid the progression of cutting-edge therapies from the lab to the clinic
- Develop standardised protocols and outcome measures for neuromuscular disorders
- Draw up standardised procedures for the production of therapeutic agents and for their toxicology, quality and safety assessment
- Encourage 'spreading of excellence' by facilitating research placements, visiting professorships and other training opportunities for scientists and clinicians
- Develop close links with patient groups worldwide
- Work closely with international specialists to ensure harmonisation on the key issues

Appendix 5:

Duchenne Muscular Dystrophy Survival:



The authors reviewed the notes of 197 patients with Duchenne muscular dystrophy whose treatment was managed at the Newcastle muscle centre from 1967 to 2002, to determine whether survival has improved over the decades and whether the impact of nocturnal ventilation altered the pattern of survival.

Results:

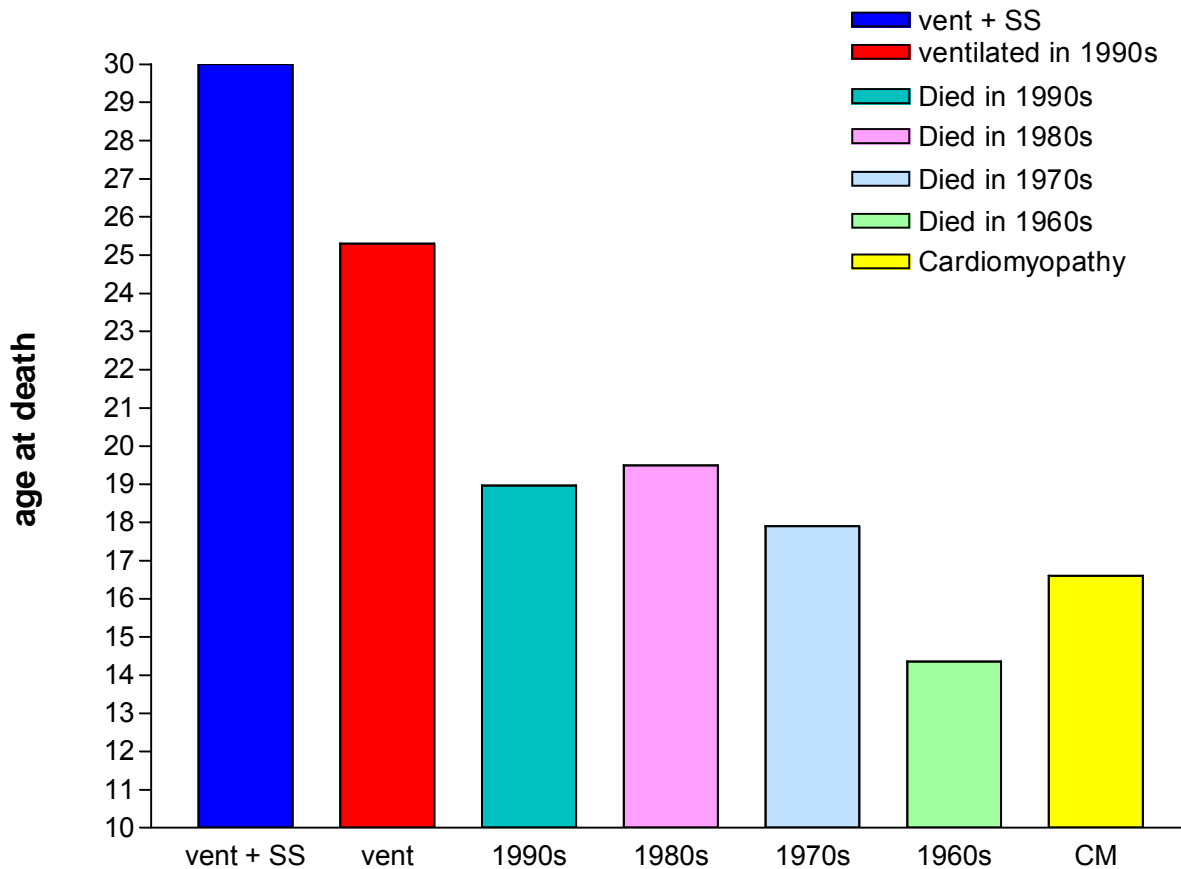
1960s: Mean life expectancy: 14.4 years - No survivors beyond 19.29 years

1990s: Mean life expectancy: 19.5 years

Improvement is due to multi-disciplinary care

Eagle et al Survival in Duchenne muscular dystrophy: improvements in life expectancy since 1967 and the impact of home nocturnal ventilation²⁸

Mean age of death



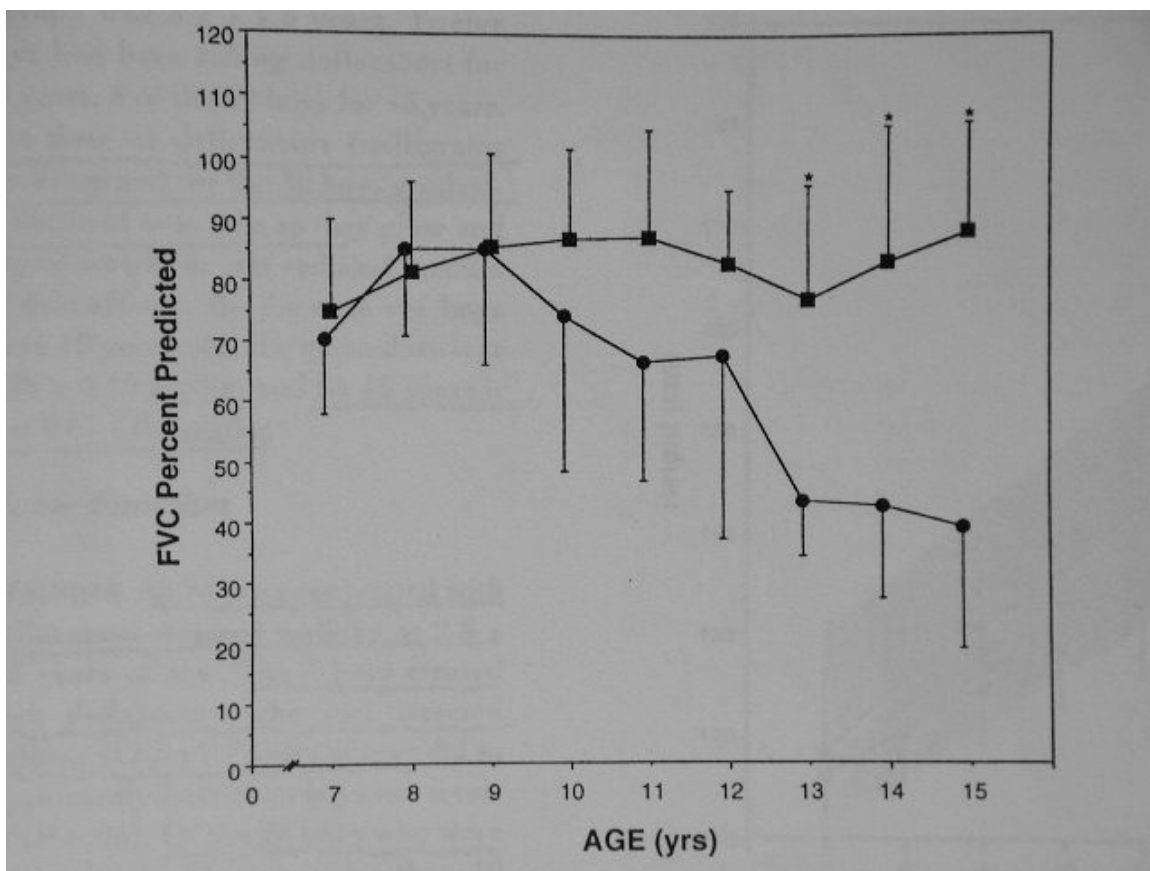
In addition, in a further peer-reviewed study from 2007, the Newcastle Centre's Michelle Eagle *et al* highlighted that patients with Duchenne muscular dystrophy treated at the Newcastle Centre and receiving home ventilation had a mean age of death of almost 30 years.²⁹ The survival rate has continued to improve.

M. Eagle, J. Bourke, R. Bullock, M. Gibson, J. Mehta, D. Giddings, V. Straub, K. Bushby (2007) Managing Duchenne muscular dystrophy – The additive effect of spinal surgery and home nocturnal ventilation in improving survival Neuromuscular Disorders, Volume 17, Issue 6, Pages 470-475.

Appendix 6:

Preserved lung function

(from Biggar WD, Harris VA, Eliasoph L, Alman B. Long-term benefits of deflazacort treatment for boys with Duchenne muscular dystrophy in their second decade. *Neuromuscular Disorders*)³⁰



The article compares the clinical course of 74 boys 10-18 years of age with Duchenne muscular dystrophy (DMD) treated (40) and not treated (34) with deflazacort.

Results for lung function:

- Deflazacort group: 88% (\pm 18%)
- No treatment Group 39% (\pm 20%)

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