Building on the Foundations: The Need for a Neuromuscular Service Serving Patients in the NHS East Midlands Region
July 2009
A report by the Muscular Dystrophy Campaign with contributions from and endorsed by the leading neuromuscular clinicians in the East Midlands region:

Dr Max Damian - Adult Neurologist, Leicester General Hospital.

Dr William Kinnear - Consultant respiratory physician at Queens Medical Centre (QMC), Nottingham

Dr Margaret Philips - Honorary Consultant and Associate Professor in Rehabilitation Medicine, Nottingham

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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 East Midlands Strategic Health Authority region to set out a number of serious concerns regarding the provision of specialist clinical services in the East Midlands for patients with muscular dystrophy and related neuromuscular conditions.

We are calling for a major shift in the way services are commissioned in the region in line with the Department of Health’s guidance that services for patients with this group of rare conditions should be regarded as specialised and therefore subject to collaborative commissioning arrangements.

The specialised commissioning of these services would be an effective way of delivering care for rare and high cost treatments. The arrangements would provide best value for money and long-term savings for the nine Primary Care Trusts (PCTs) in the region and would ensure fair access to clinically effective, first class, specialised services across the East Midlands.

Action needed:

- A short life working group should be established to carry out an in-depth review of current service provision and its vulnerability in the East Midlands. This review would involve families, clinicians, PCTs and the Specialised Commissioning Group (SCG), and would bring forward proposals in autumn 2009 to secure and develop the comprehensive, multi-disciplinary service for children and adults, including transition services for young people.

- A neuromuscular network should be established on the model of a managed clinical network. This will ensure coordination between the existing clinics and ensure that expertise is shared with all clinicians and Allied Health Professionals (AHPs) in each PCT in the region. The network should be supported by a Network Coordinator. This model has been agreed in the South West and has been successful in Scotland with the Scottish Muscle Network. It also reflects the model set out in the National Definition for specialist neuromuscular services.
• The foundations of good practice have already been laid with the establishment of a Professional’s Network in the East Midlands. We call on the SCG, together with PCTs in the region, to provide greater support to this Network.

• Three additional full-time Regional Care Advisors (RCAs) with expertise in muscular dystrophy and related neuromuscular conditions should be established and embedded in the NHS to serve the 4,000 people in the area living with these conditions.

• Ongoing physiotherapy should be provided to all adults and children with a neuromuscular condition in each PCT, supported and developed in each PCT area by enhanced specialist physiotherapy support from the specialist clinics.

• Psychological support should be provided as part of a multi-disciplinary approach to care for individuals and family members living with a neuromuscular condition in each PCT area across the East Midlands.

Our key findings include:

• Specialist neuromuscular services are vulnerable due to their reliance on charitable funding. The region’s only care advisor, based at the QMC Nottingham, is funded by the Muscular Dystrophy Campaign and we are calling for this vital post to become embedded in the NHS from April 2010.

• Two thirds of patients and their families have no access to a key worker or care co-ordinator. Four full-time RCAs are needed to serve the 4,000 children and adults in the area with a neuromuscular condition, many of whom are simply not known to providers of health services.

• Patients have very limited access in particular to ongoing physiotherapy. Specialist physiotherapists are required to support outreach clinics and provide training and professional development for community physiotherapists. Three quarters of patients in the East Midlands feel that they do not receive enough physiotherapy.

• There is no dedicated psychology service for neuromuscular patients despite its importance as part of multi-disciplinary care for this patient group with rare and very rare progressive conditions, often genetic in origin and with no known cures and only limited treatments available.

• Greater support at transition from paediatric to adult services is needed given the evidence of services being removed or greatly reduced when people leave paediatric services even though needs may well increase given the progressive nature of many conditions.
1. **Background:**

There are over 200 muscular dystrophies and related neuromuscular conditions. They are multi-system disorders, which require complex long-term surveillance and care.

Without specialist multi-disciplinary care most patients and their families experience a reduction in quality of life. Improved genetic counselling is likely to cause a small reduction in the overall incidence of these conditions but improved survival will increase their prevalence in the adult population.

2. **Demographics:**

More than 4,000 people in the East Midlands region affected by a form of muscular dystrophy or a related neuromuscular condition.

The East Midlands region has a population of approximately 4.3 million people and also supports neuromuscular patients in Sheffield, Doncaster, Barnsley and Rotherham.

Specialised care in the region is commissioned by the East Midlands Specialist Commissioning Group. The region’s nine PCTs are Northamptonshire PCT, Leicester City PCT, Leicestershire County and Rutland PCT, Derby City PCT, Nottingham City PCT, Nottingham County PCT, Lincolnshire PCT, Bassetlaw PCT, Derby County PCT. Leicestershire County & Rutland PCT hosts the East Midlands SCG.

**SCG Budget: In 2007/08 this was £236 million**

3. **Current level of essential, specialist provision in the East Midlands:**

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 local, regional and national service providers. A neuromuscular RCA is essential to provide vital specialist care, support and advice for each individual and family living with one of these conditions.

Specialist multi-disciplinary care can improve quality of life and extend life expectancy. For example, without treatment, the mean age of death in Duchenne muscular dystrophy is 19 years. With specialist care and home ventilation, life expectancy is raised to almost 30 years.

Co-ordinated and comprehensive multi-disciplinary specialist care should include a neuromuscular specialist consultant and, dependent on medical need, may also include specialist cardiac, respiratory and orthopaedic care. 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. 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 4). For a number of neuromuscular conditions, regular check ups are required irrespective of symptomatology, because deterioration can advance rapidly over the course of months.  

- **Specialist muscle clinics:**

Over half (55%) of all PCTs do not commission adult or paediatric muscle clinics in the East Midlands. The details of existing clinics are as follows:

**Paediatric Clinics:**

Paediatric muscle clinics in the East Midlands region are led by:

- Dr Peter Baxter, Consultant Paediatric Neurologist, Sheffield Children’s Hospital
- Dr Gabriel Chow, Consultant Paediatric Neurologist, Nottingham University Hospitals NHS Trust
- Dr Moira Broderick, Consultant Community Paediatrician in Derby.
- Dr Adrian Brooke, Consultant Community Paediatrician from Leicester.

**Ryegate Children’s Centre, Sheffield** - monthly paediatric muscle clinic

**Rotherham, Barnsley, Chesterfield, Doncaster** - two per year

**Grimsby** - one per year

Dr Baxter, a specialist neuromuscular physiotherapist and the RCA see children with all types of muscle conditions at the following clinics. Elaine Scott is the physiotherapist for these clinics.

**Children’s Hospital, Sheffield** – three times annually combined muscle/respiratory/spinal clinic

Attended by Dr Baxter, Dr Rob Primhak, consultant paediatric respiratory physician and either Mr Ashley Cole or Mr Lee Breakwell, consultant spinal surgeons. Children with a variety of neuromuscular conditions attend this clinic if they require input from all three consultants. It is beneficial to patients as they only have to attend one appointment instead of three.

During 2009, Dr Baxter and the RCA are holding a specific muscle clinic at Pilgrim Hospital in Boston for children within the South Lincolnshire area.

**QMC, Nottingham** - bi-monthly combined paediatric muscle/spinal clinic

Children with neuromuscular conditions at risk of developing scoliosis are seen by either Dr Chow or her registrar, or Dr Toni Wolff, a paediatrician specialising in disability.
Children are also seen by Mr Mehdian, consultant spinal surgeon or one of his team. Alison Miah, community paediatric physiotherapist provides regular input to the clinic. Jenny Grayston, a community occupational therapist normally provides support for the clinic but was on maternity leave during 2008, with no replacement.

**Children’s Centre, Nottingham City Hospital – bi-monthly paediatric muscle clinic**
This clinic is attended by young children with muscle disease and older children at a low risk of developing a scoliosis. Patients are seen by Dr Chow and/or her registrar and the Care Advisor. **It has not been possible to obtain physiotherapy and occupational therapy cover for this clinic and we are calling on the SCG, together with PCTs in the region, to address this problem.**

**Child Development Centre, Derby – bi-monthly paediatric muscle clinic**
Children with all types of muscular dystrophy or spinal muscular atrophy (SMA) are seen by Dr Broderick and the RCA. Dr Will Carroll, consultant paediatrician specialising in respiratory issues attends the clinic when possible. The specialist nurse that previously attended the clinic is no longer providing regular input. Local therapists attend with the children if possible.

We are calling on the SCG to address the matter of declining support from the specialist nurse at this clinic.

**Ash Field Special School, Leicester – termly / half-termly (as needed) paediatric muscle clinic**
Dr Brooke, school medical staff and the RCA attend this clinic for children with a variety of neuromuscular conditions who usually attend with their parents. Dr Nahin Hussain, a new consultant paediatric neurologist from Leicester Royal Infirmary now also attends the clinic.

**Leicester Royal Infirmary – three monthly paediatric muscle clinics**
Dr Brooke, Dr Nahin Hussain, consultant paediatric neurologist and the RCA see mainly younger children with a variety of neuromuscular conditions.

**Adult clinics:**
Adult muscle clinics in the East Midlands region are led by:

- Dr Margaret Phillips, senior lecturer/consultant in rehabilitation from Derby
- Dr Adrian Wills, adult neurologist, QMC, Nottingham
- Dr Will Kinnear, respiratory physician, QMC, Nottingham
- Dr Max Damian, adult neurologist, Leicester General Hospital.

**Derby City Hospital – monthly adult muscle clinic**
Patients are seen by Dr Phillips and the RCA. An occupational therapist who provides support for the clinic when needed, is part of the rehab team.
QMC - three monthly adult muscle/respiratory clinics
Patients are seen by Dr Wills and Dr Kinnear. Dr John Walsh, consultant cardiologist also attends the clinic. This clinic allows patients to see all three specialists at the same time and allows discussion of management between the various professionals.

Leicester General Hospital – monthly adult muscle clinic
Dr Damian runs a weekly muscle clinic for adults with a variety of muscle conditions.

- Regional Care Advisors (RCAs)

RCAs play an essential role in supporting individuals with muscular dystrophy and related conditions. They successfully co-ordinate their health and social care needs, provide support and information to families and help to ensure a seamless transition from child to adult services. They also save money over the long term, due to freeing up consultants' time and helping to reduce unplanned hospital admissions.

The role of the RCA is crucial to these clinics. Subjects such as work, education, equipment provision and adaptations can be discussed in advance and referrals to local services made when appropriate.

The region has only one RCA, Julie Cassell, who is based at the QMC in Nottingham. The service is highly valued with patients and families describing the RCA service as excellent or good. However, due to the service being over subscribed two thirds of patients in the region do not receive the care and support of a RCA. An extra three WTE posts (each with a case load of 1,000 patients) are needed to serve the estimated 4,000 people with muscular dystrophy and related conditions in the region, many of whom are simply invisible to providers of health services.

It is essential that additional RCAs are appointed in order to serve the whole population. We are also calling for the existing RCA to become embedded in the NHS as a matter of urgency.

The East Midlands SHA identifies the importance of a named care advisor for people with long-term conditions as part of their Strategic Plan 2009/10.⁷

“All patients with a long term condition having their own personalised care plans by 2009, which will be owned by them and reviewed at least annually.” (East Midlands SHA Strategic Plan 2009/10)

The provision of a named RCA/co-ordinator is also stated as an aim by 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.⁸ In addition, the need for a RCA was 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.”⁹
The results of the Muscular Dystrophy Campaign’s Patient Survey, September 2008, highlighted the need for an increase in RCAs, with over two thirds of patients reporting that they are not satisfied with the level of emotional support available to their families and to themselves and their families. Furthermore, only half of patients are satisfied with the amount and clarity of information available to them.

- **Diagnosis experience:**

Half of patients in the area describe their experience of the diagnosis process as either poor or very poor, with many calling for greater information and support to be given to parents and families after diagnosis.¹⁰

There is a lack of knowledge among GPs about these rare conditions, suggesting a need for greater education of the early symptoms of neuromuscular conditions.¹¹

- **Respiratory clinics:**

Breathing disorders are recognised as the leading cause of mortality in neuromuscular disease.¹² Respiratory muscle weakness is relatively common in most neuromuscular conditions and is almost inevitable in the late stages of Duchenne muscular dystrophy.¹³ However treatment, including ventilation, has been shown to improve both quality and length of life.¹⁴

<table>
<thead>
<tr>
<th>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. This compares with a mean of age of death of almost 30 years in patients with Duchenne receiving home ventilation and specialist multi-disciplinary care reported by the Newcastle group in the most recent study by Eagle et al (2007).¹⁵</th>
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</table>

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 patients can become too accustomed to their chronic illness and therefore rarely raise complaints about respiratory distress spontaneously.¹⁶

Evidence from a 2003 study highlighted that it is more cost-effective to manage respiratory issues through check ups and home ventilation than through unplanned critical hospital admissions.¹⁷

- **Cardiac clinics:**

As a number of neuromuscular conditions also affect the heart, cardiac monitoring must be part of a multi-disciplinary approach to care. The heart is affected in different ways – people affected by myotonic dystrophy and Emery-Dreifuss muscular dystrophy are
prone to abnormal heart rhythms, while cardiomyopathy is more likely for people affected by Duchenne or Becker muscular dystrophy.

Regular cardiac screenings are crucial even for conditions which 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.” 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. Without screening, cardiomyopathy can progress almost entirely without symptoms until signs of heart failure emerge, when all cardiac reserve has been eroded.

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 experience no symptoms.

○ Physiotherapy:

It is accepted that all patients with a neuromuscular condition will at some point during the course of their condition require access to ongoing and timely physiotherapy. 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. 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. Specialist physiotherapy can delay the progression of the condition, reduce pain and minimise emergency hospital admissions.

In April 2008, the Muscular Dystrophy Campaign carried out a Freedom of Information request to all NHS Trusts and Primary Care Trusts across England about the provision of physiotherapy services. Of those PCT in the East Midlands, the following picture emerged:

○ Only five out of nine PCTs responded. Of these:
  ○ Only two out of five (40%) provide ongoing physio for patients with muscular dystrophy and related conditions where required
  ○ Only two out of five (40%) have trained physios available for children.
  ○ Only two out of five (40%) have trained physios available for adults.

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).

One young adult from Chesterfield has Duchenne muscular dystrophy and has had difficulty in accessing physiotherapy services:

“My regular care worker is only 19 years old and my parents have found the cost of insuring him on their adapted vehicle very prohibitive. The fact that my care worker is so
young means my family has to pay more for me to be able to get around. Things like this should be considered when care workers are placed with people requiring care.”

A former Chair of the FSH support group who lives in Leicester said:

“Many people’s experience is that they are told there is nothing that can be done for their condition, so they are not referred to physiotherapy or any kind of specialist help.

“Some of our members decide to seek physiotherapy, perhaps through talking to other members, or hearing talks at events. When they try to get a referral to a physiotherapist there are various barriers. My local hospital has a computer system that automatically discharges patients after six months, so when I see the physiotherapist I then have to make an appointment for five months and three weeks.”

The provision of physiotherapy in short blocks of sessions is problematic for patients and indicates a clinical focus on conditions in which quantifiable improvement can be measured, rather than the maintenance of chronic and progressive conditions. For example, many hospital Trusts provide physiotherapy in six week blocks with patients to be referred back for more treatment.

We are calling for ongoing, specialist physiotherapy to be provided to those patients in the region who require it.

- Orthopaedic care

Spinal deformity, such as scoliosis is common in many neuromuscular conditions, with 90% of people affected by Duchenne muscular dystrophy for example, likely to develop a clinically significant scoliosis.23

Surgery to correct spinal deformity can 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°.24 As it is a major operative procedure, a multi-disciplinary approach, involving the paediatrician/paediatric neurologists and orthopaedic surgeons is essential in the approach to surgery.25

As an example, the best practice guidelines for patients with spinal muscular atrophy state that evaluation should take place every three to six months, and more frequently in clinically unstable non-sitters. The evaluation should include, depending on clinical need: inspection of the spine, chest x-rays and radiographic evaluations of scoliosis, swallow studies, pulse oximetry and polysomnography.26

- Rehabilitation and equipment:

Specialist neuromuscular rehabilitation clinics aim to help maintain independence and to adapt to changes which affect social and domestic life and can include a number of services including physiotherapy, occupational therapy, speech and language therapy,
wheelchair services and orthotics. Rehabilitation care can improve quality of life and delay progression of the condition. For example, poorly fitting knee-ankle-foot orthoses can severely compromise mobility and successful care. To avoid this orthotists with specific experience in neuromuscular disorders should be used to measure and supply orthotics.  

**Wheelchairs**

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 is often available in local wheelchair services. The *Muscular Dystrophy Campaign Patient Survey* revealed that a number of people are not being properly assessed or being offered appropriate equipment.

Currently, as PCTs do not collaborate to provide specialist wheelchair services, 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, and are often being forced to wait for long periods for essential equipment. Muscular Dystrophy Campaign Freedom of Information requests have shown that, with regards to wheelchair provision:

Of those five out of nine PCTs who responded:

- None provide riser or sit-to-stand chairs (80% said no, 20% did not answer)
- Average wait for a powered chair is 22 weeks (four weeks longer than national average). Children in Derby City PCT wait an average of 26 weeks (six months) for a powered chair

**Psychologists:**

Psychology support has been identified as an important aspect of multi-disciplinary care, and as a key part of rehabilitation services. There is pressing need to develop clinical and educational psychology input and support for this patient group.

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.

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**Studies have shown that the incidence of autistic spectrum disorders, attention deficit hyperactivity disorders and obsessive compulsive disorders is higher in males affected by Duchenne muscular dystrophy. In addition behavioural changes have been shown to be an adverse side effect of treatment with corticosteroids, which are used to prolong ambulation and preserve muscle strength and respiratory function. Early input from a clinical psychologist may**
help parents develop strategies with which to manage these behavioural difficulties and thus prevent the need to withdraw steroid treatment.

- 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.\(^{31}\)

However, despite the significance of this period for younger people with these progressive neuromuscular conditions, the majority do not have access to a RCA who can support their transition to adulthood.

The difficulties are shown by respondents to the *Muscular Dystrophy Campaign Patient Survey*, with three out of five families describing the transition process as ‘poor’ or ‘very poor’. Only 14% rated the process as good or excellent, with the question not being applicable to the rest of respondents.
Appendix 1:

East Midlands Demographics:

Population data taken from PCT websites/annual reports

<table>
<thead>
<tr>
<th>PCT</th>
<th>Resident population</th>
<th>Prevalence of neuromuscular conditions</th>
<th>Prevalence of muscular dystrophy</th>
<th>2009-10 PCT Revenue Allocations (£ thousands)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Leicestershire County &amp; Rutland</td>
<td>933,400</td>
<td>933</td>
<td>467</td>
<td></td>
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<tr>
<td>Northamptonshire</td>
<td>678,200</td>
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<td>339</td>
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<td>Leicester City</td>
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<td>293</td>
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<td></td>
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<tr>
<td>Bassetlaw</td>
<td>111,700</td>
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<td>Central Derby</td>
<td>236,300</td>
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<td>Derbyshire County</td>
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<td>Lincolnshire</td>
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<td>Nottingham City</td>
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<td>Nottingham County</td>
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South Yorkshire Demographics:

<table>
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<th>PCT</th>
<th>Resident population</th>
<th>Prevalence of neuromuscular conditions</th>
<th>Prevalence of muscular dystrophy</th>
</tr>
</thead>
<tbody>
<tr>
<td>Barnsley</td>
<td>224,600</td>
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<tr>
<td>Doncaster</td>
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<tr>
<td>Rotherham</td>
<td>255,000</td>
<td>255</td>
<td>128</td>
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<tr>
<td>Sheffield</td>
<td>530,300</td>
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</table>
Appendix 2:

Background to report:

The report contains:

- Evidence from the leading neuromuscular clinicians working in the East Midlands area.

- Information from the latest research papers on the impact of specialist services on those affected by muscular dystrophy and related neuromuscular conditions.

- Data from the responses to the largest nationwide survey of people affected by muscular dystrophy and related conditions, published in September 2008 by the Muscular Dystrophy Campaign. 850 people completed the survey from across the UK – including 62 families from South Central.

- The responses to Freedom of Information requests to all Primary Care Trusts and Acute Trusts regarding specialist services,
Appendix 3:

Duchenne Muscular Dystrophy Survival data 1960-1990
(Eagle et al Survival in Duchenne muscular dystrophy: improvements in life expectancy since 1967 and the impact of home nocturnal ventilation)

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
Appendix 4:

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)⁴³

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% (± 18%)
- No treatment Group 39% (± 20%)
References:

1. 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.


