

**Muscular  
Dystrophy**  
Campaign



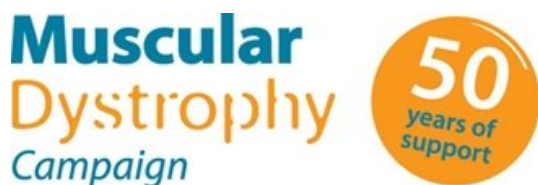
## **Building on the Foundations: The Need for a Neuromuscular Service Serving Patients in the NHS South East Coast Region**

May 2009



**A report by the Muscular Dystrophy Campaign with  
contributions from the leading neuromuscular clinicians serving  
the South East Coast region.**

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



**Building on the Foundations: the need for a neuromuscular service  
across South East Coast  
April 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 South East Coast Strategic Health Authority region to set out a number of serious concerns regarding the provision of specialist clinical services in the region for patients with muscular dystrophy and related neuromuscular conditions.

We are calling for a major shift in the way services are commissioned in the South East Coast 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 (see appendix 2).

The specialised commissioning of these services would be the most effective way of delivering care for rare and high cost treatments. The arrangements would provide best value for money and long-term savings for Primary Care Trusts and would ensure equitable access to clinically effective, first class, specialised services right across the South East Coast region.

**Urgent action needed in the South East Coast region:**

- **A short life working group should be established to carry out an in-depth review of current service provision and its vulnerability in South East Coast. This review would involve families, clinicians, PCTs, and the SCG 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.**
- **A neuromuscular network should be established on the model of a managed clinical network. Commissioners in the South West have agreed to take forward this model which has already been successful in Scotland with the Scottish Muscle Network and it also reflects the model set out in the National Definition Set for specialist neuromuscular services.**
- **At least four full-time Care Advisors/neuromuscular care coordinators with expertise in muscular dystrophy and related neuromuscular conditions should be established and embedded in the NHS to serve the 4,300 people in the area living with these conditions.**

- The existing specialist physiotherapist (based at the tertiary centre at Kings College Hospital) and local physiotherapists across the region should be supported as part of a managed clinical network to ensure that ongoing physiotherapy is provided to all adults and children with a neuromuscular condition in each PCT, supported and developed by enhanced specialist physiotherapy support from the tertiary centres.
- Resources to be allocated to ensure that a structure is in place to ensure that vital respiratory and cardiac support can be accessed by all neuromuscular patients who require it. These are key factors in extending life.
- Psychological support is a vital element of a comprehensive service that should be provided for patients and families living with a neuromuscular condition in South East Coast. These are, in the main, genetic conditions and families require psychological support throughout the lifetime of the condition and particularly during key times such as diagnosis, genetic counselling, when becoming wheelchair dependent, at transition stage, prior to spinal surgery and when end of life issues need to be addressed.

**Our key findings include:**

- Services for people with muscular dystrophy and related neuromuscular conditions in the South East Coast region are uncoordinated and patchy. There are very few PCT commissioned local muscle clinics in the region and the work of the out-of-area tertiary centres is frequently poorly supported by local primary and secondary services.
- Errors in diagnosis and delays in diagnosis were found which were not corrected until the patient was referred to the specialist tertiary centre.
- There are no regional neuromuscular care advisors in this region to provide day to day support for families and individuals living with Muscular Dystrophy.
- A widespread deficit in provision of services by allied health professionals (AHPs) was also identified with very limited access in particular to ongoing physiotherapy.<sup>1</sup> Specialist physiotherapists are required to support outreach clinics and provide training and professional development for community physiotherapists.
- 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 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 younger people leave paediatric services, even though needs may well increase given the progressive nature of many conditions.

## 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. 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 (see appendix 1):

There are some 4,300 people in the NHS South East Coast region affected by muscular dystrophy or a related neuromuscular condition.

It is estimated that there are about 1,000 people for every 1 million of the population who are affected by neuromuscular diseases. Many of these are low-incidence conditions and some are ultra-orphan affecting less than 100 people in the whole of the UK. Improved genetic counselling is likely to cause a small reduction in the overall incidence of these conditions but improved survival has increased their prevalence in the adult population. For example, the number of boys with Duchenne muscular dystrophy living into adulthood is increasing each year due to well-documented management interventions.

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

Coordinated 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.<sup>2</sup> 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 patient's lives and increase their life spans (see appendix 3). 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 also may also delay the onset of breathing complications (see appendix 5). For a number of neuromuscular conditions, regular check-ups are required irrespective of symptomatology, because deterioration can advance rapidly over the course of months.<sup>3</sup>

**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 18 years.<sup>4</sup> With specialist care and home ventilation life expectancy is raised to almost 30 years.<sup>5</sup>**

#### **4. Commissioning of specialist services in South East Coast:**

Commissioning of specialised services is managed in the South East Coast region by the South East Coast Specialised Commissioning Group (SCG). The SCG is a joint sub-committee of the eight PCTs in the region: Brighton and Hove City, East Sussex Downs and Weald, Eastern and Coastal Kent, Hastings and Rother, Medway, Surrey, West Kent, and West Sussex. It had a commissioning budget for 2008/09 of £389 million.<sup>6</sup> It is managed by West Kent PCT.

The combined budget for the eight PCTs in the region is £6.8 billion.

#### **5. Current level of essential, specialist provision in South East Coast:**

Access to local specialist multidisciplinary care is not available to all patients in the South East Coast region. The results of Freedom of Information requests have shown that no Primary Care Trusts in South East Coast commission muscle clinics for adults with muscular dystrophy or a related condition.

The majority of neuromuscular services in the region are commissioned by PCTs from tertiary centres in London; for example Surrey PCT commission services from St George's NHS Trust, while Kent and Medway PCT commission neuromuscular services from Kings College Hospital Foundation Trust and from Guys and St Thomas Hospital Trust. Almost half of all neuromuscular patients living in South East Coast travel to tertiary centres in London but links are poor between the tertiary centres and secondary/primary care.

In addition, almost one-fifth of respondents to the patient survey do not receive any specialist care: the majority of these are people over the age of 60, with the remaining third aged over 30, who have generally either not received a clear diagnosis, or were diagnosed several years ago, and told that there was no treatment or care available for their condition.

##### **o Neuromuscular care advisors:**

Care advisors (care coordinators/key workers) play an essential role in supporting individuals with muscular dystrophy and related conditions. They successfully coordinate their health and social care needs, provide support and information to families and ensure a seamless transition from child to adult services.

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".<sup>7</sup>

There are no Regional Care Advisors serving the South East Coast region. As the average workload of a Regional Care Advisor is approximately 1,000 patients, the region requires at least four whole time equivalent staff to support the families and individuals living with muscular dystrophy in South East Coast.

The Muscular Dystrophy Campaign Patient Survey further shows the need for an increase in care advisors with over half of patients reporting that they are not satisfied with the amount and clarity of information available to them. Furthermore only a third of patients are satisfied with the level of emotional support available to their families and to themselves.<sup>8</sup>

The provision of a named care advisor is also stated as an aim for the NHS 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.<sup>9</sup> In addition, the need for a care advisor 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.”<sup>10</sup>

#### Case study:

- *A Mum of a child with Duchenne muscular dystrophy said: “What we have found lacking is someone who coordinates and helps you through all the various services and professionals and makes sure you have access to everyone it is appropriate to meet for health, education and grants etc.”*

#### o **Diagnosis experience:**

Over a third 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. This reflects the lack of Care Advisor support for patients and families.

Many patients reported that their conditions were undiagnosed or misdiagnosed until they visited specialists in London. This can be due to a lack of knowledge among GPs of these rare conditions, suggesting a need for greater education of the early symptoms of neuromuscular conditions.<sup>11</sup> Delayed or incorrect diagnosis can lead to the patient being given dangerously incorrect advice regarding the management of their condition.

#### Case studies:

- *Mrs C from Surrey: “I was wrongly diagnosed and given aggressive treatments at my local hospital. I got myself into a London hospital.”*
- *Mr R from Kent: “Diagnosis took about 4½ years from seeing my GP.”*

#### o **Physiotherapy and hydrotherapy:**

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.<sup>12</sup> 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 managed 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.<sup>13</sup> Specialist physiotherapy can delay the progression of the condition, reduce pain and minimise emergency hospital admissions.

### **Case study: Damage to patient**

*A retired grandfather with a form of muscular dystrophy broke his arm when he had an accident with his wheelchair while on holiday. He was flown back to the UK and taken to his local hospital in Surrey. Without specialist knowledge of his condition the local physiotherapist conducted inappropriate exercises which led to the patient losing mobility in his arm. This could have been avoided if the local physiotherapist had contacted the specialist neuromuscular physiotherapist only 8 miles away at Kings College Hospital. A managed clinical network would have ensured strong links between the tertiary centres and primary and secondary care so that specialist knowledge about these rare conditions is shared between clinicians. (Reported by Clinician - April 2009)*

The provision of specialist physiotherapy in the region is extremely limited. There are specialist physiotherapists present at the tertiary centres in London, but they have very little contact locally with primary and secondary care physiotherapists.

All thirteen NHS Trusts and seven out of eight PCTs in South East Coast responded to a Muscular Dystrophy Campaign Freedom of Information request in April 2008 which showed that:

- 40% of PCTs and over half of the Trusts do not provide ongoing physiotherapy for patients with muscular dystrophy and related conditions where required;
- Less than a third of the PCTs, and only half of the Trusts have physiotherapists available to children or adults with specific training in muscular dystrophy and related neuromuscular conditions;

This correlates with the Muscular Dystrophy Campaign Patient Survey in which half of respondents reported that they had no access to physiotherapy. More children than adults received physiotherapy, often through 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).

Provision of hydrotherapy is even more restricted with over 80% not having access to the service.

#### *Case studies:*

- *A mum of two boys with Duchenne Muscular Dystrophy says: "The main problem we face is lack of physiotherapy. The lady who sees the boys is very good but she just doesn't have time to visit us very regularly."*
- *An FSH patient from West Sussex says: "It would be really useful to have regular physio appointments rather than once or twice a year."*
- *Mr C: "My hydrotherapy services ceased a year ago due to the lack of staff"*

The provision of physiotherapy and hydrotherapy 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 conditions.

#### *Case study:*

- *Mr F from Kent used to receive hydrotherapy and says: "I did find this helpful when I went, but you can only go for six sessions."*

### o Respiratory clinics:

Breathing disorders are recognised as the leading cause of mortality in neuromuscular disease.<sup>14</sup> Respiratory muscle weakness is relatively common in most neuromuscular conditions and is almost inevitable in the late stages of Duchenne muscular dystrophy.<sup>15</sup> However treatment, including ventilation, has been shown to improve both quality and length of life.<sup>16</sup> Furthermore, a 2003 paper showed that it is more cost-effective to manage respiratory issues through check-ups and home ventilation than through unplanned critical hospital admissions.<sup>17</sup>

**An audit of 40 sequential DMD deaths over 10 years in the Southwest 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 DMD receiving home ventilation and specialist multi-disciplinary care reported by the Newcastle group in the most recent study by Eagle *et al* (2007).<sup>18</sup>**

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.<sup>19</sup>

In South East Coast region there is a joint respiratory clinic for paediatric neuromuscular patients held regularly at Chailey Heritage Hospital and run by Dr Seddon and Dr Khan. Dr Seddon currently has five young people with neuromuscular conditions who are being treated with non-invasive ventilation, and a further 15-20 children with muscular disorders who do not yet need this support but need other types of care, and are likely to need NIV in the future.

Respiratory care is also offered in the East Kent area by Dr Bandipalyam Prathibha who provides care to adult neuromuscular patients and manages the non-invasive ventilation for these patients.

### o Cardiac clinics:

As a number of neuromuscular conditions affect the heart, cardiac monitoring should be part of a multi-disciplinary approach to care. People with myotonic dystrophy and Emery-Dreifuss dystrophy are prone to abnormal heart rhythms, while people affected by Duchenne or Becker muscular dystrophy are more likely to experience cardiomyopathy.

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.”<sup>20</sup>

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.<sup>21</sup> Without screening, cardiomyopathy can progress almost entirely without symptoms until signs of heart failure emerge, when all cardiac reserve has been eroded.<sup>22</sup>

Cardiac screening should also be offered to women who are carriers of mutations in the dystrophin gene, as they are at increased risk of cardiomyopathy, even if they experience no symptoms. Currently this only occurs on an ad hoc basis, if at all.

#### o **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.<sup>23</sup>

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°. <sup>24</sup> 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.<sup>25</sup>

As an example, the best practice guidelines for patients with Spinal Muscular Atrophy state that evaluation should take place every 3-6 months, and more frequently in clinically unstable non-sitters. The evaluation should include: inspection of the spine, chest x-rays and radiographic evaluations of scoliosis, swallow studies, pulse oximetry, and polysomnography depending on clinical need.<sup>26</sup>

#### o **Specialist muscle nurses:**

For a fully comprehensive and multi-disciplinary service, leading clinicians at the existing specialist services have identified the need for a specialist neuromuscular nurse to be provided alongside the consultants and physiotherapists. However, due to the lack of specialist neuromuscular services in the region, this is only available to 6% of patients.

#### *Case study:*

- *“I have been referred several times to a specialist muscle nurse but nothing has ever come of it.”*

The duties of a specialist nurse are wide and include managing and coordinating care packages and supporting self-management.<sup>27</sup> The National Service Framework for Long Term Conditions highlights the importance of specialist nurses and recognises the cost savings they can ensure, stating: “Specialist nurses are shown to provide effective and cost effective advice and care for people with progressive conditions ... Specialist advice and treatment can be cost neutral and may reduce admissions and length of stay and improve wellbeing.”<sup>28</sup>

#### o **Psychologists:**

Psychology support has been identified as an important aspect of multi-disciplinary care.<sup>29</sup> Children and adults with neuromuscular conditions would benefit from the input of a clinical psychologist to help families develop management strategies.<sup>30</sup> Specific issues include support at the time of diagnosis, chronic illness, loss of ambulation, transition to adulthood, times of crisis and bereavement.<sup>31</sup>

**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.<sup>32</sup> In addition behavioural changes have been shown to be an adverse side effect of treatment with corticosteroids – which is used to prolong ambulation and preserve muscle strength and respiratory function.<sup>33</sup> 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.**

Half of all respondents to the Muscular Dystrophy Campaign Patient Survey reported that they were not satisfied with the level of emotional support available to their families and to themselves. Consultants at the specialist centres have also reported that a considerable amount of their clinical time can be spent providing psychological support to patients outside of clinic sessions. There is a clear and pressing need to develop clinical and educational psychology input and support for this patient group.

### o 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.<sup>34</sup>

However, despite the significance of this period for younger people with these progressive neuromuscular conditions, there is no dedicated key worker to support their transition to adulthood and there is a lack of facilities for adolescents to support any preparation for transition to adult services.

The difficulties are shown by respondents to the patient survey, a quarter of who describe the transition process as 'poor' or 'very poor' (only 15% rated the process as good or excellent, with the question not being applicable to the rest of respondents).

#### *Case studies:*

- *Mr F from Kent rated the transition as poor, saying: "I was transferred to another clinic but that clinic is hard to access and not as personal."*
- *Mr C had fortnightly physiotherapy sessions to monitor changes in his condition, but these ended when he turned 18. He says: "transition was confusing, and poorly, if at all, coordinated. I am still reliant on children's services 3 months after turning 18."*

### o Rehabilitation and equipment:

Specialist neuromuscular rehabilitation clinics aim to help maintain independence or in adapting to changes which affect social and domestic life and can include a number of services including physiotherapy, access to communication and controls, 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.<sup>35</sup>

In South East Coast the East Kent Neurorehabilitation Unit at Buckland Hospital, Dover, is led by Dr Mohammed Sakel, Director of Neurorehabilitation Services. However, only a small number of patients with muscular dystrophy or a related neuromuscular condition are seen at this clinic. Clinicians have identified the need to expand this service to meet current patient need.

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. For example, a February 2009 Freedom of Information request has shown that children with neuromuscular conditions in Eastern and Coastal Kent wait for up to 9 months for a wheelchair, and an average of 4 months for an electric profiling bed.

## Appendix 1:

### South East Coast PCT populations

Population data taken from PCT websites/annual reports

PCT	Resident population	Prevalence of neuromuscular conditions, including muscular dystrophy (1:1000)	Prevalence of muscular dystrophy (1:2000)	2009-10 PCT Revenue Allocations (£ thousands)
Brighton and Hove City	260,700	261	130	461,469
East Sussex Downs and Weald	326,000	326	163	539,702
Eastern and Coastal Kent	710,000	710	355	1,216,563
Hastings and Rother	170,000	170	85	319,363
Medway	270,000	270	135	412,814
Surrey	1200000	1200	600	1,646,316
West Kent	655,700	656	328	977,459
West Sussex	776,300	776	388	1,232,894
<b>TOTAL</b>	<b>4,368,700</b>	<b>4,369</b>	<b>2,184</b>	<b>6,806,580</b>

Notes:

- Over 1,000 children and adults for every 1 million of the population are affected by muscle wasting neuromuscular conditions in England.
- Neuromuscular conditions include: the muscular dystrophies, the spinal muscular atrophies, the congenital neuropathies, metabolic myopathies, myasthenic syndromes, channelopathies, the myotonias and the neuropathies.

**Appendix 2:****Commissioning specialised services – the Specialised Definition Set:**

Since March 2009 neuromuscular services have been recognised as specialist by the National Specialised Commissioning Group, and have been covered in the revised neurosciences definition as follows:

**4. Specialist Clinic for Neuromuscular Disorders (children and adults)****Specialised services for neuromuscular disorders may include:**

- **Multiprofessional care including joint involvement of: neuromuscular specialist nurses, professions allied to medicine, dieticians, orthotists, speech and language therapists, psychologists, respiratory care services, orthopaedic or spinal surgical specialist services, cardiac specialist services**
- **Transitional care between paediatric and adult clinicians**
- **Joint neurogenetics services.**

### **Appendix 3:**

#### Background to report:

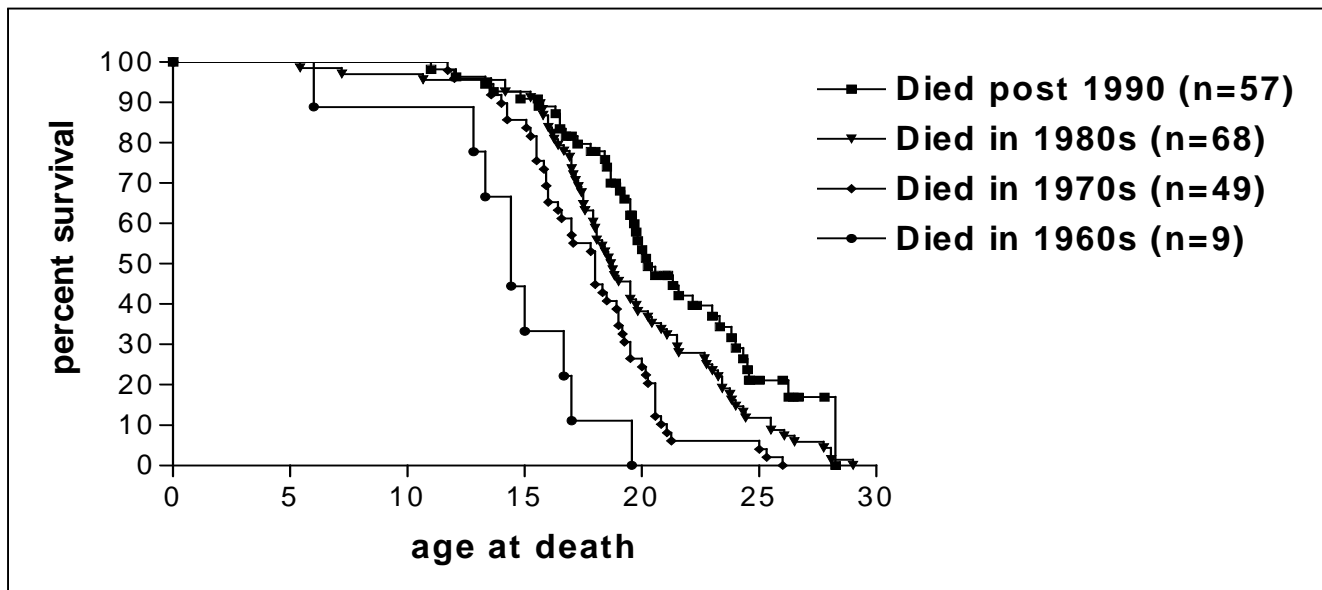
The report contains:

- Evidence from the leading neuromuscular clinicians serving the South East Coast 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 114 families from South East Coast region.
  
- The responses to Freedom of Information requests to all Primary Care Trusts and Acute Trusts regarding specialist services,

#### Appendix 4:

##### 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<sup>36</sup>)*



*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

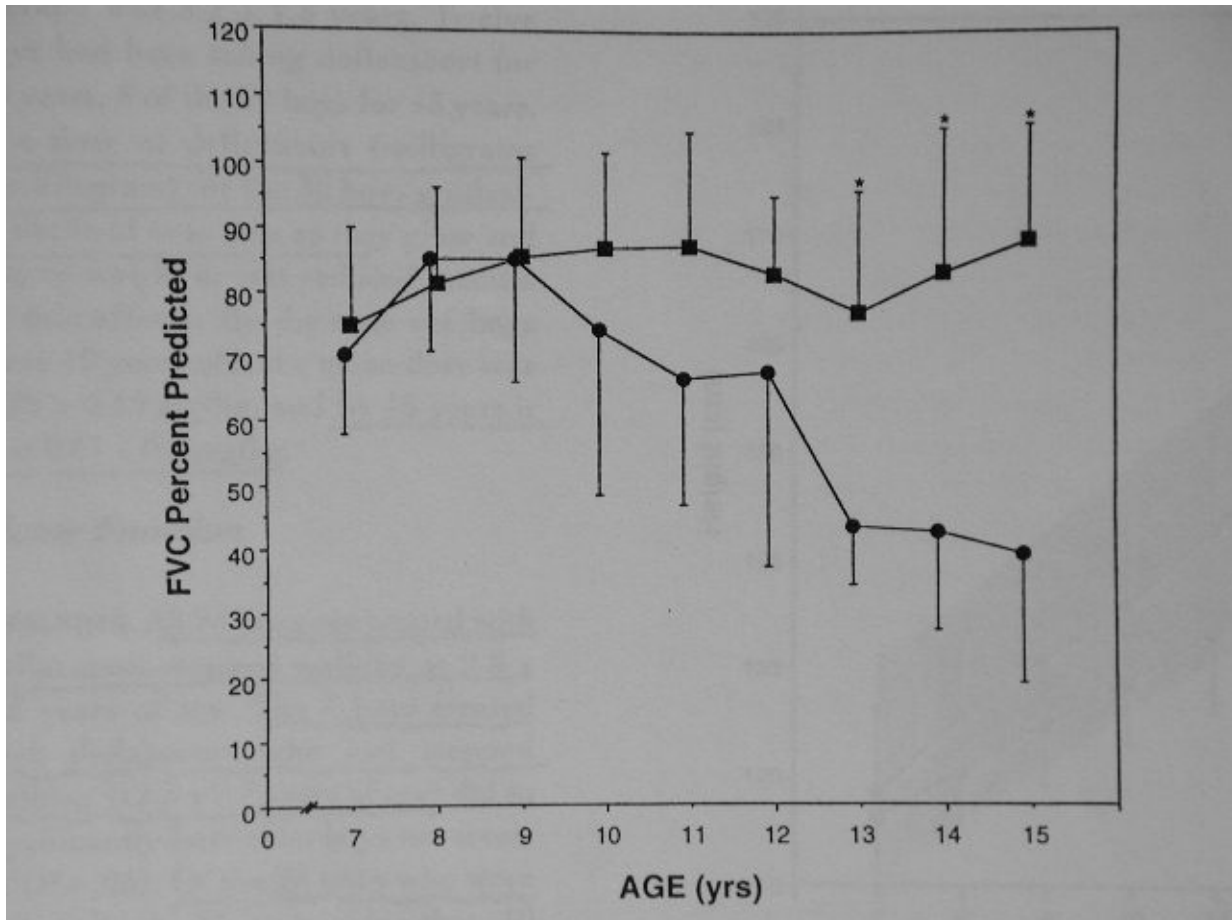
1990s: Mean life expectancy: 19.5 years

*Improvement is due to multi-disciplinary care*

## Appendix 5:

### 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*)<sup>37</sup>



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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- <sup>6</sup> South East Coast Specialised Commissioning Group: Strategic Commissioning Plan 2008-2013 (2007)
- <sup>7</sup> South West Specialised Commissioning Group Revised Neuromuscular Services Development Strategy (2009)
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