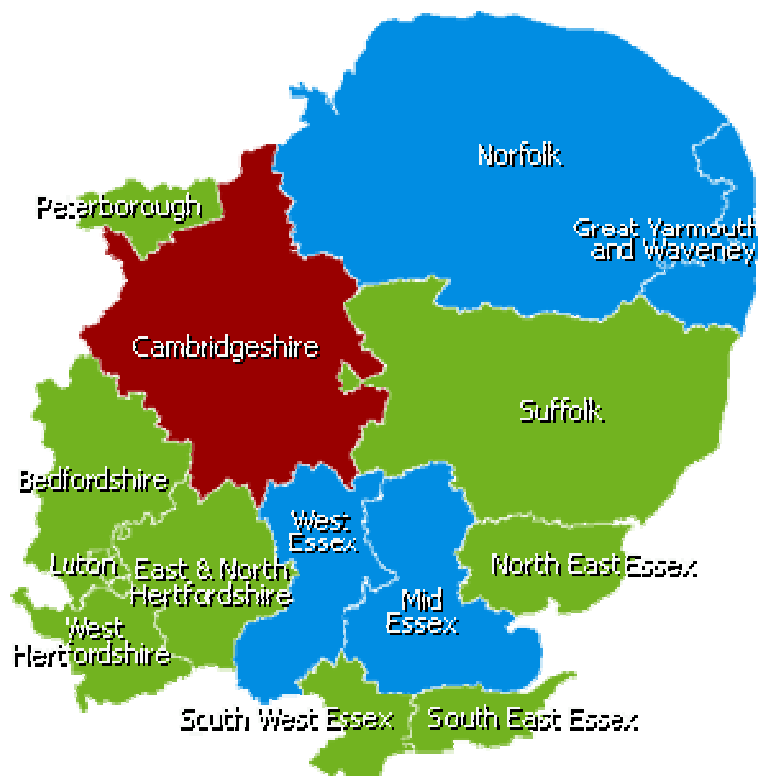


Muscular Dystrophy Campaign



Building on the Foundations: The Need for a Neuromuscular Service serving patients in the East of England Region August 2009



A report by the Muscular Dystrophy Campaign with contributions from and endorsed by the leading neuromuscular clinicians in the East of England region.

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Muscular Dystrophy Campaign



***Building on the Foundations: The Need for a Neuromuscular Service serving patients in the NHS East of England Region
August 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 East of England Strategic Health Authority region to set out a number of serious concerns regarding the provision of specialist clinical services in the East 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 fourteen Primary Care Trusts (PCTs) in the region and would ensure fair access to clinically effective, first class, specialised services across the East of England

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 of England. 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 co-ordination 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 reflects the model set out in the National Definition for specialist neuromuscular services (see appendix 5). It**

has been agreed in the South West and has been successful in Scotland as the Scottish Muscle Network.

- Five 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 estimated 5,500 people in the area living with these conditions. There are currently no RCAs serving the region.
- 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 of England.

Our key findings include:

- **Of the 19 recorded deaths of young men affected by Duchenne muscular dystrophy from 2002- 2007 the mean age of death fell from 22 to 20 years (2002-2005 mean age 22.25 years; 2005-2007 mean age 20 years) (data was retrieved by the East of England Public Health Observatory (PHO)). This compares with a published mean age at death of 30 years in the North East, and a steadily rising age at death in most of the published literature due to improvements in disease management.**
- There is no Regional Care Advisor for the East of England. RCAs play a vital role in the coordination of care, support and advice for families with a neuromuscular condition, many of whom are simply not known to providers of health services. We are calling for this vital post to be created and fully embedded in the NHS from April 2010.
- There is no tertiary care provider offering a devoted paediatric neuromuscular service or clinic. In addition, transition services from paediatric to adult services are particularly poor.
- The only dedicated paediatric muscle clinic held in the region, at the Child Development sent in Bury St Edmunds, Suffolk, was built on the back of no additional NHS funding but is instead reliant on a team who were prepared to re-organise their working practice and relied on charitable donations to buy a respiratory function kit. Therefore, it is vulnerable to the potential withdrawal of funding.
- Access to ongoing and timely specialist care remains uneven with many patients travelling over two hundred miles to receive specialist diagnosis, assessment and treatment in London.
- Patients have very limited access in particular to ongoing physiotherapy.¹ Specialist physiotherapists are required to support outreach clinics and

provide training and professional development for community physiotherapists. Two out of three NHS Trusts in the East of England do not provide ongoing physiotherapy for patients with muscular dystrophy and related conditions.

- Lack of local knowledge of muscle disease resulted in half of all patients in the region stating that their diagnosis process was either poor or very poor. It is very important that local families have access to research centres
- 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. Over half of patients are not satisfied with the level of emotional support available to themselves and their families.
- 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.
- There is an urgent need for greater clinical time. Patients often have to wait too long a between clinic appointment, which is a particular worry for children on high dose long-term steroids and those with impending potential respiratory failure.
- Local speech and language therapy and dietetics services are extremely patchy.
- Due to a lack of local provision clinicians are continually forced to access London centres for specialist orthotics.

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:

There are some 5,500 people in the East of England region affected by a form of muscular dystrophy or a related neuromuscular condition.

The East of England has a population of approximately 5.5 million.

Specialised care in the region is commissioned by the East of England Specialised Commissioning Group. The region's fourteen PCTs are:

- East and North Hertfordshire PCT
- NHS Great Yarmouth and Waveney
- NHS Luton
- NHS Mid Essex
- NHS Bedfordshire
- NHS Cambridgeshire
- NHS Norfolk
- NHS Peterborough
- NHS South East Essex
- NHS Suffolk
- NHS North East Essex
- NHS South West Essex
- NHS West Essex PCT
- West Hertfordshire PCT

SCG Budget: In 2007/08 this was £170 million

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

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 Regional Care Advisor 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.² With specialist care and home ventilation, life expectancy is raised to almost 30 years.³

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.⁴ 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.⁵

o **Specialist muscle clinics:**

- o Only three out of 14 (20%) PCTs in the East of England commission adult muscle clinics.
- o Only one out of 14 PCTs in the East of England commission a paediatric muscle clinic.
- o There is no tertiary provider in the East of England offering a devoted neuromuscular service or clinic for children.

The Muscular Dystrophy Campaign has noted that there is a significant difference in the standard of clinical care available to paediatric and adult patients. Transition is also an area of concern for many families, and this situation requires urgent redress.

Case Study:

One family travel from Suffolk to London, a journey of up to five hours each way, in order to receive specialist healthcare for their seven year old son. The long journey affects the treatment of their child, who is often exhausted and uncooperative by the time he sees the doctors.

Paediatric

The **Child Development Centre at Addenbrooke's Hospital** is the tertiary provider for the following hospitals: Kings Lynn, Peterborough, Luton, Harlow, Welwyn Garden City, Stevenage, Hertford, Bury St Edmunds, Hinchingbrooke, Ipswich, Great Yarmouth and Norwich.

They see a variety of children with various neuromuscular conditions but do not have a devoted neuromuscular clinic or service.

There is one local dedicated neuromuscular clinic held at the **Child Development Centre in Bury St. Edmunds, West Suffolk**.

- The clinic has developed multi-disciplinary expertise in the management of these long-term conditions and has access to shared care and support from the Dubowitz Specialist Centre based at Great Ormond Street Hospital, London. Here, clinic staff and patients can receive specialist advice, support and training.
- The clinic sees 20 patients every 6 months (more frequently if necessary) and its boundaries are the Norfolk border (including Thetford) to the north, Essex border to the south, Cambridge border to the west, and Stowmarket to the east.
- The clinic provides a specialist respiratory neuromuscular clinic once a year which includes sleep studies and nocturnal ventilation via Addenbrookes and Papworth, and attended by the Cambridge respiratory consultant.

- The clinic has been developed at no extra cost to the Primary Care Trust although its **vulnerability is highlighted by its reliance on charitable donations**. For example, basic equipment such as the respiratory function kit was only purchased as a result of charitable funding.
- To ensure the service is continued in the long-term, NHS funding and planning will be necessary.
- Transitional services are not in place for young adults moving from child to adult services.
- The clinic has only limited access to a psychologist.

There is a recognised need to provide further staff in order to improve the outreach service for all patients with an acute need to develop transition services. Further resources are needed to ensure multi-disciplinary care with provision of specialist physiotherapists, speech and language therapists, and specialist muscle nurses.

Adult and young adult

The Coleman Centre for Specialist Rehabilitation runs a multi-disciplinary neuromuscular clinic.

- The current service is only for adults. The service can take patients from the age of 16 years upwards.
- The service sees approximately 22 patients in clinics which run bi-annually over 2-3 days.
- The clinics are multi-disciplinary but vary in how many disciplines attend.
- Patients do not have access to psychology input.

Norwich and Cambridge

- One adult neuromuscular clinic is held each month in Norwich. The clinic is not multi-disciplinary, no specialist physiotherapist, care advisor or specialist nurse attends. This clinic sees approximately 45-50 new patients each year.
- One adult neuromuscular clinic is held each week at Addenbrooke's Hospital. The clinic is not multi-disciplinary, no specialist physiotherapist, care advisor or specialist nurse attends. An additional clinic takes place each month run by a visiting specialist from Norwich. The consultant neurologist sees roughly 160 new patients per year.

o Regional Care Advisors

Regional Care Advisors (RCAs) play an essential role in supporting individuals with muscular dystrophy and related conditions. They successfully co-ordinate patients 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 for funding bodies 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 specialist 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 no RCA.

The service is highly valued in other regions with patients and families describing the RCA service as excellent or good. **An extra five posts (each with a caseload in excess of 1,000 patients) are needed to serve the estimated 5,500 people with muscular dystrophy and related conditions in the region, many of whom are simply invisible to providers of health services.**

Case Study:

A mother from Chelmsford whose daughter has a neuromuscular condition said, "As a parent with a child with a neuromuscular condition I find one of the most frustrating things is that I am, if you like, the 'manager' of the entire care. If I count all the people I have to deal with because of my daughter's condition in one year, it probably reaches 30-40. Why can't children or adults with recognised complex care needs have a coordinator who does all of this?"

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.⁶ 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"⁷.

It is essential that RCAs are appointed in order to serve the whole population, and that the position becomes embedded in the NHS as a matter of urgency.

The results of the Muscular Dystrophy Campaign's Patient Survey carried out in 2008 further highlighted the need for an increase in RCAs, with only half of patients satisfied with the amount and clarity of information available to them.

o **Diagnosis experience:**

Half the 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.⁸

There is a lack of knowledge among local GPs about these rare conditions, suggesting a need for greater education of the early symptoms of neuromuscular conditions.⁹

Clinicians have highlighted that they are unable to get funding for screening – unless there is a family member about to get pregnant – as the cost is £600. This has a knock-on effect on genetic planning and clinical trials.

o **Respiratory clinics:**

Breathing disorders are recognised as the leading cause of mortality in neuromuscular disease.¹⁰ Respiratory muscle weakness is relatively common in most neuromuscular conditions and is almost inevitable in the late stages of Duchenne muscular dystrophy.¹¹ However treatment, including ventilation, has been shown to improve both quality and length of life.¹²

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 muscular dystrophy receiving home ventilation and specialist multi-disciplinary care reported by the Newcastle group in the most recent study by Eagle *et al* (2007).¹³

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.¹⁴

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.¹⁵

o **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 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.”¹⁶ 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 10.¹⁷ Without screening, cardiomyopathy can progress almost entirely without symptoms until signs of heart failure emerge, when all cardiac reserve has been eroded.¹⁸

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.

o **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.¹⁹ 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.²⁰ 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 in order to detail the provision of physiotherapy services. Of the fourteen PCTs in the East of England, the following picture emerged:

Only nine out of 14 PCTs responded. Of these:

- o Two out of three PCTs do not provide ongoing physiotherapy for patients with muscular dystrophy and related conditions where required;
- o Half of PCTs do not have physiotherapists available to children with specific training in muscular dystrophy and related neuromuscular conditions;
- o Eight out of nine PCTs who responded do not have physiotherapists available to adults with specific training in muscular dystrophy and related neuromuscular conditions.

13 out of 18 NHS Trusts in the East region responded and the following picture emerged:

- o Two out of three Trusts do not provide ongoing physiotherapy for patients with muscular dystrophy and related conditions where required;
- o Two out of three Trusts do not have physiotherapists available to children with specific training in muscular dystrophy and related neuromuscular conditions;
- o One in five Trusts has physiotherapists available to adults with specific training in muscular dystrophy and related neuromuscular conditions.

Case Study:

A mother from Norwich, whose 20 year old son has a neuromuscular condition said: "I am really concerned for his future. Without adequate physiotherapy support and expertise I am worried that his quality of life will be seriously affected."

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 having to be referred back for more treatment.

Case Study: *Physiotherapy services at the Coleman Road Health Centre, Norwich*

This service is delivered by specialist neuro physiotherapists. However, there are a number of service issues:

- The service is currently for Norfolk NHS residents only. Patients from Great Yarmouth & Waveney and Suffolk cannot access this service.
- The service has deteriorated following the removal of the Family Care Adviser who used to attend all clinics and provide invaluable advice and support to families.
- There is no adult access to chest physiotherapy in the community. This is often a source of concern to young adults and their parents when making the transition from the Paediatric services. Respiratory physiotherapy is a specialist skill which requires specific training beyond generic skills to be effective.
- The care pathway linking with colleagues in palliative care is not as yet well defined for neuromuscular patients, but discussions have taken place regarding end of life medical/social/psychological and spiritual needs.

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.²¹

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°. ²² 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. ²³

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. ²⁴

○ **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 KAFOs 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. ²⁵

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's September 2008 Patient Survey revealed that a significant 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.

Access to lifestyle appropriate wheelchairs and special seating and 24 hour postural control equipment for this population is an enduring issue, as the most appropriate wheelchairs are usually beyond the range of Wheelchair services, so reliance on the charitable sector is usual and the other equipment is often not available through the NHS.

Only six PCTs (out of 14) responded to a Muscular Dystrophy Campaign Freedom of Information Request on their wheelchair services. It was discovered that:

- The average waiting time for a powered wheelchair, between initial referral to wheelchair services and delivery of the chair, was **three months**. With a regional variation of between 4-6 weeks (Peterborough) and 6-12 months (South West Essex).
- Four PCTs (66%) did not contribute the full amount of funding, according to clinical need, towards these chairs.

Over half of patients fund their wheelchair out of their own pocket or thanks to a charity.

○ **Psychologists:**

Psychological support has been identified as an important aspect of multi-disciplinary care, and as a key part of rehabilitation services.²⁶ 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.

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.²⁷ 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.²⁸ 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.

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.²⁹

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

The difficulties are shown by respondents to the Muscular Dystrophy Campaign Patient Survey, with half of 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:**Demographics****East of England**

<u>PCT</u>	<u>Resident population</u>	<u>Prevalence of neuromuscular conditions</u>	<u>Prevalence of muscular dystrophy</u>
Bedfordshire	382,100	382	191
Luton	185,900	186	93
Cambridgeshire	554,700	555	278
Peterborough	157,400	157	79
South East Essex	325,800	126	63
South West Essex	377,700	378	189
Mid Essex	349,400	349	175
North East Essex	294,800	295	148
West Essex	268,800	268	134
East & North Hertfordshire	510,800	511	256
West Hertfordshire	524,800	525	263
Norfolk	707,700	708	354
Great Yarmouth & Waveney	203,400	203	102
Suffolk	557,300	557	279
EAST OF ENGLAND	5,400,500	5,500	2,750

Data correct according to Office of National Statistics, 2006.

Appendix 2:

Background to report:

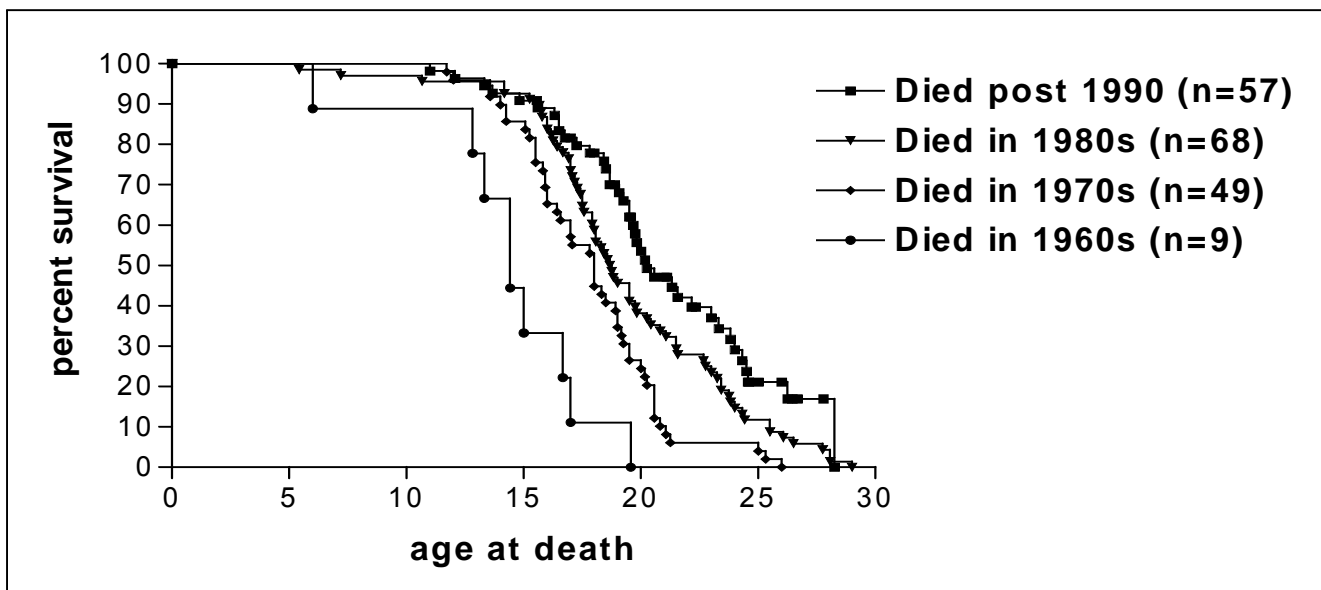
The report contains:

- Evidence from the leading neuromuscular clinicians working in the region.
- 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. Eight hundred and fifty people completed the survey from across the UK.
- 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*³⁰)



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

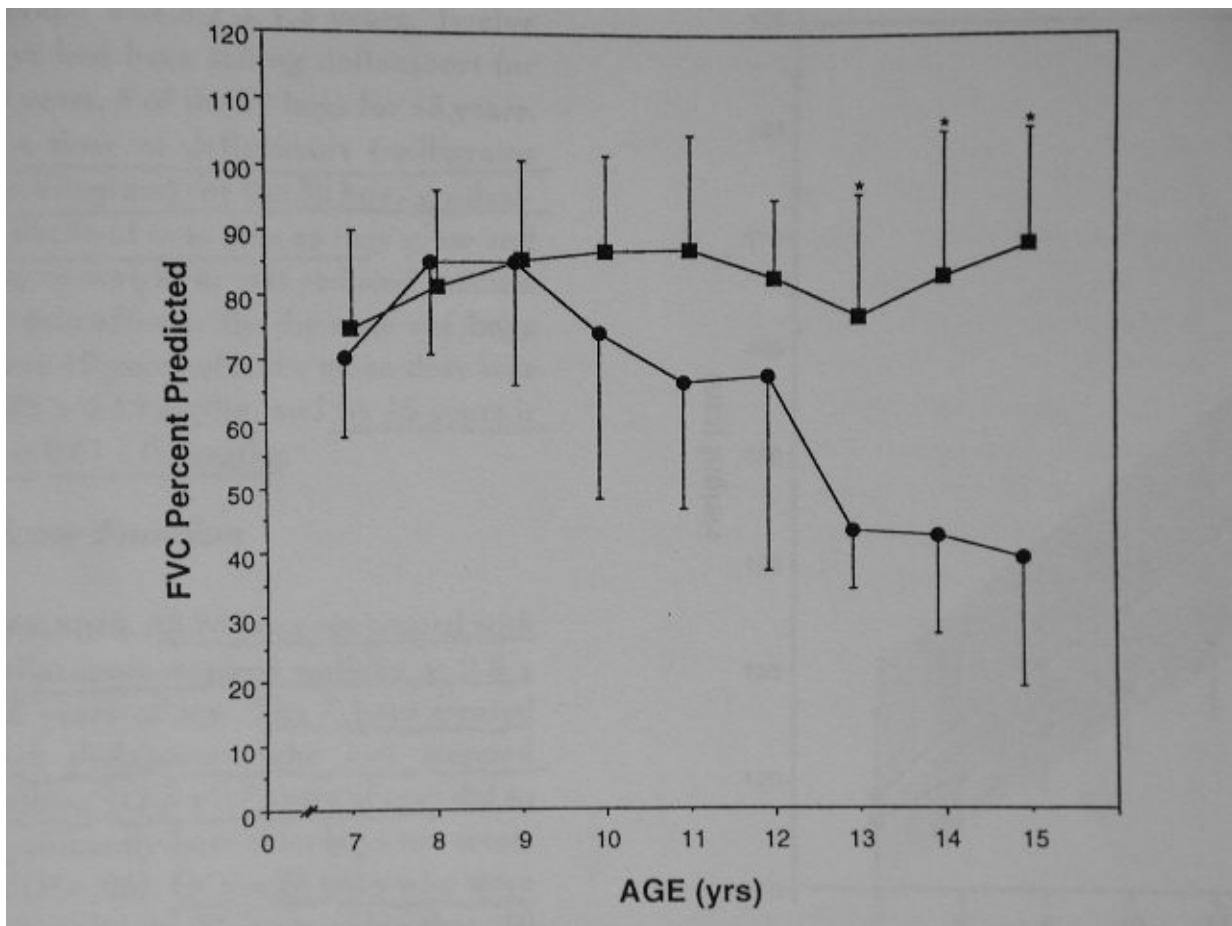
1990s: Mean life expectancy: 19.5 years

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*)³¹



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%)

Appendix 5:

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 **Specialised Services National Definition Set: 8 Specialised neurosciences services (adult)** as follows:

4. Specialist Clinic for Neuromuscular Disorders (children and adults)

Specialised services for neuromuscular disorders may include:

- Multi-professional 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.³²**

However, specialised neuromuscular services should have their own place in the National Definition Set in order to reflect the urgent need to treat these services, for both paediatric and adult, as a priority following years of under-investment and weak co-ordination, and not simply placed within the neurosciences definition.

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