

**Muscular**  
**Dystrophy**  
Campaign



## **Building on the Foundations: The Need for a Specialist Neuromuscular Service for all Patients in the NHS London Region**

October 2009



**A report by the Muscular Dystrophy Campaign with contributions from and endorsed by the leading neuromuscular clinicians in the London region:**

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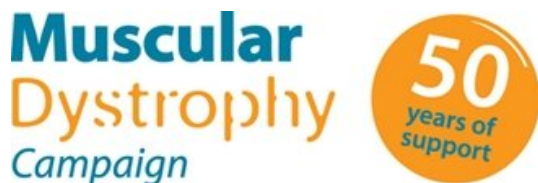
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## **Building on the Foundations: The Need for a Specialist Neuromuscular Service for all Patients in the NHS London Region October 2009**

### **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 London Strategic Health Authority (SHA). It sets out a number of serious concerns regarding the provision of specialist clinical services in London for patients with muscular dystrophy and related neuromuscular conditions.

We are calling for a major change in the way that services are commissioned in the NHS London Region. This is to be in line with the Department of Health's guidance that services for patients within this group of rare conditions should be regarded as specialised and therefore subject to collaborative commissioning arrangements.

The Specialised Commissioning of these services is the most effective way of delivering comprehensive multi-disciplinary health care for neuromuscular conditions. This arrangement would provide best value for money and long-term savings for Primary Care Trusts (PCTs) and would ensure fair access to clinically effective, first class, specialised services right across the London region.

**We believe that a National Department of Health approved Standard of Diagnosis and Care for neuromuscular conditions should be developed and agreed, which includes the gold standard requirements for muscle pathology services.**

### **Urgent 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 London, using this report as a starting point. This review would involve clinicians, patients, PCTs and Specialised Commissioning Groups (SCGs), and would bring forward proposals by February 2010 to secure and develop comprehensive, multi-disciplinary services for children and adults, including transitional services for young people.**
- **A neuromuscular network should be established on the model of a managed clinical network, and supported by a network coordinator. This reflects the model set out in the National Definition for Specialist Neuromuscular Services. This would ensure coordination between the**

existing specialist centres. In addition, it would guarantee that expertise is shared with all clinicians and Allied Health Professionals (AHPs) as there is overall a poor understanding of neuromuscular conditions amongst AHPs. The network would function as a point of contact to provide guidance and pass on information in an easy to manage and cost effective way.

- Six 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 7,500 people in the area living with these conditions. The existing position of RCA based at Great Ormond Street Hospital should be embedded in, and funded by, the NHS.
- There is a high need for further neuromuscular nurse specialists to be appointed particularly at Bart's and the London, Kings College Hospital and Queen Square, where the service is most noticeably lacking. These positions provide clinical support for late onset adult conditions, and are crucial for improving quality of life for many patients.
- Resources should be allocated to ensure that vital respiratory and cardiac support is provided for all neuromuscular patients who require it. These services are key factors in survival and extending life. 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 all PCT areas across London.
- Ongoing physiotherapy should be provided to all adults and children with a neuromuscular condition, developed in each PCT area by enhanced specialist neuromuscular physiotherapy support from the specialist clinics. The existing specialist research physiotherapist role should be funded by, and embedded in, the NHS as it is currently vulnerable and dependent upon charitable funding.
- The short life working group should also focus on the emerging crisis identified in North East London where multiple deprivations and other factors are leading to an increased rate of autosomal recessive inheritance, including spinal muscular atrophy and non-Duchenne muscular dystrophies, in an area with relatively poor NHS services.

**Key findings include:**

- Specialist neuromuscular services are uncoordinated, but also vulnerable due to their reliance on charitable funding.
  - Specialist clinics in London do not have the capacity to serve the 7,500 people living with muscle disease in the region as well as the many migratory patients who travel from other regions, and indeed other countries, to receive specialist support.
  - A number of cases of misdiagnosis of myasthenia gravis were reported, usually where diagnosis was made by a non-specialist.
  - Almost one in four patients in London currently have no access to a neuromuscular specialist and receive no specialist care.<sup>1</sup>

- **There is only one Regional Care Advisor<sup>2</sup> serving the region – funded by the Muscular Dystrophy Campaign and is in an extremely vulnerable position due to its reliance on funding. Seven full-time RCAs are needed to serve the 7,500 people with a neuromuscular condition in the area.**
- **The demands on the role of the RCA in London are more complex than elsewhere because of London's demography which includes large black and minority ethnic communities whose needs must be addressed effectively.**
- **Greater transitional support from paediatric to adult services is needed given the evidence of services being removed or greatly reduced when younger people leave paediatric services. Patients require a wide range of different services and their support requirements change rapidly, not all centres are adequately prepared to cope with transitional services. This means that patients frequently receive inadequate support at the transitional stages and, indeed, throughout their adult lives.**
- **There are major gaps in the diagnosis procedure: diagnosis of complex conditions such as Muscular Dystrophy require not only a careful initial diagnosis but continual reassessment throughout life not least to ensure prolonged life expectancy. Within London assessments and diagnosis need to be better communicated in order not only to improve local access to palliative and respiratory care, but also to ensure a complete picture of the needs of the patient.**
- **Current examples of best practice in providing specialist multi-disciplinary services are often driven by determined individuals who build services around their research interests. However, without succession planning and a clear service strategy, these services have been withdrawn when the lead individual is no longer in post.**
- **It is already proven in some areas that the cost effectiveness of specialist staff in reducing in-patient days and ensuring better clinical management is vast. Preventative investment in a neuromuscular network, to share expertise and make better use of consultant time will lead to improved services and consequential cost benefits.**
- **Similarly, although London enjoys the benefit of specialist tertiary referral centres there are major problems linking these centres with the localities. In both paediatric and adult services, there is a high risk of prolonged waiting times for specialist assessment. This could lead to the patients needs not only changing but worsening. It is, therefore, important to address the urgent need to educate more clinicians working in local Trusts and PCTs, provide outreach centres and more access to specialist care.**
- **A widespread deficit in provision of services by AHPs was also identified with very limited patient access to essential services, in particular to ongoing physiotherapy.<sup>3</sup> Specialist physiotherapists are required to support outreach**

clinics and provide training and professional development for community physiotherapists.

- Similarly, there is a limited 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.
- There are insufficient specialist muscle nurses, especially in London, where the requirement for multidisciplinary work is acute.
- A build up of clinical expertise could be followed by a hiatus in a service as it can be quickly undermined due to a lack of succession planning. For example, there is a shortage of paediatric neurophysiologists and muscle pathologists in the London region.

## 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 further reduction in quality of life. Improved genetic counselling is likely to cause a small reduction in the overall incidence of these conditions.

Proactive symptom-based multi-disciplinary team management has led to an increasing number of patients with early onset muscular dystrophy surviving into adulthood. However, the management of older patients presents very specific practical issues and the increased prevalence of adult cases has resulted in a shortfall in adult care provision.

Professor Lord Darzi, then Health Minister, claimed in 2007, that;

**Our ambition should be nothing less than the creation of a world class NHS that prevents ill health, saves lives and improves the quality of people's lives<sup>4</sup>.**

The current care for people with neuromuscular conditions in London fails to meet the ambition set out by Lord Darzi. There is an urgent need for improvement in the co-ordination, structure and scope of rehabilitation services for paediatric and adult patients with muscular dystrophy.

## 2. Demographics:

There are some 7,500 people in the London region affected by a form of muscular dystrophy or a related neuromuscular condition.

Projections from the Greater London Authority suggest that the population of London will increase from 7.6 million in 2006 to 8.2 million in 2016, with a further increase to 8.7 million by 2026. London has the most diverse population in England with over 90 ethnic minority groups making up over 33% of London's population. Over 300 languages are spoken, and 25% of England's poorest districts are in London, with areas of great affluence found in proximity to areas of significant deprivation<sup>5</sup>.

There is one Strategic Health Authority (SHA) for London – NHS London. NHS London manages the overall performance of 31 Primary Care Trusts, 25 Acute Trusts, 13 Foundation Trusts, 9 mental health trusts and the London Ambulance Service.

A number of adult neuromuscular patients from other regional SHAs regularly visit London for specialist care, tending to travel to the specialist clinics at the Centre for Muscle Disease at the National Hospital for Neurology and Neurosurgery at Queen Square, and the Lane Fox Respiratory Unit of Guy's and St Thomas'. Paediatric patients from across the UK often travel to London for specialist care at the Dubowitz Neuromuscular Centre (this clinic was previously located at Hammersmith Hospital).

Specialised care in the region is commissioned by the London Specialist Commissioning Group - a joint committee of London PCTs working in partnership with neighbouring specialised commissioning groups, NHS London, patient and public engagement groups and NHS Trusts. London specialised commissioning is currently divided between five different regional groups, North Central London, North East London, North West London, South East London and South West London. Within each sector there is a lead PCT for specialised commissioning.

**Specialised care in London had a commissioning budget for 2007/08 in excess of £600 million.<sup>6</sup>**

Also key to collaborative specialised commissioning are the Consortia groups of commissioners who have come together to plan and procure a service. Where Consortia are in place, funding arrangements are centralised and addressed through a single lead PCT.

In their strategic plan for 2009-14 the London Specialised Commissioning Group acknowledged that:

**London is a city of marked disparity in health and wealth. As a result, the major indicators of overall health: infant mortality, life expectancy and under 75 death rates are similar to the national average but differ markedly between London PCTs<sup>7</sup>.**

In section 4, this report will focus on this disparity by highlighting the poor standards of care faced by those in the North East London region. There are hidden populations in certain PCTs that commissioners are currently failing to reach. Further, these hidden populations are subject to multiple deprivations that can increase the prevalence of neuro-muscular conditions thus increasing the need for specialist, multidisciplinary care.

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 (see appendix 7).

### **3. Current level of essential, specialist provision in London:**

In their annual report for 2008/9, published in July 2009, Health Care for London acknowledged the necessity of specialist services, outlining the ambition that -

**A greater proportion of future spending should go to help people with long-term conditions stay as healthy as possible by investing in more GPs, specialist nurses and other health professionals and the services they provide.<sup>8</sup>**

People living with severely disabling and/or life limiting neuromuscular conditions need access to the appropriate interventions and support as their condition progresses. Specialist services should be delivered by a range of professionals from local, regional and national service providers. Neuromuscular Regional Care Advisors are 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.<sup>9</sup> With specialist care and home ventilation life expectancy is raised to almost 30 years.<sup>10</sup>**

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>11</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 patients' lives and increase their life spans.

Boys with Duchenne muscular dystrophy who are still ambulant should be offered the opportunity to discuss treatment using steroids such as deflazacort or prednisolone, 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, as deterioration can advance rapidly over the course of months.<sup>12</sup>

#### **Specialist muscle centres and clinics:**

London clinics receive a relatively large number of patients from outside the region, particularly from the Home Counties. For example, 26% of patients who responded to the Muscular Dystrophy Campaign patient survey in the South Central region travelled to London in order to access specialist care - primarily by children travelling to GOSH. This information is reinforced by the evidence provided by the Regional Care Advisor at GOSH, who sees 54 families from the South Central region. Patients also travel to London from a number of other counties, including Nottinghamshire, Yorkshire and Lancashire.

However, although the London region benefits from specialist muscle clinics the Muscular Dystrophy Campaign Patient Survey found that **one in four** patients in the region have no access to a specialist neuromuscular consultant. In addition, almost **four out of five** respondents have no access to a key worker or care co-ordinator. This is of particular concern for the many elderly or severely disabled patients who have difficulty travelling to clinics.

Patients currently face an unacceptable regional variation in the provision of specialist neuromuscular care, caused by the inadequate provision of specialist muscle clinics.

We are very concerned that many patients within London are not accessing specialist facilities on their doorstep, facilities that others travel hundreds of miles to use.

## North East London – an Emerging Crisis

### o North East London demographics:

North East London had a population in 2006/7 of an estimated 1.59 million people, broken down by PCT areas as follows:

Newham	260,124
Redbridge	251,430
Havering	235,627
City & Hackney	227,500
Waltham Forest	224,271
Tower Hamlets	223,300
Barking & Dagenham	170,445

The region's population is projected to grow to 1.87 million by 2020/21.

It is estimated that 1 in 1,000 people are affected by muscular dystrophy or a related neuromuscular condition. There are currently an estimated 1,500 people in the North East London area living with muscle disease.

In 5 out of 7 NE London PCTs over a third of children live in poverty.

### **Current provision of specialist muscle clinics:**

North East London suffers from a lack of specialist muscle care, with only 1 specialist muscle clinic and a lack of local clinical and therapeutic care provided by the PCTs.

Information obtained following a Freedom of Information request by the Muscular Dystrophy Campaign in early 2008:

PCT	Muscle Clinics
Newham	0 (referred out of area)
Redbridge	0 (referred GOSH)
Havering	1 (Queens Hospital)
City & Hackney	0 (referred out of area)
Waltham Forest	0 (referred out of area)
Tower Hamlets	No answer
Barking & Dag	0 (referred out of area)

Since the end of 2008 patients in the North East have also been able to attend clinic at Bart's and the Royal London Hospital.

Poor local primary care can undermine the work of the London centres of excellence, resulting in a break down in treatment and communication.

### **Commissioning of specialised services:**

As for other London regions, specialised care in the North East is commissioned by the London Specialised Commissioning Group (SCG) - a joint committee of London PCTs working in partnership with neighbouring specialised commissioning groups, NHS London, patient and public engagement groups and NHS Trusts. Specialised care in London has a commissioning budget for 2008/09 in excess of £600 million.<sup>13</sup>

The London SCG in their strategic vision document claims:

**We will build on our experience commissioning HIV and haemophilia based on novel commissioning models and models of clinical care; the focus on clinical and service outcomes; systematic monitoring of quality standards and clinical audit to extend a similar approach to all areas of our commissioning portfolio.<sup>14</sup>**

However, the London SCG is currently failing to commission for those with rare neuromuscular conditions in North East London.

### **Tower Hamlets: Case Study of an Emerging Crisis?**

Tower Hamlets PCT is a clear case of the problems suffered by hidden populations within the London region. With a population of 223,300, Tower Hamlets is ranked the highest of all UK local authorities for its rate of child poverty, with 50.9% of children living in poverty.<sup>15</sup>

#### **Demographics for Tower Hamlets PCT:<sup>16</sup>**

- Half of the population of Tower Hamlets are non-White British ethnic groups, 37% from the Asian ethnic group and 7% from the Black ethnic group.
- Over 30% of the population of Tower Hamlets are Bangladeshi of whom half are under 20 years old.
- Young Bangladeshi men living in London experience higher unemployment than most other comparable demographic groups.
- 40 per cent of children from Bangladeshi families live in workless households.
- All but one of 17 electoral wards in Tower Hamlets is among the most deprived in England.
- It has the youngest modal age of child bearing amongst London boroughs, of 24.2 years.
- Tower Hamlets has a very high birth rate compared to the national average.
- Population in Tower Hamlets PCT is projected to grow from 223,300 to 236,610 by 2016.
- It has fewer pensioners than most of London.<sup>17</sup>

The recently established neuromuscular clinic at Bart's and the Royal London Hospital has seen a steady growth in patient referrals. The majority come from North East London PCTs, of these 25% come from Tower Hamlets PCT.

Tower Hamlets is one of 18 national Health Reform Demonstration Sites (HRDS) and one of only two in London. The HRDS Programme is an NHS initiative to observe the implementation of health reform policies. In Tower Hamlets the programme is called 'Making the Breakthrough' and focuses on the largest contributors to the life expectancy gap in Tower Hamlets – stroke, heart disease, diabetes, cancer and what is termed the vulnerable elderly population.<sup>18</sup>

**While we applaud these efforts, the impact of multiple deprivations on those living with rare and very rare neuromuscular conditions needs to be addressed with equal urgency.**

#### Why:

- Studies have shown that a higher than expected proportion of families of patients with Duchenne muscular dystrophy seem to be from a deprived background. Duchenne muscular dystrophy is an X linked disorder affecting approximately 1 in 3500 male live births. The incidence remains steady in most populations, maintained by a high rate of new mutations in the dystrophin gene.<sup>19</sup>
- Evidence has recently emerged from clinical audits carried out in the Yorkshire and Humber region, which has shown that there is an alarmingly high frequency of cases of inherited muscle disease amongst ethnic minorities in deprived communities.<sup>20</sup>
- A growing number of ongoing studies have found that marriage patterns, including parental consanguinity and endogamy, can also lead to increased cases of autosomal recessive inheritance, including spinal muscular atrophy and non Duchenne muscular dystrophies.<sup>21</sup>
- A study by the Wellcome Trust of genetic risk counselling among British families of Pakistani origin, estimated that the average national risk of autosomal recessive disorders is about two in every 100 births and that the risk is doubled in first cousin marriages.<sup>22</sup>
- The national study of Progressive Intellectual and Neurological Deterioration (PIND) provides further epidemiological data on the distribution of neurodegenerative diseases in the UK child population. 1,400 children were reported in the first five and a half years of surveillance. In the five districts with the largest numbers of resident cases the majority not only came from a particular ethnic group but also had high reported rates of consanguinity.<sup>23</sup>
- Due to marriage patterns in Bangladeshi society, the community has a high rate of consanguinity and endogamy compared to the national average.
- Where there has been a longstanding tradition of consanguineous marriage across generations, the resultant level of cumulative homozygosity would predictably be

quite high, and is the reason for the high prevalence of recessive disease including neuromuscular conditions, within this population.

- Bangladeshi parents of disabled children have poor interpreting support and limited access to translated materials which makes access to appropriate information difficult.
- A national survey by the Joseph Rowntree Foundation discovered that only one in four Bangladeshi families living with a disability said they had seen a social worker over the last year.<sup>24</sup>

**Therefore, as much of the Bangladeshi population is materially deprived, young and growing, it is reasonable to anticipate that an already high instance of muscular dystrophy will increase over the next few years to reach a crisis point. Action is required now by NHS commissioners.**

### **Implications – a growing problem**

Poverty and disability can reinforce each other, contributing to increased exclusion and vulnerability. If people are denied access to the basic medical care that they need for everyday functioning they can lose self-respect, empowerment and a sense of belonging to a community.<sup>25</sup> In the context of Tower Hamlets, this serves to increase the feeling of marginalisation amongst minority groups, limiting their opportunities, sense of well being, and individual control over quality of life.

It is important to acknowledge that due to specific cultural practice and tradition, certain communities can become hotspots and suffer different problems to the population as a whole. Improved resources – both clinical and genetic – are required to provide essential support that is sensitive to culture, language and religion.

The North East London area is an example of a local hotspot that may not be picked up by nationally based surveys. Marriage patterns can affect specific population subgroups causing significant increases in the prevalence of recessive diseases, including neuromuscular conditions. Further, there is currently not enough local palliative and respite care in place to support communities in North East London living with muscle disease, and with cases likely to increase this situation will only deteriorate further.

To improve the health of communities across North East London PCTs, this situation needs to be resolved now, and the suggestions in this report implemented.

Patient survival and daily functioning is limited by the area in which they live. It is simply unacceptable that many patients are not receiving adequate levels of local care.

**Specialist Centres across the London Region (arranged alphabetically by hospital):**

**Neuromuscular Clinics at  
Bart's and the London Trust (BLT) and Queen's Hospital Romford:**

A dedicated Neuromuscular clinic was established at Queen's Hospital Romford in early 2008 and at Bart's and the London Trust (BLT) in late 2008.

The clinic is led by:

- o Clinic Director and Consultant Neurologist - Dr Aleksandar Radunovic

Neuromuscular clinics and Needle muscle biopsy clinics run weekly. BLT strengths lie in the already established services required for running of the specialist neuromuscular service. Dr Simon Lloyd-Owen, Consultant Respiratory Physician leads BLT Regional Non-Invasive Ventilation (NIV) centre which looks after 350 patients on domiciliary ventilation. Cardiac surveillance of neuromuscular patients is provided by the Heart Muscle Unit run by Dr Peter Mills, Consultant Cardiologist. Orthopaedic service for neuromuscular complications is run by Mr Mark Paterson, Consultant Orthopaedic Surgeon.

The neuromuscular clinics are at an embryonic stage and urgently require further funding in order to deal with demand, fund the attendance of other AHPs, and expand upon available resources. Dr Radunovic has received 112 referrals for his neuromuscular service at BLT. This number has steadily increased and currently averages between 10-12 new referrals per month. The majority of patients come from North East London PCTs (25% from Tower Hamlets PCT). In his Neuromuscular Clinic at Queen's Hospital Romford Dr Radunovic has received 160 referrals in the period from January 2008 to May 2009.

**Neuromuscular Service at,  
Guy's and St. Thomas':**

**Evelina Children's Hospital**

Evelina Children's Hospital (ECH) provides a comprehensive clinical service for children with neuromuscular conditions. Over 1000 neuromuscular patients are seen or contacted by the service every year. Patients across the full range of muscular dystrophies are seen in a multi-disciplinary clinic jointly by a consultant and physiotherapist. The service interfaces with other specialties including physiotherapy, occupational therapy, speech and language therapy, clinical psychology and a specialist nurse.

The neuromuscular service at ECH is the third largest paediatric neuromuscular service in the country and provides a regional tertiary neuromuscular service that covers the South East of England.

The centre is led by:

- Service Director and Consultant Paediatric Neurologist - Dr Elizabeth Wraige
- Senior Lecturer and Consultant Paediatric Neurologist - Dr Heinz Jungbluth
- Neuromuscular Physiotherapists - Jennie Sheehan and Rachael Spahr

Clinics are held either weekly, or every other week:

- General Neuromuscular Clinic - Wednesday all day – weekly (Attended by Dr Elizabeth Wraige and Dr Heinz Jungbluth)
- General Neuromuscular Clinic - Tuesday morning - alternate weeks (Attended by Dr Heinz Jungbluth)
- Physiotherapy Clinic - Tuesday morning - alternate weeks (Attended by Jennie Sheehan and Rachael Spahr)
- Myasthenia Clinic - Thursday afternoon - alternate weeks (Attended by Dr Heinz Jungbluth)

Follow up clinics are held regionally in Medway, Maidstone, Valence and Chailey Heritage.

After clinical assessment appropriate further investigation is agreed, including bloods, imaging, electrophysiology, muscle biopsy and referral to specialist in cardiac, respiratory, orthopaedic and spinal teams.

Many other departments interface with the service: cardiology, respiratory at the Lane Fox Unit (see pages 18 and 19, for more details on respiratory clinics), spinal, orthopaedic, adult neurology (joint clinics), paediatric and neonatal intensive care, cardiothoracic surgeons and nephrology.

There is also a strong research interest in neuromuscular disease at ECH, reflected in a substantial number of publications on neuromuscular topics. Close clinical and research links exist with the Diagnostic Genetics Laboratory at Guy's Hospital which provides the National Diagnostic Service for many neuromuscular conditions.

#### The Lane Fox Respiratory Unit

The Lane Fox Respiratory Unit at Guy's and St Thomas' is the largest respiratory unit in the country and provides a specialist service for chronic respiratory failure predominantly arising from chest wall or neuromuscular disease. It specialises in respiratory support, particularly non-invasive ventilation, and in weaning patients from invasive mechanical ventilation. The unit also supports over 800 people at home with a variety of breathing aids. It has recently established an outreach ventilation weaning service to provide expert advice to intensive care units in the South of England.

The centre is led by:

- Consultant Physician and Director of Lane Fox Unit - Dr Craig Davidson,
- Clinical Research Consultant and Clinical Lead for Transitional Services - Dr Nicholas Hart
- Consultant Physician - Dr Adrian Williams
- Consultant Neurologist and Clinical Lead (Neurology) - Dr Robin Howard

- Specialist Transitional Care Coordinator for Progressive Neuromuscular Disease (Specialist Physiotherapist) - Emily Ballard

However, although respiratory care can result in great benefits to patients there remain real problems with its provision. Local GPs and ventilation services often do not understand the level of specialist care needed and do not know how to best use the ventilation equipment.

Support for local developments would help Health Care for London achieve its goal of improved secondary prevention for long term conditions, as detailed in A Framework for Action.<sup>26</sup>

*Dubowitz Neuromuscular Centre at*  
**Great Ormond Street Hospital (GOSH):**

The Muscular Dystrophy Campaign provides funding for the Dubowitz Neuromuscular Centre (DNC) at GOSH. The DNC is the largest paediatric muscle clinic in the UK, and was formerly located at Hammersmith Hospital.

The DNC provides a comprehensive clinical service for children with neuromuscular conditions. Over 400 children and approximately 50 adults currently attend clinics at GOSH, a figure which is increasing annually. In addition nearly 2,000 patients are contacted by the lone Regional Care Advisor via telephone, letter and e-mail.

Currently 4 or 5 general paediatric muscle clinics are held each month; there is also the same number of disease specific clinics held on a monthly basis.

The centre is led by:

- Clinical Director and Professor of Paediatric Neurology - Prof Francesco Muntoni
- Consultant Paediatric Neurologist - Dr Adnan Manzur
- Consultant Paediatric Neurologist - Dr Stephanie Robb
- Regional Care Advisor - Martin Chainani
- Neuromuscular Nurse Specialist - Ruth Barratt

Each patient receives a comprehensive multi-disciplinary assessment that can last most of the day. Joint appointments are arranged that can include:

- Physiotherapy (2 permanent, 3 rotational staff),
- Medical treatment, patients are seen initially by a junior doctor and then reviewed by a consultant (3 Consultants, 1 Specialist Registrar, 1 Senior House Officer (SHO) rotational, 1 Senior Clinical Research Fellow & 1 Clinical Research Fellow),
- Speech and Language Therapy, Dietetics, Orthotic, Orthopaedic, Cardiac and Respiratory assessments.

In addition, a neuromuscular nurse and a Regional Care Advisor are available for specialist advice, information and support.

The centre holds joint specialist clinics with orthopaedic and spinal surgeons, respiratory and peripheral nerve specialists. Children may also be referred to other specialist services at GOSH such as cardiology, gastroenterology and paediatric surgery.

Transition services are in place for the move from paediatrics to adult care, in association with adult neurology specialists from The National Hospital for Neurology and Neurosurgery [NHNN] (for more information see sections on NHNN and Transitional Services).

The DNC is one of the members of the MRC Neuromuscular Translational Research Centre at University College London (UCL). The DNC is also part of the UCL Institute of Child Health and GOSH NHS Trust.

The clinics at DNC are vulnerable due to a reliance on charitable funding by the Muscular Dystrophy Campaign. The posts of Clinical Research Fellow for Paediatrics, Research Physiotherapist, and Clinical Trial Coordinator are funded entirely by the charity. To ensure that the services continue and are developed in the long-term, these roles should be embedded in and funded by the NHS.

*Neuromuscular Clinic at*  
**King's College Hospital (KCH):**

The Muscle Service at King's College Hospital (KCH) serves a large geographical region covering South-East London and Kent. KCH is a major London teaching hospital with the full range of general and specialist services and is part of the Academic Health Sciences Centre called King's Health Partners. This has strengthened links with Guy's and St Thomas' Hospitals and with basic science colleagues at King's College London and the Institute of Psychiatry.

The service comprises weekly clinics seeing adult patients with all muscle conditions including genetic and acquired muscle disease and myasthenia gravis.

The clinic is led by:

- o Consultant Neurologist and Lead Clinician - Dr Fiona Norwood
- o Consultant Neurologist - Dr Michael Rose
- o Specialist Muscle Clinic Physiotherapist - Joanne Reffin

There is a dedicated Pompe disease clinic run by Dr Fiona Norwood and colleagues from Royal Free Lysosomal Storage Disorders Unit, and a joint congenital myopathy clinic headed by Dr Heinz Jungbluth and Dr Fiona Norwood.

Also a weekly multidisciplinary / investigation clinic is held regionally for those with long-term muscle disease. This is run by Jo Reffin and attended by Michael Rose or Fiona Norwood in rotation. A needle muscle biopsy clinic - weekly – is performed as a regional service by Michael Rose, Fiona Norwood or one of the registrars. Open muscle biopsies are performed by a neurosurgical consultant in the area.

Through the South London NHS Trust Dr Norwood also holds a fortnightly muscle / myasthenia / motor neurone disease clinic.

There are no accessible care advisors available for Kings, or a specialist muscle clinic nurse. The team are reliant on a volunteer to assist them with the multidisciplinary clinic.

*The Centre for Muscle Disease at*  
**The National Hospital for Neurology and Neurosurgery (NHNN),**  
**UCLH, Queen Square:**

The Centre for Muscle Disease is an institute highly regarded internationally by both patients and clinicians. It has links within London and surrounding regions to a network of Directorate General of Health Services that include Watford, Northwick Park, Whittington, Whipps Cross and Homerton hospitals, in addition to providing joint appointments with Guys and St.Thomas', St Mary's, GOSH, Moorfields and the Royal Free.

The centre is led by:

- Centre Director and Professor of Clinical Neurology - Prof. Michael Hanna
- Consultant Neurologist - Dr Matthew Parton
- Professor of Clinical Neurology - Prof. Anthony Schapira

There are currently four muscle clinics per week.

A wide range of muscle diseases are observed and assessed in these clinics. They include generic young-onset and disabling muscular dystrophies. Many patients with acquired muscle disease are also seen at these clinics; including a large group of patients with autoimmune myositis which require intensive immunosuppression regimes and monitoring to achieve effective treatment.

As with the other hospitals and trusts mentioned the need for a Regional Care Advisor to support the clinic is crucial, particularly at the time of initial diagnosis. In addition, to further smooth the diagnosis pathway, greater administrative support is required to ensure that patients receive as speedy a diagnosis as possible. Both proposed posts will lead to savings in consultants' time, time that could be spent treating a greater number of patients.

There is a recognised need to improve the service for patients and to develop disease specific clinics. Further, resources are needed to ensure high quality responsive multi-disciplinary care with provision of specialist physiotherapists, speech and language therapists, and particularly specialist muscle nurses, all of which are essential.

**Regional Care Advisors:**

Regional Care Advisors (RCAs) play an essential role in supporting individuals with muscular dystrophy and related conditions. They successfully coordinate patients' health and social care needs, provide support and information to families and help to ensure a seamless transition from child to adult services.

The **London region has only one RCA**, Martin Chainani, who is primarily based at the DNC. The service is highly valued with 80% of the people who have access to this service describing it as good or excellent. However, four out of five of patients and their families have no access to the service.

*Case study:*

*Mrs K, who has limb girdle muscular dystrophy, said “It is appalling that there is no RCA to support me. In general people with muscular dystrophy have to access non specialist services: physiotherapy, hydrotherapy, community health, social services, GP and dentistry services. The level of knowledge and understanding of muscular dystrophy in these non specialist services is very poor”.*

The need for an 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.”<sup>27</sup>

*Case Study:*

*Mr V, who has FSH, has no access to an RCA and finds it difficult to co-ordinate cross-disciplinary care, said “My thinking can become very trivial when I have to worry about simple everyday tasks. All encompassing, well co-ordinated inclusive care is essential to my whole life experience”.*

Further, the provision of a named Regional Care Advisor/Coordinator is stated as an aim by Health Minister Lord Darzi in his final report High Quality Care for All, published June 2008, which set out how the Government intends to provide a more personalised level of care for people with long-term conditions.<sup>28</sup>

Similarly, Health Care for London identified the provision of services focused on individual needs and choices as the first of their five common principles in their ten year healthcare vision document A Framework for Action, published in July 2007.<sup>29</sup>

**Services focused on individual needs and choices.**

**Provision should, wherever possible, be tailored to the particular needs of each individual. This is especially important for people with ongoing health needs, such as those with long term conditions, and for those at the end of life. To help ensure this, patients should feel in control of their care and be able to make informed choices to suit their personal needs.**

The results of the Muscular Dystrophy Campaign’s Patient Survey show two thirds of patients are not satisfied with the level of emotional support available to their families and to themselves. Furthermore, only a quarter of patients are satisfied with the amount and clarity of information available when making choices about their personal needs.<sup>30</sup>

*Case study:*

*Mr G, who has a neuromuscular condition, said “I am very concerned about the opacity of support networks from the perspective of disabled people and their carers. It is a struggle for even the most proactive individuals to gain a proper appreciation of the system and the help available to them”.*

**A further seven posts (each with a workload of approximately 1,000 patients) are needed to serve the estimated 7,500 people with muscular dystrophy and related conditions in the London region. These positions should be embedded within the NHS as a matter of priority.**

### **Diagnosis experience:**

Incorrect diagnoses can cause huge unnecessary stress on families, and strain already stretched resources. Two fifths 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.<sup>31</sup> This again reflects the lack of a RCA to provide advice for patients and families.

The lack of local specialists can cause delays in starting treatment and incorrect diagnosis, which can then lead to the patient being given dangerously erroneous advice regarding the management of their condition. This situation can arise due to a lack of knowledge among GPs of these rare conditions, suggesting a need for greater education on the early symptoms of neuromuscular conditions.<sup>32</sup>

.We believe that paediatricians who have received training from specialist clinics are better able to offer more accurate diagnosis.

#### *Case study:*

*Mrs C, whose child has muscular dystrophy, said “The paediatrician had worked in the neuromuscular department at the Hammersmith Hospital as part of her rotation and so was very clued up on what tests to perform and was also able to talk directly to the consultant for us”.*

### **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>33</sup>

Physiotherapy is the physical treatment and management which enables people with neuromuscular conditions to reach their maximum physical capability 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.<sup>34</sup> Specialist physiotherapy can delay the progression of the condition, reduce pain and minimise emergency hospital admissions.

#### *Case study:*

*Mrs A, who has spinal muscular atrophy type 1, said “When I went to special schools, up until the age of 16, I received passive physio on a weekly basis. I believe that physiotherapy is very important, although I do not have access to it since leaving school”.*

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. Key findings for the London region include:

- Many of the PCTs and NHS Trusts in London do not provide ongoing physiotherapy for patients with muscular dystrophy and related conditions where required;

- Half of all Trusts and a third of PCTs in London do not have physiotherapists available to children or adults with specific training in muscular dystrophy and related neuromuscular conditions.

This correlates with the results of the Muscular Dystrophy Campaign Patient Survey which found that three out of four patients reported that they do not receive enough physiotherapy.<sup>35</sup> Currently more children than adults receive physiotherapy, often due to provision at their special school. This provision is then removed when the child leaves school or moves from paediatrics to adult services.

*Case study:*

*Mr L, who has limb girdle muscular dystrophy, said “I have had to fight and fight for physiotherapy and have eventually gone private as the support available in acute services and community services simply does not meet my need for ongoing specialist support”.*

The provision of physiotherapy in short blocks of sessions is problematic for these patients and indicates a clinical focus on conditions in which quantifiable improvement can be measured, rather than the maintenance of chronic and progressive conditions.

The current centres for neuromuscular physiotherapy are not able to provide ongoing physiotherapy in the local community. Advice is given about ongoing treatment to be provided by local physiotherapy services. If the patient requires ongoing physiotherapy management the physiotherapist will liaise with the appropriate community team. However, this system can lead to complications and unacceptable standards of care.

*Case study:*

*A consultant neurologist at King’s College London (KCL) recounted how “A patient who broke his arm was given inappropriate exercises by their fracture clinic, this led to patient disillusionment. The patient did not return, and therefore the physiotherapist thought the patient was satisfied, which was clearly a communication breakdown. The patient lost function in his arm because of the rehabilitation programme and is now unable to recover. However, the problem was avoidable, as a KCL neuromuscular physiotherapist could have explained Facioscapulohumeral muscular dystrophy to the fracture physiotherapist who didn’t know how to handle it”.*

There needs to be an increase in the critical mass of neuromuscular therapists in order to make this network work. Ongoing management is crucial, and AHPs, patients and parents need to be educated on how best to manage the condition on a day to day basis.

Hydrotherapy can also greatly improve quality of life for many people with a neuromuscular condition but many encountered problems in accessing a hydrotherapy pool locally.

**We are calling on London PCTs to conduct a review of access to hydrotherapy pools.**

*Case Study:*

*Mr V, who has FSH muscular dystrophy, said “I have been forced to use a general swimming pool, which affects my personal dignity. Hydrotherapy services exist in the*

community, for example in private gyms, and an innovative network should look outside of the facilities provided by the NHS Trust in order to allow patients to access these services”.

### **Respiratory clinics:**

Breathing disorders are recognised as the leading cause of mortality for those with neuromuscular disease.<sup>36</sup> Respiratory muscle weakness is relatively common in most neuromuscular conditions and is almost inevitable in the late stages of Duchenne muscular dystrophy.<sup>37</sup> Treatment, including ventilation, has been shown to improve both quality and length of life.<sup>38</sup>

*Case study:*

*Mr K, who has Duchenne muscular dystrophy, said “Non-invasive ventilation is amazing; I wish I had started sooner. I now have more energy, can breathe easier, and no longer suffer headaches”.*

**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 age of death of almost 30 years in patients with Duchenne muscular dystrophy receiving home ventilation and specialist multi-disciplinary care reported by the Newcastle group in the most recent study by Eagle *et al* (2007).<sup>39</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 patients can become too accustomed to their chronic illness and therefore rarely raise complaints about respiratory distress spontaneously.<sup>40</sup>

Further, 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.<sup>41</sup>

**Secondary prevention (i.e., preventing an existing condition from deteriorating) is needed as well, with proactive care for people with long-term conditions to prevent emergency admissions.<sup>42</sup>**

### **Cardiac clinics:**

As a number of neuromuscular conditions also affect the heart, cardiac monitoring should 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.”<sup>43</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>44</sup> Without screening, cardiomyopathy can progress almost entirely without symptoms until signs of heart failure emerge, when all cardiac reserve has been eroded.<sup>45</sup>

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. Currently this only occurs on an ad hoc basis, if at all.

In London patients are referred to cardiac specialists through the muscle clinics at GOSH, NHNN, Evelina Children's Hospital, and King's College Hospital. Cardiac surveillance is also provided by the Heart Muscle Unit run by Dr Peter Mills at Bart's and the London Trust.

### **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>46</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>47</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>48</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, depending on clinical need: inspection of the spine, chest x-rays and radiographic evaluations of scoliosis, swallow studies, pulse oximetry and polysomnography.<sup>49</sup>

Orthopaedic care is illustrative of the current shortfalls in adult care, and major differences between adult and paediatric services. Transition services are currently not geared up to deal with the transfer of patients, and procuring custom made equipment for adults is difficult.

Due to the complex requirements of neuromuscular patients, orthopaedic care cannot be adequately reproduced through tertiary services; therefore it is crucial that resources are allocated to ensure that vital respiratory support is provided by centralised clinics, such as that of the DNC, with effective support co-ordinated locally by a Regional Care Advisor in each PCT area.

### **Rehabilitation and equipment:**

Specialist neuromuscular rehabilitation clinics aim to help maintain independence and help patients adapt to changes which affect social and domestic life. They can include a number of services such as 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 both mobility and successful care. To avoid this situation orthotists with specific experience in neuromuscular disorders should be used to measure and supply orthotics.<sup>50</sup>

Striking difficulties often also arise with the provision of practical aids, including appropriate hoists and belts, feeding and toileting aids, and the conversion of accommodation.<sup>51</sup> Access to a Regional Care Advisor to co-ordinate these services has been shown to make a huge difference and, of course, is a cost-effective intervention.

### 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 offered appropriate equipment.<sup>52</sup>

#### *Case study:*

*Mr K, who has Duchenne muscular dystrophy, said “My old wheelchair broke down and it took three years to be provided with the right replacement wheelchair. The wheelchair services wouldn’t help and eventually I had to contact the manufacturer myself, who in turn employed a specialist occupational therapist to assess my condition and needs”.*

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.

The Muscular Dystrophy Campaign Patient Survey found that one quarter of patients pay for their wheelchair out of their own pocket or thanks to a charity, and of those that receive funding three out of four have experienced delays or difficulties in receiving the appropriate chair.<sup>53</sup>

### **Psychologists:**

Psychological support has been identified as an important aspect of multi-disciplinary care, and as a key part of rehabilitation services.<sup>54</sup> There is pressing need to develop clinical and educational psychology input and support for this patient group.

Children with chronic physical health disorders are at a much greater risk of psychosocial maladjustment compared with healthy children, and have lower emotional well being.<sup>55</sup>

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.

#### *Case study:*

*Mrs K, who has limb girdle muscular dystrophy, said “I would really like to be able to access some sort of emotional support to help me in coping with change. Even though I*

*was diagnosed as a child I have never had any kind of counselling or emotional therapy”.*

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, and 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.<sup>56</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>57</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.**

Currently there is a discrepancy in London service provision. For example, although it is thought that the standard of psychological support at Evelina Children’s Hospital of Guy’s and St Thomas’ is good, GOSH does not provide comparable levels of support.

Guidelines and standards need to be embedded in order to make sure that all patients receive essential services.

### 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>58</sup>

Parents of teenage boys with Duchenne muscular dystrophy can exhibit great stress and significant feelings of guilt when discussing their child’s condition. Parents can find discussing the disease to be very difficult, and struggle to communicate death issues. Families have been found to impose boundaries which lead to fear on the part of the person with the condition, which is compounded by badly managed and co-ordinated transition.<sup>59</sup>

However, despite the significance of this period for younger people and families with progressive neuromuscular conditions, the majority do not have access to a transition coordinator or a Regional Care Advisor who can support transition to adulthood.

The difficulties are illustrated by the respondents to the Muscular Dystrophy Campaign Patient Survey; half of whom rated their transition from childhood to adult services as either poor or very poor.<sup>60</sup>

#### *Case study:*

*Mr L, who has limb girdle muscular dystrophy, said “When I was a child all focus was on my parents. I never felt that there was anybody speaking to me, and did not until I was about 25, and able to ask questions for myself”.*

In the London region there are two transitional services available to try to rectify this situation. The services occur at Evelina and are jointly run with King's and at Guy's and St. Thomas' with the appointment of a transition coordinator.

Transitional services in KCH are quarterly clinics are held jointly at Evelina Children's Hospital by Heinz Jungbluth and Fiona Norwood or Elizabeth Wraige and Michael Rose. Strong links with Guy's Hospital are extended through Fiona Norwood and a monthly Neurogenetics clinic. In addition a weekly muscle biopsy review meeting is held by Neuropathologists in rotation. This is lead by Neuropathologist Dr S Al-Sarraj with Dr Michael Rose and Dr Fiona Norwood attending.

In November 2008 Emily Ballard was appointed to the new role of progressive neuromuscular disease and transition co-ordinator based at Lane Fox Respiratory Unit and funded through the Trust's charity. The role is designed to provide early respiratory intervention and assessment of patients and as a point of liaison with the adult respiratory unit to detect acute medical changes as patients come through to adult services.

The new position makes communication much easier between physicians and families, supports patients if they need an admission, supports the local hospitals, and provides further support through early symptom management of chest infections etc. The role is also able to address the social needs of patients with neuromuscular diseases and find information on available services and assist young people with career opportunities, social activities and respite care.

However, this role is currently unique and is limited in its geographical scope to the South East region. Other key services still suffer from a piece meal approach, and **additional transition co-ordinator positions need to be established and entrenched** to meet the needs of these vulnerable young people.

## **Appendix 1:**

### **Source of Evidence**

The report contains:

- Evidence from the leading neuromuscular clinicians working in the South Central 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 25 families from London.
  
- The responses to Freedom of Information requests to all Primary Care Trusts and Acute Trusts regarding specialist services.

## Appendix 2:

### London Muscle Clinics and staff:

#### Bart's and the London Trust

##### *Bart's and the London*

- **Dr Simon Lloyd-Owen** Lead Respiratory Physician
- **Dr Peter Mills**, Consultant Interventional Cardiologist
- **Dr Saidi (Sam) Mohiddin**, Specialist Registrar in Cardiology
- **Mr Mark Paterson**, Consultant Orthopaedic Surgeon
- **Dr Aleksandar Radunovic**, Consultant Neurologist

#### Queen's Hospital Romford

- **Dr Aleksandar Radunovic**, Consultant Neurologist
- **Prina Patel**, Senior Neurophysiotherapist

#### Charing Cross Hospital

- **Dr Wojtek Rakowicz**, Consultant Neurologist

#### Dubowitz Neuromuscular Centre: Great Ormond Street Hospital

- **Professor Francesco Muntoni**, Director and Consultant Paediatric Neurologist
- **Dr Adnan Manzur**, Consultant Paediatric Neurologist,
- **Dr Stephanie Robb**, Consultant Paediatric Neurologist
- **Dr Colin Wallis**, Consultant Respiratory Paediatrician
- **Martin Chainani**, Regional Care Advisor

#### Guy's and St Thomas' Hospital

##### *Evelina Children's Hospital*

- **Dr Heinz Jungbluth**, Consultant Paediatric Neurologist
- **Dr Elizabeth Wraige**, Consultant Paediatric Neurologist
- **Jennie Sheehan** Neuromuscular Physiotherapist

##### *Lane Fox Respiratory Unit*

- **Dr Craig Davidson**, Consultant in Respiratory Medicine
- **Dr Nick Hart**, Director of Research and Consultant in Respiratory Medicine
- **Emily Ballard**, Progressive Neuromuscular Disease Transition Coordinator

#### King's College Hospital

- **Dr Fiona Norwood**, Lead Clinician and Consultant Neurologist
- **Dr Michael Rose**, Consultant Neurologist
- **Joanne Reffin** Specialist Muscle Clinic Physiotherapist

#### National Hospital for Neurology and Neurosurgery: University College London Hospital

- **Professor Michael G Hanna**, Consultant Neurologist
- **Dr Nicholas Hirsch**, Consultant Neurologist
- **Dr Robin Howard**, Consultant Neurologist
- **Professor Dmitri Kullmann**, Consultant Neurologist
- **Dr Richard Orrell**, Consultant Neurologist
- **Dr Matthew Parton**, Consultant Neurologist
- **Dr Shamima Rahman**, Consultant Neurologist
- **Dr Mary Reilly**, Consultant Neurologist
- **Prof. Anthony Schapira**, Consultant Neurologist

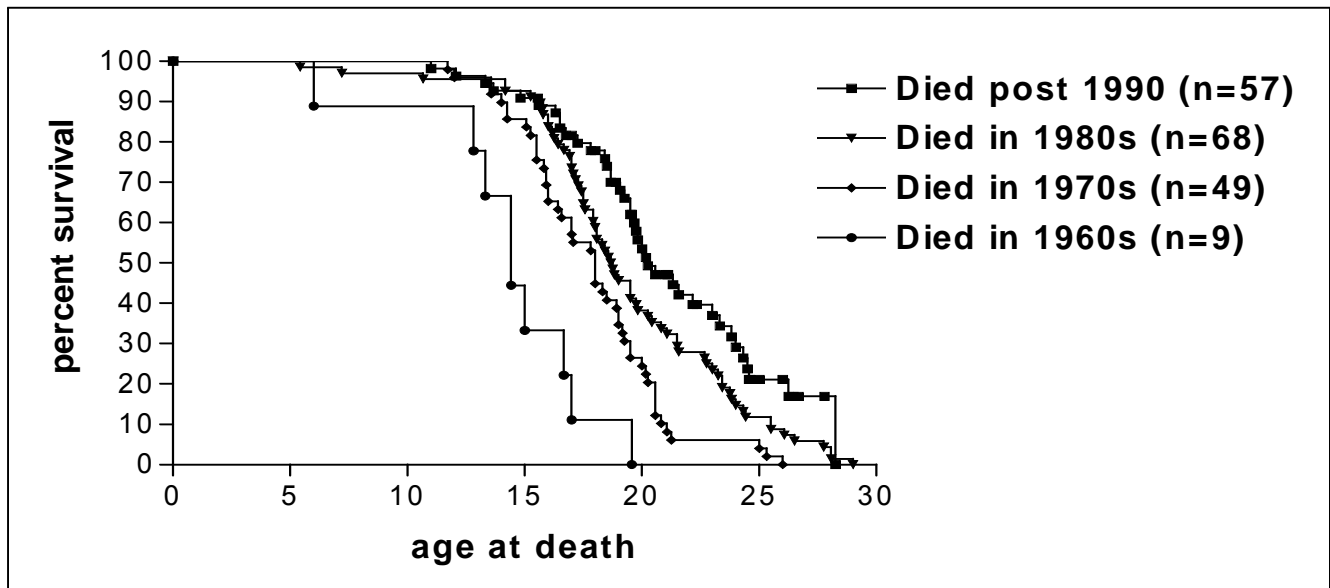
Royal Brompton Hospital

- **Michelle Chapman** – Neuromuscular Physiotherapist, Royal Brompton Hospital
- **Dr Anita Simonds**, Consultant in Respiratory Medicine

### 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*<sup>61</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:

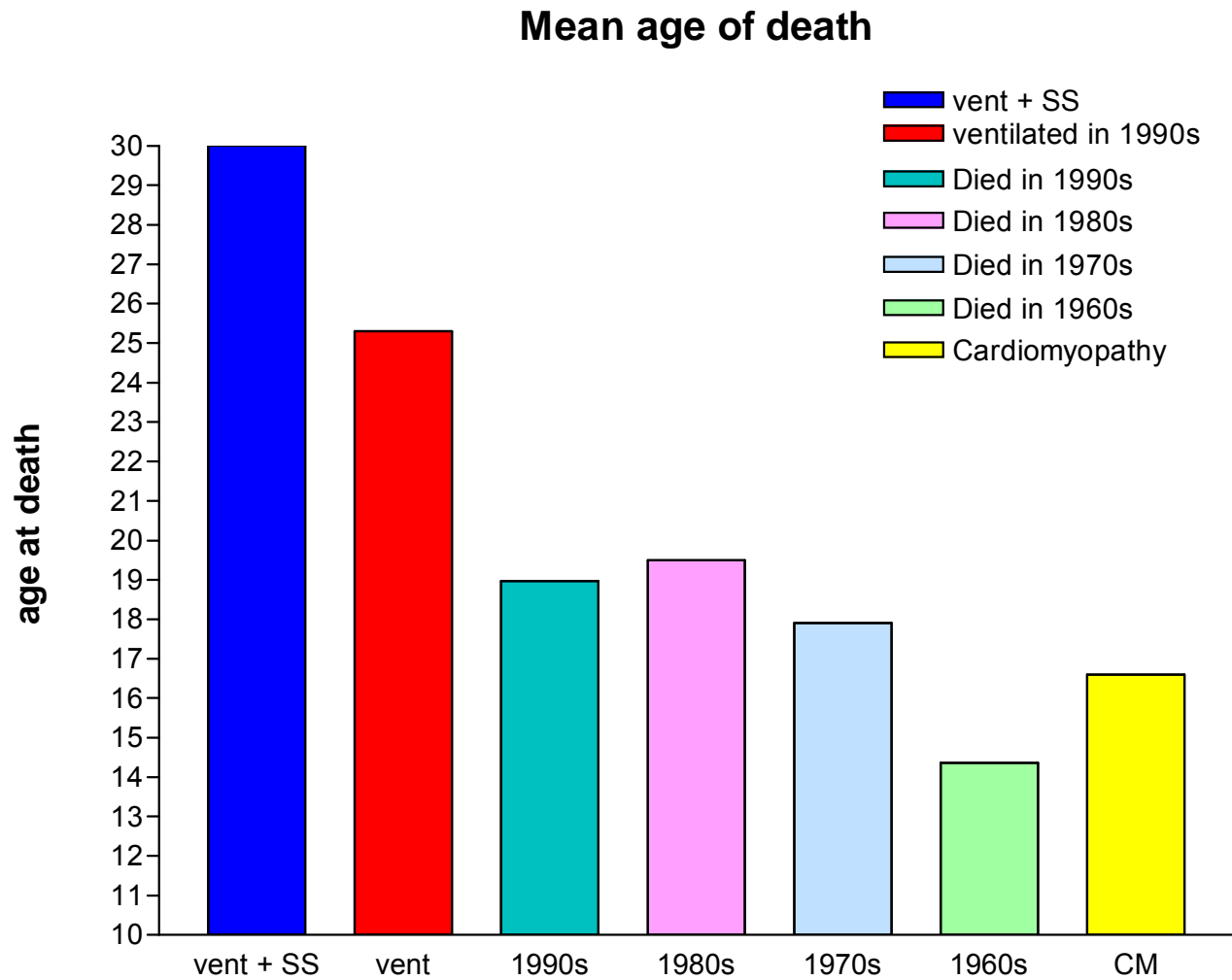
1960s: Mean life expectancy: 14.4 years - No survivors beyond 19.29 years

1990s: Mean life expectancy: 19.5 years

*Improvement is due to multi-disciplinary care*

**Appendix 4:**

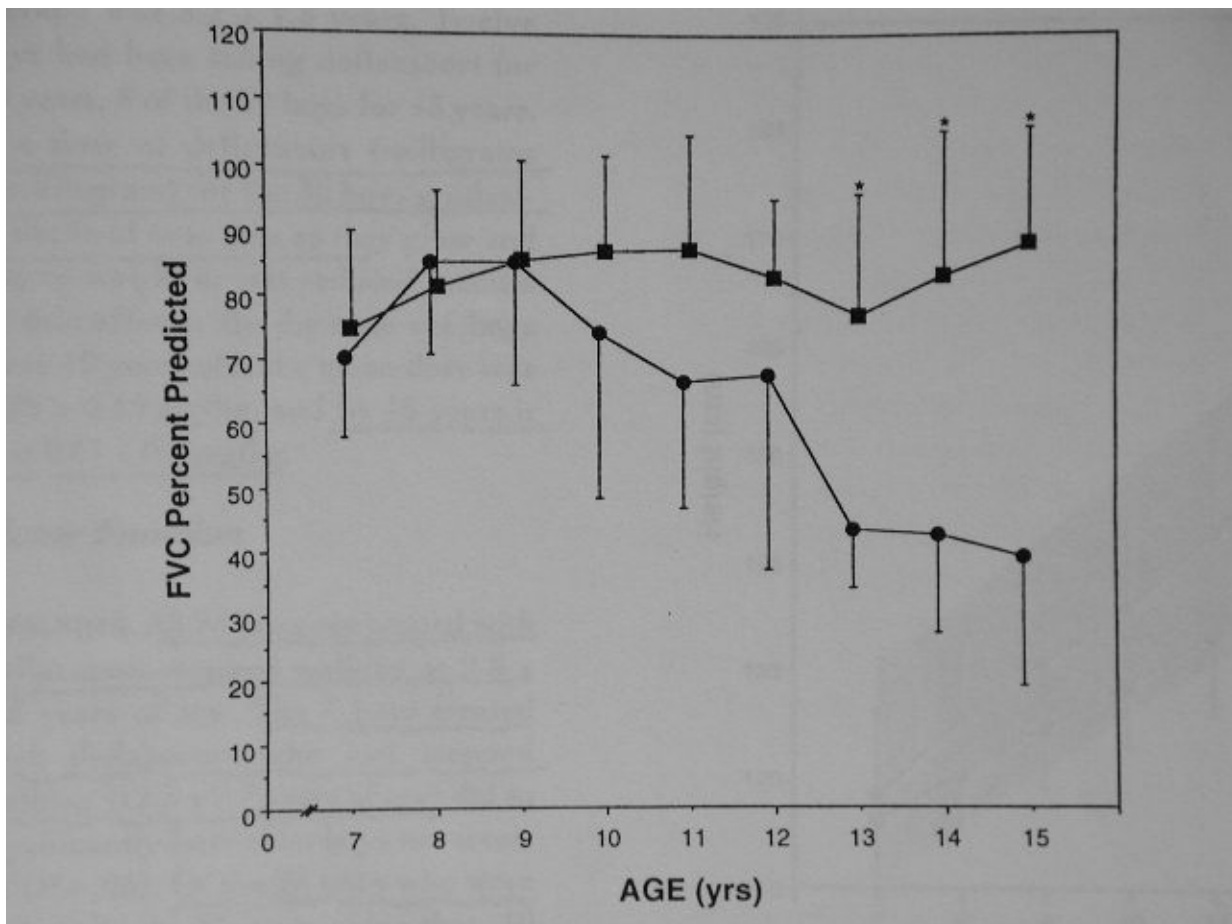
2) Eagle et al “Managing Duchenne muscular dystrophy – The additive effect of spinal surgery and home nocturnal ventilation in improving survival.”<sup>62</sup>



## 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>63</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%)

**Appendix 6:**The 31 London Primary Care Trusts:

NHS Barking and Dagenham, Barnet PCT, NHS Bexley, NHS Brent, Bromley Primary Care Trust, Camden Primary Care Trust, City and Hackney Teaching Primary Care Trust, Croydon Primary Care Trust, Ealing Primary Care Trust, Enfield Primary Care Trust, Greenwich Teaching Primary Care Trust, Hammersmith and Fulham Primary Care Trust, Haringey Teaching Primary Care Trust, Harrow Primary Care Trust, NHS Havering, Hillingdon Primary Care Trust, NHS Hounslow, Islington Primary Care Trust, NHS Kensington and Chelsea, Kingston Primary Care Trust, Lambeth Primary Care Trust, Lewisham Primary Care Trust, Newham Primary Care Trust, NHS Redbridge, Richmond & Twickenham Primary Care Trust , Southwark Primary Care Trust, Sutton & Merton Primary Care Trust, Tower Hamlets Primary Care Trust, NHS Waltham Forest, Wandsworth Teaching Primary Care Trust, NHS Westminster.

## Appendix 7:

**London PCT demographic data, projected growth and prevalence of neuromuscular conditions (NMC):**

Name of Borough	2006	2011	Estimated prevalence of NMC
Camden	201,500	205,600	202
Kensington and Chelsea	164,800	169,500	165
Westminster, City of	211,500	219,300	212
City of London	9,000	10,600	90
Central boroughs	586,900	605,000	587
Hackney	214,900	223,800	215
Hammersmith and Fulham	175,200	182,400	175
Haringey	234,000	226,300	234
Islington	187,800	197,100	188
Lambeth	283,000	290,600	283
Lewisham	258,400	267,400	258
Newham	254,400	286,400	254
Southwark	264,700	287,400	265
Tower Hamlets	218,800	252,200	219
Wandsworth	283,000	294,700	283
Rest of Inner boroughs	<b>2,366,500</b>	<b>2,516,000</b>	<b>2,367</b>
Inner London	<b>2,953,400</b>	<b>3,121,000</b>	<b>2,953</b>
Barking and Dagenham	166,800	177,700	167
Barnet	321,100	339,700	321
Bexley	215,600	213,600	216
Brent	273,300	282,700	273
Bromley	297,400	299,800	297
Croydon	329,800	338,800	330
Ealing	308,800	320,400	309
Enfield	285,100	286,600	285
Greenwich	229,900	244,400	230
Harrow	214,400	215,600	214
Havering	226,700	230,300	227
Hillingdon	244,200	243,000	244
Hounslow	220,300	234,400	220
Kingston upon Thames	152,100	155,200	152
Merton	192,000	193,800	192
Redbridge	246,000	256,700	246
Richmond upon Thames	180,400	184,300	180

<b>Sutton</b>	180,800	181,300	181
<b>Waltham Forest</b>	223,200	229,700	223
<b>Outer London</b>	<b>4,508,000</b>	<b>4,628,200</b>	<b>4,508</b>
<b>GREATER LONDON</b>	<b>7,461,400</b>	<b>7,749,200</b>	<b>7,462</b>
<b>GL (Independent)</b>	<b>7,462,500</b>	<b>7,747,700</b>	<b>7,463</b>

*Demographic data is taken from the websites of PCTs.*

## Appendix 8:

### 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.<sup>64</sup>**

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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