

Duchenne Muscular Dystrophy  
Service Program  
Dubowitz Neuromuscular Centre  
Great Ormond Street Hospital  
London

*Dr Adnan Manzur*  
*Consultant Neuromuscular Team*  
*GOSH*

# Duchenne MD

- Multisystem involvement: motor, cardiac, respiratory, nutritional, skeletal
- Course spanning from childhood into adulthood
- Alteration of natural history of Duchenne muscular dystrophy over the last 30 yrs
- Standards of care have evolved massively
- Have support services kept pace?



# Dubowitz Neuromuscular team.

1<sup>st</sup> Apr 2008. Transfer from Hammersmith Hosp to  
Great Ormond Street Hosp

# Model of Care

- Good clinical care – available when needed
- Multidisciplinary team care
- Interface with research programmes
- Facilitating translational research
- Learning from clinical practice - Audits
- Clinical networks to ensure standards of care
- Shared care with local paediatric services

# DMD clinical service: MDT Care

- **Diagnosis** – Speedy diagnosis on referrals.  
Sensitive counselling & support
- Age specific clinics: eg Young Duchenne under 7 yrs
- Problem specific clinics: eg Orthotics
- Joint Speciality clinics: eg Scoliosis surgery
- Therapy input: physio, orthotics, SLT, Dietitian
- Close links with: cardiac, respiratory and other services
- Treatment monitoring: Steroids, Vit D,
- Muscle nurse specialist & MD family care advisor
- **Transition of care to adult team** -

# Follow up appts. “One-stop shop”

- 9 year old boy on corticosteroid:
- Clinic visit schedule:
  - Physiotherapy
  - doctors assessment,
  - echocardiogram,
  - dietetic assessment,
  - DEXA scan on the same day, blood test
- Is this too much for the child and the family?
- Should these assessments be done over multiple days?

# Best model of care

- Truly multidisciplinary team / combined specialist clinics
- Senior clinical input
- Excellent orthosis
- Non-dogmatic approach
- Academic environment

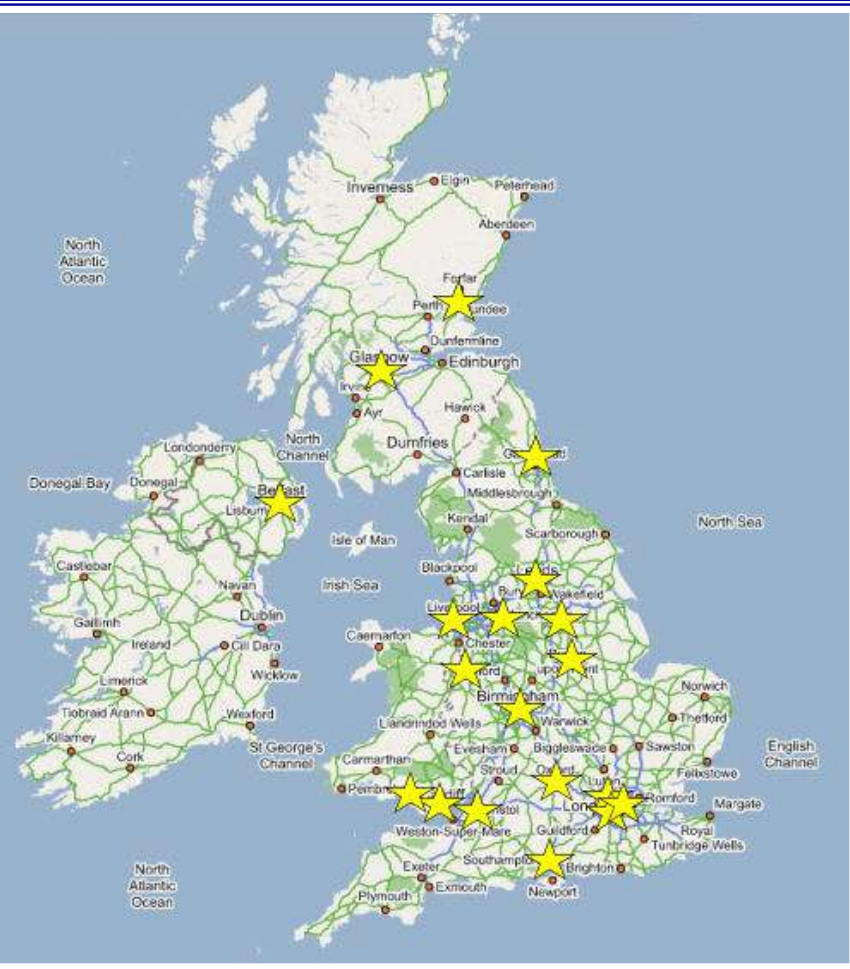
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- Physiotherapy
  - Medical treatment
  - Surgical intervention

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- Best management today
  - Therapeutic trials
  - Gene-based technologies

# “Room for improvement”

- Hospital premises designed decades ago
- Toileting and hoisting facilities to changes of disabled toileting, and hoisting facilities not uniformly available in all clinical areas
- Lack of resources to fully follow SOC
- ? Lack of psychology services, OT, seating
- Very “motor focused” – specialist care for learning / behaviour limited

# Paediatric Neuromuscular Networks



Lead Clinician/s	Centre
Dr Helen Roper	Heartlands, Birmingham
Drs Philip Jardine, Arni Ajumadar	Bristol
Drs Louise Hartley, Jane Fenton-May	Cardiff
Dr Karen Naismith	Dundee
Dr Robert McWilliam	Glasgow
Dr Anne-Marie Childs	Leeds
Dr Stefan Spinty	Liverpool
Drs Elizabeth Wraige, Heinz Jungbluth	Evelina Children's Hospital, London
Dr Catherine De Ville	GOSH
Drs Adnan Manzur, Stephanie Robb, Prof Muntoni	GOSH
Dr Imelda Hughes	Manchester
Profs Kate Bushby, Volker Straub	Newcastle
Dr Gaby Chow	Nottingham
Dr Ros Quinlivan	Oswestry
Dr Sandeep Jayawant	Oxford
Dr Peter Baxter	Sheffield
Dr Cathy White	Swansea

# NorthStar Clinical Network

2003 - Background preparatory work

2004 / 05 - Feb 04, Network coordinator in post

Consensus statement on Steroids in DMD

MREC application - ethics approval not required

Physiotherapy workshops and assessment consensus

Medical and physiotherapy assessment proformas

2006 – Database development

Parent information leaflet / consent form finalized

MREC application - ethics approval not required

Caldicott guardian approval from Hammersmith site

Web based neuromuscular database goes live

2007 – 130 patients on database,

2010 – 400 patients registered

# National Audits of DMD through NorthStar Clinical Network

- 25 OH Vit D levels in boys with DMD
- Vertebral fractures in Corticosteroid treated DMD
- Corticosteroid treatment in DMD in UK
- *Informed 2009 ENMC bone health in DMD workshop, and clinical practice*

# Great Ormond St risks losing £16m after budget review

**Sophie Goodchild**  
Health Correspondent

SERVICES for critically-ill children at Great Ormond Street Hospital could lose £16 million in cuts.

The children's trust faces a 20 per cent budget reduction under an NHS funding review, which proposes slashing top-up payments to specialist hospitals.

Opposition MPs accused the Government of reneging on a healthcare spending pledge. Shadow health secretary John Healey said: "Big health cuts to children's hospitals were not what the public expected when they heard the Prime Minister's promise to protect the NHS budget."

There are 35 centres nationwide providing specialist children's services and they receive 78 per cent more than the standard "tariff" paid to other hospitals carrying out the same procedures.

The extra funding is provided because the specialist centres treat patients with complex health problems. But new guidance from NHS deputy chief David Flory proposes a cut in the "top-up" by

**'This is not what people expected when they heard the PM promise to protect the NHS budget'**

**John Healey**  
Shadow health secretary

more than two thirds – a total of £70 million for all hospitals affected.

Great Ormond Street said: "Changes to the tariff are subject to discussion and refinement. We are exploring possible changes with the Department of Health and with other affected providers."

Health Secretary Andrew Lansley has denied the cuts would be as great as estimated by the hospital. He said the previous government had initiated the review and its conclusions were being considered to assess whether they were "accurate and applicable".

Other London hospitals that provide specialist care for children include Barts and the London NHS Trust as well as Guy's and St Thomas' NHS Foundation Trust. Barts declined to disclose the impact of the review on its children's tariff. But a spokesman said any change would be "minimal" and would be "more than compensated for" by increases in funding elsewhere.

Guy's said it had not yet assessed the impact of any tariff change. Its children's services are provided by the Evelina Children's Hospital.

Excellence in care of  
Duchenne muscular dystrophy  
is an ongoing project