

In the UK?



Action Duchenne's Response to the Governments white papers -
Liberating the NHS: Commissioning For Patients
and
Achieving Equity and Excellence for Children
October 2010

- Duchenne Muscular Dystrophy is a serious, progressive, high cost condition with no cure.
- Action Duchenne believes there has to be a budget for Rare Diseases, to ensure ring-fenced resource for rare conditions such as Duchenne.
- The DOH white paper *Liberating the NHS: Commissioning for Patients and Achieving Equity and Excellence for Children* makes no reference to how rare, serious, high cost conditions such as Duchenne will be managed.
- Action Duchenne has grave concerns regarding both the provision and standards of care for boys with Duchenne Muscular Dystrophy under the proposed changes to the NHS outlined in the White Paper.
- As highlighted in *Achieving Equity and Excellence for Children*, Standards of Care are generally poorly met. Within the UK there is extreme variability in Standards of Care, costing the lives of Duchenne patients and adversely affecting quality of life. Life expectancy for those living with Duchenne in the UK is half of that for those living in Denmark.
- The only way to improve this is through specialist led multi-disciplinary services, ring fenced budgets and high quality commissioning of the services required. The White Paper gives no indication of how this could be achieved with the GP consortia model.
- Due to the rarity and complexity of the conditions GPs are inexperienced and ill-equipped to manage or commission care for boys with Duchenne Muscular Dystrophy.
- Lack of any proposals for how to manage rare and costly conditions such as Duchenne in the new structure, can only lead to reductions in what is already a poor quality service, costing quantity and quality of life in patients and their families.



Centres of Excellence?

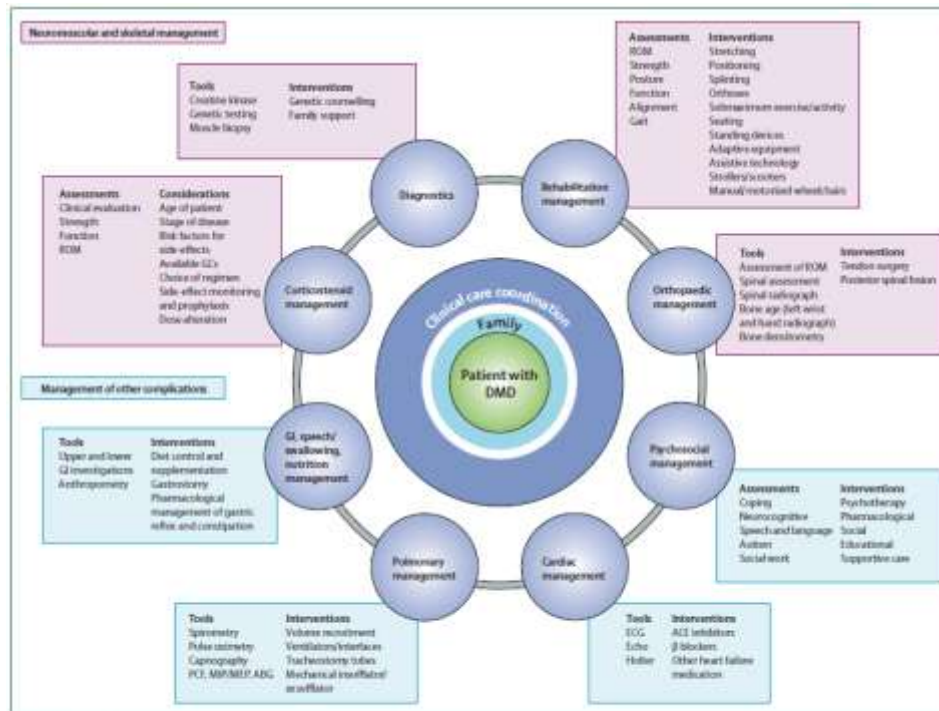


Figure 1: Interdisciplinary management of DMD

Coordination of clinical care is a crucial component of the management of DMD. This care is best provided in a multidisciplinary care setting in which the individual and family can access expertise for the required multi-system management of DMD in a collaborative effort. A coordinated clinical care role can be provided by a wide range of health-care professionals depending on local services, including but not limited to neurologists or paediatric neurologists, rehabilitation specialists, neurogeneticists, paediatricians, and primary-care physicians. It is crucial that the person responsible for the coordination of clinical care is aware of the available assessments, tools, and interventions to proactively manage all potential issues involving DMD. AEG=arterial blood gas, ACE=angiotensin-converting enzyme, DMD=Duchenne muscular dystrophy, Echo=echocardiogram, ECG=electrocardiogram, GC=glucocorticoids, GI=gastrointestinal, MIP=maximum inspiratory pressure, MIPV=maximum inspiratory pressure, PFT=peak cough flow, ROM=range of motion.



Questions

How can we ensure that all UK Duchenne patients have access to best practice standards of care? Are the Governments Health reforms going to help to improve upon existing provision? Do we need more “Centres of Excellence” to deliver improved medical and educational assessment, research and care? What is a Centre of Excellence? How will services be commissioned? How can we improve access to local services? What are the main gaps in provision? How will spending cuts impact on services for rare diseases like Duchenne? Who will pay for new treatments and drugs? How do other European countries like Denmark provide medical care and services for Duchenne families?

