

Developing consensus on assessment options for ambulant Duchenne muscular dystrophy boys

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Muscular
Dystrophy
Campaign

North Star Project Aim

To optimise patient care by achieving and practising consensus on best clinical management, with agreed assessment and treatment protocols

Objectives of the North Star Project

- To set up a UK wide **clinical network** of participating paediatric neuromuscular centres
- To develop a nationally agreed **standardised assessment protocol** to monitor change in patients with Duchenne muscular dystrophy
- To develop a **national clinical database** for a large cohort of patients with DMD to **facilitate clinical audit and review**
- The initial focus is on **optimising and standardizing steroid therapy** in ambulant children with DMD

The North Star Clinical Network for Paediatric Neuromuscular Disease Management



Identifying possible measures

- Review of those currently in use in specialist muscle centres across the UK
- Medline and CINAHL databases
- Recommendations from the international ENMC workshop on management of DMD, *Bushby et al 2004*
- ‘Consensus statement on the role of glucocorticoid corticosteroids in Duchenne muscular dystrophy’ – produced by the lead clinicians from the network centres

Nominal Group Consensus Technique

- The aim of this consensus method is to determine the extent to which experts agree about a given issue
- Consensus methods offer a format for structured discussion and prioritisation of measures
- “Agreement” takes two forms: firstly, the extent to which each respondent agrees with the issue under consideration and, secondly, the extent to which respondents agree with each other, the consensus element of these studies

Jones & Hunter 1995

Process

- Expert panel convened
- **“What are the best measures to monitor disease progression in DMD?”**
- Measurement report circulated
- Key measures identified by the group
- Discussion
- Private ranking on Likert scale

Nominal Group Physiotherapists' demographics

Physiotherapist	Paediatric experience (years)	NMD experience (years)	Estimated number of DMD patients assessed*
1	22	15	>500
2	18	17.5	>500
3	20	18	>500
4	6	0.25	>30
5	7	4	>150
6	17	5	>200
7	8	6	>500
8	20	8	>500
9	7.5	6	>100
Mean	13.9 (SD+/-6.6)	8.9 (SD+/-6.4)	

Analysis

- Likert Scale 1-9; 1=unimportant, 9 = very important
- Categories:
 - 1-3 = low importance
 - 4-6 = medium importance
 - 7-9 = high importance
- Agreement:
 - Strict – all scores within 1 category
 - Relaxed – over 2 categories
 - None – spread across 3 categories

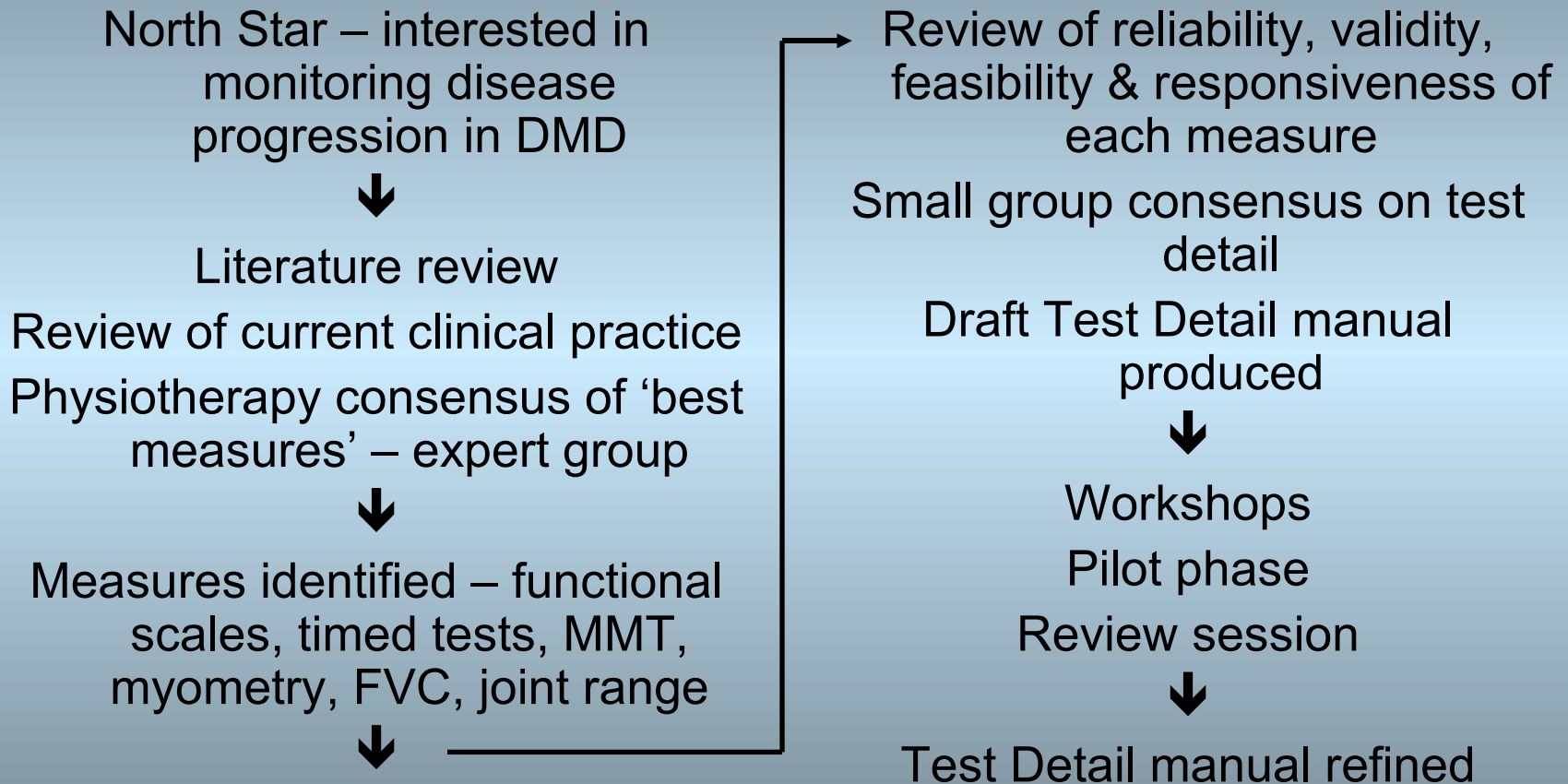
Identified measures and consensus findings

Measure	Median	Range	Comments
Hammersmith Motor Ability Scale ³	9	9	High importance, strict agreement
Timed walk 10m	9	7-9	High importance, strict agreement
Timed rise from floor	9	6-9	High importance, strict agreement
FVC	9	8-9	(x1 outlier) High importance, strict agreement
Contractures	9	6-9	High importance, strict agreement
Manual muscle testing	8	5-9	(x1 outlier) High importance, strict agreement
Myometry	8	5-9	(x1 outlier) High importance, strict agreement
Observational gait	7	4-8	(x1 outlier) Medium importance, relaxed agreement
Brooke's UL & LL Scales ⁴	7	4-8	Medium importance, relaxed agreement
Timed walk 30m	6	3-9	Medium importance, no agreement
Spinal posture	6	3-9	Medium importance, no agreement
Timed stairs	5	3-9	Medium importance, no agreement

Conclusions

- A comprehensive review of existing practice and of the literature relating to measurement in DMD identified key measures to monitor disease progression
- The use of a nominal group consensus technique gained expert agreement and confirmed seven key measures to be considered for monitoring disease progression in DMD
- These key measures have been reviewed and standardised to provide the basis of the North Star assessment protocol

Developing the assessment protocol



References

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2. Jones J & Hunter D (1995). "Qualitative research: Consensus methods for medical and health services research." *BMJ* **311(7001)**: 376-380.
3. Scott OM, Hyde SA, Goddard C & Dubowitz V. (1982). Quantitation of muscle function in children: a prospective study in Duchenne muscular dystrophy. *Muscle Nerve* **5(4)**: 291-301
4. Brooke MH, Griggs RC, Mendell JR, Fenichel GM, Shumate JB & Pellegrino R J (1981). Clinical trials in Duchenne dystrophy. I. The design of the protocol. *Muscle Nerve* **4(3)**: 186-197.