





Assessing Efficacy: Standards of Care





Are Standards of Care doing their job?

Ask questions
Provide examples
Open Discussions
Invite Feedback

Assessing Efficacy: Standards of Care

-  What should we include where?
-  What tools have we available?
-  How should we apply them?
-  What other things might be useful?

SoC: What should we include where?

-  Is outcome assessment something that should be addressed in clinical guidelines?
-  If so, what specific issues do those guidelines need to address?

SoC: What tools have we available?



One example might be the Registry of Outcome Measures (ROM)

What tools have we available? ROM



The primary purpose of ROM, at inception, was to provide the framework needed to support the systematic selection of outcome measures (OMs) 'best suited' to fill the needs of a particular clinical trial or study.



Why this might be useful?

What tools have we available? ROM

ROM enables us capture and disseminate information about a variety of OMs.




It does this in a clear, consistent, controlled manner via a web based interface.

Individualised Neuromuscular Quality of Life (INQoL)			
Objectives			
This is a neuromuscular disease specific quality of life measure for adults.			
Disease Type		Used	Validated
	Neuromuscular Junction		X
	Muscle		X
Time Information			
Time to Recall	At Assessment		
Recall Period	Not applicable		
Age Group	Adults (16 Years and Over)		
Domain Details			
Number of Domains	10		
Domain Name / Number of Items	4 symptom impact domains 5 life domains 1 treatment impact domain		
Total Number of Items	45		
Scoring			
Total Score	Yes - total score is calculated.		
Total Score - Range/Direction	0 = worse to 100 = better		
Sub-Score	Yes - sub-score is calculated.		

SoC: What tools have we available?

✧ Do you know of other tools we might use?

SoC: How should we apply them?

-  Should we apply ROM in this way, integrating information from ROM into existing Standards of Care?
-  Should we search ROM seeking to add information to Standards of Care?
-  Should we expand ROM to provide summary descriptions for SoC outcome measures that are not already recorded in the Registry?

SoC - How should we apply them?

 How would you apply other tools to add benefits?

SoC: What other things might be useful?

- ✧ Is there more we can do with an existing tool?
- ✧ For example, we are currently looking at an upgrade to ROM that will greatly improve the efficiency and capacity of the referencing element of the system.

What other things might be useful?

ROM Upgrade

The system upgrade may also provide us with an opportunity to publish Standards of Care from a single central site (i.e. integrated with ROM), using a purpose designed end to end document management system.

What other things might be useful?

ROM Upgrade

[Current Version](#) [Status and Details](#) [Associated Information](#) [Historic Versions](#) [Future Version](#) [Print](#)

STANDARDS OF CARE - DUCHENNE MUSCULAR DYSTROPHY

 [Hide Navigation](#)

Section 1 - Diagnosis of DMD

When to suspect DMD

This is the current version of this document. To view historic versions of this document click the link in the main navigation (grey) bar above.

SECTION 1 - Diagnosis of DMD

(1) The aim of care around diagnosis is to provide an accurate and prompt diagnosis, allowing initiation of appropriate interventions, continuing support and education, and minimising the length and impact of a potentially protracted diagnostic process. Diagnosis should be done by a neuromuscular specialist who can assess the child clinically and can rapidly access and interpret appropriate investigations in the context of the clinical presentation. Family follow-up and support after diagnosis will often be augmented by support from geneticists and genetic counsellors.

When to suspect DMD

(2) Suspicion of the diagnosis of DMD (figure 3) should be considered irrespective of family history and is usually triggered in one of three ways: (1) most commonly, the observation of abnormal muscle function in a male child; (2) the detection of an increase in serum creatine kinase tested for unrelated indications; or (3) after the discovery of increased transaminases (aspartate

SoC: What other things might be useful?

- ✧ Is this something that would be worthwhile pursuing, creating a central resource for SoC documentation for NMD?
- ✧ Are there other gaps (i.e. non-tool)?

Thank You!

Please provide your feedback so we can keep the discussion going and plan where to go from here.