



CARE-NMD INTERNATIONAL CONFERENCE ON DMD,  
BUDAPEST, 18-19 APRIL 2013

# **STANDARDS OF CARE FOR DMD**

## **-open issues and further developments**

Thomas Sejersen  
Karolinska Institutet, Stockholm, Sweden

# Standards of care -important achievements:

- **DMD** (Bushby et al, Lancet Neurol 2010; 9:77-93, 177-189)
- SMA
- Congenital muscular dystrophies
- Congenital myopathies



Parent Project  
Muscular Dystrophy



# Standards of care, key principles:

- Patient representatives
- Evidence when existing
- Delphi-like expert consensus building process
- 40-80 experts in key areas
- Scientific publication + user friendly version

# Standards of care, key principles:

- Patient representatives
- Evidence when existing
- Delphi-like consensus building process:
  - Key symptoms?
  - How investigate / measure these symptoms?
  - Therapeutical interventions?
- 40-80 experts in key areas
- Scientific publication + user friendly version

# DMD care guidelines, -open issues:

1. Expert consensus exists for all recommendations in present (2010) vs of DMD care guidelines?

?

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Areas missing?

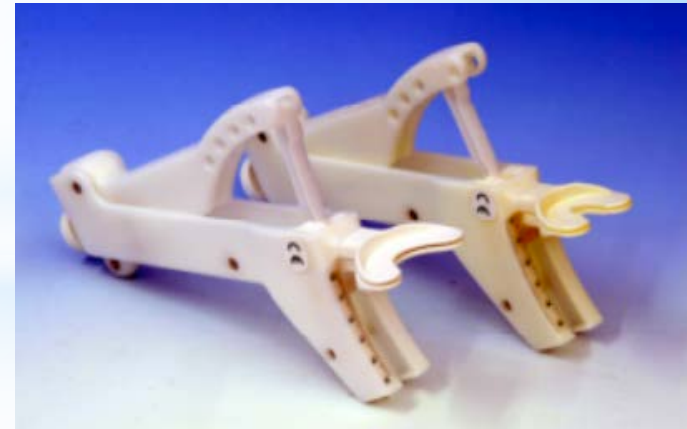
# DMD care guidelines, -open issues:

1. Expert consensus exists for all recommendations in present (2010) vs of DMD care guidelines?

Areas missing? YES!

- Oral health
- Bladder function
- (Adulthood)

# Oral health:





# Bladder function:



## Bladder dysfunction frequent in DMD!

MacLeod et al. Bladder dysfunction in DMD. Arch Dis Child 2003;88:347-349

Askeland et al. Urologic manifestations in DMD. J Urol jan 2013, ePubl ahead of time

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Revision of recommendations?

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## *Initiation of glucocorticoid therapy:*

“Initiation of glucocorticoid treatment is not recommended for a child who is still gaining motor skills....”

“Once the plateau phase has been clearly identified, usually at age 4-8 years, the clinician should propose initiation of glucocorticoids”

# Revision of recommendations?

*GC use after loss of ambulation:*

“The effectiveness of glucocorticoid treatment in preventing scoliosis or in stabilising cardiac or respiratory function in this (adult) setting is not known....”

Markham LW et al. Steroid therapy and cardiac function in duchenne muscular dystrophy. *Pediatr Cardiol.* 2005;26:768-771.

Balaban B. et al. Corticosteroid treatment and functional improvement in duchenne muscular dystrophy: Long-term effect. *Am J Phys Med Rehabil* 2005;84:843-850.

Alman B. et al. Steroid treatment and the development of scoliosis in males with Duchenne muscular dystrophy. *J Bone Joint Surg Am* 2004; 86: 519-24

McAdam et al. The Canadian experience with long term deflazacort treatment in DMD. *Acta Myol.* 2012; 3:16-20

# Revision of recommendations?

Ongoing/planned studies likely to lead to need of more revisions:

Novel medical therapies (exon skipping, premature stop codon)

Cardiac care (ACE-inhibitor,  $\beta$ -blockers)

Bone health (DEXA-scint, oral bisphosphonates)

# DMD care guidelines, -open issues:

2. Structure of the present DMD care guideline adequate?

?



## Standards of Care for Spinal Muscular Atrophy



## STANDARDS OF CARE

### GENERAL WARNINGS

- ❖ Dissemination?
- ❖ Implementation?
- ❖ Impact on health?
- ❖ "Outdated"?



# DMD care guidelines, -structure:

## *Issues to consider:*

- “To be used before xxxx-yy-zz”
- Clear information on level of evidence. Utilize GRADE?
- How best to ensure patient involvement in process? “Dialogue model”?
- Do recommendations reach out? Dissemination and implementation!
- Analyse effect of recommendation!

## **RATING QUALITY OF EVIDENCE AND STRENGTH OF RECOMMENDATIONS**

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# **GRADE: an emerging consensus on rating quality of evidence and strength of recommendations**

Guidelines are inconsistent in how they rate the quality of evidence and the strength of recommendations. This article explores the advantages of the GRADE system, which is increasingly being adopted by organisations worldwide

Guyatt GH, et al, Schünemann HJ.  
BMJ 26;336(7650):924-6, 2008

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# WHO

*Handbook  
for Guideline  
Development*



World Health  
Organization

ISBN 978 92 4 154844 1, 2012

## WHO advice on evaluation and monitoring:

“An evaluation should be done to measure the impact of the guideline. The guideline should include outcome or performance measures that can be monitored for the main recommendations. Performance measures might be related to:

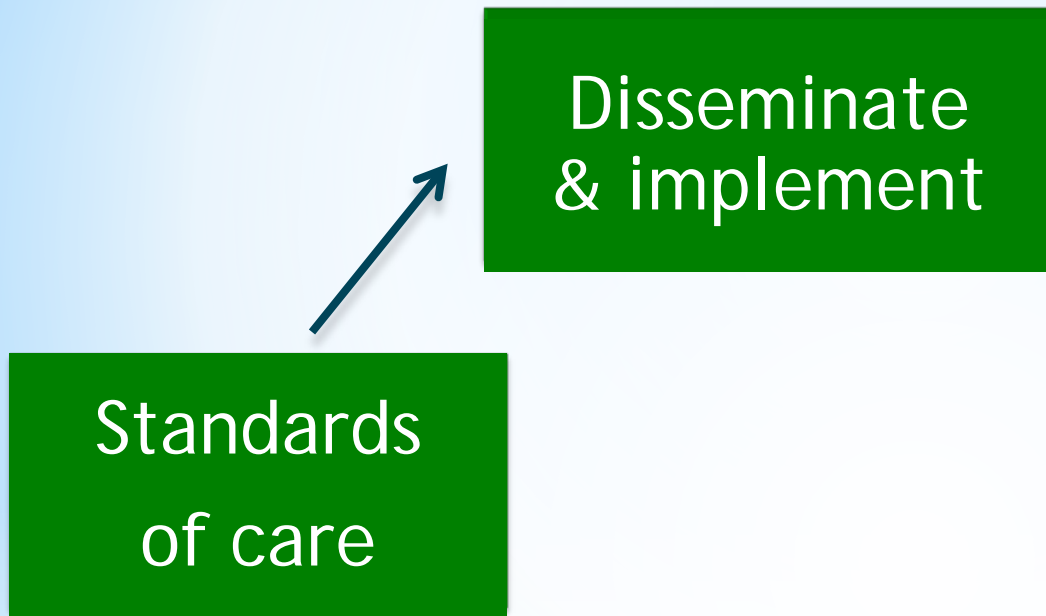
- guideline dissemination
- change in practice performance
- change in health outcomes
- change in end-user knowledge and understanding
- economic consequences.”

# DMD care guidelines, -open issues:

3. DMD standards of care, -further developments!

!

# Standards of care scheme:



## Awareness and implementation of DMD SoC

<i>Awareness of SoC guidelines:</i>	85%
<i>Document availability in the local language:</i>	
Yes	26%
In translation	20%
No	54%
<i>Implementation of SoCs:</i>	
Well implemented	15%
Partly implemented	54%
Not at all implemented	31%



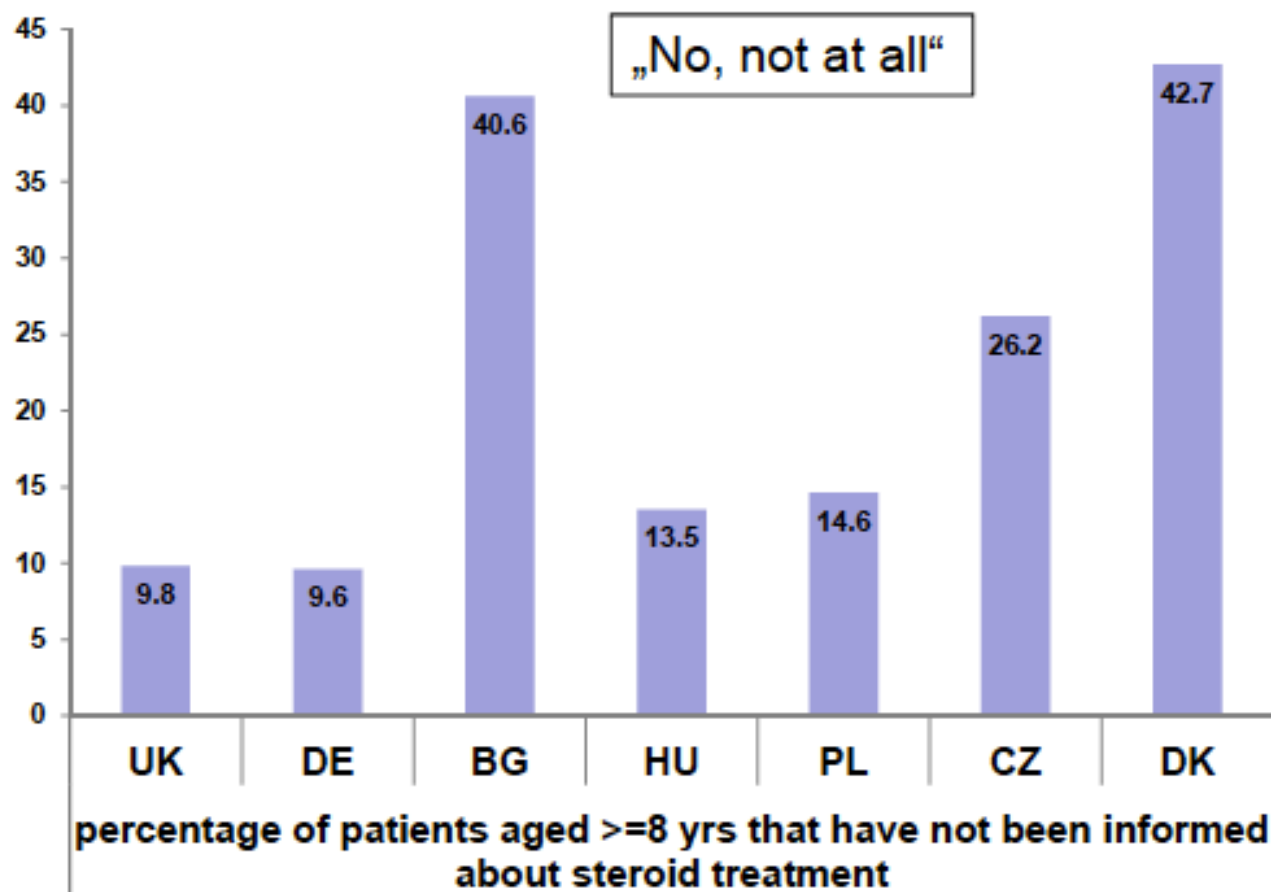


# Obstacles to implementation of SoCs

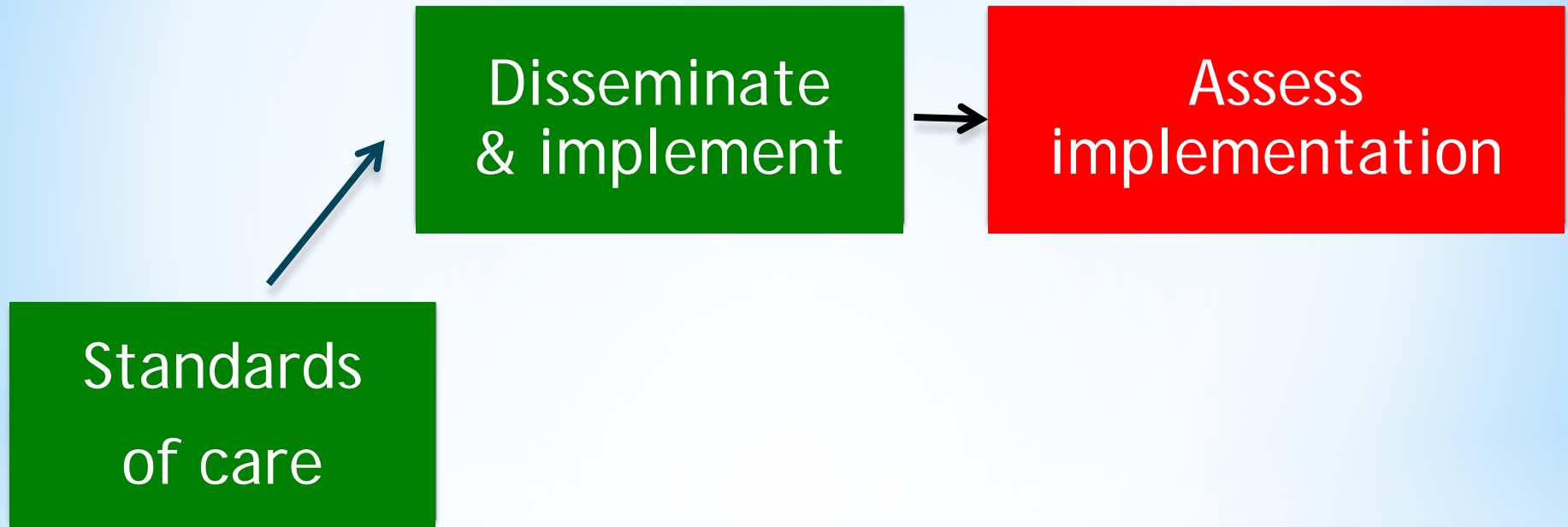
Reasons for partly or no implementation of SoC in CEE countries:

- *Financial reasons* 91%
- *Lack of support within medical community* 82%
- *Political reasons* 55%

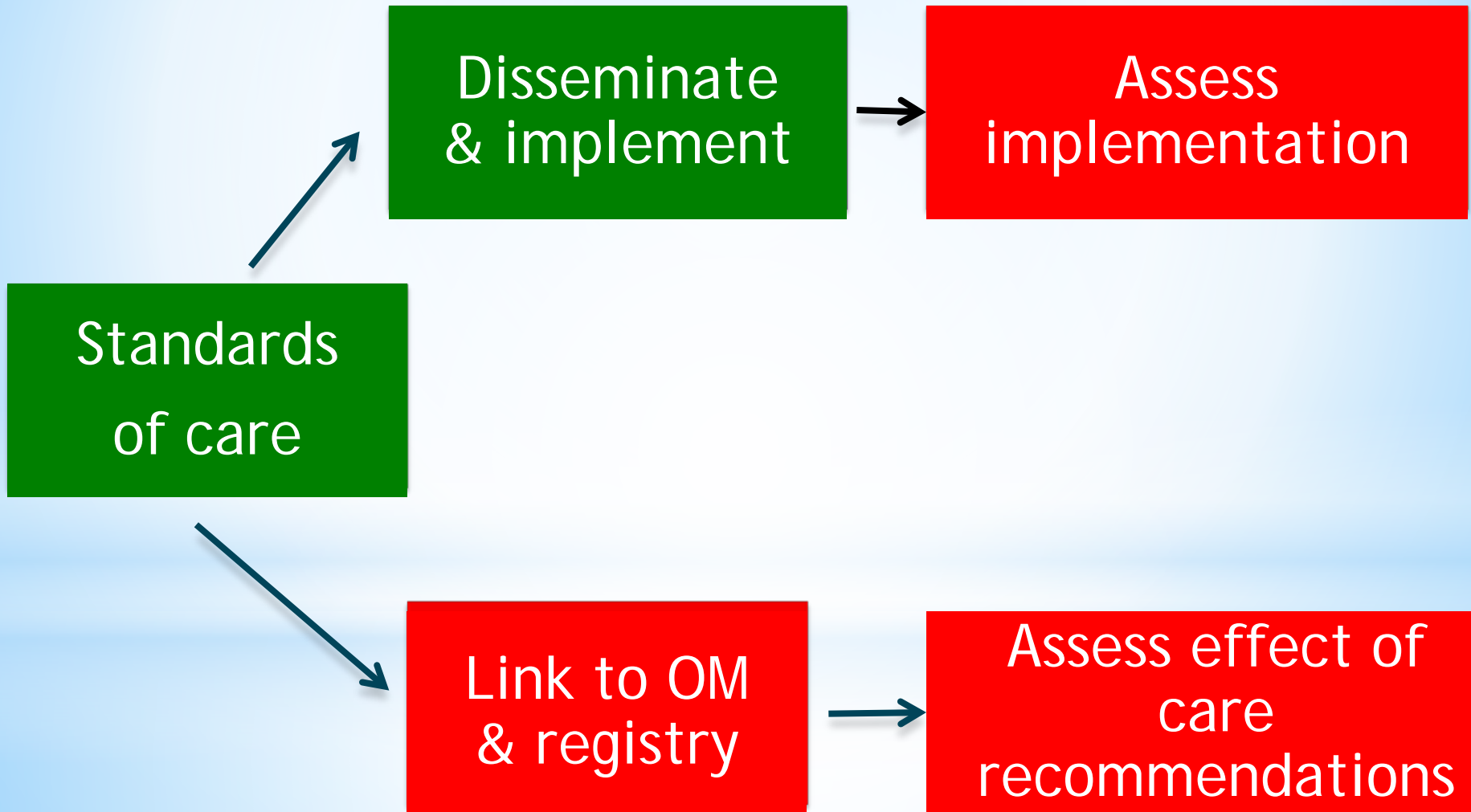
# Have you been informed about steroid treatment?



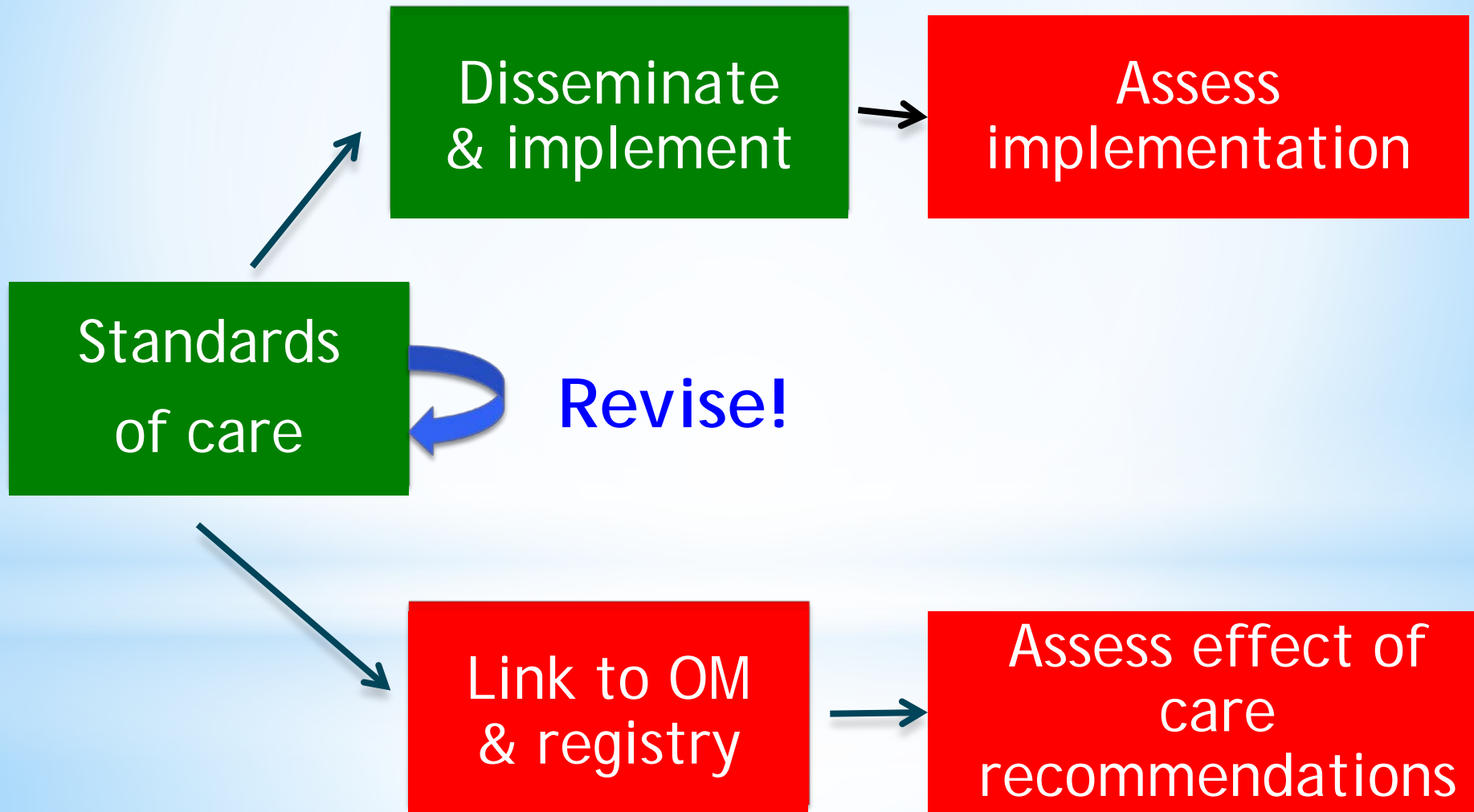
# Standards of care scheme:



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# Conclusions, -accomplished:

- **Consensus care guidelines produced** for SMA, DMD, CMD, and congenital myopathies
- **User-friendly versions** of guidelines available in **several languages**
- Full documents and (translated) user-friendly versions available at [www.treat-nmd.eu](http://www.treat-nmd.eu)
- Efforts for DMD care guideline dissemination and implementation (e.g. CARE-NMD)

# Conclusions, -next tasks:

- Update existing guidelines
- Optimise patient involvement in process
- Grading of recommendations according to level of evidence
- Assess dissemination-implementation
- Analyse health effect of guidelines

Thank you!

