

Standards of Care activities in TREAT-NMD

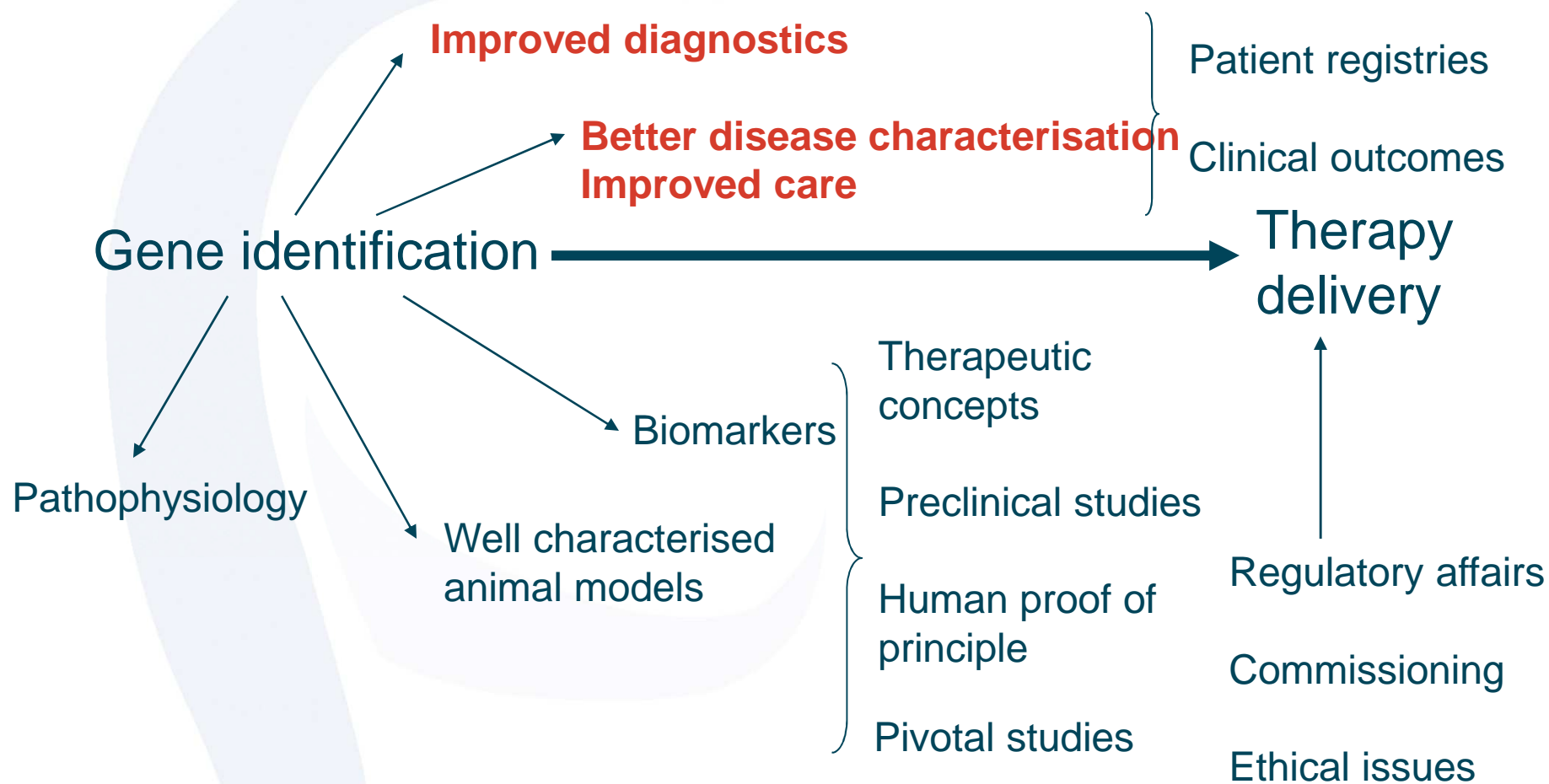


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www.treat-nmd.eu

On behalf of Thomas Sejersen, activity leader

2 | The route to therapy



Harmonisation of care for NMD

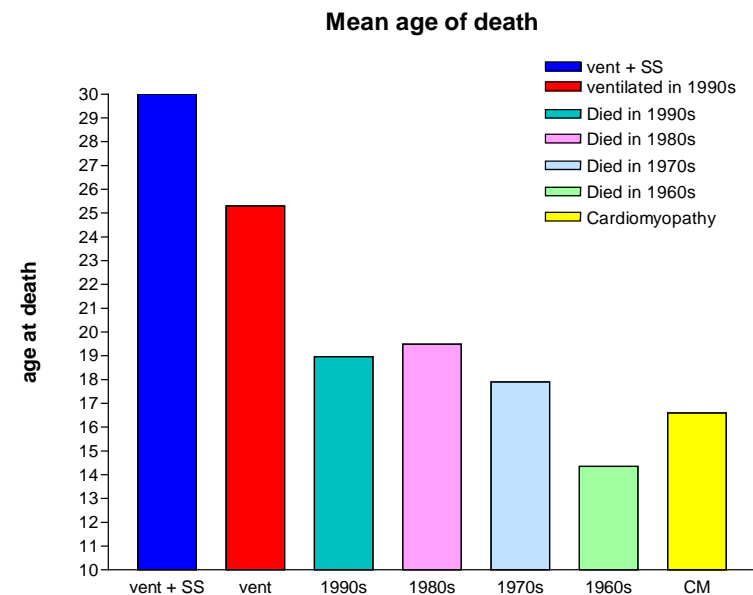
■ Why?

- To create a level playing field for patients and families
- Provision of care should not be a lottery
- To provide a tool to lobby health care providers
- To raise standards across the board
- To encourage new initiatives to encourage excellence
- To provide a basis for the introduction of trials and new treatments



Example: appropriate management improves survival in DMD today

- DMD is a treatable disease
 - Predictable complications in different systems
- Steroids prolong ambulation and delay the onset of other complications
 - In conjunction with physiotherapy regimes
- Respiratory support is proven to improve life expectancy with maintenance of a good quality of life
- Cardiac surveillance and treatment is likely to have similar benefits



However.....

- Many patients do not have access to these levels of care
- This impacts on their whole life and longevity
- When trials are being planned the difference in care standards creates additional barriers to patient recruitment
 - And jeopardises the interpretation of the results
- This is true for ALL NMD, not just DMD

Standards of care activities in TREAT-NMD

- Work with the experts in the field
- Assist where other groups have already made progress in these goals
- Utilise the whole network (“joined up thinking”) to ensure that the message gets out to the community
 - Family guides
 - Translations of family guides and academic articles
 - Dissemination via newsletter, patient organisations, care and trial site registry, patient registries, national initiatives, on site training, adjunctive projects (CARE-NMD)
- Address updating and new areas of research

Standards of care activities in TREAT-NMD

- SMA collaboration with ICC
- DMD- working with CDC led initiative
- CMD- working with CURE-CMD
- Congenital myopathies consortium

A Foundation
**Building
Strength**
for Nemaline Myopathy



Parent Project
Muscular Dystrophy
LEADING THE FIGHT TO END DUCHENNE

MDA



Principles of generation of care standards similar

■ How?

- Gather evidence from literature where this exists
- Assemble expert panels
- Ensure representation as broad as possible
- Use an unbiased method for building consensus
 - RAND/ UCLA appropriateness method (RAM)
 - Delphi methodology
- High profile academic publication
- “Joined up thinking” for dissemination and implementation
- Consistent message



EG: DMD: Consensus generation via expert groups and RAM methodology

- Key areas of clinical management identified in 8 disciplines
- >80 experts went through a three stage process of generating recommendations
- Personnel and “toolkit” defined for optimal care
- Direction for care in specific scenarios generated from results of consensus
- Multidisciplinary key issue, need for experience and co-ordination of care
- Management geared to the specific stage of condition



Panel	ROUND 1		ROUND 2		ROUND 3	
	Matrices	Clinical Scenarios	Matrices	Clinical Scenarios	Matrices	Clinical Scenarios
Diagnostics	23	402	11	136	11	50
Psychosocial	61	26,075	19	325	18	148
Neuromuscular	30	817	26	948	24	192
Rehabilitation Management	126	36,015	29	1322	22	421
Orthopedics / Surgical	48	2,284	25	442	12	33
Gastrointestinal / Nutrition	44	3,479	25	479	20	129
Respiratory	36	606	33	269	27	115
Cardiovascular	21	624	13	194	12	64
TOTAL	389	70,302	174	4,115	149	1,152

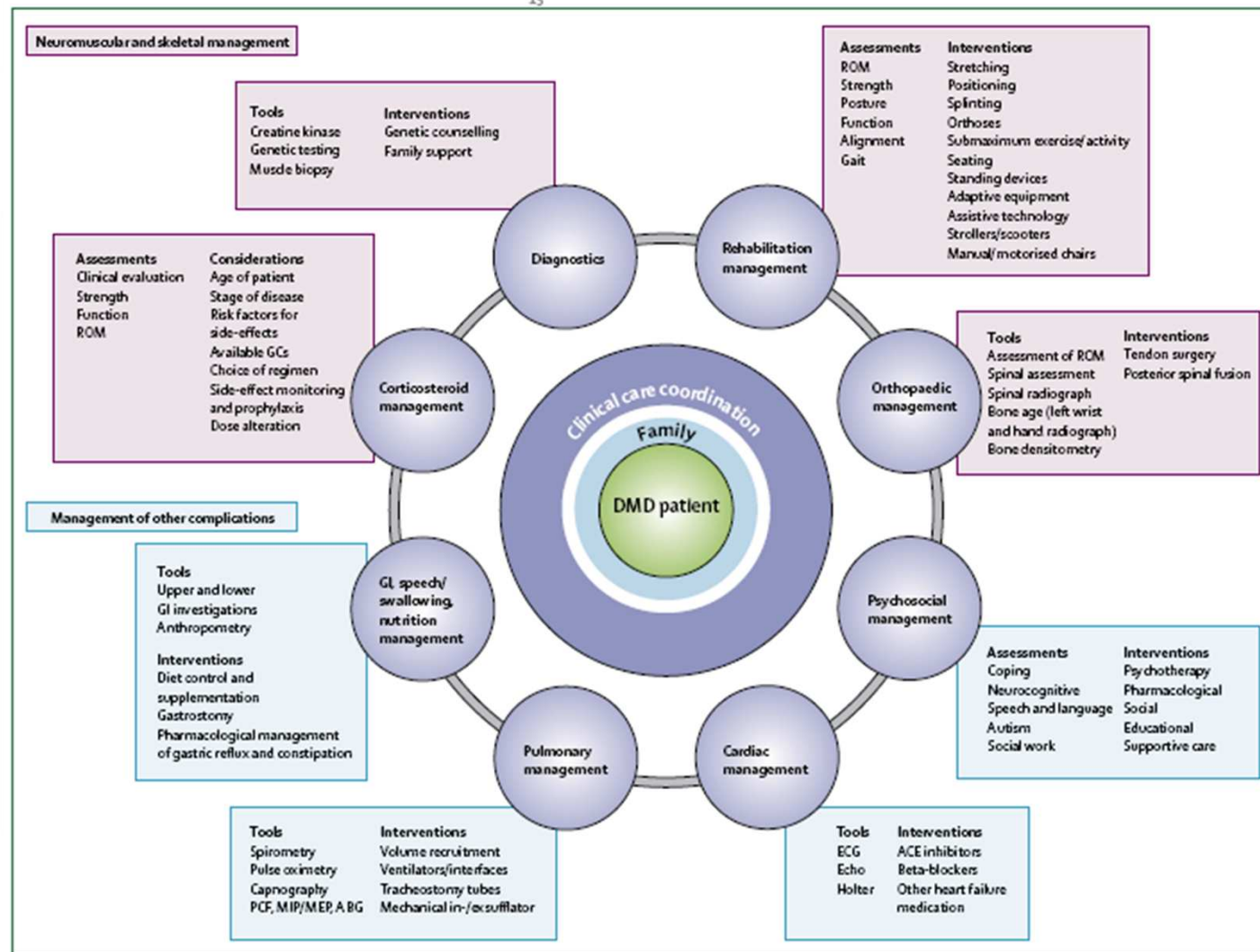
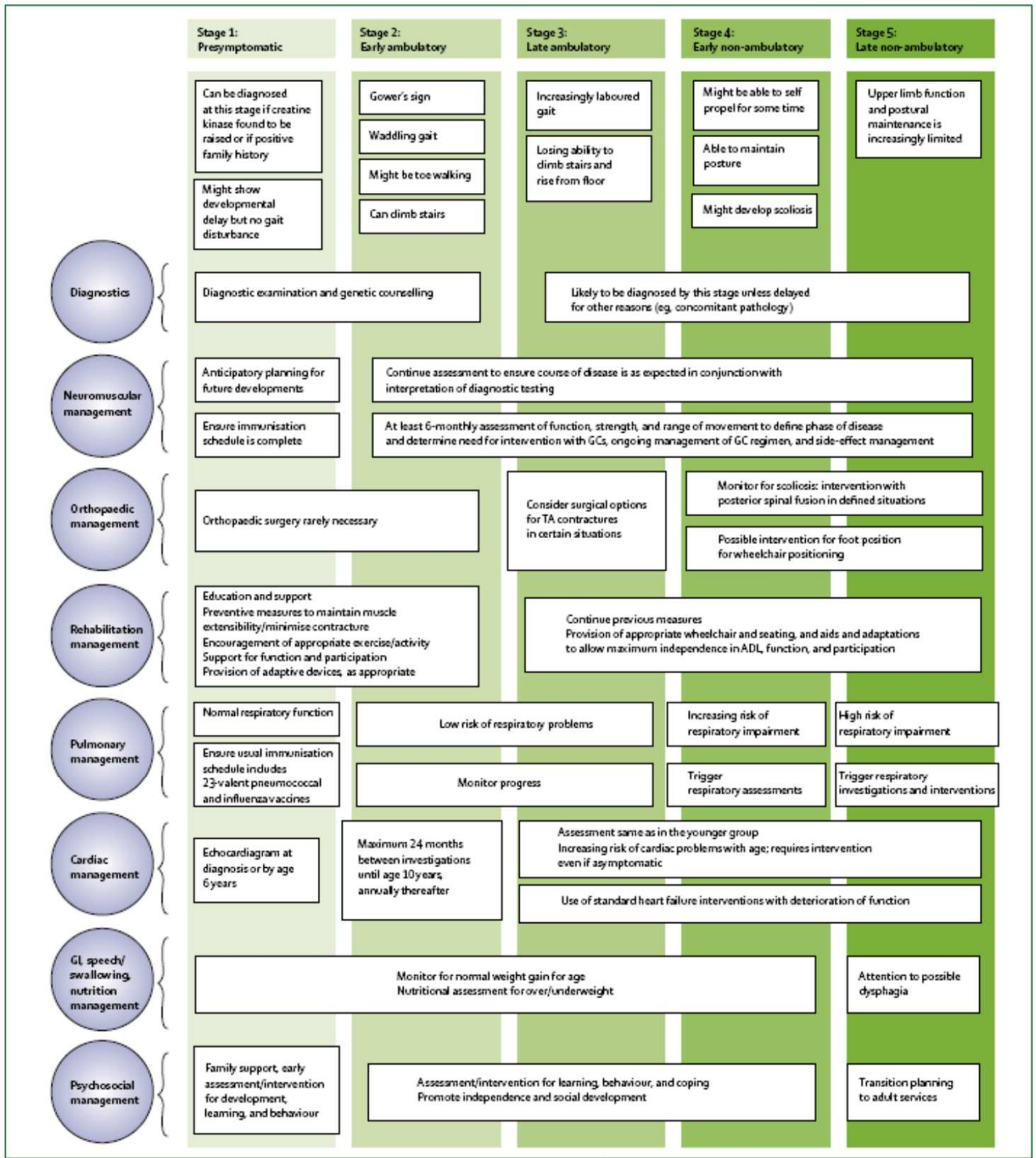


Figure 1: Interdisciplinary management of DMD



Huge effort from the CDC team, publications committee and working group!

K Bushby, R Finkel, D Birnkrant, L Case, P Clemens, L Cripe, A Kaul, K Kinnett, C McDonald, S Pandya, J Poysky, F Shapiro, J Tomezsko, C Constantin, and the DMD Care Considerations Working Group

Ted Abresch, MS, University of California, Davis; Laura Case, PT, DPT, MS, PCS, Duke University; David Atkins, MD, MPH, Agency for Healthcare Research and Quality; Linda Cripe, MD, FAAP, Cincinnati Children's Hospital Medical Center; Valerie Cwik, MD, Muscular Dystrophy Association; Jonathan Finder, MD, Children's Hospital of Pittsburgh; Pat Furlong, BSN, Parent Project Muscular Dystrophy; Aileen Kenneson, PhD, MS, Ajay Vatave, MD, MPH, or Carolyn Constantin, PhD, RNC, CDC National Center on Birth Defects and Developmental Disabilities; Craig McDonald, MD, University of California, Davis; Shree Pandya, PT, MS, University of Rochester; John Porter, PhD, NIH National Institute of Neurological Disorders and Stroke; Karen Siegel, PT, MA, Agency for Healthcare Research and Quality; Michael Sussman, MD, Shriners' Hospital for Children (Portland, Oregon); Brenda Wong, MD, Cincinnati Children's Hospital Medical Center.

Publication date: Lancet Neurology January and February 2010

Family guide production: SMA, DMD

- Collaborative work to generate a guide for families from the academic document
- PDF downloads, printed version (can be country specific)
- Translations and dissemination in collaboration with local clinicians and patient groups
- CMD, congenital myopathies, myotonic dystrophy, FSHD- in planning with relevant groups

Standards of Care for Spinal Muscular Atrophy



Downloads

[SMA précis - English](#)

[SMA précis - German](#)

[SMA précis - Dutch](#)

[SMA précis - Russian](#)

[SMA précis - Bulgarian](#)

[SMA précis - Italian](#)

[SMA précis - Polish](#)

[SMA précis - Turkish](#)

[Full consensus statement -
English](#)

[Family guide to the consensus
statement - ICC](#)

[SMA précis - Czech](#)

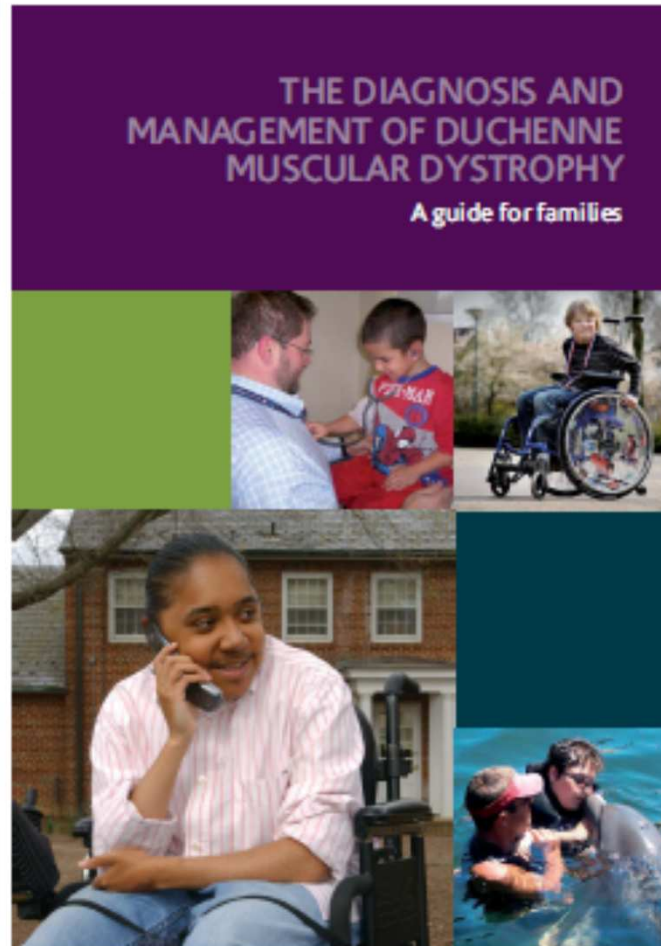
[SMA Family Guide - English
Brochure](#)

[SMA précis - Serbian](#)

[SMA précis - Portuguese](#)

[SMA précis - French](#)

Language	Family Guide brochure with images	Black and white printer-friendly version
Arabic		
Belarusian		
Bulgarian		
Chinese (Mandarin)		
Czech		
Danish		
Dutch		
English (US)		
English (UK)		
Farsi		
Finnish		
French		
German		
Greek		
Hungarian		
Italian		
Japanese		
Polish		
Portuguese		
Romanian		
Russian		
Serbian		
Slovenian		
Spanish		
Swedish		
Tamil		
Turkish		
Ukrainian		



Examples of other activities related to SOC: Opportunities!

- Lobbying health care providers based on international best practice
- Local training courses for professionals
- Patient information days.....
- CARE-NMD