

Aktuelle og kommende terapier under TREAT-NMD



Brickless Centre 2008

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www.treat-nmd.eu

Aktuelle og kommende terapier under TREAT-NMD



Mål: "advancing diagnosis, care and treatment
for people with neuromuscular diseases
around the world"

Forbedred infrastruktur for at harmonisere vejen fra laboratorium til klinisk prøve og behandling:

- Standardisation of animal and cell models
- Production, toxicology and systemic delivery methods
- Biobanks
- Protocols for molecular diagnosis
- Outcome measures
- Standards of diagnosis and care
- Clinical trial coordination
- Patient registries
- Training, communication, dissemination



TREAT-NMD collaborations - a global network



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- **Outcome measures**
- **Standards of diagnosis and care**
- **Clinical trial coordination**
- **Patient registries**
- Training, communication, dissemination



Trial readiness: outcome measures

- Development of online **registry** of outcome measures
- Assessment of available tools in clinical situation
- Identification of gaps in knowledge
- Dialogue with regulatory authorities
 - Flexibility, training, education
- Not promoting a single OM but assessing which are appropriate for different situations





Registry of Outcome Measures

[Home](#) | [View A-Z](#)

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View A-Z

You are currently looking at an alpha-numerical listing of the existing outcome measures that have been entered into the Registry. Click the relevant link to 'view summary descriptions' for a group of outcome measures.

[NUMERIC](#) [A](#) [B](#) [C](#) [D](#) [E](#) [F](#) [G](#) [H](#) [I](#) [J](#) [K](#) [L](#) [M](#) [N](#) [O](#) [P](#) [Q](#) [R](#) [S](#) [T](#) [U](#) [V](#) [W](#) [X](#) [Y](#) [Z](#)

NUMERIC (View Summary Descriptions)

» [12 Minute Shuttle Walk Test](#)

[top](#)

A (View Summary Descriptions)

» [ACTIVLIM: a Rasch-built measure of activity limitations](#)

» [Ankle Dynamometer](#)

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B (View Summary Descriptions)

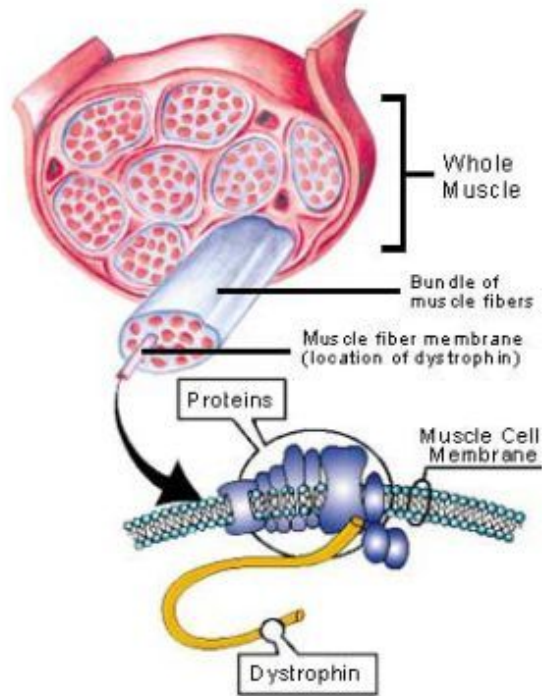
» [Balance Quality Tester](#)

» [Biodex System 3 Pro](#)

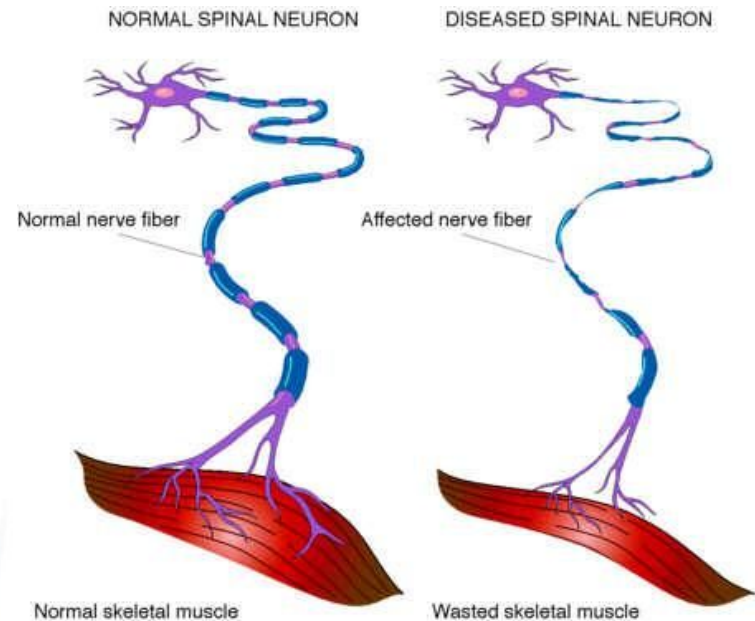
Standards of Diagnosis and Care

1. A joint basis for diagnostics and care improves quality of multinational treatment studies
2. Low prevalence necessitates expert advice being collated on a multinational level
3. Families should be offered the most informed treatment and counselling regardless of where in Europe they live

Fokus på Duchennes muskeldystrofi (DMD) og spinal muskelatrofi (SMA)



DMD



SMA

Standards of care in SMA

- **ICC Consensus statement for SOC in SMA**
TREAT-NMD/ICC précis
ICC Family guide



ICC Consensus statement for Standards of care in SMA

Standard of care committee (SCC) formed 2005 to establish guidelines

12 core members, 56 experts

How to reach consensus? Delphi technique



ICC Consensus statement for Standards of care in SMA

Five areas addressed:

diagnosis

pulmonary care

GI and nutritional care

orthopedics and (re)habilitation

palliative care

Three functional levels:

non-sitter

sitter

walker





Patient Care

HEALTH PROFESSIONALS > PATIENT CARE > SMA

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MEETINGS & EVENTS

GET INVOLVED

PARTNERS

WHAT WE DO

OUTCOME MEASURES

PATIENT REGISTRIES

PATIENT CARE

Care standards

» SMA

DMD

DOWNLOADS

Standards of care for spinal muscular atrophy

TREAT-NMD has been working with the authors of the recently published consensus statement on care for patients with spinal muscular atrophy to create useful summary factsheets based on the full published document. A TREAT-NMD working group is continuing to develop the standards for care on SMA in areas such as physical and occupational therapy, orthopaedics, nutrition and psychosocial implications. We will be looking for volunteers from amongst the partners and others to help with the generation of these further guidelines soon.

We are very interested in talking with patients and clinicians about these recommendations and suggestions for their future improvement. If you're interested, please email the TREAT-NMD team at info@treat-nmd.eu.

Translations

We believe it is crucial to have this type of information about standards of care available in patients' and clinicians' **native languages**, and we are therefore in the process of translating it into a number of languages with the generous help of multilingual staff within the TREAT-NMD network and colleagues from patient organisations. If you are interested in translating our factsheets into your language for hosting on our website, please **contact the TREAT-NMD team** at info@treat-nmd.eu - we'd love to hear from you!

Translations that are currently in progress include: Polish, Dutch, Italian, Turkish and Slovenian



Downloads

SMA précis - English

SMA précis - German

SMA précis - Dutch

SMA précis - Russian

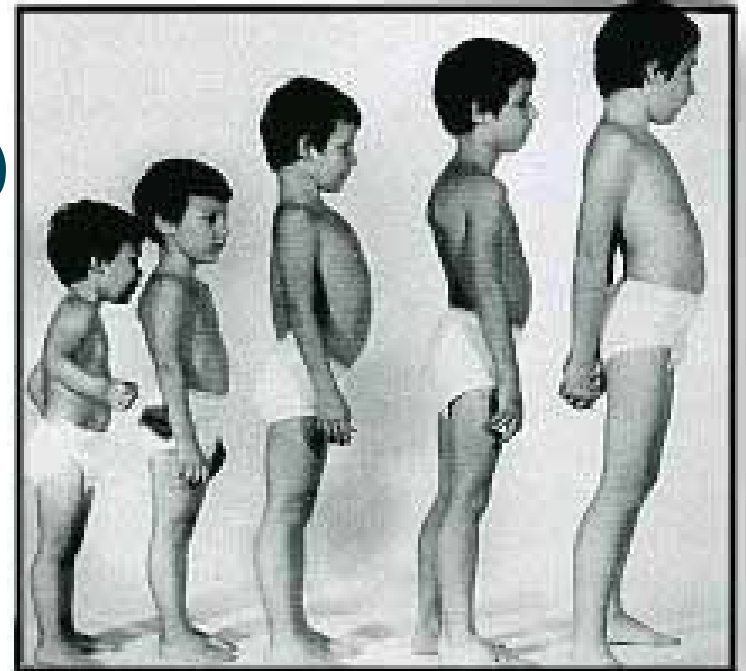
SMA précis - Bulgarian

Full consensus statement - English

Family guide to the consensus statement

TREAT-NMD interim Standards of Care recommendations for DMD

- Diagnosis
- Medical treatment (cortisone)
- Nutrition / gastroenterology
- Respiration
- Cardiology
- Orthopaedics
- Psychosocial
- Rehabilitation
- Oral and dental care



Keyword Search

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Regulatory Affairs

Standards of care for Duchenne muscular dystrophy

Introduction

The process of drawing up a comprehensive set of recommendations for standards of care in DMD on the basis of true international consensus is ongoing, under the auspices of the CDC Care Considerations project, and is likely to be complete in 2008. In the intervening period, TREAT-NMD is working to get as much useful information as possible summarised and out into the public domain. A group of experts, primarily co-authors of existing DMD management guidelines, has been invited to collaborate with the TREAT-NMD activity leaders with the aim of first collecting and comparing existing management recommendations and reaching consensus from these documents and updates of present knowledge, and subsequently presenting these consensus recommendations at a "DMD standards of care" meeting in 2008.


TREAT-NMD interim recommendations

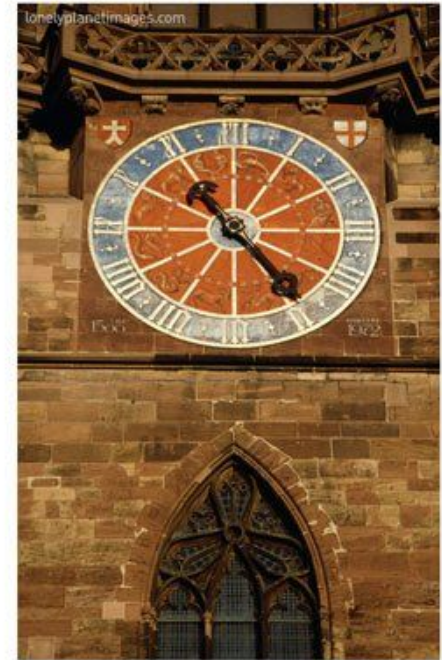
The aim of these brief recommendations is to achieve the rapid dissemination of existing knowledge in this area while awaiting the more detailed recommendations presently being drawn up by the US Center for Disease Control in collaboration with TREAT-NMD. The present guidelines are to be considered as expert opinion, and are not based on systematic review processes, although an evidence-based approach has guided the work. Use the download button to download a PDF of these TREAT-NMD care recommendations.



 download

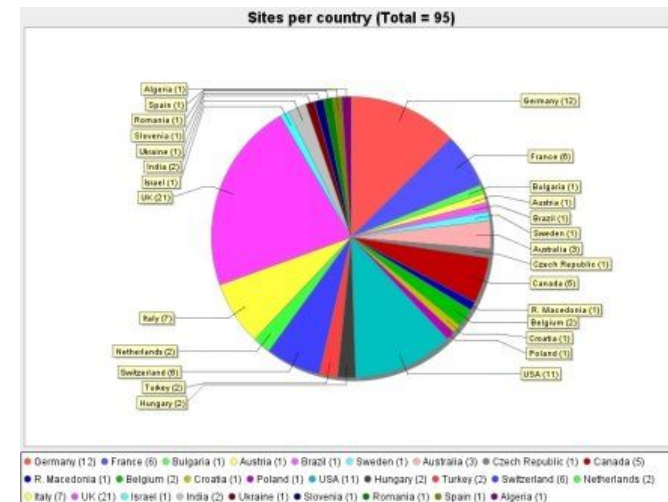
Trial readiness: clinical trial coordination centre

- University of Freiburg (MD-NET 
- Services:
 - Project and data management
 - Monitoring
 - Study assistance
 - Training
 - Bioinformatics
 - Trial site identification
 - Guidance on European regulations
 - EMEA/FDA contacts



Trial readiness: network of trial sites

- Pre-feasibility data obtained on clinical sites worldwide with trial experience and neuromuscular expertise
- Resource for industry planning trials
- Combines with patient care initiative to disseminate best practice and care standards
- Possibility of using network as platform to fast-track certain trials



How it works in practice: first feasibility study

- Enquiry from a company considering a clinical trial in DMD (Europe/US)
- Accurate clinical data required for trial planning
- Inquiry received: 5 June 2008
- Request for recently recorded or updated data only (< 1 year old!); 2 weeks for completion of study
- Report completed: 23 June 2008
 - **Dramatically quicker results ⇒ trials are suddenly more attractive**

Trial readiness: patient registries

A **global** database of patients with the genetic and clinical data necessary for trial recruitment

- Many benefits to registered patients
 - Feedback on standards of care and new research developments
 - Feeling a sense of “belonging” to a broader community
 - Not being left behind as clinical trials develop
 - A link to the research community
- Many benefits to industry
 - Easy access to patient community
 - Clear concept of target market
 - Feasibility and planning of clinical trials
 - Recruitment of patients into clinical trials



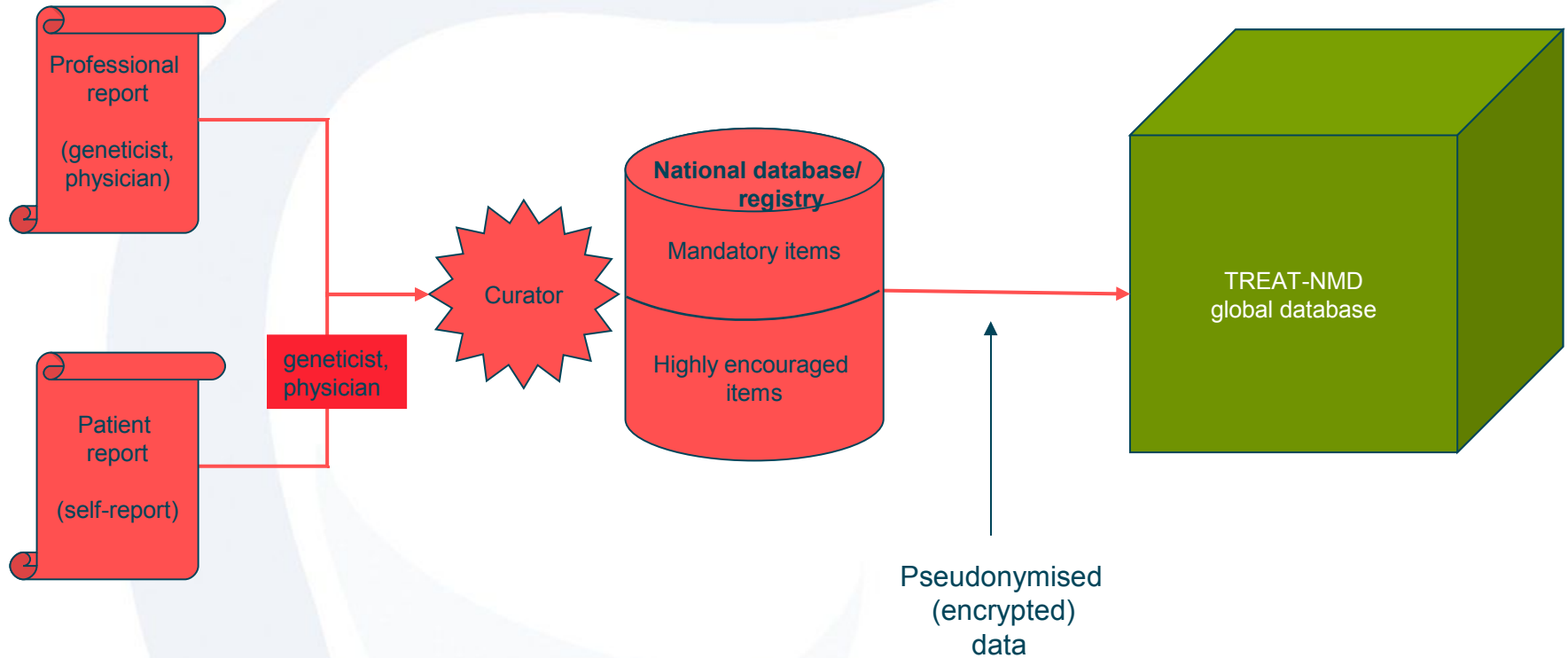
Registries for DMD: harmonized items

- Personal data of patient (name, birth date, address)
 - Diagnosis (DMD, BMD, IMD, SCarrier, not determined)
 - Mutation/Deletion
 - Motor function: ambulation
 - Medication: steroids
 - Scoliosis surgery
 - Cardiac involvement
 - Ventilatory function (Ventilator use)
 - Muscle biopsy
- Mandatory
- Highly encouraged

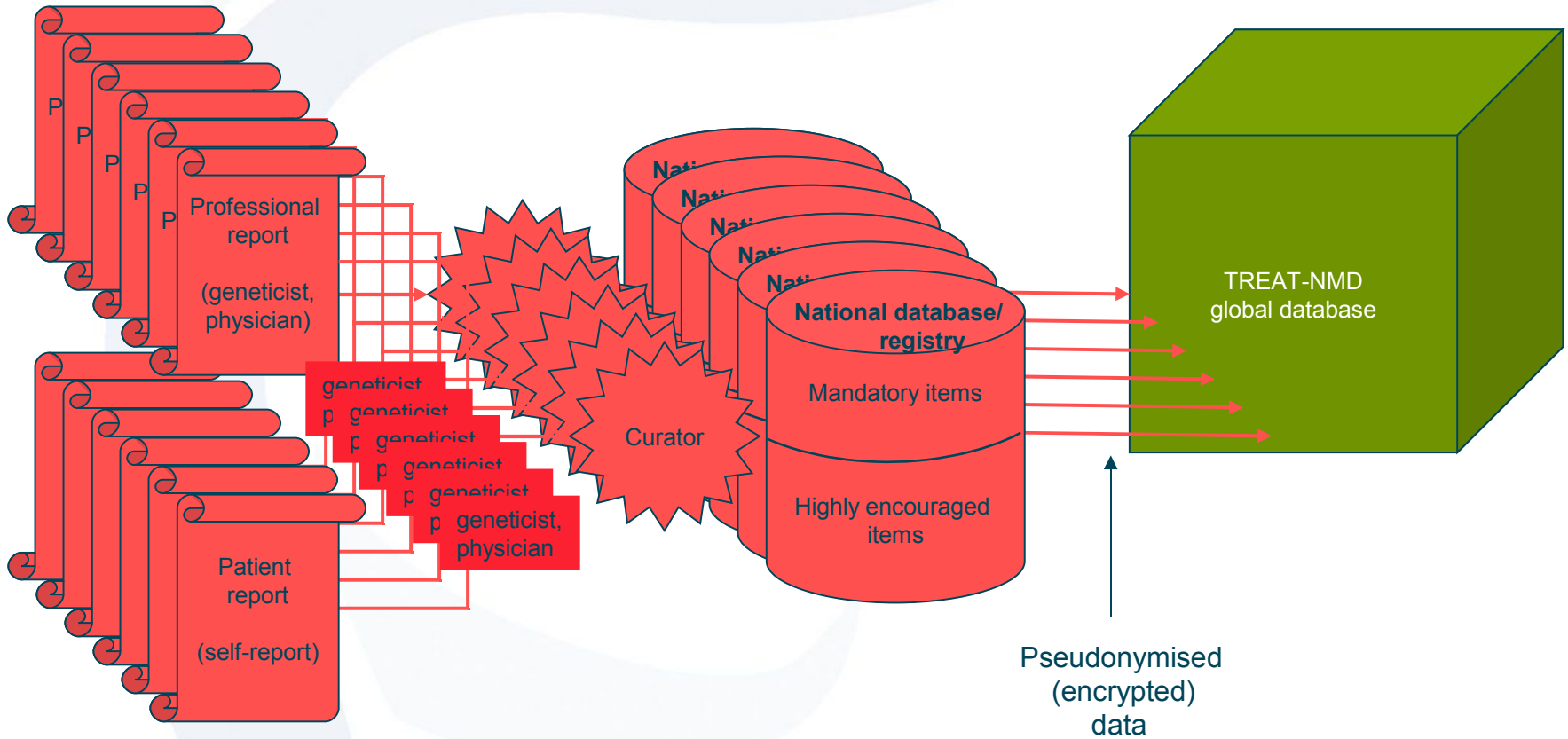
Registries for SMA: harmonized items

- Personal data of patient (name, birth date, address)
- Diagnosis (SMA, other, not determined)
- Mutation/Deletion of SMN1 gene
- Motor function: ambulation Mandatory
- Feeding function: gastric/nasal tube
- Scoliosis surgery
- Ventilatory function (Ventilator use)
- SMN2 copy number Highly encouraged
- SMA classification

Patient registries



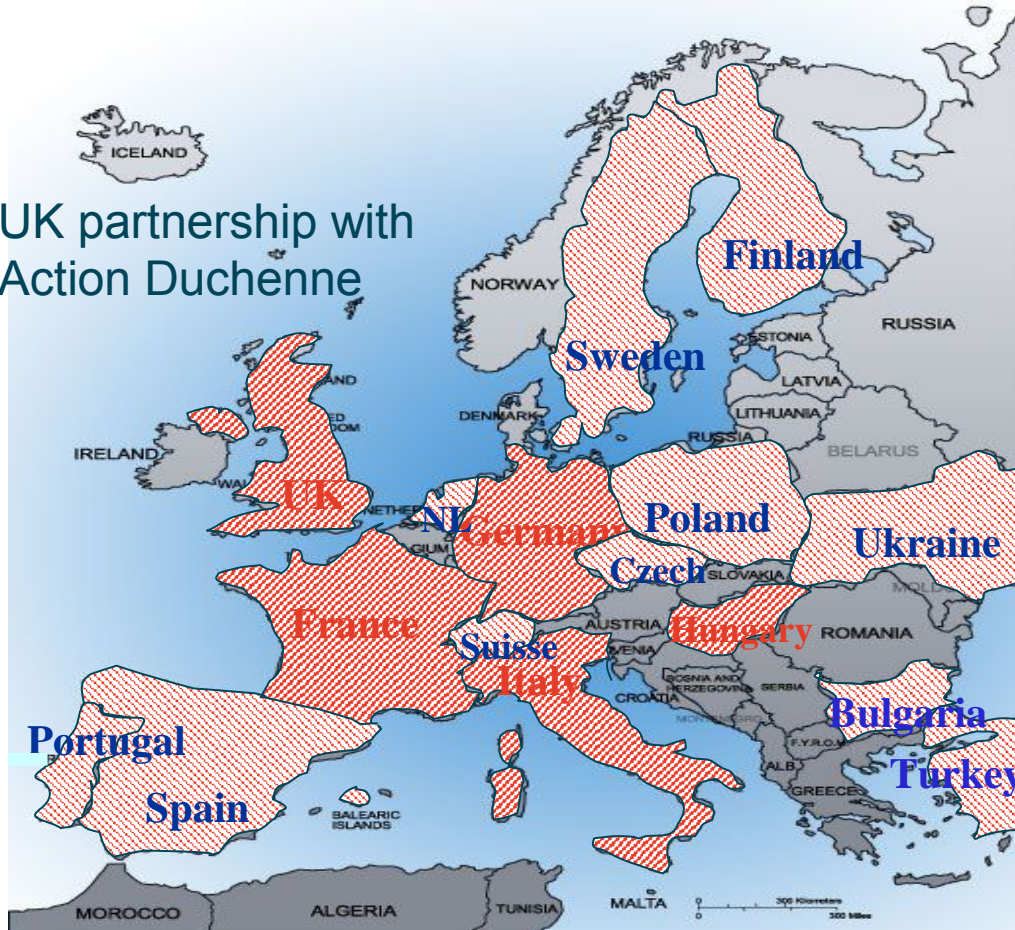
Patient registries



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Currently planned national TREAT-NMD registries on DMD & SMA (April 2008)

UK partnership with
Action Duchenne

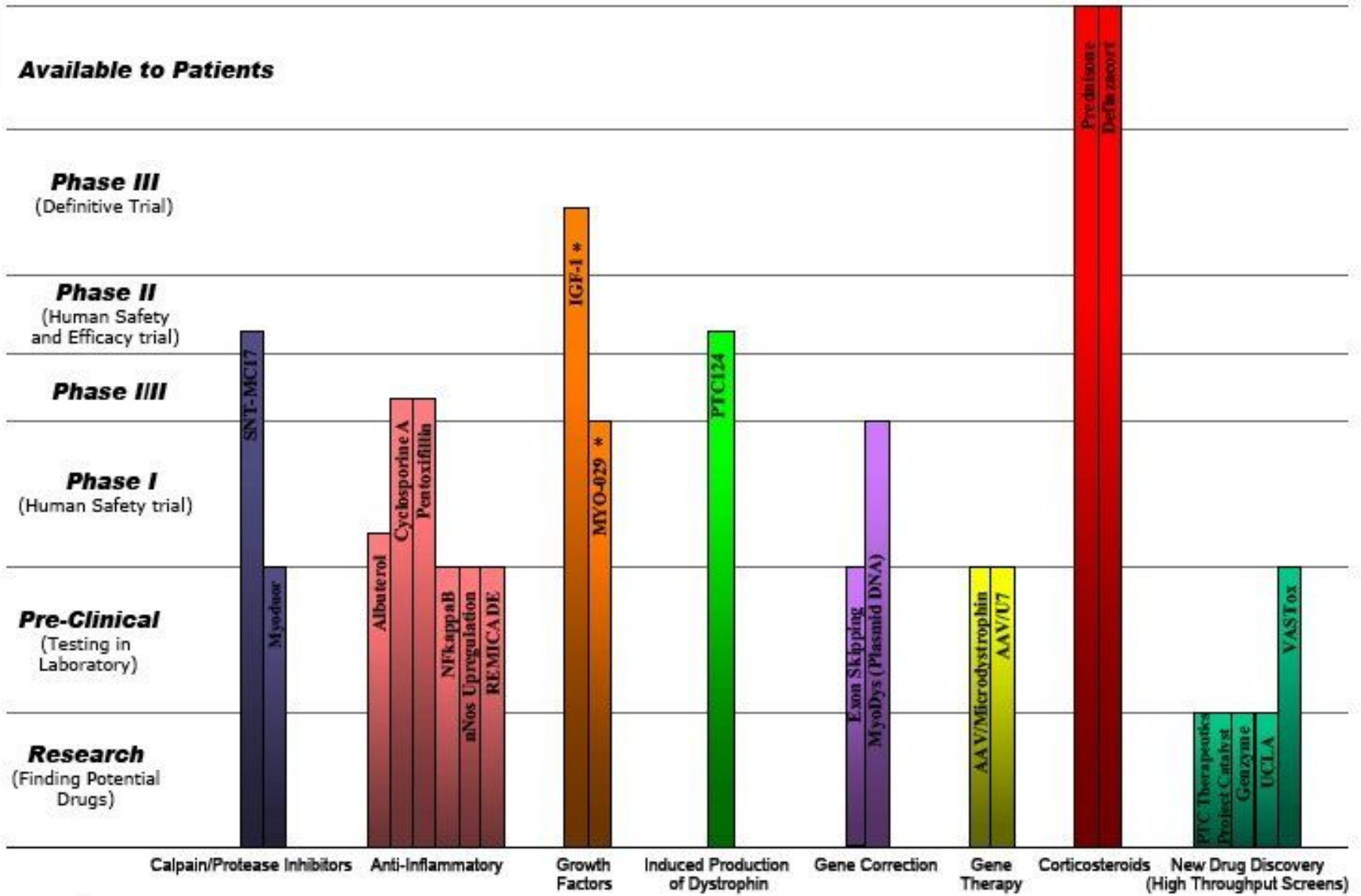


Associated
Registries:

Australia
Canada
Japan
USA

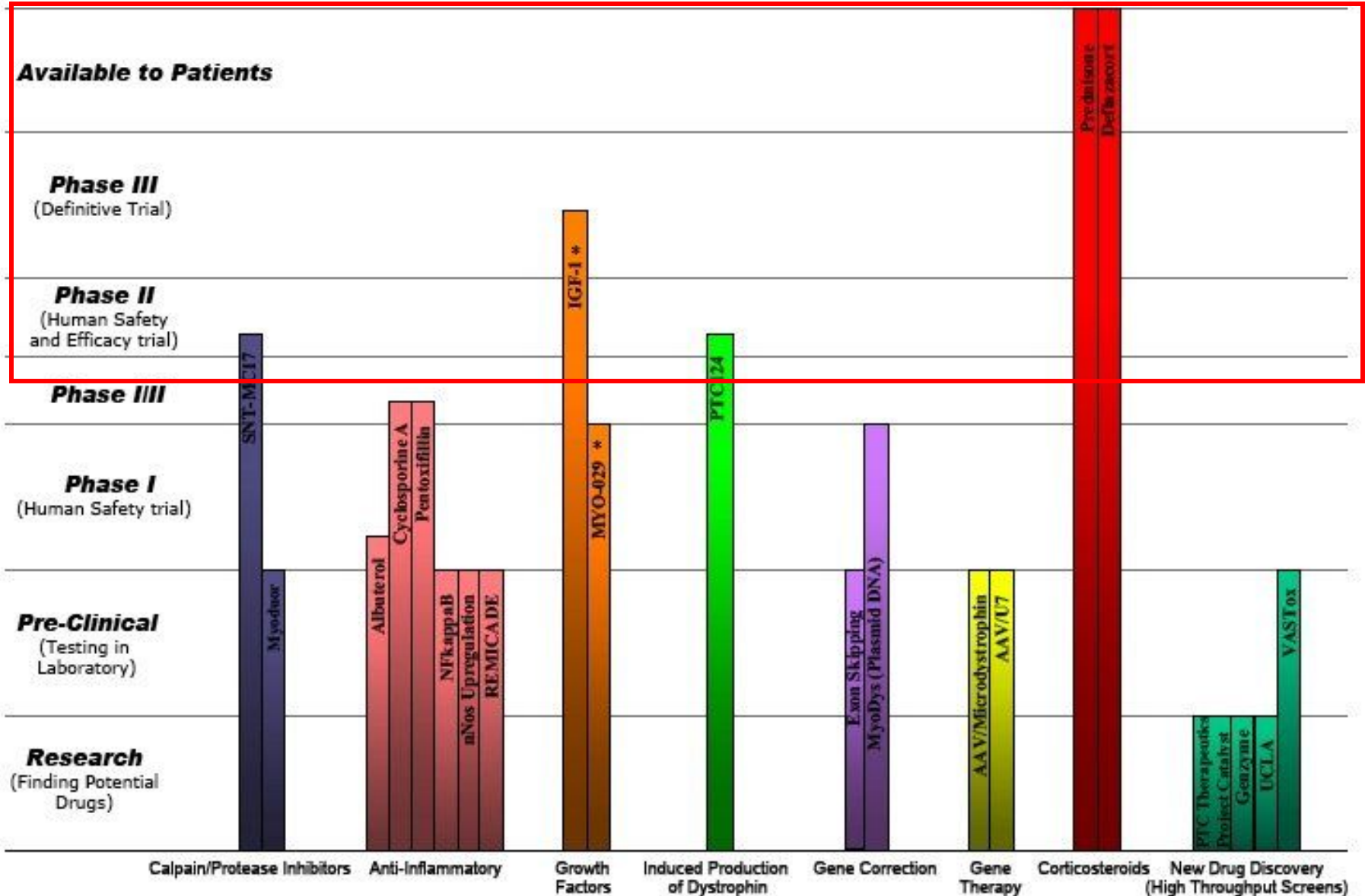


Duchenne Muscular Dystrophy Pipeline



* In Clinical Trials for other diseases, potential therapeutic benefit to DMD

Duchenne Muscular Dystrophy Pipeline



*In Clinical Trials for other diseases, potential therapeutic benefit to DMD

Kommende terapier: -vem vælger trial og på vilke grunde?

Farmaceutisk firma vælger kliniske centra

Kliniske centra vælger trial at deltage i

Enkelt person/familie vælger slutlig deltagelse

Leger / terapeuter rådgiver

Skal TREAT-NMD give "expert advice"?

Konklusion:

- TREAT-NMD arbejder for europeisk/international konsensus kring bedste nu kendte behandling
- forberedelser for kliniske studier (outcome measures, patient registries, CRCC)
- Mange kliniske trials nu eller på vej gör det viktig vælge at deltage i den rette

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Tak!

