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The TREAT-NMD Network

Rachel Thompson, TREAT-NMD Communications Officer

What is TREAT-NMD?

- A project funded by the European Commission from 2007 – 2011
- Aims to help promising new treatments for neuromuscular diseases get from the lab to the patient
- Not a research project but an “infrastructure” project
- Helping different expert centres collaborate better
- Creating the “tools” for trial-readiness in the neuromuscular field
- Improving patient care worldwide

21 core partner organisations



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Worldwide “membership”




TREAT-NMD - Treat-nmd members - Windows Internet Explorer

http://www.treat-nmd.eu/patients/membership/members/

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TREAT-NMD - Treat-nmd members

 Membership

PATIENTS & PUBLIC > MEMBERSHIP > TREAT-NMD MEMBERS

ABOUT US
NEWS
MEETINGS & EVENTS
MEMBERSHIP
Become a member!
» TREAT-NMD members
PARTNERS
OUTCOME MEASURES
BIOBANKS
PATIENT REGISTRIES
CLINICAL TRIALS

TREAT-NMD members

We are delighted to count the organisations and individuals listed below as TREAT-NMD members. Members benefit from closer ties with the network and special access to the tools and resources the network is creating worldwide, and we are working together with our members to help implement the network's goals, from improved patient care to specialist scientific training, in members' countries worldwide.

If you are interested in becoming a member, click [here](#).

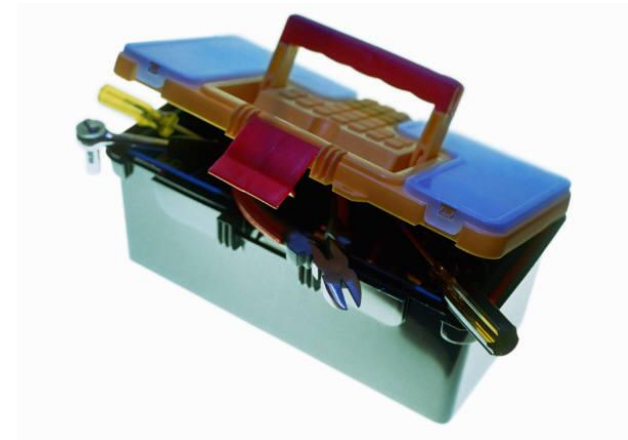
TREAT-NMD members

Name	Organisation	Country
Rodger Alexander	Neuromuscular Alliance	New Zealand

Internet 100%

Tools for trial-readiness

- § Not about individual research projects but about **infrastructure**
- § Tool-building approach addresses areas that just wouldn't get considered in normal research projects
 - Patient registries
 - Outcome measures
 - Standardization of animal models
- § All areas where international consensus and collaboration is **essential** to get things moving



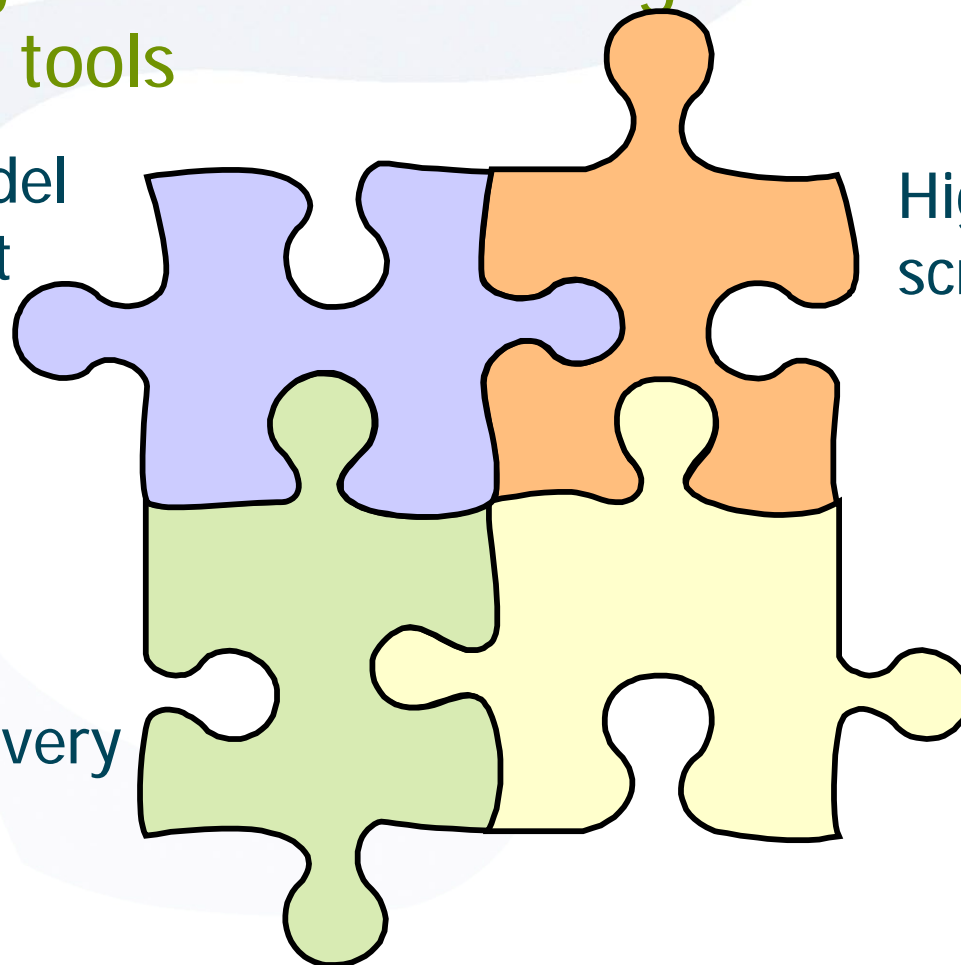
Technological and methodological tools: Preclinical tools

Animal model
assessment

High throughput
screening methods

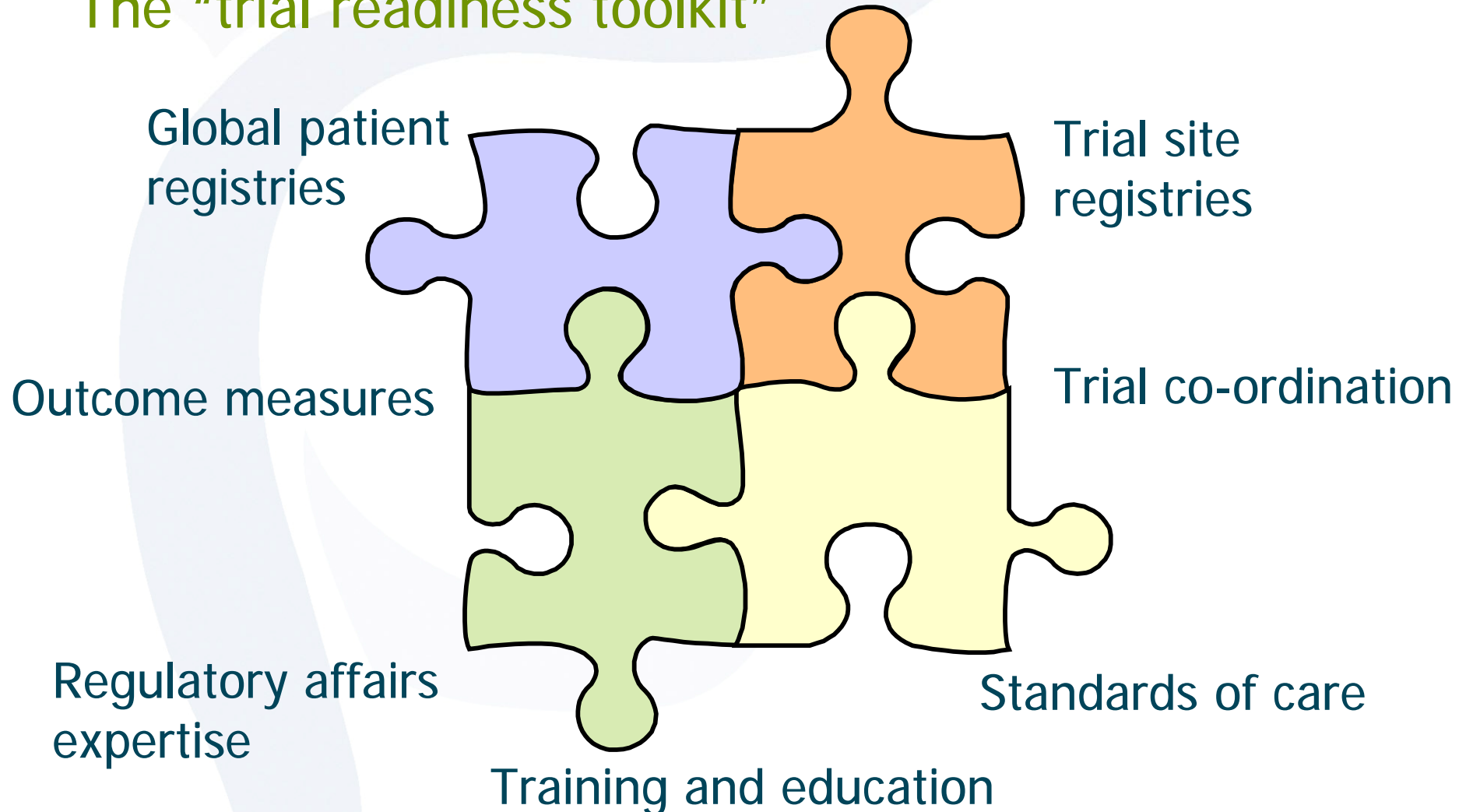
Optimising
systemic delivery
to muscle

Production
capabilities



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Technological and methodological tools: The “trial readiness toolkit”



Three particularly important areas:

- § Network of clinical trial and patient care sites
- § Standards of care
- § Patient registries

Global registries: DMD

- Pre 2007:
 - § Utah dystrophinopathy project
 - § Action Duchenne (PPUK) registry UK and Ireland
 - § French patient database
 - § Czech Parent project registry
- All independent and with different datasets
- TREAT-NMD approach
 - § Define a mandatory dataset and means to share core data
 - § Allow interface with patients to be variable from country to country
 - § Focus on the information that will be needed for clinical trials

National DMD patient registries worldwide

running

Bulgaria:



Czech R./Slovakia:



Finland:



France:



Germany/Austria:



MID-NET

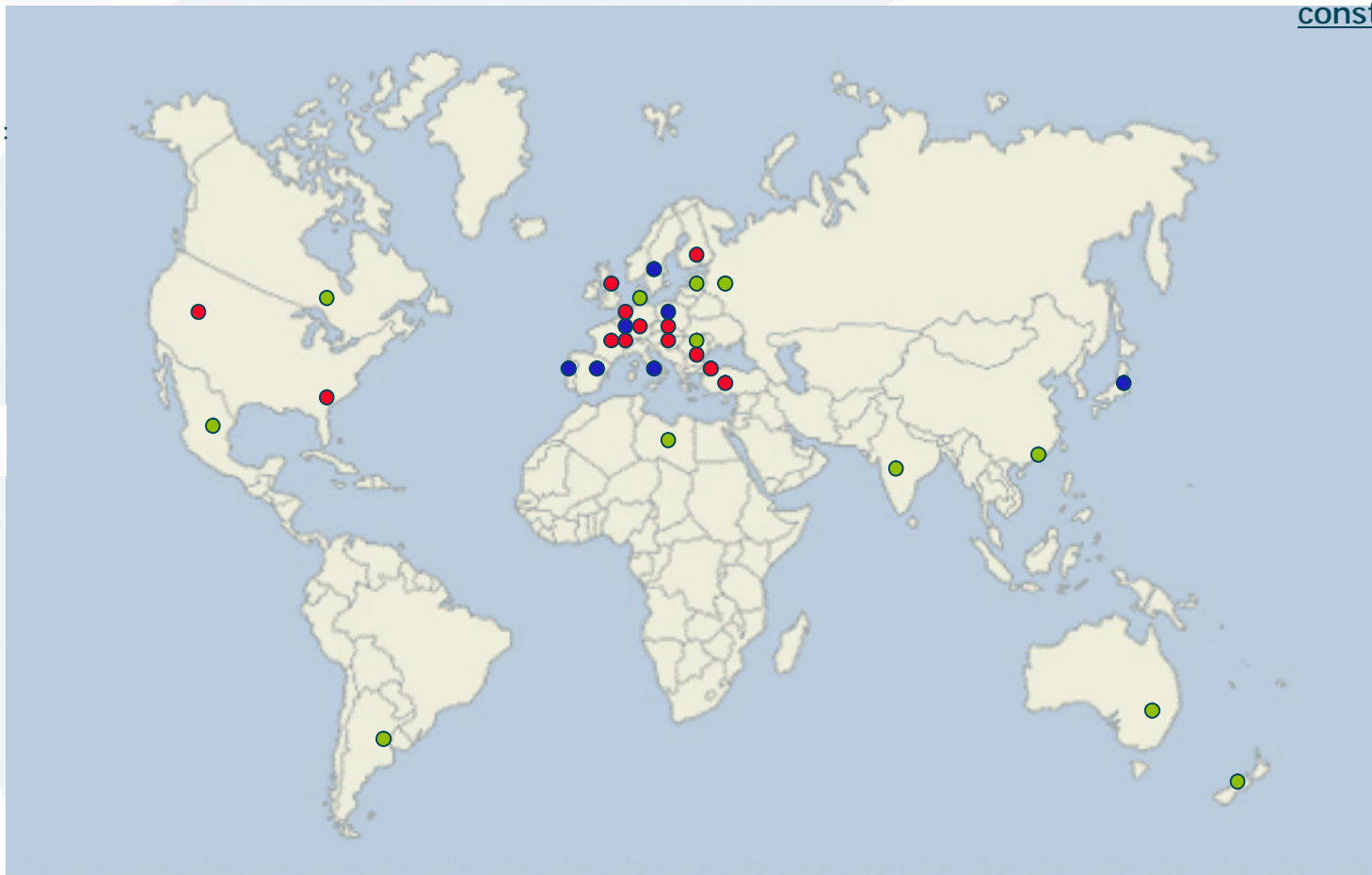
Hungary:



Switzerland:



The Netherlands:



under construction

Belgium:



Japan:



Poland:



Portugal:



INSTITUTO NACIONAL DE SAUDE

Spain:



Sweden:



planned

Turkey:



UK/Ireland: action duchenne

USA:



Algeria
Argentina
Australia

Canada
Denmark
Estonia

Hong Kong
India
Mexico

New Zealand
Romania
Russia

National SMA patient registries worldwide

running

Bulgaria:



Finland:



Germany/Austria:



Hungary:



Switzerland:



Turkey:



UK/Ireland:



Ukraine:



USA:



under construction

France:

Italy:



Poland:



Spain:



Turkey:



planned

- | | | | |
|-----------|----------------|-----------|-----------------|
| Algeria | Belgium | Denmark | New Zealand |
| Argentina | Canada | Hong Kong | Russia |
| Australia | Czech Republic | India | The Netherlands |

TREAT-NMD Global Database Oversight Committee (TGDOC)

- Formed in autumn 2008 (1st meeting on 6th September in La Grande Motte).
- Membership:
 - personal membership (not transferable)
 - each national SMA/DMD patient registry participating in the TREAT-NMD global database (they need to sign the *registry charter*)
 - other organisations, such as patient organisations
 - excluded are parties with strong commercial interests (e.g., industry)
 - list of members published on web, incl. photo & *disclosure statement*

Current TGDOC members

Pierre-Yves Jeannet
Anna Ambrosini (temp.)



Hanns
Lochmüller



Christophe
Bérout



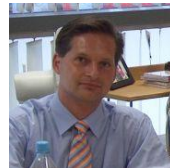
Sylvie
Tuffery-Giraud



Peter
Van den Bergh



Violeta
Mihaylova



Petr
Vondráček



Jaana
Lähdetie



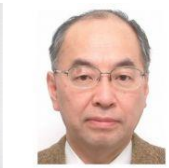
Sarah
Baumeister



Veronika
Karcagi



Filippo
Buccella



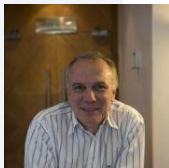
Shin'ichi
Takeda



Anna
Kaminska



M. Rosário
dos Santos



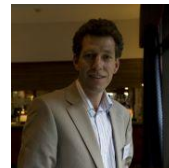
Eduardo
Tizzano



Ian
Murphy



Thomas
Sejersen



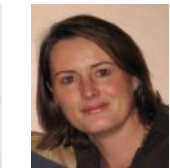
Jan
Verschuuren



Serap
İnal



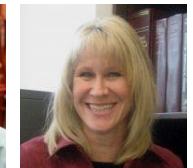
A. Ayşe
Karaduman



Pascale
Saugier-veber



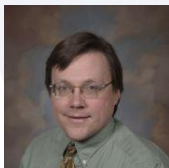
Vitaliy
Matyushenko



Jacqueline
Jackson



Vanessa
Rangel Miller



Kevin
Flanigan



Marie-Christine
Ouillade



Ria
Broekgaarden



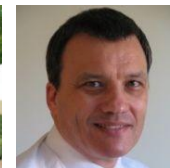
Fabrizia
Bignami



Simon
Woods



Ian Max
Huxham



Richard
Green



Janbernd
Kirschner



Nick
Catlin

Enquiries from industry: the registry is working!

AVI BioPharma, Inc., USA:

- Biopharmaceutical company specialising in the discovery and development of novel, *RNA-based drugs* targeting a wide range of important diseases, incl. DMD.
- *Phase 1 clinical trial of AVI-4658 successfully completed (21st January):*
 - *clinical trial was performed in the UK (national study)* by members of the MDEX Consortium led by Professor Francesco Muntoni
 - potential antisense oligonucleotide drug for the treatment of DMD by *skipping exon 51 in the dystrophin gene*
(to restore reading frame in mRNA sequence, thus restore dystrophin production)
 - certain types of *exon deletions* in the dystrophin gene are potentially treatable by exon 51 skipping

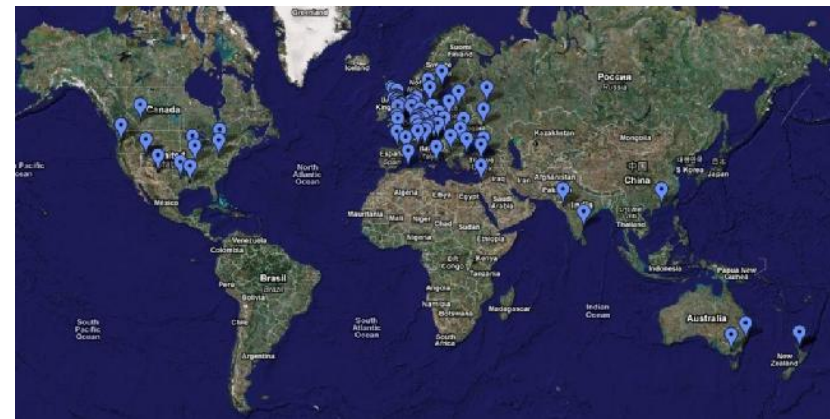
DMD exon skippable patients (patient registries)

	Exon 44	Exon 45	Exon 50	Exon 51	Exon 53	all
Belgium	11	18	11	36	14	90
Bulgaria	2	3	0	2	2	9
Czech Republic and Slovakia	8	4	6	12	13	43
France	110	138	57	154	138	597
Germany	17	7	9	32	18	83
Hungary	0	2	0	8	2	12
Italy	7	7	9	10	12	45
Japan	55	37	20	74	64	250
Poland	24	72	12	60	61	229
Portugal	9	16	7	23	7	62
Spain	18	22	13	47	26	126
Switzerland	2	5	5	11	6	29
The Netherlands	11	1	4	7	9	32
Turkey	43	38	41	87	65	274
UK and Ireland	17	24	5	30	19	95
USA	65	91	36	119	99	410
all	399	485	235	712	555	2386

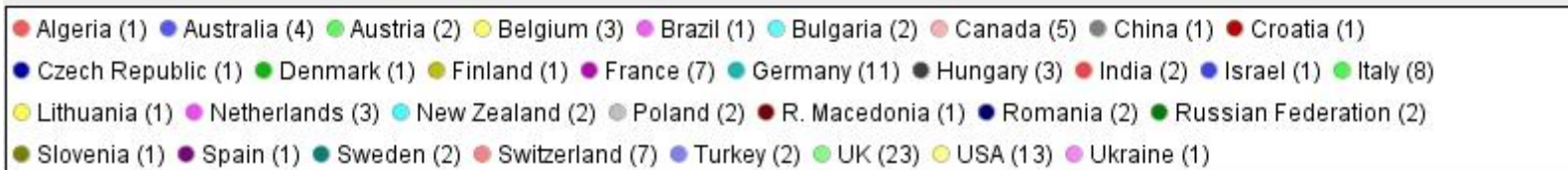
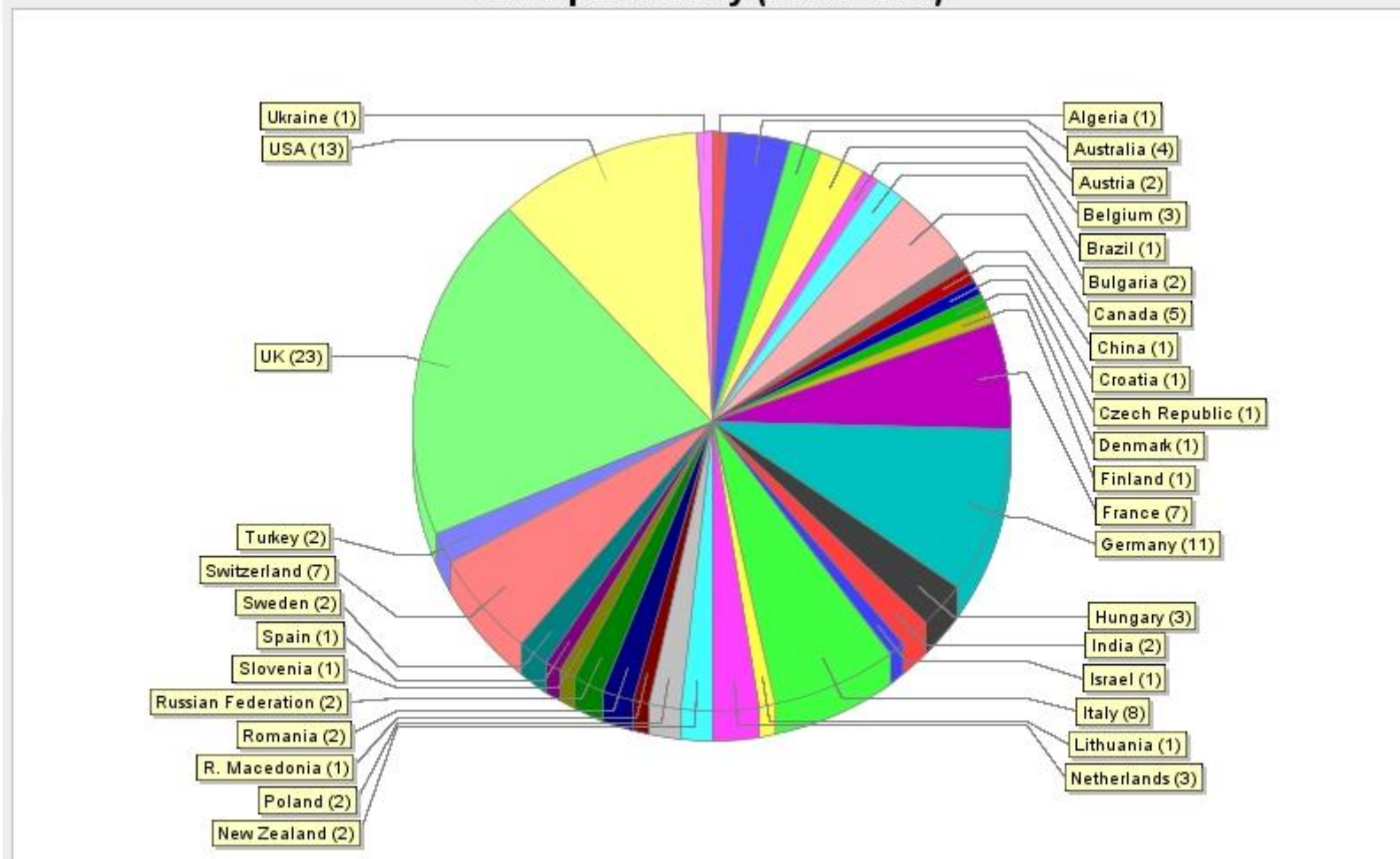
TREAT-NMD trial and clinical care sites

Managed by the Clinical Trial Co-ordination centre (Freiburg)

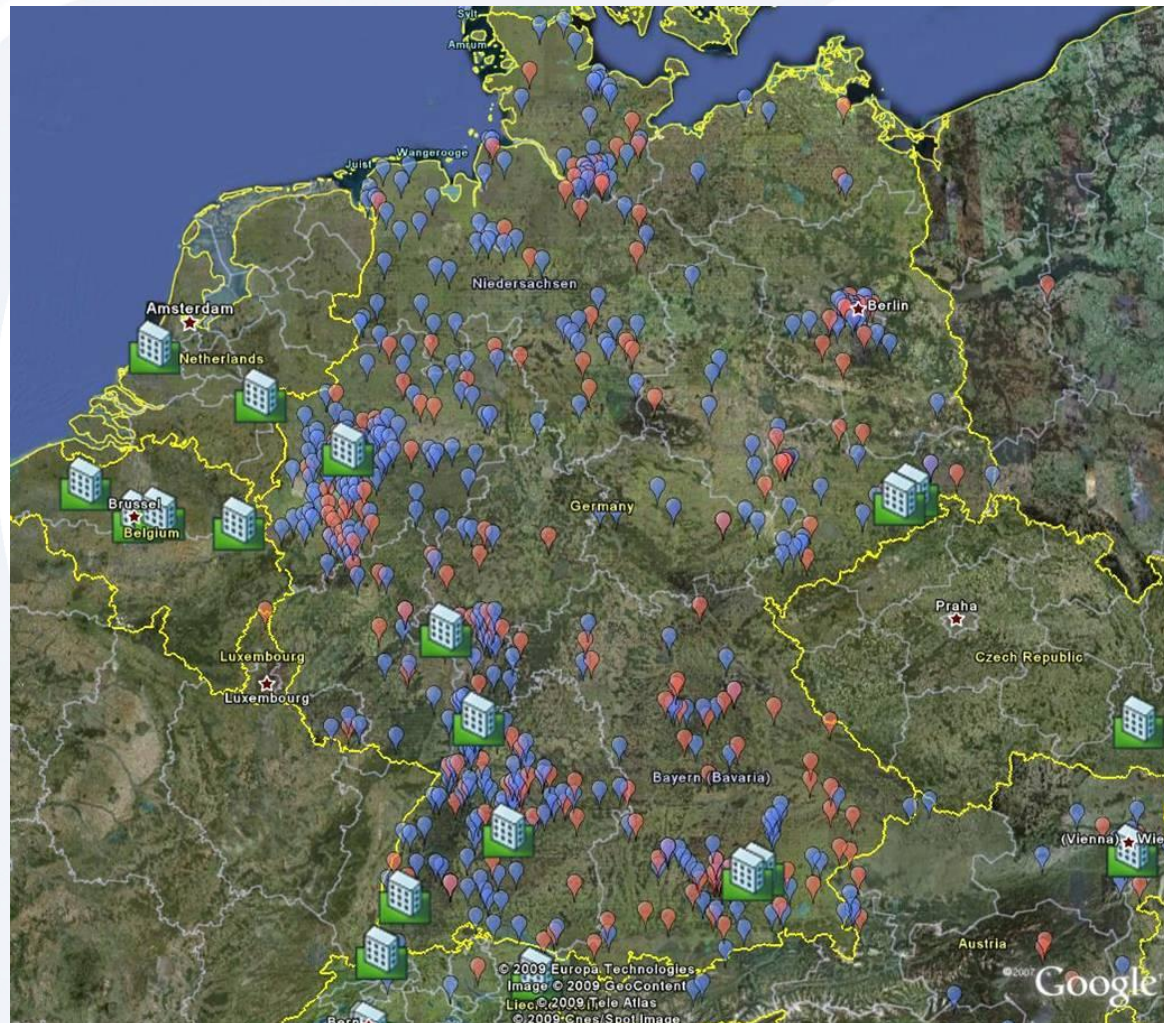
- 118 Trial Sites, caring for more than 10 000 patients with NMD
- 33 different countries
- Database contains information on
 - § Patient cohort (self-report)
 - § Trial experience and personnel
 - § Local facilities and infrastructure
- Caring for
 - § 4461 patients with Duchenne Muscular Dystrophy
 - § 2034 patients with Spinal Muscular Atrophy (Type II and III)
- Over half the sites have been identified as having previous or current experience in conducting clinical trials



Sites per country (Total = 118)

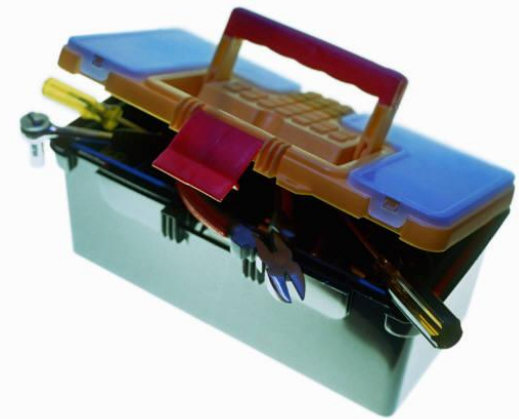


Linking trial / care sites and patients

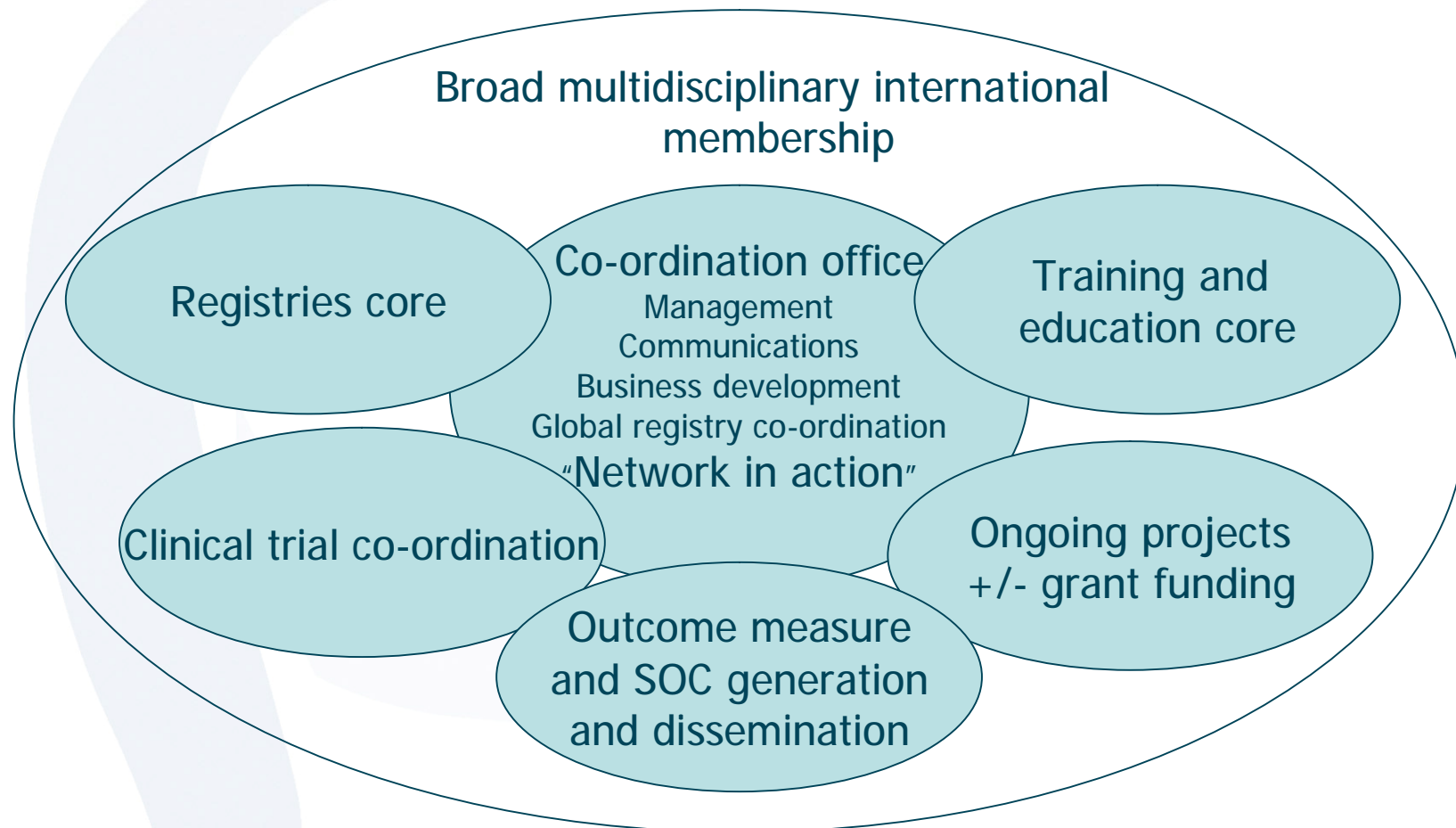


Tools for patient care

- § Even while the search for a “cure” is still ongoing, giving patients the best care makes a huge difference
- § But what is the best care?
- § A major international consensus document on DMD care is going to be published in an international journal in about 3-4 months
- § This will give guidelines that will be useful to doctors and patients



TREAT-NMD beyond 5 years: delivering treatments, ensuring optimal care



How can the Czech Republic get involved?

As doctors:

- Register in the network of patient care and clinical trial sites: www.treat-nmd.eu/trialsites
- Encourage patients to join the patient registry
- Look out for the standards of care for DMD this spring/summer
- Sign up for the TREAT-NMD newsletter to hear about conferences and training opportunities

How can the Czech Republic get involved?

As patients/parents:

- Register in the patient registry
- Look out for the standards of care for DMD this spring/summer
- When they arrive, use them as a basis for discussion with your doctor
- If there are recommendations that cannot be met, use them as a lobbying tool at a governmental level
- Sign up for the TREAT-NMD newsletter for news and information about international DMD developments

For more information

www.treat-nmd.eu

info@treat-nmd.eu