EMA Workshop on risk minimisation measures 16 September 2015

Patient perspectives on monitoring effectiveness of risk minimisation measures in the field of Multiple Sclerosis

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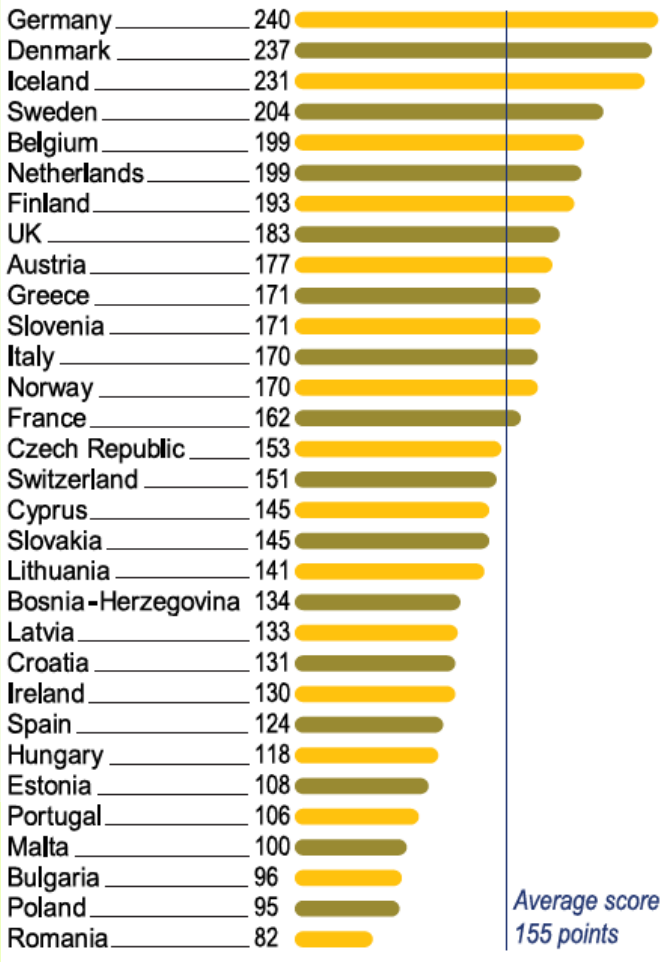
How our national data collection for analysis on European level began:

EMSP's MS Barometer – monitoring by benchmarking



How is the MS Barometer structured?

Seven priority thematic areas have been identified.The maximum score possible for each section appears in brackets.

1. Access to treatments and therapies (75 points)
2. Research agenda in MS (15 points)
3. Employment & Job retention (40 points)
4. Empowerment of people with MS (45 points)
5. Reimbursement of Costs (55 points)
6. Accurate Data Collection (25 points)
7. Medication coming to the market (15 points)

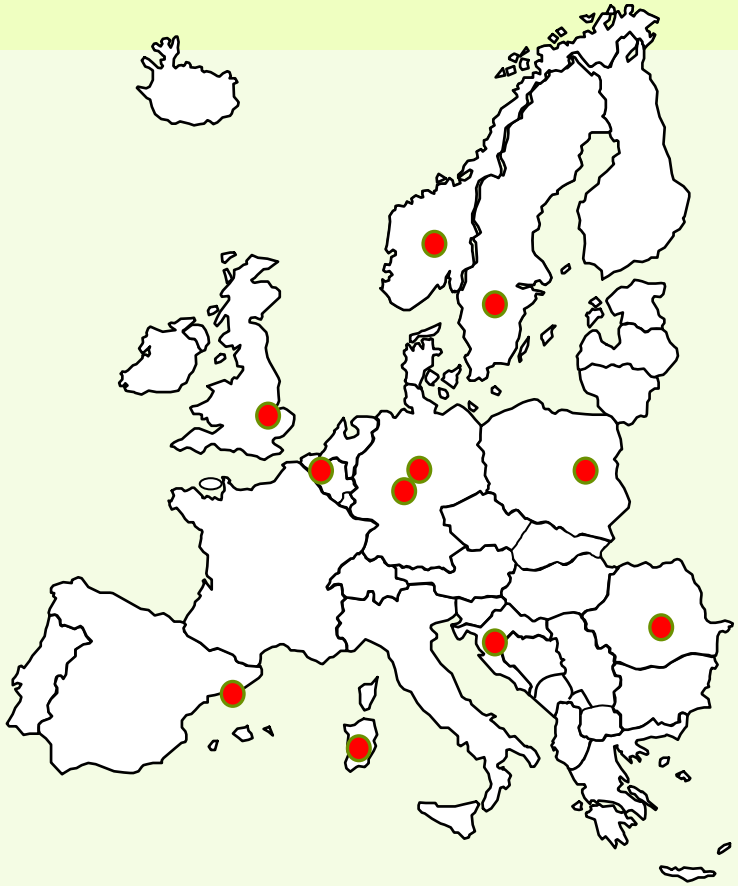
Who provides data for the MS Barometer?

The MS Barometer is completed by the national MS society in each country and endorsed by the society's Medical Advisory Board.

EUReMS project co-funded by the Health Programme 2011-2014 (European Commission, DG Sanco)

Collaborative approach to MS data: patients' representatives, Clinicians and academics

11 Project partners
incl. **5 MS Registers participating**
In 2011



Health Programme 2008-2013 Together for Health

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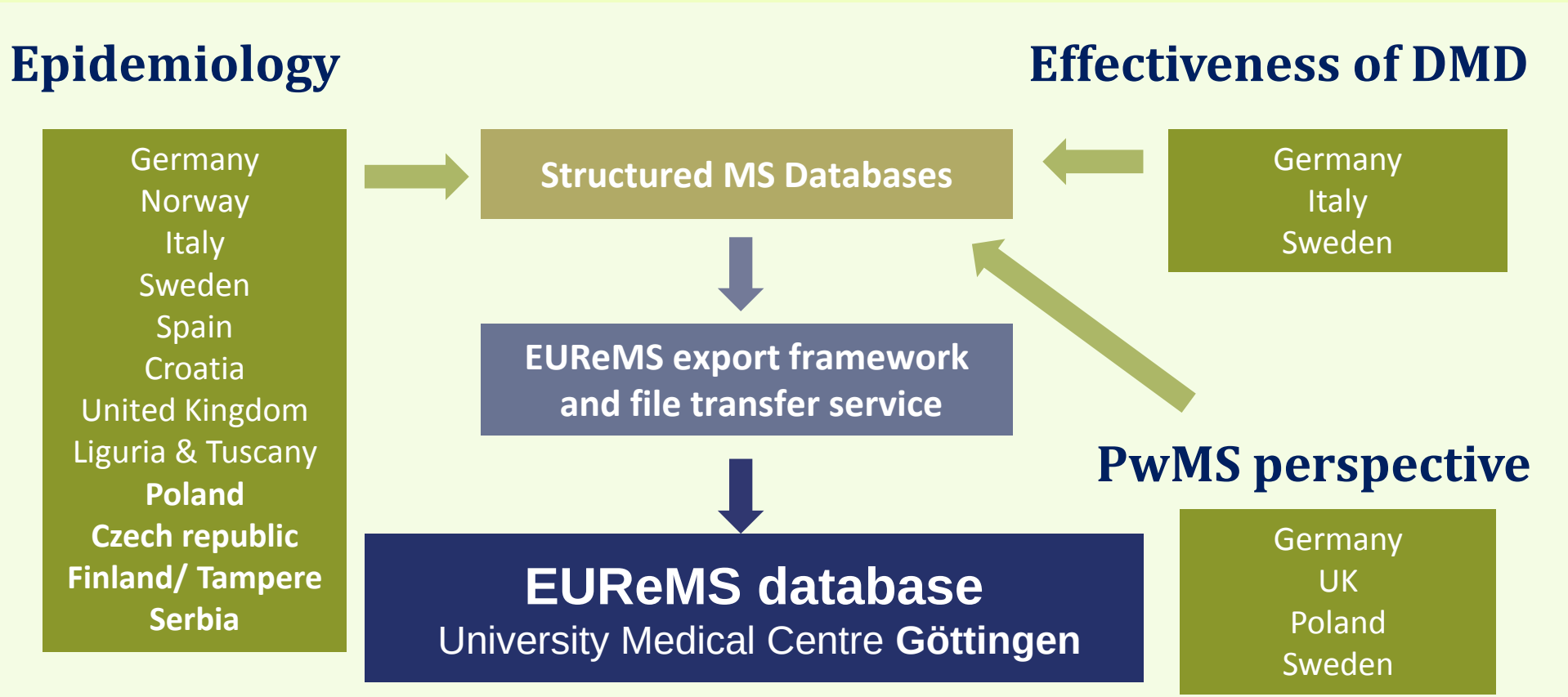
EUReMS missions

1. MS epidemiological and clinical surveillance across European countries, including the assessment of the 'MS burden' in Europe
2. Assessment of long-term efficacy, **safety** and cost-effectiveness of MS disease modifying and symptomatic treatments across European countries
3. Assessment of provision and quality of health care services across European countries, and
4. Assessment of PwMS' quality of life, the burden of symptoms and socio-economic aspects from the patient's perspective across European countries

EUReMS Core data set as starting point

Item	Description
1/ Date of birth	Month, Year
2/ Sex	Female, male
3/ Disease course	Relapse-remitting Secondary Progressive Primary progressive
4/ Year of onset	First symptoms “Onset”: terminology to be defined. Suggestion: ”Year of first symptom”
5/ Year of diagnosis	
6/ McDonald criteria fulfilled	Yes/No
7/ MRI done	Yes/No
8/ CSF done	Yes/No
9/ EDSS “describing the time course”	Number Level and time (dates) so that the development can be monitored.
10/ DMD treatments	Never/past/present With date or date for 1st treatment & type of treatment.
11/ Symptomatic treatment	Never/past/present Cannot be too extensive
12/ Working	Yes/No Income generated
13/ EQ5d	5 questions
14/ MSIS-29	2 domains

European Register for Multiple Sclerosis EUReMS 2011-2014



RESULTS by end of 2014

- From 5 data providers in 2011 to 12 in 2014
- Model contract accepted by all data providers
- Core data set accepted by all data providers
- Four Studies completed in the fields Epidemiology, DMD effectiveness and PRO
- Socio-economic data foreseen to come from various sources

EUReMS successful as a proof of concept!

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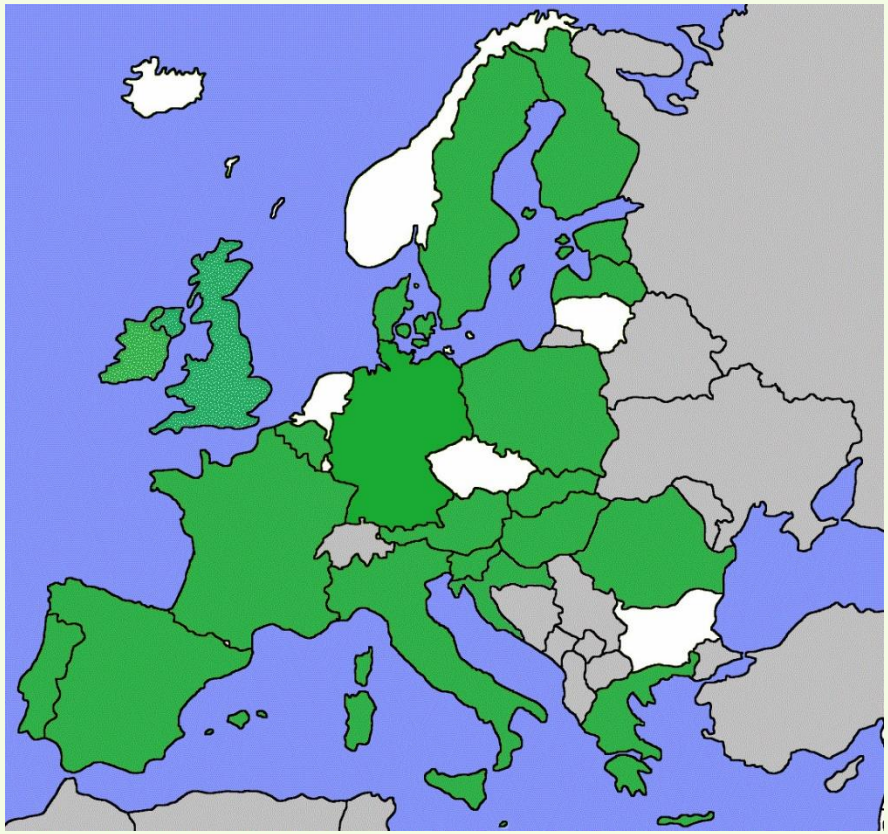
EUReMS Project 2011-2014 Outcomes

- Collaborative and geographically representative Network of MS data providers in Europe;
- Validated procedures and methodology for MS data merging;
- IT infrastructure for pooling and analysis of (pooled) MS data at UMG;
- Ethical and legal framework for cross-border MS data analysis.

PARENT, a EUReMS cooperation with European Commission & Member States: PAtient REgistries iNiTative

Aim: provide MS with guidelines and recommendations on improving registry interoperability and use of data for secondary purposes (indicators, research, etc.) -cross-border setting

May 2012 – Dec 2014
Budget: 3.4 Mio € (60% EC)
11 Associated partners
17 Collaborating partners



PARENT cross-border PAtient REgistries iNiTiative

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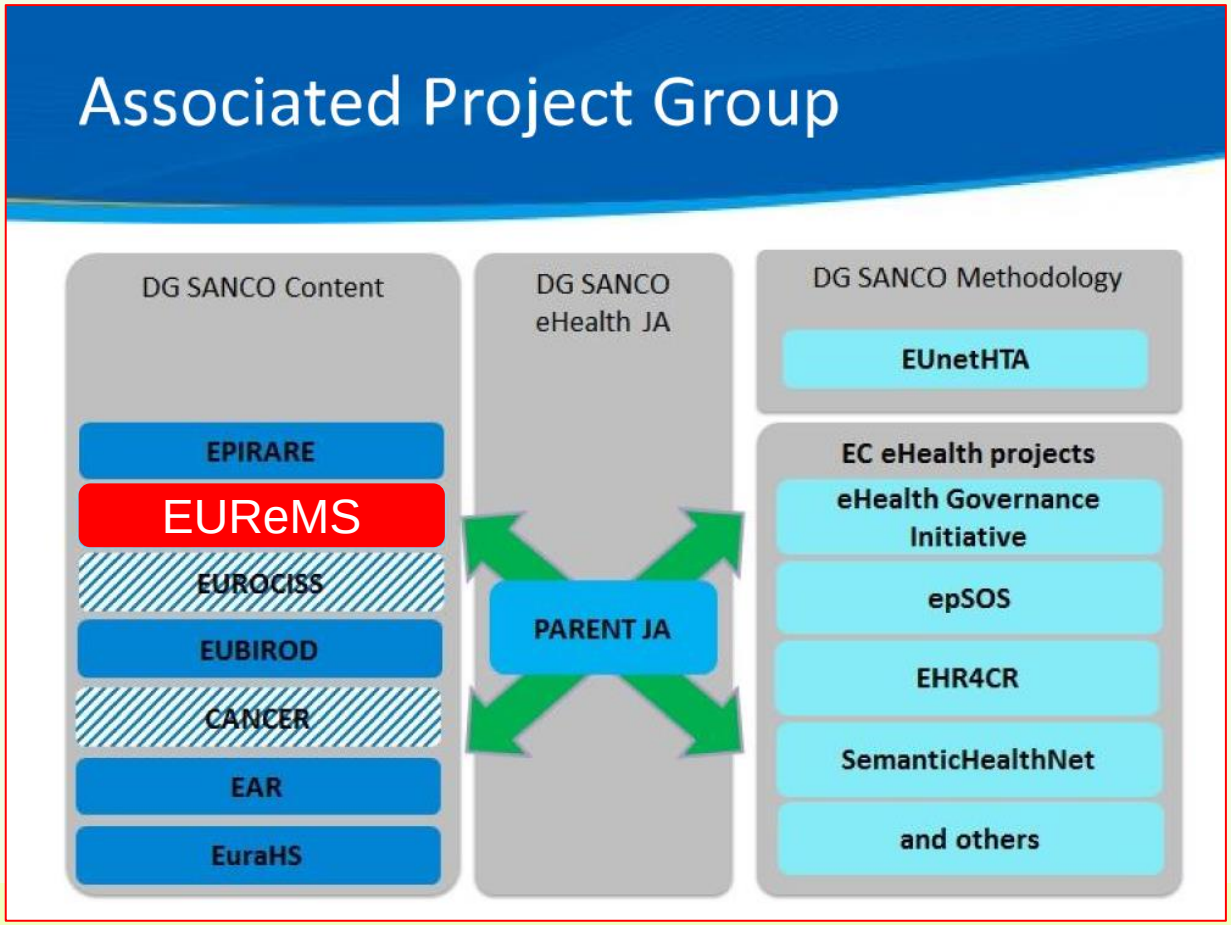
EMA: PAtient REgistries iNiTative



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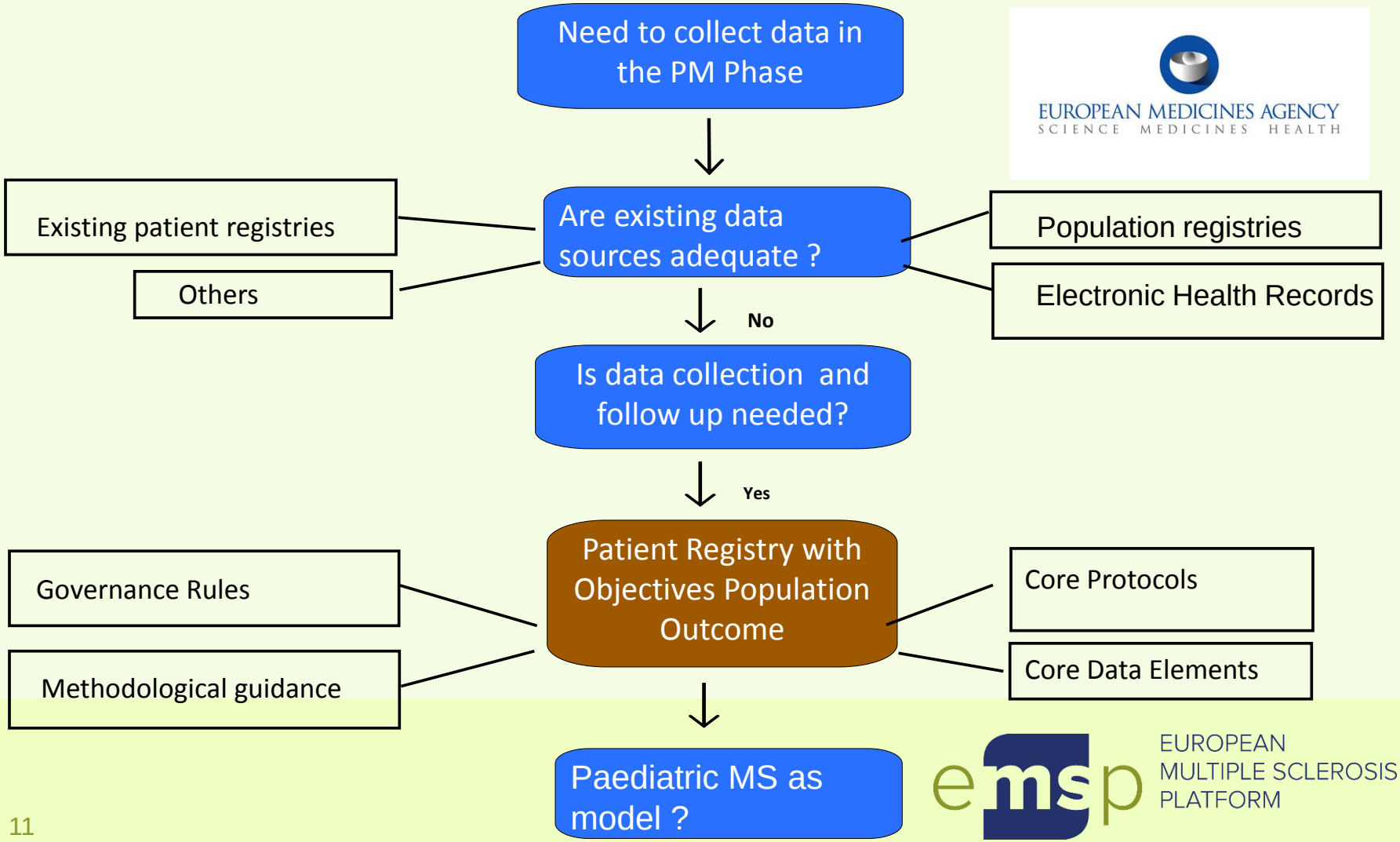
EUReMS collaboration with EMA: Cross Committee Taskforce for Patient Registries (2014-2016)



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From the "European Register for MS" to the "European Network of MS Registries"

The recently announced IMI2 call
« **Big Data for Better Health Outcomes** »(BD4BO)
could provide the opportunity to move on from EUReMS as a "proof on concept" of effective cross-border cooperation of national registries and data pooling plus centralized data analysis

Towards

a European Network of MS Registries potentially providing support to regulatory tasks such as the monitoring of risk minimization

Goal

Support the evolution towards outcomes-focused and sustainable healthcare systems, exploiting the opportunities offered by big and deep data sources

Themes/Enablers

A — Design sets of standard outcomes and demonstrate value

- Sets of target outcomes
- Clinical endpoints
- Alignment of HC stakeholders on the value of those outcomes..

B — Increase access to high quality outcomes data

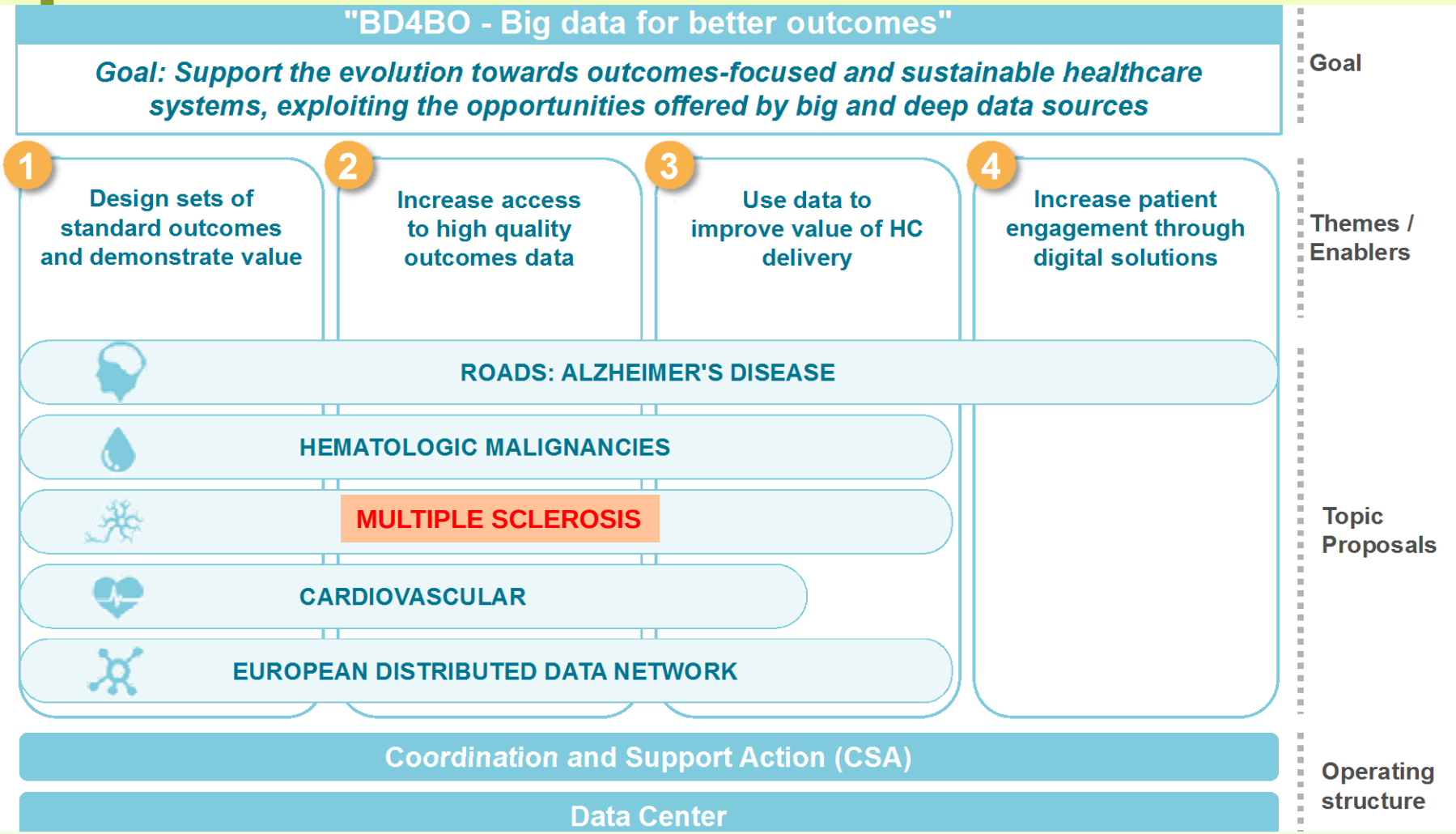
- Mapping of sources, methods and tolls for collection and harmonization
- Governance and technical standards...

C — Use data to improve value of HC delivery

- Drivers of outcomes variation
- Best clinical practices
- Methodologies to predict outcomes...

D — Increase patient engagement through digital solutions

- Patient Reported Outcomes opportunities
- Profiling patients behaviors
- Tools to increase patient engagement...



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~~EUReMS~~ /European Network of MS registries– a potential tool for risk minimisation in MS?

A risk management system is a set of pharmacovigilance activities and interventions designed to identify, characterise, prevent or minimise risks relating to medicinal products including the assessment of the effectiveness of those risk minimisation interventions.

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~~EUReMS~~ /European Network of MS registries– a potential tool for risk minimisation in MS?

On request by EMA, it currently could reach out to 12, in the near future even to twenty national registries

- To conduct sub-studies e.g. to determine :
  - real world usage (via HCP input)
  - acceptable level of risk (patient opinion via PRO?)
- To recruit investigators and sites
  - for products that need Risk Minimisation Plan

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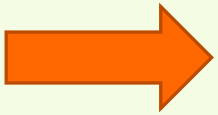
Current practice of RMM in MS

Use of special registries as part of RMP

- New Medicinal Products for MS for which Serious Adverse Drug Reactions can be expected 
  - Regulatory Body-mandated pregnancy registries for all Interferons
  - Tysabri (natalizumab) Observational Program (TOP) registry: 10yrs *(PML?)*
  - Biogen Idec Multiple Sclerosis Pregnancy Exposure Registry- tecfidera (dimethyl fumarate)- 10 yrs

Use of special registries as part of RMP e.g. Fingolimod

Preclinical studies ► Fingolimod may cause fetal harm ► RMP additional measures:

- Pregnancy prevention program:
  GILENYA(Fingolimod) Pregnancy Registry: 6yrs, multi-national, prospective, observational, >500 pregnant women collect and evaluate safety data on GILENYA
- PANGAEA : safety study of Gilenya in RRMS patients: 5 yrs
- Cardiac safety studies: REAL(Argentina), FIRST (Germany)

Dual Approach to assess effectiveness of RMM as defined by EMA

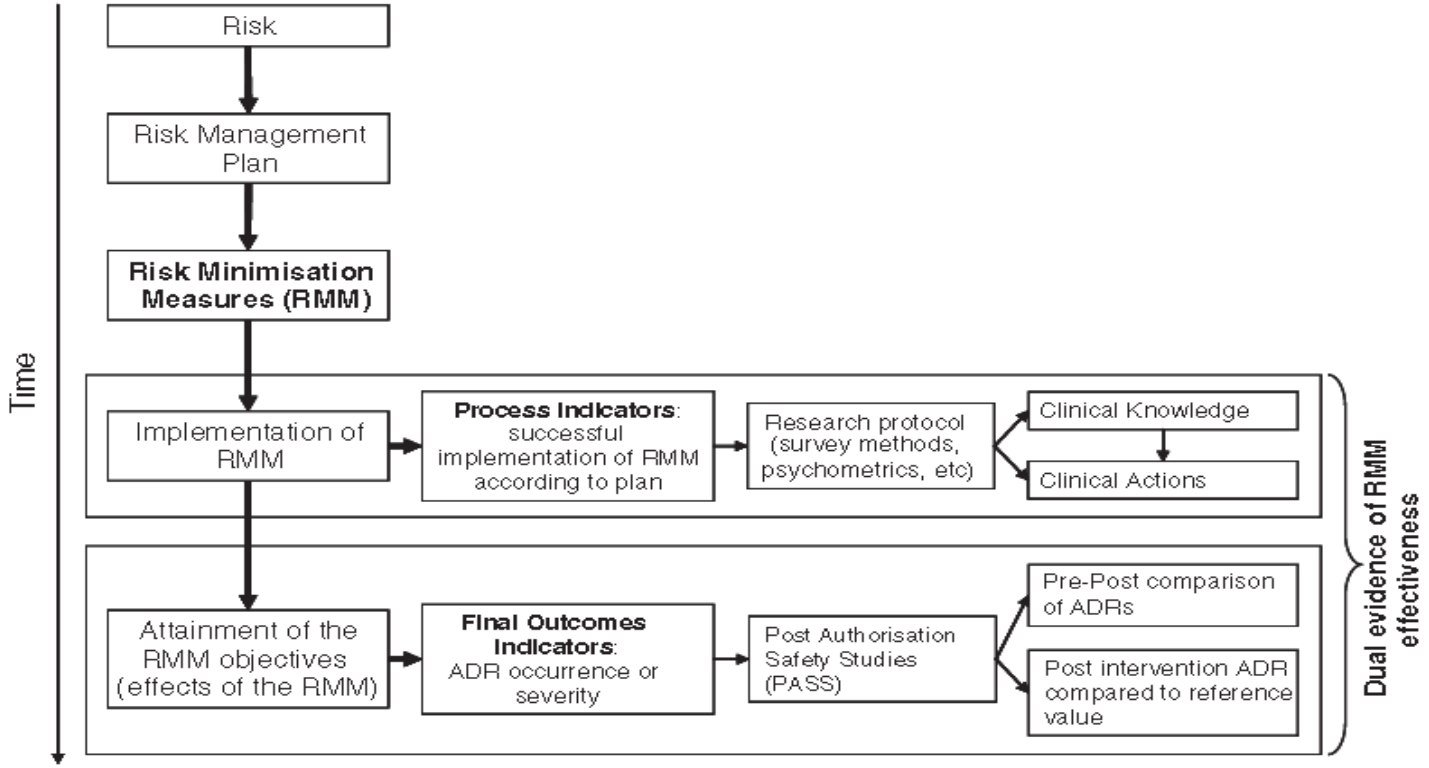


Figure 1. Evaluating the effectiveness of RMM by means of a dual-evidence approach

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Dual Approach to assess effectiveness of RMM as defined by EMA

Question to the audience:

Could European Networks of National Registries such as the one existing in MS become useful tools to help EMA with their tasks in controlling RMM? If your answer is "in principle: Yes!", which conditions would such network need to fulfil in order to match regulatory needs?

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