

Brain diseases in Europe:

MS is part of a much bigger picture

MS Facts

- 700,000 people in Europe have MS
- 70% diagnosed during prime working years
- 9 million people with neuro-degenerative diseases
- € 800 billion = cost of brain conditions in Europe

€ 1,5 million per minute*

are being spent on brain conditions in Europe



* European Brain Council, *Cost of Brain Diseases in Europe*, 2010

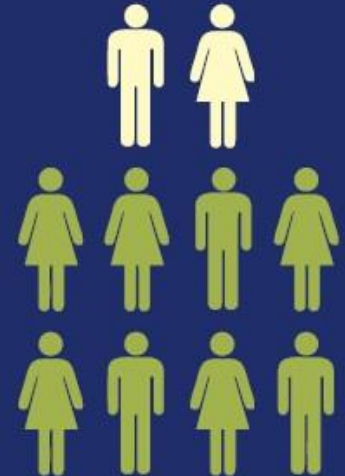
65% of the 1300 respondents to our young people with MS survey said they are currently employed or doing voluntary work ...

... but 80% usually stop working within 15 years of the onset of the disease



EUROPEAN
MULTIPLE SCLEROSIS
PLATFORM

Activity in the workplace for young people with MS



8 out of 10 stop working*

* Work Foundation, *Ready to Work?*, 2011

From the proof of concept for EMSP's European Register for MS (EUReMS) to the coming European Network of MS Registries

EUReMS achievements by end 2015:

- Collaborative and geographically representative **Network of MS data providers in Europe, capturing real-world evidence**;
- Inspiration for creation of **new national registries** already in Poland, Czech Republic, Switzerland and the UK;
- **Validated procedures** and methodology for temporary MS data merging;
- **IT infrastructure for pooling and analysis** of (pooled) MS data at Medical University Göttingen (UMG);
- **Ethical and legal framework** for central MS data analysis on the base of temporary cross border data pooling.



ncbi.nlm.nih.gov/pubmed/26611275



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Neurodegener Dis Manag. 2015 Dec;5(6 Suppl):55-8. doi: 10.2217/nmt.15.65.

Pooling real-world multiple sclerosis patient data on a European level: a true story of success.

Thalheim C¹.

• Rectangular Snip

+ Author information

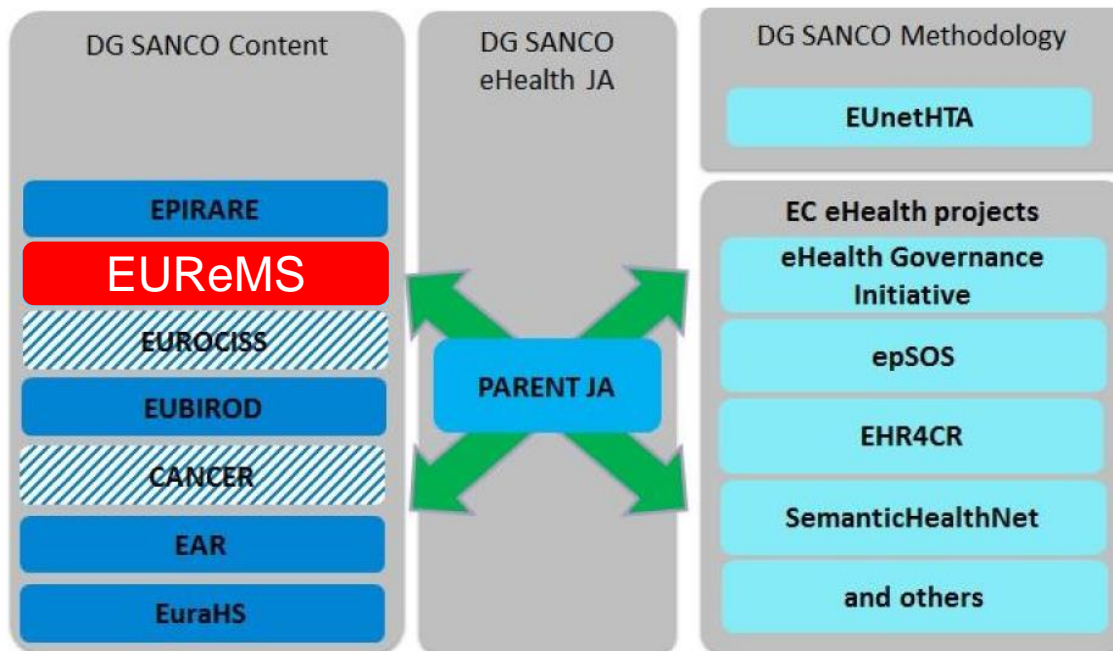
Abstract

Patient-based evidence is becoming increasingly important in budget-restricted healthcare systems as the information can help influence the direction of funds towards interventions that provide the most relevant outcomes for people living with a chronic disease. The European Multiple Sclerosis Platform (EMSP) is an umbrella organization for national multiple sclerosis (MS) patients' associations in Europe which represents the interests of >700,000 MS patients, their families and caregivers. EMSP aims to ensure the delivery of high-quality equitable treatment for persons with MS across Europe. EMSP is involved in numerous projects and activities that encompass its vision and mission. The European Network of MS Registries project has provided proof of concept that high quality MS data from previously unconnected sources can be integrated to inform research and improve patient outcomes.

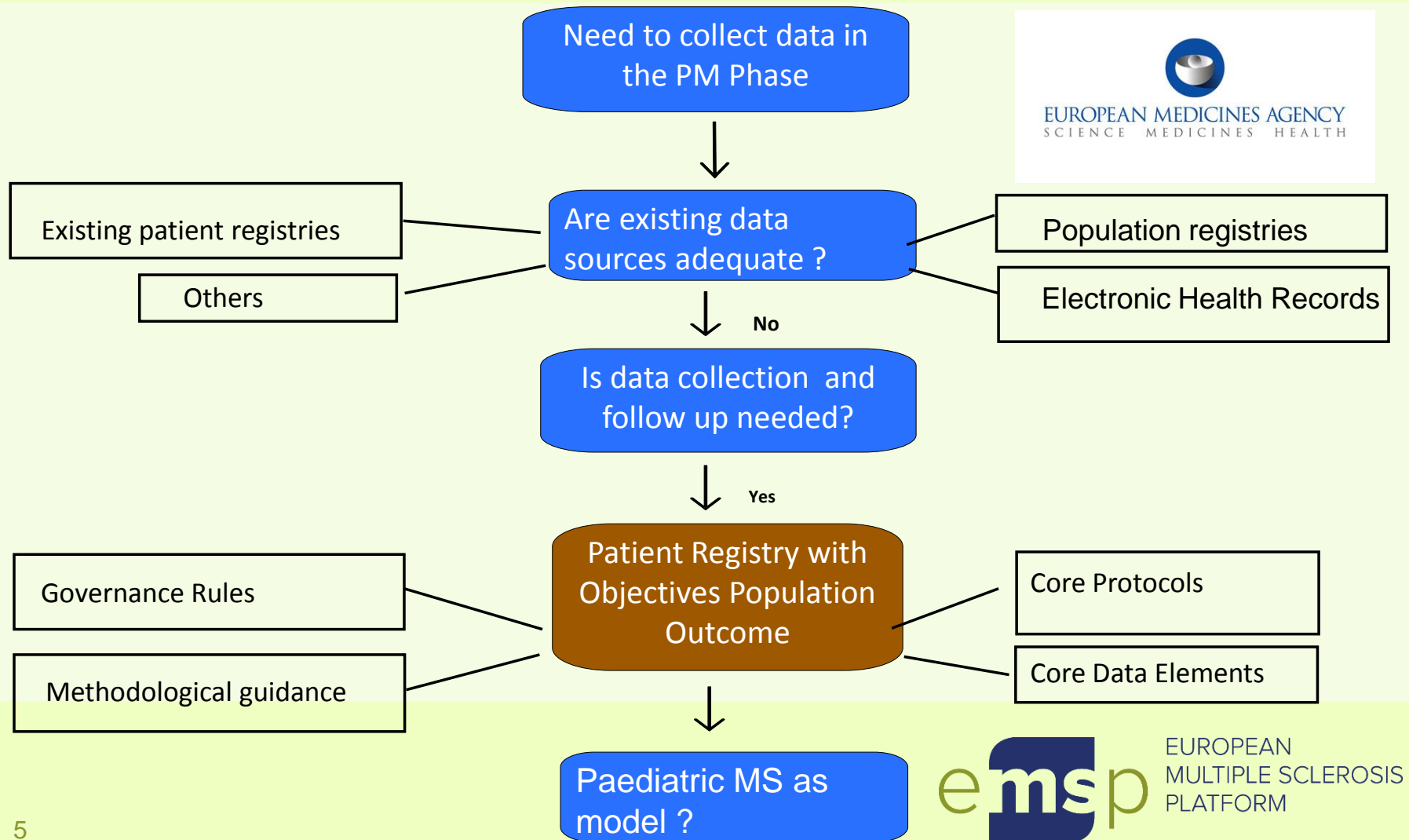
KEYWORDS: European Multiple Sclerosis Platform; European Network of MS Registries; multiple sclerosis

EMA: **P**atient **RE**gistries **iNiT**ative

Associated Project Group



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




EUREMS-/European Network of MS registries– a potential tool for risk minimisation in MS?

EMA:

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.**

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

European Network of disease specific registries – supportive tool for certain **European regulatory tasks?**

If yes – which tasks ?

- Request for MA for a new centrally approved product
- Post Authorization Safety Studies (PASS)?
- **Monitoring and optimizing risk minimization measures**
- Right treatment, right patient, right time?
- Labelling changes?
-
-

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

EUREMS-/European Network of MS registries– a potential tool for risk minimisation in MS?

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?

Our second proposal for joint action: European Network of disease specific registries – supportive tool for certain **national HTA tasks**?

If yes – which tasks ?

- Head-to Head comparison of existing with a new drug
- Patient relevant outcomes data as additional evidence base for national decision on drug reimbursement
- Right treatment, right patient, right time?
-
-

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

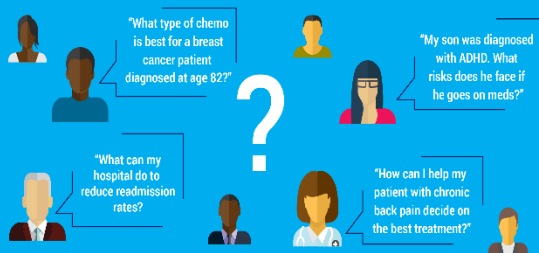
Increase patient engagement through digital solutions

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

EMSP proposal: "SINGLE EUROPEAN PLATFORM FOR PATIENT RELEVANT OUTCOMES"

- Medical data alone being derived from traditional clinical trials are no longer regarded as "golden standard", because there are not mirroring "real life" conditions, they are too expensive because the trials are too long and they rarely reflect on patient relevant outcomes
- Both clinical research and patient advocacy is longing for stronger real life evidence
- Patient Reported and Patient Centered Outcome Data are expected to grow into a major role as second criteria for future regulatory and pricing /reimbursement decisions together with clinical data from registries and clinical trials.

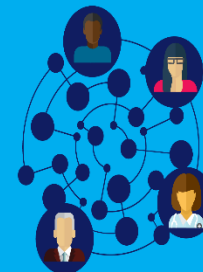
We face many important, unanswered **questions** about health care.



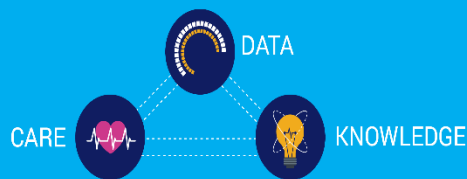
But our current research system is not set up to answer these questions in the most **useful** and **efficient** way.



We need to link **patients, clinicians, health systems, and researchers** as **partners**.



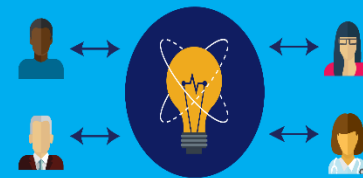
And harness health **data** to foster **knowledge** that can lead to **better care**.



Together, **partners** can decide what questions to study and how to use the data.



And get **answers** more quickly to the health and healthcare questions that matter most.



A collaborative **national resource** using the power of **partnerships** and **health data** for better research.



PCORnet is an initiative of the Patient-Centered Outcomes Research Institute

That's the vision of



With sites and partners in every state...



...And protection of patients' privacy and data security.

Patient Centered Outcomes Research network

PCORnet aims to address these issues by creating a “network of networks” that harnesses the power of large amounts of health information and unique partnerships among patients, clinicians, health systems and others.

In the process, it seeks to transform the culture of research from one directed by researchers to one driven by the needs of patients and other healthcare stakeholders.

“JOINTBRAIN” – the H2020 proposal

This is a first indicative list of WPs and might be adapted at a later stage of the proposal development.

WP-NR.	Workpackage title	Workpackage Leader
1	Coordination	
2	Governance, legal issues, ethics, IP mgmt.	
3	MS registry meta data base – Integration	
4	Existing data 1 (Course of MS)	
5	Existing data 2 (Pharmacoepidemiology)	
6	Existing data 3 (PCOs- patient centred outcomes)	
7	Prospective cohort, bio-specimen, MRI, cognition	
8	Validation of PCOs (PROs, PREMs, hQoL/novel)	
9	Expanding the base for European MS research: more and better registries	
10	A European platform for PCO collection	
11	Device generated PCOs	
12	Support to regulatory tasks by patient registry data (European level)	
13	Support of HTAs tasks by patient registry data (National level)	
14	Plan of sustainability	

www.emsp.org

Thank you!

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