

# PROPOSAL and DISCUSSION

Towards a “European One-Stop Provider” of aggregated RWE data in Multiple Sclerosis for regulators, payers, academia and patient advocates ?

Prepared by: Christoph Thalheim, EMSP

For info and discussion

February 21, 2017

## From the “European Register for MS” to the “European Network of MS Registries”

Can the analysis of « **real life** » **patient data on European level** provide some of the answers required by medical researchers, regulatory bodies, health care payers and patient advocates?

## From drug specific to disease specific registries – supportive tool for certain national regulatory and/or HTA tasks?

- YES – German IQWiG asks DMSG for data support derived from their MS Patient Registry
- YES – French NCA recommends OFSEP as data collector / data provider to pharma companies
- YES – Australian-based multinational registry MS Base collects clinical trial data for MS drugs on behalf of pharma companies as base for MA decision (in a cross-border approach to many MS Centers)

# From drug specific to disease specific registries – supportive tool for certain national regulatory and/or HTA tasks?

## If yes – which tasks ?

- Request for MA for a new centrally approved product (CAP)
- Post Authorization Safety Studies (PASS)?
- Monitoring and optimizing risk minimization measures
- Right treatment, right patient, right time?
- Labeling changes?
- .....
- .....
- Cost efficiency in the context of Conditional Approval by HTA?
- .....

# Could the “Internet of Things” in healthcare become one avenue towards vital PRO data?

## HEALTHCARE

### Meet the IoT-Powered Remote Patient

According to a 2015 report from Market Research, healthcare IoT will be worth \$117 billion by 2020. The growing industry hopes to significantly improve preventative and remote care for patients around the world.



## Registries could become the central (national) point of disease specific multi-source data collection...

- IMI projects such as RADAR-CNS (<http://www.radar-cns.org/>) are currently exploring the potential of wearable devices to help prevent and treat depression, multiple sclerosis and epilepsy – BUT:
- Where will those data be stored and linked to other health care data of the same patient?

# *EMSP's vision for Real World Evidence in Multiple Sclerosis – created through a European Network of MS Patient Registries*

## **1) Introduction of standardized core data sets with a necessary minimum of**

- **Clinical data including safety related data**
- **Patient Reported and Patient Relevant Outcomes data (PRO)**
- **Data being relevant for “Cost of Illness/ Burden of Disease” studies every 5-10 years**
- **Rehabilitation data (overlapping with PRO data?)**
- **Other data to be added?**

**2) Temporary pooling and central analysis on European level of MS relevant data coming from multiple sources such as Patient Registries, Electronic Health Records, Health Insurance Data and others (pharma industry owned data?)**

**3) Coordinated by a European Registries Network Board, consisting of representatives patient registries, cohorts and databases in MS – run in close cooperation between academia and patient advocates and supported by stakeholder advisory boards (**

*EMSP's vision for Real World Evidence in Multiple Sclerosis – created through a European Network of MS patient registries*

**4) Driven by specific queries** coming from

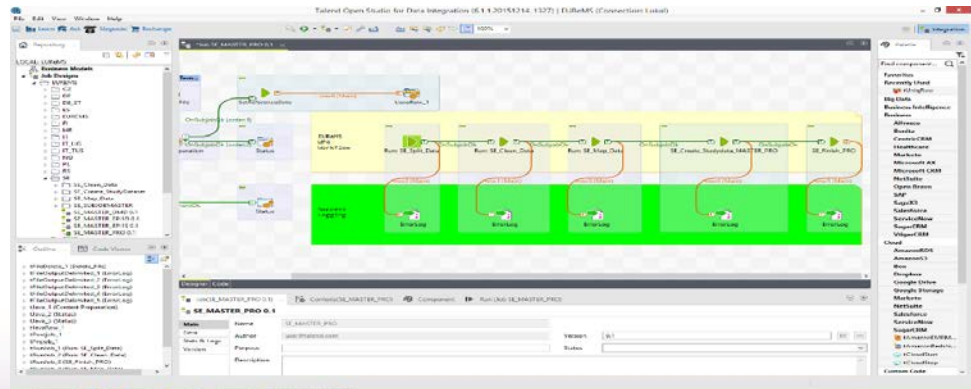
- **Regulatory bodies and /or Healthcare Industry**
- **HTA Agencies and Payers**
- **Researchers and Health Care Providers**
- **Patient Advocates**

**5) Sustainably financed** – through a public/private partnership by industry & national government funds



# Better RWE through data pooling

- Over the past years, up to 13 national MS registries have been working together in four studies, based on common agreements for core data sets.
- Data were fed cross border into a central analysis center in Germany to produce answers to researchers'- and to patient-driven questions
- As a „proof of concept“, we already made the network of European MS registries happen through EUREMS!



A first attempt of 18 project partners towards H2020 brought lots of learnings, but no funds 😞

- “Optimized exploitation of patient registries, cohorts and databases in Europe – Multiple Sclerosis as exemplar for patient centred & evidence based health care”

NOT  
Approved

~~OptiMuS~~

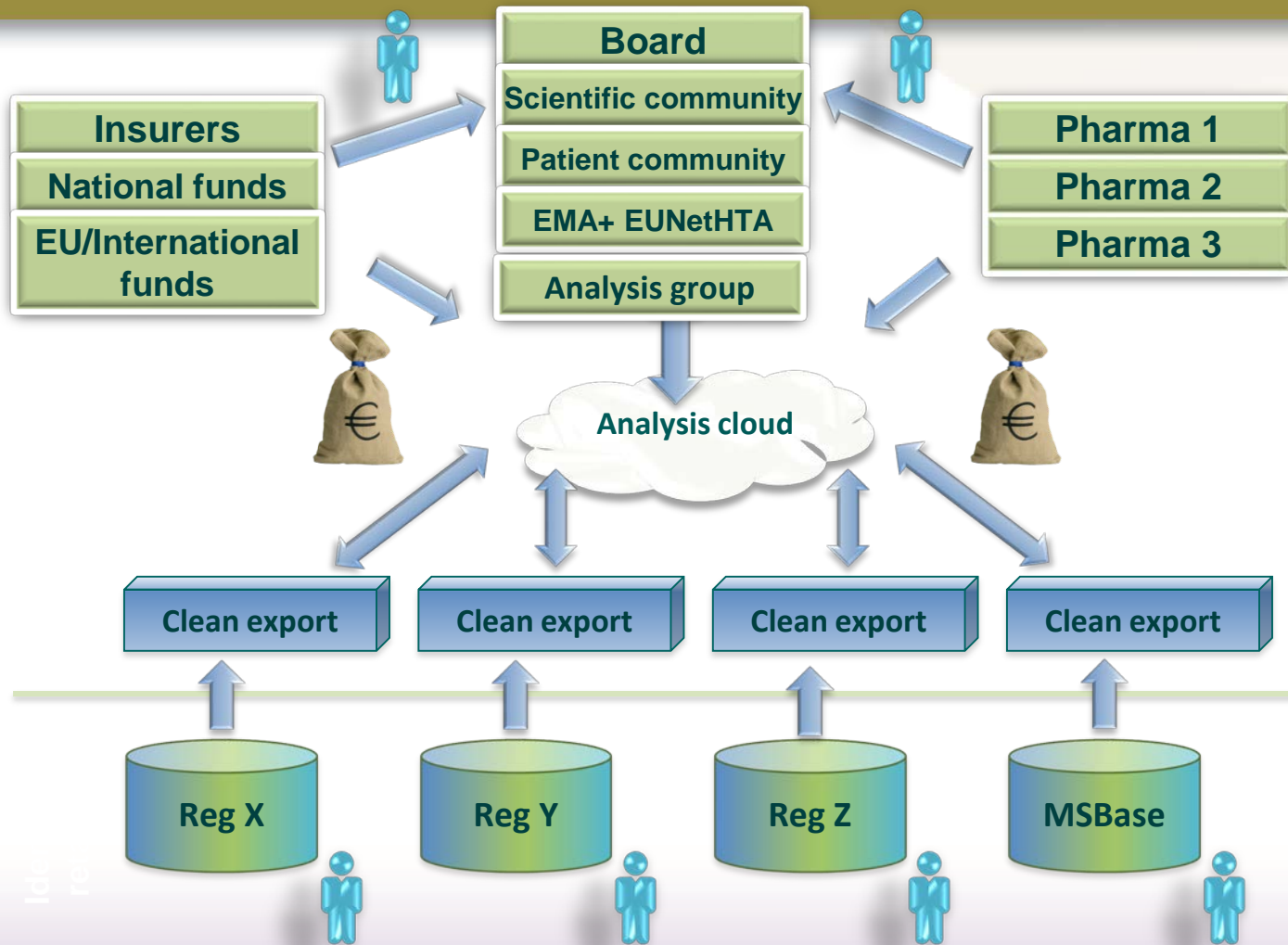
Our improved new proposal:

**EuNetMuS** –

European **Network** of

**Multiple Sclerosis** Registries

# How could a truly inclusive European Network of MS Patient registries be structured?

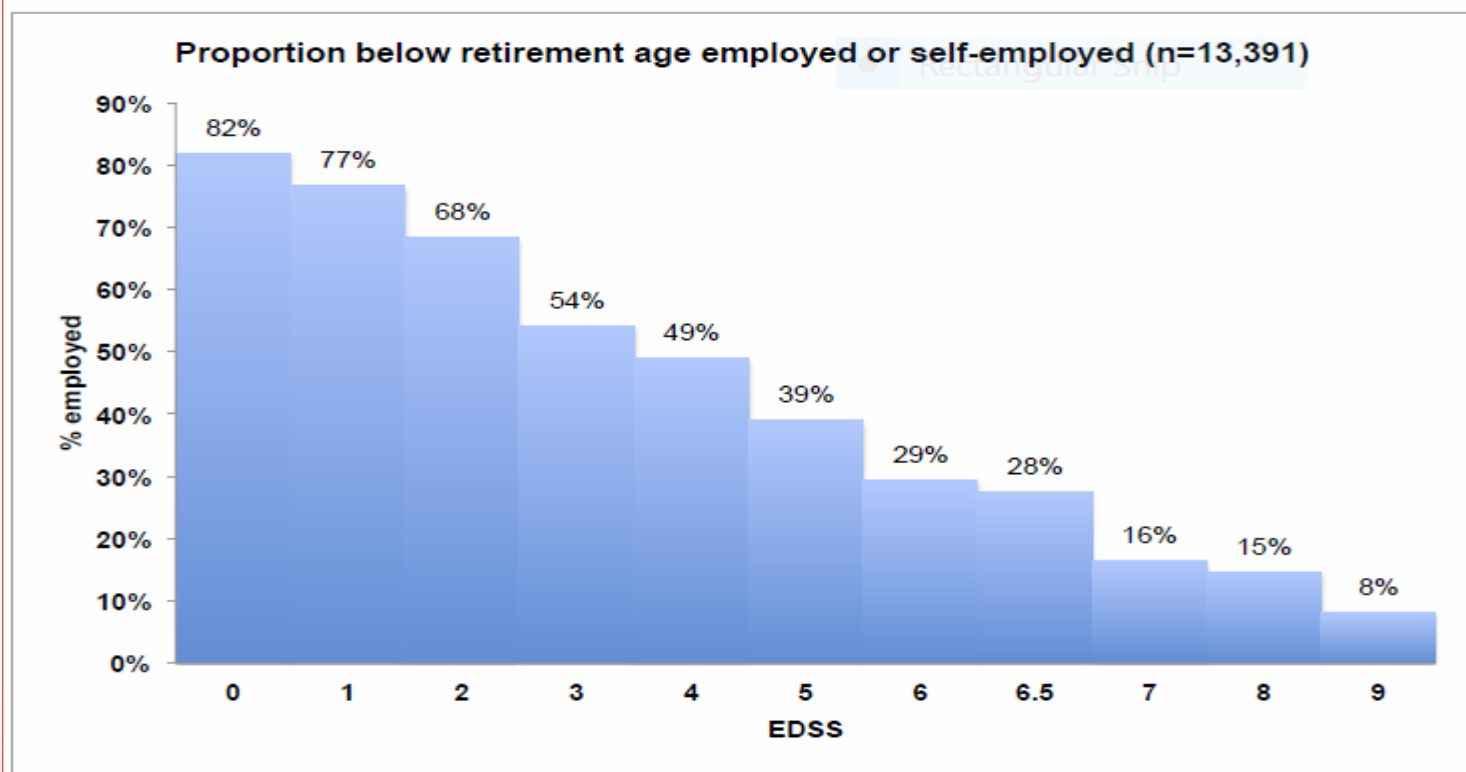


*Data of highest possible patient relevance:  
e.g. The Neuro-QoL PRO questionnaire*

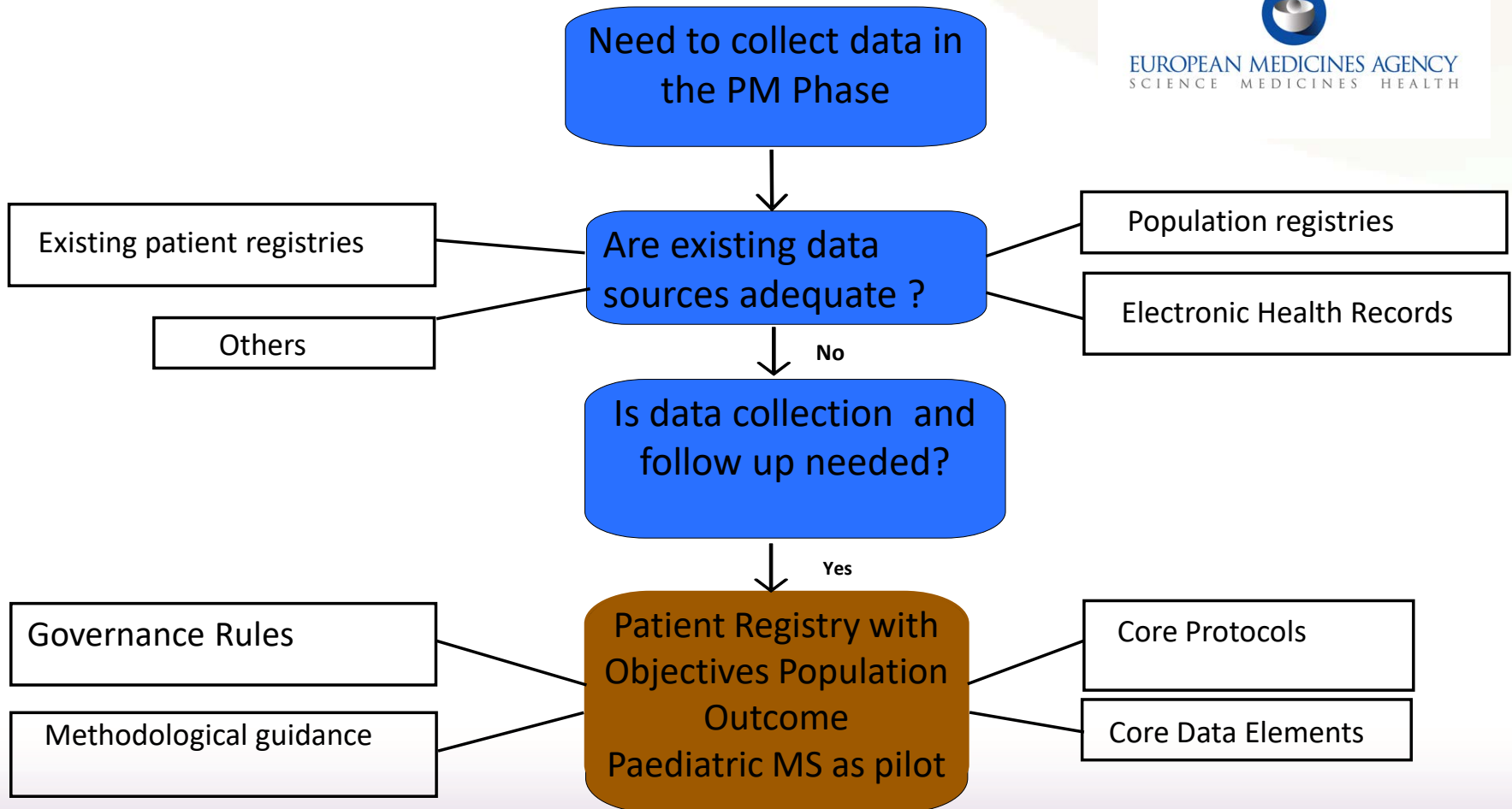
Physical Health	#	Mental Health	#	Social Health	#
Upper Extremity Function	8	Anxiety	8	Ability to Participate in Social Roles/Activities	8
Lower Extremity Function	8	Depression	8	Satisfaction with Social Roles/Activities	8
Fatigue	8	Positive Effect & Well-Being	8		
Sleep Disturbance	8	Emotional & Behavioral Dyscontrol	8		
Bowel Function	*	Cognitive Function	8		
Bladder Function	*	Communication	5		
Sexual Function	*	Stigma	8		

*Data of highest possible patient relevance: e.g. the 2017 LANCET publication  
New insights into the burden and costs of multiple sclerosis in Europe  
Gisela Kobelt PhDa, Alan Thompson MD<sup>b</sup>, EMSP, et.al.*

**Figure 5. Workforce participation: Proportion of patients below retirement age employed or self-employed (N = 13,391)**



# EMSP collaboration with Commission /EMA: Cross Committee Taskforce for Patient Registries (2014-2016)



# EMA Workshops on Patient Registries -2016

## Patient Registries Workshop

28 October 2016

Rectangular Snip

Meeting Room 2/A (2<sup>nd</sup> Floor)

European Medicines Agency, London, United Kingdom

**Report and call for action available now on EMA website!**



# ...and EMSP's proposal to EMA for a MS specific workshop on EuNetMuS

**The EMA will foster MS registry stakeholder collaboration through a face-to-face meeting bringing together invited representatives from**

- The European Commission
- The EC Joint Research Council
- EUREMS
- Big MS Data
- Marketing authorisation holders (MAHs) (MS products)
- Payers
- The EMA Cross-Committee Task Force on Registries
- Key Committee (CHMP, PRAC) members acting as Rapporteurs for MS products
- Representative from a registry group that has already agreed on key common data elements, for example, the European Cystic Fibrosis Society Patient Registry (based on suggestion to have a successful example of between-registry co-operation)

# 2017 EMA Workshop on EuNetMuS (cont.)

- **To discuss during the workshop**
- Key common outputs from registry-based studies that are needed by stakeholders including regulators, MAHs, payers
- Key data elements needed by all stakeholders in order to fulfil their common output needs
- Data validation and quality assurance measures
- Governance and data sharing, including process for collecting additional data required by various stakeholders.
- Multi-stakeholder collaborations for optimal use of MS registries
- **Output**
- Implementable recommendations that could be used by registry holders and MAHs/MAAs in respect of the data elements to be collected, protocols, consents, governance and registry interoperability.
- The EMA will act as facilitator and as such, no binding regulatory outcomes of the meeting are foreseen. The EMA will record key orientations taken at the meeting, draft the recommendations arising, circulate these for comment to the stakeholders and write the final document for sharing and publication on the EMA website. The EMA will consider whether it is uniquely placed to support any next steps in terms of fostering MS registry collaboration.
- **Meeting has been approved for the week commencing June 12, 2017, with travel reimbursement for 20 delegates**

# Next Steps for EuNetMuS

- Mapping Exercise on MS Registries in Europe has started – results expected in April 2017
- Individual presentation and discussion with pharma industry as potential funders did start / liaison with Industry Forum Progressive MS Alliaalliance needed still...
- EMA hosted workshop in mid June 2017
- Individual presentation and discussion with key registries in Europe
- Building a new project structure for EuNetMuS
- Detailed project funding proposal to industry in late summer 2017

# Can a European Network of (MS) Registries become financially sustainable ultimately?



**Marc Trusheim** of MIT Sloan School of Management and a NEWDIGS researcher, said in a recent EMA workshop on “Adaptive Pathways” :

“Our simulations and case studies on the market authorisation process show that via “Adaptive Pathways” and new approaches to data generation – e.g. through **disease specific patient registries being made fit for purpose** - the current process could become faster by years and cheaper by millions...”