

Understanding real world drug effects by
performing federated analyses across four
national multiple-sclerosis patient
registries

5th November 2025



Housekeeping



All participants will automatically be muted.



The chat box is disabled.



Please add your questions in the Q&A box. They will be answered either during the webinar or at the Q&A session at the end.



This webinar is being recorded; we will share the recording with all registrants after the webinar.

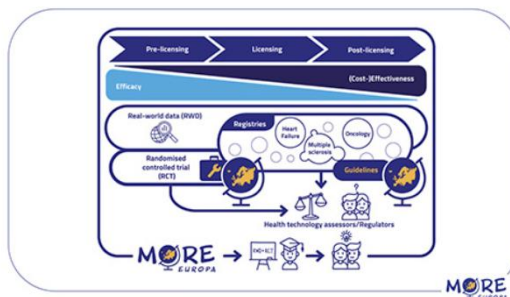
Agenda

Topic	Speaker(s)	Time Duration
More-EUROPA's aims / context for the Federated studies	Peter Mol	15 min
Federated analyses using MS registry data	Lars Forsberg	45 min
Bayesian Federated Inferences	Marianne Jonker	30 min
Q&A session	Moderator: Marlene Gylmark	30 min

Previous Webinars

Webinars

February 2025



How can real-world registry data be used to augment clinical trial data

July 2024



More-EUROPA

• Horizon-HLTH-2022-TOOL-11-02

Aims

- Establish value of registry-based RWD in augmenting RCTs
- Enable more effective and ethical use of registry data to support patient-centered regulatory and health technology assessment decision-making



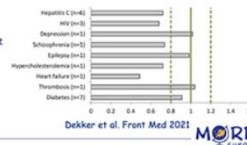
AI and ML approaches to identify and appraise registries and relevant data

November 2023



RCTs and RWD

- RCTs mainstay of drug efficacy and safety information for regulators/HTAs
- Value of RWD increasingly acknowledged
 - Transform, accelerate and de-risk decision making
 - Improve efficiency in design and conduct of trials
 - Increase public health
- Around licensing
 - Contextualize study results
 - Ensure generalisability of results to target population
- Post-licensing
 - Appreciate real-world value
 - Long-term B/R



Use of Registries in Regulatory Decision Making

<https://umcgresearch.org/more-europa-news-events>

Host and Moderator



Peter Mol is a professor of drug regulatory science at the University Medical Center Groningen, and the Dutch representative at the committee for medicinal products for human use (CHMP) at the European Medicines Agency. He is also the Principal Investigator of the More-EUROPA project.

Marlene Gyldmark holds a Masters in Economics and Policy Sciences from University of Copenhagen, Denmark, and a MPhil in Health Economics from York University, UK. Leading organisational change and strategy with focus on EU HTA organisational readiness at BeOne Medicines, Basel Switzerland as part of the Global Value, Market Access, and Pricing group. In addition, she is a long-term member of ISPOR and served on the board of directors between 2021- 2024. She also serves as Copenhagen Goodwill Ambassador.



Panellists



Lars Forsberg is an engineering physicist with a PhD in neuroscience. He is currently working as a research specialist at Karolinska Institute, where he focuses on real-world data in multiple sclerosis, and the development of federated learning methods.



Marianne Jonker studied mathematics and obtained her PhD in mathematical statistics from the VU University Amsterdam. She is currently affiliated with the Radboud University Medical Center (Nijmegen, the Netherlands). Her research focuses on survival analysis and federated inference methodology. She supervises PhD students and postdoctoral researchers and collaborates with scientists from various disciplines, including statistical physics, mathematics, and medicine.

More-EUROPA



- Establish value of registry-based RWD in augmenting RCTs
- Enable more effective and ethical use of registry data to support patient-centered regulatory and health technology assessment decision-making

Real-World Evidence in EU Medicines Regulation: Enabling Use and Establishing Value. Arlett p. et al. CPT 2021

<https://doi.org/10.1002/cpt.2479>

Registry data
complementing evidence
from clinical trials

Novel analytical tools (WP1)



Stakeholder
Evidentiary
Expectations



Tools to
augment trial
with registry data



Tools to assess
/ quantify level
of evidence



Federated
analyses



Effectiveness / safety in poorly
represented heart failure subgroups



Extend registry-based RCT evidence
on rituximab to European
multiple sclerosis registries



Complement minimal RWD dataset using
machine learning/artificial intelligence
techniques in lung cancer

Data access & usefulness WP2

Establishing value

Enabling use

Screening
tool for suitable
registries
WP3



Ethical
& Patient
perspectives
WP4



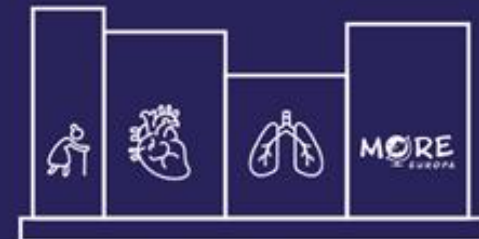
Dissemination WP5



Training



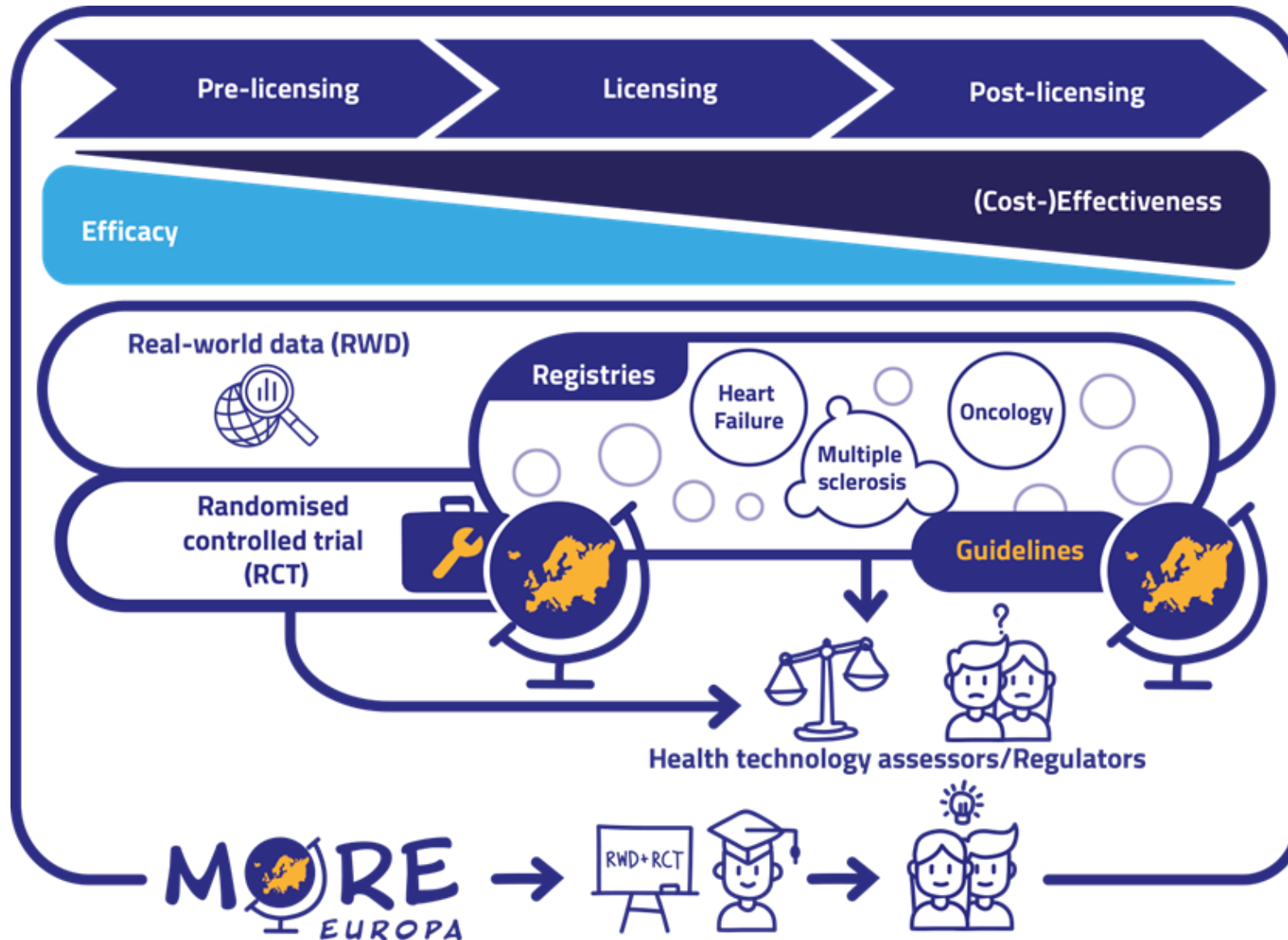
**Adoption
& Use**



**Guideline and Framework
Development**



More-EUROPA Summary



Focus on 3 registries



	Swedish Multiple Sclerosis registry (SMSreg)	Swedish Heart Failure Registry (SwedeHF)	Dutch Institute for Clinical Auditing (DICA) [‡]
Disease	Multiple sclerosis	Heart failure	Cancer (lung cancer)
Established since	1997	2000	2010
# patients captured in the registry	20,000	Till 2018, 156.000 registrations from 90.000 patients	In the pilot DICA-medicines: 10,000 patients (2018-2022) [§]
Data linkage	Cause of Death Registry National Patient Registry Statistics Sweden Prescribed Drug Registry		Hospital database (possible to scale up to nation-wide participation) PALGA (pathology) Vektis (claim database)
Age range	12-96 years	18-106 years	19-104 years
Sex	70% females	39% females	54% females
Registry-based RCT	RIFUND-MS (EudraCT 2015-004116-38)	SPIRRIT-HFpEF (clinicaltrial.gov NCT02901184)	N/A

SwedeHF



Personal number
(Tax number)

National Patient Registry

- Additional comorbidities
- Outcomes
 - Cause specific hospitalization
 - Incident comorbidity

Dispensed drug
registry

Cause of death
registry

RiksSvikt
nationellt hjärtsviktsregister

- Demographics
- Comorbidity
- Clinical (**Ejection fraction**, x-ray, ECG, BP, HR)
- Laboratory (creatinine, K, Hb, NT-proBNP)
- Medications and doses
- PRO
- Biobank (ongoing)
- Planned follow-up

Patient information		Diagnosis		Treatment	
First name	Last name	ICD-10	ICD-9	Medication	Dose
Mr. Erik	Mr. Eriksson	I50.1	402.01	Lisinopril	10 mg
Age	Sex	Admission date	Discharge date	Discharge diagnosis	Discharge status
65	Male	2018-01-15	2018-02-01	I50.1	Discharged

UCR®

Inclusion criterion:
Clinician judged HF

Personal number
(Tax number)



Statistics Sweden

- Socioeconomic characteristics (income, civil status, n. children, education)
- Matched control population (e.g. without HF)

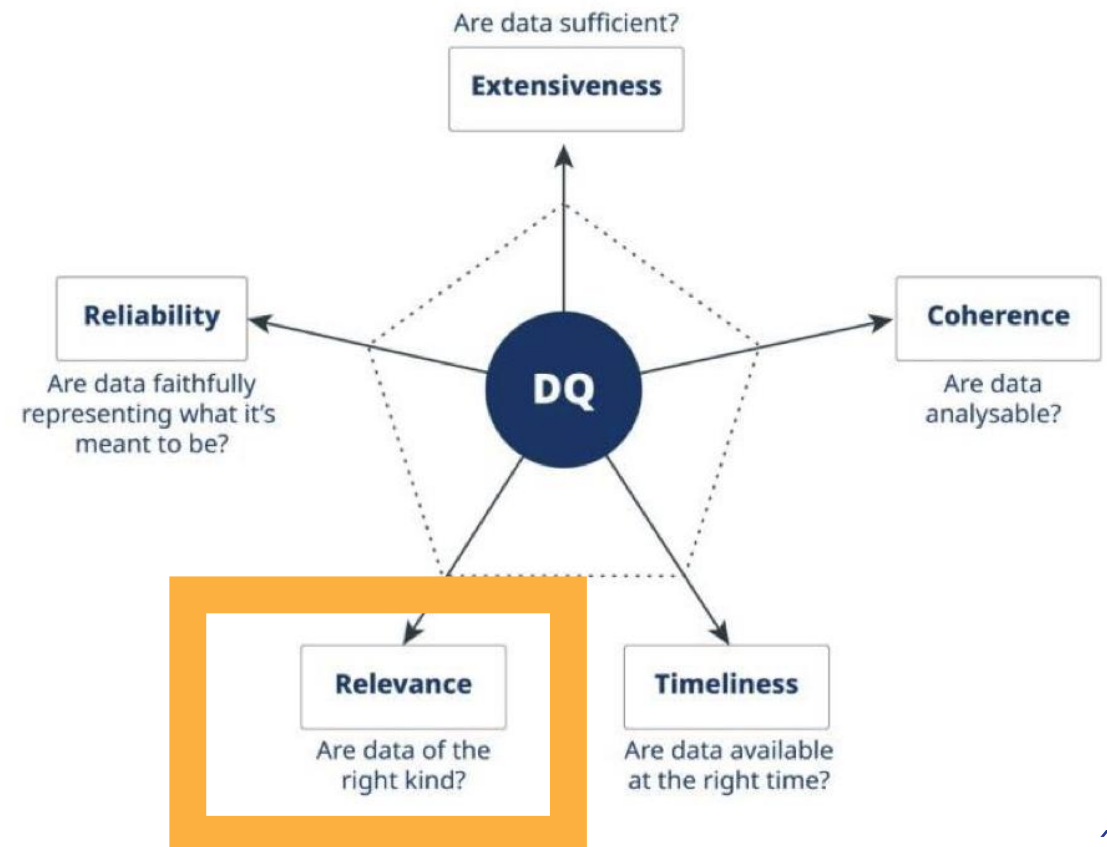
National Diabetes Registry **NDR** NATIONELLA
DIABETESREGISTRET

Data Quality Framework (key concepts)

Data Quality Determinants

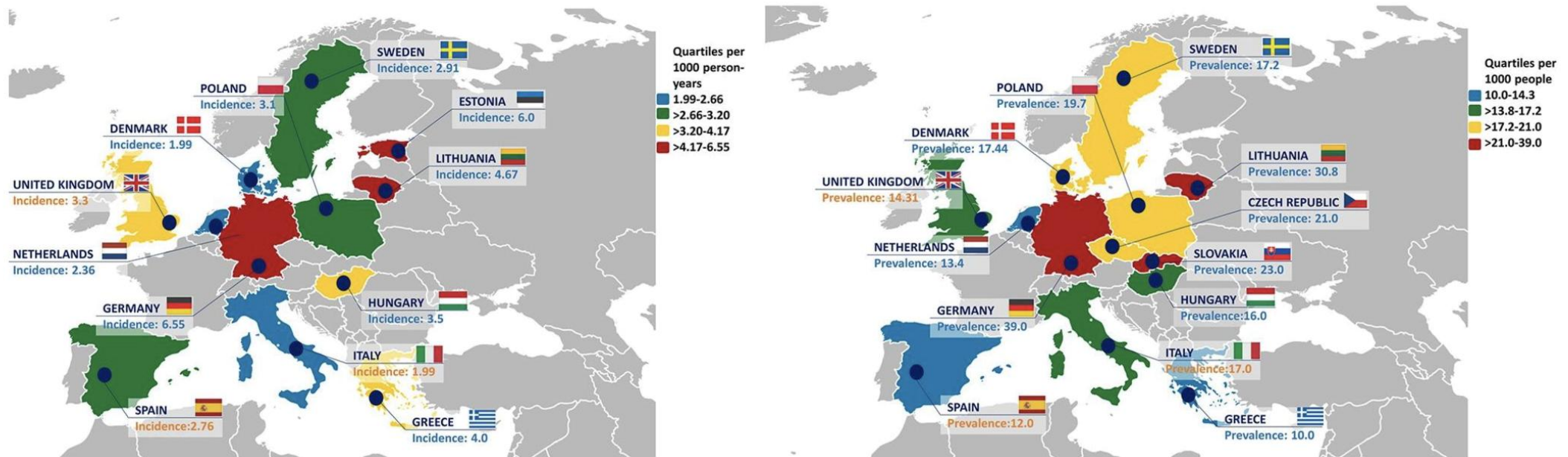


Data Quality Dimensions



European differences in epidemiology

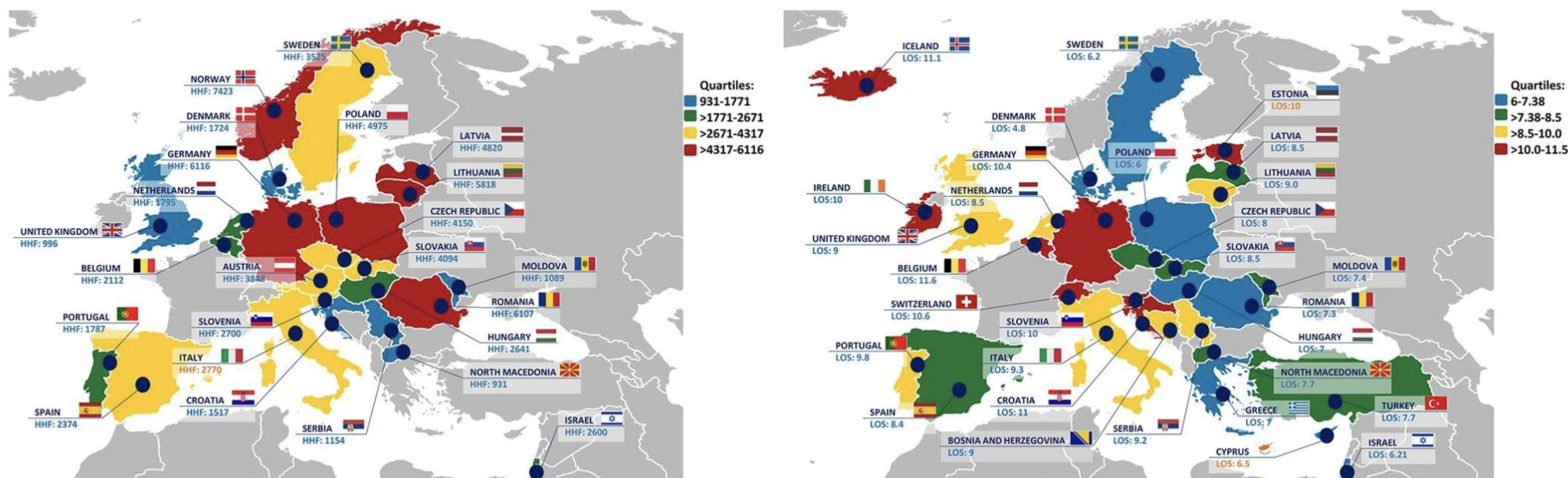
Figure 1 Incidence of heart failure per 1000 person-years (left) and prevalence of heart failure per 1000 persons (right). Adapted from Seferović *et al.*¹³



ESC Heart Failure 2022; 9: 2767–2778
DOI: 10.1002/ehf2.14076

European differences in disease management

Figure 3 Number of heart failure-related hospital discharges per million people (left) and average length of stay in hospital primarily due to heart failure (right). Adapted from Seferović *et al.*¹³



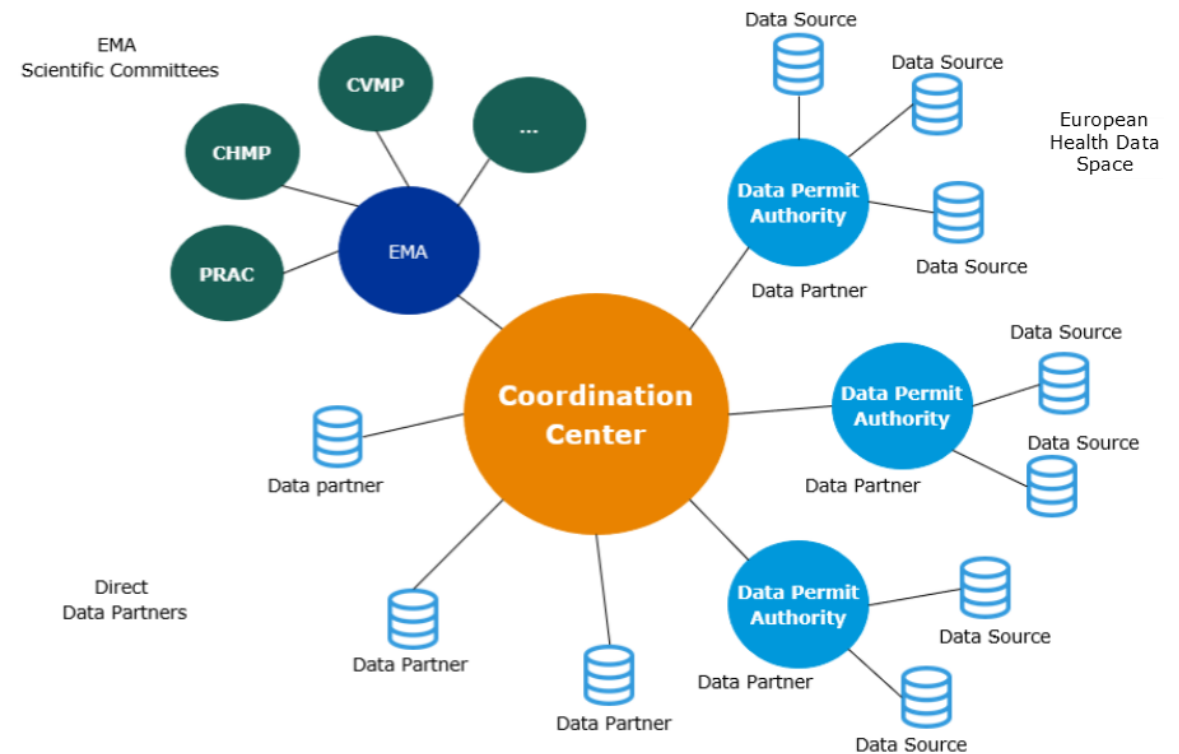
DOI: 10.1002/ehf2.14076

Relevance of RWE for EU regulators & HTA

- Do we have the right data?
 - Core data elements, DQF
- Can we get the data?
 - Governance
 - EMA's own data
 - 181 million EU inhabitants
 - 39 data sources
 - 18 countries

Key principles

- Data stays local
- A common data model will help performing studies timely and increasing consistency of results



Federated analyses using MS registry data



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Bayesian Federated Inferences



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Q&A session



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Thank you!

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