Overview of research projects: different use of RWD

Projects

EMIF

GETREAL

ADAPTSMART

BD4BO (Big Data for Better Outcome)

Digital technologies: WEB-RADR, Proactive, RADAR-CNS, RADAR-AD

IMPACT-HTA

COMED

IMI

RTD

Use of RWD

CLINICAL RESEARCH

REGULATORY DECISION MAKING

HEALTH TECHNOLOGY ASSESSMENT (HTA)

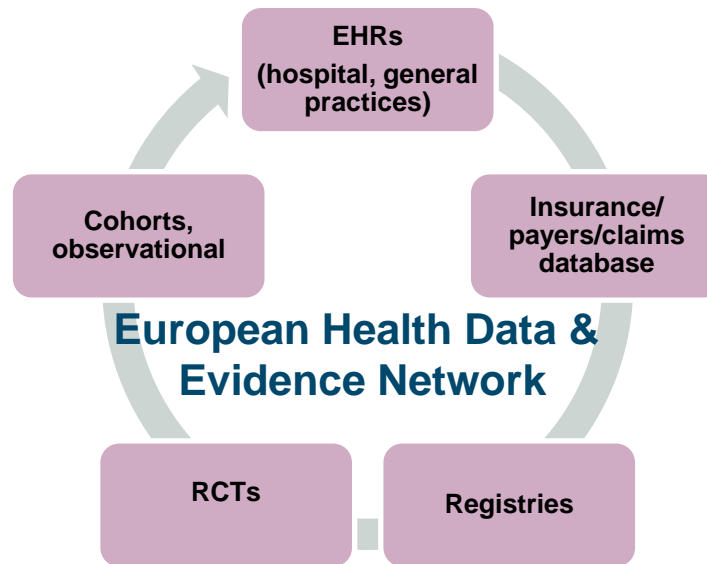*POLICY MAKING (PRICING & REIMBURSEMENT DECISION)*
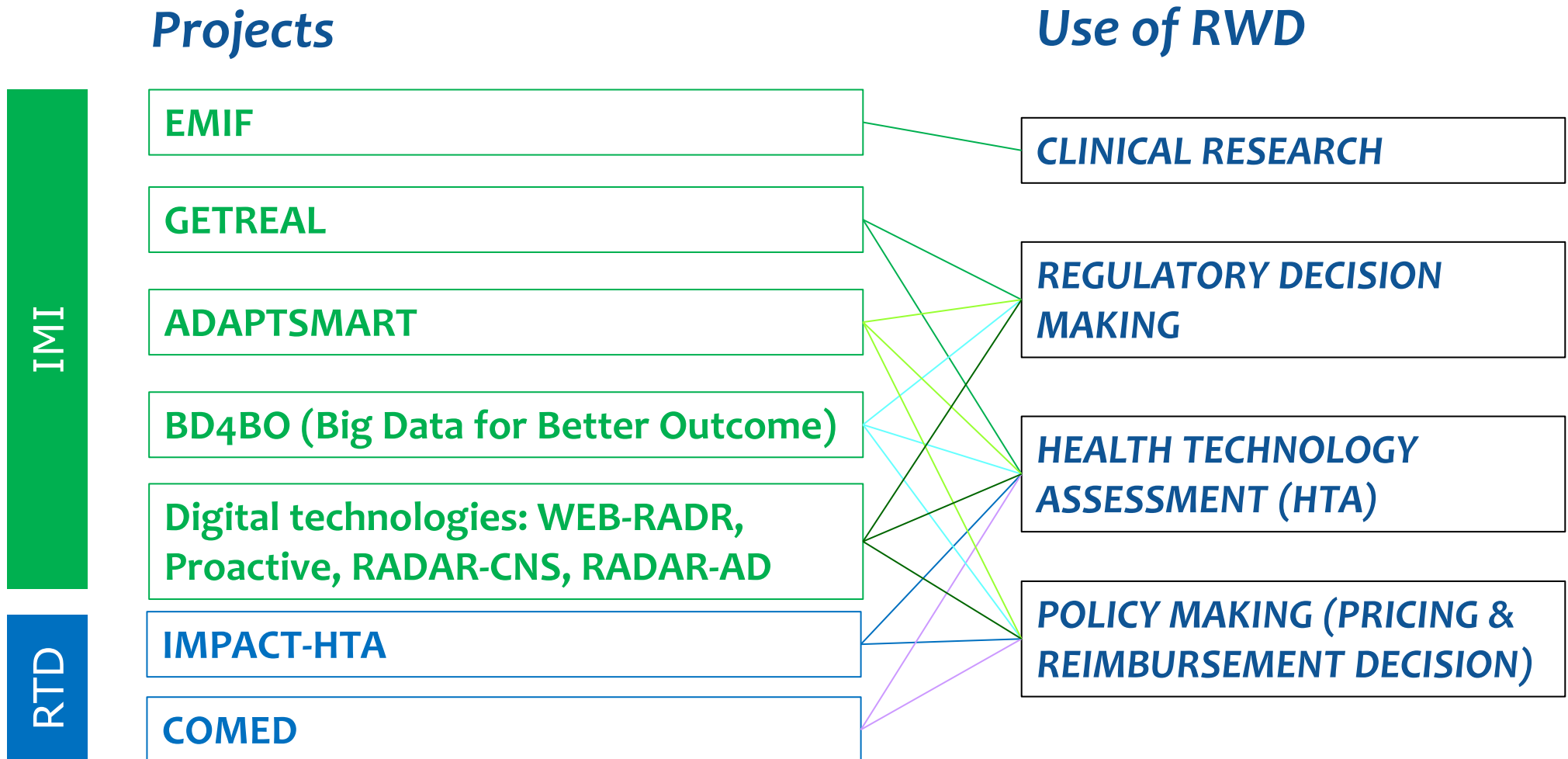
The Innovative Medicines Initiative Driving research in real-world data

Nathalie Seigneuret
STAMP meeting 08.06.2018

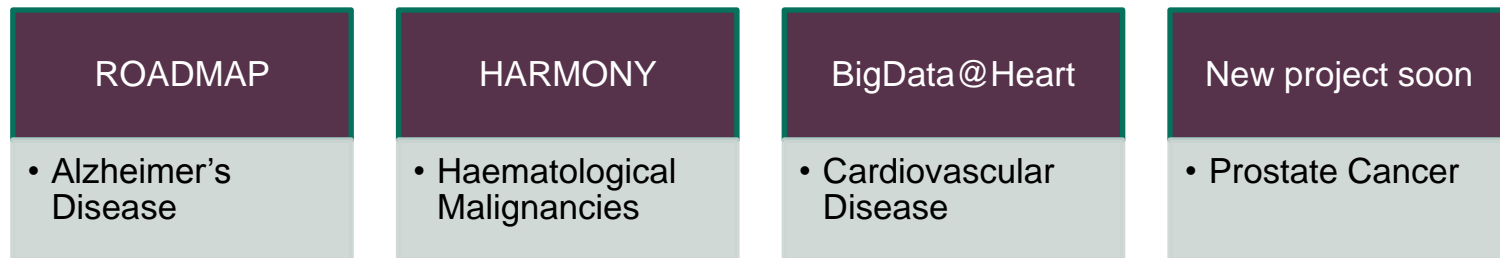# An integrated approach closer to real life practice: real world data / big data

EHRs
(hospital, general practices)

Insurance/payers/claims database

Cohorts, observational

European Health Data & Evidence Network

RCTs

Registries

Sustained through
www.insiteplatform.com

Big Data for Better Outcomes

ROADMAP	HARMONY	BigData@Heart	New project soon
• Alzheimer's Disease	• Haematological Malignancies	• Cardiovascular Disease	• Prostate Cancer

Facilitating Outcomes-focussed Healthcare Systems

GETREAL
Incorporating Rear world data into drug development

ADAPT-SMART
Medicines Adaptive Pathways to Patients

innovative medicines initiative

EMIF - Key Achievements

- 25 million patients harmonised to OMOP CDM

- Data catalogue of data sources (governed access)
  https://emif-catalogue.eu/

- Biomarker studies in AD & Metabolism

GETREAL - Key achievements

- **RWE Navigator:** interactive tool to walk a broad range of stakeholders through defining evidence gaps and study approach and design option https://rwe-navigator.eu
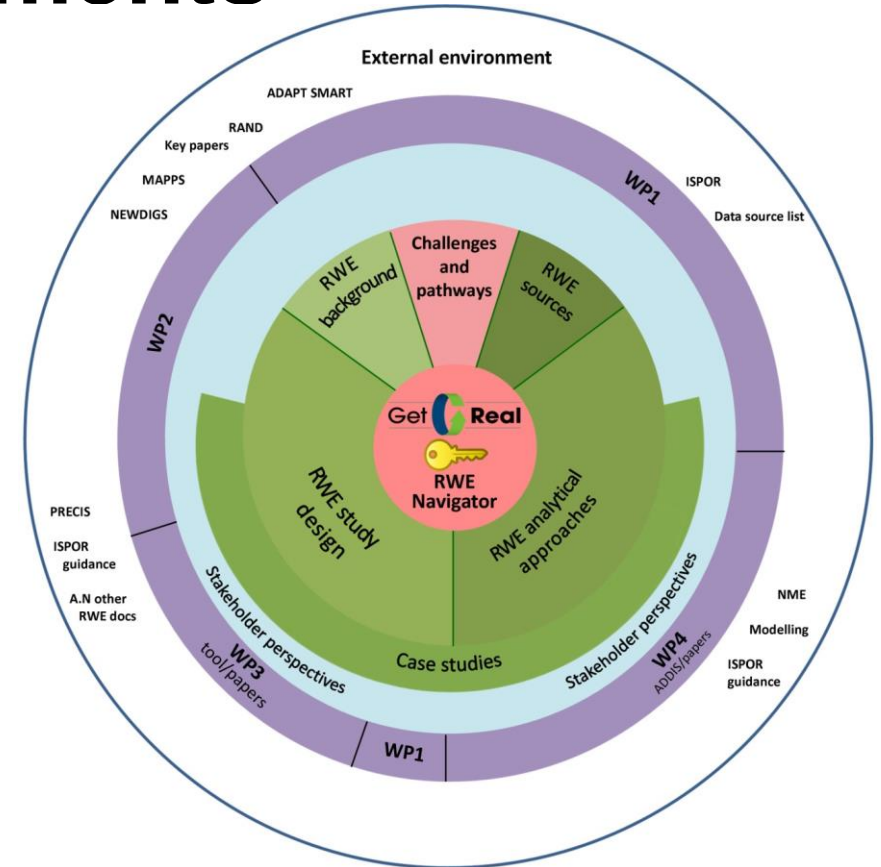
- **Toolbox:** Practical decision-making framework of tools to identify drivers of effectiveness + to get more insight into the statistical aspects for trials design
  - Practical tools to anticipate the risk for an efficacy-effectiveness gap
  - modelling techniques for designing an enriched trial before launch
  - statistical tools to analyse a pragmatic trial

GETREAL - Key achievements

- **PragMagic decision support tool** to help stakeholders to design better pragmatic trials www.pragmagic.eu

- **Synthesis and modelling approaches** to generate RWE, based on randomised and observational data (IPD meta-analysis, Network meta-analysis, mathematical modelling to predict RWE)

- Further development of **ADDIS** an advanced user-friendly software to support adoption of state-of-the art methods and tools in health care policy decision making https://addis.drugis.org

- **Real-World Evidence in Medicines Development course**

using *Elevate* e-learning platform (new planned in September-October)

- **Publication of GetReal policy recommendations on RWE**

    Further exploitation of the results in upcoming project: GetReal Initiative

innovative medicines initiative

ADAPTSMART - Key Achievements

BD4BO - Programme Goal

- **Massive amounts** of diverse healthcare data currently exist:
  - inpatient and outpatient hospital data, prescription data, claims information and patient-reported data, sociodemographic data, clinical trial data, 'omic measurements etc..

- Currently, no wide scale exploitation of these data
- Exploit the opportunities offered by **large data sets** from variable sources could lead to many **powerful insights:** increase **medical innovation** and deliver **better quality healthcare** system

- Support the evolution towards **outcomes-focused** and **sustainable healthcare systems** through engagement of key stakeholders

BD4BO: European Health Data Network

Objectives

- Harmonise data on approximately 100 million people to the OMOP common data model.

- Facilitate federated analytics on the data through standardised analytical tools

- Develop a number of use cases to demonstrate the value of the network

  - Regulatory, HTA decision making

  - Optimising care pathways

  - Continuous monitoring of effectiveness, safety

  - Expected to start Q42018/Q12019

New data sources: Digital technologies

- Wearables, smart watches, smart phones are a rich source of continuous patient-derived data
- Social media can provide direct patient insights
- Growing focus on developing digital endpoints

- **WEB-RADR:** Mobile ADR Reporting Apps & Social media monitoring
- **Proactive** – hybrid PRO tools qualified by EMA for monitoring physical activity
- **RADAR-CNS –** can data from consumer-grade wearables (eg FitBit) be used to monitor disease relapse/progression (depression, MS, epilepsy)
- **RADAR-AD -** smartphone, wearable and/or fixed home based sensors used to assess identified functional domains in Alzheimer's patients. Launch: Q4 2018.
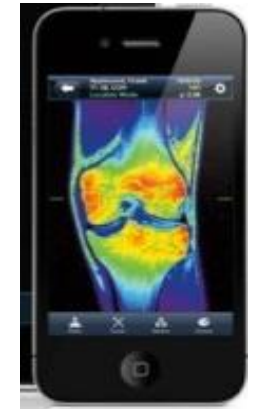
The EU Framework Programme for Research and Innovation

HORIZON 2020

HTA research projects on the use of real-world data

Leslie Pibouleau

European Commission
DG Research & Innovation
Unit E3 Public Health

Research and Innovation

HTA research projects on the use of RWD

- ✓ **On-going projects (2018-2020)**
  - ✓ *IMPACT-HTA (LSE)*
  - ✓ *COMED dedicated to medical devices (University of Bocconi)*
- ✓ **New 2018 call: Topic on HTA research to support evidence-based healthcare**
  - ✓ *Methodological work should address current concerns and uncertainties around the quality and suitability of RWD (e.g. from disease-specific registries and routine healthcare databases) for **relative effectiveness assessment** in HTA.*
  - ✓ *Budget: 10 million €*
  - ✓ *Evaluation procedure: 22 May 2018 →21 June 2018*

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Guidance on the analysis of non-randomised studies Impact-HTA (WP6)

✓ *Objective: to assess the **relative performances of available methods to adjust for confounding biases** (e.g. regression, propensity scores, instrumental variables)*

✓ ***Identify pairs of randomised and non-randomised studies** investigating the same research question (population/ intervention/comparator & outcomes) → Non-randomised studies will include cases using single arm trial for the new intervention and external data for the comparator*

✓ ***Compare estimated effect sizes obtained using different methods** to identify which methods lead to the less-biased estimates*

✓ *Develop **recommendations showing the different options** for the analysis of non-randomised studies and, for each option, the residual risk of bias*

✓ *3 workshops will be organise with key stakeholders **including regulators** to ensure the relevance & applicability of the outputs*

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Guidance on implementation of outcome-based managed entry agreements for orphan medicinal products (OMP) IMPACT-HTA (WP10)

✓ More likely to be required due to shortcomings in the available data at the time of product launch

✓ Review the use of outcome-based MEA & identify specific issues for OMP

✓ **Guidance on implementation**: criteria & conditions including the form of collaborative approach among stakeholders, operational & legal requirements, **data sources & data requirements**

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Thank you for your attention!

European Commission

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