The EU Framework Programme for Research and Innovation

HORIZON 2020

RTD – IMI projects

STAMP meeting
8 June 2018
Overview of research projects: different use of RWD

Projects

- EMI
- GETREAL
- ADAPTSMART
- BD4BO (Big Data for Better Outcome)
- Digital technologies: WEB-RADR, Proactive, RADAR-CNS, RADAR-AD
- IMPACT-HTA
- COMED

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

European Health Data & Evidence Network

- EHRs (hospital, general practices)
- Cohorts, observational
- Insurance/payers/claims database
- RCTs
- Registries

ROADMAP
- Alzheimer's Disease

HARMONY
- Haematological Malignancies

BigData@Heart
- Cardiovascular Disease

New project soon
- Prostate Cancer

Facilitating Outcomes-focused Healthcare Systems

Sustained through www.insiteplatform.com

GETREAL Incorporating real world data into drug development

ADAPT-SMART Medicines Adaptive Pathways to Patients
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](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](http://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](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
ADAPTSMART - Key Achievements

Medicines Adaptive Pathways to Patients (MAPPs) seek to foster access to beneficial treatments for the right patient groups with high unmet medical needs at the earliest appropriate time in the product life-span in a sustainable fashion. A key component of MAPPs is stakeholder collaboration.

https://www.infographic.adaptsmart.eu
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
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 domain in Alzheimer’s patients. Launch: Q4 2018.
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HTA research projects on the use of real-world data

Leslie Pibouleau
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Unit E3 Public Health
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
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**
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!