IMI ROADMAP

Carlos Díaz, SYNAPSE Research Management Partners

XI Conferencia Anual de las Plataformas Tecnológicas, Barcelona, 5-6 Marzo 2018

Real world Outcomes across the AD spectrum for better care: Multi-modal data Access Platform

www.twitter.com/IMI2_ROADMAP
www.roadmap-alzheimer.org

Current situation of Alzheimer's Disease (AD)

- Manufacturers invest in different interventions (nutrition, pharmaceutical, diagnostic tools, etc.)
- Disease starts 10-20 years before symptoms detected >> new trials target earlier stages of the disease
- Real world data and care do not address the full spectrum of the disease
- Passive attitude towards care
- Not enough efforts on collection and analysis of high quality real world evidence

What is ROADMAP?

- Formed under the Innovative Medicines Initiative (IMI) – with a total budget of 8.2M EUR (4M IMI JU Funding, 3.2M EFPIA In-kind, 1M EFPIA In-cash).

- ROADMAP is a private-public partnership (PPP) to explore the viability of a RWE platform in AD

- Focus on usefulness for HTA/regulatory agencies, patients and their caregivers

Aims

The aim of ROADMAP is to deliver a **series of data integration methods and tools for patient outcomes, developed and tested through pilot projects**, which are scalable and transferable, and which will provide the foundation for a future Europe-wide RWE platform on AD

In parallel, we will develop tools for **stakeholder engagement, understanding the ELSI context and health economics impact** of a RWE approach in AD.

In doing so, ROADMAP aims to create the **conditions for an open collaboration among stakeholders that yields consensual, efficient uses of this RWE platform for the ultimate benefit of AD patients and their caregivers**.

Objectives

- Define and catalogue methodologies for **identifying AD outcomes from routinely collected data**
- Identify and pool AD-related RWE data and establish solutions to combine different **RWE sources with RCT** data supporting pharmacoeconomic evaluation
- Develop and validate a core **disease progression model** to facilitate analysis of disease trajectories and effect of interventions on disease trajectories
- Develop a **PoC AD cost-effectiveness and budget impact model** for HTA agencies, regulators, service providers, industry, payers and carers
- Develop guiding principles and **recommendations from HTA/payers/regulators** for the development and incorporation of RWE into clinical and market access development plans for AD
- Develop and implement a **communication strategy** focussing on the needs of patients and professionals
- Develop an **ELSI framework** for the development and application of RWE in AD
- Develop a **full plan for phase 2** of the ROADMAP initiative that addresses identified gaps and pitfalls, and exploits promising solutions to their full potential for development of a European RWE platform in AD

Expected Key Deliverables

- Define **minimum set of** measurable **real-world patient outcomes**
- Develop **recommendations on RWE** appropriate AD-related cognitive, functional, and behavioural **endpoints**
- **Identify data sources &** outline a data **integration strategy** for RWE outcomes
- Develop **new methods for collecting RWE data** to improve AD health care value
- Make **recommendations on disease progression/health economic modelling**
- Under leadership of UK NICE and Dutch Regulator (MEB), deliver **guiding principles and recommendations** from HTA groups/payers/regulators for incorporation of RWE into clinical & **market access development plans** for AD

ROADMAP Consortium

EFPIA

NOVARTIS
janssen
Roche
Biogen
GE
Lundbeck
Takeda
Lilly
AC IMMUNE

Academia

UNIVERSITY OF OXFORD
Maastricht University
LUMC Leids Universitair Medisch Centrum
IXICO
AARHUS UNIVERSITY
UNIVERSITY OF GOTHENBURG
Erasmus MC
université de BORDEAUX
SYNAPSE RESEARCH MANAGEMENT PARTNERS
LSE
university of groningen
KØBENHAVNS UNIVERSITET
IDIAP Jordi Gol
THE UNIVERSITY OF EDINBURGH

EPAD European Prevention of Alzheimer's Dementia Consortium
EMIF
Dementias Platform UK Medical Research Council

EMA-NICE/HTA/payers Qualification Pilot use of RWE in AD

CBG MEB Medicines Evaluation Board

NICE National Institute for Health and Care Excellence

Alzheimer Europe

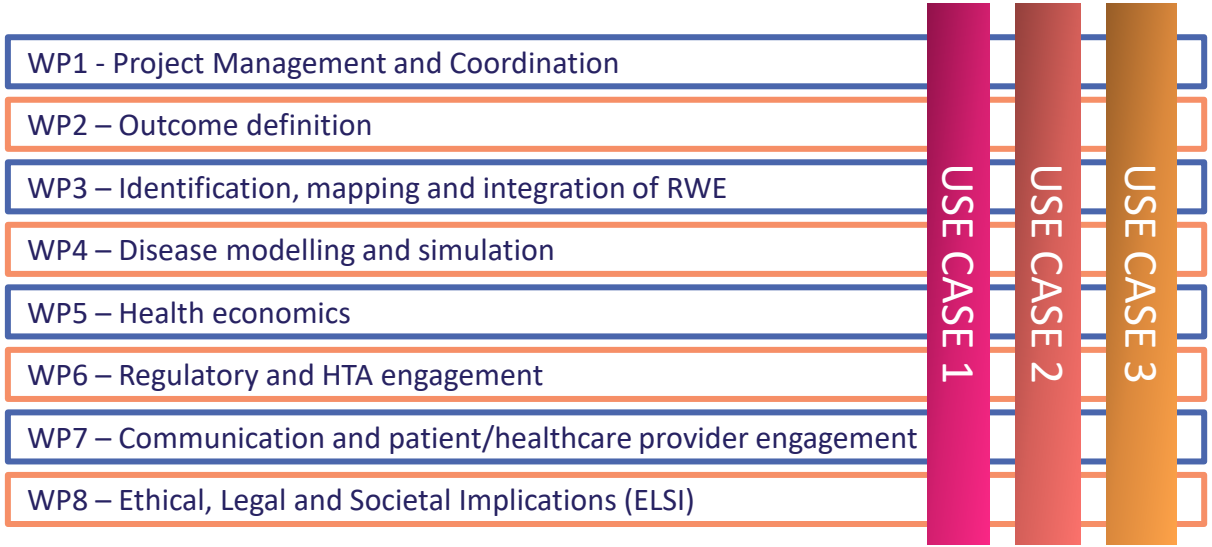
National/Regional Health Authorities to comprise an Expert Advisory Group (EXAG led by NICE):
(1) HTA bodies/payers from different archetypes
(2) Regulators
(3) Patient Associations

Related projects

BD4BO
HARMONY
BigData@Heart Big Data for Better Hearts
MOPEAD
AMYPAD Amyloid imaging to prevent Alzheimer's Disease

ROADMAP Project Plan

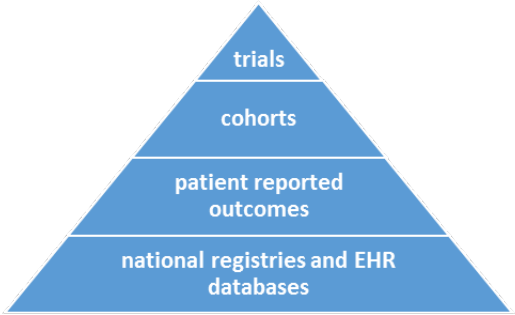
Use cases as framework to help stakeholders generate specific questions, test the ability of the integrated data systems to provide answers, and identify data gaps



ROADMAP – Strong data corpus

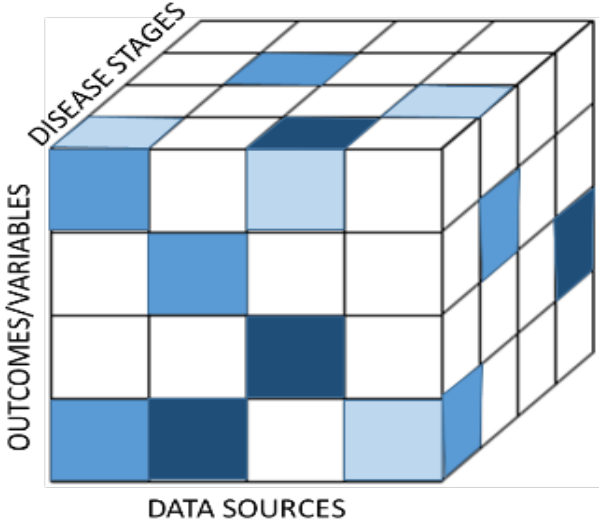
- **6 European countries** (Denmark, France, Netherlands, Spain, Sweden, UK)
- **75 national databases** and **clinical registries** n≈80M
- more than **40 cohorts** n≈2M
- several studies using **wearables** and **smart devices** n≈100K
- **5 dementia relevant trials** n≈100K

Leverage existing large data sets to perform pilots



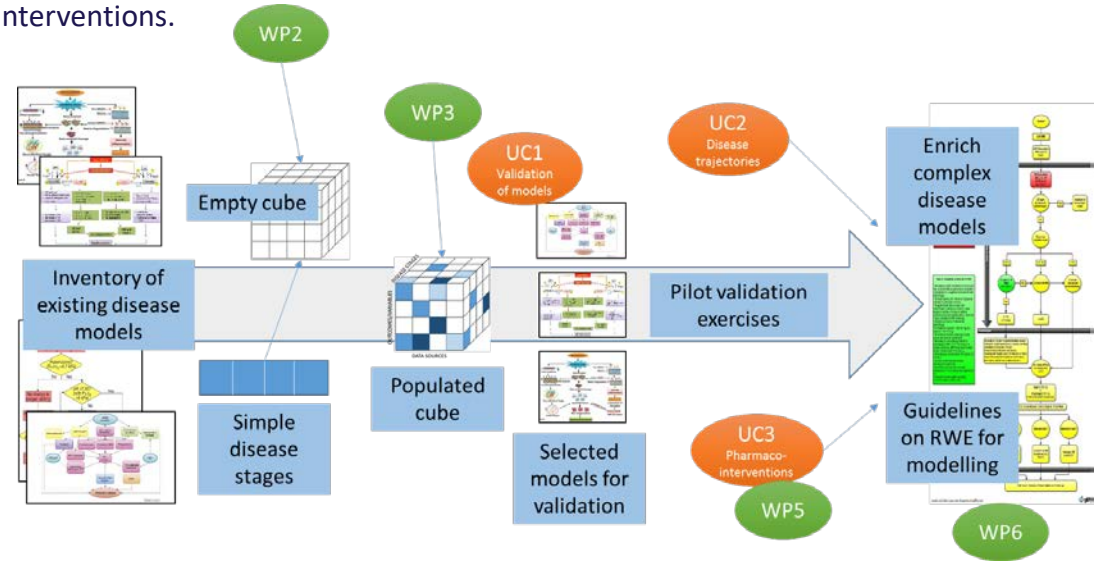
Data Cube

Integrating diverse data sources in relation to disease stage



Project structure - flow of tasks

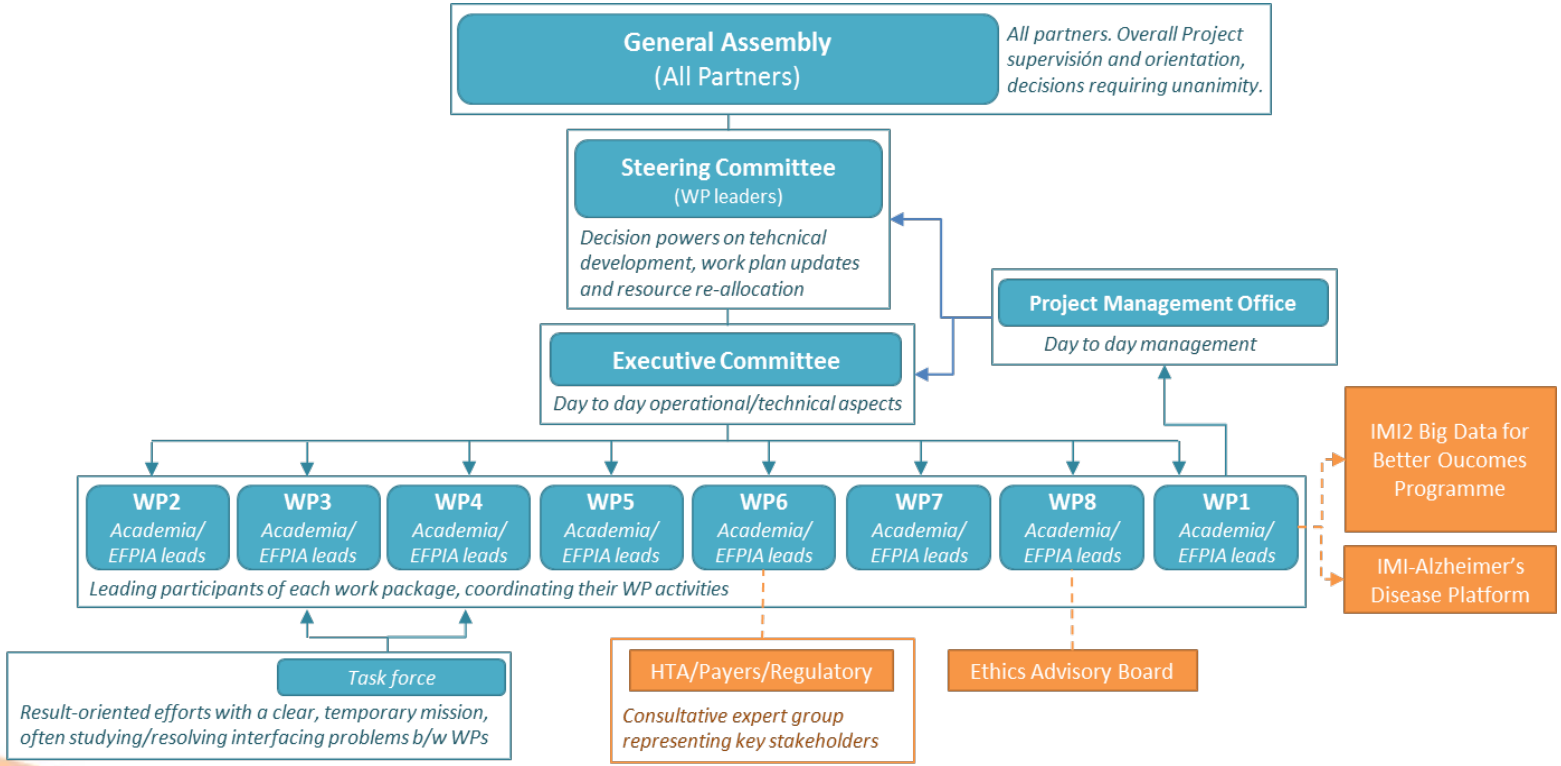
- Activities in WP4 are intertwined with other WPs and the use cases.
- WP2 provides input regarding the outcomes.
- WP3 provides input regarding the availability of data.
- Use Case 1 involves the validation of selected models.
- Use Case 2 provides input for enriching the disease models.
- Use Case 3 evaluates the effects of interventions.



Regulatory & HTA engagement

- Expert Advisory Group
- Consists of members from regulatory agencies & HTA bodies across Europe
- Meets periodically with Consortium to provide input and feedback, ensuring that ROADMAP outputs are not just of high scientific quality but also have meaningful applicability in regulatory & HTA context

Governance


General Assembly
(All Partners)
All partners. Overall Project supervisión and orientation, decisions requiring unanimity.
Steering Committee
(WP leaders)
Decision powers on tehcnical development, work plan updates and resource re-allocation
Project Management Office
Day to day management
Executive Committee
Day to day operational/technical aspects
IMI2 Big Data for Better Oucomes Programme
IMI-Alzheimer's Disease Platform
WP2 Academia/ EFPIA leads
WP3 Academia/ EFPIA leads
WP4 Academia/ EFPIA leads
WP5 Academia/ EFPIA leads
WP6 Academia/ EFPIA leads
WP7 Academia/ EFPIA leads
WP8 Academia/ EFPIA leads
WP1 Academia/ EFPIA leads
Leading participants of each work package, coordinating their WP activities
Task force
Result-oriented efforts with a clear, temporary mission, often studying/resolving interfacing problems b/w WPs
HTA/Payers/Regulatory
Consultative expert group representing key stakeholders
Ethics Advisory Board

Expected Impact and timelines

ROADMAP aims to provide the foundation for a Europe-wide integrated data environment and framework for RWE across the spectrum of Alzheimer's disease

- Phase 1 runs over 2 years from November 2016 - October 2018.
- Key findings from Phase 1 of ROADMAP will likely determine a further three-year Phase 2 project for the prospective collection of key clinical and health economic outcomes in AD

Funding

This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement No 116020 (“ROADMAP”). This Joint Undertaking receives support from the European Union’s Horizon 2020 research and innovation programme and EFPIA.