Building a sustainable approach that addresses the challenges and opportunities around treatment access at time of approval

LEARNINGS FROM THE ROADMAP PROJECT

Alzheimer Europe Conference
Barcelona, 29-31 October

Real-world evidence in Alzheimer’s disease

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WELCOME FROM THE PROJECT COORDINATOR

On behalf of our 26 partner organisations, I am delighted to welcome you to the ROADMAP sessions on “Real-world evidence in Alzheimer’s disease” at this year’s Alzheimer Europe Conference in Barcelona.

ROADMAP stands for “Real world Outcomes across the Alzheimer’s Disease spectrum for better care: Multi-modal data Access Platform” and this event marks a highlight in our journey as we move towards the end of Phase 1.

I am particularly proud that we can showcase our learnings at the conference of Alzheimer Europe, who is one of our partners and has been actively engaged throughout the whole project.

We had a great journey during the last two years. This project has probably been the most detailed and comprehensive evaluation of real-world evidence in Alzheimer’s disease that has ever been conducted.

Our teams on disease outcomes, ethics, health technology assessment and regulatory engagement as well as available data have done fantastic work and our Consortium is already passionately looking towards a potential Phase 2.

As we saw the project going through initial exploratory stages to drawing conclusions, both challenges as well as achievements have generated leading-edge learnings.

I would therefore like to invite you to join us in the following scientific discussions, which I hope you will find both fulfilling as well as engaging.

John Gallacher
PROJECT COORDINATOR, ROADMAP
I am pleased to welcome you to the ROADMAP journey here in Barcelona at the Alzheimer Europe Conference, held under the motto “Making dementia a European priority”. I find it marvelous that our Consortium has been given the opportunity to devote a plenary talk and four parallel sessions to this event, where it will present our research on various topics revolving around real-world evidence in Alzheimer’s disease.

Successfully delivering therapies to the tens of millions in need will depend on building a sustainable approach that addresses the challenges and opportunities around treatment access at time of approval. In Alzheimer’s disease this will very much depend on the integration of real-world evidence within healthcare systems to support evidence for approval as well as health technology assessment and funding allocation.

Therefore, our Consortium has worked at a fast-moving pace during the past two years and the duration of ROADMAP Phase 1. I am delighted that our Project Coordinator, John Gallacher, will set the scene by giving you an introduction to ROADMAP’s aims and the experiences we were able to gain during a plenary talk. This will be followed by four sessions, which are each going introduce you to an important element added to ROADMAP on our path towards improved healthcare for people affected by Alzheimer’s disease.

I am confident that you will find our introduction into the ambitious goals of the overall project and work streams, including the lessons learned, thought provoking and am therefore very glad to welcome you to join us in the following ROADMAP presentations during these two days.

Frédéric de Reydet de Vulpillieres
PROJECT LEADER, ROADMAP
TUESDAY, 30 OCTOBER, 8.30-10.00
ROOM: MR09-MR10-MR11

PL1 Dementia as a policy priority

PL1.5 John Gallacher (United Kingdom):
Real-world data supporting regulatory and health technology assessments: the findings of the ROADMAP project
John Gallacher, Project Coordinator, Director of the MRC Dementia Platform UK and Professor of Cognitive Health at the University of Oxford

ABSTRACT

REAL-WORLD DATA SUPPORTING REGULATORY AND HEALTH TECHNOLOGY ASSESSMENTS: THE FINDINGS OF THE ROADMAP PROJECT

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ROADMAP is a public-private partnership to evaluate the usability of multiple data sources, including real-world evidence (RWE), in the decision-making process for new treatments in Alzheimer’s disease (AD), and to advance concepts in disease and pharmaeconomic modelling. ROADMAP will identify key disease and patient outcomes for stakeholders to make informed funding and treatment decisions, deliver data integration methods and standards, and develop conceptual cost-effectiveness and disease models designed in part to assess whether early treatment provides long-term benefit. ROADMAP provides a stakeholder consensus approach to optimizing patient and societal benefit from new AD treatments. Initial findings from ROADMAP on the accessibility of real-world data, its utility for disease modelling and policy formation will be discussed.
DISEASE OUTCOMES: WHAT MATTERS TO WHOM AS AD PROGRESSES?
RELEVANT OUTCOMES IN DISEASE PROGRESSION

ABSTRACT

Alzheimer’s disease and other dementia-related diseases are affecting more and more people, as the population is getting older. Due to the increasing number of people affected by the condition, the need for new treatment alternatives and better care plans is higher than ever before, both for people living with dementia and their families but also for society at large.

In order to be able to better evaluate new emerging interventions, systems that can take into account all available healthcare information—often referred to as real-world data (RWD)—are needed. ROADMAP is a project bringing together 26 partners from across Europe, aiming to investigate how such systems should be set up to meet the needs from different stakeholders, including regulatory bodies and payers. The ROADMAP work stream “Outcome Definition” aimed to identify a priority set of real-world dementia outcomes, focussing on AD, across the disease spectrum, from a diversity of stakeholder perspectives. To achieve this, a systematic literature review and a survey were conducted. In addition, PPI consultations were carried out including among others the views of people with dementia and their carers. The aim of this session is to present an overview of the full work process including preparation of PPI consultations, methods used and the results. The outlook focusses on how these results as well as the patients’ and caregivers’ voices could impact public decision-making.
TUESDAY, 
30 OCTOBER, 
16.00-17.30

ROOM: MR08

P12  Ethics challenges for AD research and practice using real-world data
Chairperson: Christophe Bintener (Luxembourg)

P12.1 Ana Diaz (Luxembourg) and a representative of the EWGPWD: The involvement of the European Working Group of People With Dementia in the ethics work of ROADMAP

P12.2 Zuzanna Angehrn (Switzerland): Ethical and social implications of using predictive modelling for Alzheimer’s disease prevention

P12.3 Alex McKeown (United Kingdom): Ethical issues in outcome prioritisation in Alzheimer’s disease treatment

ABSTRACT

ROADMAP: ETHICS CHALLENGES FOR AD RESEARCH AND PRACTICE USING REAL-WORLD DATA

ANGEHRN Zuzanna1, DIAZ Ana2, MCKEOWN Alex3

1Analytica Laser, a Certara Company, Lörrach, Germany/Basel, Switzerland, 2Alzheimer Europe, Luxembourg, Luxembourg, 3University of Oxford, Oxford, United Kingdom

Background: ROADMAP maps the critical ethical, legal and social issues that arise from creating a real-world evidence platform, re-using the existing health data and pooling data from different data sources. It also addresses ethical concerns and topics that arose during the course of the project.

Ana Diaz will give a presentation about the consultation with the European Working Group of People with Dementia for the ROADMAP project on possible concerns that people with dementia and carers might have regarding the sharing and re-use of health data for AD research. A representative from the EWGPWD will co-present and contribute towards the discussion.

Zuzanna Angehrn will report on the results of a systematic literature review looking into the ethical concerns and social implications of using predictive modelling as a part of AD prevention strategies targeting people who do not have symptoms but seem to have an increased risk of developing AD in the future. The public will be asked to provide their perspective on the literature findings.

Alex McKeown will present on a recent paper that he and other members of the ROADMAP team have collaborated on in mapping the landscape of ethical issues in the prioritisation of health and treatment outcomes in AD. The paper ranges over issues for a wide range of stakeholders including people with dementia and their carers, clinicians, health economists, payers and others.
REGULATORY AND HTA PERSPECTIVES ON REAL-WORLD DATA IN AD: FUTURE OF PROSPECTIVE COLLECTION AND FURTHER RESEARCH

BOUVY Jacoline, O’ROURKE Diana, JONSSON Pall, XOXI Entela

National Institute for Health and Care Excellence, Manchester, United Kingdom

Regulatory and health technology assessment (HTA) engagement has been a key component in ROADMAP – an Innovative Medicines Initiative project in Alzheimer’s disease (AD). To facilitate this an expert advisory group (EXAG), consisting of regulatory and HTA experts was established to provide guidance on the use of real-world evidence in AD from a regulatory and HTA perspective.

During the course of the ROADMAP project, EXAG discussions have explored the challenges associated with preparing Europe’s healthcare systems for a disease-modifying drug for AD. Because disease-modifying drugs will likely target earlier disease stages of the disease than currently licensed treatments, it is not clear what type of evidence base might be required for these products to go through regulatory and HTA procedures.

Many of the challenges that the EXAG identified could be solved by generating better real-world data in AD. Recommendations from the EXAG included the need for the use of real-world evidence to support disease progression modelling assumptions, the need for consensus on relevant outcomes in early AD, and to provide the required information to accommodate for differences between national and regional settings as part of a pharmacoeconomic model.

This presentation and discussion will provide an overview of the lessons learned from ROADMAP Work Package 6 (regulatory and HTA engagement) and the ROADMAP EXAG, as well as reflecting on the practical implications and challenges of using real-world data in HTA and regulatory decision making.
WEDNESDAY,
31 OCTOBER,
10.30-12.00

ROOM: MR08

P28  Real-world data availability across Europe: What data are present, missing and heterogeneity of data collected?
Chairperson: Carlos Díaz (Spain)

P28.1 Glòria Garcia (Spain): Developing the Data Cube

P28.2 Johan van der Lei (Netherlands): Challenges to fill in the Data Cube, differences across data sources

P28.3 Antje Hottgenroth (Germany): Industry perspective on use and future development

P28.4 Anna Ponjoan (Spain): Data source and academic perspective on the Data Cube

ABSTRACT

P28. ROADMAP: REAL-WORLD DATA AVAILABILITY ACROSS EUROPE: WHAT DATA ARE PRESENT, MISSING AND HETEROGENEITY OF DATA COLLECTED

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Background: ROADMAP addresses the challenge of how to best inform clinical and health-policy decisions by studying how to build a population-based integrated data environment and enabling its visualization.

Method: ROADMAP has addressed the challenges of defining AD-relevant real-world outcomes based on literature reviews and consultation with experts and stakeholders. It then has surveyed a variety of data sources across Europe to find out the degree to which such real-world outcomes are captured.

Results: A Data Cube has been developed and offers a ‘landscape’ on data availability in Europe. This 3D ‘heat map’ assessment allows the visualization of the different data sources and how they are able to capture the different AD-outcomes, together with their relevance for the different disease stages.

Conclusion: Enabling the visualization of the AD-related data availability in different types of European data sources and the intrinsic gaps has proven to be a powerful tool for the design, planning and validation of the models and strategies used to guide future recommendations to enhance AD research.
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. http://imi.europa.eu/