Regulatory and HTA perspectives on real-world data in Alzheimer's Disease

Lessons learned from ROADMAP Work Package 6 (regulatory and HTA engagement) and the ROADMAP expert advisory group

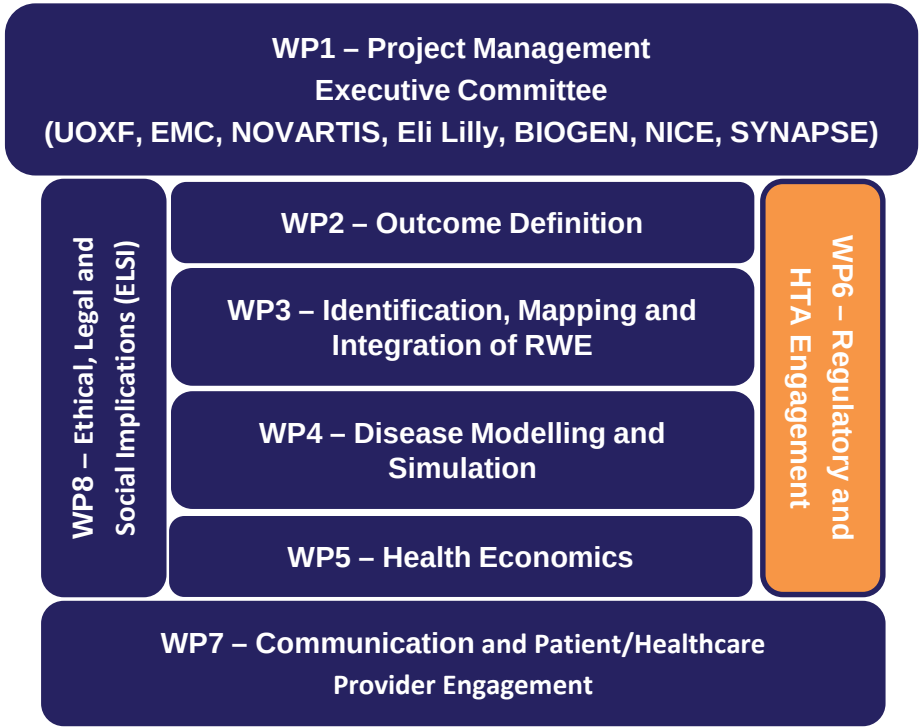
Diana O'Rourke| National Institute for Health and Care Excellence (NICE)

www.roadmap-alzheimer.org

What is ROADMAP?

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

ROADMAP's goal is to develop efficient uses of real world evidence for the benefit of people with Alzheimer's Disease and their caregivers



Why regulatory and HTA engagement?

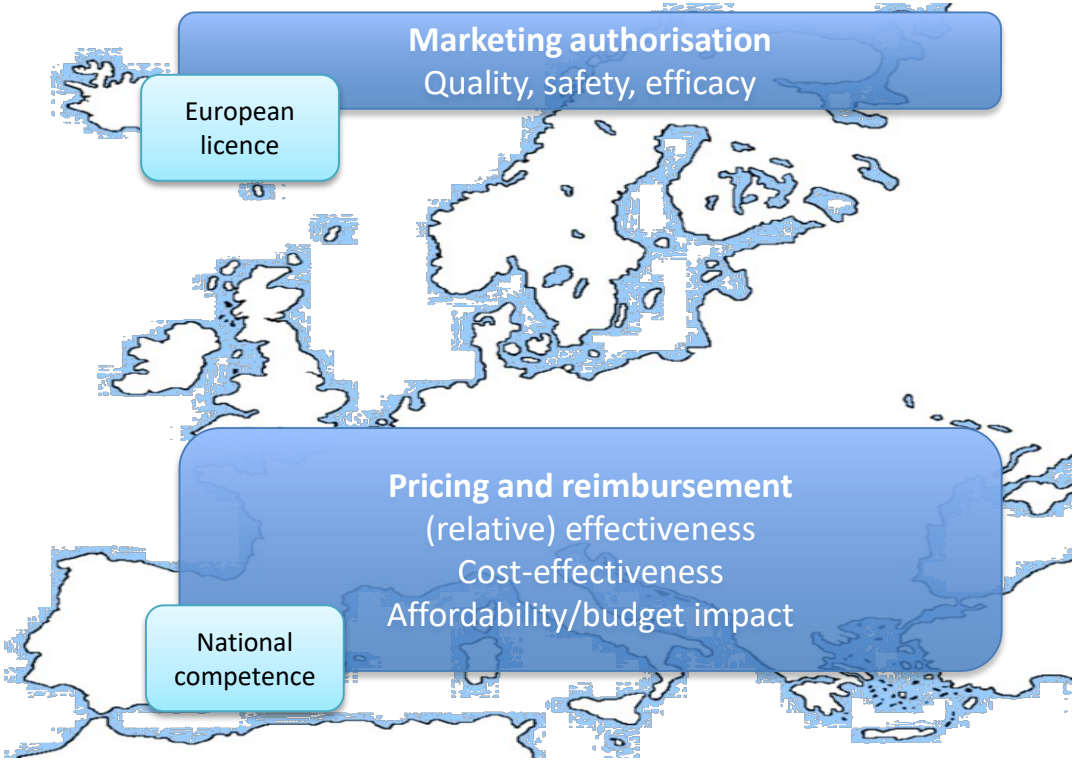
Regulatory agencies

Does the product work?

HTA bodies

Payers

How well does the product work
-Compared to what's already available?
-Does it provide value for money?



Aims of Work Package 6

- Mechanism for engagement with regulators, HTA agencies and payers
  - To ensure regulatory + HTA perspectives are taken into account in project outputs and deliverables
- Developing a real-world evidence strategy for Alzheimer's disease/dementia:
  - Guidance on the potential use of RWE for AD from the regulatory, HTA, and payer perspectives

Work Package 6: Workstreams

- ROADMAP Expert Advisory Group (EXAG)
  - Experts from regulatory/HTA backgrounds representing different European countries
  - Quarterly meetings by teleconference / face-to-face (6 meetings in total)
  - Discussion, feedback and input on project outputs and activities:
    - AD priority outcomes
    - Disease stage definitions
    - Disease-progression and economic modelling
    - Approaches for real world data collection

Work Package 6: Workstreams

- Guiding principles on use of real world evidence (RWE) in AD in the regulatory/HTA context
  - Regulatory + health technology assessment (HTA) experience with marketed AD products
  - Regulatory + HTA considerations for disease-modifying drugs in Alzheimer's disease

Lessons learned: Current AD products

- Similar evidence requirements for the regulatory and HTA approval of current AD drugs
- Limited use of RWE
- Choice of outcomes differed slightly

Lessons learned: Priority outcomes

- Lack of validated outcomes in the early disease stages
- Establish accepted outcomes for Regulators/HTAs/Payers which are able to demonstrate prevention/delayed onset of AD
- Need for validated measurement instruments which can be used across settings
- Importance of caregiver-relevant outcomes

Lessons learned: Real-world evidence in AD

- Gaps in RWE sources relating to different outcome measures/instruments
- Lack of standardisation in the collection of real-world data
- Limitations in the use of RWE to demonstrate effectiveness of a treatment

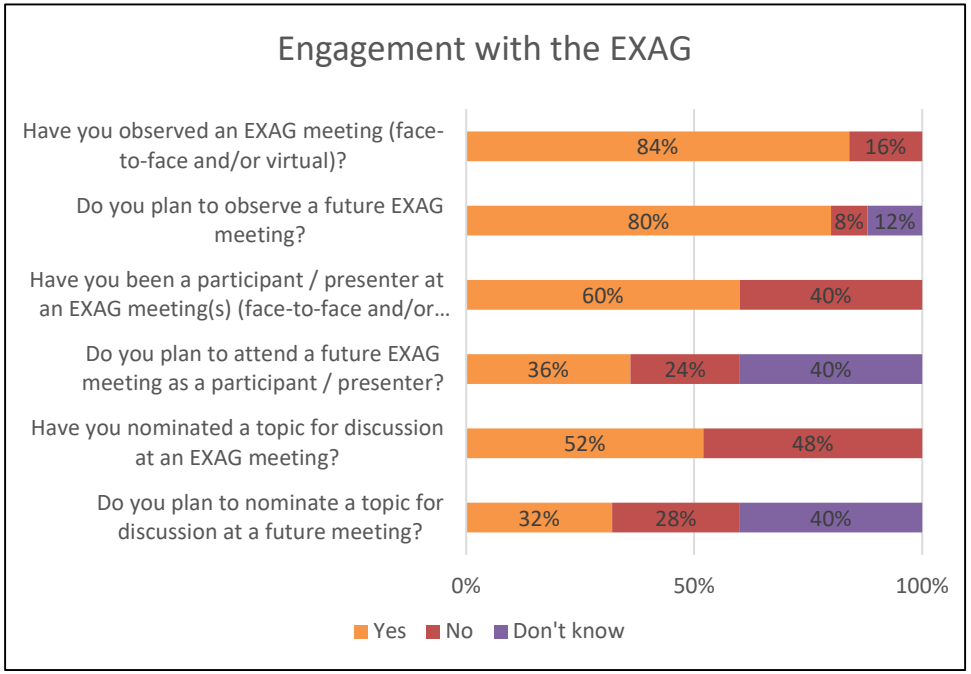
BUT

- Role for RWE in supporting disease progression and health economic modelling assumptions:
  - Correlation between disease stages and endpoints
  - Caregiver impacts
  - Information on regional/national settings

EXAG Survey – Key findings

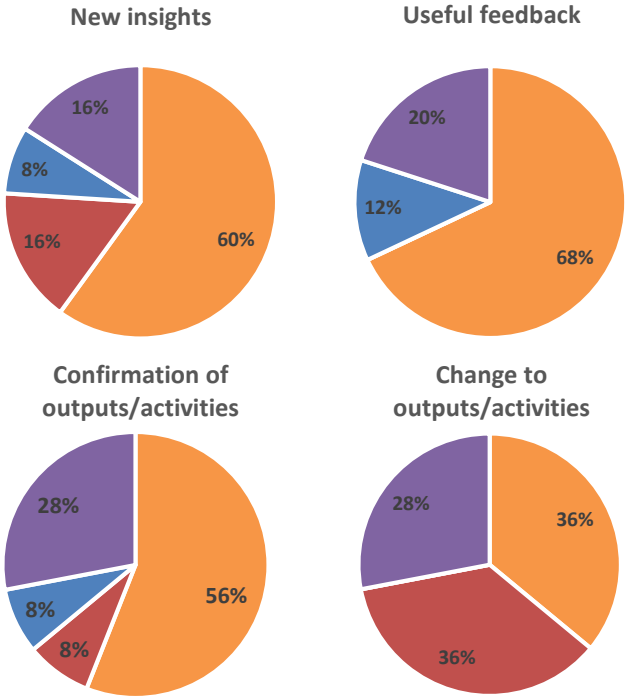
- Online survey of EXAG and Consortium members
- Responses from 25 consortium members from across 26 partner organisations and 5 EXAG members
- Aim to evaluate how well the EXAG had supported HTA and regulatory engagement and to assess the impact of the EXAG's feedback

EXAG Survey – Engagement



- High levels of engagement with the EXAG
- Less certainty about future engagement
- Reasons for not engaging included members being new to the project, not needing feedback on project outputs and not being aware that they were able to nominate a topic for discussion.

EXAG Survey – Role and impact



- Positive experiences received about the EXAG in fulfilling the roles for which it was established
- Overall, the findings demonstrate a positive impact of the EXAG's feedback on project outputs and activities
- Main benefits included:
  - Increased understanding of the perspective of regulators/HTA about specific areas of work
  - Validation of current strategies
  - Provision of feedback that would shape future work.

EXAG Survey – areas for improvement and best practice

Improvement	Best practice
Meeting length and frequency, particularly the need for more frequent face-to face meetings	**Mix of competences and perspectives** from across the regulatory and HTA landscape
Scheduling of meetings to meet all stakeholders' needs	**Right size of group** to allow for focused activity and discussion
Open Q&A sessions for all observers	**Challenging discussions** between experts and project partners
Less regimented virtual meetings to allow for more open discussion	
Broader EXAG membership to reflect the perspectives of more European countries	

"Overall, I think having the EXAG in ROADMAP is excellent as it would be good to ensure that, when there is an efficacious, safe drug to treat AD, it can be accessed by patients who need them as soon as possible."

Conclusions

- Valued role of EXAG feedback – usability of project outputs in regulatory and HTA settings
- Need for consensus on priority outcomes for regulatory and HTA decision-making on new treatments
- Clear role for real-world evidence to support decision making

Questions

- Any questions?
- Thank you!
- More info:
  - www.roadmap-Alzheimer.org
  - www.nice.org.uk/research
  - Diana.ORourke@nice.org.uk