REGULATORY AND HTA CONSIDERATIONS FOR DISEASE-MODIFYING DRUGS IN ALZHEIMER'S DISEASE

Diana O'Rourke[1], Jacoline Bouvy[1], Pall Jonsson[1]

1. National Institute for Health and Care Excellence (NICE)

Facts & Figures

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Contributions	
IMI funding:	3 998 250 €
EFPIA in kind:	4 210 843 €
Other:	1 288 €
Total Cost:	8 210 381 €
Project website:	www.roadmap-alzheimer.org
Social media:	@IMI2_ROADMAP

Challenge

New therapies are needed to improve outcomes for people with Alzheimer's Disease (AD). Disease-modifying treatments targeting the earlier disease stages of AD are being developed. However, unlike current treatments, the evidence needed to ensure successful regulatory and HTA approval is unclear. There is therefore a need to establish a consensus on regulatory and HTA requirements to ensure access to these treatments when they become available.

Approach & Methodology

ROADMAP aims to optimise the use of real-world evidence (RWE) to inform decision making on developing new treatments. An Expert Advisory Group (EXAG), consisting of European regulatory and HTA experts, was established to provide guidance on the use of RWE from a regulatory and HTA perspective.

Table 1. Topics discussed by the EXAG

Priority outcomes across key stakeholders
Disease stage definitions
Disease-progression and economic modelling
Approaches for real world data collection
The 'Data Cube' – integration of data sources with priority outcomes and disease stages

Results

During the course of the ROADMAP project, the EXAG explored a range of topics with the project's different work packages (Table 1) leading to a series of recommendations identifying the steps needed to prepare Europe's healthcare systems for a disease-modifying drug for AD (Table 2).

Table 2. Regulatory/HTA considerations

Establish outcomes for regulators and HTAs in early AD that can: • Distinguish between AD and other types of dementia • Measure a delay in AD onset; • Measure a meaningful delay in AD progression
Establish caregiver-relevant outcomes to support economic modelling
Explore the use of modern technology to measure outcomes across different settings
Real-world evidence will be needed to support disease progression modelling assumptions and to provide information on the differences between national and regional settings
The Data Cube will allow ROADMAP to identify gaps in data from different data sources

Value of IMI collaboration

ROADMAP has sought to achieve an open dialogue between a broad range of stakeholders on the use of real-world evidence for the benefit of people with AD and their carers. Regulatory and HTA engagement, through the EXAG, has been central to the project. The EXAG has provided a unique opportunity for ROADMAP to elicit the views of regulators and HTA bodies on the relevant outcomes and evidence requirements needed for decision-making for new AD treatments.

Impact & take home message

The EXAG identified a clear need to agree on the outcomes and evidence needed to inform regulatory and HTA decision-making. Once agreed, the challenge will be to better utilise existing data sources and to fill the gaps in the evidence base, particularly through the generation of real-world data. This will ensure that Europe's healthcare systems are a step closer towards being ready for a future disease-modifying drug for AD.

imi innovative medicines initiative efpia

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