INTRODUCTION:

There has been a move towards the development of disease-modifying agents in Alzheimer's disease (AD) and it is likely that early disease-modifying treatments will initially be offered to people who have positive AD markers and mild cognitive impairment (MCI). Consequently, disease-modifying drugs will have distinctive features as compared to currently licensed symptomatic treatments, which makes the implications of these new agents for regulatory and health technology assessment (HTA) processes unclear.

METHODS:

The ROADMAP (Real-world Outcomes across the AD spectrum for better care: Multi-modal data Access Platform) project provides the foundation for a European data platform for real-world evidence in AD, and established an expert advisory group (EXAG) consisting of regulatory and HTA experts. This presentation will summarize the key lessons from the first year of ROADMAP's EXAG and identifies the next steps that are required to prepare Europe's healthcare systems for a disease-modifying drug.

RESULTS:

The EXAG identified a need for: (i) establishing the rationale for the selection of priority outcomes in pre-clinical AD and MCI; (ii) establishing accepted outcomes for defining prevention of AD or delayed AD onset; (iii) exploring modern technology that could assist in testing cognition that could easily be used in clinical practice; and (iv) establishing caregiver-relevant outcomes (e.g. quality of life, loss of income, carer time) that are important to capture; and found that not all evidence to support modelling assumptions can be generated through RCTs, making the case for using real-world evidence.

CONCLUSIONS:

Many of the challenges that the EXAG identified can be solved by generating better real-world data in AD. There is a clear need to agree on the outcomes that will facilitate and inform regulatory and HTA decision-making. Once the gaps in the availability of outcomes in AD will be closed, we will be one step closer towards being ready for a disease-modifying drug.

OP171 Does Parallel Regulatory-Health Technology Assessment Reviews Affect Time To Health Technology Assessment Decisions?

AUTHORS:

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INTRODUCTION:

Timely recommendation by Health Technology Assessment (HTA) agencies for drug reimbursement is critical to ensure patient access to medicines of therapeutic value. In this study, HTA performance was examined in terms of their outcome and timing by looking at how 103 drugs, which gained regulatory approval from 2013 to 2015, were assessed by HTA agencies from 2014 to 2016.

METHODS:

Products must have received regulatory approval from one of the following regulatory agencies: EMA (Europe), Health Canada (Canada) and TGA (Australia). The first HTA recommendations were then collected from PBAC (Australia), CADTH (Canada), HAS (France), IQWiG (Germany), SMC (Scotland) and TLV (Sweden). The HTA decisions were classified as positive, positive with restrictions, negative and multiple.

RESULTS:

Eighty-four drugs were approved in Europe before Australia and Canada. Of the studied HTA agencies, PBAC had the highest percentage of products recommended within a year from regulatory approval (93 percent). In addition, Australia had the shortest median time between first regulatory submission by any of the three agencies and HTA recommendation (553 days) as compared to Europe (616 days) and Canada (722 days). This can be attributed to the TGA/PBAC parallel process. However, Australia has the highest proportion of products receiving a negative PBAC recommendation (62 percent).

CONCLUSIONS:

The majority of drugs were first submitted for reimbursement in Europe, but the time from regulatory submission to HTA decision was the fastest in Australia.

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This can be attributed to the TGA/PBAC parallel review process, which showed its benefit in reducing the overall time. A parallel review process is also available in Canada; however, it is not utilized as frequently by companies as in Australia.

OP172 Do Expedited Regulatory Pathways Affect Time To Health Technology Assessment Decision?

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INTRODUCTION:

In an effort to speed the assessment of new medicines while maintaining the quality of the regulatory review, facilitated regulatory pathways (FRPs) have been introduced in many countries. In this study, the effects of FRPs (expedited and conditional reviews) were investigated in terms of their influence on HTA outcomes and timing.

METHODS:

HTA recommendations issued between 2014 and 2016 were collected from CADTH (Canada), HAS (France), IQWIG (Germany), SMC (Scotland) and TLV (Sweden) for 90 internationalized medicines (new active substances approved between 2012 and 2016 by all regulatory agencies in the five jurisdictions). The HTA decisions were then classified into the following categories: positive, positive with restrictions, negative and multiple.

RESULTS:

Of this cohort of internationalized medicines that received an HTA recommendation, 31 percent in Canada and 28 percent in Europe were approved via a FRP. With the exception of Scotland, expedited medicines were more likely to be appraised within a year from regulatory approval and had a shorter median time between regulatory approval to HTA recommendation than standard medicines. The largest difference was seen in Sweden, where medicines were 66.5 days faster than standard pathways when it underwent the expedited pathways. Compared to standard pathways, there were generally a higher proportion of positive and positive with restrictions recommendations when expedited pathways were used. Germany reported the largest proportional difference (31 percent) between the two pathways.

CONCLUSIONS:

Medicines being designated for an expedited review pathway show a reduced time from regulatory approval to HTA decision. This finding suggests there is an alignment between regulators and HTA agencies on which medicines require expedited HTA pathways; however, from this data it cannot be assessed whether the reduced time from approval to HTA decision is attributed to the company strategy, HTA review time or both. Further investigation is required.

OP173 Eligibility Criteria For "Accelerated Access" Approval: A Global Survey

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INTRODUCTION:

Several early access schemes (EAS) exist, which aim to accelerate patient access to new, potentially life-saving therapies. While some information exists on key schemes and their modalities, the determinants that drive adoption of a new medicine under an EAS remain unclear. We aimed to map eligibility criteria for inclusion of new medicines into the different EAS available across countries.

METHODS:

Health technology assessment (HTA) stakeholders across 23 countries globally were invited via email to complete a web-survey with questions on (i) items that define product eligibility for EAS designation, (ii) standards for minimum level of evidence, monitoring, and additional evidence generation for early access products, and (iii) funding arrangements for these products across settings and types of schemes. Anonymized responses were analysed using descriptive statistics.

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