Planning Real-World Data Collection to Inform HTA Alzheimer's Disease

Report of Roundtable - 18 February 2026

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Introduction

On the 18th of February 2026, the RWE4Decisions multi-stakeholder Learning Network hosted a Roundtable virtual meeting on **Planning real-world data (RWD) collection to demonstrate real-life effectiveness and inform economic modelling, using Alzheimer's disease (AD) as a case study**. Over sixty participants representing HTA/Payers, national health ministries, regulators, EU institutions, patients, data analysts, registry holders, academics, and the pharmaceutical industry participated. The meeting was co-moderated by Niklas Hedberg (TLV) and Karen Facey (RWE4Decisions Senior Advisor). A keynote presentation and three case study presentations, was followed by panel questions, multi-stakeholder breakout dicussions and plenary reflections.

Keynote presentation: Learnings from the HMA/EMA workshop on Patient Registries for AD: Kelly Plueschke (European Medicines Agency)

Kelly Plueschke (EMA) set the stage by presenting the learnings from the HMA/EMA workshop on Patient Registries for Alzheimer's Disease, which took place on 15 December 2025. The rationale behind the workshop was as follows:

- Alzheimer's disease represents a major public health issue, accounting for 60-80% of dementia cases, with dementia being a leading cause of disability in older adults.
- The treatment landscape is evolving rapidly, with nearly 140 medicines in development.
- In 2025, two monoclonal antibodies were authorised for the treatment of mild cognitive impairment and mild dementia due to Alzheimer's disease. These medicines are associated with risks. As a result, the EMA's Committee for Medicinal Products for Human Use imposed risk-minimisation measures, which led to questions on whether RWD from patient registries could be used to address existing knowledge gaps in this therapeutic area.

Kelly summarised the feedback shared by stakeholders. From the **patient perspective**, data sharing was not considered an issue provided that data are kept safe and secure. Trust, protection of personal data and transparency were identified as essential. Patients stressed the importance of knowing how their data would be used, why they would be used and by whom, as well as whether they would still be able to access their data after collection. They need to be involved throughout data processing and highlighted the importance of smarter collaboration to reduce duplication of data entry and address the current fragmentation of data sources.

From the **industry perspective**, four main domains were highlighted to ensure registry data are fit for use. These included the need for robust data capture infrastructure that can be supported through data linkage and adapted as new data needs arise; clear and transparent governance for data collection, use and sharing; data of good quality, meaning reliable and relevant to their intended use; and appropriate expertise within registry teams, including pharmaco-epidemiology and biostatistics.

From a **regulatory perspective**, it was noted that RWD can complement clinical trial data, while acknowledging known limitations. Regulators emphasised the importance of pre-planned studies with protocols published before study initiation. The EMA catalogue of RWE studies are a mechanism to support transparency, data sharing and research on medicines. Early dialogue with regulators is feasible and recommended, noting that both EMA and FDA presented their scientific advice procedures at the workshop. In addition, the EMA qualification process was highlighted as a way for regulators to evaluate the relevance of registry data to address questions related to a specific context of use.

Regarding **HTA perspectives**, there is a recognised need to invest in registries that collect data on untreated and treated patients, on- and off-label use of medicines, and key dates such as treatment start, stop and treatment switches.

From the **payer perspective**, registries were described as useful for generating long-term RWE (real-world evidence) on effectiveness and safety to justify investment, given the modest clinical benefit of the currently authorised medicines. Registry data were also seen as a tool to verify that patients meet eligibility criteria and to prevent use outside the reimbursed population.

From the **clinician perspective**, a key point raised was the need to move towards disease-focused registries rather than treatment-focused registries, in order to address the current scattered landscape of data sources and to facilitate patient follow-up. Clinicians also noted that while high-quality data are important, data collection must remain feasible within the constraints of clinical practice, and therefore there is a need to be pragmatic in defining which data should be collected.

The output of the workshop can be summarised in three main points:

- There is a need to define a core dataset for Alzheimer's disease that aligns with the EMA data quality framework, is acceptable for as many stakeholders as possible and is fit for use, which requires collaboration to identify a minimum core dataset.
- Patients and caregivers should be involved to ensure that the data collected are relevant and beneficial to them.
- There is a need to move towards large, disease-based registries instead of treatment-focused registries to allow comparisons between treated and untreated patients, between products and between countries, while addressing data fragmentation. This may be addressed through sustainable and long-term financial support, including funding from public institutions and governments, rather than relying solely on project-based funding.

The workshop report is expected to be published in May 2026, and there is ongoing work on drafting a minimum core list of data variables. This list will be developed in consultation with experts from the regulatory network, published for broader consultation, updated following feedback and ultimately published in a peer-reviewed journal.

Case study presentations and reflections

The keynote address fed into a panel discussion which reflected on the role of RWE in Alzheimer's disease across the regulatory and HTA/Payer contexts, drawing on national registry experience, health system data, and industry perspectives on evidence gaps beyond clinical trials. The panel discussion started with three case-study specific presentations from Prof. Dorota Religa (Swedish Registry for Cognitive/Dementia Disorders), Shaun Rowark (NICE) and Matt Dixon (BMS).

Overview of the SveDem (Registry for Cognitivie/Dementia Disorders) – Data collection challenges and implications for clinical practice: Prof. Dorota Religa, Karolinska Institutet (SveDem)

This presentation offered an insight from the Swedish Dementia Registry (SveDem) and its experience in planning for, and undertaking, RWD collection in Alzheimer's disease and dementia. Prof. Religa described work in dementia as a "marathon", noting that patients are typically followed over many years in clinical practice. SveDem has been in place for almost 20 years and has registered approximately 150,000 patients, covering around 40% of incident dementia cases in Sweden, while acknowledging ongoing challenges such as underdiagnosis and stigma.

The registry is structured to follow patients throughout the care pathway, from primary care to specialist care and long-term care settings. SveDem collects data from all memory clinics in Sweden, 78% of units in primary care, 754 nursing homes and 200 home-care units. By February 2026 it contained information about 150,000 individuals with dementia or mild cognitive impairment (covering about 35-40% of incident dementia cases/year. It is financed through the Swedish Association of Local Authorities and Regions. Participating units receive regular feedback reports to support local quality improvement, and annual meetings and reports are used to discuss and highlight results. Follow-up of patients is conducted once per year, reflecting the slow progression of the disease.

The registry is designed to collect a selected set of variables, with most variables recorded as simple yes/no responses, to minimise burden on clinicians. Variables are reviewed annually, with the principle that adding new variables should be balanced by removing others. Some clinical results, such as cognitive test scores, and imaging findings can be recorded when

available but are not mandatory. Processes for automated transfer of data from medical records to the registry are currently explored and tested. As the unique personal number is used, the registry data can be linked with other national and regional health and social care datasets. Long-term follow-up of patients is essential to assess issues such as long-term effects on cognition and functional ability, and to identify prognostic factors. When a new medicine is authorised, a new drug module has been developed and is ready to be deployed to follow patients on new therapies with focus on real-world effectiveness.

SveDem provides RWD from routine clinical practice, including patients with comorbidities and varied living situations, and the registry can be scaled, linked with other data sources, and used to follow the implementation and use of new Alzheimer's disease treatments. Prof. Religa concluded by underlining the user-friendly nature of the registry and the importance of collaboration and education to further develop the registry model for future therapies.

The role of NICE in the ConnectD project informing development of a national AD registry: Shaun Rowark (NICE)

Shaun Rowark, Associate Director for Healthcare Data Analytics at NICE, presented an HTA perspective on Alzheimer's disease, dementia and the use of RWE for health technology assessment (HTA) in the UK.

Approximately 982,000 people currently living with dementia in the UK, and this is projected to rise to 1.4 million people by 2040. About two-thirds of people with dementia in the UK have Alzheimer's disease. The economic impact of dementia is substantial, with costs forecast at around £42 billion in 2024, increasing over time due to population growth and ageing. Most dementia-related costs in the UK are borne by social care and unpaid carers, while healthcare costs account for a smaller proportion, with the majority of these arising from non-elective inpatient care rather than medicines or diagnostic procedures.

NICE recommended treatments for AD include supportive care and older medicines for mild to severe AD – this relates to <6% of the dementia population. Innovative new treatments that target the underlying amyloid pathology have not been recommended due to uncertainties in cost effectiveness. Furthermore, challenges defining the patient population were noted as Alzheimer's disease is currently staged in the UK using the Mini-Mental State Examination (MMSE) but clinical trials were based on PET scans or cerebrospoinal fluid biomarkers, and neither are validated. Importantly, these recommendations on emerging technologies are currently draft guidance and do not represent a final decision.

Future Alzheimer's disease medicines are seen as potentially disruptive due to challenges in patient identification, diagnostic capacity, high patient volumes, high budget impact for the NHS, required service transformation, and strong public and media interest. Unlike other disease areas such as oncology, there is currently no clear route in the UK for generating the RWE needed to support decision-making in dementia.

NICE use of RWE in other disease areas, such as cancer, were described, as well as the limitations of applying similar approaches to dementia at present. This led to the introduction of the NHS England-funded **ConnectD project**, which is aiming to create a secure, shared dementia data system in England. It seeks to link data from five sub-national secure data environments to form a testbed for a future national dementia registry, building on data already collected in routine practice. Initially this will cover 100,000 people with dementia.

The project is currently in a proof-of-concept phase, focused on governance approvals, analyst access to the secure data environment, and defining a minimum viable dementia dataset. An initial evaluation report is planned by the end of 2026, assessing data content and quality and its suitability for NICE use cases. In subsequent phases, the project aims to expand data linkage, characterise patient cohorts, undertake modelling analyses, and establish a process to support future NICE technology appraisals in dementia.

How can RWE complement RCTs to demonstrate the burden of Alzheimer's Disease and benefits of treatment to patients and carers?: Matt Dixon (Bristol Myers Squibb)

The final presentation was delivered by Matt Dixon, Director of Global Health Economics and Outcomes Research in Alzheimer's disease at Bristol Myers Squibb, who gave an industry perspective on the role of RWE in Alzheimer's disease.

While randomised trials are essential for establishing efficacy and safety, they are conducted in controlled settings and over limited time horizons, whereas Alzheimer's disease progresses over many years and its burden extends beyond the patient. As a result, not all outcomes that matter to patients, caregivers, clinicians, payers and regulators can be fully captured within trials, leading to predictable evidence gaps.

There are several key evidence domains where RWE can add value. These include understanding durability and long-term outcomes beyond the randomised trial period, treatment persistence and safety in broader populations, and how short-term trial effects translate into longer-term clinical and system-level impacts. RWE plays a significant role in understanding real-world treatment use, including initiation, adjustment and discontinuation of therapies, transitions across care settings, and outcomes beyond traditional clinical endpoints, such as functioning, behavioral stability, avoidance of crisis-driven care, caregiver burden and, where possible, patient quality of life. Early engagement with patients and caregivers is essential to inform outcome selection and interpretation.

Matt Dixon further discussed the use of RWE to contextualise trial findings in routine practice, including its potential role in enhancing generalisability and, where appropriate, supporting external comparator approaches, while stressing the need for early alignment with stakeholders, robust methodology and transparency. He also highlighted challenges related to differences in care delivery, diagnostic pathways, specialist access and caregiver support across regions, and the need to consider generalisability and transportability of evidence.

Key challenges in generating credible RWE in Alzheimer's disease were outlined, including variable clinical detail across data sources, inconsistent measurement of cognitive and neuropsychiatric outcomes, limited capture of caregiver burden and patient-reported outcomes, and uncertainty in translating short-term outcomes into long-term value. Data must be fit for purpose and the research question should drive data strategy, often requiring triangulation across multiple data sources. These challenges are linked to the importance of defining a core dataset for Alzheimer's disease registries, as discussed earlier in the meeting.

Finally, Matt Dixon emphasised that RWE in Alzheimer's disease must be intentionally designed to extend trial findings, reflect long-term disease burden and treatment impact, and rely on transparent methodology and collaboration across stakeholders. Demonstrating value requires agreement on what outcomes matter, what can be measured, and how uncertainty is managed.

Q&A

The presentations section finished with a Q&A, which identified a number of additional points for consideration:

- Participants highlighted a "chicken-and-egg" problem: diagnostics pathways are typically only established once a treatment is available, yet new treatments cannot be recommended unless patients can first be diagnosed. This dependency creates uncertainty for HTA decision-making and delays adoption in clinical practice.
- There is uncertainty about the size and characteristics of the patient population eligible for new Alzheimer's disease treatments, and whether these patients can be reliably identified in practice. RWD could help inform assumptions used in HTA about the patients who will receive treatment according to the reimbursement conditions.

- There are difficulties in finding the right patients in existing registries due to underdiagnosis and variable diagnostic practices. This limits confidence in population definitions.
- Access to historical datasets and long-term follow-up data was seen as critical but challenging. Such data are needed to understand disease progression and long-term outcomes.
- A key challenge is extrapolating short-term trial evidence to long-term effectiveness. This was described as both a data and a methodological problem.
- Although many countries have rich data, these are often fragmented across systems. Linking registries with existing data assets is valuable but resource-intensive and challenging from a technical and legal standpoint.
- Concerns were raised about whether existing data sources are fit for purpose, given inconsistent capture of key clinical measures. Agreement on minimum or core datasets was seen as essential.

Breakout group discussions

Following the presentations, participants were divided into multi-stakeholder breakout rooms facilitated by Elena Lungu (CDA-AMC), Seamus Kent (Rotterdam University), and Christian Dehlendorff (Danish Medicines Council), with the support of scribes Dimitri Pouradier-Duteil (BMS), Gabriela Ochoa Gracia (Rotterdam University), and Prof. Dalia Dawoud (Cytel Inc.).

Discussions highlighted several key themes and challenges of generating and using RWE in Alzheimer's disease, including the definition of core datasets, the balance between standardisation and burden, the relevance of patient and caregiver outcomes, data fragmentation and linkage, and the limits of rRWE for decision-making.

- **Core datasets, standardisation and burden**
  - A core dataset or core outcome set is needed to support regulatory, HTA and payer decision-making.
  - Data collection may create burden for patients, caregivers and healthcare systems, particularly in a long-term, progressive disease.
  - In Alzheimer's disease, efforts to standardise datasets must balance the need for minimal, uniform data with the considerable variability in patient presentation and individualised treatment goals.
  - Complementary approaches should be put in place to augment registry data, such as surveys or targeted studies, and linkage can be used, as can linkage to other health system and social care data.

- **Patient and caregiver relevance**
  - Data collection must reflect outcomes that matter to patients and caregivers.
  - Patient priorities differ, with some focusing on cognition and others on autonomy or daily functioning.
  - Data collection needs to be simple, flexible and meaningful to support sustained participation.
  - Caregiver impact is a critical outcome and varies across healthcare systems and regions.

- **Innovation versus validation**
  - Digital tools and gamification offer ways to capture cognitive and functional outcomes longitudinally and in more natural settings.

  - Innovative tools risk over-monitoring and may induce anxiety if health signals are over-emphasised.
  - Validation pathways are required for new tools to be accepted by regulators and decision makers.
  - Innovation must be balanced with standardisation and methodological credibility.

- **Data landscape, linkage and transportability**
  - Existing data are fragmented across care settings and data sources, but mapping existing datasets is necessary to understand available data and remaining gaps.
  - Data linkage increases analytical value but requires substantial resources and governance.
  - National data linkage is more feasible than international linkage.
  - Differences in diagnostic practices and care pathways limit cross-country transportability of evidence.

- **Role and limits of RWE in decisions**
  - RWE supports understanding of long-term outcomes, disease progression and natural history under current care.
  - The value of RWE and its acceptability to payers remains debated, and some level of risk-sharing is likely required to support its use in decision-making.
  - While RWE can help reduce uncertainty, residual uncertainty remains and may need to be addressed through mechanisms such as managed entry agreements (MEAs).
  - Some constraints on reimbursement decisions relate to system capacity, affordability and care pathways rather than evidence gaps.
  - Early and proactive planning is required to align RWE generation with future decision needs.

Conclusion

The Roundtable discussions highlighted the evolving role of RWD and RWE in supporting decision-making in Alzheimer's disease. The presentations and case examples illustrated how RWE can complement clinical trials by improving understanding of long-term outcomes, real-world effectiveness, treatment use in routine practice, and the broader impact on patients and caregivers. Experience from national registries, health system data initiatives and industry perspectives demonstrated how RWE can help contextualise trial evidence and inform regulatory, HTA and payer considerations, particularly in a rapidly changing therapeutic landscape.

The discussions also reflected growing alignment on the value of RWE as a complementary source of evidence, alongside the importance of clear data standards, fit-for-purpose methodologies and early planning of evidence generation.

Overall, the Roundtable reinforced that sustained collaboration across stakeholders, combined with improved data infrastructure and shared frameworks, can strengthen the contribution of RWE to informed decision-making in Alzheimer's disease and other disease areas.

This Roundtable is part of the wider RWE4Decisions 2026 workplan, linking directly to ongoing activities on registries, patient-relevant outcomes, transportability and methodological approaches to the use of RWE across disease areas. By including a use case on Alzheimer's disease within this broader programme of work, the Roundtable reinforced the role of the Learning Network as a platform for cross-cutting dialogue and for advancing shared approaches to RWE beyond a single therapeutic area.

RWE4Decisions is an HTA/Payer-led multi-stakeholder Learning Network, which, in 2024, developed of a set of new Stakeholder Actions to generate better real-world evidence for HTA/payer decisions about highly innovative technologies.

The work has been commissioned by the Belgian National Institute of Health and Disability Insurance (NIHDI) and is led by a multi-stakeholder Steering Group with a wider community of contributors including HTA bodies and payers, regulatory agencies, patient groups, clinical teams, industry, analytics experts and academic experts/researchers. The RWE4Decisions Secretariat is provided by FIPRA, with sponsorship in 2026 by the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE), Astellas, AstraZeneca, Bayer, BMS, Chiesi, MSD, Roche and Takeda.

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