HMA
Heads of Medicines Agencies

EMA
EUROPEAN MEDICINES AGENCY

Joint HMA/EMA multi-stakeholder workshop on Patient Registries for Alzheimer's disease

Workshop report

15 December 2025

Contents

1. Executive summary

Dementia is the leading cause of disability and dependency in older adults, affecting 9 million people in the EU [1]. Alzheimer's disease is the most common form of dementia and is thought to account for 60-80% of cases. While a diagnosis of Alzheimer's disease changes a person's life and that of their relatives, a very active research ecosystem feeds substantial hopes for improved outcomes in the near future [2]. This includes the emergence of new classes of medicines to treat mild cognitive impairment or mild dementia due to Alzheimer's disease. However, as new medicines reach the market, knowledge gaps are being identified which need to be filled, including on the effectiveness and safety of these treatments, and on the consequent trajectory of the disease.

Clinical evidence is at the heart of well-informed decisions on the development, authorisation, reimbursement, use and monitoring of medicines. Whilst patient registries play a key role in collecting data, including in the long-term, to support some of these decisions, the current landscape of Alzheimer's disease registries is fragmented across the European Union. The fast-evolving treatment landscape creates a unique momentum to strengthen collaboration across stakeholders for a smarter and better coordinated data collection that will address evidence needs from patients, healthcare professionals, but also from regulators, health technology assessment bodies, payers, research groups and the pharmaceutical industry.

On 15 December 2025, the Heads of Medicines Agencies (HMA) and the European Medicines Agency (EMA) held a multistakeholder workshop to discuss how patient registries can be best leveraged to collect data and fill evidence gaps in Alzheimer's disease, its prevention and its treatments. After listening to the perspectives from all stakeholder groups, and learning about the evidence gaps, challenges, but also opportunities offered by existing patient registries such as *Svedem* [3] and *InRAD* [4], the participants agreed on a series of key principles and follow-up actions.

Key principles	Description
Core dataset harmonised across registries	• Stakeholders should work together to define a core dataset to be implemented across Alzheimer's patient registries that is fit-for-use in multiple contexts. Existing international consensus efforts will be considered and used as a basis. • This core dataset should meet immediate needs of patients, healthcare professionals, research groups as well as those of the regulators, especially in terms of data quality. ***Action**: EMA to launch a public consultation in 2026 on a core dataset for Alzheimer's patient registries*
Engagement with patients / caregivers and healthcare professionals	• Patients, caregivers and healthcare professionals must be involved in defining this core dataset to ensure the collected data reflect outcomes that matter to them and ultimately bring them a benefit, through better treatments, care and information, whilst balancing the feasibility and burden of data collection in routine clinical practice. • Collection and sharing of data should be handled in a secured, trusted and transparent way to ensure patients' confidence and engagement.

Key principles	Description
	***Action**: EMA to actively involve patients/caregivers and healthcare professionals' organisations in the context of the public consultation on a core dataset for Alzheimer's patient registries.*
"Disease-based" patient registries for decision-making on medicines	• Efforts should be made to progress towards large disease-based patient registries designed to capture data on all treatments allowing comparisons 1) between treated and untreated patients, 2) across treatments and 3) across countries, to avoid fragmented data collection and underpowered studies. ***Action**: Regulatory/Health Technology Assessment bodies/Payer requirements on the need to use "disease-based" patient registries instead of single treatment-based data collection systems to be clearly communicated, e.g. in the context of the revised* EMA Guideline on registry-based studies *[5], and other engagement activities with stakeholders.*
Financial support	• There is a need for sustainable support through permanent funding under clear governance principles from multiple interested parties, including public institutions (e.g., governments) and private bodies (e.g., pharmaceutical industry), rather than relying only on project-based funding. This will help patient registries to collect high-quality meaningful data in a sustainable way, adapt as treatment landscape evolves, and better integrate into healthcare systems. ***Action**: Public institutions (e.g., governments) and private bodies (e.g., pharmaceutical industry) to invest in the development of high-quality patient registries under clear governance rules where patients own their data, and to financially support their maintenance for long-term management of patients care. This important aspect will be highlighted in the revised EMA Guideline on registry-based studies [5], and awareness will be regularly raised by EMA during engagement activities with various stakeholders.*
Data linkage, use of digital tools and Artificial Intelligence (AI)	• The possibility for data linkage should be further investigated to supplement registry data and reduce the burden on clinicians and patients/caregivers. The use of digital tools/AI may be a mean to address this aspect. ***Action**: Patient registries, with the support from public institutions (e.g., governments) and private bodies (e.g., pharmaceutical industry), to explore and invest in this area in view to optimise data processing and stimulate clinicians, patients/caregivers to contribute to research on the development of safe and effective treatments.*

More information about the workshop can be found on the *dedicated EMA webpage* [6].

2. Clinical context and objectives of the workshop

2.1. Alzheimer's disease and current therapies

The treatment landscape for Alzheimer's disease is rapidly evolving. In November 2024, EMA published a *horizon scanning report* [2] describing key emerging trends in the field and highlighting challenges and opportunities related to the development of disease-modifying treatments.

The 2025 Alzheimer's disease development pipeline includes nearly 140 medicines in development, one third of which are repurposed treatments. These products target various aspects and mechanisms of the disease.

For decades, only a small number of treatments - cholinesterase inhibitors and NMDA receptor agonists - have been authorised in the European Union (EU) for Alzheimer's disease. In 2025, a new class of medicines emerged with the EU authorisation of two monoclonal antibodies targeting amyloid beta to treat mild cognitive impairment or mild dementia due to Alzheimer's disease. Both medicines are associated with potentially serious side effects (amyloid-related imaging abnormalities, ARIA), and to mitigate these risks, a controlled access programme and additional risk minimisation measures have been put in place. Knowledge gaps have also been identified with this new class of medicines, and will be addressed based on real-world clinical experience:

- safety risks, especially long-term consequences of ARIA; effects on cognition; brain atrophy;
- long-term treatment strategies, including criteria for discontinuation and retreatment, for example informed by data of amyloid re-accumulation.

Another key aspect relates to the prevention of symptomatic Alzheimer's disease, especially for patients with subtle detectable abnormalities but no functional impairment: how can pre-clinical, and asymptomatic Alzheimer's disease be investigated to allow the development of a preventive approach?

An overview of Alzheimer's disease, its epidemiology, and the unmet medical need is available here: *Introduction on Alzheimer's Disease and current therapies* [7].

2.2. EU-funded initiatives

Several initiatives are ongoing at EU level in the context of Alzheimer's disease. The EU Health programme (2021-2027) includes a 3-year Joint Action on dementia and other neurological disorders, *JADE Health* [8], which started in January 2025 and touches upon data accessibility.

In addition, as part of Horizon Europe, the European Commission's dedicated programme for research and innovation, three initiatives relevant to real-word data (RWD) including patient registries, can be highlighted:

- the 5-year public-private partnership *ACCESS AD* [9], which is part of *EU innovative Health Initiative* [10] (start: January 2026). One of its objectives is to establish a pan-European registry for Alzheimer's disease and to facilitate dialogue between all stakeholders;
- the 10-year partnership *European partnership for Brain Health* [11] (start: January 2026), which mission is to promote the translation of research findings into tailored solutions for prevention, diagnosis, treatment and care, accessible to all. By connecting stakeholders, it could facilitate the follow-up of persons using patient registries;

- *CHIEF (Collaborative Health Information European Framework)* [12] aimed to propose a framework for the sustainable collection of indicators for non-communicable diseases (started: 2023).

More details on these initiatives can be found in the *Opening remarks from the European Commission* [13].

2.3. Objectives of the workshop

This joint hybrid HMA/EMA workshop on patient registries was part of the *Network Data Steering Group workplan 2025-2028* [14], and brought together representatives of patients, healthcare professionals, registry holders, regulatory agencies, pharmaceutical industry, academia and non-for-profit research organisations, health technology assessment bodies and payers.

The main objectives were to agree on recommendations for optimising stakeholders' collaboration to facilitate the long-term follow-up of patients and enable the generation of meaningful evidence on the safety and effectiveness of medicines using patient registries. Discussions focused on:

- Raising awareness of the evidence gaps related to current and upcoming therapies that could potentially be addressed using real-world data (RWD);
- Identifying core data elements to be collected in patient registries to enable the evaluation of therapies and the effectiveness of their risk minimisation measures;
- Aligning on recommendations regarding patient consent, governance for accessing and sharing data, quality assurance and registry interoperability.

All the workshop documentation (agenda, reports, presentations, recording) can be found on the *dedicated EMA webpage* [6].

3. Stakeholders' perspectives on evidence gaps and potential of RWD

The different stakeholder groups shared their perspectives on the evidence gaps and potential of RWD, including patient registry data, in Alzheimer's disease.

3.1. Patient perspective

The importance of patient registries in monitoring the use, safety and effectiveness of treatment options was highlighted. Participation in studies is key to support progress in research and deepens understanding of the disease. Patient Reported Outcome Measures and Patient Reported Experience Measures (PROMs/PREMs) are critical tools that allow capturing not only clinical outcomes but also lived experience, including treatment burden, impact on daily life, and the quality of interaction with the healthcare system.

For patients, data sharing is not considered an issue as long as information is kept safe and secure. In other words, it should not compromise privacy nor confidentiality. Patients should know how their data will be used, who will have access to them, and whether they will be able to access them themselves. Building trust requires clear and transparent explanations of all aspects of data processing, so that patients feel involved throughout.

There is also a need for more efficient collaboration to reduce duplication of data collection and lift the burden on patients and their families, who want to see tangible benefits from sharing their data.

The importance of larger sample sizes and trials that reflect real-life experience across different countries was underlined, as well as the need to involve patients in the design and governance of patient registries. The full presentation can be found here: *Lived experience of Alzheimer's Disease (European Working Group of People with Dementia)* [15].

Alzheimer Europe presented the results of an analysis carried out to better understand European perspectives on data sharing. The project involved an opinion poll targeting the general public, focus groups with patients with dementia and caregivers, interviews with researchers complemented with a literature review.

The findings highlight that data sharing is extremely important for progressing research and finding better treatments for dementia. However, they also show that there is a need for reassurance that a general agreement to share will not lead to misuse or unexpected problems.

Key learnings include:

- Trust: the protection of privacy, in particular when sensitive genetic data are shared is critical. Accessible informed consent procedures (in lay language) and awareness-raising activities can support digital literacy and trust building;
- Communication: clear explanations and transparency about how data will be used, and continuous provision of feedback to patients on who can access their data is essential; taking carers' perspective into account is also very important;
- Fragmentation: this issue creates additional burden for patients and carers due to the scattered landscape of patient registries and inconsistent informed consent procedures.

Further information on this project can be found in *Patient representative perspective: Understanding European perspectives on data sharing [16]*, as well as in the *Dementia Research Participation and Data Sharing in Europe report* [17] published in 2026 by Alzheimer Europe highlighting opportunities to improve participation in dementia research and data sharing across Europe.

3.2. Industry perspective

Industry representatives outlined four domains essential for making a registry fit-for-use in regulatory contexts:

- Infrastructure: robust data capture system, adaptability, linkages;
- Process: transparent governance in place for data collection/use/sharing;
- Data: reliable and relevant captured core data elements that support fair and meaningful; comparisons
- Methods & deliverables: multi-disciplinary expertise represented in the registry team, including epidemiology, statistics, and data science experts.

They emphasized the importance for a patient registry to have well-defined objectives, internal validity, and recognized levels of risk in evidence needs.

Alignment between a registry-based study's objectives and a registry's purpose is critical for ensuring scientific validity. The need for a systematic feasibility assessment of the fitness-for-use in the context of a registry-based study was highlighted, as this is a critical step to ascertain whether a patient registry is suitable to answer the specific research questions. If the result of such

assessment reveals that a patient registry's quality and relevance do not support the study objectives, alternative data sources should be identified.

Finally, learnings from past post-authorisation safety studies in other therapeutic areas should be systematically leveraged to enhance the quality of Alzheimer's disease registries intended for future regulatory use.

The full presentation can be found in the *Industry perspective on real-world evidence generation: Shaping fit-for-purpose Alzheimer's Disease registries* [18].

3.3. Regulator perspective (EU and USA)

From the *EU regulatory perspective* [19], registry data can complement clinical trial data by providing information on the natural history of Alzheimer's disease, on the clinical context (incidence, prevalence, patient characteristics), and on the long-term effects of medicines to support the evaluation of their safety and effectiveness profiles. In this context, EMA has imposed conditions to the approval of the currently existing monoclonal antibodies targeting amyloid beta to conduct post-authorisation safety registry studies. Whilst acknowledging the potential limitations of data collected in the real-world clinical setting (e.g. regarding quality, or limited capture of outcome measures commonly used in trials), patient registries can provide valuable information on adverse events of special interest (e.g., ARIAs, intracerebral haemorrhage, etc.), clinical outcomes (such as disease staging) and risk factors (for example apolipoprotein E – APOE - status). The protocols for these studies must be reviewed and approved by the EMA's *Pharmacovigilance and Risk Assessment Committee* [20] before their launch, to ensure that the data source(s) (including patient registries), the design and methodological aspects of the studies are fit-for-purpose to answer the research questions.

In general, and as highlighted in the presentation by industry representatives, studies based on registry data should be pre-planned, in line with state-of-the-art methodologies (e.g., target trial emulation framework, *ICH M14 guideline* [21], and *other regulatory documents related to real-world evidence* [22]), and included in the *EMA/HMA catalogue of RWD studies* [23]. The protocol and design of such studies can be discussed a priori as part of *EMA's scientific advice procedures* [24] to ensure they generate data that can support regulatory decision-making. *EMA's Qualification procedure* [25] can also be used to assess the relevance of registry data in a specific context of use.

Similarly, the US-Food and Drug Administration (FDA) underlined in its *presentation* [26] that for sporadic Alzheimer's disease, randomised controlled trials remain essential to establish medicines' efficacy. Real world data may be used to inform the design of clinical trials or to characterize a product's effects in a broader population in the post marketing setting. The most informative data for regulatory use comes from sources with systematic and standardised data collection, based on a study protocol that specifies the assessments and outcomes of interest. Common problems with RWD include very limited access to patient-level data, data quality issues and the absence of pre-specified study protocol or analysis plan, which can lead to unreliable and biased analyses, or even uninterpretable results. In the United States of America, a registry-based study has also been requested for the approved amyloid-targeted monoclonal antibodies to assess safety outcomes. The FDA requirements are very specific about the outcomes that will be assessed and other important patient characteristics that must be collected.

3.4. HTA and payer perspectives

A representative from Health Technology Assessment (HTA) bodies highlighted several actions that would support decision-making by this stakeholder group (*HTA perspective on real-world evidence generation* [27]):

- Invest in patient registries, both for historical data and for treated patients;
- Ensure that patient registries can track all uses of the medicine, including off-label use;
- Clarify treatment initiation and discontinuation criteria, and ensure they are well understood and accepted;
- Assess whether costs in the care sector can be avoided – or merely postponed – through the use of new medicines;
- Engage in discussions about new payment models and risk sharing agreements;
- Address ethical issues and uphold the principle of solidarity, ensuring priority for those who are severely ill and have the greatest needs;
- Compare the effectiveness of therapies given as a fixed course duration versus continuous treatment;
- Maintain cooperation and information exchange between stakeholders.

A payers' representative noted that the new targeted medicines for Alzheimer's disease represent a paradigm shift as they offer for the first time the possibility of modifying the disease course rather than only treating symptoms (*Payer's perspective: Current evidence gaps and potential of real-world data* [28]). However, from the payer's perspective, this hope comes with major challenges in terms of affordability, infrastructure, uncertain long-term benefit, safety, and equity.

Patient registries provide two key-values for payers:

- Evidence on real-world effectiveness and safety: Given the modest clinical benefit demonstrated in clinical trials to date, the significant uncertainty about durability of effects and the safety concerns, payers will need to know whether real-world outcomes justify the investment;
- Enforcing eligibility criteria and reducing inappropriate use: For disease-modifying therapies, payers will almost certainly impose criteria such as Mild Cognitive Impairment (MCI) or mild Alzheimer's disease only, confirmed amyloid positivity, absence of severe comorbidities and Magnetic Resonance Imaging (MRI) clearance. A registry can help verify that patients' criteria, prevent off-label expansion, enforce step-therapy rules and ensure compliance with MRI safety protocols.

3.5. Healthcare professional perspective

A clinician representative highlighted that a structured follow-up of patients diagnosed with Alzheimer's disease is not always in place in routine clinical practice (*Healthcare professional perspective on real-world evidence generation* [29]). Significant data gaps remain, particularly regarding the diagnostic pathways of patients.

There is currently no pan-European registry, and only a few national patient registries exist, each with variable coverage, scope, and clinical settings (for example, some focus on dementia while others focus on mild cognitive impairment).

There is a need for patient registries to be "disease-focused" rather than treatment-focused, and with data collected on both patients receiving medicines and those who are not treated.

While capturing high quality data is crucial for healthcare professionals, it must also be feasible within the constraints of clinical practice. This requires:

- A minimum set of essential data points to ensure both feasibility and broad participation;
- Assessment tools that are quick and practical to use;
- Implementation of automation wherever possible, to reduce administrative burden and support consistent data collection.

3.6. Patient registries perspectives

Big Multiple Sclerosis Data Network: lessons learnt for Alzheimer's Disease registries [30]

The *Big Multiple Sclerosis Data Network MS network* [31] includes six national patient registries to allow joint analyses of very large merged or federated sets of structured clinical data. It covers 350,000 patients with multiple sclerosis (MS) to determine whether MS treatments provide long-term benefits.

Lessons learnt from the Big MS network which are relevant for patient registries in Alzheimer's disease include:

- Build a robust informatics structure from the start;
- Establish a common data model for use by each participating registry;
- Base the common data model on standards used on randomised controlled trials;
- Adapt to EMA's data quality expectations (*HMA/EMA Data Quality framework* [32]): Big Multiple Sclerosis Data Network has applied for EMA's qualification opinion to validate data quality, following the qualification advice and *letter of support [33]* received in 2022;
- Offer patient portals;
- Establish a consortium with industry partners for support and advice.

Swedish registry for cognitive and dementia disorders (SveDem) [34]

SveDem [3] is a 20-year-old registry that, as of December 2025, included 144,000 people with dementia (covering 40% of the dementia cases, but ca. 50% of expected cases are undetected) or mild cognitive impairment. The registry aims to improve health care based on national guidelines and collects data on dementia diagnoses, cognitive assessments, dementia investigations performed, treatments (with 80% of people being treated), and living and care conditions. It collects from the entire care chain: specialist care, primary care, nursing homes, and home care.

Characteristics of SveDem include:

- Cognitive ability assessment (Mini-Mental State Examination - MMSE, Montreal Cognitive Assessment - MOCA, Rowland Universal Dementia Assessment Scale - RUDAS);
- Annual follow-up of each person;
- Use of personal identification numbers for registration;
- Possibility to link with multiple national registers.

International Registry for Alzheimer's Disease and Other Dementias (InRAD): Driving international collaboration for real-world evidence in Alzheimer's disease [35]

InRAD [4] aims to establish an international clinical practice-based disease registry that includes both treated and untreated people with Alzheimer's disease, and to create a collaborative space for all stakeholders. Principles of InRAD include:

- A free-to-access cloud-based data platform and collaboration infrastructure;
- Data ownership retained by contributing clinical centres (Data Controllers), enabling collaboration within and beyond the network;
- InRAD acting as Data Processor, coordinating international research and studies;
- Participation in scientific agenda (e.g. own research; multicentre or national research studies);
- A Data Quality workstream to support regulatory use and HTA use cases;
- An open and transparent tripartite collaboration between regulators & payers / patient registries holders / marketing authorisation holders.

Following consultations with 72 international clinician and academics (50 based in Europe) and other stakeholders' representatives, InRAD defined a *Minimum Data Set (MDS) and an Extended Data Set (EDS)* [36]. The MDS includes diagnosis pathway information, clinical staging, patient demographics, MOCA/MMSE results, treatments and medical conditions/safety events. It is designed to be a realistic dataset that can be collected at the point of care.

The results of a survey performed amongst clinicians were presented. These showed willingness to systematically collect some of the InRAD MDS, in particular: demographics at baseline, diagnosis, MOCA/MMSE, disease stages and treatments and biomarker data. However, collecting a broader set of data, including medical events and treatments' safety-related information would require financial support. The survey also highlighted that 90% of the clinician responders consider that having multiple drug Phase 4 studies would have a negative impact on their time and resources, whilst 70% of them think that working with a single harmonised disease registry would help them save time and resources.

Deployment of the registry and data collection are planned to start in January 2026 with pilot centres across 21 countries (11 EU countries). The InRAD registry is open to all centres and will be used within the *EU innovative Health Initiative* [10] *ACCESS-AD* [9].

4. Outcomes of breakout sessions and plenary discussions

Three breakout sessions (BOS) ran in parallel and focused on the use of patient registries to address the following key topics:

- Safety and risk minimisation measures of medicines;
- Long-term treatment strategy (including criteria for discontinuation and re-treatment);
- Prevention of symptomatic Alzheimer's disease (including in patients with subtle detectable abnormalities but no functional impairment).

4.1. Safety and risk minimisation measures of medicines (BOS A)

This session focused on the safety of approved medicines, optimising monitoring, and understanding long-term consequences of Alzheimer's disease treatments' safety profiles:

- **Safety of approved products**
  - Specific risk minimisation measures including strict control access programs have been imposed for the recently approved monoclonal antibodies. It is desirable that patient registries collect the data necessary to fulfil these obligations, in particular to help differentiate the effect of these treatments from the standard of care.
  - Participants recognised that holders of patient registries may not always be familiar with regulatory requirements and processes, and that this may impact the fitness-for-use of the data in regulatory contexts. Engagement with holders of patient registries to raise awareness and facilitate understanding of the regulatory needs, standards and decision-making process may be useful, including on the use of relevant guidance e.g., *ICH M14* [21] on *general principles on plan, design and analysis of pharmacoepidemiological studies that utilize real-world data for safety assessment of medicines*, the *HMA/EMA Data Quality framework* [32], and the *EMA reflection paper on use of RWD in non-interventional studies to generate RWE* [37].
- **Optimising monitoring of Alzheimer's disease treatments**
  - Legal requirements in terms of data protection can limit how data can be linked or shared. There is a need to better understand how cross-country linkage can be achieved and what limitations apply. Developing patient portals can help clarify how data are used, strengthen engagement with patients, and encourage participation in studies.
- **Long-term consequences of safety profile**
  - The risk of losing follow-up of patients treated outside specialised centres was highlighted. This issue leads to limited understanding of potential long-term side effects, including after treatment discontinuation.
  - It was agreed that data linkage, portals for patient-reported outcomes and other digital tools could help reduce loss to follow-up and maintain long-term monitoring.

4.2. Long-term treatment strategy (including criteria for discontinuation and re-treatment) (BOS B)

The discussion centred around the capacity of patient registries to generate interpretable evidence to inform long-term treatment strategies:

- **Core dataset**: A core dataset should be collected across regions and treatment contexts, including treatment information (start/stop dates and reasons for discontinuation), patients' co-morbidities, co-medications and co-interventions, and measures of disease progression. The group agreed that agreement is needed on how to measure disease progression consistently and allow for comparison of treatments effect. Patient representatives also highlighted that caregiver burden should be treated as a primary outcome, not only as a cost variable.
- **Understanding long-term disease trajectories**: This includes biological markers (e.g., amyloid Positron Emission Tomography (PET) scan, cerebrospinal fluid (CSF), blood tests), cognitive decline, caregiver burden, quality of life, healthcare system costs, and time to nursing

home admission. Feasibility could be improved through innovative approaches such as Artificial Intelligence (AI)-based support tools. Patient-reported outcomes should also be collected.

- **Criteria for stopping anti-amyloid antibody treatment**: These criteria could include both amyloid measurements (PET, CSF, blood tests) and clinical indicators (CDR, MMSE/MOCA/ moderate dementia). Options such as fixed treatment durations (e.g., 12 or 18 months) followed by reassessment of amyloid clearance (or without reassessment if RWD studies show that this is not necessary anymore) were discussed.
- **Novel treatments as an opportunity to mature the healthcare system**: Lessons can be learnt from other disease areas such as multiple sclerosis where the first treatments helped introduce regular PET/MRI scans to monitor disease progression. Targeted anti-amyloid treatments should encourage more structured patients follow-up thanks to obligations imposed in relation to the use of these new medicines. The group agreed that experience gained from the accumulation of such data for a small patient population to start with, combined with progress in research, may provide evidence that scans could be replaced by other more scalable measurements like blood tests or fixed treatment period.

  Data linkage, such as what is already in place in Scandinavian countries as well as in the Netherlands, has the potential to reduce duplication of data collection. Hope was also raised thanks to the establishment of the European Health Data Space (EHDS), which should enable data sharing and interoperability in the long term.
- **Funding and sustainability**: Many funding sources exist, but they typically support projects with a specific end date. Patient registries should be part of the healthcare infrastructure rather than dependent on temporary funds. Public funding (e.g., government), but also private (e.g., pharmaceutical industry) will be needed to ensure sustainability of data collection for appropriate patients' follow-up and monitoring of all existing treatments whilst ensuring patients' ownership of their data.

4.3. Prevention of symptomatic Alzheimer's disease (including in patients with subtle detectable abnormalities but no functional impairment) (BOS C)

The third breakout explored the challenges and opportunities of using patient registries for the prevention of Alzheimer's disease.

The following challenges were identified:

- Individuals with the pathological hallmarks of Alzheimer's disease, but who have not (yet) developed symptoms, **do not have subjective complaints that may trigger a referral** to a healthcare professional. The modest positive predictive value of individual biomarkers makes the identification even more difficult at this time;
- **Need for very long-term follow-up** raises concerns about feasibility and missing data. Some of the patients identified as being at risk with the current technologies may not develop any symptoms for a very long time;
- There may be **negative psychological and practical consequences of communicating a risk** of developing symptomatic Alzheimer's disease. Careful considerations about ethical implications, consent and privacy are highly relevant, particularly in the context of genetic risk factors;

- **Sustainability of funding**, especially as the initiatives may be expected to be long, include extensive use of biomarkers, and may not be linked to approved medicinal products, funding models have to be discussed. Industry funding, complemented with public funding may be considered. The specific infrastructural need also raises some concern about ensuring adequate **geographical representation** to any future initiative.

Some opportunities have been identified:

- **Individuals often do want to take part in research**, for the benefit of the collective and of the future generations in their families. It is important to clearly communicate the value of taking part, and to provide feedback on how their data have contributed to research outcomes;
- (Older) adults participating **in patient registries for other initiatives** may be identified – for example based on blood biomarkers on materials stored in biobanks – as candidates for patient registries on the pre-symptomatic phases of Alzheimer's disease. This exercise would however require a clear research plan and consent to ensure protection of data privacy and confidentiality;
- **Digital and AI-based technologies** offer new opportunities for both the identification and the longitudinal follow up of participants. **Passive monitoring** tools could both be key for the feasibility of registries and be validated by their use in patient registries;
- Registry data should be integrated to and **linked with other healthcare records** for a more complete view of the patient journey.

4.4. Plenary discussions

Patient and caregiver representatives emphasised that, as new treatments are authorised and reach the market, a streamlined approach is required to avoid the situation where EMA requirements lead marketing authorisation holders to set up their own phase 4 trials that only follow-up the patients treated with their medicines. This model will inherently limit the "real-world nature" of the data collected, as participation would only target a small group of early-stage patients in specialist settings, and would depend on the access to specialised centres where the authorised disease-modifying treatments are administered (which is uneven across Europe and within countries). Such a scenario would lead to under-representation of patients who are not eligible for these new therapies, or who do not have easy access to specialised centres and the treatments being offered, e.g., people in remote/rural areas, from ethnic minorities or socioeconomically disadvantaged groups. It would therefore be more appropriate to establish at national or European level disease-based patient registries (as opposed to regulatory obligations or access conditions-led registries) designed for longitudinal data collection, capable of following all patients across the full trajectory of dementia, regardless of the treatments they receive or the setting in which they are treated.

A strong collaboration with clearly defined responsibilities between relevant regulatory bodies at national and European levels, data holders and pharmaceutical industry is necessary to properly shape the design of sustainable patient registries and to ensure their integration into national healthcare systems. This would allow the collection of data based on new treatment strategies and would support their evaluation over time. The need for independent, publicly guided governance was highlighted to safeguard transparency and patient trust.

5. Post-workshop considerations

This section highlights actions which arose from post-workshop discussions on concrete ways to follow-up on the key principles highlighted in the Executive Summary.

- **Core dataset harmonised across patient registries**

Preparatory step: Experts from the European Medicines Regulatory Network (EMRN) will prepare a draft core dataset considered critical to answer regulatory questions based on the workshop discussions and existing initiatives. The list will then be published on the EMA website to give all stakeholders the opportunity to highlight any missing data variables that matter to them, and which they consider feasible to be collected by patients/ caregivers/ clinicians in routine clinical practice. A mechanism for periodic review and update should be established to adapt the core dataset in line with evolving treatment landscape, biomarker science, and clinical outcome assessments.

Consultation step: The EMA will actively engage with its stakeholder groups via the dedicated associations/organisations to raise awareness and maximise their contribution in the most appropriate way. Input from patients and caregivers will particularly be important to ensure meaningful outcomes (e.g. PROMs and PREMs) are explicitly included in the core dataset. Outcomes should reflect not only clinical parameters, but also lived experience, treatment burden, impact on daily life, and the quality of interaction with the healthcare system. These aspects are critical to capture if patient registries want to increase their ability to inform decisions that truly reflect what matters to patients and families. A core dataset harmonised across patient registries which is considered acceptable by all key players will stimulate data collection, build trust in the processing and use of patients' data, and strengthen data quality in a sustainable way.

- **Use of "disease-based" patient registries for decision-making on medicines**

Advantages/opportunities: It was emphasised during the workshop that the use of disease-based patient registries collecting data on patients regardless of their treatments' assignement is favoured and should be strongly encouraged instead of "single product" data collection systems. The latter are unlikely to address a variety of scientific questions due to their limited representativeness in the absence of reference groups and information on natural disease progression. On contrary, patient registries (which encompass disease registries as defined in the *EMA Guideline on registry-based studies* [5]) provide a more holistic approach by collecting long-term data on treated and untreated patients in the real-life clinical settings, embracing different patients' characteristics with a larger sample size to support the generation of meaningful evidence on medicines.

Need for clear regulatory/HTA/payers' requirements to leverage "disease-based" patient registries: If disease-modifying therapies that have been authorised through EMA's centralised procedure based on their positive benefit-risk profile are not subsequently reimbursed at national level, the ability to collect meaningful RWD through patient registries will be significantly constrained. Therefore, requirements from regulators, HTA bodies and payers in terms of "disease-based" patient registry data to support their decisions need to be clearly communicated to encourage collaborative efforts aimed to leverage these data, minimise duplication and improve data quality in the field of Alzheimer's disease and beyond. The EMA will take the opportunity of the planned revision of the *EMA Guideline on registry-based studies* [5] to strengthen the EMRN position on the use of patient registries in regulatory contexts.

Generation of evidence for regulatory decisions: from disease-based patient registry to registry-based study: The distinction between a "disease-based" patient registry and registry-based study is important to highlight in this report. Whilst such patient registry may provide a valuable infrastructure for the collection of data to address regulatory questions, a post-authorisation safety

or efficacy study conducted within a patient registry still requires a fit-for-purpose protocol, pre-specified analysis plan, and appropriate methodological design. The accountability for the quality and interpretability of the study results remains with the study sponsor, e.g., marketing-authorisation holders (MAHs). It may happen that existing disease-based patient registries may not (yet) be able to support the specific data quality standards, outcomes, or patient characteristics needed for a given regulatory commitment. In these situations, the use of complementary or alternative data sources to meet regulatory obligations within agreed timelines may need to be considered. Early dialogue between MAHs and regulators, and possibly with patient registry holders, should help navigate through these scenarios to find solutions acceptable by all parties involved.

- **Financial support**

Patient registries require sustained financial support to help them adapt to evolving data needs linked to novel treatments, and to continuously optimise their processes, including quality management, training of staff members and engagement with individuals inputting data (e.g., patients/healthcare professionals). Moving forward, the establishment of a permanent dual source of funding coming from public institutions (such as government bodies) and private organisations (e.g., pharmaceutical industry) would enable high quality maintenance and integration of patient registries into national healthcare systems to improve patient care. Financial models should be transparently declared and clearly translated within patient registries' governance to ensure patients' ownership of the data, trust and impartiality whilst maintaining the capacity to provide relevant data to monitor medicines. These important aspects will be highlighted in registry-related EMA guidance, starting with the revision of the *EMA Guideline on registry-based studies* [5], and other engagement activities with stakeholders.

- **Data linkage, use of digital tools and AI**

The potential of digital tools and AI in reducing duplication, facilitating data entry and linkage (e.g., to electronic health records) was clearly labelled as enablers for better patients' follow-up. Financial support as mentioned above are needed in this area to help patient registries optimise their infrastructure in view to reduce burden on clinicians and patients/ caregivers, but also to strengthen their engagement in contributing to research and ultimately to the development of better treatments.

6. Abbreviations

ACCESS AD: Advancing Alzheimer's Disease care

AI: Artificial Intelligence

APO-E: Apolipoprotein E

ARIA: Amyloid-Related Imaging Abnormalities

Big MS: Big Multiple Sclerosis Data Network MS network

BOS: Break Out Sessions

CDR-SB: Clinical Dementia Rating Sum of Boxes

CHIEF: Collaborative Health Information European Framework

CSF: Cerebrospinal Fluid

EHDS: European Health Data Space

EMA: European Medicines Agency

EMRN: European Medicines Regulatory Network

EU: European Union

HMA: Heads of Medicines Agency

HTA: Health Technology Assessment

ICH: International Council for Harmonisation

InRAD: International Registry for Alzheimer's disease and other Dementias

MAH: Marketing Authorisation Holders

MCI: Mild Cognitive Impairment

MDS: Minimum Data Set

MMSE: Mini-Mental State Examination

MOCA: Montreal Cognitive Assessment

MRI: Magnetic Resonance Imaging

NMDA receptor agonists: N-methyl-D-aspartate receptor agonists

PET: Positron Emission Tomography

PREM(s): Patient Reported Experience Measures

PROM(s): Patient Reported Outcome Measures

RUDAS: Rowland Universal Dementia Assessment Scale

RWD: Real-Word Data

SveDem: Swedish registry for cognitive and dementia disorders

US-FDA: US-Food and Drug Administration

7. References

[1] Prevalence of dementia in Europe, Alzheimer Europe - https://www.alzheimer-europe.org/dementia/prevalence-dementia-europe?language_content_entity=en – Source accessed in April 2026

[2]: Joint Heads of Medicines Agency and European Medicines Agency EU-IN Horizon Scanning Report on Alzheimer's disease - https://www.ema.europa.eu/en/documents/report/alzheimers-disease-eu-horizon-scanning-report_en.pdf - Source accessed in April 2026

[3] Swedish registry for cognitive/dementia disorders, SveDem - https://www.ucr.uu.se/svedem/in-english - Source accessed in February 2026

[4] International Registry for Alzheimer's disease and other Dementias, InRAD - https://www.inradnetwork.org/ - Source accessed in February 2026

[5] EMA Guideline on registry-based studies - https://www.ema.europa.eu/en/guideline-registry-based-studies-scientific-guideline - Source accessed in February 2026

[6] Joint Heads of Medicines Agencies (HMA)/European Medicines Agency (EMA) multistakeholder workshop on Patient Registries for Alzheimer's disease - https://www.ema.europa.eu/en/events/joint-heads-medicines-agencies-hma-european-medicines-agency-ema-multistakeholder-workshop-patient-registries-alzheimers-disease - Source accessed in January 2026

[7] Introduction on Alzheimer's Disease and current therapies - https://www.ema.europa.eu/en/documents/presentation/presentation-introduction-alzheimers-disease-current-therapies-m-haberkamp_en.pdf - Source accessed in December 2025

[8] JADE Health - https://jademente.eu/ - Source accessed in February 2026

[9] ACCESS AD - https://www.access-ad.org/ - Source accessed in February 2026

[10] EU innovative Health Initiative - https://www.ihi.europa.eu/ - Source accessed in February 2026

[11] European partnership for Brain Health - https://www.brainhealth-partnership.eu/ - Source accessed in February 2026

[12] Collaborative Health Information European Framework (CHIEF) - https://joint-research-centre.ec.europa.eu/projects-and-activities/public-health_en#eu-platform-for-rare-diseases-registration - Source accessed in February 2026

[13] Opening remarks from the European Commission - https://www.ema.europa.eu/en/documents/presentation/presentation-opening-remarks-european-commission-hma-ema-multi-stakeholder-workshop-patient-registries-alzheimers-disease-t-raemaekers_en.pdf - Source accessed in December 2025

[14] Network Data Steering Group workplan 2025-2028 - https://www.ema.europa.eu/en/documents/other/network-data-steering-group-workplan-2025-2028_en.pdf - Source accessed in December 2025

[15] Lived experience of Alzheimer's Disease (European Working Group of People with Dementia) - https://www.ema.europa.eu/en/documents/presentation/presentation-lived-experience-alzheimers-disease-european-working-group-people-dementia-s-dougall_en.pdf - Source accessed in December 2025

[16] Patient representative perspective: Understanding European perspectives on data sharing - https://www.ema.europa.eu/en/documents/presentation/presentation-patient-representative-perspective-understanding-european-perspectives-data-sharing-l-duffner_en.pdf - Source accessed in December 2025

[17] Dementia Research Participation and Data Sharing in Europe Report - https://www.alzheimer-europe.org/news/new-report-alzheimer-europe-highlights-opportunities-improve-participation-dementia-research?language_content_entity=en&language=uk - Source accessed in May 2026

[18] Industry perspective on real-world evidence generation: Shaping fit-for-purpose Alzheimer's Disease registries - https://www.ema.europa.eu/en/documents/presentation/presentation-industry-perspective-real-world-evidence-generation-shaping-fit-purpose-alzheimers-disease-registries-e-grace-p-dobay_en.pdf - Source accessed in December 2025

[19] Regulator's view on RWD in Alzheimer's disease - https://www.ema.europa.eu/en/documents/presentation/presentation-eu-regulators-view-rwd-alzheimers-disease-p-mol_en.pdf - Source accessed in December 2025

[20] Pharmacovigilance and Risk Assessment Committee - https://www.ema.europa.eu/en/committees/pharmacovigilance-risk-assessment-committee-prac - Source accessed in December 2025

[21] ICH M14 guideline - https://www.ema.europa.eu/en/ich-m14-guideline-general-principles-plan-design-analysis-pharmacoepidemiological-studies-utilize-real-world-data-safety-assessment-medicines-scientific-guideline - Source accessed in February 2026

[22] EMA webpage on Real-world evidence - https://www.ema.europa.eu/en/about-us/how-we-work/data-regulation-big-data-other-sources/real-world-evidence - Source accessed in February 2026

[23] HMA-EMA Catalogues of real-world data sources and studies - https://catalogues.ema.europa.eu/ - Source accessed in February 2026

[24] EMA Scientific advice and protocol assistance - https://www.ema.europa.eu/en/human-regulatory-overview/research-development/scientific-advice-protocol-assistance - Source accessed in February 2026

[25] Qualification of novel methodologies for medicine development - https://www.ema.europa.eu/en/human-regulatory-overview/research-development/scientific-advice-protocol-assistance/qualification-novel-methodologies-medicine-development - Source accessed in February 2026

[26] US-FDA Real-world data in Alzheimer's disease - https://www.ema.europa.eu/en/documents/presentation/presentation-fda-real-world-data-alzheimers-disease-t-buracchio_en.pdf - Source accessed in December 2025

[27] HTA perspective on real-world evidence generation - https://www.ema.europa.eu/en/documents/presentation/presentation-hta-perspective-real-world-evidence-generation-n-hedberg_en.pdf - Source accessed in December 2025

[28] Payer's perspective: Current evidence gaps and potential of real-world data - https://www.ema.europa.eu/en/documents/presentation/presentation-payers-perspective-current-evidence-gaps-potential-real-world-data-c-hernandez_en.pdf - Source accessed in December 2025

[29] Healthcare professional perspective on real-world evidence generation - https://www.ema.europa.eu/en/documents/presentation/presentation-healthcare-professional-

perspective-real-world-evidence-generation-k-frederiksen_en.pdf - Source accessed in December 2025

[30] Big Multiple Sclerosis Data Network: lessons learnt for Alzheimer's Disease registries - https://www.ema.europa.eu/en/documents/presentation/presentation-big-ms-network-multiple-sclerosis-lessons-alzheimers-disease-registries-j-hillert_en.pdf - Source accessed in December 2025

[31] Big Multiple Sclerosis Data Network MS network - https://bigmsdata.org/ - Source accessed in December 2025

[32] HMA/EMA Data quality framework for medicines regulation - https://www.ema.europa.eu/en/about-us/how-we-work/data-regulation-big-data-other-sources/data-quality-framework-medicines-regulation - Source accessed in February 2026

[33] Big Multiple Sclerosis Data Network MS network letter of support - https://www.ema.europa.eu/en/documents/other/letter-support-performing-registry-based-post-authorisation-safety-studies-pass-multiple-sclerosis-ms-using-data-big-ms-data-network-bmsd_en.pdf - Source accessed in February 2026

[34] Swedish registry for cognitive and dementia disorders (SveDem) - https://www.ema.europa.eu/en/documents/presentation/presentation-swedish-registry-cognitive-dementia-disorders-svedem-registry-holders-perspectives-real-world-evidence-generation-d-religa_en.pdf - Source accessed in February 2026

[35] International Registry for Alzheimer's Disease and Other Dementias (InRAD): Driving international collaboration for real-world evidence in Alzheimer's disease - https://www.ema.europa.eu/en/documents/presentation/presentation-international-registry-alzheimers-disease-other-dementias-inrad-driving-international-collaboration-real-world-evidence-alzheimers-disease-r-perneczky-r-hyde_en.pdf - Source accessed in February 2026

[36] Real-world datasets for the International Registry for Alzheimer's Disease and Other Dementias (InRAD) and other registries: An international consensus - https://www.sciencedirect.com/science/article/pii/S2274580725000408?via%3Dihub - Source accessed in April 2026

[37] EMA reflection paper on use of RWD in non-interventional studies to generate RWE - https://www.ema.europa.eu/en/reflection-paper-use-real-world-data-non-interventional-studies-generate-real-world-evidence-scientific-guideline - Source accessed in February 2026.

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