Patient Registry workshop on Alzheimer's disease

Current evidence gaps and potential of real-world data

Payer's perspective

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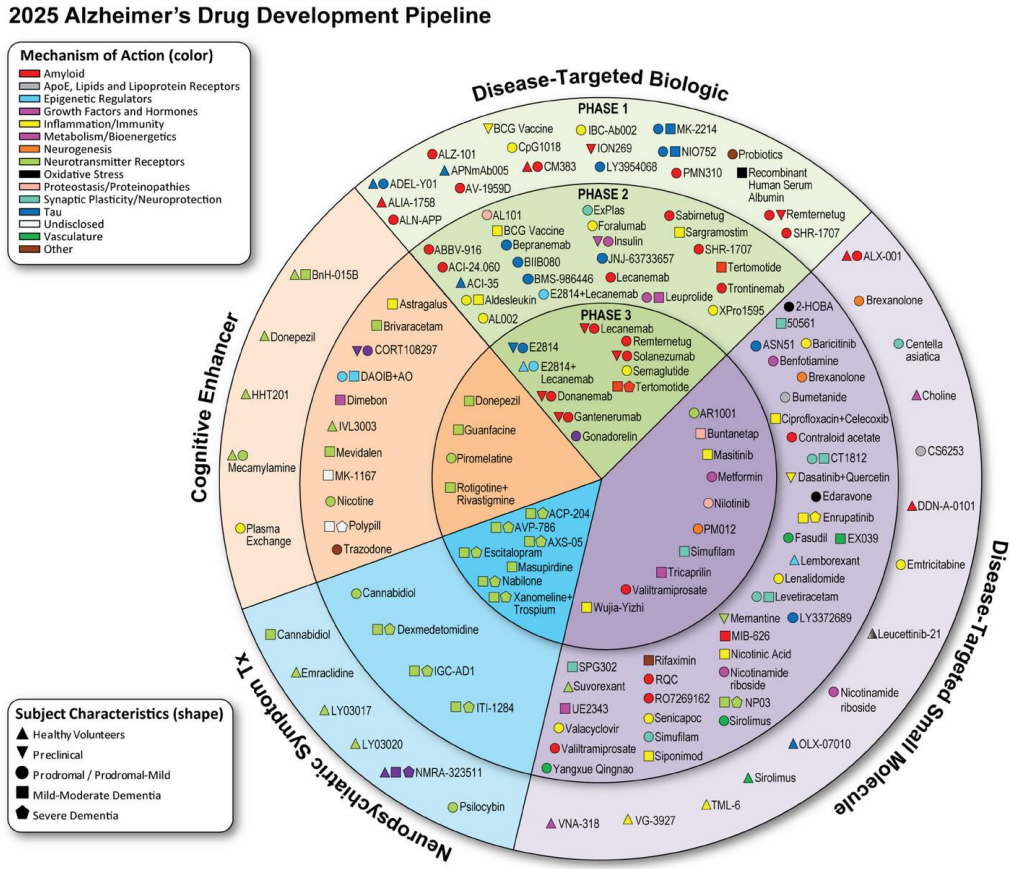
Alzheimer's disease and other dementias normally identified as high-priority health needs for all countries



Sources: Cummings JL, Goldman DP, Simmons-Stern NR, Ponton E. The costs of developing treatments for Alzheimer's disease: A retrospective exploration. Alzheimer's Dement. 2022; 18: 469–477. https://doi.org/10.1002/alz.12450 Cummings JL, Zhou Y, Lee G, et al. Alzheimer's disease drug development pipeline: 2025. *Alzheimer's Dement*. 2025; 11:e70098. **https://doi.org/10.1002/trc2.70098**

Is pharmaceutical innovation amenable to Alzheimer's disease?

1. Leading experts agreed that pharma innovation in AD is plausible, though significant gaps remain to fully realize its potential

2. Recent amyloid-targeting therapies show a modest benefit slowing in early patients. However, a strong genetic and biological rationale may point to a certain feasibility

3. Sustained public investment is needed in shared trial infrastructure, biomarker ecosystem, and prevention frameworks–areas

In summary, cautious optimisms provided we manage to align research, production, access, heath care provision, and sustainability (or even affordability)

To pay or not to pay

that is the question

- If priced as announced, would require a very large share of pharma or HC budgets
- Beyond the medicine, administering these therapies involves additional costs: imaging, genetic or biomarker testing, etc...
- The clinical benefit of these medicines is modest, only a small slowing of cognitive decline (on the order of months).
- The treatments carry non-trivial safety risks that require monitoring
- From a payer's perspective, investing heavily in a therapy with modest benefit and uncertain long-term value — plus safety risks — is a difficult trade-off…

- … however, we should not forget that we are speaking about the disease with the highest burden over society
- … that we are already paying hundreds of millions per year for medicines with even a more modest effect
- … that the objective is to offer solutions at an affordable price for a wide range of population avoiding concentration of the budget in restricted populations
- … no climb begins with anything other than the first step at the bottom of the slope
- … perhaps the question is how much money we want (and can) invest in making possible access to the molecules from my first slide

Why Registries Are Valuable for Payers

looking for the philosopher's stone

- **Evidence for Real-World Effectiveness and Safety**
  - High-cost Alzheimer treatments come to market with a modest clinical benefit, significant uncertainty about durability, meaningful safety concerns (e.g., ARIA).
  - Even with an open mind, payers will need to know whether real-world outcomes justify the investment
    - (a) treatment persistence rates, (b) real-world decline vs. clinical trial benchmarks, (c) incidence of ARIA and cost of managing it, (d) subgroups who benefit most (or no benefit); (e) mortality, hospitalization, and institutionalization rates…
- **Enforcing Eligibility Criteria & Reducing Inappropriate Use**
  - For disease-modifying therapies, payers will almost certainly impose criteria such as mild cognitive impairment (MCI) or mild AD only, amyloid positivity, no severe comorbidities, MRI clearance
  - A registry can validate that patients meet eligibility, prevent off-label expansion, enforce step-therapy rules, ensure MRI safety protocol compliance

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Key messages

Thanks for your attention

- The new Alzheimer's medicines represent a **paradigm shift** — for the first time, potentially modifying the disease course rather than only symptomatic treatment. However, from the payer's perspective, this hope comes with major challenges: **affordability**, **infrastructure**, **uncertain long-term benefit**, **safety**, and **equity**.
- Successfully integrating these therapies into real-world health systems will likely require **new approaches, payment models**, **care-pathway redesign**, **investment in diagnostic and infusion infrastructure**, and **long-term evidence generation** — as well as difficult cost-benefit and ethical decisions about who gets treated and when.
- For registries, please, **consider also the needs for payers** so **we can align research, production, access, heath care provision, and sustainability (or even affordability)**

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