



AD PACE

Alzheimer's Disease Patient and Caregiver Engagement

Understanding What Matters Most to Inform Care and Services Research

Research Summit on Dementia Care

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National Institutes of Health

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AD PACE is

- a ground-breaking patient/caregiver-led collaboration of industry, government agencies, and patient/caregiver advocates
- that will build a standing and persistent platform
- to deliver - to research, regulatory, and payer authorities - new insights into preferred treatment and health outcomes sought by those living with Alzheimer's and their caregivers.

AD PACE will

- deliver a wide-ranging, scientifically rigorous understanding of *what matters most* to individuals across the breadth of the Alzheimer's lived-experience
- match the scope and pace of the patient engagement guidances and policies to be developed by the FDA pursuant to the 21st Century Cures Act and PDUFA VI
- become the authoritative 'patient voice' to inform and improve product and clinical trial design, regulatory submissions, payer value models, coverage and payment determinations, and research on care and services.

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AD PACE Value Proposition



Delivering the voice of the AD patient and caregiver across the lived experience

Being sure that therapies in the pipeline make a difference in 'what matters most' to patients and caregivers



Aligning patient preferences with therapeutic development

- Clinical Trial Design
- Product Design
- Clinically Meaningful Outcomes
- Regulatory Endpoints
- Regulatory Reviews



Enhancing speed and quality of coverage and payment determinations

- Value Models
- Benefit Design
- Drug Price Determination
- RWE Development

Informing care and services design and outcomes.

Precompetitive. Leveraged across sponsors. Needed in AD.

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The AD PACE Assets

1. **New Knowledge on patient and caregiver insights and preferences** – generated from AD PACE What Matters Most Study, Landscape and Technical Assessment, and Gap Analysis and in time to deliver insights specific to Alzheimer's into the guidance development and processes called for under PDUFA VI & 21st Cent. Cures.
2. **The A LIST** – a standing cohort of individuals ready and willing to participate in preference studies. With a very early 'test bed-cohort' of 1300 members, from worried-well to caregivers (70%), the A LIST is ready to become the online community for hundreds of thousands of individual who wish to have their voice heard as they contribute to 'smarter science.'
3. **The AD PACE Data Commons** – a repository of datasets on patient and caregiver insights and preferences, acquired through AD PACE and through partnering relationships, to speed learnings and avoid redundancy.
4. **AD PACE Tool Box**– Repurposed or new tools that measure what is meaningful to patients, developed and adopted for use in a single trial to gain early proof of concept, validated for broad use in the field.

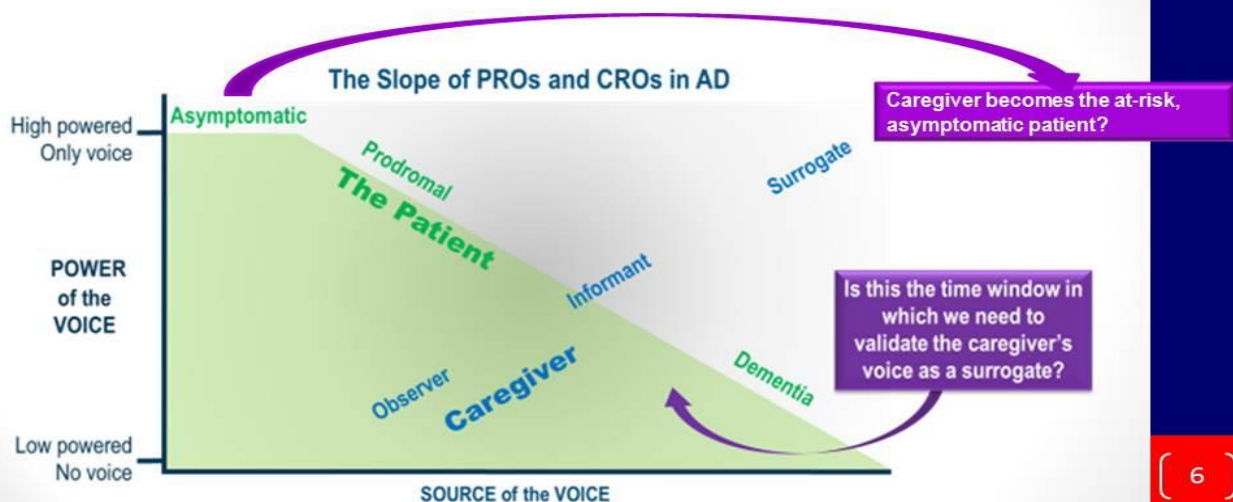


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AD PACE will
bring qualitative and quantitative research to understand the Alzheimer's lived experience

1. What Matters to YOU?
2. What Matters Most to ALL who are in your position in the Alzheimer's lived-experience – Disease stage? Similar in terms of caregiving dynamics, social economic status, racial, ethnic and other demographics that bear directly on the disease experience?
3. Can we measure this to confirm a level of certainty that creates a new evidence base for
 - ✓ **Regulatory Submissions**
 - ✓ Clinically meaningful outcomes
 - ✓ Benefit Risk calculus
 - ✓ **Value Models**
 - ✓ Benefit Design – Coverage
 - ✓ Payment Determinations, including Formulary Determinations
 - ✓ **Care and Services Research**

For all areas (Regulatory, Payment, Care and Services)
 AD PACE will work to understand this dynamic:



Where are we in this work?

Early A LIST qualitative work: What Matters to YOU? **2460 responses.**

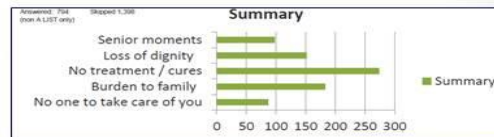
- Q: (AD / MCI Cohort):** If you could take a medication that would improve your memory and help preserve your independence, which **ONE** of the following would be **MOST IMPORTANT** to you

 - Complex tasks?
 - Right words/recognizing faces?
 - Following conversation;?
 - Finding your way?
 - Holding onto information?
 - other?

➤ **ANS:** *Performing complex tasks; holding onto information*
- Q: (Caregiver Cohort):** If the person you are caring for could take an Alzheimer's medicine that would improve his or her memory and help preserve independence, which **ONE** of the following would be **MOST IMPORTANT** to him or her from YOUR perspective?

➤ **ANS:** *Finding the right words; following conversations; holding onto information*
- Q: (Caregiver Cohort):** If the person you are caring for could take an Alzheimer's medicine that would improve his or her memory and help preserve independence, which **ONE** of the following would be **MOST IMPORTANT** to him or her from HIS OR HER perspective?

➤ **ANS:** *Following conversations; holding onto information*
- Q: (not Diagnosed / non-caregiver):**
Having learned about or seen the impact of Alzheimer's on family and friends, what one thing concerns you most about getting the disease?



As expected, lack of treatment / cures is concerning to onlookers; also substantially concerned about being a burden and loss of dignity

Consider a simple next step with the AD PACE A LIST cohort to inform care and services:

- Q: (AD / MCI Cohort):** If you could have a single **support system** in place to improve YOUR memory, function and independence, which **ONE** of the following would be **MOST IMPORTANT** to you?

 - Being kept safe?
 - Maintaining relationships with / companionships with loved ones?
 - Engaging in activities?
 - Assistance with household tasks (i.e. laundry, dishes, maintaining cleanliness)?
 - Assistance with personal care (i.e. bathing, grooming, restroom assistance, etc.)?
 - Other (fill in the blank) _____?
- Q: (Caregiver Cohort):** If the person you are caring for could have a single support system in place to improve his/her memory, function and independence, which **one** of the following would be most important to him or her from YOUR perspective?
- Q: (Caregiver Cohort):** If the person you are caring for could have a single support system in place that would improve his or her memory, and independence, which **one** of the following would be most important to him or her from HIS or HER perspective?
- Q: (not Diagnosed / non-caregiver):** Having learned about or seen the impact of Alzheimer's on family and friends, what one thing concerns you most about getting the disease in terms of YOUR ACCESS TO THE CARE AND SERVICES YOU WILL NEED?

Mayo Clinic – A LIST Partnership: What Matters Most to Caregivers?

IRB-approved research study, "Identification of Unmet Needs, Priorities, and Expectations of Family Caregivers and Providers of Persons with Alzheimer's Disease and Related Dementias: A Qualitative Study."

- ✓ **Principal Investigator**, Joan Griffin, Ph.D., Associate Professor of Health Services Research, Mayo Clinic College of Medicine, Division of Health Care Policy and Research, Department of Health Sciences Research, Robert D. and Patricia E. Kern Scientific Director for Care Experiences Program, Kern Center for the Science of Health Care Delivery.
- ✓ **Study aim** is to learn what matters most to dementia caregivers when participating in clinical visits with their loved ones and as part of clinical treatment teams.
- ✓ **Data to be used** to help train healthcare providers on better ways to communicate and support caregivers and their care recipients through their Alzheimer's journeys.
- ✓ **Two part study**: 1) online focus group with participation once a day for one week; 2) mail-survey.
- ✓ **A LIST successfully recruited** study participants who are struggling with memory impairment and their caregivers through emails to A LIST members from Meryl Comer (current caregiver) and Greg O'Brien (living with Alzheimer's).

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Consider the EU five country survey of Carers – and societal/cultural/practice differences **1,400 carers**

(Berlin, 3 October 2017; Woods, Bob, Bangor University, Wales)

- **As a Carer, are you male/female?**
 - *Women: 75.8% Netherlands; 88.4% Scotland.*
- **Would there have been an advantage to you had the person for whom you care been diagnosed earlier?**
 - *Yes: 36.5% Netherlands; 52.1% Italy.*
- **What was the most significant barrier to an earlier diagnosis?**
 - *Varying:*
 - *Nothing wrong: 33%;*
 - *Not worth pursuing a diagnosis: 6.6%;*
 - *Refusal of patient to engage in treatment: 38%*
- **What assessments were carried out as part of the process of diagnosis?**
 - *Range from*
 - *memory tests: 79% Czech Rep; 93% Finland*
 - *interviews: 62% Czech; 87% Italy*
 - *blood tests: 32% - Italy; 64% Finland*
 - *brain scans: 56% Czech; 84% Finland*
 - *lumbar puncture: 4.4% Scotland; 1.1% Finland*
- **Was the person with dementia informed of the diagnosis?.**
 - *Italy: 59.3 % were not informed; Finland: 1.1% were not informed*

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Recommendations

- As we, as a society, increasingly rely on caregiving to be provided as long as possible in a home setting, we need to develop a scientifically sound means of understanding *what matters most* to people with dementia and the individuals who care for them.
- Patient and Caregiver Preference Research can provide insights that will enable providers to conform and adjust programmatic needs and deliver to respond to different **demographic, cultural and socioeconomic backgrounds** and to improve quality of care and health outcomes across varying care settings
- Investments need to be made to develop platforms, such as AD PACE, for this work that span regulatory, value models for coverage and payment, AND systems for care and support. The patient and caregiver journey is part of an ecosystem where what matters most to them informs these multiple dimensions.

*Patient Preference Research should be a **foundational, required piece** when developing or evaluating new or established evidence-based programs and services*

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Thank you

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