"State of the Science for Pragmatic Trials of Non-Pharmacological Interventions to Improve Outcomes among Persons with Dementia & their Caregivers"

National Institute on Aging
7201 Wisconsin Avenue, Bethesda, MD 20814

Friday, December 1, 2017

MEETING RECOMMENDATIONS

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Meeting Information

- Organized by the Brown University School of Public Health and Hebrew SeniorLife
- Supported by an administrative supplement to Brown's NIA grant # P01AG027296-09S1
- A Post-summit Activity of the 2017 "Research Summit on Care and Services for Persons Living with Dementia and Caregivers," which was sponsored by the Advisory Council on Alzheimer's Research, Care, and Services of DHHS

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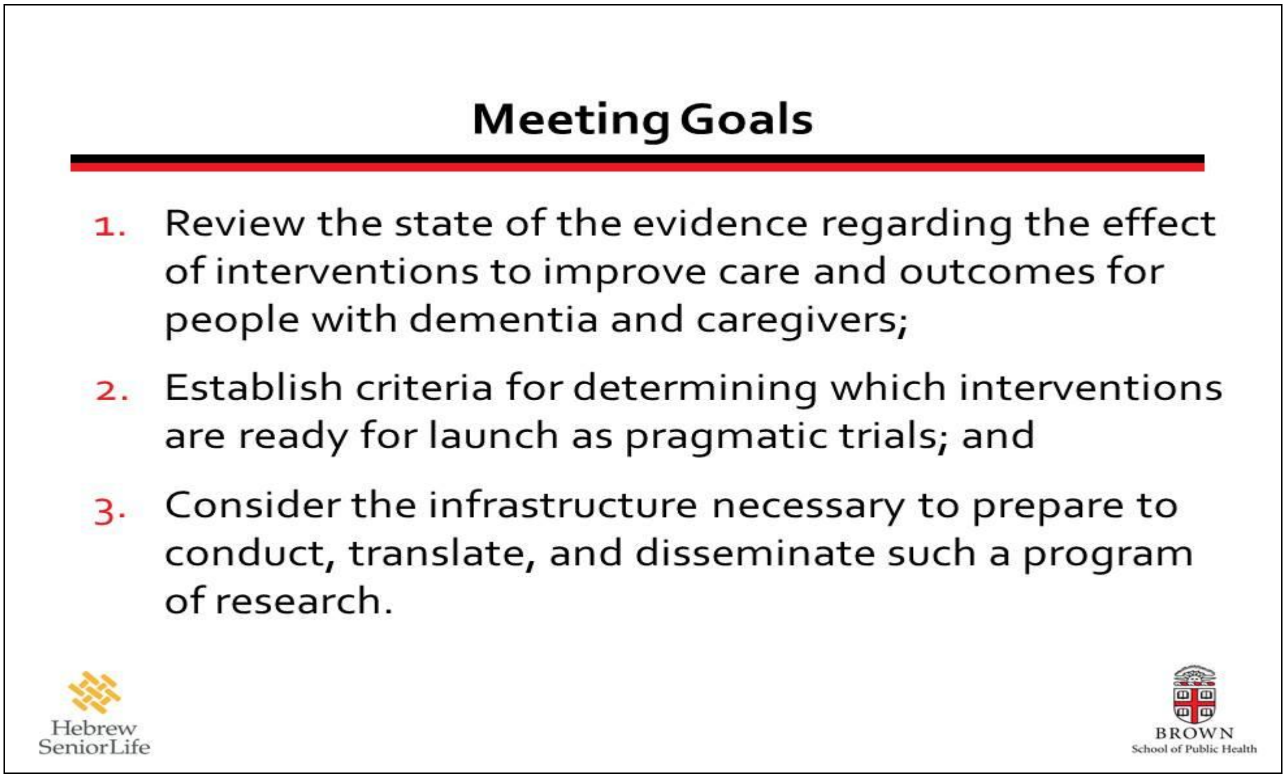
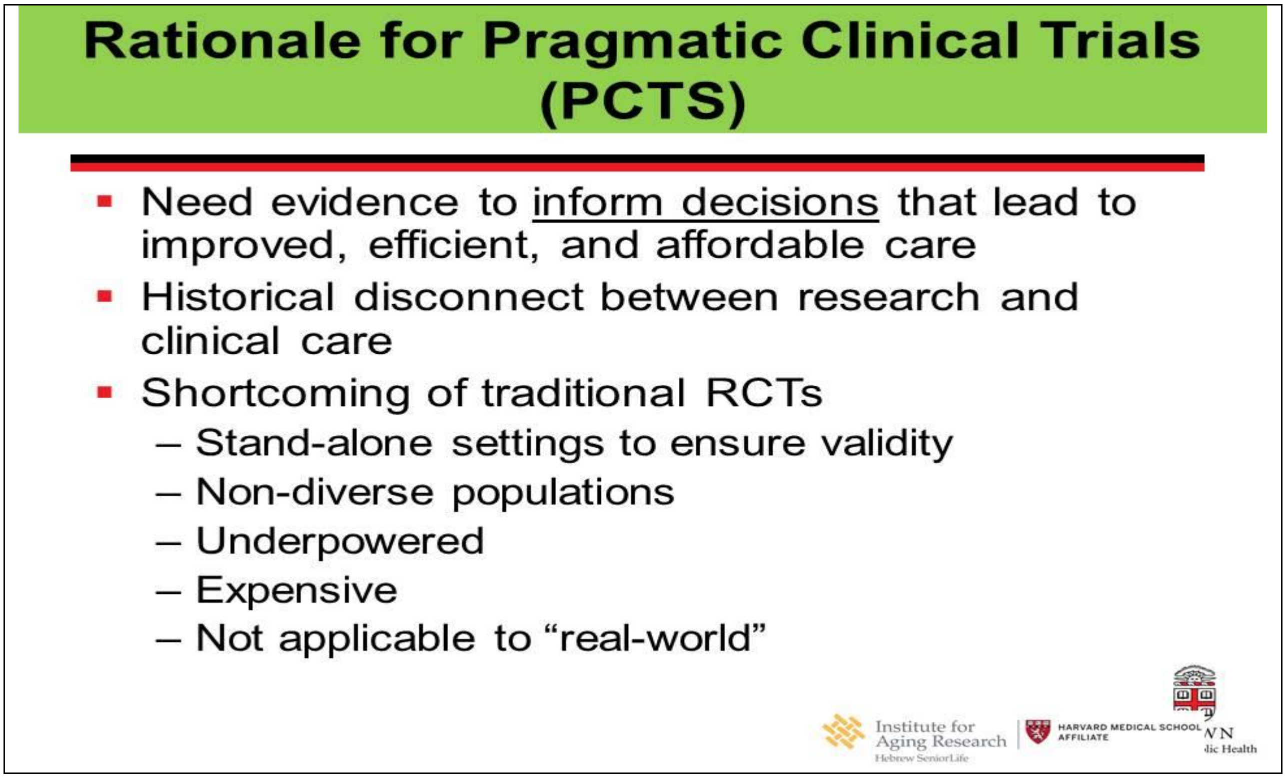


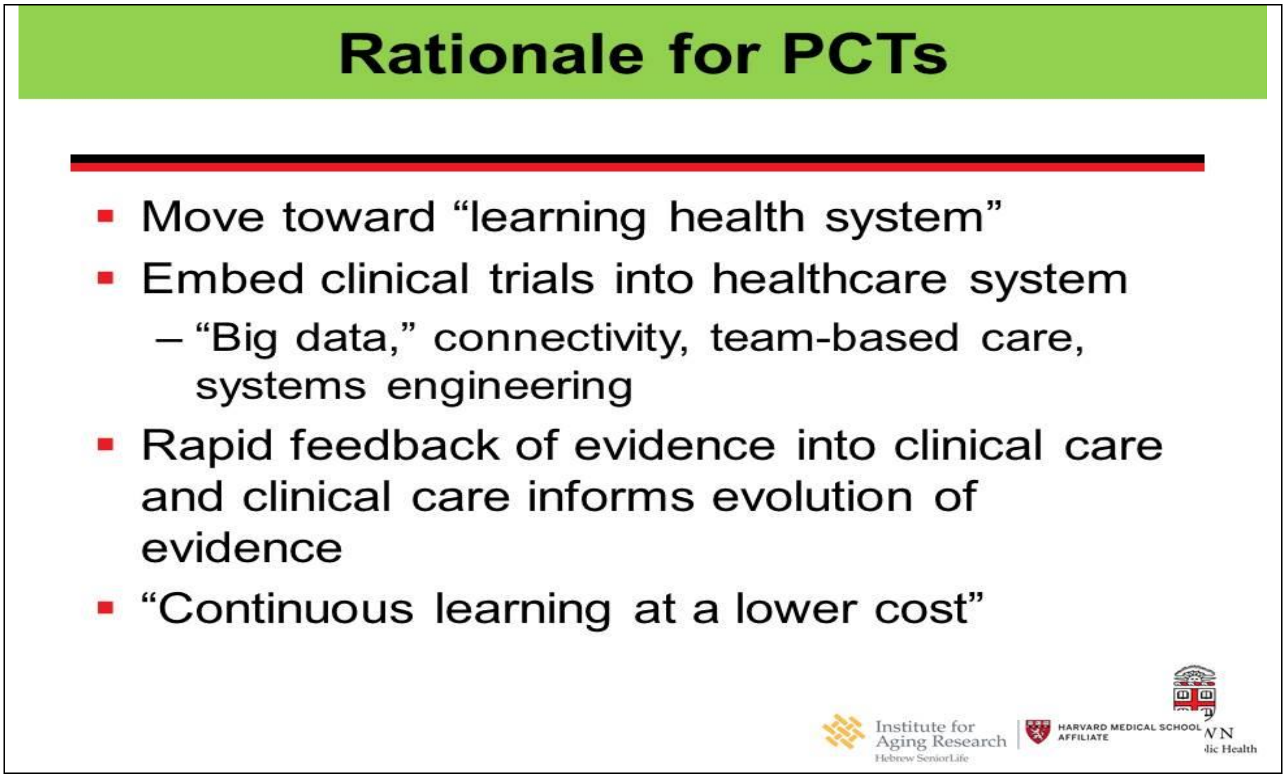
Meeting Goals

1. Review the state of the evidence regarding the effect of interventions to improve care and outcomes for people with dementia and caregivers;
2. Establish criteria for determining which interventions are ready for launch as pragmatic trials; and
3. Consider the infrastructure necessary to prepare to conduct, translate, and disseminate such a program of research.



Rationale for Pragmatic Clinical Trials (PCTS)

- Need evidence to <u>inform decisions</u> that lead to improved, efficient, and affordable care
- Historical disconnect between research and clinical care
- Shortcoming of traditional RCTs
  - – Stand-alone settings to ensure validity
  - – Non-diverse populations
  - – Underpowered
  - – Expensive
  - – Not applicable to "real-world"



Rationale for PCTs

- Move toward "learning health system"
- Embed clinical trials into healthcare system
  - "Big data," connectivity, team-based care, systems engineering
- Rapid feedback of evidence into clinical care and clinical care informs evolution of evidence
- "Continuous learning at a lower cost"

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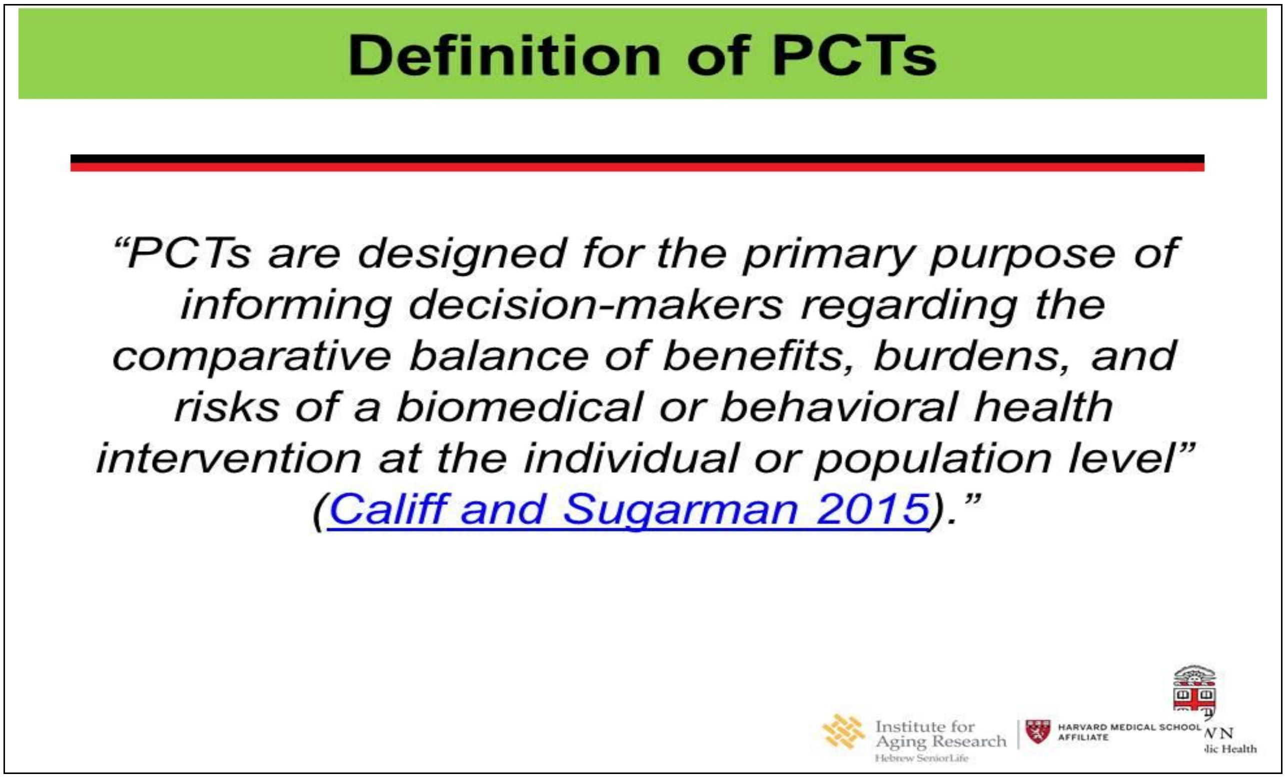

Definition of PCTs

"PCTs are designed for the primary purpose of informing decision-makers regarding the comparative balance of benefits, burdens, and risks of a biomedical or behavioral health intervention at the individual or population level" (Califf and Sugarman 2015)."

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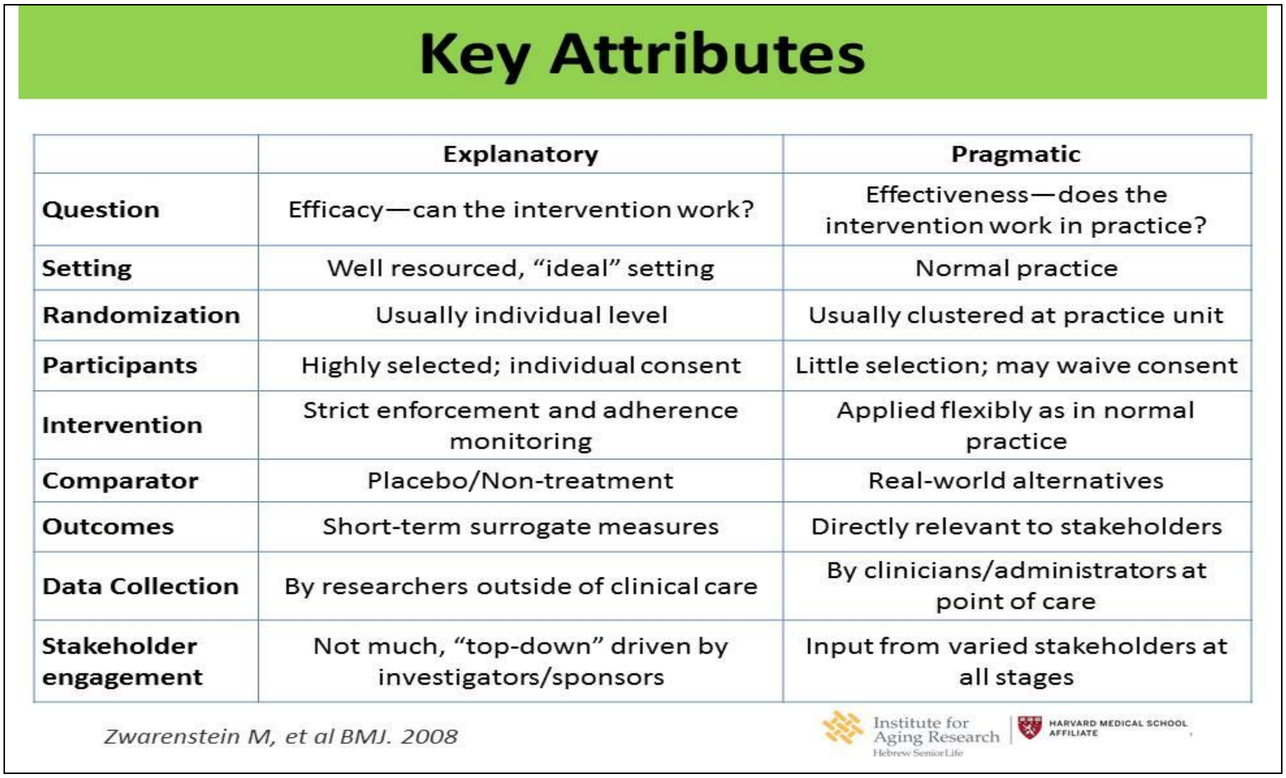

Key Attributes

	Explanatory	Pragmatic
Question	Efficacy—can the intervention work?	Effectiveness—does the intervention work in practice?
Setting	Well resourced, “ideal” setting	Normal practice
Randomization	Usually individual level	Usually clustered at practice unit
Participants	Highly selected; individual consent	Little selection; may waive consent
Intervention	Strict enforcement and adherence monitoring	Applied flexibly as in normal practice
Comparator	Placebo/Non-treatment	Real-world alternatives
Outcomes	Short-term surrogate measures	Directly relevant to stakeholders
Data Collection	By researchers outside of clinical care	By clinicians/administrators at point of care
Stakeholder engagement	Not much, “top-down” driven by investigators/sponsors	Input from varied stakeholders at all stages

Zwarenstein M, et al BMJ. 2008

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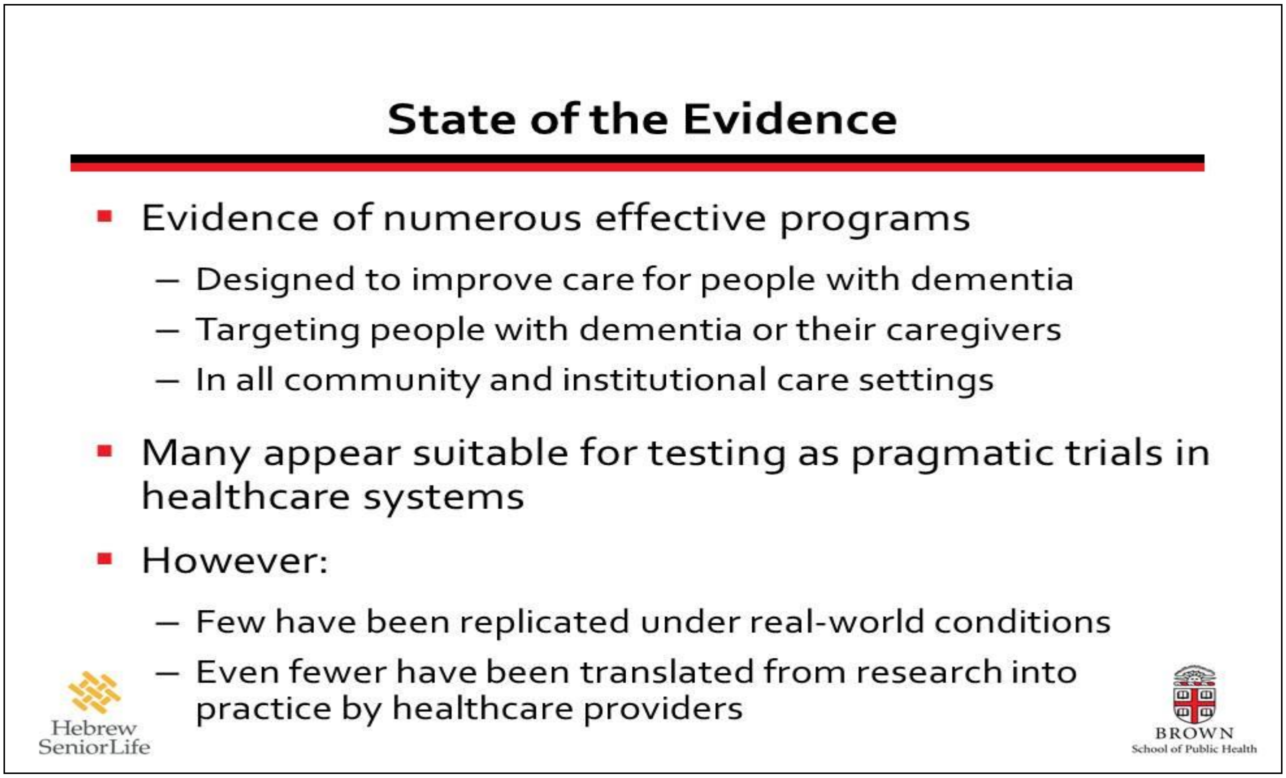

State of the Evidence

- Evidence of numerous effective programs
  - Designed to improve care for people with dementia
  - Targeting people with dementia or their caregivers
  - In all community and institutional care settings
- Many appear suitable for testing as pragmatic trials in healthcare systems
- However:
  - Few have been replicated under real-world conditions
  - Even fewer have been translated from research into practice by healthcare providers

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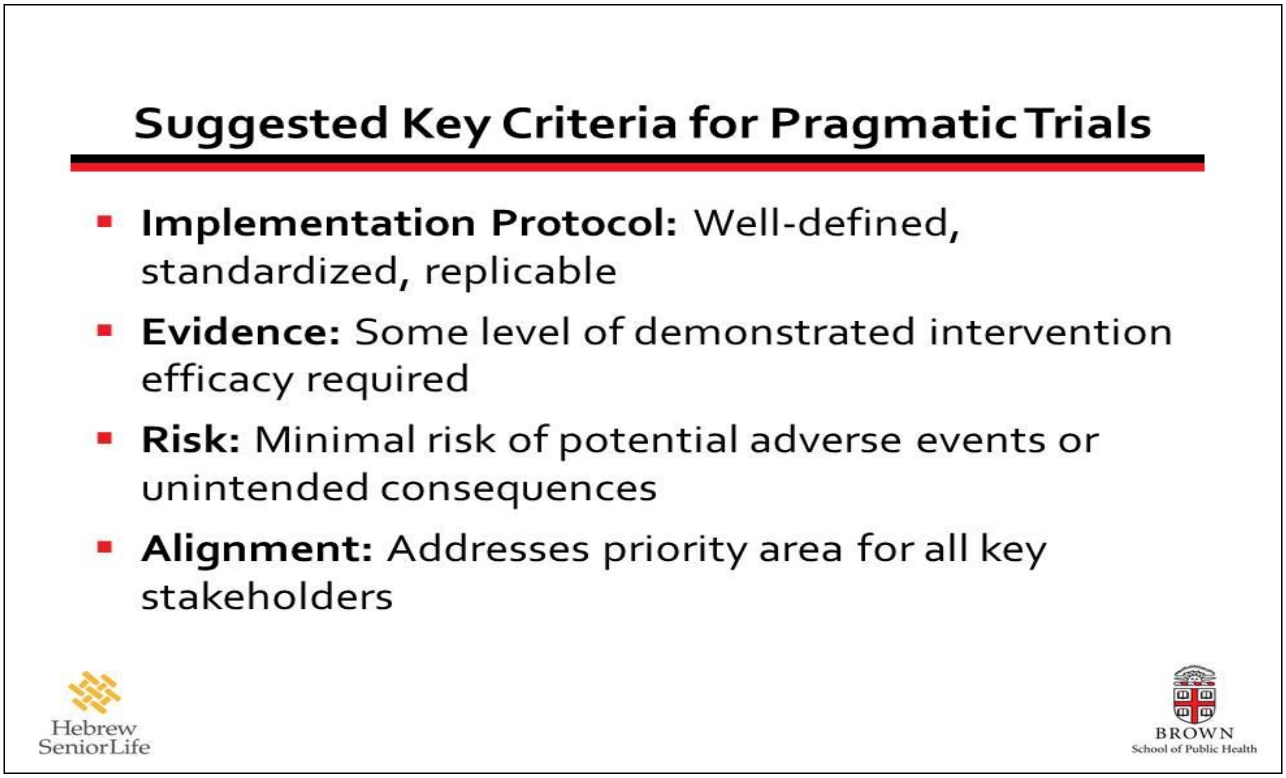

Suggested Key Criteria for Pragmatic Trials

- **Implementation Protocol:** Well-defined, standardized, replicable
- **Evidence:** Some level of demonstrated intervention efficacy required
- **Risk:** Minimal risk of potential adverse events or unintended consequences
- **Alignment:** Addresses priority area for all key stakeholders

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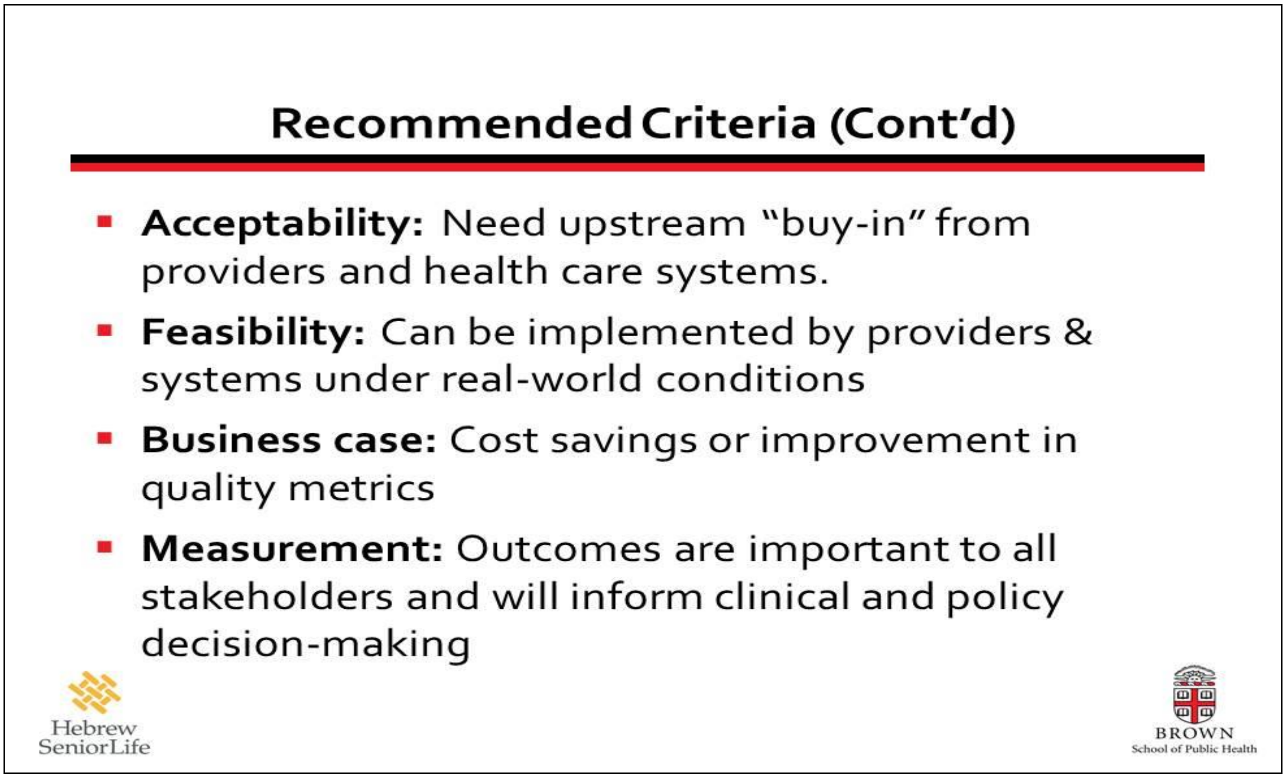

Recommended Criteria (Cont'd)

- **Acceptability:** Need upstream "buy-in" from providers and health care systems.
- **Feasibility:** Can be implemented by providers & systems under real-world conditions
- **Business case:** Cost savings or improvement in quality metrics
- **Measurement:** Outcomes are important to all stakeholders and will inform clinical and policy decision-making

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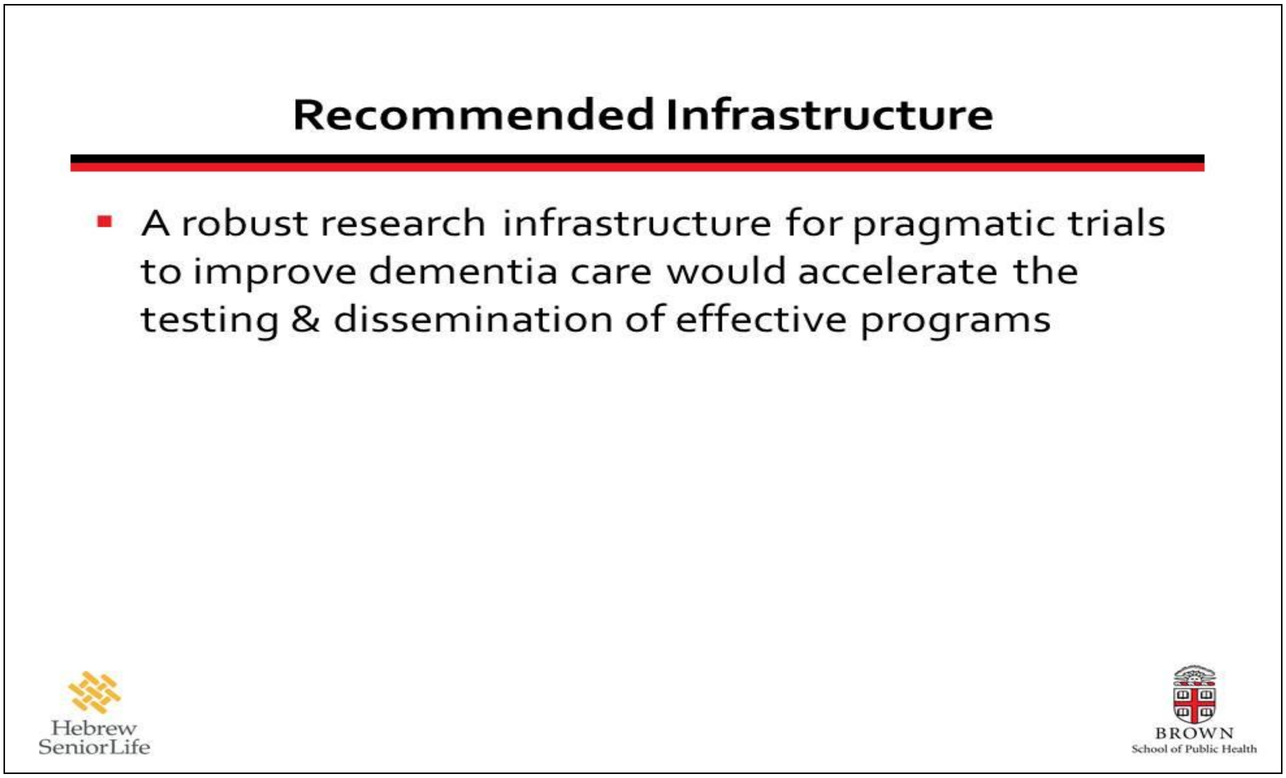

Recommended Infrastructure

- A robust research infrastructure for pragmatic trials to improve dementia care would accelerate the testing & dissemination of effective programs

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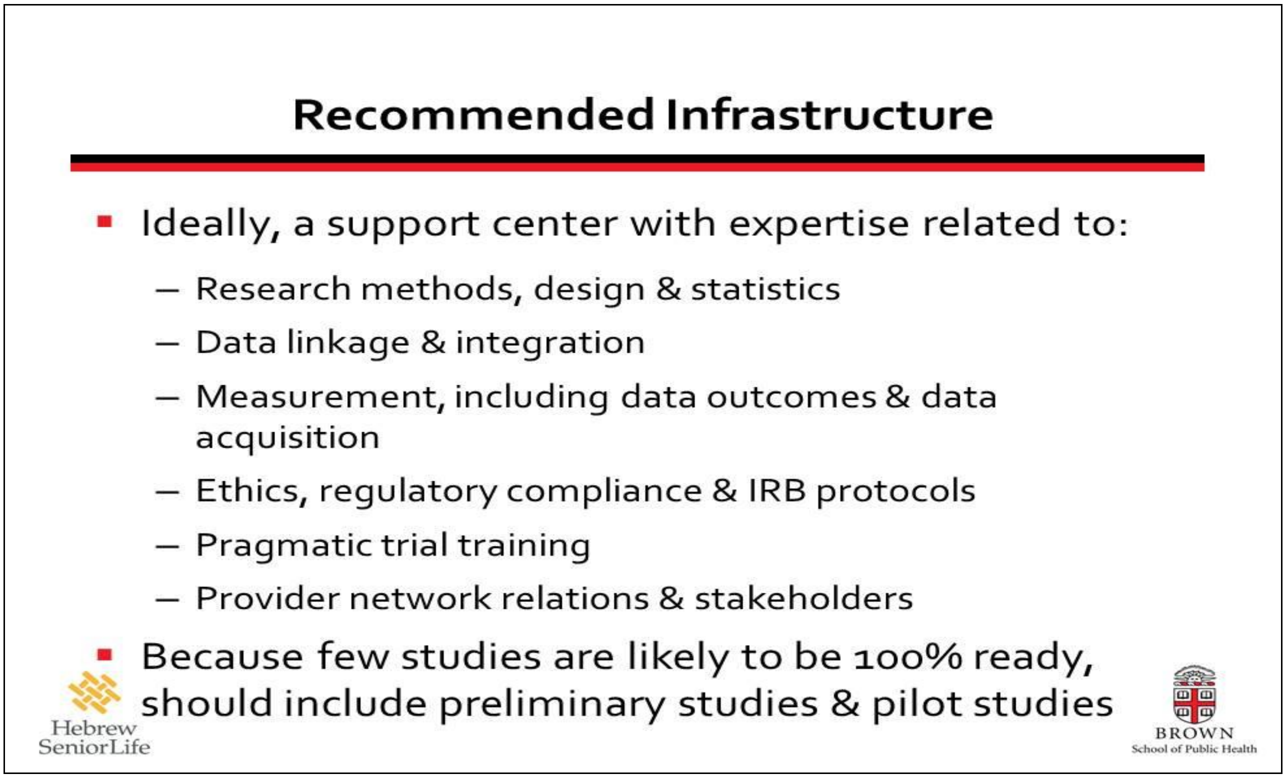

Recommended Infrastructure

- Ideally, a support center with expertise related to:
  - Research methods, design & statistics
  - Data linkage & integration
  - Measurement, including data outcomes & data acquisition
  - Ethics, regulatory compliance & IRB protocols
  - Pragmatic trial training
  - Provider network relations & stakeholders
- Because few studies are likely to be 100% ready, should include preliminary studies & pilot studies

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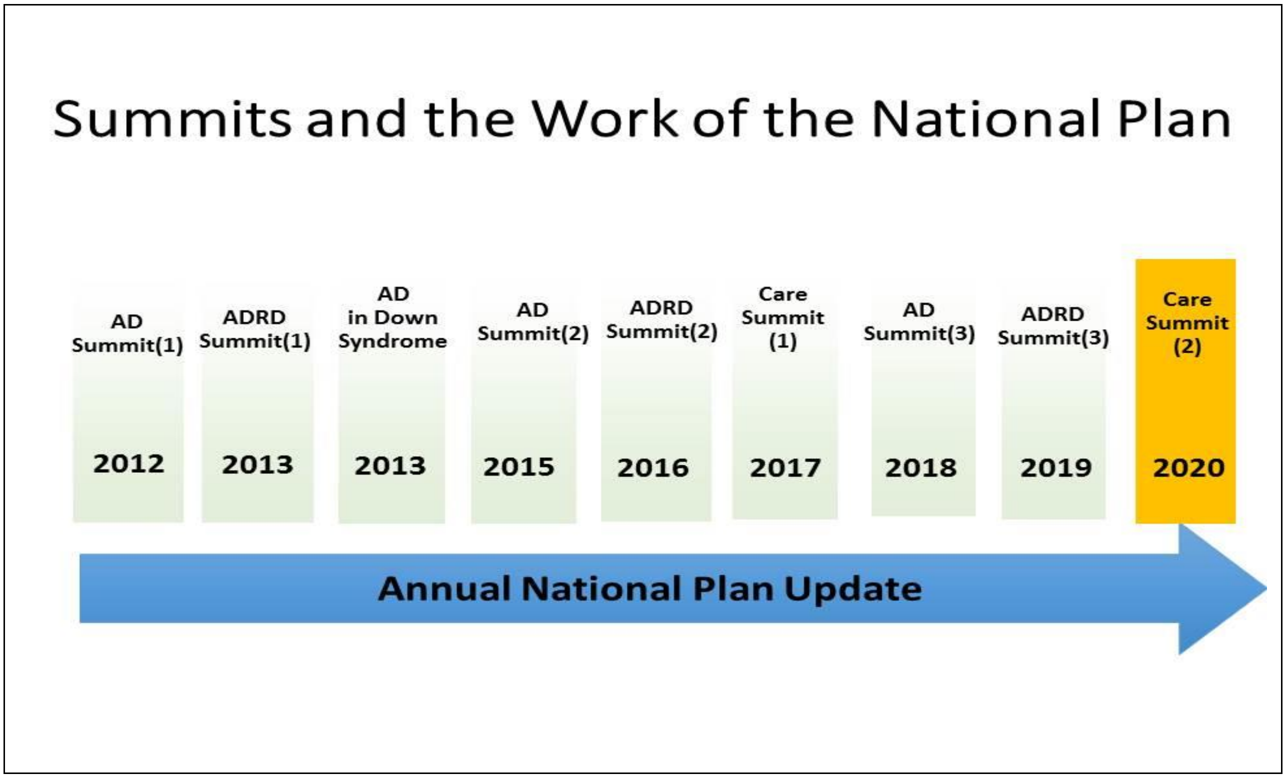
RESEARCH SUMMIT ON DEMENTIA CARE

Building Evidence for Services and Supports

UPDATE

Laura N. Gitlin and Katie Maslow

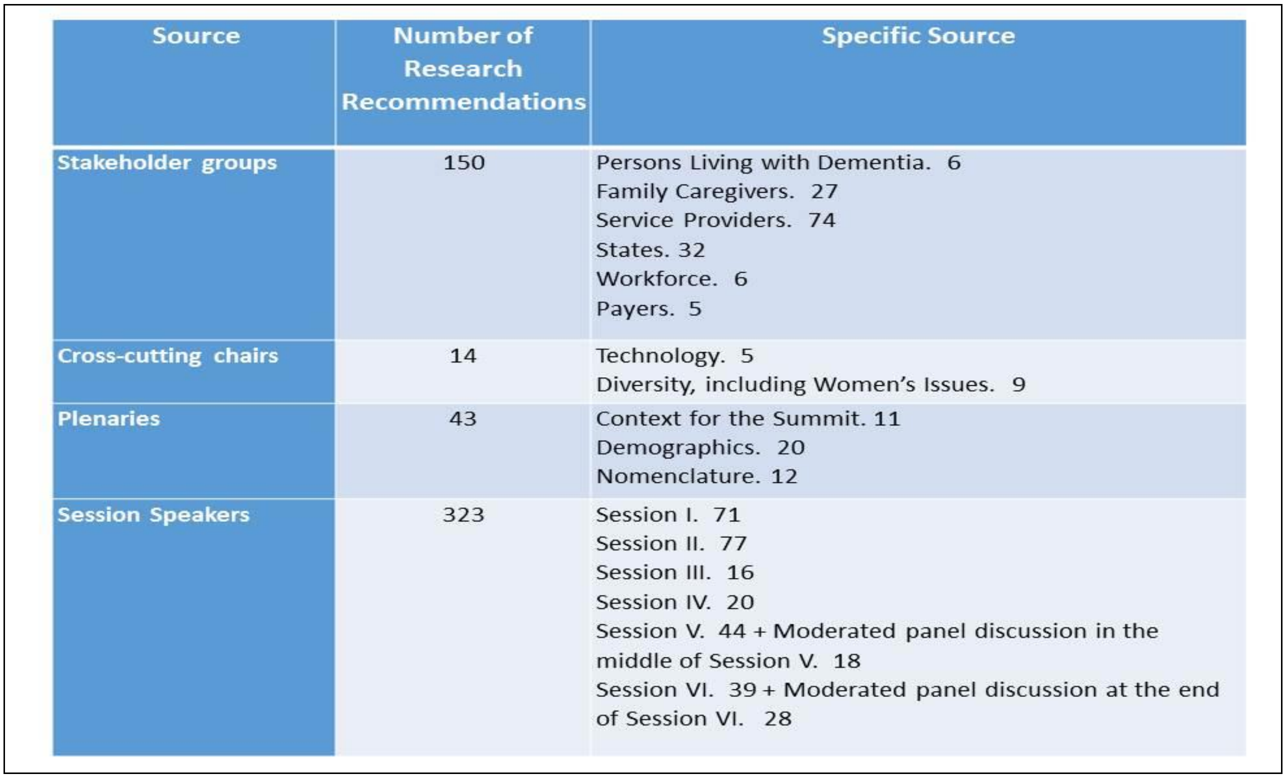
Presentation to the Advisory Council on Alzheimer's Research, Care, and Services
January 26, 2018



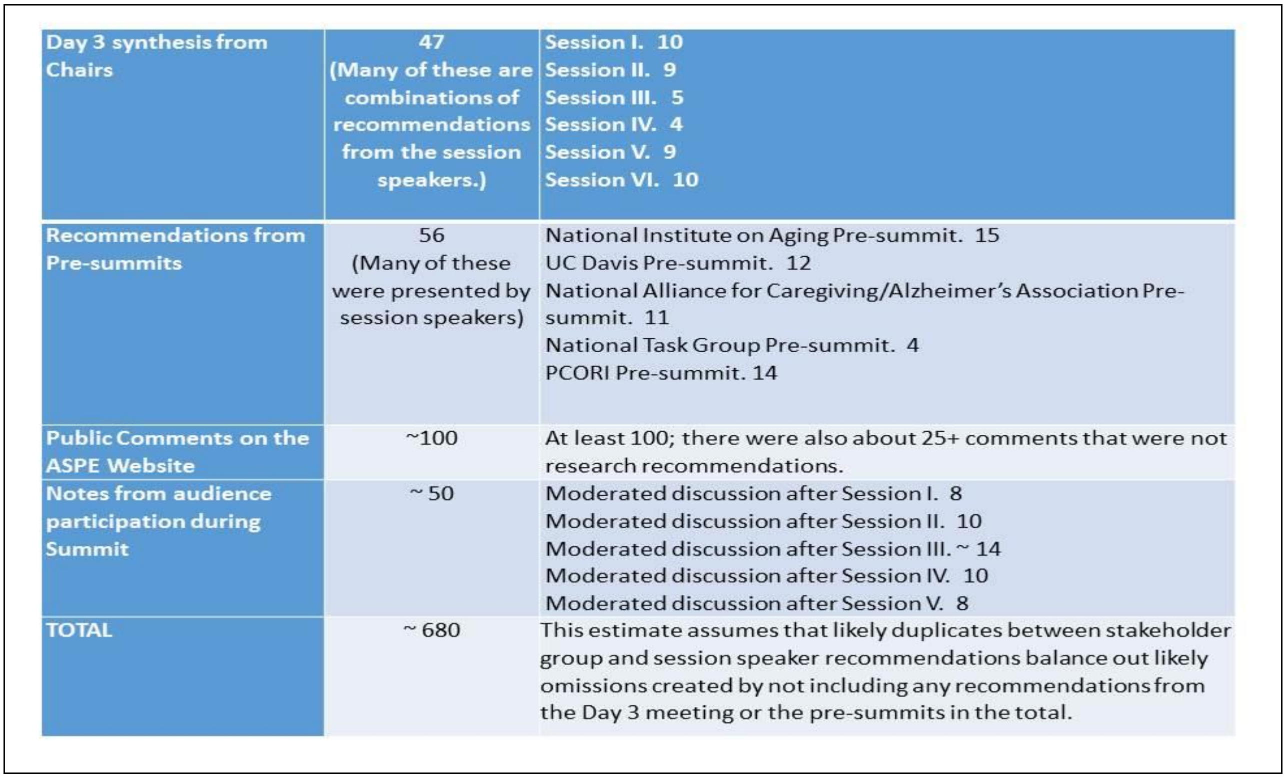
Summits and the Work of the National Plan

AD Summit(1)	ADRD Summit(1)	AD in Down Syndrome	AD Summit(2)	ADRD Summit(2)	Care Summit (1)	AD Summit(3)	ADRD Summit(3)	Care Summit (2)
2012	2013	2013	2015	2016	2017	2018	2019	2020

Annual National Plan Update



Source	Number of Research Recommendations	Specific Source
Stakeholder groups	150	Persons Living with Dementia. 6 Family Caregivers. 27 Service Providers. 74 States. 32 Workforce. 6 Payers. 5
Cross-cutting chairs	14	Technology. 5 Diversity, including Women's Issues. 9
Plenaries	43	Context for the Summit. 11 Demographics. 20 Nomenclature. 12
Session Speakers	323	Session I. 71 Session II. 77 Session III. 16 Session IV. 20 Session V. 44 + Moderated panel discussion in the middle of Session V. 18 Session VI. 39 + Moderated panel discussion at the end of Session VI. 28



Day 3 synthesis from Chairs	**47** **(Many of these are combinations of recommendations from the session speakers.)**	**Session I. 10** **Session II. 9** **Session III. 5** **Session IV. 4** **Session V. 9** **Session VI. 10**
Recommendations from Pre-summits	56 (Many of these were presented by session speakers)	National Institute on Aging Pre-summit. 15 UC Davis Pre-summit. 12 National Alliance for Caregiving/Alzheimer's Association Pre-summit. 11 National Task Group Pre-summit. 4 PCORI Pre-summit. 14
Public Comments on the ASPE Website	~100	At least 100; there were also about 25+ comments that were not research recommendations.
Notes from audience participation during Summit	~ 50	Moderated discussion after Session I. 8 Moderated discussion after Session II. 10 Moderated discussion after Session III. ~ 14 Moderated discussion after Session IV. 10 Moderated discussion after Session V. 8
TOTAL	~ 680	This estimate assumes that likely duplicates between stakeholder group and session speaker recommendations balance out likely omissions created by not including any recommendations from the Day 3 meeting or the pre-summits in the total.

Organization of Recommendations

11 Themes

- Focus areas
  - Research recommendations
    - Specific recommendations

Summit Themes

- **Heterogeneity**
- **Clinical Approaches and the Lived Experience of Persons with Dementia**
- **Caregiver Relationships, Roles, and Networks**
- **Comprehensive Models of Care Across Trajectories and Care Settings**
- **Strategies for Scaling and Disseminating Existing Evidence, Drawing Upon Implementation Science**
- **Dementia-Related Terminology and Stigma: Words Matter**
- **Financial Burden and Costs**
- **Living Arrangements, Care Settings, and Persons with Dementia who Live Alone**
- **Technology**
- **Workforce**
- **Research Methodology**

Dissemination Plan

- Initial publication ideas
  - Overview of Summit methodology and all in approach
  - Research recommendations
  - Stakeholder reports
  - Summary of evidence from each session (series of articles in a journal such as TG)
  - Other?
- Presentations at conferences
- Identification of funders for specific research recommendations
- Other (?)

January 26, 2018 -- Advisory Council Meeting #27

The meeting was held on Friday, January 26, 2018, in Washington, DC. The Research Subcommittee took charge of this meeting's theme, focusing on the process from targets to treatments. The Council heard speakers on the preclinical pipeline, the clinical trial pipeline, and the industry perspective. The meeting also included discussion of a driver diagram to guide the Council's future work, updates and a report from the October Care Summit, and federal workgroup updates. Material available from this meeting is listed below and is also available at https://aspe.hhs.gov/advisory-council-alzheimers-research-care-and-services-meetings#Jan2018.

Comments and questions, or alerts to broken links, should be sent to napa@hhs.gov.

General Information

Agenda	[HTML Version] [PDF Version]
Meeting Announcement	[HTML Version] [PDF Version]
Meeting Summary	[HTML Version] [PDF Version]
Public Comments	[HTML Version]

Handouts

Care Summit Report Themes	[PDF Version]
NAPA Driver Diagram Draft Examples	[PDF Version]
Outline for Care Summit Final Report	[PDF Version]

Presentation Slides

AbbVie's R&D Vision for Alzheimer's Disease	[HTML Version] [PDF Version]
Care Summit Report	[HTML Version] [PDF Version]
Clinical Subcommittee Update	[HTML Version] [PDF Version]
Initiatives, Partnerships and Collaboration to Help Patients with the Highest Unmet Need: Dominantly Inherited Alzheimer's Disease Trials Unit (DIAN-TU) as a Case Example	[HTML Version] [PDF Version]
Long-Term Services and Supports Committee Update	[HTML Version] [PDF Version]

NAPA Driver Diagram	[HTML Version] [PDF Version]
Overview of the Clinical Trial Pipeline for AD	[HTML Version] [PDF Version]
Overview on NIA Preclinical Pipeline	[HTML Version] [PDF Version]
Participating in an Alzheimer's Clinical Study: Perspectives on Involvement of a Person Living with Dementia and Her Study Partner	[HTML Version] [PDF Version]
Progress Since October	[HTML Version] [PDF Version]
Research Progress on Alzheimer's Disease and Related Dementias	[HTML Version] [PDF Version]
Research Subcommittee Agenda: The Journey from Targets to Treatments	[HTML Version] [PDF Version]

Videos

Updates since October meeting	[Video]
NAPA Driver Diagram	[Video]
Federal Updates	[Video]
Public Comments	[Video]
Research Subcommittee Agenda	[Video]
Care Summit Update	[Video]

Last Updated: 06/09/2018