Perspective

Public-Private Partnerships to Develop Treatments

AARR-PAD2020 Workgroup [WG]

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Preface: This document describes the mission, background and rational for a proposed Workgroup [WG] to develop recommendations for planning, organizing and managing a public-private partnership; designed to accelerate the discovery of cures for Alzheimer's disease [AD].

Objective: Convene a WG to develop actionable public policy recommendations that would foster the creation of novel *'Public-Private Partnerships'*. The objective of the action plan proposed by the WG is to provide experts' input to the ongoing *National Strategic Planning* process by the *National Alzheimer's Project Act [NAPA]*. The WG's suggestions for an action, as a *Perspective* paper, will be published in a future issue of *Alzheimer's & Dementia;* the ideas proposed could also provide the foundation for new RFAs, program or legislative initiatives by the Alzheimer's Association.

Process: The WG will operate as a *virtual think-tank;* organized to reflect a broad spectrum of perspectives. The participants will include not only a subgroup or task force from Alzheimer's Associations' Research Roundtable [AARR] but also other viewpoints or experts from academia, government and private organizations. The projected *'WG on Public-Private Partnerships'* will be organized according to the 'Memorandum of Understanding [MoU] between the Campaign to Prevent Alzheimer's Disease by 2020 [PAD2020] and the Alzheimer's Association [AA] concerning joint ventures in strategic planning. The AA-PAD2020 partnership is committed to ensuring that the full potential of NAPA is realized by creating a platform, through virtual workgroups or 'Think-Tanks', to engage the vast network of scientists in the planning process. The goal is to tap the expert knowledge of the research community to help shape the principles, concepts and recommendations that should be included in the final *National Strategic Plan*.

Background: The National Alzheimer's Project Act [NAPA] was passed by both houses of Congress and signed into law (PL 111-375) by President Obama on January 4, 2011. The Act [NAPA] authorized the:

a) Creation of National Strategic Plan to address the Alzheimer's crisis
b) Coordination of Alzheimer's disease efforts across the federal government
c) Formation of an Advisory Council and,
d) Annual Reports to Congress, which will provide
    1 – Updates on the National Strategic Plan
    2 - Recommendations for priority actions
    3 - Evaluation of all federally funded efforts in Alzheimer's research

The membership of NAPA's Advisory Council[1] is constituted to reflect the diversity of constituencies involved in different aspects of *research*, *care* and *services*. The role of the Council is to:

- make recommendations to The Secretary of the Department of Health and Human Services (DHHS), who has the ultimate authority for preparing the final National Strategic Plan
- coordinate federal agencies conducting Alzheimer's research and
- participate in the evaluation and strategic planning process

The great hope of the research community is that NAPA will finally succeed in Alzheimer research a national priority. Potentially, PL 111-375 promises to create the same successes as have been demonstrated in the battles against other diseases e.g., HIV/AIDS, influenza and pneumonia, and stroke.

The legislative mandate of NAPA covers a broad array of issues related to *research*, *care* and *services*. However, the mission of proposed WG is limited to research; they will focus on developing recommendations to shaping public policies concerning *national research resources-capabilities* in therapy development. The critical challenge for the Council, and thus the Secretary, DHHS, in this strategic planning process is the availability of well vetted credible recommendations by experts. The ultimate effectiveness of the Council/NAPA will depend on new ideas for initiatives/programs that are *transformative* rather than those that promote *business-as-usual solutions*; aimed at protecting individual turfs.

Specific Aims: The goal of the AARR-PAD2020 *'WG on Public-Private Partnerships'* is to outlining a forward looking bold vision that would outline specific mechanisms to accelerate the discovery-development of interventions to slow or prevent disease progression. The objective is to provide NAPA's Advisory Council/ Secretary, [DHHS] specific recommendations for inclusion in the final National Strategic Plan. The plan of action developed by the WG should address the critical parameters for forging strategic 'Public-Private Partnerships' by expanding the role of industry in the area of pre-competitive research.

Rationale: One of the important dilemmas for Alzheimer's research is the challenge of developing interventions to alter [delay or prevent] the progression of the disease. The solution of this problem will be difficult; thus it will require a set of public policies and/or program initiatives that would promote: a) substantial investments in drug discovery-development research and b) the formulation of new paradigms for planning, administering and funding of drug discovery-development enterprises e.g., formulation of novel mechanism such as *public-private partnership* for 'managing' or reducing scientific and financial 'risks' in therapy development. The aim of the AARR-PAD2020 WG is to explore/recommend the creation of novel organizational-administrative paradigms to facilitate the therapy development process thus enriching the pipeline of potential therapeutic options.

Presently there are no effective treatments. The lackluster productivity of current drug development paradigms mandates the need to explore different approaches to enrich the pipeline for novel

[1] *The **Federal** representations on the Advisory Council include: Administration on Aging ❖ Agency of Healthcare Research and Quality ❖ Centers for Disease Control and Prevention ❖ Centers for Medicare and Medicaid Services ❖ Department of Veterans Affairs ❖ Food and Drug Administration ❖ Indian Health Service ❖ National Institutes of Health ❖ National Science Foundation ❖ The Surgeon General. The **Non-Federal** representations (2 each) include: Alzheimer's Caregivers ❖ Alzheimer's Patient Advocates ❖ Health Care Providers ❖ Researchers with Alzheimer's Experience ❖ State Health Departments ❖ Voluntary Health Associations*

promising therapeutic targets/agents. Purely competitive research models that involve separate groups working alone simply has not delivered the solutions to the complex problem neurodegeneration. Now there is a need for a coordinated effort in a non-competitive environment for validating all of the potential therapeutic targets. An open process for sharing information across research groups (academia and industry) will enable us to: 1) quickly rule in or out particular approaches, 2) avoid redundancy and blind attacks that lead to ambiguous and costly clinical trials, and 3) rank targets with the appropriately focused strategy for development.

No single entity, in academia, industry or government, has all of the necessary ingredients [i.e., scientific knowledge, technical capabilities or resources] to accelerate the process of discovery and validation of promising interventions. Thus it is imperative for NAPA to have credible recommendations from experts on how to forge collaborative research and development [R&D] agreements among all stakeholders - government, academia, industry, NGO and voluntary health organizations. The final version of NAPA's National Strategic Plan must include an action plan for developing/promoting ***new paradigms for government-industry-academia collaborations.***

At present, the competing priorities, missions, agendas, and perspectives of stakeholders lead to program initiatives that are at cross-purposes or duplicative. The new model must eliminate organizational, administrative and, legal barriers by reengineering the structures-processes for collaborations in R & D across the full spectrum of activities, from early discovery to clinical validation of interventions. The NAPA plan must develop reasonable and fair financial incentives to industry partners both to expand research on new treatments and to collaborate with academic and government research on these projects.

The specific task of the WG is to recommend an action plan for bridging the critical gap that exists today between various phases of therapy development i.e., a) basic research, b) drug discovery and c) development. Typically, academia is the best environment at identifying new disease mechanisms, cellular pathways and new potential therapeutic targets. However, in current modes of operation academia has very limited resources, capabilities or the inclination to take basic discoveries to the next essential steps towards drug discovery-development. Meanwhile, due to the growing financial and scientific 'risks' aversion, biotechnology and pharmaceutical companies presently also are having difficulties to fund very early stage drug discovery efforts; beginning with target identification and validation. Here is where the opportunity resides, for the AARR-PAD2020 WG to develop recommendations to surmount this hurdle.

The aim is to evaluate various mechanisms/approaches/programs for joint ventures among different laboratories (in academia and industry) and suggest options/plans for . The WG will explore approaches for collaborations on various stages of drug development, including medicinal chemistry, drug metabolism and pharmacokinetics, toxicology, proof of concept in preclinical animal models, and ultimately the design and conduct of clinical trials.

The different prototypes proposed by the WG should:

- Evaluate the *merits* and the *limitations* of current ongoing programs/initiatives that provide template for some aspects of public-private partnerships e.g., ADCS, ADNI, NIH Translational Medicine, or Industry-Academia partnerships at UPenn, Duke, WashU, Harvard, UCSF others [the goal is to build upon or expand these programs rather than reinvent or replicate them]

- Propose plans that will allow collaboration in various stages of drug development and help usher more efficient approaches to therapy discovery-development
- Outline ideas to potentiate funding of collaborative projects, leading to broader exchanges of novel ideas
- Address the needed reforms concerning intellectual property issues that restrict progress
- Recommend options for novel organizational-administrative structure to plan and manage such partnerships
- Develop a 'professional judgment' budget estimate
- Define the parameters for a modest *proof-of-concept* 'demonstration program'
- Evaluate various *funding mechanism* for a pilot program e.g., a modified version 'Cooperative Research & Development Agreement [CRADA]' currently used by NIH to foster collaborative research between intramural scientist and industry