

ResearchersAgainstAlzheimer's

May 9, 2013

The Honorable Kathleen Sebelius
Secretary
U.S. Department of Health and Human Services
200 Independence Avenue, SW
Washington, DC 20201

VIA ELECTRONIC DELIVERY

Dear Secretary Sebelius:

ResearchersAgainstAlzheimer's – a group of over 350 scientists dedicated to stopping Alzheimer's disease – strongly supports the National Plan to Address Alzheimer's Disease and its bold but necessary goal 1 of preventing and effectively treating Alzheimer's disease by 2025. While this target will be a challenge – similar to past national efforts to send a man to the moon and map the human genome, we firmly believe it is achievable if commensurate resources are committed and strategies deployed. To maximize our chances at success, we strongly support the “Interim Milestones to Complete the First Goal of the National Alzheimer's Plan by 2025” that were included in the recommendations unanimously approved earlier this year by the Advisory Council on Alzheimer's Research, Care and Services, and we strongly encourage that such milestones be included in the next iteration of the national plan.

Including a specific set of time-bound milestones would be useful to all stakeholders, particularly those in medicine and science, striving to achieve the 2025 goal, as they will establish important interim markers to measure progress and determine potential amendments in the strategy. Absent such milestones, it would be far more difficult to track and measure progress against the goal.

As the Administration has clearly noted, the 2025 goal and the overall plan are national rather than federal goals meaning their success is dependent upon the active participation of multiple stakeholders including government, academia, industry, and non-governmental organizations. A tangible demonstration of this commitment is the level of resources dedicated to the task. While we recognize the fiscal challenges facing the nation as well as the reality that federal discretionary funding is determined in most cases on an annual basis, we think the milestones document would be further strengthened by including funding estimates, targets or ranges necessary to achieve the milestones. Such data would make for a more complete picture and help inform federal and non-federal funding decisions,

including public-private partnerships. It would also help inform the annual Congressional budget and appropriations process by providing lawmakers with expert recommendations they can use in establishing department, agency, and program funding levels and in setting priorities.

In addition to including funding estimates, we offer the following thoughts as to ways the milestones chart could be strengthened further:

Research Milestones

- Include throughout the document a stronger focus on basic research milestones, particularly benchmarks for discoveries of novel targets, to ensure the early-stage portion of the pipeline and process is not overlooked, as well as process reforms intended to reduce the time, the cost, and the risk of development therapies (*More under regulatory*). While we are hopeful that therapies and/or means of prevention will be available and accessible by 2025 if not sooner, achieving this goal does not negate the need for further research, particularly to develop more effective interventions and, hopefully one day, therapies to reverse the course of the disease and to cure it outright.
- Include during the 2012-2015 timeframe a milestone focused on increasing the understanding as to potential differences treatments may have based on each individual patient's condition (e.g. Apolipoprotein E genotype, amyloid status etc).
- Include during the 2016-2020 timeframe a milestone focused on the translation of promising therapeutic interventions into clinical testing.
- Include throughout the research component the establishment of a government co-investment function that is modeled upon other successful programs in other sectors and that strives to bridge the "valley of death" that is so daunting in the Alzheimer's and neuroscience space. Such an entity would complement NIH and other research efforts by leveraging relatively small amounts of funding to advance targeted, goal-oriented, and milestone driven project.

Regulatory Milestones

- As noted above, set specific regulatory goals aimed at compressing the time and cost of Alzheimer's therapy development. This effort should include specific action under all timeframes and set an overarching goal whereby the average development and review timeline for an Alzheimer's therapy would be reduced by a specific amount of time.

- Include during the 2012-2015 timeframe the issuance of FDA guidance pertaining to clinical trials involving combination therapies, and modify the “FDA adaptive trial model” to “FDA adaptive trial paradigm.”

Beyond these points, we recommend that the Department continually work to add greater levels of specificity to the milestones, particularly those pertaining to validated endpoints and approved treatments. Overall, this set of milestones must be seen as a constantly evolving, living, and breathing document and should be as comprehensive as the National Plan to Address Alzheimer’s Disease. To that end, while the NIH and FDA are perhaps the two most important agencies in terms of achieving the 2025 goals, we recommend broadening this solid foundation so all key departments and agencies participating in the plan and Advisory Council, are incorporated.

We applaud you for your leadership in striving to stop Alzheimer’s disease and thank you for considering this request. If you have any questions, please contact Dave Morgan, Lead Representative of ResearchersAgainstAlzheimer’s and CEO of the Byrd Alzheimer’s Institute at the University of South Florida at dmorgan@ResearchersAgainstAlzheimers.org.