Attachment—Additional Questions for the Record

Subcommittee on Health
Hearing on
" The Future of Medicine: Legislation to Encourage Innovation and Improve Oversight "
March 17, 2022

Jeff Allen, Ph.D., President and CEO, Friends of Cancer Research

The Honorable Michael C. Burgess, M.D. (R-TX)

1. It seems to me as though the goal for ARPA-H is to bring into focus our domestic R&D efforts to address some of the larger challenges in health care. However, I stand by the viewpoint of being intentional and not duplicative with funding and believe that there needs to be additional conversations on structure of the agency. The original intent of Center for Medicare and Medicaid Innovation Center was to fuel innovation. Are there suggestions you have for reforms to other agencies or programs such as CMMI, that could save dollars and prevent duplication within ARPA-H?

While the overarching goal of facilitating innovation may be shared, the focus of CMMI and the mechanisms that CMMI and ARPA-H utilize will enable a quite different scope of work. CMMI has leveraged its ability to conduct pilot projects to explore innovation in payment models to help support optimization and efficiencies for Medicare beneficiaries. ARPA-H will provide direct funding and contracting intended to support work much earlier in the innovation cycle including transformational capabilities with broad application across multiple disease areas.

ARPA-H should embrace the goal of developing advanced proofs of concept for breakthrough technologies with the aim of transference of intellectual property rights—or licensing options—to a partner with capacities to move those technologies forward through development and commercialization. Internal pursuit within the agency of medical product development and commercialization should be the exception and should be considered only where the private sector declines licensing and development due to a perceived absence of commercial market or where there exists a compelling reason to retain ownership in the public domain. This is a focus that is unique from any other current government program at this scale.

   a. If ARPA-H was to be established, are there additional qualities or structural aspects from other agencies that can be applied to its design?

Given the origins of ARPA-H are deeply rooted in previous efforts in other sectors, it will be important for ARPA-H to adopt operational aspects that have been successful for DARPA and ARPA-E. These may include:

- **A Director, appointed by the President and reporting to the Secretary of HHS** along with any such other direct report that may be appropriate given the placement of the agency**,** with a time-limited appointment (e.g., 4 – 8 years). He/she will have full authority to establish ARPA-H as a DARPA-like contracting agency, with contracts developed by in-house program managers who select external performers, and maintain, enhance, or terminate projects based on performance against explicit milestones. ARPA-H will set priorities to define and advance the mission of the agency as informed by a multi-sectoral board of advisors.

- **World-class program managers** who define their programs, set milestones, track progress, and communicate with leaders in the healthcare, biomedical research, and technology sectors to identify new challenges and potential solutions. As in DARPA and ARPA-E, these program managers may be recruited from industry, academia, and government for a specific length of time (e.g., 3- 5 years) for what will be viewed as a coveted 'sabbatical', allowing them to work in a unique environment supportive of collaborative, time-bound, results-driven innovation.

- **Funding available for and the ability to contract with a broad range of institutions** including universities, national labs, public sector organizations, private companies, and nonprofits.

- **Funding directed through milestone-driven contracts** of a sufficient size (up to multiple millions annually) that are structured to support high-risk ventures, allow unsuccessful ventures to fail fast and allow successful projects to proceed with the expectation that this rapid, high-risk approach will yield high rewards.

- **Latitude to appoint advisors.** Similar to ARPA-E, the Director should be given latitude to draw from existing advisory committees appointed by the Secretary of HHS or to create a new advisory committee unique to the agency to advise on specific program tasks or overall direction of ARPA-H.

In addition, the autonomy of ARPA-H should be enabled with additional flexibilities such as the authority to submit its annual budget request directly to Congress concurrently with its submission to the Office of Management and Budget.

b. Are there any consequences to ARPA-H being housed in NIH?

Hopefully the main result of ARPA-H being housed in NIH will be expedited processes for establishing the agency by leveraging current infrastructure (e.g., hiring processes, employee benefits, transferring of funds, etc.). Recognizing that the potential strength of ARPA-H will be its unique programmatic focus and approach, additional authorizing language can help maintain its appropriate independence while allowing ARPA-H to capitalize on current NIH capabilities.

2. I've noticed that the ARPA-H text calls for the establishment of an "Interagency Advisory Committee." A few examples of the participants include the Director of NIH, Commissioner of FDA, the Director of the CDC, and the list goes on.

    a. Is there a plan or benefit to these individuals working with stakeholders who have actual expertise and experience?

There would certainly be a benefit for ARPA-H to have regular collaboration with and advisement from subject matter experts. The Interagency Advisory Committee's role is to ensure projects align with the needs of the healthcare continuum. The committee's interactions with members of ARPA-H will likely largely be with project managers. In turn, the project managers will ensure that the right expertise from both the public and private sectors can help inform each specific project. Future authorizing language may help establish processes to balance any potential conflicts of interest from external collaborators and facilitate robust interactions.

3. Millions of Americans suffer from some form of psychiatric and neurologic diseases. In my home state of Texas, thousands of people suffer from Alzheimer's disease, Parkinson's disease, and other diseases or disorders of the central nervous system. Despite this, FDA reviewers denied more requests for (and granted fewer) breakthrough therapy designations among neuroscience New Drug Applications (NDAs) than they did for NDAs in other disease areas. In 2017, the FDA established a Center of Excellence focused on oncology products. And since then, the agency has seen a demonstrable increase in the number of applications and approvals of oncology products. The FDA's Oncology Center of Excellence is viewed by FDA, patients, and other stakeholders as a strong success. I believe the Committee should build on these successes by establishing a Neuroscience Center of Excellence as part of our work on the FDA user fee legislation.

    a. Can you offer your view on how a Neuroscience Center of Excellence can help accelerate the review and approval of more medical products to confront neurological diseases and disorders like Alzheimer's disease, ALS, autism, Parkinson's disease, and MS benefit the millions of Americans suffering from these diseases?

The establishment and success of the Oncology Center of Excellence is largely driven by the wealth of underlying scientific knowledge supporting cancer research, development, and care. As cancer treatments have become more complex and involve multiple types of products, multiple medical centers at FDA become involved. For example, diagnostic tests used to identify patients likely to benefit from a targeted treatment are regulated in the Center for Devices and Radiological Health and cellular therapies are regulated in the Center for Biologic Evaluation and Research. Due to this multi-modal approach to treatment, it was clear that a centralized group of oncology experts was necessary to coordinate cancer-related activities across the different FDA medical product Centers and to ensure consistency, efficiency, and optimal expertise.

In considering establishing additional Centers of Excellence for other therapeutic areas, it may be helpful to evaluate areas of potential disconnect or duplication of activities at FDA that would

benefit from increased synergy related to a specific disease state (e.g., Neuroscience), as well as the strength of scientific evidence and therapy development in the given therapeutic area.