White House Office of Science and Technology Policy & National Institutes of Health
ARPA-H Listening Session: Patient Advocacy Organizations
Wednesday, July 21, 2021
Summary

Overview

On July 21, 2021 the White House Office of Science and Technology Policy (OSTP) and the National Institutes of Health (NIH) convened a listening session to gather input to inform the scientific focus of the Advanced Projects Research Agency for Health (ARPA-H). As part of the 90-minute listening session, representatives from 22 patient groups[1] discussed scientific opportunities, approaches, challenges, and partnership strategies ARPA-H might adopt.

The session was introduced by Dr. Francis Collins, the Director of NIH. Dr. Collins welcomed participants and explained the meeting goal, which was to engage participants in a discussion about making the bold, new ARPA-H concept as productive as possible. Dr. Collins noted that COVID has been our generation's "Sputnik moment" and that ARPA-H is intended to help the United States take full advantage of this moment in scientific history to accelerate progress and prevent future pandemics. He suggested that ARPA-H would follow a model that is built upon the DARPA experience, taking on high-risk and high-reward programs that would otherwise not get support. The new ARPA-H, he suggested, should engage bold, fearless leaders with entrepreneurial character and private sector experience, recruit visionary program managers, bring new research performers and collaborations into the biomedical research ecosystem, and operate using milestone-driven approaches to monitor progress and identify failures early. He also stressed that equity needs to be built into ARPA-H's structure and culture, drawing in top talent from across the United States, looking at diseases and health from a multiplicity of lenses – from the molecular to the societal – and focusing on health conditions germane to underserved populations. Dr. Collins noted that partnerships across the research and development spectrum will be needed to accomplish these goals.

After Dr. Collins' introduction, participants engaged in a question-and-answer period, followed by two breakout sessions, reconvening the groups after each for feedback sessions moderated by Dr. Lawrence Tabak, Principal Deputy Director of NIH. The first session considered challenges and barriers in the biomedical research ecosystem as a whole and for specific disease areas, including commonalities across diseases and conditions, and critical gap areas that ARPA-H could fill using an ARPA-like model. The second session considered "game-changing" tools, technologies, and processes upon which ARPA-H might focus and the patient inclusion models and partnership strategies ARPA-H might employ to catalyze progress.

Themes

Overall, there was a strong recommendation from participants for ARPA-H to consider patient needs and apply a patient-centered approach to all aspects of the funding initiatives and processes. Multiple participants emphasized the idea of formulating ARPA-H as a patient-driven organization and consider patients' needs as its "North Star". In addition, participants suggested:

[1] Alzheimer's Association, American Cancer Society, American Diabetes Association, American Liver Foundation Chronic Pain Research Alliance, Deadliest Cancers Coalition, FasterCures, Global Alzheimer's Platform Foundation, National Health Council, New America, Patient-Centered Outcomes Research Institute, American Autoimmune Related Diseases Association, American Diabetes Association, American Heart Association, amfAR, American Association for Cancer Research, EveryLife Foundation for Rare Diseases, Genetic Alliance, JDRF, National Organization for Rare Disorders (NORD), Friends of Cancer Research, and UsAgainstAlzheimer's

- ***ARPA-H should address barriers to equitable health care access.*** Participants suggested that, for many underserved populations in particular, access to care and to new interventions is a major barrier to improving health. Therefore, ARPA-H should focus not only on developing new prevention, detection, and treatment approaches, but also new models for care delivery. Including underserved and underrepresented populations in the early stages of treatment development and clinical trials could help these groups gain better access to the benefits of the research.

- ***ARPA-H should focus on developing platforms addressing the reality that every patient is unique, with a mix of interacting disease conditions, and differing genetic, environmental, and social determinants of health status.*** Participants noted that patients' health is more than the sum of individual disease conditions that they might experience and that ARPA-H should concentrate on developing holistic treatment approaches that account for multiple, interconnected conditions. Advancing new technologies to improve the speed and affordability of whole-genome sequencing, developing novel gene therapy and gene editing approaches, and identifying and standardizing other broadly-applicable research tools for studying individual patients (e.g., predictive biomarkers) were suggested as potential platform approaches to improve health outcomes across multiple diseases and conditions.

- ***ARPA-H should focus on upstream approaches such as prevention or early detection and treatment of chronic disease.*** America is a nation plagued by multiple chronic diseases. Alzheimer's, kidney disease, chronic pain, and diabetes were identified as examples of chronic conditions where prevention or early detection and treatment have the potential to improve health outcomes and are areas where an ARPA-H might focus. For many afflicted with rare diseases, correct diagnosis allowing for treatment may take years. "Long COVID" was identified as an emerging disease area worthy of focus by ARPA-H as well.

- ***ARPA-H should address priorities associated with preclinical development, clinical research, and deployment of innovations in addition to fundamental challenges.*** Participants noted that, for ARPA-H to be successful, the innovations it fosters will need to reach patients and change existing standards of care. Participants noted a range of areas in preclinical and clinical development where ARPA-H might develop platforms valuable to the biomedical enterprise broadly, including centralized preclinical resource cores that can be accessed by investigators working across a range of disease areas including rare diseases, greater use of Phase 0 clinical trials, innovative clinical trial designs, and community-based clinical trials infrastructures to improve geographic reach and accessibility. Participants also noted that ARPA-H should foster a data sharing infrastructure and create incentives for data sharing, collaborative pre-competitive research, and testbeds that facilitate collaboration and speed research and clinical approval.

- ***ARPA-H will need statutory authorities and administrative processes designed to foster flexibility, speed, and independence.*** Participants reiterated the need for ARPA-H to be given statutory authorities already leveraged by ARPA-like organizations. Participants also recommended that ARPA-H should be given the authority and independence to develop its own culture, with leadership drawn from industry or the DARPA community to reinforce these distinct ways of working.

Next Steps and Conclusion

The White House and NIH will continue to seek perspectives on ARPA-H from stakeholders. through a series of listening sessions being convened throughout July and August with representatives from patient groups, industry, biomedical scientific societies, physical science-oriented scientific societies, non-profits, and other stakeholders. OSTP and NIH will use interagency processes to promote coordination and ensure

that ARPA-H complements the priorities of NIH and other Federal research agencies. OSTP and NIH are grateful for the participation and perspectives provided by the wide variety of stakeholders in these listening sessions. Much work remains to ensure that the biomedical ecosystem is engaged in solving some of the most pressing health challenges of our time. The Administration will continue to work to ensure that the US remains a global leader in biomedical and health innovation for the benefit of all Americans.