

**White House Office of Science and Technology Policy
U.S. Department of Health and Human Services National Institutes of Health
ARPA-H Listening Sessions**

**Listening Session 1: Advocates for Research on Cancer; Heart, Lung,
and Blood Disorders; and Environmental Health
July 22, 2021**

The first of 10 listening sessions to gather feedback on the proposed Advanced Research Projects Agency for Health (ARPA-H) was held virtually on July 22, 2021, with about 350 attendees. Advocates for research on cancer; heart, lung, and blood disorders; and environmental health shared their opinions. The National Institutes of Health (NIH) is working closely with the White House Office of Science and Technology Policy (OSTP) to establish ARPA-H to focus on ambitious and innovative projects that will shape the future of health and medicine for all Americans.

Participants

White House Office of Science and Technology Policy (OSTP)

Tara A. Schwetz, Ph.D., Assistant Director for Biomedical Science Initiatives

National Institutes of Health (NIH)

Francis S. Collins, M.D., Ph.D., Director

Gary H. Gibbons, M.D., Director, National Heart, Lung, and Blood Institute (NHLBI)

Norman E. Sharpless, M.D., Director, National Cancer Institute (NCI)

Richard P. Woychik, Ph.D., Director, National Institute of Environmental Health Sciences (NIEHS)

Stakeholders

Gary Ewart, M.H.S., Chief, Advocacy and Government Relations, American Thoracic Society (ATS), New York, NY

Lewis Hsu, M.D., Ph.D., Director, Sickle Cell Center, Professor, Department of Pediatrics, University of Illinois, Chicago, IL; Chief Medical Officer, Sickle Cell Disease Association of America, Inc. (SCDAA), Hanover, MD

Mariell Jessup, M.D., FAHA, Chief Science and Medical Officer, American Heart Association (AHA), Dallas, TX

Ursula B. Kaiser, M.D., Chief, Division of Endocrinology, Diabetes and Hypertension; George W. Thorn, M.D. Distinguished Chair, Endocrinology, Brigham and Women's Hospital; Professor, Harvard Medical School; Director, Brigham Research Institute, Boston, MA; President-Elect, Endocrine Society, Washington, DC

Karen E. Knudsen, Ph.D., M.B.A., Chief Executive Officer, American Cancer Society (ACS), Atlanta, GA

Danielle Leach, M.P.A., Chief of Community and Government Relations, National Brain Tumor Society (NBTS), Newton, MA

Suzanne Leous, M.P.A., Chief Policy Officer, American Society of Hematology (ASH), Washington, DC

Jennifer W. Pegher, M.A., Executive Director, Association of American Cancer Institutes (AACI), Pittsburgh, PA

Meeting Summary

Welcome and Opening Remarks

Francis S. Collins, M.D., Ph.D., Director, National Institutes of Health (NIH)
Tara A. Schwetz, Ph.D., Assistant Director for Biomedical Science Initiatives, White House Office of Science and Technology Policy (OSTP)
Norman E. Sharpless, M.D., Director, National Cancer Institute (NCI)
Gary H. Gibbons, M.D., Director, National Heart, Lung, and Blood Institute (NHLBI)
Richard P. Woychik, Ph.D., Director, National Institute of Environmental Health Sciences (NIEHS)

Dr. Collins welcomed participants and attendees to the first of 10 listening sessions to gather feedback on the proposed Advanced Research Projects Agency for Health (ARPA-H). NIH is working closely with OSTP on ARPA-H, which is a high priority for the Biden administration. ARPA-H is designed to catalyze ambitious ideas and approaches that will shape the future of health and medicine for all Americans. The new agency, which will follow the Defense Advanced Research Projects Agency (DARPA) model, will focus on high-risk, high-reward projects and will be guided by visionary project managers. ARPA-H will recruit researchers who might otherwise not apply to NIH for support, and its projects will be driven by clearly defined milestones. OSTP and NIH wish to gather opinions from stakeholders, who will play a critical role in the establishment and success of ARPA-H. The 10 listening sessions will focus on specific research areas and will involve NIH Institute and Center (IC) directors who represent those areas.

Dr. Schwetz said that ARPA-H will strive to be transformative for biomedical research. The United States has a strong biomedical research ecosystem that is supported by NIH-funded research. Results from these research studies have informed the pharmaceutical industry in its development of treatments for a range of conditions. However, the current system has some gaps between traditional fundamental research and industry. ARPA-H will help provide a new lens and a mechanism through which to support exciting biomedical research that can improve human health. Such ambitious and cutting-edge research requires a novel funding approach, and such approaches have been used in other areas of science. In ARPA-H, OSTP and NIH aim to create a distinct entity whose leadership will have the autonomy and resources to tackle some of the biggest challenges facing human health.

Dr. Sharpless said that obtaining external input is critical for an endeavor like ARPA-H. This is a very exciting time for cancer research, and researchers are making great progress in understanding cancers and developing new therapies. However, ARPA-H will be useful to catalyze progress in specific types of projects. Cancer research is a top priority for the Biden administration, and ARPA-H will be a new tool for making advances in this field.

Dr. Gibbons said that NHLBI is excited about the opportunities that ARPA-H will present to catalyze and accelerate transformative change in ways that are limited by the current research ecosystem. Collaborative research is essential to address complex challenges that span several research fields. For example, advances in cures for sickle cell disease can have broader applications and are likely to require collaborative efforts among multiple small biotechnology

companies and traditional researchers. NHLBI is excited about ARPA-H's potential to help establish these opportunities for both rare and common diseases.

Dr. Woychik said that NIEHS is excited about the potential of ARPA-H to advance environmental health research. NIEHS has the complex task of studying the effects of environmental exposures on health. NIEHS also studies the influence of lifestyle habits and psychosocial threats. Environmental health researchers have been successful in establishing links between single exposures and disease. However, the global environmental science community realizes that it is necessary to embrace an experimental framework that moves beyond studying individual exposures. Environmental scientists aim to study the exposome to measure the effect of environmental exposures on the function of the human body across a lifetime. This will be a major challenge and will likely benefit significantly from the innovation that will be part of ARPA-H. ARPA-H could help environmental scientists usher in a new era in precision environmental health.

Comments from Invited Stakeholders

Jennifer W. Pegher, M.A., Executive Director, Association of American Cancer Institutes (AACI), Pittsburgh, PA

Karen E. Knudsen, Ph.D., M.B.A., Chief Executive Officer, American Cancer Society (ACS), Atlanta, GA

Danielle Leach, M.P.A., Chief of Community and Government Relations, National Brain Tumor Society (NBTS), Newton, MA

Mariell Jessup, M.D., FAHA, Chief Science and Medical Officer, American Heart Association (AHA), Dallas, TX

Suzanne Leous, M.P.A., Chief Policy Officer, American Society of Hematology (ASH), Washington, DC

Lewis Hsu, M.D., Chief Medical Officer, Sickle Cell Disease Association of America, Inc. (SCDAA), Hanover, MD

Ursula B. Kaiser, M.D., President-Elect, Endocrine Society, Washington, DC

Gary Ewart, M.H.S., Chief of Advocacy and Government Relations, American Thoracic Society (ATS), New York, NY

Ms. Pegher said that AACI is dedicated to accelerating progress against cancer by enhancing the impact of its member cancer centers. AACI appreciates the Biden administration's commitment to eradicating cancer, including plans to establish ARPA-H with an initial focus on cancer. Because partnerships are crucial in advancing cancer research, ARPA-H needs to adopt a collaborative approach that involves the federal government, the biomedical research community, industry, and patients. AACI's primary public policy goal is to advocate for sustained, predictable, and robust funding; the establishment of ARPA-H should complement existing funding mechanisms and not divert funds from basic research. Also, ARPA-H needs to be committed to addressing health disparities and ensuring that clinical trials are inclusive. AACI hopes that ARPA-H will be a catalyst to quickly develop innovative advances in cancer prevention and treatment. These advances will also have a significant impact on other fields of research.

Dr. Knudsen said that ACS is excited about ARPA-H. In line with its goal of improving the lives of cancer patients and their families, ACS has invested more than \$5 billion in research to find

causes and cures for cancers. However, ACS recognizes that it cannot fund all promising projects. ACS welcomes ARPA-H as a mechanism to complement existing funding structures and fast-track equitable access to cancer prevention and cures. ARPA-H has the potential to help close the gap between patients and research through targeted innovative therapies. To be successful, ARPA-H needs to be patient-centered, built on existing research infrastructure, and transparent in its goals and project selection process. Also, ARPA-H needs to stay focused on its goal of funding innovative projects with the potential for broad impact, and it needs to provide additional avenues for research funding and not take away from existing funds. ACS will support ARPA-H's efforts to advance innovative cancer research.

Ms. Leach said that ARPA-H could help foster collaborations among all brain tumor research organizations and stakeholders. Brain cancer treatments can influence treatments in other fields. ARPA-H could fund innovative projects such as novel brain tumor biopsy methods, liquid biopsy methods, brain imaging technology, and methods to deliver targeted drugs to the brain. Clarifying how ARPA-H will coordinate with NIH and NCI in funding brain cancer research would be helpful. It would be beneficial for ARPA-H to work with existing research pipelines and the biotechnology industry and to tap into the expertise of patient advocacy organizations. NBTS supports the establishment of ARPA-H and will work with ARPA-H to advance brain tumor research.

Dr. Jessup said that AHA has reaffirmed its long-standing commitment to health equity and hopes that ARPA-H will accelerate accessible and affordable health care for all. AHA hopes that ARPA-H will be structured in a way that fosters a unique culture of innovation, entrepreneurship, and big-picture thinking that leads to transformative breakthroughs. Given the urgency of its mission and potential to transform health, ARPA-H must be focused on rapid translation, implementation, and efficacy of priorities while adhering to the highest levels of scientific standards and integrity. ARPA-H should ensure that diverse voices are part of its setup at all levels. It is important to consider how ARPA-H will coordinate with other federal agencies, such as the U.S. Food and Drug Administration and federal health insurance agencies, so that new treatments options are covered. AHA will support ARPA-H's initiatives and hopes that ARPA-H will coordinate with existing research resources and will focus on the prevention of disease.

Ms. Leous said that ASH applauds the Biden administration's initiative to establish ARPA-H. ASH believes that ARPA-H needs to be truly unique and not duplicate existing efforts at NIH. ARPA-H should focus on large-scale innovative research focusing on areas of need, such as reducing health disparities, as well as projects that are not supported by current NIH funding mechanisms. ASH believes that funds available through ARPA-H must supplement and not supplant current research investments. Finally, ARPA-H needs to have a thoughtful strategy and mission. ASH looks forward to working with NIH to support ARPA-H in its mission to fund innovative, potentially high-impact research.

Dr. Hsu said that SCDAAs applauds the idea of ARPA-H as an incubator that will test new ideas. There are several challenges in sickle cell disease research, and addressing these challenges would benefit other fields. However, ARPA-H needs to ensure that there are no unintended consequences in amplifying health disparities, and patients need to have a seat at the table. Also, ARPA-H needs to focus on several aspects of biomedical research, including social context,

health disparities, quality of life, and access to care. The current U.S. health care system does not handle care for rare and chronic diseases efficiently, and access to care remains a problem. ARPA-H could help address these issues.

Dr. Kaiser said that the Endocrine Society welcomes new approaches to drive transformational change to improve health. Endocrine science is instrumental to NIH's mission, because conditions that affect the endocrine system influence numerous other systems. Advances in endocrine research will therefore benefit several other fields. Because it will focus on use-driven research with targeted near-term deliverables, as well as high-risk, high-reward projects, ARPA-H could drive collaborations across all fields of research. ARPA-H could support projects that require partnerships with industry and might lie outside the scope of current funding mechanisms. It is important to note that some features of DARPA grants create barriers that can limit the pool of researchers to a select few at well-funded institutions. ARPA-H should give researchers the freedom to study unanticipated research questions that arise organically. ARPA-H will offer an opportunity for all stakeholders to work with new partners, and the program's leadership should design ARPA-H to incorporate diverse perspectives to maximize the benefit to society.

Mr. Ewart said that ATS enthusiastically approves of ARPA-H. ATS understands the importance of current research funding mechanisms and hopes that ARPA-H complements and does not compete with existing funding avenues. Investigator-initiated grants are not appropriate for every scientific or public health problem, and major project-based investments are sometimes essential. ARPA-H can be effective in solving such problems. ATS anticipates that ARPA-H will be open to existing researchers and traditional research institutions but should also reach out to nontraditional partners to help accelerate the scientific process. Mr. Ewart asked whether ARPA-H funds will be available to all institutions, how ARPA-H project managers will be recruited, how its priorities will be determined, and how it will ensure that it is advancing the public good. ATS has several interesting projects that would benefit from ARPA-H funding, and the society will follow up in written comments.

Discussion

Dr. Collins led stakeholders and NIH directors to discuss the following questions:

- *How will ARPA-H's program managers balance the importance of stakeholder input with selecting projects based on their potential for rapid advancement and acceleration in a use-driven way? What are the consequences of selecting projects based on the potential for progress?* Mr. Ewart said that ARPA-H will put a lot of trust in its program managers, and he asked how the agency will recruit them. Public input might be critical in the selection process. Dr. Collins said that ARPA-H's success will rely heavily on its director and program managers. Selecting a director who is an entrepreneur at heart, has private-sector experience, and is a good communicator will be important. The program manager position will have a fixed initial term of 3 years, and a program manager's tenure might be extended to 5 years. Dr. Collins said that he anticipates that most program managers will come from outside NIH and therefore will not have strong allegiances to specific individuals or Institutes. Program managers will likely have private-sector experience and will be selected based on their ideas and entrepreneurial spirit. Ms. Leous said that program managers need to come from diverse backgrounds to help combat health

inequities, and ARPA-H will need to break down silos to improve collaborations among researchers and industry. Dr. Collins said that ARPA-H will aim to have diversity among program managers, because this will be critical to the agency's success. Dr. Knudsen said that the agency's success will depend on the efficiency of its program managers, and the opinions of stakeholders are critical, because stakeholders know where there are gaps in current research funding mechanisms. Dr. Collins said that ARPA-H will consider the background of program managers as well as their skill set.

- *Will ARPA-H fund research into long-term solutions if such projects have multiple short-term deliverables?* Dr. Collins said that every program will need to have a clear set of deliverables and milestones. A significant number of programs will be focused on health equity, and such programs will need to have the same clear indications of success.
- *How do you leverage the expertise of other government agencies without increasing bureaucracy?* Mr. Ewart said that it will be important to involve the Centers for Disease Control and Prevention, the U.S. Department of Veterans Affairs, and the Environmental Protection Agency. Ms. Leous said that conditions such as sickle cell disease cut across government agencies, and it is important to involve the relevant agencies, although bureaucracy might stall innovation.
- *Will ARPA-H fund programs that are likely to lead to progress mostly in a very specific but much-needed disease application?* Dr. Jessup said that programs to develop structural platforms for one condition, such as a public-private partnership to develop information technology infrastructure to establish a community of researchers and recruit participants into clinical trials, could lead to advances in other fields. Dr. Collins agreed, adding that programs to develop community health worker models to help address health disparities might be focused on public health but could also serve as platforms that would yield improvements in health care across multiple disciplines.
- *How will ARPA-H set barriers and determine what is and is not basic research?* Dr. Knudsen said that ARPA-H should keep an open mind about what types of programs could support truly transformational research. She suggested that the agency consider the potential impact of a program and provide transparency in its project selection processes. Dr. Collins said that ARPA-H will support use-driven projects. Dr. Hsu said that selecting programs based on broad impact across multiple disease types might perpetuate inequities for rare diseases. Ms. Leach agreed, adding that there are urgent unmet needs in rare disease research that could benefit from ARPA-H. Dr. Gibbons said that the criterion of wide impact could be met by an emphasis on platforms that could be established to address a very specific need but that might have multiple uses. ARPA-H should consider programs that have the ability to scale up to address other issues. Dr. Collins agreed and said that ARPA-H could support programs that may not be commercially profitable, especially if they could be more broadly applicable, and possibly drive interest in the commercial market.

Closing

Francis S. Collins, M.D., Ph.D., Director, NIH

Dr. Collins thanked participants and attendees for their interest in ARPA-H. ARPA-H is a work in progress, and OSTP and NIH will be hosting additional listening sessions to continue gathering information to help guide its establishment. Dr. Collins invited attendees to send comments and questions to the ARPA-H comment box (ARPAHcomments@nih.gov) and to visit the [ARPA-H webpage](#).