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Advanced Research Projects Agency for Health (ARPA-H): Potential Questions for Consideration

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SUMMARY

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Advanced Research Projects Agency for Health (ARPA-H): Potential Questions for Consideration

The National Institutes of Health (NIH), an agency in the Department of Health and Human Services (HHS), proposed the creation of an Advanced Research Projects Agency for Health (ARPA-H) as part of President Biden's FY2022 budget request. The budget request seeks $6.5 billion for ARPA-H over three years to "drive transformational health research innovation and speed medical breakthroughs by tackling ambitious challenges requiring large-scale, sustained, and cross-sector coordination." The initial focus of ARPA-H would include building platforms and capabilities to try to deliver cures for cancer, Alzheimer's disease, diabetes, and other diseases.

ARPA-H would follow the model of other "ARPAs," especially the Defense Advanced Research Projects Agency (DARPA) and the Advanced Research Projects Agency-Energy (ARPA-E), including an organizational structure designed to be flat and nimble, tenure-limited program managers with a high degree of autonomy to select and fund projects, and a milestone-based contract approach. In contrast, NIH relies predominantly on the scientific peer review process to award most of its funding. Some data suggests that this investigator-driven and consensus-based process is less likely to fund high-risk, high-reward projects. Supporters of the proposal argue that high-risk, high-reward research is critical to ensuring U.S. competitiveness and addressing societal challenges.

On July 29, 2021, the House passed the Consolidated Appropriations Act, 2022 (H.R. 4502), which would provide $3 billion over three years for ARPA-H. The funds would be available only if legislation specifically establishing ARPA-H is enacted into law. As of the date of this report, no such legislation has been introduced. In addition, the Senate has not yet introduced or considered appropriations legislation related to NIH. As Congress continues its deliberations on whether and how to enact and fund the ARPA-H proposal, it may consider a number of questions discussed in this report, including

- Should Congress establish ARPA-H?
- What might ARPA-H focus on?
- How would ARPA-H compare to other NIH programs and biomedical research efforts?
- How would ARPA-H compare to other "ARPAs"?
- Should ARPA-H be part of NIH?
- What legislative authorities may ARPA-H require or warrant?
- How might ARPA-H be evaluated?
- What would be the appropriate funding level for ARPA-H?

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Introduction

The federal government has long invested in biomedical science through the National Institutes of Health (NIH). This investment has been credited with contributing to advances in treating disease and providing medical care, increasing life expectancy, and preventing millions of deaths. For much of its history, NIH has focused largely on supporting basic research: research that explores the fundamental mechanisms of biology and behavior. Such research facilitates scientific knowledge that informs medical advances. Traditionally, the private sector, such as the biopharmaceutical industry, has largely taken on the role of supporting research and development (R&D) activities aimed at bringing new technologies and products to market, such as pharmaceutical drugs.[1]

In recent years, legislation such as the 21st Century Cures Act (P.L. 114-255) and the provisions establishing the National Center for Advancing Translational Sciences (NCATS)[2] have expanded NIH's role in biomedical innovation, that is, research efforts aimed at driving new paradigms and potentially breakthrough science and technologies.[3] The Biden Administration continues this trend by proposing a new Advanced Research Projects Agency for Health (ARPA-H) at NIH in its FY2022 budget request.

This report identifies policy questions relevant to congressional considerations of whether—and, if so, how—to establish a new ARPA-H entity. The report does not explicitly address alternative options to accomplishing the goals of the ARPA-H proposal, which might include expanding upon existing efforts instead of establishing a new ARPA-H or the consolidation of existing related programs into a new entity.

Overview of Proposed ARPA-H

While the current publicly available information on the proposal lacks specifics, the Biden Administration has laid out its vision for the proposed ARPA-H in NIH's FY2022 budget request, in addition to a concept paper and an article published in *Science* magazine by NIH Director Francis Collins, the director of the White House Office of Science and Technology Policy Eric Lander, and others.[4] According to the proposal, ARPA-H would be modeled after the Defense Advanced Research Projects Agency (DARPA), which is part of the Department of Defense

[1] For more information on NIH and the process of pharmaceutical drug development see, CRS Report R41705, *The National Institutes of Health (NIH): Background and Congressional Issues*; and CRS Infographic IG10013, *The Pharmaceutical Drug Development Process*.

[2] NCATS was established by the Consolidated Appropriations Act, 2012 (P.L. 112-74).

[3] The NIH defines *innovation* as "something new or improved, including research for (1) development of new technologies, (2) refinement of existing technologies, or (3) development of new applications for existing technologies." NIH peer review criteria also uses the following criteria to evaluate innovation in a research proposal: "Does the application challenge and seek to shift current research or clinical practice paradigms by utilizing novel theoretical concepts, approaches or methodologies, instrumentation, or interventions? Are the concepts, approaches or methodologies, instrumentation, or interventions novel to one field of research or novel in a broad sense? Is a refinement, improvement, or new application of theoretical concepts, approaches or methodologies, instrumentation, or interventions proposed?" See https://grants.nih.gov/grants/peer/critiques/rpg.htm.

[4] NIH, *Congressional Justification: FY2022*, May 28, 2021, pp. 10-11, https://officeofbudget.od.nih.gov/pdfs/FY22/br/2022%20CJ%20Overview%20Volume%20May%2028.pdf; White House, *Advanced Research Project Agency for Health (ARPA-H): Concept Paper*, https://www.whitehouse.gov/wp-content/uploads/2021/06/ARPA-H-Concept-Paper.pdf; NIH, "Lander, Collins Set Forth a Vision for ARPA-H," press release, June 22, 2021, https://www.nih.gov/news-events/news-releases/lander-collins-set-forth-vision-arpa-h; and Francis S. Collins et al., "ARPA-H: Accelerating Biomedical Breakthroughs," *Science*, vol. 373, no. 6551 (July 9, 2021).

(DOD), and would contain several *DARPA model* characteristics, including a flat organizational structure designed to be nimble, tenure-limited program managers with a high degree of autonomy to select and fund projects, and a milestone-based contract approach.[5] NIH, in contrast, generally funds most of its research through the scientific peer review process—a committee-based review process to evaluate scientific investigator-driven research proposals for funding.[6] Some data suggests that this investigator-driven and consensus-based process may not adequately fund "high-risk, high-reward" projects,[7] a term often associated with projects that have high potential for meeting fundamental scientific or technological challenges and that involve a high degree of novelty and/or multidisciplinary approaches that may not be favored by traditional scientific funding models.[8]

The proposal responds to concerns by some in the scientific and patient advocacy communities that traditional funding processes are too risk averse—supporting incremental advances over high-risk, high-reward or potentially transformative research.[9] Support for high-risk, high-reward research is considered an important element in developing breakthrough technologies that address societal challenges, including health-related challenges, and in maintaining the economic competitiveness of the United States.[10] In addition, the recent rapid development of safe and effective Coronavirus Disease 2019 (COVID-19) vaccines based on novel technologies such as mRNA, driven partly by DARPA investments, has spurred some to consider the usefulness and value of the *DARPA model* or other innovative approaches for biomedical research in general.[11]

The budget request includes $6.5 billion for ARPA-H "to make pivotal investments in breakthrough technologies and broadly applicable platforms, capabilities, resources, and solutions that have the potential to transform important areas of medicine and health for the benefit of all patients and that cannot readily be accomplished through traditional research or commercial activity."[12] According to the proposal, ARPA-H is to "build platforms and capabilities to deliver cures for cancer, Alzheimer's disease, diabetes, and other diseases."[13] Additionally, the Administration has provided a list of potential ARPA-H projects, including the development of accurate, wearable, blood pressure technology; the preparation of mRNA vaccines against common forms of cancer; drug or gene therapy delivery systems that can target any organ, tissue,

[5] For more information on DARPA, see CRS Report R45088, *Defense Advanced Research Projects Agency: Overview and Issues for Congress*.

[6] See "Peer Review Process for Extramural Funding" in CRS Report R41705, *The National Institutes of Health (NIH): Background and Congressional Issues*.

[7] Chiara Franzoni, Paula Stephan, and Reinhilde Veugelers, "Funding Risky Research," *National Bureau of Economic Research Working Paper*, June 2021; Mikko Packalen and Jay Bhattacharya, "NIH Funding and the Pursuit of Edge Science," *Proceedings of the National Academy of Sciences*, vol. 117, no. 22 (June 2, 2020), pp. 12011-12016; and Pierre Azoulay, Erica Fuchs, and Anna Goldstein, "Funding Breakthrough Research: Promises and Challenges of the 'ARPA Model,'" *National Bureau of Economic Research*, June 2018.

[8] For a discussion of definitions of "high-risk, high-reward research," see pages 11-13 of Organization for Economic Cooperation and Development (OECD), *Effective Policies to Foster High-Risk/High-Reward Research*, OECD Science, Technology, and Industry Policy Papers, No. 112, May 2021, https://read.oecd.org/10.1787/06913b3b-en?format=pdf.

[9] For example see, Suzanne Wright Foundation, "HARPA: Health Advanced Research Projects Agency," https://www.harpa.org/; and Bhaven N. Sampat and Robert Cook-Deegan, "An ARPA for Health Research?," *Milbank Quarterly*, https://www.milbank.org/quarterly/opinions/an-arpa-for-health-research/.

[10] OECD, *Effective Policies to Foster High-Risk/High-Reward Research*.

[11] CRS Insight IN11446, *DARPA's Pandemic-Related Programs*; and Franzoni, Stephan, and Veugelers, "Funding Risky Research."

[12] White House, *Advanced Research Project Agency for Health (ARPA-H): Concept Paper*.

[13] NIH, *Congressional Justification: FY2022*, pp. 10-11.

or cell type; and platforms to reduce health disparities in maternal morbidity and mortality, among others.[14]

Funding is requested for a period of three years to "allow for both scale-up in FY2022 and redeployment of resources in the next two years if projects fail to meet performance milestones." The vast majority of funding would support extramural research (i.e., research conducted outside the federal government), with a smaller amount of funding reserved for staffing and administrative functions. Unlike NIH Institutes and Centers (ICs), the proposed ARPA-H would not have its own intramural research program (i.e., research conducted at NIH facilities).[15]

The FY2022 budget request describes the types of challenges ARPA-H would seek to address through its investments, including

- Support for complex research and development that requires large-scale, sustained, cross-sector coordination;
- The creation of new capabilities (e.g., technologies, data resources, disease models);
- Support for high-risk exploration that could establish entirely new paradigms; and
- The commercialization of biomedical innovations using financial incentives and other mechanisms.[16]

Most ARPA-H awards would support industry, universities, and nonprofit research institutions and may involve some agreements with other federal agencies. While the proposed agency structure would be "operationally distinct" from NIH ICs, ARPA-H would still coordinate research and activities with NIH ICs and other Department of Health and Human Services (HHS) agencies (e.g., the Food and Drug Administration [FDA]).

Congressional Action

The Consolidated Appropriations Act, 2022 (H.R. 4502),[17] which passed the House on July 29, 2021, would provide $3 billion for ARPA-H available until September 30, 2024, with the condition that funds would be available only if legislation specifically establishing ARPA-H is enacted into law. As of the date of this report, CRS has not identified introduced legislation that would establish ARPA-H. Two representatives have circulated a discussion draft that would authorize an ARPA-H, among other provisions.[18]

[14] NIH, *Congressional Justification: FY2022*, pp. 10-11; and White House, *Advanced Research Project Agency for Health (ARPA-H): Concept Paper*.

[15] NIH, *Congressional Justification: FY2022*, pp. 10-11.

[16] NIH, *Congressional Justification: FY2022*, pp. 10-11.

[17] H.R. 4502 contains the text of seven regular appropriations bills reported by the House Appropriations Committee: H.R. 4502 (Labor-HHS-Education) (Div. A), H.R. 4356 (Agriculture) (Div. B), H.R. 4549 (Energy-Water) (Div. C), H.R. 4345 (Financial Services) (Div. D), H.R. 4372 (Interior) (Div. E), H.R. 4355 (Military Construction and Veterans Affairs) (Div. F), and H.R. 4550 (Transportation-HUD) (Div. G).

[18] Congresswoman Diana DeGette, "DeGette, Upton Unveil Bipartisan Legislation to Create Biden's New Advanced Research Agency to Cure Cancer," press release, June 22, 2021, https://degette.house.gov/media-center/press-releases/CURES2.

The Senate has not yet introduced its FY2022 Departments of Labor, Health and Human Services, and Education, and Related Agencies (LHHS) Appropriations legislation (i.e., the annual appropriations bill that provides most of NIH's funding).

Potential Questions for Consideration

The following sections address key policy questions that Congress might consider when determining whether and how to enact and fund the ARPA-H proposal.

Should Congress Establish ARPA-H?

According to the Biden Administration, "through bold, ambitious ideas and approaches, ARPA-H can help shape the future of health and medicine in the U.S. by transforming the seemingly impossible into reality."[19] While the establishment of ARPA-H is one option for supporting high-risk, high-reward research, there are other research funding mechanisms that may be used to foster high-risk, high-reward research. For example, according to a study by the Organization for Economic Cooperation and Development (OECD), high-risk, high-reward research can be supported through

- Funding mechanisms specifically designed to support high-risk, high-reward research as a primary goal (e.g., DARPA; ARPA-E; NIH's High-Risk, High-Reward Research Program);
- Funding mechanisms that have high-risk, high-reward research as their primary mission within a broader set of objectives (e.g., National Science Foundation's (NSF's) Research Advanced by Interdisciplinary Science and Engineering program);
- Funding mechanisms in which supporting high-risk, high-reward research is a secondary goal or an important consideration in the proposal evaluation process (e.g., NSF's Early Grants for Exploratory Research program); and
- Funding mechanisms geared toward supporting scientific research with multiple possible goals including advancing scientific knowledge, achieving economic outcomes, or advancing societal outcomes, although there are no clear criteria for fostering high-risk, high-reward research.[20]

Additionally, the OECD identified people-based awards, scientific prize competitions, and internal or institution funding as mechanisms for fostering high-risk, high-reward research.[21] Congress might consider the use or expansion of other funding mechanisms to support high-risk, high-reward biomedical research in lieu of establishing ARPA-H.

What Might ARPA-H Focus On?

According to the NIH FY2022 budget request, ARPA-H would "build platforms and capabilities to deliver cures for cancer, Alzheimer's disease, diabetes, and other diseases … collapse barriers and speed the development, application, and implementation of urgently needed health

[19] White House, *Advanced Research Project Agency for Health (ARPA-H): Concept Paper*, p. 7.

[20] OECD, *Effective Policies to Foster High-Risk/High-Reward Research*.

[21] OECD, *Effective Policies to Foster High-Risk/High-Reward Research*.

breakthroughs."[22] Additionally, the Administration provided an illustrative list of potential ARPA-H projects, including the development of mRNA vaccines that target common forms of cancer, inexpensive and accurate wearable sensors to monitor blood sugar, and platforms to reduce maternal morbidity and mortality disparities, among others.[23]

For comparison, the Advanced Research Projects Agency-Energy (ARPA-E) within the Department of Energy addresses its mandate to advance high-risk, high-reward energy technologies by seeking to fill what it calls the *white space*, a perceived gap or opportunity in the energy technology landscape. Specifically, ARPA-E provides funding for "technological approaches that are truly novel or greatly underexplored … [or] to fill gaps left in other research or funding programs."[24] As proposed, ARPA-H may focus on the white space in the biomedical research landscape. This role for an ARPA-H is consistent with the view among some scholars that the *DARPA model* is best suited to fund projects that occupy a productive middle ground between basic and applied research and focus on unexplored potential breakthrough technologies.[25]

Identifying the white space would be the responsibility of ARPA-H program managers who, similar to DARPA and ARPA-E program managers, would have more autonomy in the decisionmaking process than is typical of other federal agencies that fund R&D. ARPA-H's potential mandate is broad—perhaps purposefully so—to provide the proposed agency with maximum flexibility in the projects and problems it may seek to address.

Some stakeholders have expressed concern about the potentially broad scope of ARPA-H.[26] Given the flexibility and open-endedness provided by the "ARPA model," Congress and the public might benefit from additional public information about processes and principles ARPA-H would use to create programs and to select and fund projects.

According to the Biden Administration, "to determine which risks should be taken and to evaluate proposed programs and projects, ARPA-H should adopt an approach similar to DARPA's 'Heilmeier Catechism,' a set of principles that assesses the challenge, approach, relevance, risk, duration, and metrics of success."[27]

Congress might consider requiring ARPA-H to make the principles used to select and fund projects publicly available. Additionally, Congress might consider requiring ARPA-H to make its strategic priorities public and solicit stakeholder input on the development of such priorities. ARPA-H's priorities could potentially fluctuate over both the short and long term; however, they could serve as an important signal to researchers and the private sector.

[22] NIH, *Overview of FY2022 President's Budget*, May 2021, p. 10, https://officeofbudget.od.nih.gov/pdfs/FY22/br/2022%20CJ%20Overview%20Volume%20May%2028.pdf.

[23] White House, *Advanced Research Project Agency for Health (ARPA-H): Concept Paper*, p. 5.

[24] National Academies of Sciences, Engineering, and Medicine, *An Assessment of ARPA-E* (Washington, DC: The National Academies Press, 2017), p. 95.

[25] Azoulay, Fuchs, and Goldstein, "Funding Breakthrough Research."

[26] Jeff Tollefson, "The Rise of 'ARPA-Everything' and What It Means for Science," *Nature*, July 8, 2021, https://www.nature.com/articles/d41586-021-01878-z.

[27] White House, *Advanced Research Project Agency for Health (ARPA-H): Concept Paper*, p. 7.

How Would ARPA-H Compare to Other NIH Programs and Biomedical Research Efforts? What Gap Could It Fill?

The Biden Administration argues that the current ecosystem of biomedical R&D—with curiosity-driven research funded by NIH[28] and the public sector and with for-profit and commercialization-driven R&D funded largely by industry—is adequate for most biomedical innovation but leaves certain critical gaps that ARPA-H could fill.[29] Specifically, project ideas that the Administration asserts are left unfunded by the current system include those that (1) are either high risk and/or require significant funding, (2) involve complex coordination among multiple parties, (3) have a focus that is too applied for academia, and (4) have a scope that "is so broad that no company can realize the full economic benefit."[30] The *Science* article asserts that ARPA-H would pursue "use-driven" ideas in comparison to NIH's typical "curiosity-driven" approach.[31]

As mentioned above, a key consideration for establishing ARPA-H is how to ensure that the new agency would concentrate its efforts on identifying "white space" in the biomedical research landscape. While no entity within NIH, or in the U.S. biomedical research enterprise at large, follows an exact *DARPA model*, NIH does have some existing programs aimed at fostering biomedical innovation. These include the Common Fund for cross-cutting and milestone-driven innovative projects; the National Center for Advancing Translational Sciences (NCATS), which focuses on innovation in medical product development; and several NIH-wide programs, such as the Accelerating Medicines Partnership, a public-private partnership aimed at transforming the processes for developing new diagnostics and treatments.[32] Certain stakeholders have argued that some existing efforts—particularly NCATS—have not been funded well enough to achieve their intended goals.[33] This suggests that ARPA-H may not be necessary if existing efforts saw increased funding. However, the Biden Administration asserts that ARPA-H would be distinct from these existing programs, although it acknowledges some potential overlap (especially with a certain NCATS program) and the need for coordination to avoid duplication.[34]

Congress may consider requiring an independent entity such as NIH's Scientific Management Review Board (SMRB),[35] the Government Accountability Office (GAO), or the National Academy of Medicine to conduct a review of all NIH programs to assess the degree of potential

[28] NIH issues three types of funding opportunity announcements (FOAs): parent announcements, which are broad FOAs allowing applicants to submit an investigator-initiated application for a specific activity code; program announcements, which are FOAs issued by one or more ICs to highlight areas of scientific interest; and Requests for Applications, which are FOAs issued by one or more ICs to highlight well-defined areas of scientific interest to accomplish specific program objectives. For more information see, NIH, "Understanding Funding Opportunities," https://grants.nih.gov/grants/how-to-apply-application-guide/prepare-to-apply-and-register/understand-funding-opportunities.htm.

[29] Collins et al., "ARPA-H: Accelerating Biomedical Breakthroughs."

[30] Collins et al., "ARPA-H: Accelerating Biomedical Breakthroughs."

[31] Collins et al., "ARPA-H: Accelerating Biomedical Breakthroughs."

[32] NIH, "About the Common Fund," June 17, 2021, https://commonfund.nih.gov/about; NCATS, "About NCATS," https://ncats.nih.gov/about; and NIH, "Accelerating Medicines Partnership," https://www.nih.gov/research-training/accelerating-medicines-partnership-amp.

[33] Jocelyn Kaiser, "Seven Years Later, NIH Center That Aims to Speed Drugs to Market Faces Challenges," *Science*, September 25, 2019.

[34] White House, *Advanced Research Project Agency for Health (ARPA-H): Concept Paper*, p. 6.

[35] The SMRB is a federal advisory committee created by the NIH Reform Act of 2006 (P.L. 109-482) that advises NIH on its organizational structure, including its use of authorities to reorganize NIH institutes, centers, and offices. See SMRB, "Charter," https://smrb.od.nih.gov/charter.html.

overlap with the proposed ARPA-H and the need for consolidation or termination of existing programs. Such a review could also identify lessons learned or best practices from NCATS or the Common Fund that could serve as a guide for ARPA-H.

Further, philanthropic funders play a significant role in U.S. biomedical R&D.[36] Some of these entities are engaged in activities that would overlap with the proposed activities of ARPA-H. Examples include the nonprofit Chan Zuckerberg Initiative, which focuses on funding biomedical science efforts that address unmet health needs and barriers to success, including support for translation of new technologies,[37] and the Bill and Melinda Gates Foundation, which funds vaccine platform technologies.[38] These are both directed at similar types of projects that the proposed ARPA-H may support. Congress and the Administration might consider both how to differentiate ARPA-H from existing NIH programs and private sector initiatives as well as how ARPA-H could interact with these programs and initiatives. DARPA, for instance, plays a role as a convener among relevant players in industry, academia, and the government.

How Would ARPA-H Compare to Other "ARPAs"?

According to the Biden Administration:

> Although DARPA is an excellent inspiration for ARPA-H, it is not a perfect model for biomedical and health research. It serves the needs of a single customer, the DOD, and its mission is focused on national security. Its projects typically involve engineered systems. By contrast, health breakthroughs (i) interact with biological systems that are much more complex and more poorly understood than engineered systems, requiring close coupling to a vast body of biomedical knowledge and experience; (ii) interact with a complex world of many customers and users—including patients, hospitals, physicians, biopharma companies and payers; (iii) interact in complex ways with human behavior and social factors; and (iv) require navigating a complex regulatory landscape. ARPA-H can learn from DARPA, but will need to pioneer new approaches.[39]

As noted earlier, the Biden Administration mentions a range of technologies and projects that the new agency could support—such as new research tools and technologies (e.g., data platforms), new drugs or treatments (e.g., mRNA vaccines for cancer), and innovative health care programs and practices (e.g., virtual midwife programs to reduce maternal morbidity and mortality). While ARPA-H may provide the initial support for such technologies and platforms, they would likely be made available for research, commercial, or clinical use through different mechanisms. Some of the proposed ARPA-H technologies, such as research technologies, may be made available through open-source platforms or licensing. Others that contribute to the development of new medical products may transition to industry for further financing and development. Innovative health care programs and practices may transition to another federal health agency or private organization or be adopted as common practice throughout the health care industry or clinical practice.

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[36] Research!America, *U.S. Investments in Medical and Health Research and Development: 2013-2018*, 2019, https://www.researchamerica.org/sites/default/files/Publications/InvestmentReport2019_Fnl.pdf.

[37] NIH also supports translational research. For example, see the Edward R. Roybal Centers for Translational Research in the Behavioral and Social Sciences of Aging at https://www.nia.nih.gov/research/dbsr/edward-r-roybal-centers-translational-research-behavioral-and-social-sciences-aging.

[38] Chan Zuckerberg Initiative, "Science Programs," https://chanzuckerberg.com/science/programs-resources/ and Bill and Melinda Gates Foundation, "Vaccine Development and Surveillance," https://www.gatesfoundation.org/our-work/programs/global-health/vaccine-development-and-surveillance.

[39] White House, *Advanced Research Project Agency for Health (ARPA-H): Concept Paper*, p. 3.

According to a Biden Administration official, ARPA-H could provide incentives for industry adoption of ARPA-H-funded innovations, or it could partner with other federal agencies to lower regulatory hurdles in getting such innovations to the marketplace.[40] Some federal agencies (e.g., the Department of Veterans Affairs) could become customers for ARPA-H-funded innovations.

Given the complexity of the health care industry as well as unique regulatory considerations, Congress might consider if it were to establish an ARPA-H whether to explicitly authorize a program within ARPA-H that would facilitate commercialization and implementation. ARPA-E, for example, has a Technology-to-Market Program specifically focused on this concern that offers services and support to ARPA-E awardees in the development and execution of a commercialization plan. ARPA-H could establish a similar program to aid in the transition of technologies and programs it supports to industry or into practice, perhaps in coordination with the FDA and the Centers for Medicare and Medicaid Services. Congress might also consider whether to require ARPA-H to incorporate considerations of equitable access to, and the affordability of, new medical products into its funding criteria or commercialization programs and how to balance these criteria with ensuring private sector investment in and uptake of ARPA-H supported innovations. In addition, Congress might consider how ARPA-H may interact or engage with other federal agencies in the pursuit of health breakthroughs and the development of programs, policies, and activities that go beyond R&D funding (i.e., early adoption of innovative products and services).

Should ARPA-H Be Part of NIH?

The Biden Administration has argued that "the goals of ARPA-H fall squarely within NIH's mission" and that placing ARPA-H within NIH will promote scientific collaboration and help avoid duplication across programs.[41] The placement of ARPA-H—whether to house the new entity within NIH or as a separate agency under HHS (NIH's parent department)—is a key debate among stakeholders and Members of Congress. The debate stems, in part, from a perception among some stakeholders that NIH's culture is relatively conventional and risk-averse. Some question if NIH's leadership and culture could affect ARPA-H's ability to succeed in research for transformational innovation.[42] Such stakeholders support placing ARPA-H outside of NIH to ensure independence and autonomy. Additionally, there is concern among other stakeholders that ARPA-H could crowd out funding for other NIH ICs in future NIH budget and appropriations considerations.[43]

There is past precedent for innovative biomedical science efforts both within NIH and at the HHS Secretarial level. At NIH, in addition to Common Fund and NCATS, NIH has supported projects such as the Human Genome Project and, more recently, the Rapid Acceleration of Diagnostics program to boost innovation for COVID-19 diagnostics.[44] At HHS, the Office of the Chief Technology Officer has managed several public-private partnerships aimed at accelerating

[40] Beth Wang, "ARPA-H Would Work with Industry, FDA, CMS to Bring Products to Market," *Inside Health Policy*, July 26, 2021, https://insidehealthpolicy.com/daily-news/arpa-h-would-work-industry-fda-cms-bring-products-market.

[41] Collins et al., "ARPA-H: Accelerating Biomedical Breakthroughs."

[42] Sarah Omermohle, "Skeptics Question If Biden's New Science Agency Is a Breakthrough or More Bureaucracy," *Politico*, July 5, 2021; Jacqueline Alemany, "Biden Has Proposed a New Agency to Turbocharge Medical Treatments. But There's a Fight over Where It Should Live," *Washington Post*, June 23, 2021.

[43] See, for example, American Society for Biochemistry and Molecular Biology (ASBMB), "ASBMB Responds to Proposal for New Health Research Agency," *ASBMB Today*, July 27, 2021.

[44] ASBMB, "ASBMB Responds to Proposal for New Health Research Agency."

innovation, including for kidney disease and Lyme disease.[45] Additionally, the Biomedical Advanced Research and Development Authority (BARDA) under the Assistant Secretary for Preparedness and Response engages in efforts to drive innovation for medical countermeasures to address public health emergencies.[46]

If ARPA-H were to be established, its ability to succeed could depend more on its statutory and programmatic design and its leadership than its placement within the federal government. Congress could develop ARPA-H legislation in a way that gives ARPA-H leadership independence to act and formulate strategies, regardless of where the new agency is housed. No matter where the new agency could be placed, it would likely need to consult with programs both within and outside of NIH to promote collaboration and avoid duplication—including DOD, BARDA, and FDA, among others. Some have argued that ARPA-H's founding director would play a crucial role in developing a unique culture that guides the agency to success; the report accompanying the House FY2022 LHHS appropriations bill (H.Rept. 117-96) "strongly encourages NIH to recruit an ARPA-H Director with extraordinary technical and leadership skills, who has a proven track-record in innovation and partnership-building."[47] Some have also suggested facilitating independence by housing ARPA-H in a different location than NIH's main campus in Bethesda, MD; NIH Director Francis Collins has publicly stated that this possibility is "on the table."[48]

What Legislative Authorities May ARPA-H Require or Warrant?

As noted earlier, the Consolidated Appropriations Act, 2022 (H.R. 4502), passed by the House on July 29, 2021, would provide funding for ARPA-H only if legislation specifically authorizing the establishment of ARPA-H were enacted into law. Such legislation may include flexibilities in hiring personnel and in the mechanisms used to acquire goods and services (e.g., contracts for R&D) that many view as key aspects of the *DARPA model*. Specifically, the Biden Administration is requesting the authority to directly hire scientific and technical experts from outside the federal government (i.e., academia, industry, and think tanks) for limited-term appointments (three to five years). It is requesting that such hiring authority exempt the agency from complying with traditional civilian personnel requirements, thereby allowing it to streamline its hiring process and increase the level of compensation it could offer.[49] The ability of ARPA-H to recruit and retain scientific and technical experts could be critical to the success of ARPA-H given the prominent role of program managers in determining the direction and success of the proposed agency. NIH already has some special hiring authorities, including an amended hiring authority (Public Health Service Act Section 228)[50] in the 21st Century Cures Act (P.L. 114-255) that expanded HHS's

[45] HHS Office of the Chief Technology Officer, "Innovation and Partnerships," May 15, 2020.

[46] HHS Office of the Assistant Secretary for Preparedness and Response, "Biomedical Advanced Research and Development Authority," https://phe.gov/about/barda/Pages/default.aspx.

[47] Omermohle, "Skeptics Question If Biden's New Science Agency Is a Breakthrough or More Bureaucracy;" Sampat and Cook-Deegan, "An ARPA for Health Research?;" and U.S. Congress, House Committee on Appropriations, Subcommittee on Labor, Health and Human Services, Education, and Related Agencies, *Report to Accompany* H.R. 4502, 117th Cong., 1st sess., July 19, 2021, pp. 165-166.

[48] An earlier version of the House FY2022 LHHS Appropriations report also recommended that "ARPA-H be housed outside of NIH's main campus in Bethesda, MD," but this language was removed in the final report version. See Lev Facher, "NIH's Francis Collins: For Biden's New Research Agency to Succeed, It Should Prepare for Some Projects to Fail," *STAT News*, July 15, 2021.

[49] White House, "Advanced Research Projects Agency for Health (ARPA-H) Fact Sheet," https://www.whitehouse.gov/wp-content/uploads/2021/06/ARPA-H-Fact-Sheet.pdf.

[50] 42 U.S.C. §237.

ability to hire qualified scientific and technical experts in the biomedical sciences. According to GAO, as of May 2020, HHS had not yet used these authorities.[51] Despite the hiring authorities provided by P.L. 114-255, ARPA-H may require additional hiring flexibilities for its program managers.

The Biden Administration is also requesting *other transactions authority* (OT authority) for ARPA-H. An OT authority is an acquisition mechanism that does not fit into any of the traditional mechanisms used by the federal government for acquiring goods or services—contracts, grants, or cooperative agreements. OT authority is generally viewed as giving federal agencies additional flexibility to develop agreements tailored to the needs of the project and its participants, who do not have to comply with the government's procurement regulations. Only those agencies that have been provided OT authority by Congress may engage in other transactions. Generally, the reason for creating OT authority is that the government needs to obtain leading-edge R&D or prototypes from commercial sources that are unwilling or unable to navigate the government's procurement regulations. Some analysts raise concerns over potential risks associated with the use of OT agreements (OTAs), including diminished oversight and exemption from laws and regulations designed to protect government and taxpayer interests.[52] Congress has provided NIH currently with several OT authorities, though many are specific to individual ICs.[53]

OT authority may prove controversial in the context of medical products; the intellectual property rights to inventions under an OTA are usually negotiable.[54] A common justification for using OTAs is that some companies may seek greater intellectual property protections than are available under traditional federal funding mechanisms when deciding to work with ARPA-H.[55] In recent years, there has been increased interest in ensuring that medical products discovered with federal support are affordable, including by having the federal government exercise some of its rights to inventions developed with federal support.[56] With these rights potentially waived in OTAs, there may be some concern about the government's ability to ensure that products developed with ARPA-H support are available and affordable commercially.[57]

Additionally, the Biden Administration is requesting that ARPA-H

- receive multiyear budget authority (i.e., funding that is available for obligation for a fixed period in excess of one fiscal year);
- be exempt from using the traditional peer review process required by NIH ICs; and

[51] GAO, *Biomedical Research: HHS Has Not Yet Used New Authorities to Improve Recruit and Retention of Scientists*, May 8, 2020, https://www.gao.gov/assets/710/707051.pdf. For NIH's hiring authorities, see NIH Office of Human Resources, "Pay at NIH," https://hr.nih.gov/benefits/pay/pay-nih.

[52] Scott Amey, "Other Transactions: Do the Rewards Outweigh the Risks?," Project on Government Oversight, March 15, 2019, https://www.pogo.org/report/2019/03/other-transactions-do-the-rewards-outweigh-the-risks/.

[53] For more information on other transactions, see GAO, *Federal Acquisitions: Use of 'Other Transaction' Agreements Limited and Mostly for Research and Development Activities*, GAO-16-209, January 7, 2016.

[54] For more information see, CRS Legal Sidebar LSB10422, *COVID-19 Medical Countermeasures: Intellectual Property and Affordability*.

[55] GAO, *Federal Acquisitions*.

[56] See National Academies of Science, Medicine, and Engineering, "The Role of NIH in Drug Development Innovation and Its Impact on Patient Access: Proceedings of a Workshop," 2019, http://nationalacademies.org/hmd/Reports/2019/role-of-NIH-in-drug-development-innovation-and-its-impact-on-patient-access-proceedings.aspx; and Alfred B. Engelberg and Aaron S. Kesselheim, "Use the Bayh-Dole Act to Lower Drug Prices for Government Healthcare Programs," *Nature Medicine*, vol. 22, no. 576 (June 7, 2016).

[57] Sampat and Cook-Deegan, "An ARPA for Health Research?"

- be explicitly granted the authority to conduct prize competitions.[58]

How Might ARPA-H Be Evaluated?

It is unclear how the Administration would measure the success of ARPA-H. Measuring the impact of R&D investments can be difficult. For example, there is generally a long lag time between R&D activities and the availability of a commercial product or service. It can also be difficult to quantify *spillover effects* (i.e., R&D knowledge applied to an area that differs from the original intent) and to understand the degree to which a specific R&D project contributed to the development of a product or innovation. The identification and measurement of noneconomic or societal impacts such as health outcomes can be challenging. Moreover, given the focus on high-risk projects, a portion of ARPA-H projects would be expected to fail. Despite such challenges, evaluation and impact assessment support decisionmaking by providing insight into the efficiency, effectiveness, and relevance of policies and programs.[59]

Congress might consider requiring an independent evaluation of ARPA-H, as it has for ARPA-E. In the America Creating Opportunities to Meaningfully Promote Excellence in Technology, Education, and Science Act of 2007 (P.L. 110-69), Congress required the Secretary of Energy to enter into an agreement with the National Academies of Sciences, Engineering, and Medicine (NASEM) to conduct an evaluation of how well ARPA-E was achieving its goals and statutory mission. In 2017, NASEM released a study that provided both an operational assessment and a technical evaluation of ARPA-E. Specifically, the operational assessment appraised the appropriateness and effectiveness of ARPA-E's structure in positioning it to achieve its mission and goals, while the technical evaluation described the most significant accomplishments or impacts of ARPA-E as of 2017, and considered how well ARPA-E's activities supported the agency's goals (i.e., whether ARPA-E programs/awards were truly focused on transformational energy technologies).[60]

An initial assessment of ARPA-H could focus on the operational aspects of the proposed agency, in addition to determining if APRA-H is actually filling a gap in biomedical innovation (i.e., supporting R&D that would not be funded or supported by other mechanisms). The evaluation could also focus on shorter-term performance measures such as publications, patenting, or private investments in technologies originally supported by ARPA-H. A subsequent evaluation or evaluations could focus more on the economic and societal impacts of ARPA-H funding. To facilitate such evaluation, Congress might consider requiring ARPA-H to develop a framework for the collection of data and other information that would allow for robust assessment. Congress could also require periodic or annual reports from ARPA-H that describe the projects being supported and how such activities are advancing biomedical R&D and the mission of the proposed agency to create breakthrough health technologies.

What Would Be the Appropriate Funding Level for ARPA-H?

The Biden Administration proposed an initial funding level of $6.5 billion for the new agency.[61] However, the House has passed legislation (H.R. 4502) that would fund ARPA-H at $3 billion.

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[58] For more information on prize competitions, see CRS Report R45271, *Federal Prize Competitions*.

[59] OECD, "Evaluation and Impact Assessment of STI Policies," in *OECD Science, Technology and Innovation Outlook 2016* (Paris: OECD Publishing, 2016), p. 1, https://doi.org/10.1787/sti_in_outlook-2016-13-en.

[60] NASEM, *An Assessment of ARPA-E*, pp. 16-17.

[61] NIH, *Congressional Justification: FY2022*, pp. 10-11.

Both proposals would provide funding for multiple years. For reference, DARPA has an annual budget of $3.5 billion, ARPA-E has a FY2021 funding level of $425 million, and fewer than half of NIH ICs have an annual budget that exceeds $1 billion (11 out of 25 accounts).[62] Biomedical research—especially medical product R&D activities—tends to be expensive relative to some other areas of technology R&D, which may explain a relatively higher funding level for ARPA-H.[63] Given that ARPA-H is an untested new agency, some argue that it should start small and grow over time depending on its success.[64] If future budget resolutions were to impose more stringent discretionary spending limits, Congress could face difficult future choices in allocating limited funds among ARPA-H, NIH IC accounts, and other discretionary health programs.[65] Starting with higher baseline funding for ARPA-H could make such choices even more challenging.

Congress might also consider whether and how to leverage private sector funding—such as from industry or philanthropy—to help support ARPA-H's efforts. Currently, NIH structures many of its medical product development and biomedical innovation programs as public-private partnerships facilitated by the Foundation for the NIH,[66] which could play a similar role in financing ARPA-H programs. Congress might also seek to support other innovative models for financing biomedical innovation, either as an alternative to or in conjunction with the ARPA-H proposal. For example, the Long-term Opportunities for Advancing New Studies for Biomedical Research Act (H.R. 3437) would create a federally backed loan program to support research and clinical trials for therapies that would address unmet medical needs.

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[62] See CRS Report R46869, *Federal Research and Development (R&D) Funding: FY2022*; and CRS Report R45088, *Defense Advanced Research Projects Agency: Overview and Issues for Congress*.

[63] GAO, *Drug Industry: Profits, Research and Development Spending, and Merger and Acquisition Deals*, GAO-18-40, November 2017, pp. 28-37.

[64] Tollefson, "The Rise of 'ARPA-Everything' and What It Means for Science."

[65] For an overview of the annual budget resolution process, see CRS In Focus IF10647, *The Budget Resolution and the Budget Control Act's Discretionary Spending Limits*.

[66] See "Public-Private Partnerships" in CRS Report R41705, *The National Institutes of Health (NIH): Background and Congressional Issues*; and CRS Report R46109, *Agency-Related Nonprofit Research Foundations and Corporations*.

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