

Developed & supported by:

Rare Disease Community Priorities for the Advanced Research Projects Agency for Health (ARPA-H)



Former NCATS
Director
Dr. Chris Austin

As representatives of the rare disease community, our goal is to ensure that as ARPA-H is formed, funded, structured, and implemented, it is done in a way that prioritizes and reflects the unique circumstances and needs of rare disease patients and families.

More than 7,000 known rare diseases impact approximately 1 in 10 Americans; the lack of FDA-approved treatments for these diseases represents a serious public health challenge. The staggering emotional and financial burdens and costs of rare diseases to families and the health system require investment and nimble, collaborative innovation.

We believe many of the priorities of the rare disease community are reflected in the vision of the new entity, including:

- greater government investments;
- use of DARPA-like authorities to recruit talent and structure funding; and
- support for high-risk, high-reward programs that represent the kind of projects traditional government grantmaking mechanisms tend not to support.

To further strengthen the mission of ARPA-H and meet the needs of the rare disease community, we recommend that any authorizing legislation reflect the following:

1. Include the Administration's requests for ARPA-E/ARPA-I/DARPA-like authorities.
2. Establish a rare disease-specific or patient advisory board with diverse rare disease patient representation.
3. Include language encouraging a stronger tie between ARPA-H and FDA.
 - FDA will play a critical role in influencing what data should be collected to meet the agency's standards, and to prepare the agency to more effectively review new and emerging technologies that it may not be currently equipped to handle. Language to "collaborate" or "consult" with FDA, while a good start, is not sufficient.
 - Consider including mechanisms for a formal conduit, such as using detailees, appointees, and including the FDA Commissioner as part of the ARPA-H leadership team.
4. Require transparency in priorities and projects.
 - Public reporting should be a key component of ARPA-H. A report should include the total funding, the amount spent on overhead vs. research, a listing of government and private-sector partners, priority projects, and how each of the projects can provide direct and indirect benefits to patients.
 - Create a mechanism for public comment to the report.
5. Provide clarity around ownership of intellectual property.

We look forward to working with President Biden, Congressional leaders, and the stakeholder community to establish ARPA-H and ensure its successful implementation.

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