

Advanced Research Projects Agency for Health (ARPA-H): A New Paradigm for Biomedical and Health Research

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Current biomedical ecosystem driven by two powerful forces

Fundamental Biomedical Research

- Performed in university, nonprofit, government labs
- Funded mostly by federal government
- Pursues important fundamental questions
- Breathtaking progress in discovering molecular and cellular mechanisms underlying health and disease
- Public good – produces knowledge available to all
- \$1 federal investment yields \$8 in economic growth
- Every new FDA approved therapeutic can be traced (in part) to NIH-supported discoveries

Vibrant Commercial Sector

- Focused largely on research, development, and marketing of specific products to bring highly sophisticated therapies and devices to patients
- Access to significant capital to develop products, provided they can generate sufficient profit
- Currently, >8000 medicines in development, including 1300 for cancer

The Opportunity/Challenge

- We are at a moment of unprecedented scientific promise...
- E.g., cancer immunotherapies; highly effective COVID-19 vaccines developed and approved in ~11 months
- Challenges us to ask:
 - What can we do to fully realize the promise by dramatically accelerating the pace of breakthroughs in medicine and health?
 - to transform how we prevent or cure cancer, infectious diseases, Alzheimer's disease, and other diseases?
 - to transform healthcare access, equity, quality, and reduce health disparities?

Critical Gap

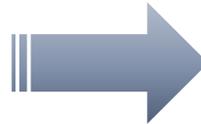
Some of the best ideas do not fit well into either half of our current system, because:

- Risk is too high
- Cost is too large
- Timeframe is too long
- Focus is too applied for academia
- Need for complex coordination among multiple parties
- Near-term market opportunity is too small to justify investment
- Goal is too broad for one company to do and fund alone

President Biden's Joint Address to Congress

April 28, 2021

*"We will see more technological change in the next 10 years – than we saw in the last 50 years. And we're falling behind in that competition. ... The National Institutes of Health, the NIH – should create an ... **Advanced Research Projects Agency for health**. To develop breakthroughs – to prevent, detect, and treat diseases like Alzheimer's, diabetes and cancer. ... I can think of no more worthy investment. And I know of nothing more bipartisan."*



FY22 budget request: \$6.5B available over three years

Mission

To make **pivotal investments in breakthrough technologies** and broadly applicable platforms, capabilities, and resources that have the potential to **transform important areas of medicine and health** and that cannot readily be accomplished through traditional research or commercial activity.

Goals

- Revolutionize prevention, treatment, and cures in a range of diseases
- Convert use-driven ideas into tangible solutions for patients far more rapidly than previously believed possible
- Make high-risk investments in broadly applicable platforms, capabilities, resources
- Foster breakthroughs across various levels – from the molecular to the societal – and drive them to the point of adoption to serve patients
- Overcome market failures through critical solutions or incentives

Many areas are ripe for transformation with right support, collaboration

Examples of Projects ARPA-H Might Undertake



Develop mRNA vaccines to prevent most cancers



Create molecular “zip codes” that target drugs only to specific tissues and cell types, to eliminate serious side effects



Holistic interventions to eliminate racial disparities in maternal morbidity/mortality rates and premature births



Highly accurate, inexpensive, non-intrusive, wearable monitors for blood pressure and blood sugar that provide real-time data to patients and providers

Culture



Seek bold
ideas and
people



Be nimble



Be open
and
transparent



Fail fast



Own it!

Structure

- Coupled to NIH to draw on vast knowledge, expertise and activities, but . . .
- Distinct entity within NIH, with unique culture, organization and independence

DARPA-like Culture, Review, Organization

- DARPA-like culture of time-limited projects with goals, benchmarks, and accountability
- Distinct project review and selection processes
- Work with performers in academia, industry, government (including ICs)
- Director with technical and leadership skills, ensuring risk tolerance, urgency, and innovation
- Creative individuals recruited as program managers for short terms, with broad autonomy to drive transformational change

Equity

- Equity should be considered in all aspects of ARPA-H mission, including:
 - Some projects squarely focused on addressing health equity
 - All projects considering impact on equity
 - Equity in staffing

Conclusions

- Many areas ripe for transformation with right support and collaboration
- The potential opportunity is extraordinary:
 - ARPA-H can help shape the future of health and medicine in the U.S. by transforming the (currently) impossible into reality.
 - Goal is to directly improve the health of Americans faster than was ever imagined possible.