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
“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.