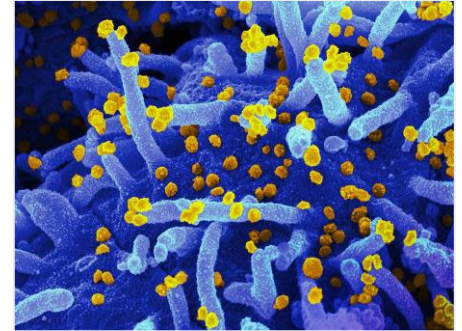
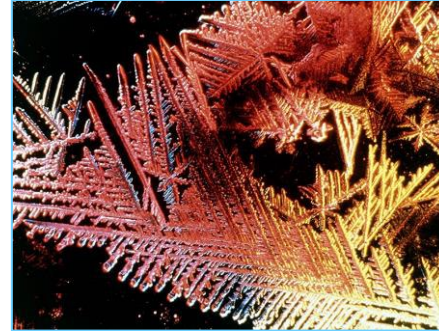
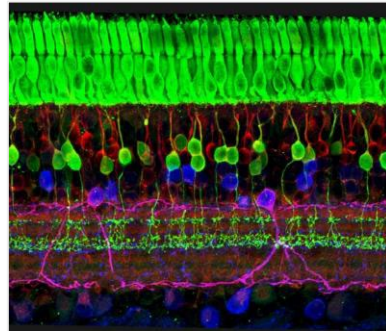
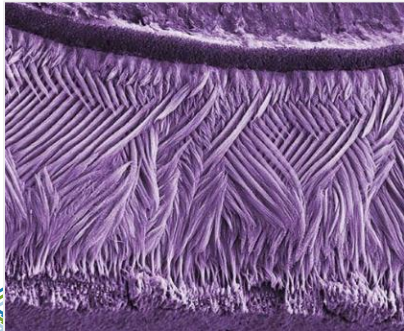
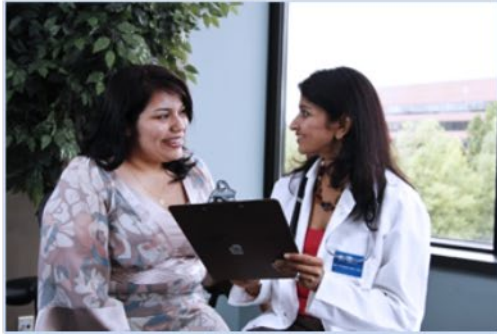




NIH...

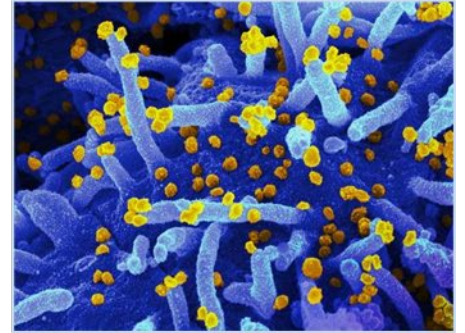
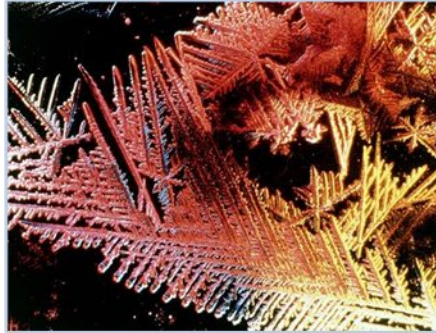
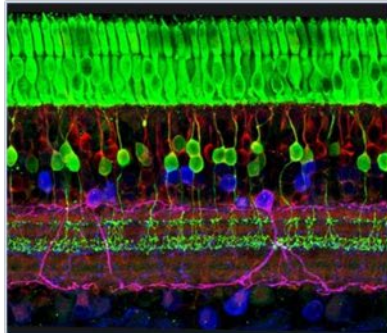
Turning Discovery Into Health





FNIH...

Building Bridges to Breakthroughs



Ambition:

Accelerate and amplify the value and impact of NIH and its research and scientific collaboration on people's health and health equity



BUILDING BRIDGES TO BREAKTHROUGHS

- Building Collaborative Science**
- Supporting Scientists**
- Earning Trust in Science**

Ambition:

Accelerate and amplify the value and impact of NIH and its research and scientific collaboration on people's health and health equity



BUILDING BRIDGES TO BREAKTHROUGHS

[Anthem Video](#)



**Building
Collaborative Science**

**Partnership
Planning
& Design**

Fundraising

Governance

**Financial
Stewardship &
Management
of Funds**

**Rigorous
Program &
Project
Management**

**Intellectual
Property
Management**

**Policy
Oversight**

**Convening
Stakeholders**

Collaborative Science: Major Public Private Patient Partnerships and Platforms

Public & private funding



\$1B+
Accelerating COVID-19
Therapeutic Interventions &
Vaccines (ACTIV)



\$783M
Accelerating Medicines
Partnership



\$220M
Partnership for
Accelerating Cancer Therapies



\$206M
Alzheimer's Disease
Neuroimaging Initiative (ADNI)



\$23M
Gene Convene



\$201M
Grand Challenges in
Global Health (GCGH)

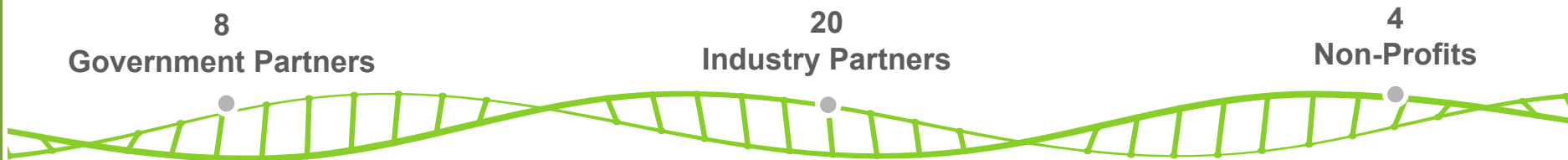


\$163M
Lung-MAP:
Master Lung Protocol Trial



\$107M
The Biomarkers Consortium

ACTIV: Accelerating COVID-19 Therapeutic Interventions and Vaccines



ACTIV: Four Major Focus Areas

Participants: over 100 scientists from NIH, industry, academia, and foundations



Vaccines

Achievements

- + Developed harmonized protocols for vaccine efficacy trials
- + Support decision-making for EUA criteria
- + Recommendations re: controlled human infection trials and threat of immune-associated disease enhancement

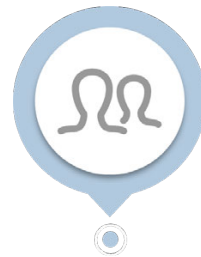


Preclinical

- + National strategy for non-human primate research
- + Inventory of in-vitro and in-vivo resources
- + SOPs for accelerated preclinical development
- + Open Data Portal at NIH



TRACE



Clinical Trial Capacity

- + International clinical site survey
✓ (63 networks, 39 CROs/SMOs, 728 sites)
- + Online geotracking tool
- + Strategies for enhancing trials in a pandemic (virtual, digital, online solutions)

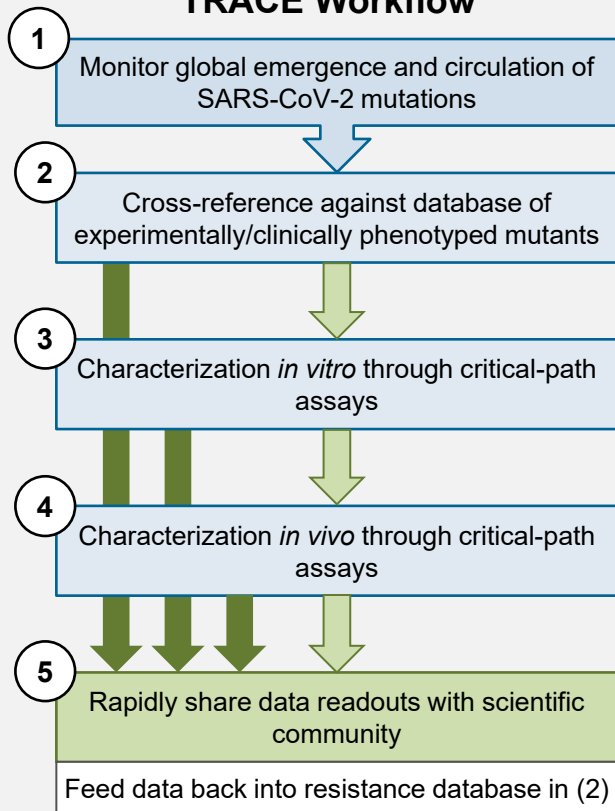


Therapeutics – Clinical

- + Select the most promising COVID treatment candidates
- + Provide accelerated, robust clinical testing of selected agents in outpatient, inpatient, and convalescent populations via master protocol trials

ACTIV TRACE (Tracking Resistance And Coronavirus Evolution): Tracking Virus Variants

TRACE Workflow



TRACE Priorities



Publish weekly TRACE report summarizing shifting trends in emerging viral variants



Collect available industry and government agency data on variants in one place



Generate additional datasets using standardized protocols and common reference reagents

ACTIV Therapeutics Clinical Trials Success Thus Far: At-A-Glance

ENROLLMENTS & ACTIVATION

23,000+ Patients enrolled into ACTIV trials

620+ Sites in partnership with **multiple networks** including ACTG, CONNECTS, DCRI, INSIGHT, PETAL, CTSN, PCORnet, CTSA, IDeA Sites, ACTT, and others



PUBLICATIONS



45 Scientific Publications on ACTIV Trials



These publications have been **cited 1,640+ times** (Google Scholar)

AGENT REVIEWS & SUCCESSES

800+

Total agents reviewed by ACTIV Tx-Clinical and CONNECTSWGs Agent Review Panels

33

Unique agent formulations/dosages or agent combinations fully enrolled through the ACTIV Master Protocols

6

Agents proven efficacious against COVID-19 in analysis of data from ACTIV Trials. Other priority agents being tested.

- ACTIV-4 work on heparin and other anticoagulants changed clinical practice
- Bii Bio monoclonal antibody combination submitted for EUA based on data from ACTIV trials
- ACTIV-1 and ACTIV-3 have shown Evusheld, Infliximab, and Abatacept decrease mortality in hospitalized patients
- 3 other monoclonal antibody products tested in ACTIV trials received EUAs based on separate, industry-supported trials
- Industry trials of Merck & Pfizer antivirals were harmonized with ACTIV protocols

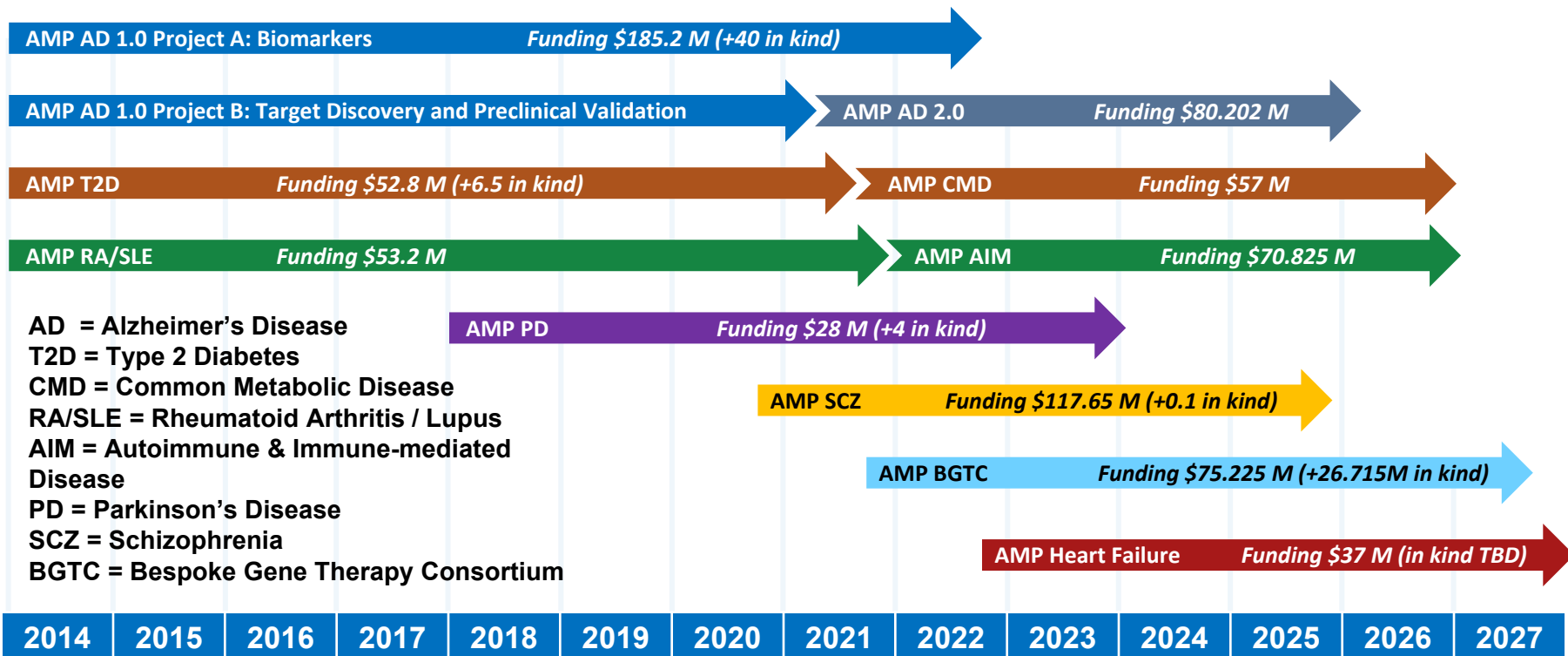
ACTIV: Some Lessons Learned

- 1. We need an active, transparent, coordinated system of global surveillance for emerging and ongoing biothreats that is capable of sharing data effectively with scientists across international borders.**
2. We should invest now to develop a robust pipeline of new, qualified, early-stage therapeutic candidates (particularly antivirals) to meet future pandemic threats.
- 3. A centralized system for selecting and prioritizing promising investigational therapies (new and repurposed) that integrates both public and private scientific expertise is essential.**
4. Broad access to repositories of standardized **sequence data, viruses, assays, and animal models can greatly accelerate preclinical research.**

ACTIV: Some Lessons Learned (continued)

4. **There is still no fully coordinated system for conducting preclinical and clinical research in the U.S. in the event of a national health emergency.** Upgrading the clinical trial ecosystem to adequate state of readiness requires:
 - A centralized inventory of both preclinical and clinical resources (including sites and their capabilities)
 - A “warm” network of sites with the capabilities and resources for rapid response (personnel, training, equipment) and - if possible - an active protocol
 - Early agreement on effective study designs and regulatory approaches
 - Standardized policies and structured contractual templates
5. We need to better **integrate community care settings** into our clinical trial ecosystem
 - Go where the patients are
 - Improve equity and diversity of clinical trial populations
6. **Robust, well-coordinated public-private partnerships are essential to mounting an effective pandemic response**

Growth in the Accelerating Medicines Partnership® (AMP®) Portfolio




AMP Type 2 Diabetes Program: Powerful Knowledge Portal with Federated Nodes in the U.S. and U.K.

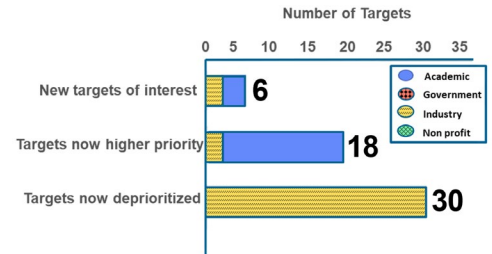


>1.5M subjects 50 countries

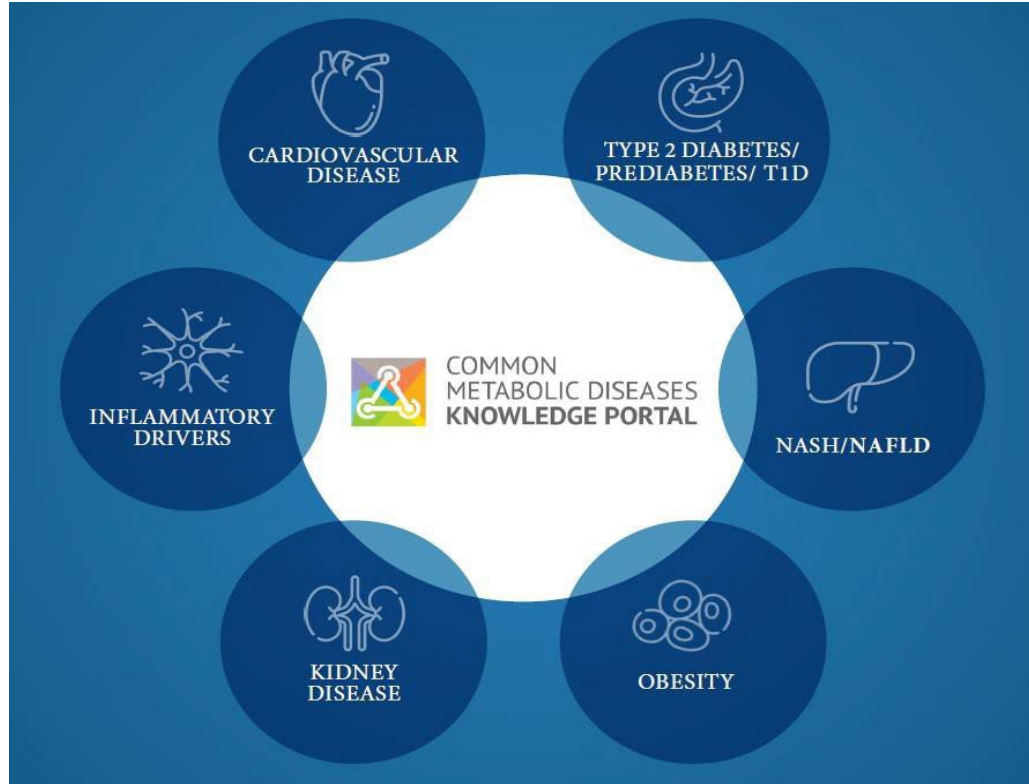
 Common Metabolic Disease Genome Atlas
Functional Genomics Data (3500+ annotations)

 **T2DKP**
Genetic Data (368 datasets, 388 traits) >77M variants

 **EMBL-EBI**
Genetic Data (comply with GDPR)



AMP Common Metabolic Disease: A \$57M partnership to expand AMP T2D Knowledge Portal and Prioritize Targets for All Metabolic Diseases

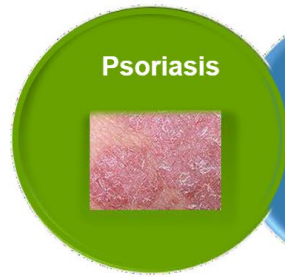
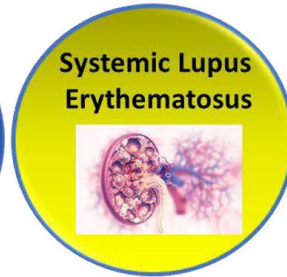


368 datasets, 388 traits
<http://hugeamp.org>



4,495 annotations
<http://cmdga.org>

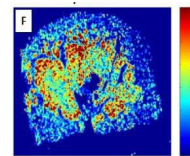
AMP Autoimmune and Immune-Mediated Disease: \$65M Investment in Systems Biology



Disease Reconstruction

- ❖ Discover how innate and adaptive cells of the immune system and tissue resident cells interact to drive inflammation and clinical disease
- ❖ Map anatomic locations, neighborhood pathology, cell-to-cell and receptor-to-ligand interactions
- ❖ Define how these cell and molecular pathologies are common across diseases and across tissues
- ❖ RA/SLE Data Portal launched Q2 2022

BIG NEWS



Key Deliverables

- ❖ Advance understanding of how cell-cell interactions activate specific mechanisms of disease through spatial analytics
- ❖ Accelerate the discovery of new mediators of disease through “interactome” analytics
- ❖ Systems level integration across tissues and diseases combining the above with epigenetics and genomics to identify target molecules in causative pathways of disease
- ❖ Public Portal Launch – RA/SLE + AIM datasets → incorporate capability for federated datasets from other consortia & studies (e.g., 3TR)

ACCELERATING MEDICINES PARTNERSHIP® (AMP®)

Accelerating Medicines Partnership (AMP)



[Alzheimer's Disease](#)

[Autoimmune and Immune-Mediated Diseases](#)

[Bespoke Gene Therapy Consortium](#)

[Common Metabolic Diseases](#)

[Heart Failure](#)

[Parkinson's Disease](#)

[Rheumatoid Arthritis and Lupus](#)

[Schizophrenia](#)

[Type 2 Diabetes](#)

Bespoke Gene Therapy Consortium

Overview

The Accelerating Medicines Partnership® (AMP®) program is a public-private partnership between the National Institutes of Health (NIH), the U.S. Food and Drug Administration (FDA), and multiple public and private organizations. Managed through the Foundation for the NIH (FNIH), the AMP program aims to improve our understanding of therapeutically relevant biological pathways and validate information that could be relevant for the development of multiple therapeutics.

The AMP Bespoke Gene Therapy Consortium (BGTC) aims to develop platforms and standards that will speed the development and delivery of customized or "bespoke" gene therapies that could treat the millions of people affected by rare diseases.

Launched in October 2021, BGTC is the first AMP initiative focused on rare diseases and the sixth AMP initiative overall. The effort aims to overcome major obstacles related to developing gene therapies and will create a gene therapy protocol book that the research community can use to make the process of developing gene therapies for rare conditions much more efficient.

On this page

- [About Rare Diseases](#)
- [Need for New Therapies](#)
- [AMP Approach](#)
- [Governance](#)
- [Budget](#)

Public-private consortium announces eight rare diseases for gene therapy clinical trial portfolio

May 22, 2023 / [Health, Latest News](#) / By [Colleen Pietras, M.D.](#)



Bespoke Gene Therapy Consortium (BGTC)

“When will we stop this madness of funding rare disease research through bake sales and road races? That's what's at the heart of what BGTC has to offer. We need dozens and dozens of BGTCs.”

- Jocelyn Duff

(mother of Talia Duff, a child with CMT 4J)

BGTC Rare Diseases Portfolio

- Charcot-Marie-Tooth 4J
- Congenital Hereditary Endothelial Dystrophy
- Morquio A Syndrome
- Multiple Sulfatase Deficiency
- NPHP5 Retinal Degeneration
- Propionic Acidemia (PCCB)
- Retinitis pigmentosa 45
- Spastic paraplegia 50

Bespoke Gene Therapy Consortium Partners

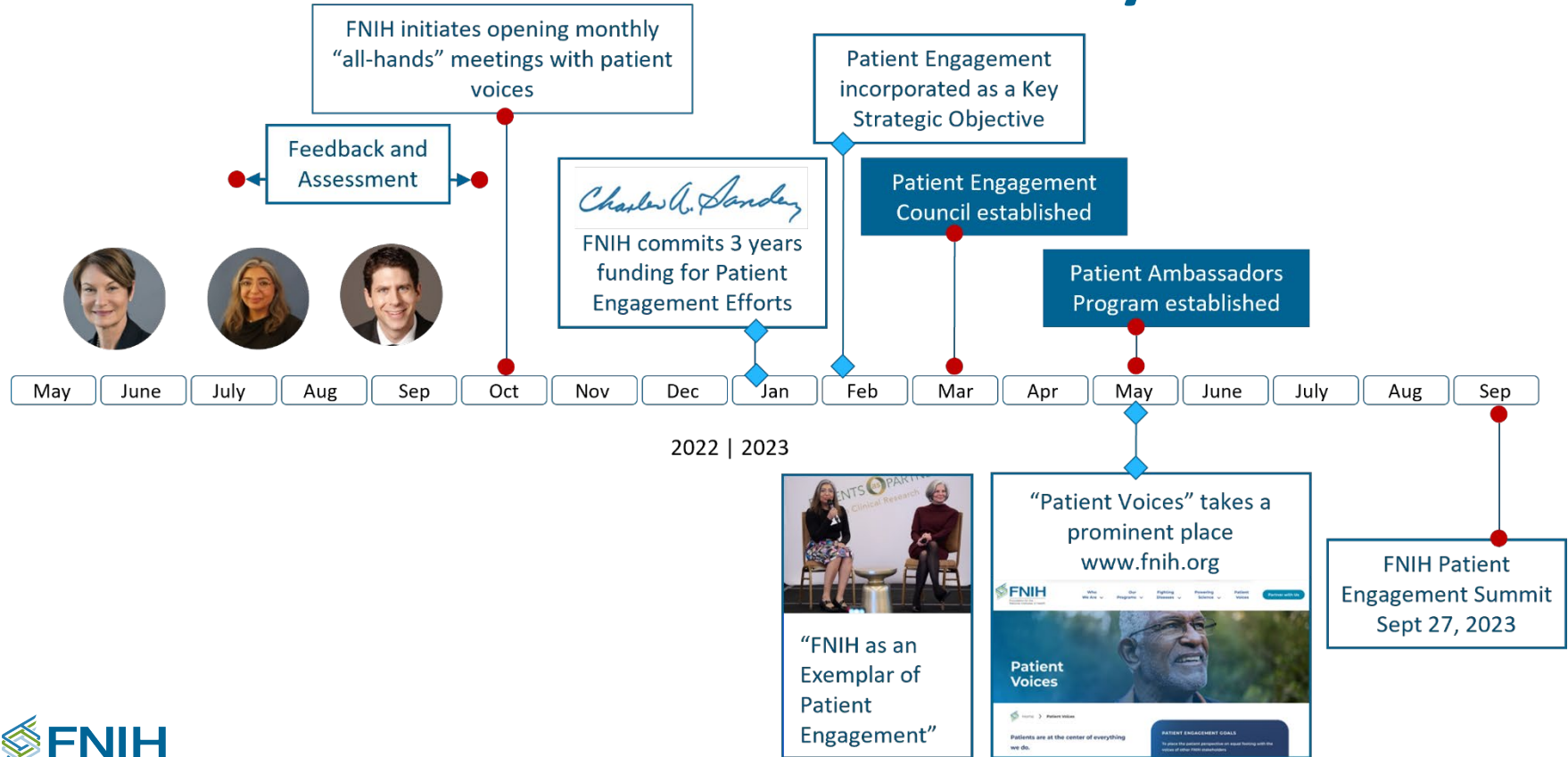
Government Partners

- NCATS
- NINDS
- NIMH
- NHGRI
- NHLBI
- NICHD
- NEI
- NIDCD
- NIDCR
- NIAMS
- BRAIN Initiative
- FDA
- CIRM

Non-government Partners

- Alliance for Regenerative Medicine
- American Society of Gene & Cell Therapy
- Biogen Inc.
- CureDuchenne
- Danaher Corporation
- Elpida Therapeutics
- Forge Biologics
- Foundation Fighting Blindness
- GENETHON
- Janssen Research and Development LLC
- National Organization for Rare Disorders
- Novartis Institutes for BioMedical Research
- Ovid Therapeutics Inc
- Pfizer Inc
- REGENXBIO Inc
- Rhett Syndrome Research Trust
- RTW Charitable Foundation
- Spark Therapeutics
- Takeda Pharmaceutical Company Ltd
- National Institute for innovation in Manufacturing Biopharmaceuticals
- Thermo Fisher Scientific
- Ultragenyx Pharmaceutical

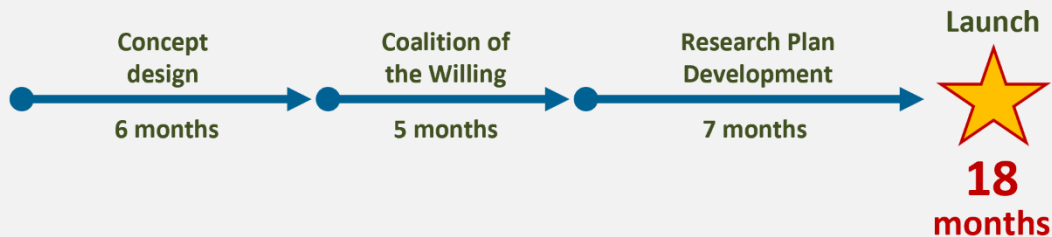
FNIH End-to-End Patient-centricity



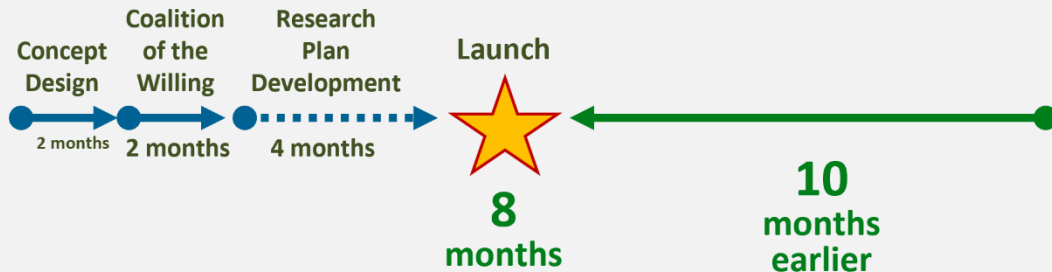
AMP ALS: Accelerating “Idea to Implementation”

- Amyotrophic lateral sclerosis (ALS) causes rapid, progressive motor weakness, leading to death 3-5 years from time of diagnosis
- No truly effective treatments, nor biomarkers, for ALS
- The ACT for ALS bill authorized \$100 million annually

AMP average development timeline (standard)



AMP ALS Development timeline (aspiration)



How Additional FNIH Financial Resources and Flexibility will Help Achieve Our Strategic Priorities and Benefit NIH

1. Expand scope, scale, reach, and impact of our scientific partnerships, platforms, and portfolios
2. Speed and enable start-up project investments “at risk”
 - a. accelerate launches before NIH fiscal year or private sector funding is complete
 - b. stimulate pilots, seed projects, and other innovative on-ramps to project design and partnership development
 - c. signal “skin in the game” credibility to motivate other investors
3. Expand recognition and support for scientific “stars” at NIH and elsewhere
4. Sustain the recruitment, development, and retention of top talent
5. Ensure long-term financial sustainability and flexibility

Building Bridges to New and Expanded Partnerships: Pipeline

23 Q1: Sickle cell gene therapy (Bill and Melinda Gates Foundation)

Lung MAP S1900G

23 Q2: AMP ALS design

AMP Systems Biology of Inflammation design

Biomarkers Consortium: PET tracers in lung cancer

Biomarkers Consortium: Preeclampsia

Pediatric medical devices

23 Q3: Genomic workforce diversity PPP

Biomarkers Consortium: Systemic Sclerosis

NIH All of Us biomedical workforce diversity PPP

23 Q4: Biomarkers Consortium: Monoclonal Gammopathy

24 Q1: AMP Parkinson's disease phase 2

Supporting Scientists

Powering science by celebrating, funding, and training scientists



The Lurie Prize in Biomedical Sciences

Recognizes outstanding achievement by promising scientists aged 52 or younger



2014: Jennifer Doudna
Nobel Laureate in Chemistry;
Professor of Biochemistry,
Biophysics and Biology,



2019: Yasmine Belkaid
President, Institut Pasteur



Trailblazer Prize for Clinician-Scientists

Recognizes early career clinician-scientists driving innovations in patient care



2022: Eliezer Van Allen
Chief of the Division of
Population Sciences,
Dana Farber Cancer Institute



2022: Nikhil Wagle
Associate Professor of
Medicine, Harvard
Medical School; Institute
Member, Broad Institute



The Charles A. Sanders Partnership Award

Recognizes people/organizations leading private-public partnerships with the FNIH

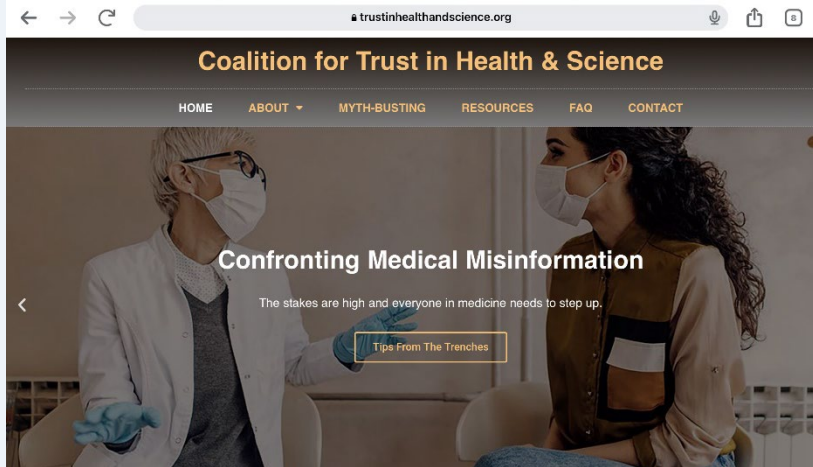


2020: Anthony Fauci
Former Director, NAID;
Former Chief Medical
Advisor to the President



2022: Amgen

Earning Trust in Science



www.trustinscienceandhealth.org

- ✓ 70+ **member organizations** cover the full spectrum of health, health care and science organizations and societies;
- ✓ Communicate directly to the public and through our organizations and their constituents;
- ✓ Correct misinformation and counter disinformation that reduces trust and harms people's health;
- ✓ Capitalize on a “living library,” a compendium of research and activities to share strategies, foster collaboration and serve as a hub of innovation and practice;
- ✓ Utilize and promote websites that provide public access to accurate information and members' resources;
- ✓ Engage in policy advocacy at national and state levels.

Coalition for Trust in Health & Science

[HOME](#)[ABOUT ▾](#)[MYTH-BUSTING](#)[RESOURCES](#)[FAQ](#)[CONTACT](#)

Debunking Myths:

“Did Damar Hamlin’s heart stop because of the COVID vaccine?”

- Mr Hamlin is a football player for the Buffalo Bills whose heart stopped after he was tackled last Monday night. He was successfully revived and is now doing better.
- Lots of speculation why it happened.
- There was an image with a date Jan. 11 showing as a CNN headline that read, “Doctor of Damar Hamlin confirms Cardiac Arrest was due to the 4th Booster Vaccine.” However, that headline was fake and was not put out by CNN!

Let’s look at what’s real though. Here are 3 facts to consider:

1. Nearly 95% of the roughly 1700 NFL players are vaccinated against COVID – none of them have had sudden death on the field.
2. One cause of sudden death in athletes is heart inflammation (myocarditis) which has happened after COVID vaccination, with young men being at higher risk. However, the risk of myocarditis is 7 times higher due to infection with the coronavirus, SARS COV-2. If you have had COVID, the risk of myocarditis is 15 times higher regardless of whether you’ve been vaccinated or not compared to those who never contracted the virus.
3. Finally, there has never been evidence found that Mr Hamlin had any heart damage from anything. Our January 9, 2023 episode of “Excuse Me Dr!” (see Dr Melissa Clarke’s You Tube channel) had a whole explaining how his cardiac arrest could have happened as explained by an Emergency and Sports Medicine physician.

Ambition:

Accelerate and amplify the value and impact of NIH and its research and scientific collaboration on people's health and health equity

BUILDING BRIDGES TO BREAKTHROUGHS

- ✓ Building **Collaborative Science**
- ✓ Supporting **Scientists**
- ✓ Earning **Trust in Science**



Thank you!!!