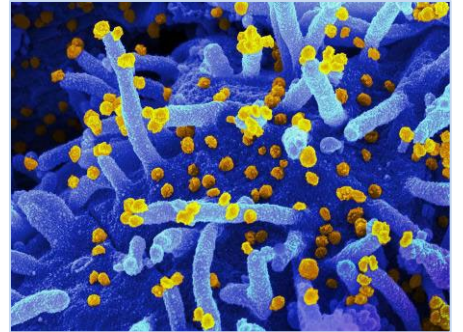
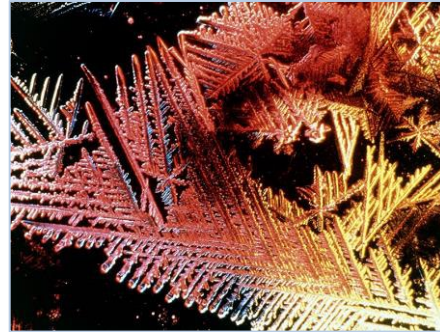
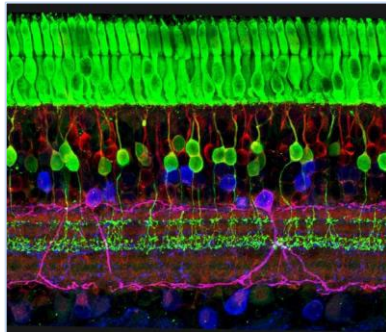
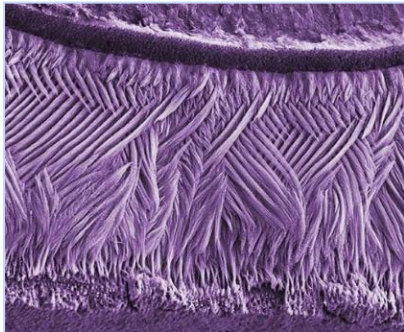
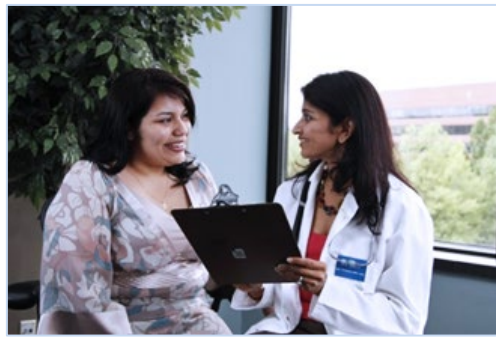


# NIH...

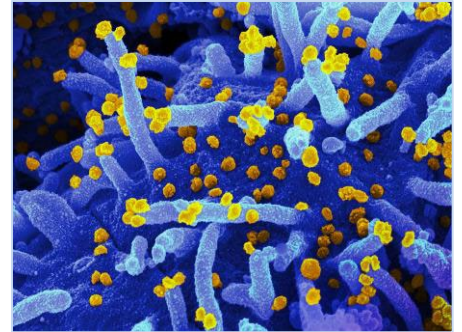
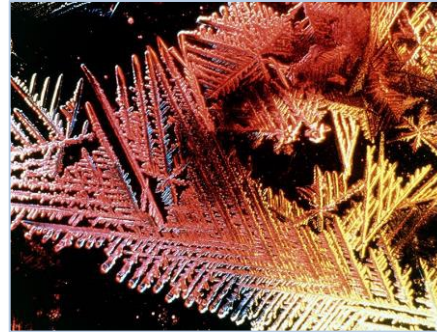
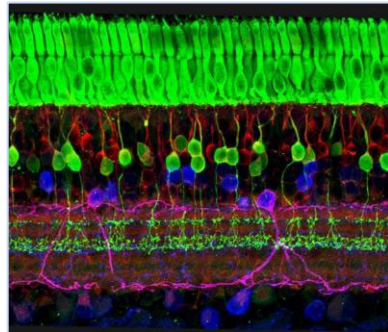
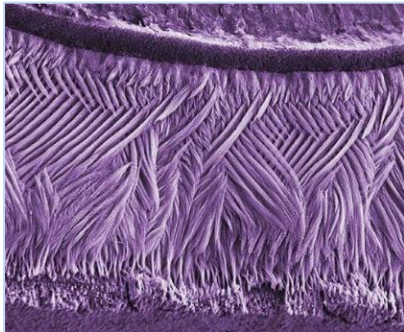
## Turning Discovery Into Health





# 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**

# FNIH Public-Private Partnership Criteria

- 1) **Significant unmet need and high potential patient impact**
- 2) **Clearly supports NIH's mission**
- 3) **Uniquely suited to a public-private partnership mechanism**
  - No single entity can achieve results as well on their own
  - Is not already being done elsewhere
- 4) **Significant value proposition for the private sector partners**
  - Is “fundable”
  - Is pre-competitive
- 5) **Executive-level sponsorship from stakeholder organizations (including NIH)**
  - Essential, especially for larger projects

## **Optional (but nice to have) attributes:**

- Partnership leverages a significant NIH contribution
- FNIH has a strong portfolio or project management role that provides clear added value (especially for research partnerships)

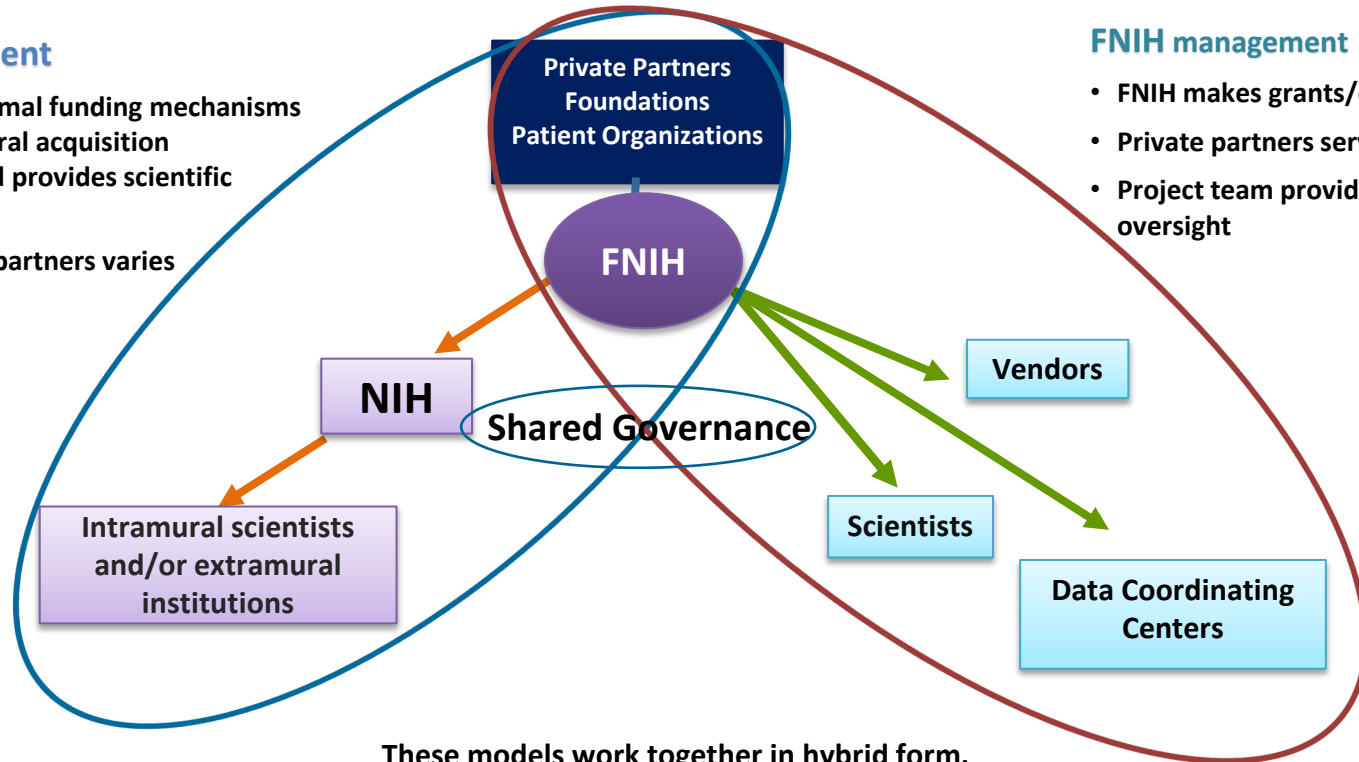
# Partnership Models and Flow of Funds

## NIH management

- NIH uses its normal funding mechanisms (subject to federal acquisition regulations) and provides scientific oversight
- Role of private partners varies

## FNIH management

- FNIH makes grants/contracts directly
- Private partners serve on project team
- Project team provides scientific oversight



These models work together in hybrid form.  
FNIH deploys private sector funds in multiple ways  
as required by specific project needs.

# Biomedical Pipeline: Partnership Opportunities for Innovation

- Basic Biology
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        - Clinical Studies
          - Regulatory Approvals
            - Post-approval Studies
              - Equitable Access and Uptake

# Biomedical Pipeline: Partnership Opportunities for Innovation

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# Accelerating Medicines Partnerships

AMP<sup>®</sup> the Numbers

**10**

Projects

**34**

Industry Partners

**\$834M**

Total Investment

**16**

NIH Institutes and  
cross-institute programs

**10**

Years

**37**

Non-Profits

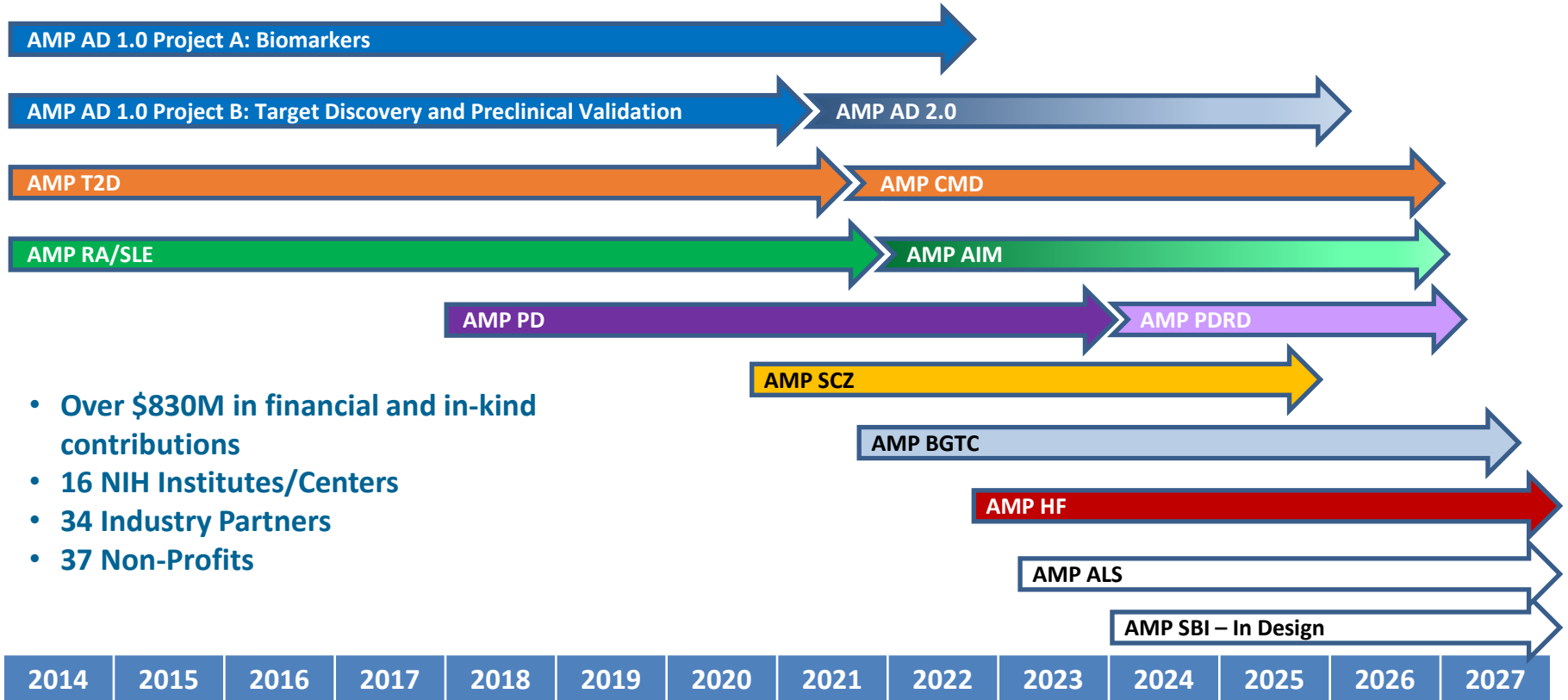
<https://fnih.org/our-programs/accelerating-medicines-partnership-amp/>



Building Bridges to Breakthroughs



# The Accelerating Medicines Partnership® Program



- Over \$830M in financial and in-kind contributions
- 16 NIH Institutes/Centers
- 34 Industry Partners
- 37 Non-Profits

AD = Alzheimer’s Disease

T2D = Type 2 Diabetes

CMD = Common Metabolic Disease

RA/SLE = Rheumatoid Arthritis / Lupus

AIM = Autoimmune & Immune-mediated Disease

PD = Parkinson’s Disease

SCZ = Schizophrenia

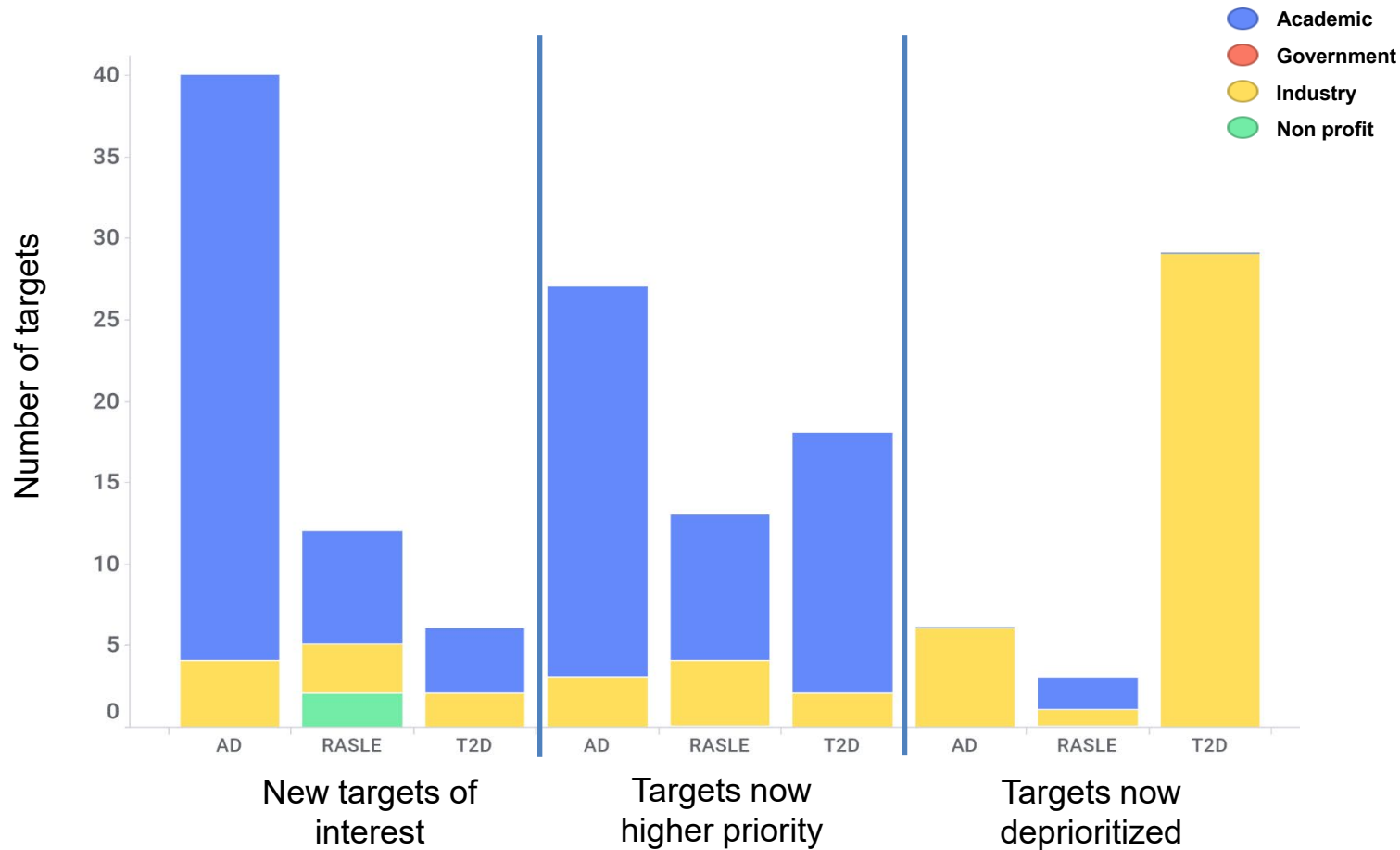
BGTC = Bespoke Gene Therapy Consortium

ALS = Amyotrophic Lateral Sclerosis

SBI A= Systems Biology of Inflammation

As of September 2024

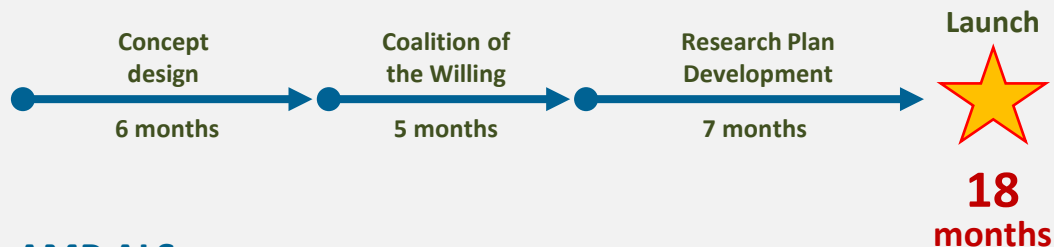
# AMP Impact on Target Prioritization



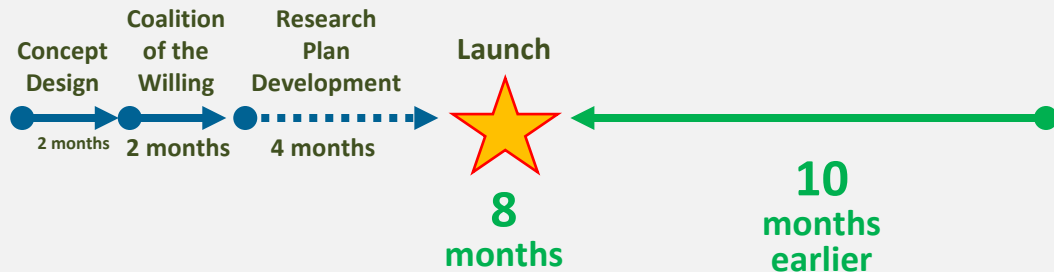
# AMP ALS: Accelerating “Idea to Implementation”

- 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)



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# ACTIV Therapeutics (COVID Clinical Trials): At-A-Glance

## ENROLLMENTS & ACTIVATION

**23,000+ Patients** enrolled in ACTIV trials

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



## PUBLICATIONS SO FAR



**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
- Brie 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: Accelerating COVID-19 Therapies and Vaccines

8

Government Partners

20

Industry Partners

4

Non-Profits



abbvie

AMGEN

AstraZeneca

Bristol-Myers Squibb

dewpoint<sub>x</sub>



Lilly



Johnson & Johnson

MERCK

moderna

NOVARTIS

RHYTHM  
THERAPEUTICS

Roche Genentech  
A Member of the Roche Group

Pfizer

SANOFI

Takeda

NOVAVAX

VIR

BILL & MELINDA  
GATES foundation



FRED HUTCH  
CURES START HERE™

IRTI  
INTERNATIONAL

FNIH

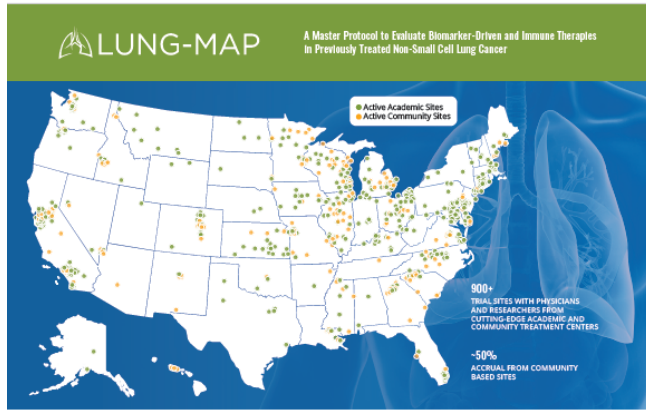
FNIH

# Biomedical Pipeline: Partnership Opportunities for Innovation

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# Precision Cancer Therapy: Lung MAP (Master Protocol)

## Nearly 30 Public and Private Partners since 2014



- +300 altered genes interrogated in each patient's tumor
- 15 investigational drugs tested (so far)
- 12-month average sub-study stand up time

### Nearly 30 Public and Private Collaborators and Supporters in Partnership since 2014

13 PARTNERING PRECISION MEDICINE DIAGNOSTIC COMPANIES AND LEADING PHARMACEUTICAL COMPANIES	7 SUPPORTING PATIENT ADVOCACY GROUPS	8 ORGANIZATIONS, INCLUDING NCI AND FDA, WORKING TOGETHER TO CONDUCT AND OVERSEE THE STUDY
15 INVESTIGATIONAL DRUGS OR DRUG COMBINATIONS TESTED	~4,250   ~650 Per Year PATIENTS SCREENED	10,000+ ANNOTATED SPECIMENS IN A TISSUE BANK TO ALLOW DEEPER SCIENTIFIC STUDIES TO INFLUENCE FUTURE TRIALS
16 Initiated   14 Completed SUB-STUDIES CONDUCTED	~2,750 PATIENTS ELIGIBLE FOR A TREATMENT IN A WELL-DESIGNED TRIAL	35+ PUBLICATIONS AND ABSTRACTS
10 AGENTS AGAINST SPECIFIC TUMOR MUTATIONS/GENETIC SIGNATURES TESTED IN NSCLC	~1,000   150+ Per Year PATIENTS TREATED WITH CUTTING-EDGE THERAPIES	<b>Additional Benefits of Lung-MAP</b> SHARED COSTS AND RISKS OF TESTING THERAPEUTICS FOR COMPANIES FOSTERING DRUG COMBINATION COLLABORATIONS BETWEEN COMPANIES ACCELERATED TIMEFRAMES FOR EVALUATION OF TREATMENT EFFICACY DUE TO THE LARGE NETWORK. STRONG SUPPORT FROM THE FDA WITH ABILITY FOR STUDIES TO HAVE REGULATORY INTENT
12 MONTH AVERAGE SUB-STUDY STAND UP TIME FROM APPROVAL BY THE LUNG-MAP DRUG SELECTION COMMITTEE TO ACTIVATION	300+ ALTERED GENES INTERROGATED IN EACH PATIENT'S TUMOR	
22 MONTH AVERAGE TIME TO TARGET ACCRUAL COMPLETION FOR ~80 PERSON STUDY, DRIVEN BY BIOMARKER PREVALENCE	24% UNDERSERVED MINORITY PARTICIPANTS ENROLLED	

### Highly Motivated Expert Partners for Trial Conduct



### Highly Motivated Expert Partners for Trial Conduct





# Biomedical Pipeline: Partnership Opportunities for Innovation

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# Biomarkers Consortium

## Mission

- To create and lead **CROSS-sector** efforts that validate and qualify biomarkers and other drug development tools to accelerate better decision-making for the development of new therapeutics and health technologies.

## Goals

- Facilitate the development and the seeking of regulatory approval for biomarkers using new and existing technologies;
- Develop evidence to help qualify biomarkers for specific applications in diagnosing disease, predicting therapeutic response or improving clinical practice;
- Generate information useful to inform regulatory decision making;
- Make consortium project results broadly available to the entire scientific community.



## Key Outcomes

- 35+ Projects launched since inception
- 14 Therapeutics advanced
- 1 Clinical safety biomarker qualification
- 6 FDA Guidance documents
- 9 Clinical tools used in drug development

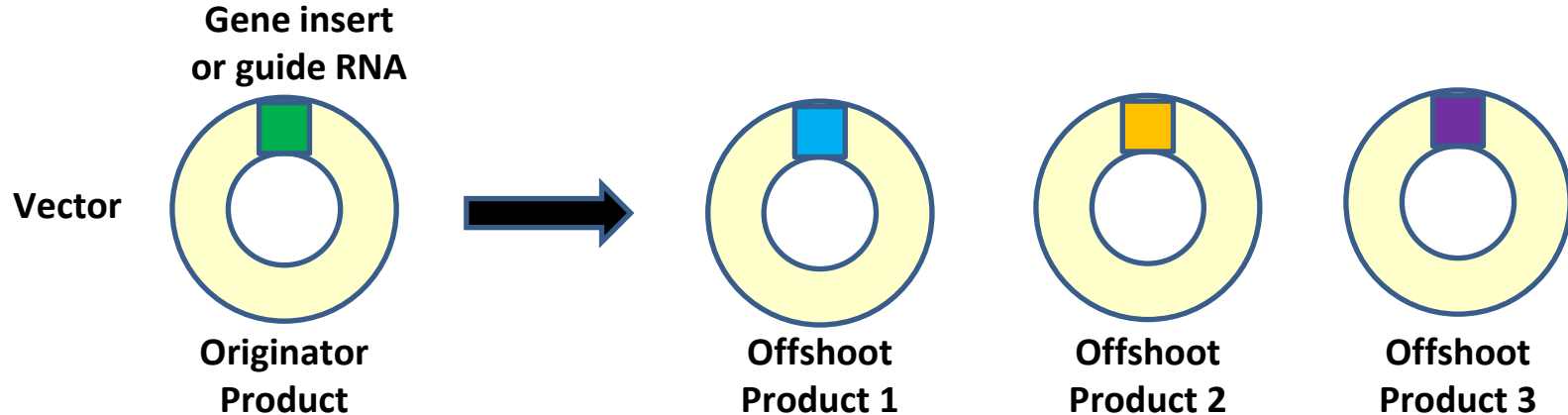
# Biomarkers Consortium Private Sector Members (as of 23 August 2023)



# Biomedical Pipeline: Partnership Opportunities for Innovation

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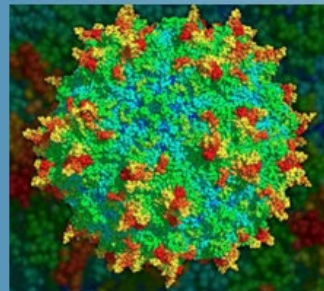
# Regulatory Innovation: Platform Technologies



## Premise

- In appropriate situations, non-clinical data and manufacturing information from one product may be able to be leveraged to another

# Accelerating Medicines Partnership® Bespoke Gene Therapy Consortium (BGTC)



<https://fnih.org/our-programs/AMP/BGTC>

# 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 Partners

## Bespoke Gene Therapy Consortium

**NIH** National Center for Advancing Translational Sciences

**NIH** Eunice Kennedy Shriver National Institute of Child Health and Human Development

**NIH** National Eye Institute  
Research Today...Vision Tomorrow

**NIH** National Heart, Lung, and Blood Institute

**NIH** National Human Genome Research Institute

**NIH** National Institute of Arthritis and Musculoskeletal and Skin Diseases

**NIH** National Institute of Dental and Craniofacial Research

**NIH** National Institute of Mental Health

**NIH** National Institute of Neurological Disorders and Stroke

**NIH** National Institute on Deafness and Other Communication Disorders

**BRAIN**  
INITIATIVE

**FDA**

**DANAHER**  
**Biogen**

**FORGE**  
BIOLOGICS

**Spark**  
THERAPEUTICS

**ultragenyx**  
pharmaceutical

**CIRM**  
CALIFORNIA'S STEM CELL AGENCY

**GENETHON**  
CURE THROUGH INNOVATION

rett syndrome  
research trust  
Making Rett History

**Janssen**

**Pfizer**

**Takeda**

Alliance for  
**Regenerative  
Medicine**

**Cure  
Duchenne**

**NIMBL**  
The National Institute for Neurological Disorders and Stroke

**RTW**  
Charitable Foundation

**NOVARTIS**

**REGENXBIO**

**ThermoFisher  
SCIENTIFIC**

**ASGC** American Society  
of Gene + Cell Therapy

**FOUNDATION  
FIGHTING  
BLINDNESS**

**NORD**  
National Organization  
for Rare Disorders

**ELPIDA  
THERAPEUTICS**



# BGTC Regulatory Playbook *Version 1.0*

**OBJECTIVE: DOCUMENT BEST PRACTICES FOR GENE THERAPY PRODUCT DEVELOPMENT, INCORPORATING BGTC MINIMUM STANDARDS AND EXPERT ADVICE**



- **Version 1.0 released February 6, 2024 for viewing using QR code below**
- **Serves as an initial framework**
- **Subsequent versions (v2.0+) will incorporate learnings from BGTC, including:**
  - **minimal CQAs and experiences gained**
  - **minimum animal toxicology and experiences gained**
  - **streamlined approaches to regulatory submissions**





**Partnership  
Planning  
& Design**

**Fundraising**

**Convening  
Stakeholders**

**Governance**



**Building  
Collaborative Science**

**Policy  
Oversight**

**Financial  
Stewardship &  
Management  
of Funds**

**Rigorous  
Program &  
Project  
Management**

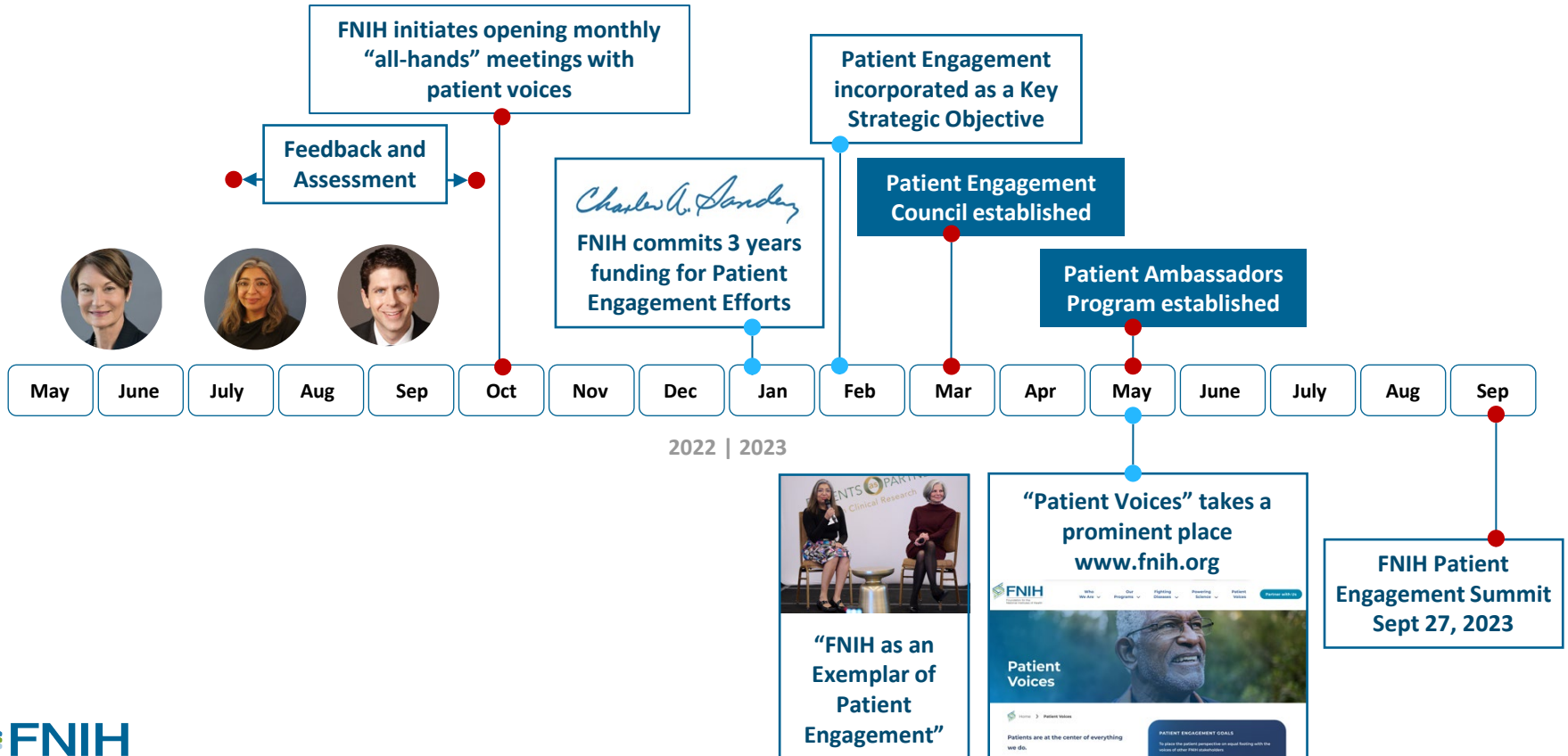
**Intellectual  
Property  
Management**

# Biomedical Pipeline: Partnership Opportunities for Innovation

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              - **Equitable Access and Uptake**

# FNIH End-to-End Patient-centricity

- Key Activities
- Key Decisions



# 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



## Trailblazer Prize for Clinician-Scientists

Recognizes early career clinician-scientists  
driving innovations in patient care



## The Charles A. Sanders Partnership Award

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

# Earning Trust in Science

[www.trustinhealthandscience.org](http://www.trustinhealthandscience.org)

## The Kovler Prize For Trust In Life Science Journalism

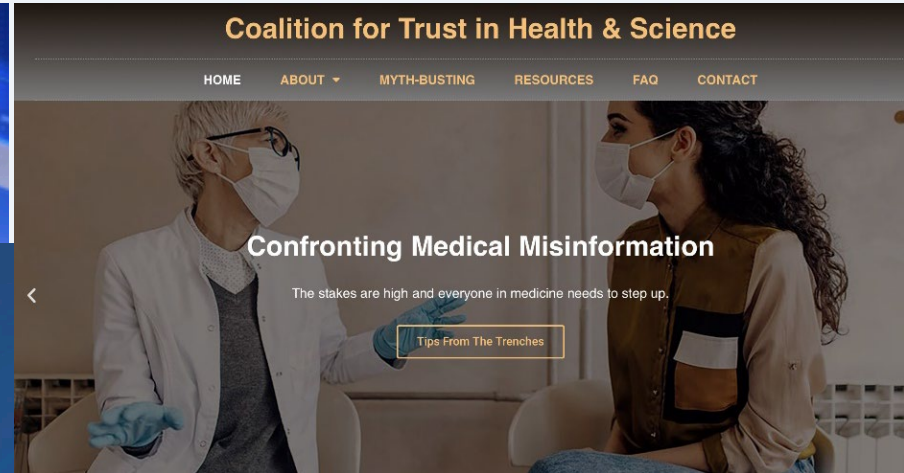
### 2024 Recipient




**Katherine J. Wu, PhD**

Staff Writer, *The Atlantic*

A scientist-turned-science reporter, Dr. Wu holds a PhD in microbiology and immunobiology from Harvard University and much of her work has focused on infectious diseases. Before joining *The Atlantic*, she was a science reporting fellow for *The New York Times*, where she covered many aspects of COVID-19. Her work has appeared in *National Geographic*, *Popular Science* and *Scientific American*. Dr. Wu has received the Eric and Wendy Schmidt Award for Excellence in Science Communication, the Science in Society Journalism Award and the Evert Clark/Seth Payne Award for Young Science Journalists.





**Ambition:**  
Accelerate and amplify the  
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*BUILDING BRIDGES TO  
BREAKTHROUGHS*

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

**Thank you for your partnership!!!**