

Novel Alternative Methods (NAMs) Validation and Qualification Network (VQN) Public-Private Partnership Plan Update

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Building Bridges to Breakthroughs

Science has the power to cure, but no single organization can do it alone.

The Foundation for the National Institutes of Health (FNIH) is a non-profit organization chartered by the U.S. Congress and launched in 1996 to support the mission of NIH.

We connect world-leading NIH researchers with the ingenuity and expertise of public and private sector leaders to accelerate medical breakthroughs.

Partnering with world-class organizations to tackle the most pressing health challenges

PUBLIC



We support the mission of the nation's premier biomedical research agency, driving discoveries that improve health and save peoples' lives

BIOPHARMA



We collaborate with leading R&D organizations to advance research that will lead to new therapies, diagnostics, and potential cures

FOUNDATIONS



We work with foundations to address urgent issues in global health and accelerate biomedical innovation across a range of diseases

Biomedical innovation to improve health

\$1.55B private funds raised

122 active partnerships

\$.90 of every dollar directly supports programs

We accelerate prevention, new therapies, diagnostics & potential cures

ACCELERATING MEDICINES PARTNERSHIP® (AMP®)

AMP® BESPOKE GENE THERAPY CONSORTIUM (BGTC)

BIOMARKERS CONSORTIUM

PARTNERSHIP FOR ACCELERATING CANCER THERAPIES (PACT)

ACCELERATING COVID-19 THERAPEUTIC INTERVENTIONS & VACCINES (ACTIV)

We advance global health & seek equity in care

MATERNAL & CHILD HEALTH

GENECONVENE GLOBAL COLLABORATIVE

GRAND CHALLENGES IN GLOBAL HEALTH

We power science by celebrating & training the next generation of scientists

LURIE PRIZE IN BIOMEDICAL SCIENCES

TRAILBLAZER PRIZE FOR CLINICIAN-SCIENTISTS

CHARLES A. SANDERS, MD PARTNERSHIP AWARD

FNIH Partnerships Cover a Spectrum of Designs

Funded exclusively by public organizations

ACTIV



Funded by both public and private organizations



Funded exclusively by private organizations

**BIOMARKERS
CONSORTIUM**

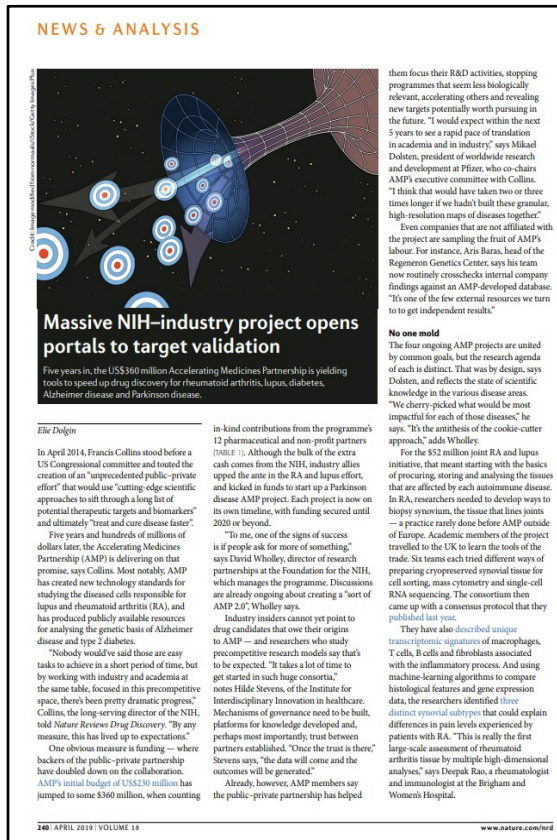
IMPROVING HEALTH THROUGH
MEANINGFUL MEASUREMENTS

Grand Challenges
in Global Health



The Accelerating Medicines Partnership® (AMP®) Program

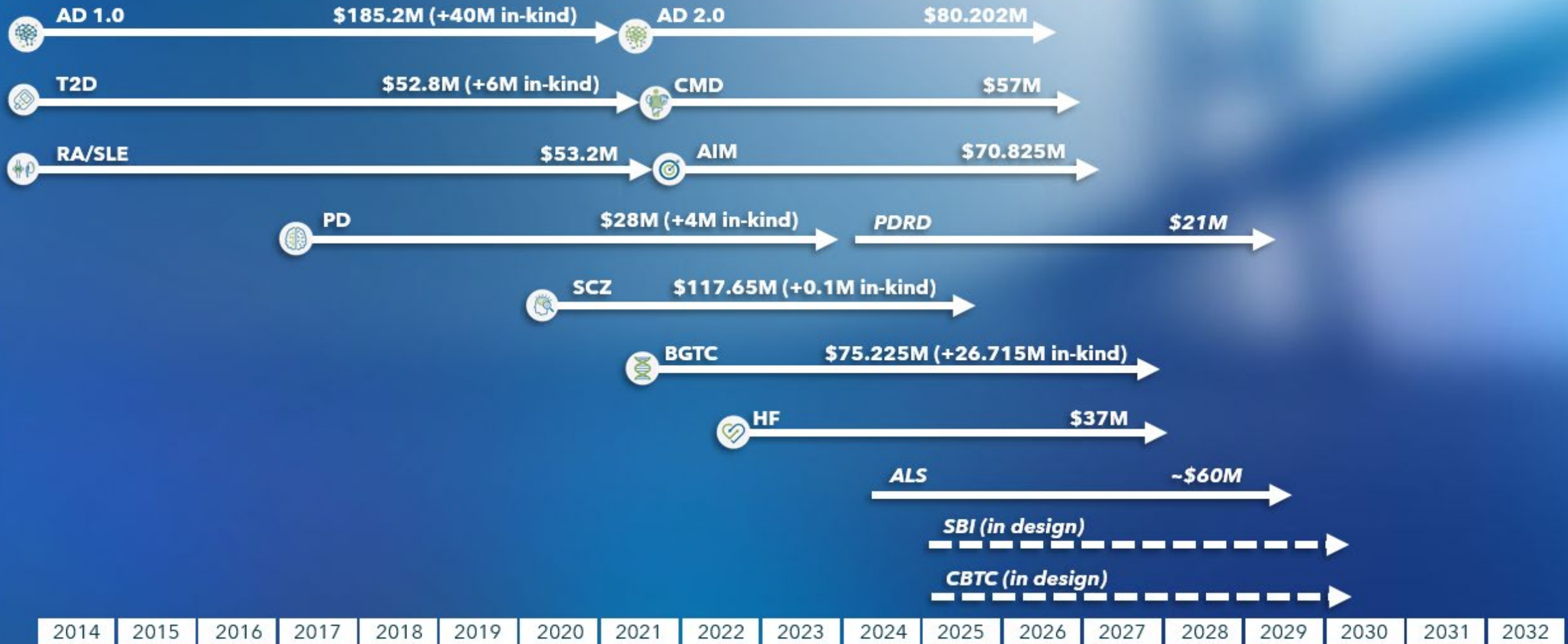
Precompetitive public-private collaboration started in 2014



- **Unite resources of NIH and private partners to improve our understanding of disease pathways and transform current models for developing new treatments by:**
 - Identifying new targets, biomarkers and development paradigms
 - Developing leading-edge tools and technologies
 - Collecting large scale datasets and supporting analytics for open analysis by the public
 - Generating consensus platforms and procedures

For an overview of the AMP Initiative, see:
Nature Reviews Drug Discovery - February 27, 2019
<https://www.nature.com/articles/d41573-019-00033-8>

AMP[®] Program Development



AMP[®] By the Numbers

12

Projects

36

Industry Partners

\$915M

Total Investment

16

**NIH Institutes and
cross-institute programs**

10+

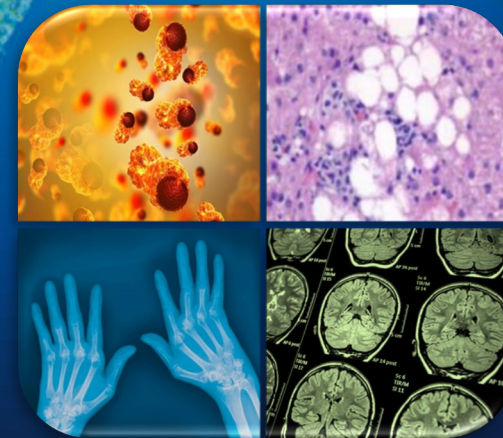
Years

43

Non-Profits

The Biomarkers Consortium

Advancing Precision Medicine



BIOMARKERS
| | | | | **CONSORTIUM**
—
IMPROVING HEALTH THROUGH
MEANINGFUL MEASUREMENTS

Driving principles of the Biomarkers Consortium



- BC projects bridge the gap between basic research and practical needs for advancing drug development and regulatory science.



- Drug development tool projects are developed collaboratively with involvement from academic, government, and industry scientists. Projects can be generated in any therapeutic area and often consult with patients and advocates.



- All work is pre-competitive, and results are released to the public as early as possible.



- All projects have specific, well-defined goals and are milestone-driven, including interim "go/no-go" funding gates.

Biomarkers Consortium

18+ years of collaboration, research, and progress

60+

active partners

40+

projects

\$108M

private funds raised

2

Biomarkers accepted by FDA for use as a surrogate endpoint

1

Qualified composite safety biomarker

14+

Therapeutics advanced based on tools generated

9

FDA Guidance documents supported by work of the BC

9

Clinical tools being used in trials for drug development

>50 publications
800+ citations

Public Private Partnerships: Role of the FNIH

The FNIH convenes the best minds around the world to tackle complex health problems through partnership and collaboration.

GOVERNANCE

Establish and manage a variety of structures appropriate to each partnership

POLICY MANAGEMENT

Provide safe harbor for interactions between companies, government, and academic entities

Policies support NIH ethical and policy standards

PROGRAM MANAGEMENT

Drive stakeholder consensus about appropriate scientific selection and execution of projects

FUNDRAISING & RELATIONSHIP MANAGEMENT

Directly solicit contributions

Steward and manage donor funds

PROJECT MANAGEMENT

Ensure projects meet established deliverables and "go/no go" milestones

INTELLECTUAL PROPERTY MANAGEMENT

Provide "pre-competitive" structures for handling intellectual property, if needed

Potential NAMS PPP

Potential of a NAMs PPP

- Creation of a public-private partnership (PPP) will establish a **community platform and a replicable process for NAMs validation**.
- Partnership with **novel existing technologies**, testing refinement initiatives and regulatory networks, will support development of a **multistakeholder validation process**.
- Provision of **process recommendations and public guidance to support implementation of NAMs** will complement existing models used in biomedical research.
- Collection and dissemination of **training modules** will support education and adoption of NAMs in the broader community.

Complement-ARIE

Better model and understand human health and disease outcomes across diverse populations.

Develop NAMs that provide insight into specific biological processes or disease states.

Validate mature NAMs to support regulatory use and standardization.

Complement traditional animal models and make biomedical research more efficient and effective.

<https://commonfund.nih.gov/complementarie>

Role of the NAMs VQN Steering Committee

Work with the Complement-ARIE to collaborate on a VQN Design Phase

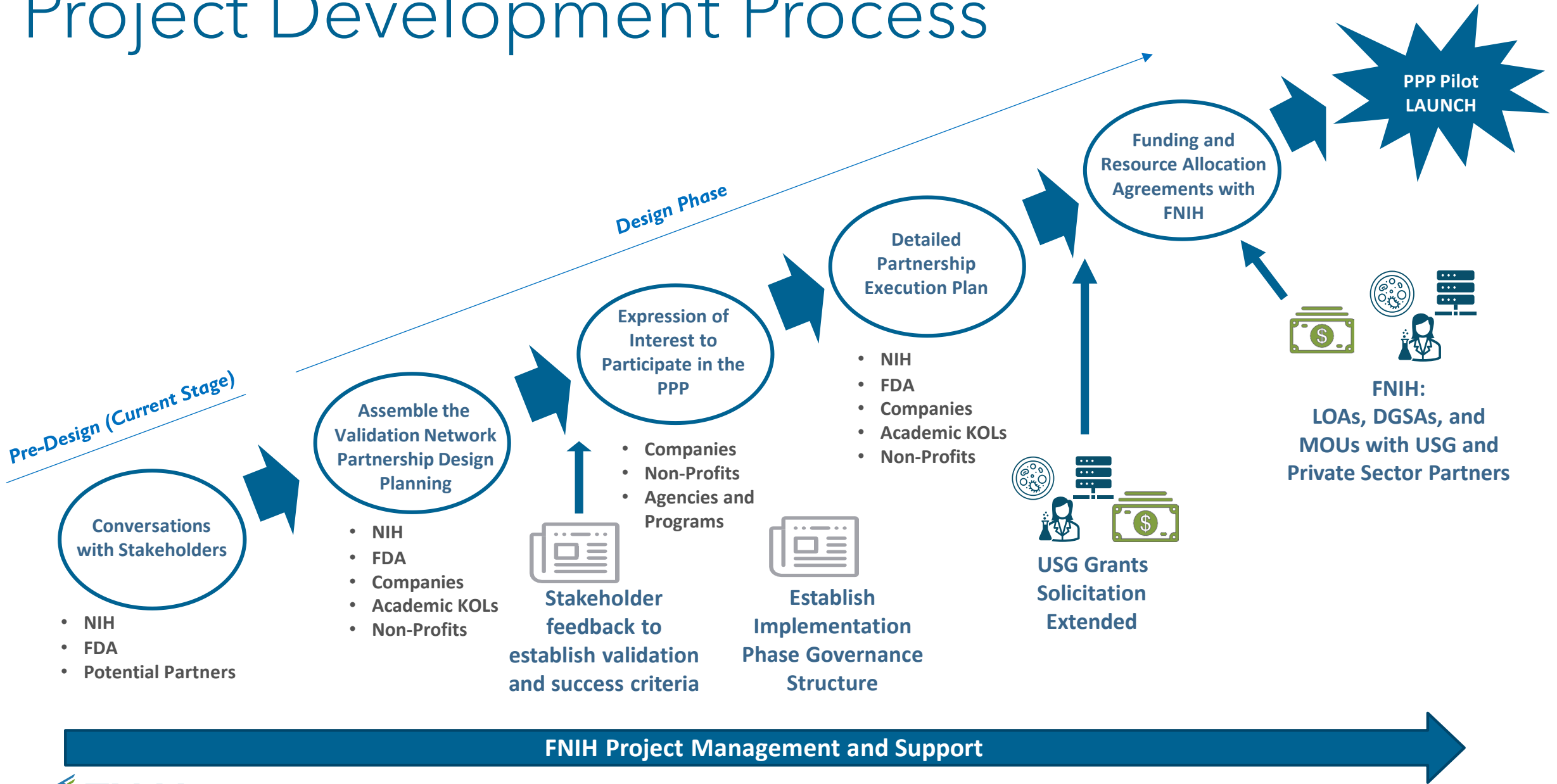
FNIH has launched in the NAMs VQN Steering Committee (SC) in advance of the RFC for the following purposes:

- Stand up working groups to focus on development of project proposals in the NAMs area that could collaborate with Complement-AIRE or operate through existing BC, AMP and other mechanisms
- Build a set of cohesive projects validate and support adoption of NAMs

SC will be instrumental for:

- Proposal Evaluation
- Project Development
- Assembling Funding and Partnership

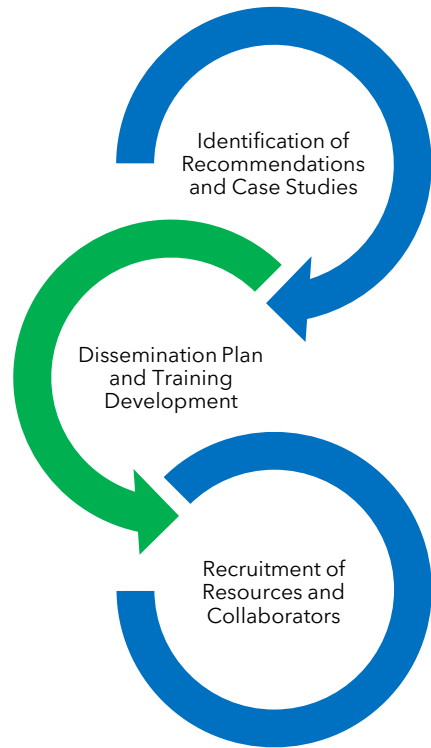
Project Development Process



Design Phase

2024 - 2025

Validation Network
Convening and Phase 1
Design



- Develop success criteria: Establish minimum information for identification of NAMs for validation and/or qualification.
- Develop governance structure: Plan, develop network with FNIH, Industry, NGOs, federal partners and regulators.
 - Identify primary “customers” and associated regulatory agencies
 - Conduct workshops and other activities to identify industry/agency priority needs
 - Seek feedback on scope of validation and qualification efforts
 - Define pre-competitive data sharing capacity for stakeholders
- Issue RFPs to solicit nominations of “late-stage” NAMs to address priority needs.

Design Phase Activity

- Convene workshop with industry, academia, NGOs, CROs, and federal partners:
 - FDA, EPA, NSF, ARPA-H, BARDA, VA, DARPA, NIST, NASA, ICCVAM, 3Rs Consortium, IQMPS, HESI, C-Path, HIS, industry and civil society partners.
- Identify and confirm interest of potential industry and federal partners, with a focus on pre-competitive and collaborative approaches
- Coordinate with ICCVAM workgroup to identify regulatory needs and support validation efforts.
 - Identify existing recommendations and international standards for dissemination.
- Determine scope of validation efforts, data-sharing, and reporting standards.
- Identify funding streams: lab work, chemical sourcing, data analysis, reporting, peer review, etc.
- Governance/criteria for selection of use cases - refine goals for the network.
 - Establish Steering Committee and Working Groups to initiate recommendations
 - Review training efforts for educational dissemination

White Paper Development

- An aggregation of interest and scope
- An advertisement for inclusion
- A baseline for negotiation
- A project team charter with:
 - Budgets
 - Timelines
 - Scoping parameters
 - Milestones
 - Expected outputs

Budgeting

- Design Phase
 - Requests NIH to work across agencies to solicit \$300,000 in contributions
 - FNIH will work with private sector partners to request \$200,000 in industry support

Design Phase Budget	
Personnel	\$232,656
Meetings & Travel	\$150,000
FNIH Direct Costs	\$382,656
Indirect Costs	\$76,531
Total Design Phase Cost	\$459,187

- Implementation Phase Plan Expects:
 - \$3-4M per year in Public Funding
 - \$6-8M per year in Private Sector

Decide How to Assemble Working Groups

- Technology
 - In chemico
 - In silico
 - In vitro
- Sector
 - Bio-Medical
 - Chemical
 - Environmental
- Use-Case
 - Safety
 - Toxicology
- Therapeutic Area
 - Cancer
 - Metabolics
 - Inflammatory Process
 - Neuroscience
 - Rare Disease
- Tissue
- Species
- Other?

Next Steps

- Current ongoing efforts:
 - FNIH Polling NAMs VQN SC participants for feedback on working group structure
 - Soliciting support from private sector partners and interest in continued participation
- Q4 2024
 - Establish Working Groups and self-select membership
 - Re-engage the full Steering Committee (SC) on a bi-monthly basis to assess progress
 - Work with Complement-AIRE on the pending documentation for collaboration
- Q1 2025 – Begin Design Phase in earnest

Discussion

THANK YOU