



**PARIS  
MASH  
MEETING**

**10<sup>th</sup> edition**

**Organized by  
Arun Sanyal & Lawrence Serfaty**



**September 5 & 6 2024  
Institut Pasteur, Paris**

# Inclusivity and Diversity in Translational and Clinical Studies

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# Conflicts

- I have no conflicts to declare

# Including Voices of Persons with Lived Experience has Several Benefits to Drug Development



**Improved Outcomes:** Treatments are more likely to meet patient needs and improve quality of life



**Building Trust** in Healthcare Systems



**Increased Adherence:** When patients are involved in the development process, they are more likely to adhere to treatment regimens



Regulatory and Reimbursement Support

# Challenges – We all agree it's the “right thing to do” but its difficult and ROI is often unclear



Difficult to Engage Patients and Caregivers, Often an Afterthought



Fear that it will Slow Down the Process of Drug Development



Several Competing Priorities



Unclear Guidance on Return of Results and Knowledge

# Pragmatic Case for Patient Engagement

For a pre-phase 2 project, the cumulative impact of a patient engagement activity that avoids one protocol amendment and improves enrollment, adherence, and retention is an increase in net present value (NPV) of \$62MM (\$65MM for pre-phase 3) and an increase in ENPV of \$35MM (\$75MM for pre-phase 3).

**Time:** ENPV increase is the equivalent of accelerating a pre-phase 2 product launch by 2½ years (1½ years for pre-phase 3).

**Money:** NPV and ENPV increases can exceed 500-fold the initial patient engagement investment

Assessing the Financial Value of Patient Engagement: A Quantitative Approach  
from CTTI's Patient Groups and Clinical Trials Project  
<https://doi.org/10.1177/2168479017716715>

# Regulatory Guidance

## 1. EARLY ENGAGEMENT IN DESIGN:

1. **Patient Input:** Involve patients and their families in discussions about disease impact, treatment goals, and outcomes that matter most to them.
2. **Patient-Reported Outcomes (PROs):** Collect data directly from patients about their symptoms, quality of life, and treatment satisfaction.

## 2. INTEGRATION INTO DRUG DEVELOPMENT:

1. **Clinical Trial Design:** Design of clinical trials (including endpoints that reflect what's important to patients), minimize patient burden, devise recruitment strategies and using advocacy networks to reach patients
2. **Regulatory Submissions:** Provide regulators with information about how a drug will impact patients in real-world settings.

## 3. COMMUNICATION AND RETURN OF RESULTS:

1. **Information Sharing:** Clearly communicate trial findings and drug benefits/risks to patients, caregivers, and the public.
2. **Patient Education:** Ensure patients understand their treatment options, including the benefits and potential risks.

## 4. LONG-TERM BENEFITS:

1. **Post-Market Surveillance:** Continue to gather patient feedback after a drug is on the market to monitor long-term effects and improve future treatments.
2. **Patient Advocacy:** Collaborate with patient advocacy groups to keep patient voices at the forefront of drug development.

# 5 Critical Barriers for Underrepresented Communities to Participate in Clinical Trials



MISTRUST



Lack of Comfort with Clinical Trial Process



Lack of Information about Clinical Trials



Time and Resource Constraints



Lack of Awareness

# 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 connects world-renowned NIH researchers with the ingenuity and expertise of leading academic and private sector partners to accelerate medical breakthroughs for patients.

# FNIH Commitment to Patient Centricity

Strategy 1: Build and Expand Innovative Public-Private Partnerships

Objective 2: Exemplify patient and community centricity

**All FNIH projects will provide timely and actionable information for the populations most impacted by the disease. To achieve this goal, we will include patient, care provider and community interests and needs in our programs through meaningful engagement during project design, execution, and dissemination of information.**

# FNIH Patient Engagement Council helps us establish our strategy for meaningful patient engagement



- FDA, Patient Focused Drug Development (FDA PFDD)
- National Institutes of Health (NIH)
- Patient Centered Outcomes Research Institute (PCORI)
- BIO
- National Health Council (NHC)
- Milken Institute (ENRICH-CT)
- National Alliance of Caregivers
- Community Engagement Experts (Grapevine Health, Touch BC)
- National Organization for Rare Disorders (NORD)



# FNIH Patient Ambassadors Program consult on patient centricity and talk about the importance of partnerships



**Carlos Larrauri Talks about:**  
Schizophrenia, Psychosis,  
Mental Health, Access to Mental  
Health



**Terry Pirovolakis Talks about:**  
Rare diseases, being a care  
provider, bucking the trend for  
regulatory approval for rare  
diseases, his son Michael



**Tony Villiotti, Talks about:**  
NASH, Non Invasive Diagnostics,  
Access to and Education about  
Liver Diseases



**Cynthia Chauhan Talks  
about:** Heart Failure, Obesity,  
Renal Failure, Living with  
multiple comorbidities, clinical  
study design, OA



**Sharon King Talks about:** Rare  
diseases, being a care provider,  
bucking the trend for regulatory  
approval for rare diseases, her  
daughter Taylor



**Kima Tozay Talks about:**  
Preeclampsia, women's health,  
maternal health, infant loss,  
healthcare access and equity



**Janet Church Talks about:**  
Autoimmunity, Sjogren's  
Disease, living with  
undiagnosed illnesses,  
leadership



**Eleni Tsigas Talks about**  
surviving pre-eclampsia, infant  
loss, supporting maternal  
health, global health equity



# Key Concepts for Meaningful Patient Engagement



1. **Engaging** advocacy orgs as well as persons with lived experience and care providers
2. **Communication plan** for health literate sharing of knowledge
3. **Resourcing** to ensure active engagement during the project lifecycle (*Project Plan budget should reflect this*)
4. **Patients in leadership roles** (when appropriate) including decision making, authoring manuscripts and presenting
5. **Building trust in communities** and maintaining a continuous channel of transparent information exchange

# **Incorporating Perspectives in Translational and Biomarker Programs**

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# Directives for Patient Engagement in New FNIH Programs



1. **Early Engagement** with PWLEs, PAOs to effectively develop projects and prioritize the questions that should be asked (*participants should be identified by the time a concept comes up for review*).
2. When appropriate, PWLEs and PAOs will be invited to meaningfully participate in **project steering, including leadership and authorship on publications**.
3. **Resourcing** to ensure active engagement during the project lifecycle (*Project Plan budget should reflect this*)
4. Strategy for **communication of results in a “health literate” manner** to people most impacted by the disease. All project plans will address how results will be shared with patients and communities most impacted. This can be done in collaboration with PAOs, PWLEs.

*PWLE: Persons with lived experience, PAO: Patient advocacy organizations*

# Accelerating Medicines Partnerships (AMP) started in 2013 and are highly successful public-private collaborations led by FNIH

**11**

Projects

**37**

Industry Partners

**\$897M**

Total Investment

**16**

NIH Institutes and  
cross-institute programs

**10**

Years

**43**

Non-Profits

# Evolution of Patient Engagement in FNIH AMP Programs

Alzheimer's Disease					
T2D, Common Metabolic Diseases					
Immune and Autoimmune Diseases					
Schizophrenia					
Parkinson's and Associated Disorders					
Gene Therapy					
Heart Failure					
ALS					



Patient Advocacy Orgs



Persons with Lived Experience/Caregivers



Patient Communication Plan and Budget



Patient in Leadership



FNIH Patient Ambassadors

# FNIH MASH Biomarker Studies: NIMBLE

NIMBLE: Non-invasive markers of metabolic liver disease (2019-2025)

NIMBLE



Stage 1 completed, progressing 10 biomarkers for qualification for a diagnostic enrichment COU.

Stage 2 to commence in October 2024.

- NIMBLE 2 Protocol was significantly amended due to input from patients and advocacy organizations
- NIMBLE 2 will be recruiting 400 patients across 3-5 sites; half the sites are in areas with strong Hispanic and Latin populations



Patient Advocacy Orgs



Persons with Lived Experience/Caregivers



Patient Communication Plan and Budget



Patient in Leadership



FNIH Patient Ambassadors



Impacted Communities

# FNIH MASH Biomarker Studies: MASHTrack

Liver-related outcomes study in MASH

NIMBLE	   
MASHTrack	    



Patient Advocacy Orgs



Persons with Lived Experience/Caregivers



Patient Communication Plan and Budget



Patient in Leadership



FNIH Patient Ambassadors



Impacted Communities

**MASHTrack:** Can NITs prognosticate LACE outcomes?

- 2-year prognostic outcomes study. Launching Dec 2024.
- Retrospective study (NASH CRN DB2), reasonable representation of Hispanic population
- Working with NASHKnowledge to communicate results in a health literate manner to impacted communities

*contact me if interested in partnering! [tkamphaus@fnih.org](mailto:tkamphaus@fnih.org)*

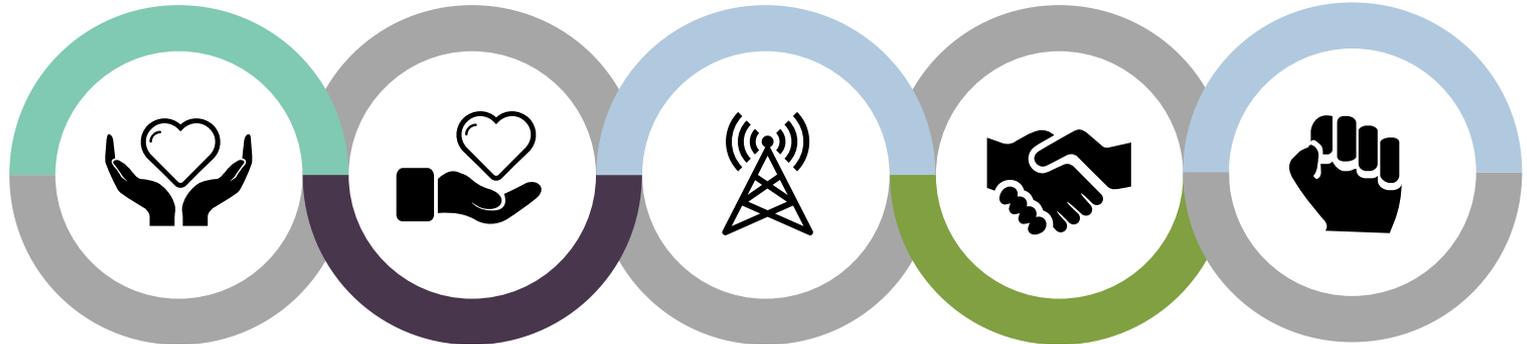


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# Our Aspiration

We believe that patients are at the center of our work, and bringing their perspectives into our partnerships will result in faster medical breakthroughs and improved health & wellbeing



Centricity

To place the patient perspective on equal footing with the voices of other FNIH stakeholders

Expansion

To inform patient groups about the FNIH mission and enable their active participation

Communication

To increase communications about the patient impact of FNIH programs

Inclusivity

To reduce barriers to inclusion of patients and patient groups in FNIH programs

Accessibility

To leverage learnings to improve the diversity, equity and inclusion of FNIH programming