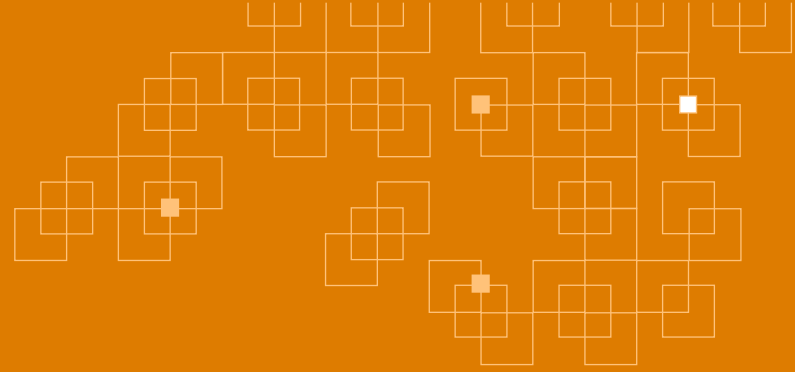




**PEDDLE**

Patient Engagement in Drug  
Development: Leading through Example



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# PEDDLE Working Group: Where We Are, and What's Next

**Founding Co-Chairs:**

**Michael Betel | Fatty Liver Alliance**

**Henry E. Chang | Fatty Liver Foundation**

**Rosemarie Sellati | Regeneron Pharmaceuticals, Inc**

# Disclosures



- **Rosemarie Sellati** is an employee and stockholder of Regeneron Pharmaceuticals, Inc.
- **Henry E. Chang** reports financial relationships with several organizations. He serves as a speaker for the Asian Health Foundation and the Mount Sinai School of Medicine. He is a consultant for Madrigal Pharmaceuticals, Novo Nordisk, and Regeneron Healthcare Solutions, Inc. He also serves on the Board of PharmaNest, a privately held company, where he holds equity shares.
- **Michael Betel** has acted as speaker or moderator for Medscape Education, Novo Nordisk, Boehringer, Mount Sinai School of Medicine and served as an advisory board member for PPD, Worldwide Clinical Studies, Regeneron, WebMD, Sonic Incytes and Madrigal.
  - **Fatty Liver Alliance** has received funding or grants from Madrigal Pharmaceuticals, Novo Nordisk, Regeneron, 89bio, Boehringer-Ingelheim, Perspectum, Aegle Medical, Medscape, Echosens, PPD/Evidera, Siemens-Healthineers, Mindray.

# Overview of PEDDLE: Patient Engagement in Drug Development Leading Through Example



## Purpose

Identify ways to integrate patient perspectives (PE) into MASH drug development processes to enhance outcomes and patient satisfaction

## Short Term Goals

- ✓ Establish a group of experts in PE and drug development to provide guidance and insights
- ✓ Identify current resources and gaps in PE practices to target key areas for improvement
- ✓ Evaluate existing tools to assess their usability across companies of varying sizes

## Long Term Goals

- Create a practical toolkit to guide industry and advocacy partners in implementing effective PE
- Develop Communication and Integration plan
- Establish metrics to assess the impact of patient engagement activities and initiatives
- Advance meaningful PE in MASH drug development by sharing insights and tools through abstracts and publications at key conferences and in peer-reviewed journals

# Multi-Stakeholder Collaboration

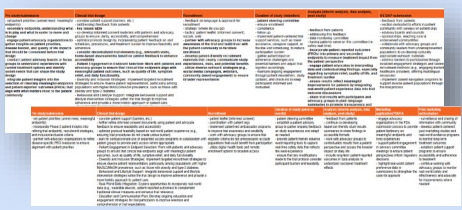


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du Foie Gras




# Laying the Foundation: Turning Patient Engagement into Action

## Milestone Achievements



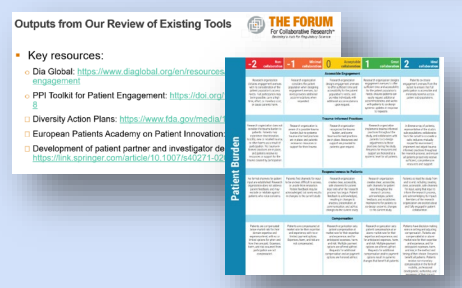
**Mapping Opportunities**

- Identified **key engagement touchpoints** across Phase 2 and Phase 3 development
- Mapped patient/advocate **contributions** alongside current industry practices
- Aligned milestones with **existing regulatory and clinical guidance**



**Establishing Precedent**

- Conducted literature audit of **70 resources** across **12+ therapeutic areas** from oncology to rare disease
- Identified evidence linking early engagement to **cost savings** and **accelerated development timelines**
- Highlighted case studies where patient input **improved treatment design and outcomes**



**Evaluating Existing Tools**

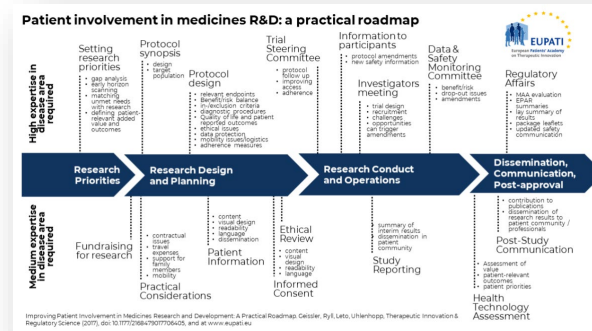
- Audited **tools from established credible organizations** (PCORI, EUPATI, FNIH)
- Assessed **stakeholder involvement** and integration into industry SOPs and guidance
- Evaluates **usability and adaptability** across company sizes and engagement maturity levels

# Building Blocks of the Patient Engagement Toolkit



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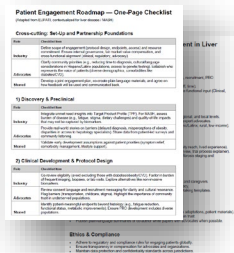
## Schematic of the Drug Development Process *A visual framework showing the various points where patients should be involved*



## Components and Features of the Toolkit

### Checklist

*A concise and actionable tool to enable consistent planning, development, and execution*



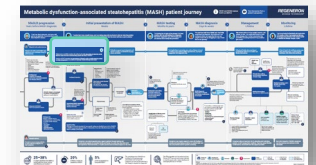
### Guidance Document

*A deeper dive into how to execute the checklist, including references, templates, and linked tools*



### Interactive Resource Hub

*Leverages the schematic as a navigable, interactive tool with easy access to resources, templates and best practices to support real world application*



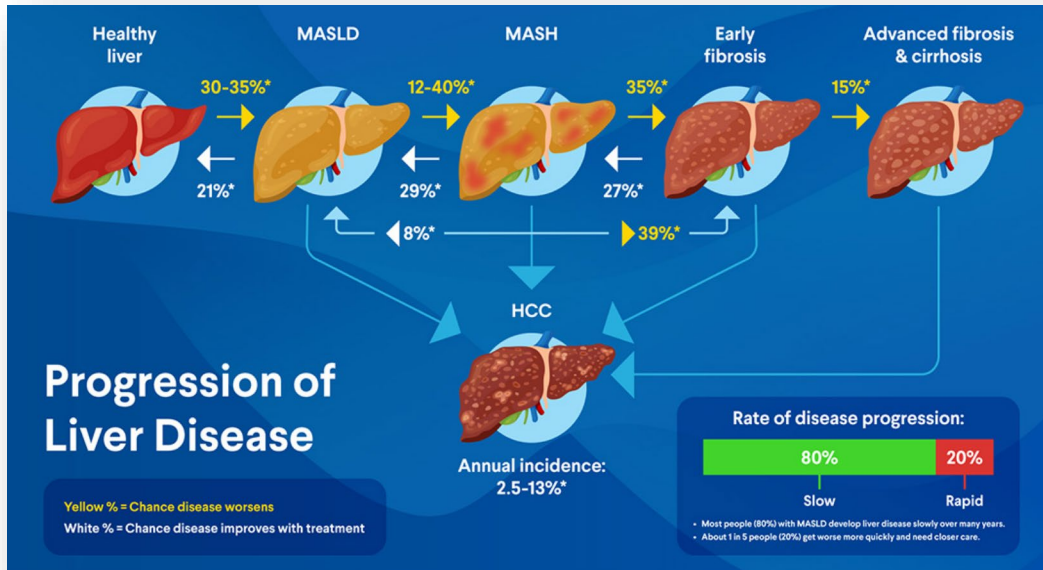


# Interactive Schematic-AACE Obesity and MASH Patient Journey map example



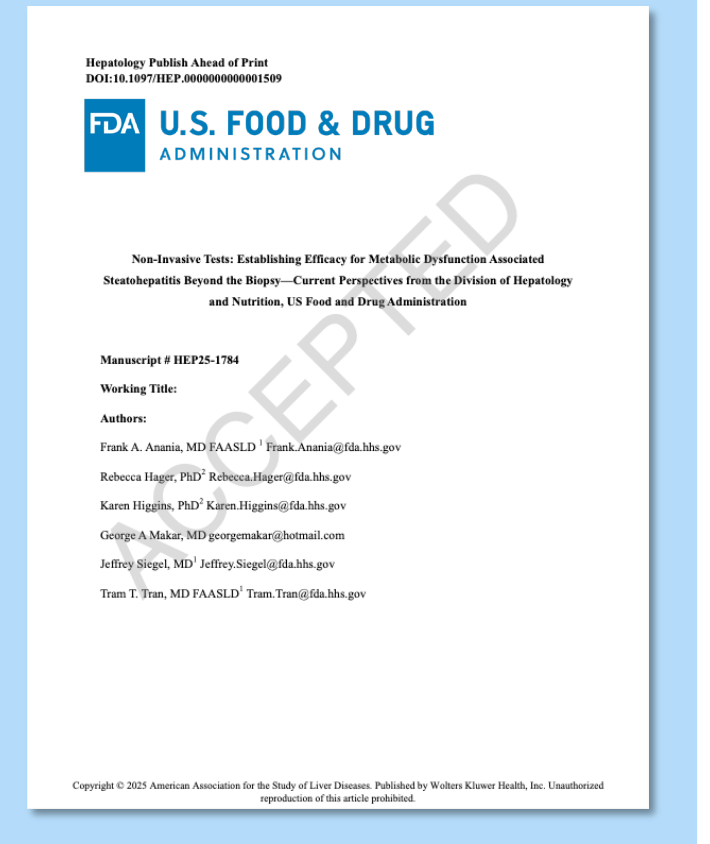
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**Interactive Schematic**— An online version of the schematic where users can click on elements to access additional details, potentially drawing from the guidance document



# Integration: Regulatory and Scientific Interfaces

- The PEDDLE toolkit is designed not only as an advocacy resource but as a **practical interface with regulatory science**.
- FDA's recent communication on **non-invasive tests (NITs)** as reasonably likely surrogate endpoints (RLSEs) marks a pivotal moment in MASH drug development<sup>1</sup>.
- Transitioning from biopsy-based endpoints to NITs has direct implications for:
  - **Trial design efficiency** (reducing invasive procedures).
  - **Recruitment and retention** (lowering patient burden).
  - **Representativeness of study populations** (broadening eligibility).
- PEDDLE's integration focus: ensuring patient perspectives are systematically applied to the **feasibility, trustworthiness, and operational deployment of NITs** within development programs.



<sup>1</sup>Anania, Frank A., et al. "Non-invasive Tests: Establishing Efficacy for Metabolic Dysfunction-Associated Steatohepatitis Beyond the Biopsy—Current Perspectives from the Division of Hepatology and Nutrition, US Food and Drug Administration." *Hepatology*, 2025, doi:10.1097/HEP.0000000000001509.



# Manuscript:

## The 5 Ws of Patient Engagement in Liver Clinical Trials (PEDDLE)



- Who:
  - Patients, caregivers, advocacy groups, sponsors, trial sites, regulators (FDA, EMA), industry partners
- What:
  - PEDDLE Working Group framework for embedding patient engagement into liver drug & diagnostic development
- When:
  - Across the full lifecycle: pre-study, during trial, and post-trial (Phase 2 → post-marketing)
- Where:
  - Clinical trials for liver diseases (esp. MASLD/MASH), where unique barriers exist (stigma, biopsies, long trial durations, few approved treatments)
- Why:
  - To improve recruitment, retention, data quality, diversity, and trust
  - To ensure outcomes reflect what matters to patients (quality of life, PROs, non-invasive diagnostics)

## Manuscript: PEDDLE Framework: Summary

- Why it matters: Patient engagement improves trial enrollment, retention, and data relevance.
- Barriers: Biopsies, stigma, long trial durations, logistical & financial burdens, lack of diversity.
- Core strategies:
  - Patient Advisory Boards (PABs)
  - Co-developed protocols & consent materials
  - Decentralized & non-invasive trial options
  - Patient-reported outcomes (PROs)
- Impact: Faster recruitment, lower drop-out, better data, and trust between patients, industry, and regulators.

# Working Group & Forum Survey: Advancing the PEDDLE Toolkit Together



## Purpose of the Survey

- Collect additional feedback and perspectives from the WG and broader Liver Forum
- Ensure the PEDDLE approach and toolkit are aligned with community needs

## Areas of Input Requested

- Approach & Toolkit Elements – Are the tools practical, adaptable, and impactful?
- Existing Frameworks & Examples – What can we learn from current models?
- Barriers & Challenges – What obstacles exist for integration into real-world settings?
- Marketing & Communication – How can we optimize dissemination, uptake, and awareness?

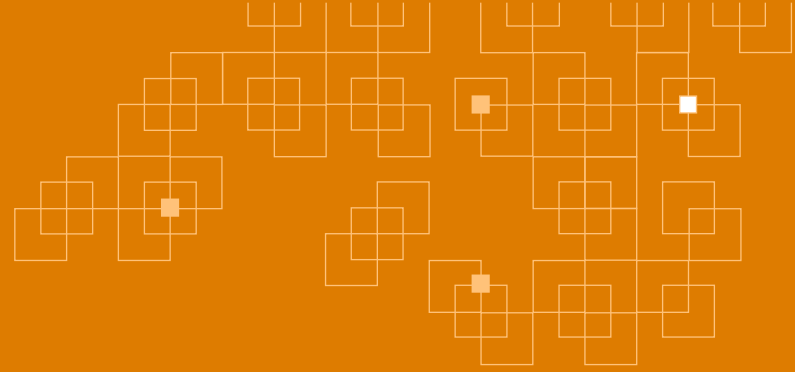
## Next Steps

- Tentative launch October 2025
- Responses will inform refinement of the PEDDLE toolkit
- Results will be integrated into the Liver Forum's broader strategy for patient-centered drug development



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**Thank You**