PEDDLE
Patient Engagement in Drug Development: Leading through Example

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PEDDLE Working Group Update and Toolkit Proposal

April 4, 2025

Founding Co-Chairs:

Michael Betel | Fatty Liver Alliance

Henry E. Chang | Fatty Liver Foundation

Rosemarie Sellati | Regeneron Pharmaceuticals, Inc.

Berkeley Public Health

Agenda

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- **Overview of PEDDLE** and its objectives (Michael)
- Value of Early Patient Engagement and our **accomplishments to date** (Rose)
  - Map precedent and value for early patient engagement in drug development
  - Map key activities and potential impact of PE along the clinical development lifecycle
  - Evaluate existing tools by leading non-profits and Industry experts (EUPATI, ICON, PCORI)
- **Toolkit Proposal and timelines** (Henry)

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PEDDLE Forward: Driving Patient-Centered Innovation in MASH Drug Development

April 4, 2025

Michael Betel | Fatty Liver Alliance

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Disclosures

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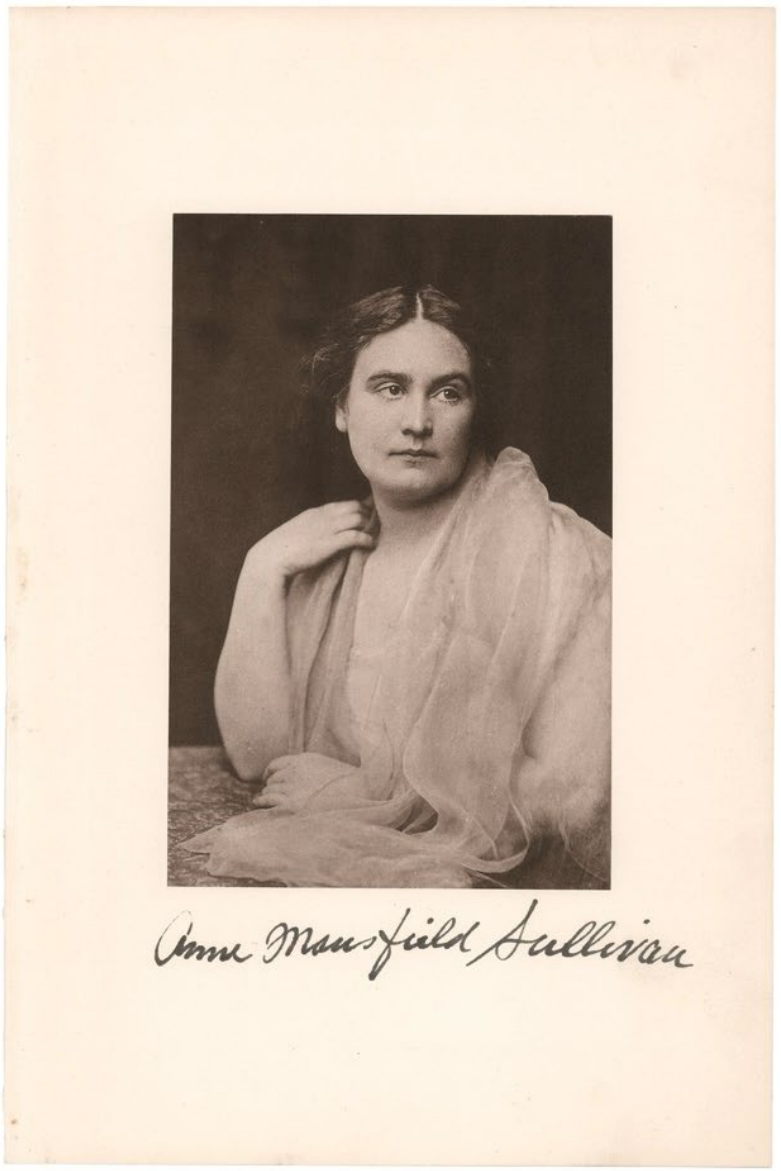
- Speaker or moderator for: Medscape Education, Novo Nordisk, Boehringer
- Advisory board member for: PPD, Worldwide Clinical Studies, Regeneron, WebMD, Madrigal
- Fatty Liver Alliance has received unrestricted funding from: Madrigal Pharmaceuticals, Novo Nordisk, Regeneron, Perspectum, Aegle Medical, Medscape, Echosens, PPD/Evidera, Siemens-Healthineers, Mind-Ray
- No conflicts related to the content of this presentation



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"Alone we can do so little; together we can do so much."

Purpose

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- Emphasize the critical role of patient insights in designing effective and patient-centric MASH clinical trials.

- Identify ways to meaningfully integrate patient perspectives into MASH drug development processes to enhance outcomes and patient satisfaction.

Liver Forum & PEDDLE Alignment of Goals

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1. Centers the Patient Voice in Clinical Trial Design: Meaningful and effective therapies
2. Builds Practical Tools for Stakeholder Engagement: Collaboration
3. Drives Best Practices for Patient Involvement: standardization, clarity, and efficiency
4. Breaks Down Barriers to Engagement: remove roadblocks in drug development
5. Accelerates Development of Personalized Therapies: advancing science-based, personalized care for MASH

Who is Involved?

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REGENERON

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From Ideas to Impact: Our Patient Engagement Journey So Far

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Rosemarie Sellati | Regeneron

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- Regeneron Pharmaceuticals, Inc. employee and stockholder

“New standard” led by regulators, legislatures, advocacy groups

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- Transformational approach to identifying and responding to the unmet needs of patients worldwide
- Global movement building since early 2010s
- Benefits to **patients**, **sponsors** and **society**
- More meaningful clinical trials with **increased feasibility**, **enhanced recruitment / retention**, and real-world **outcomes**
- **FDA Guidance** provides requirements for certain clinical studies

“The informed, empowered patient is becoming the norm.” - Drug Information Association (DIA)

- Food and Drug Administration
- European Medicines Agency
- DIA Patient Engagement
- National Institute for Health Research
- National Health Council
- Patient-Centered Outcomes Research Institute
- European Patients’ Academy on Therapeutic Innovation

Not just the “right thing” to do

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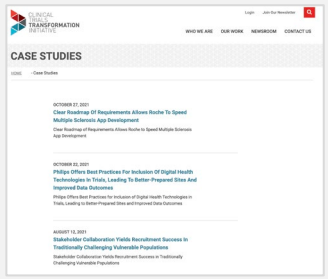
Patient engagement impacts the bottom line

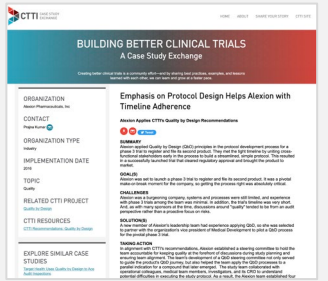
Financial Value of Patient Engagement
Study Results[1]

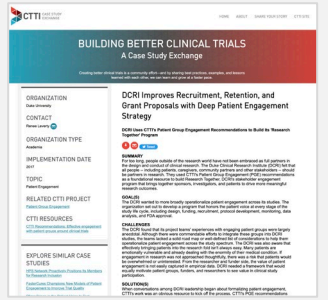
3 Months	**Earlier launch**
25%	**Reduction in enrollment time**
$65M	**Calculated median financial impact**

Dozens of case studies show:

- Quantifiable impact on **revenue**, **cost**, **time**
- Powerful expression of **good corporate citizenship**
- Improvement in **trial design/execution**
- Reduction in study **risks/mitigation** of conflicts of interest







50 percent of confirmatory Phase III trials fail. (Applied Clinical Trials, April 2016)

Estimates vary on the cost of a failed clinical trial, but figures range anywhere from $800 million to $1.4 billion (FDA)

TOTAL MEDIAN DIRECT COST FOR A SUBSTANTIAL AMENDMENT:
Phase II and Phase III protocols
$141,000 & $535,000

1. Impact of a patient engagement activity in Phase 2 Trial that avoids one protocol amendment and improves enrollment, adherence, and retention Bennett Levitan, MD, PhD,1 Kenneth Getz, MBA,2 Eric L. Eisenstein, DBA,3 Michelle Goldberg, MBA,4 Matthew Harker, MPH, MBA,5 Sharon Hesterlee, PhD,6 Bray Patrick-Lake, MFS,7 Jamie N. Roberts, MPH, MA,7 and Joseph DiMasi, PhD2 *Assessing the Financial Value of Patient Engagement, 2017*
Clinical Trial Transformation Initiative

Integrating patient needs, perspectives, and experiences throughout the Drug Development

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*Companies are increasingly conducting patient advisory boards, focus groups, and other methods to engage patients from the earliest stages of drug development and identify **unmet needs**, relevant **clinical endpoints**, and **patient-centric trial designs***

Appropriate Representation in Clinical Trials

- Reassures that new therapies are **effective** and **safe** for all patients
- Ensures that **trials reflect the patient populations** they intend to serve

Incorporating Patient-Reported Outcomes (PROs)

- Ensures that the data collected includes meaningful feedback on how a drug impacts patients' daily lives, which goes **beyond traditional clinical endpoints**

Regulatory Initiatives

- Encourages the **integration of patient input** into the regulatory process, particularly in determining what aspects of a disease matter most to patients
  - E.g., FDA's Patient-Focused Drug Development (PFDD) program

Patient Advocacy Partnerships

- Allows drug developers to stay connected to the **real-world experiences of patients**
  - Acts as a bridge to facilitate **greater engagement in trials** or understanding of the broader landscape of care and treatment

Laying the Foundation: Turning Patient Engagement into Action

Progress to Date on mapping, Measuring and Mobilizing Patient Centric Strategies

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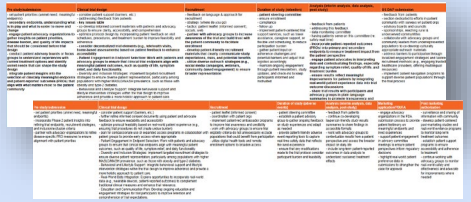
	Milestone Achievements
 **Mapping Opportunities**	• Identified **key engagement touchpoints** across Phase 2 and Phase 3 development • Mapped patient/advocate **contributions** alongside current industry practices • Aligned milestones with **existing regulatory and clinical guidance**
 **Establishing Precedent**	• Conducted literature audit 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

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Moving Forward: Building the Toolkit for Engagement

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Henry E. Chang | Fatty Liver Foundation

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- **Advisory Board**
  - Madrigal Pharmaceuticals, Novo Nordisk, and Regeneron
- **Honoraria**
  - Asian Health Foundation, Mount Sinai School of Medicine
- **Equity Holdings/Stock Options**
  - PharmaNest
- **Grants to Fatty Liver Foundation**
  - 89bio, Akero Therapeutics, Altimmune, Allergan, Applied Clinical Education, Athena Tung, Boehringer Ingelheim, Bristol-Myers Squibb, Celgene, Fibronostics, First Line Creative Media, Galectin Therapeutics, Gilead Sciences, GYRT Health, Healthy Trucking Association of America, Intercept Pharmaceuticals, Madrigal Pharmaceuticals, M3 USA, Merck, Novo Nordisk, Perspectum, Pfizer, Sonic Incytes

PEDDLE Toolkit:
A Practical Resource for All Stakeholders

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Develop actionable toolkit that support integration of patient engagement across the MASH drug development lifecycle

Grounded in precedent: Building from PCORI, EUPATI, FNIH, and other leading resources

Designed for scalability: Tools can be adopted by study sponsors regardless of size or development phase

Initial implementation will focus on the U.S. with plans to extend the toolkit's reach to the EU in subsequent stages

Building the Toolkit: Scope and Strategic Activities

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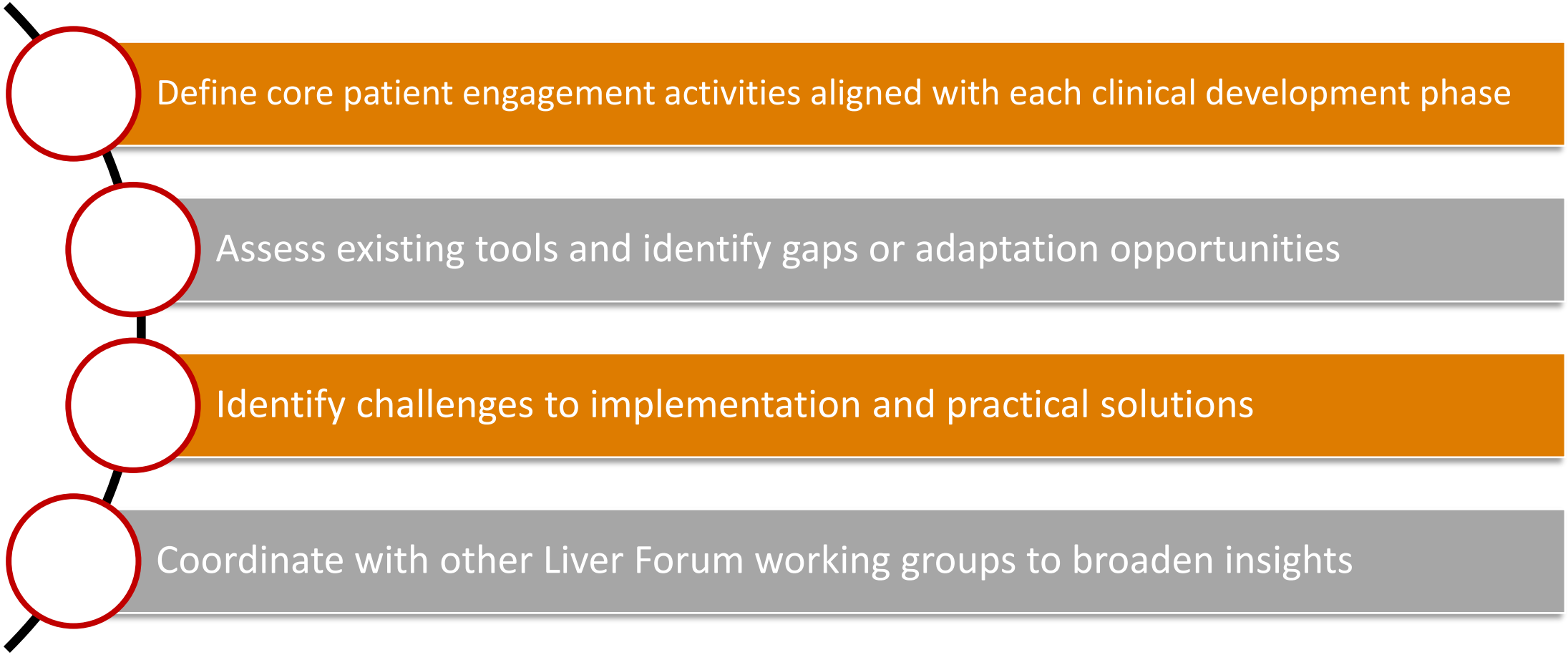

Toolkit Development Timeline: April – September 2025

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Thank You

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