Value of Early Patient Engagement

"New standard" led by regulators, legislatures, advocacy groups

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

- 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 new details on **Diversity Action Plans** required for certain clinical studies

- 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

Value of Early Patient Engagement

Not just the “right thing” to do

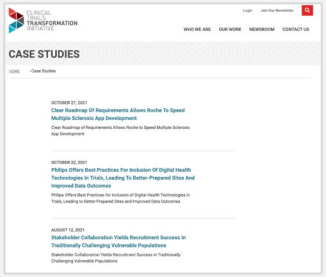
Patient engagement impacts the bottom line

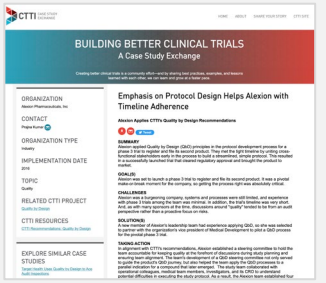
Financial Value of Patient Engagement
Study Results[1]

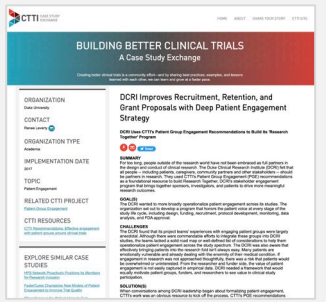
3 Months	**Earlier launch**
25%	**Reduction in enrollment time**
$65M	**Calculated median financial impact**

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*

Case Study-MASH POWER Council

POWER=Patient Organizations Who Empower Results

Overall Goals

- Establish a **Patient Advocate Council** with members representing different perspectives
- Provide **ongoing patient expertise and insights** to inform Regeneron MASH Program strategy
- Disseminate findings to **advance the field of study**

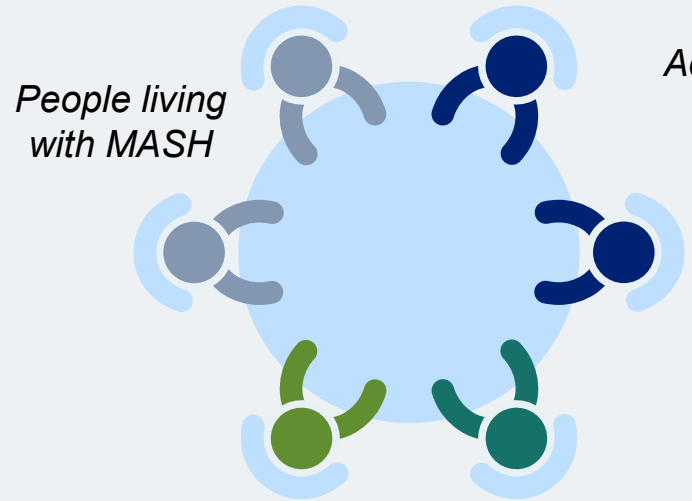
Key Objectives

- Map the **patient journey**, from symptomology through disease management, to define the patient experience from a humanistic perspective
- Identify **barriers to clinical trial participation**, including differences for subpopulations
- Understand **community engagement** models in rural and underserved regions
- Identify high impact opportunities to publish and **share Council insights**
- Build **meaningful collaborations** with patients, caregivers, and community members

Output

- Deliverables include a **patient journey map**, an **educational infographic**, **video testimonials**, and a planned **publication**

Council members providing specific expertise



Case Study-MASH POWER Council

The Council provided insights into awareness and motivation challenges, informing opportunities for meaningful engagement with Hispanic and Latino communities

Lack of Urgency

- HCPs often do not **effectively communicate the severity** of the disease, so patients may not understand the urgency to seek treatment or enroll in clinical trials
- Patients prioritize **managing their comorbid conditions** (e.g., diabetes, obesity, kidney disease, cardiovascular disease) as a more imminent need

Unawareness

- Patients are often not diagnosed until they are late stage and **ineligible for trials** due to a lack of HCP awareness
- There are **few channels** for patients to become aware of clinical trials; notable ones include **HCPs and Patient Groups**

Mistrust

- Marginalized populations may experience a deep mistrust of the healthcare system and clinical trials due to a **history of exploitation and mistreatment**
- The COVID-19 pandemic and anti-vaccination movement reinforced the fear, suspicion, and mistrust of the healthcare system

Cultural Challenges

- Patients may be motivated to enroll in a clinical trial, but receive push-back from their community, because pharma and HCPs are viewed as **"outsiders"**
- In some cultures, the use of **traditional medicine** or **healing** is encouraged over "Western medicine"

"Not putting the unmet medical needs of patients first, early in the development process, can lead to wrong priorities, wrong decisions on research design, and potentially costly late-stage failure." – Clinical Trials Transformation Initiative (CTTI)

THANK YOU

Regeneron plans to continue engaging with the MASH POWER Council and sharing insights with the broader community

AASLD The Liver Meeting®