

# TRACK 7: CLINICAL RESEARCH & TRANSLATIONAL INFORMATICS: TRANSFORMING BIOLOGICAL DATA TO CLINICAL DEVELOPMENT

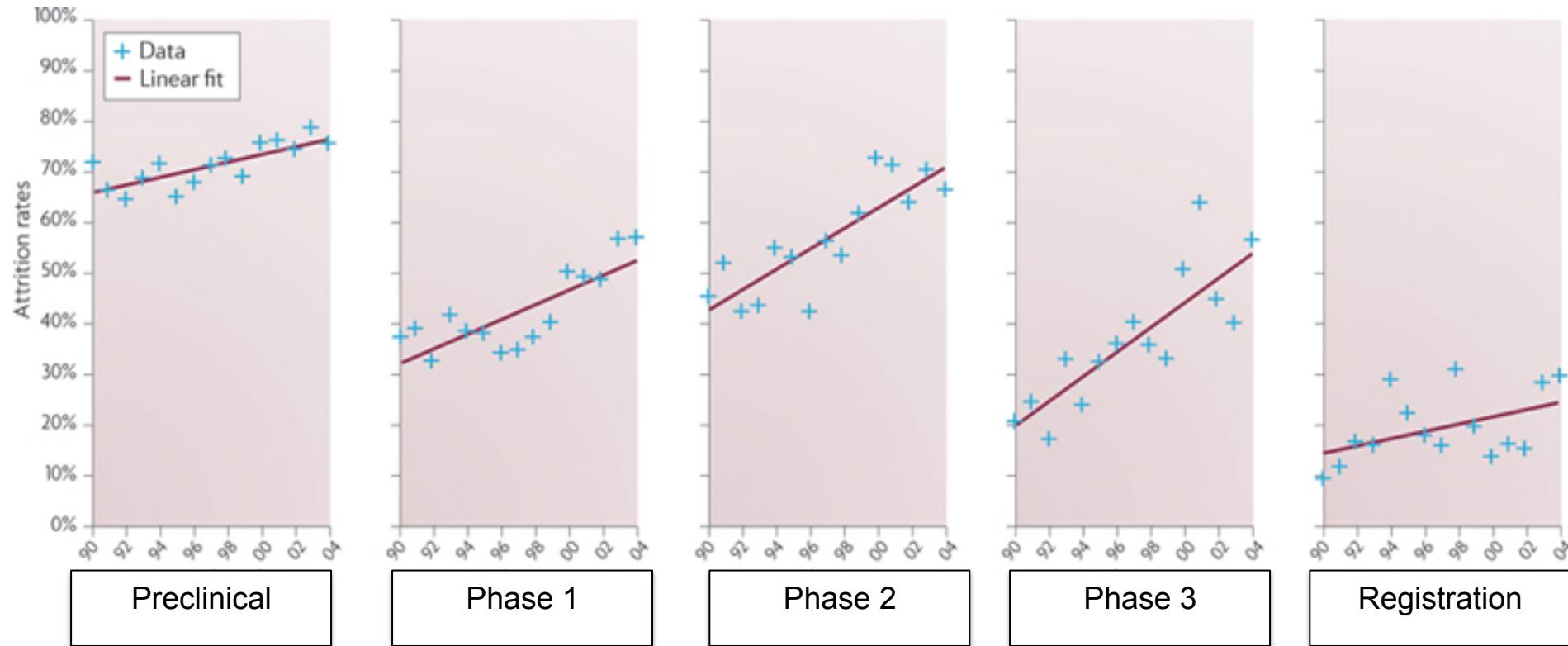
*Wednesday, April 6  
10:50 AM*

**BUILDING FRAMEWORKS,  
USING NEW TOOLS & INTEGRATING SYSTEMS  
TO IMPROVE RESEARCH PIPELINES**

# Session Outline

- 10:50 Chairperson's Opening Remarks  
*Jason Johnson, Ph.D., Executive Vice President, Head of Research & Informatics, PatientsLikeMe*
- 11:00 On the Road to Digital Biomarkers  
*Alain Nanzer, Ph.D., Global Head Safety & Development Workflows, Pharma Research and Early Development Informatics, Roche Innovation Center Basel*
- 11:30 Establishing, Improving and Sustaining Primary and Secondary Clinical Exploratory Research Pipelines  
*Jay Bergeron, Director, Translational and Bioinformatics, Pfizer*
- 12:00 pm New Informatics for New Science: From Data Acquisition to Biomarker Validation  
*Jens Hoefkens, Director, Strategic Marketing, PerkinElmer*
- 12:15 More Than Code: True Interoperability Realized in Translational Medicine  
*Philip Payne, Ph.D., FACMI, Co-Founder, Signet Accel LLC*
- 12:30 Session Break
- 12:40 Luncheon Presentation I: The Intersection of Translational Informatics with Precision Medicine  
*John Shon, Vice President, Data Science and Bioinformatics, Illumina, Inc.*
- 1:10 Luncheon Presentation II: Accelerating Insights in Translational Research: Self-Service Analytics and Visualizations at Amgen  
*George Seegan, Ph.D., Research & Development Informatics, Amgen*  
*David Hardison, Ph.D., Vice President, Health Sciences, ConvergeHEALTH by Deloitte*
- 1:40 Session Break

The setting: The vast majority of clinical candidates fail to translate into drugs, and failure rates are increasing



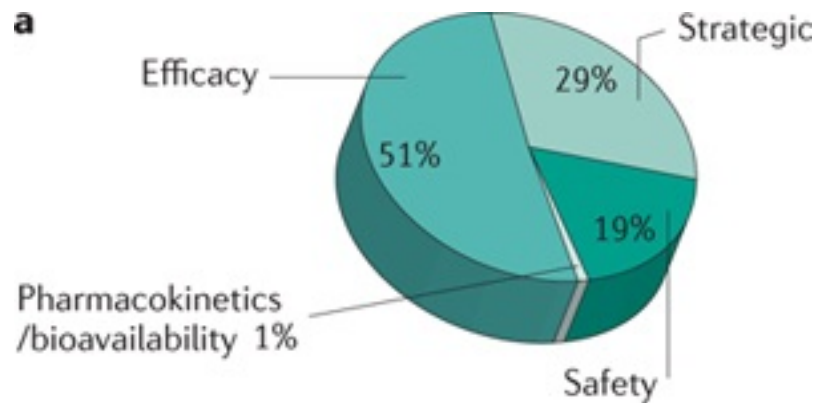
[The productivity crisis in pharmaceutical R&D](#)

Fabio Pammolli, Laura Magazzini & Massimo Riccaboni

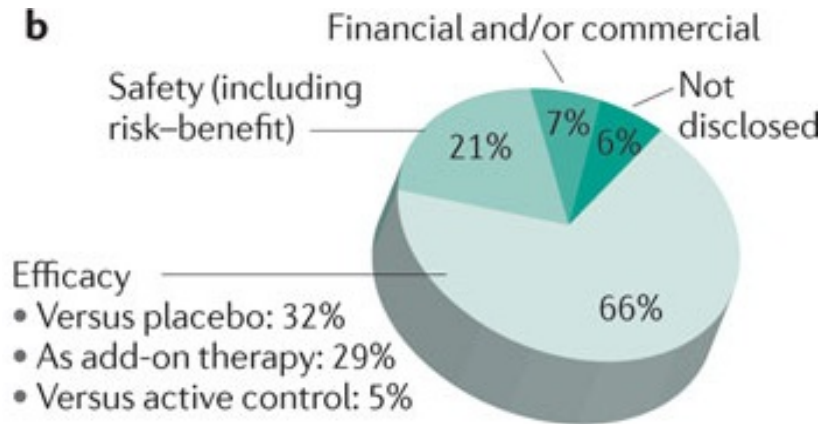
Nature Reviews Drug Discovery 10, 428-438 (June 2011)

28,000 compounds from Pharmaceutical Industry Database

# Most Phase 2 and 3 failures are for efficacy



**Phase II Failures  
2008-2010**



**Phase III and Submission  
Failures 2007-2010**

Phase 2 success rates have fallen below 20%, Phase 3 + submission below 50%

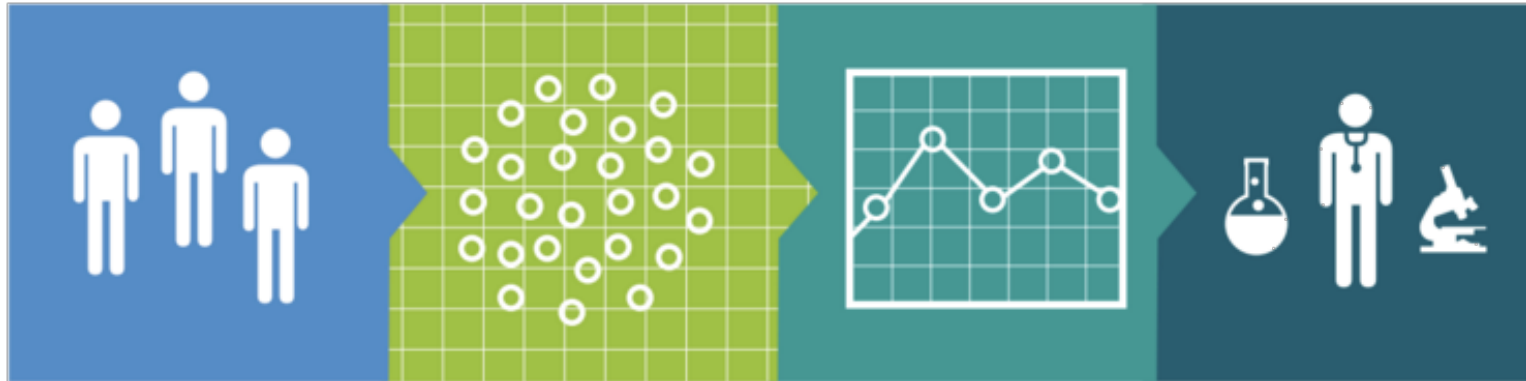
Arrowsmith, *Nature Reviews Drug Discovery* (2011) 10:328; Arrowsmith, *Nature Reviews Drug Discovery* (2011) 10:87;

## Example informatics-based responses to the translational/clinical productivity problem

- 1) Better validated targets, e.g. human genetics
- 2) Better preclinical data integration and modeling/simulation
- 3) Patient segmentation, biomarkers
- 4) Improve trial design, e.g. earlier human testing (fail earlier cheaper), adaptive trials designs for more efficient hypothesis testing
- 5) 'Real-world' data partnerships to better characterize unmet need, improve trial design and recruitment, understand natural history of disease, value of medicines, etc.

# PatientsLikeMe

We partner with leading players in the healthcare ecosystem to help them better understand the patient experience to develop better products, services and care



↑ Patients donate data into the system, and the data are de-identified, aggregated, and contextualized for partner and patient insights ←

## PLM Data Insights

Care  
Management  
(self & provider)

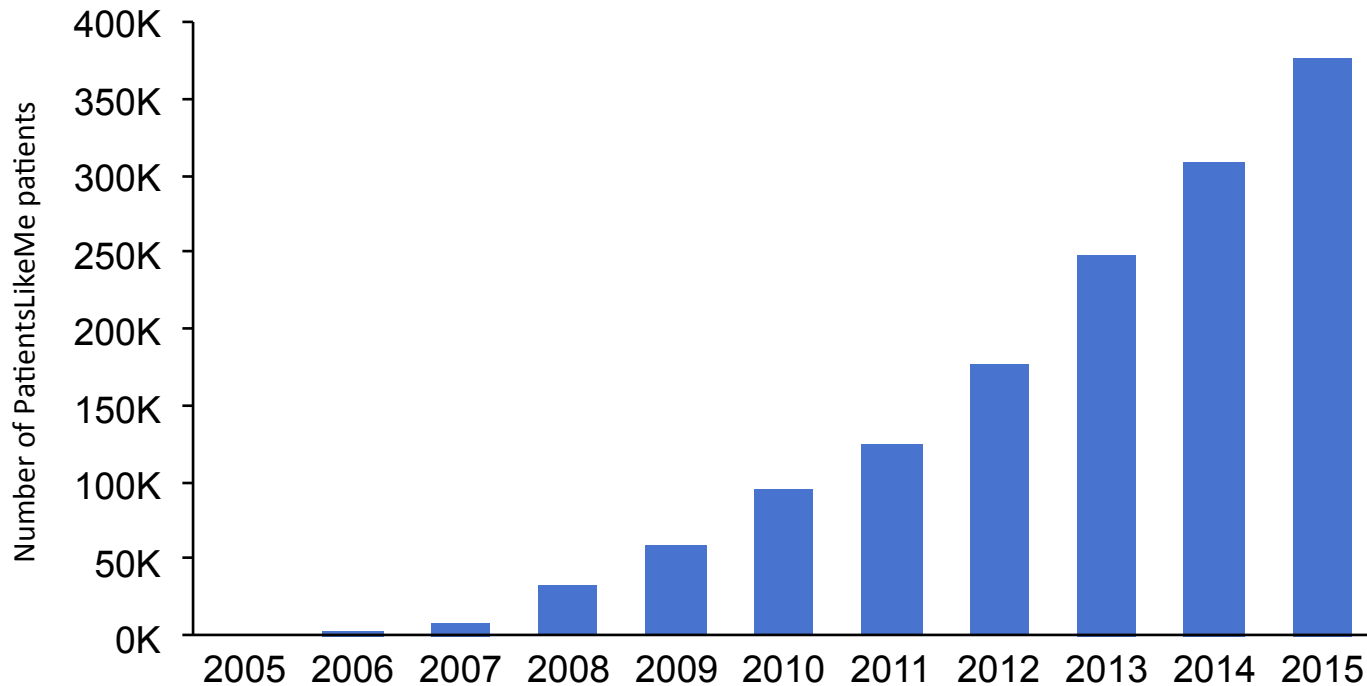
Population  
Health, Access  
&  
Reimbursement

Shared Decision  
Making

CER, PCOR,  
HEOR

Drug Approvals  
& Active Safety  
Surveillance

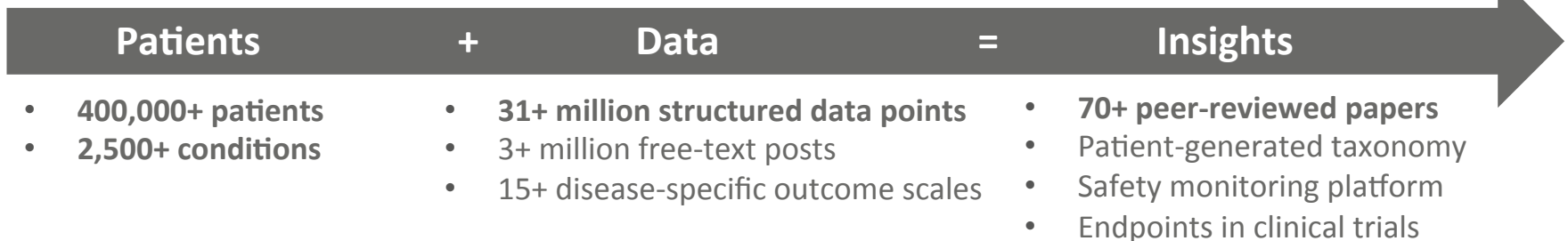
# Building a community of early patient adopters



PatientsLikeMe has been building ongoing relationships with our members since 2006, when we launched our first community for patients living with ALS.

Since then, we have scaled the platform to support increasing numbers of patients, diseases, and data.

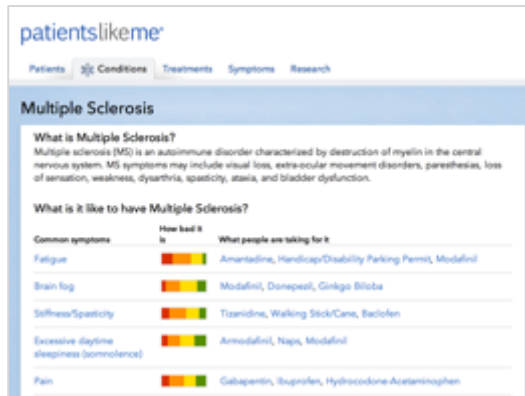
## Where we are today:



# Patient value

## Learn

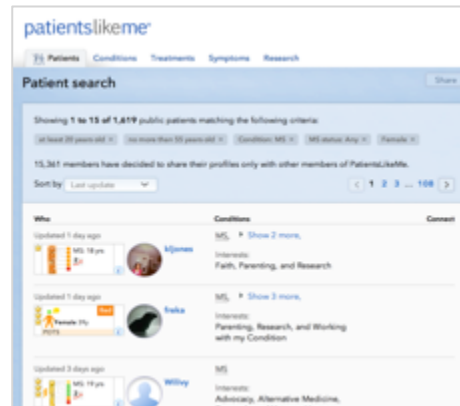
about living with & treating  
life-changing conditions



- Answer the questions, “Is this *normal?*” and “What are my *choices?*”
- Learn from aggregated patient treatment and symptom data

## Connect

with others sharing  
those experiences



- Find patients to connect with based on age, gender, disease, treatment, symptoms, interests
- Get day-to-day support from others to live better, together

## Track

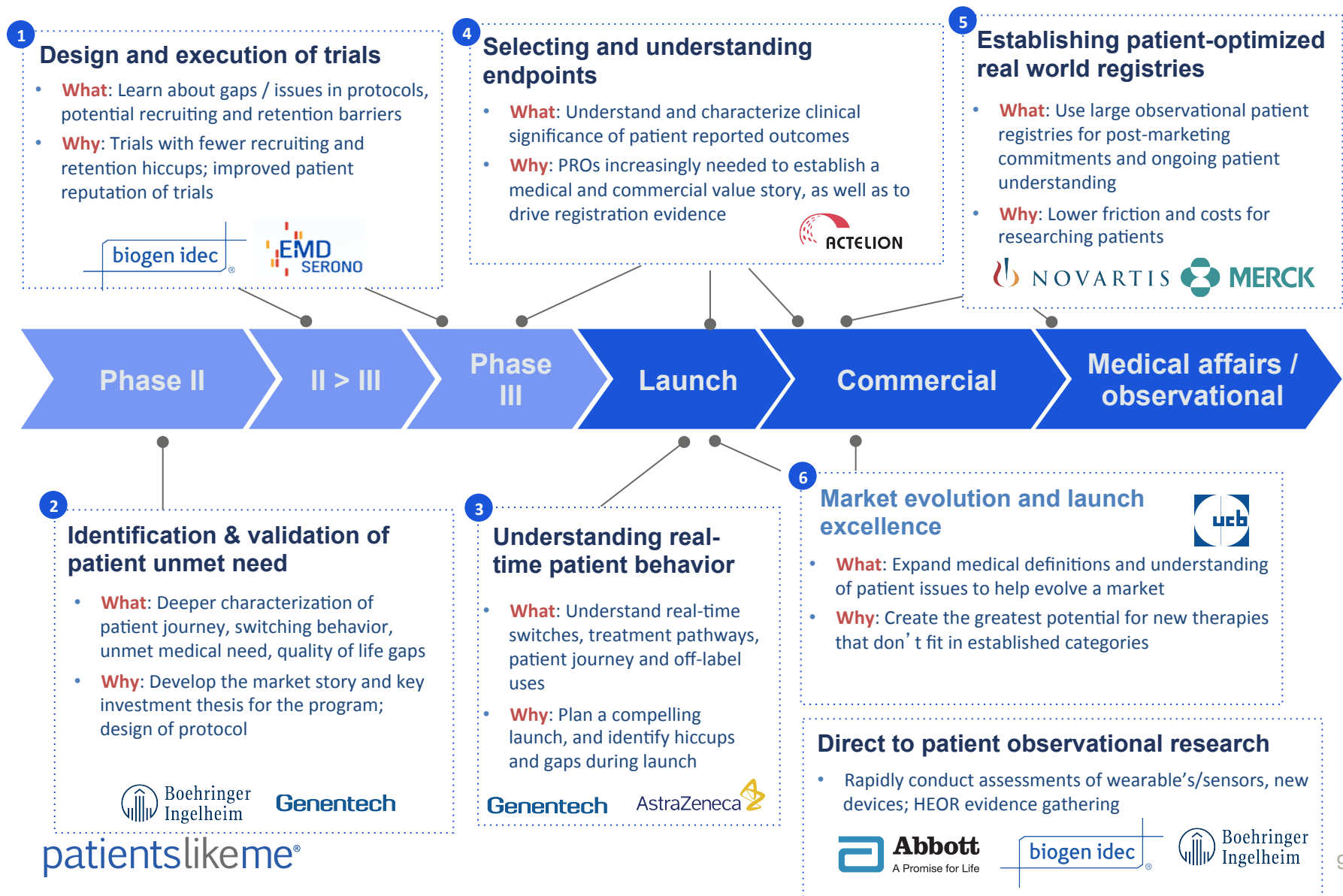
their history and  
progress over time



- Document symptom changes, treatments starts and stops, triggers of exacerbations, etc.
- Generate real-world outcomes data for use by researchers

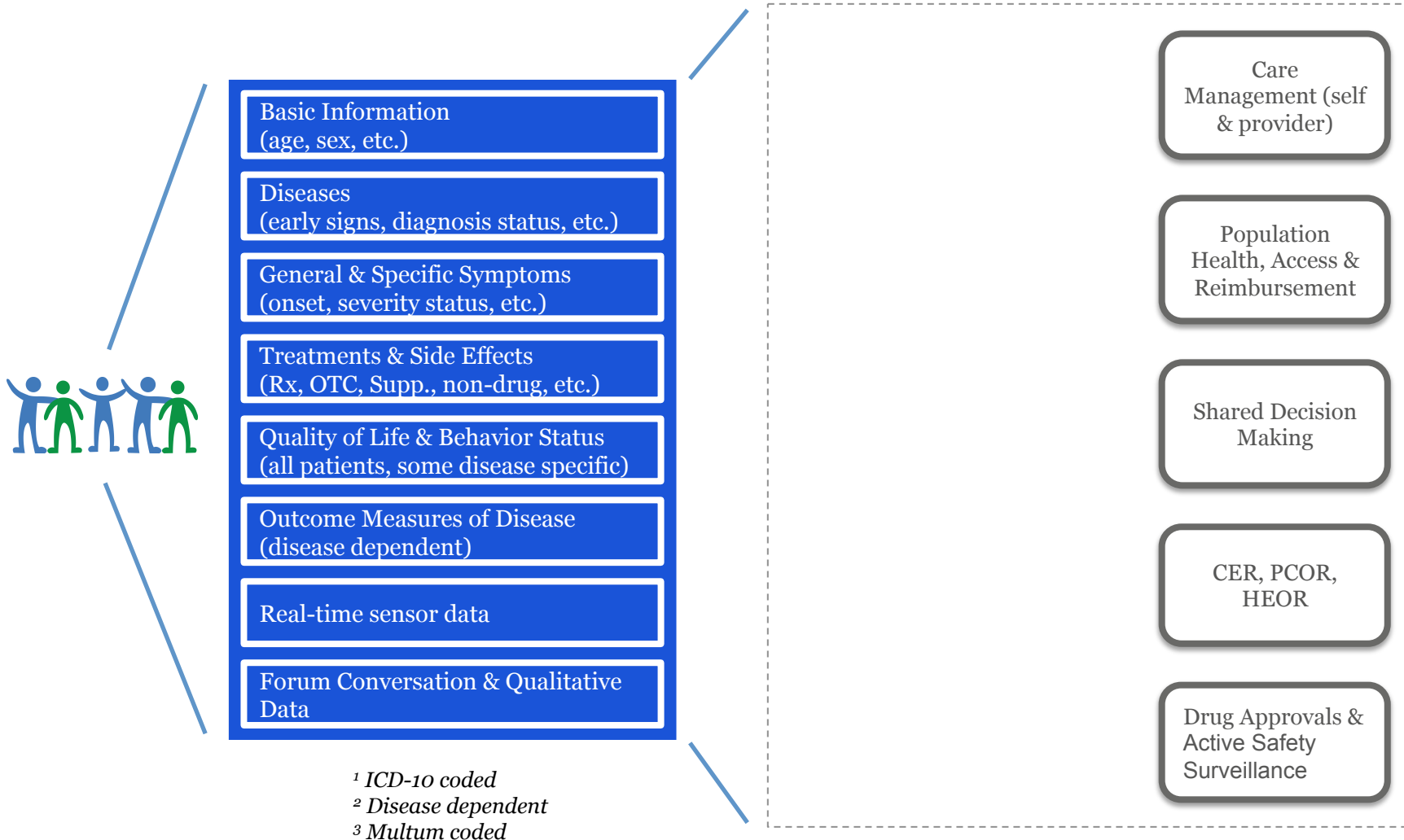


# Examples of how the life science industry has partnered with patients through the medicine discovery, development and delivery continuum



# Platform overview and data layers

Data captured on PLM is expanding beyond patient self-report to provide a holistic view of patient health that is complimentary to traditional medical records



# An Open Research Exchange for Online Patient Feedback in PRO Development

Harrington M, Heywood B, Rura S, Wicks P

PatientsLikeMe, Cambridge, MA, USA



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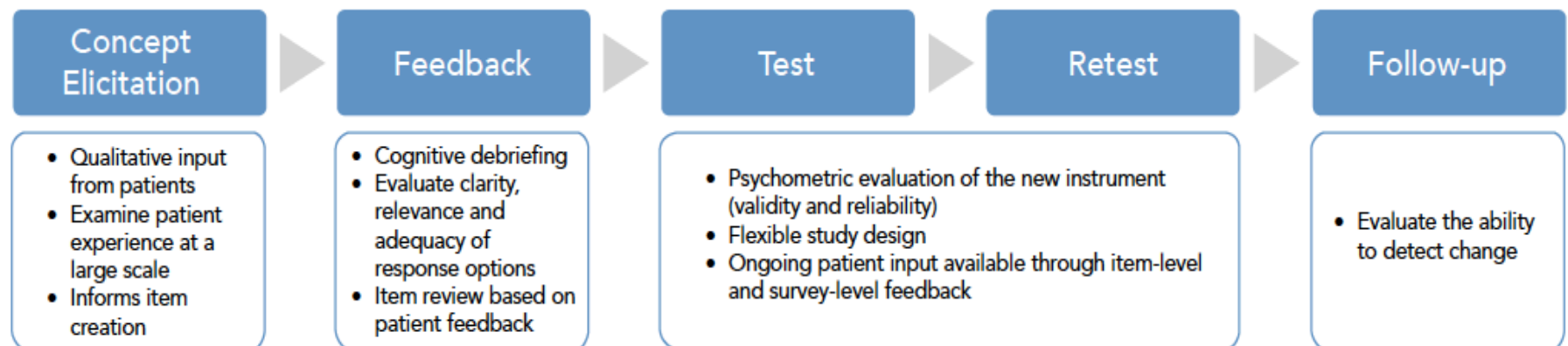
## Objectives

- Patient-reported outcome (PRO) instrument development is a costly and lengthy iterative process.
- Access to patients, logistical difficulties, and the cost involved in getting patient feedback can delay the development of measures that adequately reflect patient experience.
- To address these challenges we built the Open Research Exchange (ORE) software platform.

## Methods

- ORE is integrated with PatientsLikeMe (PLM), an online community for patients who share their data for research and support.
- ORE facilitates iterative and multi-phase development process of a PRO instrument, illustrated in Figure 1.
- Qualitative and quantitative phases are integrated to support an ongoing patient engagement throughout PRO development process. It is accomplished through access to item-level and survey-level feedback questions (see Figure 2 and 3, respectively).
- The effectiveness of the ORE platform was evaluated while developing the Insomnia Impact Questionnaire (IIQ).

Figure 1: ORE's five PRO development phases



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