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Patient *First* Drug Development:

Exploring the patient perspective

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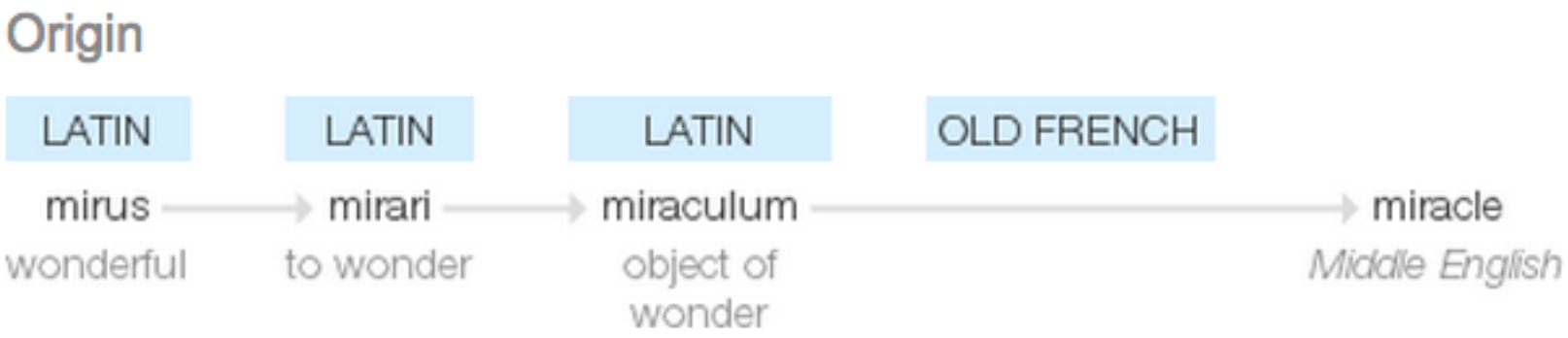
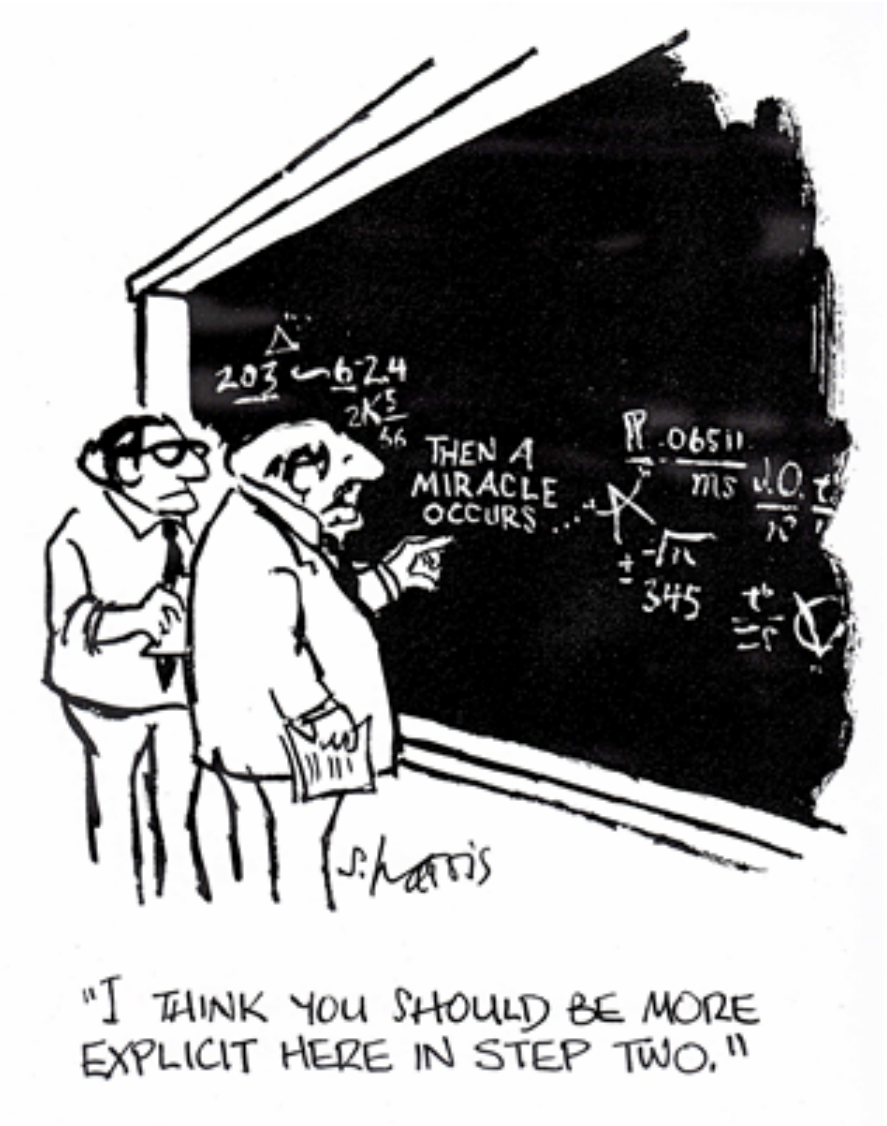
mir·a·cle *n.*

1. a surprising and welcome event that is not explicable by natural or scientific laws

2. a highly improbable or extraordinary event, development, or accomplishment that brings welcome consequences





bedside => bench => bedside...repeat

Patient-informed clinical trials: a pilot survey

Objective: To understand motivations, barriers, and opportunities to enhance clinical trial recruitment for patients with chronic illness through a patient powered research network.

Methods:

- 6,815 members active within previous 90 days
- 9 communities (MS, PD, Fibro, ALS, T2DM, Epilepsy, RA, MDD, SLE)
- 1,621 (24%) completed the survey

Pilot prompts larger study:

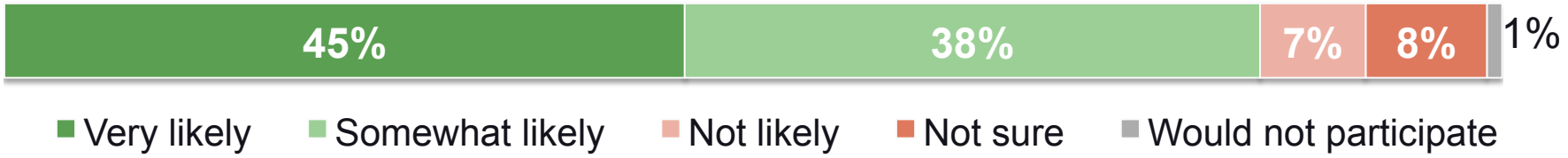
- Trends and differences in demographic and disease subgroups will be further explored in 2015

Source: PatientsLikeMe member survey Feb-Mar 2014

Illuminated roadblocks & missed opportunities

Has your doctor or other healthcare professional ever talked with you about medical research?

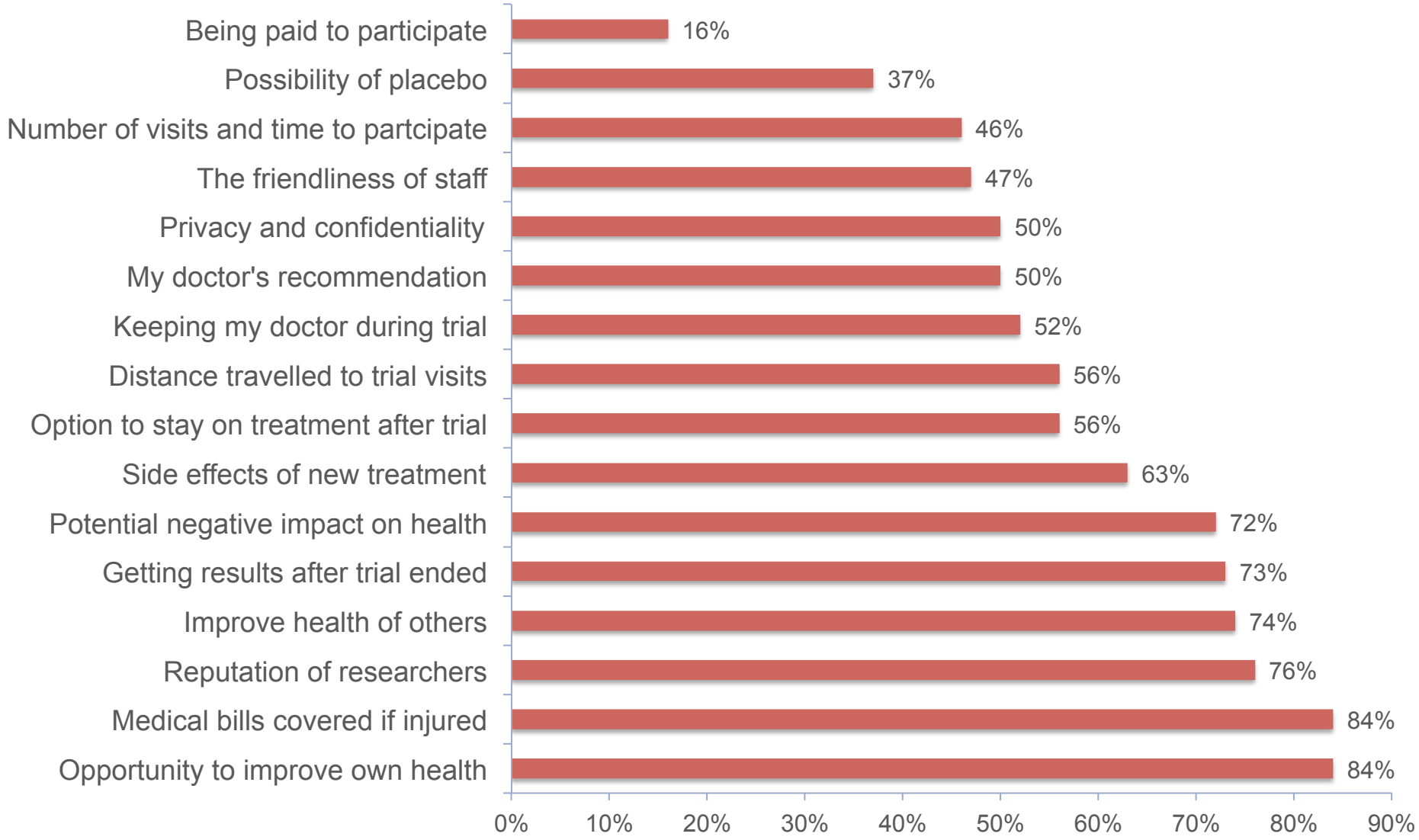
If your doctor found a clinical trial for you and recommended you join, how likely would you be to participate in a clinical trial?



Source: PatientsLikeMe member survey Feb-Mar 2014

N = 1,621

Factors that matter when considering research…



Source: PatientsLikeMe member survey Feb-Mar 2014

N = 1,621

Balancing trial participation with real life concerns

Did you ever consider withdrawing your consent, dropping out, or leaving the clinical trial early?

Why did you consider leaving the trial?

"The pre-trial screener was burdensome and poorly constructed. It worried me that the quality might be indicative of what might follow"

"I grew tired of taking the injections 8 times a week"

"It was for insomnia and required me not to take any sleeping aid. I did not think I could participate for 6 months especially if on placebo"

"All I needed was to have my blood drawn and get it to Fed-Ex…I searched for a facility that would draw my blood but no one would process it so I had to drop out"

"I found out I'd be travelling every 15 days without reimbursement"

Source: PatientsLikeMe member survey Feb-Mar 2014

N = 346

Invitation to share voice with FDA PFDD team…

Megaphone meeting @ the FDA

Hi Sally,

On September 26th, I'm headed off to the FDA for a public meeting on Patient-Focused Drug Development and would love to take what you have to say - your experiences - with me to share. You're just 20 questions away from having your voice heard at the meeting. Click below and let's shout-out your experiences, together.

The FDA is interested in hearing about how IPF impacts your daily life and your views on currently available therapies or treatments.

On behalf of PatientsLikeMe, I will be there to provide comments at the FDA meeting and highlight the experiences of your IPF community, including an overview of the community's answers to these questions. No personally identifiable information will be shared from your responses.

Answer the questions

Thank you for sharing,
Sally Okun, RN
Vice President of Advocacy, Policy and Patient Safety

More about the FDA meeting

This meeting is part of the FDA's Patient-Focused Drug Development initiative, a 5-year project focused on obtaining the patient perspective in twenty disease areas. (I've already presented one with the help of the PatientsLikeMe fibromyalgia community.) For each disease area, the FDA will hold a public meeting to discuss and learn more about how the disease impacts your daily lives, what treatment benefits matter most to you, and your perspective on available therapies for your condition.

Amplifying the voice and experience of many…

"What's daily life like with your condition?"

- Rapid response from thousands of patients about their daily life living with their conditions for FDA PFDD workshops
- Includes the questions of interest to FDA collected using quantitative & qualitative data collection methods
- Survey results submitted to public docket in comprehensive white papers for specific conditions
- Our research-based PFDD framework is amenable to conditions of interest for patient groups, regulators, industry.

A perspective from an engaged and empowered patient

Primary condition: ALS

First symptom: Mar 2010
Diagnosis: Dec 2010

As a person with ALS (diagnosed on 12/8/2010) – I am very much interested in providing the critical ALS patient perspective as a part of the ALS Community decision-making process involving:

- Clinical trial design
- Benefit – Risk
- Approval
- Early Approval
- Accelerated Approval
- Expanded Access

Action: Move definition from *"as is"* to *"to be"*

PFDD is an initiative from the FDA intended to bring patient perspectives into an earlier stage of product development.

The goal is that patients will be able to:

- provide context for benefit-risk assessments and input to review discussions, and
- aid in development of new assessment tools, study endpoints, and risk communications.

Participation: FDA Patient Rep Program

About the program

We are committed to making more opportunities for patients to participate in FDA decision-making. Our Patient Representative Program brings patient voice to the discussions about new and already approved drugs, devices and policy questions.

We recruit Reps on an *as-needed* basis to:

- Help advise us on drugs, devices and biologics that are currently being considered for approval
- Give us input early in the regulatory medical product development and review process.

Role of the Patient Rep

Patient Reps provide FDA with the unique perspective of patients and family members affected by a serious or life-threatening disease. As an FDA Patient Rep, you MAY serve one or more ways. Even if you are selected as a Patient Rep it is possible you will not serve at all. Some of the ways a Patient Rep may serve are:

- On FDA advisory Committees where you will offer the patient perspective, ask questions and give comments to assist committee in making recommendations
- As a consultant for the review divisions
- As presenters at FDA meetings and workshops on disease-specific or regulatory and health policy issues.

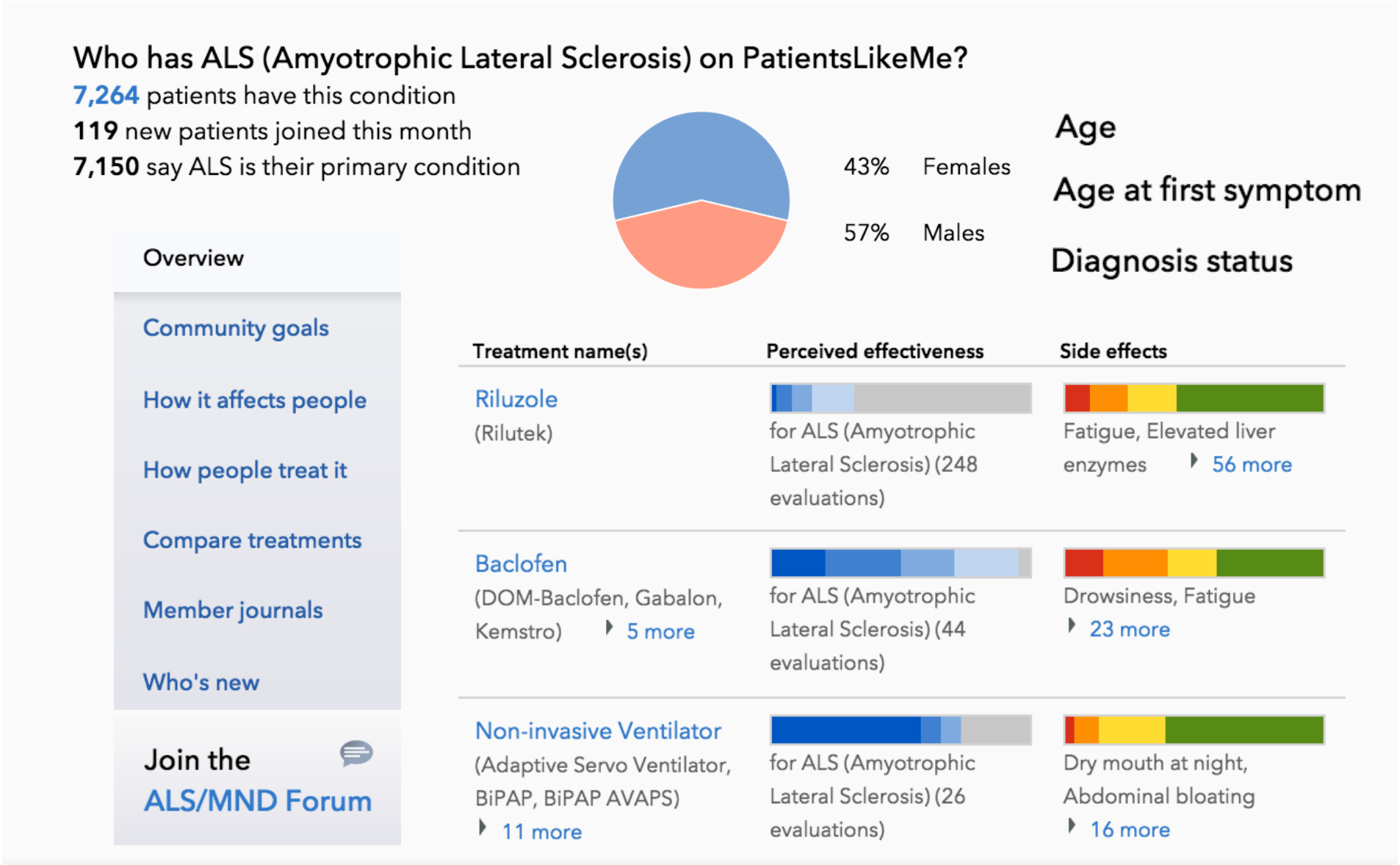
Hear Us: A real example from ALS Community

FDA Accelerated Approval of Genervon's GM604 for Use In ALS

Genervon met with the FDA in February 2015 and made a potentially life-changing request on behalf of the ALS community; they asked the FDA to promote GM604 to the Accelerated Approval Program with Post-Marketing Phase 4 Requirements, so all ALS patients can have legal access to GM604 now. Under the FDA's Accelerated Approval Program, the treatments would be covered by health insurance. Although Genervon knows that this request might complicate their relationship with the FDA, they were willing to take a stand and do everything they can to help the ALS community because it is the right thing to do. The FDA made no final decision during the meeting and we expect that decision to occur in March or April 2015. **If the FDA does not grant Accelerated Approval, it will likely be 3 more years before patients are able to access this drug -- meaning that most people currently living with ALS will not live to see it reach market.**

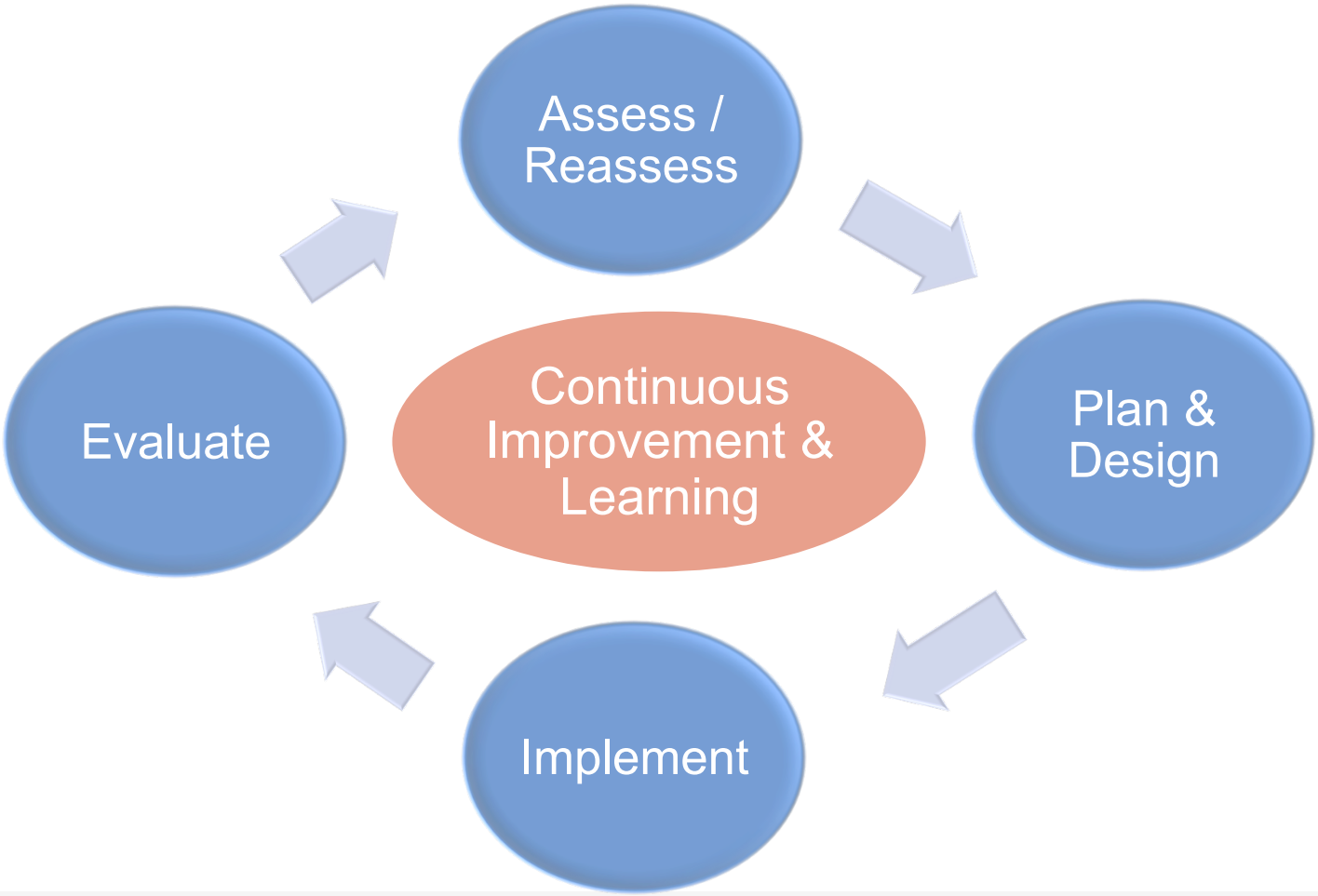
It is one thing to listen … but another to demonstrate with visible results that you actually heard

Scale: seek insight from broader “community”



PFDD "as is" reads well but for it "*to be*" we'll need …

…a plan that supports a collaborative culture of continuous improvement and learning



Continuously improving & learning… TOGETHER!

- Meaningful endpoints
- Real life protocols
- Therapeutic gaps
- Unmet needs
- Natural history
- Targeted recruitment
- Comorbidity influence
- Evaluate satisfaction
- Real world benefit / risk
- PRO development
- Faster bench to bedside
- Heterogeneity impact
- Real world tolerability
- Early safety signals
- Longitudinal view

Patient *First* Drug Development…scaling globally

Engage & Empower

TOGETHER

Etiquette of Engagement

- LISTEN…FREQUENTLY
- HEAR…AUTHENTICALLY
- INVITE…INTENTIONALLY
- ACT…RESPECTFULLY
- ASK…MEANINGFULLY
- SHARE…REGULARLY
- ILLUMINATE…INSIGHTFULLY
- COLLABORATE…CREATIVELY
- MEASURE…RIGOROUSLY

patientslikeme®

To learn,
listen well to
impressions voiced by
patients first.

~ okun 2010 ~