Patient First Drug Development: patients leading the way...

EveryLife Foundation for Rare Diseases
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leading

1. most important *adj.*
2. guidance or leadership *n.*

leading the way

1. To show a course or route by going in advance
2. To be foremost in an endeavor or trend.
About PatientsLikeMe
Our mission is to improve the lives of patients through new knowledge derived from shared real-world experiences and outcomes

• Founded in 2004 as a direct response to family’s experience with chronic disease
• Built as an open, patient facing research based community
• Launched as ALS community in 2005 and opened to any condition in 2011
• Deep patient data and experience in 30-40 chronic life-changing conditions
• It is free to join and there’s no advertising

Our Journey – 10 years in...

<table>
<thead>
<tr>
<th>Patients</th>
<th>Data</th>
<th>Insights</th>
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<tbody>
<tr>
<td>&gt;350,000 patients</td>
<td>25+ million structured data points</td>
<td>60+ journal publications</td>
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<tr>
<td>&gt;2,500 conditions</td>
<td>3+ million free-text posts</td>
<td>Patient-generated taxonomy</td>
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<td>15+ PROMs</td>
<td>Safety monitoring platform</td>
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<td>Open Research Exchange (ORE)</td>
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Diverse personas, similar questions...

Ethnography approach to patient & caregiver journey illuminates insights into common events, feelings and questions.
Patient/caregiver journey framework

- **Having symptoms**
  - Notice something
  - Explain it away
  - Want an answer!
  - Seek information
  - Seek doctor Dx, maybe multiple
  - Maybe many tests
  - Patient knowledge often discounted

- **Seeking diagnosis**
  - Want an answer!
  - Seek information
  - Seek doctor Dx, maybe multiple
  - Maybe many tests
  - Patient knowledge often discounted
  - Lots of info…or little
  - Don’t know what questions to ask
  - May need to make decisions quickly
  - Dx may be surprise
  - Life changes in an instant

- **Getting diagnosis (that you believe)**
  - Seek info (control!)
  - Seek support, acknowledgement, encouragement
  - Share with / involve loved ones
  - Maybe seek activism
  - Maybe new MD(s)
  - MD provides plan
  - Discuss any options
  - Experiment: Effective? Fits life? Change life & work
  - Manage loved ones
  - Manage & logistics
  - Find resources
  - Learn vocabulary, concepts, skills

- **Making sense of it**
  - May see new MD(s)
  - MD provides plan
  - Discuss any options
  - Experiment: Effective? Fits life? Change life & work
  - Manage loved ones
  - Manage & logistics
  - Find resources
  - Learn vocabulary, concepts, skills

- **Finding a plan**
  - Getting better at:
    - Connecting MDs
    - Asking questions
    - Absorbing nuance
    - Trusting self
    - Accepting limits
    - Learning to:
      - Optimize Dx, Tx
      - Make Tx trade-offs
      - Feel capable again
      - Reclaim life
      - Tell story (to process)

- **Optimizing & adjusting**
  - Need less info & support
  - Identity has shifted
  - Some: help others
  - Accept new normal
  - Some: info-browsing
  - Fewer MD visits
  - MD conversations: how are you?
  - Less tracking
  - New Dx may become focus

- **Living with it**
  - Mostly acceptance
  - Depression
  - Lingering fear
  - Resigned…or hopeful
  - “It could be worse”

**QUESTIONS**
- Is this a big deal?
- Should I see a doctor?
- Is this real, or am I overreacting?

**FEELINGS**
- Worry
- Denial
- Avoidance
- Shocked
- Overwhelmed
- Scared
- Relieved
- I can’t deal with this
- I can’t react now

**Disease progresses, new symptoms**
- New town, doctor, insurance, life change
- New diagnosis
- Treatment stops working
- Great new treatment available
Something just doesn’t seem right…

EVENTS

- Notice something
- Explain it away
- Watch and wait
- May seek information

FEELINGS

- Worry
- Denial
- Avoidance

QUESTIONS

- Is this a big deal?
- Should I see a doctor?
- Is this real, or am I overreacting?
Telling the story...

EVENTS
- Want answers to explain what’s happening
- Seek doctor, maybe more than one
- May have tests
- Patient’s knowledge often discounted

FEELINGS
- Worry continues – isn’t this urgent?
- Frustration – no one seems to be listening
- Helplessness – can they treat what they can’t diagnose?

QUESTIONS
- Is this the right doctor to diagnose me?
- Does someone specialize in this?
- Am I crazy? Am I alone?
Hearing the news...

- **Amount and quality of information varies**
- **May need to make decisions quickly**
- **Diagnosis may be surprise**
- **Life changes in an instant**

- **Shocked, overwhelmed, scared**
- **Maybe a sense of relief**
- **I can’t deal with this**
- **I can’t react now**

- **Are they sure this the right diagnosis?**
- **Why me?**
- **What will it do to me – will I die?**
Getting beyond diagnosis...

**EVENTS**
- Seek info (control)
- Share with loved ones
- Seek acknowledgement, support, encouragement
- Maybe get activated
- Maybe find new team

**FEELINGS**
- Scared, vulnerable
- Overwhelmed, grieving
- Worried about loved ones
- Alone
- Sole focus: “I have____!”, “What can I do?”

**QUESTIONS**
- What can I do?
- What does diagnosis mean?
- Do I want to know?
- Tell: who, when, how?
- How will life change?

**Finding a plan**
- MD provides plan
- Discuss any options
- Manage loved ones
- Manage work, school, life, etc.
- Find resources
- Learn vocabulary, concepts, skills
- Feeling: Scared, vulnerable
- Identity: in flux
- Confused
- Control: out of reach
- Want independence, strength

**Experiencing a change**
- Still in shock
- Identity: is in flux
- Confused
- Control: out of reach
- Want independence, strength

**Living with it**
- What treatment is best for me?
- Which doctor?
- Where is the best care?
- How will I manage the details?
- Can I afford this?
I can do this...with help

**EVENTS**

- Disease progresses, new symptoms
- New town, doctor, life change
- New diagnosis
- Treatment stops working
- Great new treatment available

**FEELINGS**

- Less overwhelmed
- More confident
- Less vulnerable
- Tired of educating others including HCPs, loved ones

**QUESTIONS**

- What do others do?
- Will I need more treatments – what will they be like?
- What hope is there?
- Will I ever be like I was before this diagnosis?

**Getting better at:**

- Connecting HCP’s
- Asking questions
- Absorbing nuance
- Trusting self
- Accepting limits

**Learning to:**

- Optimize diagnosis & treatments
- Making treatment trade-offs
- Being capable again
- Reclaiming life
- Tell story to help process

**Getting diagnosis**

(that you believe)
From here to there...

**EVENTS**
- Having symptoms
- Seeking diagnosis
- Getting diagnosis (that you believe)

**FEELINGS**
- Mostly accepting
- Depression
- Lingering fear
- Resigned…or hopeful
- “I suppose it could be worse”

**QUESTIONS**
- How am I, really?
- How will I be, really?
- Is there more coming?
- Will a time come when I won’t need to think about this anymore?

**For many:**
- Need less info and support
- Identity has shifted
- Begin accepting new normal
- Fewer medical appointments with different conversations

**Some may:**
- Help others
- Track less, browse more
- Track more, browse less
- Face recurrence, complications, new diagnosis

- Disease progresses, new symptoms
- New town, doctor, life change
- New diagnosis
- Treatment stops working
- Great new treatment available
Measurement principles

Measurement principles should inform and help to create patient-centric health measures.

- **Clear**: I understand what it means
- **Answerable**: It's information I have
- **Efficient**: It respects my time
- **Relevant**: Describes my experience
- **Educational**: What is and isn't part of my condition
- **Harmless**: Doesn't make me sad and symptomatic
- **Actionable**: Helps me change, discuss with MD

Design principles

We exist to:
- Get the data that makes a difference.
- Help people achieve better outcomes.
- Promote informed decisions & actions

Every patient wants us to:
- **See me as a whole person.**
  - My doctors often don't. That's part of the problem.
- **Come with me on my journey.**
  - In different times & places, I need different things.
- **Help me capture my truth.**
  - It bugs me if I can't accurately reflect my own experience.
- **Let me define who is like me.**
  - They share my experiences and values.
- **Help me feel in control.**
  - Life feels out of control; I need to know I'm in charge.
- **Put my needs first.**
  - Address my needs before demanding I address yours.
- **Inspire confidence.**
  - Show me that P/LM is worthy of my effort and trust.
- **Build on what I already want to do.**
  - I already spend time on my health; take advantage of it.
- **Prioritize.**
  - I have limited energy; show me where to put it.
- **Minimize my work.**
  - I may have dexterity, fatigue, cognitive, or memory challenges.
Case Example: evolution of rare disease community

✔ **Pre-2012:** Less than 50 Idiopathic Pulmonary Fibrosis patients

✔ **November 2012:** 2 months of ethnography and user design

✔ **December 2012:** 4 months of feature build begins; patient engagement strategies identified

✔ **Feb 2013:** Large scale engagement activity begins

✔ **September 2013:** Over 1600 IPF patients members

✔ **November 2014:** Two new drug launches

✔ **January 2015:** Prospective monitoring of post-launch patient experience

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**Cumulative IPF patients since Nov 2012**

- **Q1 2013:** 254
- **Q2 2013:** 642
- **Q3 2013:** 1,222
- **Q4 2013:** 1,600
- **Q1 2014:** 1,927
- **Q2 2014:** 2,223
- **Q3 2014:** 2,546
- **Q4 2014:** 2,886
- **March 2015:** 4,001

Source: Data as of March 30, 2015
Prospective post-launch monitoring

For the first time, two medications were approved by FDA on the same day for a rare disease that previously had no available treatments.

Member of IPF community are reporting on their treatment experiences during this unique period with real world post-launch observations.
### Real-world treatment observations: early findings

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<tr>
<th>Category</th>
<th>Findings</th>
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| **Treatment Awareness**   | • Awareness higher for Drug A (67%) than Drug B (44%)  
• 32% of patients not taking any treatment because unaware of them  
• 6% of patients don’t want to take *any* drug for their IPF |
| **Doctor Discussion**     | • Drug A users more likely to engage in decisionmaking with MD (47%) than Drug B (15%)  
• Slowing progression and doctor’s recommendation most important reasons for patient starting treatment |
| **Access Barriers**        | • Patients report issues with patient support services, insurance companies, and VA  
• Some physicians have told patients that treatments are not yet available or did not mention the new-to-market treatments at all |
| **Treatment Experience**  | • Real-world side effects vary greatly from reported rates in trials for both drugs  
• About half of patients on both treatments perceive minimal improvement/efficacy in early usage |
| **Discontinue Treatment** | • 66% of Drug A users and 73% of Drug B users are “not at all likely” to discontinue their treatment  
• Few patients for either drug stopped or reduced their dose due to side effects |
Every step...engage with patients & listen well