Success from Start to Finish: Multi-Stakeholder Engagement

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Disclaimer

The views and opinions expressed in this presentation are those of the individual presenter and do not necessarily reflect the views of the Clinical Trials Transformation Initiative.
Agenda

- Patients Evolving Expectations
- Understand the Disease Landscape & Advocacy Groups
- Bringing Everyone Together to Find Mutual Goals/Objectives
- Partnering Together on Common Grounds
- Patient Voice Can Be Powerful and Empowering
- FDA & Patient Groups
Patient expectations are evolving FAST

Proper incentives will impact behavior change and influence outcomes

Proactive disease prevention and wellness programs and pushing patients to health exchange websites

Patient support services and education help manage disease state

Providing convenience and decreasing time and cost of care

Monitoring tools to actively manage wellness and healthcare

Focusing on value and quality of care for members, shifting costs to patients

Provider

Pharmaceutical Company

Retail

Technology

Employer

Payer

Patient/Consumer
Patient expectations are being shaped by other industries and evolving FAST

### Supermarkets focusing on wellness

> “Whole Foods Market is combating rising costs through a range of innovative health-care and wellness plans” - John Mackey - CEO of Whole Foods Market

### Supercenters creating economical healthcare

> “We are setting a new retail price in the health care industry” - Jennifer LaPerre - Walmart U.S.

### Drugstores providing full service retail clinics

> “Take Care Clinics now provide the most comprehensive service offering within the retail clinic industry” - Dr. Jeffrey Kang - Walgreens

### Apparel & footwear focusing on digital & atheleisure lifestyle

> “The infusion of new technology and innovation into training apparel remains one of the most exciting elements of sports performance,” Kevin Haley - Under Armour
Types of Patient Engagement

Research
- Incorporate patient feedback in the design of trials
- Design drugs and packaging for patient and caregiver convenience
- Develop starter packs
- Develop patient programs and resources for engagement and adherence improvement

Clinical Development
- Match patients to trials based on specific profiles and individual needs
- Collaborate with patients to get on the forefront of legislation (PDUFA IV draft)

Manufacturing & Packaging
- Inform patients on trials to accelerate recruitment
- Partner with patient advocacy groups on venture philanthropy to fund research
- Leverage crowdsourcing technology to gather a pulse on research topics

Marketing
- Provide informational videos and interactive instructions for drug products
- Develop educational tools (online, mobile apps, programs, etc.) to share disease and drug information

Managed Markets
- Develop tools to capture and share patient reported outcomes with pharma companies, payers and providers

Adherence Improvement
- Incorporate patient feedback in the design of trials
- Design drugs and packaging for patient and caregiver convenience
- Develop starter packs
- Develop patient programs and resources for engagement and adherence improvement

Patient Identification
- Match patients to trials based on specific profiles and individual needs
- Collaborate with patients to get on the forefront of legislation (PDUFA IV draft)

Accelerating Research
- Inform patients on trials to accelerate recruitment
- Partner with patient advocacy groups on venture philanthropy to fund research
- Leverage crowdsourcing technology to gather a pulse on research topics

Data Sharing
- Develop tools to capture and share patient reported outcomes with pharma companies, payers and providers

Patient Education
- Develop educational tools (online, mobile apps, programs, etc.) to share disease and drug information
Setting Internal Expectations

- Gain internal key stakeholder buy-in on primary interest
  - Medical, Clinical Development, Marketing etc.

- One lead for internal efforts to ensure comprehensive advocacy approach across all departments

- Incorporate learnings from patient group and patient advisory boards

- Solidify internal procedures for engaging advocacy groups with legal and compliance
Why Work with Advocacy Groups

- Advocacy groups are the voice of the patient and have loyal communities that rely on them for information about their disease, updates on research, clinical trials and the latest treatments.

- Many advocates are patients and survivors.
  - Adds credibility through their REAL personal experiences.

- Important in treatment, research and policy capacities.

- Engaged in the development of treatment guides, clinical trial designs, safety monitoring.

- Participate in scientific, medical and healthcare policy decision-making panels.

- Reach millions through websites and online communities.
Understand the Disease Landscape

- Who are the advocacy groups in the disease area?
  - What do they think of Industry?

- How is the disease discussed?
  - Are there any misunderstandings?

- What is being said about treatments? Clinical trials?

- Who/where are patients going to for information?

- What other companies are in this space?
Bringing Everyone Together

- Learn about their priorities, past/present programs and what their goals are
- Understand capacity – Many groups are in rare disease areas and are small and limited to what they can do
- Stimulate thinking about relevant support, education and advocacy programs to assist this population
- Patient advocacy groups can assist in bringing the patients perspective or actual patients
- Develop common goals and objectives
Case Study - Myelofibrosis
Evaluating the Disease

Myelofibrosis (MF) (or primary myelofibrosis) is one of a group of conditions known as myeloproliferative disorders (MPDs). These disorders affect how the bone marrow works.

Myelofibrosis is a serious bone marrow disorder that disrupts your body's normal production of blood cells. The result is extensive scarring in your bone marrow, leading to severe anemia, weakness, fatigue, and often, an enlarged spleen and liver.

Myelofibrosis (MF) is a chronic blood cancer.

Myelofibrosis is a myeloproliferative disease characterised by proliferation of haemopoietic cells, and

Primary myelofibrosis (also called chronic idiopathic myelofibrosis, agnogenic myeloid metaplasia) is a disorder in which normal bone marrow tissue is gradually replaced with a fibrous scar-like material. Over time, this leads to progressive bone marrow failure.
Finding Mutual Goals/Objectives

- Strive for a long term project not a one time project
- Make the project manageable, strategic, and achievable
- Be relational not transactional
- Identify areas of common interest:
  - Clinical trials input – (symptoms)
  - Clinical trial education and recruitment (additional sites)
  - Updates on new treatments (transparency)
  - Disease awareness information (consistency)
  - Media for an event or publication (additional distribution)
Partnering Together – Common Grounds

Partner with patient advocacy groups to provide quality disease education for HCPs, patients and caregivers:

- Understand best practices in communication and dissemination of appropriate educational materials for patients and caregivers
- Ensure groups are appropriately briefed when new information is available (disease state education, new research findings, new treatment options)
- Provide easily accessible tools to increase disease understanding
  - Educate in the pathogenesis of the disease
  - Expand understanding - diagnosis, prognosis, role of treatments
  - Increase awareness of the clinical significance of symptoms
Evaluating the Disease

**What Is Myelofibrosis?**

Myelofibrosis (MF) belongs to a group of diseases called “myeloproliferative neoplasms” (MPNs). It is sometimes called a “blood cancer” or “bone marrow disorder.” People with myelofibrosis have a defect in their bone marrow that leads to overproduction or underproduction of various blood cells.

Primary myelofibrosis (also called idiopathic myelofibrosis) is a rare bone marrow cancer. It is one of a related group of blood cancers known as “myeloproliferative neoplasms (MPNs)” in which bone marrow cells that produce the blood cells develop and function abnormally. The resulting fibrous scar tissue formation leads to severe anemia, weakness, fatigue and an enlarged spleen and liver.

MF is a chronic blood cancer that is often advanced at the time of diagnosis. Patients may develop one or more of the following conditions:

- **Splenomegaly**: Enlarged spleen
- **Liver enlargement**: Enlarged liver
- **Gastrointestinal bleeding**: Internal bleeding due to the accumulation of scar tissue within the gastrointestinal tract
- **Bone pain**: Due to bone involvement
- **Fatigue**: Due to anemia and anemia-related effects
- **Low platelet count**: Due to bone marrow involvement

**Health Information Technology Institute (HITI)**

The Health Information Technology Institute (HITI) is one of a group of 160+ health improvement organizations working together to improve the health of Americans. The HITI team is committed to building a network of expertise and tools to improve the quality and productivity of health care. The HITI team is continuously working to reduce the burden and burden of disease and promote health and well-being for all people.
Advocacy Feedback

- Wanted to know more about what was causing symptoms
  
<table>
<thead>
<tr>
<th>Spleen-related</th>
<th>Non-spleen-related</th>
</tr>
</thead>
<tbody>
<tr>
<td>Abdominal discomfort</td>
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- Wanted to know more about JAK 1 vs. JAK 2

All people with myelofibrosis are believed to have overactive JAK signaling, even if they do not have a JAK2 mutation.
Using REAL patents in materials
Using REAL patents in materials

“Since I started on Jakafi, my spleen is significantly reduced, and I’ve noticed a decrease in my symptoms.”

—Dan
Living with myelofibrosis since 2009

“I didn’t know what was causing some of my symptoms. My doctor told me that my early feeling of fullness, itching, night sweats, and bone/muscle pain may be caused by myelofibrosis.”

—Susan
Living with myelofibrosis

Possible Benefits of Jakafi
While Jakafi may not work the same way in every person, you may experience an improvement in some of your symptoms and a reduction in the size of your spleen.

In studies of people with myelofibrosis, almost half of those who took Jakafi had an improvement in a group of symptoms* including:

- Spleen-related symptoms
  - Abdominal discomfort
  - Early feeling of fullness
  - Pain under the left ribs
- Other symptoms
  - Itching
  - Night sweats
  - Bone/muscle pain
Patient Voice: Powerful & Empowering

- Understanding patient and family needs will enable companies to develop better solutions that will help patients manage their disease beyond prescription medicines.

- Will become a crucial partner to all patients and their families.

- Increased patient involvement demonstrates companies are truly committed to patient engagement and overall improvement in health.
FDA & Patients

- FDA seeks input from patients and caregivers regarding their treatment options and needs
- The FDA has included the patient perspective at advisory committee meetings
- FDA suggests the use of patient-reported outcome measures in clinical trials
  - Quality of life claims
- Friends of Cancer Research working with the FDA on breakthrough designation
  - As of December 29, 2014 the FDA has given 16 approvals to drugs designated as Breakthrough Therapies
  - 11 of them first time approvals for novel drugs
Conclusions

- Patient and caregivers are expecting MORE
- Early engagement is better and more powerful outcomes
- Relationships between academic, industry, FDA, patient & advocacy has significantly progressed
- Multi-stakeholder engagement is critical in the development of clinical trials and drug approval
- Optimal outcomes through common ground
  - EVERYONE benefits from a genuine partnership