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Success from Start to Finish: Multi-Stakeholder Engagement

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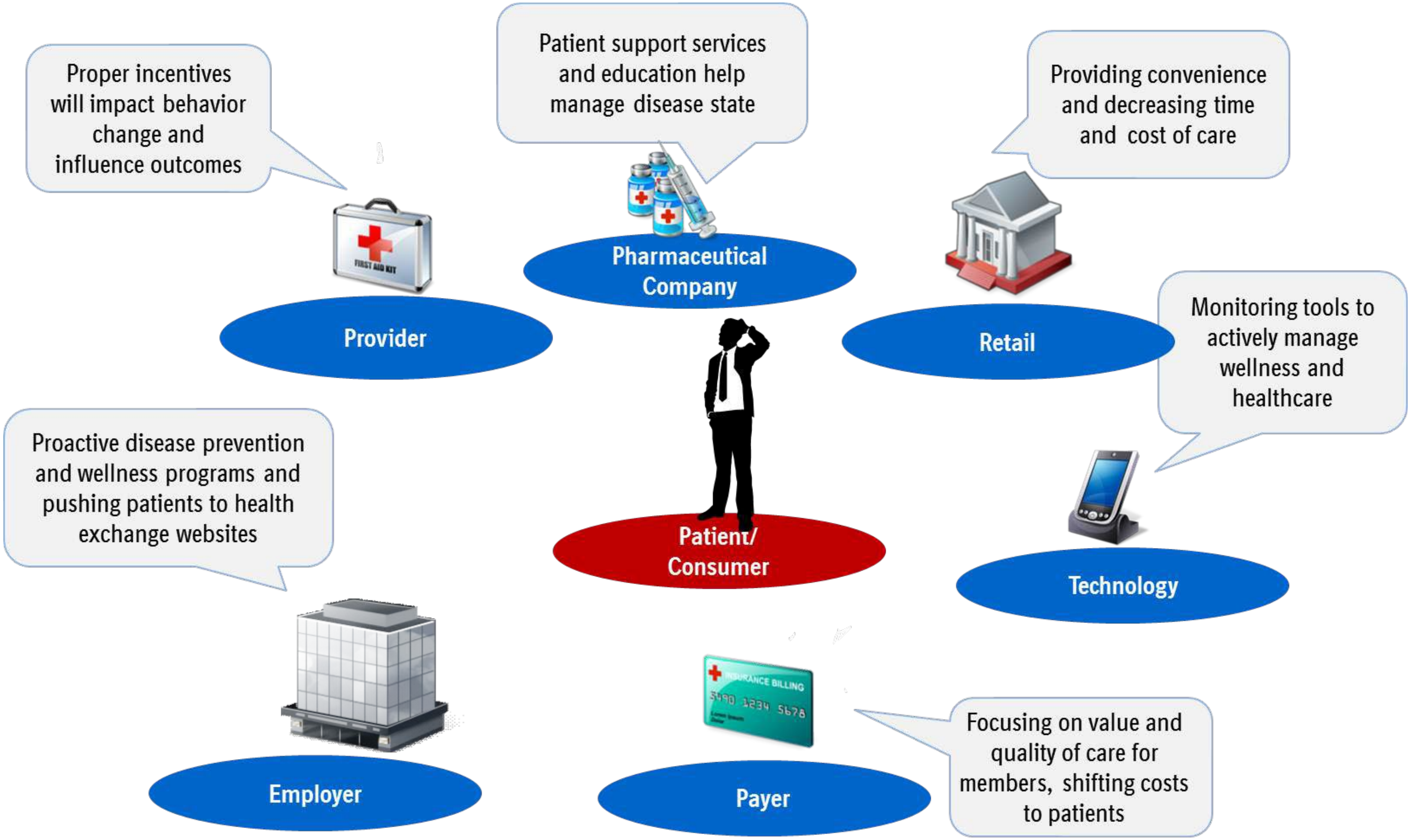
Disclaimer

- 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.
- The presenter is an Employee of Duke University. Salary support comes from pooled membership fees of the Clinical Trials Transformation Initiative and from FDA Cooperative agreement.

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

Provider

Patient support services and education help manage disease state

Pharmaceutical Company

Providing convenience and decreasing time and cost of care

Retail

Monitoring tools to actively manage wellness and healthcare

Technology

Patient/ Consumer

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

Employer

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

Payer

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*

SAFEWAY Ingredients for life...
WHOLE FOODS MARKET
Kroger
Pathmark
Wegmans
Publix SUPER MARKETS
H-E-B

Supercenters creating economical healthcare

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

Walmart Save money. Live better.
Sam's CLUB
TARGET
COSTCO WHOLESALE

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

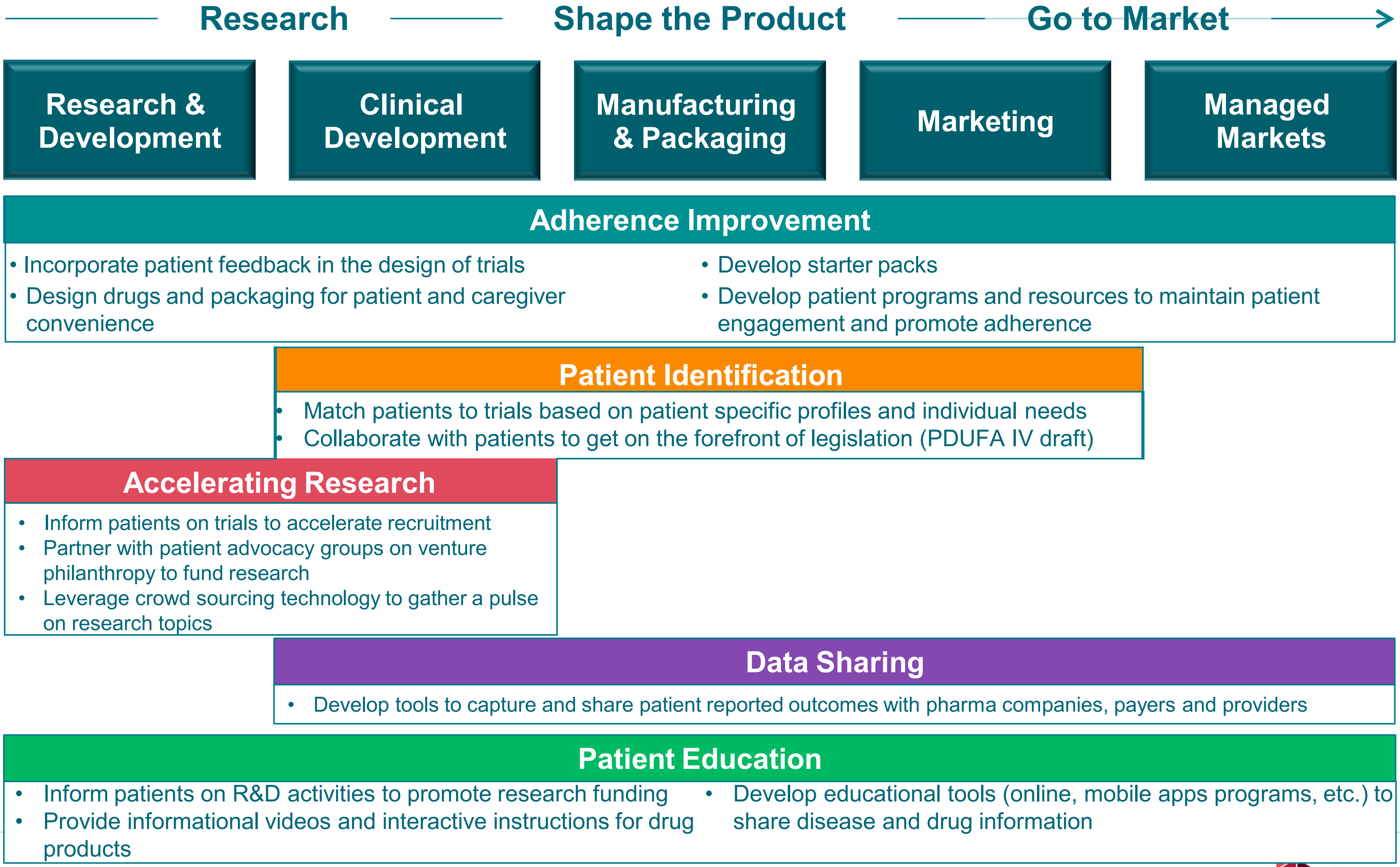
Walgreens
CVSHealth
RITE AID

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*

UNDER ARMOUR
adidas
NIKE
Levi's
ATHLETA
lululemon athletica

Types of Patient Engagement



Research — Shape the Product — Go to Market →

Research & Development | **Clinical Development** | **Manufacturing & Packaging** | **Marketing** | **Managed Markets**

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 to maintain patient engagement and promote adherence

Patient Identification

- Match patients to trials based on patient 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 crowd sourcing 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

- Inform patients on R&D activities to promote research funding
- Provide informational videos and interactive instructions for drug products
- 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 the 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

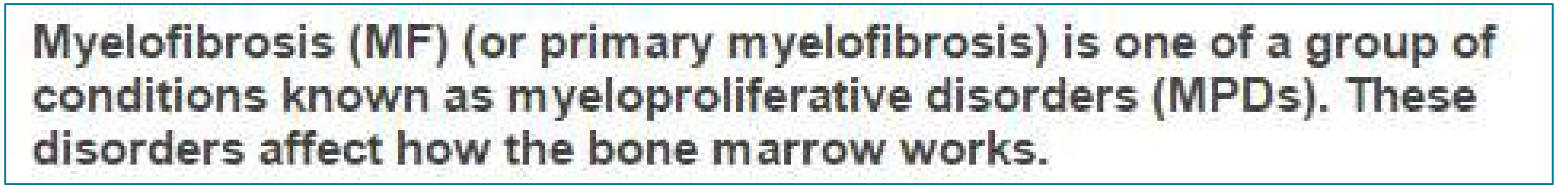
- Who are the advocacy groups in the disease area?
  - What do they think of Industry?
- How is the disease discussed?
  - Are there any misunderstandings?
- What is being said about treatments? Clinical trials?
- Who/where are patients going to for information?
- What other companies are in this space?

Bringing Everyone Together

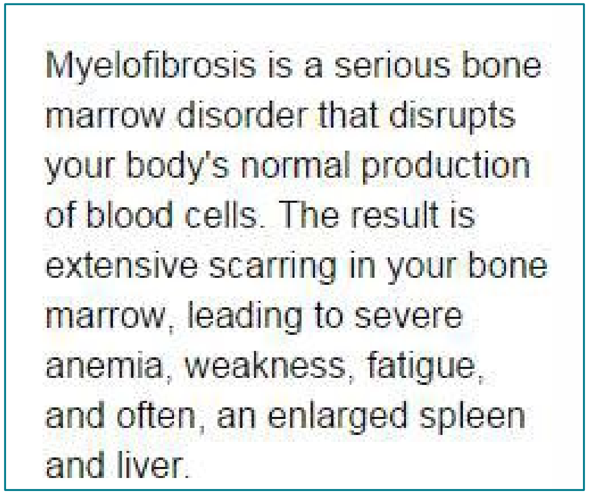
- Learn about their priorities, past/present programs and what their goals are
- Understand capacity – Many groups are in rare disease areas and are small and limited to what they can do
- Stimulate thinking about relevant support, education and advocacy programs to assist this population
- Patient advocacy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

Case Study - Myelofibrosis

Evaluating the Disease



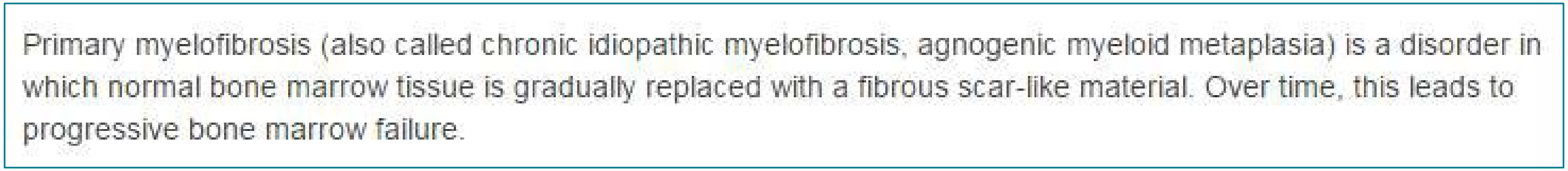
Myelofibrosis (MF) (or primary myelofibrosis) is one of a group of conditions known as myeloproliferative disorders (MPDs). These disorders affect how the bone marrow works.



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,



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.

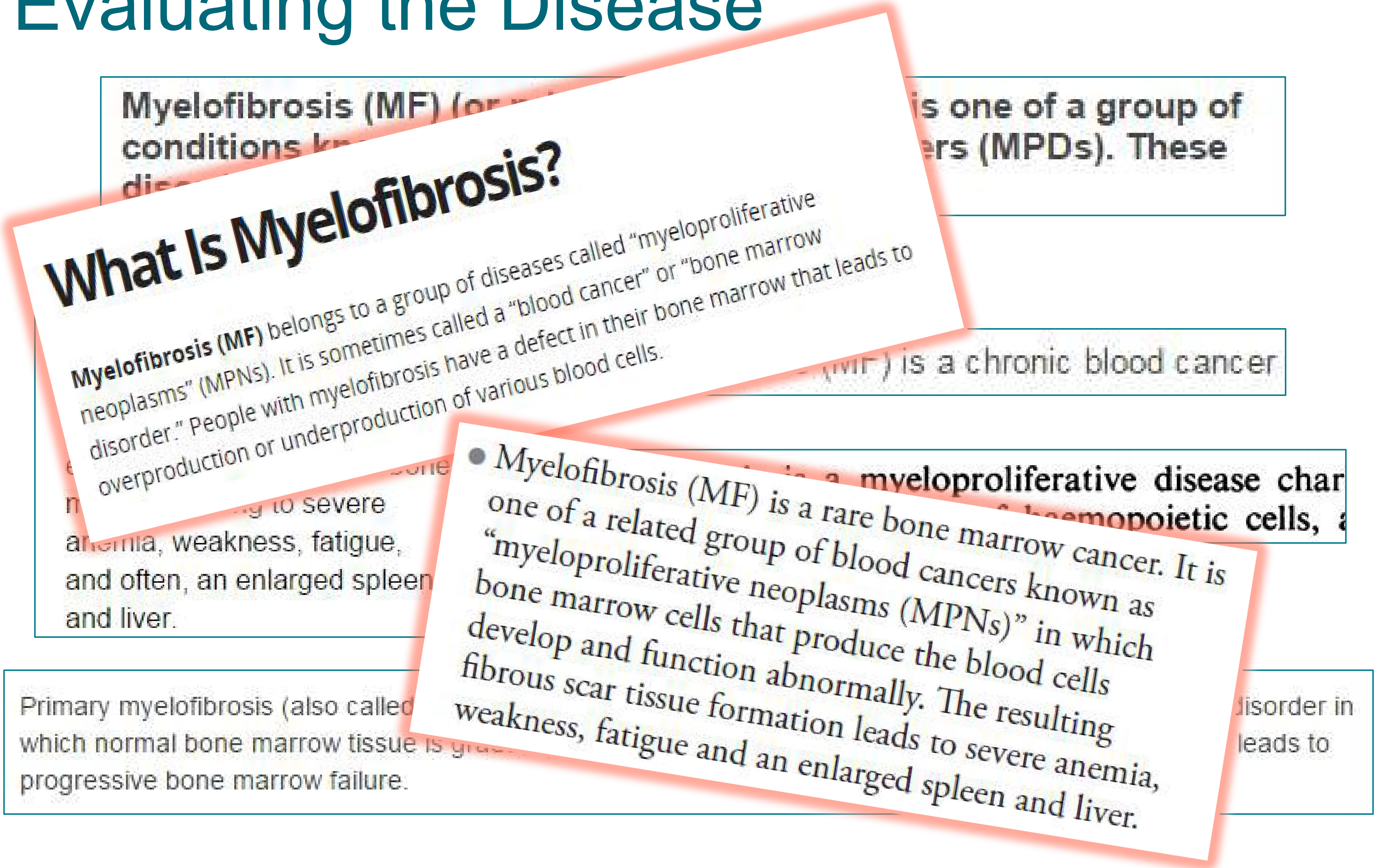
Finding Mutual Goals/Objectives

- 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



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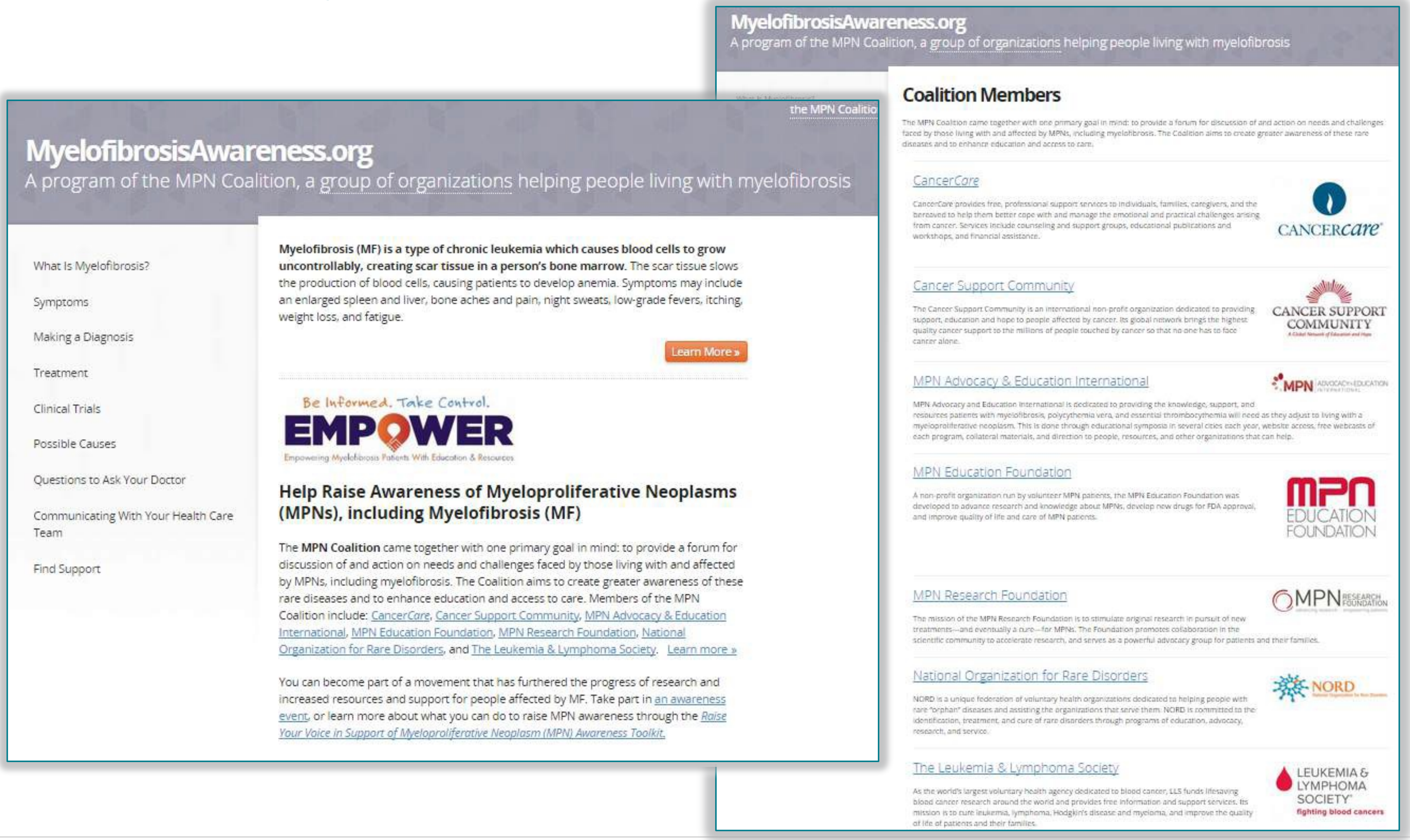
What Is Myelofibrosis?

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.

- Myelofibrosis (MF) 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.

Advocacy Group Collaboration



MyelofibrosisAwareness.org

A program of the MPN Coalition, a group of organizations helping people living with myelofibrosis

Myelofibrosis (MF) is a type of chronic leukemia which causes blood cells to grow uncontrollably, creating scar tissue in a person's bone marrow. The scar tissue slows the production of blood cells, causing patients to develop anemia. Symptoms may include an enlarged spleen and liver, bone aches and pain, night sweats, low-grade fevers, itching, weight loss, and fatigue.

Help Raise Awareness of Myeloproliferative Neoplasms (MPNs), including Myelofibrosis (MF)

The **MPN Coalition** came together with one primary goal in mind: to provide a forum for discussion of and action on needs and challenges faced by those living with and affected by MPNs, including myelofibrosis. The Coalition aims to create greater awareness of these rare diseases and to enhance education and access to care. Members of the MPN Coalition include: *CancerCare*, Cancer Support Community, MPN Advocacy & Education International, MPN Education Foundation, MPN Research Foundation, National Organization for Rare Disorders, and The Leukemia & Lymphoma Society. Learn more »

You can become part of a movement that has furthered the progress of research and increased resources and support for people affected by MF. Take part in an awareness event, or learn more about what you can do to raise MPN awareness through the *Raise Your Voice in Support of Myeloproliferative Neoplasm (MPN) Awareness Toolkit.*

MyelofibrosisAwareness.org

A program of the MPN Coalition, a group of organizations helping people living with myelofibrosis

Coalition Members

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CancerCare

CancerCare provides free, professional support services to individuals, families, caregivers, and the bereaved to help them better cope with and manage the emotional and practical challenges arising from cancer. Services include counseling and support groups, educational publications and workshops, and financial assistance.

Cancer Support Community

The Cancer Support Community is an international non-profit organization dedicated to providing support, education and hope to people affected by cancer. Its global network brings the highest quality cancer support to the millions of people touched by cancer so that no one has to face cancer alone.

MPN Advocacy & Education International

MPN Advocacy and Education International is dedicated to providing the knowledge, support, and resources patients with myelofibrosis, polycythemia vera, and essential thrombocythemia will need as they adjust to living with a myeloproliferative neoplasm. This is done through educational symposia in several cities each year, website access, free webcasts of each program, collateral materials, and direction to people, resources, and other organizations that can help.

MPN Education Foundation

A non-profit organization run by volunteer MPN patients, the MPN Education Foundation was developed to advance research and knowledge about MPNs, develop new drugs for FDA approval, and improve quality of life and care of MPN patients.

MPN Research Foundation

The mission of the MPN Research Foundation is to stimulate original research in pursuit of new treatments—and eventually a cure—for MPNs. The Foundation promotes collaboration in the scientific community to accelerate research, and serves as a powerful advocacy group for patients and their families.

National Organization for Rare Disorders

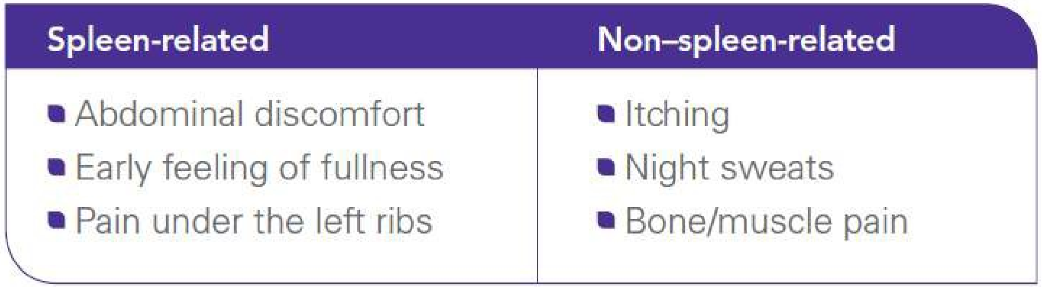
NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and service.

The Leukemia & Lymphoma Society

As the world's largest voluntary health agency dedicated to blood cancer, LLS funds lifesaving blood cancer research around the world and provides free information and support services. Its mission is to cure leukemia, lymphoma, Hodgkin's disease and myeloma, and improve the quality of life of patients and their families.

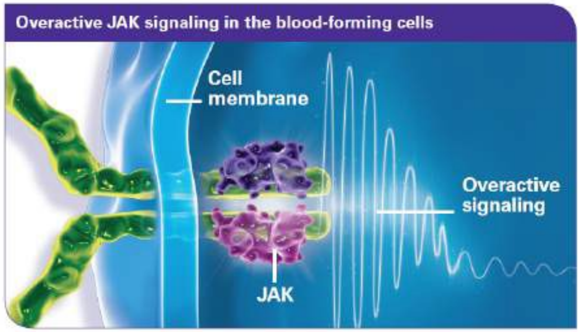
Advocacy Feedback

- Wanted to know more about what was causing symptoms



Spleen-related	Non–spleen-related
Abdominal discomfort Early feeling of fullness Pain under the left ribs	Itching Night sweats Bone/muscle pain

- Wanted to know more about JAK 1 vs. JAK 2



Overactive JAK signaling in the blood-forming cells

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

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

"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

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

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