Advocacy Group Engagement Matters: To Patients, To Industry, To the Future of Drug Development

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Disclosures

- No relevant financial disclosures to report
- Opinions expressed are solely my own and do not express the views or opinions of my employer.

Agenda

I. What patient advocacy groups mean to patients
II. What patient advocacy groups mean to industry
III. Best practices for industry
IV. Best practices for patient advocacy groups
V. What each stakeholder in the room can do today

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What Patient Advocacy Groups Mean to Patients

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Told by your GP that based on your blood test, your eGFR continues to be low and they want to refer you to a nephrologist

Glance at the paperwork from your doctor but your next step at home is to Google “Stage 3 Kidney Disease”

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American Association of Kidney Patients
aakp.org

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Patient advocacy groups: a trusted source

Patient advocacy groups serve as a source of information and support that patients and caregivers likely are not able to get from their healthcare providers. This information and support is provided at a critical time in the patient journey and a connection is forged with the group. This group becomes a trusted source for information.

Disease education, the latest research,

Access to specialists, treatment options, and clinical trials

Support services, support groups, educational events

Community events, fundraising events, legislative activities

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Paradigm shift to patient centricity

Physician Centered

Reactive
Prescriptive

Patient Centered

Proactive
Collaborative

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Successful partnerships between industry and PAG

Sarepta Therapeutics and the Duchenne Muscular Dystrophy Community

April 2016: Hundreds of Duchenne muscular dystrophy (DMD) patients, parents, caregivers and family members testify at FDA hearing but the FDA Advisory Committee votes 7 to 3 against approval

May thru August 2016: MDA and PPMD mobilized their community to express the importance of approval to the FDA. The FDA received over 2500 emails and letters

September 2016: FDA approves eteplirsen after listening to the concerns of the co[illegible]ty

Vertex and the Cystic Fibrosis Foundation

Late 1990's: Dr. Beall develops a venture philanthropy model for non-profits to invest in drug development with for-profit companies

2000: First investment made to Aurora Biosciences, now Vertex

2012: Kalydeco approved by FDA

December 2014: CFF sells royalty rights for CF treatments developed by Ve[illegible]

Patient input is gold standard in rare disease

Principles for interactions with biopharmaceutical companies: the development of guidelines for patient advocacy organizations in the field of rare diseases

Susan Stein[1], Elizabeth Bogard[2*], Nicole Boice[3], Vivian Fernandez[4], Tessa Field[5], Alan Gilstrap[6], Susan R. Jane Larkindale[8] and Toni Mathieson[9]

EFPIA CODE OF PRACTICE ON RELATIONSHIPS BETWEEN THE PHARMACEUTICAL INDUSTRY AND PATIENT ORGANISATIONS

Initially approved in 2007
Amended by decision of the General Assembly in June 2011

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Regulatory recommendations

FDA-led PFDD Meetings

From 2012 to 2017, under the fifth authorization of PDUFA (PDUFA V), FDA conducted disease-specific PFDD meetings to obtain the patient perspective. FDA's PFDD meetings have provided key stakeholders, including FDA, patient advocates, researchers, drug developers, healthcare providers, and others, an opportunity to hear the patient's voice.

21st Century Cures and PDUFA VI

FDA will develop a series of guidance on the collection of patient experience data, and the use of such data and related information in drug development. FDA will develop these guidances over a period of five years to implement provisions of the 21st Century Cures Act and to fulfill commitments under the sixth authorization of the Prescription Drug User Fee Act (PDUFA VI).

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Industry wants to engage patients

Patient advocacy groups want to help drive therapeutic options

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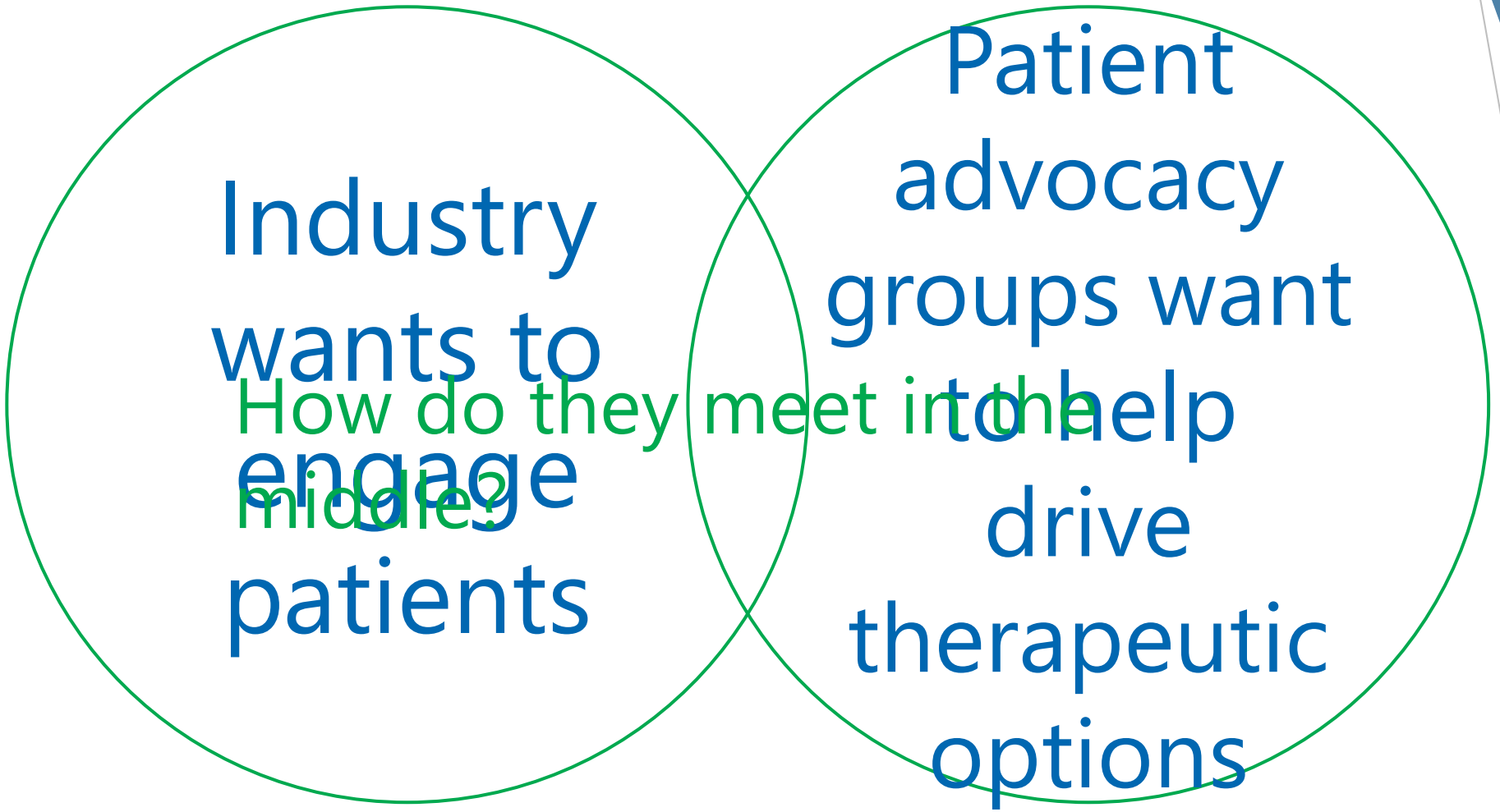

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Best Practices for Industry

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Start early: Phase III is too late

Protocol development

- Protocols are written by scientists with an aim for regulatory approval
- Are they recruitable?
- Ask the patients!

Patient Advisory Boards

- Use PAGs to help find engaged patients to participate
- Review recruitability of protocol
- Keep engaged and meet regularly to ask important questions

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Patient engagement is not the same as patient outreach or recruitment

- Outreach is unidirectional while engagement is bidirectional.
- Engagement **requires** getting input from patients and showing them how it has been used to inform research
- The goal of recruitment is to enroll study participants
- Engagement makes the research relevant

"Engagement means the participant community is somehow involved in the design, even the prioritization before the design, the implementation, and the dissemination of the work."

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Stay involved and engage often

Avoid a transactional relationship

- Make a long-term investment in the patient community, not just to enroll your study
- Stay involved with the patient community; this is critical for diverse populations

Aim for true partnership

- Work with the PAG to educate their constituency about your trial as one of many potential therapeutic options
- Sponsor events, show up to walks, educate your company
- Communicate results of your clinical trial

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Transparency is key

Educate patients & PAGs about regulations

- Work with your regulatory and compliance colleagues to understand what you can and cannot do
- Teach your partners about regulatory restrictions to set clear expectations

Be careful of overstepping

- A note about closed Facebook groups

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Best Practices for Patient Advocacy Groups

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Determine the organization's philosophy about working with industry

Guidelines for industry interactions

- Draft guidelines and internal practices for how your organization can and cannot interact with industry
- Understand your limitations for accepting funding due to 501(c)3 or 501(c)4 status
- Educate your partners
  - Share your guidelines with industry partners
  - Help them understand 501(c)3 governance issues

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Match goals and priorities

Check out your industry partner's website

- How are they talking about supporting patients?
- Are they offering resources to potential patients looking at their website?

Understand the role and function of your industry contact

- Patient Advocacy? Medical Affairs? Focus on educational grants?
- Depending on the size of the company and the role, the priorities of your contact may be specific or more broad

Align goals and priorities

- Keep a dialogue going with your industry partners to meet in the middle and align

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Aim for true partnership by thinking beyond sponsorship

Find out what is most important

- Work together to achieve mutual WIMIs
- Help your industry partner understand the importance of true patient engagement
- Industry comes to PAGs because of your expertise on patient insights and the patient voice

Get creative with partnership opportunities

- Sponsorship is important but what else?
- Program development opportunities
- Clinical trial education and outreach

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What Does This Mean For Each of You?

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Industry

If you aren't engaging with patients or patient advocacy groups, **start today!**

Start with your **compliance and regulatory colleagues** to determine the parameters you need to work within.

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Patient Advocacy Groups

Determine your organization's philosophy on **working with industry** and draft **policies and guidelines.** Then **socialize** that information **internally** and with **all of your partners.**

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Patients & Caregivers

Get involved today!

- Make sure you are receiving updates from the groups that are important to you
- AAKP has a specific place on their website: aakp.org/research which has clinical trial listings and market research opportunities

Be your own advocate

- You are the **EXPERT** on your own health
- Share your story
- Speak up with your physician if you want to explore a clinical trial. Bring the information to them!

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Healthcare Professionals

Know the patient advocacy groups that serve your patients

- Help them find trusted resources for information and support instead of Dr. Google
- Most of these groups have fantastic resources for HCPs as well

Discuss clinical trials with your patients.

- Be open if they bring you information about a trial.
- Talk to **ALL** your patients about clinical trials.

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Thank you!

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