Effective Patient Engagement in Drug Development

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Effective Patient Engagement in Drug Development

• Patient Advocacy In Industry
• Best Practices
• Relationship life cycle – from discovery to commercialization and beyond
• Case studies
• Questions
The Role of Patient Advocacy in Industry

• To advocate for and partner with patient leaders and the overall community in the following areas:
  • Disease awareness and education
  • Public Policy and Access
  • Drug development
  • Unmet needs of patient population

• The voice of the Patient Community within the company

“If it is good for the patients that we serve and the physicians that treat them, then it is good for the company”
- Vin Milano
A few words on compliance…
Select Best Practices - Industry

- Define the global advocacy approach at the CEO/management team level; communicate and set clear expectations at all levels of the organization
- If possible, designate staff with the sole role of advocacy
- Form and nurture relationships with advocacy organizations as early as possible
- Identify all advocacy organizations within the disease area
- Update the entire company on advocacy – make it real
- Never prompt branded discussions when meeting with an advocacy organization; especially upon first contact
PAG Strategic Intent - EXAMPLE

• At ViroPharma the patient comes first
• Patient Advocacy Groups (PAG) are authoritative representatives of patients’ needs which will be represented in everything that we do.
• It is ViroPharma’s intent to connect with PAG in order to forge relationships which address clear unmet needs in the patient community, in a manner that is transparent and compliant with all applicable regulations, industry codes of conducts and internal PAG and ViroPharma policies
• In working with PAG ViroPharma will primarily focus on programmes in the following areas:
  • Education
  • Public Policy
  • Future therapy areas
  • Access to high quality care
• As a consequence of the relationship between PAG and ViroPharma patients will benefit through improvements in the standard of and/or access to care
Select Best Practices for Industry Engagement

• Form and nurture relationships with industry partners as early as possible
• Do your best to understand the rules and regulations that govern these relationships
• Set expectations of the relationship
  • Accountability
  • Interactions – proactive vs reactive
• Transparent and open communications are essential - meaning share the good and the bad
• Obtain resources through sponsorships, grants and in-kind services
It works like any relationship…

- Relationship building
  - Trust
  - Transparency
  - Proactivity
  - NO EGOS!

Trust builds over time
Sharing knowledge speeds progress

YOU are the expert
In Rare Diseases...there are no borders
The FDA Drug approval process
Select Areas of Partnership

Preclinical/Clinical Phase
• Natural history
• Registries
• Clinical trial design
• Clinical trial site selection
• Key opinion leaders/experts
• Recruitment
• Disease education
• Meeting and event support
• Educating industry staff

Commercialization and beyond
• Advisory Committee participation
• Unique programming
• Aid in communication
• Community feedback
• Patient identification
• Market research
• Pricing and reimbursement
• Post marketing obligations
Case Studies
Unmet need: Support for teens and young adults

Cystinosis Teen Adventure
About Cystinosis and Challenges

• Cystinosis is a LSD affecting approximately 2000 people worldwide, 500 in the US
• The disease affects all systems in the body, but presents first in kidneys and eyes
• With approved treatments over the last decade, people with Cystinosis are thriving well into adulthood
• But, these treatments have unpleasant side effects and rigorous dosing schedules
• Transition from pediatric to adult care can be difficult
Cystinosis Teen Adventure
Educating to Empower

Survey Statistics
• 100% of teens reported positive experience at the program
• 100% of teens reported that they would attend again
• 87.5% of teens said they would travel 200+ miles to attend a similar program
• 75% of attendees surveyed plan to discuss the weekend with the Cystinosis community
Unmet need: Awareness, Advocacy and Physician Education

HAE: Learn About It, Talk About It
Swell Documentary
Faces Book
About HAE

- Hereditary Angioedema (HAE) is an “orphan” or rare disease characterized by painful, unpredictable, recurrent attacks of inflammation affecting the hands, feet, face, abdomen, urogenital tract, and the larynx.
- HAE is estimated to occur in at least 6,000 people in the US.
- Average time to diagnosis is more than 10 years.
- If left untreated, HAE can result in a mortality rate as high as 40% primarily due to upper airway obstruction.
Challenges...

- HAE is a very rare disease and largely under diagnosed and misdiagnosed
- People suffering from HAE may present at a number of different specialists and/or the ER due to swelling in various locations
- Many of these specialist have limited or zero awareness of the disease
- Typically, allergist manage people suffering with HAE in the United States
- Consultation between specialists difficult in the case of rare diseases due to lack of awareness
HAE: Learn About It, Talk About It

Program Overview

• Global, physician-focused program educating a variety of HAE-naïve specialists on how to recognize and diagnose HAE, and refer to an HAE treating allergist
• Partner with professional organizations to leverage credibility and reach
• Designed to easily onboard additional third parties and expand globally and regionally over time
• Designed to drive leads to sales team and OL contacts to medical affairs in a compliant manner
• Allows for novel interaction with OLs through participation in program elements (roundtables, podcasts, content review)
HAE: Learn About it, Talk about it
Foster Clinician Relationships via Multiple Touch Points

Engage in 1:1 conversations with clinicians; drive to diverse educational offerings
Leverages ACAAI Allergist Locator to Enable Immediate Patient Referral

Available to physicians via computer and iPhone App
My Life – Photo Book

• Highlight advocates and everyday people living and thriving with HAE
• Provide a ‘counselling tool’ for physicians to explain to patients that they are not alone
• Provide patient organisations with a booklet with inspiring patient stories and pictures
  • Include local PAG logo and contact information
Examples of patient profiles

“Being part of a patient organisation has helped me; when you tell your story to the person next to you, you think --Wow! He understands me! He’s going through the same thing!”

My father passed away as the result of a fatal swelling of the throat. He wasn’t diagnosed and the doctors thought it was some type of allergy. He was only 29 when he died, and my mother was left to raise two daughters.

I was told I had my first attack when I was only a baby. All the attacks were abdominal so it was very difficult to know exactly what was going on. My parents had me undergo many allergy tests. It wasn’t until the attacks became external that I was tested for B12. My attacks were very frequent. I would suffer abdominal cramps. It took two to three times a month. I would have hardly recovered when a new episode would start again. Of course, it never happened with my life. I would be unable to go to school, I missed many recitals and I didn’t have resources to share it with, this is something you live with... alone.

It took 12-13 years to know exactly what it was. I was very scared when I was diagnosed since my father had such a terrible illness.

Now, I’ve come to terms with it. Since my treatment was changed earlier in the year, I feel happy and more independent. I enjoy traveling. As I start to feel more confident, I would like to go on longer trips. I am also interested in visualization techniques because I feel this is good for my condition. Yoga or pilates are on my “to do” list.

Let the doctor find the treatment that best suits you and try to lead a normal life. Join a patient group and above all, keep calm.
"Nowadays I am much more relaxed; I can go on holiday without a worry, and have even been all the way to Australia!"

At age 16, I experienced my first HAE episode. As it was known that my grandfather and mother had the disease, I was tested promptly and HAE was detected early.

At age 16, I experienced my first HAE episode. As it was known that my grandfather and mother had the disease, I was tested promptly and HAE was detected early.

Without any management options at the time, I was forced to "cope". Jaws that swelled when I had an attack made eating difficult. I experienced attacks mainly in my arms, legs, feet and hands, and later started having attacks in my abdomen and upper body.

My first large-scale attack happened while I was hitchhiking on a motorway. I panicked, I couldn't breathe. Finally, someone clamped my pulse and called an ambulance. That man saved my life.

Over the years, my attacks increased, but luckily my employer understood the disease and has allowed me to take a lot of time off work.

At one point, I was experiencing two attacks a week. My quality of life was not good. When I had an attack, I didn't want to see anyone, and I had to take more days off work.

It was then that the physician spoke to me about self-injecting how I was managing my attacks.

Since I changed my routine, I take it every three days and it works well. My life has changed. Nowadays, I am much more independent, can do what I want, and at work I am much more relaxed. Yes, HAE is bothersome, yes, it is very unpleasant, but the best of it, when it is gone again, you rise up again.
Questions?????
Effective Patient Engagement in Drug Development

Kyle Bryant

*Patient Advocate, Friedreich’s Ataxia Founder/Director, rideATAXIA*
Me
Researchers & Physicians

Patients and Families

Government NIH, FDA

Pharma & Biotech
Researchers & Physicians

Patients and Families

Pharma & Biotech

Government NIH, FDA

FARA
FARA Mission & Strategy

Mission – is to marshal and focus the resources and relationships needed to cure FA.

Guiding Principle - Collaboration and Partnership not Isolation and Confrontation
  • As a rare disease we can not afford to seek progress in isolation

Strategy
  • Growing the scientific community
  • Diverse and Deep Pursuit of Treatments
  • Advocacy
Patient Organization

- Industry Benefits from Infrastructure and Insight
- Data from Natural History Study
- Recruit trials in hours using the Patient Registry
- Connection to the patient, trust
Patient Engagement

- Early and often
  - Preclinical – pre-discovery – find out what is important to patients
- Constant/proactive Communication
- Company visits – meaningful work
- Informal connections
- Make it happen
FARA Engagement Across the R&D Continuum

**Discovery**
- Grant funding
- Access to academic experts & clinical network
- Assays
- Mouse models
- Cell lines and biorepository
- Natural history database
- Gene expression database

**Pre-Clinical**
- Patient registry / recruitment
- Patient engagement & access, retention
- Trial design advice
- Funding support / patient costs
- Serve on DSMBs
- Endpoint, biomarker advice & development

**Phase 1**
- Clinical network sites for trials
- Clinician / Site recommendations
- FDA advocacy in post phase 2/3 meetings
- Serve on FDA advisory committees
- Provide testimony at FDA hearings
- Patient engagement / education

**Phase 2/3**
- Patient access & communications
- Website/newsletter/blog, social media articles
- Seminars & conferences - Co-present results
- Payer engagement
- Assist w/ post-market surveillance initiatives

**FDA review & approval**

**Approved Therapy**

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