Building a Digital Home to Provide Patient Support, Education and Clinical Trial Awareness

unlocking the potential of multi-stakeholder communities and the families it serves
Rare Drug Development and Commercialization Philosophy

Science – typically there is a dearth of information on rare diseases and less on products. Little to no natural history data and nothing on the patient and caregiver journey. Establishing a scientific rationale for the rare drug is critical.

Community – engaging with the community, or even creating it if it does not exist, is fundamental in rare diseases. The engaged community can become a de facto field force for the company.

Access – access has many definitions – in this instance, we are referring to pricing and reimbursement of rare drugs. Without science and community readily established, access fails. Without access, you have no drug.

If anything in your plan does not fall under one of these 3 pillars don’t do it.

Science

Community

Access
PACs are the Primary Shapers of Their Rare Disease Ecosystem ... so Involve Them
Rare Market Dynamics – The Evolution of Market Shapers

Transformation of decision-making process

- Decision making in rare includes a 3rd party in addition to the physician and payer who must be considered throughout development and commercialization.

PAC especially critical in rare

- PAC plays a variety of roles and as such has become the new market shaper in rare disease.
- They can make or break a product’s success.

Engaging the PAC

- Identifying, understanding, and engaging the PAC in meaningful ways from development through commercialization is the new wave.

Collaboration is key

- Among academia, pharma, patients, researchers, advocates, and caregivers. Those who do it, will lead the pack.

Non-orphan decision makers – non-orphan drug development and commercialization typically focuses on health care practitioners and payers for success.

PACs have become market shapers in rare – breaking the mold of traditional commercialization, rare companies must become entrenched with the new market shapers to succeed.
Real-world Community Case Studies

Pharma Company #1
- Ultra rare disease – patients very difficult to find for clinical trials
- Influential, social media active parent did not get the response she was hoping for from company
- Reached out to network of parents and actively encouraged them not to enroll their children in the company trial

Pharma Company #2
- Drug approved in the US for first time
- Limited, meaningful pre-launch interaction with community
- Announced price that was “equal to the average price of rare disease drugs in the US
- Community uproar
- Company no longer exists

Pharma Company #3
- Equivocal results of small registration trial (N=12), no control arm
- Patient/caregiver FDA testimonies very moving and influential
- Drug approved 7-3 with 3 abstentions
- First drug approved for the indication
Evolution of Online Communities

since 1980s list serves and aol chat rooms, people have been gathering online and demanding greater specialization and personalization

Traditional communities = Website + Facebook

Custom communities =

social wall +
curated knowledge +
clinical trial resources

[Diagram showing the evolution of online communities from 1980s list serves and aol chat rooms to modern custom communities with various labels like onevoice, one FAPvoice, oneEDSvoice, and oneSCDvoice]
Community Survey promoted for 1-week on Facebook

Facebook metrics:
- 5,000+ “talking about it”*
- 1,300+ “likes”
- 300+ "shares"
- 45+ “top comments”
- 2,000+ click throughs

Viral opportunity:
- 300+ new members (15% conversion)
- 200+ survey responses (66% conversion)

*TALKING ABOUT IT: is an important metric because it emphasizes interactions beyond an initial Facebook Like. The people who actually come back to the page including activities such as comments, likes to a post, shares, etc. Usually the # of TAI is a small % of the # of likes – this reverse result shows the viral opportunity.
A need for Easy-to-access Vetted Information

Patient survey results show that patients want information and they want it in one place – where they can talk about it.

Vetted disease information is ranked “extremely important” across all categories.

- Expert healthcare centers & advocacy organizations
- Written patient education materials
- Expert medical / counselor / influencer profiles
- Scientific / medical papers / guidelines
- Emergency rooms near you
- Research grants awarded
- News & meetings & events
- Scientific / medical papers / guidelines
- Videos / infographics / slide presentations

Q: choose a phrase to complete this sentence:

“Having a one-stop-shop for Community, Disease Information & Clinical Trials would…”

- "...be fantastic" 50%
- "...make better treatment choices" 39%
- "...make me more confident living with SCD" 28%
- "...transform my understanding of SCD" 22%
- "...not make any difference to me" 6%
Feedback on Concept – Community Built by the Community for the Community

"A resource like this would be fantastic because the community can hear patient challenges and provide credible solutions. We can help each other."
- Health Worker, Philadelphia

“I am very interested in learning more about this platform. I commend COMPANY because it is placing resources in the hands of patients and proving your commitment to the SCD community.
– Advocate, IN

“Thank you for allowing me to provide feedback. I am very excited to learn more about this platform and providing it to my community. Having access to a tool like this is out of reach of the SCD community and it can be a great resource for COMPANY to provide to us.”
Advocate, MD

“...It would provide better options for me, help me understand and educate others better, and help me to advocate more effectively.”
Patient,
Improve Goodwill and Reputation by Building a Customized Community

create an online community and improve reputation 15% more than Facebook

Patients want to interact with pharma

Respondents agree that they would choose drugs from pharmaceutical companies that are engaged in their outcomes vs. those who are not.

75%

Americans are open to virtual support service options (e.g., video conference calls) with pharmaceutical companies to help them understand medications.

60%

Americans would share their experiences and direct feedback about medications with a pharmaceutical company to help improve their abilities to develop and support new medications.

83%
What did one Company Hope to Achieve by Building such a Community?

Pharma-sponsored community platform, oneSCDvoice:
- created in unique collaboration between Sickle cell healthcare providers and advocacy leaders
- designed to provide credible disease-specific information and enable self-advocacy

Coalescing a fractured advocacy landscape of 150+ groups
Building trust with historically distrusting cty.
Long-term relationship building
Creating future where new opportunities for connection occur
Creating insights sharing them w/cty. continued learning
Community Dashboard Provides Real-time Insights

See what’s going on as it happens

- Membership sign up by role
- Average time on site
- Top video of the month
- Search term frequency
- Top content
- Stakeholder mix
- Geographic distribution
- Poll/questionnaire results
Demand for PED Drives Next Growth Phase of Digital Communities

- Clinical outcome: An outcome that describes or reflects how an individual feels, functions or survives
- Careful assessment of patients' views on benefits and risks are an important part of regulatory decision making

Collect PED Early, Longitudinally, and Engage Patients as Partners
- The FDA permits starting as early as discovery and continuously throughout product development
- Furthermore, the FDA recommends “actively” engaging patients as advisors and “partners”

Genentech Real World Example: Rituxan hyceia - reducing administration time with PED
OneVoice protects member trust.

Trust is critical for our community members – and we are willing to take a phased approach to getting to know any one individual in our community in the world can access 75% or any one individual in our community. we consider ourselves trusted custodians of members’ identities and data.

In the community... you can choose to be private or identifiable.

But in all cases... we strip identities before we pool to analyze.

Data collection that is safe.

Data collection that is compliant.
Impact of Digital Educational Communities

1. TRUSTWORTHY RESOURCES
   the best of the internet vetted by experts

2. SOCIAL WALL FOR EMOTIONAL SUPPORT
   emotional support and peer-to-peer sharing

3. CLINICAL TRIAL EDUCATOR
   patient education, recruitment & retention

COLLABORATE & CUSTOMIZE WITH COMMUNITY LEADERS:

- Invite patients, advocates, caregivers, and healthcare professionals to guide the platform’s development
- Advisory Councils guide engagement & curation strategies from pre-launch and regularly thereafter
- Create an authentic environment for discussion and relationship building

WHEN & WHY TO DEPLOY A DIGITAL PATIENT-CENTRIC COMMUNITY?

- **Phase 2**: understand unmet needs in early development, build advocacy relationships & optimize clinical program
- **Phase 3**: educate and drive disease awareness while uncovering strategic insights
- **Phase 4**: capture in-market patient experience data for potential label expansion & patient perspectives

Consider online custom community building platform that combines rare disease leaders and technology for the community’s benefit
Thank You!

*Digital, validated rare disease PAC communities*