Engaging Patient Advocacy Groups:

A Key Piece of the Puzzle

Patient advocacy groups play an increasingly important role in the drug development and regulatory process. Biopharmaceutical companies that truly want to be patient-centered should engage these groups early, often, and enthusiastically.

Written by:
Pooja Doshi, Engagement Manager
Laney Monsey, Head of Expert Research Solutions
Chris Von Seggern, Partner

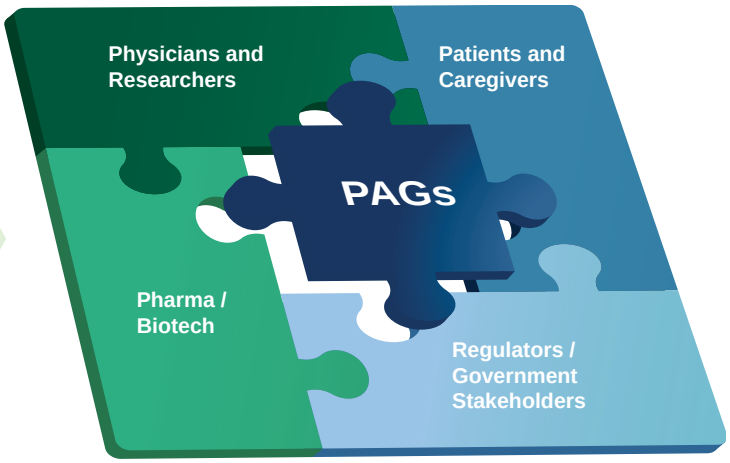
"If I had a disease, I would not want to have a disease that had an ineffective patient advocacy group," FDA Commissioner Robert Califf said at a November 2016 meeting convened by the advocacy organization Friends of Cancer Research (FOCR). It's increasingly unlikely he'd have that specific worry: patient advocates are becoming savvier navigators, instigators, and research engines, and in the process making themselves indispensable partners for the biopharmaceutical industry.

That influence extends well beyond patient advocacy groups' regulatory impact, though that can be significant. Patient advocacy groups (PAGs) can be powerful and pivotal allies, helping to shape decisions about clinical trials or even what sort of drugs to attempt to develop in the first place. At one end of the spectrum these groups can be dogged and determined lobbyists, as was seen with the recent approval of Sarepta Therapeutics' Exondys 51 for Duchenne muscular dystrophy. At the other end, they can be crucial to unraveling the natural history of a disease to steer biopharma companies toward appropriate interventions.

PAGs serve as a conduit between patients, the caregivers, clinicians, researchers, and regulators who serve those patients, and the biopharmaceutical industry (Figure 1).

FIGURE 1

PAGs play a critical role in connecting various stakeholders and providing a holistic perspective to pharma



When partnered with appropriately, PAGs can play an important role in ensuring the patient remains at the center of drug discovery and development. They often have the ear of key regulators, or can help payers to better understand patient priorities and the value of medical interventions. PAGs can help translate the raw emotion of patients and patient communities into measurable endpoints.

For biotech and pharmaceutical companies, partnering with PAGs should not be a question of if, but how and when. Here we discuss strategies for engaging with these organizations that have become so crucial to the wellbeing of their patient communities and drug companies' future success.

The Importance of Patient Advocacy Groups

PAGs may vary in size, structure, and scope depending on the patient community they support and how well the PAG itself is established within that community. A PAG may seek to establish a community in the first place, particularly in cases of rare diseases where patients can be geographically separated from one another and treatment or research opportunities.

PAGs play a critical role in addressing the feeling of isolation that patients and families may feel by creating connections between patients, physicians, and other key stakeholders. They may exist to maintain or raise awareness of more common conditions, where public concern and attention must be continually stoked to generate R&D funding or encourage disease prevention efforts. Larger, established organizations may recruit, maintain, and coordinate networks of key opinion leaders, clinicians, companies and even seek to influence regulation and policy. They may provide patient education and services, act as a clinical trial matchmaker, or create or rearrange research priorities and fund those priorities themselves. These PAGs are often the lynchpin holding together an entire community of stakeholders.

"Often times in the rare disease space these are areas where there are only a couple thousand patients," and only sparse scientific literature describing the disease and its underlying biology, Beatrice Biebuyck, head of global regulatory policy and intelligence at Alexion Pharmaceuticals noted at the November FOCR forum. In those cases, patient identification is a huge challenge for even well-established biotechs like Alexion, and having an established PAG is very helpful to access those patients and identify appropriate clinical trial sites early on in the development process, she said. Alexion works with and supports a legion of PAGs around the globe to help address the needs of patients who receive its ultra-orphan therapies.

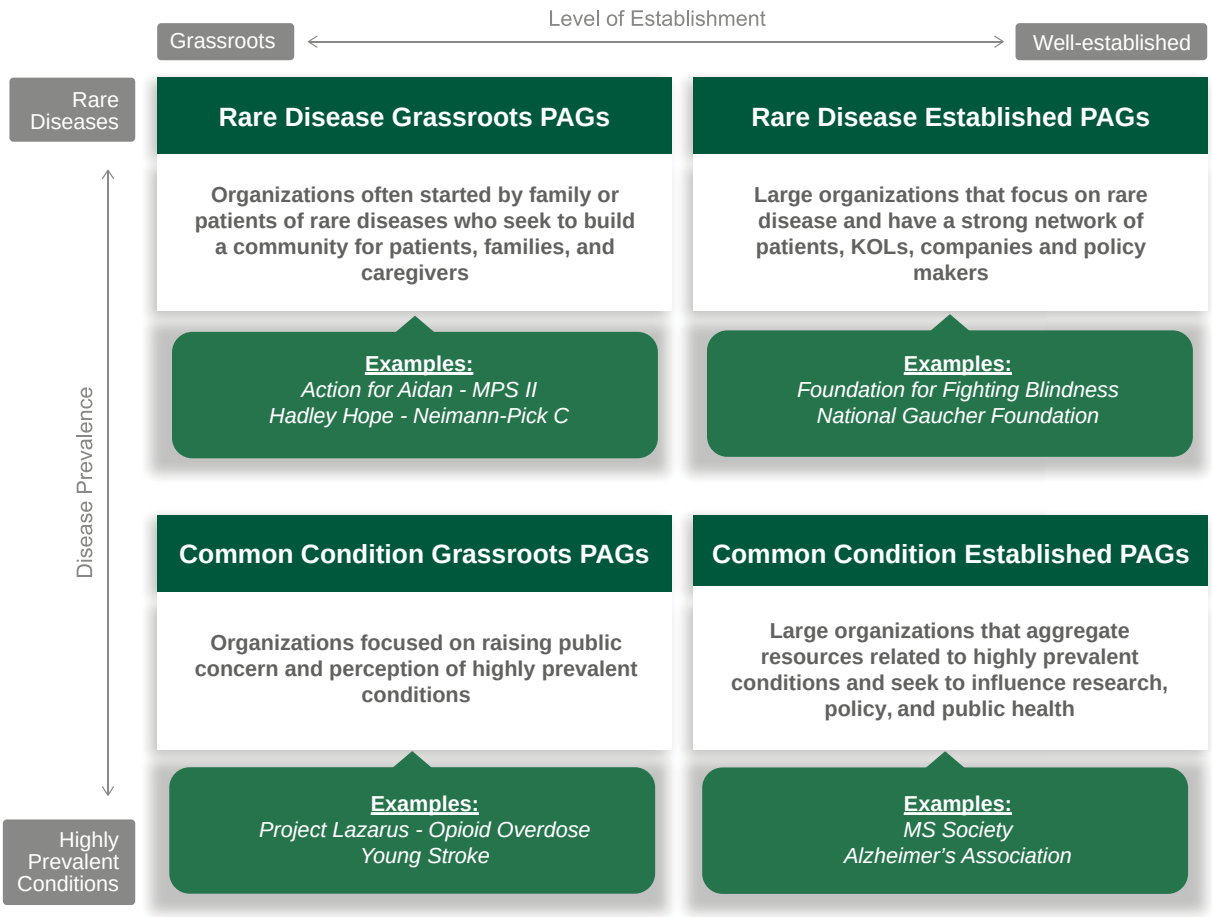
PAGs can work with biopharmaceutical companies to help them understand what efficacy or safety improvements might persuade a patient or his/her physician to switch from a standard-of-care therapy to something new.

These organizations can be instrumental in collecting or shaping clinical trial feedback so future testing can reduce patient burdens. This input on the clinical trials may help to ultimately shape drug discovery to truly meet the unmet need within the space.

Perhaps most importantly these groups have unique insight into patients' unmet needs and patients' priorities. Whether a PAG is a small organization founded by patients or patients' families or a large, established foundation seeking broad public health initiatives, they are often trusted patient partners.

FIGURE 2

The role a PAG plays within a community is largely influenced by organization size and disease prevalence, which may have implications for engagement strategies



Strategies for Engagement

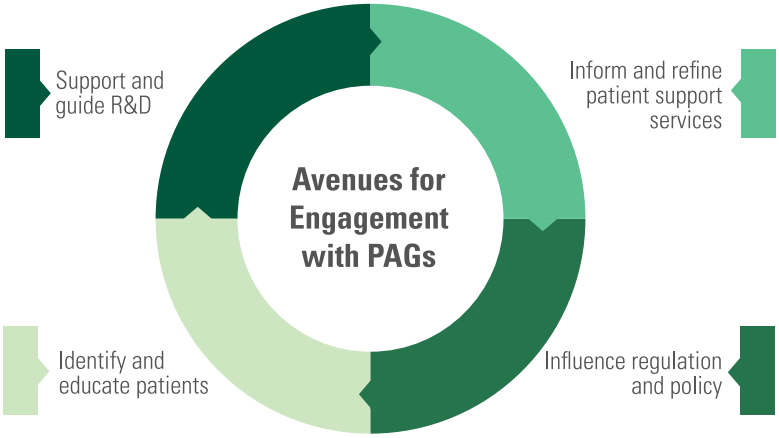
We see four main strategies that biopharmaceutical companies can deploy in engaging patient advocacy groups (Figure 3). Determining which approach to pursue may rely on a company's stage in the drug discovery and development process, or how well developed and sophisticated a particular drug market may be from a regulatory and market access perspective.

Support and Guide R&D

For companies focused on drug discovery and development, input and support from PAGs can help to guide R&D. This is particularly important for assets in the earlier stages of development, and for innovative drug candidates that are potentially first-in-class therapies or new therapeutic modalities. In areas where few or no therapies are available or standard care is particularly lacking, PAGs can work with biopharma companies to push drug candidates to potentially treat their diseases or conditions ahead of other options in the R&D queue. In many ways the poster child for PAG/biopharma partnering success, the Cystic Fibrosis Foundation's (CFF) long and impressive history of funding and coordinating CF research eventually led to Vertex Pharmaceuticals' development and eventual approval of the CF transmembrane conductance regulator Kalydeco (ivacaftor), the first medicine to treat the underlying cause of CF in patients with a certain genetic mutation.

The CFF's original deal with Aurora Biosciences in 2000 (Vertex acquired Aurora a year later) helped drive drug discovery in the pulmonary disease and funded a large portion of CF drug development at Vertex for years. That alliance has been expanded several times, led to a second approval (Orkambi [lumacaftor/ivacaftor]), and has beget more success as CFF has been able to plough its considerable fortune earned from the success of Vertex's programs into further CF research and patient support initiatives.

Another example of successful collaboration is Roche and AstraZeneca's partnership with PatientsLikeMe, an online community that connects patients suffering from more than 2,500 different conditions, and one that has sparked several ongoing research initiatives. Companies have accessed its extensive patient-reported data to better develop new medical evidence and identify endpoints that might be suitable for determining the efficacy of their drug candidates. Working with PatientsLikeMe also enables biopharmas to better communicate with patient communities to gain insights into lifestyle factors that might affect outcomes or quality of life. These interactions can help guide the future of care in areas like oncology or respiratory disease by taking into consideration patients' treatment preferences and real-world disease management and care decisions.



Patient Support Services

For biopharmas with therapies about to launch or already on the market, collaboration with PAGs to inform and refine patient support services can be critical to those drugs' success.

Advocacy groups are intimately familiar with the patient's disease journey and often have a clearer perspective on patients' pain points and other experiences before, throughout, and after the treatment process. These insights can be critical to companies developing successful patient support services. Inviting patient or PAG input into the design and implementation of these services also emphasizes patients' roles as partners that have ownership of and control over the treatment process.

Not only does this bond help to improve the patient experience – through better communication, for example – but it can create loyalty to a specific company or therapy, particularly in the rare disease setting. Engaging with and soliciting patient feedback on support services through in-person workshops, public forums, or even social media can help to refine patient services and build trust between patients and pharmaceutical companies. Often these support services offer personalized approaches, particularly in the case of patients with rare diseases. The rare disease specialist Shire has created a program called OnePath to support patients with a variety of rare diseases including Gaucher disease, Hunter Syndrome (mucopolysaccharidosis II, or MPS II), and hereditary angioedema. OnePath's support services were developed and will continue to be refined as Shire tracks the unmet needs of its patients, based on the company's engagement with PAGs. Through OnePath, each patient is assigned a specific case manager who assists with information about the disease and its treatment, reimbursement, access to care, and financial assistance.

This not only provides personal assistance to each patient, but allows for refinement of services at even a micro –level, which can help to adjust approach as needed.

Identifying the Right Patients

In addition to providing support services to patients and their caregivers, PAGs often have the resources necessary to educate the broader community about diseases. This education can drive disease awareness and help to identify patients that may be suffering from a particular condition. Acting as a conduit between potential patients and physicians, these PAGs can then help drive treatment uptake.

The gene therapy company Spark Therapeutics has begun to raise awareness about inherited retinal diseases (IRD), a group of rare eye disorders that can eventually lead to blindness. The company's lead product is in late-stage clinical trials to treat one such IRD, and in October 2016 the company unveiled an initiative to get more eligible patients access to genetic testing and counseling to identify the root cause of their retinal diseases. The program, dubbed ID your IRD, was borne out of Spark's conversations with advocacy groups and patient families and their concern that lack of access to genetic testing was a large barrier to diagnosis. By identifying potential patients, Spark can connect them and their health care professionals to relevant educational resources about their conditions and possibly screen them for inclusion in a future clinical trial.

A Voice in Policy

As trusted partners, not only can PAGs speak for patients in their dealings with biopharma companies, they can be patients' voice to regulators and payers as well. In 2012, the U.S. Food and Drug Administration began meeting with PAGs to better understand patients' viewpoints. This official push toward greater patient-centeredness in development and regulation was codified in that year's reauthorization of the prescription drug user fee act (PDUFA) law. It has since taken on new momentum as the advocacy community itself has evolved and grown.

What began as FDA's attempt to establish a framework and best practices for using patient information to inform key decisions on their own terms has morphed into PAGs themselves establishing workshops in which FDA is invited to participate alongside patient advocates. Patient-reported outcomes are gaining ground as clinicians, companies, and regulators strive to more fully measure the impacts of potential new therapies on patients' quality of life. PAGs can also provide evidence-based guidance to regulators that need to hear patients' voices in a scientific and rigorous way.

And once products are approved, payers often have patient advocates on review boards. For example, the Center for Medicare and Medicaid Services requires a patient advocate to take part in its Medicare Evidence Development and Coverage Advisory Committee meetings to keep the patient viewpoint at the center of its decision making. Private payers also increasingly rely on patients and other health care consumers in decision making processes. PAGs can possibly play a role in emerging drug value assessment frameworks that help payers shape coverage decisions and guide physician prescription options.

Engage!

PAGs play an increasingly important role in drug development and marketing, and partnering with these organizations can be mutually beneficial for biopharma, patients, and the overall disease-specific community. Best practices for engaging PAGs will be determined by the nature of a biopharma company's pipeline and where a company is in development.

Engagement strategies will vary by disease and by the maturity of the condition's patient support networks, but all PAGs can play a critical role in ensuring that the patient is and remains at the core of biopharma company strategies. For industry, the question is not whether to engage with patient advocacy, it's how.

ClearView Healthcare Partners is a boutique consulting firm providing superior strategic consulting services to companies and investors seeking to drive growth and value creation within the life science sector of the healthcare industry.

ClearView works with business leaders across the life science sector to address a range of strategic growth issues. Our projects span corporate growth, therapeutic area / franchise growth, and disease or asset-specific strategies and initiatives. Our firm expertise spans therapeutics, diagnostics, medical devices, and lab tools.

We have an unrelenting focus on driving to actionable insight. We achieve this by pairing deep domain expertise with robust analytical approaches together with the appropriate mix of client collaboration and external research. Our unique expertise and capabilities enables us to help clients make well informed strategic decisions, enabling them to move forward with confidence within the evolving life science landscape.

For more information please contact the author at chris.vonseggern@clearviewhcp.com

To request information regarding Clearview contact info@clearviewhcp.com Boston-New York