

Investing in Rare Disease Patient Advocacy Groups



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Patient Advocacy Groups (PAGs) excel in bringing together multiple stakeholders – KOLs, physicians, caregivers, policy makers, and patients – throughout development. Close involvement with PAGs helps pharmaceutical and biotech companies advance therapeutics more easily and often more rapidly through the development cycle and regulatory approval process.

Investing early in PAGs' success is a sound strategic move that may increase a company's chances of success by forming a collaboration that enhances patient access and embraces advocacy as this team works together toward compatible goals. Potential strategies to engage with PAGs are discussed in our previous whitepaper, [Engaging with Patient Advocacy Groups](#).

PAGs are invaluable partners for pharmaceutical companies and should be approached early in any therapeutic development cycle. For drug developers, engaging with PAGs is an investment of time, money, and expertise that yields a significant return on investment. Here, we discuss the three stages of investment and what drug developers can expect.

The Natural Evolution of PAGs

Each PAG is unique, yet they tend to start the same way: as organizations helping people with a disease. Often they were formed by caregivers eager for information and desperate for solutions.

PAGs form gradually. Often, people begin blogging about their experience with a rare disease. Patients and caregivers find one another through social media and form online groups to share their experiences. Eventually, the support groups begin to search the Internet for information to share. As the community organizes, it begins to host local events, such as fundraisers, which attract media attention, and community awareness grows.

As time passes, these young PAGs begin to consolidate disease awareness materials for patients and caregivers. They play a role in providing resources to educate others about the disease broadly, as well as ongoing scientific and clinical research. As the group grows, physicians and health care providers may increasingly participate. KOLs emerge, either as leading researchers or visible members of the community, and serve as conduits to clinical trials. These experts begin to share their insights into current research and trials within their specialty with the PAG, thus deepening the knowledge base. As the PAG grows, offshoots may organize throughout the nation or a larger group may form as independent groups consolidate, helping the PAG raise funds for research while attracting more members, which enables more fund raising.

As a PAG grows, its activities may expand beyond strict patient advocacy and support. Some PAGs begin to fund research grants for corporate or academic investigators. Several of the largest, most sophisticated PAGs go a step further and hire researchers of their own. They also tend to become more active politically, lobbying for scientific funding and seeking to influence regulators in terms of the choice of endpoints for clinical trials, how therapeutics for rare diseases are assessed, and even why their disease is a priority.

The U.S. HAE Association (HAEA) followed the typical path as a PAG. It was founded by hereditary angioedema (HAE) patients and caregivers in 2000

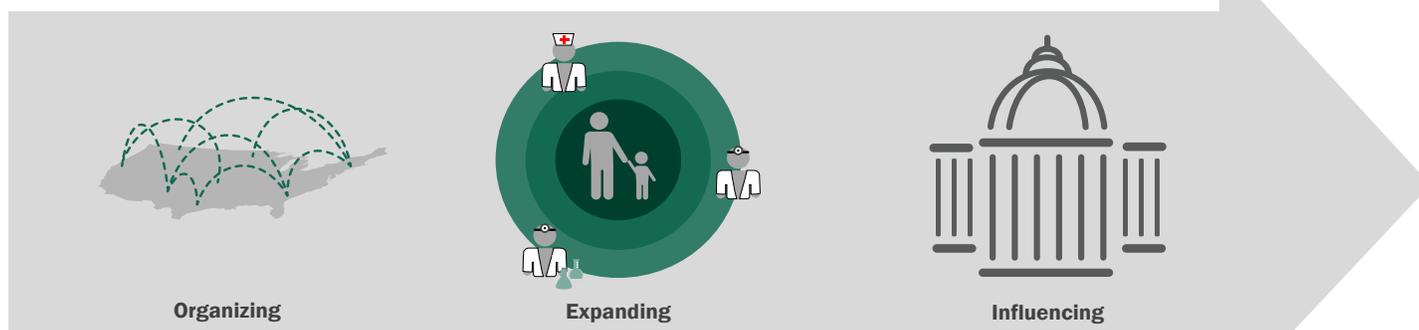


FIGURE 1 The natural evolution of PAGs can be defined as three key phases: (1) Organizing communities of patients and caregivers; (2) Expanding the community to include physicians and researchers (3) Influencing policy makers to increase awareness and funding for research.

to provide information to patients and physicians. By 2005, it had built a social media presence with chat groups and Facebook pages. That expanded in 2015 to include a business Facebook page, Twitter and, importantly, the U.S. HAEA Café. This Café provides an interactive online community where patients and caregivers can come to chat about the condition and therapeutic options.

As the organization has grown, there has been an increased focus on continuing to expand the community of patients, caregivers, physicians, and researchers as well as advocating for awareness of the disease. For example, HAEA organizes an international scientific meeting for physicians and HAE thought leaders, along with a scientific registry of patient data and accredited continuing medical education classes for healthcare providers. Additionally, HAEA organized Capitol Hill Day, an effort to put patients and advocates in front of lawmakers to raise awareness and increase policy support.

Another example of a PAG that has gone through this evolution is the SMA Foundation. Founded in 2003 by parents of a child with spinal muscular atrophy (SMA), the organization has since focused on accelerating the development of a treatment. It

operates as a blend of not-for-profit, venture capital, and biotech entities and has provided more than \$110 million in funding for basic, translational, and clinical research, and \$30 million to develop research tools. The foundation is building industry/academic networks and boasts some 30 affiliations with drug developers. Other activities include educating policy-makers and the broad community about this rare disease.

These organizations are typical of those serving people afflicted by the more than 7,000 rare diseases recognized in the United States. Approximately 260 PAGs are members of the National Organization of Rare Diseases (NORD), which reports that 84 percent of its members are actively involved in research and that 79 percent are engaged in funding research.

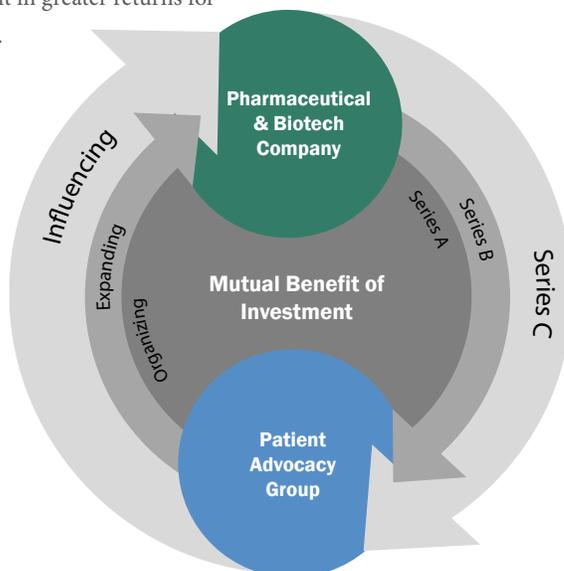
While PAGs may vary in size and sophistication, they all are passionate about their missions. Consequently, they are tireless advocates for patients and their caregivers. Drug developers investing in PAGs should expect engagement and mutual sharing. PAGs prefer to work with drug developers as equals to share information and opportunities to improve the lives of real people.

Investing in PAGs: A New Approach

Investing in PAGs provides a unique opportunity for drug developers active in rare diseases to expedite therapeutic development and build more robust programs. These investments can take many forms. They may, for example, involve direct financial support to help a rare disease foundation support specific programs or fund its general operating budget. On the other hand, investment of expertise may help further the scientific discussion and development within a disease area. What is imperative is that the investments benefit both parties.

A pharmaceutical or biotech company's role in the development of a PAG is similar to that of a

FIGURE 2 Early and consistent investment in PAGs can accelerate the evolution of PAGs, and ultimately result in greater returns for pharma companies.



venture capitalist's role in company development. Both are investing time, money, and expertise in the anticipation of future gains. Thinking of involvement in terms of Series A, Series B, and Series C investments – just like financial capital – helps define a natural evolutionary engagement pathway that capitalizes on the distinct investment opportunities at each phase.

Series A investments in PAGs, as in biotech companies, occur when a PAG is at the early stage of development. It's organizing its community and beginning to build its network. Its potential is aspirational at this point. Drug developers can be invaluable in helping with patient and caregiver outreach, and providing support – both financial and in terms of experience – to help the PAG build better platforms to communicate; develop education materials for patients, caregivers, and healthcare providers; and by so doing, reach more people. The business case for pharmaceutical and biotech company involvement is to build goodwill by demonstrating a commitment to this patient group that will enhance access to members later, thereby

providing a ready pool of patients for clinical trial recruitment and, eventually, commercial users.

Series B investments correlate to a PAG's early expansion. At this point, the PAG starts building its expert network by reaching out to KOLs and physicians. Pharmaceutical and biotech companies can help make introductions to specific leaders, which helps solidify the community. This is an important stage for PAGs, in which they prove their value by providing needed resources for their constituents and information about ongoing research. Beyond the significant trust and goodwill this engenders, involvement at this stage gives drug developers the opportunity to help define the disease, consolidate the diagnostic process, and standardize the treatment approach through the mutual sharing of data and information.

Series C investments occur after the PAG has built its community and established its network and is becoming active in scientific research, typically by providing scientific research grants or hosting scientific meetings. At this point, the PAG

THE MUTUAL BENEFITS OF PAG INVESTMENT

PHARMA COMPANIES

Pharma companies may invest time and resources to find and build networks of patients who may be eligible for a novel therapeutic

Series A

PATIENT ADVOCACY

This investment allows PAGs to increase outreach to patients and caregivers and continue to grow and strengthen the community

Building connections and goodwill among key KOLs can help position pharmaceutical companies as key partners within the disease area of interest

Series B

Investments in this stage help PAGs expand the community and provide greater resources to patients regarding research and treatment

Supporting advocacy activities can create a favorable regulatory environment and encourage support for trials within a disease area

Series C

Greater funding and technical knowledge can accelerate research and increase availability of a therapy

is beginning to influence the scientific community and legislators, and can benefit from investments that will accelerate research and increase patient access to emerging therapies. Drug developers benefit by gaining additional advocates. This may manifest as testimony in FDA hearings, where patients detail the real world differences a particular therapeutic makes in their lives and provide insights that regulators increasingly are seeking. Their involvement helps clear the path for accelerated approval or priority review of a therapeutic. Sometimes, a PAG's role is simply to highlight the fact that a drug is needed. PAGs' pleas to legislators also have increased visibility and garnered valuable research dollars and policy support for specific diseases.

The Value of Investing Early

Unlike venture capital, where late-stage investors typically reap the greatest returns, drug developers have the most to gain by investing early. Unlike venture capital, there is no risk. Although successful commercialization can't be assured, investing in rare disease PAGs yields partners for what may be a long-term endeavor.

There's no downside to investment. Win, lose, or draw, companies that invest in PAGs will have built goodwill. That intangible asset often leads to sharing of data that promotes deeper, non-obvious insights

that inform the choice of goals and improve clinical trial design in ways that develop endpoints that matter to the community. And, if they matter to the community, they matter to regulatory bodies. Considering these points early, therefore, clears the path toward commercialization. Drug developers looking to enter the rare disease space should invest early in PAGs, for the benefit of both.

Even if the unthinkable happens and the therapeutic doesn't achieve commercial success, the drug developer will, through its engagement with a PAG, have built relationships that can help it explore alternative approaches in the future. If the therapeutic succeeds, the company will have helped build a community for a rare disease and, thereby, created a loyal customer base.

For pharmaceutical and biotech companies, successfully commercializing rare disease therapeutics requires leveraging multiple relationships among key stakeholders. PAGs are uniquely situated to bring together these players. Drug developers, therefore, are well-advised to cultivate relationships with rare disease PAGs early and to engage with them frequently as partners in the quest to improve the quality of life or eventually to develop a cure.

Cultivate Developing-Region PAGs, Too

Patient advocacy groups (PAGs) are as important for scientists, patients, and caregivers in emerging markets as well as in the developed world.

While the goals of engagement may be the same as in developed regions, there are certain differences that drug developers must understand. Although PAGs function as gatekeepers for patient access and serve as information clearinghouses, often they are not recognized as the voice of the patient. Drug developers, therefore, can offer training and expertise in terms of community organizing and public relations to help PAGs gain visibility and importance. The efforts

expended now may yield great returns later.

Because rare disease infrastructure and regulations may not exist in developing regions, drug developers have an opportunity to help craft regulations relevant to rare diseases. In such an ecosystem, scientists and physicians may have greater flexibility to engage patients, especially if no other therapies exist for life-threatening conditions.

Whatever their size or level of influence, PAGs bring stakeholders together and, therefore, are important for long-term commercial success. By investing in them, companies can help shape the world in which rare diseases exist.

About ClearView Healthcare Partners

Founded in 2007, ClearView Healthcare Partners is a global strategy consulting firm serving the life science sector.

The firm combines international industry knowledge and deep scientific expertise across a range of therapeutic areas with an extensive network of external stakeholders to deliver practical and actionable recommendations to our clients' most complex challenges. The firm's projects include cross-functional support at the corporate, franchise, and product levels for pharmaceutical, biotech, medical device, and diagnostics companies worldwide.

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