

THIRTY YEARS *of* PHARM EXEC



# IMPROVING ADVOCACY

BY

## LEAPS *AND* BOUNDS

The rise of advocacy groups have helped patients find their voice, but the power to change health profiles remains an elusive goal

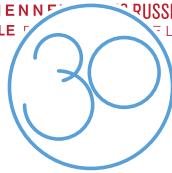
By Jennifer Ringler, Associate Editor

“One small step for man, one giant leap for mankind,” were Neil Armstrong’s famous words when he became the first man to set foot on the moon. That small first step became the jumping-off point for a whole new world of discovery and space exploration. Similarly, the first humble steps of the patient advocacy movement started decades ago with a few hopefuls treading lightly onto uncharted territory. Their efforts have paved the way for those after them, leading to new legislation, a more patient-centric approach to pipeline development, and new strategic partnerships between those that run the industry and those they serve.

The first “giant leap” in giving patients a voice in industry came in the 1980s, with the AIDS epidemic as the catalyst. “Prior to the AIDS epidemic, patient advocacy groups were supporting traditional research and timelines, and were still in the March of Dimes mindset—family members raising money to give to a particular cause for typical academic research,” says Greg Simon, senior vice president of the Patient Engagement Group at Pfizer. “But when people are dying so rapidly—that causes a crisis that’s very different from diseases such as Alzheimer’s, for instance.”

Because of the shock of the epidemic and the nature of the disease, Simon says, a new way of getting things done—getting drugs pushed through the FDA review process and getting legislation passed—was crucial. Advocacy groups such as the AIDS Coalition to Unleash Power (ACT UP) rose to the occasion. “They just walked in and broke all the rules,” says Simon. “They occupied offices. They wrote inflammatory letters calling the senior people at NIH like Tony Fauci inhuman idiots. And when Fauci was asked how these groups got his attention, he said, ‘Well, when they keep writing articles with headlines that say Tony Fauci is an idiot, you start paying attention.’”

And it worked. Fauci invited the ACT UP advocates into his office, and



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from those conversations, according to Simon, Fauci “realized that he had to change the way we go about doing research.” Part of that change was allowing AIDS patients who were not en-

stitutionalized as the Expanded Access Programs). Though the method was unprecedented, says Simon, “it was about accelerating the effort and breaking the rules to allow desperate people

tive, in which the FDA defines therapeutically important new drugs and gives them special treatment in moving them through the registration process. This form of accelerated approval was formally endorsed in congressional legislation in 2007, and is seen as a major victory for patients with diseases representing a serious unmet medical need.

According to Simon: “The lesson of the AIDS advocates isn’t that you have to go around breaking windows. It’s that you have to bring about change—you have to break the paradigm.”

### Singular Voices, Collective Ideas

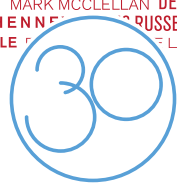
In the early '80s, as patients and physicians everywhere were grappling with AIDS and wondering what to do, another group of patients—or rather, several small groups—had other diseases on the mind. The National Organization for



The FDA's Fast Track initiative is seen as a major victory for patients with diseases representing a serious unmet medical need

rolled in clinical trials to try the new, pre-approved drugs anyway, because they might not have the time to wait for approval (a process now fully in-

to try things we wouldn't let them try if they were not terminal.” Pressure from the AIDS community also helped strengthen the FDA Fast Track initia-



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Rare Disorders (NORD) was founded in 1982, when Abbey Meyers realized that the trouble she was having in getting pharma to manufacture a drug for her son's rare disease was actually a problem across the board (see Abbey Meyers profile, page 104). Meyers' efforts and the work of NORD quickly spawned the Orphan Drug Act of 1983—a clear example that a solitary voice, when banded with others, could be heard.

Today, NORD is continuing to aim for the goals set by Meyers 29 years ago. "We see ourselves as the voice for patients with rare diseases," says Pe-

smaller pharmas have traditionally been the ones developing orphan drugs, Big Pharma is now getting in the game. "Big Pharma has a different kind of business model," cautions Saltonstall. "They historically have not been plugged into the patient as much as I think they need to be. So NORD's task is to educate them, to help them master what it is to bring a rare disease therapy to market."

Thankfully, he says, Big Pharma welcomes the expertise NORD brings to the table. "One of the big players has come to us and asked us to help structure their rare disease program. We're

the cause. But we don't have anyone making those statements—Alzheimer's doesn't have any survivors."

Like NORD, the AFA is an umbrella organization, uniting the efforts of smaller advocacy groups across the nation. But the main focus here is not on R&D and finding a cure—it's on supporting the patients and caregivers through their daily struggle with managing the disease. When the AFA was founded in 2002, says Hall, "One thing I sensed was that there was an enormous amount of attention being paid to research, and the idea that we would find a cure, and that was the most important objective. There was so much attention being paid to research for a cure, but in the meantime, there are all these other needs."

Based on the number of individuals currently affected with Alzheimer's and demographic projections, Hall emphasizes the need to focus on proving support for patients and caregivers, and on what to do about the financial impact Alzheimer's will have on the country. "This is an almost unprecedented impact on the national economy—in the absence of a cure, what happens? How does any one country end up caring for this onslaught?"

AFA has helped carve a noticeable chunk out of the challenges associated with Alzheimer's through such efforts as National Memory Screening Day, a magazine devoted to caregivers, a training program for healthcare professionals called Dementia Care Professionals of America, and an online support community for teens who are assisting with the at-home care of a grandparent or other Alzheimer's patient.

As the AFA and other patient advocacy groups continue working toward their goals, Hall, like Saltonstall, says the most important element going forward will be collaboration. "It's natural for organizations to be focused on their own objectives, and to have their own goals and missions ... it's not easy to say, 'This won't be about us. We'll do it for the greater good.' But AFA is an



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ter Saltonstall, president and CEO of NORD. The organization serves as an umbrella for many smaller rare disease advocacy groups across the nation—providing advice, raising funds, shaping legislation, and offering expertise to those smaller groups in an effort to pool resources and collaborate on behalf of the patients.

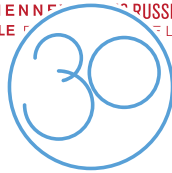
A current focus for NORD is assessing the cost of bringing a drug for a rare disease to market versus *not* bringing that drug to market, especially in terms of burden on the healthcare system. "I ran a large hospital in Boston, and I can tell you that patients who didn't get diagnosed or didn't have their ongoing medication for whatever reason would always end up at the emergency room," says Saltonstall.

Going forward, Saltonstall believes that increased collaboration between pharma and NORD (and all advocacy groups) will be key. Though he says that

helping them think through what this whole business unit needs to look like, what elements it needs to be successful from our perspective. They sought us out—that's very encouraging."

Uniting Efforts

For some patients, however, storming the NIH or writing letters to *The New York Times* to call attention to their cause just isn't an option. "Let's be honest—the Alzheimer's population is not as vocal in advocacy as others, because the disease really restricts and sometimes removes that voice," says Eric Hall, president and CEO of the Alzheimer's Foundation of America (AFA). "All the breast cancer walks and other advocacy efforts of other groups are great. They are able to find individuals who get up and say, 'I had this illness, and thanks to leading therapies and treatment and care, I'm now cancer free.' And that's marketing for



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example of what can happen when organizations come together. ... I think advocacy is best served when organizations unify their voice.”

**Partnering Hope with Fight**

For patients with late-stage cancer, advocacy means exhausting all possibilities. And so the goal of the International Cancer Advocacy Network (ICAN) is to be the patients’ “advocate, clearinghouse, and sounding board, with a meticulous eye to every detail of their medical records, history, co-morbidity conditions, and treatment plan, while mindfully positioning several moves ahead at all times in terms of available strategic options,” says Marcia Horn, ICAN’s president and CEO.

That sounds like no easy feat, but ICAN breaks these goals down through such actions as recommending special-

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ists to patients on a case-by-case basis, offering counseling for patients and families, interpreting the impact of patient gene expression profiling reports, and referring patients to relevant clinical trials. “We often make referrals to oncologists who, like us, are ‘manic’ about not missing anything in the drug pipeline,” says Horn. “A patient can go from one oncologist who says, ‘There is ab-

solutely no hope and you might as well check into hospice,’ to someone we refer them to, who will launch a focused and determined fight for the many months or years that patient has left.”

So where other advocacy groups might look at the big picture, acting as the voice for an entire population in a specific therapeutic area, ICAN works differently—from the bottom up—pro-



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viding advocacy for each patient one at a time, in a way that is tailored to the individual, not to the whole. “What we are best known for is providing and explaining comprehensive information,” explains Horn. “It’s not unusual for us to hear that our initial phone call or email exchange with a patient is far more detailed than their entire medical team’s interaction with them.”

Horn emphasizes the need to work closely with pharma; a need which has only increased in the past 30 years. “Patient advocacy organizations are becoming far more important to pharmaceutical and biotech companies than ever before. The industry realizes that organizations that deal with proactive patients need a seat at the table to provide insight into patient choices,” she says.

Especially when it comes to cancer, Horn says, no one individual or entity has all the pieces of the puzzle. “Our most repeated line in any given day when talking to patients is, ‘We don’t know, but we will find out for you.’ I don’t think that we as a community know even a small fraction of what we ultimately need to know in order to tame cancer into a maintenance disease and then eradicate it altogether. But patients who are armed with highly trained advocates are greatly advantaged in having an ally who can help them sort, filter, issue-spot, and translate difficult concepts for them.”

### Dawn of a New Era

Whether working on rare diseases, speaking for the silent, or encouraging innovation in oncology, the minds behind every patient advocacy group *Pharm Exec* spoke with agreed on one thing—there’s still work to be done. And technology is helping us do it in ways we never thought possible. Like putting a man on the moon, the Internet has opened up worlds of possibilities, allowing patients and advocates to connect with one another, organize their goals, and act on them efficiently. “Before the Internet, the flow of information and the

opportunity to broaden that scope was limited. But advocacy groups who didn’t have this ability 10 years ago can now go on Facebook and meet someone who will say, ‘I happen to be doing research on this,’ or, ‘I know someone working in that area,’ and then follow that trail,” says Saltonstall. NORD is leveraging such technology to create disease-specific online communities, where patients can communicate with one another and decrease the sense of isolation that having a rare disorder often constitutes.

In fact, NORD and other national advocacy groups have partnered with Inspire, a company whose purpose is to build online communities for patients and caregivers and to help life sciences organizations connect with online community members for clinical trial recruitment and related services.

line resource called Remission Coach, which Horn says will be a “comprehensive medical information search engine.” In addition to Remission Coach, Horn and her staff frequently use LinkedIn, which she says has exponentially expanded ICAN’s reach to professionals in pharma, academia, and government. “With the rise of the Internet and so much information at their fingertips,” she says, “patients and their families are more informed and empowered than ever before.”

As we look to the future, all stakeholders must ask themselves five questions about patient advocacy organizations: Will the patient movement shift from a homegrown initiative into a more global, broad-reaching endeavor? Will there be further transition from a focus on specific diseases and therapeutic ar-

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“Patients have always connected with one another, whether in hospital waiting rooms or doctors’ offices,” says Brian Loew, cofounder and CEO of Inspire. “But what is new—what we have now—is the incredible benefit of these forces working together: the organic, self-organizing activities of patients online, enhanced by the trusted, strategic efforts of patient advocacy organizations. What this means for pharma, biotech, and device companies is that if they’re looking for active, engaged patient populations, here they are.”

ICAN, as well, is leveraging the power of the Internet for advocacy. The organization is currently in the fundraising and planning stages of creating an on-

line resource called Remission Coach, which Horn says will be a “comprehensive medical information search engine.” In addition to Remission Coach, Horn and her staff frequently use LinkedIn, which she says has exponentially expanded ICAN’s reach to professionals in pharma, academia, and government. “With the rise of the Internet and so much information at their fingertips,” she says, “patients and their families are more informed and empowered than ever before.”

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