

## Insight & Intelligence™

# Pharma Builds Bridges with Advocacy Groups

## Healthcare Reform Underscores the Need to Engage Patients Before Launching Clinical Trials

Alex Philippidis

The FDA's recent approval of Procsybi (cysteamine bitartrate) for nephropathic cystinosis brought attention to Natalie Stack, the young woman who, 10 years ago at age 12, used a restaurant napkin to write out her wish: "To have my disease go away forever."

Natalie's wish inspired her parents to create the Cystinosis Research Foundation (CRF), which over the past decade built a community of families affected by the rare kidney disease. Joining the foundation and researchers in that effort has been Procsybi's developer, Raptor Pharmaceutical.

Raptor kept communication lines open by furnishing the cell number of an executive willing to answer questions about the development of Procsybi. "That struck a chord in our community," Natalie's mother, Nancy Stack, told GEN. "We're such a small community, and we were so grateful that a pharma company came along to pick this drug up and march it through the FDA."

Raptor also encouraged cystinosis families to register with the first cystinosis patient registry, established by CRF.

"We got them involved in our family conferences, in terms of providing updates along with our researchers who were doing presentations. Raptor was always there talking and answering questions, and sharing what they were doing," Stack said.

From up-and-comers like Raptor to corporate giants, big and not-yet-bit biopharmas will need to relationship-build with patients to achieve the cost and time savings needed to bring new drugs to market in the brave new world wrought by the Patient Protection and Affordable Care Act (PPACA).

### Perfect Storm

The federal overhaul of the U.S. healthcare system enacted by President Barack Obama established the private, nonprofit Patient-Centered Outcomes Research Institute (PCORI) to conduct and fund comparative-effectiveness research intended to provide best-available evidence on prevention, treatment, and healthcare options.

PCORI and other federal efforts, such as faster FDA reviews, are "creating a perfect storm that has caused industry to look at this," Marc Boutin, J.D., executive vp and COO for the National Health Council, told GEN.

The coalition is controlled by patient advocacy organizations, with members from insurance and biopharma.

"A number of companies are doing self-assessments to look at where they engage, when and how, and start to look at what might be a strategy from beginning to end, with an eye towards shortening the process and making better products," Boutin said.

"It really requires a cultural shift within the researchers and within the organizations. And it requires that multiple silos within any pharmaceutical company have to come together and figure out what is that strategy going to be."

For a change, money doesn't appear to be the obstacle—most likely since many engagement initiatives aren't too costly, or are cost-shared among several stakeholders. A larger challenge, according to Boutin, is changing phar-



**Biopharmas will need to relationship-build with patients to achieve the cost and time savings needed to bring new drugs to market in the post-Affordable Care Act world.**

© Monkey Business - Fotolia.com

ma's historical focus on winning over regulators to the safety and efficacy of new drugs, rather than to the value of new drugs to patients.

"You would put together a strategy around patient engagement that would include a variety of different kinds of modalities, and you'd bring the strategy to market. I could envision doing that for certainly under \$500,000, so it's not a great cost. You could do it for less or you could do it for more, depending on the specific strategy," Boutin said. "It could be a focus group. It could be in-person interviews. It could be surveys. It could be crowdsourcing. It could be deliberative juries."

### Waiting for Guidance

Alexina Fredman, senior consultant with the healthcare professional services firm Falcon Consulting Group, cited FDA inaction to date on closely watched industry rules. The agency has yet to finalize a 2011 draft guidance on responding to unsolicited requests for information—for example, a question about off-label use.

"As soon as there are a few more regulations put into place, whoever takes advantage of that is going to have a good competitive edge over new customers, putting the patients first, having the patients be able to share their experiences with others, and do a bit more word-of-mouth marketing and really utilize social media in the way that other industries have," Fredman added.

At Eli Lilly, a company-initiated "community conversation" uniting local leaders and people with severe, persistent mental illness resulted in improved access to psychiatric treatment.

"The reason so many people were not accessing their treatment and therefore getting arrested or living on the streets, was there was no bus line to the main community psychiatric center. The only people who could make it there were those who could walk several miles, or those who had transportation," Michele M. Oshman, director, global advocacy & professional relations with Lilly, told GEN. She could not furnish the municipality's name at deadline.

Lilly's engagement also funds the Welcome Back and Reintegration annual awards of the National Council for Community Behavioral Healthcare, whose 2,000 member organizations treat eight million adults and families annually. Winners receive awards of \$5,000 to \$10,000 that must be donated to nonprofits that assist other patients.

"We will never be aligned on every issue. They have other business interests. But in places where we are aligned, certainly around issues of access to treatment, we're able to work together in coalitions, and we're much more powerful together," Linda Rosenberg, the national council's CEO, told GEN. "They really put their money into really supporting patients as well as, obviously, making important products."

### Bracing for Patent Cliff

Lilly says its engagement effort is separate from its drug marketing. But the pharma giant is bracing for the loss of \$5 billion in annual revenue as its patent on antidepressant Cymbalta® (duloxetine hydrochloride) expires in December. The company's pipeline includes edivoxetine (LY2216684), an antidepressant in a Phase III trial for major depressive disorder patients who responded partially to a selective serotonin reuptake inhibitor, and a Phase II/III trial for ADHD.

"It's fair to point out that we engage in communities where we have mutual interests. Our areas of expertise at Lilly are definitely in the neurosciences, oncology, and diabetes. Because of the work we've done in those spaces, we have a unique opportunity to build an advocacy community there," Oshman said.

Big pharmas can also learn about engagement from Acorda Therapeutics, which markets Ampyra® to improve walking in people with multiple sclerosis, as well as Zanaflex® capsules and tablets to manage spasticity in people with MS or spinal cord injury.

In addition to supporting the National Disabilities Institute's Real Economic Impact Tour, which provides free tax preparation and financial counseling to people with disabilities, Acorda is in its third year of working

with the institute to develop and underwrite webinars on financial wellness for people with MS.

### Making a Difference

Tierney Saccavino, senior vp, corporate communications at Acorda, recalled how a decade ago, before winning approval for its first MS drug, the company used resourcefulness to make up for its lack of cash when groups came calling for support.

"We made our contributions to advocacy groups by providing practical support instead of financial support. And it made us really focus on doing things that made a very concrete difference in peoples' lives," Saccavino told GEN. "I really prefer not to write a check and get my logo on an email. We don't do a lot of galas. But we really look for programs whose goal is similar to our therapeutic mission, which is to restore function and improve lives."

As its drugs restore physical functioning for people with MS, Saccavino said, so Acorda's engagement efforts aim to restore patient function within society: "We might provide equipment or help make people's homes or lives more accessible. I'd rather do that than sponsor an event."

Like Lilly, Acorda views its engagement as separate from drug marketing, though the company acknowledges at least one benefit—tightening its connection with the communities of MS and spinal cord injury patients.

Obamacare will compel many biopharmas to relationship-build along Acorda's lines, while big pharma can be expected to expand wellness efforts, which include GlaxoSmith-Kline's Triple Solution for a Healthier America and Merck & Co.'s Merck-Engage. Beyond the patient-friendly language & healthy advice, companies face growing pressure to expand access to medicines beyond the poorest patients by containing drug costs, with Washington now joining payers and providers in the squeeze. How well companies balance that pressure with their desire for profit will determine the success, or failure, of their patient engagement efforts. **GEN**