

# RARE VIEW

Online resources (both branded and non-branded) for patients with rare diseases help replace feelings of isolation with a community that offers emotional and educational support, **Marc Iskowitz** finds

**J**anet Long's childhood memories are painful ones. As early as age 7, she remembers being besieged by bouts of sudden, excruciatingly painful attacks in her stomach that caused her abdomen to swell up like a balloon.

The symptoms mystified her mother. "At that point, the only thing she could do for me was give me a hot water bottle and a couple of baby aspirin," Long recalls.

It took 40 years, but after numerous misdiagnoses Long learned she had the rare disease hereditary angioedema (HAE), a genetic disorder marked by attacks of uncontrolled swelling in various parts of the body including the stomach, hands, feet, face and throat.

"I had never met anyone with this disease," Long says. So the first thing she did was what most people with a health question would do—Google HAE and locate support. She quickly found an online patient group and "was thrilled to find people I had never met and couldn't see face-to-face responding to me with warmth and understanding."

Across the rare-disease community, patients and caregivers are using the internet to enlarge their support network. In fact, those with rare conditions have an outsized tendency to go online for a virtual shoulder to lean on and for practical advice in coping with day-to-day situations.

## One to many

According to a 2011 report by the Pew Internet & American Life Project, which surveyed people with chronic conditions as well as those with rare disorders, the people living with rare disease who responded far outpaced those in the chronic group in tapping the web to access peer networks or those with the same condition.

This is often by necessity, writes Susannah Fox, author of the

## The HAE pipeline

There are two products in late-stage development for treating HAE, a rare disorder marked by uncontrolled swelling. Both are already on the market in Europe, under the brand names Firazyr and Ruconest.

Product	Company	Indication	Status
Firazyr	Shire	Acute angioedema attacks from C1-inhibitor deficiency	Filed
Rhucin	Pharming NV	Acute angioedema attacks from C1-inhibitor deficiency	Phase III

Note: Following the FDA's previous refusal to review each of their biologic license applications, Firazyr's new PDUFA date is 8/25/11 and Rhucin's newest Phase III trial will not be completed until 2012.

Source: The companies

"Peer-to-Peer Healthcare" report and a Pew associate director, since rare-disease respondents are unlikely to live near to the people who share their conditions.

Turning to the web to seek out someone experiencing a similar change in physical health is nothing new, as any member of Weight Watchers can attest. But, "Rare disease seems to amplify this need to spread one's network far and wide," Fox writes. The connections inform, support and empower. As in Janet Long's case, they also help to replace isolation with community.

"HAE is such a rare disease that sometimes a virtual connection is the best one," says Long, who is now executive VP of the US HAE Association (HAEA), a patient advocacy organization with back-



ing from all the major stakeholders, including pharma. The group's online offerings include a listserv (at haea.org); Facebook group; physician CME program; and a Patient Empowerment Program (at haedu.com) with videos that help users take charge of their HAE.

Pharmaceutical companies, too, play a role in reducing feelings of isolation among the relatively small numbers of patients suffering from orphan diseases. To help these patients connect with others, specialty pharma companies are launching online hubs tuned to the needs of the orphan community.

"We consider each of these patients to be as important as a patient with a much more common disease," says Will Roberts, VP of corporate communications for ViroPharma, which markets the drug Cinryze. The biologic was the first product approved by the FDA for patients with HAE.

Prior to approval of Cinryze in late 2008, Roberts says, patients with HAE largely felt alone. The firm launched a support program last year, Ryze Above, designed to dovetail with HAEA's offerings.

HAE afflicts only about 6,000-10,000 patients in the US, yet three products are approved for the disease, and more are in development (see text box). All three are supported by branded and unbranded educational websites, and while some of the marketers acknowledge the significance of social networking for these patients, they are awaiting FDA regulation on the topic. To help increase awareness of the need for earlier diagnosis, research and access to treatments, some of the companies held events around Rare Diseases Day in February.

Cinryze is the only HAE drug approved for prophylactic use, as opposed to acute. Patients infuse the biologic every three to four days to prevent attacks, and Roberts says the goal of the Ryze Above program is to "build a sustainable relationship" with them and to offer emotional and other kinds of support.

"If we do our jobs with Cinryze, we are allowing these patients to have a normalized life without the fear of attacks," he says. "We've designed this community to create ways for patients to share their experiences to make sure they are well represented, well taken care of and that they have all the information they need to maintain a normal lifestyle."

Therese Heimbold, assistant director for Cinryze marketing, who

conceived of the Ryze Above program, says it can "inspire and motivate Cinryze patients through stories from real patients, one-on-one patient support and relevant HAE resources."

The program was designed by agencies Dudnyk and Cadient. Branded site ryzeabove.com, by Cadient, offers a therapy tracker, electronic reminders for doctor appointments, tips on living with disease, access to a journal to record attacks, and travel kits. It doesn't have a social media component yet.

"Social media is a very important platform for ViroPharma programs; it's something we will consider for this program, as well," says Roberts, adding that the firm is awaiting FDA guidelines on the issue.

### Band of others

Adding a real-time, social component might enhance the offering, although companies are hesitant to host such forums for lack of regulatory direction, and of resources for adverse-event monitoring. Still, like messages on an online bulletin board, even patient stories persist and can be a tremendous benefit to others.

"People living with rare disease, their own or a loved one's, have honed their searching, learning and sharing skills to a fine point," observes Fox in the Pew report. "They endlessly scan resources for clues to try and cope with and mitigate the inevitable complications and setbacks that come from rare diseases. What was once a solitary expedition for one person or family, however, has become a collective pursuit taken on by bands of brothers- and





**Dyax developed HAEHope.com, a non-branded disease website, to educate patients and create awareness about the condition**

sisters-in-arms who may never meet in person.”

It’s clear, Fox notes, that people who participated in Pew’s online survey—which included 2,156 members of the National Organization of Rare Disorders (NORD)—have taken what she calls the “social life of health information” to a higher level. This quality of being highly motivated to seek out support can be pharma’s opportunity.

“Because patients with rare chronic conditions don’t have as many places to turn for information and support, they are open to a direct relationship with a brand—if that brand is perceived to deliver value,” explains Wendy White, founder and president of Siren Interactive, a relationship marketing agency specializing in rare disorder therapies.

“So there is a really good opportunity for brands to provide tools, support and services directly and through the internet to people in these communities,” White says by e-mail. “Since these are complicated therapies, people only switch for very good reasons. The payoff of providing value is brand loyalty and better adherence.

“In this case,” she continues, “pharma and patients have similar goals—they both want patients to live longer, healthier lives on therapy (even if their motivation comes from different sources).”

**Different sources**

Still, when it comes to getting an accurate diagnosis and finding information about prescription drugs, health professionals are the most popular source, even among this highly networked group, the Pew report notes. Advice from peers merely supplements what a doctor or nurse may say about a health situation. Acting in a supportive capacity to the HCP is a role pharma plays well.

ViroPharma competes for attention against online HAE resources launched by the other two approved drugs in this disease state—IV infusion drug Berinert, for treating acute attacks in the face and abdomen, and Dyax’s Kalbitor, which is approved for acute attacks in all locations and is the only HAE drug that can be injected under the skin.

Berinert’s marketer, specialty company CSL Behring, offers personalized interactive tools through an unbranded site, AllAboutHAE.com. Launched in 2007, the site’s resources include a journal patients can use to

keep track of HAE attacks, as well as doctor appointments, medication, diet and exercise. A “Family Tree” section aims to help patients understand how HAE presents in families.

The Berinert Expert Network, a support program CSL Behring started last year, helps patients secure access to the drug and offers additional resources via a 24/7 phone line. Patients, caregivers and physicians do not need to register to access the information provided by either the Berinert Expert Network or AllAboutHAE.com.

Dyax has developed two websites relevant to the HAE community—HAEHope.com, a non-branded disease website designed to educate patients and create awareness, and Kalbitor.com. Patients can register to receive newsletters, but this is required for only one feature—submitting an HAE-related question to an expert on HAEHope.com.

To keep content fresh, visitors are encouraged to submit questions. Personal success stories from Kalbitor patients are also featured on Kalbitor.com. “The main idea is to make the sites personal, applicable and interactive for our users,” says Paul Fletcher, director of marketing at Dyax. “Ultimately, we try to get insights from our users as to what information they would like to receive, and then we strive to create programs to make it available to them.”

He says Dyax is exploring several interactive features based on their needs, including social media as a means to communicate with patients and caregivers. “Of course FDA guidance will help inform these initiatives.”

Dyax has also used print advertising to draw attention to the condition and to online resources, including a journal ad that won an MM&M Gold Award in 2010.

**Rare unity**

ViroPharma requires patients to register in order to access online features but has not released the exact number of those who have. At the end of 2010, the firm said that more than 600 patients were taking Cinryze.

To drive patients to ryzeabove.com, ViroPharma uses personalized emails, direct mail and SEO, said Roberts. The reason for putting features behind a firewall has to do with demographics and the specific needs of patients, he said, as well as patient privacy. Care coordinators have access to patients’ phone numbers.

ViroPharma said the most heavily used part of its site is the section where patients can listen to other Cinryze patients share experiences. Updates to this section are planned for this year. Says Roberts: “We do know [users] identify with these stories and want to make sure that part of the site is as up-to-date and fresh as it can be.” ■

