

The effectiveness and value of big-budget DTC in mega-mass venues—a TV spot during the Super Bowl, say—continues to be debated, particularly when you consider the general public's perception of industry that these kinds of ads tend to perpetuate. But that doesn't mean that even the most zealous social media proselytizers have, in quiet times, lusted after the scale available to traditional broadcast.

But what if scale isn't the issue? What if your total patient population, in the US and Western Europe combined, is only eight to 10,000 patients? "We take a far different approach to promotion," says David Hallal, SVP, commercial operations, Americas, at Alexion Pharmaceuticals. Alexion manufactures and markets Soliris (eculizumab), a monoclonal antibody indicated for the treatment of paroxysmal nocturnal hemoglobinuria (PNH), a blood disorder.

[hematologists] had heard of PNH, but most had never seen a case," says Irving Adler, senior director, corporate communications at Alexion, adding that in some cases they probably were seeing it, but couldn't identify the symptoms as PNH. To educate physicians, Alexion launched professional journal advertising "focused on the disease, and which patients are more likely to have it, and who [physicians] should test," says Hallal. Online, Alexion launched a brand site for Soliris—Soliris.net—and also an unbranded education site, PNHSOURCE.com. Built through a partnership with Cambridge BioMarketing, PNHSOURCE.com offers a plethora of information and resources for physicians, including incentives for learning more about the disease. For example, physicians that view an educational presentation geared toward helping identify PNH patients receive a Bronze PhRMA Card—a \$25 gift certificate for

An Orphan's Tale

When your patient population is just 10,000, the pharma marketing rulebook goes out of the window. **Ben Comer** examines one of the most successful ultra orphan campaigns of all time



der. PNH causes cells to be destroyed with symptoms ranging from extreme fatigue and shortness of breath to death. In fact, approximately 35% of PNH patients die within five years of diagnosis, and 50% die within 10 years of diagnosis.

Soliris, which contains a boxed warning on the risk of meningococcal infection, was designated an orphan drug by the FDA and approved in March of 2007. It was clear from the beginning that outreach to physicians, in terms of educating them on the disease, would be priority number one. "All of the [hematologists and

red blood cells, leading to hemolysis, and shortness of breath]

medically relevant gifts. A separate presentation offers a complimentary one-year subscription to Epocrates, a mobile drug and disease database service.

For patients and caregivers, PNHSOURCE.com offers information and a glossary of terms, as well as a link to PNHdisease.org, an independent patient community where individuals discuss their experiences with the disease, treatment and other related topics. Hallal says giving patients options—to jump from one website to another—is an important way to help them learn about their condition. Although Alexion is not officially connected to PNHdisease.com, the company does collaborate with the community, and monitors interactions to learn how best to serve PNH patients.

Prior to the launch of Soliris, Alexion launched One Source, a program that pairs PNH patients with a registered nurse case manager. “A majority of support programs are outsourced to third parties,” says Hallal. “We have a case management suite on site, staffed by nurses with experience in insurance reimbursement and hospital care.” Patients can enroll in the One Source program through their physician or by calling Alexion directly. Each individual patient is assigned to one nurse case manager, said Hallal.

Soliris is used as an infusion—every week for the first five weeks, and then every other week chronically—so providing patients with a support program, as part of the treatment, is important. Nurses educate patients before the first infusion, so they know what to expect, and are available to answer questions about the disease and Soliris. They also advise patients on access to the treatment, including coding and billing information, reimbursement and how to appeal a denied claim—an important offering, given Soliris’s

PNH is destructive, progressive, and life threatening.

Soliris®: The Power to Protect.
The first humanized monoclonal antibody (mAb) to reduce hemolysis and its harmful effects in all PNH patients.

- 86% reduction in hemolysis (as measured by LDH)
- Fewer thrombotic events were observed with Soliris in clinical trials
- The majority of patients (63%) received concomitant anticoagulant therapy
- The effect of anticoagulant withdrawal during Soliris treatment has not been studied
- 73% reduction in the need for transfusions across all patient populations
- 78% clinically meaningful improvement in fatigue; significant improvement in level of fatigue (as measured)
- Adverse event profile similar to placebo

Visit go.pnhinsights3.com or call 1.888.765.4747 to learn more about the benefits of Soliris.

WARNING: SERIOUS MENINGOCOCCAL INFECTIONS
Soliris increases the risk of meningococcal infections. Meningococcal infection may become rapidly life-threatening or fatal if not recognized and treated early.

- Vaccinate patients with a meningococcal vaccine at least 2 weeks prior to receiving the first dose of Soliris, re-vaccinate according to current medical guidelines for vaccine use
- Monitor patients for signs of meningococcal infections; evaluate immediately if infection is suspected, and treat with antibiotics if necessary

Soliris is generally well tolerated. The most frequent adverse events observed in clinical studies were headache, dizziness, back pain, nausea, and fatigue. Please see brief summary on the back page, including boxed WARNING regarding serious meningococcal infection.

SOLIRIS®
(eculizumab)

Alexion's Soliris is the most successful ultra orphan drug to date, with projected sales of between \$368 million-\$378 million for 2009

price tag, which exceeds \$300,000 annually.

Orphan drugs, as classified by FDA, are drugs serving patient populations of less than 200,000, and as such, receive exclusivity for seven years—an incentive for industry to develop treatments for rare diseases—under the Orphan Drug Act of 1983. Soliris is considered an “ultra orphan” drug, given the population of PNH patients. Within the ultra orphan drug subset, Soliris stands to become the most successful treatment to date, with global sales for 2009 estimated to be between \$368 million and \$378 million, according to statements

Tips from an expert

Kristin Keller, VP, client services at Compass Healthcare, worked on the launch of Alexion's Soliris, and has developed social media platforms and other programs for Biomarin's Kuvan, an orphan treatment for PKU, and Ipsen's Somatuline Depot, an injection for acromegaly.

Rare diseases tend to be serious, chronic conditions, and that is reflected in a community's dynamic. “Patient communities tend to be somewhat cohesive—you have key influencers and thought leaders within the patient community,” says Keller. To that end, marketers should be aware of not just individual personalities in the patient group, but also the group's collective personality—because they differ dramatically, says Keller,

PNHSource®
Personalized nocturnal hemoglobinuria (PNH)

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Bronze PhRMA Card
Patients in your practice may have PNH. View an interactive, educational presentation that can help you identify these patients by using the PNH Diagnostic Pathway. When finished, receive a complimentary* Bronze PhRMA Card (a \$25 value).

[Click here](#)

Paroxysmal nocturnal hemoglobinuria (PNH) is a progressive and life-threatening disease that causes thrombosis, end organ damage, impaired quality of life, and increased mortality.¹⁻³

Important facts about PNH:

- Chronic hemolysis is the underlying cause of progressive morbidities and mortality in PNH¹
- Thrombosis is the leading cause of death in PNH¹⁻³
- Early diagnosis drives effective PNH management
- Monitoring hemolysis with lactate dehydrogenase (LDH) provides a more accurate assessment of PNH
- Evaluate your patients with myelodysplastic syndromes (MDS) at diagnosis and your patients with aplastic anemia (AA) at diagnosis and annually

PNHSource.com offers information about PNH, a glossary of terms and links to PNHdisease.org, an independent patient community

made during Alexion's second quarter conference call in June. During the second quarter, Soliris garnered \$92.3 million in net product sales, up from \$59.6 million during the second quarter of 2008—a 55% increase. In sequential quarters, sales of Soliris increased by \$11 million, or 14%. Physician outreach and education has played a key role in Soliris's success, as evidenced by the increasing portion of patients on Soliris that are newly diagnosed PNH patients, said Leonard Bell, Alexion's CEO, on the call.

According to Wolters Kluwer Pharma Solutions, Soliris currently ranks 65th in sales among all orphan drugs, ultra or no, at \$42.5 million in sales for the 12-month period ending in August. By comparison, the top three selling orphan drugs, Centocor's Remicade (\$3.2 billion), Amgen's Epogen (\$2.7 billion) and Genentech's Avastin (\$2.6 billion), have all received additional indications since their initial FDA approval, that extend their reach to larger patient groups.

Alexion hopes to extend Soliris into other indications as well, and is currently conducting clinical trials to study the drug as a possible treatment for atypical hemolytic uremic syndrome (aHUS) in the US and several European countries. Like PNH, aHUS is also an orphan disease, and is caused by a genetic mutation that causes the hemolytic destruction of red blood cells. Alexion is also evaluating Soliris in high-risk kidney transplant patients.

On top of new indication possibilities, Soliris is moving into new markets. Last February, the treatment was approved for PNH in Australia, and was approved in Canada in July. Alexion plans to launch the drug in Japan during the second half of 2010, and will begin investigating markets in the Middle East, according to second quarter reports. ■

adding that the PKU.com community is much different than the acromegaly community.

Keller says small patient groups allow marketers to invest in more meaningful and value-added resources, and that's what motivates a behavioral change.

“A heartburn drug would never spend as much as an orphan drug per patient,” she says. “Brand goals are the same, but it's the scale of the measurement that can change. It's important to lay out the goals upfront.”

