

By Kim Ribbink

A Nuanced Message: *Marketing to the Rare Disease Community*

Rare disease marketers must establish trust with a community of stakeholders, including the patient and their circle of care, as well as payers and policy makers.

Check traditional marketing strategies at the door, because when it comes to rare diseases they simply don't apply.

Experts say established methods of direct- ing campaigns toward physicians don't work because unlike large therapeutic categories, such as cardiovascular or diabetes, the patients and their caregivers must be front and center.

"Patient-centricity in both product development and commercialization is the key consideration with rare diseases," says Alain Gilbert, co-chairman of Bionest Partners. "Orphan drugs are often initiated by patient associations that work to motivate companies — typically biotech companies — to develop products for these smaller populations, whereas traditional pharma companies tend to define the patient need and then develop a program. This is the biggest difference between rare disease and more traditional pharma."

Denise Von Dohren, VP, Access Solutions, at RxCrossroads Specialty Solutions, says because the patient population for rare diseases is much smaller than with other diseases, a targeted marketing approach must be used to the patients and caregivers as they have a strong desire for continuous learning regarding their disease.

"Considering more than 50% of rare diseases touch children, reaching parents, caregivers, and advocacy groups should be the first stage of outreach, and incorporating frequent and personalized methods of engagement into a support program is extremely important," she says. "Caregivers of young patients thrive on information, so consideration should be given to strong digital campaigns using social media and viral marketing."

Information must be comprehensive and specific for multiple stakeholders, Ms. Von Dohren says. "Healthcare professionals, patients, and caregivers need information regarding therapy access, financial support, patient-assistance programs, clinical nursing support, and disease education," she notes.



In rare diseases, it is plausible for pharmaceutical marketers to have direct relationships with literally all of their patients, says Matthew Howes, executive VP, strategy and growth, Palio, an inVentiv Health company. "In fact, that should be their goal," he says. "Once the relationship is established, marketers can then co-create content and deliver content interventions that address gaps in care beyond the product."

This includes help in overcoming stress and depression, and providing patients with an understanding of their condition, a sense of control, a network of support, and an outlet for emotional frustration, Mr. Howes adds.

"For rare diseases, support must be for patients and families through every step of the journey, including disease education, drug

information, reimbursement help, and lifestyle management," he says.

Among the most important voices for patients with rare diseases are the patient associations.

Indeed, patient associations have been central to the development of products for patients suffering from rare diseases, and these organizations work to incentivize companies to develop products for patients suffering from these conditions.

"Patients and caregivers often join rare disease communities in order to connect with others who are experiencing similar patient journeys because so little information may be available," Ms. Von Dohren says. "Helping with this disease engagement assists the patient in receiving a comprehensive patient experience."

Those associations are very influential in terms of access to patients, and rare diseases companies work closely with them to organize focused events and build personal relationships with the patient, says Anne Sophie Demange, manager, Bionest Partners.

Building relationships with advocacy groups can go a long way in connecting with patients, as well as getting disease education, treatment information, and support, Ms. Von Dohren adds.

"Partnering with patient advocacy groups that have relationships with patients will provide feedback on the best way to communicate with a specific patient population," she says.

It is critical to engage with patient organizations early in the development process when operating in the rare disease space, says Bradley Campbell, president and chief operating officer at Amicus Therapeutics.

"At Amicus, we have a chief patient advocate who not only is responsible for helping ensure that Amicus advocates on behalf of patients in the community but also ensures that we advocate for patients internally as well," Mr. Campbell says. "As an example, Amicus was one of the first companies to use patient advisory boards to incorporate the patient



From inception, we have successfully created strong relationships with patient organizations and we have a dedicated team engaging with patient communities. We have regular patient advocates speak to our employees to create a thorough understanding of the patient's hopes and needs.

DANIEL DE BOER
ProQR

voice throughout the development lifecycle. More broadly, we work closely with patient advocacy organizations around the world to not only support the patient community but also provide transparent information to them in an appropriate and compliant way.”

Mr. Campbell cites as an example the patient-friendly tools and services Amicus has developed in partnership with a patient advisory board to help patients with Fabry disease remember when and how to take Galafold (migalastat) to get the most from their medication. Galafold was approved in Europe in May 2016.

Helping the Physician

The role of the physician remains vital in rare disease marketing, but pure marketing strategies are ineffective in this space.

“Everything is around medical initiatives rather than mass marketing as would be the case with large disease categories,” Ms. Demange says.

In rare disease marketing, strategies often include disease awareness initiatives that are focused on helping specialty healthcare professionals better understand and recognize underappreciated diseases, says Christopher Tobias, Ph.D., president of Dudnyk.

“These initiatives are generally tailored to messaging about pathophysiology, clinical presentation, and diagnosis to help arm the target specialist with the ability to identify and treat a rare disease,” Dr. Tobias says. “In our experience, these initiatives are still missing an important component of education and that is to help specialists understand the burden a rare disease can put on a patient's life.”

Mr. Campbell says because there is low awareness among physicians for many diseases Amicus targets, reaching the right audience in an authentic and educational way is critical. It is, therefore, important to identify who the thought leaders and key patient organizations are and how to speak to them on an appro-



The top consideration for rare disease marketing is that information must be comprehensive and specific for multiple stakeholders.

DENISE VON DOHREN
RxCrossroads Specialty Solutions

appropriate scientific or lay level, while also understanding where to go to identify new patients.

“Medical congresses become more important in rare diseases as there are fewer forums to share data and have quality interactions with the medical community,” he says. “Healthcare providers are more engaged and collaborative, and we have the opportunity to make a greater impact on patient care.”

One way to reach physicians is through access programs before authorization, Ms. Demange says, who cites France's Temporary Authorisations for Use program as an example.

“The idea of such programs is to get support from physicians before the drug is commercially approved by giving them experience with the drug and helping them understand how it works,” she says.

Mr. Howes agrees that marketing therapies in rare diseases begins long before the first promotional tactics appear in the market, noting that during the clinical development process it's about identifying unmet needs, managing trial design, and patient recruitment.

Movement Marketing in Rare Diseases

According to an Interpublic Group, Emerging Media Lab Survey from 2010, 92% of consumers said they have a more positive image of a product/company when it supports a cause and 87% of consumers (when price and quality are equal) are more likely to choose a brand associated with a cause. One way this is being tackled is through what Siren Interactive, a Dohmen company, refers to as movement marketing, which empowers patients by rallying them around a cause and motivating them to take action. Movement marketing is on the rise, partially because the impact social media has in making movements more powerful and broader in reach.

Movement marketing is a natural for rare disease communities because:

- ▶ Patients are already on social media — it may be the only way they can find each other.
- ▶ They care deeply about the cause — they have to in order to get properly diagnosed and treated.
- ▶ They want to help others — they know how hard the journey can be and they will do everything they can to help others along the way.

Guidelines for Success

- ▶ Align with the needs of the community. What is it they want to do: raise money for research, increase diagnosis, help educate HCPs and legitimize patient's concerns?
- ▶ Partner with trust agents and, if necessary, be a bridge builder for relevant patient organizations.
- ▶ Tie into important events in the community or patients' lives.
- ▶ Provide tools — real or virtual — that have significance and are easy to share
- ▶ Crowd source content — stories, photos, messages, etc.
- ▶ Connect to the emotional center — the thing people in the community are passionate about.





A shift in strategic ideology from a sole focus on either specialists or patients to a more unified approach will help the audiences achieve the best interaction possible in a rare disease setting.

DR. CHRISTOPHER TOBIAS
Dudnyk

According to Ms. Von Dohren, with such small patient populations there are a limited number of physician experts across the United States and locating and educating these “centers of expertise” about support services available to these patients by pharmaceutical companies is critical.

Michael Carlin, VP of HCP engagement at TrialCard, says orphan drug marketers need to understand how to map the diagnostic patient journey so they can better identify points in care management to educate physicians on who is and who is not an appropriate patient for their therapy.

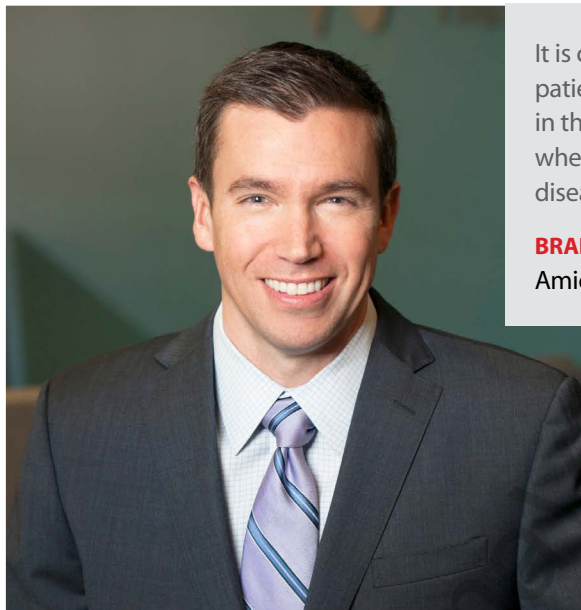
“But with physicians only seeing perhaps one patient with a particular disorder in their career, this education can be challenging,” he says. “This makes finding patients a tremendous challenge for marketers.”

Resources are needed to simultaneously educate physicians while determining if they have a patient who would benefit from the therapy, Mr. Carlin says.

“The approach needs to be broad enough to be in front of the physician while that patient is top of mind,” he says. “Conventional orphan field forces face geographical challenges in doing that. They need account support services that help maximize their time in the field.”

Physician education programs need to provide appropriate diagnostic and product efficacy messaging and offer an understanding of the economic burden of not treating the patient, Mr. Carlin says, who adds that this is a large message to deliver to an audience that may see very few patients with a specific disease.

Dr. Tobias notes that because patients live



It is critical to engage with patient organizations early in the development process when operating in the rare disease space.

BRADLEY CAMPBELL
Amicus Therapeutics

lishing trust with a community of stakeholders that includes payers, policy-makers, patients and their circle of care: family, social workers, nurse practitioners, nurse navigators, doctors, advocacy groups, and many others.

“To create a sense of trust, marketers must take an empathetic approach to patients and caregivers,” he says. “They must walk in their audience’s shoes and learn that in rare disease, patients and caregivers wear many hats.”

Since rare disease development addresses smaller patient populations, prices tend to be high. Patients with rare diseases are often concerned about their ability to pay for their therapy and other health-care, Ms. Von Dohren says.

“Offering support services such as financial support, patient-assistance programs, clinical nursing support, and disease education are imperative to marketing these therapies,” she says. “Patient education, including nurse home visits for self-administered products to show proper administration is also key to alleviating the patients’ concern about their therapy.”

Mr. Carlin recommends a mix of quantitative data such as patient registries, ICD-10 and physician behavior activities, as well as a qualitative element to finding patients.

“For example, employing a highly trained specialist to canvas through outbound calling and Internet services to talk to the offices about rare and ultra-rare patients,” he says. “The combination of quantitative and qualitative increases efficiencies and success rates in finding patients.”

He adds that patient registries are an invaluable way to engage with patients, saying manufacturers should be in communication with these communities of patients to appropriately inform patients and caregivers about opportunities.

According to Mr. Howes, because corporate reputation is a critical component of any marketing strategy for a rare disease, it must

Patient organizations have tremendous influence in terms of access to patients, so rare diseases companies tend to work closely with those organizations.



ANNE-SOPHIE DEMANGE
Bionest Partners

with symptoms for years or even decades, they may see several specialists in search of a correct diagnosis, while also experiencing feelings of loneliness, frustration, and despair.

“Helping specialists understand the impact of the disease on the patient allows them to fully apply the knowledge gained from a disease awareness initiative,” he says. “Shifting from a sole focus on either specialists or patients to a more unified approach will help the two audiences achieve the best interaction possible in a rare disease setting.”

Mr. Campbell adds that it’s important to have tailored patient tools and services in rare diseases. With its lead molecule migalastat, which is approved in Europe under the brand name Galafold, the company has created the first online genetic lookup table where physicians can simply enter their patients’ mutations into the search function and determine whether migalastat may be suitable for their patient.

Finding the Right Mix

Mr. Howes says the primary challenge of marketing therapies for rare diseases is estab-



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HIGH-TOUCH

Commercializing an orphan drug requires designing solutions to improve the treatment journey. In-home inventory management solutions combined with sophisticated logistics expertise makes participating in a clinical trial more convenient, easing recruitment and reducing withdrawal rates. Increasing patients' access to treatment, improving adherence, reducing costly emergency visits, and enhancing the quality of life for both patients and caregivers takes high-tech solutions and high-touch patient support. It takes a committed commercialization partner. It takes AmerisourceBergen.



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be carefully managed long before promotional activities of a drug begin.

Since 2010, the number of compassionate use requests has risen by nearly 25%. Many of these requests are for rare diseases with small patient populations, with highly engaged patients and caregivers, connected with vocal advocacy groups.

The FDA has clear guidelines on requirements for meeting expanded access, but it's up to companies to say which cases to support and which to deny. Many companies are unprepared to respond quickly, leaving their corporate reputations vulnerable.

One of the better-known examples happened several years ago when Andi Sloan, a Texas lawyer, petitioned BioMarin for access to its experimental Phase III ovarian cancer drug. When BioMarin refused access, Ms. Sloan began a social media campaign that generated widespread support.

Mr. Howes says companies in the rare diseases space should anticipate an increase in compassionate use requests and be ready to respond.

Some experts say the rare diseases space is difficult for large pharma and tends to be better managed by smaller, more agile biotech companies.

The problems are multi-factorial, Ms. Demange says. Since many rare diseases aren't properly diagnosed it's difficult to know where the patients are or how many there are. Rare diseases are also not consistently managed and the patient journey is not straightforward. Additionally, in rare diseases, companies need to work with a much wider network of stakeholders than with diseases that have many patients.

"The challenge for marketers in larger companies is that they have been educated around the notion of prescriber-focus and are accustomed to working with doctors and key opinion leaders, whereas with rare diseases the focus is the patient," Mr. Gilbert says.

However, Mr. Howes says the advantage big pharma companies have is that they have years of experience with co-payment assistance programs that connect patients to resources such as nonprofit foundations that assist with co-payments and help improve access and retention.

According to Mr. Carlin, the interest that some bigger companies are showing in orphan drugs has brought more attention to rare diseases. Recent industry deals have brought visibility to the product model, demonstrating the financial and extended exclusivity advantages of these products, but there is a lot more to it.

"There's also an energy seen in teams when they discover they just connected a patient to a



The biggest issue when marketing in the rare diseases space is identifying the patients; it's like finding a needle in a haystack.

ALAIN GILBERT
Bionest Partners



The goal of pharmaceutical marketers in rare diseases should be to have direct relationships with literally all of their patients.

MATTHEW HOWES
Palió, an inVentiv Health company



There's an energy seen in teams when they discover they just connected a patient to a therapy that will make their life better, or even save their life.

MICHAEL CARLIN
TrialCard

therapy that will make their life better, or even save their life," he says. "It makes it personal."

Market Changes

Ms. Demange says the approach in very rare diseases hasn't changed much but in some areas increased focus has had a significant effect. Years ago, when Avonex, the first drug for multiple sclerosis was being investigated, MS was considered a rare disease. But as more companies have entered the MS market, many more patients with the disease have been identified and today MS has become a competitive market.

One significant change is the way rare diseases are viewed by drug manufacturers, many of which understand that this is a growing market with increased competition.

"The cystic fibrosis field has become more crowded, but there are still so many diseases with no satisfactory treatments," says Daniel de Boer, founding CEO of ProQR, a biotech company focused on development of drugs to treat severe genetic disorders.

Mr. Campbell notes that having multiple treatment options leads to more emphasis on engaging with the community in a way that is differentiated from other companies in the space while still ensuring that the message is trustworthy and engaging.

In addition, Ms. Von Dohren says payers are becoming more focused on rare diseases and are putting cost pressures on manufacturers to make them affordable to patients.

"Comprehensive patient support services

including therapy access, financial support, patient assistance programs, clinical nursing support and disease education have become the expectation for patients and their caregivers," she says.

Among new marketing strategies, social media presents both interesting challenges and opportunities for rare diseases-focused companies, experts say.

"We are still working to understand how best to utilize social media to reach more people more quickly with a more intimate conversation that still fits within our regulatory and compliance framework in the pharmaceutical industry," Mr. Campbell says. "As patients and physicians adopt social media as their standard communication channel, this should force a continued evolution of this communication medium."

Beyond the efforts of specific companies there have also been some public campaigns designed to turn attention to rare diseases. One of the more successful such campaigns was the ice-bucket challenge to promote awareness of amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig's disease.

"By raising awareness about potential therapies, the ice-bucket challenge also helped to increase the number of patients diagnosed," Mr. Gilbert says.

In July 2016, the ALS Association announced that thanks to funding from the Ice Bucket Challenge, the University of Massachusetts Medical School and researchers from 11 countries identified a new ALS gene, NEK1. **PV**

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