

How to Effectively Engage in the Rare Disease Space

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What Are Rare Diseases?

The rare disease landscape is complicated. According to the National Institutes of Health, there are nearly 7000 known diseases deemed “rare” because each affects fewer than 200,000 Americans, and some of these diseases, deemed “ultra-rare,” affect only a few hundred individuals. Taken together, approximately 30 million Americans and 350 million people worldwide have a rare or ultra-rare disease.

Over the past 4 decades or so, the arsenal of treatments for rare diseases has grown from single digits to a few hundred, and the pace of discovery continues to pick up. There are about 460 medicines for rare diseases in clinical development or under FDA review addressing rare cancers, genetic disorders, neurologic disorders, and infectious diseases, among others.

Challenges Faced by Health Care Practitioners Treating Rare Diseases

Most physicians never see patients with rare diseases. Insufficient exposure prevents them from building familiarity with the processes of diagnosing and treating these illnesses. Simply getting a diagnosis is often a long and difficult quest that takes patients and their families through a series of health care practitioners. Diagnoses are often missed, delayed, or inaccurate, even when an effective therapy is available. For many rare diseases, physicians’ lack of awareness is compounded by a limited opportunity to network with other physicians about these diseases, insufficient attention from professional medical organizations to these diseases, and difficulty coordinating patient care with other physicians.

The physicians who are aware of rare diseases and work with these patients put their patients at the core of everything they do. Since treatment options for rare diseases are limited relative to the number of available options for more prevalent diseases, physicians tend to be more aggressive in treatment and more willing to spend time supporting patient advocacy as it relates to both treatment and reimbursement.

Challenges Faced by the Field Selling Products Indicated for Rare or Ultra-Rare Diseases

In general, there is a lack of patient data, market data, and information on how to interact with customers in the rare disease market. Given this situation and the low level of familiarity of most physicians with these diseases, the first challenge facing a sales force marketing a therapeutic in this space is raising clinicians’ awareness of the disease so that diagnoses are made in a timelier manner. A closely related challenge is the need to provide tools for physicians to help them identify patients with rare diseases. Since about 80% of rare diseases have a genetic origin, it is important to ensure that physicians understand the hereditary component underlying these diseases. Diagnostic genetic tests are available for some rare diseases, though are still absent for most. For those rare diseases that have a diagnostic test, recognizing which family members of afflicted individuals to screen is important. Being able to address issues raised by physicians regarding test validation and interpretation of results is also needed.

From here, a careful balance of science, economics, and humanitarianism must be delivered. The next challenge is telling a plausible scientific story that will increase understanding of the underlying pathophysiology of the rare disease, which is often more complex than that of common diseases. Because their patient populations are limited, these diseases are more difficult to study. Yet, the ability to explain the scientifically intricate mechanism of a disease and the mechanism of action of a new treatment is vital.

The third challenge of marketing a therapeutic in the rare disease space is being able to paint a picture of how patients and their families are impacted by the disease. Understanding these emotional aspects and conveying them to health care providers is of the utmost importance: it will heighten physicians’ sensitivity when they speak with patients and their families, and will make them more aware of why it is so important to take the time to listen to their patients and answer their questions.

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The fourth challenge for the sales force is being able to appreciate the value provided by a therapeutic for a particular rare disease. Standard metrics used for common diseases may be irrelevant for rare diseases. The value of a therapeutic includes the quality of patients' responses, and not just the number of responses in a given patient population. New metrics, such as prolongation of stable disease, requirement of longer time to additional treatment, ability to partake in more activities of daily life, and the emotional satisfaction of not being passed over by the health care system, must be taken into account.

The final challenge is being able to thoroughly address the economics of orphan drugs such that payors and national health services will support a price that is fair for both manufacturers and the concerned public. There is a high cost for treatment associated with developing and manufacturing complex biologics versus chemical drugs whose production is typically simpler and less expensive. Understanding how payors feel about the product, and being able to communicate about the product's value as a potentially lifelong and life-altering therapy for a chronic illness, is mandatory.

What Do Health Care Practitioners in the Rare Disease Space Require?

It is important for industry sales representatives to help physicians build familiarity with these rare illnesses and available diagnostics and therapies in order to facilitate more accurate and timely diagnoses and ultimately to improve patient outcome. This requires representatives to be thoroughly knowledgeable, equipped to address clinicians' difficult questions and issues, and sensitive to patients', caregivers', and physicians' challenges.

When physicians are asked how interacting with sales professionals about rare diseases and treatments differs from interacting about common ailments, they claim to want more timely, frequent, and in-depth information about available diagnostic and treatment options from representatives. They also desire assistance with establishing and growing their network of professionals contributing to the rare disease landscape, including other physicians, researchers, advocates, medical associations, and reimbursement specialists. This weak support system must be strengthened to grow physicians' abilities and confidence, so they can better serve their rare disease patients.

What Do Sales Teams Who Market in the Rare Disease Space Require?

Several common medical disorders, such as diabetes, hypertension, and hyperlipidemia, are well defined and have clear paradigms as a result of large historical trials. To gain similar ground with rare diseases, pharma and biotech companies developing drugs and tools in the rare disease space must first create "medical champions" who are knowledgeable and passionate about these diseases. The companies must then help develop the pathways needed for the differential diagnosis and management of the rare disorders. The sales forces must then engage and educate customers so that something rare is still top of mind.

Training to sell in the rare disease space is different than training to sell for common disorders. A small but knowledgeable sales force must be able to target academic and community specialists. These sales representatives MUST be trained to be one step ahead of the medical community, more informed and conversant than the physicians they call on, and better able to provide guidance on how to generate a differential diagnosis.

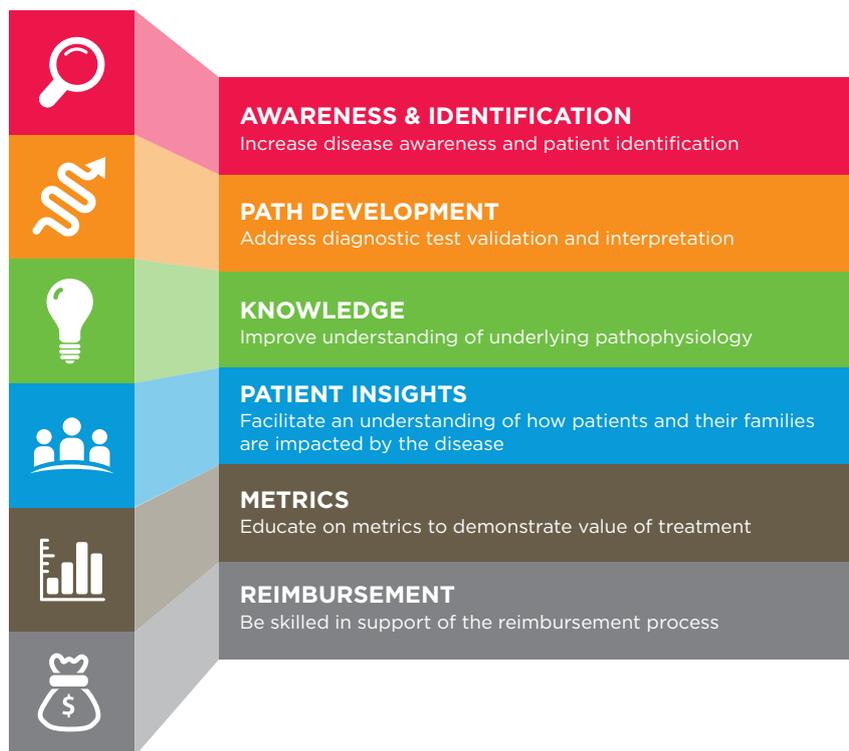
A successful sales representative needs to better understand their customer—inside and out. It's not enough to know only about the drug or to understand the treatment algorithm. They must also comprehend the patient's journey to diagnosis and the CUSTOMER'S journey to diagnosis, as well as how they can support the reimbursement process for the product. Training managers say that training in the rare disease space is therefore longer and more intense.

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Medical platforms that endure longer for common conditions become outdated more quickly for a rare disease. Follow-up training must be more frequent. Sales representatives may be pulled out of the field as much as twice a year for additional live training. Core material is reviewed and new material is added. Companies selling in the rare disease space continuously collect insights from the field, the marketing team, and advisory boards to guide follow-up training. Training managers will directly ask their sales forces "What do you need?"

The representatives have more of an active role in their ongoing training, since they are routinely asked about their daily challenges and their responses guide the new training. Sustainable training can be further facilitated by collecting questions from rare disease communities and the EURORDIS Round Table of Companies (ERTC) workshops to address topics of relevance to stakeholders regarding treatment. Ongoing training must continuously improve the ability of sales forces to effectively communicate with physicians so that they can, in turn, address patients' questions and concerns.

Hired sales representatives in this space tend to be seasoned and able to comprehend the complex science behind the rare disease they represent. All training is medically driven and particularly content heavy; representatives are often given a more robust version of the content found in typical medical speaker slides. As much as 98% of training is focused on the hard clinical science, whereas the remainder addresses the emotional aspects of the disease and may include unscripted patient and family testimonials.



Challenges for Sales Forces Marketing Products for Rare Diseases

What Role Does a Sales Force Effectiveness Vendor Play in the Rare Disease Space?

Companies that sell products for rare diseases need sales force effectiveness vendors who understand the landscape and are fully committed to the care of patients with rare diseases. An appropriate vendor will have in-house medical content experts who can stand toe-to-toe with the company training managers and discuss the detailed scientific aspects of the disease mechanism, manifestations, and diagnostic pathways; methods of assessment of disease severity or flare; and treatment goals. The vendor will also understand the spheres of influence, concerns, and considerations of medical specialists, and be adept at training seasoned representatives to develop a mastery of the clinical science to be able to deliver on all the challenges they face.

Ultimately, to achieve further progress in the rare disease space, a coalition among patient-focused training solutions experts, primed pharmaceutical and biotechnology companies, and informed health care providers must exist.

About the Authors



Roz Blumenthal, PhD, has 25 years of scientific experience since receiving her PhD in biomedical science, with a specialization in physiology and biophysics. Roz has an additional 10 years of experience in medical communications working in promotional medical writing,

health care advertising, scientific strategy, and sales training. She has worked on numerous agents in prelaunch and launch across multiple therapeutic categories, including hematology, oncology, immunology, women's health, neurology, endocrinology, and rare diseases.



Brandy Weidow, MS, ELS, brings nearly a decade of experience in scientific and medical communications to her position as medical editor with MXM Health. Brandy has bachelor's and master's degrees in microbiology, with minor studies in statistics and English,

as well as some post-graduate training in scientific writing. Her therapeutic areas of expertise include oncology, women's health, respiratory diseases, immunotherapy, and drug design.

About MXM Health

Meredith Xcelerated Marketing (MXM) Health is focused on providing targeted, custom solutions for training pharma and biotech sales representatives who interface with health care practitioners. They have acquired and groomed remarkable expertise—in science and medicine, communications, adult learning, and technology—that positions them to be leaders in sales training on therapies and diagnostics for the rare disease space. In the last 2 years alone, they have teamed with several industry leaders to produce engaging custom training solutions on over a dozen rare or ultra-rare diseases.

Please contact **Roz Blumenthal, PhD at 248.246.5398 or e-mail her at roz.blumenthal@mxm.com** for further information. MXM Health, part of the broader MXM group, is the premier agency in the health care training and marketing arena, offering full-service learning design and development solutions for the life sciences industry.

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