Impactful Rare Disease Campaigns Are Grounded in Medically Inspired Approaches

Patient-rooted strategies are essential in rare disease communication and commercialization.

No matter what stage a company is at, or what therapeutic area it is serving, one thing is certain: Rare diseases present unique commercialization and communication challenges that require a deep understanding of not only the disease but also the vast impact it can have on patients and care partners, as well as healthcare practitioners.

It takes a holistic, coordinated approach from day one to make an impactful difference. That means deeply understanding every rare disease touchpoint and bringing all of those stakeholders — doctors, patients, care partners, access experts, strategists, creative superstars, and more — together as early as possible to ensure a thoughtful, unique approach every step of the way.

Fingerpaint, a full-service health and wellness agency, reached across its companies to bring together the leaders who are experts in their respective fields to share their approaches and perspectives on executing successful communication and commercialization strategies in the rare disease space.

Medical Foundation

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Medical Strategist
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With over 7,000 rare diseases, no two are alike, yet they are each scientifically complex and require a deep understanding of the pathophysiology and mechanisms of disease. In addition, treatments for rare disease often include novel mechanisms of action and cutting-edge science that requires a high degree of scientific literacy to elucidate. Adding to this complexity is a dearth of scientific literature, diagnostic algorithms, and treatment guidelines that often comes hand-in-hand with the rare nature of these conditions.

Not only are the diseases themselves complicated, but patient and prescriber needs are likewise often complex. The highly educated nature of many patients with rare diseases raises the stakes when it comes to understanding and empathizing with the conditions that these patients live with every day. After all, a rare disease is anything but rare to the person suffering from it.

To effectively communicate with a person affected by a rare disease or a physician treating one, every single member of a communications team must have the same thorough and comprehensive understanding of the condition and underlying scientific challenges. A dedicated medical team brings a deep scientific understanding of the subject that allows them the unique ability to simplify complex science without sacrificing the nuance necessary to effectively communicate with patients and prescribers.

Medical strategists know the condition inside and out and help ensure that everyone on the team is educated, aware, and understands the unique issues associated with the condition. Arming everyone with a solid grounding in disease knowledge is a key component to producing impactful, engaging, and moving work.

Patient Perspective

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All chronic health conditions place demands on people who live with them. With rare diseases, these demands are greatly amplified, and resources may be harder to access. Patients and families may find roadblocks throughout their medical journey, such as obtaining a timely and accurate diagnosis, finding and accessing safe and effective treatments, and coordinating care among specialists and other providers for more routine health needs. Along with these challenges, families face significant social and financial strains as well, such as the time and costs of travel to specialized care settings, balancing the needs of the child (or adult) affected by the rare disease as well as the needs of other family members, and the isolation they may feel due to restrictions related to the affected person's abilities or treatment needs.

> Companies and brands in the rare disease space have the power to change lives.

Yet many families display incredible resilience in managing these issues. They seek information — often written for healthcare professionals — and use it for their own knowledge and in conversation with the extended healthcare team. They speak openly about their needs not only in the examination room but at school and workplaces and in public policy forums as they advocate for access to treatment and services and funds to support research.

For the companies dedicated to developing and providing treatment for rare diseases, it is critical to learn from these families in meaningful ways. While inviting a family to speak or sharing video clips from an ethnographic study at a leadership meeting can be impactful, there is much more to learn and do.

Consider ways for cross-functional teams, such as clinical operations, medical affairs, market access, and marketing, to participate in an immersion program co-created by family members (who are compensated for their time), advocacy partners, and training leaders. Look for ways to bring to life the integrated patient experience, including clinical care, financial impact, work and school settings, and social activities.

The Commercialization Difference

Dan Schroen
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Photo 51, a Fingerpaint company

Approaching rare disease commercialization with a

deep understanding of the medical intricacies of the disease and the patient's perspective, combined with seasoned leaders in all aspects of market access and commercialization, is advantageous when bringing new, advanced therapies to market.

It starts with prioritizing the understanding of a client's situation, challenges, and needs before developing personalized solutions. Applying a responsive and collaborative core team to support the needs of the fast-paced rare disease industry allows for comprehensive service across the entire drug development spectrum. By providing dedicated resources, laser-focused to overcome complex scientific and operational obstacles, the goal is to bring rare disease drugs to market faster and better through customized solutions, personal experience, and data-led decision-making.

What are some challenges unique to rare diseases, and how do we turn them into opportunities met? We encounter hurdles that at their essence boil down to scarcities in knowledge, expertise, coordination, preparation, and incentive. For instance, educating and involving various demographics yields an informed, comprehensive public health approach. Understanding and navigating legislative, economic, and regulatory functions incentivizes and catalyzes development of rare disease drugs or repurposing of existing therapies to rare diseases.

The Complex World of Access

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The Orphan Drug Act has incentivized manufacturers to research and commercialize therapies for treating rare conditions. Orphan drugs are potentially effective treatment options; however, payers are averse to them because of their perceived high costs and the relatively sparse clinical data on their use. Additionally, once the number of orphan drugs for which the Food and Drug Administration (FDA) has also approved nonorphan indications is factored in, payers' perspectives become more complex. The challenges in achieving market access begin in clinical development. There are a limited number of — and a probable lack of heterogeneity in — individuals eligible for any given study. These factors put the reliability of the study design, sample size, and power at risk. Because the Orphan Drug Act does not create a unique statutory standard for the approval of orphan drugs, FDA approval does not guarantee payer coverage. Regulators and payers may disagree in terms of comparators, endpoints, and details of the overall efficacy and safety. Most important, the evidence informing coverage decisions varies among economic stakeholders.

> Patient-rooted strategies are essential in rare disease communication and commercialization requiring a holistic approach.

Achieving coverage and payment requires substantial evidence of the clinical and economic benefits of a newly approved orphan product. Manufacturers who neither prepare for market access requirements nor consider health economics in the clinical development plan may struggle to gain reimbursement after FDA approval.

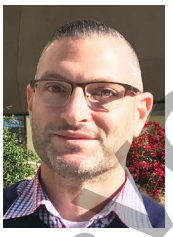
Even for patients whose health plans cover orphan drugs, payers may attempt to contain costs via utilization controls. To minimize control implementation, manufacturers should demonstrate to payers, whenever possible, that the overall cost exposure to a plan is low — a function of disease prevalence, the number of indications, and the potential for off-label use.

Finally, access can vary widely and should not be conflated with affordability. For many patients with serious illnesses, rising premiums, deductibles, and copayment/coinsurance requirements create economic hardships. Manufacturers must be prepared to support patients with financial assistance programs.

Manufacturers should not operate under the assumption that an orphan therapy designation for a small patient population justifies high prices.

A complete value assessment is required from clinical development to commercialization to ensure equitable and sustainable access to life-changing therapies.

A Creative Lens



Craig Mattes
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Discussing the approach to creative work in the rare disease space as a whole is challenging because every single rare disease is completely unique. There are, however, three common threads critical to breaking new ground for rare disease brands: empathy, insight, and empowerment.

It's so important to place oneself in others' shoes and imagine what it must be like to deal with a rare condition that's disrupting a person's, or their child's, life. In some cases, it can take years to even find the correct diagnosis. While an empathetic approach certainly goes beyond rare disease, it is particularly important in approaching rare disease creative.

Performing creative work within rare disease requires an extraordinary amount of listening. People with rare diseases, their care partners, and their physicians have incredible, inspiring stories to tell. It's a creative team's job to boil these stories down to simple, digestible efforts that bring attention to the disease and connect more people with potentially life-changing treatments.

After listening and considering the mindset of the audience, it's time to start doing disruptive creative work. Look for opportunities to produce messaging and imagery that will inspire people to become more aware and ultimately take action. This might require the creation of a dire scenario that brings attention to a potentially devastating condition or to create hope and reassurance. Knowledge is power, and the rare disease universe is searching for clarity. It's that empowerment that is injected into all of our creative work.

Companies and brands in the rare disease space have the power to change lives. In turn, the creative work has the power to give them the voice they deserve, to capture attention, and to be the catalyst to take action. PV

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