2020 VISION: FOCUS ON RARE DISEASES AND ORPHAN DRUG MARKETING

An Amplity Health White Paper

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Amplity HEALTH

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Introduction

Rare diseases — those afflicting fewer than 200,000 individuals in the United States — individually affect relatively small groups of patients. But taken together, rare diseases afflict as many as 30 million Americans, nearly 10% of the US population. To date, as many as 8,000 rare diseases have been identified, and 250-280 new rare diseases are described annually.

Up to 80% of these disorders have a genetic origin; in fact, most are caused by defects in a single gene. Rare diseases take a large toll on children:

- Approximately 50% of those affected by rare diseases are children
- 30% of children with a rare disease will succumb to it before reaching 5 years of age
- Rare diseases cause 35% of deaths occurring during the first 12 months of life

Taken together, rare diseases afflict as many as **30 million Americans**, nearly 10% of the population.

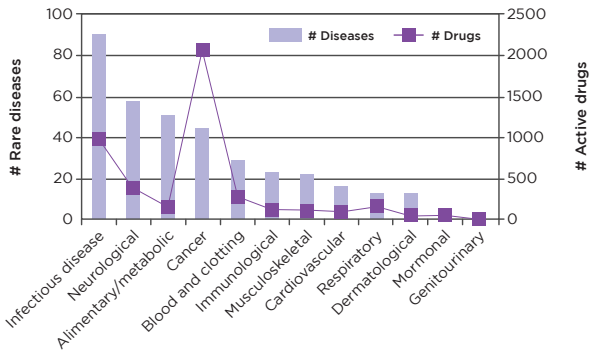
To date, as many as, **8,000 rare diseases** have been identified, and roughly **250 new rare diseases** are described annually.

Historically, rare diseases were not a common focus of mainstream pharmaceutical companies. But that began to change with the 1983 implementation of the US Orphan Drug Act, which ushered in a new era in the development of drugs to treat rare diseases. As midsize and large pharmaceutical companies started to respond to the opportunity to support rare disease patients, they increased their investment in the market. And as scientific understanding of rare diseases improves, the pharmaceutical industry continues to transform its approach to drug therapy, leading to new hope for millions who suffer from rare diseases.

The FDA has now granted more than 7,000 orphan designations and approved more than 600 orphan therapies. What's more, the number of orphan drugs is steadily increasing: in 2018, 34 of the 59 novel drugs the agency approved carried orphan designations.

Worldwide orphan drug sales are forecast to reach $262 billion in 2024, according to EvaluatePharma's Orphan Drug Report 2018. The compounded annual growth rate of orphan drugs between 2018 and 2024 is forecasted to be 11.3 percent, about twice that of the non-orphan drug market. By 2024, the industry analysis service forecasts that orphan drugs will represent 21.7 percent of worldwide prescriptions, up from just 16 percent in 2017.

Rare Disease Drugs in Development by Therapeutic



From Stephens J. Blazynski C. Rare disease landscape: will the blockbuster model be replaced?

Opportunities in the Rare Disease/Orphan Drug Marketplace

Fresh from recent successes, the orphan drug marketplace continues to represent a significant opportunity for research-based biopharmaceutical firms. Some of these products become blockbusters; one drug in this category has more than $1 billion in annual spending, and another seven have more than $200 million. The emergence of this opportunity is largely the result of the confluence of several key factors, described briefly below.

Unmet Medical Need

The rare disease marketplace represents a massive unmet medical need. As noted earlier, rare diseases afflict as many as 30 million Americans, nearly 10% of the US population. Yet only 5% of rare diseases currently have FDA-approved drug treatments.

Improved Science

Groundbreaking progress in genomic science over the past 25 years has yielded a far clearer understanding of the molecular basis of diseases. Twenty-five years ago, scientists had identified the genetic etiology of just 50 diseases; today, the number is more than 4,500, a >90-fold increase. These breakthroughs give companies a roadmap to develop targeted drugs.

Continuing Revenue Stream

The vast majority of patients with rare diseases are battling chronic or recurring illnesses. As a result, those who gain access to effective, affordable, and well-tolerated therapy are likely to remain on therapy for extended periods. Orphan drug makers can often realize recurring revenue with little or no competition.

Premium Pricing

The median annual cost for an orphan drug was recently over $46,800. Several factors support such high prices. First, drug manufacturers need high prices to recover research and development costs and have only a small number of patients from which to draw that revenue. Second, many first-to-market manufacturers haven't been forced yet to battle competitive products that could spur price reductions. Third, payers have limited negotiating power because of the high unmet need and profoundly negative optics that could result by blocking access to these life-saving therapies.

Efficient Marketing

Whereas the typical non-orphan small-molecule marketing plan may target 50,000 prescribers, orphan drug marketers may target fewer than 100. But differences in scale are just the beginning; the orphan drug marketplace is fundamentally distinct. Rare disease researchers, advocacy groups, patients, and clinicians often form close-knit communities that can significantly alter — and sometimes simplify — the marketing mix. These communities are described in some detail in the Grassroots Stakeholders section.

Legal and Regulatory Incentives

The Orphan Drug Act includes a number of incentives, including protocol assistance by the regulatory authorities, potential access to accelerated approval strategies, reduced or waived regulatory fees, the opportunity for tax credits and subsidies for clinical trials in the rare disease space, and 7-year market exclusivity.

Through the Rare Pediatric Disease Priority Review Voucher (RP-PRV) program, any company that gains regulatory approval for a drug aimed at a pediatric rare disease is rewarded with an RP-PRV. The company can either use it to expedite the review of any future product in its own pipeline (rare or mass market), or sell it to another manufacturer, thereby generating a cash influx for the seller. When the voucher is applied to a downstream new drug filing, the FDA attempts to make its approval decision within six months, rather than the usual 10 months.

Reduced Trial Size

Orphan drug development often includes smaller-scale clinical trials. For example, the mean Phase III trial size for non-orphan drugs is almost 800 patients, while for orphan drugs the mean is approximately 300 patients.

Higher Probability of Regulatory Approval

Orphan drugs have a 38% higher approval rate than non-orphan drugs.

Taken together, the opportunity in orphan drugs seems clear. But there are also challenges.

Challenges in the Rare Disease/Orphan Drug Marketplace

Challenging Regulatory Pathway

The orphan drug regulatory path can be treacherous. Because they are rare, orphan diseases are often not well understood even by researchers and regulators. As a result, drug developers must themselves become regulatory experts, able to engage authorities in complicated scientific discussions where there is little, if any, well-established regulatory guidance.

Difficult Development Process

Because patients are few and geographically dispersed, conducting orphan drug clinical trials can be challenging. Some trial sites may be able to enroll only a few patients. What's more, in the case of a disease where there is no approved therapy, patients may be unwilling to be in a placebo-controlled trial if there is a potential therapy being provided to treat a condition without one.

Increasing Competition

The opportunities in orphan drugs are drawing new players. While orphan markets were once the purview of smaller manufacturers, Big Pharma is now positioning itself to compete for orphan treatments. Recently, leading companies have formed research units specializing in rare diseases. Top-tier companies have also begun partnering with — or acquiring — dedicated orphan companies.

Restricted Access

Payers have begun to restrict access to some orphan drugs by establishing managed-entry agreements or by threatening to deny coverage outright unless prices are reduced. Furthermore, payers are beginning to require prior authorization or step edits for certain orphan drugs as part of the reimbursement process, or requiring pretreatment clinical diagnostic tests. Orphan products that provide few differentiated benefits are likely to face close scrutiny from payers.

Limits on Reimbursement

Governments and other payers are starting to question reimbursement of expensive orphan drugs. Coinsurance percentages for specialty drugs have risen steadily, recently averaging 20%. Families with a $60,000 annual income (near the US national median) can ill-afford to pay thousands of dollars in coinsurance on top of premiums and deductibles. And for diseases with multiple orphan drug alternatives, payers are giving preferred formulary status to certain agents and non-preferred status to others.

Unique Marketing Environment

Many of the most-experienced pharmaceutical marketers learned their craft in the traditional small-molecule arena, and are relatively unaccustomed to the close-knit rare disease communities in which patients are fully informed and engaged, patient advocate groups are highly active, expert resources are accessible, and clinicians impart their knowledge and personal experiences openly. These communities are unique in the biopharma space, and navigating them successfully requires a deft hand.

Difficult Path to Diagnosis

Because rare diseases are, by definition, rare, the path to a correct diagnosis is often long and tortuous. In fact, a patient with a rare disease will visit an average of 7.3 physicians and it will take 4.8 years from symptom onset to an accurate diagnosis. Fortunately, improvements in genetic testing are improving the path to diagnosis. By 2018 a genetic test was available for around 3,600 rare diseases, compared with only 2,200 in 2010. In fact, since 2013, diagnostic efficiency has increased from ~10% to 30-50%, a considerable step forward for rare disease patients.

Understanding the Grassroots Stakeholders

Successful orphan drug marketers are those that have built up credibility with each of the rare disease stakeholder constituencies: patients, families, and caregivers; advocacy groups; primary care providers; specialty providers; specialized rare disease centers; and specialty pharmacies.

Patients, Families, and Caregivers

Dealing with the physical challenges of a rare disease is tough enough, but in many cases, the challenges facing rare-disease patients and their caregivers extend well beyond the disease itself. These challenges, which can be overwhelming at times, include the following:

- Finding suitable medical care: patients and families face a two-phased journey; first, it may take many years of playing "rotating provider" to arrive at a correct diagnosis, and second, it is often difficult to locate hard-to-find rare disease expert clinicians after diagnosis
- Interacting with healthcare professionals who are unfamiliar with the disease: by the time patients are properly diagnosed and receiving appropriate treatment, they often know more about the disease than do many of the medical professionals who are treating them
- Managing co-morbid conditions and/or side effects: providing family-based care for these complications — which are common in many rare diseases — often requires specialized training and support
- Handling the financial challenges that often accompany rare diseases: bills for special care, travel to find specialists, and for some, the inability to work while managing their disease
- Coping with the emotional challenges a rare disease presents: a majority of rare disease patients and their caregivers reported that the disease caused worry, depression, anxiety, stress, and isolation from friends and family

Advocacy Groups

The physical, emotional, and financial impact of a rare disease can motivate those suffering from it to work to ease the burden for others that are or may be affected by the same disease. These individuals often join together to form support and advocacy organizations (more than 800 to date); some are focused on individual conditions, while others encompass a number of related conditions. Still others, such as the Global Genes™ Project and the National Organization for Rare Disorders (NORD) act as umbrella organizations for large numbers of disparate groups.

Rare disease advocates often seek to impact the broader community through public awareness and fundraising efforts. Disease-specific advocacy groups sponsor events such as runs/walks, telethons, and celebrity appearances in order to focus public attention on rare conditions and the families dealing with them. Umbrella groups engage in an array of activities intended to build broad-spectrum awareness of rare diseases. One example is Rare Disease Day, an annual international event coordinated by a host of national alliances and patient organizations.

Advocacy groups can be the key to increasing awareness and recruiting patients for rare disease clinical trials. They can also significantly affect the perception of a company, its intentions, and the success of its orphan product. Many rare disease communities wield tremendous influence; through their own interconnectedness — particularly via social media — they can augment or subvert the work done by a company's field-based clinical and sales teams.

In some cases, marketing to a circumscribed rare disease community can be accomplished with a modest investment in a carefully constructed marketing mix. Over time, manufacturers can build long-lasting alliances with patients and advocacy groups. Working with these organizations can help smooth a path from research to approval, since these groups are often well-structured, vocal, and energetic. Furthermore, such alliances play a crucial role in a host of commercialization activities, from sourcing clinical-trial patients to promoting the availability of new treatments.

Patients, families, and advocacy organizations also actively promote research into rare diseases and the development of medicines to treat them. In fact, for some groups, research is the principal aim. Among research-focused organizations, some concentrate primarily on direct fundraising for research and gaining more public research funding, while others take more active research roles.

Despite continuing growth in the number and strength of advocacy groups, only about half of rare diseases have a dedicated foundation or support organization. And even when a rare disease does have an advocacy group, marketers must still deliver their message to appropriate clinicians.

Primary Care Providers

In about 75% of cases, primary care providers (i.e., FP, GP, IM, and pediatricians) are the first point of contact for patients suffering from what is eventually diagnosed as a rare disease. And although a majority of primary care physicians (PCPs) welcome the challenge of rare diseases and want to be part of the diagnostic journey, a seminal study that still offers valuable insight (Engel et al) reports that 4 of 10 PCPs say they don't have time to do a workup for a rare disease even if they suspect one. Furthermore, the survey shows that as many as 80% of PCPs believe that because certain other specialists/experts have more experience, they prefer to refer suspected rare disease patients to other providers. Nevertheless, about 10% of rare disease diagnoses are made by PCPs.

Care of a patient with a rare disease requires detailed knowledge of the condition, and many PCPs don't feel confident in providing that care — especially early in the course of treatment. In this same survey, among PCPs who treat at least one patient with a rare disease, more than half rated their initial knowledge of the disease as only fair or poor. After they gained experience with the disease the number dropped to just over 20% fair or poor, while almost 60% rated their knowledge as good or excellent.

As PCPs begin to engage in a possible rare disease case they take several actions to augment their knowledge. The most common actions are, (1) conducting an assessment of the medical literature to obtain information, (2) discussing the case with local colleagues, and (3) contacting local or national experts to obtain advice or guidance.

Specialty Providers

Specialty providers (e.g., cardiology, genetics, neonatology, neurology, etc.) most often engage nascent rare disease cases as referrals from PCPs, according to the Engel survey. However, in more than a quarter of cases patients first present to a local specialist (17% of cases), regional specialist (7%), or national specialist (3%). Among the approximately 90% of rare disease cases that are diagnosed by specialists, the diagnosing physician is most often a local specialist (44% of cases) rather than a regional specialist (28%) or a national specialist (17%).

Compared with PCPs, specialists tend to have roughly 15 times as many patients with rare diseases in their practices (201 vs 13) and feel much more confident in their rare disease knowledge. The same survey showed that among specialists who treat patients with rare diseases, 60% rated their initial knowledge of the disease in question as good or excellent. After they gained experience, that number increased to 77% good or excellent.

Despite their higher engagement and greater confidence with these patients, specialists also take specific actions to augment their knowledge. The most common actions are, (1) conduct an assessment of the medical literature to obtain information, (2) contact local or national experts to obtain advice or guidance, and (3) discuss the case with local colleagues. Additionally, specialists are much more likely than primary care physicians to attend a conference or seminar or contact the National Institutes of Health to obtain information.

Specialized Rare Disease Centers

In some cases, healthcare centers or physician practices dedicated to the diagnosis and treatment of a specific rare disease (or group of associated diseases) can advance the quality and uniformity of care. In addition, these specialized providers can offer expert consultation to outside clinicians, cultivate care guidelines based on up-to-date evidence, and serve as referral sites when local expertise is insufficient. Finally, they can serve as an important research base.

Specialty Pharmacies

Because orphan drugs are low-volume products that tend to be costly, they require extra care in supply chain management and distribution. In many cases, specialty pharmacies provide value-added, high-touch services that are crucial to efficient marketing:

- Benefit investigation/verification for meeting payer's prior-authorization requirements
- Reimbursement support
- Clinical and nursing support
- Patient education
- Data collection for patient outcomes
- Specialized storage, handling, and delivery of the products

Building Orphan Drug Success Through Clinical, Field Sales, and Remote Engagement Support Teams

It's not uncommon for pundits to declare that because orphan drug marketers focus on a smaller target audience, the promotional mix "requires less investment" or "doesn't need expensive advertising." Indeed, orphan drug marketers may target fewer than 100 prescribers while the typical non-orphan, small-molecule marketing plan could target as many as 50,000. But that simplistic metric fails to capture the complexities of participating in the rare disease marketplace. While an orphan drug marketing plan may indeed have a smaller sales force footprint, it may need to overweight its investment in clinical and support teams in order to best serve its key stakeholders.

The real art is in deploying the right resource in the right amount to support the right stakeholder at the right time. In the end, marketing success in the orphan drug space depends — perhaps to a greater degree than in any other biopharma space — on crafting the right mix of sales, support, and clinical teams. Moreover, because many orphan drug companies are under-resourced in terms of both money and staff, the economic importance of establishing the right communication mix can hardly be overstated.

Working with Advocacy Groups

While the vast majority of marketers and field team leaders would say they "want to work with patient advocacy groups," it's a tough battle. As noted earlier, only about half of the 8,000 rare diseases currently have a dedicated foundation or support organization. And even among rare diseases that have advocacy groups, sophistication varies greatly. While some foundations have strong paid leadership, many others are volunteer-only and have less-developed skill sets; some are one-person organizations formed by a desperate parent with a sick child. Regardless of the degree to which a manufacturer can identify and partner with an associated advocacy group, marketers still must deliver their message to appropriate clinicians.

Asking the Right Questions

How does an orphan drug marketer begin the process of crafting the right mix of HCP-focused clinical, support, and sales teams? By asking the right questions. The answers to these questions will guide the process. As Amplity Health works with clients to craft commercialization teams, we utilize a 75-question checklist. The checklist includes questions like these:

- How many patients have the disease? How many are diagnosed each year? What age are they at diagnosis? Where are they located?
- What are the emotional, financial, and quality-of-life issues faced by patients and caregivers?
- What is the diagnostic path for the disease? Who typically makes the final (correct) diagnosis? How long does it take to reach that correct diagnosis?
- How many specialized centers focus on the disease? Where are they located?
- Who are the prescriber targets? Only specialists, or are some PCPs included?
- In how many MSAs are the HCP targets located? In just a few large MSAs, or more dispersed?
- How accessible are the key thought leaders who have a significant impact on the brand?
- What is the status of disease-specific advocacy groups? How many? How well established? How accessible?
- Does the brand have a complex or novel mechanism-of-action (MOA) requiring in-depth education?
- Does your brand treat a chronic disease state that has significant patient compliance, self-administration, or adverse event issues that require management?
- Can your current field sales force adequately cover all of your target physicians with the optimal frequency?
- Do you have a significant number of "no-see" or "hard-to-see" healthcare professionals on your target list?
- Do hospitals and/or clinics impact prescribing of your brand? If so, are there under- or unreached healthcare professionals (e.g., 2nd- and 3rd-shift staff)?
- What other healthcare professionals influence your brand, such as pharmacists, office staff, physician extenders, or hospital staff?

As the commercialization team collects the answers to these questions (and many more), they typically begin to gain a sense of the crucial components of an appropriate communications mix. The "right" mix for any individual brand is typically crafted from one or more of several types of specialized clinical teams, field sales teams, and remote engagement support teams:

Clinical Teams

A well-developed team of non-promotional clinical communications specialists can execute a variety of tasks that are essential to orphan product success:

- Identify and interact with KOLs
- Place and support clinical trials
- Improve health outcomes and adherence by educating HCPs, patients, and caregivers
- Provide support to key specialists in metro areas, institutions, and white space
- Deliver disease-state education and support to patients, caregivers, and/or physicians
- Maintain an engaged partnership with disease advocacy groups and prepare these groups to lobby for access

The 3 most common clinical communications roles are Medical Science Liaisons, Patient Diagnosis/Disease Awareness Liaisons, and Clinical Health Educators:

Medical Science Liaisons (MSLs)

MSLs serve as a non-promotional link between your brand and the medical community. Staffed by healthcare professionals such as PharmDs, PhDs, MDs, etc., they work to educate key opinion leaders, clinical trial investigators, and other thought leaders about your clinical initiatives. Amplity's MSLs can also be deployed through our 100% healthcare-focused contact center.

Patient Diagnosis/Disease Awareness Liaisons

Patient Diagnosis/Disease Awareness Liaisons maintain comprehensive clinical knowledge of an assigned therapeutic area, and the evolving clinical landscape related to your portfolio. They focus on increasing disease awareness related to targeted physicians and collaborate with your Medical Affairs organization to deliver focused responses to external requests for medical-scientific information regarding your products/portfolio.

Patient Diagnosis/Disease Awareness Liaisons represent the physician/market from the field perspective. This can be extremely impactful during planning and strategy meetings. Liaisons can serve on cross-functional teams, as a conduit for accurate and updated medical/market communication, medical/market intelligence, emerging clinical trends, and HCP feedback.

Clinical Health Educators

Clinical Health Educators provide disease-state education and support to patients, caregivers, and/or physicians. Clinical health educator teams are staffed by healthcare professionals, such as RNs, who work to optimize health outcomes by facilitating patients' adherence and retention to drug therapies and other healthcare regimens.

Field Sales Teams

Physicians place high value on informative and unbiased sales presentations, and published research demonstrates that field-based sales representatives continue to be a highly effective component of the biopharmaceutical marketing mix:

Field Sales Representatives

Traditional, geographically defined field-based sales representatives are often the backbone of the communications mix; recent studies demonstrate that many PCPs and specialty physicians prefer in-person engagement with sales representatives. The size of the field sales force required to launch, grow, or sustain any particular orphan drug can be determined only after careful analysis of a host of factors, including the location and size of advocacy groups, patient identification and correlation, and the distribution and location of prescribers. As in the small-molecule market, these sales representatives are important components of the typical orphan drug marketing mix. However, many orphan drugs can be supported with relatively small sales forces.

Hybrid Sales Teams

The hybrid representative concept blends the best of both worlds — a consistent, dedicated representative combined with the flexibility to time shift and communicate through both in-person and remote channels. Because hybrid teams can make in-person calls on local targets and technology-supported remote calls on any target HCP regardless of geography, they can be an ideal solution for orphan drugs that have a small and geographically dispersed base of prescriber/target physicians.

Diagnostic Field Sales Teams

As pharma companies continue to build and invest in targeted therapy pipelines, new companion diagnostic platforms and tests are being developed to identify the patients most likely to respond to a given treatment. The role of the Diagnostic Field Team is to help clinicians identify appropriate patients and to better understand the precision medicine landscape that continues to evolve at an exponential rates. Geographical differences in the access to various testing modalities and reimbursement must be accounted for in go-to-market strategies.

Remote Engagement Support Teams

Manufacturers also deploy a variety of remote engagement teams to support stakeholders:

Remote Service Teams

Remote Service Teams provide on-demand disease- or product-specific assistance to HCPs and their patients via phone and/or text-based chat. These teams are often staffed with highly trained, non-credentialed, clinical health associates who answer common questions and triage calls to credentialed clinical health educators as needed. For example, clinical help desk teams respond to calls from patients and caregivers regarding storage, handling, dosing, etc., and from HCPs or staff members regarding access, reimbursement, etc. Live-chat care counselors respond to click-to-chat requests from visitors at patient- and/or prescriber-focused websites. These types of inside support teams are becoming increasingly important components of orphan drug plans.

Remote Sales Teams

In addition to field-based representatives, many life sciences companies are also deploying an array of inside message delivery teams. These inside teams can supplement field initiatives and be available to dialogue with HCPs on demand and at times when traditional field representatives are not typically available (eg, early morning or after-office hours). Inside teams can offer particular value for orphan drug marketers, whose targeted clinicians are often widely distributed geographically. Some of the more effective channels, include:

- **Live Video Detailing:** Inside professional sales representatives (with field-level training) conduct live, person-to-person details using the Web and phone. Most live video details are initiated on demand by healthcare providers who click a brand-site Web link.
- **TeleDetailing:** Experienced contact center sales representatives use their advanced training to accurately deliver key product messages to targeted prescribers.
- **TeleService/TeleSampling:** Inside representatives make live phone calls to target offices in order to develop relationships, uncover needs (e.g., prescriber education, patient materials, etc.), fax and retrieve sample request forms, and forward requests to the client's fulfillment supplier.

Vacant Territory and White Space Management: Comprehensive vacant territory and white space management can ensure coverage when a field sales representative is not available. Companies using a variety of highly customizable vacancy management programs can use a combination of teleDetailing, teleService, teleSampling, and eSampling. Additionally, outbound live video detailing can be employed.

Remote Credentialed Sales Specialists

In addition to the "traditional" remote sales teams just described, some orphan drug manufacturers need the power of HCP-to-HCP promotional conversations. These peer-to-peer conversations are made possible by inside credentialed sales specialists, who are healthcare professionals (e.g., RPhs, PharmDs, RNs, MDs) that engage physicians and other high-level targets in complex, one-on-one product discussions held via phone and/or live video. Inside credentialed specialists are often particularly effective in reaching target HCPs who work in institutional settings where in-person sales representatives are prohibited. This type of promotional support can be ideal for orphan drugs, as it enables high-science peer-to-peer discussions without the restrictions of geographic boundaries.

Summary

Taken together, rare diseases afflict as many as 30 million Americans, nearly 10% of the US population. To date, as many as 8,000 rare diseases have been identified, but only 5% of rare diseases currently have any FDA-approved drug treatment.

The orphan drug marketplace continues to represent a significant opportunity for research-based biopharmaceutical firms, but the path to success is fraught with complexities, including a challenging regulatory pathway, a difficult development process, and increasing competition.

Importantly, the rare disease community comprises several stakeholder constituencies: patients, families, and caregivers; advocacy groups; primary care providers; specialty providers; and specialized rare disease centers. Each of these groups carries its own communication challenges.

The process of crafting the right orphan drug communications mix begins by asking questions; the answers to these questions typically lead to a message-delivery lineup including members from one or more of several types of specialized teams, including clinical teams, field sales teams, and remote engagement support teams.

Despite the many difficulties, marketing an orphan drug can be one of the most rewarding professions in the life sciences industry. Few experiences in the career of a pharmaceutical brand manager can compare with witnessing the joy of a parent whose child can enjoy a fresh start in life as a result of receiving a new life-saving therapy.

Resources

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