Amplity HEALTH

Orphan Drug Success:

Engaging Key Stakeholders to Deliver Positive Outcomes

A Amplity Health Whitepaper

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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 roughly 250 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

Given the limited number of patients suffering from any one disease, rare diseases historically 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 provided several important business incentives to companies that develop drugs specifically for rare diseases. Early on, small biotechnology firms led the charge for orphan drug development, and biologics still account for up to two-thirds of the orphan drug market. However, as midsize and large pharmaceutical companies began to realize the revenue potential of orphan drugs, they started to increase their investment in the market. And as scientific understanding of rare diseases improves, the pharmaceutical industry continues to transform its

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

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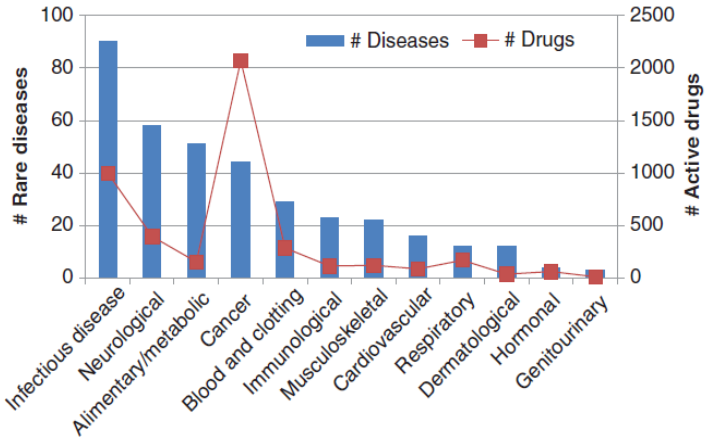
approach to drug therapy, leading to new hope for millions who suffer from rare diseases.

The FDA has now granted more than 3,100 orphan designations, and approved more than 450 of these products for marketing. What's more, the number of orphan drugs is steadily increasing. According to a 2015 GlobalData report, during 2014, CDER approved 17 new molecular entities for orphan drugs, the most ever.

Rare Disease Drugs in Development by Therapeutic



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

By some estimates the worldwide orphan drug market could grow to $127 billion by 2018; this 6-year compound annual growth rate (CAGR) of 7.4% is double that of the overall non-generic prescription drug market. By 2018, orphan drugs are expected to represent nearly 16% of the overall non-generic prescription drug market, up from just 5% in 1998. As shown in the graph above, most of the current rare disease research is focused in 10 therapeutic areas; the 2 areas with the greatest number of drugs in development are cancer and infectious disease.

Note: This report contains information from numerous sources that Amplity Health believes to be reliable but for which accuracy cannot be guaranteed. Amplity Health does not accept responsibility for any loss incurred by any person who acts or who fails to act as a result of information published in this document.

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. Many of these products become blockbusters; in fact, almost a third of orphan drugs generate more than $1 billion in annual sales. 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 20 years has yielded a far clearer understanding of the molecular basis of diseases. Twenty years ago, scientists had identified the genetic etiology of just 50 diseases; today, the number is 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 enjoy recurring revenue with little or no competition.

Premium Pricing

Of 2013's new orphan drugs, most have prices exceeding $90,000 per patient per year. It's not uncommon for new orphan biologicals to cost between

$200,000 and $400,000 per patient per year. 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.

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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, currently including expanded access to the Investigational New Drug Program, grants for drug development, a waiver of user fees charged under the Prescription Drug User Fee Act (PDUFA), fast-track approvals, tax credits, 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 huge

cash influx for the seller. When the voucher is applied to a downstream new drug filing, the FDA will 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 median phase III trial size for non-orphan drug trials is 2,234 patients, while for orphan drugs the median is just 528 patients (and in some cases, far fewer).

Higher Probability of Regulatory Approval

Orphan drugs have a 27% 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, regulators have begun to signal a shift toward higher standards. As clinical trial requirements become more rigorous, developers of orphan drugs will likely encounter higher research costs.

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 such as GlaxoSmithKline and Pfizer 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 require pretreatment clinical diagnostic tests. Orphan products that provide few differentiated benefits are likely to face close scrutiny from payers.

Limits on Reimbursement

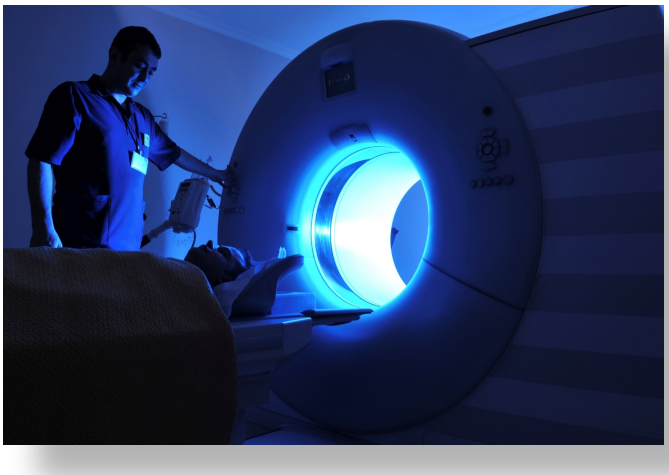
Governments and other payers are starting to question reimbursement of expensive orphan drugs. On average, coinsurance percentages have risen in the past decade to 28% from 15%. And for diseases with multiple orphan drug alternatives, such as chronic myeloid leukemia and colorectal cancer, payers are 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, according to the *Rare Disease Impact Report* (Shire, 2013) survey of US patients, it takes 7.6 years to arrive at an accurate diagnosis. In order to get that final correct diagnosis, a patient typically visits 4 primary care clinicians and 4 specialists and receives 2 to 3 misdiagnoses along the way.



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 patients with rare diseases 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 comorbid 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: these can include 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 patients with rare diseases 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 impacts 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; 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.

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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 EURORDIS and 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 focus primarily on direct fundraising for research and gaining more public research funding, while others take more active research roles.

Some advocacy groups focus on helping patients/families locate physicians with expertise treating those with extremely rare conditions. Organizations may provide a list of affiliated physicians; for example, the website of the International Fibrodysplasia Ossificans Progressiva (FOP) Association lists physician contacts, including FOP researchers at the University of Pennsylvania.

Despite continuing growth in the number and strength of advocacy groups, only about 15% of the 8,000 rare diseases currently 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 three fourths of cases, primary care providers (ie, 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 recent physician survey (Engel et al, 2013) reports that 4 of 10 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 patients with suspected rare diseases 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 (eg, 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 10 times as many patients with rare diseases in their practices (200 vs 20) 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.

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 they are low-volume products that tend to be costly, orphan drugs 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 Sales, Support, and Clinical 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 support and clinical teams.

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 15% 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 sales, support, and clinical 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 200-question checklist. For more details, refer to our white paper, *Implementing a Life Sciences Sales Force: A Process and Tactical Blueprint*. 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 (eg, 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 sales, support, and clinical teams:

Field Sales Teams

Sales representatives are important components of the biopharmaceutical marketing mix. Physicians place high value on informative and unbiased sales presentations, and published research demonstrates that sales representatives continue to be a highly effective component of the marketing mix. For more on this, see our recent white papers, *2020 Vision: A Short- and Long-Term Prognosis for Life Science Sales Forces* and the 2014-2015 edition of *What Physicians Want!*

Field-based sales teams are often the backbone of the communications mix; recent studies demonstrate that majorities of both 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.

As in the small-molecule market, sales representatives are important components of the typical orphan drug marketing mix. However, many orphan drugs can be supported with relatively small sales forces. For example, Alexion launched Soliris in 2010 with just 32 sales representatives. And Aegerion's Juxtapid was supported by only approximately 25 sales representatives focused on key specialists.

Inside 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:

- **Live Video Detailing**: Inside professional sales representatives (with field-level training) conduct live, person-to-person details using the Web and phone. Healthcare providers who click a brand site Web link initiate most live video details on demand.
- **TeleDetailing**: Experienced contact center sales representatives use their advanced training to accurately deliver key product messages to target prescribers.
- **TeleService/TeleSampling**: Inside representatives make live phone calls to target offices in order to develop relationships, uncover needs (eg, 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.

For more, see our white paper, *Field-Alternative Channels: Enhancing the Message Delivery Mix*.

Inside Credentialed Sales Specialists

In addition to the "traditional" inside sales teams described above, 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 (eg, 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.

Inside Support Teams

Inside support representatives 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, noncredentialed, 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.

Hybrid Teams

A newer representative type includes hybrid representatives, who make face-to-face, in-person visits to HCP targets within a defined local geography, but can also reach both local and remote targets through each HCP's preferred non-personal channel (live video, text-based chat, or phone) and at the HCP's preferred time (including after hours and weekends).

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.

Clinical Teams

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

- Identifying and interacting with KOLs
- Placing and supporting clinical trials
- Improving health outcomes and adherence by educating HCPs, patients, and caregivers
- Providing support to key specialists in metro areas, institutions, and white space
- Delivering disease-state education and support to patients, caregivers, and/or physicians
- Maintaining an engaged partnership with disease advocacy groups and preparing these groups to lobby for access

The two most common clinical communications roles are Medical Science Liaisons (MSLs) and Clinical Health Educators:

- **MSLs** serve as a nonpromotional 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. Touchpoint's MSLs can also be deployed through our 100% healthcare-focused contact center. For more, see our white paper, *Implementing a Medical Science Liaison Team*.
- **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 regimes. For more, see our white paper, *Clinical Health Educators: Improving Health Outcomes One Patient at a Time*.

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. Many of these products become blockbusters; in fact, almost a third of orphan drugs generate more than $1 billion in annual sales. However, the path to orphan drug success is fraught with complexities, including a challenging regulatory pathway, a difficult development process, and increasing competition.

The rare disease community comprises several rare disease 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 comprising members from one or more of several types of specialized teams, including sales teams, support teams, clinical teams, and hybrid 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.

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