LaVoie Group

Life Science and Pharmaceutical Communications

A Primer: Solving the Unique Problems of
Orphan Drug Communications Programs

Introduction

When communicating about orphan diseases and development of pharmaceuticals, it is essential to recognize the special characteristics of the rare disease environment, and to create integrated programs that respond to those idiosyncrasies. This paper outlines the considerations that must be taken into account when planning orphan disease drug communication programs and the best practice approaches to implementing them successfully, through pre-approval, approval, and post-approval phases.

Scope

In this paper, the term “communications” refers to any and all communication materials released by or for developers of products intended to treat orphan diseases. Communications programs must target, engage, and inform a wide range of distinct audiences. They may include communications created for investors and others with financial interests, for the medical community, for marketing and educational purposes, for patients, families, advocacy groups and other stakeholders, and for key opinion leaders and the general public.

The media possibilities are as diverse as the audiences and the objectives of the communications: press releases, financial documentation, road shows, corporate presentations, websites, medical communication, events, social media, teleconferences and any or all of the other tools available to modern communicators.

Types of Messaging

As is the case with all pharmaceuticals, different types of communications are required as the development process proceeds. When and what to communicate may be contingent upon whether the company is public or private, and on other factors. In a public environment, for example, some targeted messaging could be in conflict with the goals and objectives of the company's communications to other stakeholders. It is important to have an understanding of your objectives for your company's communications to determine your business case and to create the messaging that effectively supports it.

For regulatory filings, clinical trial data updates, and other general corporate news, communicating through press releases and other public disclosures are standard just as in communicating for any product or product candidate. What makes orphan disease communications different is the patient population. Orphan diseases often have close knit patient communities who are often dealing in a disease area in which there is no effective therapy. If you are a public company, the balancing act between communicating with and pleasing Wall Street, while making patients and their providers comfortable with your company and its brand become integral components of building relationships in your market. When targeting communications to a particular audience, it is important to remember that other audiences are always listening.

Without a doubt, large and small companies may have different disclosure and communications requirements because of internal policies, general commercial views, and regulatory compliance issues. These factors can sometimes create challenges, particularly for larger companies that have more policies in place with regard to regulatory compliance. That is not to infer that small companies are violating the law, but many times they have more flexibility in their approach, and thus have the advantage of being able to move quickly.

Audiences

Throughout the process, audiences may include physicians and providers, regulatory agencies, foundations, patients, families, and advocacy groups, as well as investors and others. For orphan diseases, governments are also audiences, since many are involved in providing access under treatment protocols to patients prior to approval and also provide the manufacturer with a supply agreement in advance. This is particularly important in countries like Brazil, France, Israel and others where governments are purchasing drugs on behalf of their citizens.

This does not apply to traditional pharmaceutical products except in rare cases. Orphan drugs are often in a class by themselves. For example, the number of patients in a

pivotal trial in which the new drug application is filed under is often smaller than traditional biotech and pharmaceutical products. In some cases, there are accelerated approval processes. As a result, there are communications considerations such as managing expectations of drug access to patients, their physicians and other key stakeholders -- including investors who often are trying to understand and project the company's potential product revenue and resulting profit to help them put a value on the company.

The Environment

An orphan disease environment is often more personal and more intense than large-scale pharmaceutical development. While the treatment of any disease or illness has its obvious personal and emotional overtones, an orphan situation can be more challenging, given the low disease awareness and limited therapies.

Orphan drugs communities are relatively small but have powerful patient voices who are partners with the industry in drug development. There are personal relationships and a special bond among patients, families, physicians and patient advocacy groups. Physicians develop deep and personal relationships with their patients and their families because of their small number and the intensity of their treatments. Those working to find treatments and cures are often personally motivated and know the patients and their families. Foundations and other advocacy groups are founded and heavily populated by people with personal interests together families, industry, government, physicians and others are working toward finding a treatment, a cure or a way to save the life of a family member.

As a result, it is not unusual for company medical science liaisons (MSLs) to personally get to know the physicians, patients and their families. Orphan disease communications programs thus need to be different to support the special nature of the closeness of the community and the manufacturer. Communicators need to understand that patients and their families, physicians, researchers, advocacy groups and other stakeholders have profound personal stakes in gaining recognition of the importance of the disease and its devastating consequences, the searches for effective treatments, and the importance of their roles in the process.

They also need to recognize that there may be differences of opinions about care, treatment, and research directions. Advocacy groups and others may be in competition with each other. Each orphan world is small, and each struggle for recognition and funding is often very personal. Competition for support can be intense and, as in any complex environment, there are conflicting interests.

Given also these factors, communications must be appropriate to the nature of the orphan environment that is both rewarding and tricky at the same time

Tone

As a result, the content of communications must be carefully considered. It is important to understand the specific needs of each of the audiences and the factors that may prejudice their perceptions. In that context, it is important to recognize that all stakeholders can see all communications and balancing conflicting demands (such as those of financial stakeholders and patient advocates) requires experience and sensitivity.

When framing communications, it is essential to understand the personal motivations of audience members, including physicians, scientists and other professionals. Materials aimed at them need to have a personal tone; and reflect an understanding of the importance of the problem to those immediately impacted by it.

At the same time, investor relations and other financial and business releases and medical communication materials must be professional and regulatory compliant– prepared without forgetting that they will also be read by people with very personal interests.

Master Planning

A master plan is key to the success of any communications program. It needs to begin with basics: the definition of the program's objectives; the themes and messaging that will help to realize those objectives; the audiences, strategy and tactics; the use of key opinion leaders and patients, talking points and media.

Milestones and content also need to be included: when to start talking about the drug candidate, clinical trial initiatives, pricing, patient assistance programming, patient numbers, and other matters that will satisfy legitimate audience interest and help to achieve program objectives as well as build corporate reputation.

The challenges to be met include managing milestones and filing dates, marketing and price expectations, controlling speculation about filings, competition etc., and providing adequate information and data to patients without overstepping regulatory rules.

Following approval, it is important to avoid off label discussions, and to take great care with communications about matters such as patient numbers and access in markets where the drug has not yet been approved or covered under special access. Close collaboration with medical and regulatory affairs is important.

When and where possible, the plan should be based on experience, not only of the parties involved in preparing the plan, but also the success and failure experiences of others. Knowing what to do is important; in a complex environment such as orphan drug development, knowing what not to do can be just as important.

Integration

Integration is key to any multi-faceted communications program. It creates the synergy that maximizes the effectiveness of all the program's components. A pharmaceutical communications program is a complex assemblage of virtually all contemporary methods and media, content and messages, all aimed at a very broad mix of audiences with different agendas. An effectively integrated program will capitalize on the strengths of the various methods and media designed to help the program achieve its objectives, to enlist support where and as needed, and to

do more. Integration is also key in terms of working across internal constituencies, including legal, regulatory, marketing and medical affairs.

The integration of orphan drug communications programs involves more than the typical large-scale pharmaceutical development. As noted above, the numbers may be smaller, but the audiences can be just as diverse, and their emotional involvement is likely to be much higher.

The integration must also soften the edges among differences in priorities as well as other considerations. Some differences are universal: shareholders would like to see pricing that will enhance their returns; payers want pricing that will save them money. The more innovative the therapy or drug, the better chance of demonstrating its value to payers. Cost effectiveness will continue to be important in demonstrating how the innovation helps to save money in other areas. Ultimately, saving lives is a critical message.

Other differences may be less readily apparent. Various stakeholders may have differences in treatment approaches, priorities, and many other factors related to the development process, scientific issues, pricing, schedules, and virtually anything else related to the development of a drug and its use and distribution. As noted above, there are also likely to be competitions for recognition and funding among patient/advocacy groups.

Communicators need to take all of these differences into consideration, to tailor their messages to provide positive reinforcement where appropriate, and to avoid offending or otherwise exacerbating problems with stakeholders that have conflicting positions –

all in an integrated program will help to support the expeditious development of the orphan drug and its successful use by the largest possible number of patients.

Summary

Planning and executing a communications program for any pharmaceutical development, launch, and follow-up is a series of complex challenges. While orphan drugs have much in common with any pharmaceutical products, there are differences that impact the strategy, organization, execution and content of communication programs. Their environment is more intense and more complex, and their success requires higher levels of experience and expertise.

Orphan diseases impact relatively small communities and champions in the search for their treatment are often driven by personal relations with patients. As a result, there can be difficult balances to maintain in communication programs – a need to demonstrate a personal connection with patients on the one hand and a need to be focused on the bottom line in investor communications, for example. The small size, intensity, and personally motivated nature of most orphan disease communities create both challenges and opportunities for communicators.

Challenges can be met and opportunities realized with carefully planned and intelligently integrated communication programs that focus on best practices and solutions that balance the diverse needs by using experience, key learnings from mistakes as well as successes.

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