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A marketing model for orphan drugs

Abstract

It is not uncommon for rare disease patients, that it takes more than 10 years to receive a definite diagnosis after the initial physician visit. In the orphan business, pharmaceutical companies are expected to take a role to promote early diagnosis and medication to prevent disease progression, in addition to drug development and securing a stable drug supply. In this context, MRs are required to provide physicians with patient/case-based information and consider suitable marketing activities based on disease and drug profile. This article discusses characteristics and keys to success in the orphan drug business, as well as expected roles of MRs, challenges they are likely to face as well as latent issues that pharmaceutical companies must address.

Increasing presence of orphan drug business

Looking at orphan drugs launched in Japan, it is evident that a wide range of therapeutic areas is targeted, including cancers and genetic diseases. This coverage is expected to be even wider in the future.

On the other hand, only a handful of pharmaceutical companies have succeeded in deploying a promotion model considering special characteristics of the orphan drug business. Many companies have specialty MRs for the orphan market, but the main focus of their activities remains message delivery. For rare diseases, there is a great demand from physicians for information on actual cases of drug usage and how other physicians dealt with problems during treatment since there are few patients and thus limited evidence and articles are available for treatment decision making. MRs cannot really satisfy physicians' needs unless they deliver information for the individual patient based on disease status and symptoms.

Orphan drug business – a different landscape, different strategies

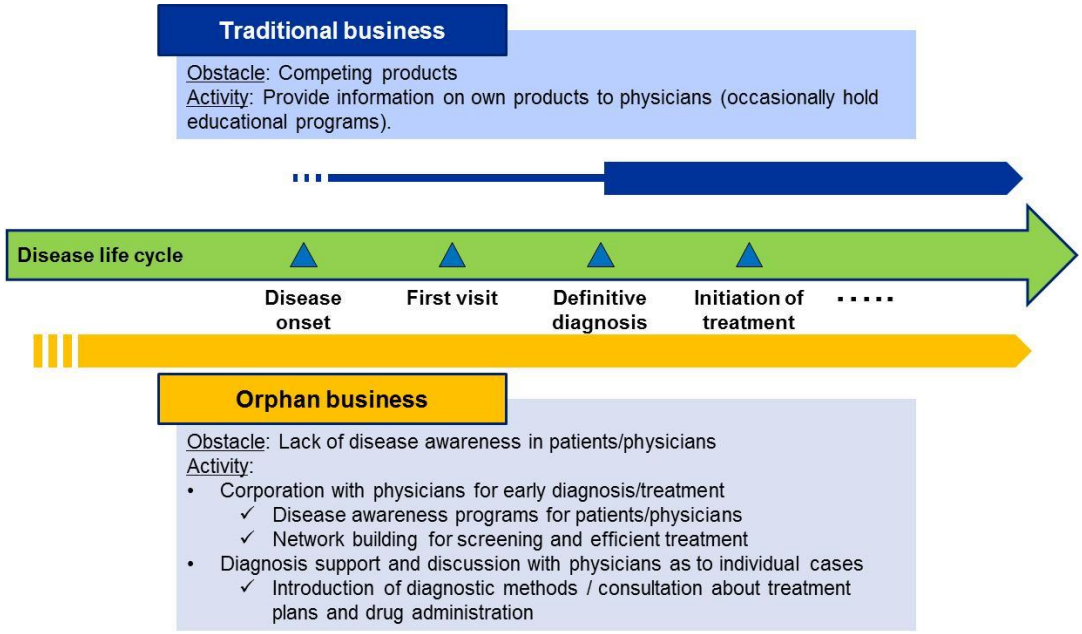
As the orphan drug market has a different landscape from traditional mass markets, strategies and activities at HQ must also be customized. Common issues that HQ often encounters include:

- From marketing perspective:
  - How to determine target hospitals / physicians when patient population is so small
  - What kind of measurements to be taken for each stage of product life-cycle?
- From sales perspective:
  - Will physicians respond to the same promotion as for other specialty market?
  - Is the knowledge of own MRs / medical affairs enough to meet physicians' information needs?

To address the above questions, we would like to introduce ideal promotional activities at three stages of the disease life cycle: 1) prior to disease onset /initial visit, 2) from disease onset /initial visit to the definitive diagnosis/initiation of treatment, and 3) after the definitive diagnosis/initiation of treatment (Figure 1).

1. **Prior to disease onset /initial visit**
   The orphan drug business is characterized by its activities targeting the general population, before disease onset or realizing symptoms. Especially for infant onset genetic diseases, early diagnosis / treatment initiation are crucial since the diseases are usually progressive and life-threatening, and greatly affect quality of life. To achieve this, neonatal screening is an effective measure but requires collaboration with academia, patient associations and local governments. Another key to success in this stage is how pharmaceutical companies can support physicians to understand the importance of screening.

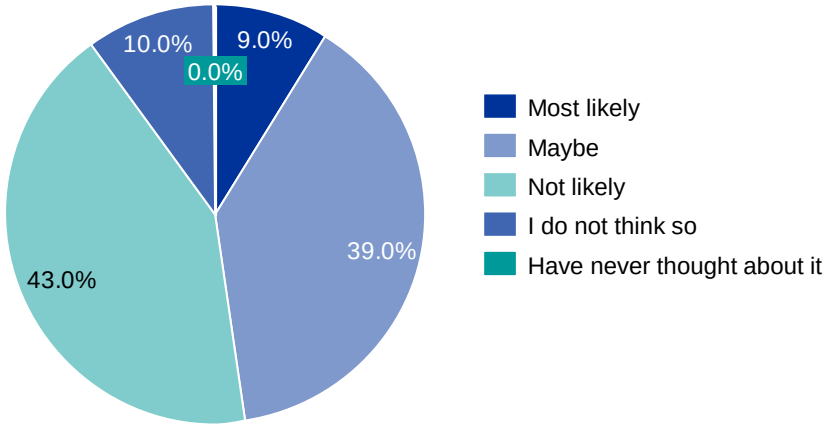
Figure 1: Traditional vs. orphan drug business – activities through the disease life cycle



2. From disease onset /initial visit to the definitive diagnosis/initiation of treatment

One might deduce that the general population is unfamiliar with rare diseases, however, fears and concerns for such diseases are not small. A Deloitte survey shows that 85% of parents of children aged 6 years or younger worry about such diseases, and about 50% of them have consulted with their family doctors. Conversely, another survey reveals that only 50% of physicians think they will have a chance to see rare disease patients (figure 2). Pharmaceutical companies should offer educational programs to make physicians realize that rare diseases are an immediate issue and a matter of themselves.

Figure 2: Result of physician survey
Q: Do you think you will actually have a chance to see rare disease patients, including suspected cases? (n=100)



Source: Deloitte Survey

3. **After the definitive diagnosis/initiation of treatment**
   Even after making a definitive diagnosis, many physicians run into difficulties in treating a patient since they do not have enough experience in using the appropriate drug and there are only a few articles to refer to. Naturally, they ask MRs for detailed information such as case-specific advice on treatment, appropriate dosage and possible adverse events. To comply with such needs from physicians, pharmaceutical companies need to establish a well-structured cross-sectional knowledge management framework so that MRs share information on individual cases and medical affairs publish articles based on PMS data in a timely manner.

Acting as a partner of physicians in making treatment decisions for individual patient is how orphan MRs should be, and probably, this is also how every MR should be and what many MRs dream to be. Of course, such activities are only feasible when HQ back them up. Pharmaceutical companies have to establish a system to support MR activities and promote personalized medicine.

Orphan business model

To achieve profitability in the orphan drug business, designing a small field force that has nationwide cover and establishing close corporation between sales and marketing for effective promotional activities is key.

In this model, MRs must prioritize core hospitals in a region, university hospitals for instance, and attempt to educate and build close relationships with physicians so that they can consult the company for definitive diagnosis immediately when they see suspected rare disease patients.

Figure 3 indicates the patient flow until they receive a definitive diagnosis and actions that the companies should implement. In addition to the provision of drugs and information, pharmaceutical companies are expected to take a role to promote early diagnosis and medication to reduce the burden of patients and society.

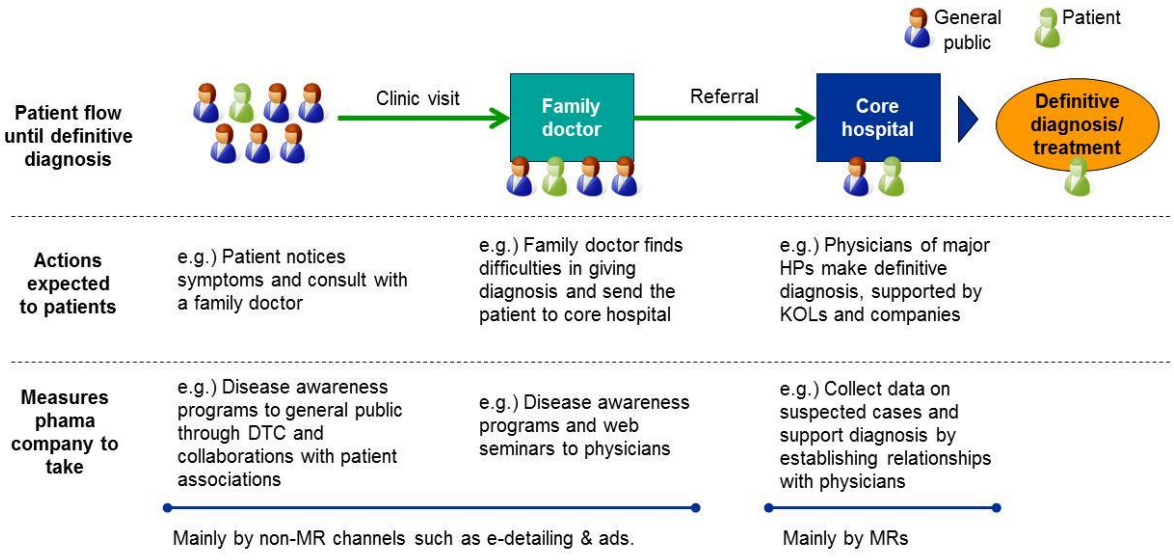
In this model, MRs are supposed to visit only core hospitals in a region. Clear targeting of hospitals and physicians clarifies roles of MRs in order to promote early diagnosis and treatment, resulting in successful marketing with a small number of MRs.

In this orphan drug business model, the effectiveness of marketing should be evaluated in terms of the market response, in addition to conventional sales measurement such as prescription / sales outcomes. ROIs in orphan drug business model include:

- If the awareness of rare diseases increased among general public including patients (e.g. conduct a qualitative research)
- If the awareness of rare diseases increased among physicians (e.g. conduct a qualitative research)
- If the number of physicians interested in the rare diseases (e.g. observe trends in a number of participants in seminars)

Currently, it is often the case that a single drug is available for a rare disease indication thus companies seem to focus on generating new prescriptions rather than ensuring securing existing prescriptions. To be prepared for future competition and not to go back to conventional SOV metrics, companies must offer information and support for physician which satisfy their demands and differentiate their products.

Figure 3: Image of typical patients flow until definitive diagnosis and breakdown of actions companies should take



Transforming into next-generation pharma company

Successful companies in the orphan drug business have accumulated know-how in relationship building with physicians and have realized an effective channel mix in promotion, including DTC advertisement and disease awareness programs. This experience is a strong advantage to succeed in the future market where personalized medicine is expected to become more dominant.

Some of the elements of this orphan drug business model can also be adapted to other markets for sales / marketing cost reduction, especially for restructuring and streamlining MR activities and for promoting multi-channel marketing.

Considering the advent of personalized medicine, an ever changing healthcare environment, necessity to carefully address patients' needs as well as the situation that companies are more focusing on patient centricity, success factors in the orphan drug business might hold the key to success in transforming into pharmaceutical companies of the next generation.

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