Ensuring Launch Success in Rare Disease Areas by Creating a Value Story

A number of trends (both in science and on the regulatory front) bode well for the development and availability of drugs to treat rare diseases. Currently, there are hundreds of requests under consideration by the US Food and Drug Administration (FDA) for orphan drug status.[1] However, given continued pressure on payers to curb healthcare spending, launch success will still depend upon having a strong value story — one that resonates with all stakeholders. Drug companies are, of course, practised in developing evidence dossiers that address the needs of regulators, payers, and prescribers, but have less experience in incorporating insights from patients and caregivers in their development work. Taking this step for therapies treating rare diseases is every bit as important as in other therapeutic areas, but even more challenging because of the scarcity of patients.

We believe that a company's ability to appreciate the thoughts, values, and preferences of patients and caregivers will be a primary differentiator for launch success for products to treat rare diseases. Fortunately, patient awareness, understanding and involvement in clinical development are becoming more sophisticated, making it less challenging for drug developers to seek their perspectives. In fact, a project supported by the Innovative Medicines Initiative in the EU is designed to educate and train patients so that they can be more effective partners in clinical development. And, we've observed that many sponsors now hold regular meetings with patient advocacy groups to gather feedback for consideration in their development plans.

In the current environment, it is essential that a patient-centric approach is taken early in development so that work starts with the end in mind. In fact, *it is never too early in the development cycle to consider what will be required for commercial success.* There's a tool that is commonly used to ensure these activities are planned for at the start of a clinical trial — the Integrated Medical Plan (IMP). The IMP is a best practice for ensuring that the development strategy will be successful is to create an IMP that incorporates the Medical Affairs Evidence Plan as well as the Clinical Development Plan. The IMP should be in place from the start of first-in-human (FIH) trials and extend all the way through commercialisation to define what evidence needs to be collected.

While the Clinical Development Plan outlines what regulators will need, the broader IMP addresses the needs of other stakeholders. The IMP should specify why, where, when, and how the drug will be used — in other words, it will lay out the value story. It should then identify the evidence needs for the key stakeholders and any existing evidence gaps that will drive operational plans to gather the necessary evidence. These are critical steps in being ready for successful commercialisation.

There are many mechanisms for identifying the evidence needs of each stakeholder group, but at a basic level, it involves asking a set of key (but different) questions to each group that are then effectively answered in the value story. The IMP is an ideal place to store the pertinent information you gather for each of your key stakeholders. It is necessary to gather patient information like this across geographies as views may vary from region to region. For example, research should allow marketers to devise answers to the following questions from patients/caregivers:

- Why do I need this medicine?
- What do I need to understand about how this medicine works?
- Is your drug more efficacious than the alternatives?
- What side-effects may I suffer and how will they be managed?
- How long do I need to take this medicine?
- What support will I be offered to manage my illness more effectively?
- Can I afford this medicine?

In the end, it will be necessary not only to answer these questions for patients and caregivers, but also to have the evidence to substantiate those answers. Patient and caregiver ability to digest data and to make informed decisions about their care is improving, a phenomenon that will be advantageous to those companies that cater to patient and caregiver information needs.

Taking the time to map out evidence needs in this way significantly increases a company's chances for a successful launch. Developing an IMP requires an interdisciplinary approach within sponsor companies and a collaborative approach with patient communities and advocacy groups. And, making best use of the insights gathered to speed development will require greater cooperation between all stakeholders.

REFERENCES

1. https://www.fda.gov/downloads/AboutFDA/ReportsManualsForms/Reports/BudgetReports/UCM488554.pdf

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