Look before you leap: How a joined-up development strategy pays off for early-stage biotechs

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Developing new therapies is an enormously exciting endeavor—after all, what can be more rewarding than developing an important new therapy for patients? But that excitement and optimism can sometimes lead biotechs to launch into a clinical program without having the opportunity to properly dissect future regulatory and payer challenges, and that can undermine value.

A well-thought-out strategic development plan that brings together nonclinical, clinical, regulatory and commercial experts can improve efficiencies, cut costs, shorten timelines, and increase the chances of success for a new drug program.

Clinical development is a biotech's biggest investment, and success can unlock the biggest rewards. Still, many companies fail because they do not align clinical execution with a clearly defined regulatory strategy or due consideration of the commercial realities their product may face if it gets approved for marketing.

Many biotechs are operating to a just-in-time model, and in the early stages of a development program, the complete picture is difficult to see when they are concentrating on the next inflection point for the business, such as the outcome of scientific studies, the readout of a clinical trial, or the next meeting with potential investors.

"Sometimes biotechs are hyper-focused on the science or technology early on—particularly when they are running "lean and mean" with a small team. They have a target identified, and they are moving through preclinical development with an eye on starting clinical trials swiftly," according to Nate Akers, Vice President, Business Development East Coast, at Parexel Biotech.

It is, however, important to take the time to reflect—even if the CEO of the company is pushing for rapid progress—and make sure the clinical development plan for a compound is the best one to take forward, and flexible enough to allow the company to pivot if faced with unexpected developments.

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A frank and honest appraisal of the available competencies in-house, as well as the gaps where external help is needed, is invaluable.

Bringing the clinical, regulatory and commercial elements together to build an integrated value story can make the clinical development process smoother, increase the likelihood of delivering a new, approved therapy to patients, and avoid additional, duplicative work that might be needed if the approach is not right the first time.
A well-thought-out and defined regulatory strategy early on is critical, and biotechs should start at the target identification or asset acquisition stage.

Early alignment with a regulator's thinking is key to reducing risk, and allows a biotech to get feedback on preclinical data, as well as a trial's design before starting human studies. A clear roadmap also helps a sponsor have a consistent story that can be communicated both within the organization—making sure everyone is pulling in the same direction and resources are allocated appropriately—and to the external world, for example, to potential investors. And it needs to be dynamic, not a one-time exercise that stays static through the duration of the project.

Risk reduction

Some of the benefits of an integrated approach are reducing risk during the drug development process, increasing the chances of getting the project right the first time, and setting realistic timelines.

For example, biotechs will want to consider whether the indication that seems the most promising or accessible at the outset is the best option. Similarly, the choice of clinical trial design may have room for improvement—sometimes, a modification to the design can reduce the number of subjects and/or investigation sites that are needed, cutting the time to completion and the cost of running the study.

That could include introducing decentralized tactics to make it easier to recruit patients, or adaptive studies designs that can test multiple indications at once. Based on a medical and biostatistical appraisal, it may be possible to develop a study design that incorporates an interim data readout that provides an early go/no go decision point, or could allow a dialogue with regulatory authorities over a shorter route to filing.

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Path to approval

While the temptation may be for biotechs to develop drugs for their home market, in some cases, a broader view may be beneficial, according to Vicky Hsu, Parexel Biotech's Head of Biotech Operations in Asia.

There may be a compelling case to develop a medicine first for a market with a high incidence of a disease. For example, a drug candidate for hepatocellular carcinoma—the most common form of liver cancer—could benefit from a program that includes a cohort from China, which accounts for almost half of all new liver cancer cases worldwid[1].

"If a biotech wants to get a drug approved in China, they will need to consider a study design that meets the local regulatory requirements of the Chinese regulatory authorities," says Hsu.

That could have a bearing on how many patients in the study are enrolled within China, but it may be possible to design a regionally and/or globally focused trial that meets the varied requirements of multiple regulatory authorities in order to maximize the return on a single protocol.

That is an increasingly important consideration for biotech, as Asia has emerged as a growth area for clinical research, outstripping North America and Europe as a destination thanks to a large population of treatment-naïve patients and high-tech/low-cost clinical trial centers, according to Frost & Sullivan[2].

Reimbursement

Of course, many biotech companies are already applying an integrated strategy to their development projects, considering from the outset the competitive landscape, making sure there are fast-follower candidates to back up the lead molecule, and the regulatory and commercial aspects.

But some—and particularly those born out of academia—sometimes miss out on that opportunity to carry out that level of due diligence. And while clinical and regulatory teams often work hand in hand, commercial and reimbursement considerations may be more challenging.

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One of the transitions a biotech can make is going from a clinical organization to a commercial organization with a product to sell and revenue expectations. Many biotechs can find challenges as they try to onboard a commercial group and develop a commercial strategy in the months leading up to a regulatory decision on a product. However, there are opportunities for sponsors to pursue considering commercial strategies and ways to increase asset value much earlier in the development process that can pay dividends for them downstream. A lot can be done early in clinical development in regards to collecting data that is minimally burdensome on patients, while not impacting the cost of conducting a clinical trial, that is meaningful to both payers and partners. The right strategy can increase asset value and support a commercial team that will be put in place downstream.

Moreover, doing that work during the development process could sidestep the need for a post-marketing trial or for gathering real-world evidence after approval to make a case for including their new product on formularies or winning the backing of health technology assessment (HTA) agencies.

At the same time, those discussions can be an advantage even if biotechs are not planning to commercialize a therapy themselves—for instance, if the plan is to out-license after showing proof-of-concept—as the value of the asset can be increased. And demonstrating that value can be a major consideration given that emerging biotechs generally rely heavily on investment funding.

The best CROs will look at a molecule as if it is their own and act as an extension of the sponsor team and—if they can provide expertise across the clinical, medical, regulatory, and commercial spectrum—they can serve as a one-stop shop for customers, according to Hsu.

That avoids needing multiple CRO partners, which can present challenges when integrating all those data sources into a unified, cohesive whole.

"Many of the companies we work with are small, and they need a CRO to be a knowledge bank that delivers reliable outcomes using comprehensive data. The CRO should have a personalized approach, contact flexibility, and a lean structure," says Hsu.

"We can support them in identifying the right regulatory and reimbursement strategy, the market potential, the competitive landscape, and even which indications they should go for based on the data."

References

(1) Liver cancer incidence and mortality in China: Temporal trends and projections to 2030 - Chin J Cancer Res. 2018 Dec; 30(6): 571–579.

(2) Global Contract Research Organization (CRO) Market: Frost & Sullivan; 2020

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