You have a promising, potentially life-changing or life-extending drug in a Phase III clinical trial. What are the chances of doctors prescribing it? And the likelihood of patients taking it, considering its cost and type of administration? Will insurers give it an optimal formulary placement? Will the safety data satisfy regulators?

The answers can make a big difference in the success of a drug, its safety and benefit for patients, and acceptance by regulatory authorities. That is why real-world evidence (RWE) studies are more essential today than ever before. The growing need is driven by reimbursement agencies, health care providers and regulators.

Are contract research organizations (CRO) prepared to help sponsors uncover the likely real-world success of the investigative drug?

RWE studies complement clinical studies to provide a more comprehensive picture of a product’s clinical and economic value to patients, payers, and prescribers and also to the sponsoring life sciences company. The demand for RWE is increasing as more and more health care providers progress toward linked computerized records, and decision-makers at biopharmaceutical companies as well as external health care entities become increasingly aware of its value.

The results of RWE studies can create relevant evidence to gain appropriate reimbursement from public and private payers, and help sponsors develop products with optimized safety, pricing, utilization, patient outcomes and standards of care.

This article explains the importance of RWE studies, the expanded role for CROs and the advantages of strategic partnering with a highly qualified CRO for the seamless delivery of both clinical and real-world studies.

**THE VALUE OF RWE STUDIES**

Capturing data about the actual experience of patients outside the carefully controlled clinical setting can help fill the knowledge gap between clinical trials and actual clinical practice. The information generated can enable providers, innovators, health plans, researchers and others make faster, more efficient and less costly advances in medical research and clinical treatment. Life sciences companies can use the data to explore the benefits and risks of treatment options, expedite enrollment in clinical trials and identify new targets for research and development.³

Reimbursement agencies, regulators and providers want to encourage and incentivize high-quality, relevant RWE generation. Payers and clinicians are eager to see more detailed health outcomes data to make more informed prescribing and reimbursement decisions. These entities need to be clear about what evidence they want and how they want it to be generated. Where appropriate, they need to partner with the life sciences industry to generate high-quality, relevant RWE. Payers may require validation of a product’s real-world clinical value and cost-effectiveness to determine optimal formulary placement. A regulatory authority can use RWE to determine real-world safety.

At the earliest stages of drug development, RWE can be generated on the disease, its natural history, treatment patterns and treatment outcomes. This RWE can be used to characterize and quantify the burden of illness the new product will try to alleviate. The RWE also can be used to inform clinical study design. RWE on the new product itself can only be generated after the product has been approved and it has entered the “real world.” There is a “Catch-22” here because RWE is needed to gain optimal formulary positioning, but optimal formulary positioning is needed to generate true RWE of product performance and value.

In 2013, the European Medicines Agency (EMA) issued guidelines for RWE studies, requiring risk-benefit data in addition to post-authorization safety studies. The U.S. and other countries are following the EMA guidelines, while the U.S. Food and Drug Administration (FDA) is in the process of developing them.

**PARTNERING FOR CLINICAL AND RWE STUDIES: AN EMERGING ROLE FOR CROS**

As clinical studies continue to be more complex, global and costly with more rigorous regulatory requirements, sponsors have been increasingly relying on outsourcing services, working with one or a few contractors as strategic partners. Outsourcing of pharma and biopharma contract services has grown exponentially during the past 15 years, as CROs evolve their services in response to and in anticipation of changing industry needs.⁵ A 2015 Nice Insight survey indicates that 73% of emerging biopharmaceutical companies are interested in a strategic partnership with a CRO or contract manufacturing organization (CMO) in the next 12-18 months.⁶ The global CRO market is forecasted to grow at a CAGR of 9.8 percent over the period 2014-2019, according to a recent report by Infiniti Research Ltd.⁷

What do sponsors seeking both clinical trials and RWE studies expect of their CROs? Why and how should CROs strengthen their capabilities in this area?

RWE studies include randomized clinical trials and pragmatic research in real clinical practice to better reflect the value of a new medicine. Data collected in these and in non-interventional, non-experimental settings offer an unbiased view of RWE, which supports decision-making. The advantage of RWE over clinical trial data is that it often represents a broader population over a longer timeframe, and provides information on comparators and outcomes that are not part of the clinical trial protocol.

An impactful RWE study design is enhanced by a deeper understanding of the real-world environment into which a new product will be launched. To achieve this, sponsors or their research collaborators (e.g., CROs) must have access to robust real-world health data, and the technology and analytics capability to

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GETTING REAL

Partnering for clinical and real-world evidence studies

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REAL-WORLD OUTCOMES

effectively analyze that data, including administrative claims data and other clinical data sources. Other prerequisites for real-world pragmatic trials are scientific excellence, therapy area expertise, the scope and scale of operations to efficiently execute studies, expertise in clinical trial design and health economics and outcomes research (HEOR), as well as a knowledge of related regulatory requirements and guidelines.

CROs that lack the full range of required capabilities will need to develop a strategy for acquiring, partnering or gaining access to the appropriate missing resources. There are many research consultancies that can provide the scientific expertise and capability. However, there are fewer sources of relevant and accessible real-world health data. Having consistent access to a comprehensive, real-world health data set through a partnership or alliance is preferred to ad-hoc collaborations with several health data providers with different data sets of different quality and scope.

With the growing availability of electronic health record (EHR) data, there are now opportunities to collect clinical detail on health outcomes, together with traditional data mined from insurance claims, such as prescription use and physician and hospital visits, to provide a comprehensive picture of what the current standards and patterns of care are. Using EHR data will require the CRO and data provider to be fully understanding of and compliant with health data privacy regulations.

HOW ARE CROS BOLSTERING THEIR CAPABILITIES?

To meet the increasing demand for these studies, CROs are ramping up their capabilities in this growing area of interest, building the necessary expertise to leverage the opportunities offered by real-world data and bring clients the full gamut of research services they need. Adapting to industry needs, CROs are scaling up internal capabilities to provide RWE research and late-stage study activities, utilizing advanced IT systems and technology.

As an example, PPD and Anthem’s HealthCore recently formed an alliance to expand their respective services in the RWE research market. The alliance combines clinical trial design, HEOR, medical affairs research, epidemiology services, access to large databases of medical and pharmacy claims and lab results, real-world research designs, analytics and qualitative research. Together, their RWE capabilities include: observational research; post-approval safety studies; Phase IV trials; patient, product, and disease registries; patient-reported outcomes; and economic evaluation.

Their combined services aim to create relevant evidence to help sponsors gain appropriate reimbursement and develop products with optimized patient outcomes.

BENEFITS OF STRATEGIC PARTNERING

As the trend of long-term strategic collaborations with global service providers continues, most sponsors prefer to rely on a single strategic CRO partner to conduct both their clinical trials and RWE studies for optimal efficiency and effectiveness. They expect their CRO to have the capabilities, technology and expertise to provide the full range of development services they need.

The major advantages of partnering with a single CRO include the seamless integration of development services and consistency and efficiency of working with the same team through all phases of product development and post launch. Preparing one all-inclusive research contract for clinical trials through to post-approval research also saves considerable time. Another time- and cost-saving benefit is a greater return on the company’s investment in educating and training only one CRO about the investigational product.

Strategic partnerships between drug developers and CROs can dramatically decrease a drug’s time to market. According to the Tufts Center for the Study of Drug Development, clinical trials conducted by CROs are completed, on average, 30% faster than those conducted in-house. Strategic partnerships also build commitment and trust over time.

SELECTING A CRO AS A STRATEGIC PARTNER

Look for a service provider offering integrated development services, including all clinical phases and RWE studies. After initial due diligence and determining the best candidates, conduct an audit history and look for gaps in specific expertise. Acquire information on the prospect’s quality record and project management expertise. A positive regulatory history is also important.

In addition to operational, methodological and clinical experience, the CRO must have expertise in clinical and RWE studies. Also look for advanced technical capabilities, reliability, high quality of services/reputation, history of on-time delivery, strong quality record, etc. Work collaboratively with the sponsor to develop operating procedures, and have a dedicated project manager.

LOOKING FORWARD

Today, more education and guidance are available on RWE and other non-interventional approaches, and groups such as the Agency for Healthcare Research and Quality (AHRQ), the Patient-Centered Outcomes Research Institute (PCORI) and the National Institutes of Health (NIH) are moving closer to an agreement on uniform research standards governing this area. There is increasing demand from the FDA for drug manufacturers to conduct observational studies on a new product’s effectiveness, and payers and clinicians seek more detailed health outcomes data to support prescribing and reimbursement decisions. In this emerging environment, CROs will benefit by meeting industry demands for strong capabilities in providing RWE studies.

References

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