FIVE CRITERIA TO USE WHEN SELECTING A CRO FOR A **GENE THERAPY STUDY**

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CHOOSING A CRO FOR GENE THERAPY TRIALS

Contract research organizations (CROs) have been an integral part of drug development since the 1920s, first as providers of preclinical testing services, then as organizations that could manage clinical trials on behalf of their sponsors. While this makes the majority of CROs knowledgeable experts in the management of clinical trials, especially for traditional small-molecule therapies, not all CROs are equally suited to oversee trials of next-generation medicines such as cell and gene therapies (CGT).

This reality comes at a time when such therapies are quickly making their way from theory to clinical practice. Just four years ago, the first CGT treatment was approved by the FDA. Today, nine therapies are approved, and, according to PhRMA, the number of treatments in clinical trials increased by 25% over the past year, going from 289 to 362.

Even more treatments are in preclinical stages and are expected to enter the clinical trials pipeline. Given this, many biopharmaceutical companies are looking for CRO partners with significant experience conducting CGT-focused clinical studies. Here are five criteria to consider when evaluating organizations:

1. EXPERIENCE WITH THE INDICATION OR TREATMENT MODALITY

First and foremost, a CRO must be able to demonstrate their experience with gene therapy trials. They should be able to provide the number of CGT projects completed, organized by indication and modality. A CRO that has performed studies involving cell therapy, gene therapy, gene editing and related modalities is an attractive partner, as is a CRO that has experience with rare diseases, such as hemophilia, muscular dystrophy, Huntington's disease or any of the other approximately 7,000 diseases covered under the Orphan Drug Act.

2. ACCESS TO PATIENTS AND FOCUS ON PATIENT CENTRICITY

The patient recruitment capability of a CRO is also important. CROs often maintain relationships with many hospitals and investigators, so they can recommend sites with high enrollment potential, which can be critical to completing trials on time and obtaining quality data. CROs with a patient-centric approach earn high marks because cell and gene therapy studies require access to unique patient populations and specialized training and support resources. CGT trials also require innovative approaches, such as long-term patient follow-up strategies and even decentralized trials in which technology is used to communicate with study participants and collect data.

3. GEOGRAPHIC REACH AND REPUTATION

The geographic reach of a CRO becomes more important later in clinical development. For example, Phase III multicenter trials typically require the involvement of several countries. For studies focused on rare diseases, check to see that local services, such as regulatory services and onsite monitoring, are available in each of the countries where the research is conducted. During an assessment, ask whether a CRO can conduct studies in various countries, but also ask about a CRO's reputation internationally, which may vary from region to region.

There is a promising future ahead for drug discovery and development worldwide. CROs will continue to be an integral part of new drug research and development around the world. With many options available, the importance of vetting and choosing the right CRO – one with preclinical expertise and facilities for an IND application – is becoming increasingly important.

Sponsors should expect that part of a CRO's qualifications include having a formal IND service offering, a senior team of scientists and a global presence. Abiding by these standards can lead to a more fruitful partnership and help make it easier to advance promising compounds into new drugs approved for patients.

4. EXPERTISE WITH THE CLINICAL LABORATORY IMPROVEMENT AMENDMENTS (CLIA)

The objective of the CLIA program, which is managed in the United States under the Centers for Medicare & Medicaid Services (CMS), is to ensure quality laboratory testing. CLIA certification is needed to conduct testing for patient enrollment and post-marketing surveillance

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purposes since patient-specific results may be reported. That means it's important to work with a CLIA-certified laboratory to ensure developed assays comply with CLIA regulations from the start and can be carried through all stages of development. A CRO with a dedicated CLIA director and with CLIA certification from all 50 states can be a significant advantage.

5. DIVERSE PORTFOLIO OF SERVICES AND ROBUST QUALITY MANAGEMENT SYSTEM

Ideally, a CRO will have scientists on staff who have proven experience designing custom nucleic acid assays for drug modalities as well as pharmacodynamic (PD) markers. They will also have multiple technology platforms at their disposal, including hybridization ELISA, ultra-performance liquid chromatography/high-resolution mass spectrometry (UPLC-HRMS), UPLC-MS/MS, hybridization-LC-FLD, LC-UV, and quantitative polymerase chain reaction (qPCR). Finally, CROs that bring cutting-edge innovations to the table, such as data-centric technologies and artificial intelligence, may offer some advantages.

The same holds true for quality management. According to the International Conference on Harmonization Good Clinical Practice (ICH GCP) guidelines, sponsors are ultimately responsible for implementing and maintaining quality assurance and quality control systems with written SOPs to ensure that trials adhere to Good Clinical Practice (GCP) and applicable regulatory requirements. However, the CRO is also required to implement quality assurance and quality control. Sponsors can ask to conduct audits of potential CROs to authenticate quality management systems, with the goal of understanding how the organization handles protocol deviations, whether its corrective actions and preventive actions (CAPA) plans are effectively managed, and whether the company is ISO 9001 certified.

CRO SELECTION CHECKLIST

Clearly, the demand for cell and gene therapies will accelerate as the industry continues to advance nucleic acid drug delivery technologies and as patient advocacy groups raise awareness of rare and orphan diseases and their impact on patients. Biopharmaceutical companies that embrace this new frontier must find a CRO that is a true partner. Sponsors should promote transparency and willingness to share information, while CROs should focus on responding in a timely manner and participating actively in strategic planning for clinical trials, as well as for commercialization efforts. Even more important, CROs need to be prepared to address the unique challenges that CGT trials present. These challenges could be related to off-target toxicity, delivery or cost management. Whatever the particular issue, experienced CROs will anticipate challenges and be ready to offer solutions with agility, flexibility and speed.

Selecting a CRO with proven CGT experience is an important challenge. A thorough and thoughtful examination of CRO candidates, using the criteria above to develop a checklist, can improve the odds of finding a suitable partner who can help successfully bring a gene therapy drug to market.

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