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Executing Early Phase Research In Hematological Oncology: What Matters To Biotech?

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Oncology continues to lead the way as a therapeutic focus for the pharma industry. In the latest Pharma Intelligence R&D Review 2022, there were 7,772 anticancer drugs currently in development: an increase of 14% from the previous year, accounting for 39% of the total pharmaceutical pipeline.[1]

Within the area of oncology, one of the most prominent areas is hematological malignancies. According to recent data, hematological malignancies are the fifth most common type of malignancy globally, and the second major cause of death in the United States.[2] As a result, pharma has reacted and an increasing number of treatments for these diseases are reaching the stage of clinical trials.

Early phase studies are a critical stepping-stone, as they are the first time an investigational medicinal product (IMP) is tested on humans. They are complex to execute, and any mistakes can be incredibly costly for sponsors looking to progress closer to commercialization. For smaller biopharma companies with limited financial resources,

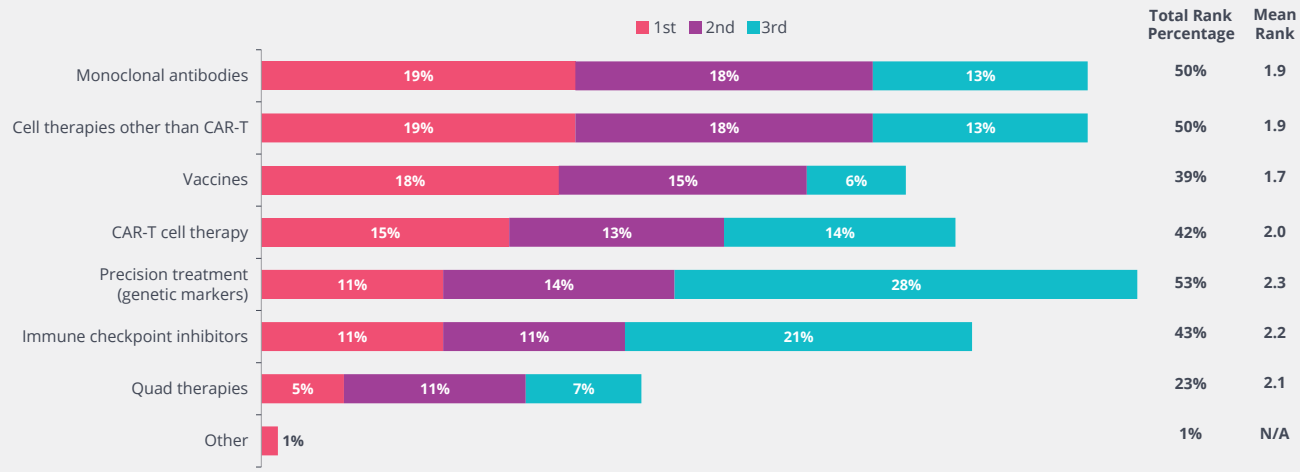
expertise, and significant pressure from investors, the stakes are particularly high. Pharma Intelligence and Allucent conducted a survey of biotechs in the hematological oncology space to understand the challenges they have faced in their early-phase research, what support they need to execute trials successfully, and what the future of these studies looks like.

What's On The Horizon For Hematological Oncology Trials?

The pharma industry is focusing on the need for treatment of hematological malgnancies, with its market size expected to reach $120.56 billion in 2028 at a compound annual growth rate (CAGR) of 10.7%.[2] Of the respondents who participated in the Allucent and Pharma Intelligence survey, 74% were developing therapies for more than one hematological malignancy. When asked which hematological malignancies posed the greatest challenge for drug development, the top four were Acute Myelogenous Leukemia, Acute Lymphocytic Leukemia, Chronic Myelogenous Leukemia, and Myelodysplastic Syndrome.

As well as identifying a target disease, the decision as to which therapeutic agent will be most effective in treatment is critical. Survey respondents ranked monoclonal antibodies (mAbs) and non-CAR-T cell therapies at the top for hematological oncology (both ranked first by 19%) followed very closely by vaccines (18% ranked first) (see Figure 1). While mAbs and cell therapies are becoming established in oncology drug development, therapeutic vaccines have seen a new wave of interest following the success of mRNA technology during the COVID-19 pandemic.

Figure 1: Agents' Potential In Treating Hematological Malignancies In The Future



Question: Please select and rank up to 3 agents that have the most potential in treating hematological malignancies in the future?
(Please select up to 3 agents and assign a value/rank from 1 up to 3 for each item, where 1 is the most important and 3 is the least important. Value/ranks may not be repeated)
Base: All respondents: Pharma & Biotech companies: annual R&D $999M or lower (n=217); multiple answers permitted.

The expected growth of the market and promise of different modalities offer great potential for patients suffering from hematological malignacies, but early phase trials can be a stalling block to treatments reaching them. Proving this, 64% of survey respondents have experienced delays in their early phase hematological oncology studies (see Figure 2). Somewhat reassuringly, the main reason cited was the COVID-19 pandemic, which halted many trials in its onset due to regional lockdowns and social distancing requirements. As the world moves towards a new normal, delays due to COVID-19 are less likely to arise. However, other key factors included patient recruitment and start-up timelines, which are more entrenched challenges in drug development.

Figure 2: Delays In Early Phase Hematological Oncology Studies

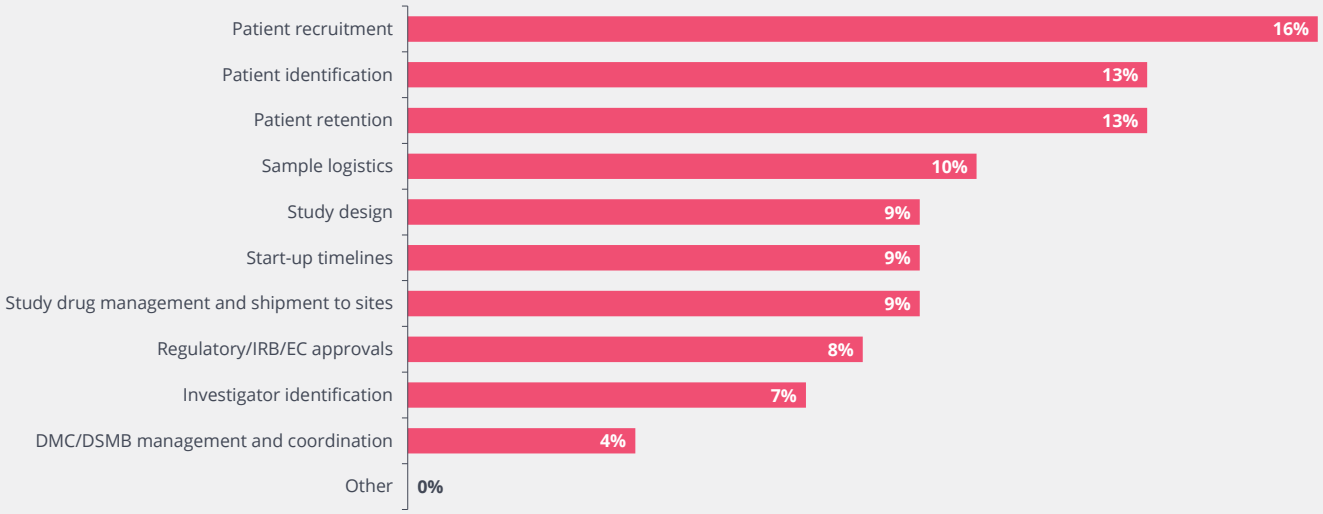
Question: Have your previous early phase hematology/oncology studies faced delays?
Base: All respondents: Pharma & Biotech companies: annual R&D $999M or lower (n=55).

Question: What were the reasons for these delays? (Please select up to three reasons)
Base: All respondents who experience Early Phase Hematology/Oncology Study delays (Pharma & Biotech companies: annual R&D $999M or lower) (n=91); multiple answers permitted.

Adapting Trial Strategies To Overcome Challenges

Respondents indicated that the challenges of early phase hematological oncology clinical trials correlate with those frequently cited as barriers to studies more generally, all relating to patient participation. The top three concerns were patient recruitment (16%), patient identification (13%), and patient retention (13%) (see Figure 3).

Figure 3: Oncology And Hematology Early Phase Clinical Study Challenges

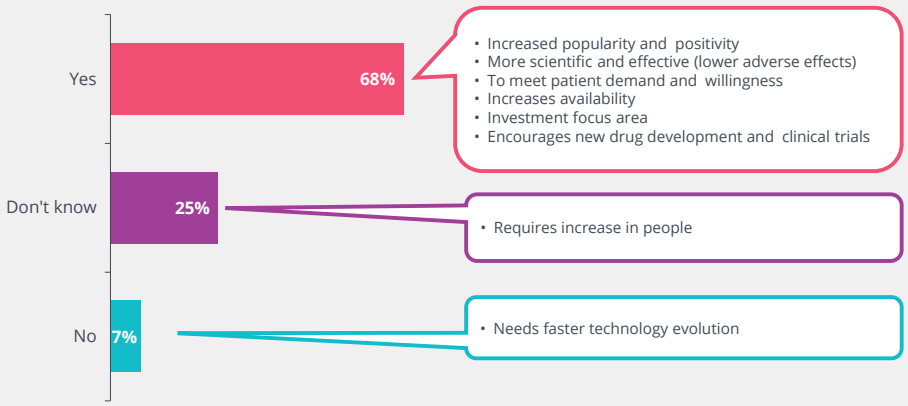


Question: What are the most significant challenges of conducting oncology and hematology early phase clinical studies? (Please select all that apply)
Base: All respondents: Pharma & Biotech companies: annual R&D $999M or lower (n=212); multiple answers permitted.

Since the onset of the COVID-19 pandemic, decentralized trial models have been more widely implemented to continue clinical research while travel and social mixing were limited. Leaders in the industry have seen the opportunity to increase participation potential in the longer term, as decentralized trials minimize the amount of time and effort associated with site visits. Unfortunately, in hematological oncology these models are more difficult to implement than other therapeutic areas. This is due to treatment administration, which is usually intravenous and therefore extremely difficult to conduct in a home setting. Moreover, early phase trials are the first time a drug has been tested on humans, so monitoring patients in a clinical setting is key to ensuring their safety and gaining the data required for pharmacokinetic and efficacy assessments.

Early phase hematological oncology studies could be made more effective through the use of adaptive trial designs. This model enables sponsors to review data from the trial on a scheduled interim basis and make pre-specified changes to its course based on the insight gained. Changes that benefit patient-centricity could include refinement of sample size, changing the allocation ratio of patients to trial arms and identifying those most likely to benefit from the treatment.[3] The opportunity this presents has not been overlooked by the industry, with 93% of respondents stating they were currently using adaptive designs in their clinical trials. Moreover, 44% of respondents stated that these models accounted for over half of their current studies. Adaptive design prevalence is only set to grow, with 68% of survey participants indicating they would expect to increase the number of these trials in the future (see Figure 4).

Figure 4: Future Adaptive Trials



Question: Do you think the adaptive trials will be more common in future and why/why not?
Base: All respondents (n=28).

Nonetheless, there are challenges to implementing adaptive design trials. Of those who utilized these models, they noted that statistician expertise was the most significant issue they had encountered. As the changes made from adaptive designs all hinge on the analysis of trial data to make informed decisions, the lack of ability to interpret results is a key barrier to efficiency and reaping potential benefits.

Another key factor for the success of hematological oncology early phase research, irrespective of trial design, is to partner with sites that have the necessary attributes to execute trials. When asked to select the qualities most important to them when assessing site readiness, respondents ranked having the resources for the delivery of innovative therapies as the top priority (19%). This was followed by patient population (16%) and relationships with

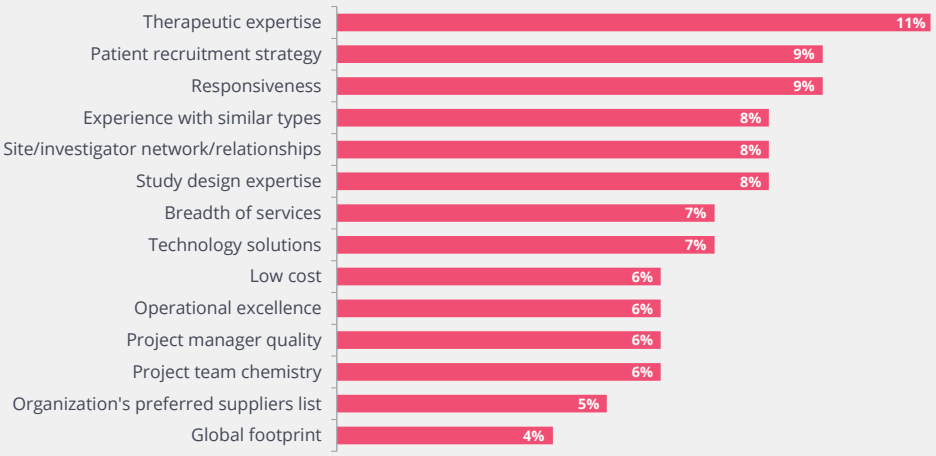
referring clinics/hospitals (14%). There has been an exponential growth of advanced treatments such as cell, gene and RNA therapies in recent years, with 3,579 therapies in development as of April 2022.[4] The level of interest in the space and its potential for hematological oncology treatments mean it is more important than ever that sites have evolved at the same pace as science, and are fit for purpose to facilitate these clinical trials. This is especially important for biotech companies, who need early phase trials to be executed efficiently and generate data for investor milestones.

What Do Drug Developers Need From CRO Partners?

For biotechs whose hematological oncology treatments are reaching the clinical trial stage for the first time, navigating these early phase studies can be a daunting prospect. Partnering with a contract research organization (CRO) that can provide expertise and resources can be critical for the prospects of their treatment, and enable them to progress to later trial phases and commercialization.

For respondents, the key influencers when choosing to partner with a CRO for hematological oncology early phase trials were therapeutic area expertise (11%), responsiveness (9%), and patient recruitment strategy (9%) (see Figure 5). Given the complex nature of hematological oncology treatments and precise delivery requirements, it is unsurprising that being an expert in this area is highly attractive to biotech companies. Responsiveness is also valued by these sponsors, as once treatments reach clinical trial stages there is a great amount of pressure and funding potential contingent on the success of the study.

Figure 5: Key Influences When Deciding A CRO Partnership



Question: What are the key influences for your organization when deciding to partner with a CRO? (Please select the top 5 benefits)
Base: All respondents: Pharma & Biotech companies: annual R&D $999M or lower n=315); multiple answers permitted.

Working with a CRO that specializes in working with biotech companies and can offer both the therapeutic expertise and project management they need is a significant advantage. Allucent is specially equipped to support innovators in the hematological oncology space, which is particularly important given the nuances of each malignancy. The company also has proven experience in meeting patient enrollment targets early,[5] which was cited as a key influencer for respondents when choosing a CRO partner.

When respondents were asked about their preference in terms of CRO size, these same concerns were largely reflected. Mid-sized CROs were seen as the most popular partner (36%), with top reasons for this including their flexibility, expertise, patient recruitment capabilities, and responsiveness. However, there were also challenges when deciding to outsource trial operations. The top three concerns were quality (15%), followed closely by time delays (14%) and cost (13%). By the time a drug reaches Phase I-II trials, significant capital has already been invested into its development. Sponsors therefore place great importance on executing studies to the highest quality standard, for the most robust data, as quickly as possible. This enables them to move closer to securing regulatory approval and commercialization.

Meeting The Potential Of The Hematological Oncology Pipeline

There is great promise within the hematological oncology pipeline, with innovative new treatment options progressing through drug development and reaching early phase trials. These studies are often complex to implement, making it critically important for biotech companies to surround themselves with expertise and acquire the necessary resources to complete successful studies. Partnering with specialist CROs in the space is a key solution to gain a competitive advantage, and ensure treatments can reach patients as quickly as possible.

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About Allucent

Allucent is on a mission to help bring new therapies to light by solving the distinct challenges of small and mid-sized biotech companies. The company is purpose-built through the convergence of leading providers to address this unmet need. Allucent is a global provider of comprehensive drug development solutions, including consulting, clinical operations, biometrics and clinical pharmacology across a variety of therapeutic areas. With more than 30 years of experience in over 60 countries, Allucent's individualized partnership approach provides experience-driven insights and expertise to assist its clients in successfully navigating the complexities of delivering novel treatments to patients.