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2024 INDUSTRY TRENDS:

CLINICAL TRIALS

The pharmaceutical industry continues to feel the effects of disruption from the COVID-19 pandemic. Lessons learned in drug development during this period have provoked a faster adoption of new techniques within clinical trials. Sponsors and their vendors are embracing clinical trials' digital transformation and orienting their focus on the patient and FDA regulation to guide these efforts. DE&I considerations lay at the core of patient recruitment while real-world data is enabling new avenues to reach patients during studies. Further, outsourcing continues to be a strong capital allocation for sponsors; these same sponsors are feeling the effects of the recently passed Inflation Reduction Act, hitting at the margins of new drugs. New technologies like artificial intelligence provide enticing possibilities for data analysis and patient identification.

In this report, we dive into these key trends and their impact on the clinical trials industry for 2024.

DIGITAL TOOLS EMPOWER INDUSTRY TRANSFORMATION

DE&I REMAINS AT THE CORE OF CLINICAL TRANSFORMATION EFFORTS

REAL-WORLD DATA MANAGEMENT IS INTEGRAL TO FUTURE CLINICAL TRIALS

OUTSOURCING CLINICAL RESEARCH CONTINUES AT A FAST CLIP

ARTIFICIAL INTELLIGENCE COULD INCREASE CLINICAL TRIAL EFFECTIVENESS AND EFFICIENCY

SPONSORS FEEL THE PRESSURE OF THE INFLATION REDUCTION ACT

1 DIGITAL TOOLS EMPOWER INDUSTRY TRANSFORMATION

The COVID-19 pandemic had transformative effects across the entire healthcare industry. This disruption has driven transformation within the clinical trial industry, in particular, where typical patient research options (i.e. on-site testing) have been supplemented by home trials and studies away from the traditional clinical test site. Patients who may not be close to a testing site, or ones who – for social, economic, or health reasons - can't participate in on-site trials, have benefited from more flexible options.

Digital tools play a large part in this industry transformation. Digital health technologies (DHTs) establish a closer relationship with the patient during trials, as their symptoms can be more closely monitored. The patient's data is recorded concurrently while they participate in a clinical trial; the patient can also provide primary data of their symptoms with their mobile phone. Digital tools also enable sponsors to conduct decentralized clinical trials away from the clinical site.

At the core of clinical trial transformation is a focus on the patient and FDA regulations. Sponsors and vendors are departing from a purely operational focus on clinical trials in favor of a focus on patient safety and risk and quality approaches. As an example of this patient focus, sponsors continuously seek high participation on enrollment schedules. This is particularly tricky when the patients required for the trial are a subpopulation with a rare disease or condition. What's more, some clinical trials can only take place in one trial (*as is the case of cell therapy drug development*). Any delay in clinical development from patient identification could set back schedules and increase operating costs.

Sponsors can increase participation in clinical trials and reduce barriers for underrepresented groups yet technological hurdles do still exist. Patients must conform to the usage of digital health technologies, and companies must also consider the implementation costs and challenges to implement new research techniques. The FDA is giving more and more guidance to handle digital health technology that will aid this transformation.

Pre-COVID, 28% of sponsors and CROs ran decentralized clinical trials. Post-COVID (2023), 87% currently use decentralized clinical trials or plan to use them within 12 months. – SOURCE

2 DE&I REMAINS AT THE CORE OF CLINICAL TRANSFORMATION EFFORTS

Clinical trial sponsors face patient recruitment challenges in underrepresented groups. A lack of diversity in clinical trials threatens data and output bias. Researchers may misrepresent the intended target population of a drug product if its patient recruitment doesn't meet expectations.

Underrepresentation within clinical trials can stem from a variety of factors: the targeted community may already have established distrust in the clinical trial process due to poor treatment in the past. There could be a lack of reliable information being transmitted/marketed to the target population, or strategies to engage with these groups could be non-existent.

Regulation is continuously being added to help improve diversity, equity, & inclusion in clinical trials. In December 2022, the United States Congress signed a bill (*Food & Drug Omnibus Reform Act*) with the provision that research sponsors will have to submit Diversity Action Plans (DAPs) during studies to meet certain enrollment goals. Further, the FDA has also recently introduced guidance that companies can continue studies of under-represented groups post-Phase III if the therapy is deemed unique and needed.

Sponsors are integrating more 'frontier' sites into their network of clinical research sites to mitigate a lack of diversity in clinical trials. Frontier sites include local doctors' offices, community clinics, and pharmacies; employees at these sites collect and send basic patient data like side effects to the coordinating site of a clinical trial (usually an academic medical center). Frontier sites can exist in almost any community, so patients don't have to travel as far to participate in a clinical trial. Sponsors benefit from this 'hybrid' clinical trial approach as they can widen their geographical outreach to patients without expending capital for more costly clinical researchers.

Overall, more capital and different approaches should be devoted to DE&I efforts to adapt. A transformative approach to increase DE&I in clinical trials is needed, like leveraging culturally relevant marketing, building better patient relationships, and educating communities. Sponsors can also avoid a traditional paper enrollment process, as this process can be transferred to a mobile phone, where everything is done in real time. Firms can also leverage various digital marketing techniques like social media advertising, search engine optimization (SEO), content marketing, and email to help increase their outreach to different patients.

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3 REAL-WORLD DATA MANAGEMENT IS INTEGRAL TO FUTURE CLINICAL TRIALS

Real-world data (RWD) is data that is collected outside of a typical clinical study setting (i.e., at the clinical site). RWD feeds into real-world evidence (RWE), which drives clinical research. The proliferation of digital tools in clinical trials like smart-phones, telehealth applications, and digital health technologies allows researchers to collect more primary data from patients. Patients can directly report their own symptoms or primary data to a doctor or their smart phone; this data could also be tracked on a constant basis with wearables – a type of digital health technology that transmits patient data directly to clinical trial researchers. The device is placed on the patient and the sponsor collects the real-world data that is produced.

RWD has several use cases within medical research and drug development: RWD can help select the right site for eligible patients and plan for statistical considerations, such as sample size and variability of patients. RWD can help identify populations with unique risk profiles and increase the number of adverse events that are caught during a clinical trial. RWD can also help develop new hypotheses about diseases and an understanding for real patient demographics. When a RWD platform is created, it's intertwined with the trial design. This can take different forms along the timeline of the clinical trial. When utilized in the pre-approval phase, RWD can help accelerate study timelines and shorten time to market. Within the post-approval phase, RWD can help demonstrate safety and efficacy and increase the chance of commercial success.

The FDA wants to safely accelerate the trend of real-world data and evidence in clinical research. The agency recently founded the "Advancing Real-World Evidence Program," where sponsors who are selected into the program can meet with FDA staff – "before product development or study initiation" -- to discuss RWE in medical product development. The FDA has also released new guidance regarding RWD (ICH-E6) that discusses clinical trial design and provides guidance on what companies should consider when beginning their own RWD platform.

RWD could help bridge the gap for DE&I gaps for underrepresented groups. RWE collected during decentralized clinical trials can reach groups who may not want to follow a typical randomized clinical trial. It can also help to bolster the quality and amount of primary data collected from patients, enabling sponsors and CROs to make informed clinical decisions and analyze more data to contribute to efficacy studies. Sponsors should expect challenges when setting up their RWD platform for clinical trials, however; the amount of data that companies must process will exceed the normal amount of data for clinical trials (e.g., observational studies), so companies will have to establish strategies to handle this influx. When setting up a RWD platform, companies should ensure that they consider the different indications and sub-industries they will deal with, as this could change the overall RWD strategy.

4 OUTSOURCING CLINICAL RESEARCH CONTINUES AT A FAST CLIP

Outsourcing clinical trials research to a contract research organization (CRO) can help a sponsor company reduce operational expenses. CROs provide drug discovery, pre-clinical, clinical, and post-approval research services to pharmaceutical sponsors. CROs can help a sponsor get a drug quicker to market and can reduce the need to hire specialized profiles for clinical research. Sponsors can also outsource manufacturing of their drug pipeline materials to contract manufacturing organizations (CMOs), which can help a firm reduce its labor cost and speed up the drug research timeline during clinical trials.

CMOs and CROs exist across different subindustries or indications. Sponsors must balance the specific needs of a drug's indication and subindustry with the CRO or CMO they will contract. This becomes especially important when identifying patient channels for rare diseases/small target populations.

In order to find the right contractor for a clinical trial, sponsors should prepare a proper vendor management strategy. Sponsors should establish a cross-functional team to break down internal and external collaboration barriers. Considering the nuance of different subindustries and indications within clinical research, a tier-based approach to vendor management should consider the risks and impact of processes between sponsor and contractor. Finally, clearly defining expectations and roles between the sponsor company and the vendor can best help mitigate any changes in processes, materials, or systems that could take place.

Data analytics also comes with additional guidelines and regulations. In addition to HIPPA, the World Medical Association (WMA) has a declaration based on ethical considerations regarding health databases and biobanks called the Declaration of Taipei. For any data being used, it's vital to ensure privacy and autonomy, especially with patient data. Companies need to be transparent about their data policies with patients, particularly when it comes to dignity, confidentiality, and discrimination. Abiding by the ethical principles and governance arrangements explained in the Declaration of Taipei will help labs feel secure in their practices with data.

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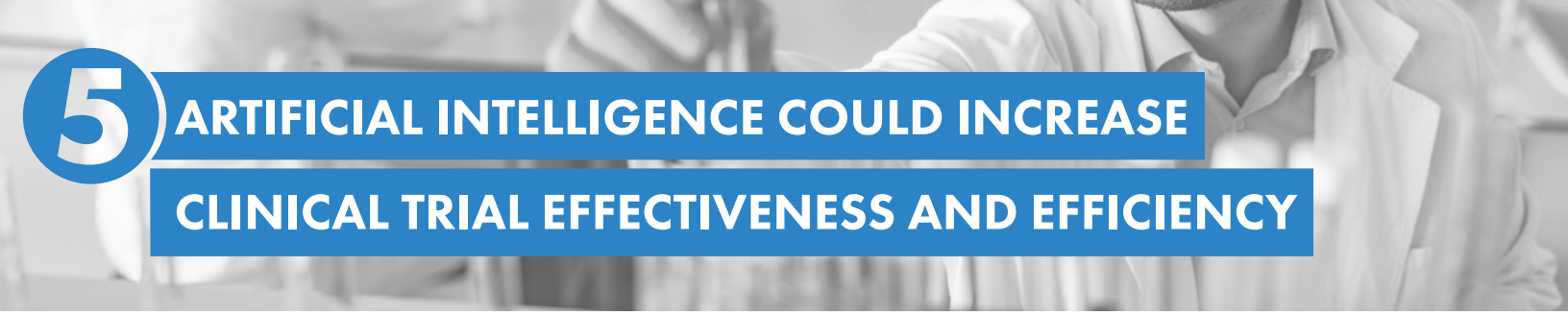


5 ARTIFICIAL INTELLIGENCE COULD INCREASE CLINICAL TRIAL EFFECTIVENESS AND EFFICIENCY

Due to the proliferation of digital health devices and collection of real-world data, the amount of structured and unstructured data has increased for clinical researchers. Artificial intelligence (AI) applications in clinical research can help analyze large amounts of data and help decrease the timeline of clinical research. With such large repositories of data, clinical researchers should take advantage of larger data sets connected to patients.

AI and machine learning applications can also aid in the identification of patient sites for clinical testing based on historical data. In trial design optimization, AI can help predict risks that may occur along the research lifecycle. Also, this technology could help predict which patients are more likely to drop out of a clinical trial, opening the possibility to reduce churn.

As an extension of artificial intelligence, digital twins can be useful for clinical trials. Digital twins can help bolster the process of estimating a patient's outcome in comparison to the patient's observed outcome. With the proliferation of wearables and mobile devices that provide RWD for clinical trials, more data is collected from patients which could be further analyzed within digital twins.

Artificial intelligence also poses challenges for sponsors in the areas of safety and data privacy. The cost to implement and maintain these applications are higher than normal data analysis for a company. This is why companies should have a proper plan and vision when implementing AI systems, such as employing the right talent who can deal with these technologies, avoiding poorly structured data, employing a proper AI/ML governance strategy, and enacting robust change management.

The investment of AI in drug discovery could amount to a $50 billion market in the next 10 years. – SOURCE

6 SPONSORS FEEL THE PRESSURE OF THE INFLATION REDUCTION ACT

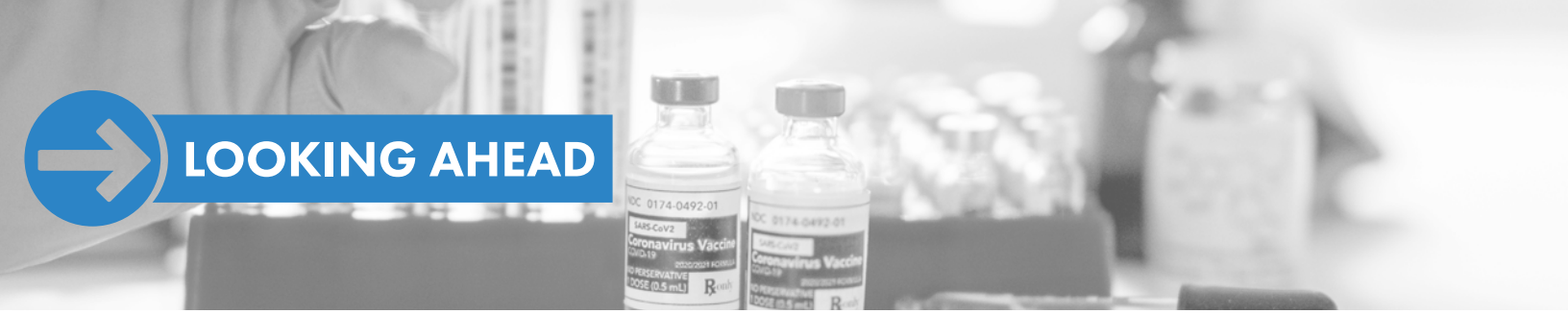
In 2022, the Inflation Reduction Act was signed into law. This legislation has caused sponsors to reevaluate how they approach their clinical development process. Specifically, the law reduces the amount of time it takes for a drug product to be included in the Medicare drug-pricing negotiations program. A quicker readjustment of price means margins for new products could be reduced on a quicker timeline; the risk-based strategy to asset selection in clinical development is now put under tighter scrutiny.

Pharmaceutical executives from prominent firms like Lily or Pfizer have spoken out publicly against this policy. Further, companies like Merck and Bristol Myers Squibb have filed lawsuits against the Department of Health and Human Services concerning the IRA in 2023. Other industry peers have supported the legislative efforts of the two companies.

Sponsors could have to decide which assets they would like to develop, and which to put on hold. This was the case for Novartis, as the company dropped some of the cancer drugs in its pipeline because of Medicare drug-price negotiations. As cost pressures increase, downstream effects of this policy could even be felt in sponsors' R&D vendor strategy. The response to the IRA is still evolving, and companies must now grapple with a more nuanced approach to clinical asset selection.




The Congressional Budget Office estimates that provisions related to prescription drugs in the Inflation Reduction Act could reduce pharmaceutical revenues by $237 billion from 2022 to 2031. – SOURCE



Sponsors and vendors are following a more patient-centric approach to work in conjunction with new FDA guidance. Digital transformation caused by the COVID-19 pandemic has continued at a high pace, with many factors to consider in the digital tools space. Diversity, equity, and inclusion remain a top priority within patient recruitment, while real-world data could help increase patient engagement and even help in the recruitment of underrepresented patient populations. Sponsors continue to outsource their manufacturing and research processes to vendors, but must now deal with the effects of the Inflation Reduction Act, whose concrete consequences remain to be realized. Clinical development, like many other industries, seeks to embrace artificial intelligence technologies. We anticipate these trends will continue to impact the clinical trial space as we move into 2024 and beyond.

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