

The clinical trial process is widely acknowledged as slow, inefficient, and expensive. More intelligent clinical trial protocol designs can effectively leverage new ways that trials can engage patients and successfully address trial challenges.

The Value of Intelligence in Clinical Protocol Designs

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Questions posed by: IBM Watson Health

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Q. What are the biggest challenges in life science R&D today?

A. Most of the major medical needs of the world are unmet today and extremely difficult problems to solve, with limited understanding of the disease mechanism of action and no clear paths forward. That said, the life science industry has unprecedented access to new data, with the potential to provide new patient and population-level insights into disease.

At the same time, scientific discovery and technological innovation are increasingly delivering more effective treatments and disease cures to patients, with next-generation therapeutics recognizing patient-specific metrics with direct relationships to both patient outcomes and drug safety. These patient-specific metrics, however, add their own challenges, making clinical trials significantly more complex and eligible patients even harder to find and recruit for trial participation.

Q. What are the key barriers to progress in clinical development?

A. Both companies and regulators acknowledge that the clinical trial process is slow, inefficient, and expensive. Several innovative new approaches to clinical development have been proposed, including adaptive, virtual, and multi-drug trial designs, each with its own specific advantages and limitations. Complicating efforts to improve trial conduct are concurrent new trial requirements that are adding more complexity to trials, including new biomarker-based patient segmentation, complicated trial protocols, and growing needs to amend trial protocols during trial conduct based on insights learned since trial designs were finalized.

Patient recruitment remains an increasingly intransigent barrier to bringing new drugs to market. In the drug industry overall, 85% of all clinical trials fail to gather enough patients to advance trial efforts. With most near-term industry efforts focused on targeted therapeutics, especially biomarker-segmented cancers, the available pool of patients eligible to participate in a clinical trial grows smaller, with every new patient-specific biomarker decreasing the pool of eligible patients.

Q. What is the role of data, information, and knowledge in clinical trial protocol development?

A. In designing a clinical trial, there is a wealth of available data, both within and outside of the organization, that could be leveraged to improve trial performance and increase the potential for trial success. Prior related trial experience provides direct insights on recognized pitfalls to be avoided, including sites with zero patients, sites with problematic data collection, and overly enthusiastic trial timelines. Completed trials also provide insights on effectively implementing complex trial designs and approaches that are more likely to achieve successful outcomes.

As a relatively new and increasingly important data resource, real-world data (RWD) is providing new and important insights to support better trial study design. From an operational perspective, RWD can improve patient recruitment by identifying where trial-eligible patients reside and how hard (or easy) it will be to access these patients. From a medical perspective, RWD can provide early insights on which specific patients would be best for trials, based on patient-specific medical data that could provide early and better insights into the eligible patient population (e.g., meet core trial inclusion/exclusion criteria). From a population perspective, RWD can be leveraged to correlate patient co-morbidities that track better with target disease progression, providing valuable insights beforehand on how a new therapeutic treatment will perform in the real world.

Q. How might clinical trial protocols evolve and change in the future?

A. We expect clinical trial protocols to become much more customized to the specific needs, requirements, and goals of the specific trial in the not too distant future. Many of these innovations will be directly enabled by more effective use of available real-world data, information, and knowledge. Infusion of data into the process will be enabled by more intelligent analytics that will become commonplace, integrated, and core to study design. Beyond legacy and patient-specific data used in developing trial protocols, intelligent analytics will become integral in protocol execution, enabling better and more real-time trial insights from inception to database lock.

As virtual, remote, and other innovative trial approaches become reality, clinical trial protocols will need to adapt to more effectively leverage the new ways that trials can and will engage patients. As an important innovation that should increase potential trial participant pools and potentially increase patient satisfaction with participation, these new trial approaches will be key to overcoming intransigent trial recruitment concerns. Whether improving patient outreach through the use of social media or patient advocacy channels, leveraging Internet of Things (IoT) sensors and other remote data collection technologies to gather key data insights, or embedding intelligent

analytics to more rapidly detect potential adverse drug events or identify success milestones in the trial, next-generation clinical trial protocols will deliver significant improvements over existing industry best practices.

Q. What benefits can organizations expect to see from the implementation of more intelligent clinical trial protocol designs?

A. Clinical trial protocols are the operating script for the execution of clinical trials. Historically, these protocols have been built on tried-and-true best practices, relying on experienced investigators and clinical trial managers to avoid pitfalls and barriers. The growing availability of clinical trial operational and patient-specific data, supporting analytics, and intelligent tools is enabling the development of better, more intelligent clinical trial protocols. Intelligent trial protocols are needed as trials become increasingly complex and appropriate and eligible patients for trial participation become harder to find. Intelligent trial protocols offer investigators significant potential benefits, including:

- » The ability to more easily leverage prior trial experience and best practices, which allows investigators to benefit from insights from experienced investigators
- » Better and more systematic processes and procedures to more efficiently and effectively identify and recruit patients for trials, including accommodation for more adaptive protocols (e.g., virtual and adaptive trials) where existing experience and insights are less available
 - Intelligent protocols can also leverage real-world data to gain insight into the effect of inclusion/exclusion criteria on the eligible patient population and more efficiently identify patient-rich geographies. In addition, these protocols can leverage patient-specific data to better identify the right patient for the trial.
- » Trial protocol designs that require fewer protocol amendments and mid-study updates based on pre-execution trial insights foretelling potential future trial issues, resulting in reduced trial delays and better cost management compared with traditional protocols
- » Minimizing patient dropout throughout trial execution by using intelligent analytics to actively determine whether a patient is likely to drop out, enabling trial managers to act before the patient leaves
- » Increased trial transparency, including the analytics supporting the ability to dynamically identify and monitor:
 - Success milestone metrics
 - Key adaptive trial metrics
 - Early indicators of potential adverse drug events

About the Analyst



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Alan is responsible for directing the IDC Health Insights Life Sciences practice. Dr. Louie manages a group of analysts who provide research-based advisory and consulting services for life sciences companies, technology vendors, and IT service providers across the life sciences value chain. Within the practice, Dr. Louie leads IDC Health Insights' Life Science R&D Strategy and Technology research service with coverage of ongoing innovation and best practices in life science R&D and a further emphasis on technology and innovation in clinical development, analytics, translational research, and precision medicine.

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About IBM Watson Health

Decision making during clinical trial study design can often feel like high impact prediction. Fortunately, data to inform the trial design process exists – clinical researchers just need easy access to the right information in order to add more patient data and evidence-based insights to the study design process.

Learn how you can make data-driven study design decisions at <https://www.ibm.com/watson-health/products>

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