The Future of Clinical Trials: 2025 Outlook

Signant Health

As we look ahead to 2025, clinical trials will continue to evolve in response to technological innovation, regulatory changes, and emerging therapeutic needs. Let's explore some trends that are poised to shape the industry, from breakthrough therapies to sustainable trial designs.

Therapeutic Developments

Advancements in GLP-1 Receptor Agonists

GLP-1 receptor agonists (GLP-1 RAs) have proven to outperform traditional antidiabetic medications in glycemic management and weight reduction. They also enhance patient outcomes, particularly for those with type 2 diabetes mellitus (T2DM) and cardiovascular disease, by reducing severe adverse cardiovascular complications. New and ongoing trials investigating GLP-1 RAs will focus on enhanced efficacy, novel formulations, and expanded therapeutic applications.

GLP-1 RA Trial Solutions | Signant Health

The Evolution of Patient-Reported Outcomes (PROs) in Early-Phase Oncology Trials

In 2025, the inclusion of patient-reported outcomes (PROs) in early-phase oncology trials is expected to become increasingly emphasized, driven by the need for a comprehensive understanding of tolerability, in addition to early efficacy profiles.

Regulatory guidance and sponsor priorities are converging to incorporate PROs into early-phase oncology trial designs, particularly as they offer critical insights into symptomatic adverse events and patient tolerability, which are vital for optimal dose determination and safety evaluation. While currently not mandated, regulatory bodies may increasingly expect sponsors to integrate PROs into early-phase trials to build comprehensive safety and tolerability profiles. A good example of this is FDA's Project Optimus initiative. As Phase I trials evolve to include more personalized and targeted therapies, PRO data will likely become integral in shaping precision dosing and optimizing patient experiences.

Learn how Signant can help sponsors and CROs generate high-quality PRO data efficiently in phase I oncology trials.

Early-Phase Oncology Trials | Capture High-Quality Data

Rare Diseases and Gene Therapy

Recent advancements in rare disease research emphasize tailored clinical strategies and evolving regulatory frameworks to address the unique challenges of small patient populations and limited datasets. Regulatory bodies like the EMA and FDA have increasingly endorsed adaptive trial designs and patient-centered endpoints, enabling faster access to innovative therapies.

Moving into 2025, these trends are expected to evolve further with a stronger focus on patient-reported outcomes (PROs) and artificial intelligence (AI) applications in trial optimization. AI's capacity for real-time data analysis and predictive modeling may bridge gaps in patient recruitment and trial feasibility for rare conditions.

As gene therapy technologies mature, innovations such as genome editing and AI-driven patient identification could further transform clinical development in rare diseases. Regulatory frameworks are likely to evolve alongside these advancements by mandating post-marketing surveillance to assess long-term safety and efficacy while balancing the need to expedite approvals for treatments.

Signant's comprehensive rater training programs and AI-supported data analytics solutions can help sponsors navigate these complexities by ensuring consistent endpoint assessment across sites, optimizing data quality, and leveraging predictive analytics to enhance site selection, trial efficiency, and success rates.

For supplemental reading about optimizing endpoint quality in rare disease trials, visit this blog:

5 Tips for Success in Rare Disease Studies - Signant Health

Adolescent Schizophrenia

Adolescent schizophrenia trials face significant challenges, particularly due to the lack of validated assessment tools tailored to this demographic. Trials often rely on adult-oriented scales, like the Positive and Negative Syndrome Scale (PANSS), which may not adequately capture developmental nuances or symptoms described by younger patients and caregivers. efficient data collection.

Recent efforts such as developing an abbreviated PANSS for adolescent use, led by Signant's clinical vice president and renowned pediatric psychiatry expert Joan Busner, aim to improve treatment sensitivity and reliability while addressing these limitations. However, these tools remain underutilized, leading to potential inconsistencies in evaluating efficacy and safety outcomes in adolescent populations.

Moving forward, regulators and researchers must prioritize the development and validation of adolescent-specific scales, integrating input from patients, caregivers, and clinical experts to ensure accurate representation of treatment impacts in this unique population.

Explore Signant's extensive research posters on the development of an optimized PANSS for adolescent use.

Vaccines and Disease Surveillance

The landscape of vaccine development is experiencing significant advancements, emphasizing innovative platforms like mRNA, nanovaccines, and adaptive clinical trial designs. Disease surveillance has become central to vaccine development, with enhanced pharmacovigilance plans incorporating real-time monitoring and comprehensive risk assessments across diverse populations. Regulatory frameworks have evolved to accommodate these innovations, with harmonized international guidelines focusing on immunogenicity, safety, and efficacy.

Looking ahead to 2025, the emphasis is likely to shift further towards global harmonization of vaccine regulatory standards, with a focus on rapid scalability during public health crises. Regulatory agencies are expected to strengthen frameworks for real-world data integration to support long-term safety and efficacy monitoring. The growing use of AI and machine learning for predictive analytics in vaccine design and trial optimization could further revolutionize development timelines and regulatory evaluations.

Signant is prepared to support these advancements with comprehensive solutions, global operational resources, and infectious diseases expertise.

Transforming Vaccine Trials | Signant Health

Cognitive Testing

Sleep and Cognition

The relationship between sleep quality and cognitive performance will become increasingly important in clinical trials, particularly for neurodegenerative and mental

health conditions. By combining sleep data with cognitive assessments, researchers can detect subtle cognitive changes earlier and gain clearer insights into treatment efficacy. The Signant SmartSignals® Cognitive Drug Research (CDR) System, a leader in computerized cognitive testing, will play a pivotal role in these advancements. Its ability to detect subtle changes early and provide millisecond-accurate data makes it indispensable for capturing the nuanced effects of poor sleep on cognitive health.

Disease Applications

Cognitive testing with Signant's CDR System will continue to be crucial in neurodegenerative disease trials, including Alzheimer's, Parkinson's, and multiple sclerosis. Its sensitivity in detecting early cognitive changes, monitoring disease progression, and measuring treatment effects will make it indispensable for these trials.

Beyond central nervous system disorders, cognitive assessments will play vital roles in various therapeutic areas:

- Epilepsy studies will focus on cognitive impacts of seizures and medications
- Migraine research will examine cognitive function during and after episodes
- Phase I trials will incorporate cognitive testing for neurological safety monitoring
- Heart failure studies will track cognitive function as a key comorbidity measure

In these ways, the proliferation in adoption of cognitive testing will support more accurate and patient-centered clinical data.

Data, Technology, and AI

Decentralized Elements

As last year, the industry will continue to thoughtfully adopt decentralized elements with the focus on simplification of study participation for patients and sites. This will continue the drive towards optimized trials, as opposed to fully decentralized trials – trials that use technology components in the way that best fits the patient population, the study sites, and the protocol. Sponsors will continue to demand proven solutions that can operate at scale, supported by robust and mature operational processes.

Leveraging PRO data beyond the label

While PRO data are often used to support regulatory approval decision making and labeling, they will continue to have increasing importance beyond the label. HTA assessment, including pricing and reimbursement activity across the globe, will

increasingly account for patient experience data alongside conventional economic analyses. Further, these data will be important in pharmaceutical sponsors' Inflation Reduction Act activities.

Artificial intelligence and machine learning

AI will continue to amaze and excite in 2025. Improvements in speed and quality will continue to be driven through incorporation of AI/ML based activities in clinical trial processes – from eClinical solution design, to data cleaning. AI will play a pivotal role in improving endpoint reliability by reducing variability, identifying trends in real-time, and ensuring consistent data quality. Machine learning and big data analytics will enable the development of novel digital biomarkers and sensor-based functional outcomes based on rich streams of sensor data which may provide more sensitive measures of treatment impact.

Diversity and Sustainability in Clinical Trials

Diversity in Clinical Trials

Ensuring representative trial populations, and responding to FDA requirements for diversity action plans, will remain a top priority. Overcoming these challenges will benefit both patients and sponsors, and drive the approval of new treatments that are fully generalizable across patient groups.

Explore our white paper which examines underrepresentation in clinical research.

Diversity in Clinical Trials | Signant Health

Sustainability Initiatives

From reducing waste to optimizing trial logistics, sustainability will gain further momentum in the industry.

Conclusion

The clinical trial landscape for 2025 promises innovation and adaptation. To stay ahead, the industry must leverage technology, focus on patient needs, and commit to sustainable practices.