AI in Clinical Trials

by Flora Shimi and Anthony Nagle

The **Primary Intelligence** Company

Artificial Intelligence in Clinical Trials

Clinical trials are the gatekeeper between laboratory research and clinical practice, and the use of AI in clinical trials may change the face of medicine

Artificial Intelligence (AI) has experienced rapid growth and adoption across various industries, including healthcare. The significance of AI is only just becoming apparent, and its impact on clinical trials will be no exception. Clinical trials, being the vital bridge between scientific discoveries in the lab and real-world clinical practice, play a critical role in determining the safety and efficacy of new treatments and medical interventions. AI is already being used to accelerate this process.

"**The healthcare system should be integrated into an intuitive real world evidence generation system**, with clinical research and clinical care going hand in hand … methodological advances and future AI-based analyses of all data will provide deep evidence to realize the goal of personalized medicine"

Vivek Subbhiah: The next generation of evidence-based medicine, Nature Medicine, 2023

The landscape of clinical research is complex and challenging. Patients need to be recruited with minimal dropouts, data needs to be collected and verified, every process needs to be managed and the ethics assessed, sites across the world need to adhere to the protocol, and overall this leads to a significant price tag to drive a medication through the regulatory approval goal posts.

AI is already being used to accelerate these processes using data analysis and automation. Further, as adoption of AI in clinical research becomes mainstream, it also presents opportunities for entirely new models of clinical research. However, the integration of AI-driven technologies raises important questions about data privacy, algorithmic fairness, and the potential for unintended consequences.

This White Paper aims to provide an overview of the current and emerging applications of AI in clinical trials, while also addressing the challenges and opportunities that lie ahead as this transformative technology continues to evolve. Approaching competitive intelligence analysis with an AI lens will play an increasingly important role in being able to accurately forecast timelines for new medicines' development and regulatory approval.

From Folklore to Evidence Based Medicine

The Birth of Modern Clinical Trials: James Lind and the Legacy of 'Limeys'

James Lind, a Scottish physician, is often credited with conducting the first clinical trial in history, which took place in 1747 aboard the HMS Salisbury. Lind sought to identify a treatment for scurvy, a debilitating disease commonly experienced by sailors due to vitamin C deficiency. He conducted an experiment with 12 sailors, dividing them into six groups and providing each group with a different potential treatment. The group that received citrus fruits, specifically lemons and oranges, showed significant improvement compared to the others. This discovery not only led to the nickname 'Limeys' for British sailors, who were later provided with a daily dose of grog as a preventative measure, but also laid the foundation for the modern clinical trial process.

Since Lind's pioneering work, the field of clinical trials has advanced significantly, leading to widespread implementation of the **Con**solidated **S**tandards **o**f **R**eporting **T**rials (CONSORT) and **S**tandard **P**rotocol **I**tems: **R**ecommendations for **I**nterventional **T**rials (SPIRIT). CONSORT and SPIRIT are sets of guidelines designed to promote transparency, consistency, and clarity in trial set-up and reporting. In 2020, the guidelines were extended to include AI enabled trials in anticipation of the future use of this technology in medical research, CONSORT-AI and SPIRIT-AI.

The Power of Simplicity: Randomized Controlled Trials

The randomized controlled trial (RCT) is widely regarded as the gold standard in clinical research, and its simplicity is a key factor in its success. By randomly allocating participants to different intervention groups, RCTs effectively minimize the potential for bias, ensuring that any observed effects can be attributed to the treatment under investigation. This straightforward design enables researchers to draw reliable conclusions about the efficacy and safety of new interventions, ultimately leading to more accurate and trustworthy evidence for healthcare providers and patients alike. As the foundation of evidence-based medicine, the simplicity of RCTs remains a cornerstone of clinical research.

Trials and Tribulations: How AI May Help Researchers Overcome Obstacles of RCTs

RCTs are slow, costly, potentially biased, and may have limited real-world application, which can compromise the reliability and applicability of results.

Stuck in the Slow Lane? RCTs Lead to Medical Advancement at a Snail's Pace

- Lengthy processes delay patient access to newer, more effective interventions
- In rapidly evolving medical fields like oncology, infectious diseases, and precision medicine trial results may be out of date by trial readout

Many trial processes can be performed with automation. Incorporating digital tools can speed up trials.

Pricey Progress? The High Cost of RCTs Hampers Medical Research

- The cost of conducting RCTs runs into billions of dollars, which is eventually passed onto payors through higher prices
- High costs also lead to disproportionate focus on high-revenue drugs with little incentive to research the unprofitable

The replicability of digital tools can reduce the costs associated with clinical trials.

Untangling the Truth? Unavoidable Biases in RCTs Reduce the Reliability of Results

- Biases in RCT design, recruitment, sample population, and data analysis can compromise reliability and generalizability of results
- Biases emerge from issues like poor randomization, unrepresentative samples, and lack of blinding

AI tools can be used to audit an RCT trial design to identify human biases

Beyond the Lab? Real-World Data is Crucial to Determine a Durable Response

- RCT findings may not reflect real-world settings due to trial conditions
- Individual characteristics that influence treatment outcomes are not captured in traditional clinical trials
- Limited durability and finite durations

Real-world and long-term follow-up studies can be supported with AI tools to identify patterns in large data sets

While the simplicity of RCTs is a key factor in their success. AI based technologies can help overcome blind spots, streamline processes, enhance decision-making, and boost data analysis in a bid to increase the efficiency and cost-effectiveness of trials. New technologies are being incorporated into the research landscape, and industry and governments are taking note of its transformative potential.

How Regulators are Responding

Regulators see the need to take a proactive approach to AI in clinical trials, they see data-driven medical research as a promising opportunity

United States

in 2019, the FDA published its 'Enrichment Strategies for Clinical Trials' guidelines on patient selection in clinical trials for designs that are more efficient and targeted. Later, in 2021, the FDA laid out its regulatory framework for AI in medicine: 'Framework for AI/ML-Based Software as a Medical Device'.

These documents underpin the FDA's response to AI in clinical trials and demonstrate its focus in using AI systems to enhance medical research. The FDA established a comprehensive approach through its "Total Product Lifecycle (TPLC)" regulatory model for AI systems, which includes best practices, pre-market review, real-world performance evaluation, and a protocol for adapting to algorithmic changes, ensuring the safety and effectiveness of AI-driven clinical trials.

European Union

The EU's AI act is the legal framework for AI applications. The 'European Health Data Space' and the EMA's 'Regulatory Science to 2025' suggest the EMA focus on data sharing. The Clinical Trials Information System (CTIS) provides a centralised platform for data sharing between EU member states.

The European Medicines Agency (EMA) published a five-goal regulatory strategy titled "EMA Regulatory Science to 2025," outlining its approach to evidence generation and evaluation. Key points include fostering innovation in clinical trials, optimizing capabilities in modelling, simulation, and extrapolation, and leveraging digital technology and AI in decision-making processes to enhance the efficiency and effectiveness of regulatory science. In 2023, all EU clinical trials must be registered on the CTIS system, which enables member states to share data on clinical trial progress.

United Kingdom

In April 2023, the Medicine and Healthcare products Regulatory Authority (MHRA) published its guidance 'Software and AI as a Medical Device', and much of the approach mirrored the FDA's 2021 framework with an important omission – the MHRA did not stipulate a protocol for algorithmic changes.

In 2021, the Medicines and Medical Devices Act received royal ascent, moving the UK away from EU civil law to common law approach, and the TIGRR taskforce outlined an approach for the future of clinical trials in the UK to bring research closer to the point of care. This is being achieved through NHSx, a data-spine across health systems and regulatory bodies to leverage the NHS to conduct population-level research. AI is an important tool for this, and the MHRA is taking an evolutive regulatory approach to flexibly accommodate development and deployment.

In the Wild: Symbiotic Relationships

AI-powered clinical trials from science fiction to science future

Over the past 75 years, the pharma industry has experienced significant consolidation in part due to the cost of RCTs. AI has the potential to disrupt not only how RCTs are conducted, but the industry as a whole. An explosion of innovation is anticipated comparable to the 1970's and the dawn of biotechnology 50 years ago.

AI algorithms learn and improve with data and training. For pharma, this means undergoing data transformation now to deploy AI systems in clinical research is likely to yield greater competitive advantage over time. Partnerships with tech are predicted to play a significant role in data transformation in clinical research as tech talent and big data infrastructure can be more easily brought in from external sources than built in-house. Therefore, an organisation's competitive advantage may be assessed by identifying its specific combination of tech-based partnerships in clinical research.

Furthermore, as AI reduces the cost of trials, these partnerships may create a paradigm shift in pharma pipelines in the future. As AI tools reduce the cost of RCTs to a critical point, smaller, research-focused pharma and biotech start-ups can access late stage development for their therapies. This will reduce the reliance on an acquisition pathway by larger pharmaceutical giants and thereby changing the development paradigm.

In 2016, Cedars Sinai chose Deep 6 for its Accelerator Program, using AI to enhance patient recruitment for oncolgy trials

> …I use the tool when I'm assessing feasibility for study design, especially one that's going to be enrolling in my institution. This is important because our local numbers vary so much from the national numbers… *Cedars Sinai*

Cedars Sinai

DEEP 6 AI

MERCK

saama

In August 2020, Saama announced a deal with Merck for Life Science Analytics Cloud (LSAC) for clinical data management

> …By integrating a ML platform across our clinical functions we aim to fuel significant process efficiencies and elevate the user experience for our talented clinical teams…
> *Merck Research Laboratories*

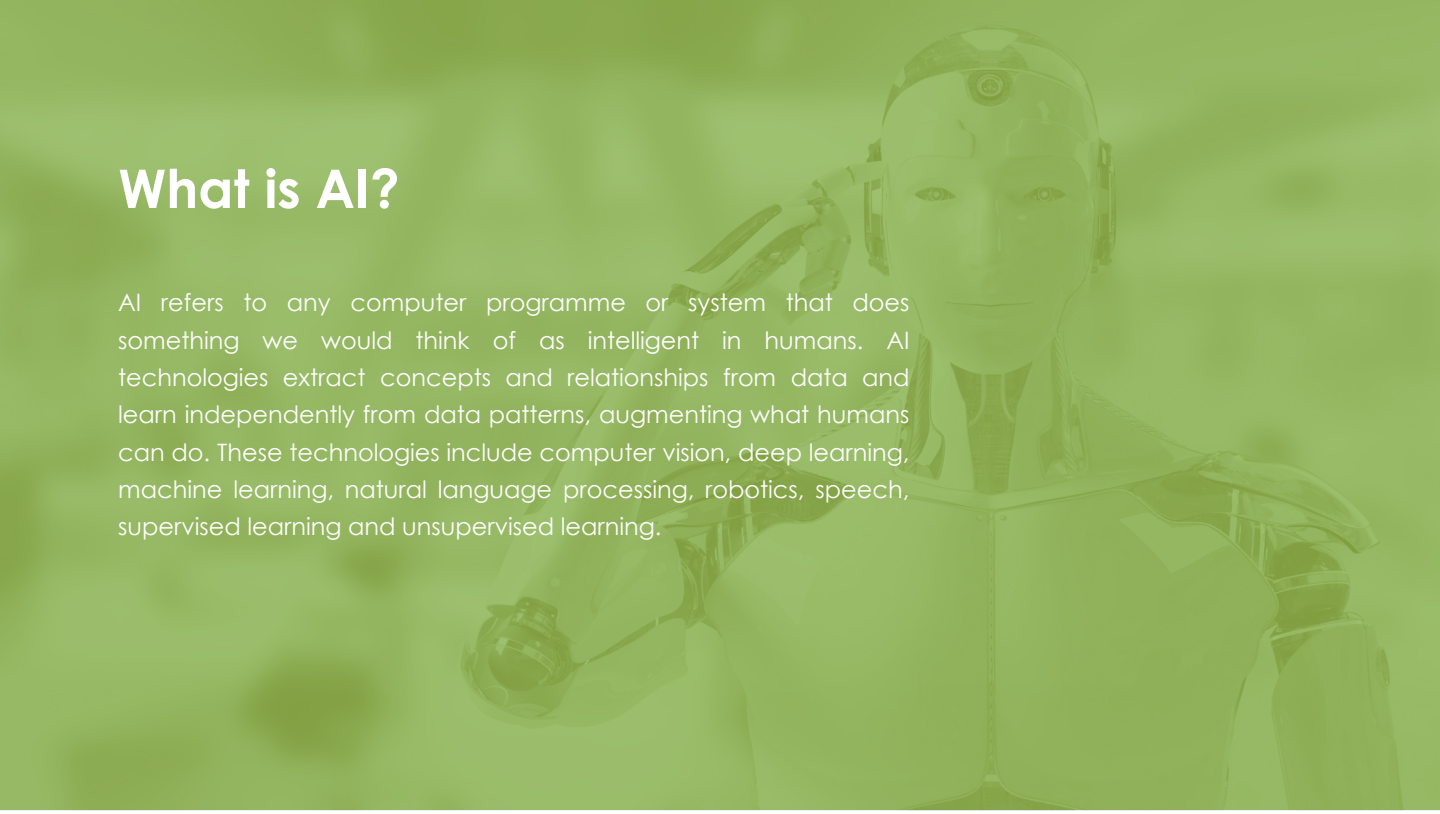
2023, Launch Therapeutics chose Medidata's AI system to power P3 trials for companies focused on early stage research

> …Adding unique insights from our Intelligent Trials solution will support [Launch Tx's] mission to disrupt the late-stage development paradigm…
> *Medidata AI*

LAUNCH THERAPEUTICS

medidata

What is AI?



AI refers to any computer programme or system that does something we would think of as intelligent in humans. AI technologies extract concepts and relationships from data and learn independently from data patterns, augmenting what humans can do. These technologies include computer vision, deep learning, machine learning, natural language processing, robotics, speech, supervised learning and unsupervised learning.

Machine Learning…
…involves the use of statistical models to analyze and identify patterns in data. Effective for tasks that require supervised learning, such as image or speech recognition.

Deep Learning…
…refers to the use of neural networks with multiple layers of interconnected nodes. Effective for more complex tasks than ML such as natural language processing or autonomous driving.

Supervised Learning…
…trains algorithms using labeled data, which can be used for tasks such as predicting patient response to treatment based on previous outcomes.

Unsupervised Learning…
… trains algorithms on unlabeled data, which can identify patient subgroups based on similar characteristics or disease progression patterns.

Natural Language Processing (NLP)…
…involves the use of algorithms to analyse, interpret, and generate human language. Effective for tasks such as data extraction and analysis from electronic health records and patient-reported outcomes.

Speech…
…NLP algorithms can be used to transcribe speech into text, allowing for automated transcription and analysis of speech data. NLP algorithms can also analyze speech data to identify patterns, sentiments, and other insights.

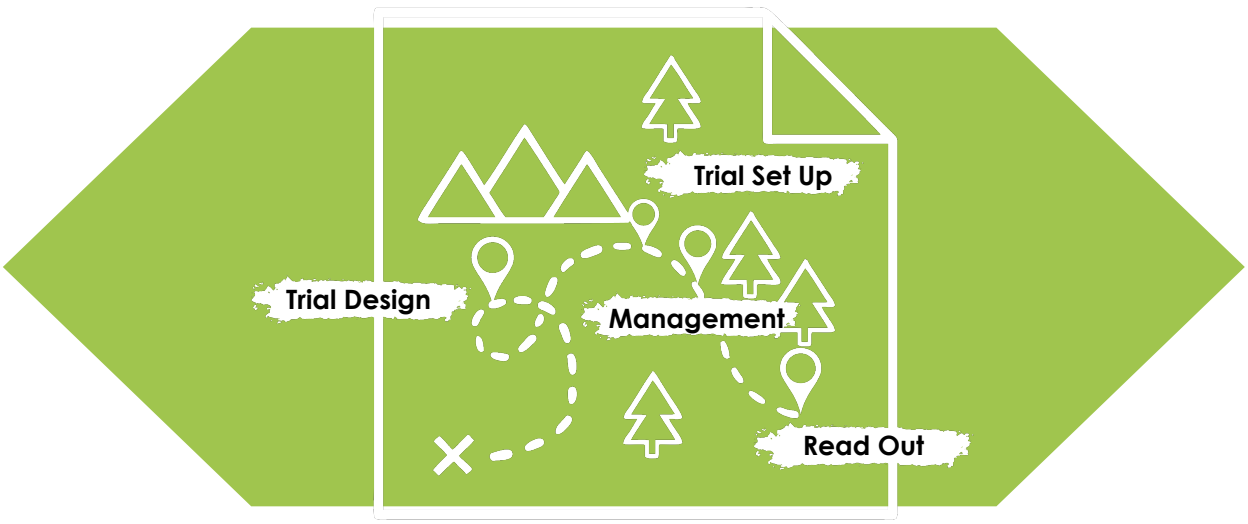
Computer Vision…
… involves the use of machine learning algorithms to enable computers to interpret and analyze visual data, which can be useful in clinical trials for tasks such as image analysis and disease diagnosis.

Robotics…
…is the engineering of machines programmed to perform tasks autonomously or guided. Robots use AI to perceive, interpret and interact with their environment, to make decisions and perform tasks autonomously.

Applications of AI in Clinical Trials

Navigating the Potential of AI Across the Clinical Trial Continuum

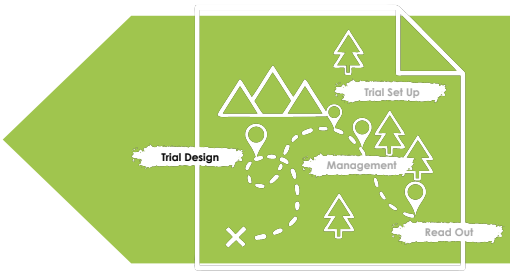
Artificial intelligence (AI) offers opportunities to enhance various aspects of the clinical trial process, from trial design to readout. While AI may help researchers create more efficient, cost-effective, and reliable trials, it is essential to carefully consider the potential risks and ethical implications involved. In this section, we provide an overview of AI's role across the clinical trial continuum, discussing its applications in trial design, setup, management, and readout while addressing the challenges and concerns that must be navigated to ensure responsible implementation.



Current possibilities for improving trial design using AI:

- AI can accelerate clinical trial design by optimizing protocols, streamlining decision-making processes, and flagging potential risks and ethical considerations.
- During trial setup, AI has the potential to enhance site selection, patient recruitment, and the efficiency of data collection.
- Throughout trial management, AI-powered tools may contribute to better monitoring, protocol adherence, and real-time data analysis.
- Finally, AI could streamline trial readout by facilitating data analysis and accelerating the translation of research findings into clinical practice.

This technology doesn't just promise efficiency gains, it enables innovative trial designs such as decentralised trials and population insights from N-of-1 trials that were not previously feasible. Adoption of these methods enables clinical research to be brought into the clinical care setting.



Trial Design

AI is being used in trial design to generate and improve protocols for new trials, and optimise site selection based on predicted performance

Protocol Optimisation

AI tools can fetch, organise, and analyse the body of data generated by clinical trials, including failed ones, and can extract meaningful patterns of information to design an optimal protocol for a trial. By training an ML algorithm with an optimised protocol, it is also able to automate the creation and maintenance of the study database and case report forms (CRFs).

How?

Train an AI model with:

- Existing protocol data and health library information
- Optimised protocols
- Current and past clinical trials, patient support programmes and post-market surveillance

Why?

- Shorter design times
- Greater accuracy
- Fast way to assess how amendments and disruptions would impact the protocol
- Can also create a bespoke study database and CRF

Site Selection

AI models can analyse site parameters like resource availability, clinicians with in-depth experience, enrolment, compliance and administrative procedures, and then make predictions about the potential performance of a site for the trial.

How?

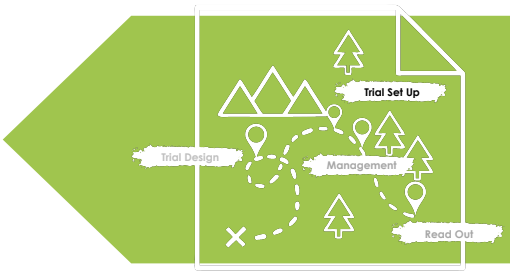
- NLP extracts terms around a specific indication to identify relevant sites and investigators
- Assessment and ranking of sites based on other factors such as recruitment potential and GCP compliance

Why?

- Speed up selection of sites
- Identification of sites with capacity, skills and ability to meet the demands of the trial
- Sites with familiarity with the disease area are more likely to ensure data quality and integrity

AI has the potential to enhance clinical trial design through protocol optimisation and improved site selection, but caution must be taken as:

- Biases in the training data can lead to skewed protocols and trial designs;
- If the AI model is overly complex or too tailored to the training data, the protocol may not apply in other disease areas or for treatments that require a different approach;
- The AI system might introduce errors or inconsistencies in the protocol, causing confusion or misinterpretation among researchers and trial participants.



Trial Set Up

Mining real-time electronic health records (EHR) can accelerate recruitment, and AI-enabled smart contracts can accelerate site-sponsor negotiations

Patient Recruitment
Real-time analysis of electronic health records (EHRs) can identify potential candidates for inclusion in a study and automatically notify their healthcare providers to aid in recruitment. This significantly expands the recruitment pool as patients and healthcare providers that would not usually be aware of appropriate clinical trials are proactively notified and invited.

How?
- Using deep learning and natural language processing to mine and analyse EHRs, medical imaging and 'omics' data
- Identify patients with best fit to inclusion/exclusion criteria listed in the optimised protocol

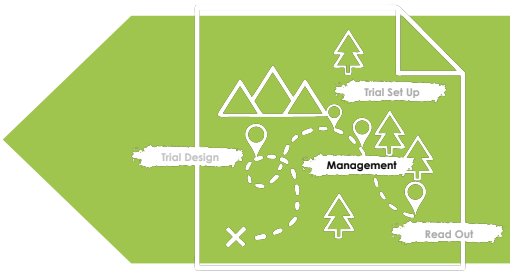
Why?
- Increased speed of recruitment
- Lower screening failures
- More accurate prediction of cost
- Target specific populations to enrich the clinical trial and improve statistical power of the study

Smart Contracts
Site-sponsor negotiations present a complex and critical bottleneck for clinical trial site activation. Smart contracts are computer protocols that facilitate, verify, or enforce a contract. The creation of these contracts can be automated as AI algorithms can be used to create, test, deploy and manage automated contracts. This accelerates site-sponsor negotiations, as well as those for auxiliary services.

How?
- AI can generate, test, and auto-fill site-sponsor contracts
- Self-executing contracts stored on a blockchain provide an immutable record of the agreement and actions taken

Why?
- To speed up the process of contract execution
- To reduce the cost of contract enforcement
- To increase the accuracy of contract execution
- Automate repetitive processes

While real-time EHR analysis and digital platforms for remote trial management can accelerate patient recruitment and negotiations, care must be taken to:
- Ensure the security and privacy of sensitive patient data within EHRs and digital platforms;
- Enable humans to provide necessary input and maintain control over the negotiation process as contracts become increasingly complex and automated.



Trial Management

AI can streamline electronic data capture (EDC), improve source data verification (SDV), and automate query management

Data Capture Automation and Smart SDV

Automation of EDC and digitised clinical assessments enable data to be automatically shared across systems, reducing human error in data entry and validation. For example, AI algorithms in combination with wearable technology can enable continuous patient monitoring and automation of SDV. Real-time analysis provides continuous insights into the safety and effectiveness of treatment while also predicting the risk of dropouts.

How?

- Data captured across channels is fed into EDC case report forms
- EDC data is analysed in real time by models trained on similar trials
- Site staff can upload images and an ML model compares images with EDC data for automated SDV

Why?

- Real time safety assessment
- Draw insights from dropouts to reduce dropout rate in real time
- Improves efficiency and accuracy – computers are superior to humans for repetitive data checking tasks

Queries Management

An ideal query should identify what type of issue is at hand, where the potential data error lies, and prompt the user for action with simple instructions. Many queries are raised in clinical trials, and it takes a lot of time to respond to them. Many of these questions are redundant and time consuming and use valuable HCP resources to respond in adequate tine.

How?

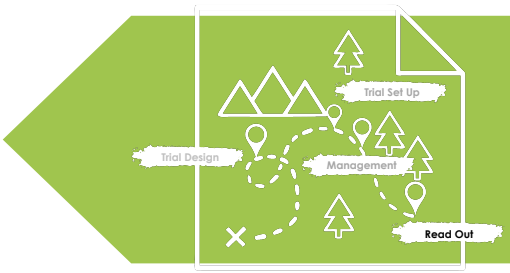
- ML to analyse historical trial data for queries and responses for different field items
- Only errors and outliers are flagged to the data manager
- The data manager's decision is fed back into the ML model

Why?

- Queries from misconfigurations in the EDC can be managed in bulk
- Discrepancy trends in individual sites can flag underperforming sites for targeted inspection
- Continuous improvement as the model learns from human input

Automating EDC systems, digitalizing clinical assessments, and employing AI algorithms with wearable technology can streamline clinical trials and provide real-time insights. However:

- Over-reliance on AI for query management may result in the loss of valuable human expertise, as staff may not have the opportunity to develop their skills in identifying and resolving data issues;
- Integrating AI based data capture and SDV systems into legacy systems may be challenging and may lead to errors, confusion or misinterpretation.



Trial Readout

AI can be used to find covariates to improve a trial's statistical power and medical writers can use AI to autogenerate much of the Clinical Study Report

Covariate Adjustment

Covariate adjustment is a statistical technique to adjust for pre-specified baseline characteristics (covariates) that may influence the outcome of the study. It aims to control for potential confounding factors and improve the precision of the estimated treatment effect, ultimately enhancing the power of the study and the validity of the results.

How?

- AI models work to identify relevant covariates that may impact the primary endpoint
- Statistical models are applied to model the relationship and adjust for identified covariates

Why?

- Increase statistical power without increasing the sample size
- Identify non-responder traits and demonstrate efficacy even when a trial may not have demonstrated significance in wider population

Automation of CSRs

AI-driven automation for generating Clinical Study Reports (CSRs) presents a potential solution for improving efficiency in the documentation process. This method may expedite the review and editing process for medical writers, especially when paired with ML models that clean and analyse the clinical data exported from the EDC systems.

How?

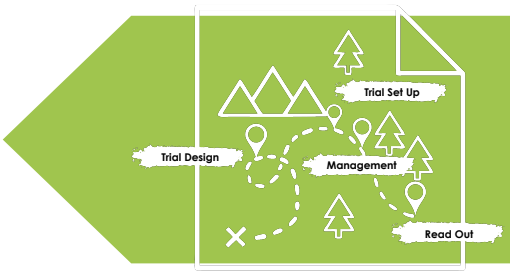
- ML models clean and analyse clinical data exported from EDCs
- Using NLP, AI models can automate CSR writing, drawing upon the protocol and data
- Medical writer time then spent on sections that require thought

Why?

- Reduces the time for a medical writer to produce a CSR report from weeks to days
- NLP can change the language depending on the regional regulatory authority
- Can improve submission quality

AI-driven automation of Clinical Study Reports (CSRs) and covariate adjustment in clinical trials can streamline documentation processes and home in on responsive patient populations. Even so…

- covariate adjustment can sometimes produce complex results that are difficult to interpret. If researchers or regulatory authorities misinterpret these results, they may approve drugs that do not provide meaningful benefits to patients;
- Reliance on AI may reduce the number of adequately trained medical writers, and important mistakes may be overlooked.



Across Trial Continuum

Other tools can be used across the trial continuum from design to readout

Risk Based Monitoring (RBM)

RBM can be employed during clinical trials to reduce risks by identifying potential issues early. Predictors such as Enrolment, Safety Compliance and Data Quality can be used to assess the performance of a site and anticipate any potential risks. Additionally, therapeutic area and other trial variables can be considered when calculating risk.

Clinical Trial Analytics

There is a wealth of information in clinical trials, however insight generation from pooled learning of clinical trials is rare for globally active sponsors. Open data standards can foster collaboration and integration and provide insights across vital metrics. Incorporating a self-learning system, designed to improve predictions and prescriptions over time, together with data visualisation tools can proactively deliver reliable analytics insights to users.

Medical Coding

Medical terms can be partially auto-coded using regular programming, with medical coders reviewing and manually coding the remaining terms. ML algorithms, trained on coding libraries from various therapeutic areas, can automate this process and accurately match text with the appropriate terms in specialty dictionaries.

Chatbots

Chatbots are a straightforward way to improve user experience and reduce support staff workload. They are powered by machine learning and can be contacted by voice or text. They understand natural language and context and can provide fast, accurate responses to various queries from patients, site users, and CRO staff.

AI has the potential to bring about a transformative impact on clinical trials by reducing the need for manual intervention, optimizing trial design and patient recruitment, and flagging patterns in big data impossible through human cognition alone. However, AI in clinical research is still by and large unchartered territory and presents risks such as the loss of interpretability as complexity increases, with potentially life-threatening unintended consequences. While they hold revolutionary promise, there remains significant work to responsibly deploy AI-driven technologies into clinical trial procedures.

AI Powered Clinical Trial Solutions

AI solutions span patient recruitment to decentralized trial platforms

Organisations, from large scale CROs to nimble start-ups are bringing AI technology into the clinical trial setting. Services offered include: accelerated patient recruitment; setting up and managing clinical trials; automating repetitive tasks; synthetic control arms; patient centric systems which capture digital biomarkers; behavioural variables to predict participant behaviour and response to treatment; social media insights to recruit for clinical trials; and AI powered platforms for running decentralised clinical trials.

Building these systems requires technical expertise and digital infrastructure that may be challenging for large organisations to emulate. With the exception of Deloitte and Medidata, the majority of companies operating in this space are disruptive start-ups, with teams of engineers. They offer partnerships with pharma for SaaS, and require a big-tech cloud provider such as AWS, Microsoft or Google to support their infrastructure.

The majority of use cases for AI systems in clinical trials are not ground-breaking, they align with the status quo of the RCT process. AI in clinical trials is being deployed in a way that accelerates processes that are already commonplace, such as automating routine tasks. Therefore, currently they pose little threat for maligned outcomes and provide motivating promise for sponsors, CROs and governments seeking to make research more efficient.

As we become comfortable with the use of AI in clinical research, the gap between research and care will converge. As data extracted from the care setting can be analysed to extract insights.

What's Next on the Horizon

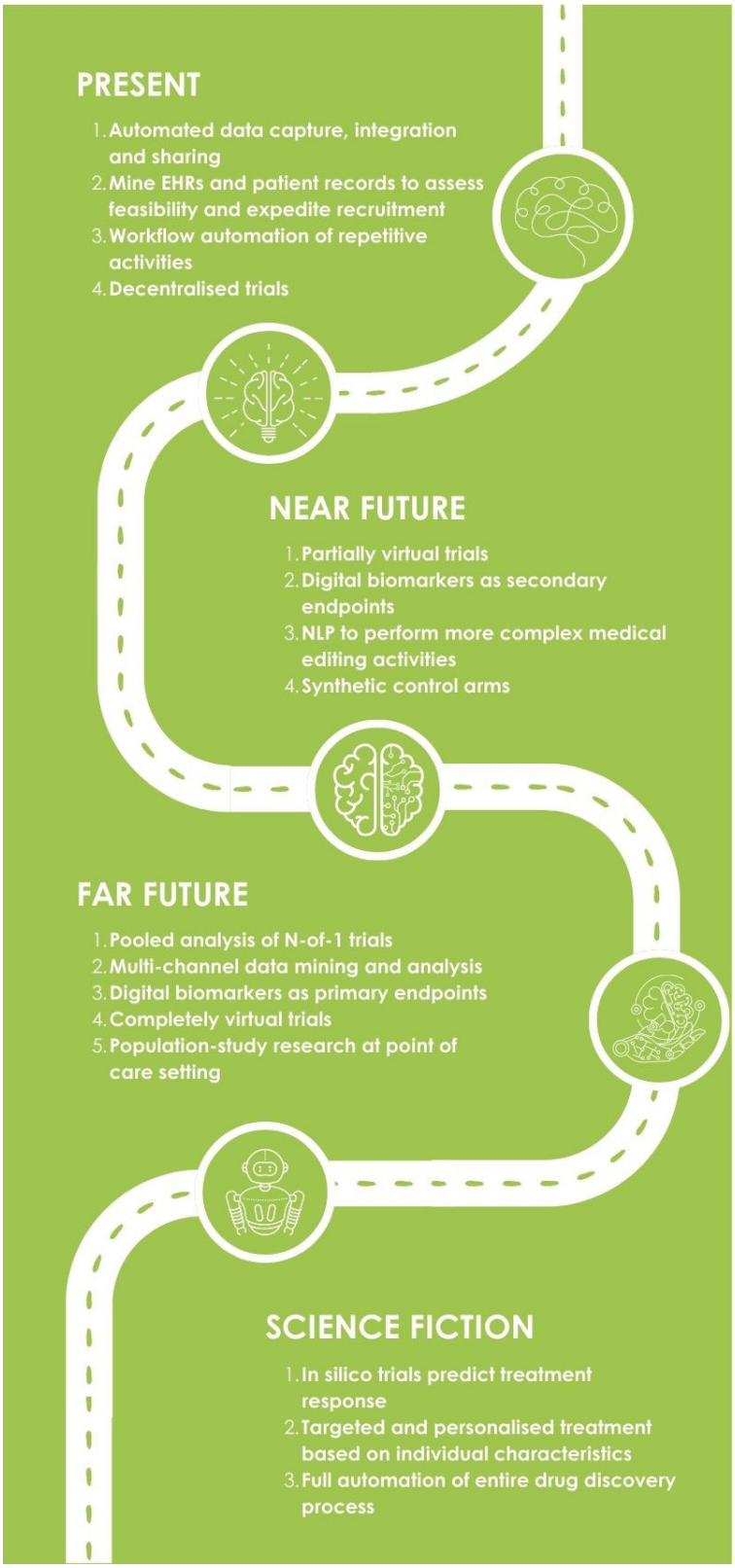
Looking to the future of AI in clinical trials

There is data in everything we do. Imagine a health system that mines and analyses large sets of natural history data, genomics, all other omics, all published clinical studies, real world data, data from smart devices and wearables, all readable to provide next-generation evidence for medicine. As health data becomes more legible by machine learning systems, this will bring clinical research and care together into the same package.

We have only scratched the surface of the potential impact of AI on the clinical research landscape. As AI is incorporated into complex decision-making, the role of human oversight becomes increasingly important. As it stands, AI systems are not generally 'interpretable', it is not currently possible to understand how a certain output is generated, and so we are a long way from incorporating AI into clinical research and practice responsibly.

Nonetheless, the potential gains from early adoption of AI in clinical trials, such as efficiency, speed and data-driven insights, are already compelling motivators for sponsors, CROs and governments to foster adoption, before it may be responsible to do so.

At present, AI enables faster and cheaper clinical trials, as well as alternative trial models such as decentralised clinical trials. Further progress may lead to N-of-1 trials and in silico trials becoming a new normal in a science-fiction future.



Decentralised Clinical Trials

How digital systems put the patient at the centre of medical research

A decentralized clinical trial (DCT), sometimes referred to as a virtual or remote clinical trial, is a research study that employs a flexible, patient-centric approach as compared to traditional site-based clinical trials. In a decentralized trial, various aspects of the study, such as patient recruitment, data collection, and monitoring, are conducted remotely, using digital technologies and tools.

Remote patient recruitment:
Patients are enrolled through online platforms, telemedicine consultations, or other digital channels, removing the need for physical visits to a trial site.

Remote monitoring and data collection:
Participants' health data is collected remotely using EHRs, wearables, apps, or other digital tools, enabling continuous monitoring of patients' health status.

Telemedicine consultations:
Study participants can interact with trial investigators and healthcare professionals through video calls or other virtual communication methods, reducing the need for in-person visits.

Direct-to-patient medication delivery:
Investigational drugs or other trial-related materials can be shipped directly to patients' homes, eliminating the need for patients to travel to trial sites for treatment administration.

\+ Our Future Health

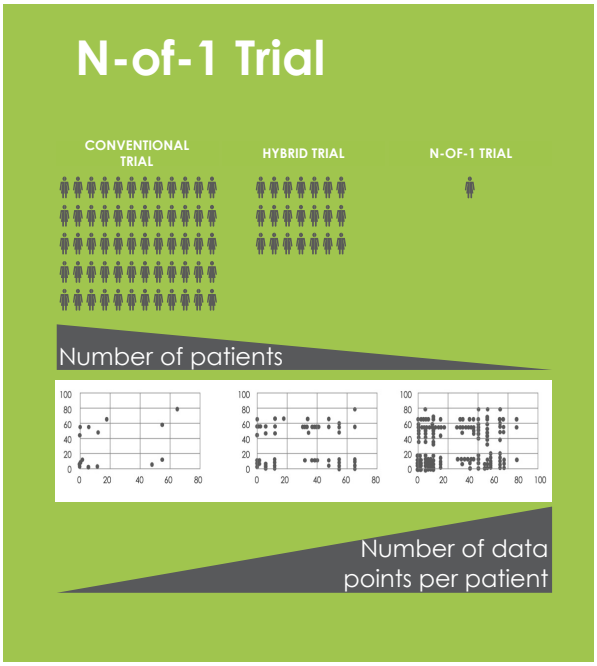
The NHS initiative, Our Future Health, was launched in 2021. The program aims to recruit up to 5 million volunteers, making it one of the largest population health research studies globally. The participants will contribute their health data, such as electronic health records, biological samples, and lifestyle information, over a long period of time, potentially up to several decades. By analysing this data, researchers hope to gain insights that can inform the development of new treatments, prevention strategies, and healthcare policies.

COVID-19 accelerated adoption of aspects of decentralisation of clinical trials such as telemedicine, and increased HCP and patient comfort with using the technology. 'Bringing the clinical trial to the patient' increases accessibility, improves retention and reduces costs. The model of using a central digital platform to run a trial also enables large scale trials for population level insights. However, fully virtual trials are currently only suited to well-characterized drugs with few adverse events in a mild indication, with end points suited to remote measurement. Complex cases require traditional site-based trials or a hybrid model.

Alternative Models for Clinical Trials

A stepping stone towards evidence based personalised medicine

The complexity of tailoring to individual characteristics in personalised medicine may shift prescribing power from doctors and towards AI algorithms. Personalised medicine tailors treatment to the patient, and significant data on the patient is required to recommend optimal treatment. Testing on a digital twin or conducting an N-of-1 trial could support clinical decision-making with a level of personalisation impossible through current practices. In this way, N-of-1 and in silico trials may bring statistical evaluation of medicines into the point of care setting and be used to recommend treatments.



N-of-1 trials are trials performed on a single patient to determine if a treatment for a chronic disease is working. The patient monitors biomarkers as they switch from treatment to placebo and back again to determine their individual response to treatment. This enables physicians and patients to minimise misdiagnoses, to find treatments, and to determine whether the prescribed treatment is providing clinical benefits. AI-based tools, such as ML algorithms and predictive analytics on pooled N-of-1 trial data, can be leveraged against patient-specific factors that influence treatment responses, such as genetic, epigenetic and environmental variables, to analyse for patterns.

In Europe, the Virtual Physiological Human (VPH) Institute is working to create a digital model of the human body for research and clinical trials. A physician could examine a patient's virtual body to predict how it would react to a medication, as well as how additional medications would react with one another.

In silico trials would analyse vast amounts of data from preclinical studies, clinical trials, and real-world data from multiple channels to build predictive models. The patient's response can be tested against the in silico prediction to improve the model.

Enhancing Competitive Intelligence Strategies

Key insights and recommendations for CI in a shifting research landscape

In the dynamic world of AI-powered clinical trials, staying informed about competitor moves is essential to proactively adapt to this shifting landscape. Five recommendations for competitive intelligence professionals to keep track of trends:

1. AI tools in clinical trials will increasingly influence medical research. **Monitor how regulators are overseeing AI in the context of clinical trials and the potential shortening of approval timelines for early adopters.**

2. Expect a rise in strategic alliances between big tech and big pharma**. Incorporate tech-pharma partnerships when benchmarking the competition, as it may have an impact on clinical trial timelines**.

3. As AI systems learn from big data, CROs able to leverage substantial datasets will play a more prominent role in conducting late-stage research. **Keep an eye on the adoption and advancements of AI systems by CROs to identify potential market leaders.**

4. Companies focused on early-stage research may shift exit strategy from acquisition by big pharma to partnership with a CRO for late-stage research and commercialization. **Adjust your competitive lens to capture the evolving landscape of collaborations between early-stage research companies and CROs.**

5. There is an increased importance of large anonymized data repositories leveraged by academic institutions and governments, as seen in initiatives like Israel's National Program for Digital Health and Our Future Health in the UK. **Stay informed about relevant diagnostic and treatment plan recommendations emerging from these programs.**

"…more companies will use outsourcing to obtain the necessary expertise, especially in advanced technologies, such as AI, cloud computing and robotics. Sector growth will be driven by strategic partnerships with academia, analytics companies and big tech, as well as CROs, as biopharma companies focus on strengthening their R&D capabilities…."

Deloitte
'Global Life Sciences Outlook 2019'

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Concluding Remarks

The use of artificial intelligence (AI) in clinical trials has immense potential to transform the way medicine is practiced. The integration of AI-driven technologies has already been shown to accelerate the process of clinical trials, improve patient recruitment, streamline data collection and analysis and generate study reports. Alternative models for clinical trials, such as N-of-1 and in silico trials, may increasingly influence medical research in the future. Presently, AI-powered clinical trials are being deployed in real-world settings by pharma and biotech companies in partnership with start-ups developing AI SaaS.

However, as with any new technology, there are challenges and risks associated with using AI in clinical trials. Data privacy and algorithmic fairness must be considered. Regulators in the US, EU, and UK have established frameworks to oversee AI-driven medical research, however, compliance with these regulations doesn't necessary spell safe and ethical use of AI in clinical trials as the technology is in the early days of development and there are still many unknowns.

As AI becomes increasingly integrated into the clinical research setting, it may enable the adoption of alternative models for clinical trials, further shaping the landscape of clinical research. However, AI produces complex results that are difficult to interpret, and it is essential that researchers and regulatory authorities understand how to interpret these results to retain human oversight and accurately minimise the risk of unintended consequences. Mistakes could potentially trigger reactionary regulatory brakes that dramatically halt the development of an industry that promises a route to realising the vision of evidence based medicine with care personalised to the individual.

Realising the potential of AI requires a multi-stakeholder approach - open data standards will need to be embraced to enable self-learning systems to improve predictions and prescriptions over time. Reliable analytics will be needed to facilitate insight generation from pooling a wide array of data channels. Partnerships between pharma and tech will multiply, both with start-ups and established AI and cloud computing providers to leverage capabilities, and there is likely to be an associated shift in approval timelines based on an organisation's clinical research partnership stack.

In summary, AI will foster a new era of medicine and competitive intelligence professionals that stay informed about competitor moves in this shifting landscape will provide more accurate insights. AI is currently shortening clinical trial timelines, but in the future, developments may impact the entire clinical research process as AI contributes to a paradigm shift where clinical research is incorporated into the point of care and enables the development of data-driven personalised medicine.

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