

AI IN CLINICAL TRIALS

Practical Applications and Regulatory Expectations

Emerging Technologies Are Transforming Trial Delivery

AI, decentralized strategies, modeling and real-world data (RWD) are helping clinical development teams move beyond traditional models and make earlier, better-informed decisions.

- ✓ Efficiency
- ✓ Scalability
- ✓ Flexibility
- ✓ Better decision-making

What AI Is Enabling

AI is being applied across clinical development to support faster, more confident and more data-driven decisions.



Earlier development decisions



Improved recruitment and enrollment efficiency



Proactive identification and risk mitigation

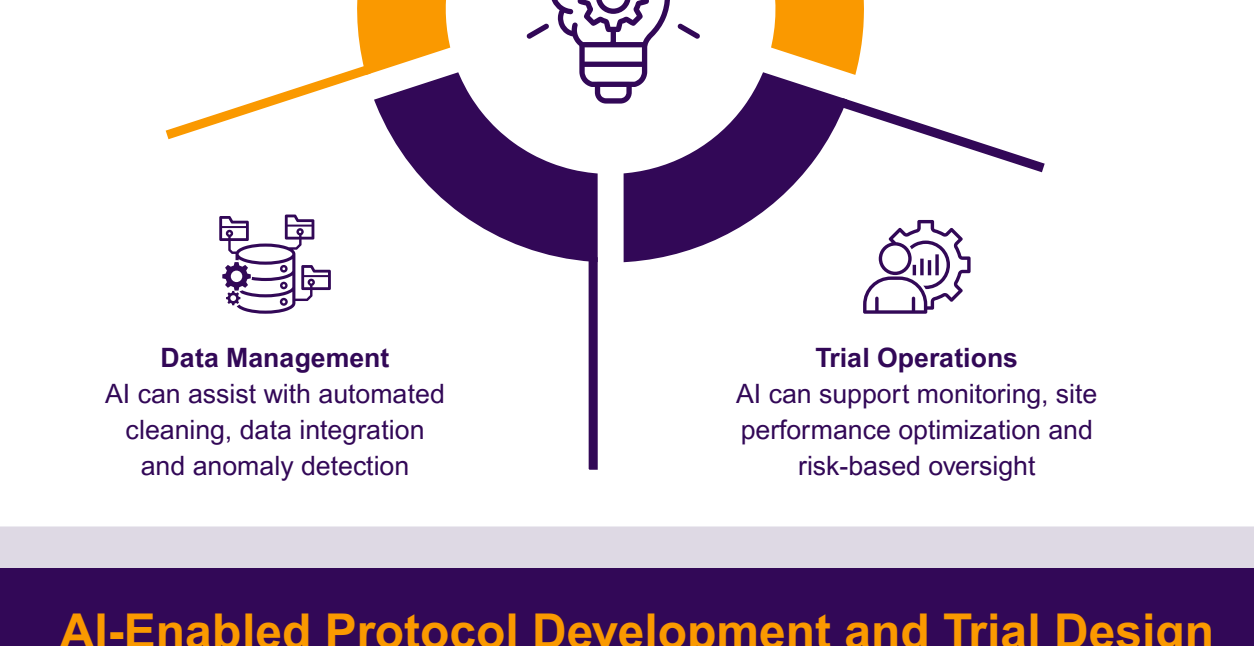


Better use of complex and diverse datasets



More streamlined trial execution

The Clinical Trial Lifecycle: Where AI and Advanced Technology Add Value



AI-Enabled Protocol Development and Trial Design

At the protocol stage, AI and advanced technologies can help teams write streamlined protocols, simulate adaptive designs and use real-world data to build stronger comparator strategies.

AI-Powered Protocol Authoring

- ✓ NLP tools analyze historical protocols to flag design risks and ambiguities
- ✓ Can identify overly complex eligibility criteria
- ✓ Can find inconsistencies between endpoints and statistical plans
- ✓ Can benchmark against regulatory precedents and competitor studies

Adaptive and Innovative Study Designs

- ✓ Simulation engines model adaptive dose-finding
- ✓ Support seamless Phase II/III transitions
- ✓ Bayesian frameworks enable pre-planned interim decisions
- ✓ Decentralized elements (ePRO, wearables, telemedicine) reduce patient burden

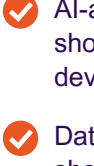
RWD and Synthetic Control Arms

- ✓ EHR and claims data can help inform comparator arm construction
- ✓ Propensity-matched external controls may reduce placebo arm size
- ✓ Biomarker-driven enrichment can improve signal-to-noise in small populations

Regulatory Considerations in Study Design

AI in Trial Design Must Be Regulatory-Ready

AI in study design is as much a regulatory consideration as it is a technical one. Sponsors need transparency, validation and early agency engagement from the start.



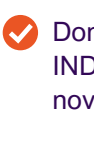
Data Transparency

- ✓ AI-generated recommendations must be traceable
- ✓ Maintain audit-ready logs of input data, model versions, outputs and rationale
- ✓ AI-assisted monitoring should flag protocol deviations in real time
- ✓ Data governance should define data provenance, access controls and version management



Model Justification

- ✓ Models used in trial design or patient selection must be analytically validated
- ✓ Show training data characteristics, performance metrics and known failure modes
- ✓ Avoid black-box approaches for critical decisions
- ✓ Demonstrate fitness for purpose for the exact use case, population and endpoint



Early FDA Alignment

- ✓ Request Type B or C meetings
- ✓ Consider FDA's ISTAND Pilot Program
- ✓ Stay aligned with evolving FDA AI/ML guidance
- ✓ Don't wait until after IND filing to discuss novel AI elements

AI in Feasibility and Study Start-Up

AI can help teams identify stronger sites, evaluate more options, and accelerate start-up activities.

Intelligent site identification can assess:



- Enrollment velocity, patient density and protocol fit
- EHR data, publication history and prior trial performance
- 'Hidden gem' sites beyond standard investigator lists
- Competing trials and overlapping patient pools

Accelerated start-up tools can support:



- AI-assisted IRB/EC document preparation, reducing drafting time by 40-60%
- Smart contract tracking for budgets and site agreements
- Automated regulatory submission readiness checks against country requirements
- Digital site activation dashboards with real-time milestone tracking

AI-enabled feasibility and start-up approaches may support a 30-50% reduction in feasibility timelines, evaluate 2-3x more site options and potentially accelerate first patient first visit by 60%.

Selecting Better-Performing Sites

AI can help connect eligible patients to investigators, institutions and geographies.

Predictive site performance may consider:



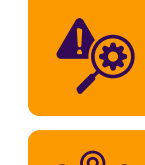
Historical enrollment metrics, prior performance by indication and phase



Retention rates, screen failure rates



Data quality, compliance history



Investigator workload



Patient diversity



Competing trials in the same geography or therapeutic area

Fewer, better-performing sites, with the option to support just-in-time models such as standby sites.

Finding Eligible Patients More Efficiently

AI can process structured and unstructured data against complex inclusion and exclusion criteria.



Scan EMR, real-world data and clinical notes



Identify obvious and "hidden" eligible patients



Re-evaluate patients as new data emerges



Improve speed, accuracy and diversity



Keep investigators involved in decision-making

Personalized Patient Recruitment

AI-enabled recruitment can help teams move from broad rescue tactics to targeted, site-specific interventions.



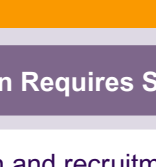
Continuous patient matching



Early detection of enrollment slowdowns



Identification of screening inefficiencies



Referral strategies



Digital outreach



Online pre-screening



Tailored patient messages using personalization factors

Regulatory Considerations for Patient Selection and Recruitment

Recruitment Innovation Requires Safeguards

AI can improve patient selection and recruitment, but this is also where ethical and regulatory scrutiny is especially high.



Regulatory watchpoints



AI-driven eligibility & enrichment



Diversity, equity & inclusion



Informed consent & data privacy

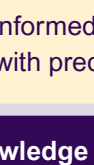


Decentralized & hybrid recruitment

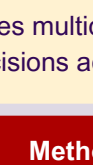
Decentralized Trial Tools Can Expand Access

Decentralized and hybrid tools can reduce patient burden and support more flexible trial participation.

Remote patient onboarding



eConsent through secure web portals, tablets and smartphones

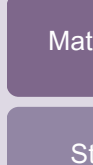


Video support and telehealth enrollment

Remote data capture



ePRO, eCOA and eDiary tools



Mobile apps and web portals



Automated reminders and notifications



Real-time study database integration

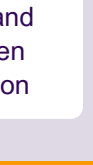
Remote care and monitoring



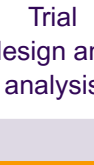
Routine check-ups and follow-ups



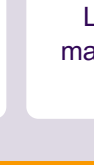
Medication management and prescription refills



Chronic disease management



Mental health counseling



Patient education and health monitoring

What Is Model-Informed Drug Development?

Model-informed drug development (MIDD) combines multidisciplinary scientific knowledge with predictive models to support better decisions across drug development.

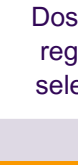
Knowledge base includes:



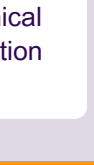
Biology



Pharmacology



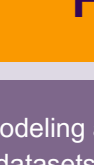
Medicine



Mathematics



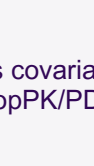
Engineering



Statistics



Epidemiology

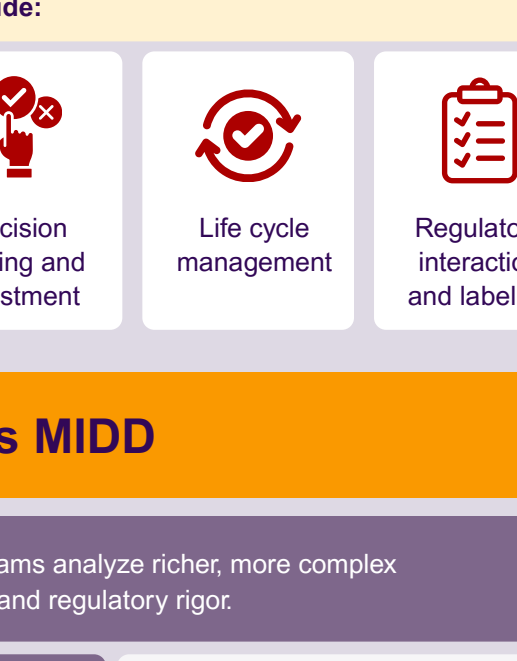


AI



Computer Science

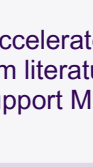
Methods under the MIDD umbrella include:



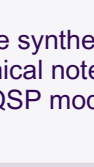
MIDD benefits include:



Dose and regimen selection



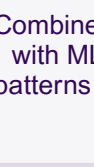
Preclinical translation



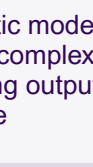
Trial design and analysis



Decision making and investment



Life cycle management



Regulatory interaction and labeling

How AI Expands MIDD

AI builds on modeling and simulation by helping teams analyze richer, more complex datasets while maintaining scientific and regulatory rigor.



Finds covariates faster in popPK/PD models



Identifies biomarkers from omics, imaging and wearable data



Accelerates evidence synthesis from literature and clinical notes to support MBMA and QSP models



Combines mechanistic models with ML to capture complex patterns while keeping outputs interpretable

Critical requirements:

- ✓ High-quality data
- ✓ Documented assumptions
- ✓ External validation
- ✓ Transparent outputs

Responsible AI Adoption: 5 Essentials

Responsible AI adoption is not primarily a technology project. It is an operating-model change involving people, process, governance, platforms and scientific standards.

Governance

Define where AI can and cannot be used. Assign accountability and maintain human review.

Context of Use

Clarify whether AI supports productivity, QC or regulatory evidence generation.

Traceability

Capture prompts, inputs, outputs and review decisions. Maintain version control.

Technology and Data Controls

Use secure environments, monitor performance and integrate with validated systems.

People and Capability

Upskill teams and align statistics, data science, regulatory, quality and IT.

Clinical trials are moving beyond traditional models, with AI, MIDD, decentralized approaches and data-driven decision-making helping improve trial design, execution and regulatory alignment.