FORUM

The Academy of Medical Sciences

Regulating AI and computational models in clinical trials

Monday 4 September 2025

Academy of Medical Sciences' FORUM roundtable, held in partnership with the Regulatory Innovation Office (RIO)

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Executive summary

In September 2025, the Academy of Medical Sciences and the Regulatory Innovation Office (RIO) held a FORUM roundtable on regulating AI and computational models in clinical trials. The roundtable involved approximately 40 experts from across sectors, including academia, industry, regulation, government, funding bodies, healthcare, charity organisations and public contributors. The event opened with talks from industry and the Medicines and Healthcare products Regulatory Agency (MHRA), followed by a panel discussion where senior industry and regulatory experts discussed the opportunities of these technologies to streamline medicine development. Discussions then focused on next steps to support their use in clinical trials through regulation.

The overall message was that there is an opportunity for the UK to become a global champion in the regulation of AI and computational models in clinical trials. The MHRA's intention is to adopt a pragmatic, innovative approach to become an international standard-setter in risk-proportionate regulation. Participants welcomed this ambition and identified several opportunities for the UK to lead in this area. The Academy of Medical Sciences is committed to working with RIO, the MHRA, and the Centers of Excellence for Regulatory Science and Innovation (CERSIs) and other partners to advance these opportunities and enable regulatory leadership.[1] Opportunities highlighted include:

1. **Pioneering AI and computational models for clinical trials where gaps or unmet medical needs exist, diverging from global approaches as necessary**. The UK could lead internationally in areas where traditional clinical trials are unfeasible or challenging. Prioritising applications that address unmet needs could generate insights to shape clear frameworks for future use. If implemented quickly, this could secure international investment in the development of new medicines and drive economic growth.
2. **Identifying core principles for the regulation of AI and computational models in trials**, balancing international divergence and harmonisation. The UK could align internationally on key principles and international data quality standards to support use in multinational trials.
3. **Establishing risk-proportionate regulatory frameworks** for the use of AI and computational models in clinical trials, building on existing initiatives. This could include:
    - Guidance and case studies on risk categorisation for AI and computational models.
    - Information on how different technologies for use in clinical trials should be classified.
    - Clear expectations on validation, tailored to the technology's function and trial phase
4. **Creating an early dialogue mechanism for innovators to test AI and computational modelling approaches confidentially with regulators**. This could be modelled on the European Medicines Agency's (EMA) Innovation Taskforce, which establishes early dialogue with regulators through briefing meetings for innovators. Other bodies, e.g. the Health Research Authority (HRA) and funders, could help clarify ethical, research and regulatory expectations early in the process.
5. **Adopting an influential role for regulators to enable better data access and sharing**, including through demand signalling and making the case for better access to data.
6. **Building trust by communicating openly and transparently** with trial participants and the public on how the use of AI in clinical trials is assessed, and how AI can benefit medicine development and patients. This should include ensuring participants can give informed consent by communicating transparently about the use of their data during and after the trial.
7. **Improving AI literacy** among regulatory, industry, and healthcare staff to support the uptake and utility of these technologies in clinical trials.

[1] UK Government (2025). *AI for Science Strategy.* https://www.gov.uk/government/publications/ai-for-science-strategy/ai-for-science-strategy#introduction-the-ai-for-science-opportunity

Introduction

The United Kingdom has an outstanding track record for the development of new medicines, built upon a strong research ecosystem and unique infrastructure.[2] However, challenges such as its small market share and slow clinical trial set-up times and recruitment undermine the UK's global competitiveness as a destination for clinical trials.[3] Innovative approaches to clinical trial delivery and complementary regulatory oversight could boost trial efficiency, enabling patients to access medicines quicker and bolstering the UK as a hub for innovation and drug development. AI and computational modelling technologies – such as digital twins, in-silico trials and synthetic control arms – could help investigate and predict drug efficacy and safety, enrich trial populations, save costs, improve timelines and reduce burden on patients.[4]

There is growing interest in the transformative potential of these technologies for clinical research, with many pharmaceutical companies investing heavily in AI tools and integrating them across the drug development lifecycle.[5] Across industry, AI and computational models are currently being applied to improve efficiency and trial success rates through the prediction of drug efficacy, patient identification and recruitment, intelligent trial design, enhanced monitoring and more.[6] The opportunities are numerous, with machine learning already being used to guide early biomarker-driven trials in breast cancer.[7] Synthetic control arms are also being employed to enhance early-phase oncology trials.[8]

To realise the benefits of AI and computational modelling in trials, it is important to clarify the regulatory approach needed in the UK to support the use of these technologies.[9] In addition, many pharmaceutical companies and researchers conduct clinical trials across multiple countries, requiring compliance with each regulatory jurisdiction. A degree of alignment and harmonisation will be needed to support engagement with multinational trials.

Risk-proportionate regulation enables innovation, delivering benefits to patients while enhancing the competitiveness of UK research and development.[10] The UK is in a unique position to be a regulatory innovator due to the strength of its scientific base and its ambitions to be a thought leader in this space. The Medicines and Healthcare products Regulatory Agency (MHRA) also recently committed to implementing a flexible and risk-proportionate approach to clinical trials regulation and is working

[2] Academy of Medical Sciences (2016). *The UK drug discovery landscape* https://acmedsci.ac.uk/file-download/71272985
[3] ABPI (2025). *Creating the conditions for investment and growth* https://www.abpi.org.uk/publications/creating-the-conditions-for-investment-and-growth/
[4] Hutson, M. (2024). *How AI is being used to accelerate clinical trials* https://www.nature.com/articles/d41586-024-00753-x
[5] McKinsey (2024). *Generative AI in the pharmaceutical industry: Moving from hype to reality* https://www.mckinsey.com/industries/life-sciences/our-insights/generative-ai-in-the-pharmaceutical-industry-moving-from-hype-to-reality
[6] Artefact (2024). *AI Breathes New Life into Clinical Trials: Perspectives and Challenges* https://www.artefact.com/blog/ai-breathes-new-life-into-clinical-trials-perspectives-and-challenges/
[7] Amandolare, S (2024). *AI's Drug Revolution, Part 1: Faster Trials and Approvals* https://www.medscape.com/viewarticle/ais-drug-revolution-part-1-faster-trials-and-approvals-2024a1000qqz?form=fpf
[8] Medidata (2022). *Celsion and Medidata to Present Findings on Use of Synthetic Control Arm to Estimate Treatment Effect in Ovarian Cancer Trial* https://www.medidata.com/en/about-us/news-and-press/celsion-and-medidata-to-present-findings-on-use-of-synthetic-control-arm-to-estimate-treatment-effect-in-ovarian-cancer-trial/
[9] KPMG (2024). *In Silico Regulatory Evidence Utilisation within the Life Science Sector (Version v1)*. https://doi.org/10.5281/zenodo.12735158
[10] Tait, J., Banda, G. & Watkins, A. (2017). *Proportionate and adaptive governance of innovative technologies (PAGIT): A framework to guide policy and regulatory decision making*. http://www.innogen.ac.uk/sites/default/files/2019-04/PAGIT%20FrameworkReport-Final_170717.pdf

with the Regulatory Innovation Office (RIO) to consider how the ecosystem can support the use of AI in drug discovery and development.[11]

On 4 September 2025, the Academy of Medical Sciences' FORUM hosted a roundtable in partnership with the Regulatory Innovation Office (RIO) to bring together experts from across academia, healthcare, charities, industry, venture investors, regulators, government, funding bodies and public contributors to consider what regulatory landscape is needed for innovators to use AI and computational models in clinical trials. Participants identified the opportunities and next steps required to support the use of these technologies in trials through the regulatory framework. The consensus among participants was that the UK is uniquely positioned to be a leader in the adoption of these technologies, which can be achieved if UK regulators champion agile, risk-proportionate regulation that enables innovation while ensuring patient safety.

The roundtable was chaired by **Professor Deborah Ashby OBE BMedSci FRS**, Dean of the Faculty of Medicine and Chair in Medical Statistics and Clinical Trials at Imperial College London, and **Dr Dan O'Connor**, Director of Regulatory Policy and Early Access at the Association of the British Pharmaceutical Industry (ABPI). The event opened with an overview of the MHRA's current risk-proportionate approach (RPA) for Clinical Trial Authorisation (CTA) from **Professor Andrea Manfrin**, Deputy Director of Clinical Investigations and Trials at the MHRA.[12] This was followed by an industry perspective on CTA and AI and computational technologies by **Dr Virginia Acha**, Associate Vice President of Science and Regulatory Policy at Merck Sharp & Dohme (MSD). An expert panel discussion then took place, involving:

- **Dr Virginia Acha**, Associate Vice President of Science and Regulatory Policy at Merck Sharp & Dohme (MSD)
- **Dr Alison Cave**, Chief Safety Officer, MHRA
- **Professor Alejandro Frangi FREng**, Bicentenary Turing Chair in Computational Medicine and Chair of the UK Centre of Excellence for In-Silico Regulatory Science and Innovation (UK CEiRSI)
- **Professor Andrea Manfrin**, Deputy Director of Clinical Investigations and Trials at the MHRA
- **Natalia Kotchie**, Senior Vice President, R&D Applied Data Science Center, IQVIA

Following the panel discussion, participants moved to breakout groups to discuss the current regulatory landscape for AI and computational models, the opportunities for the UK to lead in regulation for these technologies and the next steps required to support innovators to adopt these technologies at scale in clinical trials.

[11] UK Government (2025). *Life Sciences Sector Plan* https://assets.publishing.service.gov.uk/media/687653fb55c4bd0544dcaeb1/Life_Sciences_Sector_Plan.pdf. Also see: https://www.gov.uk/government/news/clinical-trials-regulations-signed-into-law

[12] Manfrin, A. (2025). *Evaluation of the Medicines and Healthcare products Regulatory Agency's introduction of a risk-proportionate approach for clinical trials: An analysis of 4617 applications assessed between September 2023 and August 2024.* Br J Clin Pharmacol. 2025; 1-8. doi:10.1002/bcp.70308

Championing AI and computational models in clinical trials

AI and computational models offer transformative potential to **enhance clinical trials and accelerate drug development** by offering solutions to long-standing challenges such as participant recruitment, trial set-up, cost and lengthy timelines. Applications include:

- In-silico clinical trials refers to the development of patient-specific models to form virtual cohorts for testing the safety and/or efficacy of new drugs and medical devices.[13] In-silico trials can simulate both control and efficacy arms and ultimately optimise patient recruitment and drug protocols.[14] Digital twins (virtual replicas of individual patients) can also be used in simulation-based trials to predict outcomes and optimise protocols.[15]
- Synthetic patient data, which can be understood as computer-generated data modelled from existing datasets. Machine-learning models can be trained on synthetic datasets, and they can also be used to supplement real-world evidence.[16]
- Computational modelling can be used to make predictions based on real-world, synthetic or simulated datasets, depending on whether the models are derived from real-world data, synthesised using statistical/AI methods (phenomenological models), or derived from first principles (mechanistic models). It can also help enrich samples and supplement data in areas where recruitment has traditionally been difficult, e.g. in rare diseases and small populations.[17] Computational modelling can optimise dosing and novel endpoints, including patient-reported outcome measures (PROMs), and identify the most effective and safest dose for different patient groups. This, in turn, could potentially reduce trial sizes, reduce completion time, and lead to improved outcomes.

Unlocking the transformative potential of these technologies will require **agile, risk-proportionate regulation**.[18] Participants agreed that the UK has an opportunity to be a champion for the use of AI in clinical trials by leading the development of flexible frameworks that evolve with technology. The UK is well placed to lead globally in this space, thanks to its rich data assets, regulatory pragmatism and strong scientific base. Participants also noted that the UK has strong collaborative culture, meaning it could take the lead in activating interdisciplinary groups to establish what best practice looks like.

Becoming a standard-setter in this space could be a unique opportunity for the UK to shape key principles and standards – including internationally – and learn and adapt through practical initiatives such as airlocks or sandboxes. Expanding and building on existing initiatives such as the MHRA's scientific advice meetings and AI Airlock or the Innovative Devices Access Pathway (IDAP) could provide insights into the application of AI in clinical trials.[19] The novelty of this area and the current

[13] Pappalardo, F. et al. (2019). *In silico clinical trials: concepts and early adoptions.* Brief Bioinform. **20 (5)**, 1699-1708. https://doi.org/10.1093/bib/bby043
[14] Katsoulakis, E. et al. (2024). *Digital twins for health: a scoping review*. npj Digit. Med. **7**, 77. https://doi.org/10.1038/s41746-024-01073-0
[15] Pammi, M et al. (2025). *Digital twins, synthetic patient data, and in-silico trials: can they empower paediatric clinical trials?*
[16] Myles, P et al. (2023). *The potential synergies between synthetic data and in silico trials in relation to generating representative virtual population cohorts*. Prog. Biomed. Eng. 5 013001. https://doi.org/10.1088/2516-1091/acafbf
[17] Pammi, M et al. (2025). *Digital twins, synthetic patient data, and in-silico trials: can they empower paediatric clinical trials?* The Lancet Digital Health **7**, 5. https://doi.org/10.1016/j.landig.2025.01.007
[18] Tait, J., Banda, G., & Watkins, A. (2017). *Proportionate and adaptive governance of innovative technologies (PAGIT): A framework to guide policy and regulatory decision making*. http://www.innogen.ac.uk/sites/default/files/2019-04/PAGIT%20FrameworkReport-Final_170717.pdf
[19] UK Government (2025). *AI Airlock pilot cohort*. https://www.gov.uk/government/publications/ai-airlock-pilot-cohort/ai-airlock-pilot-cohort; UK Government (2025) *The Innovative Devices Access Pathway (IDAP)*.

stage of regulatory development make this an ideal moment for the UK to shape the global approach.

The **MHRA is particularly well placed to lead this effort**, given its global reputation as a trusted source of guidance and willingness to innovate. Participants also highlighted the transparency of its decision-making processes and its openness to discussions with innovators as key enablers. In his talk, Professor Andrea Manfrin outlined the opportunity to build on the new risk-proportionate approach (RPA) for Clinical Trial Authorisation (CTA) by **developing a tailored RPA for trials that incorporate AI and computational models**, in collaboration with the life sciences sector.[20] He noted opportunities for international collaboration, such as with the World Health Organization (WHO).

When creating these regulatory frameworks, the UK can **rely on existing principles and guidance**. The development of AI regulation for medical devices or learnings from the MHRA's AI Airlock could offer valuable insights and principles to shape future approaches. Existing work on model credibility assessments – including those published by the U.S. Food and Drug Administration (FDA) and the American Society of Mechanical Engineers (ASME) – could also be built upon.[21]

Participants stressed the importance of **international regulatory harmonisation**, which is highly influential in decision-making processes about trial design and trial location. As clinical trials are frequently conducted across multiple countries, maintaining a degree of regulatory harmonisation globally is important to ensure the UK remains an attractive and competitive destination for clinical trials, while still allowing space for innovation and leadership in regulatory approaches.

However, participants saw an opportunity for the UK to **pioneer AI and computational models in areas where gaps or unmet medical needs exist**, diverging from global approaches as necessary. This could enable stakeholders to collaborate on potential applications and use these insights to create clearer guidelines and frameworks for future applications. For example, the UK could lead in areas where global frameworks are lacking, such as using AI and computational models to support clinical trials for rare conditions or supporting more representative trials by enriching data for populations where traditional trials are unfeasible or challenging, such as in children, pregnant women and those with rare diseases. To this end, horizon scanning and working with the sector to identify priorities and opportunities for innovation will be important. If implemented quickly, this approach could help secure international investment in the development of new medicines.

While the use of innovative AI and computational technologies in in-silico clinical trials is growing, widespread adoption is still some way off. However, participants noted that the UK's strong research capability presents opportunities, including **leading in areas such as early-stage innovation and Proof of Concept (POC) testing** where the efficacy of a drug candidate is evaluated to determine its potential for further development. This approach could help attract companies to pursue further drug development and research within the UK.

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https://www.gov.uk/government/publications/the-innovative-devices-access-pathway-idap/the-innovative-devices-access-pathway-idap-pilot-phase

[20] Manfrin, A. (2025). *Evaluation of the Medicines and Healthcare products Regulatory Agency's introduction of a risk-proportionate approach for clinical trials: An analysis of 4617 applications assessed between September 2023 and August 2024.* Br J Clin Pharmacol. 2025; 1-8. doi:10.1002/bcp.70308

[21] FDA (2023) *Assessing the Credibility of Computational Modeling and Simulation in Medical Device Submissions.* https://www.fda.gov/regulatory-information/search-fda-guidance-documents/assessing-credibility-computational-modeling-and-simulation-medical-device-submissions; ASME (2018) *V V 40 - Assessing Credibility of Computational Modeling through Verification and Validation: Application to Medical Devices***.** https://www.asme.org/codes-standards/find-codes-standards/assessing-credibility-of-computational-modeling-through-verification-and-validation-application-to-medical-devices

As different applications of these technologies emerge, there is a **need to prioritise** and decide what types of products or applications should be the focus. Not all AI and computational models are equally ready to be used in clinical trials, for example, cell and gene therapies are first generation technologies, meaning there is limited data to inform computational models. Regulators could **pilot a well-defined use case** where AI or computational models could realistically add value, with a focus on where validation is possible to help stakeholders feel confident in the process. This could be approached as a testing phase, with the aim of using the data to inform regulation and help scale up AI approaches responsibly. This should be done collaboratively between regulators, industry, academics, patients and others. As a first step**, a small cross-sector expert group** could be formed to characterise a group of potential therapies, focused on mechanisms of action, to guide prioritisation.

Participants also suggested that the UK could **pioneer the use of synthetic data and modelling** for testing in populations where traditional trials are unfeasible or challenging – such as pregnant women, children, or those with rare diseases. They discussed the potential for predictions based on synthetic data and AI-based modelling to be conducted before Paediatric Investigation Plans (PIPs), with the aim of then validating these predictions against real-world outcomes. This would help refine and improve the accuracy of computational modelling and build a more informed data package to support future trial design and regulatory decision-making.

AI's analytical capabilities can be applied to large, complex datasets generated during trials (e.g. from wearables) to uncover insights that would otherwise be missed and **identify new endpoints**. It could, for example, predict long-term patient outcomes to enhance understanding of drug effectiveness and safety, by modelling the effects of a treatment months or years after it is administered. Participants identified an opportunity for UK regulators to encourage industry to include AI-derived data as exploratory endpoints in clinical trials, which could help to test feasibility, develop new outcome measures, and build evidence for future validation, without influencing the primary endpoint. This could help create clearer pathways for integrating AI into clinical trial design, although there are concerns around potentially using unvalidated or poorly understood data.

Participants noted that, as well as specific steps to enable the adoption of AI and computational models in clinical trials, **challenges in the UK's clinical trial infrastructure**, including slow set-up times, access to data at scale and NHS capacity for research, must also be addressed to enhance the UK's competitiveness in trial delivery.

Next steps for regulation

1. Establishing clear regulatory frameworks and guidance

A key challenge raised by participants was a lack of clear guidance or frameworks for the use of AI and computational models in clinical trials. The MHRA is already exploring a Risk Proportionate Approach (RPA) for Clinical Trial Authorisation (CTA) specifically tailored to trials incorporating AI and computational models, indicating its willingness to be innovative in its approach to regulating these technologies.[22] It has an opportunity to build on this approach by **providing clear, risk-proportionate frameworks and guidance** for the use of AI and computational models in clinical trials. This will increase confidence from industry in the likelihood of trial approval and encourage the adoption of these technologies.

Specifically, regulators could publish **clear guidance on the categorisation of risk for specific AI and computational technologies** for innovators, highlighting the distinction between high-risk and low-risk applications. Guidance should include how AI technologies for use in trials might be classified (for example, as medical devices) and a repository of case studies that is regularly updated to provide examples of the types of technologies being approved. This would allow for better informed decision-making by innovators and higher confidence in the likelihood of approval. Participants recommended involving patients and industry partners in the development of any classifications. The UK could also agree on a risk-tiered approach where lower-risk technologies, for example those that automate processes, are approved for use in trials through a streamlined pathway.

Establishing core principles for approaching the regulation of AI and computational models in clinical trials could help overcome international regulatory fragmentation while maintaining flexibility over specific guidance in the UK to enable forward thinking. Participants suggested agreeing on a common definition of AI (e.g. the Organisation for Economic Co-operation and Development's definition)[23] and clarifying subcategories through a dedicated working group. They also suggested aligning with international data quality standards to ensure data meets the high standards set by bodies like the European Union (EU), International Organization for Standardisation (ISO) and professional medical records organisations.

Participants raised concerns about the **accuracy of generative AI tools and methods to validate and explain them**. AI systems – particularly non-deterministic and generative models – often function as black boxes, where the internal decision-making processes are opaque or inaccessible. This lack of transparency challenges traditional validation methods and makes it difficult to assess the reliability of outputs. To address this, **regulators could work with developers to publish guidance that covers validation expectations**. Participants suggested that validation should be tailored to the AI's function and trial phase, as the way AI is validated may vary significantly across disease states and phases of a clinical trial.

Regulators have an opportunity to encourage innovators to adopt these technologies by establishing a **formal, confidential pre-advice meeting structure** for AI-enabled trials. Participants suggested

[22] Manfrin, A. (2025). *Evaluation of the Medicines and Healthcare products Regulatory Agency's introduction of a risk-proportionate approach for clinical trials: An analysis of 4617 applications assessed between September 2023 and August 2024.* Br J Clin Pharmacol. 2025; 1-8. doi:10.1002/bcp.70308

[23] OECD (2024). *Recommendation of the Council on Artificial Intelligence.* https://legalinstruments.oecd.org/en/instruments/OECD-LEGAL-0449

this be modelled on the EMA's Innovation Taskforce, and could bring together the HRA and other relevant bodies, such as funders, to clarify ethical and regulatory expectations with innovators early in the process. If the trial appears likely to meet regulatory requirements, it could be approved and progressed to the next assessment phase. In more complex cases, innovators could be offered an airlock procedure to further refine the approach. An airlock procedure could be particularly valuable for companies that are new to using AI and computational models in clinical trials.

To enable the adoption of these technologies in trials, regulators must have the skills necessary to evaluate AI and computational models. A **skills assessment** could be carried out to identify gaps, which could subsequently be addressed through **appropriate workforce planning, training and secondments**. Identifying and overcoming skills gaps will be a continual requirement in this field as technologies progress and should be done in collaboration with relevant organisations such as the Centres of Excellence for Regulatory Science and Innovation (CERSIs).

2. Data use and governance

Access to and linkage of high-quality, representative datasets are vital for training and developing AI models and building synthetic data. The UK's rich data assets offer significant potential for AI development and use, but this is undermined by **siloed datasets and infrastructural, legal, and governance barriers**. To address this, regulators could take a more active role in ensuring better access to high-quality data through demand signalling. They could use their influence to make the case for better access to data and ensure data sharing is framed positively, highlighting the potential for the development of safer and more efficacious medicines. The Yellow Card Biobank was highlighted as a positive example of how regulatory leadership can help unlock data assets.

The **responsible and transparent use of data** is crucial to **building public trust in AI technologies**. Trial participants must be provided with clear information about how their data will be used during and after the trial so that they can provide informed consent.

Inclusion and exclusion criteria can introduce systemic **bias**, affecting model performance and limiting applicability across diverse populations. To address this, regulators must develop clear frameworks to certify data quality and ensure that AI tools are trained on representative, clinically meaningful datasets.

In light of the roundtable discussions, Professor Andrew Morris, President of the Academy of Medical Sciences and Director of Health Data Research UK drew on his expertise to suggest that regulators, in partnership with industry and academia, could lead a whole-system surveillance of the safety, effectiveness and value of medicines and devices. This could involve **establishing a UK platform of routinely collected data linked to health outcomes**, similar to the Data Analysis and Real World Interrogation Network (DARWIN EU).[24] This platform could harness the UK's rich data assets while overcoming existing challenges such as siloed datasets and infrastructural, legal and governance barriers.

3. Communication and building public trust

There is a relatively low understanding of AI technologies among healthcare professionals and the public, contributing to **reduced confidence**. Negative public perceptions of AI risk undermine the credibility of these technologies and their adoption in clinical trials. Concerns around transparency,

[24] European Medicines Agency (n.d.). *Data Analysis and Real World Interrogation Network (DARWIN EU)*. https://www.ema.europa.eu/en/about-us/how-we-work/data-regulation-big-data-other-sources/real-world-evidence/data-analysis-real-world-interrogation-network-darwin-eu

accountability, and the perceived 'black box' nature of AI systems further fuel scepticism. **Open communication and transparency** around how AI is used and assessed, and how it benefits patients and the public, is needed to address these concerns.

Improving AI literacy across the system through training and scaling will be key to the adoption of AI and computational models in clinical trials. Clinicians and procurement teams need to be aware of how these models work if they are to be successfully integrated into trial design and delivery.

While narratives around AI and computational models in clinical trials are broadly positive, regulators could **strengthen these narratives** by communicating the benefits to the delivery of research and new therapies. Publishing case studies where AI technologies successfully enhanced clinical trials could help to illustrate the benefits to patients and the public. Regulators should be open about **how AI technologies in clinical trials are assessed and approved**, including how risk-proportionality is decided. In addition, developers will need to be transparent with regulators about the development process and the validation of algorithms. Regulators could also engage trusted sources (e.g. large medical research charities) to update the information communicated to patients about clinical trials and AI. Participants also suggested engaging journalists, politicians and opinion leaders to build positive media narratives around AI in clinical trials.

To align in-silico trials with what matters to patients while maintaining rigorous safeguards, regulators should **embed participant perspectives and societal acceptance into risk-proportionate frameworks**. State-of-the-art practice involves eliciting patient preferences (for example, on endpoints, trade-offs and acceptable uncertainties) and translating them into counterfactual scenarios within computational models, enabling exploration of 'what-if' trajectories that reflect those preferences. This requires access to high-quality, representative, and provenance-tracked data; transparent model validation and uncertainty quantification; and structured preference elicitation methods (such as discrete choice experiments, conjoint analysis or multi-criteria decision analysis) that can be operationalised in simulation. Feasibility depends on strengthening AI literacy among participants and healthcare professionals, clearly communicating model assumptions and limitations, and ensuring governance addresses condition-specific risk tolerance, individual expectations, and societal norms. When implemented with robust technical controls, auditing, and patient-centric design, this approach can inform proportionate regulatory decisions, enhance trust in model credibility, and better align trial design and evidence with patient values.

To better reflect the target participants' perspectives, workshop participants suggested **building on existing patient and public involvement and engagement (PPIE) reporting in clinical trial design** when conducting AI- and computational-model-based trials. Involvement must be genuinely meaningful and engage the right patient groups, and these groups should be trained appropriately to contribute.

Conclusion: shaping the future of AI and computational models in clinical trials

The UK has a strategic opportunity to position itself as a global leader in the regulation of AI and computational models in clinical trials. By championing an agile, risk-proportionate regulatory approach to these innovative methods, the MHRA could help to boost trial efficiency, enabling patients to access medicines quickly, and bolstering the UK as a hub for innovation and drug development. Roundtable participants highlighted an opportunity for the UK to be a standard-setter while maintaining the necessary international alignment required to support multinational trials – particularly through agreement on core principles and data standards. It could pioneer AI and computational models for clinical trials where gaps or unmet medical needs exist to encourage collaboration and generate insights to shape clear frameworks for future use.

To enable this, participants highlighted the need for clear guidance and risk-proportionate regulatory frameworks that balance innovation with patient safety. Key priorities include developing clear guidance and qualification pathways for different tools, establishing mechanisms for early dialogue between innovators and regulators, and promoting transparency to build public trust. Improving AI literacy across sectors and enabling secure data access were also identified as essential enablers. By advancing these next steps, the UK can ensure that regulatory innovation keeps pace with technological progress, shaping a future where AI-driven and computational model approaches accelerate medicine development both responsibly and effectively. The Academy of Medical Sciences is committed to working with RIO, the MHRA, CERSIs and other partners to advance these opportunities and enable regulatory leadership.[25]

[25] UK Government (2025). *AI for Science Strategy.* https://www.gov.uk/government/publications/ai-for-science-strategy/ai-for-science-strategy#introduction-the-ai-for-science-opportunity

Annex 1: Attendee list

- **Virginia Acha,** Associate Vice President for Science & Regulatory Policy, MSD
- **Dr Sadaf Alam,** Chief Technology Officer (CTO), University of Bristol
- **Professor Deborah Ashby OBE FMedSci FRS,** Dean of the Faculty of Medicine and Chair in Medical Statistics and Clinical Trials, Imperial College London
- **Dr Alison Cave,** Chief Safety Officer, MHRA
- **Dominique Chu,** Deputy Director, Scientific Data and Insight, MHRA
- **Professor Dave Delpy CBE FRS FREng FMedSci,** Emeritus Professor of Medical Photonics, University College London (UCL)
- **Joao Duarte**, Head of Translational New Therapeutic Areas, UCB
- **Lindsay Edwards,** Chief Technology Officer and President of Platform, Relation Therapeutics
- **Professor Stuart Elborn FMedSci,** Pro-Vice Chancellor for Medicine Health and Life Sciences, Queens University Belfast
- **Joshua Fleming,** Director, Regulatory Innovation Office (RIO)
- **Professor Alejandro Frangi FREng,** Bicentenary Turing Chair in Computational Medicine, University of Manchester and Director, UK Centre of Excellence on In-Silico Regulatory Science and Innovation (UK CEiRSI)
- **Dr Felicity Gabbay FMedSci,** Senior Vice President, tranScrip
- **Sam Genway,** Scientific Director in Machine Learning and AI, LifeArc
- **Dr Vishal Gulati,** Founder and Managing Partner, Recode Ventures
- **Dr Philip Hines,** Associate Director, Life Sciences and Health Policy, IQVIA
- **Dr Felix Jackson,** Medical Director, medDigital
- **Ismael Kherroubi,** Participant Panel & Ethics Advisory Committee member at Genomics England and Founder, Kairoi
- **Natalia Kotchie,** Senior Vice President, R&D Applied Data Science Center, IQVIA
- **Raj Long,** Senior Advisor Consultant, Gates Foundation
- **Andrea Manfrin,** Deputy Director, Clinical Investigations and Trials, MHRA and Visiting Professor within the School of Pharmacy and Biomedical Sciences, University of Central Lancashire
- **Dr Gita Moghaddam,** Principal Investigator, Department of Clinical Neurosciences, University of Cambridge
- **Professor Andrew Morris CBE FRSE PMedSci,** President, Academy of Medical Sciences
- **Professor James Naismith FRS FRSE FMedSci,** Head, MPLS Division and Professor of Structural Biology, University of Oxford and Vice-President of the Academy of Medical Sciences
- **Dr Dan O'Connor,** Director, Regulatory Policy & Early Access, ABPI
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