AI, Regulation and Sustainability in Drug Development

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Agenda

1. **The regulatory landscape and how it is perceived**
   Timeline; survey on regulator awareness and guidance

2. **FDA's framework and the joint FDA and EMA principles**
   Context of use; seven-step credibility framework; ten principles

3. **Frameworks that apply to clinical trials**
   ICH E6(R3), E8(R1), M15, E11A; FDA's early-phase AI pilot

4. **Organizational readiness**
   Survey findings on governance, data, trust, challenges and skills

5. **Global regulatory approaches**
   EU, Canada, United Kingdom, China, Japan, Brazil

6. **Sustainability and recommendations**
   Environmental gap; recommendations for sponsors and vendors

Regulatory expectations for AI are emerging as principles and draft guidance



2021

Good Machine Learning Practice principles

FDA, Health Canada and MHRA

Sept 2024

EMA reflection paper on AI in the medicinal product lifecycle

Principles based

Jan 2025

FDA draft guidance on AI to support regulatory decision making

Still a draft as of September 2026

Jan 2026

Joint FDA and EMA Guiding Principles of Good AI Practice

Ten non-binding principles

Apr 2026

NMPA AI + Drug Regulation opinions; FDA request for information on AI in early-phase trials

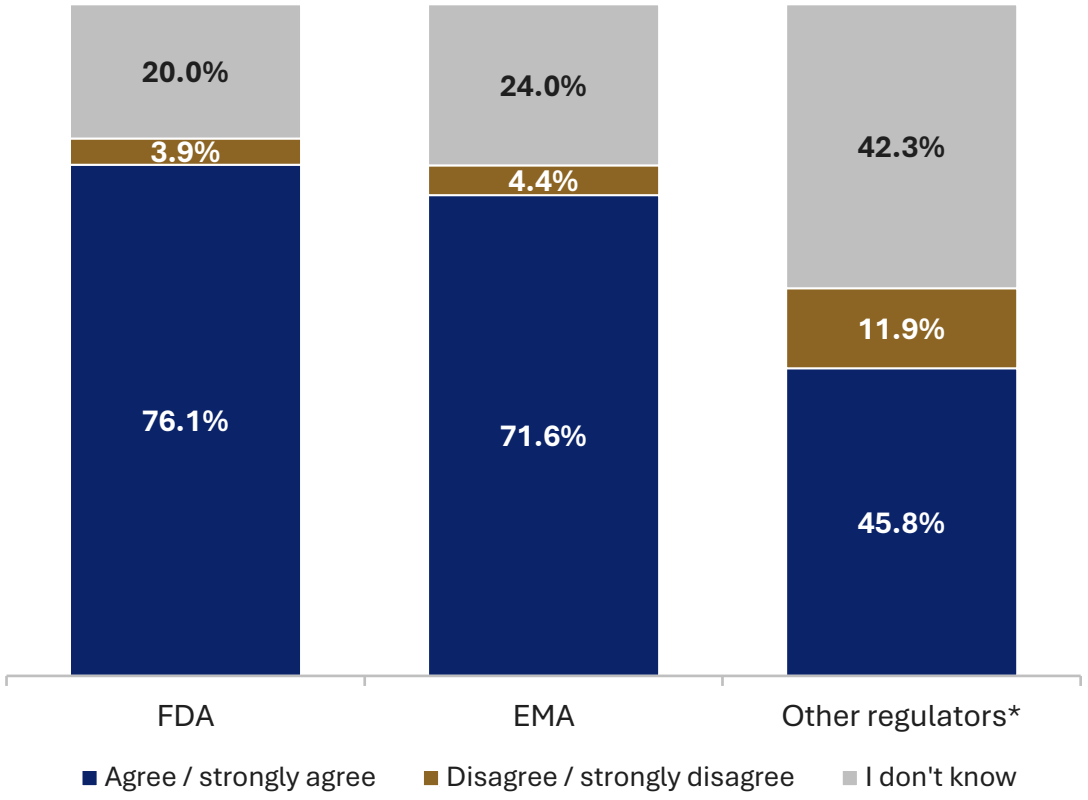
China; United States

More than 500 CDER submissions with AI components since 2016, and more than 560 at CBER through 2023

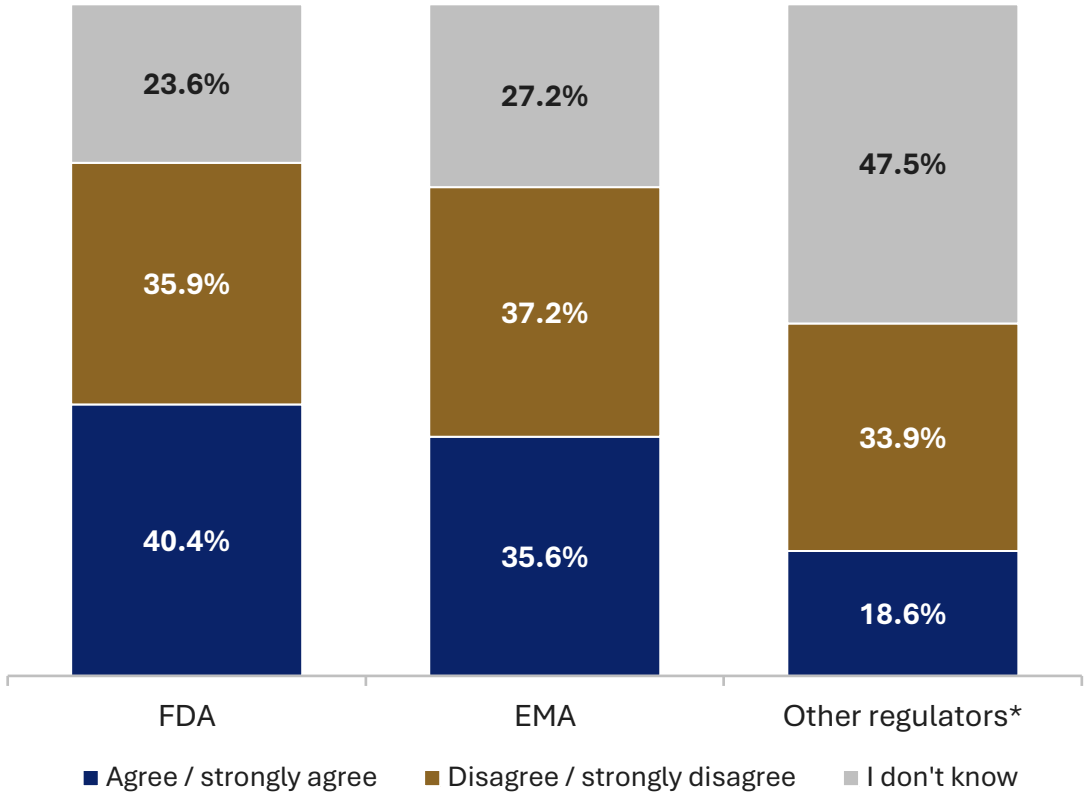
Sources: FDA; EMA; FDA and EMA joint principles (January 14, 2026); NMPA (April 2, 2026); Federal Register 91 FR 23100.

Respondents see regulators as aware of AI issues, but guidance as less specific

Health authority exhibits awareness of potential issues related to the use of AI/ML



Health authority offers specific guidance on the appropriate use of AI/ML



Source: Tufts CSDD AI Study (2025). Lamberti, Florez, Do, et al. n = 302. *Other regulators named: AIFA (Italy), ANVISA (Brazil), HPRA (Ireland), MHRA (UK), PMDA (Japan), Thailand Health Authority.

Context of use is the organizing concept of FDA's draft framework

Context of use (COU)

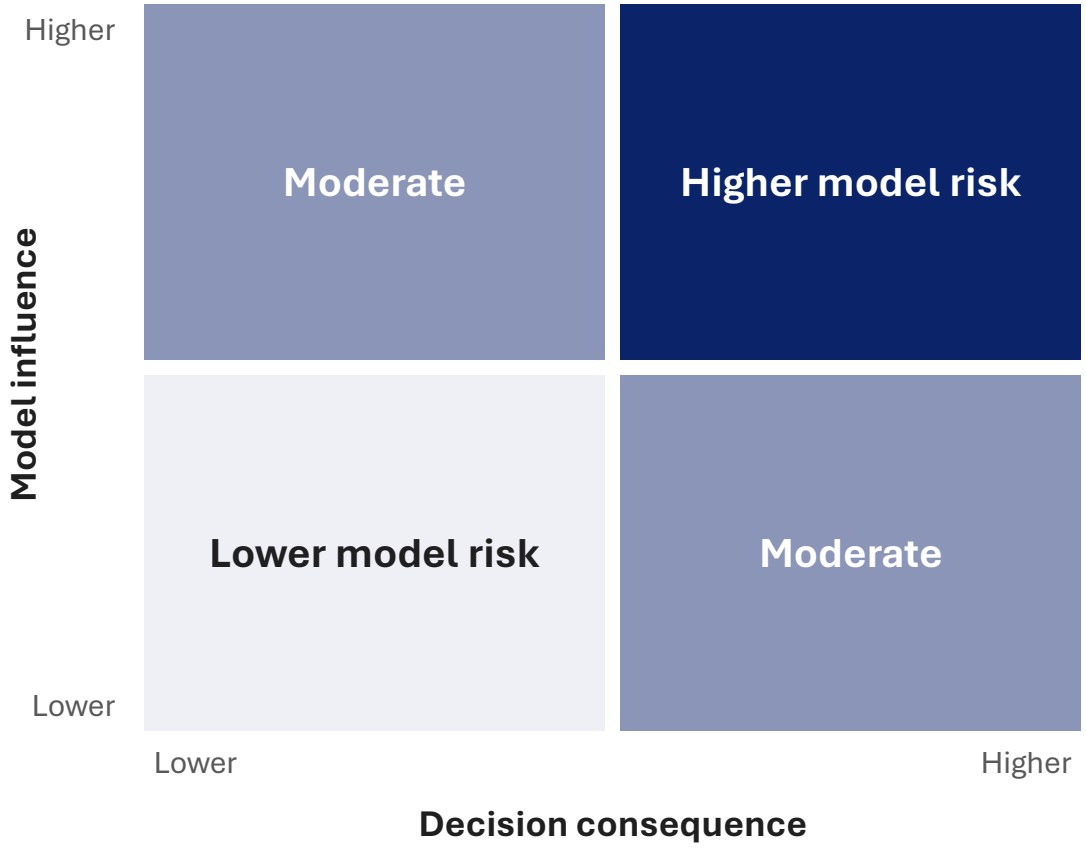
The specific role and scope of an AI model used to address a defined question of interest

Model risk depends on two factors

Model influence how much the AI output contributes to the decision, relative to other evidence

Decision consequence the significance of an adverse outcome if the decision is wrong

The rigor of credibility activities scales with model risk



Source: FDA, draft guidance on AI to support regulatory decision making for drug and biological products, January 2025.

FDA's draft guidance sets out a seven-step credibility framework

DEFINE		ASSESS	ESTABLISH			
1	2	3	4	5	6	7
Define the question of interest	**Define the context of use**	**Assess AI model risk**	**Develop a credibility assessment plan**	**Execute the plan**	**Document the results**	**Determine adequacy**
The decision or concern the model addresses	The model's role, scope and how its output is used	Model influence and decision consequence	Commensurate with model risk	Carry out the planned activities	Report results and any deviations from the plan	Is credibility sufficient for the context of use?

If credibility is not adequate, the framework allows iteration: add evidence, increase the rigor of the assessment, add controls, or revise the model or its context of use

Source: FDA, draft guidance FDA-2024-D-4689, January 2025. Still labeled a draft as of September 2026.

FDA and EMA released ten joint guiding principles in January 2026

1. **Human-centric by design**
2. **Risk-based approach**
3. **Adherence to standards**
4. **Clear context of use**
5. **Multidisciplinary expertise**
6. **Data governance and documentation**
7. **Model design and development practices**
8. **Risk-based performance assessment**
9. **Life cycle management**
10. **Clear, essential information**

Non-binding. Applies across the life cycle, from research and clinical trials to manufacturing and safety monitoring.

Source: FDA and EMA, Guiding Principles of Good AI Practice in Drug Development, January 14, 2026.

Existing ICH guidelines already apply to AI used in clinical trials

E6(R3) Good Clinical Practice

EU effective July 2025; FDA final Sept 2025; Annex 2 EU effective Jan 2027

- Computerised system validation, access control and audit trails; data governance across the data life cycle
- Annex 2 covers decentralised elements, pragmatic elements and real-world data

E8(R1) General Considerations

Step 4 October 2021; FDA final April 2022

- Quality by design and critical-to-quality factors, operationalized in E6(R3)
- Covers non-interventional and real-world data designs

M15 Model-Informed Drug Development

Step 4 January 2026; published by FDA June 2026

- Scope explicitly includes AI and machine learning methods
- Pediatric extrapolation given as an example question of interest

Pediatrics E11A and EU reform

ICH E11A final 2024; EU pharma package agreed December 2025

- E11A: modeling, simulation and Bayesian extrapolation
- EU pharma legislation, which also reforms paediatric rules, accommodates broader use of AI (EMA)
- No FDA pediatric guidance specific to AI identified

Of these ICH guidelines only M15 names AI; AI tools in trials fall under E6(R3) computerised-system expectations

Sources: ICH E6(R3) and Annex 2; ICH E8(R1); ICH M15; ICH E11A; FDA and EMA implementation notices.

FDA is seeking input on AI in early-phase clinical trials

Published	April 29, 2026 (91 FR 23100); comments closed June 29, 2026
Scope	Phase 1 and early Phase 2 trials: patient selection, dose optimization, safety monitoring, go/no-go decisions
Standard	Trustworthiness of AI aligned with the NIST AI Risk Management Framework
Status	Request for information; no requirements or guidance created

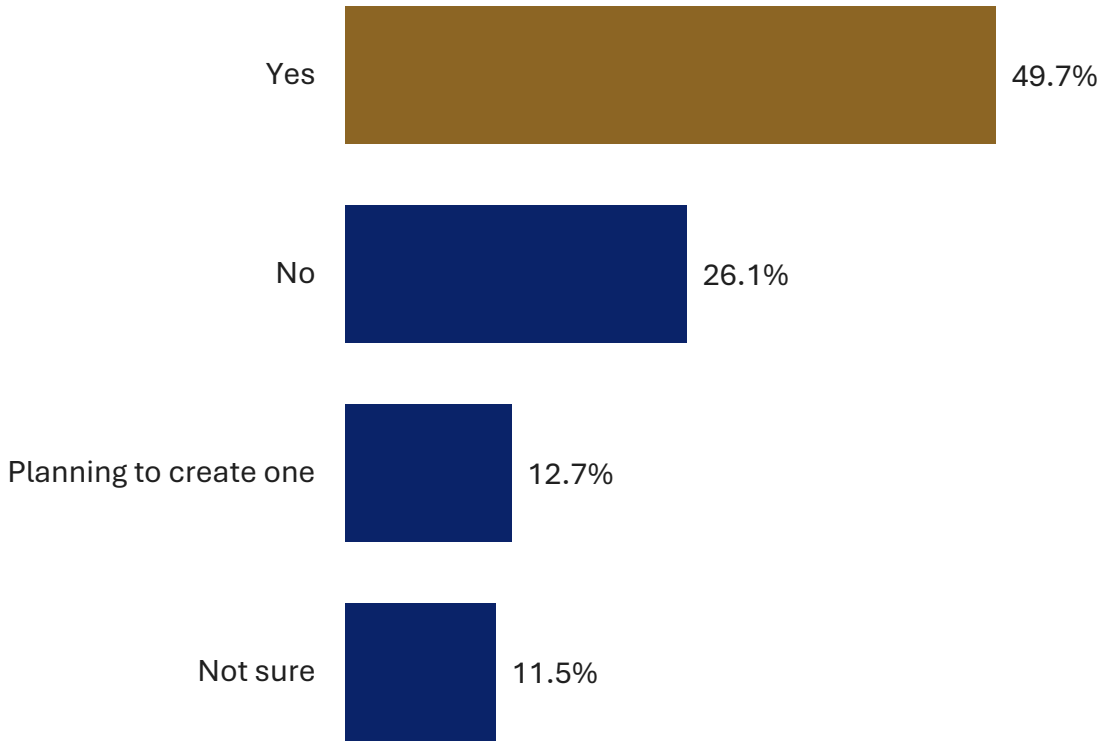
Evaluation metrics FDA asked about

- Qualitative outcomes
- Trial efficiency and speed
- Decision quality
- Participant safety and data integrity
- AI system performance
- Trustworthiness
- Comparative evaluation

Sources: Federal Register, 91 FR 23100 (April 29, 2026); extension notice FR Doc. 2026-10602 (May 28, 2026).

About half of respondents report a central AI governance function

Presence of an AI governance function



46% of those with a governance group place it within a central governance group; 30% have a stand-alone function

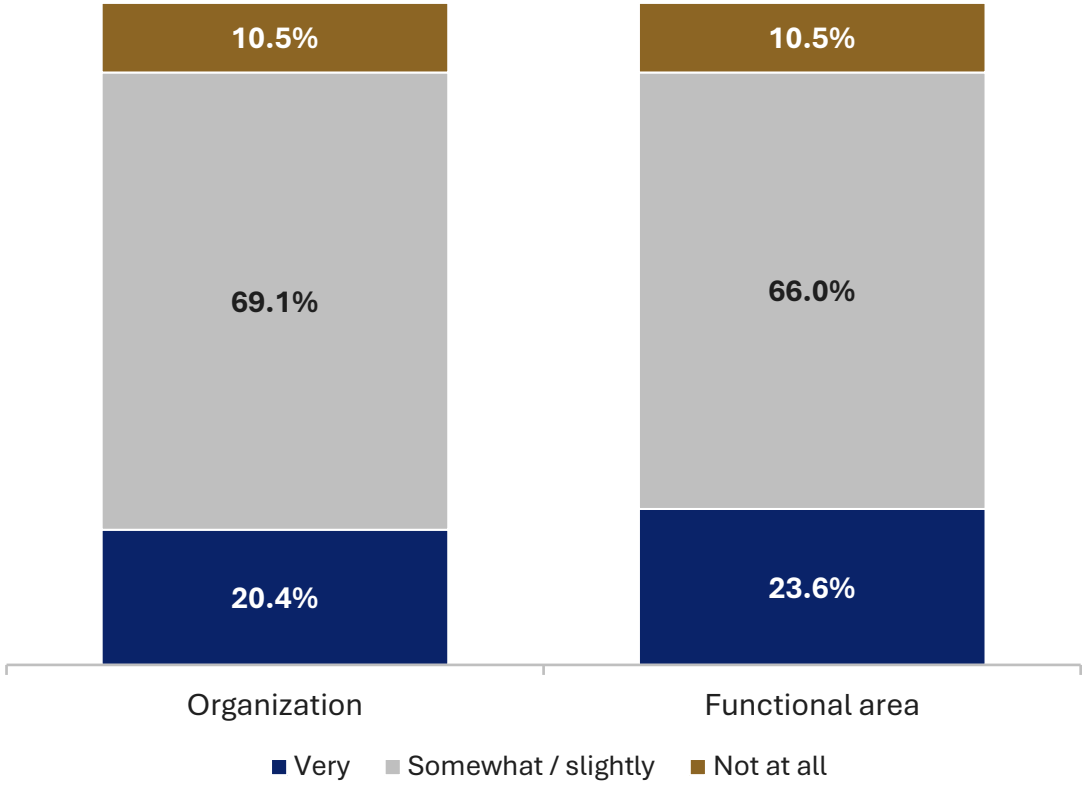
About 40% believe their governance function is effective; 32% are unsure

p < 0.05 Organizations with fewer than 25 trials were more likely to be planning a function; those with more than 100 were more likely to have one

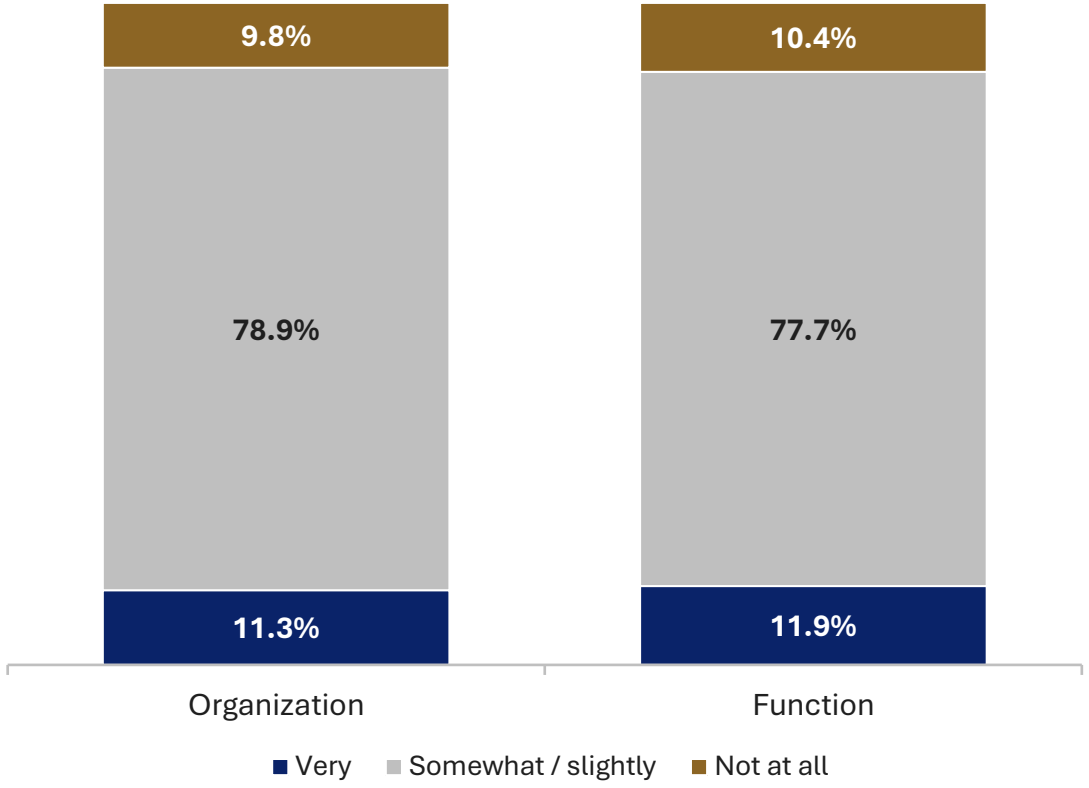
Source: Tufts CSDD AI Study (2025). Lamberti, Florez, Do, et al. n = 302.

Few respondents are very confident in training data or in AI over human judgment

How confident is your organization and functional area that the data used to train AI/ML is accurate or clean?



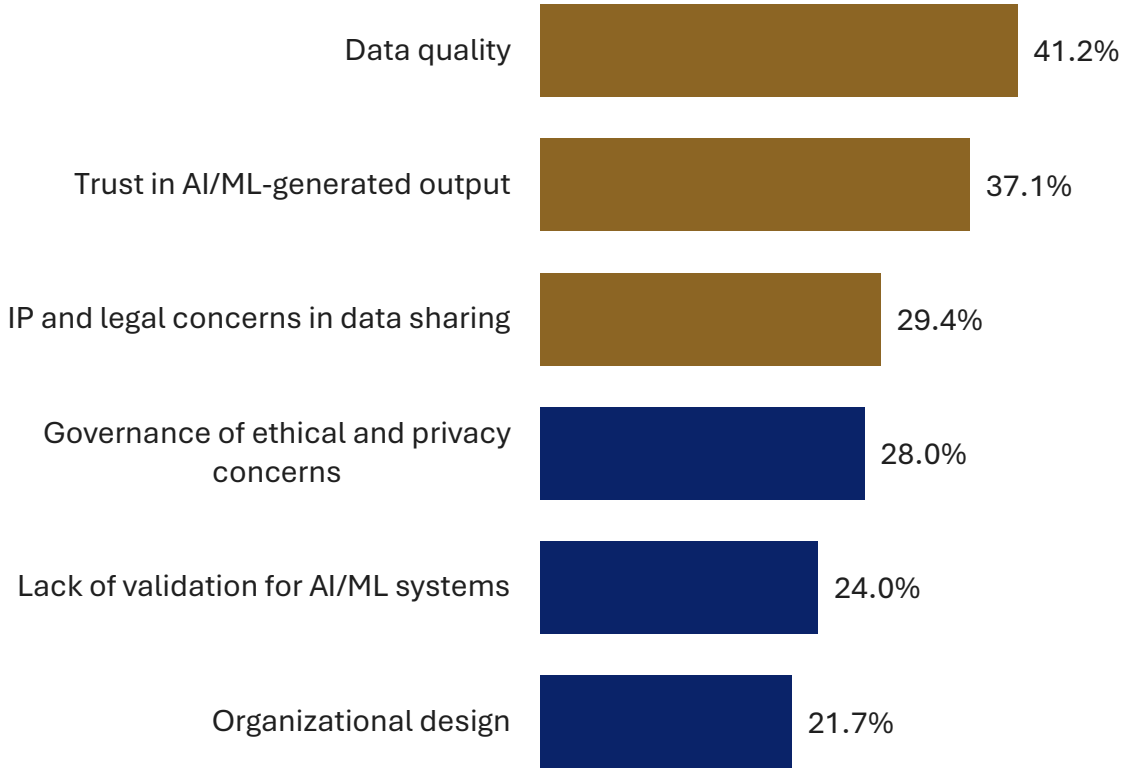
How likely is your organization and function to trust AI/ML recommendations over a human's?



Source: Tufts CSDD AI Study (2025). Lamberti, Florez, Do, et al. n = 302.

Reported challenges center on data and trust; respondents link trust to experience

Top reported challenges to AI/ML implementation

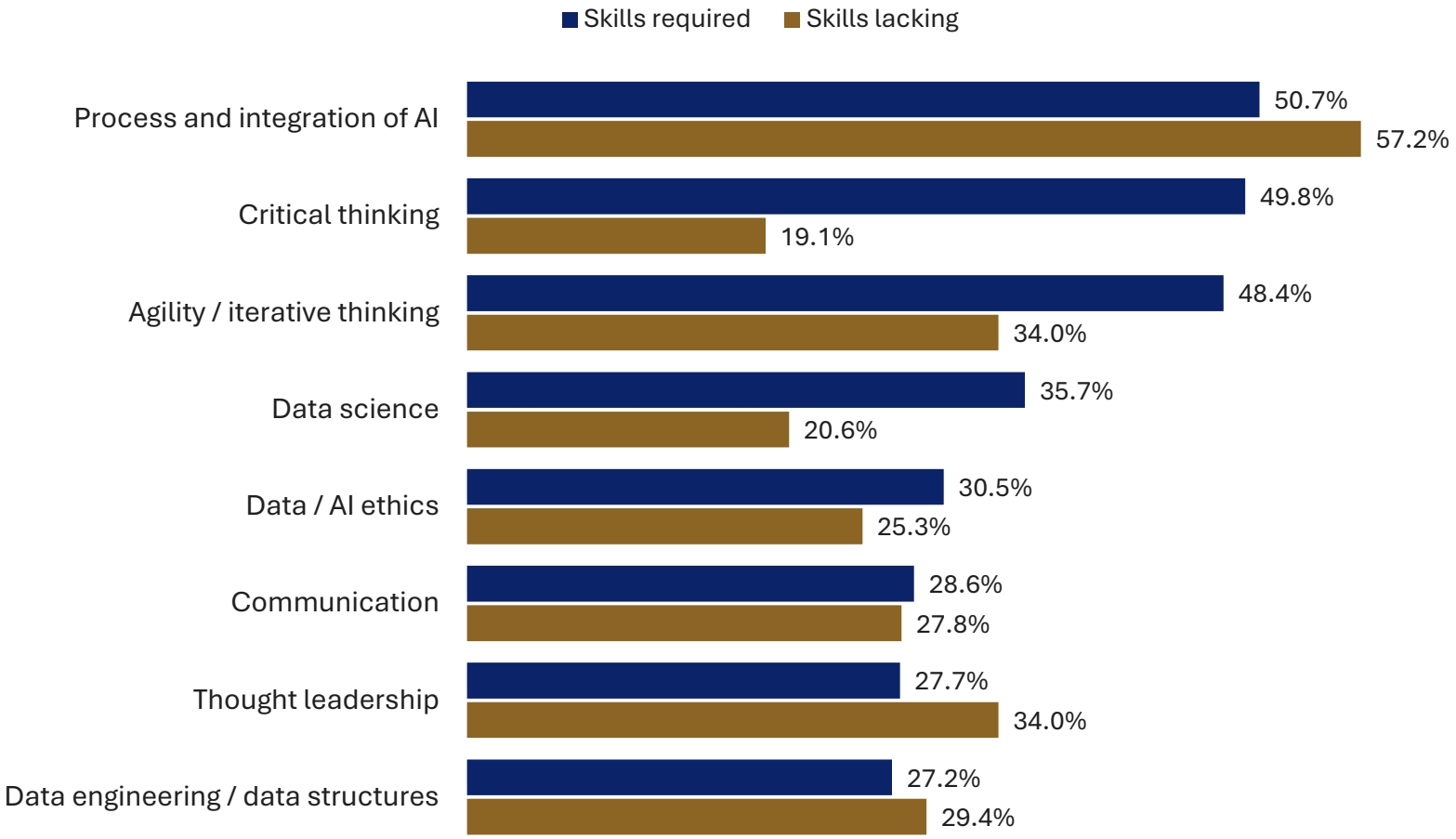


Top reported methods to increase trust in AI/ML



Source: Tufts CSDD AI Study (2025). Lamberti, Florez, Do, et al. n = 302. Multiple responses permitted; each bar is the share of all respondents.

The largest reported skills gap is in the process and integration of AI



57.2%

report lacking skills in the process and integration of AI, the most frequently required skill (50.7%)

Critical thinking

required by 49.8%, lacking for 19.1%

Source: Tufts CSDD AI Study (2025). Lamberti, Florez, Do, et al. n = 302. Multiple responses permitted; skills shown were asked in both questions.

FDA and EMA share principles; the EU is adding its own instruments

Shared FDA and EMA ground

- Ten shared principles covering research, clinical development, manufacturing and safety monitoring
- Risk-based approach anchored in a clear context of use
- Data governance, documentation and life cycle management as core expectations
- FDA's draft guidance adds a procedural seven-step framework; EMA's 2024 reflection paper is principles based

EU instruments and developments

- EU guideline development underway, building on the 2024 reflection paper
- First EMA qualification opinion for an AI tool (AIM-NASH, March 2025): locked model, requalification for major changes
- Pharma package agreed December 2025, applying around 2028; accommodates AI and controlled testing
- Proposed Biotech Act: shorter trial authorisation timelines, sandboxes, AI testing environments
- EU AI Act in parallel; high-risk obligations deferred to December 2027 and August 2028

Sources: FDA (2025); FDA and EMA (2026); EMA (AIM-NASH qualification, March 2025); EU pharma package; Biotech Act proposal; Regulation (EU) 2026/1744.

Canada and the United Kingdom: convergent principles, different instruments

Canada

- **GMLP principles** co-authored with FDA and MHRA (2021)
- **Canada's Drug Agency (CDA-AMC),** the HTA body: position statement on AI in evidence generation and reporting (April 2025)
- **CDA-AMC guidance** on AI methods to generate real-world evidence for HTA (July 2026)
- **Health Canada:** no guidance specific to AI in drug submissions identified

United Kingdom

- **AI Airlock:** regulatory sandbox for AI as a medical device; Phase 2 report June 2026, Phase 3 call October 2026
- **Medicines AI sandbox** (June 2026) on safety assessment and reducing animal testing
- **Draft guidance** on change control plans for adaptive AI devices expected by December 2026

Sources: CDA-AMC (April 2025; July 2026); MHRA AI Airlock Phase 2 programme report (June 2026); MHRA (2026).

China is building AI into regulation itself; others are still forming positions

China | NMPA, April 2, 2026

Implementation Opinions on "Artificial Intelligence + Drug Regulation"

2030 Integrated system for regulation and AI: regulatory datasets, vertical large models, AI agents, human-machine collaborative review

2035 A new paradigm of smart drug safety governance

Focus is the regulator's own operations, not a sponsor-facing framework

Japan | PMDA

AI Action Plan for internal operations (September 2025). A basic approach to AI use is planned for publication this fiscal year.

Brazil | ANVISA

About R$25 million invested in AI tools for internal review. No dedicated guidance on AI in drug development identified.

Sources: NMPA (April 2, 2026); PMDA AI Action Plan (September 2025); ANVISA (2025 and 2026).

Environmental sustainability is a gap in guidance and in practice

In drug regulators' guidance

FDA's draft guidance and the joint FDA and EMA principles do not address energy use or environmental impact

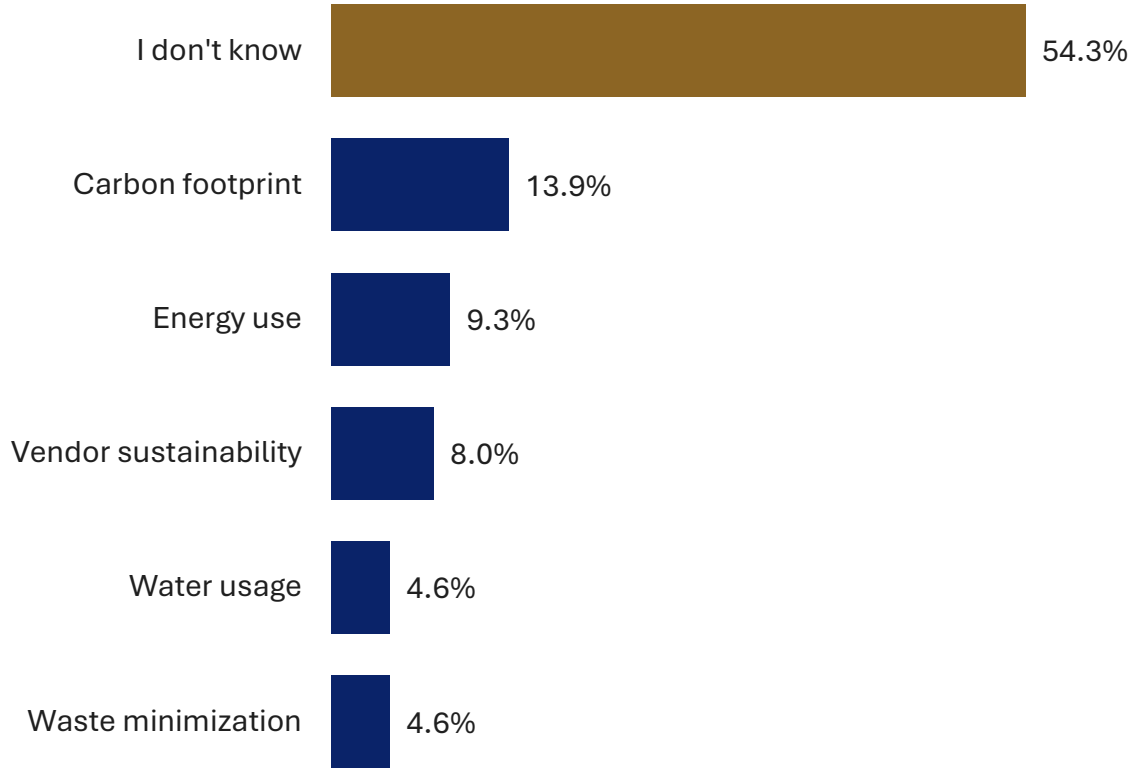
In other frameworks

EU AI Act: general-purpose AI model providers document computational resources and estimated energy consumption (Annex XI), in force since August 2025

In organizations

More than half of respondents do not know which metrics their company uses for AI-related sustainability goals

Metrics used to achieve company's AI-related sustainability goals



Sources: FDA (2025); FDA and EMA (2026); Regulation (EU) 2024/1689, Annex XI. Source: Tufts CSDD AI Study (2025). Lamberti, Florez, Do, et al. n = 302.

Recommendations for sponsors

		Basis
1	**Map AI uses by context of use and model risk**	FDA draft framework; joint principles 2 and 4
2	**Establish or formalize cross-functional AI governance**	49.7% report a central function; 12.7% are planning one
3	**Document data provenance and performance across the life cycle**	20.4% very confident in training data; principles 6 and 9
4	**Engage regulators early on novel uses**	FDA draft guidance encourages early engagement
5	**Plan for jurisdictions that diverge**	EMA guideline development; EU AI Act; China's NMPA
6	**Build integration skills and define sustainability metrics**	57.2% lack AI integration skills; 54.3% unaware of metrics

Recommendations for vendors

		Basis
1	**Provide documentation that supports sponsors' credibility assessments**	Data lineage, versioning and performance evidence; FDA steps 4 to 6
2	**Support the sponsor's context of use analysis**	What the tool informs and the consequence of error
3	**Report validation evidence**	Lack of validation 24.0%; trust in output 37.1%
4	**Design for human oversight and explainability**	11.3% very likely to trust AI over a human; principle 1
5	**Disclose energy and resource use where known**	EU AI Act Annex XI; 8.0% of respondents track vendor sustainability
6	**Map products to the ten joint principles**	Principles apply across the life cycle

Summary

1

Converging, not yet binding

Regulators share core principles (risk, context of use, life cycle), but most expectations remain principles or draft guidance and differ across jurisdictions

2

A perceived guidance gap

Respondents see FDA and EMA as aware of AI issues, but fewer than half agree they offer specific guidance; readiness on governance, data and trust is partial

3

Sustainability is largely unaddressed

Drug regulators' AI guidance does not address environmental impact, and most respondents do not know their company's metrics

Questions and discussion