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AI Ethical Considerations in Drug Development and Regulatory Review

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ABSTRACT

The hype surrounding ChatGPT has persisted for some time, prompting significant discussion about ethical implications of AI and Generative AI. As our understanding of these challenges evolves, it's crucial for us to assess how this deeper insight affects the development and implementation of AI technologies in drug development and regulatory review and what are the challenges. As both a regulator and user of AI, the FDAss needs to ensure that AI technologies in healthcare are safe, effective, and ethically sound, while also using AI to enhance its own processes. This presentation will delve into some prominent issues impacting drug development and regulatory review, including algorithmic bias, transparency, data integrity, and patient privacy protection, etc. Additionally, we will discuss some immediate steps that stakeholders can take to ensure transparency and auditability throughout the AI lifecycle, thereby maintaining public trust and upholding ethical standards in drug development and review.

INTRODUCTION

In Gartner's CEO Survey for 2025, 74% of CEOs responded that AI will have the most significant impact on their industries over the next three years. Leaders who are deploying AI across multiple business functions saw 40% higher ROI on their AI investments compared to less mature peers. However, 60-75% of enterprises haven't really matured their AI Strategy and capabilities to generate value. Achieving the full potential value from AI requires sophisticated governance, a clear strategy and the right enablers. According to Gartner, 85% of AI projects fail due to three critical gaps - strategic misalignment, trust deficits, and organizational readiness. However, in 2025, leaders are pushing their teams to innovate with AI in 2025.

AI technology has had a profound impact on various industries, and drug development and regulatory review are no exception. The utilization of AI in these sectors has not only accelerated processes and enhanced accuracy but has also brought about a paradigm shift in decision-making. However, it is crucial to acknowledge the ethical implications that come hand in hand with the integration of AI in these fields to ensure its ethical and responsible application.

DOING THE RIGHT AI AND DOING AI RIGHT

The pursuit of AI trustworthiness has transcended into a multidisciplinary field, underscoring the criticality of prioritizing user requirements and adopting an ethical approach encompassing people, processes, and technology. While it is a technical challenge, organizations should take a human-centered, sociotechnical approach to implementing AI. To equip teams with the ability to leverage responsible AI technology effectively, regulatory reviews must be equipped with tools supporting decision-making, strategic reskilling, security guardrails, and data-driven decision support.

In the realm of drug development, AI is proving instrumental in personalized medicine and drug discovery. By analyzing vast datasets, AI algorithms can identify potential drug candidates and predict patient responses, thereby enabling more precise and customized treatments. Additionally, the integration of AI in clinical trials and regulatory procedures is enhancing operational efficiency. However, ethical challenges arising from AI's involvement in decision-making processes raise concerns about transparency, accountability, and bias.

Trust in AI-driven decisions hinges on five pillars: explainability, fairness, robustness, transparency, and privacy. Recent case studies highlight the successful application of AI in drug development, illustrating its transformative potential in the pharmaceutical sector. Yet, ethical debates surrounding AI bias and data privacy persist as significant obstacles requiring meticulous attention. Updates to regulatory frameworks for AI implementation in healthcare are crucial to ensure the ethical and responsible deployment of AI technologies in drug development and regulatory review processes.

Establishing AI trustworthiness within an organization necessitates the implementation of a comprehensive AI governance framework. This framework facilitates the responsible design, development, and utilization of AI technologies, ensuring the incorporation of trustworthiness considerations across the entire AI lifecycle—from strategy and planning to development, operations, and monitoring. Organizations cannot achieve AI governance

without aligning it with their culture, processes, and technological infrastructure.

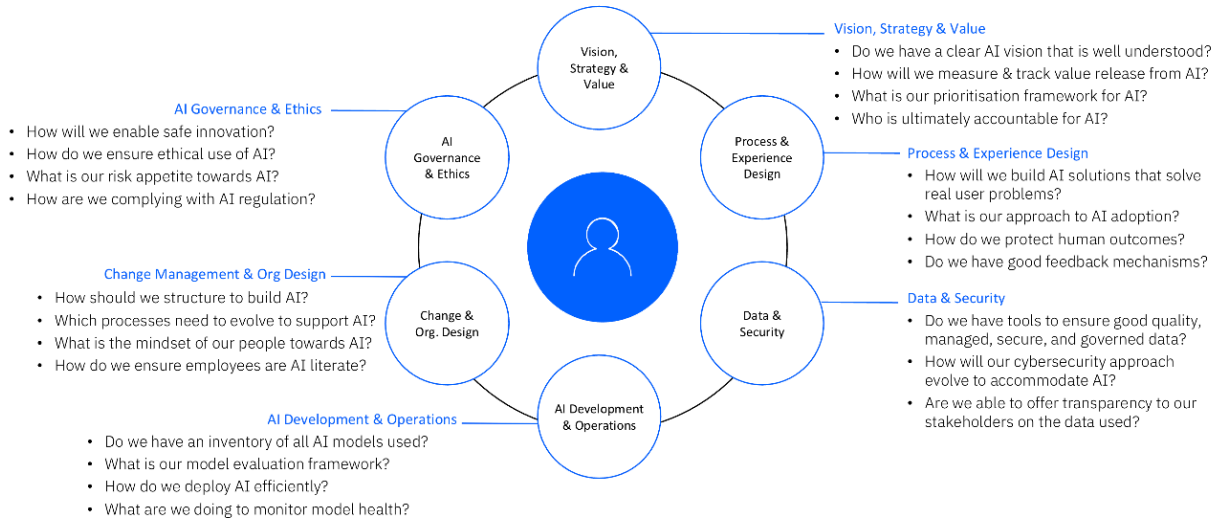

Figure 1. AI Governance Framework

AI ADOPTION IN DRUG DEVELOPMENT AND REGULATORY REVIEW

Pharmaceutical companies are increasingly investing in AI to enhance research and development, while regulatory bodies are leveraging AI to streamline review processes, accelerate approvals, and ensure drug safety. Key applications include:

- **Drug discovery and development**: AI models can predict potential drug candidates by analyzing biological data, molecular structures, and protein interactions. This accelerates the identification of promising compounds and reduces costly failures in later development stages.
- **Clinical trial optimization**: AI algorithms help identify suitable patient populations, predict trial outcomes, design more efficient protocols, and monitor patient safety signals in real-time.
- **Regulatory submission review**: Regulatory bodies use AI to analyze the vast documentation submitted for drug approvals, identifying inconsistencies, potential safety concerns, and compliance issues more efficiently.
- **Post-market surveillance**: AI systems continuously monitor real-world data to detect adverse events, drug interactions, and efficacy concerns that weren't apparent during clinical trials.
- **Manufacturing quality control**: Computer vision and machine learning systems monitor pharmaceutical production lines to detect defects and ensure consistent product quality.
- **Supply chain optimization**: Predictive analytics improve inventory management, reduce shortages, and enhance distribution efficiency.
- **Public communication**: AI rapidly analyzes vast amounts of health information, creating personalized educational content across literacy levels, monitoring for misinformation, and delivering transparent safety updates
- **Back-office operations**: AI automates complex document processing, accelerates financial reconciliation, streamlines regulatory compliance workflows, and enables predictive maintenance of manufacturing equipment - ultimately reducing costs and human error while increasing operational efficiency.

These AI applications are helping address longstanding challenges in both drug development and regulation, potentially reducing the time and cost of bringing new treatments to market while maintaining or improving safety standards.

EMERGENCE OF AGENTIC AI

The emergence of Agentic AI represents a pivotal shift in AI capabilities, particularly within highly regulated industries such as pharmaceuticals. Unlike traditional AI systems that operate within narrowly defined parameters, agentic AI demonstrates unprecedented levels of autonomy, utilizing multiple specialized agents that collaborate to pursue complex objectives. One significant application of Agentic AI in pharmaceuticals is the automation of regulatory submission creation. The process of compiling new drug applications is essential but notoriously time-consuming and prone to errors. Traditionally, pharmaceutical companies have relied on labor-intensive, manual efforts to compile extensive reports, leading to inefficiencies, inconsistencies, and high costs. Key challenges include:

- **Labor-Intensive and Prone to Errors**: Regulatory modules and report writing demand significant human effort, requiring skilled professionals to spend months compiling extensive documentation.
- **Inconsistencies and Quality Issues**: Manual processes often lead to discrepancies, causing delays and compliance risks.
- **Prolonged Time to Market**: Inefficiencies in report preparation and the extensive review process, exacerbated by inconsistencies, slows down drug approvals, delaying critical treatments for patients.

IBM's Intelligent Content Creation solution based on Agentic AI addresses these inefficiencies by leveraging a multi-agent system that automates and optimizes regulatory submission creation. This system consists of various AI-driven agents, Writer Agent, Evaluator Agent, and Retrieval Agent, that work in unison to extract, summarize, validate, and refine contents.

The Agentic AI system functions through a synergy of autonomous agents and human experts:

1. **Source Data Processing**: AI retrieves and structures data from regulatory documents.
2. **Multi-Agent Collaborate to produce draft reports**:
    a. The **Extractive Summary Agent** gathers key data.
    b. The **Abstract Summary Agent** synthesizes information and creates cohesive narratives.
    c. The **Evaluation and Feedback Agents** assess quality and compliance and provide feedback.
    d. The **Traceability Agent** maintains document lineage for transparency.
    e. The **Agent Group Chat Agent** facilitates communication between specialized agents.
3. **Human, Augmented by AI Assistant Review** AI-generated drafts, providing feedback to refine the output.
4. **Final Submission and Approval**: The optimized report is submitted for final regulatory review and approval.

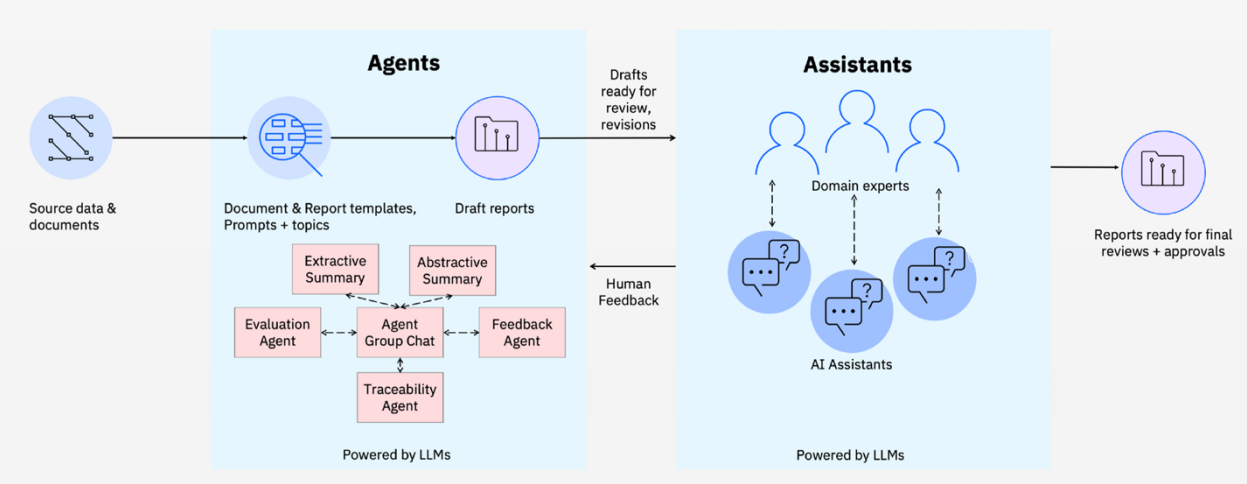

Figure 2. Agentic AI

Through the Agentic AI Intelligent Content Creation solution, IBM has helped the pharmaceutical company and regulatory agency benefit from:

- **Reduced Costs**: Automation cuts operational expenses related to document preparation and compliance.
- **Enhanced Efficiency**: AI accelerates the approval process, ensuring that life-saving drugs reach patients sooner.
- **Improved Compliance and Transparency**: AI-driven traceability ensures adherence to regulatory standards, reducing the risk of rejections and rework.

AGENTIC AI ADDRESSES ETHICAL ISSUES WHILE INTRODUCING NEW CHALLENGES

Agentic AI presents great opportunities in addressing some ethical concerns on AI, including transparency and hallucination concerns:

1. **Enhanced Transparency**: Agentic AI systems that employ chain-of-thought reasoning make their decision-making processes more visible and interpretable to users and regulators, increasing accountability.
2. **Reduced Misinformation**: Dedicated verification agents can cross-check facts and monitor for hallucinations in real-time, significantly improving information reliability in sensitive domains like healthcare and finance.
3. **Improved Autonomy in Decision Support**: In pharmaceutical settings, agentic AI can autonomously

navigate complex research literature and regulatory documents to provide more accurate guidance with clear reasoning traces.
4. **Ethical Reasoning Capabilities**: Specialized ethics agents can evaluate potential actions against established ethical frameworks, helping ensure compliance with industry standards and human values.
5. **Self-Correction Mechanisms**: Agentic systems with critic agents can identify their own limitations and mistakes, leading to more trustworthy outputs in high-stakes scenarios like drug safety evaluation.

While this evolution promises remarkable improvements in transparency and reduced misinformation, it simultaneously introduces profound ethical challenges related to decision-making autonomy, responsibility attribution, and potential emergent behaviors:

1. **Amplified Autonomous Action**: Higher levels of autonomy introduce questions about appropriate boundaries for AI decision-making, especially in regulated environments where human oversight has been traditionally required.
2. **Responsibility Attribution Complexity**: When multiple specialized agents collaborate on tasks, determining accountability for errors becomes more complex for regulators and companies.
3. **Ethical Framework Misalignment**: Different agents within a system may operate under competing ethical frameworks, potentially creating inconsistent approaches to sensitive decisions.
4. **Illusion of Infallibility**: The sophistication of agentic systems may create overconfidence in their outputs, potentially reducing critical human review of important communications or decisions.
5. **Privacy Boundary Navigation**: More autonomous systems may make independent judgments about what data to collect or analyze, creating new privacy concerns without explicit human direction.
6. **Regulatory Framework Gaps**: Existing regulations rarely address the unique capabilities and risks of agentic systems, creating uncertainty around compliance requirements.
7. **Emergent Behaviors**: Complex interactions between specialized agents may produce unexpected behaviors that weren't apparent during testing, particularly in open-ended communication scenarios.

Before implementing a novel automation system such as agentic AI in mission-critical functions, it's essential to explore potential risks through a human-centered design thinking framework. Risk mitigation cannot be left to technical means alone. Use of AI in organizations requires human accountability and funded mandates for effective governance. Teams conducting risk-mitigation exercises should represent a wide range of roles, opinions, experiences and ideologies; the more diversity is increased, the more error is reduced. Conducting design thinking exercises can also help stakeholders understand the scope and impact of implementing agentic AI in various use cases.

As pharmaceutical companies and regulatory bodies begin integrating these advanced AI systems into critical workflows spanning research, compliance, and public communication, establishing appropriate governance frameworks and guardrails for its autonomous capabilities becomes essential to harness their transformative potential while mitigating novel risks.

CONCLUSION

The convergence of AI and ethics in drug development and regulatory review offers a unique opportunity to propel healthcare forward while addressing critical ethical considerations. By having a holistic human-centered, sociotechnical approach to implementing AI in healthcare, fostering dialogue and collaboration among stakeholders, and advocating for transparency and accountability, we can navigate the intricate ethical terrain of AI in drug development and regulatory review. Together, we can harness the potential of AI to foster innovation while upholding ethical standards in healthcare.

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