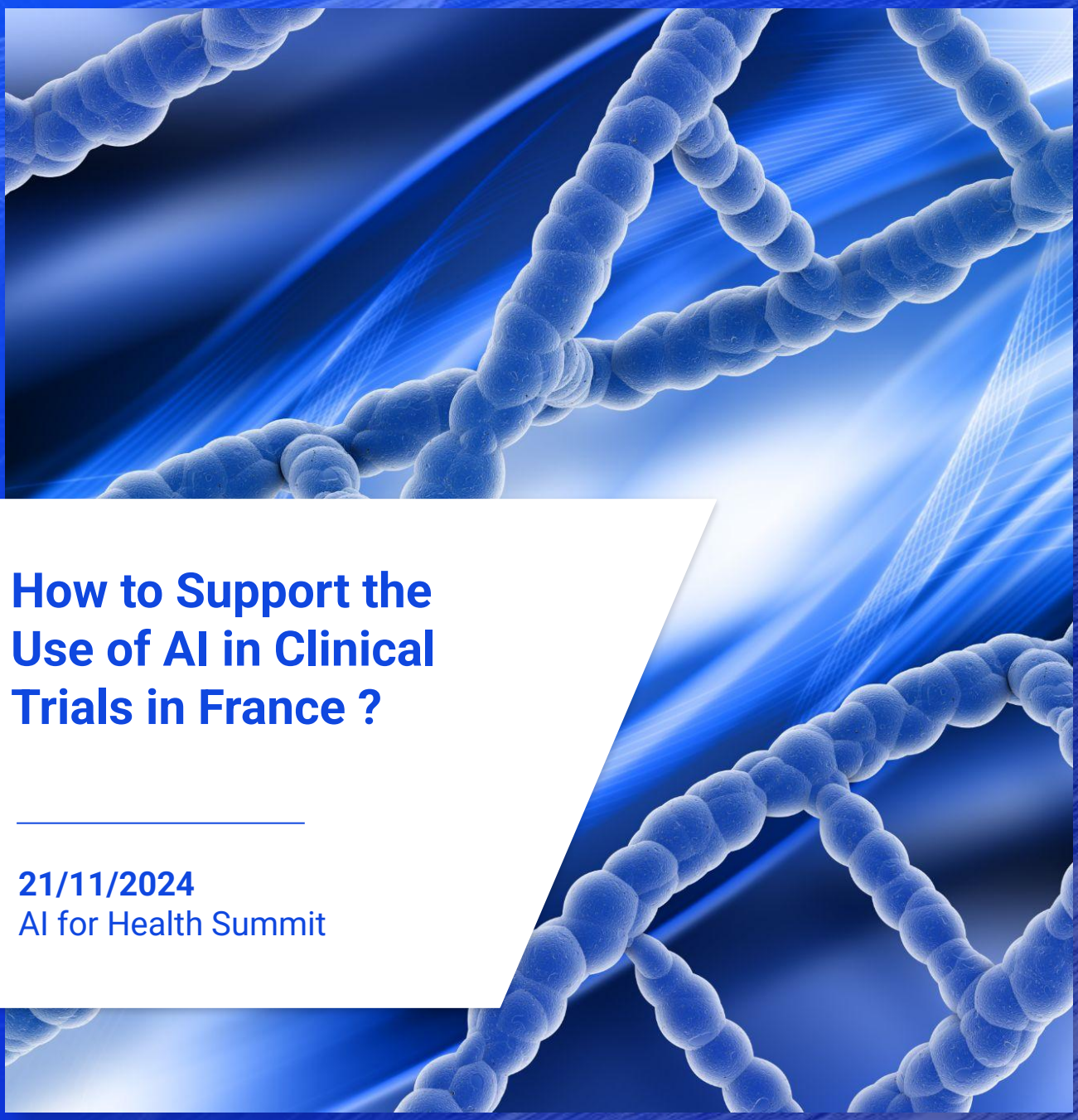
WHITE PAPER

AI FOR HEALTH



How to Support the Use of AI in Clinical Trials in France ?

21/11/2024
AI for Health Summit

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Executive Summary

Key points of the white paper summarized here

Artificial intelligence (AI) could play a pivotal role in transforming clinical trials, a cornerstone for developing new treatments and medications. With the potential to improve recruitment, optimize complex data analysis, and reduce costs, AI represents a promising opportunity to accelerate these processes. France, with its rich ecosystem of health data, is well-positioned to harness this potential and promote the use of AI in implementing clinical trials.

However, several **challenges and obstacles** need to be addressed:

- **Incentive Framework for Development :** A lack of attractiveness could hinder the emergence of innovative projects in France.
- **Awareness :** Limited awareness of AI's potential benefits in clinical trials may hinder the engagement and support of healthcare professionals, researchers, and citizens.
- **Patient Trust :** A lack of transparency about research methodologies, objectives, and the handling of personal data can lead to mistrust, complicating recruitment efforts.
- **Data Quality & Governance :** Challenges related to the quality, reliability, representativeness, and governance of health data used.
- **Validation Framework for AI Methods :** The need for clarity on methodologies to evaluate the effectiveness and functioning of AI systems.
- **Collaboration :** The lack of sufficient mechanisms for cooperation and coordination may result in delays and misalignment between protocols and actual patient needs.

We propose the following **solutions**, which are detailed further in the white paper:

- **Incentive Framework for Development :** Establish an effective incentive framework to encourage clinical trials that integrate AI systems.
- **Awareness Among Healthcare Professionals and Researchers :** Conduct awareness campaigns, such as workshops, to highlight AI's contribution to clinical research. Develop and disseminate a classification of AI use cases in clinical trials.
- **Patient Trust & Involvement :** Include patient associations in the design of protocols. Engage patients through working groups to help them understand research protocols and new methodologies. Create informational and consent documents to improve literacy about AI applications in healthcare.
- **Data Quality & Governance :** Accelerate the implementation of health data repositories by strengthening their governance to facilitate data availability in compliance with regulations. Enhance the representativeness of data used for AI algorithms.
- **Validation Framework for AI Methods :** Develop clear criteria to evaluate the accuracy, transparency, and robustness of AI systems.
- **Collaboration :** Establish a working group bringing together professionals and regulators to map use cases and support the implementation of AI in clinical trials.

With its particularly rich health data repositories and fertile ground for innovation, France has the necessary assets to become a global leader in integrating AI into clinical trials. By addressing the identified challenges and mobilizing its resources, the country could strengthen its role in advancing therapeutic treatments for the benefit of patients.

OVERVIEW

AI FOR HEALTH

Introduction

AI for Health Summit - 7th edition
November 21 at Station F
Collective intelligence workshop*

**Methodology available in the appendix*

AI can serve as a powerful tool to optimize the various processes involved in conducting clinical trials, which are essential for developing new treatments and medical devices. The primary objective of clinical trials is to **evaluate the safety and efficacy of a healthcare product**—be it a drug, medical device, or cell and gene therapy—on healthy or ill volunteers. If the results are conclusive in France, the product can receive **marketing authorization (MA)** issued by the **French National Agency for Medicines and Health Products Safety (ANSM)**.

Clinical trials are organized into several successive phases:

- **Preclinical Phase**: This preliminary phase uses in vitro models and animal testing to **evaluate initial hypotheses regarding the safety and efficacy of a treatment**. If the results are promising, the next phase begins.
- **Phase I**: This phase involves administering the molecule to humans for the first time, typically to a small group of volunteers. The goal is to **assess the toxicity and kinetics of the substance** in the body through comprehensive testing.
- **Phase II**: This phase targets ill volunteers to **analyze the molecule's tolerance and efficacy**, particularly focusing on identifying the minimum effective dose with a reduced risk of adverse effects.
- **Phase III**: This phase broadens the participant pool to **evaluate the product's therapeutic value** through comparative trials.
- **Phase IV**: Conducted after market authorization, this phase **monitors the product's long-term use under real-world conditions**, identifying potential rare side effects or late complications.

Framework for Clinical Trials and Health Data Ecosystem in France

In France, the **conduct of clinical trials is strictly regulated** to ensure both participant safety and respect for their rights. Before commencing, clinical trials must receive favorable opinions from a **Committee for the Protection of Persons (CPP)**, authorization from the ANSM, and undergo formalities with the French Data Protection Authority (CNIL). These formalities may involve a declaration of compliance with a standard methodology or, failing that, obtaining explicit authorization from CNIL.

These steps ensure that the methodologies used, the anticipated benefits, and potential risks meet ethical and scientific standards. They also verify that participants are fully informed about the study's objectives, procedures, benefits, constraints, and the handling of their personal data. Participants must receive a detailed information document and provide free and informed consent before participating. This process aims to ensure transparency and build the trust essential for conducting rigorous clinical trials.

Initiatives in France's Health Data Ecosystem

France has a particularly rich health data ecosystem. The **National Health Data System (SNDS)**, which consolidates medico-administrative and hospital data, is renowned for its comprehensiveness and quality. Additionally, the **Health Data Warehouses (EDS)** housed within healthcare institutions, along with numerous national cohorts and registries, facilitate the implementation of a wide range of research projects. The **Platform of Health Data (PDS)** further enriches the French health data ecosystem.

In France, various institutions and stakeholders actively contribute to the health data ecosystem.

Since 2023, the **French Agency for Innovation in Health (AIS)** and the **French Clinical Research Infrastructure Network (F-CRIN)** have been leading a working group tasked with defining the framework for using new clinical research methodologies as complements to traditional randomized controlled trials. This group brings together around 30 experts from regulatory agencies (e.g., **DGOS, ANSM, CNIL**), healthcare institutions, public research organizations, biotech and medtech companies, as well as digital health and AI firms. The group focuses on three main themes:

- **Optimizing current clinical trial designs** (e.g., combined trials, platform trials).
- **Incorporating external information** (e.g., Bayesian trials, digital health/EHR data).
- **Extrapolating benefit-risk assessments** (e.g., using external control groups like registries or cohorts, or mechanistic models such as synthetic arms or "*in silico*" trials).

Several works have already been published, and the group continues its work in preparation for a call for expressions of interest to select pilot cases.

In 2024, the **CNIL** (France's data protection authority) published several practical guides offering recommendations on developing and using AI, as well as FAQs addressing the interplay between the AI Act and the General Data Protection Regulation (GDPR), and the use of generative AI systems. Specifically regarding health data processing, CNIL authorized over 30 studies involving AI systems. It also launched a consultation in 2024 to update its reference frameworks in collaboration with relevant stakeholders, particularly to account for AI developments. Initial results from this consultation, shared publicly through webinars, highlighted necessary updates to existing frameworks and additional needs within the ecosystem. Responding to the expressed need for guidance, CNIL will soon publish practical guides specifically for using AI in healthcare. Additionally, further collaboration with the ecosystem will be essential to adapt existing frameworks to the unique requirements of AI applications, ensuring their development while respecting individuals' privacy.

A public consultation was held from September 30 to November 5, 2024, on a national strategy for the secondary use of health data. Conducted by the **Ministry of Health and Access to Care** in collaboration with key stakeholders, this initiative aimed to frame and optimize the use of health data while ensuring an ethical and secure framework. This effort aligns with the European Health Data Space (EHDS) regulation and France's ambitions for a performant and innovative digital health ecosystem. Following this consultation, the strategy document, *Interministerial Strategy to Build Our National Health Data Heritage 2025–2028*, was published, outlining four key areas to address with specific actions.

Most recently, the **Digital Health Delegation (DNS)** organized the 4th edition of the *Assises Citoyennes du Numérique en Santé* on January 22, 2025, focusing on the European Health Data Space (EHDS). Recommendations from the citizen committee help guide DNS decisions. The committee concentrated on the EHDS regulation, adopted in April 2024, which aims to harmonize the use of health data in two areas: primary use for direct patient care and secondary use for research, innovation, and policymaking. For further insights, refer to our white paper, *How to Make the European Health Data Space an Asset for France*?

The **secondary use of health data**, which involves repurposing data for objectives distinct from their initial collection, serves as a critical driver for research, innovation, and improved care. Current applications include designing algorithms to detect certain diseases early, conducting real-world studies to evaluate treatment outcomes, and creating dashboards for managing health crises. This approach relies on a broad definition of health data, consistent with the **GDPR**, encompassing medical, medico-administrative, and research data.

Use of AI for Clinical Trials

In this context of a robust health data ecosystem, **AI can position itself as a key tool to optimize clinical trials and enhance therapeutic approaches,** for example through the following use cases:

- Virtual arms: AI can facilitate the design of protocols that include virtual arms, where fictitious patient groups are simulated using predictive models, thereby reducing the number of participants needed.
- Digital twins: AI can create digital twins—virtual representations of specific patients based on their medical data—to predict treatment responses without directly exposing patients.
- Synthetic data: AI can generate synthetic data to enrich existing cohorts or train robust predictive models when real-world data is insufficient.

Beyond these innovations, AI can **streamline patient recruitment** by rapidly analyzing vast datasets to identify eligible candidates. It can also enable real-time monitoring of participants through connected devices, quickly detecting adverse effects or anomalies. Moreover, AI can personalize trial protocols based on collected data, offering a more flexible and tailored approach. It also helps identify and **correct biases** in trial design or collected data, thereby improving the validity of results.

AI further enhances the precision of analyses by **processing large volumes of complex data**. For instance, machine learning algorithms can identify subtle correlations between biological, genetic, or environmental factors that are difficult to detect using traditional methods. This capability deepens the understanding of underlying interactions and improves the reliability of conclusions drawn from clinical trials. Additionally, AI can model various scenarios to **predict the probable outcomes** of a trial before its launch, helping prioritize the most promising studies.

These approaches demonstrate how AI can make clinical trials more efficient, precise, and better suited to the challenges of contemporary medical research.

Recently, several regulatory agencies have explored the potential role of AI in developing therapies and new drugs:

- **European Medicines Agency (EMA)**: The EMA published a reflection paper inviting feedback on the use of AI across the entire lifecycle of medicines, from discovery to post-authorization monitoring. This document, developed in collaboration with various EMA committees, highlights promising applications such as reducing animal testing, optimizing patient recruitment for clinical trials, and enhancing pharmacovigilance processes. However, the EMA emphasizes a human-centered approach that adheres to existing regulatory requirements. The public consultation on this paper, launched on July 19, 2023, concluded on December 31, 2023.
- **Food and Drug Administration (FDA)**: The FDA also issued draft guidelines open to suggestions and comments on the use of AI to support regulatory decision-making for drugs and biologics. The public consultation, launched on January 6, 2025, remains open until April 7, 2025. These recommendations promote the responsible use of AI, with a focus on transparency, reproducibility, and reducing algorithmic biases.

These initiatives by major regulatory agencies underscore a shared commitment to exploring AI's potential while addressing implementation challenges to ensure the safety and efficacy of new therapies.

This deliverable, developed as part of the 7th edition of the AI for Health Summit, explores some challenges and solutions for better integrating AI into clinical trials in France.

For a more detailed analysis on enabling the secondary use of health data, including several sections on clinical trials and practical recommendations, we recommend the Marchand - Arvier report: Federate the actors of the ecosystem to unlock the secondary use of health data.

Challenges and Solutions
to Support the Use of AI in Clinical Trials

The integration of AI in clinical trials raises a series of complex challenges, summarized and grouped here into six major themes. These challenges are accompanied by proposed solutions from workshop participants, aimed at leveraging the promises of AI in this sector, particularly in France.

1. Incitative Framework for Development

As highlighted in the Draghi report, an overly strict regulatory framework can harm innovative initiatives. France and the EU have all the capacities to consolidate their positions as hubs of excellence in research. The integration of AI innovations requires closer collaboration between institutional and professional stakeholders. Barriers related to adoption costs and evaluation processes by health authorities also remain a central issue.

Proposed solutions: Establish an **effective incentive framework to develop clinical trials integrating AI through financial measures and by clarifying the interplay between various applicable regulations** depending on the qualification of the project: the clinical trials regulations, the medical devices regulation, provisions in the French Public Health Code, AI regulations (AI Act), as well as, in cases of health data reuse, the GDPR, the European Health Data Space regulation, and the "*Informatique et Libertés*" law. It could be advantageous to **encourage collaboration** with French and European regulatory authorities to consider **transversal actions** to be implemented. Similar to the pilot phase on decentralized and dematerialized trials launched by the ANSM, the Ministry of Health (DGS and DGOS), and the CNIL in 2024, a pilot phase on the use of AI in clinical trials could make it possible to: identify various use cases, support sponsors by establishing recommendations in collaboration with the ecosystem to promote their development and deployment, and map the uses. Finally, an incentive framework could be developed through work on the **economic model** of clinical trials using AI, taking into account the high entry costs and by valuing the data generated by AI and the companies that process it.

2. Awareness

The uses of AI and its benefits in clinical trials are not always sufficiently highlighted, which can hinder the engagement of citizens, patients, and healthcare professionals.

Proposed solutions: **Conduct awareness campaigns** to **promote the benefits** of AI in clinical research. These campaigns could rely on **awareness workshops** presenting concrete and robust use cases, thus strengthening the understanding and interest of the general public, patients, and professionals in the integration of AI in clinical trials. Consider as well **drafting and disseminating literature reviews** on AI use cases in clinical research.

3. Patient Trust

Clinical trials may encounter difficulties in recruitment, particularly for certain pathologies such as rare diseases, and a potential lack of transparency that can undermines patient trust. The modalities for using AI in clinical trials must be clearly explained to participants and framed to ensure transparent and fair use of AI systems.

Proposed solutions: **Include patient associations in the development of protocols** and **draft information and consent documents** for participation in research to improve literacy regarding the use of AI in health. Encourage sponsors to **highlight the contributions of AI** in clinical trials when sharing overall results.

4. Data Quality & Governance

In cases of reusing health data, for example, to expand cohorts or generate artificial cohorts, it is necessary to ensure the quality and representativeness of the reused databases to guarantee the methodological reliability of trials and the robustness of analyses. The European Health Data Space regulation requires data holders to provide the organization responsible for data access with the necessary documentation to justify the quality or utility label of their datasets. To facilitate the availability of this data and verify its quality, governance must be established. In some cases, the collected data may lack representativeness, particularly regarding patients with rare diseases or from underrepresented populations. The documentation on the quality and reliability of the data may also remain insufficient.

Proposed solutions: **In addition to a decentralized approach, accelerate the establishment of health data warehouses** suitable for integrating AI tools. These available databases must also be cataloged. Access to data could be facilitated, and **clear criteria** must be defined to assess their quality and reliability. The quality of available data must be strengthened, as well as the diversity of data sources, to ensure better patient representation. Additionally, **transparency in data processing and data security must be guaranteed**. Finally, collaboration with health authorities could help further highlight innovative methods. The Marchand - Arvier report provides in-depth context on the secondary use of health data in France and includes detailed proposed solutions. Furthermore, you can explore the different health data warehouses through the following mapping published by the CNIL.

5. Validation of AI Methods

In January 2025, the FDA published a set of recommendations open for comments and contributions on the use of AI for the development of drugs and biological products. This document proposes a risk-based evaluation framework to establish and assess the credibility of AI models in specific use contexts, particularly to demonstrate their safety, efficacy, or quality. Moreover, in 2024, the EMA published a reflection paper on the use of AI in the drug lifecycle, aiming to regulate its use, assess its risks, and promote harmonized standards at the European level.

Proposed solutions: **Develop specific standards and guidelines to define clear criteria** for evaluating the accuracy, transparency, and robustness of AI tools in clinical trials. Several aspects could be included: the **technical validation of models**, based on diverse and representative datasets, to ensure reliability and minimize biases; **clinical validation**, through comparisons with conventional approaches or human experts, to strengthen the credibility of AI tools by proving their relevance in real-world contexts; the **transparency of algorithms**, with clear documentation on their functioning and **methodological choices**, to establish lasting trust among professionals and regulators.

6. Collaborations and Synergies

Deeper collaboration among various stakeholders would positively contribute to the development of AI in clinical trials in France. The lack of cooperation and coordination mechanisms can cause delays and mismatches between protocols and the actual needs of patients.

Proposed solutions: **Facilitate the implementation of AI tools in collaborative projects** by strengthening exchanges between key stakeholders and providing technical and methodological support for their adoption. The development of **recommendations specific to various use cases**, including testable and adaptable regulatory and scientific aspects, could also help translate the rich legal framework into specific use cases to create operational recommendations. Additionally, **data and model sharing to encourage their reuse in other studies** would improve the quality of completed work. Establishing **working groups to map various use cases** involving different stakeholders could support the integration of AI innovations in a more practical manner. From a broader perspective, **disseminating best practices internationally**, maintaining **active monitoring of developments in other countries**, and **supporting French initiatives with an international scope** would be favorable for national projects in order to enhance their visibility.

Conclusion

AI could redefine the paradigms of clinical trials by making them faster, more precise, and more patient-centered. In France, a rich ecosystem of health data provides a solid foundation for maximizing the contributions of AI to clinical trials and taking full advantage of this revolution. However, some complex challenges remain, particularly concerning data quality and governance, regulation, attractiveness, patient trust, and incentive mechanisms. These challenges require a **collective, collaborative, structured, and proactive response**.

To address these challenges, it is essential to **strengthen alignment among stakeholders**: researchers, healthcare professionals, industry players, decision-makers, regulators, patient associations, innovators, and legislators. This alignment must **rely on transparent exchanges**, **concrete collaborative actions**, and **structured initiatives**. The development of an **incentive framework for innovation** that integrates scientific, regulatory, evaluative, and ethical considerations is essential to promote the adoption and development of AI in clinical trials in France.

In a European context marked by regulatory harmonization and infrastructures such as the Clinical Trials Information System (CTIS), the EMA plays a central role in facilitating multicenter trials. These trials, which are indispensable for ensuring data diversity and accelerating processes, illustrate the intrinsically international dimension of clinical trials involving AI. By leveraging national, European, and global dynamics, France can contribute to developing global standards while reinforcing its position in this interconnected ecosystem. The integration of AI into clinical trials transcends borders, necessitating close collaboration among stakeholders.

By committing to a common and global strategy, **France can establish itself as a leader in integrating AI into clinical trials**. AI can contribute to developing new treatments and therapies, reducing research costs and clinical trial durations, and addressing potential patient shortages for cohorts. This integration can make clinical trials more agile and even better suited to the needs of citizens.

In the appendix, we present **three use cases** illustrating examples of AI applications in clinical trials, as showcased during our workshop by a variety of participants.

Use Case

Clinical Trials Simulation Platform

Introductory French Use Case

Presented by nova IN SILICO ariis Alliance Recherche & Innovation Industries Santé

The **Jinko** platform, developed by **Nova in Silico**, is based on mechanistic models and *in silico* simulations. It aims to provide a solution in the field of clinical trials to potentially reduce costs, accelerate development timelines, and address challenges such as ethical concerns and data scarcity.

A Simulation Tool for Clinical Trials

Jinko is based on a mathematical and mechanistic approach that allows for the simulation of virtual clinical trials. The platform relies on three main pillars:

- **Disease models:** These formal representations translate the biological and pathophysiological processes underlying a given pathology, such as tumor growth or the progression of atherosclerosis.
- **Drug models:** They analyze the pharmacokinetics and mechanisms of action of administered substances, allowing the prediction of their concentration and efficacy in target tissues.
- **Virtual patients:** Generated from parametric distributions, these simulated patients reproduce inter- and intra-patient variability, providing an infinite population suitable for rare or complex diseases.

Building on these components, the Jinko platform could test thousands of scenarios, optimize inclusion/exclusion criteria, and reduce the sample sizes needed.

Potential Uses and Benefits

Addressing ethical challenges more effectively

By limiting the need to expose patients to experimental treatments, the platform could better address the ethical issues related to clinical trials.

Acceleration of timelines

Virtual simulations can significantly shorten the time required to design and validate protocols.

Complementarity with traditional trials

The Jinko platform does not aim to replace physical clinical trials but to enhance them by addressing data gaps (especially for rare diseases or underrepresented populations) and enabling better decision-making.

Jinko offers a new approach to clinical trials, aiming to optimize processes and strengthen ethical considerations.

Use Case

Patient Stratification

European Use Case

Presented by

The case of the Portuguese startup iLoF demonstrates how health startups can leverage European funding mechanisms to support their development. Founded at the University of Porto, iLoF works in the field of personalized medicine with their advanced technology and a structured funding strategy.

A Technology Applied to Clinical Trials

The platform developed by iLoF uses a combination of optical lasers and AI to analyze blood samples. It creates digital molecular profiles, which are compared to a biological database stored in the cloud. This approach shows promising applications in the context of clinical trials:

- **Reduction of recruitment costs and time:** The technology could reduce costs by 40% and participant screening time by 70%.
- **Improvement in patient stratification:** By limiting heterogeneity due to factors such as disease stage, the precision of trials could be enhanced.
- **Non-invasive method:** Based on blood samples, the method is less burdensome for patients, which could encourage their engagement.

These potential applications highlight the possible role of AI and data analysis tools in optimizing clinical trials and improving the process for patients.

Strategic Financial Support by EIT Health

The development of iLoF benefited from support by EIT Health, an organization dedicated to health innovation in Europe. In 2019, the startup won the EIT Jumpstarter program and the Wild Card program, securing an initial €2 million in funding. This support facilitated additional fundraising, reaching €8 million in 2020. In 2023, iLoF also received funding from the EIC (European Innovation Council), including a €2.5 million grant and €3.5 million in equity investment to accelerate the commercialization of its platform.

A Model to Help Overcome Funding Challenges

iLoF's experience illustrates several useful strategies for startups seeking to address financial challenges in clinical trials:

- **Leveraging European programs:** Initiatives such as EIT Health's Wild Card or the EIC Accelerator provide funding as well as mentorship and networking opportunities.
- **Strategic support:** EIT Health enabled iLoF to access technical resources and tailored training.
- **Technological validation:** By testing its solutions in real-world contexts, iLoF attracted investors, strengthening its credibility.

The journey of iLoF highlights that structured funding, combined with appropriate support, can be offered to startups in Europe, providing an interesting example for French and European initiatives.

Use Case
Augmented Cohorts

Presented by Prof. Stéphanie Allassonnière

Augmented cohorts, based on artificial intelligence, rely on advanced statistical models and simulations. This approach aims to offer an innovative alternative in the context of clinical trials to address several challenges, including data limitations, ethical constraints, and process optimization, while potentially reducing costs and associated development timelines.

Principle of Augmented Cohorts

The creation of augmented cohorts relies on the use of mathematical models to learn and reproduce the statistical distribution of data from a population. These models, whether classical statistical methods or deep learning-based, generate artificial data representing fictitious but relevant individuals in relation to the observed population. These artificial patients are built from existing data, ensuring they reflect the characteristics of real populations without introducing bias or novel traits.

Applications of Augmented Cohorts in Clinical trials

1. **Augmented control arms:** These artificial patients can complement or partially replace control groups in trials, thereby reducing the need to recruit a large number of real participants. This is particularly useful in contexts where recruitment is challenging.
2. **Optimization of inclusion criteria:** Instead of relaxing inclusion criteria to include more participants, augmented cohorts allow strict criteria to be maintained while ensuring a sufficient sample size.
3. **Reduction of temporal bias:** Artificial patients help mitigate discrepancies arising from prolonged participant recruitment, ensuring temporal consistency in the collected data.
4. **Patient retention:** By maximizing access to medical innovations through better-structured trials, these methods can improve participant retention.
5. **Interim trial evaluations:** Artificial data can enrich both studied groups (treatment and control), enabling more robust interim analyses and informing decisions on trial continuation or termination.
6. **Cohort rebalancing:** Preferential sampling of existing data can correct imbalances to align cohorts with current demographic and epidemiological knowledge.

Key Advantages of Augmented Cohorts

Augmented cohorts offer several key benefits, including:

- Enhanced statistical power of analyses.
- Improved representation of diverse populations.
- The ability to conduct multicenter studies with anonymized data.

By generating artificial patients and augmenting cohorts, AI can provide significant added value to clinical trial methodologies. It can optimize protocols, strengthen the robustness of results, and accelerate access to therapeutic innovations.

Glossary

Augmented cohorts : Patient groups enriched through the integration of additional data, such as genomic, phenotypic, or data from external databases, to enhance analyses and patient stratification.

Augmented control arms : Methodological approach in clinical trials where external data, often from databases or other studies, are used to supplement traditional control arms to reduce the number of participants required while maintaining statistical robustness.

Bayesian trials : Trials using classical methodology (randomization, etc.), but whose quantitative data analysis is conducted within a Bayesian rather than frequentist framework.

Clinical Trials Information System (CTIS) : Digital platform facilitating the management, submission, and monitoring of clinical trial authorizations in the European Union.

Committee for the Protection of Persons (CPP) : Independent body in France responsible for providing opinions on research protocols involving human participants to ensure their safety, rights, and dignity.

Control groups : Groups of participants receiving a placebo, standard treatment, or no intervention, serving as a reference to assess the efficacy and safety of the experimental treatment.

Data representativeness : Characteristic of a data sample accurately reflecting the features of the target population.

Deep learning : Branch of AI based on deep artificial neural networks, enabling the processing and analysis of massive volumes of complex data, particularly in medical imaging, genomics, and clinical trials.

Digital twin : Dynamic virtual representation of a physical object, system, or individual, created from real data and used to simulate, predict, and optimize performance.

European Medicines Agency (EMA) : European institution responsible for evaluating, monitoring, and regulating human and veterinary medicines within the European Union.

FDA (Food and Drug Administration) : U.S. federal agency responsible for regulating food, drugs, medical devices, and many other products to ensure their safety, efficacy, and quality.

Financial incentives : Economic measures, such as subsidies or tax credits, to encourage the development and adoption of new technologies.

GDPR (General Data Protection Regulation) : European regulation aimed at protecting the personal data of European Union citizens by governing their collection, processing, and storage.

Health Data Platform (PDS): French platform aggregating health data to facilitate its use in innovative projects.

Health Data Warehouses (EDS): Digital infrastructures enabling the collection, structuring, and organization of medical data from various sources.

***In silico* simulation :** Use of computer models to simulate clinical trials or biological phenomena to reduce experimentation on patients.

Inclusion/Exclusion criteria : Conditions used to select eligible or ineligible participants in a clinical trial.

Machine learning : Branch of AI enabling systems to automatically learn from data without being explicitly programmed.

Glossary

Marketing Authorization (MA) : Authorization issued by a competent authority (in France, ANSM or EMA at the European level) allowing the marketing of a medicine after an assessment of its benefit/risk ratio.

Mechanistic models : Mathematical representations describing the biological processes underlying a disease or the effect of a treatment.

Multicenter clinical trials: Studies conducted simultaneously across multiple centers or institutions to evaluate the efficacy and safety of a treatment on a larger and more diverse sample of participants.

National Agency for the Safety of Medicines and Health Products (ANSM): French public body responsible for evaluating, authorizing, and monitoring medicines and other health products to ensure their efficacy, quality, and safety.

National Health Data System (SNDS) : French database aggregating pseudonymized medical information for large-scale analyses.

Patient stratification: Process of identifying subgroups of patients sharing similar characteristics to personalize treatments or clinical trials.

Pharmacokinetics : Study of the mechanisms of absorption, distribution, metabolism, and elimination of drugs in the body.

Pharmacovigilance : Set of activities related to the detection, assessment, understanding, and prevention of adverse effects or any other drug-related problems.

Predictive model : Mathematical or computational tool used to predict future or unknown outcomes based on historical and current data.

Synthetic arm : Virtual control group created from computer models and existing data within the framework of a clinical trial.

Synthetic data : Artificially generated data by computer algorithms to simulate real data, used to enrich databases or train AI models.

Transversality : Approach aimed at connecting and harmonizing different sectors or disciplines to solve complex problems.

Underrepresented population : Patient groups under-included in trials, such as the elderly or pregnant women, which may introduce biases.

Virtual patients : Computer-simulated populations representing biological variability among individuals to test various clinical scenarios.

Methodology

To address the topic, the methodology and structure of the workshop followed a structured approach in several stages:

1. **Introduction**

A general introduction was given to set the framework and prepare participants for the workshop's theme.

2. **Presentation of concrete use cases of AI in France**

Concrete examples of AI usage in the French context were presented, illustrating relevant and current applications.

3. **Open discussion: "What are the challenges to overcome in France regarding the use of AI in clinical trials?"**

This discussion followed the 1-2-4-All method, which encourages progressive and collective reflection:

4. **Presentation of examples of AI usage internationally**

Relevant initiatives and use cases outside France were shared to broaden perspectives and explore innovative solutions.

5. **Collective reflection: solutions and actions to implement**

Participants worked together to identify concrete action plans and strategic recommendations.

6. **Group work presentations**

Each group presented its conclusions and proposals, enabling a rich and interactive exchange.

This methodology allowed for:

- Discovering and learning : Innovative examples of AI usage were shared, enriching participants' understanding.
- Gathering and confronting perspectives : Different viewpoints were highlighted during plenary discussions.
- Encouraging global and inclusive collaboration : The workshop fostered dialogue between public and private actors, facilitating a shared vision of challenges and opportunities.

Nota Bene

This white paper reflects the thoughts and discussions from a two-hour workshop, which naturally limits the depth and level of technical detail addressed. While we identified key points and strategic issues, a more in-depth analysis and additional clarifications would be necessary to address certain complex aspects. We encourage readers to keep this limitation in mind when interpreting the information presented here and to consider this document as a basis for further discussions.

AI FOR HEALTH

If you wish to further explore the ideas in this report, participate in our upcoming workshops, share your opinion, or be part of our next Summit, please contact:

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