InSilicoTrials

Harnessing AI and Regulation to Hyper Accelerate Pharmaceutical R&D

CMC Strategy Forum Europe 2025

Basel 22 October 2025

Luca Emili - CEO

Computer simulations are standard technology for many industries

100% of new cars and airplanes are developed and tested in silico

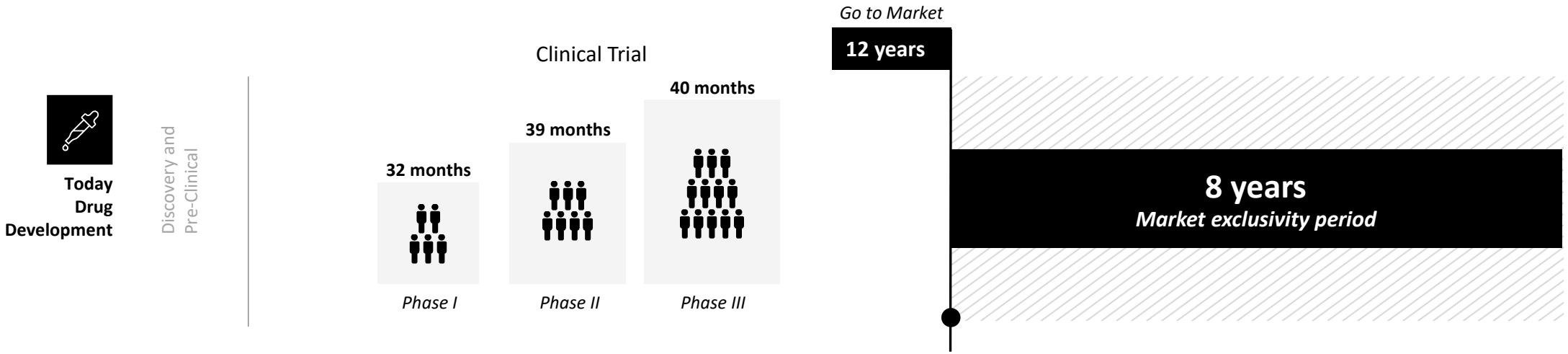
Using modeling and simulation

Automotive

Aerospace

But modeling and simulation is underutilized in the healthcare industry

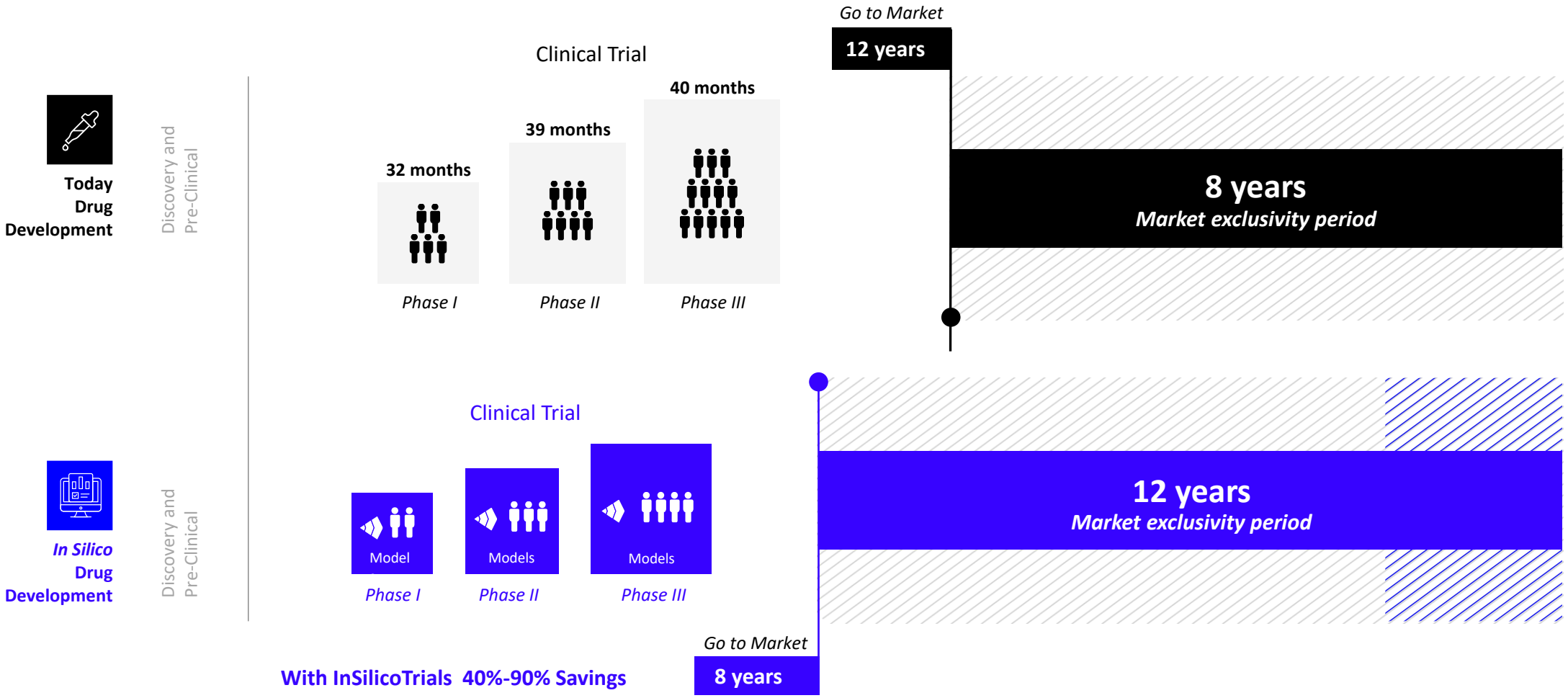
Today’s problem



Drug development takes on average 12 years and $2.2Bn
Only 7.3% of Ph1 trials lead to approved drugs

Solution: integrate traditional development with in silico

Accelerate “go-to-market” and extend market exclusivity period

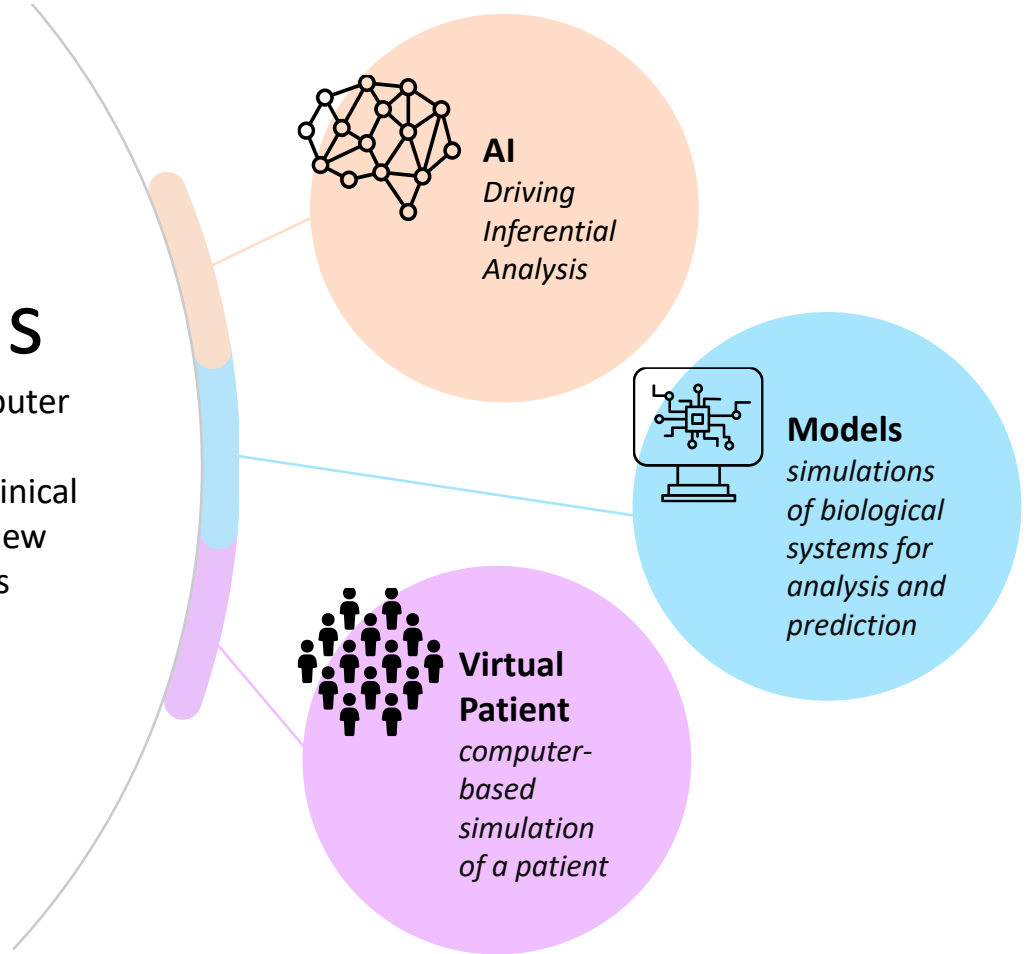


What is an *in silico* trial?

Mainstream definition by scientists and regulators

in silico trials

the use of individualized computer simulations in the design, development or nonclinical, clinical and regulatory evaluation of new drugs, devices or interventions



Main advantages of conducting *in silico* trials:

- Cost Reduction
- Time Efficiency
- Reduced Ethical Concerns
- Exploration of Complex Systems
- Prediction and Optimization

Adopted by Major Industry Players

Regulatory Bodies

FDA U.S. FOOD & DRUG ADMINISTRATION

Pharma

Pfizer · MERCK · Eisai · ucb · IBSA · Dr.Reddy's · AUROBINDO · INSUD PHARMA · FAES FARMA

Universities

RUTGERS THE STATE UNIVERSITY OF NEW JERSEY · TU Graz · University of Maribor Faculty of Logistics

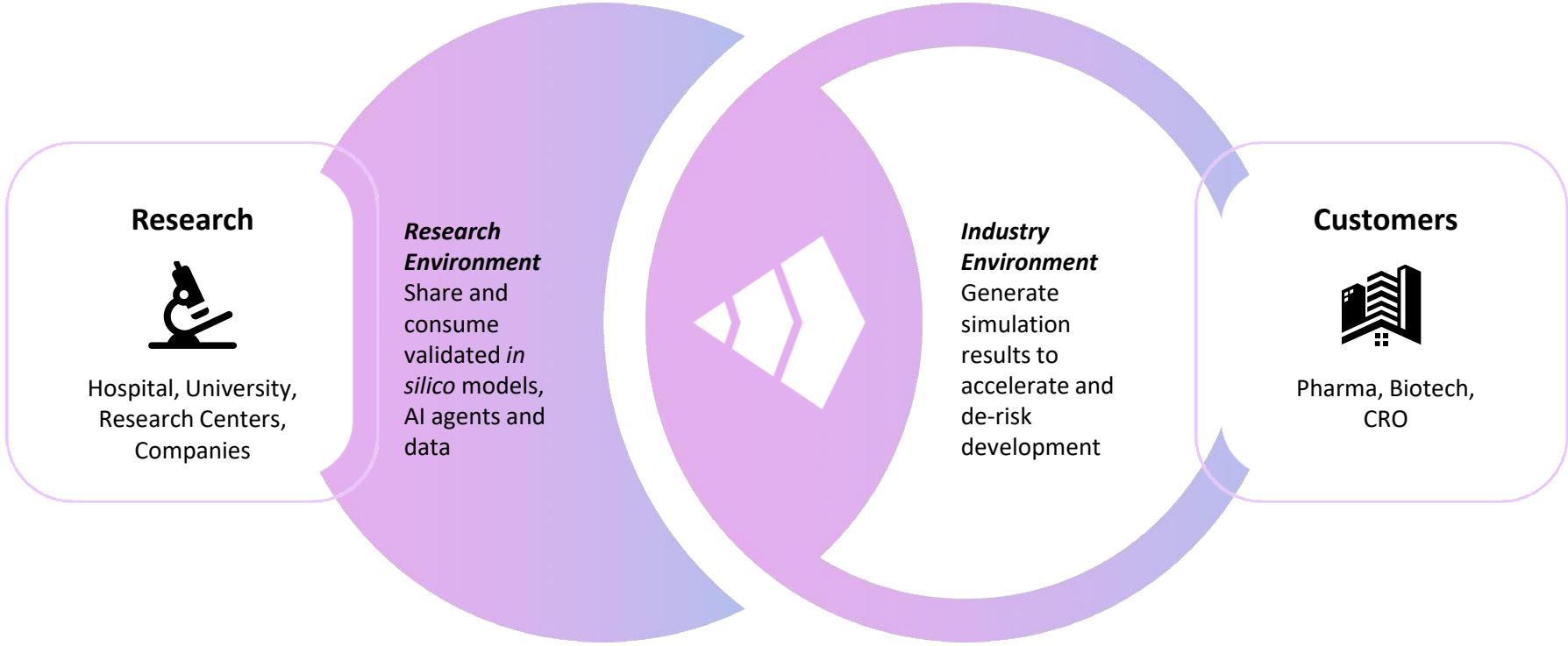
Biotech

MIRA Pharmaceuticals · axoltis pharma · HEALTHY FOR LIFE RejuvenateBiomed · TELOMIR ORAL STEM CELL THERAPY · CELLPHIRE THERAPEUTICS · Consegna Pharma · ONCOTELIC · GAIN THERAPEUTICS · MyMD · StromaCare

InSilicoTrials Leading Scientific Platform for In Silico Evidence Generation

A Collaborative Ecosystem of 70+ renowned Universities and Research Centers and 50+ Data Providers

Fully aligned with regulatory guidance by US FDA and EMA



U.S. FOOD & DRUG ADMINISTRATION

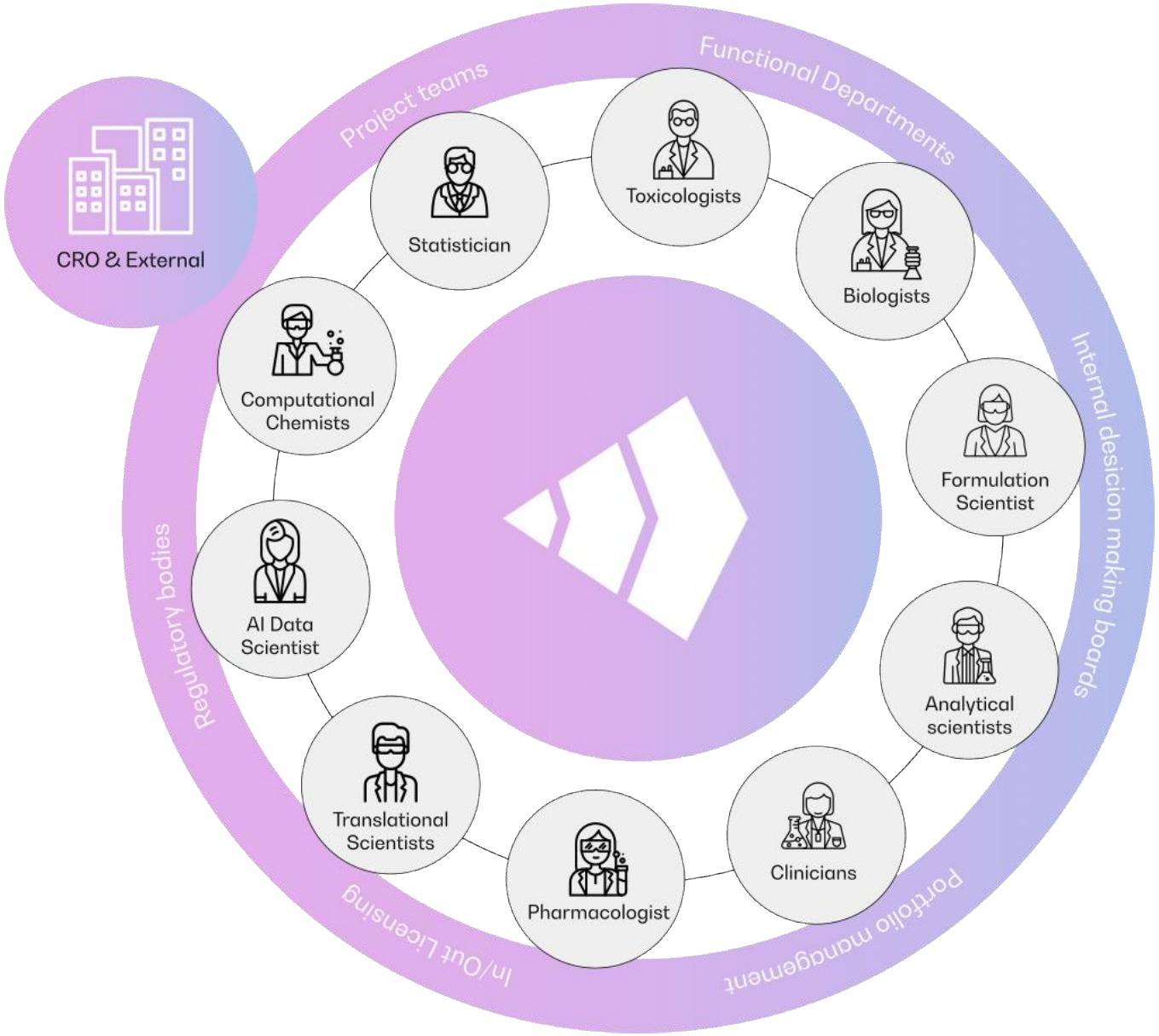
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

InSilicoTrials' Model & Data Providers

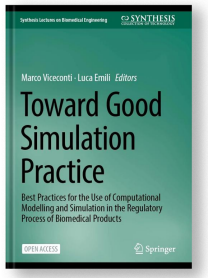
We are building the largest *in silico* research and data network worldwide

InSilicoTrials Platform: Secure & Collaborative Workspace

- **Centralized Information Storage:** A unified repository for critical data
- **Cross-Departmental Access:** Ensures visibility for authorized users
- **Role-Based Permissions:** Controlled access based on user roles
- **Seamless Collaboration:** Enhances teamwork and communication
- **Improved Efficiency:** Reduces silos and streamlines workflows



Major public achievement 2024 & 2025



Written with FDA

Publication on **Nature** Springer of **“Toward Good Simulation Practice”** with an FDA foreword.
Over 47,000 copies distributed since February 2024.

FDA U.S. FOOD & DRUG ADMINISTRATION

InSilicoTrials **awarded by the FDA** as one of the top 5 teams winning the **“Precision FDA” Generative Artificial Intelligence (GenAI)** competition

Microsoft

NVIDIA

Entered into **Startup Program** from both **Nvidia and Microsoft** as first step of developing strategic partnerships.

InSilicoTrials won the Pitching Competition at the **London AI Summit 2025.**

accenture

InSilicoTrials is showcased at **Accentures’s Innovation Center.**

InSilicoTrials received the **AI Startup of the Year award** at Startup Grind Global 2024 in Silicon Valley, California

Benefits

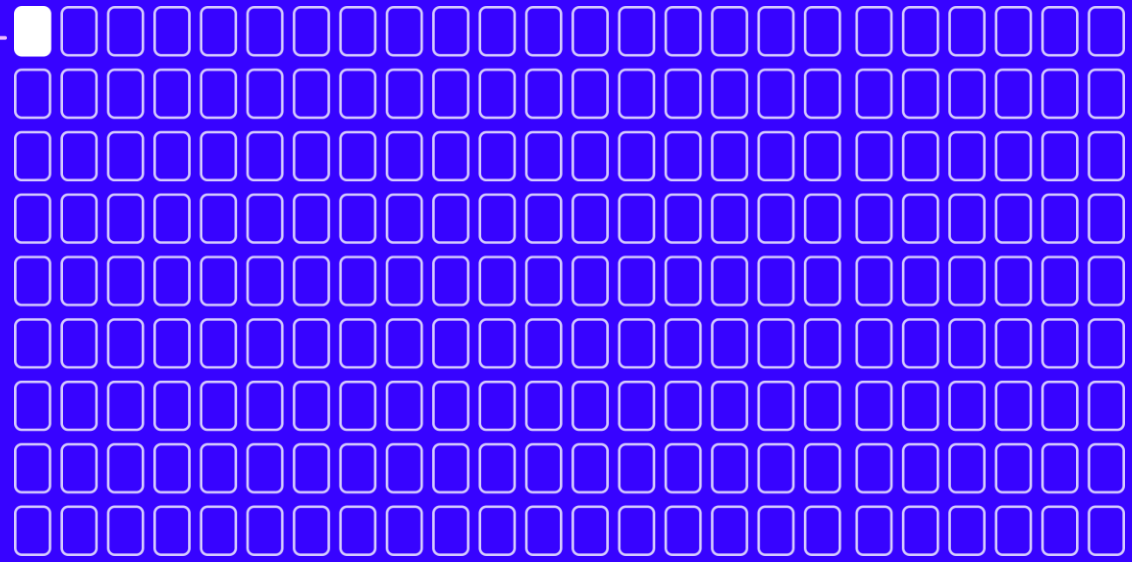
AI-generated prognostic digital twins for clinical trials simulation in MS

1 DAY

Simulation Time with **InSilicoTrials**

MERCK

Multiple sclerosis disease progression and treatment effect on more than 3,000 MS Patients in just 1 day of simulation time



Clinical Trials with Traditional Approach

4 YEARS

Benefits

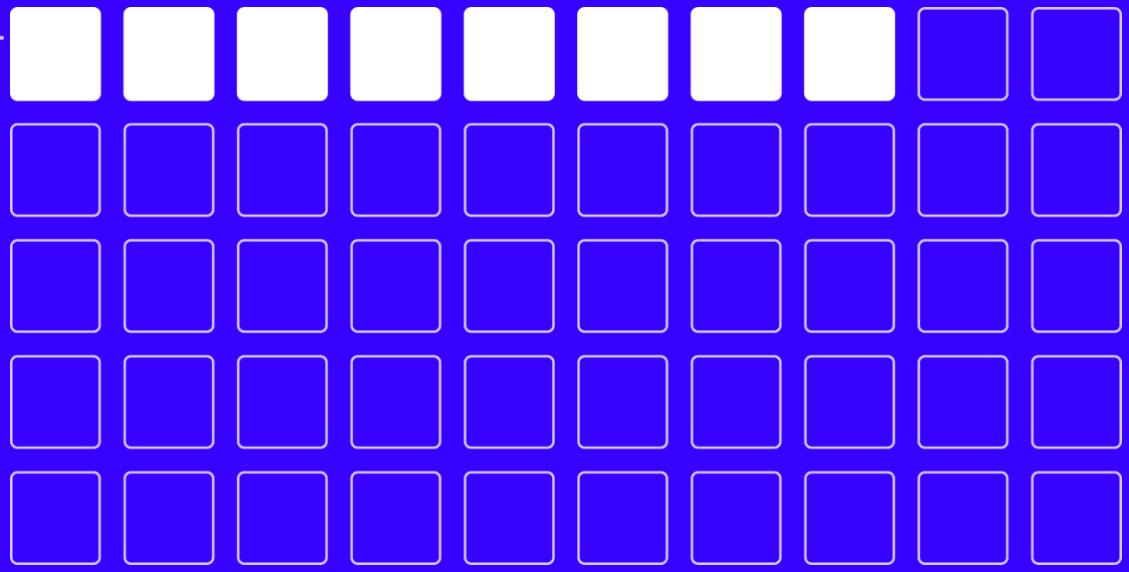
GPU-powered digital twins to predict and optimize granular processes

2 WEEKS

Simulation Time with **InSilicoTrials**

FDA U.S. FOOD & DRUG ADMINISTRATION

Successful *in silico* study of powder flow and mixing, handling up to **100 million particles**, for the evaluation of advanced manufacturing processes control strategies



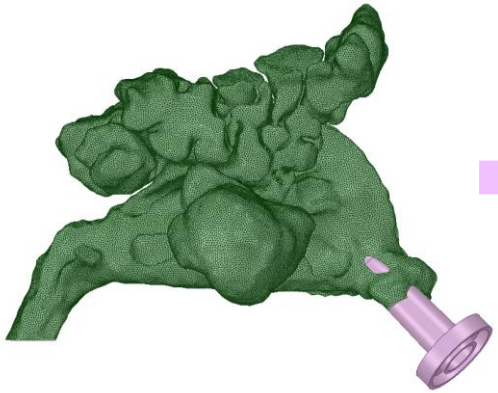
Multiple Trials with Physical Experiments

3 MONTHS

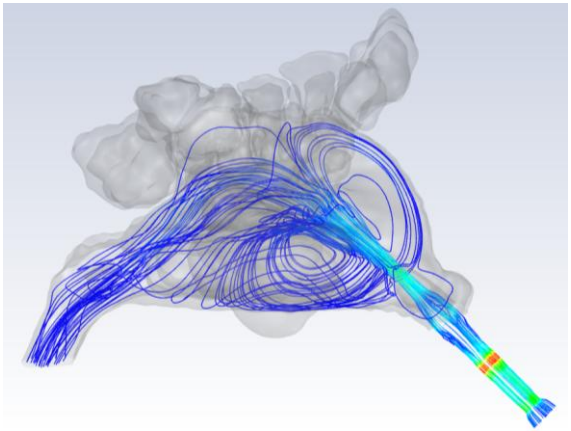
Drug delivery

Simulations to optimize the design of a drug-inhaler combination

3D reconstruction of the nasal cavity anatomy coupled with the inhalation device



Computational fluid dynamic simulation of the particle flow in the nasal cavity



Model-driven recommendations to optimize drug particle deposition in the nasal cavity

https://insilicotrials.com

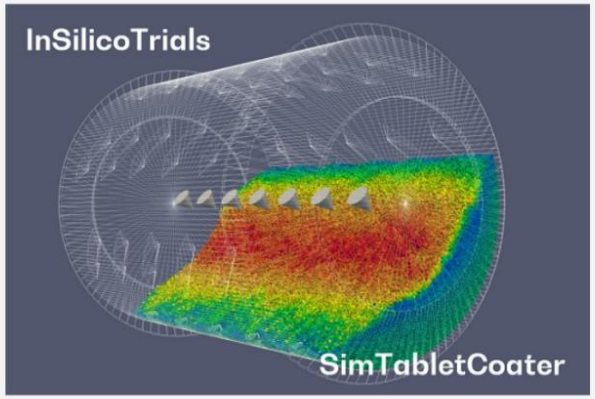
InSilicoTrials

SimTabletCoater Home Activity Account Logout

SimTabletCoater

Shedding light on your tablet coating process

Find the sweet spot of the coating process for your tablets and drastically shorten an otherwise laborious parameter space exploration by manipulating parameters such as spray, mass of tablets, or coater rotation speed from the comfort of your web browser.



Validated Model

Validated for investigation of the tablet coating process with industrially relevant settings

3D Visualisation

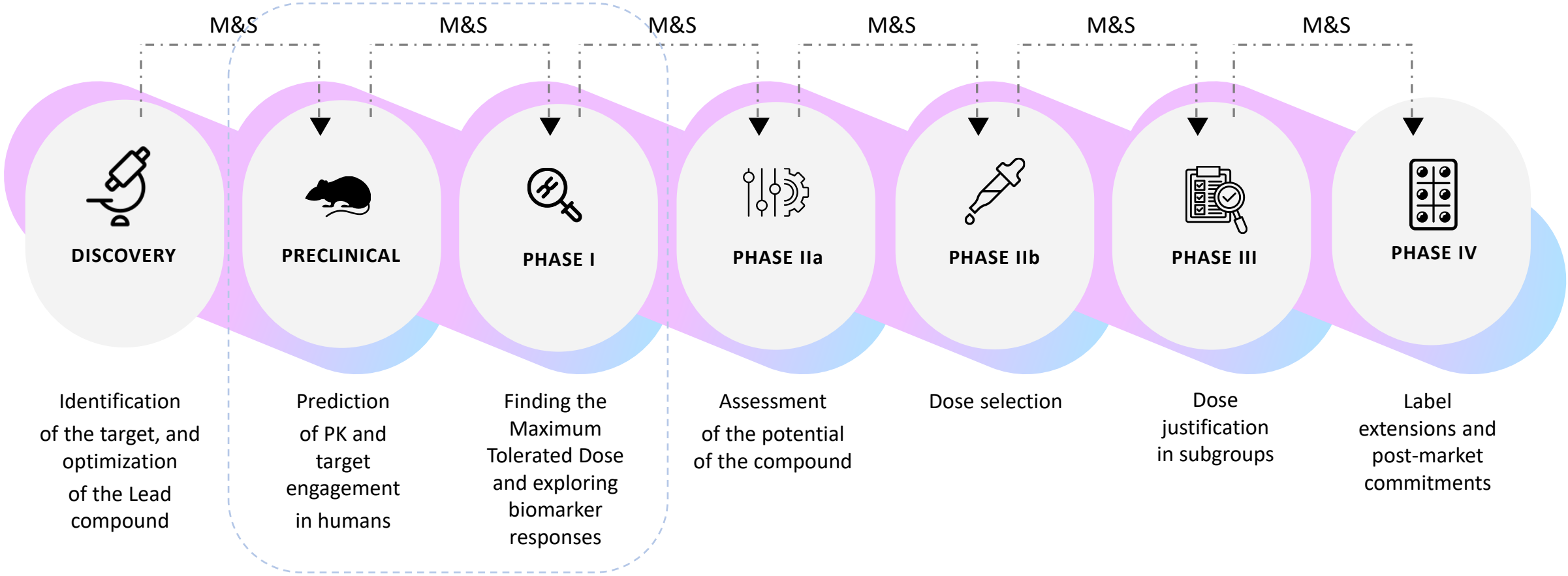
See the simulation configuration and the simulation results in three dimensions

Easy to Use

Well-designed wizard that guides through the setup of the simulation step-by-step

SimTabletCoater by InSilicoTrials Technologies
Terms and Conditions apply.

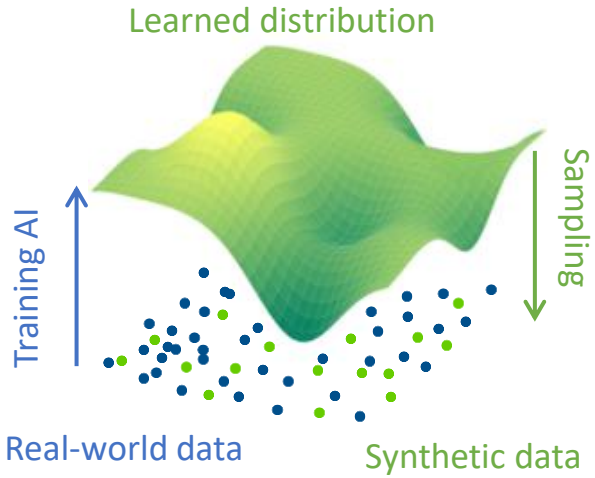
Drug development and model building



Learn / confirm / predict throughout the entire drug development program
Increase the confidence in decision making

Using generative AI to enrich RWD with synthetic data

- Use of generative AI to learn the **data distribution of patients**
- Sample new synthetic patients, ensuring RWD features, and statistical distributions are mirrored
- Generate synthetic patients to address **underrepresentation in minority groups**
- Effectively augment **statistical power** and shrink variance for minority groups
- Helps explore **outcomes with better confidence** for different subpopulations



Juwara L, et al. An evaluation of synthetic data augmentation for mitigating covariate bias in health data. Patterns. 2024 Apr 12;5(4).
A. J. Rodriguez-Almeida et al., "Synthetic Patient Data Generation and Evaluation in Disease Prediction Using Small and Imbalanced Datasets," in IEEE Journal of Biomedical and Health Informatics, 2023
D'amico S, et al. Synthetic data generation by artificial intelligence to accelerate research and precision medicine in hematology. JCO Clinical Cancer Informatics. 2023

MIDD to address 'key questions' during drug development

- How **high** in dosing can we go (single Phase 1a vs multiple Phase 1b dosing) given drug **exposure limits**?
- What is the **probability** for a new drug to be **superior** to the marketed therapy of choice in Phase 2a?
- What is the probability of selecting the **right dose** given the planned Phase 2b design including 4 dose levels?
- What is the **optimal dose** (benefit/risk) in pediatric patients based on Phase 3 results?
- What is the **probability** the new formulation is **bioequivalent** to the previously approved formulation ?

Drug Safety Suite

Complement in vitro testing to assess drug-induced proarrhythmic risks in drug candidates early screening

- QT/TdP Risk Screen

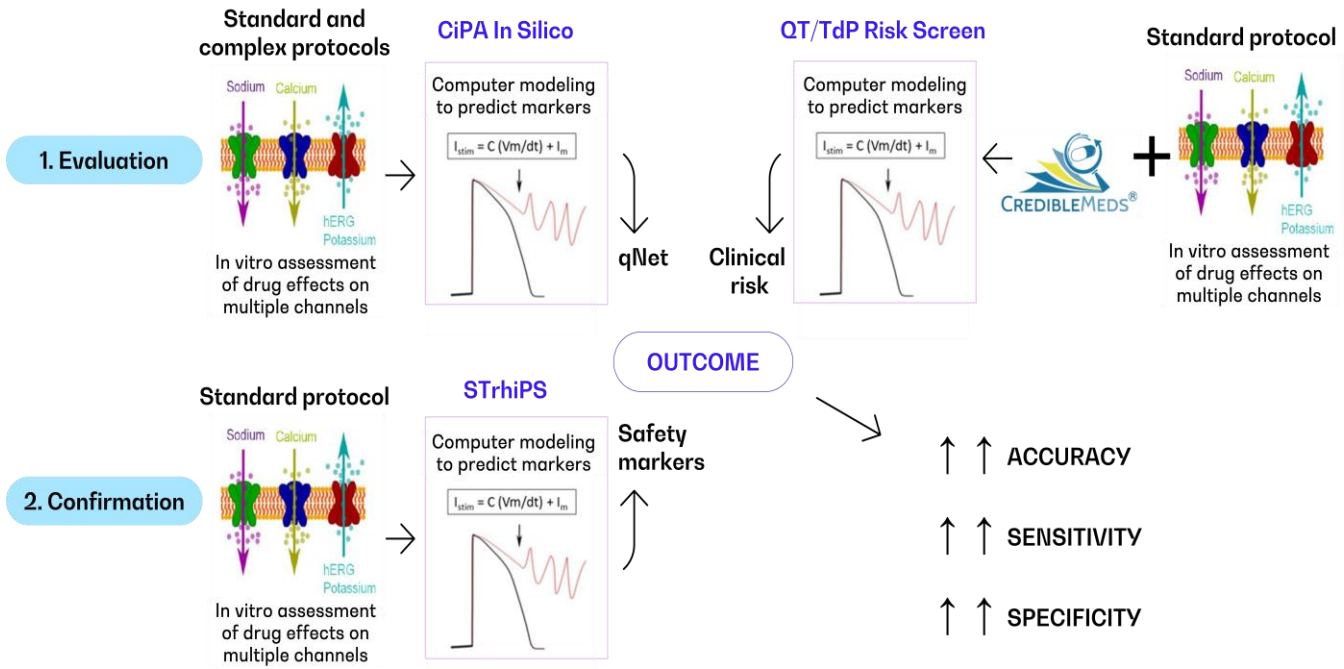
  Real-time early screening of clinical risk

- STrhiPS

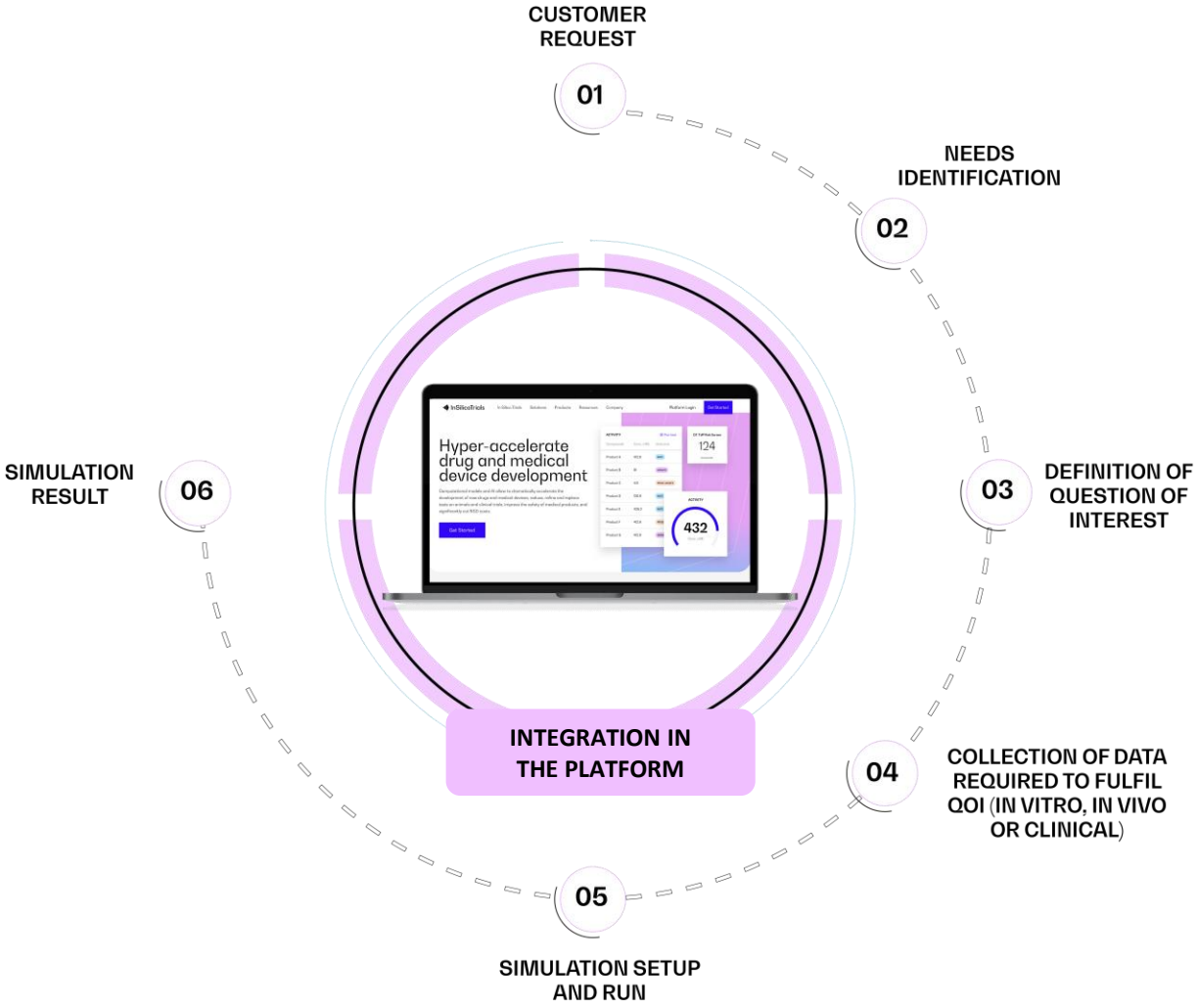
  Safety Trials on Human Induced Pluripotent Stem Cells

- CiPA In Silico

  Comprehensive in vitro Proarrhythmia Assay In Silico



Model Acquisition



Acquisition Criteria

Source reputation

Peer-reviewed publication

Validation status

Reproduction of standard tests

Relation to Regulatory Bodies

MIDD to address 'key questions' during drug development

- How **high in dosing** can we go (single Phase 1a vs multiple Phase 1b dosing) given drug exposure limits?
- What is the **probability** for a new drug to be **superior** to the marketed therapy of choice in Phase 2a?
- What is the **probability** of **selecting the right dose** given the planned Phase 2b design including 4 dose levels?
- What is the **optimal dose** (benefit/risk) in elderly women with decreased renal function based on Phase 3 results?
- What is the **probability** the new formulation is **bioequivalent** to the previously approved formulation ?

Our engagement in Regulatory Science

We authored the book Toward Good Simulation Practice

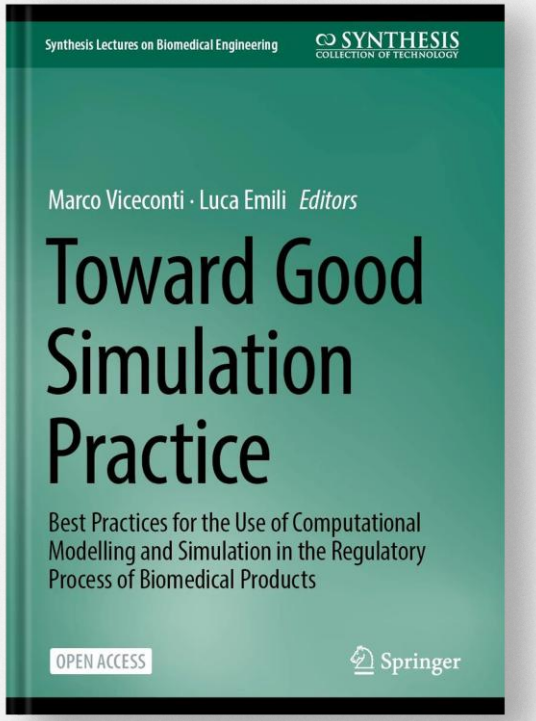
13
FDA members

U.S. FOOD & DRUG ADMINISTRATION

&

130+
researchers from university and industry

Johnson & Johnson
Boston Scientific
Medtronic



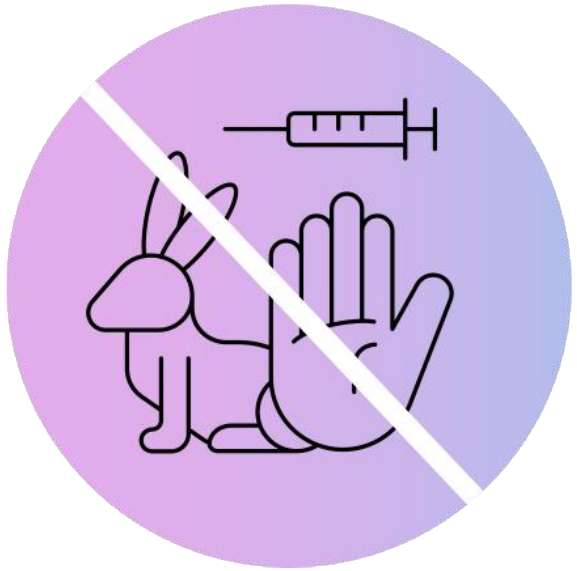
Download your free book insights

*Nature Publishing Group: **Over 47,000 copies distributed since February 2024.**

Regulatory tail wind

Implications of Recent FDA Announcement to Phase Out Animal Testing for mAbs and Other Drugs

Over 90% of drugs with safety and efficacy in animal do not receive FDA approval due to human safety and efficacy issues



Animal-based data poor predictors in multiple common diseases including cancer, Alzheimer and inflammatory diseases

Focus on New Approach Methodologies (NAMs):

- In vitro human-based systems
- In silico modeling
- Other innovative platforms evaluating immunogenicity toxicity and pharmacodynamics in human

FDA's roadmap

- Reduce animal testing while improving drug development

FDA Announces Plan to Phase Out Animal Testing Requirement for Monoclonal Antibodies and Other Drugs | FDA

FDA Deploys Elsa:
First AI-Assisted Scientific Review Completed

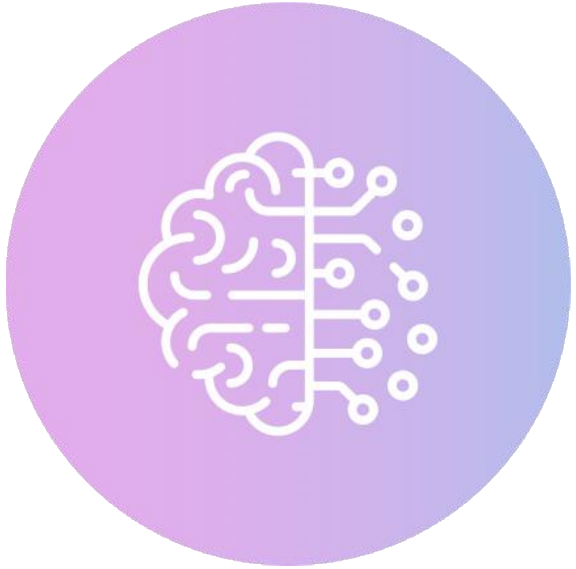
The FDA has completed its **first AI-assisted scientific review using Elsa**, a secure generative AI tool. Elsa streamlines scientific and regulatory workflows, allowing reviewers to complete complex tasks in minutes instead of days.

The agency will deploy Elsa across all centers by June 30, 2025, marking a new era of efficiency and innovation in regulatory science.

FDA Deploys Elsa:
First AI-Assisted Scientific Review Completed

Elsa in Action:

- **Accelerate** clinical protocol reviews
- **Shorten** the time needed for scientific evaluations
- Identify **high-priority inspections** targets
- Perform **faster label comparisons**
- **Summarize adverse events** to support safety profile assessments
- **Generate code** to help develop databases for nonclinical applications



"Today marks the dawn of the AI era at the FDA with the release of Elsa, AI is no longer a distant promise but a dynamic force enhancing and optimizing the performance and potential of every employee,"

Jeremy Walsh
FDA Chief AI Officer

IST Takes the Stage after NVIDIA CEO at PMWC

Key takeaway

- **AI** can **dramatically reduce time and cost** of drug development de-risking the investment

- Regulators are adopting **AI to review submission**

- Regulations are starting to be defined to support the **better use of this kind of technology**

- Drug development **de-risking the investment**

We are ready for the future of medicine, are you?

Thank you.