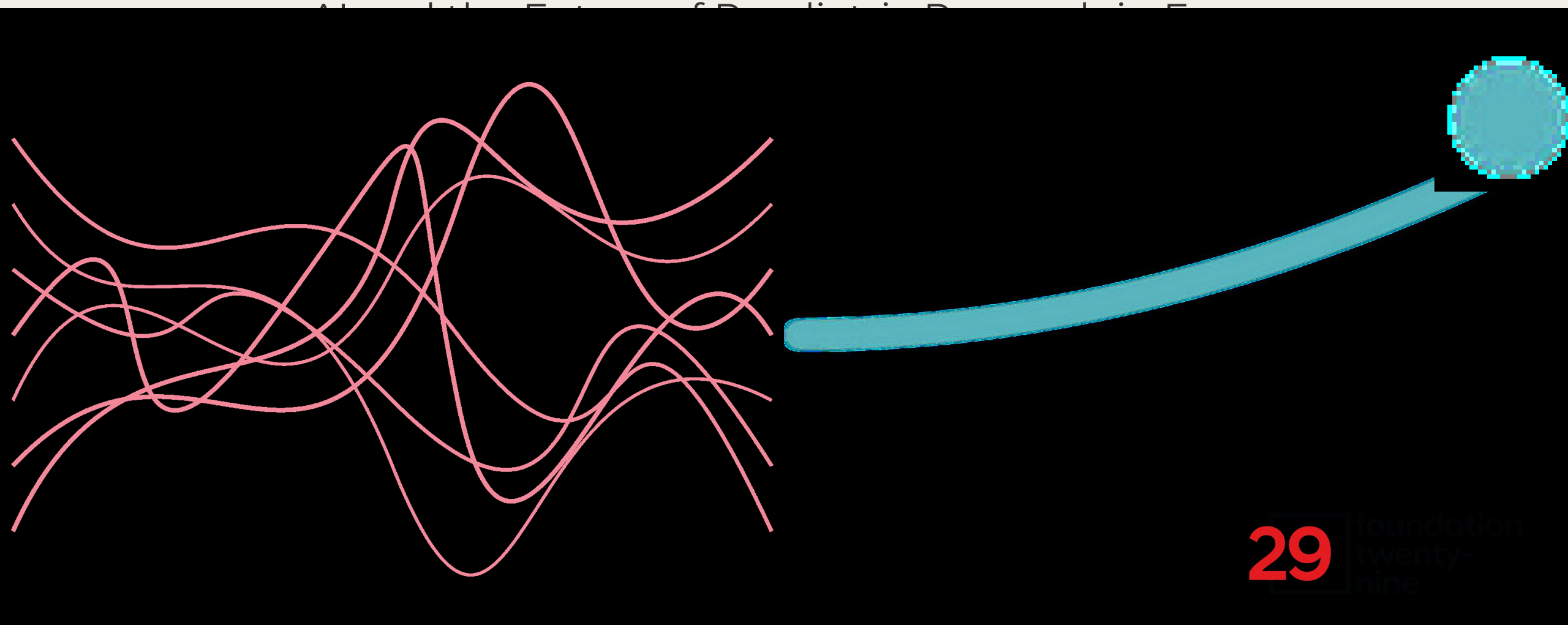
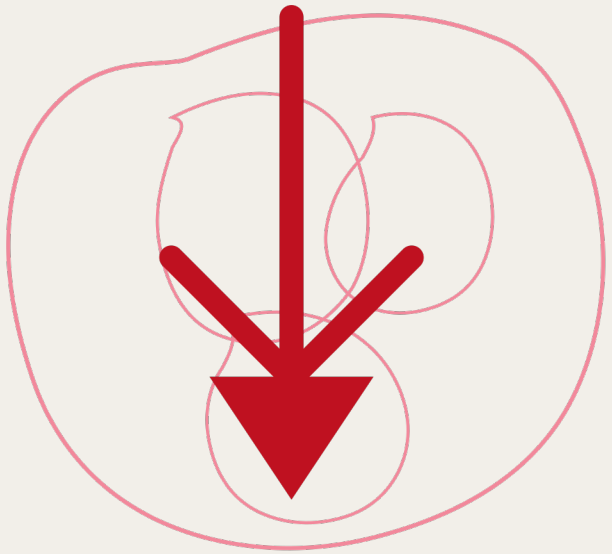
Restoring Agility



Europe is losing research

−60,000

Fewer clinical trial places for European patients.

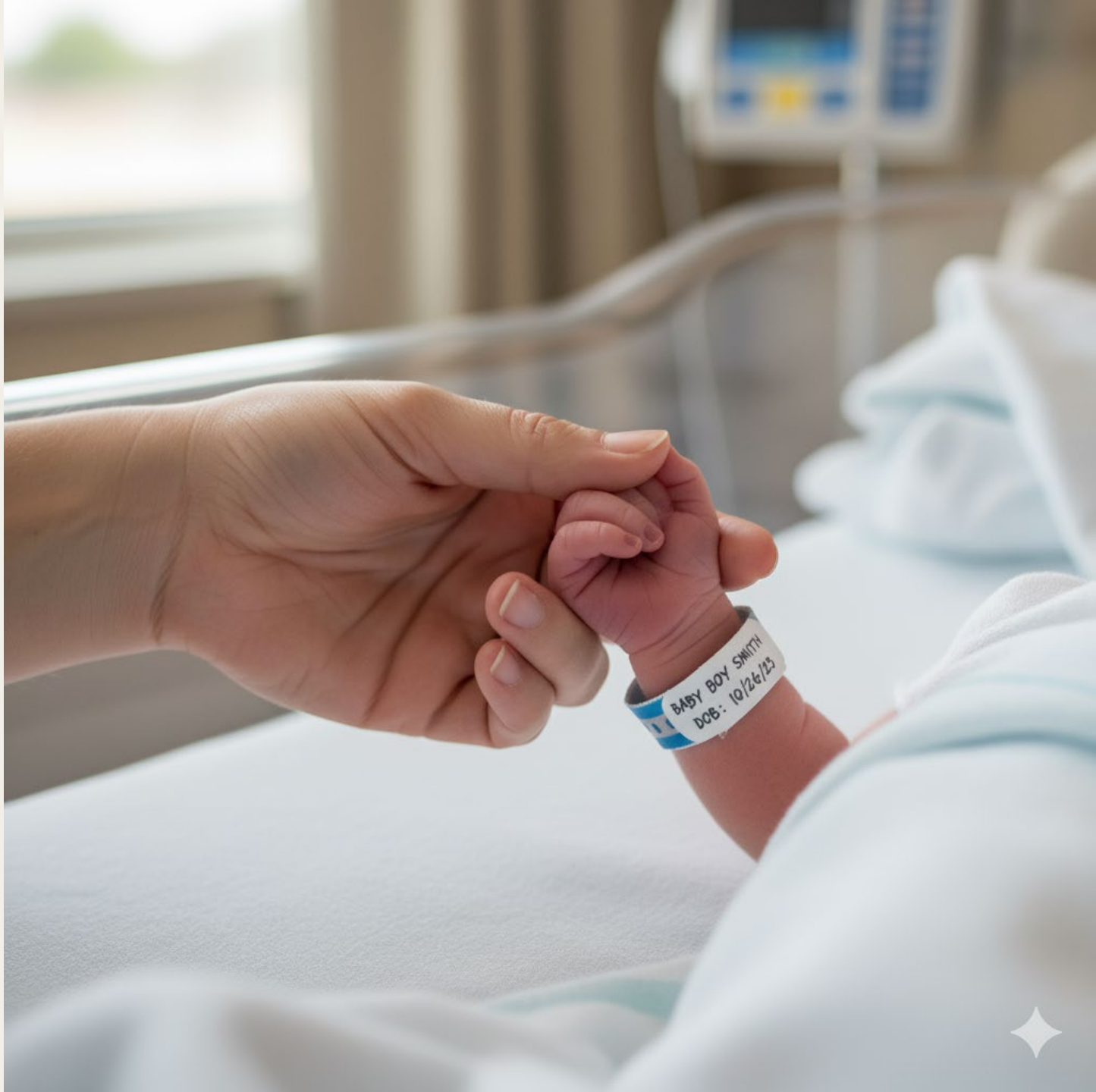


EEA Global Share of Trials:

22% (2013) ➔ 12% (2023)

This is not a statistic

"For many, a clinical trial is the only treatment option."



The most vulnerable are hit hardest

Paediatric & Rare Disease

Global

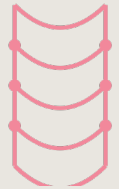
Rare Disease Trials:

GROWING

Europe (EEA)

Rare Disease Trials:

FELL 4%



Advanced

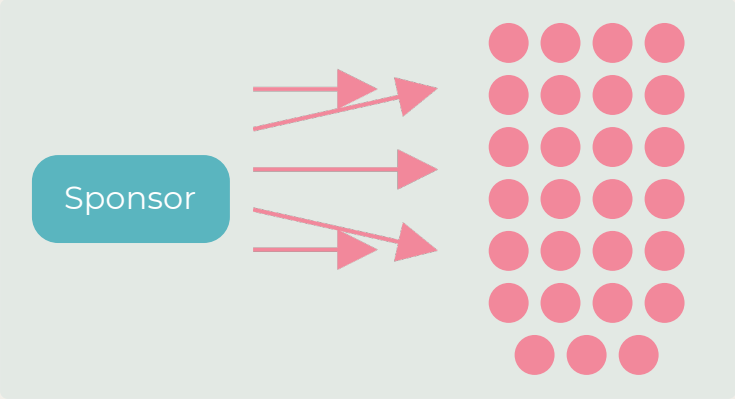
EEA Share of Cell & Gene Therapies:

Collapsed from 25% ➔ 10%

The "Solution" became the problem

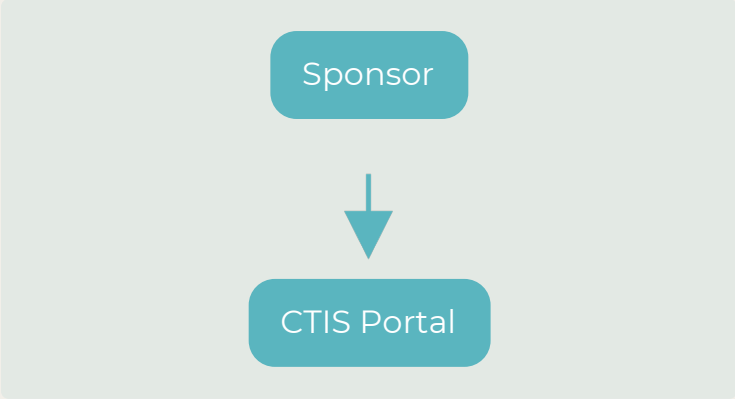
The EU Clinical Trial Regulation (CTR)

BEFORE



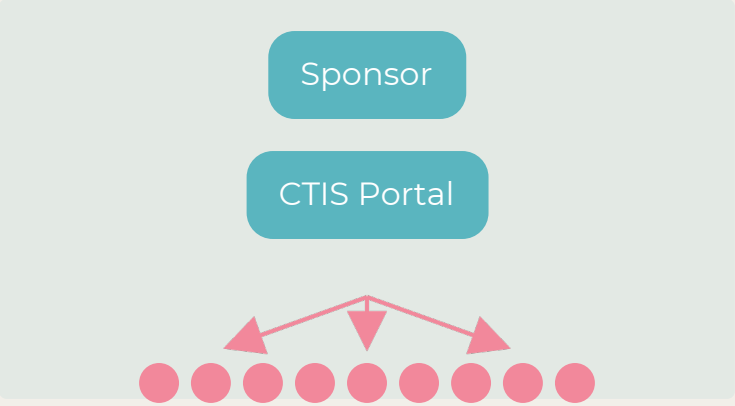
27 Member States

THE GOAL



A single communication from the sponsor to the portal

THE REALITY

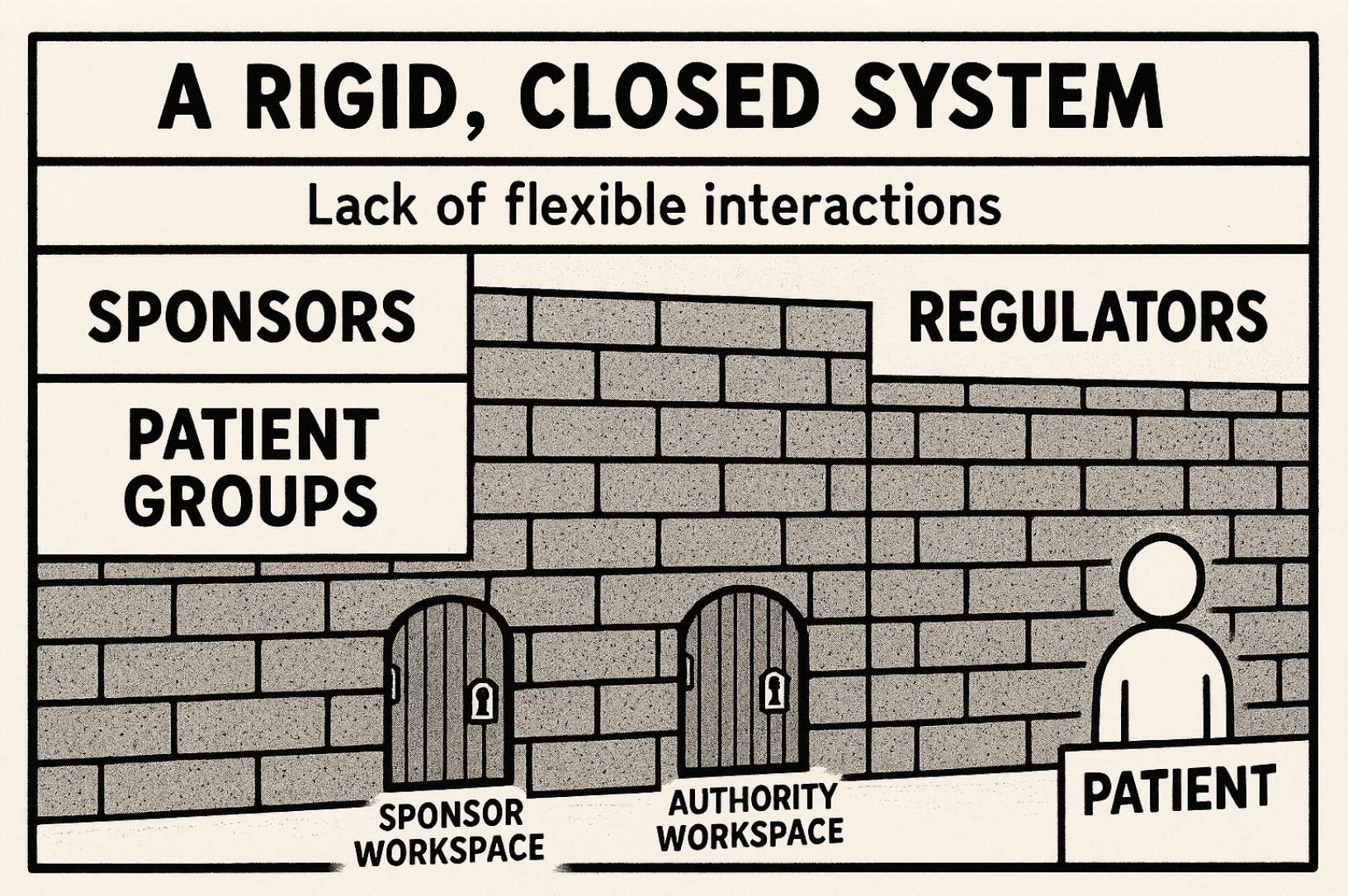


CTIS Portal as an additional layer over the fragmented system

A rigid layer was added, complexity was not removed.

Extreme complexity

The Patient: Excluded From The Development Process



Innovation is being bypassed

A gene therapy trial for Dravet Syndrome

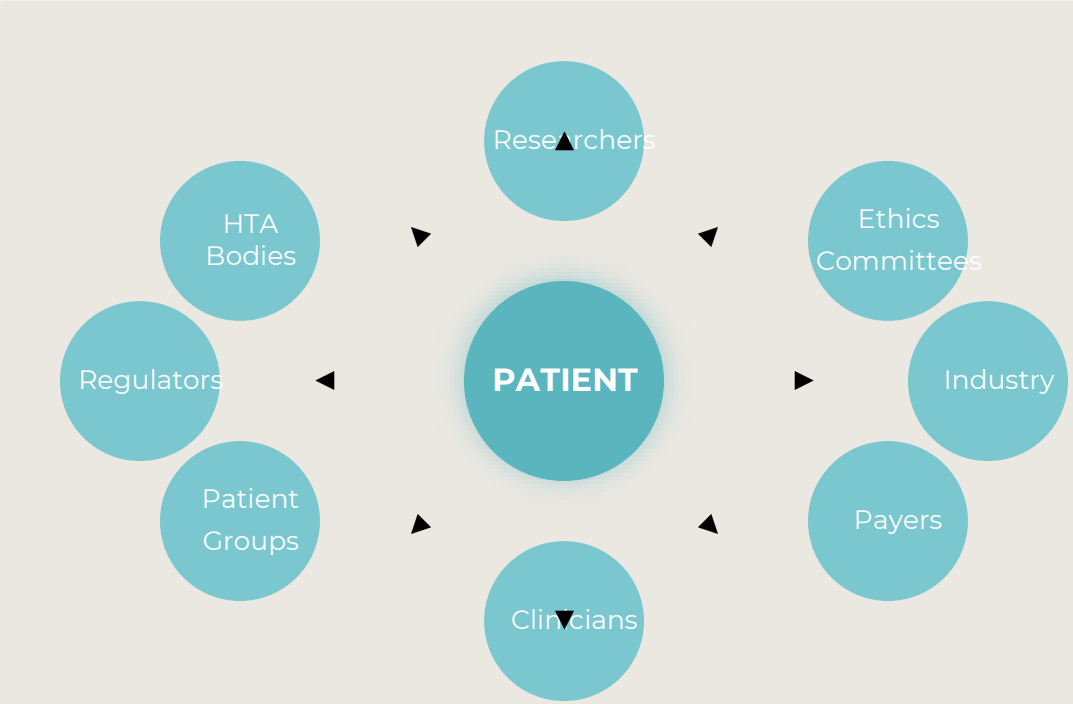
ACTIVE TRIALS: United States, United Kingdom

NO TRIALS AVAILABLE (Including Europe)

Patients must be co-creators

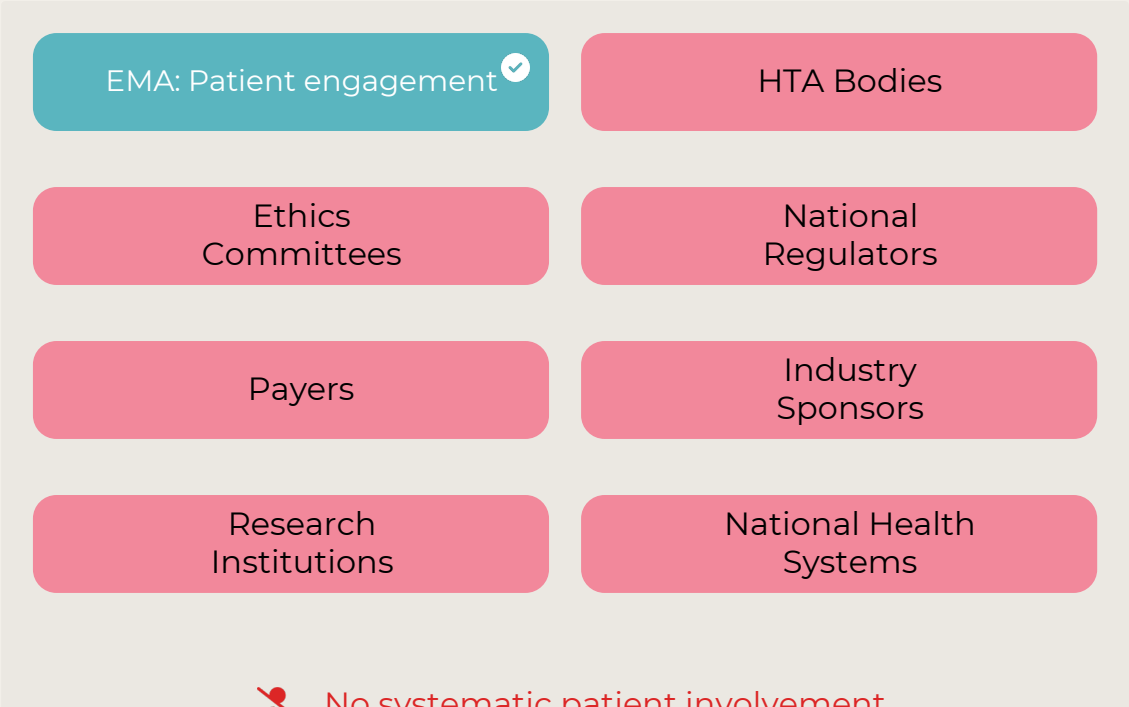
Involved Throughout the Entire Development Process

THE IDEAL: Patient as Co-Creator



Collaborative network with patient at center of all interactions

THE REALITY: Fragmented Involvement



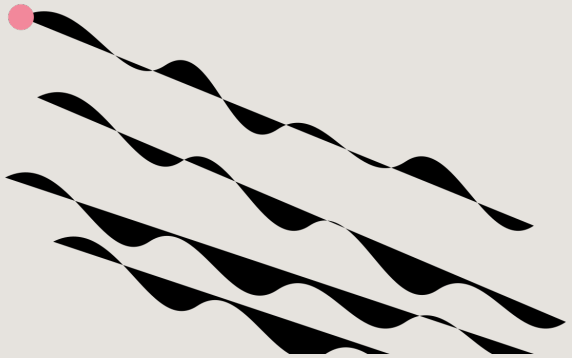
Isolated stakeholders with minimal patient engagement

Currently, only the EMA fulfills this mission of true patient engagement

We need an agile, intelligent system

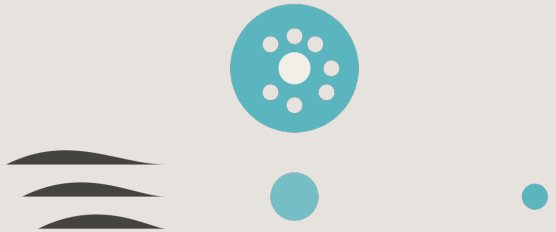
From Process-Driven to Data-Driven

CURRENT PROCESS



Rigid, complex and fragmented system

AI SOLUTION



Intelligent, agile, data-oriented system

Artificial intelligence can simplify complex processes,
extract value from data and generate actionable results

AI solution 1: Digital Twins

Model natural history. Create in-silico virtual control arms.

STEP 1

Real Patient

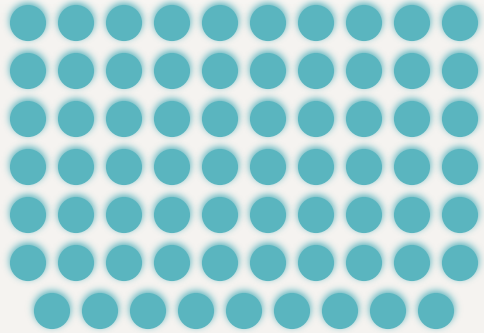
Limited data due to disease rarity

STEP 2

Digital Twin

Mathematical model based on patient data

STEP 3



Virtual Trial

Multiple virtual control arms

Solution to the small patient population problem in rare diseases

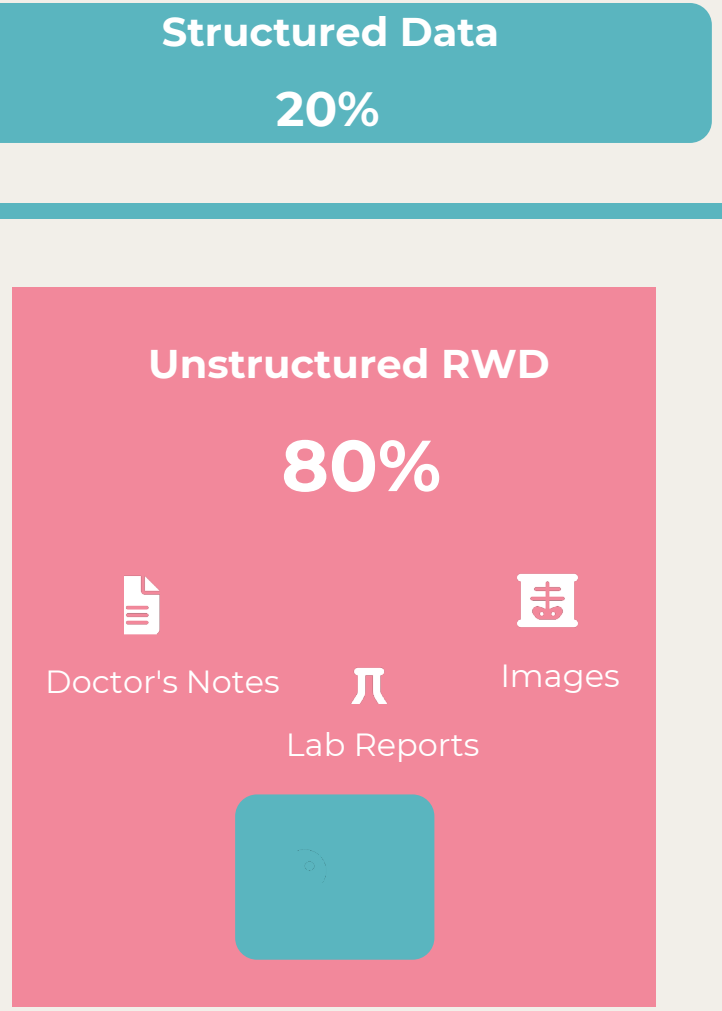
AI solution 2: Unlocking Real-World Data

80%

of clinical data is 'locked' in unstructured notes

Generative AI
Unlocks hidden knowledge

→ Actionable RWE
Data ready for research use



AI can fix the process

Agile protocols. Faster recruitment. Less burden.

Protocol design

AI simulation
before submission

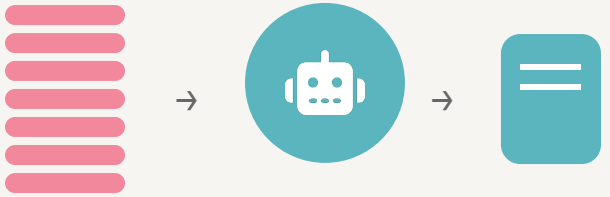
Optimizes protocols for success by simulating outcomes and avoiding common errors

Patient recruitment

Automated matching
from EHRs

Identifies ideal candidates from large clinical datasets

Admin burden



AI Scribe converts
paperwork to data

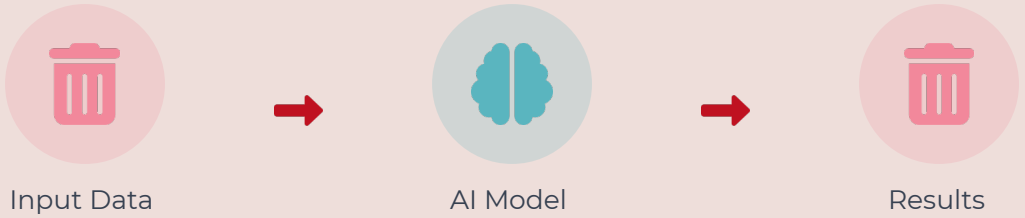
Reduces paperwork burden and accelerates document processing

AI optimizes the entire process cycle, from design through implementation and management.

The risks we must govern

Data Privacy (GDPR) & Algorithmic Bias

BIAS



"Garbage In, Garbage Out"

AI trained on ADULT data fails children

PRIVACY

Complex ethics of consent for minors

Who provides consent?

How do we protect sensitive developmental data?

What is the balance between privacy and research?

A Child-Centric framework

Reform the Process. Govern the AI.

PAEDIATRIC-FIRST RESEARCH

PROCESS REFORM

Flexible and adaptable systems

AI GOVERNANCE

Ethics, transparency and safety

The two fundamental pillars for transforming paediatric research in Europe

Thank you

> "Let's build a system that is Agile, Intelligent, and Trustworthy."

Julian.isla@foundation29.org

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