Passion for Innovation.
Compassion for Patients.™

Welcome

17th EFSPI Statistical Leaders Annual Meeting 2026

May 11-12, 2026 – Daiichi Sankyo Munich

17th EFSPI Statistical Leaders Annual Meeting 2026

AGENDA 11-May-2026

Time (CEST)	Subject
1130 – 1200	Arrivals
1200 - 1215	Welcome – Emmanuel, Anna, Giacomo
1215 – 1300	NextGen introductions & Icebreaker/Team activity Invited guests: Thilo Welz (Daiichi Sankyo), Nadeesha Lihinikadu Arachchige (Bayer), Lukas Schroeter (Boehringer Ingelheim)
1300 - 1345	Lunch
1345 - 1400	Invited Guest: Masahiro Kato, Managing Director / CEO, DS Europe
SESSION 1: Global Industry Shifts and Europe's Position in a Multi-Centered World (Leads: Giacomo, Veronique)	
1400 – 1415	Session introduction – Giacomo Mordenti
1415 - 1445	Keynote "Geopolitical Landscape", Sean Byrne, EFPIA
1445 - 1545	Panel discussion (Steffen Ballerstedt Novartis, Mike Branson UCB, Sean Byrne EFPIA, Andrea Manfrin MHRA, Armin Schueler BfArM, Benjamin Hofner PEI)
1545 – 1600	Networking break
1600 – 1645	Round table discussion
1645 – 1730	Round Table debriefs and key actions for EFSPI Stat Leaders and EFSPI council
1730 - 1745	Group photo, close Day 1 and logistics for dinner – Daiichi Sankyo Team
1745 – 1815	Hotel check-in
1815 - 1900	Transport/walk to restaurant
1900 - 2200	Dinner

17th EFSPI Statistical Leaders Annual Meeting 2026

AGENDA 12-May-2026

Time (CEST)	Subject
0800 - 0830	Arrival with coffee/tea
0830 – 0845	Welcome to Day 2, key reflections from Day 1 – Veronique/Giacomo
Session 2: AI and Statistics (Leads: Mike, Arne, Baldur)	
0845 – 0915	Session introduction – Arne, Nigel, Mike
0915 – 1000	Keynote "Evidence Before Tools: Why the Next AI Wave Must Go Through Clinical Development", Alexandre Templier, Independent consultant – Augmented Evidence Generation Strategy
1000 – 1015	Networking break with coffee/tea
1015 - 1030	Introduction to Round Table topics – Mike, Arne
1030 – 1115	Round Table Discussions
1115 – 1200	Session 2 debriefs and key actions for EFSPI Stat Leaders and EFSPI council
1200 – 1245	Lunch
Session 3: Engagement with Regulatory Agencies and Methodological Innovation (Leads: Richardus, David)	
1245 – 1300	Session introduction
1300 - 1515	Panel discussion including presentations from invited guests - Regulators: Benjamin Hofner (PEI), Armin Schueler (BfArM), Elina Asikanius (FIMEA). EFSPI Methods Leaders: Mouna Akacha, Tobias Mielke
1515 – 1530	Close Day 2: Summary and key highlights – Emmanuel

INTRODUCTION

Slides – Anna Wiksten, President EFSPI

EFSPI

European Federation of Statisticians in the Pharmaceutical Industry

Promoting Professional Standards in Europe

European Federation of Statisticians in the Pharmaceutical Industry (EFSPI)

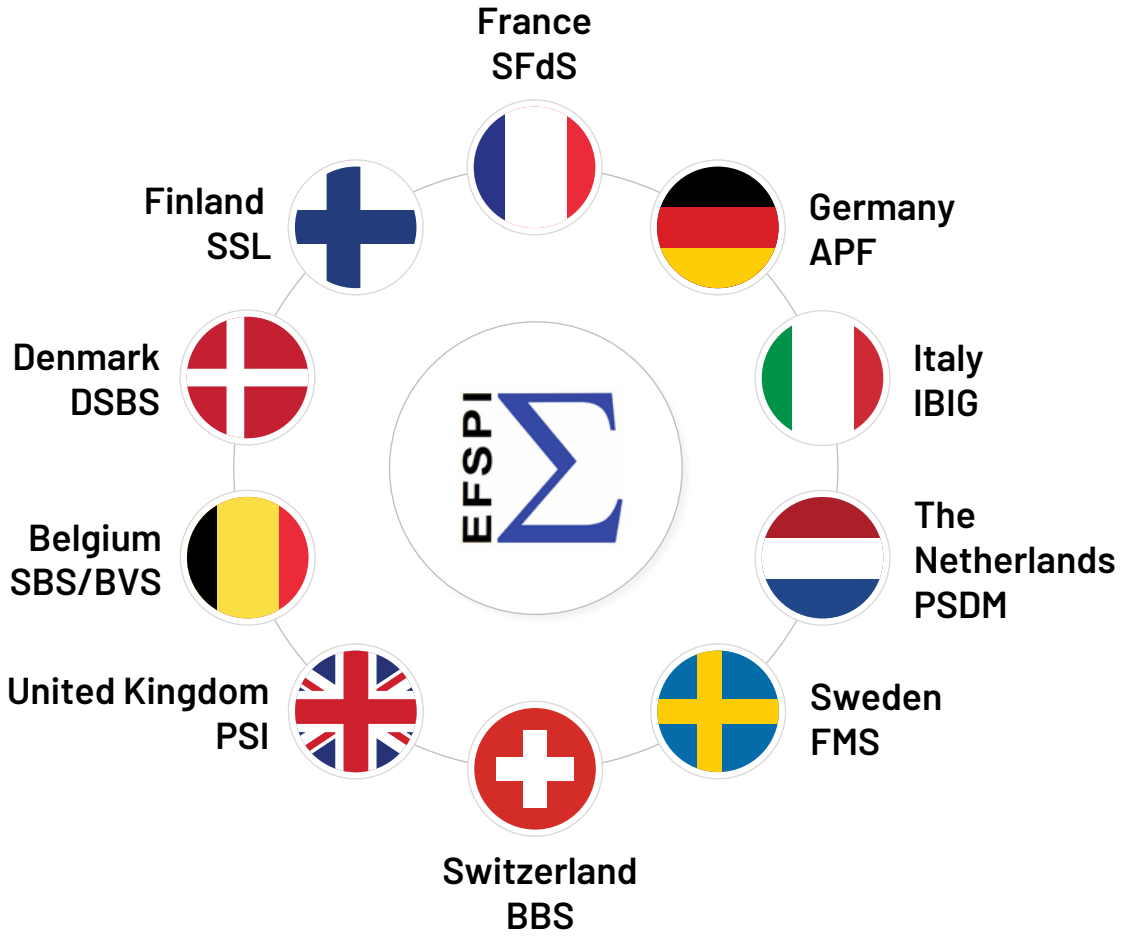
May 2026

What is EFSPI?

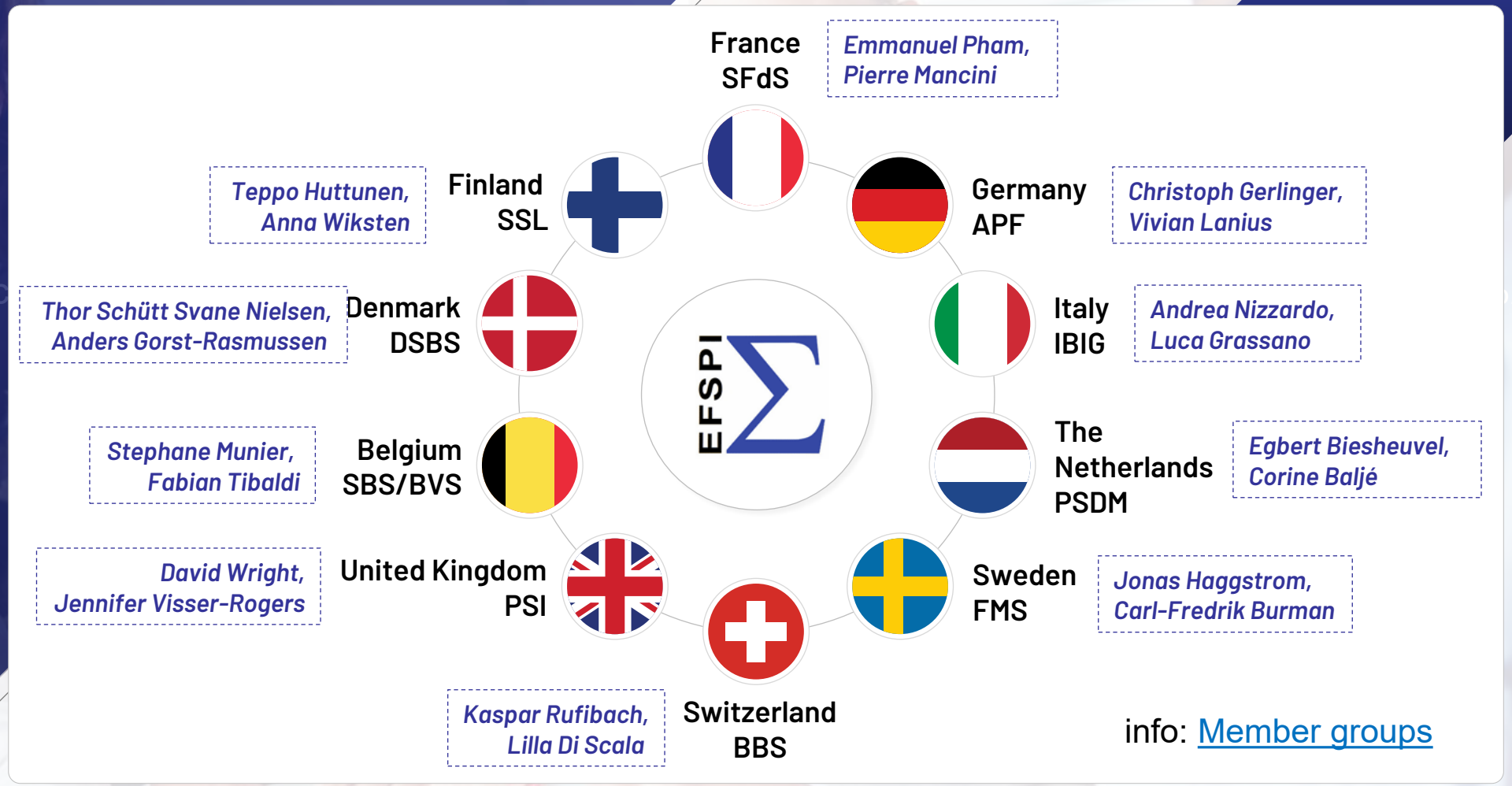
- **EFSPI = European Federation of Statisticians in the Pharmaceutical Industry**
- Founded in 1992
- EFSPI is an “umbrella”, non-profit making organisation
- A federation of National European Groups
- Now have 10 national groups
- No individual members
- Our national organisations collectively represent 2200 members
- Each organisation has 2 members on the EFSPI Council
- Website: www.efspi.org

Constitution

EFSPI Members



EFSPI Council Members



April 2026

EFSPI Objectives

- To **promote professional standards of statistics** and the standing of the statistical profession in the pharmaceutical industry
- To offer a **collective expert input on statistical matters** to national and international authorities and organisations
- To **exchange information** on and **harmonise attitudes to the practise of statistics** in the European Pharma Industry and within member groups

EFSPI Officers and Key Roles

President –
Anna Wiksten

Vice-President –
Egbert Biesheuvel

Treasurer –
Lilla di Scala

Director (legal entity) –
Thor Schütt Svane Nielsen

Scientific Lead –
Axel Krebs-Brown

Regulatory Lead –
Fredrik Öhrn

Special Interest Groups (SIGS) –
Emmanuel Pham

Statistical Methodology Leaders –
Kaspar Rufibach, Mouna Akacha

Statistical Leaders Chair –
Christine Fletcher

Stats Regulatory Workshop
Vivian Lanius

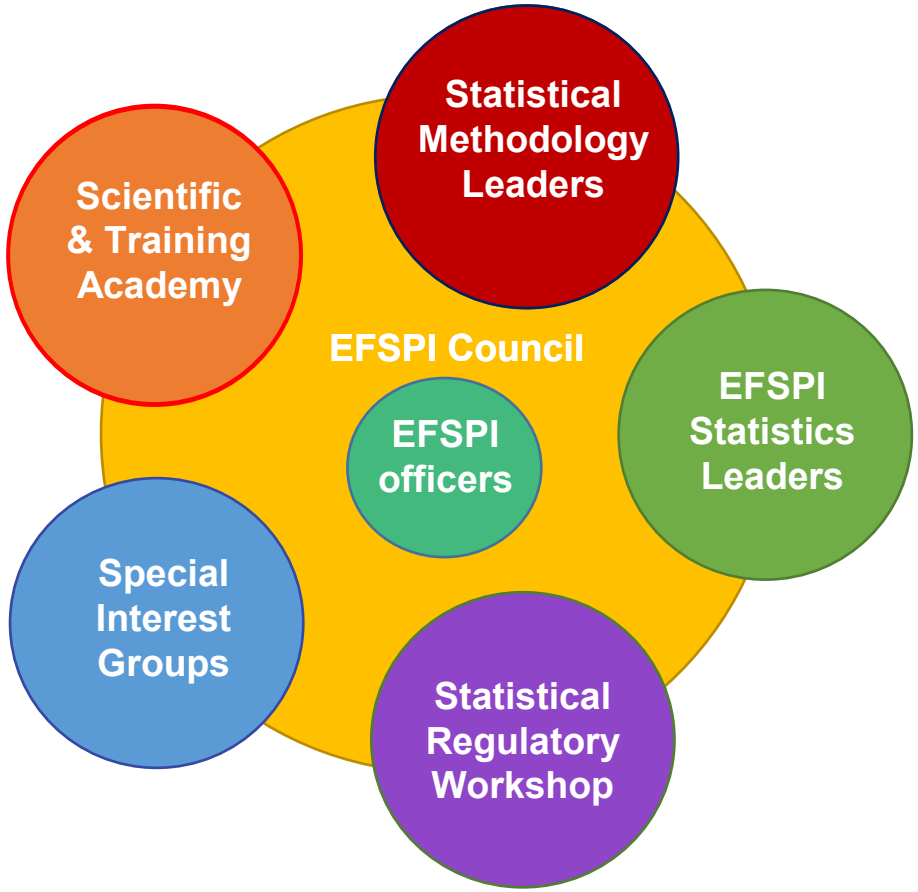
Communications Officer –
Andrea Nizzardo, Luca Grassano

EFSPI Executive Office –
Lenka Roper, Sue Rogers, Moore Engage

EFSPI Officers

EFSPI Activities

Ecosystem



EFSPI Activities – Current ESIGs

SIGS (psiweb.org)

AI and ML	AIMS	Benefit-Risk	Biomarkers
Causal Inference	CMC Statistical Network Europe	CSM/QTL	Data Science
Data Sharing	Decision Making	Early Phase	Historical Data
HTA	Launch & Lifecycle	Medical Devices	Multi-Component Endpoints
Neuroscience-Estimands	Oncology-Estimands	Patient-Focused Drug Development	Pre-clinical
Randomisation	Regulatory	RWD	Small Populations
Software Engineering	Treatment Effect Heterogeneity	Vaccines	Visualisation

11th EFSPI Regulatory Statistical Workshop

Regulatory Workshop

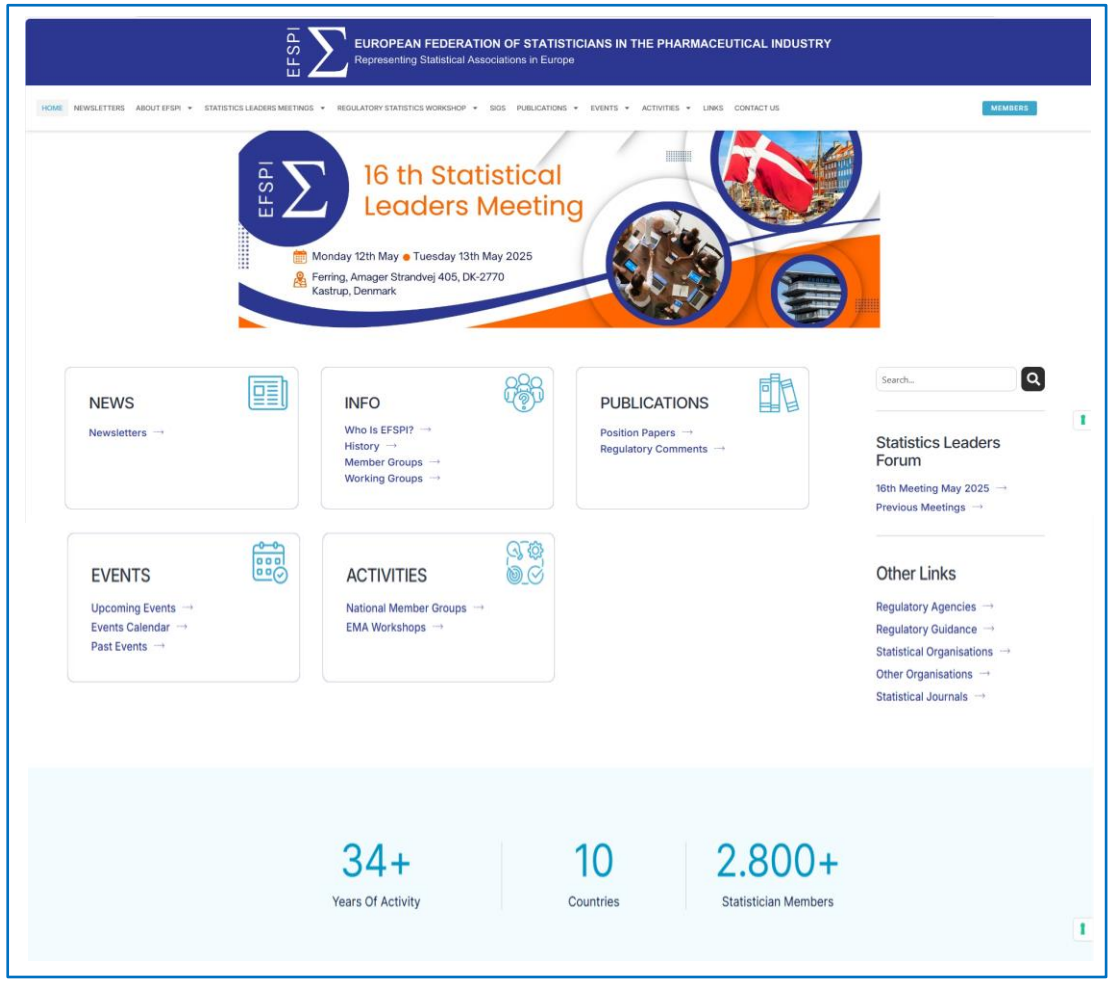
19-20 August

- Workshop with unique atmosphere of collaboration and open discussion on statistical and regulatory science in general.
- Agenda is under construction by the Scientific Committee
- Possible to submit a short topic for discussion with a panel of regulators
- Registration fees (€300 / €100(virtual))
- Block booking for face-to-face attendance:
  starting at 8400 Euro (up to 50 participants) – see website
- Block booking for unlimited virtual attendance:
  2800 Euro (break-even with 40 colleagues)

https://efspieurope.github.io/workshop/2026.html

EFSPI – Communications

www.efspi.org



NextGen Introduction

Slides – Nadeesha Lihinikadu Arachchige, Bayer
Thilo Welz, Daiichi Sankyo
Lukas Schroeter, Boehringer-Ingelheim

Nadeesha Lihinikadu Arachchige

My job: Senior Clinical Statistician at Bayer AG.

Located in Reading UK

Education and career path:

- M.Sc. in Applied mathematics (Wichita state university)
- M.Sc. in Statistics (Kansas state university)
- Lecturer of Stat/Business analytics and Director of statistical consulting center Saint Cloud State University MN

Professional interest/ topics of interest:

- Phase I and III clinical trials in oncology and cell and gene therapy
- PRO related end points

Hobbies: Running, yoga, gardening cooking, baking and cake decorating

Thilo Welz

- Biostatistician at Daiichi Sankyo Europe GmbH for 3 years, primarily working on oncology HTA
- PhD in Statistics at TU Dortmund University
  - Research on meta-regression and robust (sandwich) estimators
- Master in Mathematical Biometry (Ulm University)
  - Erasmus Semester in Vienna, Austria
  - 1 year working student at Daimler R&D Ulm
- Live with my wife near Augsburg
- In my free time I enjoy hiking, running, playing with our dog, chess, coffee and Lindt chocolate balls

Lukas Schröter

Clinical Data Scientist/Biostatistician working as trial and project statistician in oncology at Boehringer Ingelheim

- **How did I become a biostatistician?**
  - M.Sc. in Mathematics from Ulm University
  - Work on biostatistics started with an internship at Boehringer during my studies
  - Master's thesis in collaboration with Boehringer about meta-analytic Bayesian models for oncology dose-finding trials (2020)
  - Started as trial/project statistician in oncology at Boehringer in 2021

- **Professional interest/focus topics:**
  - Clinical statistics in oncology across Phase I to III trials, particularly in lung cancer
  - Bayesian methods for dose-finding trials based on up-and-down titration

- **Hobbies:** cooking, sport/fitness, soccer (at least, watching it ☺), astrophotography

Boehringer Ingelheim

SESSION 1

Global Industry Shifts and Europe's Position in a Multi-Centered World

Leads: Giacomo, Véronique

Passion for Innovation.
Compassion for Patients.™

Daiichi-Sankyo

17th EFSPI Statistical Leaders Meeting
SESSION 1: Global Industry Shifts and Europe's Position in a Multi-Centered World

Introduction

11 May 2026 – Giacomo Mordenti


WELCOME
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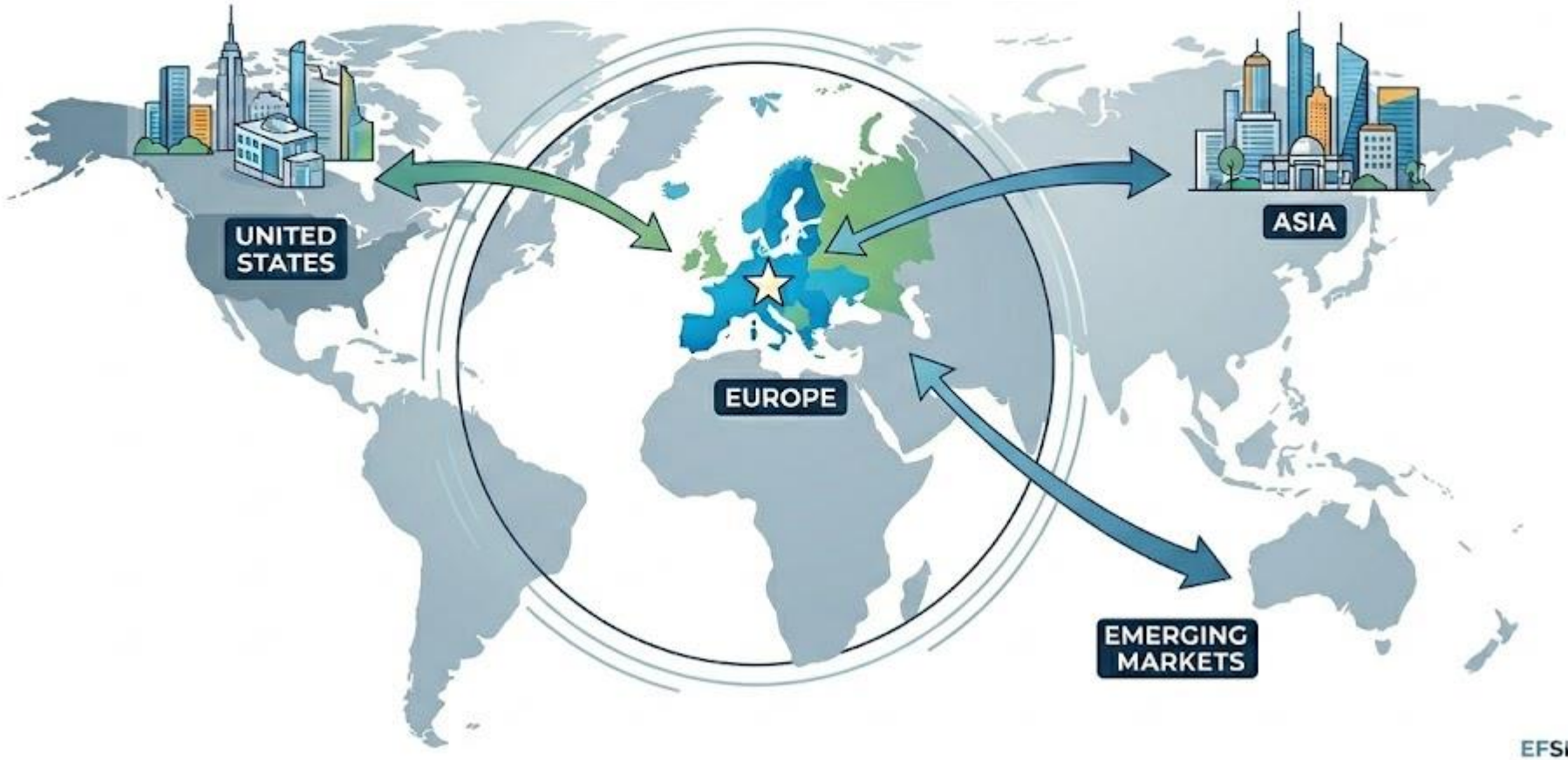
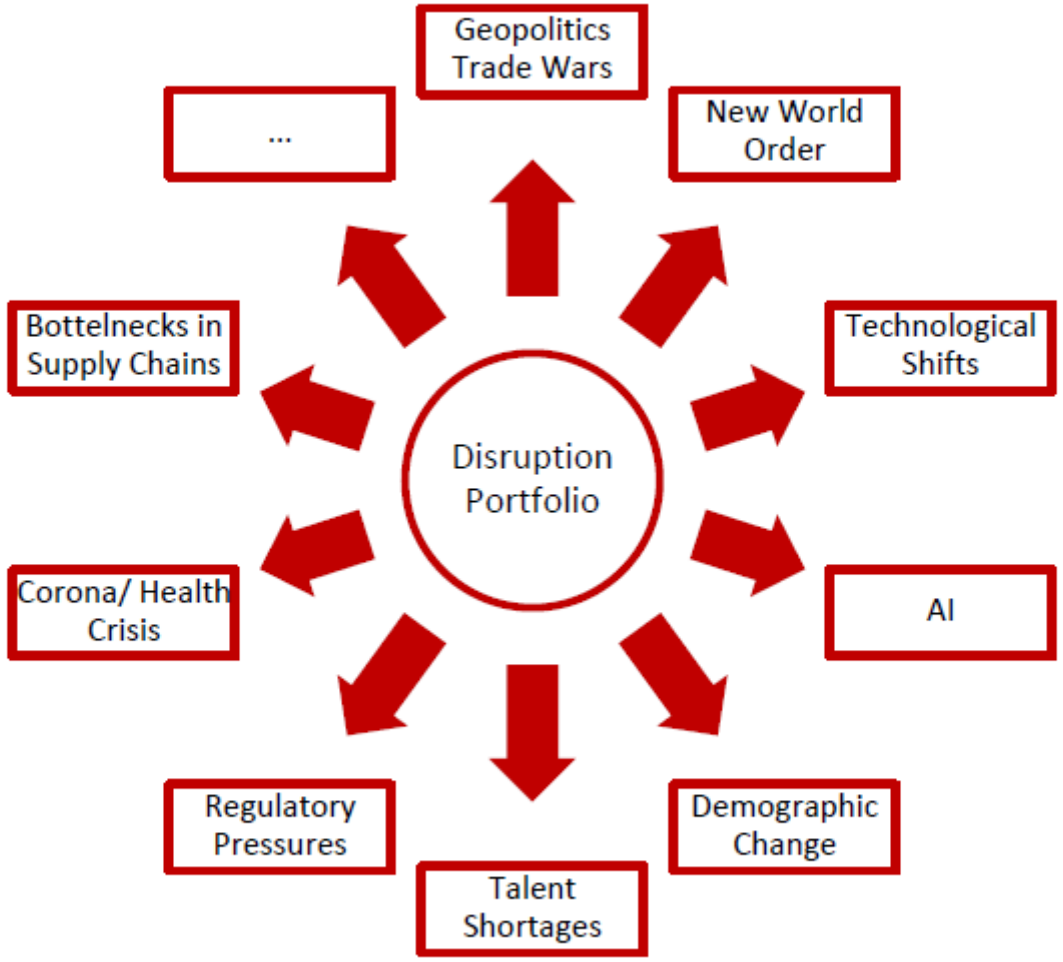
P(A|B) = P(B|A) * P(A) / P(B)

EFSPI SESSION 1: GLOBAL INDUSTRY SHIFTS AND EUROPE'S POSITION IN A MULTI-CENTERED WORLD

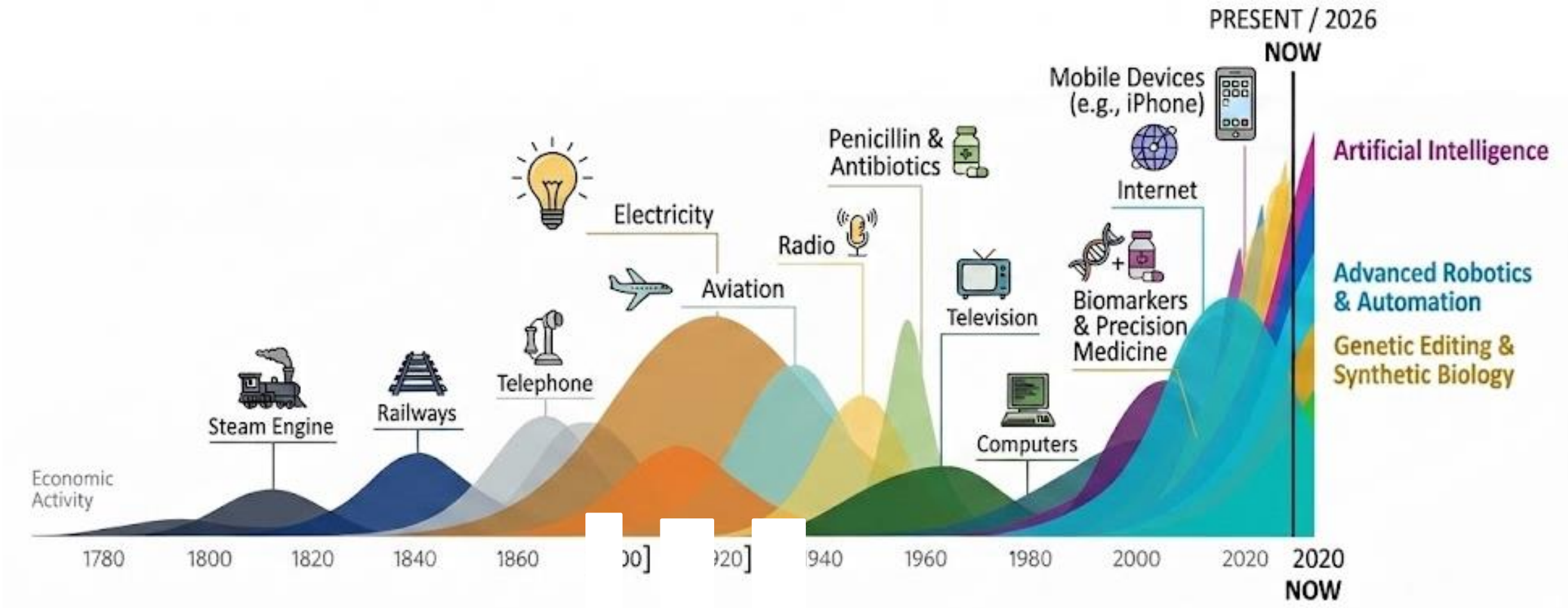



LOW
HIGH
HIGH
LOW
HOW WELL CAN YOU PREDICT THE RESULTS OF YOUR ACTIONS?
COMPLEXITY
MULTIPLE KEY DECISION FACTORS
VOLATILITY
RAPID AND UNEXPECTED CHALLENGES
VUCA
TOO MANY 'UNKNOWN UNKNOWNS'
AMBIGUITY
PENDING CHANGE: KNOWN UNKNOWNS
UNCERTAINTY

The disruption portfolio, everything is happening at the same time



The overlap of Technological Breakthrough
Ongoing Strategy Validation Required



Rise of a new global player in the pharma world

Daiichi-Sankyo

DRUG DISCOVERY & DEVELOPMENT

DRUG DISCOVERY | WOMEN IN PHARMA AND BIOTECH | ONCOLOGY | NEUROLOGICAL DISEASE | INFECTIOUS DISEASE

Chinese firms landed 6 of 26 major pharma deals in 16 months, worth $53 billion

By Brian Buntz | April 20, 2026

FINANCIAL TIMES

HOME WORLD US COMPANIES TECH MARKETS CLIMATE OPINION LEX WORK & CAREERS LIFE & ARTS HOW TO SPEND IT

Pharmaceuticals sector | + Add to myFT

Why investors are chasing US biotechs with Chinese characteristics

Drugs developed in Asian nation are increasingly being used to seed US start-ups

Pinsent Masons

Expertise People Thinking

... > News > Europe risks 'being left behind' as China fills global pharma pipeline gaps

OUT-LAW / YOUR DAILY NEED-TO-KNOW

OUT-LAW NEWS 1 min. read

Europe risks 'being left behind' as China fills global pharma pipeline gaps

ING

Home Markets Economy Sectors Webinars Forecasts Meet the team

Healthcare

Articles | 20 January 2026 | 10 min read | Italian version

Hello, Goodbye: Europe loses ground as China becomes powerhouse of innovation

Reuters

World Business Markets Sustainability Legal Commentary Technology

China biotech licensing boom to hit record in 2026 as pipeline swells

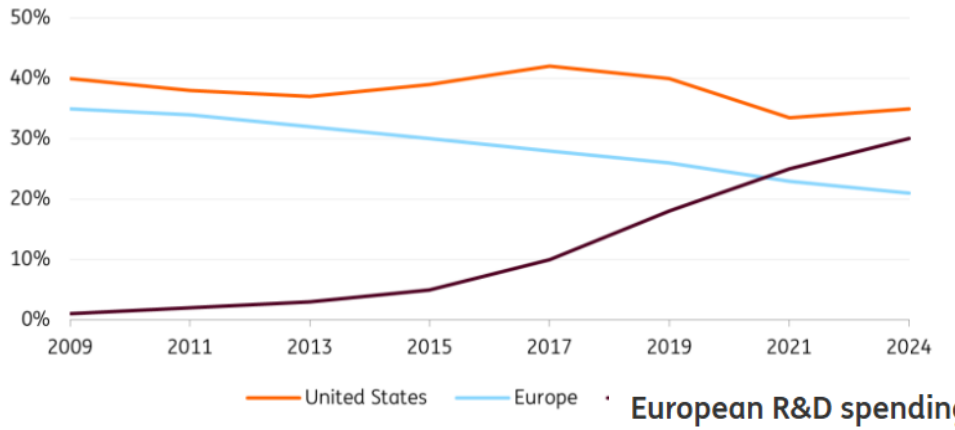
By Kane Wu and Andrew Silver

February 13, 2026 10:24 AM GMT+1 · Updated February 13, 2026

The lost opportunities for Europe

China has surpassed Europe with the percentage of started clinical trials

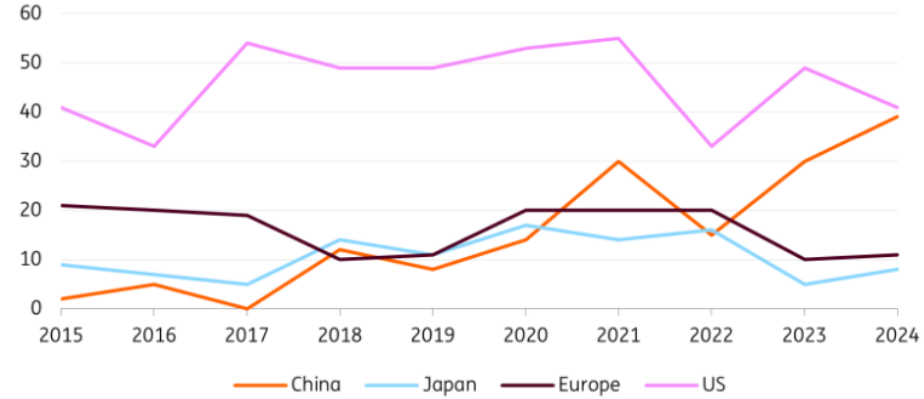
Clinical trial started by company location in percentages of global total



Source: IQVIA

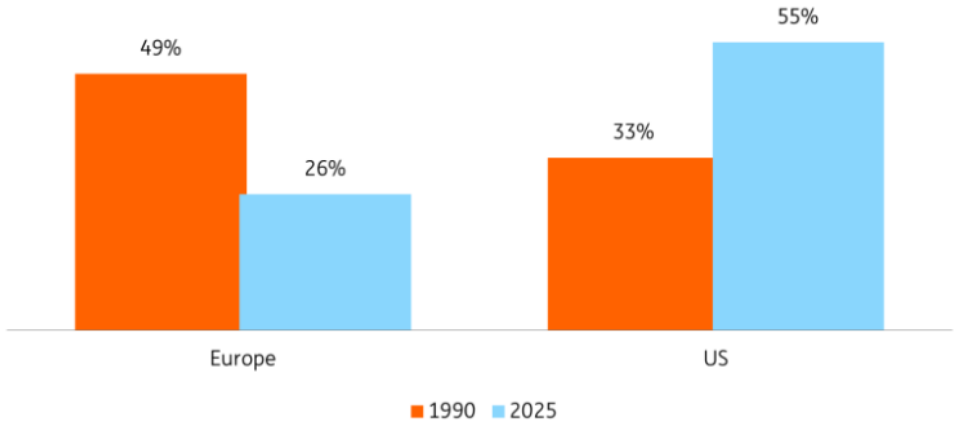
China has surpassed Europe in global drug approvals

Distribution of first global approvals of innovative drugs, 2015-24



European R&D spending has declined rapidly compared to the US

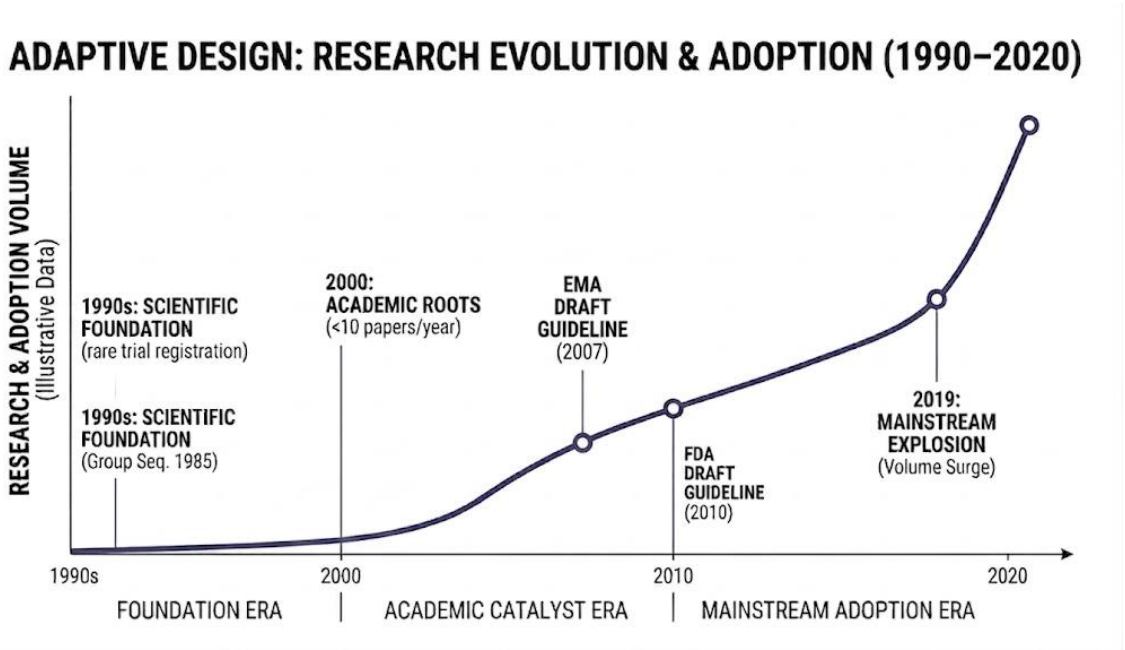
European and American biopharmaceutical R&D spending as a percentage of global spending in 1990 and 2025

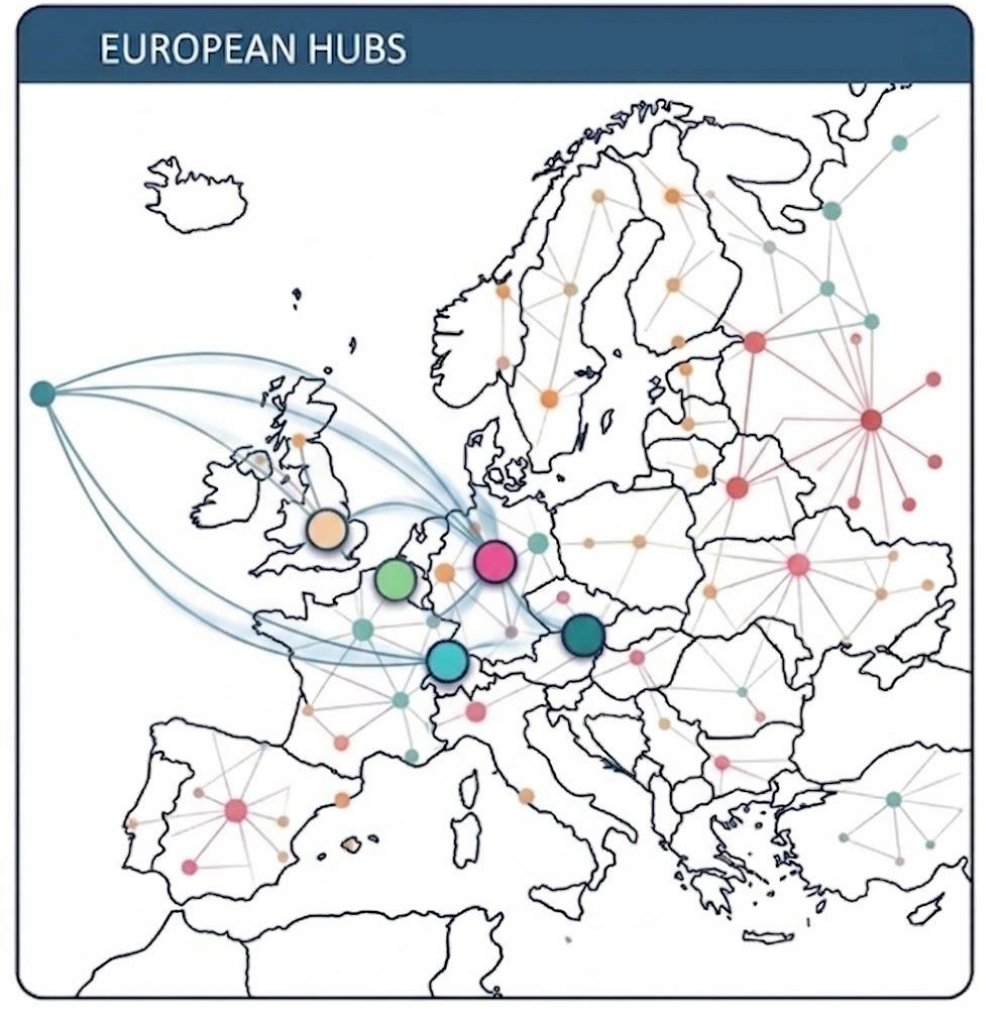


Source: ITIF; ING

https://think.ing.com/articles/why-it-is-the-eleventh-hour-for-european-pharmaceutical-innovation/

We have a success story we can be inspired by





A call for action

What is our role as European Statistical Leaders in:

- Navigating divergent regulatory expectations
- Ensuring coherent global evidence strategies with structured tailoring
- Remaining flexible to adapt and seize emerging opportunities
- Driving Innovation Adoption
- Adapting to Evolving Organizations and Capability Distribution

SESSION 1: Global Industry Shifts and Europe's Position in a Multi-Centered World (Leads: Giacomo, Veronique)	
1400 – 1415	Session introduction – Giacomo Mordenti
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1445 - 1545	Panel discussion (Steffen Ballerstedt Novartis, Mike Branson UCB, Sean Byrne EFPIA, Andrea Manfrin MHRA, Armin Schueler BfArM, Benjamin Hofner PEI)
1545 – 1600	Networking break
1600 – 1645	Round table discussion
1645 – 1730	Round Table debriefs and key actions for EFSPI Stat Leaders and EFSPI council

SESSION 1 – Breakout Groups

Group 1 – Room Westminster

- Anna Karina Trap Huusom (F)
- David Wright
- Dominik Heinzmann
- Guillaume Desachy (S)
- Mike Branson
- Per Soerensen
- Del Jones (online)
- Emmanuel Zuber
- Sean Byrne (online)
- Andrea Manfrin (online)
- Kate Peacock (online)
- Jianchang Lin (online)

Group 2 – Room Louvre

- Alessandro Previtali
- Egbert Biesheuvel (F)
- Florence Caset-Semanaz
- Jennifer Visser-Rogers (S)
- Kim Hacquoil
- Nigel Howitt
- Randi Gron
- Steffen Ballerstedt
- Torsten Westermeier
- Benjamin Hofner
- Lukas Schroeter

Group 3 - Room Hyde Park

- Anna Wiksten
- Thilo Welz
- Jianchang Lin
- Lara Wolfson (F)
- Pantelis Vlachos (S)
- Richardus Vonk
- Susan Lovick
- Amanda Darekar
- Armin Schueler
- Mickael Guedj

Group 4 - Room Big Ben

- Arne Ring
- Estelle Lambert
- Julia Igel (S)
- Lilla DiScala
- Lucy DeCosta
- Paula Edwards-Holmes
- Rene Kubiak
- Simon Wilcock (F)
- Josie Wolfram
- Nadeesha Lihinikadu Arachchige

F Facilitator S Scribe

Session 1 Feedback

Global Industry Shifts and Europe’s Position in a Multi-Centered World

Leads: Giacomo, Véronique

Group 1

R&D Investment & Clinical Trials in Europe

R&D spending in Europe is consistently outpaced by the US and faces increasing competition from China. The number of global clinical trials conducted in the EU has declined, resulting in fewer clinical trial opportunities for European patients and sites.

What challenges and opportunities arise in this context, and, if EFSPI were to lead a single coordinated action to support increased clinical trial activity in Europe, what should that priority be?

Group 1

R&D Investment & Clinical Trials in Europe

Context:
R&D spending in Europe is consistently outpaced by the US and faces increasing competition from China. The number of global clinical trials conducted in the EU has declined, resulting in fewer clinical trial opportunities for European patients and sites.

Discussion Questions:

Challenges

- What are the key statistical or operational challenges your teams face due to these evolving trends in clinical trials in Europe?
- How does the competitive landscape in R&D investment between Europe, the US, and China impact your statistical planning, clinical trial designs or development plans?

Opportunities

- What statistical strategies or advanced statistical methods could help in this context?
- How can statistical teams contribute to improving clinical trial or development plan efficiency and competitiveness in Europe?

Call to Action

- If EFSPI were to lead one single coordinated action to support increased clinical trial activity in Europe, what should that priority be?

US: .7% of GDP invested in drug innovation

EU: .36% of GDP invested in drug innovation

What are the key statistical or operational challenges your teams face due to these evolving trends in clinical trials in Europe?

- **Fragmented development:**
  Often two trials needed to get to market (EU vs US)
- **Comparator choice dilemma:**
  Shifting standards of care, pricing pressure, misaligned regulators
- **Shrinking EU footprint:**
  EU might lose drug innovation
  Fewer EU patients → harder extrapolation justifications?
- **Future feasibility risks:**
  Right comparator unavailable in the EU; uneven EU/US data quality
- **Structural imbalance:** US far more expensive per patient, yet increasingly favored for investment
- **Regulatory friction:** EU vs US vs China data-sharing constraints

What statistical strategies or advanced statistical methods could help in this context?

- Historically, China last-in with data borrowing
  What if the EU was forced to move to that model?

- If EU footprint reduced, investigate how to better use Real-World Evidence and External Control Arms

How can statistical teams contribute to improving clinical trial or development plan efficiency and competitiveness in Europe?

- Strengthen dialogue with the agencies
  💡Discuss with the regulators multiple times a year
  - Endpoints convergence between regulators
  - Expand accelerated approvals, including strengthening use of biomarkers

- Further share the constraints we are under

Recommendations for EFSPI Council

- Increase frequency of discussions with regulators
- Discussions need to be targeted
- Bridge working groups through ASA and EFSPI

Group 2

Statistical Innovation & Regulatory Environment

Context:
Differences in regulatory approaches between the FDA and EMA contribute to variations in approval timing, volume, and patient access, raising strategic considerations for global pharma. At the same time, recent FDA initiatives signal a broader shift toward innovation in regulatory science in the US, including, for example, FDA's endorsement of Bayesian methodologies.

Discussion Questions:

Challenges

- What are the key statistical risks and challenges presented by the differing regulatory environments across regions?
- What has been the most significant technical compromise your team has made to manage these divergences?

Opportunities

- Which statistical innovations or methodologies could be leveraged or adapted to improve regulatory submissions across regions?
- Where do you see the greatest opportunities within these evolving trends?

Call to Action

- If EFSPI were to lead one single coordinated action to enhance regulatory success and alignment across regions, including acceptance of innovative statistical methods, what should that priority be?

Group 2

Challenges

- What are the key statistical risks and challenges presented by the differing regulatory environments across regions?
  - Different regulatory approaches
    - Is it really different – or is it just better communication? Bayesian isn't new
    - FDA has a different platform
    - FDA have explicit guidance docs and publications – doesn't mean that EMA wouldn't accept
  - Can trigger conversations that we are not ready for
  - Bayesian, RWE, single pivotal, real-time trials
  - Should be a better dialogue between technical experts and "politics"
  - Biggest challenges are where differences in approaches affects the design of the study
- What has been the most significant technical compromise your team has made to manage these divergences?
  - Estimand compromises and multiple endpoints for different regions

Group 2

Opportunities

- Which statistical innovations or methodologies could be leveraged or adapted to improve regulatory submissions across regions?
  - Extrapolation to other populations/treatment regimens
  - Don’t use advanced tools in these areas
- Where do you see the greatest opportunities within these evolving trends?
  - In Europe we can stand up and talk about innovation
  - Get better at communication
  - How do we speed this up – probably more robust but the system makes it slow
  - Can we have global guidelines

Group 2

Call to Action

- If EFSPI were to lead one single coordinated action to enhance regulatory success and alignment across regions, including acceptance of innovative statistical methods, what should that priority be?
  - Could EFSPI lead the production of case studies of actual pivotal studies that have been done
  - We could use the EFSPI expertise to go out and educate
  - Prioritisation of main “pain points”

Group 3

Market Access & Evidence coherence

Longer EU post-approval time-to-access, alongside evolving US pricing reforms, is reshaping global market access strategies, including potential shifts in launch sequencing across regions. Together, these dynamics raise increasing concerns about the attractiveness of the EU as a launch market for global pharmaceutical companies.

What challenges and opportunities arise in this context, and, if EFSPI were to improve Market Access either in terms of speed or extent, what should that priority be?

Key messages/discussion points

- Implementation of JCA was supposed to speed up approval. It is really getting longer?
- **Challenges**
  - EU evidence expectations, comparator choices, population definitions, national payer requirements diverge
  - Not just speed is needed, but coherence of evidence
  - For sponsors: Misalignment on target populations, subgroups, estimands, endpoints, comparators, external controls
  - For patients: uneven access

Key messages/discussion points (cont)

- **Opportunities**
  - JCA can reduce duplication, make evidence expectations clearer
  - EFSPI can position biostats as the discipline that connects regulatory validity, HTA relevance, and decision making
  - Plan for trials and RWE plans rgar are useful BEYOND regulatory approval

Recommendations for EFSPI Council

Promote early joint evidence planning

- Estimands, endpoints, comparators, subgroups
- Make evidence package more decision-ready
- Increase market access intelligence in clinical trial design and execution
- Articulate principles for good design collection and analysis of patient collected data
  - Educate clinops teams in issues like PRO completion
  - Build templates for evidence generation
- Use SIGs for sharing and education
  - Clarify, educate on differences on by country access

Recommendations for EFSPI Stats Leaders

- Equip statisticians to understand the dynamics of the changing policy landscape
- Be part of the discussions
  - Go beyond the fundamental skills and invest in time and education
- Set up a team, build good network

Group 4

Sustaining Europe as a Strategic “Center of Excellence”

Europe possesses unique value, particularly through its scientific rigor, data quality, and methodological leadership. The European Union continues to advance its regulatory and statistical frameworks, focusing on accelerating innovation, enhancing patient access, and strengthening regulatory science. There is a need to explore pathways to strengthen Europe’s global influence and ensure it remains a strategic "Center of Excellence" in the evolving multi-centered pharmaceutical landscape.

What challenges and opportunities arise in this context, and, if EFSPI were to lead a single coordinated action to ensure Europe remains a leading “Center of Excellence” and asserts greater influence within the global industry, what should that priority be?

Key messages/discussion points

- Are we clear about our own value?
- How is the quality of statistical talents in Europe perceived by others? How can we influence that?
- How do we sell ourselves?
  - Cost vs expertise in low-cost countries
  - Recommendations on following slides
- Where do we run clinical trials, who are the stakeholders of those trials and where do we have associated staff?

Recommendations for EFSPI Council

- Do good things **and talk about it**
- Increase visibility globally, present European Statistical community at global conferences, EFPSI on tour (poster, presentation) – not limited to Stats
- Set SMART objectives / KPIs for SIGs - outcome and the impact?
- Not only compare with US, look East as well
- Use EFSPI Academy to promote European Stats community
- Guidelines for curricula at universities

Recommendations for EFSPI Stats Leaders

- Advocate for EFSPI outcomes and deliveries
- Ensure we have adequate contributors, encourage statisticians of ALL levels to contribute
- Create an environment to help emerging talents grow and get out of their “purely statistical corner”
- Understanding cultural differences

17th EFSPI Statistical Leaders Annual Meeting 2026

AGENDA 12-May-2026

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1515 – 1530	Close Day 2: Summary and key highlights – Emmanuel

SESSION 2

AI and Statistics

Leads: Mike, Arne, Baldur

AI and Statistics

Session 2 of the 2026 EFSPI Statistical leaders meeting

12-May-2026

AI and Automation in Statistics: Leading into the Future

- ***German Head of Stats 2026***
  - AI is not optional–it is the electricity of our time
  - We must make AI work for us
  - It is not a question of either (statistics) or (AI) – it has to be used together
  - Do we choose to shape –or to react?
- ***EFSPI: 2 topics***
  - Automation and AI (Nigel)
    - Role of statisticians in defining the capabilities of automation and AI tools in drug development and evidence generation
    - Navigating the landscape of AI stakeholders across organizations.
  - AI and statistics (Alexandre)
    - cultivating next-generation biostatistics talent and evolving our professional culture;
    - driving innovation through AI and empirical modeling;

AI – Considerations from a CRO perspective

Nigel Howitt

May 2026

Nigel Howitt – My journey with AI

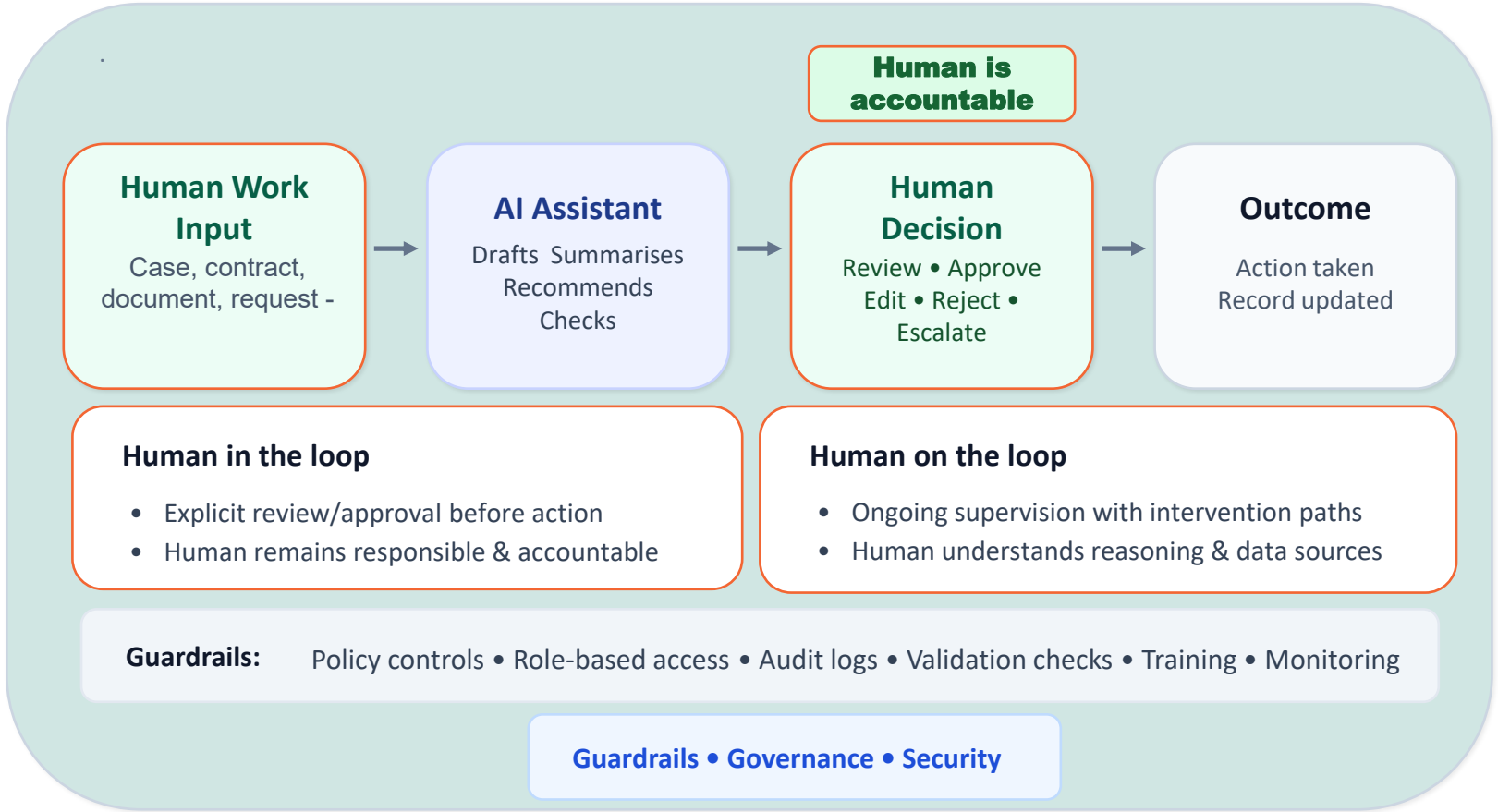
VP Biometrics and Functional Delivery (ISS)

- Global leader of Biometrics and Functional Delivery
- 32 Years of experience in CRO Industry
- Global leader for 17 years
- Previous VP of Biostatistics and Statistical Programming, FSO and FSP at Fortrea
- Previous President of EFSPI and Chair of PSI
- Postgraduate Diploma in Mathematical Statistics from Cambridge University
- Postgraduate Diploma in Business Administration

Why AI investment is becoming unavoidable for CROs?

Survival

Accountability and Data Security



What this means for you

- People stay in control: AI proposes, humans decide
- Clear accountability: the human role owns outcomes
- Transparency: reviewable outputs and audit trail
- Safety: guardrails and escalation paths by design
- AI is an aid to the human role — not a replacement for judgment, responsibility or accountability

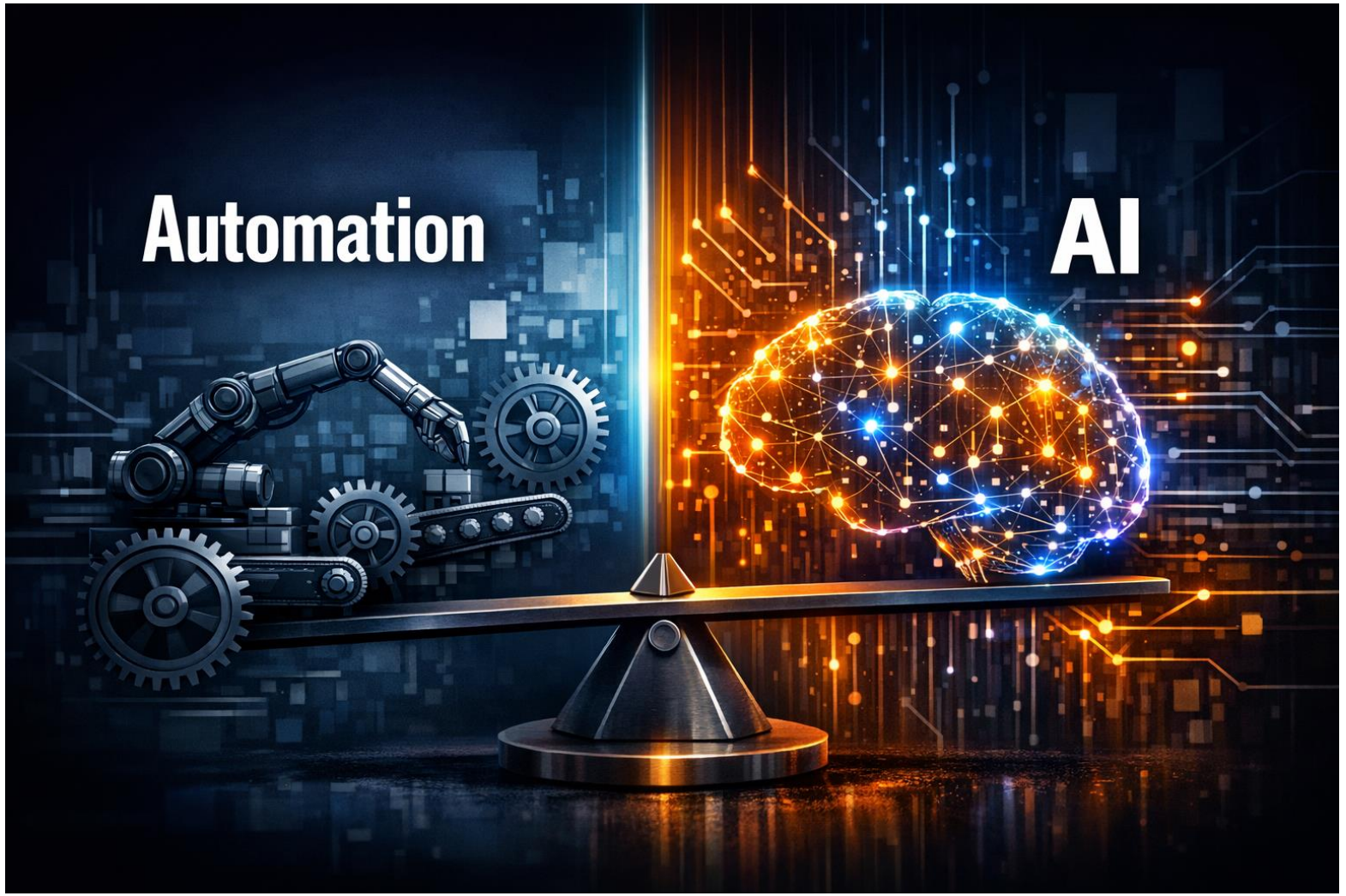
Deployment & Data

Security of your Data

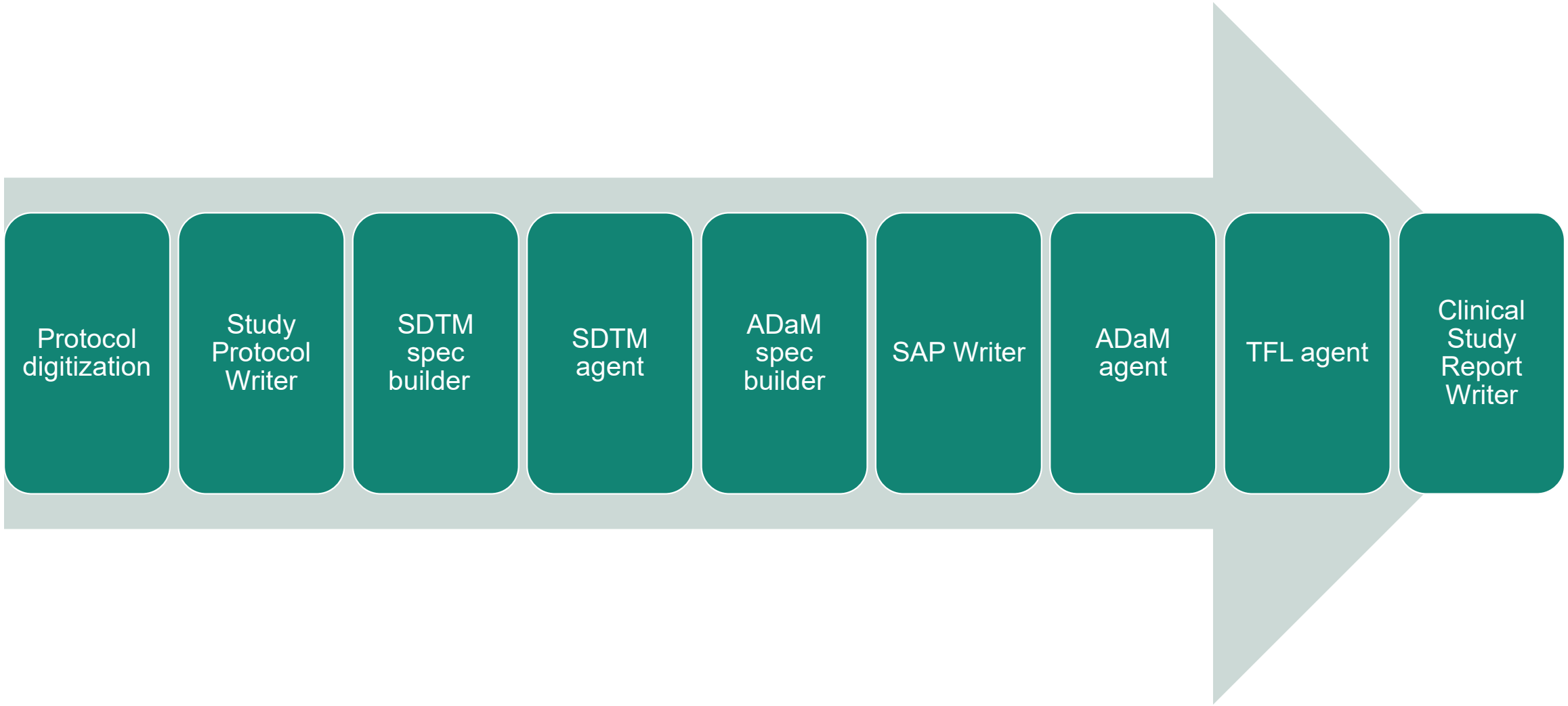
Enterprise controls:

- **Isolation • Access control**
- **Auditability • Monitoring**

What do I if I have reliable automation tools in place?



What types of AI Agents are possible?



What levels of efficiency are possible in biometrics using AI?

Statistical Programming
Key opinion leader = 20%
Boston Consulting = 40-60%

Medical Writing
CSR AI Agent = 30%

ICONplc.com

AI and statistics

Roundtable discussion

Where are we today, where are we heading, and how do we lead our organizations through this transition?

Core themes:

- Cultivating next-generation biostatistics talent and evolving our professional culture
- Driving innovation through AI and empirical modeling
- Our responsibility as shepherds of rigorous and credible evidence
- Engagement with regulators and HTAs

Surface key actions and commitments

AI and statistics

Pre-read questions

- Given the advancement of AI technology, what do you think the world of pharmaceutical statistics (or, more broadly, drug development) will look like 1 year from now? 3 years? 5 years?
    - To help sharpen the lens: Reflect of one decision your team made in the last year that AI will likely handle (or significantly inform) within five years. How does that shape your view of your role as a leader?
- What are the key opportunities you see for our discipline in this regard?
    - What is one thing about how we currently develop biostatisticians that you think most needs to change, and what would you replace it with?
    - What can EFSPI do to enable statistical leaders to be successful?

Shape it, or inherit it: those who wait to engage will engage on someone else's terms

SESSION 2

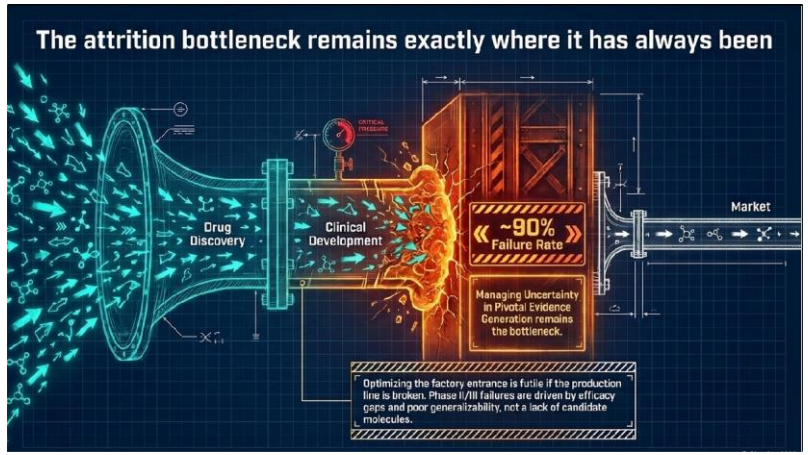
Keynote “Evidence Before Tools: Why the Next AI Wave Must Go Through Clinical Development”

Slides – Alexandre Templier, Independent consultant – Augmented Evidence Generation Strategy

Spoken commentary
Innovations are neither bad nor good in themselves. What matters is how we understand them, embrace them, and use them. In the next 20 minutes, I will walk you through the risks and opportunities of Machine Learning in clinical evidence generation - intentionally leaving aside Generative AI and Large Language Models which - in themsleves - deserve a specific and separate set of discussions. My hope is that you leave this room with a sharper sense of the role statisticians must claim right now, before others claim it for them.

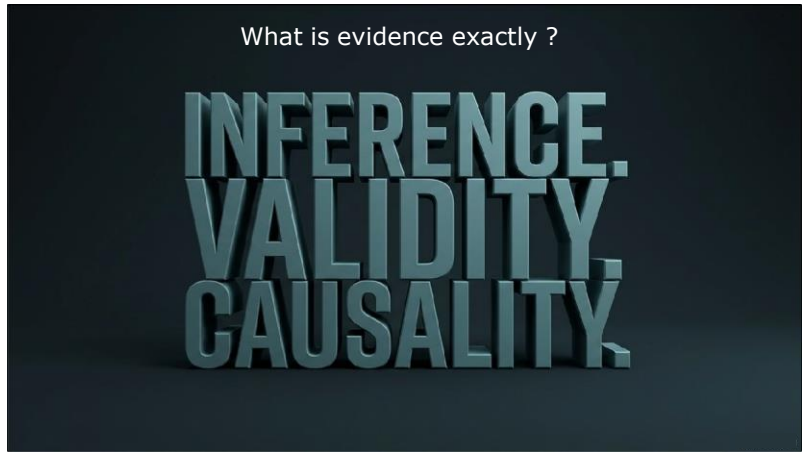
Extended note
During the post-session conversations, several participants articulated something that I had not formulated explicitly in the talk but which seems to me central in retrospect. Conversational generative AI (LLM) is only as good as you believe it to be. It is also only as bad as you believe it to be. But it can also be as bad as you do not know you are. The technology functions as a resonance amplifier of the user's intellectual honesty, of the rigor of their methodological framework, and of their willingness to be challenged. For a community of biostatisticians whose entire training is built around discipline and skepticism, this should be a comparative advantage, not a threat.

Spoken commentary
Everyone is focused on AI in drug discovery. Faster molecule identification, generative chemistry, target prediction.
That is where most of the industry investment has been going over the last 10 years.
But look at the actual number.
Ninety percent failures. 9 out of ten products tested in Phase I do fail for 1 being successful in Phase III. For decades.
Despite genomics, despite biomarkers, despite every wave of innovation this industry has been celebrating.
The number has not moved.
So before we talk about what AI changes, we need to be honest about what has not changed at all - and why.

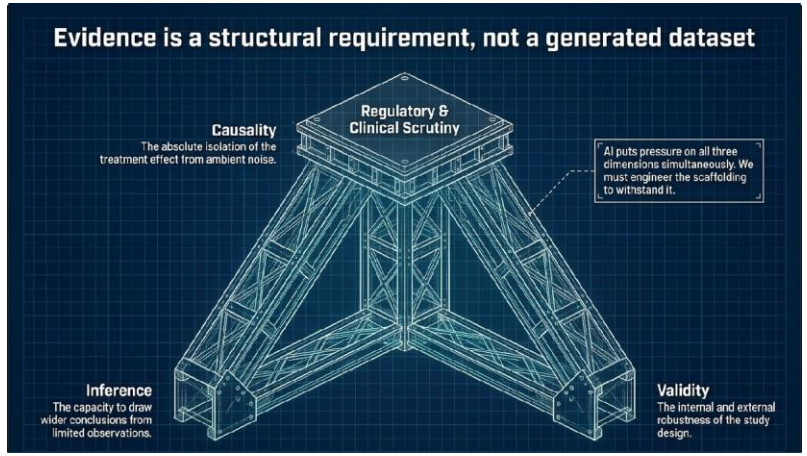


Spoken commentary

The bottleneck is - and has always been - clinical development.
Specifically: managing uncertainty in pivotal evidence generation.
Phase Ⅱ and Ⅲ failures are driven by efficacy gaps and poor generalizability, not by a shortage of candidate molecules.
We have been optimizing the factory entrance while the production line remains broken.
Applying AI upstream of this bottleneck is like adding lanes to a highway that leads to a wall.
That is the kind of reframe this industry needs.



Spoken commentary
Before we talk about what AI can do to evidence, we need to be precise about what evidence actually is.
It is not data. It is not a dataset, however large, however synthetic.
Three properties define it - and they are not methodological preferences.
They are structural requirements.

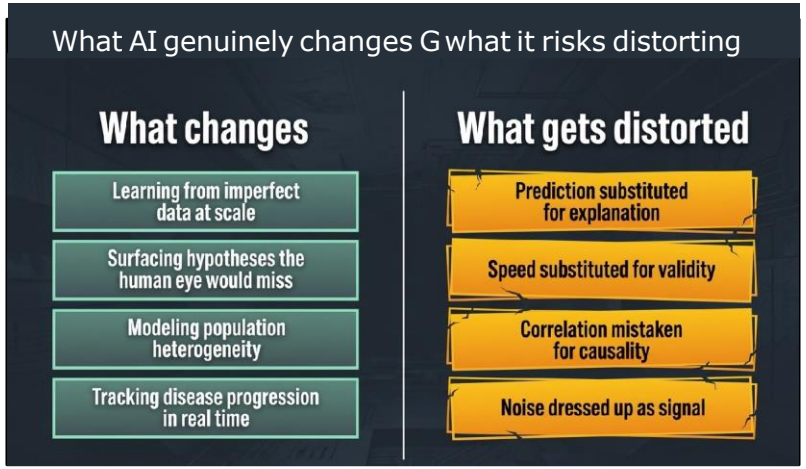


Spoken commentary

Causality: the absolute isolation of the treatment effect from ambient noise.
Inference: the capacity to draw wider conclusions from a limited set of observations.
Validity: the internal and external robustness of the study design.

Remove any one of these three, and you no longer have evidence.
You have data. Possibly large data. Possibly fast data. But not evidence.
That distinction is not semantic. It is the difference between a drug that reaches patients and one that does not.

AI puts pressure on all three simultaneously.
That is not a reason to reject AI.
It is the architectural challenge we need to engineer around - with statisticians in the room, not observing from the outside.



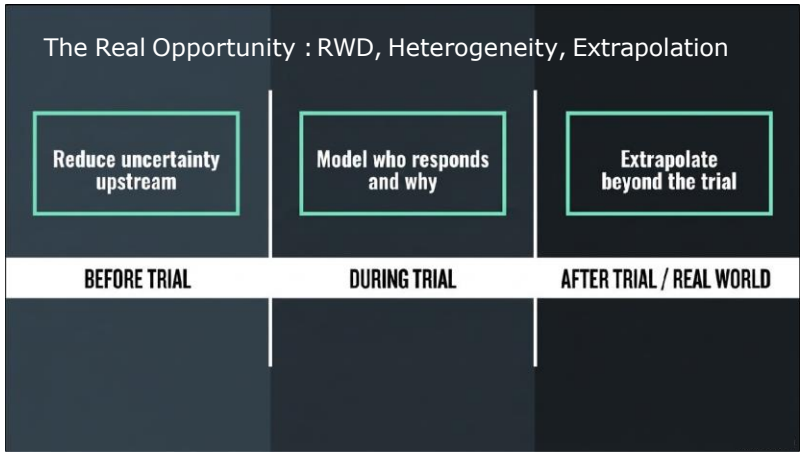
Spoken commentary

Here is the honest map :
Left side: what AI genuinely changes.
- Learning from imperfect data at scale.
- Surfacing hypotheses that conventional stratification would never reach.
- Modeling population heterogeneity with much higher resolution.
- Tracking disease progression in real time.

These are real contributions, happening as we talk.

Right side: what gets distorted when AI is applied without statistical governance.
- Prediction substituted for explanation.
- Speed substituted for validity.
- Correlation mistaken for causality.
- Noise dressed up as signal.

The problem is not AI. The problem right now is using the left-hand list to justify ignoring the right-hand list.

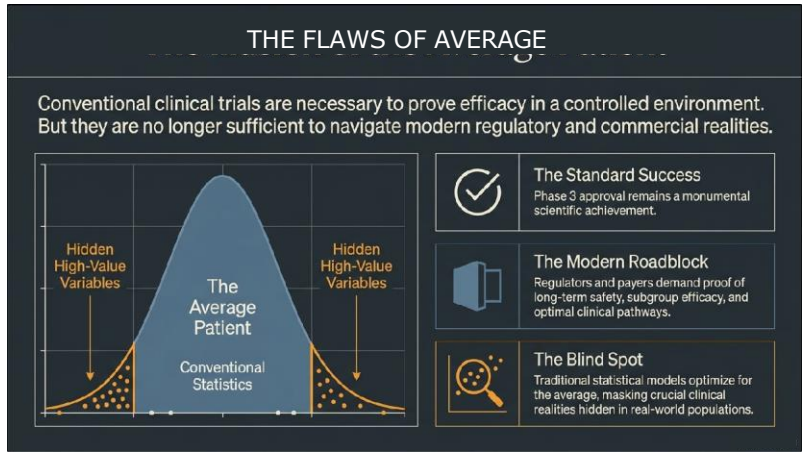


Spoken commentary

If we focus on how the evidence generation process is actually structured, three moments stand out :

- **Before the trial:** we lock designs without reducing upstream uncertainty as much as we could.
- **During the trial:** we treat the population as uniform, when heterogeneity is the clinical reality.
- **After the trial:** we fail to extrapolate outcomes into the real world beyond the enrolled population.

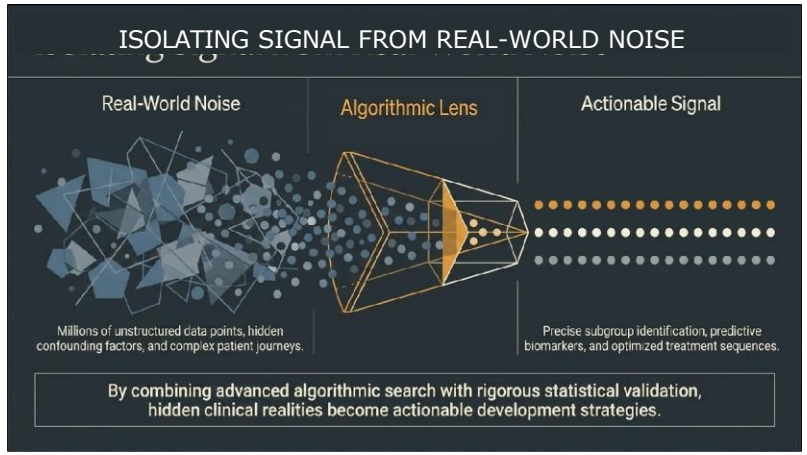
AI has something genuinely useful to offer in all three phases - **but only if statisticians are shaping how it is used**, not receiving its outputs at the end.



Spoken commentary

The conventional clinical trial remains indispensable.
Phase 3 approval is a monumental scientific achievement - I want to be very explicit about that.

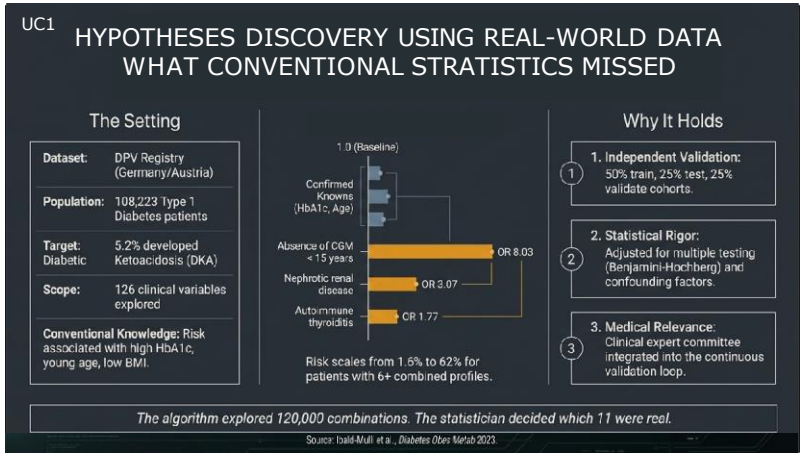
But it has a structural blind spot : It optimizes for the average patient.
A patient who, in most therapeutic areas, does not actually exist.
The bell curve captures the center. What it systematically misses are the tails - high-value variables that predict who responds and who does not.
Regulators and payers now demand exactly what the average cannot deliver: subgroup efficacy, long-term safety, optimal clinical pathways. Conventional statistics is not wrong. It is no longer sufficient on its own.



Spoken commentary

The algorithmic response is not to replace statistical reasoning - it is to extend it. What machine learning does well is search: exploring combinatorial spaces that would take decades by hand.
What statisticians do well is decide: determining which combinations represent a real clinical signal and which is noise.
Read the bottom line of this slide carefully. That is the collaboration model.

Now let's see what it looks like in practice through a few examples.



Spoken commentary

First case (https://dom-pubs.onlinelibrary.wiley.com/doi/10.1111/dom.15039)

108,223 Type 1 diabetes patients from the DPV registry in Germany and Austria.
126 clinical variables explored simultaneously.

The goal of this project, in collaboration with Pr. Holl from Ulm University, was to identify new risk factors of Diabetic Ketoacidosis (DKA) in T1D.
Published consensus said:

- high HbA1c,
- young age,
- low BMI.

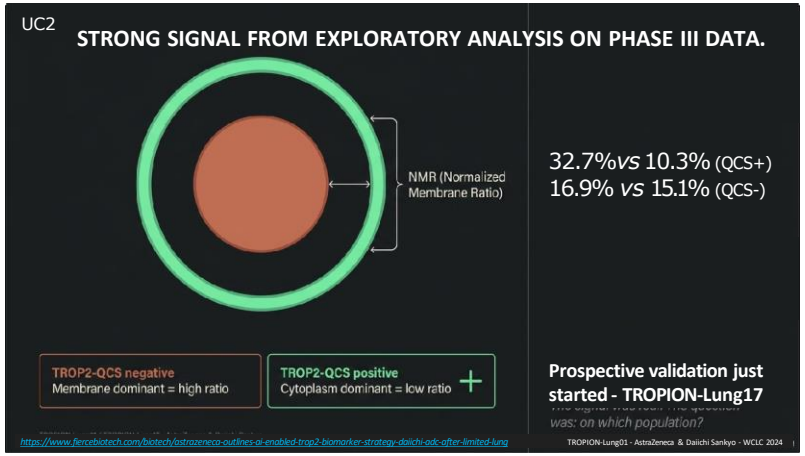
That was the risk map.

The algorithm looked at the same 108,223 patients and said: «you are missing something ».

- Absence of continuous glucose monitoring for patients under 15 years of disease - odds ratio of 8. Not 1.5. Not 2. Eight.
- Nephrotic renal disease: odds ratio of 3.
- Autoimmune thyroiditis: 1.77.

Combined, these profiles scaled DKA risk from 1.6% (no profile) to 62% (all profiles).
That is not a refinement of the existing risk model. That is a different map of the same territory.
Validated across independent cohorts, adjusted for multiple testing using Benjamini-Hochberg, reviewed by a clinical expert committee.

The algorithm explored the space.
Statisticians defined what counted as evidence.



Spoken commentary

Second case. (https://www.fiercebiotech.com/biotech/astrazeneca-outlines-ai-enabled-trop2-biomarker-strategy-daiichi-adc-after-limited-lung)

AstraZeneca and Daiichi Sankyo, TROP2 expression as a biomarker in lung cancer.

Conventional IHC scoring produced an equivocal signal.

A computational pathology approach - cell-by-cell quantitative continuous scoring - told a completely different story.
In TROP2-QCS positive patients: 32.7% response versus 10.3% in the control arm. In QCS-negative patients: 16.9% versus 15.1%. Essentially flat. The same drug. Radically different outcomes depending solely on how you measured the biomarker.

This is an exploratory analysis on Phase III data - prospective validation is now underway in the TROPION-Lung17 trial.

Strong signal. Appropriate epistemic humility.
That is what responsible AI-statistics collaboration looks like in practice.

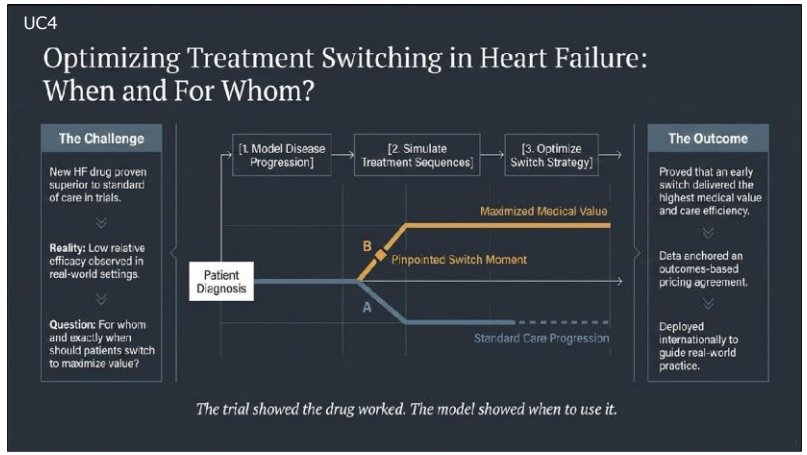
And for those who missed it: the key to this biomarker was not how much TROP2 was expressed, but where in the cell it was located. That level of spatial resolution is not accessible to conventional pathology. It required a computational lens.

Spoken commentary

Third case - and perhaps the most instructive for this room (https://www.thelancet.com/journals/lanonc/article/PIIS1470-2045(16)30661-1/abstract)

Padeliporfin for low-risk prostate cancer met two co-primary endpoints in Phase 3. That is success by every conventional standard. And yet it was not sufficient. Regulators required five-year safety projections against standard of care - data the trial was not designed to provide.
The solution: a simulation engine combining RCT efficacy data with real-world patient journeys, projecting long-term adverse event profiles.
Simulations successfully demonstrated the safety profile.
The drug reached market authorization.

Phase 3 proved efficacy. Real-world simulation proved safety over time.
Both were required. One without the other was not enough.



Spoken commentary

Last case. (Anonymized for confidentiality reasons)

A new heart failure drug proven superior in trials. In real-world settings, relative efficacy was lower - expected, and not disqualifying.

The question that mattered was: for whom and exactly when should patients switch?

A modeling approach combining real-world patient journeys with trial outcomes showed that early switching delivered a 30 to 50% reduction in first hospitalization and death over 10 years - and a 2 to 4 year gain in lifespan for patients five years post-diagnosis.

That evidence anchored an outcomes-based pricing agreement in th US.

The trial showed the drug worked.
The model showed when to use it.

Spoken commentary

All four cases share one thing : they were conducted under explicit statistical governance.

Several emerging practices deserve both your attention and scrutiny. For instance :

- Synthetic data without «quantity of information metrics »,
- Black-box models on primary endpoints,
- Unbounded extrapolation without external validation,

These examples are not just theoretical.
They are in active development, in your companies, right now.
The question is not whether they will be used.
The question is whether statisticians will define the conditions under which they are acceptable - or whether someone else will.

Extended note

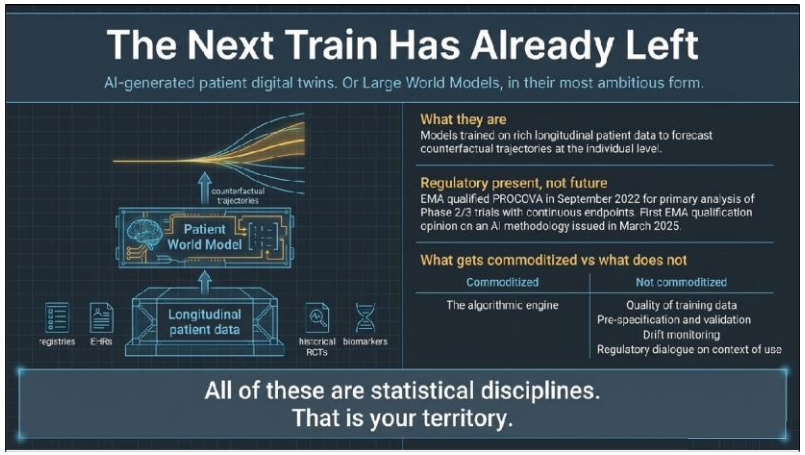
The roundtable discussions surfaced a tension that deserves to be named explicitly.
Statisticians today are expected to play two roles that pull in opposite directions.

On one side, they remain the guardians of statistical orthodoxy.
Their credibility, both internally and toward regulators, depends on the consistency of that role.

On the other side, the current pace of methodological change requires precisely the opposite posture: openness, initiative, willingness to question established practice, and ability to engage with methods that do not yet have a closed regulatory framework.

This is not a contradiction to resolve. It is a productive tension to inhabit.

The statisticians who will shape the next decade are the ones who can hold both postures simultaneously: rigorous when defending the integrity of pivotal inference, ingenuous when exploring what the next generation of methods might look like. The unique value of this community is that it is one of the very few in the pharmaceutical industry that has the methodological depth to do both.



Spoken commentary

Before I close, one more train - and this one has already left the station, more quietly than the LLM hype but with much greater regulatory traction.

Call them what you like: AI-generated digital twins, patient-level disease progression models, or - in their most ambitious form - large world models (LWM).

They share one architecture: they are trained on rich longitudinal patient data to forecast counterfactual trajectories at the individual level.

Here is my prediction for the next five to ten years : the algorithmic engine - the model itself - will be commoditized. Open-sourced, available, undifferentiated. Exactly what happened to deep learning architectures.

What will not be commoditized :

- The quality of the longitudinal data required to train these models on a specific therapeutic area.
- The pre-specification and validation of the model before unblinding.
- The robustness checks against distribution drift.
- The regulatory dialogue about context of use under the FDA-EMA January 2026 principles.

All of these are statistical topics. Not AI topics.
That is your territory.
And it is about to become more valuable, not less.

Extended note :

A point that emerged with particular clarity in the post-session discussions: among all the functions currently driving innovation in evidence generation - data science, computational pathology, AI engineering, real-world evidence teams - statisticians are the only ones who natively speak the language of regulators.
This is not a small detail. It is a structural advantage that no amount of technical sophistication on the other side can replicate quickly.

For a long time, the phrase "innovation in evidence generation" has carried an implicit tension, almost an oxymoron.
Evidence is supposed to be conservative, established, scrutinized.
Innovation suggests speed, novelty, disruption.
The bridge between the two is precisely what statisticians are uniquely positioned to build.
If they do not build it, the bridge will be built by others, and it will be weaker.

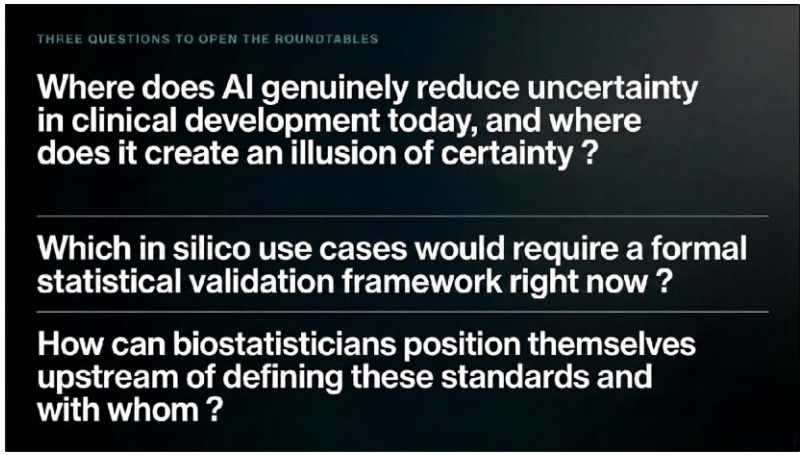
Spoken commentary

Which brings me to the only question that ultimately matters for this room :

In five years, statisticians in pharmaceutical companies will be in one of two positions :

- Downstream validators: receiving AI outputs, checking them, signing off. That role exists. It is increasingly commoditized, and it is not where this community should be heading.
- Or upstream architects: co-designing the evidence strategy, determining which AI methods are scientifically defensible, shaping what counts as evidence before a single data point is collected.

The upstream architect role is not waiting for you.
It will not be offered. It has to be claimed - by people who understand evidence well enough to govern the tools that are redefining it.
You are those people.
The only open question is whether you act like it.



Spoken commentary

Three questions to introduce your roundtable discussions :

- Where does AI genuinely reduce uncertainty in clinical development today - and where does it only create the illusion of certainty?
- Which in silico use cases require a formal statistical validation framework right now?
- And how can biostatisticians position themselves upstream of defining those standards - and with whom?

These are not rhetorical.
They are the questions your field needs to answer.
And you are the right people to answer them.

Closing reflections from the day : Several threads from the roundtables and the informal conversations deserve to be carried forward by participants in their own organizations :

On true listening as a methodological discipline.
The reminder, contributed during one of the roundtables, that the most underrated form of listening is listening to understand rather than listening to respond.
In a field where defense of methodological positions can become reflexive, the discipline of pausing to fully integrate what the other party is actually saying - rather than preparing the counter-argument - may be one of the most valuable habits a senior statistician can model for their team.

On the role of naive questions.
Expertise has a hidden cost: it tends to suppress the questions that look unsophisticated but in fact carry the most signal.
In creative and exploratory work (design thinking), the divergence phase is where these questions belong.
Protecting that phase, both for oneself and for one's team, is a methodological practice in its own right.

On the philosophical dimension of AI.
Generative AI in particular holds up a mirror to its users.
It reveals as much about the questioner as it produces in answer.
This is not a peripheral observation. It is a working principle that affects how teams adopt these tools, how they communicate their outputs, and how they distinguish genuine insight from sophisticated repetition.

This document may be shared freely within EFSPI and partner organizations.
For follow-up discussions, please contact Alexandre Templier directly.
(alextemplier@me.com)

SESSION 2 – Breakout Groups

Group 1 – Room Westminster

- Alessandro Previtali (S)
- Emmanual Zuber
- Guillaume Desachy
- Lilla Di Scala (F)
- Pantelis Vlachos
- Randi Gron
- Susan Lovick
- Del Jones (online)
- Armin Schueler
- David Wright
- Kate Peacock (online)
- Jianchang Lin (online)

Group 2 – room Louvre

- Anna Wilsten
- Estelle Lambert (S)
- Jennifer Visser-Rogers
- Lucy DeCosta
- Paula Edeards-Holmes
- Richardus Vonk
- Torsten Westermeier (F)
- Thilo Welz
- Tobias Mielke
- Mickael Guedj

Group 3 – Room Hyde Park

- Dominik Heinzmann (S)
- Florence Casset-Semanaz
- Kim Hacquoil
- Per Sorensen
- Simon Wilcock
- Baldur Magnusson
- Josie Wolfram (F)
- Veronique Robert
- Nadeesha Lihinikadu Arachchige

Group 4 – Room Big Ben

- Anna Karina Trap Huusom
- Egbert Biesheuvel
- Giacomo Mordenti
- Julia Igel
- Lara Wolfson
- Nigel Howitt
- Rene Kubiak (F)
- Steffen Ballerstedt
- Amanda Darekar (S)
- Lukas Schroeter
- Mouna Akacha

F Facilitator **S Scribe**

Session 2 Feedback

AI and Automation in Statistics: Leading into the Future

Leads: Mike, Nigel, Baldur, Arne

Group 1

Statistics and AI

- Given the **advancement of AI technology**, what do you think the world of pharmaceutical statistics (or, more broadly, drug development) will look like 1 year from now? 3 years? 5 years?
  - To help sharpen the lens: Reflect of **one decision your team made in the last year** that AI will likely handle (or significantly inform) within five years. How does that shape your view of your role as a leader?
- What are the **key opportunities** you see for our discipline in this regard?
  - What is one thing about how we currently **develop biostatisticians** that you think **most needs to change**, and what would you replace it with?
  - What can EFSPI do to enable statistical leaders to be successful?

Key messages/discussion points

- Decisions vs tasks
  - Decisions → decisions that are not part of to ensure we are in relevant discussions
  - Tasks → AI agents (competitive advantage?)
- Long term
  - New platform with regulators? Flat dossier architecture
  - Regulators review/QC implementation
- Self perception - get away from the concept of process owners and get into the concept of driving the strategic thinking
  - What can give up to AI and what cannot and keep in the statistical framework
- Data democratisation and role of statistician in it
- Example/use case: questions (eg prompt) mastering and the key input that stats can do with it
  - Quality of the questions (evidence, goal, purpose, estimand, etc…)
  - Opportunity to provide creative questions vs defensive questions
  - Opportunity to ask new/divergent questions
- Be onboard or be excluded
  - Invest in development and advertise ours better – time and resources
  - Training experience will be different from the past (eg imperfect case-study – real life experience)
  - Critical thinking – learn to ask the right questions
- Build/develop a aelf-learning AI ecosystem
  - Role of Generative vs static (interaction) : Generative → Static in an AI environment

Recommendations for EFSPI Council

- **Training academy: forefront of training to develop critical thinking for all gen**
  - Case-study / hands-on / action labs
  - Case study for governance discussions
  - Listen to understand not to answer

Recommendations for EFSPI Stats Leaders

- Collective voice from statisticians (otherwise somebody else will do that)
  - High-level document from regulators is already available
  - Control the environment
  - Position paper
- Ensure democratization of AI for all (eg CRO, Sponsor, Regulator, etc…)

Group 2

Automation and AI:

- Which are potential areas of **statistical responsibilities** that would be **benefit from either automation or AI** ?
  - Which tasks **have recently been automated** in your work area (or are currently worked on), at which cost (time, license fees), and how much do/will they save?

  - HTA submission: standardized outputs (i.e german HTA submission)
  - SLR automation
  - Statistical reports (outside the CSR) -> pilot before CSR automation
  - SAP, TFL shells, ADAM spec (testing phase) -> QC validated by human in the loop
  - Stat spent a lot of time by drafting SAP, ADAM spec -> automation part could save time
  - Dashboards generation
  - Use automation will help statisticians to focus more on complex and strategical statistical questions (designs, estimands…)
  - What it costs today (almost free) will maybe not be the same in the future

Automation and AI:

- What are **reservations and key risks for using AI**?
  - What would be reasons not to engage with AI ?
  - Ideas to **overcome** these reservations?

  - Risks: Clinicians start to use IA to replace statisticians for designing/optimising the trials
  - Generating nice SAP with IA should not prevent statisticans to understand and being able to explain all the rules
  - Delegating draft version is not maybe the right way to use automation but more for having the nice and final version
  - Preserve data integrity
  - Generating code that we do not understand
  - First code draft could be generating by IA but the QC code should still be done by human
  - Risk with automation is to do always in the same way and miss innovation
  - Trainings stat with prompts generation – being very explicit/very precise
  - Questions to tracability

Automation and AI:

- Which **functions** in addition to statisticians are **involved in automation projects** (e.g. Non-Clinical IT Programmers, project managers, (senior) management etc.?
  - How do you experience the collaboration, how can you get most out of it?

  - Computational system validation
  - Teaching people about what is useful with IA
  - Sharing case studies, best practices on how to use IA tools
  - DM and programmers (automation)
  - Being facilitators between all the functions (PV, clinicians…)

Recommendations for EFSPI Council

- External engagement with others council, highlighted the importance of validation

Recommendations for EFSPI Stats Leaders

- Training statisticians on the right way to use IA tools
- Trainings on prompts generation
- Making sure that we have QC process
- Highlight the importance of sanity check
- Statistical competencies are still relevant

Group 3

Statistics and AI

- Given the **advancement of AI technology**, what do you think the world of pharmaceutical statistics (or, more broadly, drug development) will look like 1 year from now? 3 years? 5 years?
  - To help sharpen the lens: Reflect of **one decision your team made in the last year** that AI will likely handle (or significantly inform) within five years. How does that shape your view of your role as a leader?
- What are the **key opportunities** you see for our discipline in this regard?
  - What is one thing about how we currently **develop biostatisticians** that you think **most needs to change**, and what would you replace it with?
  - What can EFSPI do to enable statistical leaders to be successful?

Key messages/discussion points

[Stats in the loop]

- Entire data & analysis pipeline is supported by tools (automation)
- AI relies on data – our roles in defining **company data strategy**
- A lot of non-stats (incl clinicians) can do analyses – how to set up (scientific rigor) governance?
  - We build tools Rshiny…), but risk they are used without stats in the loop
  - Accountability changes (??)

[Stats evolution]

- What are we doing with the (AI) free-updated time
- Overall: Stay ahead of curve as what is needed today is not what we need tomorrow
- Being more strategic & quantitative drug development / bring our valuable **E2E**
  - E.g **involved more in iEP** / beyond CDP
  - Bring a holistic "data science" perspective to the table
  - More proactive
- Enhanced influential skills (also "real-time" / empowered in cross-functional discussions)
  - More proactive
- **(New) Multi-modal data** including (digital) sensors, health tech tools
- *Inspiration?: Leveraging "spirit/learning" from stats in research?*

Key messages/discussion points

[Stats “basics”]

- How we train stats of the future (and what profile we recruit) to be able to assess AI output/insight
- How we “reasonable check” / oversight model

[Scaling stats learning = Productization]

- Digital twins, in silico trials

Recommendations for EFSPI Council & Stats leaders

- Consider different segments of stats (leaders, …)
- Drug development **significantly** changes overall, not only for stats
  - Stats Leaders can get involved in the broader discussions
- **Must win:** E2E / iEP involvement – "broader horizon" (technical, drug development but also leadership/influential) skills needs to be developed/enhanced
- Foster creativity / risk taking (if people will even challenge you more using AI "reality checks")
- **We are not alone – Sense of stats community**
  - **Share (stats) experimentation across companies / regulators**

Recommendations for EFSPI Stats Leaders

- See previous slide

Group 4

Automation and AI:

- Which are potential areas of **statistical responsibilities** that would be **benefit from either automation or AI** ?
  - Which tasks **have recently been automated** in your work area (or are currently worked on), at which cost (time, license fees), and how much do/will they save?
- What are **reservations and key risks for using AI**?
  - What would be reasons not to engage with AI ?
  - Ideas to **overcome** these reservations?
- Which **functions** in addition to statisticians are **involved in automation projects** (e.g. Non-Clinical IT Programmers, project managers, (senior) management etc.?
  - How do you experience the collaboration, how can you get most out of it?

Key messages/discussion points

- Reducing hand offs between stats and prog
  - Cutting out manual tasks between individuals
  - Saves time, reduces frustration, increases job satisfaction?
  - Automation itself didn't work well – could have used AI more to help with this
  - Going forward – where will we need to use programming? Unless its for new standards
  - Standardisation of all aspects from the start, to ensure downstream tasks can be automated
- Use automation to do standard tasks, and also for knowledge transfers, distilling information from documents, to free up time to think outside the box
- Useful for systematic data reviews, data extraction, data validation – but need to think statistically about guard rails.
  - Need to ensure enough meta-data to ensure LLMs are providing accurate outputs and basic rules are followed. Need to 'micro-manage' AI.
- Fewer people, or different people? Or the same people doing a different role? Still need to have the basic knowledge to ensure accuracy of work
  - Need to think beyond basic automation – this is the low hanging fruit, but there is more we should be able to develop and influence
- Challenges in asking staff to do non-routine work
  - How do we encourage more innovative thinking?

Reservations/Key risks

- Must work with Programming leads to also understand their challenges
- QC…different agents for production and QC
  - Utilize statistical quality control to ‘check’ QC is being done correctly?
  - Can we/should we use a ‘Critical statistician’ agent?
- Or are there more opportunities than reservations when we start using AI and how quickly it develops?
- How do we ensure data/results are not mis-used or mis-interpreted
  - Real time data flow/analysis?
  - Standardisation of what data/results/tables are available externally?
- Is there enough discussion/knowledge of the reliability, relevance and validity of data going into models?
- Do non-statisticians realise how biased LLMs/generative AI can be?
  - Need to tell AI to ensure output is valid (statistically and otherwise)
- Public opinion of fully automated drug development

Recommendations for EFSPI Council

- Outline the added-value and responsible use of AI
  - Work with other organizations – e.g. ASA/PhUSE/CDISC for coordinated messaging, maybe a White Paper
  - EFSPI should focus on the more statistical aspects of AI use as opposed to data standards
  - EFSPI Stats Methods Leaders will also be covering this
  - Time critical to ensure we are leading this conversation
- Need to work together to enforce better quality and reliability of data

Recommendations for EFSPI Stats Leaders

- Give statisticians the opportunity to learn - and fail!
- What direction should we give to upskill statisticians – of all levels of experience
  - Recommend and make material available to all
  - Or tailor training for statisticians to motivate curiosity and to cater for different ways of learning
  - Technical training vs cultivating the right mindset for embracing AI
  - Share learnings, lead by example
- Be present in the eco-system!

SESSION 3

Engagement with Regulatory Agencies and Methodological Innovation

Leads: Richardus, David

Session 3: Engagement with Regulatory Agencies and Methodology Innovation

David Wright and Richardus Vonk

Abstract

- How proactive dialogue with regulators can support the adoption of innovative methods.
- Opportunities and challenges in implementing novel statistical and biometrical approaches in clinical trials.
- Best practices for aligning methodological rigor with regulatory requirements.
- How evolving regulatory frameworks shape the future of evidence generation.

Speakers

- Regulatory – Bejamin Hofner (PEI), Armin Schuler (BfArM)
- EFSPI Stats methods leaders – Mouna Akacha (Novartis), Tobias Mielke (J&J)
- Panel discussion – all (including online – Elina Asikanius (FIMEA))

Common issues for EFSPI Stats Leaders, EFSPI Stats Methods Leaders and Statistical regulators

Actions for EFSPI Stats Leaders

Messages to EFSPI Stats Methods Leaders

Messages to Regulators

Daiichi-Sankyo

SESSION 3 – Panel Discussion

Engagement with Regulatory Agencies and Methodological Innovation

Benjamin Hofner, PEI

Armin Schueler, BfArM

Elina Asikanius, FIMEA

EFSPI Methods Leaders:

Mouna Akacha

Tobias Mielke

SESSION 3

Slides – Benjamin Hofner, PEI

Engagement with Regulatory Agencies and Methodological Innovation – **The HOW**

Benjamin Hofner
Head of Data Science & Methods

Bundesinstitut für Impfstoffe und biomedizinische Arzneimittel
Federal Institute for Vaccines and Biomedicines

Das Paul-Ehrlich-Institut ist ein Bundesinstitut im Geschäftsbereich des Bundesministeriums für Gesundheit.

The Paul-Ehrlich-Institut is an Agency of the German Federal Ministry of Health.

Paul-Ehrlich-Institut

Disclaimer

These slides are partially based on work of others and contain **input from the SIA LT** (most notably Elina Asikanius and Frank Pétavy) for which I am very grateful.

However, the **views are my own** and **do not** necessarily represent the views of

- any of my colleagues,
- the Paul-Ehrlich-Institut (PEI),
- the European Medicines Agency (EMA), or
- any other European regulatory agency (NCA).

We are here to **listen and learn**.
We cannot promise any solutions.

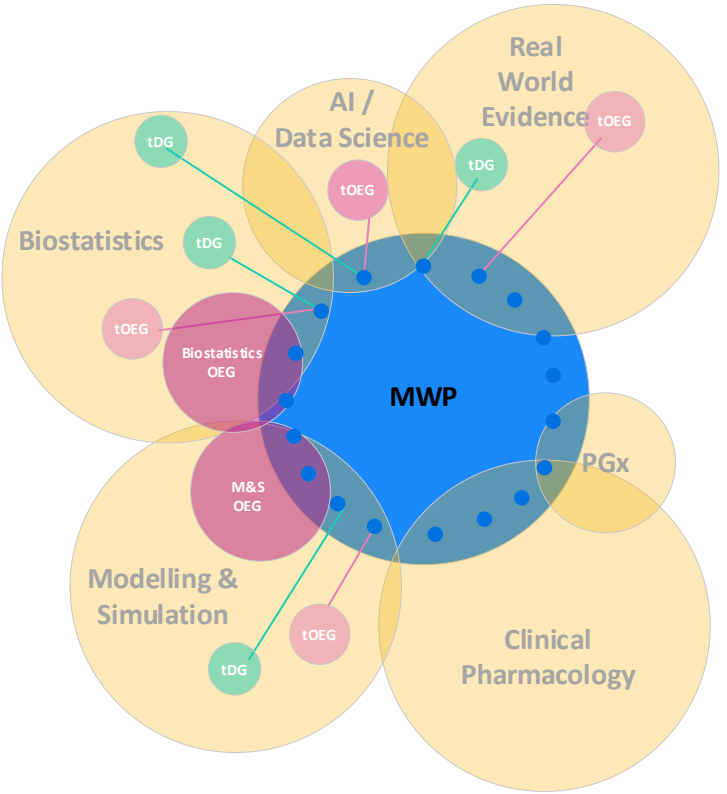
HOW WE WORK

STRUCTURE AND GOVERNANCE OF METHODOLOGY DOMAIN

Structure of Methodology Domain

- Methodology Working Party (**MWP;** Source)
  - Currently consists of 24 members endorsed by CHMP
  - Primary role/mandate:
    - Governance of domain
    - Alignment across disciplines
    - Implementation and oversight of guideline drafting groups
    - Strategic role on methodologies
    - …
- **MWP Chairs**:
  - Kit Roes
  - Kristin Karlsson

} Primary point of contact for overarching topics (concerning whole domain)

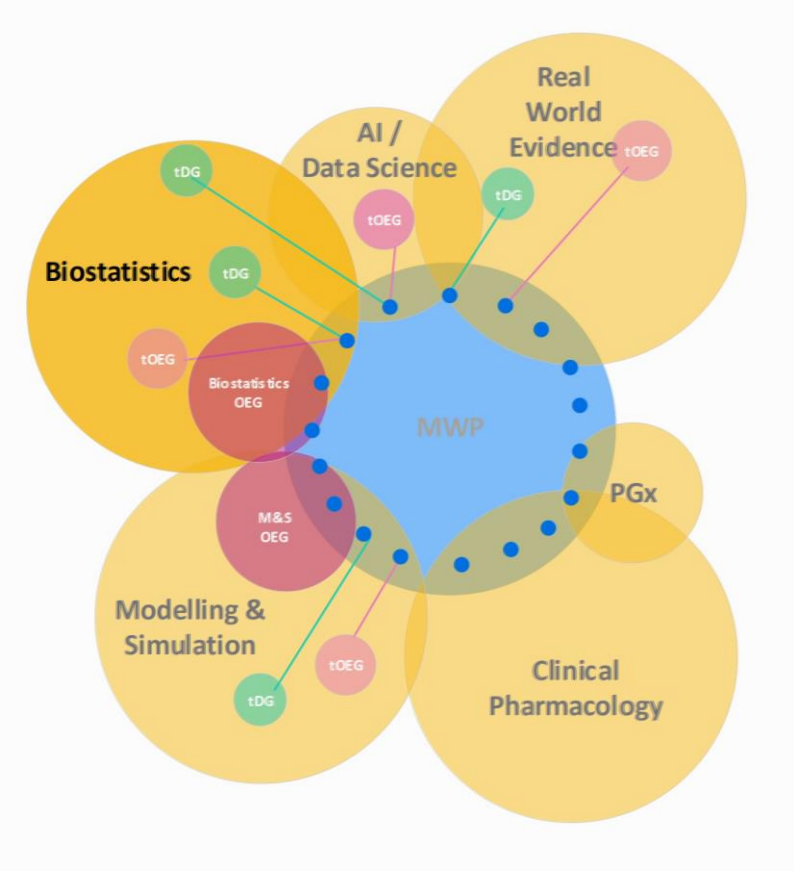


Source: List of MWP members

Structure of Methodology Domain

- Methodology European Specialised Expert Community (**Methodology ESEC**; Source) supports MWP (> 250 experts)
  - Divided into 6 (overlapping) domains, so called Specialized Interest Areas (**SIAs**); Composition primarily based on interest (rather than expertise)
  - This is where the actual work is done (in drafting groups etc.)
- **Biostatistics SIA** consists of > 100 members from 20 countries + EMA
  - Not all are statisticians! Not all are actively engaged in network activities!
  - But many are engaged in activities (and numbers are increasing).
  - Drafting Groups are often multi-disciplinary.
  - Each SIA has a dedicated Leadership Team (**SIA LT**).
- Biostats SIA LT:
  - Andreas Brandt (BfArM)
  - Benjamin Hofner (PEI)
  - Elina Asikanius (FIMEA)
  - Florian Klinglmüller (AGES)
  - Frank Pétavy (EMA)
  - Lukas Aguirre Davila (PEI)

Primary point of contact for biostatistics



Source: List of ESEC member

FRAMEWORK FOR INTERACTION

Principles for stakeholder interactions

Fundamental principles

- transparency
- independence and integrity
- accountability
- appropriate interaction
- broad representation
- effective communication
- continuous improvement

Specific goals

- Avoiding Conflicts of Interest (COI)
- Maintain balance among stakeholders / companies

Source: European Medicines Agency (EMA) stakeholder relations

Stakeholder interaction framework

Objectives for stakeholder interaction:

- provide a platform to **exchange views** and promote dialogue with stakeholders on topics concerning medicines for human and veterinary use;
- improve communication and provide **efficient, targeted and timely information** in a proactive manner;
- enhance stakeholders' **understanding of the EU medicines regulatory framework** and the role of the regulators and enrich the EMA's understanding of issues that are pertinent from the industry perspective for product development and licensing;
- build on existing interactions between industry (including SMEs), academia and other stakeholders in the overall science, medicines, and healthcare arenas by **co-operating with established networks** and alliances;
- increase **transparency** of stakeholders engaging with the EMA and report on the interaction.

Source: https://www.ema.europa.eu/en/partners-networks/pharmaceutical-industry#framework-for-interaction-12362

- **Levels of interaction:**
  - **Inform** (to enable feedback e.g. news items, Q&As, information days)
  - **Consult** (via written consultation e.g. guidelines development, public consultations on deliverables, focus groups)
  - **Consult & Involve** (based on direct interactions e.g. focus groups)
  - **Co-operate** (jointly engaging towards a common technical goal e.g. technical expert groups)
- Interactions are possible for listed stakeholders (> List of eligible industry stakeholder organisations)
  - ✓ EFSPI (and PHUSE) are listed as stakeholders

Mandate of WPs

- WP mandate further governs interactions with interested parties and specifically **allows interactions**
  - with industry stakeholder organisations (preferably >1 at a time)
  - via public-private partnerships

7.7. Contacts with Interested Parties

Where relevant and as agreed at the domain level, lists of stakeholders for consultation, also referred to as “interested parties”, will be established in line with each domain/WP workplan.

WPs may meet with interested parties to discuss general matters or specific scientific topics as foreseen in their workplan, and/or emerging topics with the agreement of the CHMP/CVMP/domain.

WPs may establish links with:

- Patient and consumer organisations;
- Learned societies and healthcare professional organisations;
- Academia, such that it acts as a hub for information flow from key European academic stakeholders into WPs, in those areas falling under the remit of the WP;
- **Industry stakeholder organisations; such an interaction should primarily be carried out at EU industry (trade) organisation level, and only exceptionally at company level;**
- **Public-private partnerships and consortia;**
- Other stakeholders which have an interest in or are influenced by the work of the specific WP.

The WP should not reach any formal decisions in the presence of interested parties.

Source: Mandate, objectives, and rules of procedure for working parties under the quality, non-clinical, methodology, clinical and veterinary domains

EXAMPLES OF INTERACTIONS

Interactions within regulatory framework

- Standard regulatory procedures
  - ITF Meetings
  - Scientific Advice
  - Qualification advice/opinion
  - Assessments

✓ Carefully read advice, assessment reports (and guidelines)
✓ Share experience within and across companies

- ICH guideline drafting groups
- Public consultation on EMA guidelines & corresponding workshops
- Note that we also have regulatory exchange (between regions):
  - Cluster meetings (with PMDA / FDA)
  - Possibility to speak about procedures & access FDA advices
  - Trying to align / exchange were possible

Research-based interactions

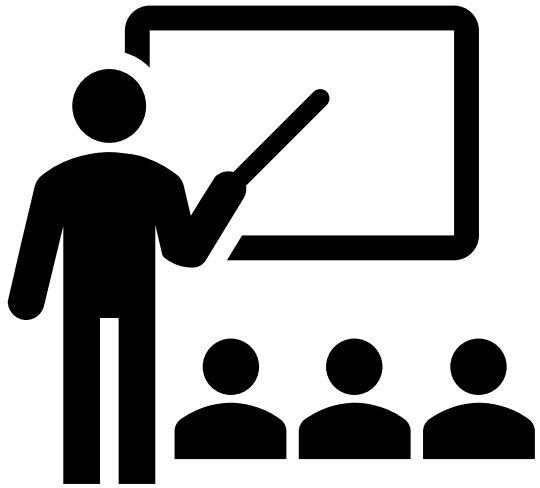
- Common research projects (e.g. via IMI/IHI)
  - Recent example were I was involved in:
    IMI project EU-PEARL on platform trials
  - EMA is currently compiling a list of relevant topics (research priorities)
- „Ad hoc" research collaborations
  - See e.g. terminology paper (DOI: 10.1186/s13063-025-08942-3)
- Public workshops / trainings
  - E.g. by EFSPI but also other organisations/hosts
  - "Private" training by industry for EMA/EMRN not possible/sensible

Other interactions

- EMA stakeholder meetings
  - E.g. Methodology Working Party (MWP) interested parties meeting
- EMA workshops
  - E.g. on external controls
- Exchange on specific use cases much more fruitful
  - These need to be public (at least shared with other companies)
- Direct interaction with other (leadership) teams
  - EFSPI Statistics Leaders
  - EFSPI Statistical Methodology Leaders

Outlook and points for discussion

- Venues for exchange exist and are used already.
- Going beyond these remains challenging.



Questions to industry

- What do you not know or need to understand better?
- What would you expect from us in terms of interaction?
- Why do you want to expand / change the current interactions?

"Our tasks"

- How can you internally spread the word and make sure that your teams are aware of interactions that took place and corresponding learnings?
- What can you do to improve interactions?

"Your tasks"

SESSION 3

Slides – Armin Schueler, BfArM

Federal Institute
for Drugs
and Medical Devices

Engagement with Regulatory Agencies and Methodological Innovation – A personal view

2026-05-12

Armin Schüler, BfArM

Disclaimer

Views expressed in this presentation are the author's personal views and not necessarily the views of BfArM.

Agenda

1. Introduction
2. Opportunities and Challenges
3. Best Practices
4. Reflection

1. Introduction

Introduction

The aim of the session is to discuss

- How proactive dialogue with regulators can support the adoption of innovative methods.
- Opportunities and challenges in implementing novel statistical and biometrical approaches in clinical trials.
- Best practices for aligning methodological rigor with regulatory requirements.
- How evolving regulatory frameworks shape the future of evidence generation.

Introduction

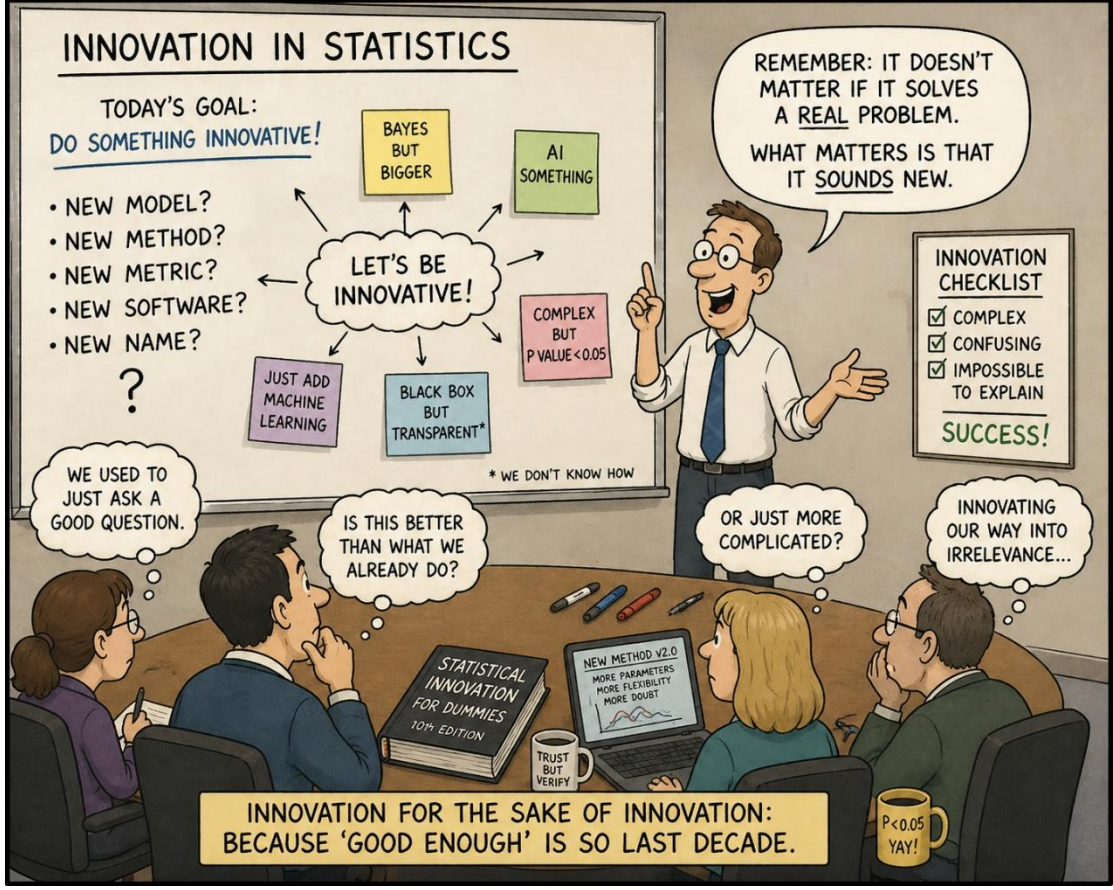
Focus of this presentation

- How proactive dialogue with regulators can support the adoption of innovative methods.
- Opportunities and challenges in implementing novel statistical and biometrical approaches in clinical trials.
- Best practices for aligning methodological rigor with regulatory requirements.
- How evolving regulatory frameworks shape the future of evidence generation.

Excursus – what is innovation?

Innovation := creating new value by

- developing new methodology?
- transforming / adapting / improving well known methodology?
- changing current practice?
- just doing something different?



Source: ChatGPT

Different types of innovation might need different ways of interaction.

2. Opportunities and Challenges

in implementing novel statistical and biometrical approaches in clinical trials

Opportunities and Challenges

+ Regulators are in general open for innovation and eager to **learn** and understand
– The challenge is finding the time to make oneself familiar with new methodology

+ Regulators are in general open for innovation and eager to learn and **understand**
– The challenge is sometimes to understand the rationale for the innovation proposed. Especially if not clearly and transparently communicated why there is a deviation from the standard approach or the EMA guidelines.

Opportunities and Challenges

+ Innovation enables to discover terra incognita which may leads to new discoveries
– Is the innovation still helping to answer the clinical question of interest?
  E.g. I have seen justifications via better power or better effect size, but the proposed methods changed the associated Estimand => answer a different clinical question

+ Innovation enables to discover terra incognita which maybe leads to new discoveries
– Going new ways bears the risk of losing consistency with previous decisions

3. Best Practices

for aligning methodological rigor with regulatory requirements

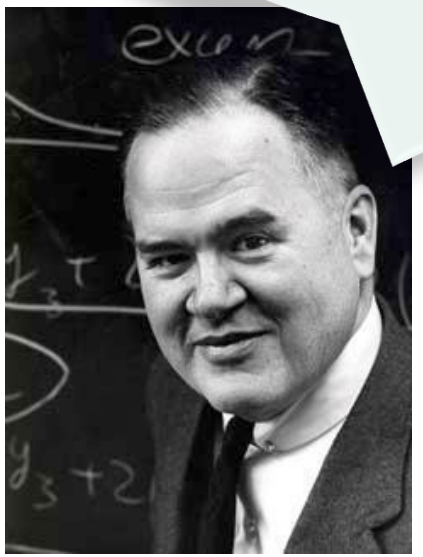
Best practice

- **Follow the Estimands Thinking**

John Tukey (1962)[1]

Far better an *approximate* answer to the *right* question, which is often vague,

than an *exact* answer to the *wrong* question, which can always be made precise.



[1]The future of data analysis. Annals of Mathematical Statistics 33 (1), (1962), p.13

Federal Institute for Drugs and Medical Devices

Best practice

- An assessment of a MAA is a benefit – risk evaluation

- Any innovation should not lead to a less qualified benefit-risk evaluation.
  E.g. a methodology which has advantages in controlling the FWER should be accompanied by an associated estimation of the treatment effect

Best practice

- Choose the right pathway for innovation. As more complex an innovation is, as more it’s essential to till the field
  - Introduction via congresses and publications
  - Contribution to guideline development
  - Via EMA qualification
  - Interactive (national advice) vs non-interactive advice formats (SAWP advise)

- Be transparent why you innovate. If you deviate from a guidance provide the **real** reason. It happens to often that I think to myself…. Have they ever read EMA guidance xxxx?

Back-up

Abstract

Session Brief: Engagement with Regulatory Agencies and Methodological Innovation

This session, co-organized by Richardus Vonk and David Wright, looks at how early and constructive interaction with regulators can help bring new statistical methods into practice and support stronger evidence in drug development. As clinical research evolves with advanced analytics and novel statistical frameworks, working effectively with regulatory expectations has never been more important.
Our guest speakers, Armin Schüler and Benjamin Hofner, will share perspectives from their extensive experience at the intersection of regulatory science and innovative methodology. Through case examples and strategic insights, they will discuss:
*] How proactive dialogue with regulators can support the adoption of innovative methods.
*] Opportunities and challenges in implementing novel statistical and biometrical approaches in clinical trials.
*] Best practices for aligning methodological rigor with regulatory requirements.
*] How evolving regulatory frameworks shape the future of evidence generation.

The session aims to provide participants with practical guidance, real-world lessons, and a clearer understanding of how methodological innovation can thrive within a robust regulatory environment.

SESSION 3

Slides – EFSPI Methods Leaders:

Mouna Akacha

Tobias Mielke

Engagement with Regulatory Agencies and Methodological Innovation

Mouna Akacha and Tobias Mielke
(on behalf of the EFSPI Stats Methods Leaders)

17th EFSPI Statistical Leaders Meeting 2026 – May 12th, 2026

EFSPI
EUROPEAN FEDERATION OF STATISTICIANS IN THE PHARMACEUTICAL INDUSTRY
Representing Statistical Associations in Europe

Agenda

- **Innovation in drug development: Why it matters and what is holding it back?**
- **From concept to practice: Two examples of quantitative innovation in action**
- **Strategic engagement: A critical enabler**
- **Key takeaways and discussion**

DISCLAIMER:

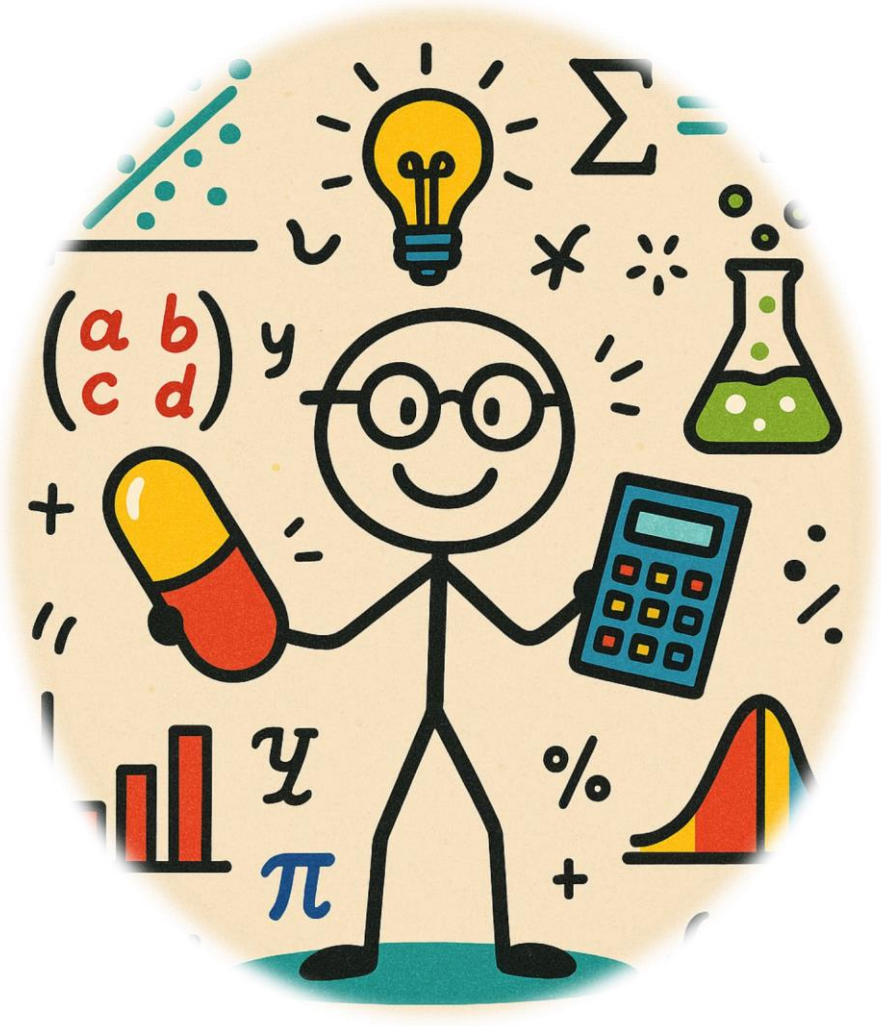
- Slides represent thoughts which are all open to discussion.

Innovation in drug development: Why it matters and what is holding it back?

Perspective of a quantitative drug developer

Our goal is to:

- Enable evidence-based medicine
- Facilitate faster, ethical and safe access to treatments
- Collaborate for meaningful outcome
- Innovate for impact

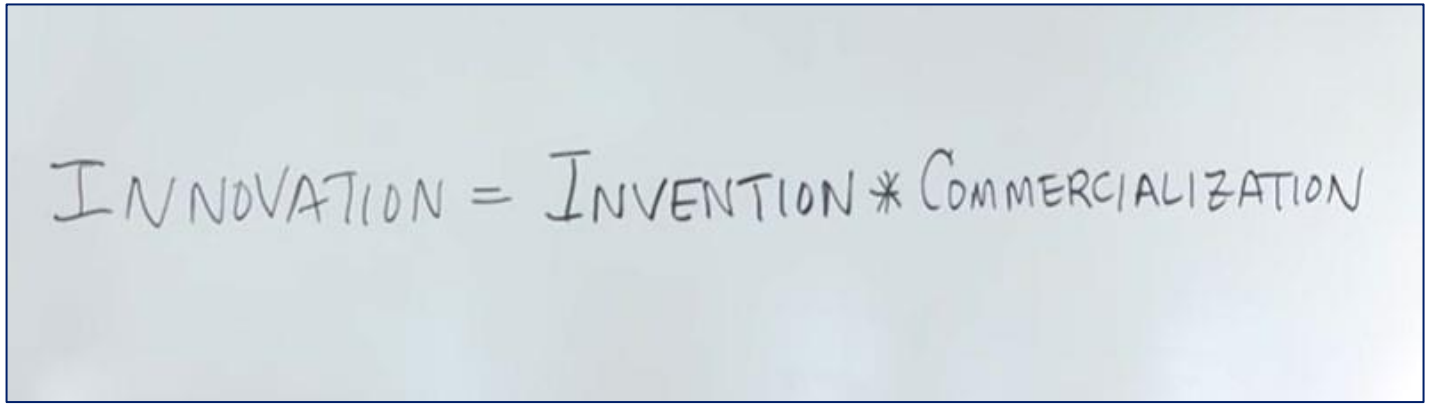


Innovation in drug development

- **World and health care are changing at very fast speed**
  - More complex questions - new modalities, precision medicine, real-time clinical trials
  - More data than ever before - and more diverse (RWD, digital, genomic)
  - More powerful tools - AI, advanced modelling, simulations

- **Quantitative scientists and leaders are essential to harnessing innovation and driving meaningful impact**

What is ‘innovation’?



*

*see https://www.youtube.com/watch?v=oD7X3KvJAVk

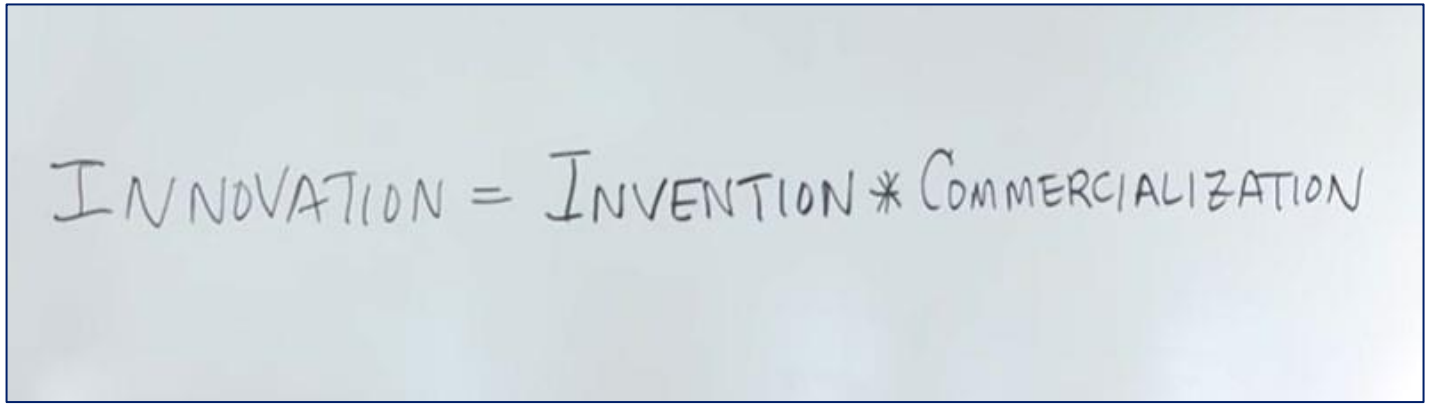
What we can innovate

- The way we think, learn and lead
- Our processes
- Our technologies
- Our **methodologies**
- ...

Categories of innovation

- Disruptive
- Incremental
- Lateral
- ...

Quantitative inventions often face adoption hurdles



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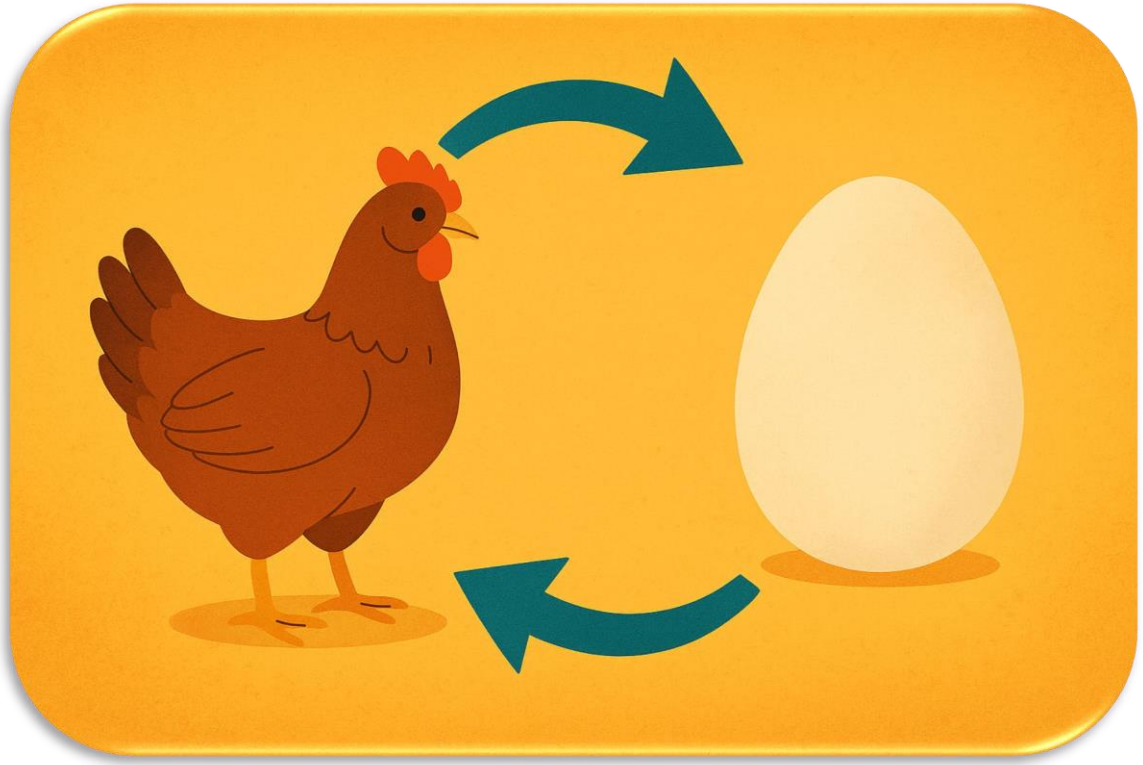
*see https://www.youtube.com/watch?v=oD7X3KvJAVk

Examples include:

- Multiple imputation
  - Developed by Rubin in 1978; applied for the first time at Novartis in 2011
- Bayesian adaptive dose-response designs
  - "Despite these advantages and recommendations, Bayesian adaptive designs have not been widely adopted in practice." (Andy Grieve, 2011 at a BBS seminar)

What makes adoption so difficult?

- Traditional thinking
- Risk aversion
- Lack of precedent
- Lack of regulatory feedback
- Missing skills
- Lack of tools and processes

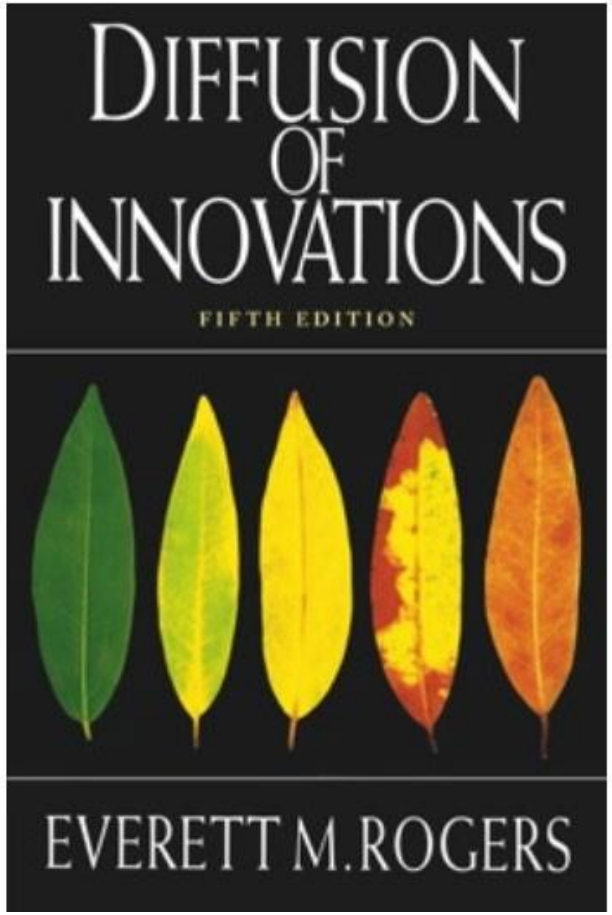


Opportunities and challenges

Methodological innovation is not enough

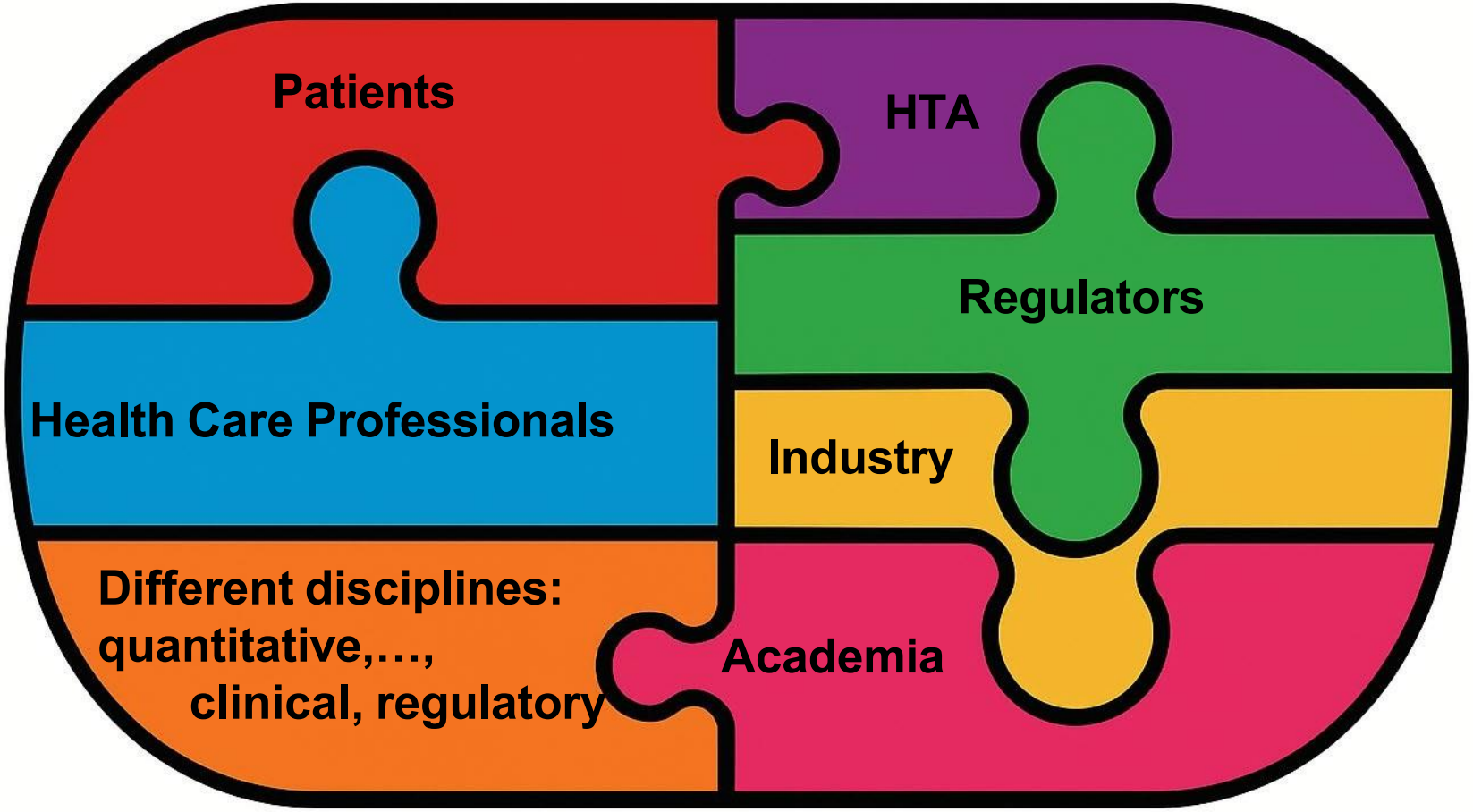
Impact, predictability, transparency and consistency

- Any proposed novel approach should bring clear utility
- There needs to be some predictability on the impact
- There needs to be transparency
- Added utility needs to offset the lack of consistency vs. previous precedence
  - *E.g.: ICH-M15: risk proportionate*

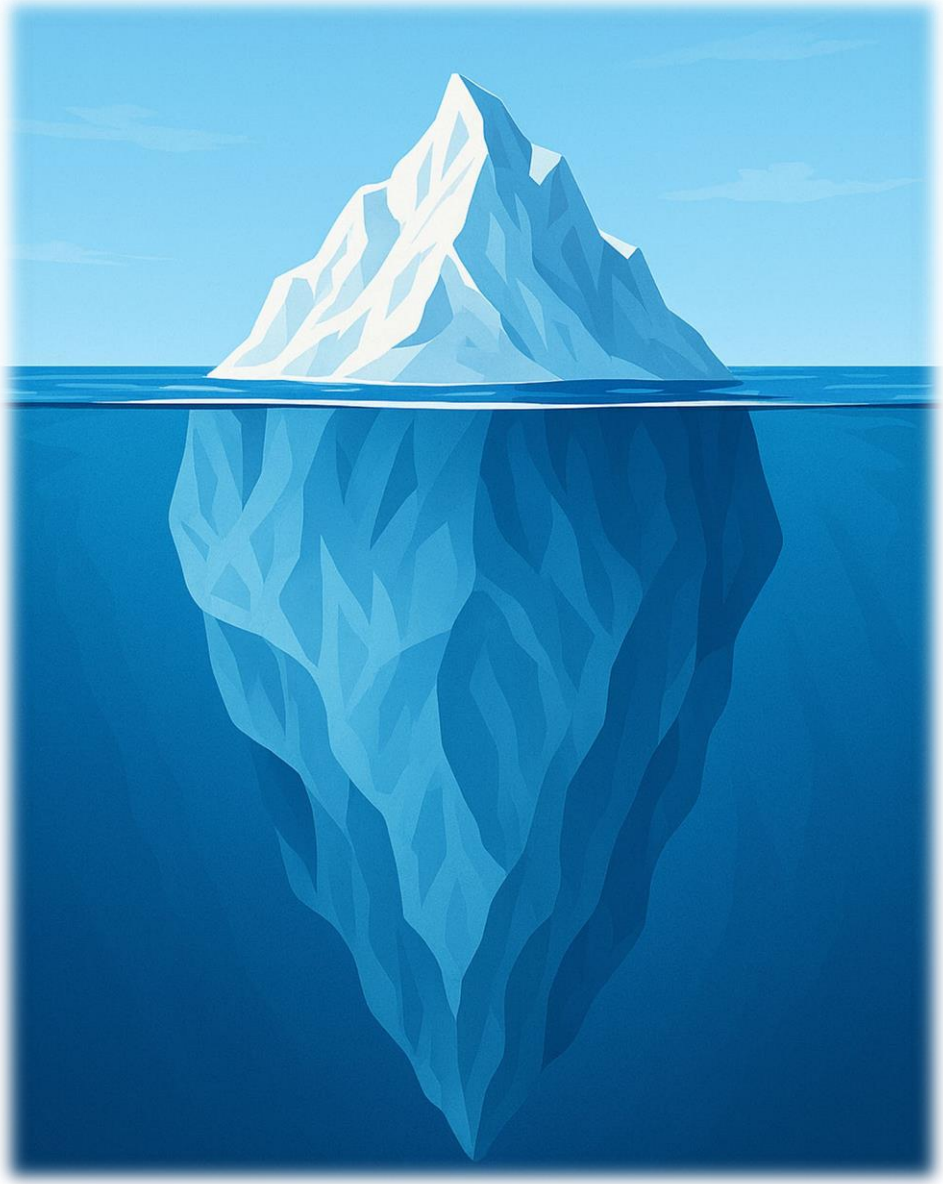


Quantitative drug developer in industry

We are just a small piece of the puzzle



What we see vs. what we don’t see



Methodological developments, publications, guidelines,…

People & Skills

Leadership

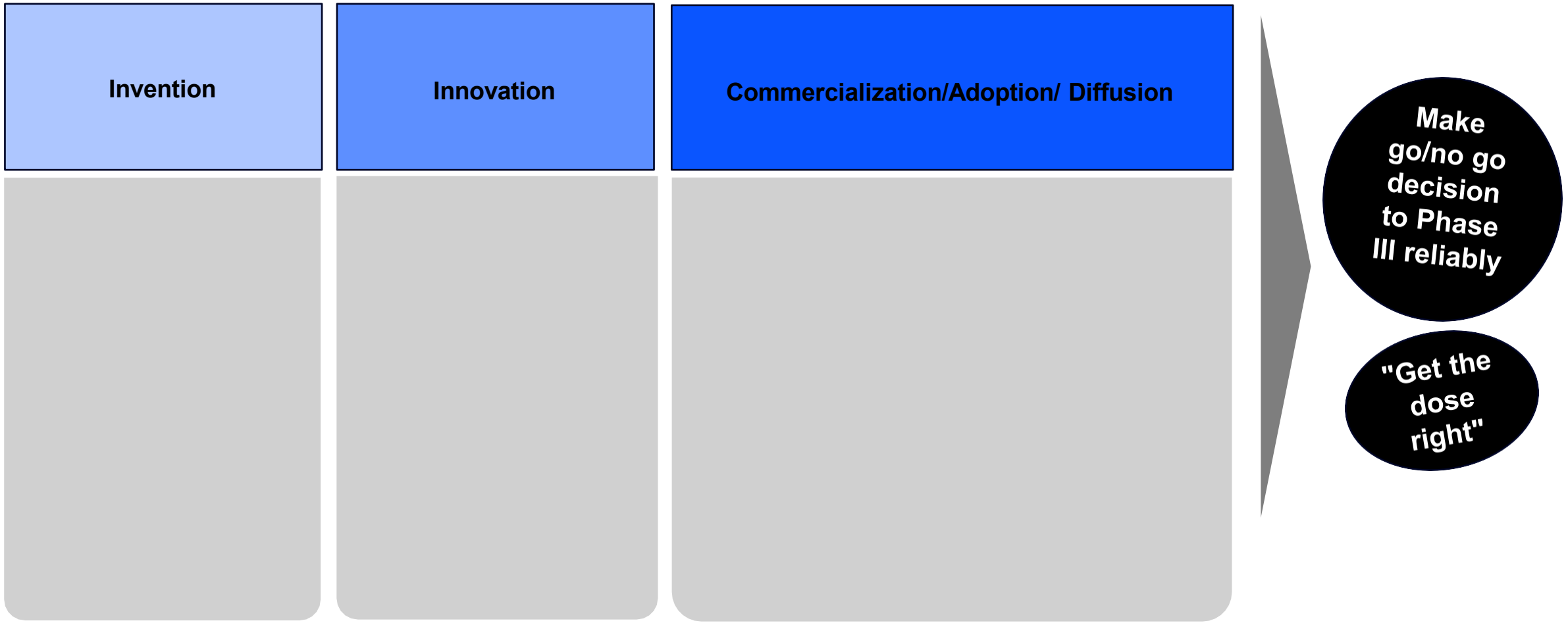
Culture

Tools & Technology

Dealing with complexity (legal, finance, data privacy,...)

From concept to practice: Two examples of quantitative innovation in action

Example: MCPMod



Example: MCPMod

Invention	Innovation	Commercialization/Adoption/ Diffusion
MCPMod developed in 2003 for design and analysis of Phase II dose-finding studies	**MCPMod applied as supp. analysis in 2004 and as primary analysis in 2005/2006**	**MCPMod routinely considered and implemented across Novartis and by other companies**
Key enablers ✓ Collaboration with academic consultants ✓ In-house skills and resources ✓ Mandated by Biostatistics Management (Dose Finding Initiative started in 2002)	**Key enablers** ✓ Collaboration with academic and industry partners ✓ Engagement with project teams and review boards to identify adequate pilots ✓ Various cross-functional activities on how to improve dose-finding (2004 "Delphi" / 2007 "Get the dose right")	**Key enablers** ✓ Dissemination through publications, internal/external presentations and trainings ✓ PhRMA working group on adaptive dose-finding studies + white paper in 2007 ✓ Continued advocacy and collaboration with internal/external stakeholders ✓ Tools (S Plus in 2005, SAS in 2007, R in 2010) ✓ EMA qualification in 2014 + Fit for purpose designation by FDA in 2016

Make go/no go decision to Phase III reliably

"Get the dose right"

Example: Platform Trials

Invention	Innovation	Commercialization/Adoption/ Diffusion
Master protocols enabling evaluation of multiple interventions within a shared, ongoing trial infrastructure Early academic and public-sector initiatives demonstrating feasibility of **multi-arm, adaptive, perpetual trials** **Key enablers** • Advances in adaptive and Bayesian trial methodology • Academic leadership and publicly funded trial networks • Recognition that classical two-arm trials are inefficient for complex diseases	Successful implementation in large, disease-defining platform trials, including ▪ STAMPEDE ▪ I-SPY 2 **Key enablers** ▪ Central governance and master-protocol infrastructure ▪ Close interaction with regulators during design and execution	Platform trials effectively implemented at scale and in rapid response to Covid pandemic: ▪ Recovery ▪ Principle **Key enablers** ▪ Regulatory engagement (FDA in particular) ▪ Extensive publications with lessons learned ▪ Collaborative research (e.g. EU-PEARL)

Share resources

"Treat the patient best"

Are we there yet?

Example: Platform Trials

WAS

WHO ~~IS~~ INVOLVED?

efpia

imi innovative medicines initiative

EUROPEAN UNIVERSITY HOSPITAL ALLIANCE (EUHA) HOSPITALS

Vall d'Hebron Institut de Recerca · Vall d'Hebron · Erasmus MC · Assistance Publique Hôpitaux de Paris · UniSR Università Vita-Salute San Raffaele · Medizinische Universität Wien · King's College London · KU Leuven · Charité

OTHER HOSPITALS

LMU Klinikum

UNIVERSITIES

University of Oxford · Manchester 1824 The University of Manchester · Tartu Ülikool · Università degli Studi di Milano · Newcastle University

PATIENT ORGANISATION

EPF European Patients Forum

DATA, STATISTICS

The European Institute For Innovation Through Health Data · Berry Consultants · CUSTODIX

REGULATORY

Paul-Ehrlich-Institut · euroscan

PROJECT MANAGEMENT

teamit research

EUROPEAN RESEARCH INFRASTRUCTURES

eatris · ECRIN European Clinical Research Infrastructure Network

BIOPHARMACEUTICAL COMPANIES/EFPIA/ASSOCIATED PARTNERS

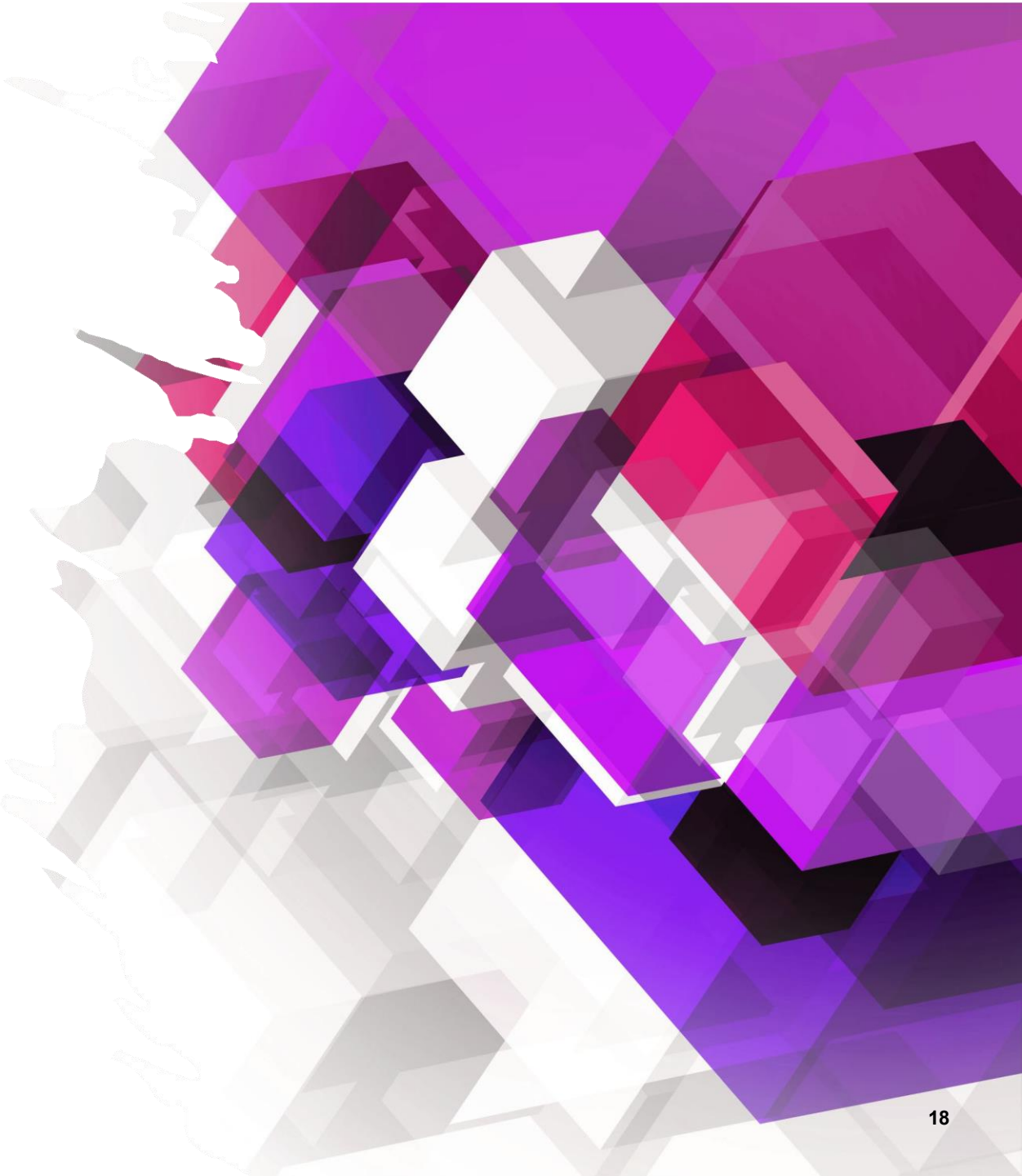
AstraZeneca · Novartis · Janssen Pharmaceutical Companies of Johnson & Johnson · Allergan · novo nordisk · Otsuka · teva · Sanofi · Pfizer · TB Alliance Global Alliance for TB Drug Development · SpringWorks Therapeutics · Children's Tumor Foundation Ending NF Through Research · Servier

EU-PEARL as an Example

Work-Package 2: Methodological Considerations for Platform Trials

- **Joint work across academia, regulatory and industry**
- **Common objective: Increase understanding & enable platform trials**

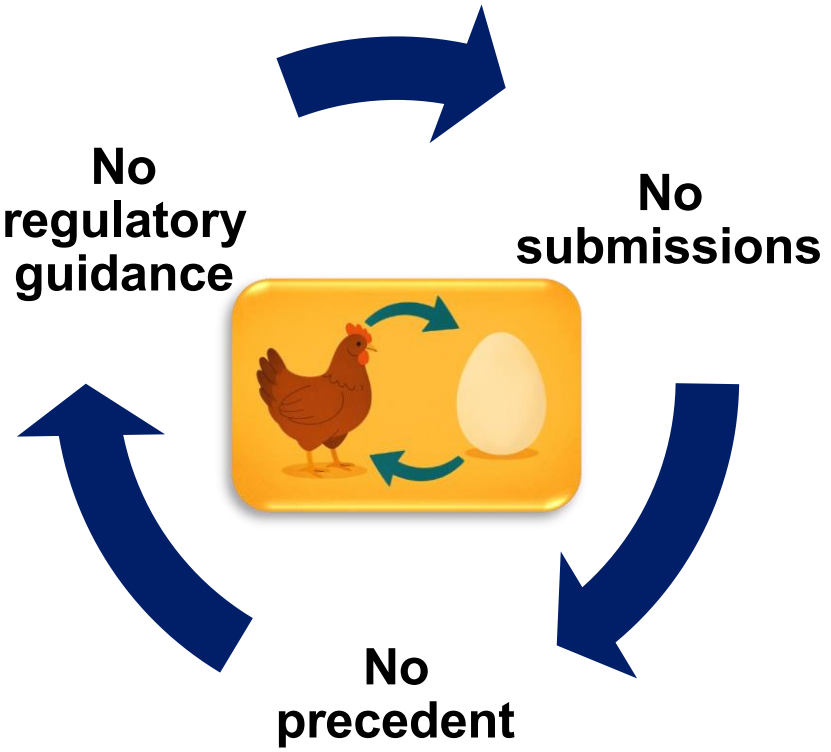
Strategic engagement: A critical enabler



Role of strategic engagement

- **Internally: Crucial to enable pilots and adoption**
  - Regulatory engagement starts with internal alignment
- **Externally: Crucial to ensure acceptability by key stakeholders**
  - Immediate opinion on innovative solutions is often not available

- **Need for a strategic approach**
  - **Dissemination:** peer-reviewed publications, presentations to receive feedback and provide independent 'validation'
  - **Collaboration:** working groups (e.g. ICH), research consortia, internships, sabbaticals by academic partners etc.
  - **Engagement:** trade associations, societies, conferences and workshops help to build relationships, gather input, and seek feedback
  - **Formal regulatory pathways:** pilots, qualification procedures, commenting on regulatory guidance documents etc.



Formal regulatory pathways

Bayesian Statistical Analysis (BSA) Demonstration Project

CDER Center for Clinical Trial Innovation (C3TI)

Complex Innovative Trial Design Meeting Program

Streamlined Trials Embedded in clinical Practice (STEP) Demonstration Project

CDER Center for Clinical Trial Innovation (C3TI)

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Qualification of novel methodologies for drug development: guidance to applicants

Selective Safety Data Collection (SSDC) Demonstration Project

CDER Center for Clinical Trial Innovation (C3TI)

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Selective Safety Data Collection (SSDC) offers an innovative approach to facilitate the conduct of large-scale efficacy and safety trials through the purposeful reduction in the collection of certain types of data for drugs or biologics with a well-characterized safety profile. C3TI aims to promote the adoption of SSDC principles into appropriate drug development programs. The SSDC demonstration project aims to partner with sponsors to

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Home > Human regulatory: overview > Research and development > Innovation Task Force briefing meetings

Innovation Task Force briefing meetings

The Innovation Task Force (ITF) briefing meetings offer developers early dialogue with the European Medicines Agency (EMA) on innovative medicines. They address regulatory, technical and scientific concerns.

Human | Veterinary | Innovation

Proposal for strategic engagement

- **Engage early, engage constantly**
  - Not a one-off scientific advice - a sustained relationship
  - Conversations outside a concrete protocol often valuable
  - No “us versus them” attitude

- **Be transparent about WHY we are innovating**
  - State the value proposition, risks and mitigation steps

- **Smart risk-taking through supplementary analyses**
  - Propose innovative methods **alongside** traditional ones
  - Build precedent and experience
  - Today's supplementary analysis is tomorrow's primary analysis

It’s not about ideas.
It’s about making ideas happen.
Scott Belsky

Leading by example: EFSPI Statistical Methodology Leaders

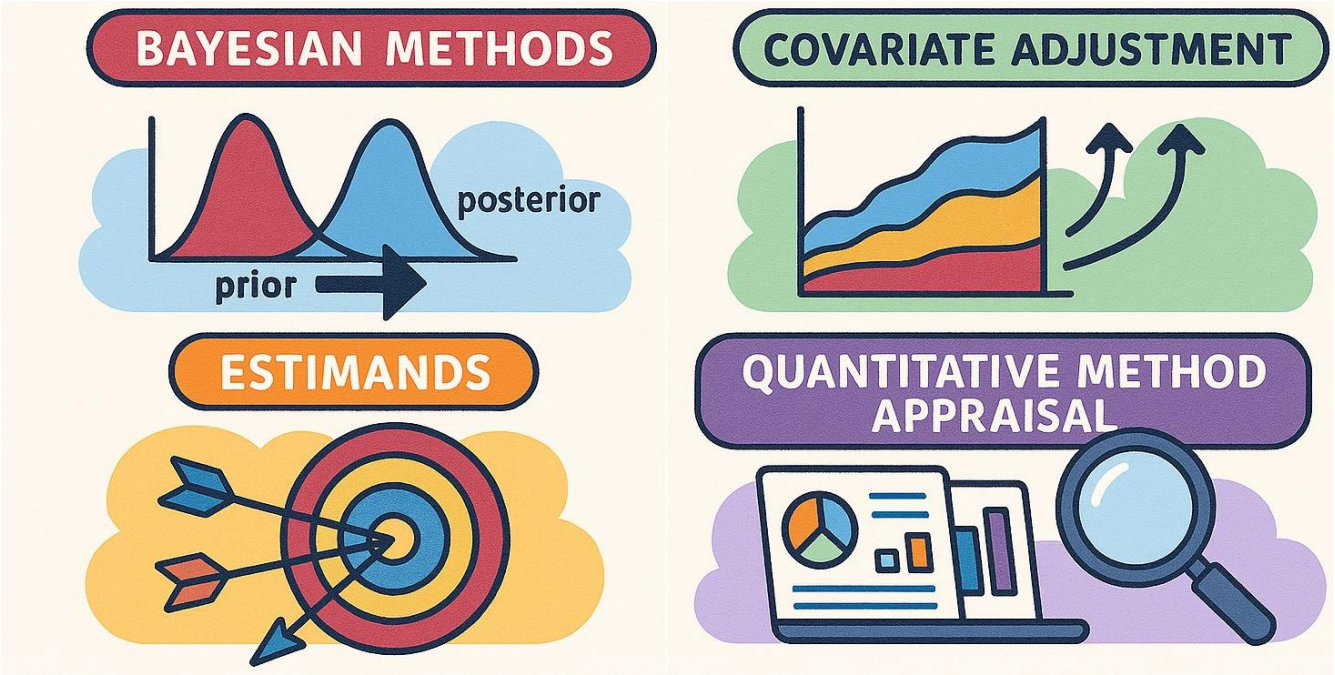
- **Formed in September 2023**
- **Aims to capitalize on opportunities for synergies**
  - Pre-competitive collaborations, e.g. on trainings and policy matters
  - Co-develop and scale innovation, e.g. through strategic engagement
  - Anticipate and shape future trends
  - Support career development of methodologists

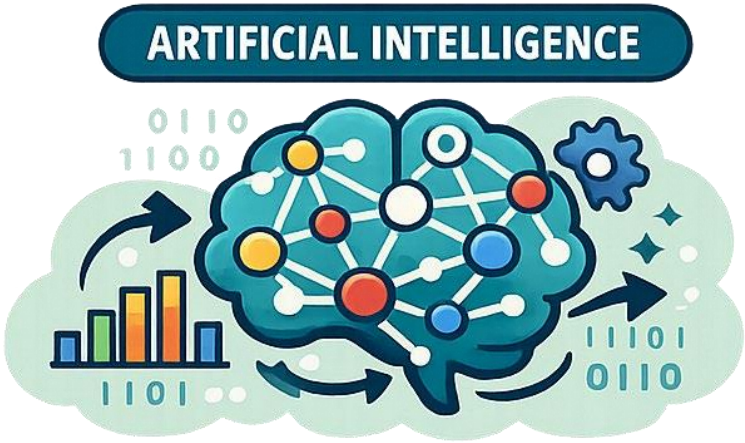
Check out our web page

Strategic priorities for 2026

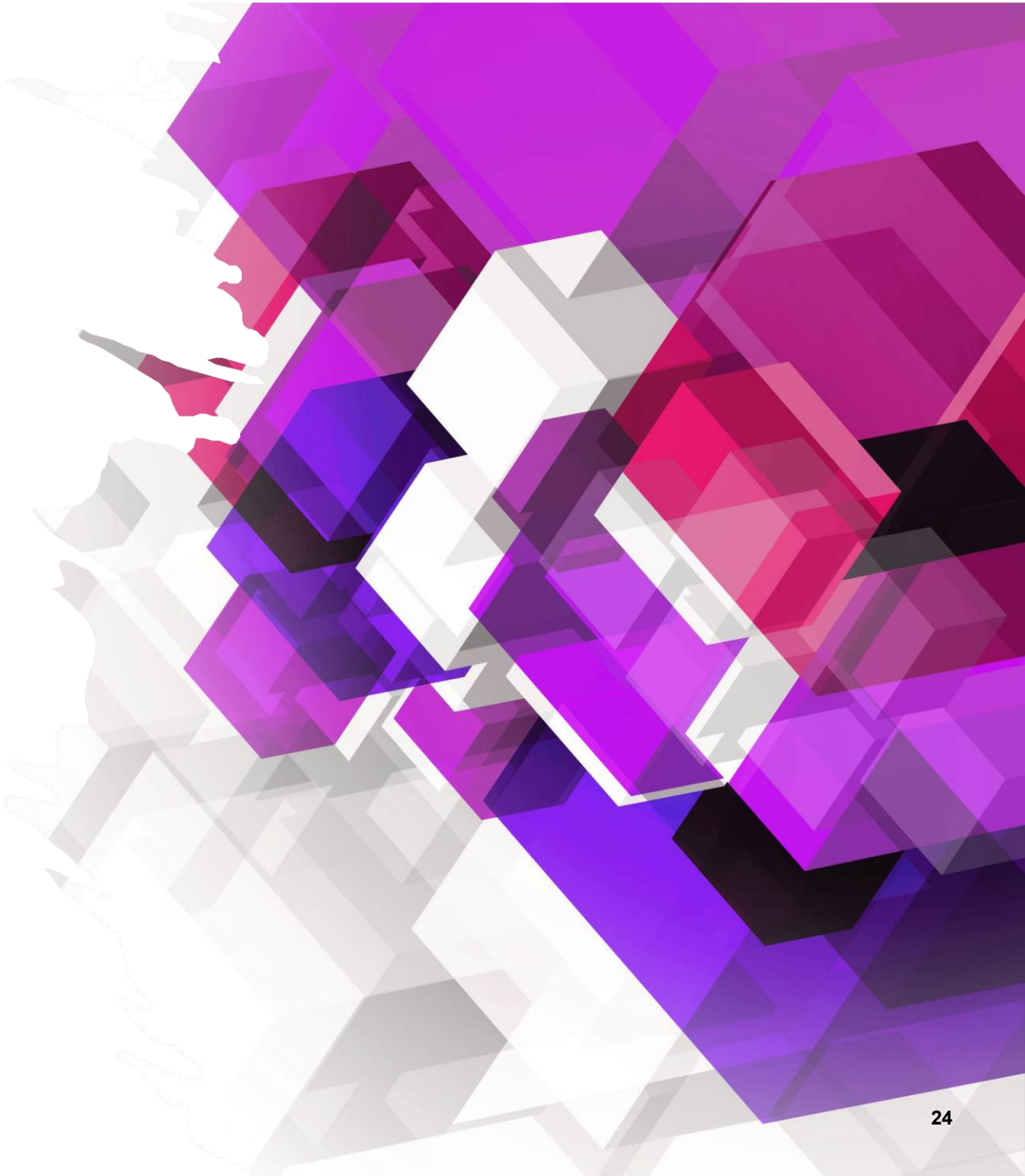
Five priorities defined through:

- Needs and opportunities in modern drug development
- Regulatory landscape and priorities





Key takeaways and discussion



From innovation to adoption: Call to action

Champion innovative submissions	**Build the precedent**: submit innovative proposals even as supplementary; engage in pilots
Engage with regulatory colleagues and other stakeholders	**Build the relationships:** early, often, transparently
Invest in capability building and tools	**Build the knowledge and infrastructure:** quant. scientists, clinicians, regulatory affairs, …
Share examples - successes and failures	**Build the adoption culture:** encourage experimentation and smart risk-taking
Support collaborations and consortia	**Build the internal and external ecosystem**

Thank you!

Question to you

Kit Roes on evidence generation: EFSPI Workshop 2025, Basel

Evidence generation

Going beyond the (prospective) individual experiment **is a fundamental step in scientific inference**.

Augmenting control groups in RCTs, digital twins, Bayesian borrowing, external controls, platform trials leveraging non-concurrent controls, model based extrapolation of adult to pediatric data,.....

Moves us essentially into meta-analysis, evidence synthesis, indirect comparisons, while reducing the amount of prospectively collected data.

Strategic priority to achieve the benefits: Progress fundamentally and coherently.

EFSPI Workshop 2019
Oekolampad Church

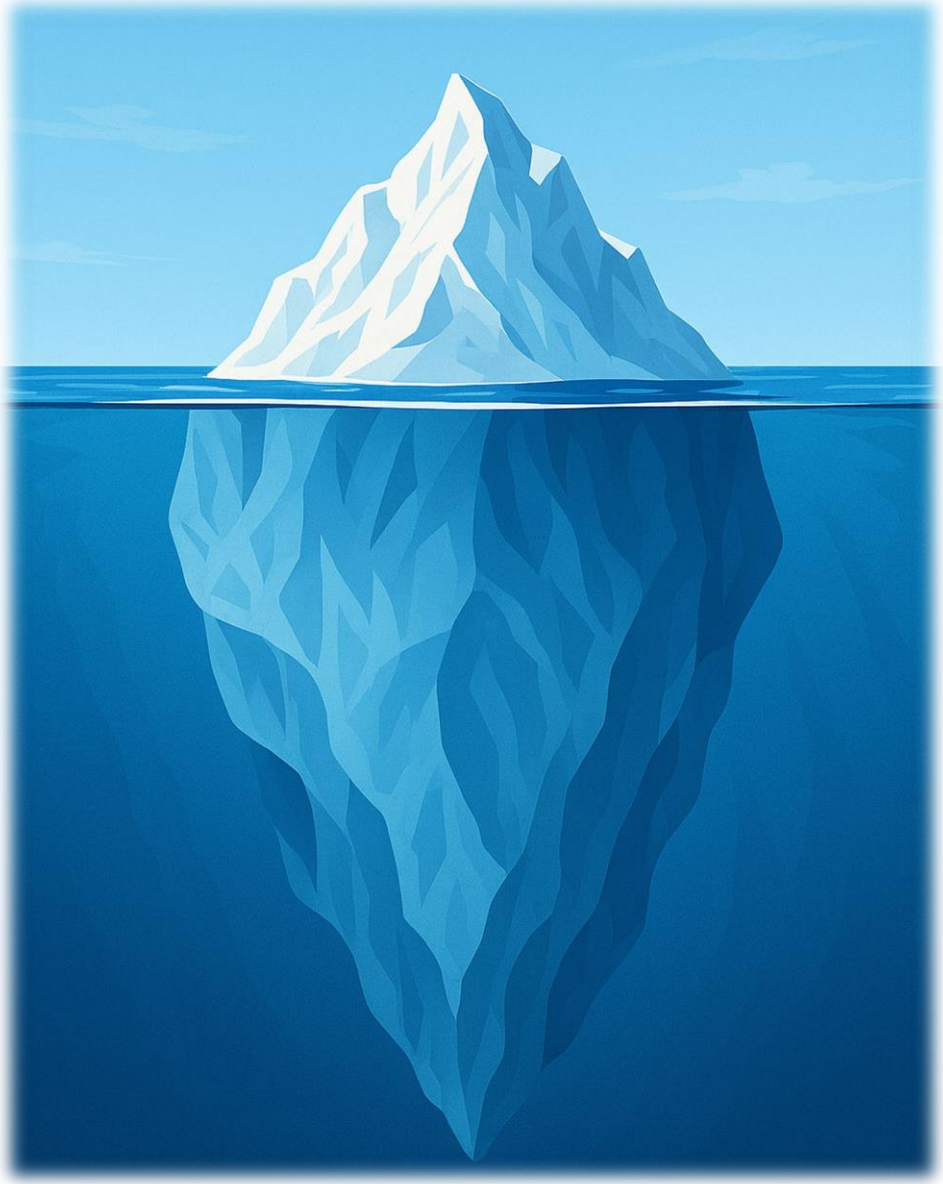
If this is our vision **to achieve** in ~10 years:

- Do we still discuss “yes/no” or do we invest more into the “how”?
- What are the steps we need to do now to get us there?

Panel Discussion - Goals

- How to align methodological rigor with regulatory requirements?
- What are the opportunities and pressing challenges in 10 years?
- What do we all need to enable those opportunities?

What we see vs. what we don't see



Transfer of Technology From Statistical Journals to the Biomedical Literature

Past Trends and Future Predictions

Douglas G. Altman, Steven N. Goodman, MD, PhD

From innovative thinking to pharmaceutical industry implementation: Some success stories

Rosalind Walley, Nigel Brayshaw

First published: 12 July 2022 | https://doi.org/10.1002/pst.2222 | Citations: 1

Phases of methodological research in biostatistics—Building the evidence base for new methods

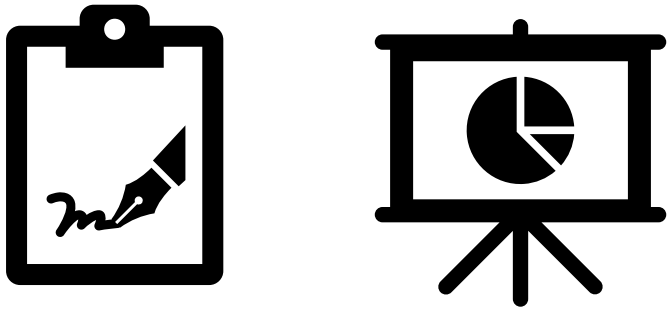
Georg Heinze[1], Anne-Laure Boulesteix[2], Michael Kammer[1,3], Tim P. Morris[4], Ian R. White[4] on behalf of the Simulation Panel of the STRATOS initiative

Implementation of Statistical Innovation in a Pharmaceutical Company

Kaspar Rufibach, Marcel Wolbers, Jenny Devenport, Godwin Yung, Chris Harbron, Alun Bedding, Zhiyue Huang, Ray Lin, Herbert Pang, Daniel Sabanés Bové & Jianmei Wang


Thank you for attending the 17th EFSPI Statistical Leaders Annual Meeting 2026.
We wish you a safe and pleasant journey home.

Post Meeting Survey results



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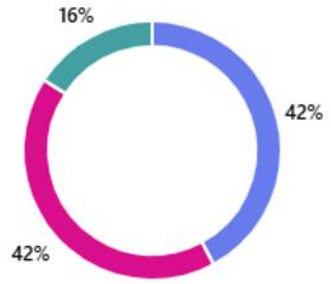
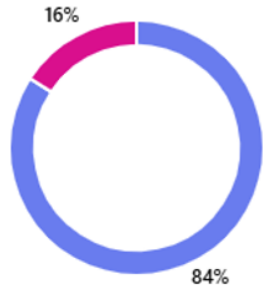
1. How relevant was each session topic to your interests and needs? SESSION 1: Global Industry Shifts and Europe's Positi on in a Multi Centered World (Leads: Giacomo, Veronique)

Very relevant	8
Somewhat relevant	8
Neutral	3
Somewhat irrelevant	0
Very irrelevant	0



2. How relevant was each session topic to your interests and needs? SESSION 2: AI and Statistics (Leads: Mike, Arne, Bald ur)

Very relevant	16
Somewhat relevant	3
Neutral	0
Somewhat irrelevant	0
Very irrelevant	0



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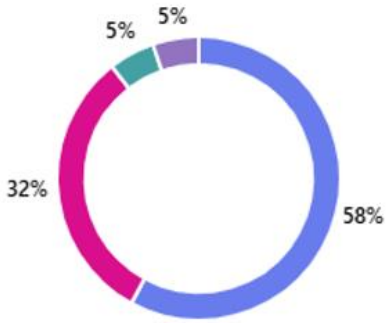
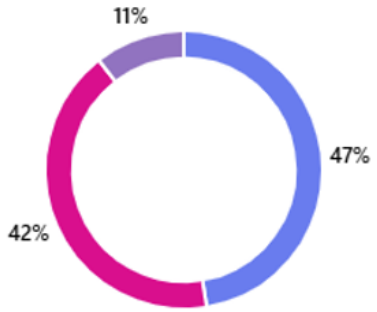
3. How relevant was each session topic to your interests and needs? SESSION 3: Engagement with Regulatory Agencies a nd Methodological Innovation (Leads: Richardus, David)

Very relevant	11
Somewhat relevant	6
Neutral	1
Somewhat irrelevant	1
Very irrelevant	0



4. Were the session topics covered in sufficient depth?

Yes, they were very comprehensive	9
Mostly, but more depth would have been helpful	8
Neutral	0
Some topics were too shallow	2
No, they lacked depth	0



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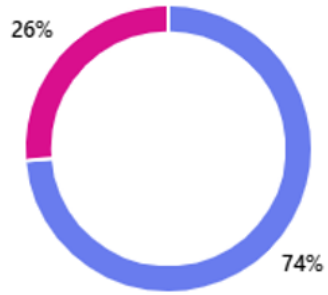
5. How would you rate the overall quality of the presentations?

Excellent	9
Good	10
Average	0
Below Average	0
Poor	0

6. How organized was the event?

Extremely organized	14
Very organized	5
Somewhat organized	0
Slightly disorganized	0
Very disorganized	0



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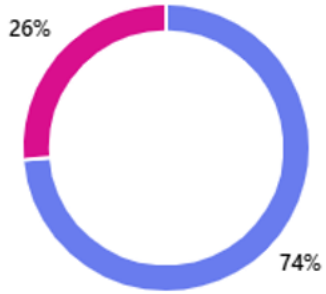
7. How would you rate the opportunities for networking during the event?

Excellent	14
Good	5
Average	0
Below average	0
Poor	0



8. Were there sufficient breaks and opportunities to interact with other attendees?

Yes	17
No	2

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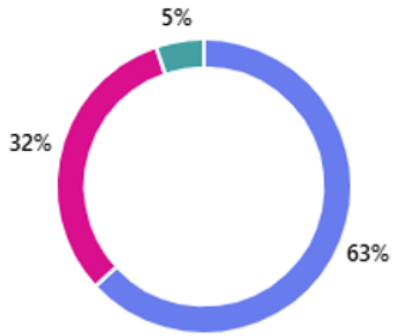
9. Overall, how satisfied are you with the meeting?

Very satisfied	17
Somewhat satisfied	2
Neither satisfied nor dissatisfied	0
Somewhat dissatisfied	0
Very dissatisfied	0

10. How satisfied are you with the following aspects of the meeting? Overall content

Very satisfied	12
Somewhat satisfied	6
Neither satisfied nor dissatisfied	1
Dissatified	0
Very dissatisfied	0



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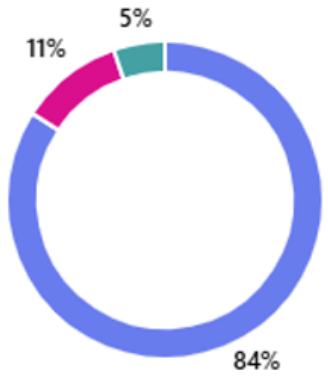
11. How satisfied are you with the following aspects of the meeting? Time management

Very satisfied	16
Somewhat satisfied	2
Neither satisfied nor dissatisfied	1
Dissatisfied	0
Very dissatisfied	0



12. Were your expectations of the event met?

Beyond my expectations	8
Met as expected	11
Below my expectations	0

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13. What did you like most about the meeting?

14 Responses

ID ↑	Name	Responses
1	anonymous	Exchange of views. Getting inspired by the discussions
2	anonymous	The breakout sessions and the restitution from each group . great discussions
3	anonymous	Overall the experience was extremely positive!
4	anonymous	Openness and willingness of attendees to network. Taking time to think about and hear from others on industry wise topics.
5	anonymous	Particularly the format of the 3rd session
6	anonymous	Variety of topics, all of relevance
7	anonymous	Networking aspect, and sharing common challenges across industry
8	anonymous	Networking
9	anonymous	The good discussions in the panel discussions and break outs
10	anonymous	It was the first time I had attended this meeting, so was a bit apprehensive, but I needn't have been! It is a very welcoming group, with plenty of opportunity to network. All of the topics covered were very relevant and I appreciated having the smaller breakout sessions to foster more discussion.
11	anonymous	the open informal atmosphere with trust.
12	anonymous	I thoroughly enjoyed it and it was the right amount of people
13	anonymous	1) Break out sessions with discussion topics. As a first time attendee this made it easier to contribute & get to know individuals. I also liked that there was a deliberate mix of backgrounds, eg Pharma, CRO, regulatory in each group 2) Evening meal - facilitated networking & getting to know people better 3) Extremely welcoming to stats leaders from all backgrounds (as someone representing a smaller CRO). Discussions set up so diversity of viewpoints was encouraged -> led to that warm & fuzzy feeling that I made a meaningful contribution
14	anonymous	The presence of external experts, in particular from regulatory agencies

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14. How could the meeting be improved?

11 Responses

ID ↑	Name	Responses
1	anonymous	Session 2 .Not convinced by the talk of Alexandre Templier to trigger discussion in break out sessions . The content of the 3rd session was not new. We had last year similar presentation of the EFSPI methodo .
2	anonymous	There could be even more interactive roundtable discussions. This is a minor comment.
3	anonymous	More diversity in panel discussion members. I think the first session didn't really cover the topic and merged into AI and methodology aspects. I don't feel it truly addressed the topic. I don't think we made the most of the next gen attendees.
4	anonymous	More time for networking (longer breaks); a restaurant (although of excellent quality) less noisy and more opportunities to move around and connect (perhaps a buffet?)
5	anonymous	Some topics in more deep, make it more tangible with concrete examples so leaders could more easily define action items for their daily work.
6	anonymous	That is a difficult one. Perhaps, to get more involvement from the major CROs.
7	anonymous	Very little to improve - the meeting was well-paced, well-organised and as mentioned before all of the topics were very relevant!
8	anonymous	more time for discussions, in particular for the break-out groups, and less presentations. Send them as pre-reads.
9	anonymous	More interactions - the breakout sessions were good but they could have been longer or had more of them. Perhaps even gather people's thoughts on the topics prior to the meeting and then during the meeting recommendations could be agreed upon.
10	anonymous	No comment on improvements. I particularly liked the late start and early finish which permitted attendance for 2 days only. Even with a disrupted travel I was able to attend the majority of day 1 and still contribute to the discussions. I only missed the ice breaker.
11	anonymous	I felt that in some circumstances the debate was focused on industry vs regulators rather than being focused on science

17th EFSPI Statistical Leaders Annual Meeting 2026

15. Do you have any suggestions for topics you would like to see covered in next year´s meeting?

9 Responses

ID ↑	Name	Responses
1	anonymous	how development is changing and continue the discussion on AI topic . Regarding the new stat genertaion , they were less present, less interaction.
2	anonymous	I think it would be interesting to have a topic of how the ways of working and team interactions etc are changing with wave of AI. In the same context the it could be discussed how the resourcing and people management changes or remains the same.
3	anonymous	Elements related to or having aspects of both CRO and Pharma, as the success of the function is reliant on both of these companies.
4	anonymous	Would like to see the plan for the EFSPI Training academy: what's up next, what is the calendar and are there topics that the Stats Leaders could directly influence? Would also like to see the SIG Calendar for the whole year ahead: what are the events planned, the Webinars and any other activity such as planned publications.
5	anonymous	Not at this stage
6	anonymous	There was a lot of discussion/mention about empowering statisticians to be better communicators/more influential and to be more rounded 'drug developers'. Maybe a session on how we can best encourage this in our teams at all levels and hear some success stories.
7	anonymous	1) impact of AI on role of statisticians and clinical drug development, 2) How can we, as EU stats leaders, remain impactful in a rapidly changing global environment? For example: are we striking the right tone to remain relevant to our management? Do we need some training and refreshers?
8	anonymous	1) Continuation of focus on how we develop future statistical leaders 2) Challenges in Europe were discussed. Can next year focus on how we reconnect with statisticians in US/Asia?
9	anonymous	AI will undoubtedly remain a central topic of interest. Another important area is academia–industry partnerships, particularly in how we build sustainable pipelines for innovation and talent. In parallel, there is growing interest in redefining the role of statistical leaders (from a traditionally supportive function to true strategic partners shaping drug development decisions). Causal thinking also appears to be making a strong return, with increasing recognition of its importance in moving from correlation-driven analyses to decision-focused evidence. Finally, it would be highly valuable to explore how clinical trials might look in the next 15–20 years. Envisioning this future could help clarify the direction we should take as a community, potentially linking back to the broader impact of AI and emerging technologies.