EFSPI Stats Leaders Meeting Boudry

How leaders are promoting innovation in drug development

Mouna Akacha, Cornelia Kunz, Kaspar Rufibach

Tuesday, 17:00 – 17:30

Who we are & Why we care about this topic

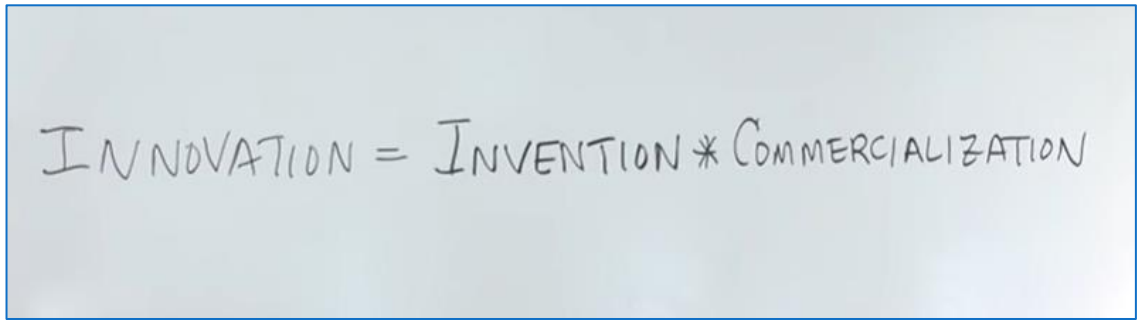
Mouna

Cornelia

Kaspar

EFSPI Statistical Methods Leaders

What is innovation, and why do we need in pharmaceutical drug development?



What we can innovate

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

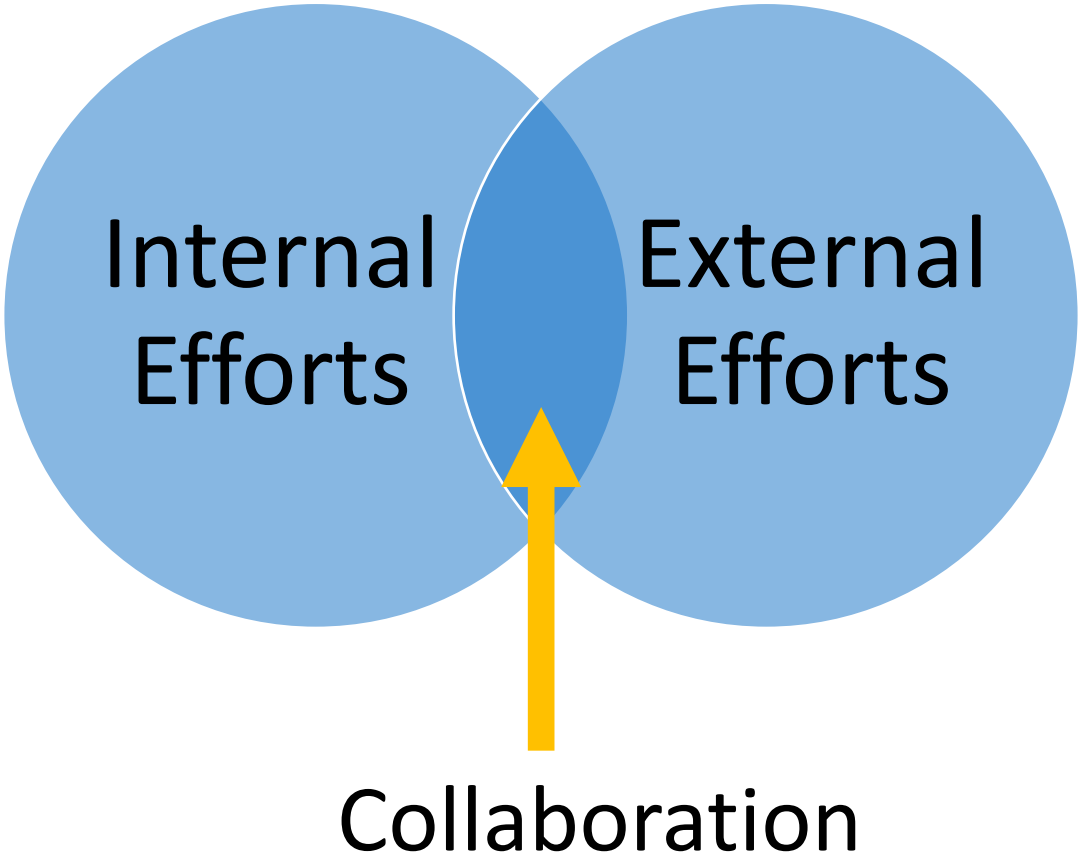
- Pharma business model more and more under pressure
- Need of innovation and adaptation to maintain profitability and relevance in a rapidly evolving world (medical + quantitative research, technology, access to data, culture,…)
- Ensure competitive advantage and success in terms of
  - Impact we generate
  - Talent that we attract
- Many opportunities to drive innovation and stay ahead of the curve exist!!!

*https://startupistanbul.com/blog/2014/11/how-to-embrace-future-changes-for-your-startup/

Embracing both internal and external sources of innovation

Develop innovative solutions in-house

- Initiatives / workstreams
- Methods groups or individuals
- …



Implement innovative solutions developed by others

- Peers
- Academics
- Start-ups
- Service providers
- …

Embracing both internal and external sources of innovation

What is the role of Statistical Leaders to promote innovation?

Collaboration

Objectives of the session

- Appreciate the interplay of invention and commercialization
- Share learnings from driving statistical innovation in industry
- Consider the role Statistical Leaders play in promoting innovation
- Acknowledgment for collaboration opportunities (or even needs) across companies
  - E.g. for coordinated and fast evaluation of «new» methods
- Map how Statistical Leaders and Methods Leaders can collaborate, within EFSPI and within / across companies

Agenda

Now

- Discuss survey results

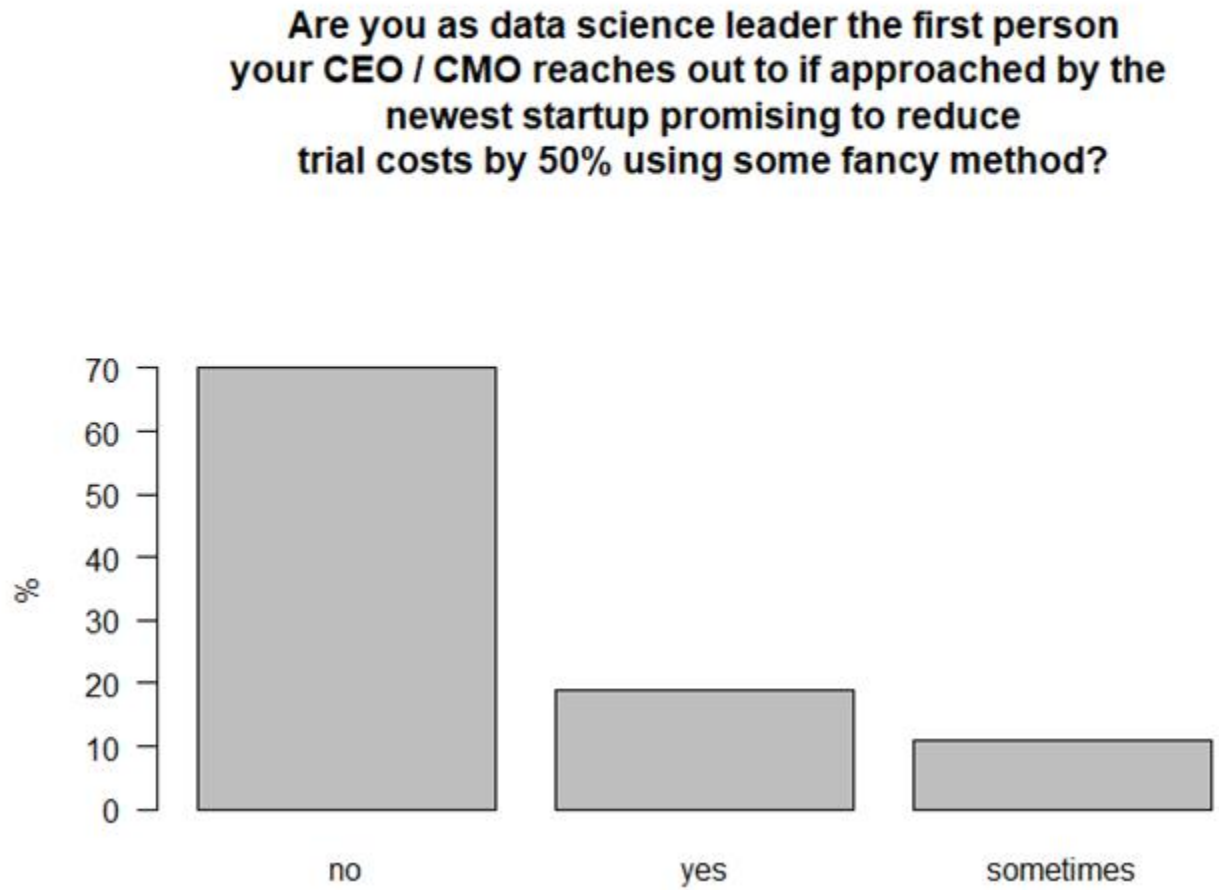
Tomorrow

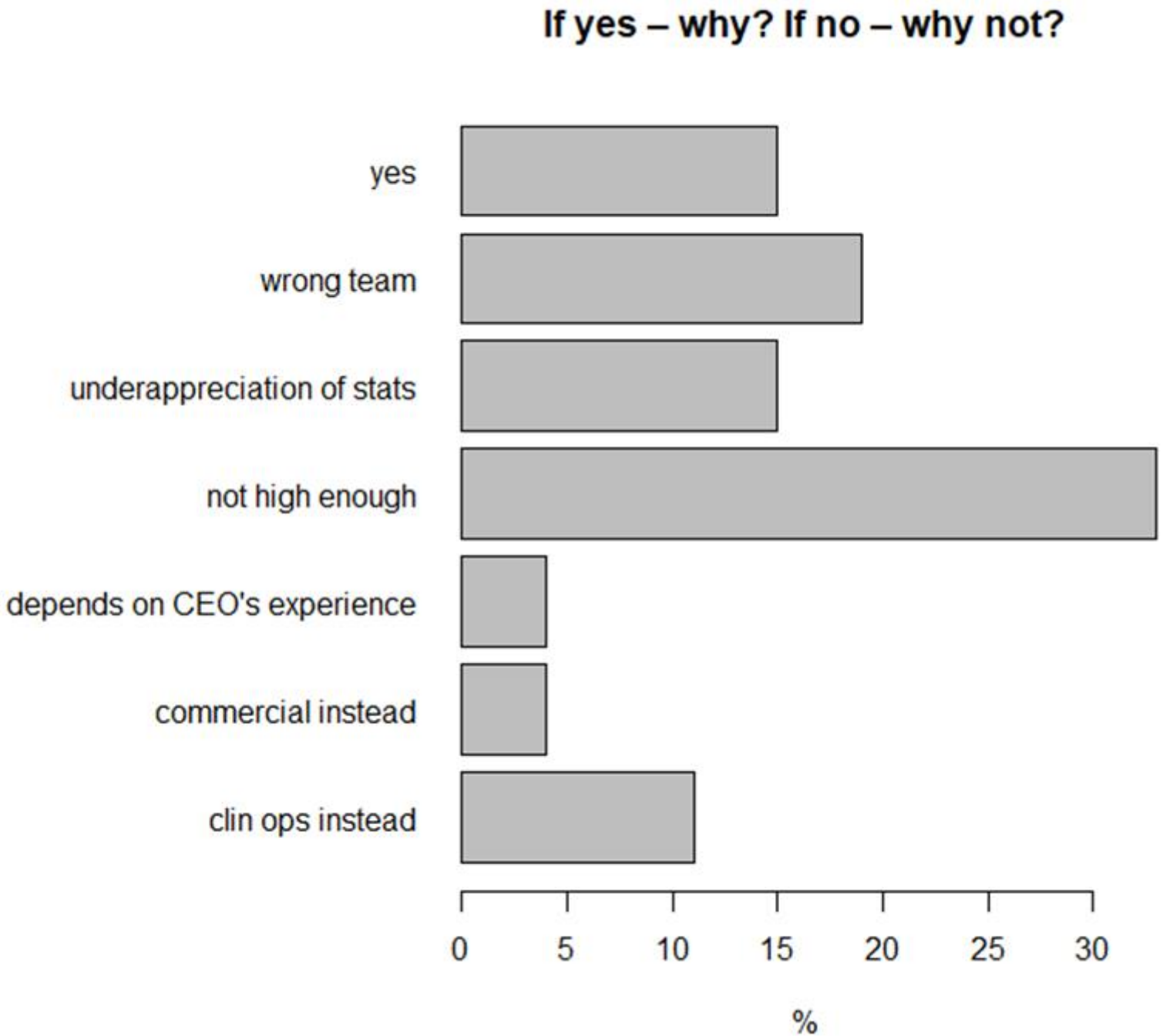
- Setting the scene
  - Introduction to the innovative work of “AI COV”
- Break-out sessions
  - Address ‘How leaders can promote innovation in drug development’
- Summary and key-take away messages
  - Consider how Statistical Leaders and Methods Leaders can/should collaborate

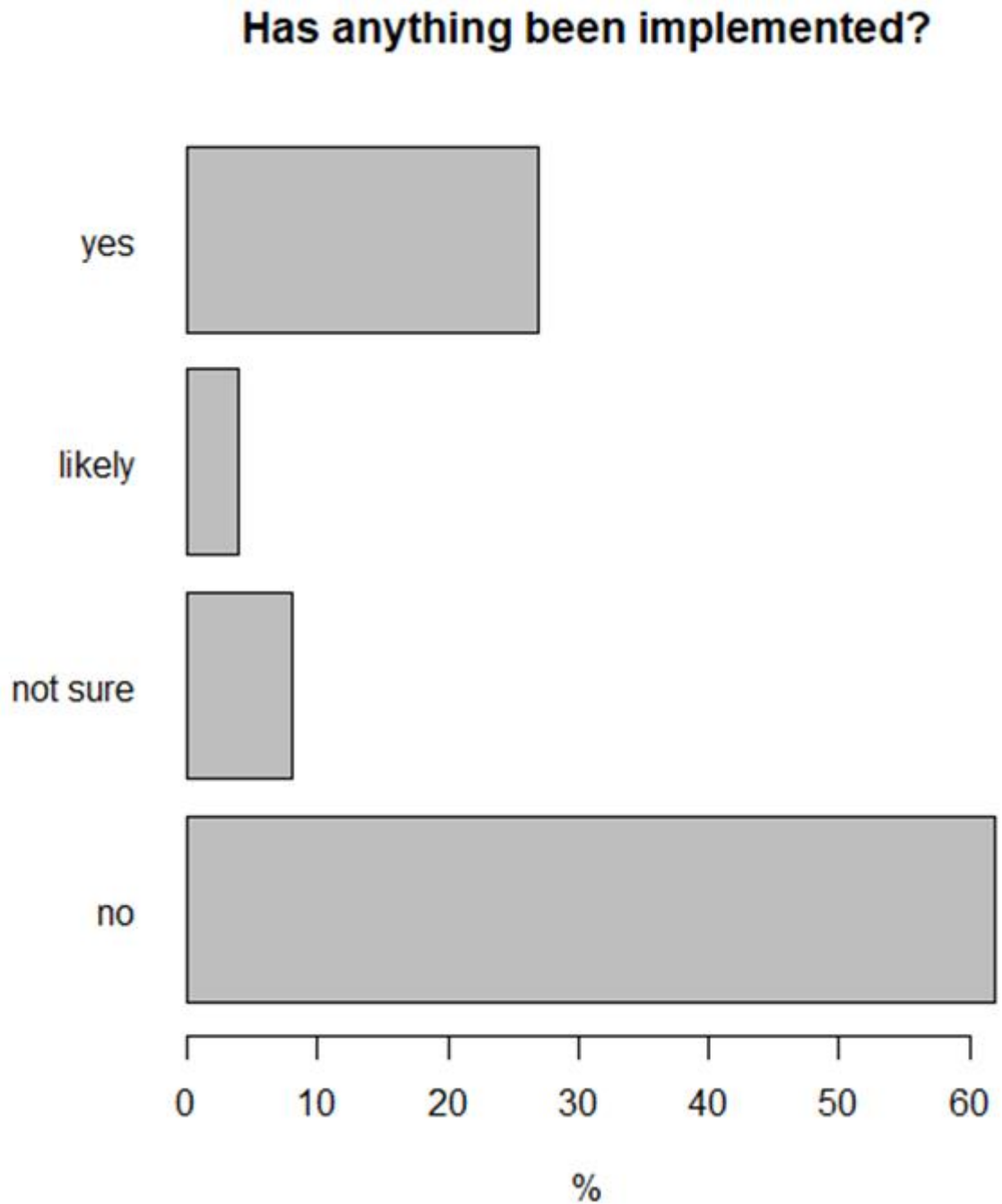
Results of the Survey

Questions pre-meeting survey

- Q1: Are you as data science leader the first person your CEO / CMO reaches out to if approached by the newest startup promising to reduce trial costs by 50% using some fancy method?
- Q2: If yes – why? If no – why not?
- Q3: Has any such «innovation» coming from a fancy startup been implemented on broad scale in your company in the last 10 years?
- Q4: Which quantitative method(s) are currently underutilized in drug development?


Are you as data science leader the first person
your CEO / CMO reaches out to if approached by the
newest startup promising to reduce
trial costs by 50% using some fancy method?
%
70
60
50
40
30
20
10
0
no
yes
sometimes


If yes – why? If no – why not?
yes
wrong team
underappreciation of stats
not high enough
depends on CEO's experience
commercial instead
clin ops instead
0
5
10
15
20
25
30
%


Has anything been implemented?
yes
likely
not sure
no
0
10
20
30
40
50
60
%

Observations

- Rarely data science leaders are the first to be contacted.
- Others (clin ops, commercial, dev teams) seem to be further up in line.
- It appears that rarely such «fancy approaches» get implemented.

Which quantitative method(s) are currently underutilized in drug development?

- AI / ML / LLM (5x)
- M & S (5x)
- Quantitative decision – making (5x)
- Causal inference (5x)
- Bayes (4x)
- Estimands (under / misutilized, 2x)
- Interim analysis (2x)
- Learn from failures (2x)

Mentioned once:

- Disease progression / predictive models // Statistical modeling outside clinical data // Productivity monitoring // Adaptive randomization // Subgroup analysis // Inform priors in later phases through early phase data

What have your companies prioritized?

- AI (e.g. For programming, prediction models, 7x)
- Bayes (7x)
- Better end to end data curation (5x)
- Quantitative decision making and de-risking drug development, probability of success (5x)
- Automation (4x)
- RWD (4x)
- Estimands, also in non-pivotal trials (3x)
- Visualization (2x)
- Causal inference and TMLE (2x)

Mentioned once:

- Insillico drug development // Centralized monitoring // Complex designs // Non-SAS software // Decentralized trials // Digital projects // Group-sequential designs // Innovative and complex designs // Adaptive randomization // Subgroup modelling to drive designs

Observations

- In general alignment between «underutilized» and «prioritized».
- AI / ML tops both lists.
- Many core statistical / quantitative topics that remain considered «underutilized»: quantitative decision-making, Bayes, M&S, ...
- Key new topic: causal inference.

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Wednesday, 10:15 – 12:30

- Setting the scene (10:00 – 10:15)
  - Introduction to the innovative work of “AI COV”
- Break-out sessions (10:15 – 11:45)
  - Address ‘How leaders can promote innovation in drug development’
- Summary and key-take away messages (11:45 – 12:30)
  - Consider how Statistical Leaders and Methods Leaders can/should collaborate

AI COV

- Source: How Digital Twins Are Reinventing Innovation (mit.edu)

CEO

I have been approached by a startup that has revolutionized statistics! They use all available clinical trial data to feed in their method and can thereby cut trial costs by half! Their approach can be deployed within a few weeks! Their stuff is just great and does not need input from us! I'll work with our clinicians so that they can quickly implement it in all our programs.

Clinician

You won’t believe what I learned at the medical congress! This is going to revolutionize everything! It’s called AI COV. It combines digital twins with covariate adjustment. It could save us millions and speed up drug development like you wouldn’t believe! It involves complex algorithms, artifical intellegence and machine learning. And there's something about adjusting for variables that… vary? The congress speaker made it sound so simple and a drug of our main competitor was approved based on AI COV.

Statistician

One of our DevTeam used this new software AI COV which compares two models: one model included the subgroup as a covariate; the other model used only the sugbroup data. They were wondering which analysis is correct as they observed different results. Using matrix algebra, I have analyzed both models. Based on inverting block matrices, we can compare the models directly. I have also done some simulations on this…

Break-Out Sessions

Break-Out Sessions

- Question 1: Why is uptake of useful inventions (think estimands, MCP-mod, futility analyses, adaptive designs, covariate adjustment, statistical engineering, ...) so heterogeneous between companies?

- Question 2: How can we better manage, sometimes avoid, large-scale implementation of inappropriate or insufficiently evaluated methods (think responder analysis, overuse of single-arm trials). How can we generate the trust with senior leaders that they reach out to us to evaluate “new” methods?

- Question 3: What can statistical leaders do to promote innovation in drug development?

- Question 4: Where and how is potential for collaboration to address the above questions?