
Implementation of statistical innovation in a pharmaceutical company - it can be done!

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I will tell a story.

I have not been part of that story.

**Story is partly discussed in
Rufibach et al. (2025).**

**I acknowledge Roche
statistics team + collaborators.**

Imagine the following situation:

**Pharma company runs two very large
very expensive Phase 3 trials in
key indication.**

**Treatment policy strategy for ICE
of treatment discontinuation.**

**Estimation via MMRM. Uses data as it
comes, inference valid under MAR.**

MAR not plausible for this estimand.

What was needed?

Primary estimand and estimator aligned.

**Statistical theory \Rightarrow conditional mean
imputation + jackknife.**

(validated) Software \Rightarrow `rbmi` package.

Output templates.

**Stats team made the company change
primary analysis in these trials.**

No statistical method.

No validated implementation.

No reporting templates.

No regulator had ever seen it.

Estimated effect smaller than with MMRM.

BUT THEY DID IT.





HOW?

Invention: new method.

**Commercialization: scaled-up
implementation of invention.**

Innovation = invention \times commercialization.

Implementation of Statistical Innovation in a Pharmaceutical Company

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ABSTRACT

Innovation, defined as the successful implementation at scale of a new invention, is key for continued success of the drug development enterprise. In this article we focus on statistical innovation in the pharmaceutical industry. We discuss both components, *invention* and implementation at scale, typically called *commercialization*, of innovation for statistical methods. These concepts are illustrated using three examples that we successfully implemented in our company. We summarize factors that foster or hinder invention and commercialization. A discussion of the mindset we consider supportive of innovation and how organizational leaders and professional organizations can generate an environment that fosters innovation concludes the article.

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Innovation; Invention;
Collaboration;
Commercialization; Statistics

Rufibach et al. (2025)

Potential hurdles for commercialization

- Insufficient knowledge.
- Lack of case studies.
- Lack of user friendly software.
- Regulatory position.
- Company-internal buy-in.
- Inertia.
- Statistician's mindset.

Yes, but have you thought about the assumptions, and this and that and blablabla. Ah, and by the way your proposal is great!

Rufibach et al. (2025): details & how to overcome hurdles.

Some success factors to innovate

- **Entrepreneurial spirit:**
 - Experts who are capable and can invest resources at their leisure.
 - Ownership, accountability.
 - Trust, reputation, credibility, network.
- **Organizational culture:** Accept failures without compromising on quality of work.
- **Specialized capabilities & investment.**
- **Software engineering.**
- **External collaboration.**
- **Designated project leadership.**
- **Timelines / urgency.**
- **Tools to facilitate adoption.**
- **Collaborations:**
 - Internally in other functions.
 - Industry, regulators, academics.

**What statisticians do we need
for innovation?**

Curiosity!



What statisticians do we need for innovation?

- Solid experience in drug development.
- **Invention:**
 - Broad methodological competence in statistics relevant to drug development.
 - Curiosity.
 - Park statistician's mindset of emphasizing everything that could go wrong.
 - Trust and credibility.
 - Collaborative: other methodologists, wherever they sit.
 - Remain connected to the drug development business.
- **Commercialization:**
 - Self-starter and pro-active.
 - Collaborative: Statistical engineering; statisticians working on projects; cross-functional molecule teams; senior management; regulators.
 - Persistence.
 - Modesty and teamwork. Sharing credit with collaborators who commercialize an invention is essential.

Hire for both!

Innovation = invention × commercialization.

Hurdles and how to overcome them.

Curiosity! And a few other things.

It can be done!

Thank you for your attention.

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**Slides can be downloaded on
www.kasparrufibach.ch**

References

- ▶ Rufibach, K., Wolbers, M., Devenport, J., Yung, G., Harbron, C., Bedding, A., Huang, Z., Lin, R., Pang, H., Sabanés Bové, D. and Wang, J. (2025). Implementation of statistical innovation in a pharmaceutical company. *Statistics in Biopharmaceutical Research* **17** 113–124.

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