

The Role of Model Assumptions in Statistics

Christian Hennig

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1 "The Death of Homeopathy"

A standard random effects meta-analysis

From Shang et al. "Are the clinical effects of homoeopathy placebo effects? Comparative study of placebo-controlled trials of homoeopathy and allopathy" (The Lancet, 2005)

Famous study, prompted Lancet-editorial "The Death of Homeopathy".

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n = 8. For trial *i* : p_i emp. prob. that "placebo works", q_i emp. prob. that "homeopathy works", $L_i = \log \frac{p_i/(1-p_i)}{q_i/(1-q_i)}$,

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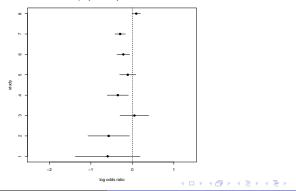
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 η_i trial specific effect, e_i within trial variation. σ_i standard error of L_i (small if n_i large),

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Homeopathy meta-analysis - artificial data



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Shang et al. find overall estimate $\hat{\theta} = -0.13$, 95%-confidence interval [-0.43, 0.17] for θ , conclude (because 0 is in CI) "no evidence for homeopathy better than placebo."

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Model assumptions:

- 1. Independence of trials, η_i and e_i
- 2. Additive model for L_i .
- 3. Normal distribution for η_i .
- 4. Normal distribution for e_i .

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How to check whether these are "true"? ... or at least "approximately true", "appropriate", "helpful" etc.

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What do the model assumptions mean? Independent repetitions Can frequentist model assumptions be checked? The goodness-of-fit paradox

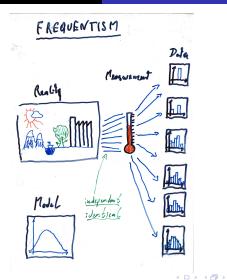
2. Frequentist model assumptions

(more general, "aleatory", incl. propensities etc.) **2.1 What do the model assumptions mean?** "We think of the situation as"

- Potentially infinite repetition (of experimental conditions)
- ► P(A): relative frequency limit of occurrence of A (e.g., normal distribution is defined by P(A) ∀A.)

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- Potentially infinite repetition (of experimental conditions)
- ► P(A): relative frequency limit of occurrence of A (e.g., normal distribution is defined by P(A) ∀A.)

This is obviously an idealisation - what constitutes a "repetition"?

"Whatever can be distinguished cannot be identical." (B. de Finetti)

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2.2 Independent repetitions ("i.i.d.")

Frequentism relies on "repetitions of experiments", e.g. results from different patients in clinical study.

Possible to *define* i.i.d. in terms of probabilities (in order to get results about distribution of L_i from independent patients) but this cannot justify defining probabilities in terms of i.i.d. repetitions.

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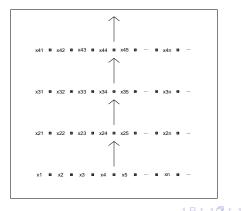
In order to define "i.i.d." sequences, i.i.d. repetitions and defining repetitions are required on different levels.



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In practice, there's only one level of repetition.

The effective sample size for (in-)dependence assumptions is usually 1.

But independent repetition *of some kind* is always required in order to learn from data.

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But independent repetition *of some kind* is always required in order to learn from data.

Independent repetition is constructed by conscious decision to ignore potential dependencies and differences.

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2.3 Can frequentist model assumptions be checked?

Goodness-of-fit/misspecification tests

If something modelled as very unlikely happens, the model is interpreted to be *falsified*.

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For example, could "falsify" independence from data where patients examined on same day have similar results.

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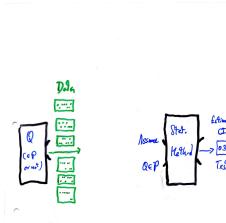
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2.4 The goodness-of-fit paradox (H, 2007)

Checking the model assumptions violates them automatically because the *possibility* of unlikely events is constitutive part of the models.

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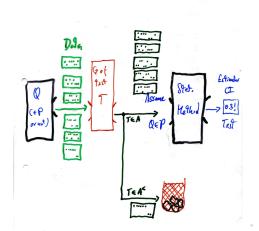
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3. Mathematical models and reality

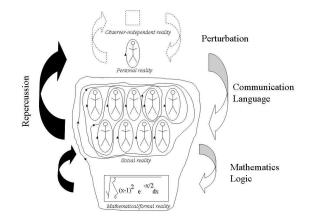
General mathematical modelling (broad and naive): mapping reality to mathematical objects.



Identification of items of perceived reality with mathematical objects and interpretation of results of mathematical operations in terms of the items.

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A constructivist view (H 2010, Foundations of Science)



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Implications for mathematical modelling

Science: establishing agreement in open exchange.

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Implications for mathematical modelling

- Science: establishing agreement in open exchange.
- Mathematics is about creating a system that makes absolute agreement possible (but only *within mathematics*).

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Implications for mathematical modelling

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- Mathematical modelling is not about how things are, but about how we think and communicate about them. (Models communicate and change views of reality.)

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Implications for mathematical modelling

- Science: establishing agreement in open exchange.
- Mathematics is about creating a system that makes absolute agreement possible (but only *within mathematics*).
- Mathematical modelling is not about how things are, but about how we think and communicate about them. (Models communicate and change views of reality.)
- It cannot be formally analysed how formal models are related to informal reality.

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4. "Frequentism-as-model"

$$\begin{aligned} & \mathcal{L}_i = \theta + \eta_i + \boldsymbol{e}_i, \quad i = 1, \dots, n, \\ & \eta_i \sim \mathcal{N}(0, \sigma_0^2), \quad \boldsymbol{e}_i \sim \mathcal{N}(0, \sigma_i^2). \end{aligned}$$

Not: is this (approximately) true? **But:** can we learn what we want to learn about our subject matter (homeopathy) if we decide to look at the data "through" this model?

What does this view imply?

Is model consistent with our (agreed) perception of data?

(Frequentist) models are useful to...

- communicate researcher's perception of situation,
- inspire methodology,
- check quality of methodology in situations with known (made up) truth. (Tukey, Davies)

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This is helped by... Model-based mathematical theory:

- How well does method work under ideal conditions?
- How much variation to expect?
- Assume model, derive optimal method.
- Other properties of method (equivariance etc.)

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Also informs about

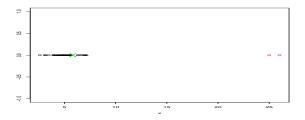
effects of model deviations, e.g., data outliers.

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Some violations of assumptions harm interpretation of results

Reasonable model checking:

 $\begin{array}{l} \text{Outliers not expected under } \mathcal{N}, \\ \text{affect } \mathcal{N}\text{-based statistics.} \end{array} \end{array}$



Some violations are *not* problematic (e.g., g.o.f.-testing, often).

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Re-formulation of "model checking":

Find out whether data could lead statistical method (derived from model) astray.

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Aspects of homeopathy meta-analysis

$$\begin{split} L_i &= \theta + \eta_i + \mathbf{e}_i, \quad i = 1, \dots, n, \\ \eta_i &\sim \mathcal{N}(0, \sigma_0^2), \quad \mathbf{e}_i \sim \mathcal{N}(0, \sigma_i^2) \text{ independent.} \end{split}$$

"Trial effect" is treated as result from i.i.d. repetition, \Rightarrow "effective sample size" is n = 8, not $\sum n_i > 5,000$, bad power, i.e.,

non-significance can easily happen under $\theta < 0$.

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Furthermore, trials apply homeopathy differently; only 2 trials treat "classical homeopathy".

Modelling all as i.i.d repetitions implies that how homeopathy is applied is not "difference of interest". \Rightarrow "Classical homeopathy" paradigm is not tested by this study.

5. Beyond classical frequentism

5.1 Bayesian philosophy and methodology

- ► Prior distribution on parameters θ allows posterior probability statements P(θ ∈ B) =?
- Epistemological rather than aleatory probability (although in practice often ignored)

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(Subjective) Bayes Madel Rulity D.t. Future out comes

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5. Beyond classical frequentism

5.1 Bayesian philosophy and methodology

- Prior distribution on parameters θ allows posterior probability statements P(θ ∈ B) =?
- Epistemological rather than aleatory probability (although in practice often ignored)
- Requires very similar idealisation; ultimately same modeling issues as frequentism.

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5.2 Nonparametrics/machine learning

Often claim "no assumptions",

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- generalisation theory still assumes frequentism/i.i.d., (can be seriously harmed by bad data quality),

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5.2 Nonparametrics/machine learning

- Often claim "no assumptions",
- big datasets allow very flexible fitting, but...
- generalisation theory still assumes frequentism/i.i.d., (can be seriously harmed by bad data quality),
- implicit structural assumptions (e.g., class shapes, similarity).

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6. Conclusions

 Decision whether a model is appropriate/helpful is quite different from checking whether it's true (the latter can't be done, not even approximately)

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- Major role of model assumptions: understanding methods under idealised conditions

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6. Conclusions

- Decision whether a model is appropriate/helpful is quite different from checking whether it's true (the latter can't be done, not even approximately)
- Major role of model assumptions: understanding methods under idealised conditions
- Rather than "model check": Will data lead method astray?

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