Questions, Aims & Objectives, & Hypotheses
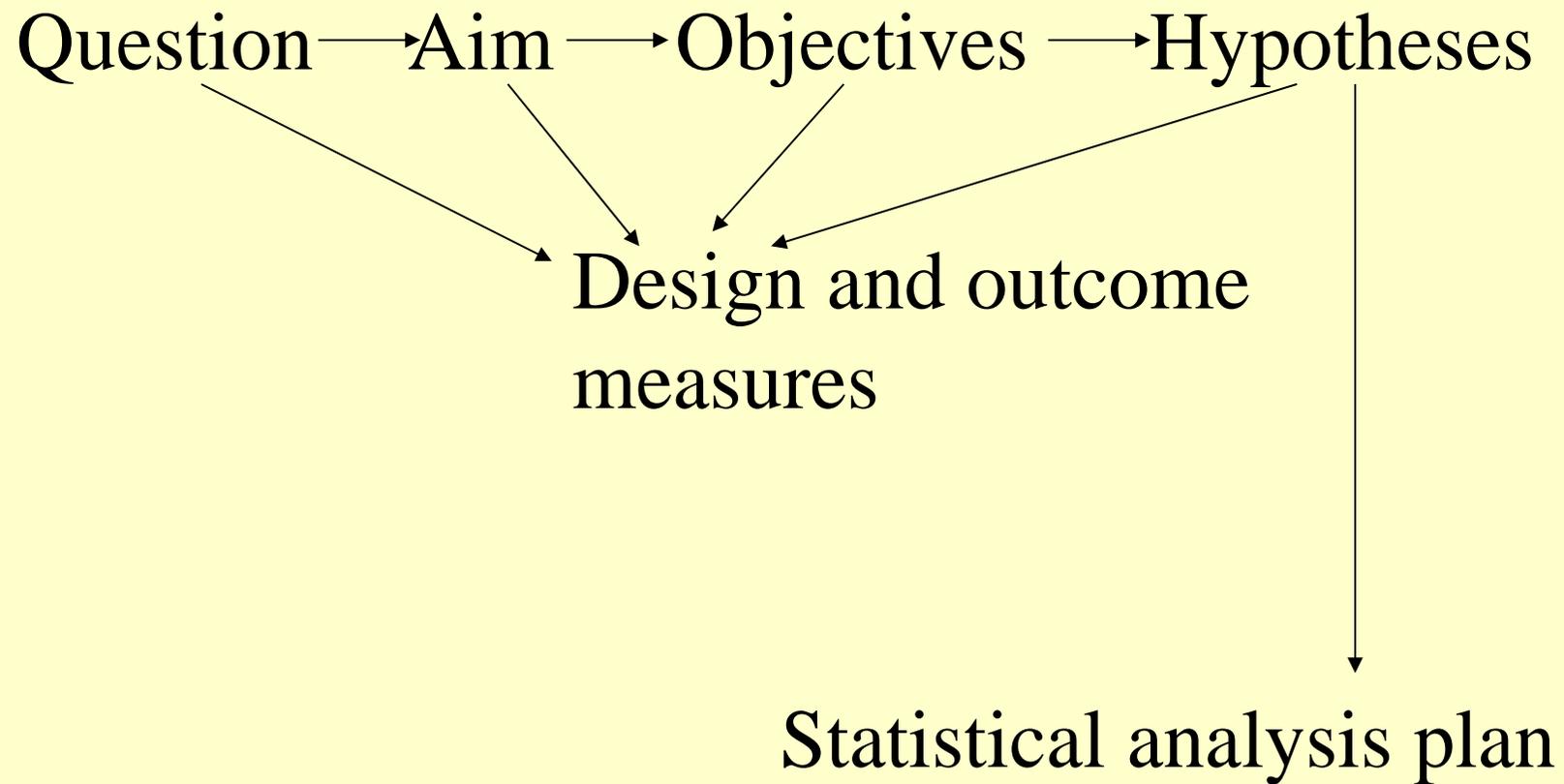
Martin Prince, with thanks to Claire Henderson, KCL

From the question to the hypotheses

Question $\longrightarrow$ Aim $\longrightarrow$ Objectives $\longrightarrow$ Hypotheses

Design and outcome
measures

Statistical analysis plan

The hypothesis

- Characteristics
  - *a priori* (rather than *post hoc*)
  - Clearly stated
  - Testable and refutable
  - Not a mere research question or objective
  - Leading to a sample size calculation, and an appropriate design and analysis

- Advantages
  - Greater credence given to validity of findings
  - Less risk of type I error ('false positive' results)
  - Ease of replication

Research

- "Research can be defined as the attempt to derive generalisable new knowledge by addressing clearly defined questions with systematic and rigorous methods."

- *Research Governance Framework for Health and Social Care, 2nd edition, 2005*
  http://www.dh.gov.uk/PublicationsAndStatistics/Publications/PublicationsPolicy AndGuidance/PublicationsPolicyAndGuidanceArticle/fs/en?CONTENT_ID=41 08962&chk=Wde1Tv

Research questions

- Deficits and uncertainties in important areas of knowledge and understanding
- Potential sources:
  - Clinical observations
  - Theory
  - Academic discourse
  - Literature

1.The research question

- Starting point
- Clearly framed
- Justified (important, original, answerable)
- Question e.g. 'does psychotherapy help patients with anorexia nervosa'
- Problem statement e.g. 'there is little available evidence on the efficacy of psychotherapy for patients with anorexia'
  - Is this OK as a starting point?
  - How might we move from here to objectives and hypotheses?

2. Aim

Distinction vs. objectives

- The aim of the study is to answer the research question and is a summary statement
- Objectives specify the components of how the aim will be met.

Aim indicates design

Example

Question: Does the use of a joint crisis plan reduce coercion in mental health care

Aim: to establish whether joint crisis plans are effective in reducing coercion in mental health care

This is a better question….

What design might be implied here?

3. Objectives

- Unpack aim into operationalised study components
- Some may be formative
- Otherwise, primary and secondary objectives translate directly into primary and secondary hypotheses, and thence outcome measures
- Prioritise (limit) objectives to avoid too many hypotheses and outcomes.
- Present them in active language, e.g. 'to quantify', 'to determine'

4. Hypothesis

'...*Omission of hypotheses is a major error in that your reviewers can criticize you for conduction a "fishing expedition". This is one of the most common criticisms made by NIH reviewers'.*

http://www.niaid.nih.gov/ncn/grants/default.htm

Karl Popper

- *- '…It is easy to obtain confirmations, or verifications, for nearly every theory — if we look for confirmations.*
- *- Every genuine test of a theory is an attempt to falsify it, or to refute it.*
- *- Some genuinely testable theories, when found to be false, are still upheld by their admirers —by introducing ad hoc some assumption, or by reinterpreting the theory ad hoc in such a way that it escapes refutation. Such a procedure is always possible, but it rescues the theory from refutation only at the price of destroying, or at least lowering, its scientific status.'*
- POST HOC SUB-GROUP ANALYSES!!

Hypothesis statement

- Freeman and Tyrer (1992) offer examples:

- **Vague hypothesis**: 'Does psychotherapy help patients with anorexia nervosa?'

- **Precisely formulated hypothesis**: Does psychotherapy, in the form of cognitive therapy, when given for ten weeks, lead to significantly greater gain in weight in anorectic patients than in those not receiving cognitive therapy?'

- **Null hypothesis**: There is no difference in the weight gain of patients with anorexia nervosa when treated with cognitive therapy compared with a control procedure.'

- Are we happy with these?

Specific Hypotheses

- Base population – patient group or sub-group, context etc
- Intervention, and the counterfactual (e.g. treatment as usual, placebo etc)
  - Duration, dose, delivery
- Outcome(s)
  - Which outcomes, observed over what period, or at what time (endpoint)
- Effect size
- Sub-group analyses and any interactions to be tested (generally secondary hypotheses)

Null hypothesis

- The **null hypothesis** is set up to be nullified or refuted in order to support an *alternative hypothesis*.

- This approach to setting up & testing of hypotheses is an essential part of statistical inference.

- In each problem considered, the question of interest is simplified into two competing claims - the null hypothesis, denoted H0, against the alternative hypothesis, denoted H1.

Null hypothesis

- When used, the null hypothesis indicates "there is no phenomenon", any observed differences arising from chance.

- These two competing claims / hypotheses are not however treated on an equal basis, special consideration is given to the null hypothesis.

- H0 presumed true until statistical evidence - a hypothesis test indicates otherwise.

Objectives and hypotheses: how many is too many? (1)

Balance between

maximising the opportunity to obtain information

Vs

Overburdening respondents and research assistants and looking as though you are fishing and have no model

Objectives and hypotheses: how many is too many? (2)

Things to consider:

Does my model (e.g. ToC) for this intervention really predict all these outcomes?

If the primary hypothesis is supported, then how important is it to test all of the secondary hypotheses?

Can I prioritise some of the secondary hypotheses?

Prioritising objectives and hypotheses

- Importance of outcomes to be measured
  - Consider views of different stakeholders: service users, carers, clinicians, managers, govt.
- Theoretical strength of outcomes based on model-proximity to proposed mechanism of action
- Technical measurement issues
  - Likely correlation between outcomes
  - Quality of existing measures (validity, precision)
- Ease of data collection (routine data, length, cost, burden)

Sample size calculation

- Based on the primary hypothesis
- Confidence, power, randomisation ratios and minimum detectable effect
- Minimum detectable effect?
- Not effect sizes reported in other similar trials
- Smallest effect that, if true, might change policy or practice

Primary and secondary hypotheses

The primary hypothesis to be tested is whether Joint Crisis Plans significantly reduce the proportion of service users detained or treated under a section of the MHA during the 18 month follow-up period, compared with the control group.

Secondary hypotheses will be to determine if compared with the control condition, JCP use will result in significant improvements in: total costs, perceived coercion, service user engagement, therapeutic alliance.

Sub-analyses will examine the effectiveness of the JCP in reducing use of the Mental Health Act for Black (Black Caribbean and Black African) service users.

Thornicroft G et al. CRIMSON [CRisis plan IMpact: Subjective and Objective coercion and eNgagement] Protocol: A randomised controlled trial of joint crisis plans to reduce compulsory treatment of people with psychosis. Trials 11: 102-8, 2010.