

# Are patients better treated in clinical trials?



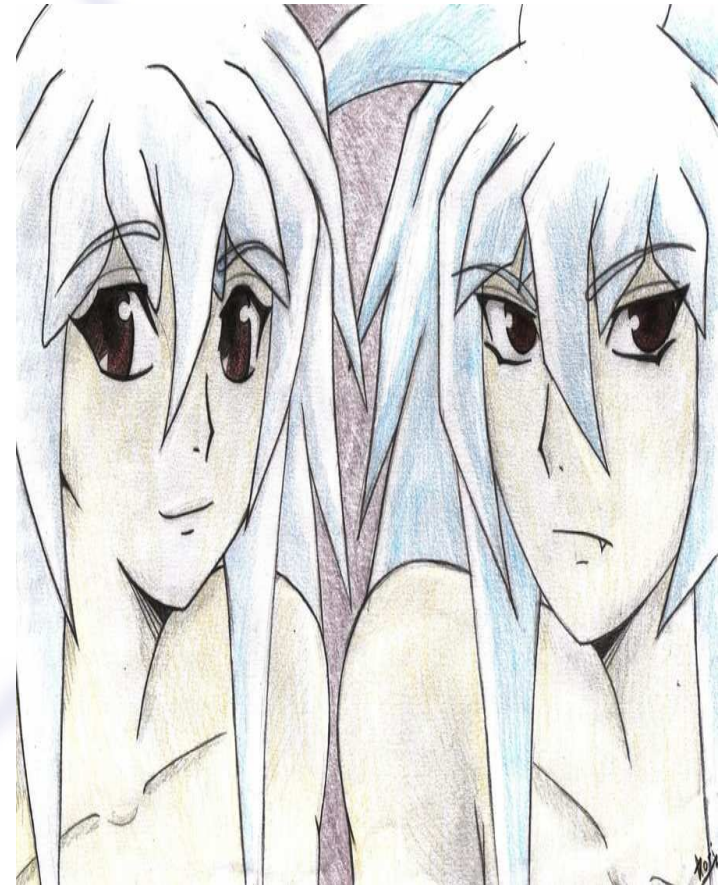
**YES - NO**

D. VERHOEVEN



not the same as.....,similar but  
so different !

- Are patients treated well in clinical trials?
- Must we include patients in clinical trials?



# ASCO

- “Treatment in a clinical trial is often cancer patient’s best option”
- Political statement to increase accrual and third party payment



# US National Comprehensive Cancer Network

- Position with patients and doctors :
  - “the best management for any patient with cancer is in a clinical trial”
  - [www.nccn.org/patients/guidelines/breast/index.html#/40/](http://www.nccn.org/patients/guidelines/breast/index.html#/40/)

# Definition of a clinical trial

Clinical trials are experiments with as purpose:

1. determine the value of a treatment
2. key components
  - Results
  - Answers to questions
  - Society and company driven

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# Right of the patients

- Good medicine everywhere
- To have a superior physician
- Clinical trials not available everywhere
  - No trials
    - 95% patients
    - 40% children
- Fear to become a guinea pig
- Informed consent



# Phase I trials

- Clinical benefit minimal (perhaps growing?)
- Competition for patients and inclusion between centres
- Independent ethical considerations
- Much examinations and hospital visits
- Knowledge of palliative care can be critical



# Selection criteria for phase 1 trial

- AIM: toxicity and dose finding of a new drug
- Challenging criteria
  - 90 day mortality: 14%
  - 0,5% toxic death





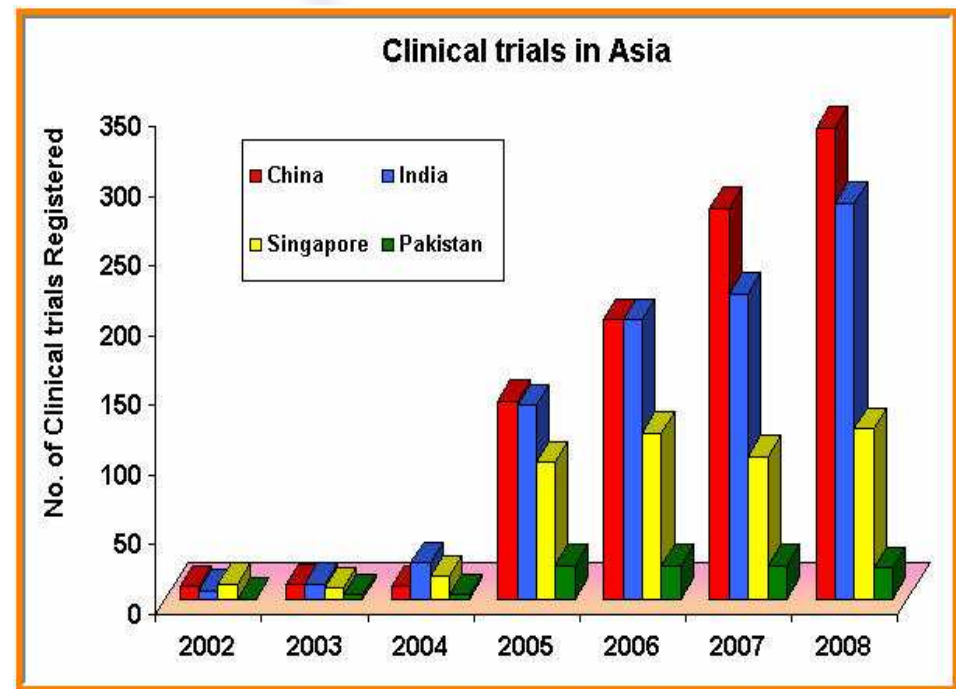
# Phase 3 study

- AIM: to answer questions
  - Conventional versus new, promising treatment
- Promising treatment can be inferior, more toxic (or more costly)
  - Herceptin studies
  - ALTO study
  - Beth study



# Clinical Trials in developing countries

- Growing market
- Rules and ethics less restrictive
- Lower cost and method to obtain new drugs



# Regulations of clinical trials

- Bureaucracy
- Stopping rules
- Clear endpoints
- GCP NOT in function of a company !
- Value of informed consent
  - Research biopsies in Clinical Trials
- Benefit patient comes first

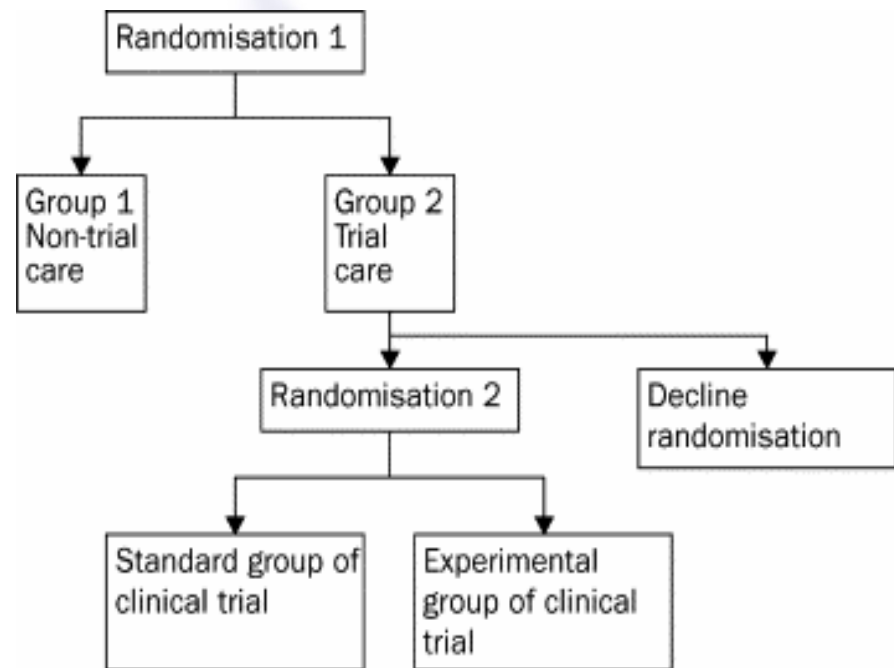


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# Comparison of outcomes in cancer patients treated within and outside clinical trials: conceptual framework and structured review, Peppercorn et al ,2004,Lancet

- Large retrospective study (only ethical way)
- Conceptual framework for comparison of trial and non trial patients
- Search of the medline
- 26 comparisons from 24 published trials



# Possible reasons for improved outcome ?

- Experimental treatment effect
- Participation effect
- Prognostic favourable subset
- Method of data gathering
- Publication bias

**=> Very little unbiased evidence of outcome improvement is available**

# Systematic review to determine whether participation in a trial influences outcome, Vist G, 2005, BMJ

Results of dichotomous main outcomes : trial versus non-trial participation

Comparison of mortality :trial versus non-trial participation

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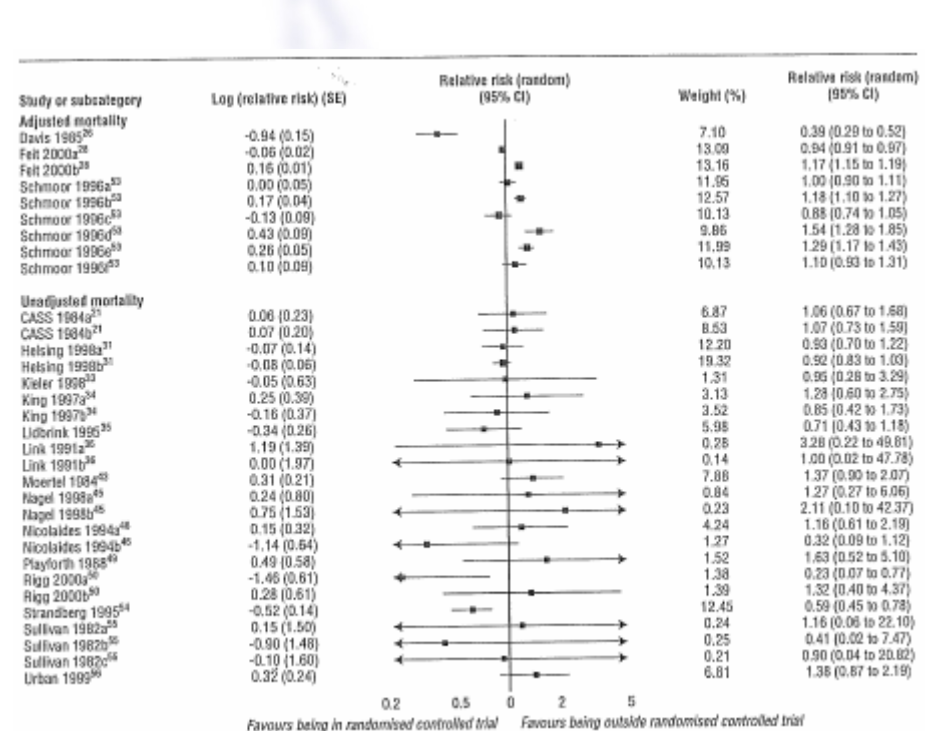
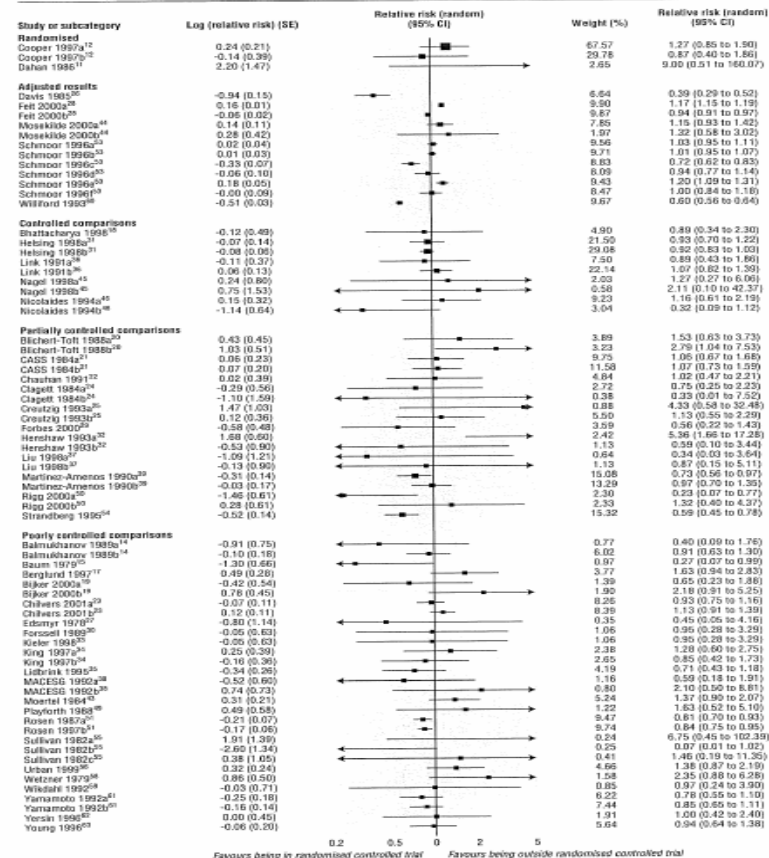


Fig 2 Results of dichotomous main outcomes in participants of randomised controlled trials and comparable non-participants who received the same or similar treatment

# Patients do better at hospitals with clinical trials

- Clinical trials are performed in bigger hospitals
- Bigger hospitals have better teams
  - Physician leadership
  - Shared team goals
  - Administrative support
  - Credible feedback





# Conclusions



- **PRO**

- Chance that new treatment will improve outcome
- Participation will improve medical care and follow-up

- **CONTRA**

- Change that new treatment will NOT improve outcome
- More toxicity
- Feel like research subject
- More time spent in the study, administrative burden!
- Less attention to palliative care

OH, Christoph...patients are treated well in clinical trials, TOO...,but...

- Do not use one-liners!
- Insufficient data to claim trial effect
- Enrol on the basis of improving treatment for future patients!



# Acknowledgement

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