Patient involvement in clinical trials

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Clinical studies

- non for profit
- for profit

Clinical studies

- investigator initiated
- industry sponsored
Clinical studies

- Investigator initiated
- Industry sponsored
- Industry supported
- Non-industry supported

Clinical studies

- Investigator initiated
- Patient initiated
- Industry sponsored
- Industry supported

Industry-supported studies

- Study conduct
- Data analysis
- Translational science
Industry-supported studies

- study design
- study conduct
- data analysis
- translational science
- ...

Phase I
- dose!
- $\checkmark$ MTD

Phase II
- $\checkmark$ OR
- $\checkmark$ OS
- $\checkmark$ QoL

Phase III
- activity!
- efficacy!

state of the art

Advisory role to the academia
Advisory role to the industry

Pharma

Researchers

Patients
Clinical decision-making

Methods to combine evidence

New study designs

Organization of studies

Study design

Joining forces for action
The Bayesian probability...

The frequentist probability in clinical trials...

\[ p < 0.05 \]
$R < p \leq 0.05$

$R < p \leq 0.05$

$R < p \leq 0.05$
Shared decision-making...

### Ethics and Models

- **Authoritarian**
- **Paternalistic**
- **Liberal**
- **Social**

#### Principles

- **Beneficence**
- **Non-maleficence**

#### Models

- **Deontologic**
- **Utilitarian** (individual)
- **Utilitarian** (social)

#### Equity

- **Autonomy**
- **Social**

- **Beneficence**
- **Non-maleficence**
Ethics needs principles—four can encompass the rest—and respect for autonomy should be "first among equals."
R Gillen
J Med Ethics 2010;36:267-272

Study design

The equipoise...
The ethics of clinical research require equi- poise — a state of genuine equipoise on the part of the investigator — as a necessary premise for the ethical conduct of clinical research involving human subjects. The premise of equipoise is that the treatment is superior to the control condition and that the investigator believes the treatment to be either superior or worse than the control condition. The current understanding of this equipoise, which rests on the principle that the investigator himself determines whether the treatment is better or worse than the control condition, is not sufficient for ensuring the ethical conduct of clinical research involving human subjects. This paper reviews the implications of the concept of equipoise with the potential to improve the ethical conduct of clinical research involving human subjects.
Attitude toward risk...

RESEARCH ETHICS
Should desperate volunteers be included in randomised controlled trials?
P Allcock, S Hancox

CONCLUSION
It can be ethical to include BCTs that recruit desperate volunteers provided there is collective engagement throughout the course of the trial (as assessed by the BMSF and Trial Inception Committee).

J Med Ethics 2006; 32: 548

«Salvage» therapies...

P > 0
U > 0
New drug
BSC
Phase I
- dose
- activity
- MTD

Phase II
- activity
- OR
- OS
- QoL

Phase III
- efficacy
- state of the art

Natural end-points

«Surrogate» end-points
Study end-points

- Overall survival
- Quality of life
- Relapse-free survival
- Progression-free survival
- Tumor response

\[ R < p < 0.05 \]