

Meaningful Patient Involvement in Clinical Trial Endpoints



Society for
**Participatory
Medicine**

Bringing together e-patients and health care professionals.

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The Nuffield Trust
FOR HEALTHCARE LAW, ETHICS
AND POLICY

The Autonomous Patient

Ending paternalism in medical care

Angela Coulter

The Jolles Fry Fellowship, 2002

Angela Coulter



Paternalistic caring

Sensible – up to a point

“No, honey –
you don’t know
what you need.”

“I’ll take care of you.”

“I’ll decide for you.”



**When does
autonomy
become
appropriate?**



Empowerment

“An empowering approach to participation treats poor people as co-producers

with authority and control over decisions and resources

devolved to the lowest appropriate level.”

World Bank's definition

Empowerment

“An empowering approach to participation treats poor people as co-producers

with authority and control over decisions and resources

devolved to the lowest appropriate level.”

Empowerment

“An empowering approach to participation treats patients as co-producers

with authority and control over decisions and resources

devolved to the lowest appropriate level.”

Institute of Medicine – Sept 2012

Major New Report: “Best Care at Lower Cost”

TABLE S-2 Characteristics of a Continuously Learning Health Care System Science and Informatics

Real-time access to knowledge—A learning health care system continuously captures, curates, and delivers the best available evidence to guide, support, and improve clinical decision making and care safety and quality.

Digital capture of the care experience—A learning health care system captures the care experience on digital platforms for real-time generation and application of knowledge for improvement.

Patient-Clinician Partnerships

Engaged, empowered patients—A learning health care system is anchored in patient perspectives and promotes the inclusion of patients, families, and other members of the continuously learning care team.

Incentives

**Yes, the IOM itself
says e-patients are an
essential part of
tomorrow's healthcare.**

Patient-Clinician Partnerships

Engaged, empowered patients—

A learning health care system is

**anchored on patient needs and
perspectives**

and promotes the inclusion of patients,
families, and other caregivers as vital
members of the continuously learning
care team.

**Who gets to say
which outcomes
are important??**

**Who gets to say
which outcomes
should be goals?**

**Who gets to
declare which
endpoint
we measure?**

**Who gets to say
what's in the
patient's best
interest?**

Paternalistic caring

Sensible – up to a point

“No, honey –
you don’t know
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**I predict a new
battlefield:
time versus certainty**

**To the scientist,
the time when we
find the truth
is irrelevant to its worth.**

**But to a patient
with a progressive
disease,
time is everything**

Time Value of Money

“A dollar today
is worth more
than a dollar next year”

Patients are proposing there's a

Time Value of a Treatment

**“A treatment today
is worth more than the
same treatment next year”**

**For patients with a
progressive disease ...**

**“A treatment today
may be worth more than a
better treatment next year”**

**What factors merit tracking
as a patient's condition
and development of a treatment
proceed separately but
simultaneously?**

Factors in an equation for the time value of a treatment

- **Efficacy**

- We assume efficacy will improve as we continue developing and testing the intervention

- **Certainty** of the benefit (low p)

- More studies, and improvements in development, should improve certainty over time

- **Utility** to the patient (need, benefit, etc)

Time Value of a Treatment

as time progresses:

$$V_{tx,t} = f(\text{efficacy}_t, \text{certainty}_t, u_t)$$

**Scientists quantify
efficacy and certainty.**

**Only the individual patient
can assess the utility.**

June 11, 2014

www.bmj.com/content/348/bmj.g3726

Announced –

- Patient advisory panel
- Patient peer review of research papers
- Authors are to document how they involved patients in defining the question and outcome measures



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- Parkinson's slides - who gets to say?
- Angela Coulter autonomous patient
- BMJ patient advisors
- IOM patient needs and perspectives
- Paternalism slides / Zoe in back seat
- Karolinska / science didn't have a way to understand yet but it was still true.
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