MADRID 2014 ESMO congress

CHORDOMA FOUNDATION

Why is it so important to do research together with patients instead of doing it about or for patients ?

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Disclosure and Personal

- Nothing to disclose, just a patient
- Hans Keulen MIM, co-own IT firm, 57, married, 3 children.
- Chordoma patient since 2009
- Several surgeries, participated in clinical trials
- Progressive disease
- European liaison of Chordoma Foundation

About Chordoma

- A devastating bone cancer of the skull and spine
- Strikes people of all ages
- Incidence 0.8 : 1.000.000
- 7-9 year average survival
- Major impact on quality of life
- 20-30% overall cure rate
- No effective drugs (yet)

Patient involvement now

Trial time-line:

- Clinical/Scientific question
- Trial Design (criteria, endpoints)
- Trial preparation (protocol, consent)
- Patient recruitment
- Manage trial

Patients are mostly only involved in last steps….

Challenges

- Trial designs are not nature's laws
- Good science is not always good healthcare
- Patients are not Guinea pigs
- HTA
- Quantity vs. Quality of Life
- The randomized placebo controlled trial as holy grail
  - Ethics
  - Statistics (personalized medicine-smaller groups)

Patients

- Look at other ways to disease than doctors, only patients knows what it is like to be a patient.
- Feel the sense of urgency and therefore are highly motivated
- Are (as a group) heterogeneous
- Want to understand the odd's and evens before enrolling (risks, side-effects etc.)

What should change ?

- Have patients(-organizations) participating from the design phase throughout the entire trial
  - we know what is relevant in a trial for patients
  - we know what adds value to a patients life
- See patient organizations as equal partners in the trial
- (Mutually) respect all the stakeholders with their (different) knowledge and capabilities
- Make trials more adaptable; use experience gained in the trial

Wins

- Less risk, more comfort, better info for patients participating in trial
- More balanced criteria an endpoints. Therefore better judgment on real benefit of treatment
- Easier HTA process
- Easier access to and recruitment of patients
- And as a spin off:
  - Joint forces towards politics and regulations
  - More sources for funding

One more thing…

Just a naughty idea

- Patient groups could be the sponsor (initiator) of a trial…..

- Especially when re-using existing drugs.
- Especially in (small scale) Phase II trials.