Patient reported outcome measurements in clinical trials

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One of the challenges in evaluating clinical trials is 'How do you measure what you think you are measuring?' In 1984 the treatment for breast cancer was mastectomy as a primary surgical treatment with little attention paid to the quality of life such an approach may have for the individual. The intention was to get rid of the cancer and job done. Informed consent was an arbitrary consideration.

Malcolm Baum & Leslie Fallowfield 1984 were involved in a clinical trial to evaluate the benefit of mastectomy versus breast conservation. The trial failed to recruit in the quantities required as women did not want to be randomly selected for a mastectomy or breast conservation. However 100 patients who were willing to be involved in a study showed that even if you had breast conservation that significant psychological factors remained as you had to cope with a life threatening disease and body image issues. The perceived view of the medical community had been that women with breast conservation would have no psychological problems.

Today we have had significant improvements in surgical treatments, radiotherapy, chemotherapy and adjuvant treatment. Treatments however continue to have side effects as no treatment is risk free. Patients are required to give informed consent and should take into account their preferences. Quality of life measures are important in assessing the patient experiences and consequently doctors fill in forms to check on the treatment burdens. As these forms are completed it becomes apparent that the doctor subjectively selects the ones to concentrate on based often on their ability to solve the issue. The patient often quantifies their quality of life issues differently.

When palliation is the goal patient reported outcomes, it could be argued should be the most important measure. Complete responses to treatment are rare instead partial responses or stable disease is the goal. The side effect of continuing treatment may outweigh the benefit of quality of life and patient reported outcomes become central. The doctors recording of quality of life therefore needs to coincide with the patient's own measures and their preferences. Life at all costs may be the doctor's stance but for the patient this might be different.

What evidence do we have that doctors, nurses and patients outcome measures are aligned?

Research from Stromgren 2001, Greimel 2011, Basch 2006, Fellowfield 2007 all identify areas where the patient reported symptoms are significantly at variance with the professionals. The case recorded forms show little concordance between life-threatening versus quality of life. If as professionals the mechanisms to accurately reflect the experiences of our patients are poor then over prescribing and disease burden achieve little tangible benefit.

Clinical trials need to have the ability to reflect all outcomes accurately to do so needs-effective measures. Measurements available at present are:

EORTC QLQ -30 + modules. FACT -G subscales.

The case for patient recorded outcomes grows; the medical community needs to look to ways to effect changes so that trials reflect accurately the patient experience. The government aim for patient centred care must do more to address the experiences of the patient. Patient reported outcomes are the foundation to build on. Clinical trials need to improve their recording of data and construct trials where the patience experience is the main focus rather than a bolt on afterthought.

The notion that professional outcome measures are more objective and scientific than patients is misguided. The patient deserves that decisions made reflect and are in concordance with their own experiences of their medical care.

Report compiled by Jill Bartrop ICPV, Sept 2012