My husband is a guinea pig

Robyn Wheldall

My husband is a guinea pig and I'm very happy about it! This may sound like a strange thing to say, but when one is faced with a life-threatening disease potentially taking over someone who you love, your perspective changes pretty quickly. Kevin has been engaged in research for over 50 years and I for over 30 years. We now find ourselves on the other side of the table, with K being considered for a clinical trial to treat his multiple myeloma. When one is familiar with the all-important steps in designing and implementing research studies, it is fascinating to be a part of this process when you are a 'subject' or participant.

Sometimes the holy grail of double-blind randomised controlled trials to assess the efficacy of a new medication is just not possible when we are dealing with human beings. The perfect experiment may be neither possible nor desirable when there are conflicting responsibilities. Medical researchers are often doctors first and foremost and are bound by the Hippocratic Oath, 'First, do no harm'. When medical researchers are planning clinical trials they must carefully assess the risks and benefits of what they are proposing. If a participant is in the experimental group, are the researchers confident that a new medication has the real potential to have a positive effect on containing a destructive disease? Might this new medication have an unintended negative consequence on the patient? Might it have no effect at all and while the experiment runs its course, a patient's disease has progressed to an irreversible point? And what about the patients in the control condition? Will they be put at risk by the treatment they receive? Will they have nothing but a placebo? Fortunately, reason and humanity prevails in the medical research we are involved in.

The clinical trial that Kevin is being assessed for will ensure that the participants are treated with a great deal of care and caution. What are the hallmarks of this approach?

1 **Transparency.** There is no attempt to keep secret whether patient participants have been allocated to the experimental or control condition. In the case of the clinical trial K is hopeful of joining, we know there is only a 25 per cent chance that he will pass the first hurdle – which is to have a certain chromosomal translocation, assessed by an invasive bone marrow biopsy. Even if K were to have this characteristic, he then only has a one in two chance of getting the new 'experimental' drug because he will be allocated randomly to the experimental or control condition. So, overall, there is only a one in eight chance that he will gain a potential benefit from the new drug by participating in this research. It is important to be prepared for this disappointment if you are the patient participant who misses out. However knowing that the control condition receives the medication you would otherwise get had you not participated in the research trial – the best 'business as usual' next line of treatment – is a comfort.

2 **Informed consent.** Knowledge is power and although one has no means of influencing the genetic makeup of one's disease, it is possible to arm oneself with facts about the research you are being asked to participate in. Human ethics committees (sometimes frustrating for researchers) provide a safety net for participants to be well-informed about the proposed experiment and provide

the means by which participants can opt out at any time without consequence, if things become too difficult for them (or they just change their mind). While participant attrition is a blow to researchers, it does provide a level of comfort to participants and arguably encourages more people to participate in research in the first place.

3 **Data.** Research is all about data. In the medical sciences, this inevitably involves research participants being subjected to an increased number of physical tests, some of which can be quite invasive and/or take considerable time. This is certainly the case in the trial that K is being screened for. Appropriate screening procedures are critically important to make sure the right type of participants are being recruited into the trial. There is no point selecting a patient with an X characteristic if the experimental treatment is trying to positively affect a Y characteristic. This inevitably means that there will be a lot of testing and screening of people who do not make it into the trial. This may lead to the dashing of hope, but it is an essential element of good research design – recruiting the right target group.

4 **Consistency.** Making sure tests and assessments are being conducted in the same way and subject to the same analysis is really important. In our case, all of the blood samples for a particular test are to be flown to Singapore (from pathology collection centres all over the world) so they are all analysed in exactly the same way for this experiment. This seeks to reduce variations that may be introduced into the analysis using slightly different methods or machines. Precision is important.

5 **Monitoring.** It's great to have a neatly designed study but when we are dealing with human beings, things can, and do, go wrong. The research protocol for clinical trials involves a good deal of monitoring, as it should. Data-based decision-making is a key feature of the clinical trial. This is good for the experiment and good for the patient participant. Researchers can see what is happening almost in real time and this provides important feedback to not only the researchers but to the patient and their physician too. If things are not going well for the patient then a discontinuation can be effected quickly. Patients leaving trials is valuable data too.

6 **Collaborative partnership.** A successful research study requires a great deal of collaboration, communication and goodwill from all parties. Having a responsive contact person heading up the research implementation is just as important as having talented researchers conceptualising the research. Research studies can fall over where there is not sufficient attention to detail and clear communication.

Reflecting on the experience that we are currently having in the medical research world has led both me and K to comment on how similar the process is when conducting educational research in real-world contexts. It's resource intensive and requires all involved to keep their attention on what it is that they have to do. The six elements outlined above – transparency, informed consent, primacy of data, consistency, monitoring and collaboration – are also the hallmarks of effective educational research. Yes, it's hard. Yes, there are often problems. Is it worth it? Absolutely.

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After having tested literally thousands of children over the course of his research career, K is more than happy to be involved in research as a participant himself. He has benefited from the research participation of unknown others for many years. He is very pleased now to be 'doing his bit' in advancing the knowledge in the best approaches to treating disease.

Robyn Wheldall, Joint Editor

P.S. K qualified for the clinical trial BUT was randomly allocated to the control condition!