Trials are not for the benefit of patients

Peter Kapitein

Inspire2Live, Noorderakerweg 271b, 1069 LT Amsterdam, The Netherlands

Correspondence to: Dr. h.c. Peter Kapitein. Patient Advocate, Inspire2Live, Noorderakerweg 271b, 1069 LT Amsterdam, The Netherlands.
Email: peter.kapitein@gmail.com.

Submitted Dec 15, 2015. Accepted for publication Dec 17, 2015.
doi: 10.3978/j.issn.2305-5839.2015.12.50
View this article at: http://dx.doi.org/10.3978/j.issn.2305-5839.2015.12.50

Trials are not for the benefit of patients. They are set up to enable the pharmaceutical industry to register new drugs. And the only thing the industry needs is a drug that performs better than the one it is meant to replace, or a placebo.

Trials are not set up for the benefit of patients. They are for the benefit of research, to test whether a drug can repair a certain defect or not. Moreover, the outcomes must be scientifically significant. There must be an evident, yet limited, difference between the test group and a control group, which is mostly given a different drug.

If trials are not for the benefit of patients, why should you, as a patient, want to participate in them? Because you are terminally ill and have set your hopes on this new drug, which may be your last hope. But the criteria for inclusion are a nightmare: you are required to meet a long list of criteria to be eligible for participation, as the main objective of the trial is of course drug registration and scientific data collection.

Most patients are simply unable to meet all of these criteria—which contributes to the problem of disappointing efficacy results in practice, when the drug at last becomes available to a larger group of patients. The compassionate use programme may offer some consolation. However, the industry is not altogether happy about this programme, since it may reveal adverse effects, which could have a negative impact on the trial and the intended registration process. Nevertheless, new drugs get ample media attention and active patient advocacy groups such as Inspire2Live call on the industry to make these new drugs available on a wider scale. Take Olaparib, for example. It has been registered for BRCA-mutated ovarian cancer, and is also known to be effective against BRCA-mutated prostate, breast, pancreatic and other cancers. But it is not made available to patients. While this may be due to a variety of reasons, one of them is that trials are currently underway to prove that Olaparib is effective in BRCA-mutated breast cancer. We already know that it is which means that patients are dying unnecessarily as a result.

Science argues that making available drugs that have not completed the full trial process (which can take years) carries too many risks, as there may be unknown adverse effects in the long term. This is a very odd argument, as many patients would be over the moon to be able to experience effects in 5-year time: it would mean that they would still be alive by then. The perspective of the pharmaceutical industry is to prove that drugs are effective and safe. Patients do not play any role at all—it's only research that counts.

At the Inspire2Live Annual Conference, Bettina Ryll of Uppsala University explained why the trial process is so problematic. Sabine Tejpar from KU Leuven said at that time there were no trials for intestinal cancer that had any benefits for patients. And it is easy to see why: patients are never asked for their opinion on which trials should be set up, and neither are they involved in the trial design. Only when all decisions have been made do they get to see the patient information, and then only to check whether they understand what they are reading.

Why are patients never involved from the outset, on an equal basis, why are they never asked "Which trials would have the most added value for patients?" or "How can we set up a patient-friendly trial?" This could lead to drugs that are actually beneficial to patients, instead of just extending their life by a single month. They want to grow old, just like everyone else. And the benefits would be mutual, as patients would become the biggest ambassadors for these new and effective drugs. Registration would then become a formality,

© Annals of Translational Medicine. All rights reserved.

and science could benefit from closer cooperation with patients. There are no risks for science, but there are for patients. Their life is at stake, and they should be allowed to decide on their own treatment. They are perfectly capable of making such decisions.

But there's hope (there's always hope). Recently I came across the Dutch 'IClusion' initiative, which seeks to connect patients, doctors, medical centres and the pharmaceutical industry with the aim of allowing patients to participate in trials more quickly and easily. It's a start! The next step should be to involve patients in the set-up of such trials and, most importantly, in the decision as to which trials are most needed. Step by step towards creating a better world. It's time to get going!

Cite this article as: Kapitein P. Trials are not for the benefit of patients. Ann Transl Med 2016;4(1):22. doi: 10.3978/j.issn.2305-5839.2015.12.50

Acknowledgements

None.

Footnote

Conflicts of Interest: None, the author in the financial industry and his volunteer organization Inspire2Live gets no money from industry.

© Annals of Translational Medicine. All rights reserved.