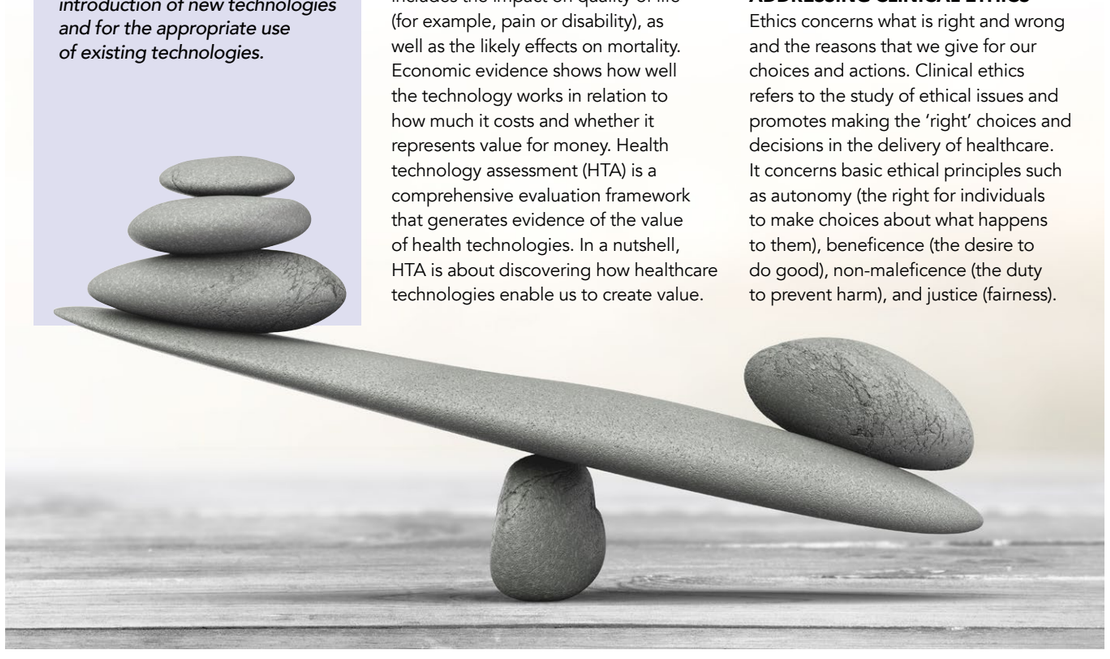
Values and evidence meet:

Appropriate healthcare assessment for vulnerable patients.

Healthcare technology assessment is about discovering how healthcare technologies enable us to create value. A key factor is clinical ethics, yet historically this has been overlooked. Researchers Gert Jan van der Wilt, Herbert Rolden, Janneke Grutters and Angela Maas at Radboud University Medical Centre explore the ethical and social implications of health care technologies. Their research aims to develop concepts and methods that enable a patient-centred, comprehensive approach to help inform decision-making, both in the introduction of new technologies and for the appropriate use of existing technologies.



In recent years, the number and variety of health technologies developed have increased at a rapid rate. These include new medications, diagnostic tests, devices, surgical methods, medical procedures and systems: all are developed to solve a health problem and improve our quality of life. We often take it for granted that these technologies are effective and that they show benefits to the patient, but to be adopted they also need to represent value for money.

The effectiveness and cost of health technologies are assessed by a rigorous process. Clinical evidence is obtained to show how well the technology works – the health benefits. The evidence includes the impact on quality of life (for example, pain or disability), as well as the likely effects on mortality. Economic evidence shows how well the technology works in relation to how much it costs and whether it represents value for money. Health technology assessment (HTA) is a comprehensive evaluation framework that generates evidence of the value of health technologies. In a nutshell, HTA is about discovering how healthcare technologies enable us to create value. It is a systematic, evidence-based mechanism that evaluates and prioritises new technologies from economic, social and ethical perspectives.

VALUE OF INFORMATION ANALYSIS

Economic evaluations are increasingly used to inform decisions in healthcare; however, decisions remain uncertain when they are not based on adequate evidence. Value of Information analysis is a systematic approach to measure decision uncertainty and assess whether there is sufficient evidence to support new health technologies. Essentially, it is a decision support tool for the allocation of resources to scientific research.

ADDRESSING CLINICAL ETHICS

Ethics concerns what is right and wrong and the reasons that we give for our choices and actions. Clinical ethics refers to the study of ethical issues and promotes making the 'right' choices and decisions in the delivery of healthcare. It concerns basic ethical principles such as autonomy (the right for individuals to make choices about what happens to them), beneficence (the desire to do good), non-maleficence (the duty to prevent harm), and justice (fairness).

ETHICAL DILEMMAS IN CLINICAL RESEARCH

Given the tremendous rate of development of health innovations, it's important to carefully consider the research questions that will be addressed: how, where, when and by whom? These questions pose a challenge when the patients participating in those studies are vulnerable.

Patients may be vulnerable for a variety of reasons. Firstly, because they are unable to fully appreciate the implications of their participation (or non-participation) in a clinical study (e.g., children and elderly people with compromised cognitive abilities). Secondly, patients may be vulnerable because they are more likely to sustain adverse outcomes, for instance, because of co-morbidities, such as diabetes or heart failure. Thirdly, their participation may be considered as exploiting some sort of disadvantage (e.g., they being poor or dependent). Conversely, despite the importance of these considerations, not providing vulnerable patients with the possibility to participate in clinical research results in the uncertainty of treatments being safe and beneficial for them. The participation of vulnerable patients in clinical research presents ethical dilemmas.

This is where the work of researchers Gert Jan van der Wilt, Janneke Grutters, Angela Maasand Herbert Rolden comes in. This dedicated team of researchers have worked together to investigate methods for exploring the ethical and social implications of health care technologies.

SPECIFYING NORMS

Henry Richardson of Georgetown University, Washington DC (1) established the method of specifying norms as a method of moral argumentation – as a way of resolving ethical dilemmas in a transparent and systematic way. Richardson's unique framework recognises that generic moral principles can be specified in multiple ways, also that it offers several rules for preserving the moral import of the original, unspecified principle. Giving the example of a severely malformed newborn child, whose parents wish that their child should be allowed to die, Richardson proposes three moral norms facing the healthcare provider:

These questions pose a particular challenge when the patients participating in those studies are vulnerable.

Norm 1. It is wrong to kill innocent people.
Norm 2. We should respect the reasonable choices of parents regarding their children (i.e. respecting autonomy).
Norm 3. We should act in the best interest of persons who have been entrusted to our care.

Richardson points out that there is always a gap between general moral norms (such as respecting autonomy) and judgments as to what follows from our commitment to such norm in concrete situations.

One way to bridge this gap is by making the norm more specific. For example, norm 1 can be conceived as a specification of a still more general norm, expressing respect for persons:

Norm 1: It is wrong to kill innocent people who have attained self-consciousness or who have the potential to develop self-consciousness over time.

As Prof van der Wilt explains: "According to this framework when we find ourselves in a dilemma, the key task is to develop alternative specifications of the various moral principles. In other words, we need to find out what follows from our commitment to a particular moral principle in a specific situation, taking into account that one or more other moral principles should be observed, too."

Building on Richardson's work, the team present a framework that makes explicit the moral principles that guide decisions in a concrete situation and that are the cause of the ethical dilemma in hand. Professor van der Wilt says: "These could include, for example, our desire to respect patients' autonomy, our desire to be able to act truthfully, our desire to treat all people fairly, and our desire to spend resources wisely. We then need to realise that such principles are quite abstract and generic and that in order to decide what follows from our commitment to these norms in concrete situations, we need to specify them."

In addition to assessing safety, clinical benefits and cost-effectiveness, it is important to consider the social and ethical factors to guide decision-making.

A NEW FRAMEWORK

The team at Radboud University neatly illustrate the application of the framework in a recent publication. The team used the case study of pre-menopausal women with atrial fibrillation, posing the question whether these women should be invited to participate in clinical studies of a new type of blood thinners, novel oral anticoagulants (NOAC). These women could be considered vulnerable since they are at increased risk of substantial bleedings that are difficult to control and that may have serious consequences. Due to their non-participation in key trials, there is uncertainty for this cohort whether the risks that are associated with these drugs are outweighed by the advantages, compared with conventional treatment. They addressed the question of whether research of this new class of drugs for these women would be appropriate, both from an ethical and economic perspective.

SHOULD PREMENOPAUSAL WOMEN WITH AF HAVE BEEN INCLUDED IN TRIALS OF ANTICOAGULANTS IN THE FIRST PLACE?

Using Richardson's method of specifying norms as a wider framework the team proposed how the apparent ethical dilemma may be resolved, considering patients' considerations and the need to spend resources for clinical research wisely. The team concluded that in fact inclusion of premenopausal women with AF in trials of NOACs would have been the ideal option.

Incorporating the question whether research of NOACs in pre-menopausal women with atrial fibrillation can be justified on economic grounds was determined using the value of information analysis. The research team concluded that further clinical research on NOACs in premenopausal women with atrial fibrillation is justified – both on ethical and economic grounds.

The team elegantly exemplify that addressing apparent ethical dilemmas by employing the use of a method such as specifying norms can improve the quality of public practical reasoning. Their work demonstrates that the method has substantial value to inform health policymakers by using solid scientific evidence on the medical, social, economic and ethical implications of investments in health care. In addition to assessing safety, clinical benefits and cost-effectiveness, it is important to consider the social and ethical factors to guide decision-making.

Behind the Research

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Research Objectives

A team of researchers at Radboud University Medical Centre explore the ethical and social implications of health care technologies. Their research aims to develop concepts and methods that enable a patient-centred, comprehensive approach to help inform decision-making, both in the introduction of new technologies and for the appropriate use of existing technologies.

Detail

Bio

Gert Jan van der Wilt (Researcher ID: H-8120-2014) is professor and head of Health Technology Assessment (HTA) at Radboud University Nijmegen Medical Centre.

Herbert Rolden is currently a policy advisor for The Council for Health and Society in The Netherlands.

Janneke Grutters works as associate professor and junior principle investigator at Radboudumc.

Angela Maas is a clinical cardiologist and is currently one of the most influential female doctors in Dutch healthcare.

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Personal Response

In your view, how much importance should the perspective of the patient have in the evaluation of healthcare technology?

"The patient perspective plays a key role. HTA is about collaboratively exploring how health technologies enable us to better realise particular social and ethical values. This requires carefully integrating empirical analysis and normative inquiry. All stakeholders, and notably patients, should be able to recognise and endorse the choices that are made in the context of an HTA and agree with the interpretation of its findings."

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