EXPERIMENTAL CARE

Experimental care provided by health care is patient care that has not been approved as part of the usual care practice, generally because its effects have not been sufficiently investigated. It can refer to the use of a medicine which is available on the market for a purpose or a patient category for which it has not been registered, but it can also be something else than medical care. In this position statement, experimental care means experimental care provided by health care and health care professionals.

Some of the current health care methods have their origin in experimental care. Among other things, organ transplants, which have become established as care practices, began as experimental activities. Since then also research evidence has been obtained to support them. Medicines and methods already in use have been tried in clinical cancer research for the treatment of cancer patients in categories that had not been part of the original research set-up. Based on the evidence from experimental care, many medicines for intensive care of newborns have been adopted as a part of treatment methods. Care and treatment started experimentally and already utilising medicines available on the market is also an important part of the attempt to find effective forms of care for rare diseases. Without experimental care, newborns and people suffering from rare diseases are in danger of being left without effective forms of treatment. Among others, the European Union has drawn up regulations to promote the care for these groups (see EU Paediatric Regulations and EU regulations for rare diseases).

The National Advisory Board on Social Welfare and Health Care Ethics ETENE regards it important to stimulate debate on the ethical questions of experimental care. This is why ETENE decided to take the issue under examination. Recently, the matter has been discussed in the press and in television. In addition to the position statement, ETENE also provides a report on the subject to serve as material for the continuation of the debate. The report consists of a compilation of material related to experimental care, and it summarises the viewpoints of the discussion at ETENE on the topic. The report was drawn up by B.Soc.Sc. Kristiina Felt, a trainee in national administration.

Legislation related to experimental care

In Finland, there are no special regulations concerning experimental care. If the experimental care is among the activities of health care, it is subject to general health care provisions, the most important of which are the Act on Health Care Professionals (559/1994) and the Act on the Status and Rights of Patients (785/1992) later referred to as the Patient Act. The Act on Health Care Professionals specifies the rights and responsibilities of the persons meant by the act. According to it, health care professionals in their professional activities must employ generally accepted and empirically justified methods in accordance with their training, and they should make continuous efforts to supplement their knowledge and skills. They must weigh the benefits of their professional activity to the patient and its possible hazards. According to the Patient Act, the patient has the right to either accept or refuse the care and treatment offered. The basis of either the consent or refusal should be the information the health care professional is obliged to give to the patient. The activities of health care

professionals are supervised by the National Supervisory Authority for Health and Welfare (Valvira).

Medical research on humans is regulated by the Medical Research Act (488/1999). According to that act, the research must be based on a research plan. A prior estimate must be drawn up of the risks and possible benefits of the intervention used in the research, and the person subjected to the research must be provided with all the information that has any bearing on the decision to participate. The experiment must not be started before the research subject has given a written consent about his/her participation, a consent that is based on the information.

Currently, introduction of new medicines is normally preceded by clinical trials for which Finnish Medicines Agency (Fimea) gives a permission and which it supervises. Ethics committees evaluate medical research projects beforehand. Prior evaluation of clinical trials is carried out by the National Committee on Medical Research Ethics TUKIJA, unless it has transferred the evaluation to a regional ethics committee. A research permit can be granted only for research for which an ethical committee or TUKIJA has given their favourable opinion.

Issues related to experimental care

Experimental care and medical research resemble each other to a great degree, especially if there is only a small amount of information about the effects of the care available to the patient or group of patients subjected to the research. However, they also differ from each other in that experimental care is generally not carried out in accordance with any research programme: the dosage, duration and follow-up methods are tailored on the basis of the patient's symptoms and condition. In recent years, so-called care evaluation groups have been set up in university hospitals, but otherwise experimental care is not necessarily evaluated beforehand. For persons participating as research subjects in a clinical trial, the outpatient clinic visits and the products studied are free of charge, whereas persons participating in experimental care pay themselves the clinic visits and medicines according to where the treatment takes place.

The use of medicines that are generally available on the market for conditions other than the illnesses for which the medicine was registered has become more widespread in recent years, especially in private medical care. The number of reports the National Supervisory Authority for Health and Welfare (Valvira) has received about these kinds of practices is greater than in the past. Inquiries have revealed that the patients have not always been aware of the experimental nature of the care and treatment and that they have not been monitored with diligent care and long enough to detect possible harm caused. Not all the patients had been carefully studied, and for this reason one cannot exclude the possibility that the symptoms were not due to the medical condition for which the treatment provided was intended to have its effect.

As experts in the matter heard by ETENE were Master of Laws Paula Vartiainen from the University of Helsinki, Chief Evaluation Physician Kimmo Mattila from the Helsinki and Uusimaa Hospital District, Professor Akseli Hemminki from the University of Helsinki, Research Professor Marjukka Mäkelä from the National Institute for Health and Welfare, Director Lauri Pelkonen from the Pharmaceuticals Pricing Board of the Ministry of Social Affairs and Health, and Chief Medical Officer Markus Henriksson from Valvira. In addition, the advisory board has familiarised itself with the reports and report drafts collected by Kristiina Felt.

Some considerations related to experimental care

ETENE draws attention to the fact that the boundary between experimental care and medical research is fickle and difficult to determine. Experimental care is regulated solely by general health care regulations, and it is supervised by the health care authorities. Medical research, on the other hand, is regulated in a fairly detailed manner. It is pre-evaluated, and many kinds of follow-up mechanisms for the effects of treatment and care are applied especially in clinical trials. Experimental care and medical research resemble each other in that the result of the care or experiment is uncertain and the risk that the care is harmful or ineffective is greater than in normal care. A written consent based on relevant information is required from the subject participating in medical research. The practice has been to ask for a similar kind of consent also if the treatment in the experimental care is given with a medicine that is still at the research stage. In cases where a doctor in a private practice meeting a patient proposes the use of a medicine, which is already on the market, as experimental treatment for something for which the medicine has not been registered, it hasn't so far been possible to always verify whether the patient has been aware of the experimental nature of the care and about the kind of information on which the patient's consent has been based.

According to ETENE, experimental care is part of the care development, especially for the needs of small groups of patients. However, it should be kept in mind that the knowledge obtained from experimental care is more uncertain than knowledge obtained from a research set-up. For this reason, attempts should be made to move from experimental care to a research design where the effect of a medicine could be measured and compared with a group getting different care.

If the knowledge about the benefits of the care and its harmful effects is insufficient, the health care professional looking after the patient has an ethical obligation to monitor the patient's condition more carefully than when providing care based on the normal care practice. This corresponds to the obligation of a professional conducting medical research to ensure that the best interests of the patient under study are realised for example by monitoring the patient's condition and taking care of the reporting of benefits and harmful effects.

ETENE's proposals

ETENE proposes that a notification procedure for experimental care should be introduced for use in Finland: the person or the body providing the care would notify a national authority about any experimental care that deviates from the care practice. However, no notification would be required for the treatment or care of a single patient.

The notification procedure would give the care-monitoring body a possibility to monitor the carrying out of the care and its effects. The notification procedure could also include an obligation for the provider to monitor the effects of the care if it continues over a long period and especially if the treatment is given for several persons. If there is no research-based information about the treatment, the supervising authority would have a possibility to propose to the care provider that a plan should be made about how the effects of the treatment could be better evaluated, to encourage the provider to draw up a research plan and carry out the actual research. The national authority monitoring experimental care could also keep an eye on that the results of the care would become public.

The proposed method could help in switching the focus from monitoring to early recognition of problems. That would expedite data collection about the effects of care and of effective care. It would also encourage the expansion of experimental frameworks into research and prevent the use of ineffective and harmful treatments in health care.

Patient's right for self-determination is realised through the information given to the patient and the consent based on that information. In all experimental care, ETENE recommends the use of a written information sheet and a specifically written or at least documented consent based on the information. Based on the consent documentation, it will also be possible to assess whether the information the patient has received about the experimental nature of the care has been sufficiently clear.

With the development of medicine, treatments are directed, more and more, for example in line with individual patients' genetic profile. Already there are examples of cancer treatments directed only to patients with a certain mutation in their genetic/hereditary factors. Development of new treatments for those suffering from rare diseases is based today, and probably will be based also in future, on experimental care. It is important to encourage professional health care staff to carry out care experiments, on the basis of which it may be possible to find effective treatments. Future reimbursement schemes for health care should be developed to a direction where they could ensure patients' right for effective care corresponding to their needs.

On behalf of the Advisory Board

Chairperson Jaana Hallamaa

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