Presidenza del Consiglio dei Ministri

COMITATO NAZIONALE
PER LA BIOETICA

Abstract

SINGLE PATIENT CARE AND NON-VALIDATED TREATMENTS (THE SO-CALLED "COMPASSIONATE USE")

27 February 2015

The opinion addresses the issue of the so-called “compassionate use” of drugs, meaning the use of individual or group treatments for patients affected by serious and often lethal pathologies, in case doctors consider that type of treatment to be without alternatives and to be able to bring benefits or improvements to the patient’s living conditions. What is being discussed is not an alternative to the well-known paths of pharmacological trial already approved and shared, but rather the possibility to envisage an exception, for particular and strictly defined situations, in order to offer all the possible resources to the patient’s hope for survival.

Such a situation engenders difficult problems with regard to the scientific assessment, the acceptance of responsibilities, the distribution of resources, and the evaluation of informed consent. In order to outline a more complete picture of this problematic case, the document addresses it from the points of view of the patient, the doctors, and the institutions.

The Committee wishes a different expression from “compassionate use” could be determined, in order not to mix it with legitimate feelings of empathy towards seriously-ill and incurable patients. The proposed alternate expression is “non-validated treatments for personal and single use”, hoping that an international “consensus conference” could promote its use.

Access to such treatments has to occur in exceptional situations, in situations lacking validated therapies, in serious cases of urgency and emergency for patients with life-threatening conditions, and it can never represent an alternative, whether explicit or surreptitious, to clinical trial. In any case, such treatments need to have a reasonable and sound scientific basis: data published on international peer-reviewed scientific journals, with strong scientific evidence, at least on animals and preferably resulting from phase I clinical trials. The prescription has to be issued by a panel of experts, appointed by public health institutions, in conditions of full transparency: absence of conflicts of interest, publication of both the products’ composition and the treatment’s results, exhaustive explanation to the patients on the potential dangers related to non-validated treatments, drugs’ costs charged to producers, and monitoring carried out by public health institutions.

Only under such conditions, “compassionate” treatments can be considered ethically licit and be included in the general right to health.

The Opinion is completed by a detailed juridical note.