



Ethical Challenges in Compassionate Use of Experimental Drugs

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DESCRIPTION

Compassionate use of experimental drugs represents one of the most delicate intersections between clinical research and bioethics, raising profound questions about justice, beneficence, and the rights of patients facing life-threatening illnesses. When conventional treatments fail, patients and their families often turn to unapproved therapies in the hope of survival or relief. While the desire to provide access to potentially lifesaving options is understandable, it is accompanied by ethical dilemmas related to safety, fairness, and the possible undermining of ongoing clinical trials. Striking the balance between compassion and caution is a constant challenge for researchers, regulators, and clinicians alike.

The origins of compassionate use programs stem from historical crises, particularly the HIV/AIDS epidemic in the 1980s, when patients demanded access to experimental antiretroviral before regulatory approval. This movement not only accelerated drug development but also highlighted the ethical principle of respecting patient autonomy in desperate situations. Since then, many countries have developed frameworks that allow patients with serious conditions to access investigational drugs outside clinical trials. However, such frameworks vary widely in scope, transparency, and oversight, creating inequities in who benefits from them.

One of the central ethical concerns is patient safety. Experimental drugs by definition lack comprehensive evidence of efficacy and risks. While compassionate use may offer hope, it also exposes vulnerable individuals to uncertain harms. In some cases, the use of untested drugs has resulted in severe adverse effects, leading critics to argue that patients are being used as de facto test subjects without the protections afforded in formal trials. Ensuring that patients are fully informed of the risks and uncertainties is thus paramount. Yet, informed consent in such contexts is difficult to guarantee, as patients facing imminent mortality may overestimate potential benefits and underestimate dangers due to desperation.

Another ethical challenge lies in fairness and justice. Access to compassionate use is often limited to patients who are well-connected, financially able, or treated at prestigious institutions where experimental drugs are available. This creates disparities between patients based not on medical need but on socioeconomic status and geography. In addition, pharmaceutical companies may prioritize compassionate use requests from countries with favorable market potential, further exacerbating inequities. From a bioethical standpoint, compassionate use should be guided by principles of fairness, ensuring that all patients with similar conditions have equal opportunities to access investigational therapies.

Compassionate use also risks undermining the integrity of clinical trials. Clinical research relies on rigorous protocols to generate reliable data on safety and efficacy. If large numbers of patients bypass trials to access experimental drugs, the ability to recruit participants for controlled studies may be compromised. This could slow down the approval process for treatments that could eventually benefit millions. Therefore, compassionate use programs must carefully balance the urgent needs of individuals with the broader societal goal of obtaining robust evidence through clinical trials. Some ethicists argue that compassionate use should be allowed only when a patient is ineligible for ongoing trials, ensuring that research progress is not jeopardized.

The role of pharmaceutical companies in compassionate use programs introduces additional ethical complexities. Companies may fear liability for adverse outcomes, lack incentives to provide scarce supplies of experimental drugs, or worry about negative publicity if outcomes are poor. At the same time, denying access can provoke moral criticism and erode trust. Ethical responsibility suggests that companies should adopt transparent policies on compassionate use, guided not by commercial considerations but by the principles of human dignity and beneficence. Regulators and policymakers can support this by establishing clear guidelines that balance patient needs with research imperatives.

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Emerging fields such as personalized medicine and gene therapy further complicate compassionate use decisions. These treatments are often highly individualized, costly, and resource-intensive, making widespread access impractical during development stages. Ethical debates center on whether limited doses should be allocated to a few desperate patients or reserved for controlled trials that might accelerate availability for larger populations. No easy answers exist, but a consistent ethical framework that prioritizes transparency, fairness, and informed consent is essential.

Public trust in the medical research system is at stake in how compassionate use programs are managed. Patients and families must believe that decisions are guided by compassion and fairness rather than profit or bureaucracy. Transparent communication about what compassionate use can and cannot offer, as well as open dialogue between patients, clinicians, researchers, and regulators, helps maintain this trust. Moreover, documenting and sharing data from compassionate use cases contributes valuable insights that can inform clinical development and benefit future patients.