Health disparities: Exploring the ethics of orphan drugs

Health disparities are inequalities in both health and access to health care in different populations based on race, gender, location, or socioeconomic status. Patients in need of orphan drugs are afflicted by an inequality that is typically overlooked. This population faces rare diseases with enormous costs for treatment. Approximately 80% of orphan diseases have a genetic basis and are chronic, life-altering conditions that place huge burdens on those involved.

An orphan disease is a rare disease that affects fewer than 200,000 people in the United States. Efforts by the National Organization for Rare Disorders and the Office of Orphan Products Development, in addition to the Orphan Drug and Rare Disease Acts of 1983 and 2002, provided a turning point for the treatment of patients with orphan diseases. Since 1983, about 335 new and successful FDA-approved orphan drugs have been developed, compared with just 10 in the years before.

Orphan drugs carry an enormous financial burden, some costing more than $200,000 per year per patient. Granting orphan drug status also drives up the cost for existing therapies, as seen with the sixfold increase in the cost of sildenafil citrate when used for the treatment of pulmonary hypertension (an orphan indication) versus erectile dysfunction. With such high costs, ethical issues on how to budget have been raised.

The moral dilemma surrounding orphan diseases is the substantial amount of resources required to treat these rare conditions. There is a strong belief that society has a duty not to abandon individuals in need. Furthermore, the medical community has an obligation to pursue new therapies.

In health care, the most common interpretation of justice is providing basic health care for everyone because it is the right and fair thing to do. This is the concern of distributive justice, or the fair way to allocate resources in society. Distributive justice is very useful in public policy but does not cover the issues of orphan diseases and their treatments; these are issues of social justice. The goal of social justice is to treat each individual with the dignity and respect he or she inherently deserves as a human being. Accordingly, legislators have acted to offer treatment for the incidental inequality that is orphan diseases.

Gerick et al. expand on the principle of social justice, dividing it into two theories: utilitarianism and rights-based justice. Utilitarianism seeks to maximize the principle of utility—the greatest good for the greatest number. Maximizing health care for the greatest number, or distributing resources based on the greatest need, leaves out those afflicted by orphan diseases. Rights-based justice argues that a minimum level of health care is required for everyone.

Another pertinent ethical principle is beneficence, or the notion of actively doing good. It relates primarily to orphan drugs through the concepts of nonabandonment and scientific advancement. Nonabandonment is the notion that society recognizes the importance of improving the health of patients with rare diseases that are severe and lack alternative treatments.

A greater importance has been placed on health care because it is required, at some minimum level, for any person to function normally. With diminished health, these members of society no longer have an equal opportunity. This is why a moral compulsion exists to further orphan drug understanding, research, and development; without it, a subset of the population’s normal functioning and opportunities is restricted.

As a society, the value placed on orphan drugs and the incentives for com-
panies to develop them are vital due to their high risk and cost. Our society has been shown to be socially just throughout history, recently with advocates and acts passed to aid in the ethical dilemmas confronting orphan drugs. We need to continue to move forward with these guiding principles and find ways to better develop, diagnose, and treat orphan diseases to eliminate this health disparity.


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