We thank Professor Davis for taking an interest in our recent article highlighting why orphan drugs merit provision even at very high prices.1

The guiding principle for physicians is to ensure that the well-being of patients is the primary concern.1,2 This does not mean to thoughtlessly prolong life if this is not in the patient’s best interest. However, monetary cost should not determine who merits treatment: those most afflicted are often the least able to defend their claim to treatment.1,3

Judging the quality of life of others is notoriously difficult:2 premature decisions may be badly in error and should be avoided—not least, lest our own lives be found wanting and we be consigned to a category to reduce planetary overcrowding. While there may be an ever-expanding global population, this may be best dealt with before conception since it is difficult to justify that children born with inherited diseases should be singled out. This latter approach has involved the medical profession before, most notably in Nazi Germany and Austria, with infamous consequences.4 An individual’s fundamental rights must not be sacrificed for what is perceived to be in society’s or the planet’s interest at large.1,2

Since the first introduction of the so-called orphan drug legislation nearly 35 years ago, there have been striking therapeutic advances; these have been accompanied by a step-change in the efficacy of molecular therapies for rare and ultra-rare genetic diseases. Our arguments have been developed in this context: in practice, timely introduction of an innovative agent can greatly improve the well-being of patients; indeed complete rescue from the irreversible effects of a progressive disease is not unknown.5

We contend that as more orphan drugs become available, there are many practical matters to resolve including negotiations about cost. However, ultimately the provision of expensive therapies is an ethical and societal matter for wider discussion, and case-by-case decision-making on the basis of sound ethical principles, rather than intertempore exclusion as a result of judgements made by physicians working alone.

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References