Justifying insurance coverage for orphan drugs

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How can insurers justify spending hundreds of thousands of dollars per patient per year on "orphan drugs" – extremely expensive medications for rare conditions that are mostly chronic and life-threatening -- when this money could provide greater overall health benefit if spread out among many other patients? Those spending decisions reflect the "rule of rescue," the value that our society places on saving lives in immediate danger at any expense. But the broad application of the rule of rescue will be increasingly difficult to support as "personalized medicine" produces more drugs genetically targeted to relatively small groups of patients, concludes an article in the *Hastings Center Report*. For example, rather than a new blockbuster drug that treats millions with hypertension, new targeted therapies will treat only those few thousand with a particular genetic makeup.

"Orphan drug coverage decisions highlight the tension that can arise in health care between doing the most good possible with scarce health care resources and the desire to assist identifiable individuals regardless of cost," write the authors, Emily A. Largent, a candidate in the Ph.D. Program in Health Policy at Harvard University, and Steven D. Pearson, a visiting scientist in the National Institutes of Health Department of Bioethics and president of the Institute for Clinical and Economic Review at Massachusetts General Hospital.

The number of orphan drugs has dramatically increased since the passage of the Orphan Drug Act of 1983, which, in response to patient advocacy and public pressure, has provided an incentive for orphan drug

development. In the decade before the act was passed, only 10 new drugs for rare diseases were developed. Twenty-five years later, orphan drugs represented roughly one-third of all newly approved drugs and biologics, the article reports.

The authors propose an ethical framework to guide coverage and reimbursement decisions for expensive orphan drugs based on a critical analysis of the arguments embedded in the rule of rescue.

The first argument is that we have a greater moral impulse to help patients when we can see them as individuals rather than as anonymous members of a group. Advocacy organizations for rare illnesses sometimes publicize patients with photo campaigns and other means. But the authors conclude that "no persuasive rationale exists for using identifiability in resource allocation at the policy level, and, indeed, strong ethical arguments can be made against it." Appeals to identifiability can be unethical, the authors state, because they give an unfair advantage to patients whose condition produces visible signs of illness.

The second argument is that we should give priority to saving people whose lives are endangered. But the authors conclude that this argument cannot justify coverage for orphan drugs without also considering the outcomes: in general, lifesaving orphan therapies and therapies that restore or maintain capacities central to functioning in society should be covered and those that do not achieve these health outcomes should not be covered.

The third argument concerns opportunity costs. Advocates have long presumed that the opportunity costs of expensive orphan drugs are low because the small number of people using them represents a small overall expense to an insurer. But, the authors state, this may no longer be accurate as more orphan drugs enter the research pipeline, a trend

that personalized medicine will exacerbate. They call for opportunity costs to be explicitly, and transparently, included in any coverage decision.

"Tomorrow's medical care will feature a growing number of expensive therapies that offer benefit only to small populations," the authors conclude. They add that their "conceptual framework offers an advance over current decision-making practices" about coverage for orphan drugs. Instead of identifiability being a factor in decision-making, "potential health gains must be evaluated in context to determine whether they provide a meaningful benefit beyond what is already available, and the opportunity costs must be weighed to determine whether they are acceptable."

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