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Does Incorporating Cost-Effectiveness Analysis Into Prescribing Decisions Promote Drug Access Equity?

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Abstract

Using cost-effectiveness analysis (CEA) to inform prescribing can promote equitable drug access from a utilitarian perspective. Some theorists of equity, such as Rawls or Powers and Faden, however, would not consider CEA as promoting equity, as they endorse nonutilitarian theories of equity. Novel advances in CEA methodology seek to integrate broader equity concerns but may raise transparency concerns. We argue that incorporating CEA into qualitative multi-criteria decision analysis to inform prescribing decisions could promote equity more effectively and transparently than using CEA alone. Such applications should be implemented, along with recommendations, at the health system level rather than be carried out by individual clinicians alone.

Cost-Effectiveness and Health Equity

Rising prescription drug costs contribute to drug inaccessibility.[1] When clinicians prescribe medications that are not cost effective, insurers subsidize these medications to the detriment of making cost-effective medications more affordable and therefore more accessible. Traditional cost-effectiveness analysis (CEA) promotes economic efficiency by prioritizing health care interventions that maximize health gains across a population within a given budget. Specifically, when a physician selects among several medications to treat a certain condition, using CEA might favor medication that is both more effective and less expensive than the alternatives or medication that delivers the greatest health gain per dollar spent. Incorporating CEA into prescribing guidelines and decisions has potential to shift insurance subsidies toward more efficient drugs, thus increasing their accessibility to patients.

Yet some ethicists, policymakers, and clinicians worry that the use of CEA fails to promote health equity.[2,3,4] Equity refers to the fair distribution of morally relevant goods among groups; that is, under a fair distribution, differences among groups are ethically permissible only if the differences are justifiable as not unfair.[5] Health is one morally relevant good. In the context of CEA, we take health equity to refer to fair distribution of health outcomes or gains. We argue that prescribing guidelines can and ought to incorporate CEA as part of a larger endeavor to promote both health equity and equity

more broadly construed. However, CEA alone, as traditionally applied, is not sufficient to promote equity.

Cost-Effectiveness Analysis and Theories of Equity

Utilitarianism. Traditional CEA is based on a *utilitarian* theory of equity or fair distribution. When applied to health gains, this theory has 2 key parts. First, traditional CEA is designed to inform identification of health services that produce the greatest health gains per dollar spent.[6] CEA is thus based on a *consequentialist maximization* theory of fair distribution, or the view that we ought to maximize good outcomes. Second, many applications of CEA are cost-utility analyses (CUA) that characterize health gains for a target patient population in terms of health-related utility typically measured in quality-adjusted life years (QALYs). (One QALY is equivalent to one year of life lived in perfect health, capturing both morbidity and mortality.) CEA as CUA rests on a type of *welfarist* view in which well-being should morally be the focus of distribution; a welfare maximization approach is known as utilitarianism.[7] On utilitarian theory, a fair distribution is straightforwardly one in which welfare is maximized; the relative distribution of welfare within a population is unimportant. Thus, for a utilitarian, the application of CEA is equity promoting with respect to health gains.

Other theories of equity. Aside from utilitarianism, other theories of equity suggest that incorporating CEA alone would not be equity promoting with respect to health gains. John Rawls rejected utilitarianism and proposed what's known as a "maximin" principle, whereby inequalities in wealth and income are fair as long as those who are the least well off on this distribution are better off than they would be on any other possible distribution.[8] This approach to equity is *prioritarian* and differs from utilitarianism because a distribution whereby overall good is maximized would likely be inconsistent with the maximin principle. Additionally, for Rawls, distribution of *primary goods*—income and wealth as well as certain rights and respect—as opposed to welfare is what matters morally.[8] Later, Norman Daniels extended Rawls' account to include the fair distribution of health and health care.[9,10] Alternatively, Madison Powers and Ruth Faden advocate a theory that can be roughly described as *sufficientarian* about *capabilities*,[7] which builds on the work of Amartya Sen.[11,12] On this view, all people should enjoy a sufficient level of some central capabilities, such as health, self-determination, and the ability to form important social relationships. Unlike Rawls' approach, on this view, the least well off are not strictly prioritized; rather, all should enjoy a minimally acceptable level of well-being in terms of these capabilities.

In light of these varying justice theories, an important question is whether CEA methodology can be adapted to further the goal of equity, both with respect to health gains and more generally. Cookson et al summarize some novel approaches for including equity considerations in CEA analyses, such as distributional CEA (DCEA) and extended CEA (ECEA).[4] DCEA can compare the distribution of health effects and health opportunity

costs of different interventions by subgroup. DCEA has been used to understand how targeted versus universal health reminders for improving cancer screening uptake affect the distribution of health gains analyzed by sex, ethnicity, and social deprivation.[13] ECEA can assess the distribution of both health effects and protection against illness-related impoverishment. For example, ECEA has been used to compare the health gains and financial risk protection by income group of a potential cigarette excise tax in China.[14] Both DCEA and ECEA enable decision makers to apply nonmaximization theories of equity, like prioritarianism, by permitting comparison of costs and benefits to a whole population with costs and benefits to subgroups of special concern. Furthermore, ECEA adopts in part a Rawlsian primary goods approach to equity by measuring costs and benefits in terms of income or wealth and not simply health. There have also been efforts to develop nonwelfarist measures of effectiveness for use in CEA. For instance, a capability measure known as ICECAP assesses the impact of health care on capabilities such as autonomy and attachment rather than simply on QALYs.[15]

While these approaches suggest it is possible for CEA to promote equity given value pluralism about what constitutes a fair distribution, they raise an additional ethical concern. A primary worry is that methods like these, as Faden and Sirine Shebaya note, "obscure controversial moral considerations from public view and deliberation" and are thus antidemocratic approaches that could harm institutional legitimacy.[16] Because CEA is a complex methodology that requires expertise to understand and apply, addressing equity concerns in CEA—and doing so in a highly technical manner—could mean that many people are unable to identify and challenge the values informing CEA analyses with which they disagree.

To be sure, this objection can be levelled at traditional CEA itself, an approach based on several value assumptions with implications for equity. For example, health gains are typically considered equally valuable regardless of age or illness severity; different discounting rates for long-term costs or effectiveness assign different value to current versus future lives and assign different value to prevention versus treatment; and there is in-built impartiality regarding whether and when large benefits to a small population should outweigh small benefits to a large population, an issue catapulted into public consciousness when Oregon proposed covering tooth capping but not appendectomies under Medicaid.[17]

How to modify CEA models to align with different views on equity is a complex matter about which reasonable people will likely disagree. Transparency about values at play in CEA—achieved by publishing and disseminating either outcomes of decision-making processes that use CEA or the full rationale behind those decisions—in a way that is both accessible and comprehensible to members of the public[18] is necessary for informed and accessible debate about which values should inform our health care practices and policies.

Alternative Approaches

Given the potential for a lack of transparency about the values at play in CEA, another way to promote equity would be to retain traditional CEA for its value in promoting efficiency—and equity from a utilitarian perspective—but consider it *alongside* analyses that capture other equity theories' core values. In multicriteria decision analysis (MCDA), for example, decision makers evaluate a set of potential interventions across several criteria to determine which interventions should be prioritized. That is, rather than building additional considerations into a single analysis as in DCEA and ECEA, MCDA enables cost effectiveness to be weighed alongside equity-relevant considerations intended to target certain subpopulations defined, for example, by disease severity, age, or socioeconomic status.[19] Importantly, *qualitative* MCDA eschews the mathematical aggregation of scores across multiple criteria and instead relies on decision makers' deliberation about the relative value of these criteria in order to prioritize subgroups or interventions.[20] In this way, qualitative MCDA can better promote transparency than approaches that quantify equity considerations and integrate them into a single analysis, as in traditional CEA, DCEA, or ECEA.

Justice-enhanced CEA is another approach being developed to assess equity within the context of drug-resistant tuberculosis and other infectious diseases. This method, influenced by the work of Powers and Faden,[11] aims to assess health care interventions' impact on core aspects of social justice, such as agency, association with others, and self-respect or social respect. These social justice impacts can then be considered alongside outputs of traditional CEA in order to improve equity.[21,22] For instance, novel medications for drug-resistant tuberculosis allow treatment regimens to be shorter, thus reducing the time during which patients endure social stigma due to this specific illness. Although these novel drugs might be less cost-effective than existing regimens, they might better protect patients from social exclusion. In theory, the influence of different health care interventions on agency, association, and self-respect could also be considered in qualitative MCDA approaches to equity.

Prescribing Policies

Unlike some other developed countries, the United States does not have organizations that provide guidelines for coverage and prescribing based on CEA. The Patient-Centered Outcomes Research Institute (PCORI), for example, created by a clause in the Patient Protection and Affordable Care Act, is not allowed to use CEA to inform recommendations.[23] Considering this limitation at the federal level, our recommendation is instead for health systems—hospitals, physician groups, or health centers—to issue prescribing guidelines informed by traditional CEA *and* qualitative MCDA that includes explicit and diverse equity considerations like those discussed above. For example, health care organizations' boards or panels of clinicians and ethicists could deliberate regularly using MCDA to (1) assess interventions' cost-effectiveness and impact on

various dimensions of equity and (2) issue recommendations to clinicians about new interventions or those already in use.

We believe that this approach is superior to a system in which individual clinicians alone incorporate CEA in their prescribing decisions. Involving clinicians directly in cost containment measures has been criticized,[24,25] and, in general, bedside rationing raises a number of complex ethical issues[26,27] and may be too burdensome for individual clinicians to implement alone. As we have argued, a qualitative MCDA approach can better promote transparency about the reasons for a decision. Decision-making processes that incorporate MCDA should also include other elements of a fair process, such as opportunities for clinicians and patients to appeal decisions,[28,29] given that reasonable people are likely to disagree agree about what promoting equity demands. Whether CEA promotes equity depends on the theories of equity one supports and on the values incorporated in different CEA models. Traditional CEA can help expand access to cost-effective interventions, and, when used alongside explicit equity considerations in a deliberative manner, can help more appropriately balance efficiency and equity impacts.

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