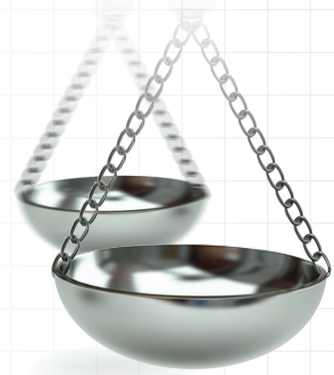
White Paper

For reprint orders, please contact: reprints@futuremedicine.com



Journal of **Comparative Effectiveness Research**

Cornerstones of 'fair' drug coverage: appropriate cost sharing and utilization management policies for pharmaceuticals

Steven D Pearson*[,1], Adrian Towse[2], Maria Lowe[1], Celia S Segel[1] & Chris Henshall[2]

[1]Institute for Clinical & Economic Review, Boston, MA 02109, USA

[2]Office of Health Economics, London, UK

*Author for correspondence: spearson@icer.org

At the heart of all health insurance programs lies ethical tension between maximizing the freedom of patients and clinicians to tailor care for the individual and the need to make healthcare affordable. Nowhere is this tension more fiercely debated than in benefit design and coverage policy for pharmaceuticals. This paper focuses on three areas over which there is the most controversy about how to judge whether drug coverage is appropriate: cost-sharing provisions, clinical eligibility criteria, and economic-step therapy and required switching. In each of these domains we present 'ethical goals for access' followed by a series of 'fair design criteria' that can be used by stakeholders to drive more transparent and accountable drug coverage.

Tweetable abstract: New ICER policy analysis sets design criteria to determine whether insurance coverage is providing fair access to drugs through cost sharing, clinical eligibility criteria and step therapy.

First draft submitted: 4 February 2021; Accepted for publication: 12 February 2021; Published online: 1 March 2021

Keywords: cost sharing • drug coverage • pharmaceuticals • prior authorization • step therapy

At the heart of all health insurance programs, public or private, lies inescapable ethical tension between two desired goals: first, that insurance maximizes personal choice so that patients and clinicians can use healthcare services as they see best to save lives and to improve the quality of life of those who are ill; and second, the need to make healthcare affordable, and to manage resources fairly within the budget constraints that exist throughout the entire health system.

Nowhere is this tension more fiercely debated today than in the area of health benefit design and coverage policy for pharmaceuticals [1]. Although patient groups, drug makers and payers can now talk (or argue) more concretely about determining what would be the 'fair price for a drug', a question far less examined is how to determine whether insurance coverage is providing 'fair access to a drug' [2]. Plan sponsors and payers need a conceptual framework of ethical design criteria to guide their decision making. Patients, clinicians and all other stakeholders need to contribute to this framework and should then be able to use it to help make consistent judgments of when specific cost-sharing provisions or utilization management protocols are fair and transparent. To present such a conceptual framework and associated design criteria is the purpose of this paper.

To inform the development of our framework we conducted a literature review and ten stakeholder interviews with representatives from patient community organizations, employer plan sponsors, pharmacy benefit managers (PBMs), commercial health plans, health policy research organizations, specialty medical organizations and health benefit consultants. Representatives from patient organizations and clinical specialty societies joined senior policy leaders from 25 payer and life science companies at a 2-day policy summit in December 2019 to discuss an earlier version of this paper, debate the concept of fair access and provide suggestions for revisions to the framework proposed.

Future Medicine

Scope of analysis

We provide elsewhere a more comprehensive examination of elements related to fair access to pharmaceuticals in a full White Paper. Due to length restrictions, in this paper we will focus narrowly on the three areas over which plan sponsors and payers (defined here as inclusive of both PBMs and insurers) have direct control and over which there is the most controversy about how to judge whether drug coverage is appropriate: cost-sharing provisions, clinical eligibility criteria, and economic-step therapy and required switching.

In each of these domains we present 'ethical goals for access' followed by a series of 'fair design criteria'. These criteria provide the set of standards by which we believe the goals of ethical cost sharing and utilization management can be realized, and with which we suggest that all participants in the health system can find a more transparent and concrete method to judge the appropriateness of benefit designs and coverage policies. These are summarized in Table 1. The context and rationale for these ethical goals and design criteria are set out in the remainder of this paper.

In combination with these ethical goals and design criteria there are essential 'implementation criteria' that payers should follow. These criteria focus on elements of transparency, flexibility and appeals procedures without which even the best designs for cost-sharing provisions and prior authorization protocols cannot achieve reasonable ethical outcomes. These implementation criteria are also presented in the Table 1, but due to length constraints are not discussed further in this paper. They are described in full detail in the White Paper [3].

Cost sharing

Ethical goals for access

One common rationale for requiring cost sharing by patients is to reduce overuse of healthcare services that may be driven by the 'moral hazard' of the lack of direct financial consequence when patients are covered by insurance [4]. Recent evidence confirms that patients can be very sensitive to differences in out-of-pocket costs, and there is a strong consensus among plan sponsors and insurers that cost sharing is necessary to help control overall insurance premiums [5]. According to this view, the ethical benefit of cost sharing is that, if exercised responsibly and properly structured, it does not harm patients and can reduce spending on drugs for which there are less expensive alternatives, improving the affordability of health insurance for all.

However, it is clear that cost sharing can prove to be a blunt tool and, especially for lower-income patients, often reduces use of necessary care as well as unnecessary care, increasing the risk of adverse clinical consequences and higher overall costs [6–9]. The broader aim of cost sharing, therefore, should be to design the drug benefit in a way that addresses moral hazard and can steer patients to clinically appropriate lower-cost alternatives without undermining the basic purpose of insurance, which is to spread financial risk over broad populations to protect individuals from direct harm.

Translating ethical goals for access into fair design criteria

If the stated ethical goals of cost sharing are to address moral hazard, reduce overall spending on drugs and improve affordability of health insurance, then two fair cost-sharing provisions are clear:

- There should be no cost sharing for cost saving, high-value medications for chronic conditions. This has been the focus of extensive policy development and advocacy as part of the drive for value-based insurance designs [10,11].
- Cost sharing for the patient should be based on the net price to the payer, not on the list price, which is irrelevant to efforts to control costs for the population.

Beyond these two criteria, we must take into account important contextual considerations. For example, if a lower-cost alternative is not available, the drug should ideally be placed on the lowest tier in the formulary, subject to the lowest level of cost sharing. It is, however, important to consider whether the drug is fairly priced. We believe consideration of a fair price is important because placing any drug that does not have competition on the lowest tier will disincentivize those drug makers from negotiating lower prices, which can misalign incentives and increase costs for all patients in a way that creates affordability barriers to accessing care. ICER advocates for an approach centered on a cost–effectiveness evaluation of whether the price is in proportion to the long-term added benefits of a drug. Our approach also explicitly considers broader contextual factors and the potential budget impact given the intended patient population. But whether a payer adopts the ICER approach or another one is less important than that the approach be transparent, explicit and applied consistently.

Table 1. Ethical goals for access and fair design and implementation criteria for drug coverage.

Element of access	Ethical goals for access	Fair design criteria	Implementation criteria
Cost sharing	• The purpose of differential cost sharing for drugs should be to provide positive incentives for patients and clinicians to select higher-value treatment options that are clinically appropriate • Cost sharing should not be structured primarily to shift healthcare costs to patients when they have few or no lower-cost options that are medically appropriate • The level of cost sharing should not serve as a major barrier to patients being able to afford needed treatment	• Patient cost sharing should be based on the net price to the plan sponsor, not the unnegotiated list price • All medications identified by the Internal Revenue Service as high-value therapies should receive predeductible coverage within high-deductible health plans • At least one drug in every class should be covered at the lowest relevant cost-sharing level unless all drugs are priced higher than an established fair value threshold • If all drugs in a class are priced so that there is not a single drug that represents a fair value as determined through value assessment, it is reasonable for payers to have all drugs on a higher cost-sharing level • If all drugs in a drug class are priced so that they represent a fair value, it remains reasonable for payers to use preferential formulary placement with tiered cost sharing to help achieve lower overall costs • As part of economic-step therapy, when patients try a lower-cost option with a lower cost-sharing level but do not have an adequate clinical response, cost sharing for further therapies should also be at the lower cost-sharing level as long as those further therapies are priced fairly according to transparent criteria	• Transparency to consumers prior to health plan selection: cost-sharing policies should be presented clearly to consumers prior to health plan selection, allowing all individuals to understand what cost sharing they will face for treatments they are currently taking or are considering. Any significant change to formulary or cost-sharing structures should not occur mid-cycle unless plan sponsors include this as a qualifying event allowing plan enrollees to switch plans • Transparency to clinicians and patients during care: at the point of care, clinicians and patients should be able to rapidly determine the cost-sharing requirements for any treatment along with cost sharing for other alternatives
Prior authorization protocols: clinical eligibility criteria	• Given that prior authorization has some inherent risk of causing patient harm through limiting rapid access to desired care options, and that it always imposes an administrative burden on clinicians, patients, and payers themselves, it should only be used in one of two circumstances: when necessary to protect patients from inappropriate use of treatments, or when necessary to manage costs of expensive interventions and to negotiate lower prices for drugs that are priced beyond a fair price range • The administrative burden of documenting clinical eligibility should be streamlined and transparent to avoid creating a significant barrier to appropriate care • Clinical eligibility criteria should not extend beyond reasonable use of clinical trial inclusion/exclusion criteria to interpret or narrow the US FDA label language in a way that disadvantages patients with underlying disabilities unrelated to the condition being treated • Arbitrary eligibility cut offs used in pivotal clinical trials for age, severity or other clinical characteristics should be subject to reasonable flexibility in coverage determinations • Development and application of clinical eligibility criteria must address the role of racism and bias in clinical trial evidence and must ensure that insurance criteria recognize distinctive benefits and harms of treatment that may arise for biological, cultural or social reasons across different communities	• Payers should offer alternatives to prior authorization protocols such as programs that give feedback on prescribing patterns to clinicians or exempt them from prior authorization requirements ('gold carding') if they demonstrate high fidelity to evidence-based prescribing • Payers should document at least once annually that clinical eligibility criteria are based on high quality, up-to-date evidence, with input from clinicians with experience in the same or similar clinical specialty • Clinical eligibility criteria should be developed with explicit mechanisms that require payer staff to document that they have: a) Considered limitations of evidence due to systemic under-representation of minority populations; b) Sought input from clinical experts on whether there are distinctive benefits and harms of treatment that may arise for biological, cultural or social reasons across different communities; and c) Confirmed that clinical eligibility criteria have not gone beyond reasonable use of clinical trial inclusion/exclusion criteria to interpret or narrow the FDA label language in a way that disadvantages patients with underlying disabilities unrelated to the condition being treated For all drugs: clinical eligibility criteria that complement the FDA label language may be used to: • Set standards for diagnosis; and/or • Define indeterminate clinical terms in the FDA label (e.g., 'moderate-to-severe') with explicit reference to clinical guidelines or other standards; and/or • Triage patients by clinical acuity when the payer explicitly documents that triage is both reasonable and necessary because: a) The size of the population included within the FDA label is extremely large, and there is a reasonable likelihood that many patients would seek treatment in the short term; b) The clinical infrastructure is not adequate to treat all patients seeking care and/or broad coverage would create such substantial increases in short-term insurance premiums or other financial strain that patients would be harmed through loss of affordable insurance; c) Acuity can be determined on objective clinical grounds and waiting for treatment will not cause significant irremediable harm • For drugs with prices or price increases that have not been formally deemed unreasonable: except for the three purposes outlined above, clinical eligibility criteria should not deviate from the FDA label language in a manner than would narrow coverage • For drugs with prices or price increases that have not been formally deemed unreasonable: documentation that patients meet clinical eligibility criteria should represent a light administrative burden, including acceptance of clinician attestation in lieu of more formal medical record documentation unless documentation is critical to ensure patient safety • For drugs with prices or price increases that have been formally deemed unreasonable: clinical eligibility criteria may narrow coverage by applying specific eligibility criteria from the pivotal trials used to generate evidence for FDA approval if implemented with reasonable flexibility and supported by robust appeals procedures as described in the implementation criteria • For drugs with prices or price increases that have been formally deemed unreasonable: documentation requirements to demonstrate that patients meet clinical eligibility criteria may represent a modest administrative burden, including requirements for medical record confirmation of key criteria instead of simple clinician attestation. In all cases, however, administrative burden should not result in major barriers to care for patients who meet criteria, and payers should perform and post publicly annual evaluations for each drug of rates of ultimate coverage approval following initial coverage denial due to documentation failures	• Transparency of policies to consumers prior to health plan selection: individuals considering health plan enrollment should be presented with clear information allowing them to understand whether they meet the insurers' clinical criteria for the treatments they are currently taking. The policies should also set out the rationale behind them and be readily understandable • Transparency of policies to clinicians and patients during care: clinicians and patients should be able to rapidly determine the clinical criteria for any treatment and view the clinical rationale supporting these criteria. The referenced clinical information should be readily available to the prescribing/ordering provider and the public • Reasonable flexibility: when coverage is requested for patients who come close to meeting arbitrary cut offs for age, severity or other clinical characteristics used for clinical trial eligibility and coverage criteria, adjudication should show flexibility, especially if the requested treatment is for a serious condition for which there are no other treatment options. In any denial of coverage on the basis of patients failing to meet clinical criteria, payers should document that the case has undergone a review process to determine whether patients are close enough to meeting clinical criteria that coverage should be granted because there is no explicit rationale for why they would be less likely to benefit from treatment Implementation of clinical criteria for patients entering a new benefit plan should offer a minimum 60-day grace period for any prior authorization protocols for patients who are already stabilized on a particular treatment upon enrollment in the plan. During this period, any drug regimen should not be interrupted while the clinical criteria requirements are addressed • Updating with new evidence: evidence should be reviewed annually at a minimum to ensure that clinical eligibility criteria are consistent with the latest evidence on drugs' relative safety, clinical effectiveness and cost–effectiveness • Evaluation of impact: insurers should evaluate annually the impact of clinical eligibility criteria to determine rates of exceptions, rates of coverage denials overturned on appeal and feedback from clinical experts and patient groups on the impact of restrictions on care and on patient outcomes

10.2217/cer-2021-0027

Table 1. Ethical goals for access and fair design and implementation criteria for drug coverage (cont.).

Element of access	Ethical goals for access	Fair design criteria	Implementation criteria
Prior authorization protocols: economic-step therapy and required switching	• Given that economic-step therapy policies add to the complexity and burden of healthcare and have the potential to limit the ability to tailor care to the needs of individual patients, these policies should only be used when all ethical design and implementation criteria are met • Economic-step therapy policies should ensure that patients are able to receive first-line therapy that is clinically appropriate for them and that will reduce the overall costs of care, not just costs for drug spending • Policies seeking to reduce costs by requiring that patients switch from a well-tolerated therapy to a less costly option offer no medical benefits to patients and frequently may not even result in lower cost sharing for the affected patients Thus, required switching policies can only be justified in very limited circumstances when the risks of harms from inadequate response or new side effects with the lower cost agent are minimal	• In order to justify economic-step therapy policies as appropriate, payers should explicitly affirm or present evidence to document all of the following: a) Use of the first-step therapy reduces overall healthcare spending, not just drug spending b) The first-step therapy is clinically appropriate for all or nearly all patients and does not pose a greater risk of any significant side effect or harm c) Patients will have a reasonable chance to meet their clinical goals with first-step therapy d) Failure of the first-step drug and the resulting delay in beginning the second-step agent will not lead to long-term harm for patients e) Patients are not required to retry a first-line drug with which they have previously had adverse side effects or an inadequate response at a reasonable dose and duration • In order to justify required switching policies as appropriate, payers should explicitly affirm or present evidence to document all of the following: a) Use of the required drug reduces overall healthcare spending b) The required switch therapy is based on the same mechanism of action or presents a comparable risk and side effect profile to the index therapy c) The required switch therapy has the same route of administration or the difference in route of administration will create no significant negative impact on patients due to clinical or socioeconomic factors d) Patients are not required to switch to a drug that they have used before at a reasonable dose and duration with inadequate response and/or significant side effects including earlier use under a different payer	• Transparency of policies to consumers prior to health plan selection: individuals considering health plan enrollment should be presented with clear information allowing them to understand whether the treatments they currently take or envision taking will be subject to nonmedical step therapy or switching policies • Transparency of policies to clinicians and patients during care: clinicians, pharmacists and patients should be able to rapidly determine the requirements related to step therapy and switching policies and be able to easily view a full justification from the insurer • Reasonable flexibility: implementation of economic-step therapy or required switching for patients entering a new benefit plan should offer a minimum 60-day grace period for patients who are already stabilized on a particular treatment upon enrollment in the plan. During this period, any drug regimen should not be interrupted while the step therapy or switching requirements are addressed. In addition, switching should not require new prior authorization to be completed by the prescribing clinician • Robust appeals procedures: appeals of economic-step therapy and required switching should be readily available and handled within a short timeframe commensurate with the clinical seriousness of the condition. In the case of emergencies, decisions should be made within 24 h of the request and for non-emergencies, 72 hours is a reasonable time. Clinical experts in the relevant field should be consulted as part of the internal adjudication process • Updating with new evidence: evidence should be reviewed annually at a minimum to ensure that economic-step therapy and required switching policies are consistent with the latest evidence on drugs' relative safety, clinical effectiveness and cost–effectiveness • Evaluation of impact: Insurers should perform annual evaluations of the impact of their economic-step therapy and required switching policies to determine rates of exceptions, rates of coverage denials overturned on appeal, and feedback from clinical experts and patient groups on the impact of restrictions on care and on patient outcomes

If the net price of a drug meets the 'fair price' standard established by a payer, and there are no other appropriate options, then it seems reasonable to place the drug on the tier with the lowest applicable cost sharing. In contrast, if the drug is deemed to be priced above a fair price threshold, then, as an alternative to excluding the drug from coverage, it seems reasonable for payers to use tiering and higher cost sharing to try to create negotiating leverage with the drug maker to achieve a lower price. The role of manufacturer copay coupons in the private market has greatly complicated, and in some cases eliminated the business case for using tiering as a negotiating tool to achieve more reasonable pricing, but the ethical argument for linking tiering to a transparent judgment of fair pricing remains.

One implication of linking judgments of fair pricing to tiering is that in some situations, even when patients have no other clinical option, it could be appropriate for payers to place an unreasonably priced drug on a high tier. Some commentators have argued that it seems unfair to 'penalize' patients by requiring higher cost sharing for their medication just because their medication is not fairly priced [12]. However, based on data related to individuals and families delaying care, foregoing care and even dropping out of the health insurance pool due to increasing health insurance premiums, we believe that there is adequate evidence to demonstrate that paying more than appropriate for health gains in one segment of the patient population does more harm than good [13,14]. Therefore, we believe that a strong ethical argument can be made to retain the option for plan sponsors and payers to require higher cost sharing for drugs that are not reasonably priced, albeit within levels that will not serve as a major barrier to access [15].

A different set of considerations comes into play when multiple drugs are viewed as clinically appropriate and all are priced fairly according to the payer's evaluation. In this situation, the ethical goals of fair access would suggest that at least one of these drugs should be on the lowest relevant tier. However, if at least one is placed on the lowest tier, fair design criteria would allow payers to continue to use tiering and graduated cost sharing to leverage competition in hopes of even lower prices for all drugs in the class.

Unfortunately, most of the benefit designs that payers administer require that certain categories of drugs, for example, 'specialty drugs', have their own designated tier or tiers in a formulary. This may make it impossible to place a brand drug on the lowest tier within the entire formulary, a tier which is often restricted to preferred generic options. To meet the ethical goals for fair access, it would be preferable for any drug, no matter how expensive, be eligible for placement on the lowest tier if the drug is fairly priced. However, if the plan sponsor does not agree to make this option available, then it seems reasonable for payers to assign at least one fairly priced drug to the lowest tier available for that type of drug.

We address specific fair design criteria for economic-step therapy later in this paper, but the question relevant to cost sharing is how much the patient should be asked to pay out of pocket when they have been a 'good soldier' by trying the first-step therapy and, through no fault of their own, have not received adequate results, and therefore must move on to the second-step option. If the second-step agent is priced above the reasonable price standard, then higher cost sharing would be justifiable. But if the second-step agent is fairly priced, then the ethical goals of fair access would suggest that it should be on the lowest tier, consistent with its drug class. Unfortunately, this approach may be prohibited in certain rebate agreements that require the payer to assign all second-step agents to a higher tier. Wherever possible, payers and manufacturers should avoid this approach in step therapy contract negotiations.

Clinical eligibility criteria

Ethical goals for access

The ethical goals that should be met by the language and implementation of clinical eligibility criteria are fundamental to fair access. Prior authorization protocols can create a risk of delayed or abandoned care that harms patients [16,17]. However, the reason prior authorization remains a reasonable policy tool, in principle, is because without prior authorization there is also a risk – sometimes significant – for overuse or misuse of treatments, leading to harm for patients and to wasted resources [18]. Therefore, although for many drugs prior authorization may not be necessary at all, when it is, clinical eligibility criteria play an important role not only in controlling costs but in ensuring appropriate care. The ethical challenge lies in finding the right balance between the burden and barriers posed by clinical eligibility criteria and the health and financial gains that can result from prudent application.

It is also important to note several other ethical goals for appropriate clinical eligibility criteria. First, coverage criteria should ensure that the US FDA label is not interpreted or narrowed in a way that specifically disadvantages patients with underlying disabilities unrelated to the condition being treated. Second, the entire process of creating

10.2217/cer-2021-0027

and implementing clinical eligibility criteria must be sensitive to the impact of racism and other forms of bias on the development of evidence supporting new treatments. Systemic racism has resulted in patterns of clinical trial design and conduct that lead to underrepresentation of black Americans and other minorities among enrolled patients [19].

A third ethical goal for clinical eligibility criteria is that they should not use the eligibility criteria from of the pivotal trials supporting FDA approval as arbitrary red lines for coverage unless the FDA has included them in the label to ensure a positive risk–benefit balance. The eligibility criteria from pivotal trials often include relatively arbitrary cut offs for age, severity and functional status. Patients with specific comorbidities may be excluded from the trials entirely. Since patients who did not meet these eligibility criteria were not able to participate in the trials, it is technically true that there is 'no evidence' on the drug's safety or effectiveness for patients like them. It can be appropriate for pivotal trial eligibility criteria to be applied to coverage determinations, but only with reasonable flexibility, especially for patients who fall just outside arbitrary cut offs.

Translating ethical goals for access into fair design criteria

The key issues for fair design criteria relate to whether and how clinical eligibility criteria can be used to narrow coverage from that implied or explicitly included in the FDA label. Given that the FDA has resources, time and evidence evaluation expertise beyond that available to payers, some would argue that fair design criteria should universally prohibit using clinical eligibility criteria to narrow coverage, and that payers should therefore always use the FDA language without revision so that clinicians and patients can exercise broad latitude in considering the risks and benefits for individual patients. However, we believe there are several purposes for which it is not *a priori* unreasonable to adopt more specific or narrower coverage language:

- To clarify indeterminate diagnostic or clinical terms. The FDA may not have included language in its label on how the diagnosis of the condition should be defined or verified. Payers may seek to use clinical guidelines, the eligibility criteria from the pivotal clinical trials, or other sources to create a more specific definition of the patient population for which a drug will be covered;
- To triage patients by clinical acuity when both reasonable and necessary. In extraordinary situations, payers may feel that providing broad coverage according to the FDA label may not be in patients' best interests because a lack of qualified providers or other infrastructure constraints would not make treatment available to all in the short term, and the sickest patients might find it difficult to 'compete' for treatment with other patients who are able to wait. Another justification for limitation of broad coverage arises rarely when the financial impact of rapid, broad treatment of a sizeable patient population would create intense short-term budget constraints that would negatively affect the delivery of other services or lead to near-term increases in health insurance costs that would make insurance unaffordable for many plan sponsors and families [20]. Arguably, this was the situation that faced Medicaid and other health systems at the time of the initial launch of novel treatments for chronic Hepatitis C in 2013 [21]. Importantly, using this justification to narrow coverage would only meet ethical goals for fair access if acuity can be determined on objective clinical grounds and waiting for treatment will not cause significant irremediable harm;
- To add requirements for specific clinical characteristics consistent with the patient eligibility criteria used in the pivotal trials underpinning FDA approval. When there is a mismatch between the broad language used in the FDA label and the types of patients for whom there is direct evidence on the risks and benefits of treatment, when, if ever, is it appropriate for payers to narrow coverage to include only patients who meet the original eligibility criteria for the clinical trials? We believe the answer to this question should hinge, in part, on whether the drug is fairly priced. If a payer does not formally and transparently deem that a drug is unreasonably priced, then access to that drug should not be constrained by coverage criteria narrower than the language of the FDA label, albeit with accommodation for coverage criteria that define indeterminate terms used in the FDA label, as described above.

However, for drugs that the payer has deemed, via a transparent, explicit and consistently applied approach, to be overpriced, the ethical balance shifts. Resources spent above a threshold for the health gained ultimately reduce health disproportionately due to increases in insurance premiums. Although the risks of harm from narrow eligibility criteria and greater documentation burden are real and should not be ignored, we believe that the broader benefits for patients across the health system can justify a fair design criterion that allows payers to consider using

clinical eligibility criteria to narrow coverage to reflect the patients studied in the pivotal trials. Accompanying implementation criteria are particularly critical to these fair design criteria: there must be reasonable flexibility and support by robust appeals procedures, as well as documentation requirements that do not result in major barriers to care.

Economic-step therapy & required switching

Ethical goals for access

Economic-step therapy policies should be distinguished from 'regulatory'-step therapy policies that embed into insurance eligibility criteria the same clinical requirements for prior medication use found in the FDA label. Economic-step therapy, in contrast, is added by payers as a requirement that patients try a lower-cost treatment before receiving coverage for the drug they are seeking to use. Economic-step therapy policies can be justified in principle by payers' obligation to seek the prudent use of limited healthcare resources, but these policies have the potential to cause harm should they block patients from receiving the treatment that is the most clinically appropriate for them.

Required switching, sometimes labeled as 'nonmedical switching', is a less common payer policy but functions much like a retrospective form of economic step therapy. In this case, patients who are currently taking a medication are required to switch to another, less expensive drug even if they are stable and satisfied with their current treatment. Some patients are asked to switch to medications they have previously taken and 'failed on'. Earlier iterations of required switching were introduced when generic medications first became increasingly available. The perceived risk to patients was viewed as extremely low if they were asked to switch from an expensive brand name version of a drug to a less expensive generic formulation of the same drug. More recently, however, as cost pressures on drug spending and overall health insurance costs have grown, required switching policies are being considered in which patients would be required to switch from one brand name drug to a different brand name drug.

For both economic-step therapy and required switching, the ethical tension inherent in maintaining fair access becomes extremely visible to patients and clinicians. Some studies demonstrate negative effects on patient outcomes related to economic-step therapy, and there has been a consistent push from many patient groups, clinical societies and commentators to add meaningful consumer protections and transparency to economic-step therapy and required switching policies [22].

Translating ethical goals for access into fair design criteria

Economic-step therapy

In our approach to this issue, we draw heavily upon work by Nayak and Pearson [23] to propose that economic-step therapy policies can only achieve the ethical goals for access if they meet the following set of five fair design criteria:

- The first-step therapy is clinically appropriate for all or nearly all patients and does not pose a greater risk of any significant side effect or harm. Payers should review potential policies with clinical experts, and whenever possible, patient representatives. Payers should ask whether there are certain kinds of patients for whom the proposed first-step drug is inappropriate and should abandon plans for step therapy if there are reasonable clinical grounds to exempt even a modest number of patients from the first-step drug unless a delay in receiving treatment is truly inconsequential;
- Patients have a reasonable chance to meet their clinical goals with first-step therapy. What threshold represents a 'reasonable' chance of meeting patients' clinical needs depends on the clinical context. If the clinical condition has a substantial impact on patients' quality of life such as severe psoriasis, then instituting economic-step therapy would be reasonable only if there is a high likelihood of success with the designated first-step therapy. A high chance of success would also be needed to justify economic-step therapy among drugs with side effects that could cause significant short-term harm;
- Failure of the first-step drug and the resulting delay in beginning the second-step agent will not lead to long-term harm for the patient. Many economic-step therapy policies involve dermatologic, antihypertensive and gastrointestinal motility drugs that treat conditions for which short-term failure with the first-step drug poses extremely little risk of any significant long-term harm. However, the situation is different if considering economic-step therapy policies for drugs treating cancer, mental health or seizure conditions. Failure of a first-line agent may involve some degree of irremediable harm to the patient, even if a transition is made rapidly to a second-line agent. It may be permissible to implement economic-step therapy when there is a very small possibility of an

10.2217/cer-2021-0027

irremediable harm, but only when there is strong evidence that the first-line therapy is equally effective and possesses equivalent risks for serious side effects;
- Patients are not required to retry a first-line drug with which they have previously had adverse side effects or an inadequate response at a reasonable dose and duration. Payers must explicitly build into economic-step therapy policies an exemption for patients who have already tried the first-line drug and had adverse side effects or an inadequate response at a reasonable dose and duration. The one possible exception to this general rule is when patients have tried the first-line drug before but did not receive an adequate dosage or duration of treatment before abandoning it. If the reason the patient stopped the drug was side effects, then requiring the patient to take the drug again is not appropriate;
- Use of the first-step therapy reduces overall healthcare spending, not just drug spending. Some drugs may be more expensive initially but lead to fewer clinician visits or hospitalizations in the long term. Economic-step therapy should not penalize use of such drugs in search of isolated lower-drug spending.

It is important to consider whether economic-step therapy should be applicable to drugs that payers have been determined to be reasonably priced. One argument is that the risks and burdens of step therapy should not be acceptable when a drug already reflects prudent healthcare spending. However, it is our view that applying economic-step therapy to fairly priced drugs should not be prohibited. For example, when cost–effectiveness analysis is used to judge a fair price, it is almost always applied to recommend a price ceiling – a price that at the margin represents the threshold at which the good from using the drug at that price matches the negative harms caused by the opportunity costs of that spending. Therefore, allowing market forces to achieve even lower prices than peak 'fair prices', seems reasonable because it will ultimately provide more resources and/or lower overall health insurance costs to all patients.

Required switching

We believe that the same general fair design criteria proposed for economic-step therapy apply to required switching policies, but given that patients are already receiving satisfactory clinical benefits, the intended new drug must meet the following more stringent requirements to assure that switching poses minimal risks:

- The required switch therapy is based on the same mechanism of action or presents a comparable risk and side-effect profile to the index therapy. In general, this design criterion can be met only with required switching between brand and generic formulations of the same drug or between drugs in the same drug class (including brand and biosimilar biologics), which have the same mechanism of action and therefore identical or nearly identical side effect profiles. Requiring a switch among drugs with different mechanisms of action is only reasonable if the clinical consequences of treatment failure are minimal and the required drug does not have distinctive significant risks compared with the index therapy;
- The required switch therapy has the same route of administration or the difference in route of administration will create no significant negative impact on patients due to clinical or socioeconomic factors. For some patients there will be substantial clinical or socioeconomic consequences of switching among oral, injectable or intravenous infusion treatments. For example, some patients will have chosen an intravenous infusion treatment option because they are unable to administer a self-injectable drug and they live alone. Sometimes, however, relatively minor differences in route of administration will be very unlikely to cause any major concerns for patients and their clinicians;
- Patients are not required to switch to a drug that they have used before at a reasonable dose and duration with inadequate response and/or significant side effects, including earlier use under a different payer;
- Use of the required switch therapy reduces overall healthcare spending, not just drug spending.

Conclusion

With ongoing attention to determining and achieving fair drug pricing, there is an equal need for further policy analysis of fair access to pharmaceuticals. But this paper is likely to fully satisfy no one. It will leave some patient advocates and clinician representatives feeling that too much weight has been given to the importance of managing limited healthcare dollars, and that too much discretion has been allowed to payers to construct policies that put patients at risk. Conversely, many payers will feel that this paper questions unfairly their moral compass; that their

commitment to evidence is discounted, while their efforts to make sure that patients are not hurt by inappropriate prescribing lie undefended from misplaced suspicions that the bottom line drives their actions.

This paper can therefore only represent a first step toward greater understanding and dialogue. Even if it is by disagreeing with the assertions made here, we hope that all involved in these issues will gain some insight into the experience of others and perhaps appreciate that simple answers to what is 'fair' are not possible. However, we believe that there is broad consensus on key ethical goals for access, and that it is possible to translate these goals into fair design criteria to guide the development of specific policies, and, importantly, to provide a framework that patient groups, clinicians and all participants in the health system can all use to engage in discussions around the appropriateness of benefit designs and coverage policies.

Executive summary

- While patient groups, drug makers and payers can now talk (or argue) more concretely about what would be the 'fair price for a drug', a question far less examined is how to determine whether insurance coverage is providing 'fair access to a drug'.
- This paper sets out ethical goals and design criteria to determine whether insurance coverage is providing fair access to a drug in three areas: cost sharing, clinical eligibility criteria, and step therapy and required switching.
- The broader aim of cost sharing should be to design the drug benefit in a way that addresses moral hazard and can steer patients to clinically appropriate lower-cost alternatives without undermining the basic purpose of insurance, which is to spread financial risk over broad populations to protect individuals from direct harm.
- Fair design criteria would allow payers to continue to use tiering and graduated cost sharing, even for drugs that are fairly priced, in order to leverage competition in hopes of even lower prices for all drugs in the class.
- Prior authorization remains a reasonable policy tool, in principle, because without it there is a risk – sometimes significant – for overuse or misuse of treatments, leading to harm for patients and to wasted resources.
- If, however, a payer does not formally and transparently deem that a drug is unreasonably priced, then access to that drug should not be constrained by coverage criteria narrower than the language of the US FDA label.
- Step therapy policies can be justified in principle by payers' obligation to seek the prudent use of limited healthcare resources, but they obviously have the potential to cause harm should patients not be able to receive the treatment that is the most clinically appropriate for them.
- We draw heavily upon work by Nayak and Pearson to propose that economic-step therapy policies can only achieve the ethical goals for access if they meet five fair design criteria.
- We believe that there is broad consensus on key ethical goals for access, and that it is possible to translate the goals we set out into fair design criteria to guide the development of specific policies, and, importantly, to provide a framework that patient groups, clinicians and all participants in the health system can all use to engage in discussions around the appropriateness of benefit designs and coverage policies.

Disclaimer

In development of this White Paper, ICER hosted senior leaders from membership companies for an in-person meeting to deliberate on key policy recommendations for appropriate cost sharing and utilization management policies for pharmaceuticals. No assertion, judgment or recommendation included in the White Paper should be viewed as representing the opinion of any participant or their company. ICER alone is ultimately responsible for the final content.

Please find a list of 2019-2020 members: Allergan, America's Health Insurance Plans, Alnylam Pharmaceuticals, Anthem Blue Cross Blue Shield, AstraZeneca, Biogen, CVS/Caremark, Blue Shield of California, Boehringer Ingelheim, Editas Medicine, Express Scripts, Genentech, GlaxoSmithKline, Harvard Pilgrim Health Care, Health Care Service Corporation, Health First, Health Partners, Johnson & Johnson, Kaiser Permanente, LEO Pharma, Mallinckrodt, MedSavvy, Merck, National Pharmaceutical Council, Novartis, Premera Blue Cross, Prime Therapeutics, Regeneron, Sanofi and UnitedHealthcare.

Financial & competing interests disclosure

ICER receives support from pharmaceutical and health insurance companies for its Policy Leadership program including the annual ICER Policy Summit. Current members include: AbbVie, Alnylam Pharmaceuticals, America's Health Insurance Plans (AHIP), Anthem, AstraZeneca, Biogen, Blue Shield of CA, Boehringer Ingelheim, CVS Caremark, Envolve Pharmacy Solutions, Express Scripts, Genentech, GlaxoSmithKline, Harvard Pilgrim Health Care, Health Care Service Corporation (HCSC), Healthfirst, Humana, Kaiser Permanente, LEO Pharma, Mallinckrodt Pharmaceuticals, Merck & Co., National Pharmaceutical Council (NPC), Novartis, Pfizer, Premera Blue Cross, Prime Therapeutics, Regeneron, Sanofi, United Healthcare and uniQure. ICER also receives funding from government grants and nonprofit foundations including Arnold Ventures, the California Health Care Foundation and the

Kaiser Foundation Health Plan, Inc. The Office of Health Economics received a research grant from ICER to support its work in relation to the Policy Summit. The Office of Health Economics receives funding from pharmaceutical companies and from other sources for its research programs, and also operates a consulting arm, OHE Consulting. C Henshall received a fee from ICER for the time he spent contributing to the 2019 ICER Policy Summit, the background paper for it and this article. C Henshall receives fees for consultancy services relating to health technology assessment and health outcomes research and policy from various for-profit and other not-for-profit organizations working in the life sciences and health system sectors, including in 2019 the Office of Health Economics, Craig Rivers Associates International (London), Evidera Market Access Ltd and Janssen Pharmaceutica N.V. Adrian Towse received funding from ICER for providing scientific content for the meeting. He is employed by the Office of Health Economics, a registered charity, which receives funding from a variety of sources, including consulting and research income from pharmaceutical companies. The authors have no other relevant affiliations or financial involvement with any organization or entity with a financial interest in or financial conflict with the subject matter or materials discussed in the manuscript apart from those disclosed.

No writing assistance was utilized in the production of this manuscript.

References

1. Teagarden J. Managing heterogeneity in prescription drug coverage policies. *J. Manag. Care Pharm.* 20(6), 564–565 (2014).
2. Robinson JC. Stat News. Mending the broken social contract for pharmaceutical pricing and innovation. (2020). https://www.statnews.com/2020/03/13/mending-broken-social-contract-pharmaceutical-pricing-innovation/
3. Pearson S, Lowe M, Towse A, Segal C, Henshall C. Institute for Clinical and Economic Review. Cornerstones of 'fair' drug coverage: appropriate cost-sharing and utilization management policies for pharmaceuticals. (2020). https://icer.org/assessment/cornerstones-of-fair-drug-coverage-2020/
4. Pauly MV. The economics of moral hazard: comment. *Am. Econ. Rev.* 58(3), 531–537 (1968).
5. Robinson JC, Brown T, Whaley C. Reference pricing changes the 'choice architecture' of health care for consumers. *Health Aff.* 36(3), 524–530 (2017).
6. Hamel L, Wu B, Brodie M, Aliferis L, Stremikis K, Antebi E. The health care priorities and experiences of California residents: findings from the Kaiser Family Foundation/California Health Care Foundation California Health Policy Survey. (2019). https://www.chcf.org/publication/poll-shows-californians-health-priorities-new-governor-legislature/
7. Brook RH, Keeler EB, Lohr KN *et al.* The health insurance experiment: a classic RAND study speaks to the current health care reform debate. (2006). https://www.rand.org/pubs/research_briefs/RB9174.html
8. Brooke RH, Ware JE, Rogers WH *et al. The Effect of Coinsurance on the Health of Adults: Results from the RAND Health Insurance Experiment.* The Rand Publication Series, CA, USA (1984).
9. Huskamp HA, Keating NL. The new Medicare drug benefit: formularies and their potential effects on access to medications. *J. Gen. Intern. Med.* 20(7), 662–665 (2005).
10. Centers for Medicare & Medicaid Services. Medicare advantage value-based insurance design model. (2020). https://innovation.cms.gov/innovation-models/vbid
11. Chernew ME, Iver AJ, Shah M *et al.* Evidence that value-based insurance can be effective. *Health Aff (Millwood).* 29(3), 530–536 (2010).
12. Graff J, Shih C, Barker T *et al.* Does a one-size-fits-all cost-sharing approach to incentivize appropriate medication use? A roundtable on the fairness and ethics associated with variable cost sharing. *J. Manag. Care Spec. Pharm.* 23(6), 621–627 (2017).
13. Kirzinger A, Muñana C, Wu B, Brodie M. KFF Health Costs. Data note: Americans' challenges with health care costs (2019). https://www.kff.org/health-costs/issue-brief/data-note-americans-challenges-health-care-costs/
14. Vanness DJ, Lomas J, Ahn H. A health opportunity cost threshold for cost-effectiveness analysis in the United States. *Ann. Intern. Med.* 174(1), 25–32 (2021).
15. Pearson SD. The patient not in the room. *Ann. Intern. Med.* 174(1), 109–110 (2021).
16. Robeznieks A. 1 in 4 doctors say prior authorization has led to a serious adverse event. (2019). https://www.ama-assn.org/practice-management/sustainability/1-4-doctors-say-prior-authorization-has-led-serious-adverse
17. American Medical Association. 2018 AMA prior authorization (PA) physician survey. (2018). https://www.ama-assn.org/system/files/2019-02/prior-auth-2018.pdf
18. Friedhoff SG, Hankoff JF, Sherman MS. Washington Examiner. Prior authorization is smart medicine. (2020). https://www.washingtonexaminer.com/opinion/op-eds/prior-authorization-is-smart-medicine
19. Oh SS, Galanter J, Thakur N *et al.* Diversity in clinical and biomedical research: a promise yet to be fulfilled. *PLoS Med.* 12(12), e1001918 (2015).
20. Saltzman E. Demand for health insurance: evidence from the California and Washington ACA exchanges. *J. Health Econ.* 63, 197–222 (2019).

21. Treatment Action Group. Hepatitis C: no more treatment rationing; universal access to generic medicines everywhere. Press release: https://www.treatmentactiongroup.org/statement/hepatitis-c-no-more-treatment-rationing-universal-access-to-generic-medicines-everywhere/.

22. Snow J, Feldman MA, Kappel J. Xcenda AmerisourceBergen. The impact of step-therapy policies on patients. (2019). https://www.xcenda.com/-/media/assets/xcenda/english/content-assets/white-papers-issue-briefs-studies-pdf/impact-of-step-therapy-on-patients_final_1019.pdf?la=en&hash=A7BB3FA4DAC189D9240CF8B724B435A8942E91DF

23. Nayak RK, Pearson SD. The ethics of 'fail first': guidelines and practical scenarios for step therapy coverage policies. *Health Aff.* 33(10), 1779–1785 (2014).

10.2217/cer-2021-0027