



CORNERSTONES OF “FAIR” DRUG COVERAGE: APPROPRIATE COST-SHARING AND UTILIZATION MANAGEMENT POLICIES FOR PHARMACEUTICALS

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Introduction

The Challenge

At the heart of all health insurance programs, public or private, lies an inescapable ethical tension between two desired goals. The first goal reflects a noble feature of our society: that insurance allow patients and clinicians to use health care services as they see best to save lives, to restore function, and to improve the quality of life of those who are ill. Drugs and other treatments that improve the lives of patients and their families can advance this goal. But standing in tension with the aim that insurance should allow personalized choices among all available medical services is the fact that the resources—the money—available for health care will always be limited. Whether the limits come from the amount of money people can spend on their insurance premiums and co-payments for specific services, or by the amount of business or government revenues that are allocated to public health insurance, the resources available for health care will always be limited.

Individual patients within an insurance system are protected from most of the direct financial trade-offs involved in paying for health care, but this function of insurance creates a risk that clinicians and patients may “overuse” services that have unknown or limited benefits, or that are more expensive than options that offer equivalent outcomes. Thus, in all insurance systems, on behalf of patients, consumers, and taxpayers, it falls to health plan sponsors and payers to create benefit designs and coverage policies that constrain the scope of covered services and that guide utilization within available options in an effort to exercise prudent stewardship over pooled, and ultimately limited, resources.

But creating constraints on which services will be covered and how inevitably leads to the risk that individual patients will face barriers to the care that is, for them, not only the most effective but the most cost-effective. Using cost-sharing to steer patients to more cost-effective options may create financial toxicity for patients who must use the more expensive option for legitimate clinical reasons. Using coverage policies to narrow access to care across all members of an insurance plan runs the risk of missing the important exceptions to every rule, thereby creating barriers to the right care at the right time. The administrative cost for clinicians of navigating coverage approval requests, and the costs for insurers of adjudicating these requests, adds another layer of potential waste on top of a resource-constrained system. And in a competitive private insurance market, there may even be pressure on insurers to develop and implement coverage policies that are more stringent than those of competitors, in order to lower costs and gain employer clients.

This ethical and practical tension between providing insurance that maximizes personalized choices and the need to manage resources fairly within budget constraints exists throughout the entire health system but it may be that it is nowhere more fiercely debated today than in the area of health benefit design and coverage policy for pharmaceuticals.¹ The cost of drugs has become one

of the largest components of health spending, and is projected to continue to increase relative to other spending areas.² State governments have responded to cost pressures by implementing a variety of potential remedies, from overseas reimportation of less expensive drugs, to price transparency laws mandating disclosure of drug price increases, to application of independent value assessment reports as part of enhanced negotiating targets with drug makers.^{3,4} At the federal level, policymakers have also advanced numerous proposals, including elimination of the drug rebate system, international reference pricing, and direct Medicare negotiation.^{5,6}

In the commercial insurance market, many employers and other plan sponsors have sought to address cost pressures by adopting benefit designs with steep out-of-pocket cost-sharing requirements for patients, including increased deductibles and higher cost sharing even when lower priced options are not available.⁷⁻⁹ Plan sponsors have sought to revise contracts with insurers and pharmacy benefit managers (PBMs) to reduce the role that rebates play in supporting higher list prices.¹⁰ Insurers and PBMs, among other measures, have expanded their use of drug value assessment reports from the Institute for Clinical and Economic Review (ICER) to support price negotiation,^{11,12} and have moved to broadly intensify their application of prior authorization protocols, step therapy, and preferential formulary status as leverage to obtain lower prices and manage drug utilization.^{13,14}

As these efforts by policymakers, plan sponsors, insurers, and PBMs have advanced, the balance between the goals of unfettered access and the need for cost control has become more contentious. Much of the focus in this debate has been on drug prices. ICER has contributed substantially to thinking in this area and has strongly influenced policymakers' views of how to determine when a price fairly aligns with patient benefits.^{15,16} But if payers and other stakeholders can now talk concretely (albeit without full consensus) about the conceptual framework for 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*. It appears widely agreed that cost sharing and drug coverage criteria serve everyone's interest when they can steer patients and clinicians toward evidence-based use of treatments that achieve equal or better outcomes at lower costs. But this level of conceptual agreement does little to help advance thinking on how to assess and judge specific cost-sharing provisions and prior authorization protocols. Is it fair to have patients pay at the highest cost-sharing level when there is only a single drug available in a drug class? What are the circumstances in which step therapy is a reasonable approach to limiting coverage? When is it appropriate for the clinical criteria required for coverage to be narrower than the Food and Drug Administration (FDA) labeled indication? And how should the pricing of a drug, whether it is deemed reasonable or not, factor in to whether certain strategies to limit or steer patient access are appropriate?

The answers to these and other related questions are central to whether insurance coverage is striking the right balance between the goals of cost control and freedom to choose without restrictions among available care options. With ongoing attention to determining and achieving fair

drug pricing, there is an equal need for further policy analysis of fair access to pharmaceuticals.¹⁷ 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 criteria is the purpose of this White Paper.

Scope and Structure of the White Paper

The many potential barriers to access run a broad gamut including problems related to health literacy, disability status, provider availability, and access to affordable insurance coverage.¹⁸ In this paper, we will focus narrowly on two areas over which plan sponsors and payers (defined here as inclusive of both PBMs and insurers) have direct control: cost-sharing provisions and the design and implementation of utilization management. We feel this focus will concentrate the analysis on illuminating options over which plan sponsors and payers have significant discretion. We also feel these are the areas in which there is the most controversy about how to judge whether drug coverage is appropriate.

Given this focus on cost sharing and utilization management, we will not be addressing many other important areas of coverage policy, including thresholds for the number or type of drugs needed within drug classes; coverage for off-label prescribing; potential changes to the current rebate system; high-deductible benefit designs; and the role of copayment coupons. Instead, this paper directly addresses the following five domains:

- Cost-sharing provisions and tier placement as part of the drug benefit design
- Timing of development of prior authorization protocols following FDA approval
- Clinical eligibility criteria
- Step therapy and coverage requirements to switch medications
- Restrictions on prescriber qualifications.

To provide context, the Background section of this paper will describe the current policy landscape and existing approaches to cost sharing and utilization management. Next, for each of the five major domains listed above, we will provide a conceptual analysis of the ethical and practical trade-offs that plan sponsors and payers must navigate, along with a description of the factors and contextual considerations that should be taken into account when deciding how to strike a reasonable balance between cost control and less constrained access. We will then present specific fair design criteria by which to develop and assess benefit designs and utilization management. To accompany these design criteria, we include implementation criteria that are also required for cost sharing and utilization management to be judged as appropriate.

Methods

To inform the development of our framework we conducted a literature review and stakeholder interviews as follows:

Literature Search

- A search of Medline was conducted utilizing PubMed and the following Medical Subject Headings (MeSH): Formularies as Topic, Managed Care Programs, Prior Authorization, Prescription Drugs. A keyword for “Step therapy” was used as well. This search returned 71 unique citations. After a review of the articles’ titles and abstracts, the team felt that 40 warranted a full-text review. Additional references were identified by our interviewees, through additional manual searches conducted by the team, and through the review of the reference lists for included articles.

Interviews

- Ten stakeholder interviews were conducted with a total of 20 interviewees using a structured interview guide. Interviewees represented patient community organizations, employer plan sponsors, PBMs, commercial health plans, health policy research organizations, specialty medical organizations, and health benefit consultants.

Policy Summit Meeting

- Representatives from patient organizations and clinical specialty societies joined senior policy leaders from 25 payer and life science companies at a two-day meeting in December 2019 to discuss an earlier version of this paper, debate the concept of fair access, and provide suggestions for revisions to the paper. The participants in this meeting are shown in Appendix E. None of these participants or their organizations should be considered as having approved of any element of this paper.

Background

This section summarizes the complex current state of play in cost sharing, formulary development, and prior authorization. A basic understanding of how these policies and processes function is essential prior to exploring criteria to ensure their ethical design.

How are Cost-Sharing Provisions Structured?

The first consideration in drug coverage is the overall benefit design, which determines categories of covered benefits and the level of cost sharing for all members of an insurance plan. As will be described below, most elements of cost sharing for drug coverage are determined by statute for patients in Medicaid, Medicare, and state health exchanges under the Affordable Care Act (ACA). In the commercial market, there are certain limits to cost sharing that are governed by the ACA, but otherwise cost sharing is largely determined by employers and other plan sponsors, sometimes working in conjunction with health benefit design consultants, and frequently selecting from a set of standard benefit designs marketed by payers. Although in some cases there are general guidelines for adequacy of coverage stipulated by state departments of insurance or CMS, plan sponsors and payers have wide latitude to use different approaches to drug coverage and formulary development, including the number of drugs in certain classes, the methods by which to create formulary tiers, and the level of cost sharing associated with different tiers.

Cost sharing is integrated into the structure of formularies, of which there are two fundamental types: open and closed. Open formularies, which are now rare, generally cover all FDA-approved drugs, whereas a closed formulary is structured to allow for the exclusion from coverage of certain drugs. Although either type of formulary can have multiple tiers with differential cost sharing, closed formularies have risen to dominance because they give preferential formulary placement greater power to drive market share in favor of drugs for which the drug maker is willing to offer a lower list price and/or greater rebates.¹⁹ According to the 2018 Pharmacy Benefit Management Institute (PBMI) survey, 79% of employer-sponsored plans surveyed report utilizing closed formularies.²⁰ It is important to note that closed formularies always have some process for patients and/or providers to request exceptions to a drug not on the formulary if there is demonstrable medical necessity.²¹

For drugs in the formulary, nearly all insurance plans except Medicaid use tiers with differential cost sharing. The first, or lowest, tier—associated with the lowest level of cost sharing—is often designated for lower cost generic drugs. Branded drugs are typically placed in one or more higher tiers with varied levels of cost sharing. When there are multiple brand name drugs available to

treat a condition, a common approach is to designate one of them as the “preferred” brand drug and place it on a relatively low tier, often the second tier, with all “non-preferred” options allocated to a higher tier that requires a higher cost share. Brand name drugs can be preferred due to superior efficacy, lower net cost to the plan sponsor, or lack of available alternative/generic equivalents. An increasing number of commercial plan sponsors and insurers now have expanded formulary tiers to include special tiers for the highest cost specialty drugs. For these drugs, some benefit designs shift from copayments used in lower tiers to some form of coinsurance (often 20-50%) that is almost always linked not to the lower negotiated price but to the list price of the therapy.

Patients requiring chronic use of therapies in higher tiers, particularly patients with multiple medications, often accrue out-of-pocket costs so high that early in the calendar year they hit out-of-pocket maximum thresholds set by the benefit design.²⁰ Across all commercial insurance plans, out-of-pocket limits for all services, including prescription drugs, are set each year by the ACA. For 2019, limits were \$7,900 for an individual plan and \$15,800 for a family plan. These maximum limits apply to commercial plans as well as health exchange plans, but do not apply to Medicare Part D plans. For these plans, beneficiaries pay 25% of their prescription drug costs until they have paid \$5,100 out of pocket.²² Once beneficiaries reach that spending level, they enter the “catastrophic” coverage phase of their benefit in which they pay 5% for all subsequent prescription drug costs. While a 5% coinsurance cost may not seem onerous, for financially vulnerable families and for all who require expensive specialty pharmaceuticals it can still represent a daunting sum, and Medicare Part D does not have a cap on out-of-pocket spending. Total annual out-of-pocket costs in 2019 for Part D beneficiaries averaged more than \$8,000 across 28 specialty tier drugs.²³ We set out in Appendix A further details on cost sharing arrangements for different types of benefit designs.

How are Formularies Developed?

Formulary development occurs quite differently in public and private insurance systems, and even within the same insurance system there is substantial variability. Different names are used for the groups and committees involved, different approaches are used to gather and evaluate evidence, and different mechanisms exist for integrating financial considerations. But, at its heart, formulary development can be viewed as a combination of three basic elements:

- Assessment of clinical trial evidence by payer staff, focusing on safety and effectiveness
- Consideration of relevant specialty society guidelines and formal evidence reviews
- Deliberation on this evidence, with key evidence votes or judgments on formulary status delegated to an independent Pharmacy and Therapeutics (P&T) Committee
- Integration of economic considerations, often performed by a “financial/contracting” group including payer staff involved in negotiating drug prices and tiering, and in some

cases, provider contracts.

There are two common ways in which these three elements are brought together in an overall process of formulary development. The first approach features a strict separation of evidence assessment, P&T Committee functions, and economic considerations.

This approach is intended to ensure that economic considerations do not subvert a fair assessment of the evidence on a drug's relative safety and effectiveness. In this model, clinical evidence assessment is performed by the payer's Evidence Assessment team and then presented to a P&T Committee comprised of clinicians and methodologists (with, rarely, a general patient representative) independent of the payer. Payers structure the decisions to be taken by their P&T Committees in various ways. Some ask the P&T Committee to vote on three formulary options for a drug: 1) *should not* be on formulary; 2) *must* be on formulary; or 3) *may* be on formulary. Other payers have their P&T Committees approve recommendations from the Evidence Assessment team that include not only whether the drug should be on formulary but with what basic utilization management requirements.

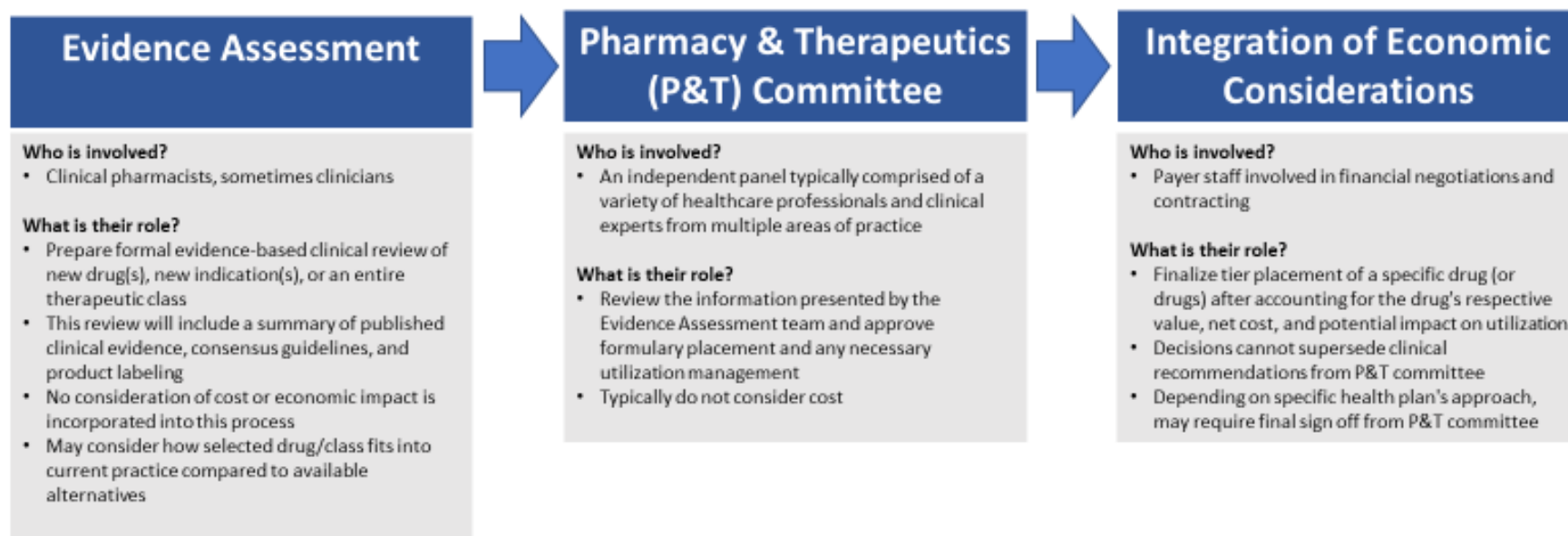
In this model of formulary development, following the P&T Committee decisions, a separate group of payer staff comprised of financial/contracting personnel meets to discuss economic considerations that may determine the final tier placement of a drug within the formulary and whether step therapy will be a part of coverage. Negotiations on pricing, rebates, and other terms with drugmakers are central to these decisions, but the process is intended to let the clinical determination of the P&T Committee set the boundaries for whether step therapy can be considered appropriate. If not already taken into consideration, this group will also need to ensure that tiering and utilization management is consistent with specific state laws. Depending on the situation and payer, any step therapy policy or other specific prior authorization criterion suggested by the Economic Considerations group may need to return to the P&T Committee for final signoff to ensure that it is clinically reasonable.

Figure 1 on the following page illustrates the above approach.

Figure 1. Formulary Development Process Type

1

Formulary Development Process Type 1: Separation of Clinical Assessment and Economic Considerations



An alternative procedural model for formulary development merges the functions of evidence assessment and economic considerations at the first step (see Figure 2 on the following page). There remains a strong emphasis on the principle that assessment of the evidence should not be subsumed by economic considerations, but this approach reflects a view that it is best to incorporate economic factors early on when framing recommendations to the P&T Committee on prior authorization and step therapy provisions. In this model, the P&T Committee may be made aware of the economic rationale for specific coverage recommendations coming out of the evidence assessment phase, and the P&T Committee itself may discuss the economic impact (in general terms) of different coverage policy options. Appendix B contains further details on these two approaches to formulary development.

Formulary Development Process Type 2: Integration of Clinical Assessment and Economic Considerations

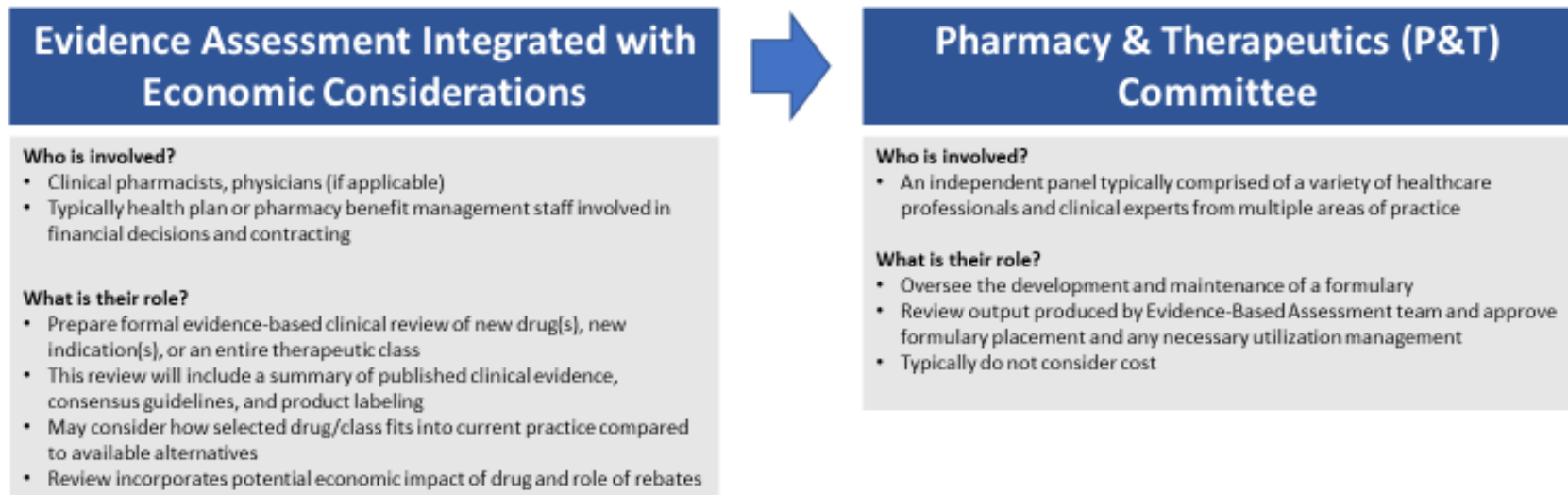


Figure 2. Formulary Development Process Type 2

Key Elements of Formulary Design

Timing of Coverage Following FDA Approval

Immediately following approval by the FDA, “new-to-market” drugs are not formally covered by insurers until the internal evidence review and formulary placement process has had time to occur. In the interim, new-to-market drugs often fall into a form of coverage limbo in which providers must go through a formal exception process and seek coverage on a case-by-case basis. It is routine for FDA label criteria alone to guide these early ad hoc coverage decisions, but patients and clinicians can often experience wide variation across payers. Centers for Medicare and Medicaid (CMS) guidance stipulates that payers must make a “reasonable effort to review a new chemical entity within 90 days, and will make a decision on each new chemical entity within 180 days of its release onto the market, or a clinical justification will be provided if this timeframe is not met....”²⁴

Prior Authorization Protocols and Step Therapy

Prior authorization protocols require that the clinician or the patient demonstrate that certain criteria have been met before coverage is granted. These protocols include clinical criteria that must be met by the patient, including criteria on method of diagnosis and level of severity; whether patients must be taking another medication or avoid certain medications; whether patients must have tried another medication earlier and not had adequate response; and whether prescribing clinicians must meet certain qualifications. Prior authorization protocols also may set limits on the quantity of drug that will be covered and stipulate conditions needed to receive renewal of coverage after an initial period of coverage.²⁵

The use of prior authorization has expanded during the last decade across all types of insurers. Prior authorization requirements increased from 8% to approximately 24% of covered drugs on Medicare Part D plans between 2007 and 2019, and, according to the 2018 PBMI survey, 94% of commercial payers report utilizing prior authorization.^{20,26} Major formularies in some instances now require prior authorization for established generic products that have no obvious lower cost substitutes, including topical corticosteroids, oral immunosuppressive agents, HIV anti-retroviral medications, sulfonylureas for diabetes, and oral antineoplastic drugs for cancer.²⁷

As described above, prior authorization protocols are developed during formulary development. In many cases, much of the prior authorization language matches word-for-word the criteria included as part of the FDA label. The P&T Committee has the responsibility of confirming or modifying prior authorization protocols, sometimes following the input of a financial/contracting group within the payer. We consider the main categories of prior authorization protocols below.

Clinical Eligibility Criteria

Prior authorization protocols frequently include clinical criteria that must be met by patients in order to receive coverage for a drug. For example, payers may require specific tests be done and documented to confirm the diagnosis. Often, clinical eligibility criteria will require documentation of some kind of the severity of the condition, and the absence of any clinical comorbidities or other treatments that are known contraindications to treatment. For each element of clinical eligibility in a prior authorization protocol, confirmation may require different levels of information, ranging from simple provider “attestation” to the submission of extensive medical record documentation.

Step Therapy and Required Switching

The sequential use of therapies, or “step therapy,” is a common feature of appropriate clinical treatment, especially in areas such as oncology or autoimmune disorders. Its analogue in insurance coverage is to deny coverage for a requested treatment unless patients have tried one or more other treatments first and not had adequate response, aka the treatment has “failed.” Documentation of the use of the required first-step therapy may be based on clinician attestation or on the submission of more extensive medical records.

Importantly, there are two different types of step therapy in drug coverage with completely different rationales. The first, which can be called a “regulatory” step therapy policy, is when the prior authorization protocol requires prior unsuccessful treatment with another treatment because the FDA labeled indication includes this same requirement. This happens when there is an existing treatment that may be safer, equally effective, and/or effective for many patients, and therefore only patients who have not had adequate response to that treatment are enrolled into the clinical trials testing the newer treatment. A regulatory step therapy policy should be viewed as an integral part of the clinical eligibility criteria of a prior authorization protocol, meant to ensure that only clinically appropriate patients receive the treatment.

The second type of step therapy in drug coverage is not linked to FDA label requirements and instead is intended to favor less expensive treatments when there is a clinically appropriate choice among two or more options. This “economic” step therapy is often used among drugs with the same mechanism of action, such as tumor necrosis factor (TNF) inhibitor drugs for inflammatory conditions, but it can also be applied in limited circumstances to drugs in different classes when their risks and benefits are judged to be similar enough to provide clinically appropriate treatment for most patients.²⁸ Whereas regulatory step therapy coverage policies should be considered part of clinical eligibility criteria, economic step therapy is better considered as a separate coverage mechanism, and it raises the greatest questions about appropriate design and implementation.

Related to economic step therapy but distinct in several important respects is a coverage requirement to switch from current treatment to a different treatment that is deemed to be equivalent in effectiveness and less expensive to the payer. This required switching, often labeled as “non-medical switching,” has been used in the past primarily when a generic becomes available for an existing branded drug. Today, however, switching policies are now also being frequently implemented in European health systems when lower cost biosimilar agents are available to compete with originator brands. In the US, some plan sponsors have adopted payer formularies that allow for required switching from one brand drug to another (usually in the same drug class) in order to favor the drug with lowest net cost to the plan sponsor. Sometimes a lower price is offered by a drug maker only if their drug is preferred in the formulary, and therefore all participants in the drug pricing and coverage process play a role in required switching.

For patients, required switching from a drug on which they are satisfied represents an even greater perceived risk than economic step therapy that limits their first choice when beginning therapy. As required switching gains a foothold in coverage policies, the concerns of patients and clinicians are exacerbated further when requirements to switch occur without notice and when switching can occur back and forth across different drugs in different years, or even within the same year.

Restrictions on Prescriber Qualifications

Coverage for some drugs may be restricted to certain clinicians with specialty training in the relevant field or who have access to regular consultation with specialists. These restrictions may be put in place when drugs have important risks or known side effects that require specialist clinician management. In addition, specialty prescriber restrictions may be used when the diagnosis of the condition or its overall management require a high level of experience and expertise. This approach can address some concerns that a new drug will be overused or misused. But prescriber restrictions may create important barriers to gaining coverage, particularly in areas underserved by specialty clinicians. This is one reason why the option of allowing prescribers who are not specialists but who are able to consult with relevant specialists is frequently used by payers.

Principles of Appropriate Access to Drugs

Described in Prior Literature

Several previous studies have explored criteria for fair and appropriate drug coverage policy²⁹⁻³² (See Appendix C, Tables C1-C5). In putting forward our criteria, we draw in particular on five previous sets of recommendations: American Society of Health-System Pharmacists (ASHP); the American Medical Association (AMA); Graff et al. (2017), Nayak et al. (2014) and Robinson et al. (2018). From these prior efforts a number of key points emerge.

- **Balancing clinical and economic considerations:** The ASHP state the importance of “cost-effective drug therapy” but also of “basing formulary system decisions on cost factors only after safety, efficacy, and therapeutic need have been established.”²⁹ It is not clear how cost effectiveness is to be operationalized. Robinson et al (2018) propose that meeting a “value-based” benchmark would be key to determining the burden of utilization management measures and cost sharing that a drug faced.³³
- **The need to look at long-term costs and benefits across all health spending:** Graff et al. emphasize that the balance of clinical and economic considerations should take into account “total health care costs,”³² and Nayak and Pearson set out the need to “weigh cost savings against long-term outcomes” and the impact on “total cost throughout the health care system.”³¹ The implication of both arguments is that the spending on drugs should not be managed as a siloed budget in a way that might lower short-term drug spending at the expense of the broader health and financial interests of the members of an insurance plan.
- **Frequent review:** The ASHP emphasize the need for the formulary to be updated “in light of new drugs, new indications, uses, or warnings affecting existing drugs” (ASHP).²⁹ This goal is echoed by Nayak and Pearson arguing for “rapidly reviewing new evidence.”³¹
- **Formal appeal process and mechanisms to allow use of non-formulary drugs:** The ASHP set out the need for “a formal appeal process if a request for a non-formulary drug is denied” and the need for a “well-defined process for the physician or other prescriber to use a non-formulary drug when medically indicated.”²⁹ This is reinforced by a statement that policies should “state that practitioners should not be penalized for prescribing non-formulary drug products that are medically necessary.” The AMA calls for an expedited (24 hour) appeal process when determined necessary by a provider, and for no prior authorization to be required for use of a drug in emergency care.³⁰
- **Transparency:**
 - *About the formulary:* The ASHP set out the need for patients and physicians to be able to understand how formulary decisions are made.²⁹ The AMA adds to this the

need for all individuals during insurance enrollment to understand how utilization management requirements are applied to specific drugs.³⁰ The AMA also argues for “statistics regarding prior authorization approval and denial rates” to be published.

- *With a patient*: The ASHP argues that transparency is required for any coverage determination “when requested” by a patient and their physician.²⁹ The AMA is stronger, stating that “utilization review entities should provide detailed explanations for prior authorization or step therapy override denials, including an indication of any missing information.”³⁰

Fair Design and Implementation Criteria

In the following section of the paper, we propose Ethical Goals for Access and a corresponding set of Fair Design Criteria for cost sharing and prior authorization protocols. To accompany these design criteria, we suggest additional implementation criteria that are also required for cost sharing and prior authorization protocols to be judged as appropriate. As noted earlier, we focus on the following five areas:

- Cost-sharing provisions and tier placement as part of the drug benefit design
- Timing of development of prior authorization protocols following FDA approval
- Clinical eligibility criteria
- Step therapy and coverage requirements to switch medications
- Restrictions on prescriber qualifications

Cost Sharing

Ethical Goals for Access

One common rationale for requiring cost sharing by patients is to reduce overuse of health care services that may be driven by the “moral hazard” of the lack of direct financial consequence when patients are covered by insurance.³⁴ Deductibles can send a market signal to consumers to be prudent consumers of health services, but the graduated cost sharing associated with tiered drug formularies is often ascribed a very specific goal: to create incentives that will steer patients to select lower cost clinically appropriate treatments when there is a choice among several treatment options. Recent evidence from trials of varying cost sharing 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, in an era of rapidly increasing drug spending, cost sharing is necessary to help control overall insurance premiums.³⁵ 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 the entire insured population.

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.³⁶⁻³⁹ High deductible health plans are viewed as particularly prone to unintended adverse effects on patients, but rising cost-sharing levels within tiered formularies have also been found to have negative effects.⁴⁰

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 in order to protect individuals from direct harm. We propose three specific ethical goals that represent this broader aim.

Ethical Goals for Fair Access: Cost Sharing

- 1. 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.**
- 2. Cost sharing should not be structured primarily to shift health care costs to patients when they have few or no lower cost options that are medically appropriate.**
- 3. The level of cost sharing should not serve as a major barrier to patients being able to afford needed treatment.**

Translating Ethical Goals for Access into Fair Design Criteria

Some implications of these ethical goals for the design of fair cost-sharing provisions are clear. For one, cost sharing for the patient should be based on the net price to the payer, not on the list price, which is ultimately irrelevant to efforts to control costs for the population. It must be acknowledged that linking cost sharing to the net price is complicated by the countervailing need to retain confidentiality surrounding net price in a way that supports the negotiating power of payers, but payers are now finding administrative ways to make this possible.⁴¹

Another fair design criterion that can be deduced from the ethical goals for access is related to high-value medications for chronic conditions. Eliminating cost sharing for high-value, cost-saving treatments has been the focus of extensive policy development and advocacy as part of the drive for value-based insurance designs (VBID).^{42,43} In 2019 the IRS released updated guidance adding drugs for a range of chronic conditions to the list of preventive care benefits that may be provided by a high deductible health plan (HDHP), allowing insurers to cover 14 services for chronic diseases like diabetes and asthma before patients hit their deductibles.^{44,45} Insurers are not legally allowed to add further services to this list on their own, but they should take every opportunity to work with their client plan sponsors to include all of the services on the list when designing their HDHP offerings.

Beyond these relatively straightforward examples, any effort to translate the ethical goals for fair access into specific fair design criteria must take into account important contextual considerations. First, in determining formulary tier placement for a drug, and the corresponding cost-sharing level, one must consider whether there are other appropriate options for patients. Appropriateness in this context should include considerations not only of “clinical” appropriateness, but of broader

considerations such as whether the delivery mechanism and location of care will be feasible for patients across a diverse spectrum of living situations and socio-economic status. If other appropriate options are not available, the ethical goals for fair access would suggest that incentivizing a lower cost alternative is not possible, and therefore, the drug should be placed on the lowest tier in the formulary, where it would be subject to the lowest level of cost sharing.

In this situation, however, it is important to consider whether the drug is fairly priced. Payers who wish to claim that their use of tiering is intended to guide patients toward appropriately priced treatments should adopt a transparent approach to determining whether the price for a drug is reasonable. 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. Any approach should also explicitly consider broader contextual factors and the potential budget impact given the intended patient population. But whether a payer adopts any one particular approach is less important than that it be transparent, explicit, and applied consistently.

If the net price of a drug meets the standard established by a payer for “good value for money” and there are no other appropriate options, then it seems most 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 co-pay 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 would not always be inappropriate for payers to place an unreasonably priced drug on a high tier, albeit within levels of cost sharing that will not serve as a major barrier to access. 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. For example, Graff et al. found that participants in their roundtable discussion felt that cost-sharing scenarios were least acceptable when patients had to bear higher out-of-pocket costs due solely to their biological circumstance.³² 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.⁴⁶ 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. As long as the overall level of cost sharing does not create major barriers to access, it seems that all patients’ long-term interests will be better served by a system in which

payers can use this policy approach as a tool to seek fair prices and thus better health outcomes across all insurance plan members.

A different set of considerations comes into play when multiple drugs are viewed as clinically appropriate and all are fairly priced 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. Drugs can be good value given their clinical benefits even if they are very expensive, and fair design criteria should require payers to honor their commitment to fair access by providing low cost sharing for drugs that are fairly priced in line with their added patient benefits and other factors.

However, when multiple competing drugs in a class are deemed to be priced at reasonable levels, as long as 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. In other words, when considering multiple drugs in a class that are fairly priced, it would not be inappropriate for a payer to negotiate even lower prices by agreeing to place only one drug on the lowest tier, and to place other "non-preferred" drugs on higher tiers. There is obviously a spectrum to "reasonable" pricing, and payers should be allowed to exercise all the tools at their disposal, including tiering, to seek the lowest net cost for plan sponsors. In all cases, however, whether there is just one or multiple drugs in a class that are deemed to be priced reasonably, at least one drug should be placed on the lowest relevant cost-sharing tier.

Unfortunately, in the current landscape of formularies designed by benefit consultants, payers, and plan sponsors, one important practical consideration may limit payers' ability to select at least one drug in every class for the lowest tier. Most of the benefit designs that payers administer will require that certain categories of drugs, e.g., "specialty drugs," have their own designated tier or tiers in a formulary. This benefit design 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.

Economic step therapy presents another important element to consider in regard to fair cost sharing. We address specific fair design criteria for economic step therapy later in this paper, but the question relevant to cost sharing is whether patients who have tried a first-step medication and have had to move on to the second step should have to pay a higher level of cost sharing for the second-step agent. This question is sometimes framed as 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. As in our earlier discussion, this situation intersects with questions regarding whether the second-step agent is fairly priced or not. If the second-step agent is priced

over a 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.

With all these contextual considerations in mind, we propose the following Fair Design Criteria for cost sharing within a drug benefit.

Fair Design Criteria: Cost Sharing for Pharmaceuticals
Patient cost sharing should be based on the net price to the plan sponsor, not the unnegotiated list price.
All medications identified by the IRS as high-value therapies should receive pre-deductible 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.

Implementation Criteria: Cost Sharing for Pharmaceuticals

In addition to content criteria necessary for fair design, there are also two implementation criteria that must be met in order for any cost-sharing scheme to achieve the ethical goals in this area and be judged appropriate. Both of these criteria are related to transparency.

Implementation Criteria: Cost Sharing for Pharmaceuticals
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.

Transparency on cost-sharing policies prior to health plan selection is needed to help consumers understand their options, if any, among benefit designs and specific insurers. Knowing prospectively all cost-sharing requirements will also help consumers make adequate financial plans to be able to cover out-of-pocket costs. Unfortunately, there has been no business incentive for

plan sponsors, their consultants, or payers to provide this information, and the idea has languished without any prominent examples of best practice.

To achieve the goals of transparency in guiding consumer choice, it is equally important that cost-sharing requirements for specific drugs not be changed during the plan year unless patients have the chance to evaluate the implications of the changes for them and select another plan. Here too, there has been no business case for protecting patients in this way, nor has any standard setting body established this as a requirement.

Transparency of cost sharing at the time of the clinical encounter, however, has received greater attention. Recent federal rulemaking now requires Medicare Part D plans to adopt tools that provide clinicians with information that they can discuss with patients on out-of-pocket costs for prescription drugs at the time a prescription is written.⁴⁷ And in November 2019, the Trump Administration released draft Transparency in Coverage Rules that would require certain self-insured plans and other health insurance issuers to disclose price and cost-sharing information for pharmaceuticals to their members through an internet-based, self-service tool as well as in paper form upon request. Private insurers are deploying a variety of tools to give real-time information on cost sharing to clinicians and patients, but the degree of penetration of these tools into practice is difficult to gauge. Clearly, however, if the purpose of cost sharing is to incentivize choices by the patient and clinician for lower cost alternatives, the cost sharing for a particular drug, along with the cost sharing for alternative options, should ideally be made available at the point of care before the first prescription is written.

Timing of Coverage Following FDA Approval

Ethical Goals for Access

When a drug is first approved by the FDA, patients and clinicians may be eager to use it immediately, particularly for serious, progressive conditions. Payers have a responsibility to make appropriate, effective treatments available as quickly as possible, but payers may require some time in order to digest the information available on the drug, determine its formulary placement, and negotiate its price with the manufacturer. Thus, there may be an inevitable tension between the goal of rapid access and the time needed to determine appropriate access.

What should never happen, however, is for the time required to develop a formal coverage policy to be used as a delaying tactic to reduce access and, therefore, expenses for the payer. Even though competitive forces among payers may create a perverse incentive to delay coverage as long or longer as one's competitor, payers should create internal systems to anticipate new drugs, to provide full review and coverage policies as quickly as possible, and to install a process for ad hoc coverage determinations during any interim period that is reliable and responsive. This leads us to the following ethical goals for access.

Ethical Goals for Fair Access: Timing of Coverage Following FDA Approval

- 1. Payer evaluation of treatments should be expeditious to minimize delays in access. Evaluation of drugs that address severe conditions without alternative treatment options should be started before FDA approval whenever possible.**
- 2. The amount of time taken to develop and implement prior authorization protocols should not be used as an indirect method to reduce utilization or to divert clinicians and patients to other insurers.**

Translating Ethical Goals for Access into Fair Design and Implementation Criteria

As we noted earlier, CMS guidance states that P&T Committees must make a “reasonable effort to review a new chemical entity within 90 days, and will make a decision on each new chemical entity within 180 days of its release onto the market...plans must make access to new drugs available to enrollees when medically appropriate via exceptions processes even before this deadline.”²⁴

However, despite this guidance, performance across many payers may not meet these goals. For example, Stuart et al. (2018) evaluated formulary placement for 33 newly approved drugs in eight therapeutic classes in 863 Part D plans with continuous CMS contracts between 2009 and 2013.⁴⁸ They found significant heterogeneity in time taken for new drugs to be placed on the formulary, with only 56.7% of plans placing each new drug within six months of the National Drug Code assignment date. In discussion with a wide range of payers, we elicited a consensus that today it is reasonable to require that new-to-market drugs be placed on the formulary within 90 days.

One way payers can accelerate their review and ensure more timely completion of formulary placement is to employ more robust horizon scanning for potential upcoming FDA approvals and to start evaluation and discussions with manufacturers before FDA approval on those drugs that appear likely to be approved. There will always be limited resources available to do early drug evaluations, and it is not infrequent for the final language in the FDA label to surprise analysts, which would require a revision of any evaluation. In addition, to the extent that the negotiated price influences formulary placement, it may be impossible to engage the manufacturer in a price discussion until FDA approval. Despite these factors, an increasing number of payers are now beginning drug evaluations far earlier than in previous years, allowing them to minimize the gap between FDA approval and the timing of formal coverage language and formulary placement.

No matter how aggressively payers move to accelerate the coverage process, there will always be some drugs for which more time is needed to determine the full contours of a coverage, formulary, and pricing arrangement. As noted earlier, therefore, to meet the ethical goals of access all payers must have a robust system for interim coverage decisions. During this phase, in lieu of other coverage language, it is most appropriate for payers to adopt the FDA label language and seek to apply it transparently and consistently to all requests for coverage that are made.

Taking these factors into consideration we arrive at the following Fair Design Criteria.

Fair Design Criteria: Timing of Coverage Following FDA Approval
“New-to-market” evaluation procedures needed to develop formal coverage policy should be structured to be completed within three months of FDA approval unless the manufacturer has announced it will delay the drug’s launch.
For treatments of rapidly progressive and/or fatal conditions for which there is no alternative approved therapy, payers should implement one of two approaches: <ul style="list-style-type: none">• Coverage should be provided immediately following FDA approval using FDA label language; or• Evaluation procedures should begin prior to FDA approval so that tailored coverage policies are available at the time of FDA approval.
During any early time period after FDA approval when prior authorization protocols have not yet been determined, clinicians should be able to rapidly seek coverage approval based on the FDA labeled language without documentation requirements that lead to substantial burden or delay.

Implementation Criteria: Timing of Coverage Following FDA Approval

There are also two implementation criteria that must be met in order for policies related to the timing of coverage following FDA approval to achieve the ethical goals in this area and be judged appropriate. One of these criteria is related to transparency, the other requires robust appeals procedures. Meeting both implementation criteria will require payers to expend resources to create and maintain important resources for patients and clinicians.

Implementation Criteria: Timing of Coverage Following FDA Approval
Transparency: Clinicians and patients should be able to rapidly determine the status of new-to-market drugs and procedures to request coverage.
Robust appeals procedures: Procedures to request appeals of non-coverage decisions during the phase between FDA approval and formal coverage policy should be transparent, easy to pursue, and expeditious. Adjudication of coverage during the interim should be based on application of language in the FDA label and should be managed by staff with good access to clinicians whose training and experience is in the same or a similar specialty.

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. With prior authorization there may be a risk of delayed or abandoned care that could harm patients.⁴⁹ For example, a 2018 Physician Survey conducted by the AMA on prior authorization found that 65% of providers had to wait, on average, at least one business day within the past week before receiving a prior authorization decision from a health plan and 26% of providers waited three business days or more.¹⁴ It should also always be remembered that prior authorization protocols impose an administrative burden on patients and clinicians that

can, by itself, pose a risk to fair access. Clinicians have cited the burden of prior authorization as one of the leading causes of burnout.⁵⁰

But the reason prior authorization remains a reasonable policy tool, in principle, is because without prior authorization there is a risk—sometimes significant—for overuse or misuse of treatments, leading to harm for patients and to wasted resources.⁵¹ 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 challenge lies in finding the right balance between administrative burden and the health and financial gains that can result from prudent clinical eligibility criteria.

The language in a prior authorization protocol to define the clinical eligibility criteria should find its foundation in the language of the FDA approval. Payers should also consider input from clinical experts in the condition and should perform their own review of high-quality, up-to-date evidence. The ethical goal is to structure clinical eligibility criteria so that patients who will benefit from the treatment are able to receive coverage without delay, whereas coverage is not automatically given when the clinical characteristics of the patient are such that the balance of risks and benefits of treatment is not well established, particularly when there is a risk of harm from the treatment.

Since much of the ethical tension arises in situations in which the clinical eligibility criteria serve to make coverage narrower or more specific than the language used for the FDA label, it is important to note several other ethical goals. First, clinical eligibility criteria should ensure that the FDA label is not interpreted or narrowed in a way that specifically disadvantages patients with underlying disabilities unrelated to the condition being treated. As will be discussed below, it is potentially justifiable for payers to use the exclusion criteria from a drug's pivotal clinical trials as exclusion criteria for insurance coverage as well, even if the FDA label is silent on this point. But what is never acceptable is for a payer to create subpopulations without reference to clinical trial exclusion criteria that are based on blunt measures of current or prospective functional status that would have the effect of excluding from coverage those patients solely on the basis of a pre-existing chronic condition.

Second, the entire process of creating 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.⁵² The evidence base thus fails to provide adequate insights into differential benefits or harms of any particular treatment among these underrepresented communities. The ethical goal should be for payers to recognize the limitations of the evidence being used to design clinical eligibility criteria and to seek input from clinical experts serving underrepresented communities when evaluating language that might narrow coverage in a way that does not reflect the best interests of these

patients. Clinical eligibility criteria must reflect explicit attention to distinctive benefits and harms of treatment that may arise for biological, cultural, or social reasons across different communities.

A final ethical goal for access is related to how the eligibility criteria of the pivotal trials used to support FDA approval are used when designing the clinical criteria within a prior authorization protocol. The eligibility criteria from pivotal trials often include relatively arbitrary cutoffs for age, severity, and functional status. Patients with specific co-morbidities 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. But the ethical goal should be for these trial criteria not to serve as arbitrary red lines for coverage unless the FDA has included them in the label as a reflection that specific cutoffs are judged necessary to ensure a positive risk-benefit balance.

As will be described below, it can be appropriate to use pivotal trial eligibility criteria not mentioned in the FDA label to specify or narrow the clinical eligibility criteria in a prior authorization protocol, but the ethical goal is for these trial criteria to be applied to coverage determinations with reasonable flexibility, especially for patients who fall just outside these arbitrary cutoffs.

Taking the key elements from these considerations, we suggest the following ethical goals for fair access related to clinical eligibility criteria:

Ethical Goals for Fair Access: Clinical Eligibility Criteria

- 1. 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: 1) when necessary to protect patients from inappropriate use of treatments; or 2) when necessary to manage costs of expensive interventions and to negotiate lower prices for drugs that are priced beyond a fair price range.**
- 2. The administrative burden of documenting clinical eligibility should be streamlined and transparent to avoid creating a significant barrier to appropriate care.**
- 3. Clinical eligibility criteria should not extend 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.**
- 4. Arbitrary eligibility cutoffs used in pivotal clinical trials for age, severity, or other clinical characteristics should be subject to reasonable flexibility in coverage determinations.**
- 5. 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.**

Translating Ethical Goals for Access into Fair Design Criteria

To ensure that prior authorization is only used when necessary to ensure appropriate care, payers must develop and offer alternatives that can achieve the same goal without the potential negative impact of administrative burdens on fair access. Many payers have implemented programs that can take the place of prior authorization for certain treatments or for nearly all treatments for certain clinicians. The most prominent example of an alternative to prior authorization is “gold carding,” a general term for programs that analyze prescribing patterns for individual clinicians or even group practices and, for clinicians who demonstrate a track record of high fidelity to evidence-based prescribing, exempt them from the requirement to seek prior authorization.⁵³ Designing clinical pathways that represent high-quality prescribing is often a critical feature of a gold-carding program, and is an area that has been advanced most prominently in the field of oncology, where clinical pathways have been developed that adhere to standards for engaging clinical experts, managing conflicts of interest, and setting up systems for regular updates.⁵⁴

In order to translate many of the ethical goals for fair access into fair design criteria, it is necessary to focus on the process through which eligibility criteria are developed. First, when prior authorization is judged to be necessary, it is self-evident that to achieve fair access, clinical eligibility criteria should never be based on cost alone. It is also important that criteria be developed using up-to-date evidence, and there should be explicit mechanisms through which the criteria are developed or reviewed by clinicians in the same or similar specialty. Moreover, it is important that the process for the development of clinical eligibility criteria include several explicit requirements that will serve as a check on the impact of systematic underrepresentation of minorities in the clinical evidence base and the risk that clinical criteria discriminate against minorities or patients with unrelated disabilities. Efforts to hold payers accountable for these procedural fair design criteria will require access to internal documents that is not generally provided by payers.

Outside of these procedural development 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:

1. 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. When there are risks that this imprecision might lead to inappropriate patient selection for treatment, payers may decide to require a specific form of diagnosis. In

addition, the FDA may use vague terms in its label, such as approving a drug for the treatment of “moderate-to-severe” forms of a condition. 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.

2. 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 there are either real constraints on infrastructure that would not make treatment available to all in the short term, or because 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 families. When new treatments for chronic hepatitis C became available in 2013, it was hotly debated whether these conditions applied and made it reasonable to narrow coverage to patients with more advanced hepatitis.⁵⁵ When considering whether fair design criteria should allow for this kind of triaging by severity or acuity, it seems possible to allow consideration for this approach only if it would not put patients at risk for irremediable harm from delayed coverage. Therefore, we suggest that fair design criteria could allow a narrower set of clinical eligibility criteria only if the following are explicitly provided as a public rationale for the policy:

- 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; AND
- 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; AND
- c. Acuity can be determined on objective clinical grounds and waiting for treatment will not cause significant irremediable harm.

3. To add requirements for specific clinical characteristics consistent with the patient eligibility criteria used in the pivotal trials underpinning FDA approval.

The FDA may not include in its label all of the specific criteria used by the sponsor in the enrollment criteria for its clinical trials—criteria for categories like minimum age, prior medication use, concurrent medication use, or absence of certain clinical comorbidities. Often these criteria do not appear in the label itself because the FDA judges that the evidence from pivotal trials supports approval language for a broader set of patients with the condition, particularly if there is an important unmet medical need and it will be unlikely that further trials will be done in broader patient populations in the near term. When the FDA takes this approach, it means that there is a technical mismatch between the broad language used in the 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 use their clinical eligibility criteria to narrow coverage to include only patients who meet the original eligibility criteria for the clinical trials?

In seeking to meet the ethical goals for fair access, we believe the answer to this question should hinge, in part, on whether the drug is fairly priced. Unless a payer formally and transparently deems 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. It must be acknowledged that cost-effectiveness analyses used to determine whether the price fairly aligns with clinical benefit are limited to using data from available clinical trials, i.e., they may be limited to data on a narrower set of patients within a broad label. But if the price is determined to be fair for that population based on available trial data, we believe that the ethical balance needed for fair access would require payers to abstain from using clinical eligibility criteria to narrow coverage.

It should be noted that even if the clinical eligibility criteria are exactly the same as in the FDA label, when the FDA label contains certain requirements, such as prior use of another medication, payers have a choice in the mechanisms through which they require clinicians to document whether patients meet the criteria or not. Payers can require the submission of detailed medical records, a cumbersome process that may require patients and clinicians to obtain records from prior providers or payers. It may be justified to require this kind of documentation when payers need to be certain that patients do not have clinical characteristics that would put them at substantial risk from a particular treatment. But otherwise, for drugs that are fairly priced, it seems reasonable for payers to accept clinician attestation that the patient meets the applicable clinical eligibility criteria. Clinician attestation can be “gamed” and result in inappropriate prescribing, but unless there is a serious safety issue involved, it appears the best option to maximize appropriate utilization without creating an undue administrative burden that unduly constrains access.

For drugs that are deemed not to be reasonably priced, however, the ethical balance shifts. Pricing may be unreasonable either through formal evaluation of the price according to transparent standards, or from a price increase that is similarly deemed unreasonable on the basis of a lack of clinical justification from new evidence. In both cases, resources spent above a fair clinical value contribute to a net harm in the insured population by increasing insurance premiums out of proportion to the health gained. It is true that using clinical eligibility criteria to create a boundary for coverage that is consistent with the known direct evidence base can create a risk that patients within the broader FDA label may not receive coverage for a treatment that would benefit them. But retaining the prerogative to narrow coverage or to require more extensive documentation of patient eligibility for drugs that are not fairly priced are the two primary mechanisms that payers have in the US system to incentivize drug makers to maintain the affordability of drug prices. Therefore, 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 justify a fair design criterion that allows payers to consider using these coverage

policy tools when a drug's price has been formally deemed unreasonable if the tools can be exercised in a transparent fashion.

Because narrowing coverage from a broader FDA label raises the greatest risk of potential harm to patients, it is important to note the special importance of two implementation criteria, both of which must be fully met in order for a narrower coverage policy to be potentially appropriate. The first is a requirement for "reasonable flexibility" in the application of trial-based enrollment criteria to coverage determinations for individual patients. There is often no clinical justification for the specific cut-offs used for clinical trial enrollment criteria, whether it be the cut-offs used to determine trial eligibility based on age, severity of illness, or other clinical characteristics. Therefore, when coverage is requested for patients who fall just outside any of these arbitrary cut-offs, coverage adjudication should show flexibility, especially if the requested treatment is for a serious condition for which there are no other treatment options. Flexibility should also be granted when specific cutoffs are used for a clinical characteristic that can vary from day to day, such as muscle or lung function. In such cases it may be that a patient will not meet the clinical eligibility criteria on any particular day in the clinician's office, in which case an "average day" approach to adjudicating the prior authorization request will be more reasonable.

The second implementation criterion of particular relevance for the application of narrower coverage policies is a requirement for robust appeals procedures. Accessible, efficient appeals procedures are important to support all elements of insurance coverage policy, but they are especially important when implementing coverage policies that are narrower than the FDA label. In particular, appeals procedures must provide an internal mechanism through which clinicians and patients can rapidly challenge judgments that the patient is not "close enough" to the clinical eligibility criteria that they should be granted coverage. Internal appeals procedures need to be swift and transparent, particularly when the clinical situation is urgent, and there should always be an ultimate option available for an external appeals procedure if needed.

In summary, taking all these factors into consideration we arrive at the set of Fair Design Criteria displayed on the following page.

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:

- Considered limitations of evidence due to systemic under-representation of minority populations; and
- 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
- 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:
 - 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; AND
 - 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; AND
 - 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.

Implementation Criteria: Clinical Eligibility Criteria

There are several implementation criteria that must be met in order for policies related to clinical eligibility criteria to achieve the ethical goals in this area and be judged appropriate. As with all areas of fair design criteria, there are requirements for transparency and appeals procedures. As noted above, there is also a heightened importance that the clinical eligibility criteria be implemented with reasonable flexibility. This reasonable flexibility must extend to two aspects of implementation: 1) flexibility for adjudicating whether patients can meet arbitrary cutoffs for clinical characteristics based on clinical trial enrollment criteria; and 2) flexibility for patients entering the payer's system who are currently taking the drug but who do not appear to meet the payer's clinical eligibility criteria. For these patients, flexibility is shown by ensuring that there is an adequate (minimum 60-day) grace period during which their coverage is not interrupted while full consideration can be given to whether the patient merits ongoing coverage. It is very important to note, however, that drug makers themselves must agree to "grace period" terms when they negotiate formulary placement with payers. Drug makers seeking preferential formulary placement must be willing to accept terms that allow payers to provide grace periods to new plan entrants.

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.

Robust appeals procedures: Appeals of coverage denials due to patients not meeting clinical criteria for coverage should be readily available and handled within a short timeframe commensurate with the clinical seriousness of the condition. Clinical experts in the relevant field should be consulted as part of the internal adjudication process. In the case of emergencies, decisions should be made within 24 hours of the request and

for non-emergencies, 72 hours is a reasonable time.

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.

Economic Step Therapy and Required Switching

Ethical Goals for Access

As with the use of clinical eligibility criteria to narrow coverage from a broader FDA label, the introduction of economic step therapy as a means of limiting access in a coverage policy raises important questions on how to achieve fair access. As described earlier, economic step therapy policies are 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 treatment with a lower net cost to the plan sponsor 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 health care 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.

Required switching, sometimes labeled as “non-medical 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. This switching could be required at entry into a new health plan, when patients might be joining the payer with a history of taking a non-preferred brand drug, or it could even apply to patients who have been with the same health plan for some time should payer negotiations lead to a shift in which the brand drug is preferred within a drug class or treatment area.

For both economic step therapy and required switching, the ethical tension inherent in maintaining fair access becomes extremely visible to patients and clinicians. The choice of medication is here

constrained not just by differential cost sharing but by an absolute coverage on-off switch. Not surprisingly, and backed by some studies demonstrating negative effects on patient outcomes related to economic step therapy, 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.⁵⁶

With great attention to the fair design and implementation process criteria described below, we believe that there are ethical goals for access that can be supported by both economic step therapy and required switching. The ethical goals for access all address in some manner the underlying risk of harm to patients that must be addressed fully when payers are considering whether to introduce these policy mechanisms into their coverage policies. These ethical goals for access related to economic step therapy and required switching are detailed below.

Ethical Goals for Access: Economic Step Therapy and Required Switching

- 1. Given that economic step therapy policies add to the complexity and burden of health care 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.**
- 2. 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.**
- 3. 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.**

Translating Ethical Goals for Access into Fair Design Criteria

Economic Step Therapy

As with other facets of prior authorization, the essential element in translating ethical goals for access into fair design criteria is to establish safeguards whereby the goal of appropriate prescribing and cost control can be achieved without risk of significant harm to patients. In our approach to this issue, we draw heavily upon work by Nayak and Pearson³¹ 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. All must be supported by equally important implementation criteria, including the requirement for a transparent and rapid appeals process. Our design criteria require payers to justify economic step therapy policies by affirming explicitly and/or presenting evidence to document the following:

1. **Use of the first-step therapy reduces overall health care spending, not just drug spending.** Payers should affirm publicly that economic step therapy is only considered when they are incurring substantial costs from the use of one drug when the use of another drug would lead to lower overall health care costs, not just lower 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 spending on drugs.

As with the use of clinical eligibility criteria, 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 health care spending. But 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. Of course, as will be emphasized throughout this section, the use of economic step therapy to prices lower than already established fair prices can only be appropriate if patients are protected by fulfillment of all the other fair design criteria.

2. **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.** To ensure that economic step therapy policies are clinically appropriate, 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. A first-step drug could be inappropriate due to specific patient clinical characteristics, including disease severity, comorbidities, or concomitant prescriptions. Importantly, economic step therapy might also be considered inappropriate because the proposed first-step therapy, although producing comparable overall health benefits, might offer a distinctly different set of side effects that are valued very differently by individual patients.
3. **Patients have a reasonable chance to meet their clinical goals with first-step therapy.** The burden on clinicians and patients of requiring them to accept a first-step drug that is not their first choice should be acceptable only if patients have a reasonable chance of having their clinical needs met with the designated first-step drug. What threshold represents a “reasonable” chance of meeting patients’ clinical needs depends on the clinical context. If the clinical consequence of treatment failure is minimal, e.g., in the treatment of toenail fungus, then fair design criteria would allow a low threshold of chance of success with the first-step drug. However, if the clinical condition has a greater impact on patients’ quality of

life, such as severe psoriasis, then instituting economic step therapy only if there is a higher likelihood of success with the designated first-step therapy would be more reasonable. In either situation, a lower chance of success can be accepted if there are no risks of serious side effects, whereas higher chances of success would be needed in order to justify economic step therapy among drugs with side effects that could cause significant short-term harm.

4. **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.** Economic step therapy can only be appropriate if failure on the first-step therapy allows for rapid transition to another option with minimal risk that the preliminary treatment failure will cause negative long-term consequences. Consideration should be given to the risks of any wash-out period that might be required in the transition from a first step to a second step agent. 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. But 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 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.

5. **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 be consistent with the ethical goals for access, 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. This includes trials of the first step drug under a previous payer. It is true that patients can sometimes be re-challenged with a drug and have better outcomes, but it is unreasonable to seek cost savings by asking patients to bear the risks of trying the first-line drug again. 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, but it can be reasonable to require that patients receive a full, clinically appropriate dose and duration of a less expensive medication before receiving coverage for a more expensive alternative.

Finally, as noted in the section on fair design criteria for cost sharing, we believe that higher cost-sharing requirements should not always apply for patients who try the first-step therapy but must advance to the second-line agent due to side effects or lack of effectiveness. As described, we

believe that if the second-step agent is priced over a fair price standard, then higher cost sharing would be reasonable, even though the patient has been a good soldier. But if the second-step agent is fairly priced, then the ethical goals of fair access would suggest that it should be on the lower/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.

Required Switching

Required switching involves consideration of many of the same issues for fair design criteria. But because patients are already taking a medication and likely satisfied with the drug's clinical performance, the ethical burden on payers to justify a required switch is higher than it is for economic step therapy at the point of a patients' choice of a first drug. For many patients, it can be a clinical odyssey to find the right treatment that works well for them, and so the anxiety they may feel at needing to try something new, and the risk for real harm should the new drug not work well, are both factors that deserve such weight that the scope of appropriate required switching and step policies will be quite narrow.

We believe that the same general fair design criteria proposed for economic step therapy apply to required switching policies, including those policies that would require patients to switch to a new first-step therapy when joining a new payer. The key differences lie in the requirements for the new therapy: given that patients are already receiving satisfactory clinical benefits, the intended new drug must meet more stringent requirements to assure that switching poses minimal risks. Therefore, the set of fair design criteria for required switching requires payers to justify their policies by affirming explicitly and/or presenting evidence to document all the following:

1. **Use of the required switch therapy reduces overall health care spending, not just drug spending.**
2. **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.** As noted earlier, because patients are already being treated with a drug they have selected with their clinician, a required switch policy should involve drugs whose risks and benefits are identical or differ in ways that present minimal differences in serious risk and side effect profiles. In general, this design criterion can be met only with required switching among drugs in the same drug class, which have the same mechanism of action and therefore identical or nearly identical side effect profiles. Switches between brand and generic formulations of the same drug would always be able to meet this criterion, and we believe that switches between branded biologics and biosimilars would also be considered appropriate. In contrast, 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 to the index therapy.

3. **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 socio-economic factors.** For some patients there will be substantial clinical or socio-economic consequences of switching among oral, injectable, or IV infusion treatments. For example, some patients will have chosen an IV 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. Payers considering requiring patients to switch to a drug with a different route of administration should therefore reflect on the diversity of patient needs and affirm that the switch would adversely affect a very small number of patients, if any.

4. **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.**

In summary, we propose the Fair Design Criteria below for economic step therapy and required switching policies.

In order to justify economic step therapy policies as appropriate, payers should explicitly affirm or present evidence to document *all* of the following:

- Use of the first-step therapy reduces overall health care spending, not just drug spending.
- 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.
- Patients will have a reasonable chance to meet their clinical goals with first-step therapy.
- 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.
- 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:

- Use of the required drug reduces overall health care spending.
- 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.
- 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 socio-economic factors.
- 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.

Implementation Criteria: Economic Step Therapy and Required Switching Policies

As noted earlier, there are several critically important implementation process criteria that must accompany the design of economic step therapy and required switching policies.

<p>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 non-medical step therapy or switching policies.</p>
<p>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.</p>
<p>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.</p>
<p>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 hours 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.</p>
<p>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.</p>
<p>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.</p>

Prescriber Qualification Restrictions

Ethical Goals for Access

Utilization management protocols can include requirements that clinicians have certain qualifications in order to prescribe the drug. Sometimes these requirements relate to institutional features, such as the availability of certain surgical or intensive care services that may be needed immediately to address complications of care. More often, however, consideration is given to whether coverage will only be provided for prescribers with specialty training in care of the relevant condition or some other qualification that would increase the likelihood that the prescription and the overall care of the patient will be managed appropriately.

In some cases, adding a prescriber qualification can minimize the need for detailed clinical eligibility criteria, the assumption being that specialists will be able to identify suitable candidates for the drug without tight management of the process. When used in this fashion, it is possible for prescriber qualifications to reduce the administrative burden of a prior authorization protocol. Requiring specialist training for prescribers may also help ensure that accurate diagnosis, that important comorbidities are recognized and managed, that patients are counseled about the risks and benefits of the treatment and other options, and that long-monitoring for potential harms as well as benefits is performed appropriately.

Despite these potential benefits, these policies also may constrain access to the treatment in ways that harm patients. Seeing specialists often has a higher cost sharing requirement and may prove a burden over multiple visits. Patients in isolated rural areas may have to travel significant distances to gain access to certain specialists, and even in urban centers it can be challenging to find specialists who are taking new patients or who have reasonable access for their existing patients. This is particular concern for patients with insurance coverage featuring narrow provider networks. The ethical goals for access for prescriber qualifications, therefore, require that the potential negative impact on access be recognized, that restrictions on prescribers be done only when the perceived clinical benefits outweigh the narrowing of coverage, and when there are clear options available for patients for whom direct access to a specialist clinician is not feasible.

This leads us to the following ethical goal for access:

Ethical Goal for Access: Prescriber Qualification Restrictions

- 1. In order to maintain broad access, restrictive qualifications for prescribers should be designed not to reduce utilization but used only when necessary to ensure appropriate patient selection, adherence to evidence-based guidelines, dosing,**

monitoring for side effects, and overall care coordination.

Translating Ethical Goals for Access into Fair Design Criteria

To translate this ethical goal for access into fair design criteria, it is necessary to detail the characteristics of the clinical context that would make the restriction on access prudent from a clinical perspective. In addition, if the ethical goal is to improve the appropriateness of overall care, then fair design criteria should include the alternative of requiring the prescribers be a specialist or attest that they are caring for the patient “in consultation with” a clinician with relevant specialty training. With these elements in mind we propose the fair design criteria:

Restrictions of coverage to specialty prescribers are reasonable when payers explicitly affirm one or more of the following justifications:

- Accurate diagnosis and prescription require specialist training, with the risk that non-specialist clinicians would prescribe the medication for patients who may suffer harm or be unlikely to benefit.
- Determination of the risks and benefits of treatment for individual patients requires specialist training due to potential for serious side effects of therapy.
- Dosing, monitoring for side effects, and overall care coordination require specialist training to ensure safe and effective use of the medication.

Requiring that non-specialist clinicians attest they are caring for the patient in consultation with a relevant specialist is a reasonable option when the condition is frequently treated in primary care settings but some elements of dosing, monitoring for side effects, and/or overall coordination of care would benefit from specialist input for many patients.

Implementation Criteria: Prescriber Qualification Restrictions

As with other elements of prior authorization protocols, there are several implementation process criteria that are important complements to the fair design criteria.

Transparency of policies to consumers prior to health plan selection: Individuals considering health plan enrollment should be able to easily find information related to coverage criteria, including prescriber qualifications, for drugs that they or family members are currently taking.

Transparency of policies to clinicians and patients during care: Clinicians and patients should be able to rapidly determine whether there is a restriction on prescribing for any treatment. Insurers should provide ready assistance to primary care clinicians seeking connection with a relevant specialist for consultation as needed.

Reasonable flexibility: Application of prescriber qualification restrictions should show flexibility if the requested treatment is for a serious condition for which there are no other treatment options.

Implementation of prescriber qualification restrictions 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 provider qualification restrictions are addressed.

Robust appeals procedures: Appeals of prescriber qualification restrictions 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 hours of the request and for non-emergencies, 72 hours is a reasonable time.

Updating with new evidence: Many drugs are initially prescribed primarily by specialists and then, as experience accumulates, primary care clinicians begin to become more competent in their use. Insurers should track the evolution of clinical practice to determine when prescriber qualifications may be broadened for drugs that were initially covered only for specialist prescribers. Reconsideration of the need for prescriber qualification restrictions should be performed annually at a minimum.

Evaluation of impact: Insurers should regularly evaluate 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.

Conclusion

With ongoing attention to determining and achieving fair drug pricing, there is an equal if not greater need for further policy analysis of what it means to ensure fair access to pharmaceuticals for all patients. We have addressed a small part of that question, focusing on the most controversial elements of drug coverage: cost sharing and the development and implementation of certain utilization management protocols. What we have sought to describe is how these insurance features represent an attempt to manage the inherent ethical tension between prudent cost control and the freedom for clinicians and patients to access a wide array of options based solely on what might have some potential benefit to the individual. Whether that tension is managed appropriately will always be contestable, but 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 with which patient groups, clinicians, and all participants in the health system can use to engage in discussions around the appropriateness of benefit designs and coverage policies for all forms of pharmaceuticals.

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 health care 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. We wish to acknowledge and thank the participants in the ICER Policy Leadership Forum, and the special invited representatives from patient and clinician groups, who helped inform this effort. None should be viewed as supporting the analysis or proposed design criteria presented in this paper, and all errors in the paper are solely the responsibility of the authors. All deserve our thanks and our praise for their honesty and willingness to pursue a common goal from different starting points.

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Appendices

Appendix A. Cost Sharing by Plan Type

Cost Sharing under Medicaid

Medicaid now covers one in five Americans and serves very diverse populations. Over two-thirds of Medicaid beneficiaries are enrolled in private managed care plans that contract with states to provide comprehensive services, and others receive their care in the fee-for-service system.⁵⁷ Benefit designs for Medicaid drug coverage are relatively fixed in statute. States have the option to establish cost-sharing requirements for their Medicaid enrollees, but maximum out-of-pocket costs are strictly limited. To encourage the use of lower-cost drugs, states are allowed to establish different copayment levels for generic versus brand-name drugs or for drugs included on a preferred drug list. At the high end, for people with incomes above 150% of the federal poverty limit, copayments for non-preferred drugs may be as high as 20% of the cost of the drug.⁵⁸ For people with income at or below 150% the federal poverty level, copayments are limited to nominal amounts.

Cost Sharing under Medicare

Medicare Part B

Medicare Part B covers drugs administered by providers and those at dialysis centers. Medicare beneficiaries have an annual deductible (\$185 in 2019) and 20% coinsurance. There is no out-of-pocket maximum for traditional Medicare Part B, but Medicare Advantage plans have an annual maximum of \$6,700 for drugs and other services received in-network and \$10,000 for out-of-network services.

Medicare Part D

Medicare drug plans through Part D are offered by insurance companies and other private companies approved by Medicare. Beneficiaries can get coverage through Medicare Prescription Drug Plans (sometimes called PDPs) or through Medicare Advantage Plans.⁵⁹ Medicare Advantage Plans use a variety of combinations of deductibles, copayments, and coinsurance, but standard PDP coverage includes a deductible (up to \$415 in 2019), followed by an initial coverage phase with 25% coinsurance. However, virtually all PDP plans place drugs costing \$670 or more per month on a specialty tier where they may be subject to up to 33% coinsurance during this phase.⁶⁰

Once total (patient plus plan) prescription spending hits an initial coverage limit (\$3,820 in 2019), beneficiaries enter a coverage gap, or “donut-hole” phase. Whereas beneficiaries were originally responsible for 100% of their drug costs during this phase, the ACA has gradually reduced this obligation by requiring drugmakers to provide a discount on brand-name medications. Today,

beneficiaries pay 35% coinsurance for brand-name medications during the donut hole; the recent Bipartisan Budget Act of 2018 has now reduced this to 25%.

During the coverage gap, once out of pocket spending reaches a catastrophic coverage limit (\$5,100 in 2019), beneficiaries are required to pay 5% coinsurance for the remainder of the coverage year. The annual benefit resets on January 1 of the following year and the cycle begins again.⁶¹

Cost Sharing in ACA State Exchanges

Health insurance plans available through the ACA marketplace are widely variable in terms of their cost-sharing provisions for drug coverage, with great flexibility allowed to use different levels of deductibles, copayments, and coinsurance. Plans are standardized, however, on an actuarial value into “metal” tiers—bronze, silver, gold, and platinum—ranging from most to least potential exposure to out-of-pocket costs. For example, a bronze plan has an actuarial value of approximately 60%, meaning that the insurer expects to pay for 60% of total costs associated with covered medical services, including drugs, and that—as a group—the enrollees would pay for the remaining 40% of total costs through their combined copayments, coinsurance, and deductibles. All patients in the exchanges share the same maximum out-of-pocket limit established by the ACA, which in 2019 was \$7,900 for individual coverage and \$15,800 for family coverage.⁶²

The majority of bronze and silver plans have what are called “combined” deductibles, meaning that there is a single deductible for both medical services and prescription drugs. The plan typically will not begin covering most medical or prescription services until the deductible has been met. The average combined deductible for 2019 was nearly \$6,258 for bronze plans and \$4,375 for silver plans.⁶³ But most gold and platinum plans, and many silver plans, have separate medical and drug deductibles (or, in some cases, have no deductible for prescriptions). Enrollees in plans with separate deductibles will begin to receive payment towards their prescriptions once they meet their prescription deductible (or immediately if there is no deductible) even if they have not yet met their medical deductible. Many silver plans with separate deductibles (58%) and most gold and platinum plans with separate drug deductibles (65% and 93%, respectively) have \$0 drug deductibles and, therefore, begin to pay toward prescriptions immediately.⁶⁴ Patients pay the copayments and coinsurance within the terms of the plan.

Cost Sharing in Commercial Insurance Plans

Cost sharing is increasingly used by plan sponsors and payers to manage drug spending and overall health care costs.⁶⁵ Back in the 1990s, most plans utilized a two-tier copay system and the average difference between brand and generic copays was \$5. The latest PBMI survey findings reported that only 12% of commercial plans had one- or two-tier formularies; the majority of health plans (52%) had a three-tier formulary, and 38% reported having four or more tiers. In addition, 56% of respondents reported using a separate cost-sharing tier for specialty drugs.²⁰

Specific tier placement for individual drugs may be based on safety and clinical effectiveness, acquisition cost, and the availability of alternative options. Cost sharing takes the form of a range of flat copay values (e.g., \$5 for generics, \$25 for preferred brand name products, and \$40 for non-preferred brand name products) or varied coinsurance that is tied to the drug's cost (e.g., 10% for generics, 25% for preferred brand name products, and 35% for non-preferred brand name products).²⁰

Appendix B. Formulary Development

Summarizing the Formulary Development Process

Conceptually, formulary development can be viewed as an integration of three basic elements.

Evidence Assessment Team

First, there is a process of evidence assessment focusing, at least initially, on clinical evidence. This process is led by a team within the payer organization that usually includes clinical pharmacists, and sometimes clinicians. This team prepares a review of each new drug as it reaches the market, bringing together information from the FDA label, clinical guidelines from specialty societies, and the available published literature. Members of this team may have considerable expertise in evidence evaluation, and will perform an analysis of the strength of evidence on safety and effectiveness, as well as present a view on the potential role in therapy that the drug is envisioned to have. Some clinical assessment teams will use a formal evidence rating system for safety and/or effectiveness, and often will develop a formal recommendation on formulary placement and any need for prior authorization protocols to guide later discussions by the P&T Committee.

Clinical evidence assessment is dominated by considerations of safety and clinical effectiveness. Many payers have an explicit firewall between this process and later consideration of cost and other financial aspects of formulary development. But some payers integrate cost considerations within this first step in the process, using early discussion of rebates and other cost issues to inform recommendations to the P&T Committee for tier placement and any use of prior authorization protocols, especially non-medical step therapy.

P&T Committee

The work of the clinical evidence assessment team feeds into the deliberation and decisions made by a P&T Committee. All payers, both public and private, are required to have P&T Committees comprised of external experts in clinical practice, and many P&T Committees also have members with expertise in clinical epidemiology, biostatistics, and other methodological foundations of evidence review.^{21,29} The goal of requiring a P&T Committee comprised of non-payer staff is to ensure that formulary placement and drug policy decisions are developed objectively with a foundation in evidence-based medicine.^{21,24}

Payers organize their P&T Committees in different ways and have them take votes on different kinds of decisions. Some committees are asked to provide a simple three-option decision for each

drug: 1) must be on formulary; 2) optional; or 3) should not be on formulary. Other P&T Committees are involved from the first consideration of the drug in determining not only whether the drug should be listed on the formulary, but with what prior authorization protocol, including consideration of non-medical step therapy. To inform P&T Committee decisions, AMCP and CMS recommend that committees consider some or all of the following types of information: medical and clinical literature, treatment guidelines, comparative effectiveness evaluations, pharmaco-economic assessments, outcomes data, FDA-approved prescribing information, information provided by patients and physicians with experience with the treatment under review, economic data, drug and other health care cost data, and provider recommendations. CMS requires no less frequent than quarterly P&T Committee meetings for Medicaid and Medicare PDPs to ensure formularies are kept up to date.^{21,24}

Financial/Contracting Group

As noted earlier, all P&T Committees are oriented to consider clinical information as their primary guide to the decisions and recommendations they make on formulary composition. For many payers, this is institutionalized by the creation of a separate group that receives P&T Committee decisions and only then uses financial considerations to fine tune formulary tiering and prior authorization protocols. Although many staff overlap between the clinical assessment team, the payer staff who convene the P&T Committee, and this “financial/contracting” group, additional payer staff participating at this stage include those with primary responsibility for negotiating contracts with drugmakers.²¹

A common situation in which financial considerations are applied at this stage in formulary development is when more than one medication is considered to produce similar effectiveness and safety results. In this situation, the financial/contracting group will examine cost, distribution concerns, or other business factors in making a decision about tiering and prior authorization parameters such as step therapy.²¹ If prior authorization criteria are changed from those initially recommended by the P&T Committee, they must receive sign-off from the Committee before implementation.

The Role of the PBM/Plan and the Plan Sponsor

PBMs and major commercial health plans create different formularies to offer to plan sponsors, and most payers have several different versions with distinctly different structures of tiering and cost sharing. Some of these formularies will have different drugs included, or will have the same drugs but on different tiers. Plan sponsors can choose to accept one of these “standard” benefit design offerings, but can also choose to modify the cost-sharing and/or prior authorization protocols. This approach creates a “customized” benefit design and if prior authorization protocols are changed from standard designs, the approval process becomes internal to the plan sponsor and they will need appropriate processes to manage this process. The health plan contracted by the sponsor will

then administer the customized benefit. Similarly, a plan using a PBM can customize the benefit design package provided by the PBM and the modified benefit will then be administered by the PBM.

Appendix C. Principles from the Literature

Table C1. ASHP’s Guiding Principles (Adapted from ASHP’s Principles of a Sound Drug Formulary System²⁹)

<p>1. Formulary system decisions are based on scientific and economic considerations that achieve appropriate, safe, and cost-effective drug therapy.</p> <ul style="list-style-type: none"> a. Clinical decisions are based on the strength of scientific evidence and standards of practice that include, but are not limited, to the following: <ul style="list-style-type: none"> i. Assessing peer-reviewed medical literature, including randomized clinical trials (especially drug comparison studies), pharmacoeconomic studies, and outcomes research data. ii. Employing published practice guidelines, developed by an acceptable evidence-based process. iii. Comparing the efficacy as well as the type and frequency of side effects and potential drug interactions among alternative drug products. iv. Assessing the likely impact of a drug product on patient compliance when compared to alternative products. v. Basing formulary system decisions on a thorough evaluation of the benefits, risks, and potential outcomes for patients; risks encompass adverse drug events (adverse drug reactions and medication errors, such as those caused by confusing product names or labels). b. Economic considerations include, but are not limited, to the following: <ul style="list-style-type: none"> i. Basing formulary system decisions on cost factors only after the safety, efficacy, and therapeutic need have been established. ii. Evaluating drug products and therapies in terms of their impact on total health care costs. iii. Permitting financial incentives only when they promote cost management as part of the delivery of quality medical care. Financial incentives or pressure on practitioners that may interfere with the delivery of medically necessary care are unacceptable.
<p>2. The formulary system encompasses drug selection, drug utilization review, and other tools to foster best practices in prescribing, dispensing, administration, and monitoring of outcomes.</p> <ul style="list-style-type: none"> a. The formulary system: <ul style="list-style-type: none"> i. Provides drug product selection and formulary maintenance (see above). ii. Provides drug use evaluation (also called drug utilization review) to enhance quality of care for patients by assuring appropriate drug therapy. iii. Provides for the periodic evaluation and analysis of treatment protocols and procedures to ensure that they are up-to-date and are consistent with optimum therapeutics. iv. Provides for the monitoring, reporting, and analysis of adverse results of drug therapy (e.g., adverse drug reactions, medication errors) to continuously improve the quality of care.
<p>3. The P&T Committee, or equivalent body, comprised of actively practicing physicians, pharmacists, and other health care professionals, is the mechanism for administering the formulary system, which includes developing and maintaining the formulary and establishing/implementing policies on the use of drugs.</p> <ul style="list-style-type: none"> a. The P&T Committee: <ul style="list-style-type: none"> i. Objectively appraises, evaluates, and selects drugs for the formulary.

<ul style="list-style-type: none"> ii. Meets as frequently as is necessary to review and update the appropriateness of the formulary system in light of new drugs and new indications, uses, or warnings affecting existing drugs. iii. Establishes policies and procedures to educate and inform health care providers about drug products, usage, and committee decisions. iv. Oversees quality improvement programs that employ drug use evaluation. v. Implements generic substitution and therapeutic interchange programs that authorize exchange of therapeutic alternatives based upon written guidelines or protocols within a formulary system. (Note: therapeutic substitution, the dispensing of therapeutic alternates without the prescriber's approval is illegal and should not be allowed). vi. Develops protocols and procedures for the use of and access to non-formulary drug products.
<p>4. Physicians, pharmacists, and other health care professionals provide oversight of the formulary system.</p> <ul style="list-style-type: none"> a. Health care organization policies should ensure appropriate oversight of the P&T Committee and its decisions by the medical staff or equivalent body.
<p>5. The formulary system must have its own policies, or adhere to other organizational policies, that address conflicts of interest and disclosure by P&T Committee members.</p> <ul style="list-style-type: none"> a. Formulary system policies should: <ul style="list-style-type: none"> i. Require P&T committee members to reveal, by signing a COI statement, economic or other relationships with pharmaceutical entities that could influence Committee decisions. ii. Exclude product sponsor representatives from P&T committee membership and from attending P&T Committee meetings. iii. Require P&T committee members to adhere to the formulary system's policy on disclosure and participation in discussion as it relates to conflict of interest.
<p>6. The formulary system should include educational programs for payers, practitioners, and patients concerning their roles and responsibilities.</p> <ul style="list-style-type: none"> a. Formulary system policies should: <ul style="list-style-type: none"> i. Inform physicians, pharmacists, other health care professionals, patients, and payers about the factors that affect formulary system decisions, including cost containment measures; the procedures for obtaining non-formulary drugs; and the importance of formulary compliance to improving quality of care and restraining health care costs. ii. Proactively inform practitioners about changes to the formulary or to other pharmaceutical management procedures. iii. Provide patient education programs that explain how formulary decisions are made and the roles and responsibilities of the patient, especially the importance of patient compliance with drug therapy to assure the success of that therapy. iv. Disclose the existence of formularies and have copies of the formulary readily available and accessible. v. Provide rationale for specific formulary decisions when requested.
<p>7. The formulary system should include a well-defined process for the physician or other prescriber to use a non-formulary drug when medically indicated.</p> <ul style="list-style-type: none"> a. Formulary system policies should: <ul style="list-style-type: none"> i. Enable individual patient needs to be met with non-formulary drug products when demonstrated to be clinically justified by the physician or other prescriber. ii. Institute an efficient process for the timely procurement of non-formulary drug products and impose minimal administrative burdens. iii. Provide access to a formal appeal process if a request for a non-formulary drug is denied. iv. Include policies that state that practitioners should not be penalized for prescribing non-formulary drug products that are medically necessary.

Table C2. AMA Prior Authorization and Utilization Management Reform Principles (Adapted from the AMA Prior Authorization and Utilization Management Reform Principles³⁰)

<p>Clinical Validity</p> <ol style="list-style-type: none"> 1. Any utilization management program applied to a service, device, or drug should be based on accurate and up-to-date clinical criteria and never cost alone. The referenced clinical information should be readily available to the prescribing/ordering provider and the public. 2. Utilization management programs should allow for flexibility, including the timely overriding of step therapy requirements and appeal of prior authorization denials. 3. Utilization review entities should offer an appeals system for their utilization management programs that allows a prescribing/ordering provider direct access, such as a toll-free number, to a provider of the same training and specialty/subspecialty for discussion of medical necessity issues. 4. Utilization review entities should offer a minimum of a 60-day grace period for any step therapy or prior authorization protocols for patients who are already stabilized on a particular treatment upon enrollment in the plan. During this period, any medical treatment or drug regimen should not be interrupted while the utilization management requirements (e.g., prior authorization, step-therapy overrides, formulary exceptions, etc.) are addressed. 5. A drug or medical service that is removed from a plan’s formulary or is subject to new coverage restrictions after the beneficiary enrollment period has ended should be covered without restrictions for the duration of the benefit year. 6. A prior authorization approval should be valid for the duration of the prescribed/ordered course of treatment. 7. No utilization review entity should require patients to repeat step therapy protocols or retry therapies failed under other benefit plans before qualifying for coverage of a current effective therapy.
<p>Transparency and Fairness</p> <ol style="list-style-type: none"> 1. Utilization review entities should publicly disclose, in a searchable electronic format, patient-specific utilization management requirements, including prior authorization, step therapy, and formulary restrictions with patient cost-sharing information, applied to individual drugs and medical services. Such information should be accurate and current and include an effective date in order to be relied upon by providers and patients, including prospective patients engaged in the enrollment process. Additionally, utilization review entities should clearly communicate to prescribing/ordering providers what supporting documentation is needed to complete every prior authorization and step-therapy override request. 2. Utilization review entities should provide, and vendors should display, accurate, patient-specific, and up-to-date formularies that include prior authorization and step therapy requirements in electronic health record systems for purposes that include e-prescribing. 3. Utilization review entities should make statistics regarding prior authorization approval and denial rates available on their website (or another publicly available website) in a readily accessible format. The statistics shall include but are not limited to the following categories related to prior authorization requests: <ul style="list-style-type: none"> • Health care provider type/specialty; • Medication, diagnostic test, or procedure; • Indication; • Total annual prior authorization requests, approvals and denials;

- Reasons for denial such as, but not limited to, medical necessity or incomplete prior authorization submission; and
 - Denials overturned upon appeal.
 - These data should inform efforts to refine and improve utilization management programs.
4. Utilization review entities should provide detailed explanations for prior authorization or step-therapy override denials, including an indication of any missing information. All utilization review denials should include the clinical rationale for the adverse determination (e.g., national medical specialty society guidelines, peer-reviewed clinical literature, etc.), provide the plan’s covered alternative treatment and detail the provider’s appeal rights.

Timely Access and Administrative Efficiency

1. A utilization review entity requiring health care providers to adhere to prior authorization protocols should accept and respond to prior authorization and step-therapy override requests exclusively through secure electronic transmissions using the standard electronic transactions for pharmacy and medical services benefits. Facsimile, proprietary payer web-based portals, telephone discussions and nonstandard electronic forms shall not be considered electronic transmissions.
2. Eligibility and all other medical policy coverage determinations should be performed as part of the prior authorization process. Patients and physicians should be able to rely on an authorization as a commitment to coverage and payment of the corresponding claim.
3. In order to allow sufficient time for care delivery, a utilization review entity should not revoke, limit, condition, or restrict coverage for authorized care provided within 45 business days from the date authorization was received.
4. If a utilization review entity requires prior authorization for non-urgent care, the entity should make a determination and notify the provider within 48 hours of obtaining all necessary information. For urgent care, the determination should be made within 24 hours of obtaining all necessary information.
5. Should a provider determine the need for an expedited appeal, a decision on such an appeal should be communicated by the utilization review entity to the provider and patient within 24 hours. Providers and patients should be notified of decisions on all other appeals within 10 calendar days. All appeal decisions should be made by a provider who a) is of the same specialty, and subspecialty, whenever possible, as the prescribing/ordering provider and b) was not involved in the initial adverse determination.
6. Prior authorization should never be required for emergency care.
7. Utilization review entities are encouraged to standardize criteria across the industry to promote uniformity and reduce administrative burdens.

Alternatives and Exemptions

1. Health plans should restrict utilization management programs to “outlier” providers whose prescribing or ordering patterns differ significantly from their peers after adjusting for patient mix and other relevant factors.
2. Health plans should offer providers/practices at least one physician-driven, clinically based alternative to prior authorization, such as but not limited to “gold-card” or “preferred provider” programs or attestation of use of appropriate use criteria, clinical decision support systems, or clinical pathways.
3. A provider that contracts with a health plan to participate in a financial risk-sharing payment plan should be exempt from prior authorization and step-therapy requirements for services covered under the plan’s benefits.

Table C3. Graff et al. Guiding Principles in Which it is More (or Less) Acceptable to Require Higher Cost Sharing (Out-of-Pocket Costs) (Adapted from Graff et al.³²)

<ol style="list-style-type: none">1. Try and fail is important: Important to “reward the good soldier.”2. Higher out-of-pocket costs are acceptable unless higher cost treatments have statistically significant and clinically meaningful benefits as compared with treatment alternatives. If they do, they should be lower.3. Need to balance treatment benefits and harms against total health care costs, i.e., incentivizing use of higher cost medicines with lower out-of-pocket costs was appropriate when overall health care costs would reduce.4. Penalties because of “bad luck,” not preferences should be mitigated.5. Differences in out-of-pocket should be lowered but not eliminated. “OOP [out-of-pocket] cost-sharing differences were needed to incentivize patients and providers to start with lower-cost, lower-tier treatments.”
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Table C4. Nayak et al. Criteria for Ethical Design of Step Therapy (Adapted from Nayak RK et al.³¹)

<ol style="list-style-type: none">1. Weigh cost savings against long-term outcomes: need to consider impact of a step on the total cost throughout the health care system.2. Ensure that first-step drugs are clinically appropriate: review potential policies with a broad set of clinical experts and whenever possible, patient representatives.3. Give patients an excellent chance to meet clinical goals: patients need to have a reasonable chance of finding success with the drug they are forced to step through.4. First-step failure should not cause long-term harm: need to consider the reversibility and extent of harm possible by forcing patients to step through a drug they might fail.5. Opting out on clinical grounds should be quick and easy.6. Clearly define “failure.”7. Rapidly review new evidence.8. Rationale and roles should be explicit and transparent.
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Table C5. Robison et al. A Framework for Linking Price and Improved Patient Access (Adapted from Robison et al. [2018])

<ol style="list-style-type: none">1. Drugs priced at or below the value-based levels should face streamlined and more modest prior authorization, step therapy, and cost-sharing requirements.2. Drugs priced above value-based benchmarks can continue to encounter more stringent requirements.3. Drugs charging value-based prices should be placed in the preferred formulary tier without coinsurance.

Appendix D. Ethical Goals for Access and Fair Design and Implementation Criteria for Drug Coverage

1. 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.
 2. Cost sharing should not be structured primarily to shift health care costs to patients when they have few or no lower cost options that are medically appropriate.
 3. 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 IRS as high-value therapies should receive pre-deductible 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.
- A. **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.
 - B. **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.

1. Payer evaluation of treatments should be expeditious to minimize delays in access. Evaluation of drugs that address severe conditions without alternative treatment options should be started before FDA approval whenever possible.
 2. The amount of time taken to develop and implement prior authorization protocols should not be used as an indirect method to reduce utilization or to divert clinicians and patients to other insurers.
- “New-to-market” evaluation procedures needed to develop formal coverage policy should be structured to be completed within three months of FDA approval unless the manufacturer has announced it will delay the drug’s launch.
 - For treatments of rapidly progressive and/or fatal conditions for which there is no alternative approved therapy, payers should implement one of two approaches:
 - Coverage should be provided immediately following FDA approval using FDA label language; or
 - Evaluation procedures should begin prior to FDA approval so that tailored coverage policies are available at the time of FDA approval.
 - During any early time period after FDA approval when prior authorization protocols have not yet been determined, clinicians should be able to rapidly seek coverage approval based on the FDA labeled language without documentation requirements that lead to substantial burden or delay.
- A. **Transparency:** Clinicians and patients should be able to rapidly determine the status of new-to-market drugs and procedures to request coverage.
 - B. **Robust appeals procedures:** Procedures to request appeals of non-coverage decisions during the phase between FDA approval and formal coverage policy should be transparent, easy to pursue, and expeditious. Adjudication of coverage during the interim should be based on application of language in the FDA label and should be managed by staff with good access to clinicians whose training and experience is in the same or a similar specialty.

1. 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: 1) when necessary to protect patients from inappropriate use of treatments; or 2) when necessary to manage costs of expensive interventions and to negotiate lower prices for drugs that are priced beyond a fair price range.
 - 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:
 - Considered limitations of evidence due to systemic under-representation of minority populations; and
 - 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
 - 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.
 2. The administrative burden of documenting clinical eligibility should be streamlined and transparent to avoid creating a significant barrier to appropriate care.
 3. Clinical eligibility criteria should not extend beyond reasonable use of clinical trial inclusion/exclusion
 - **For all drugs:** Clinical eligibility criteria that complement the FDA label language may be used to:
 - Set standards for diagnosis; and/or
- A. **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.
 - B. **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.
 - C. **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

criteria to interpret or narrow the FDA label language in a way that disadvantages patients with underlying disabilities unrelated to the condition being treated.

4. Arbitrary eligibility cutoffs used in pivotal clinical trials for age, severity, or other clinical characteristics should be subject to reasonable flexibility in coverage determinations.

5. 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.

- 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:
 - 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; AND
 - 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; AND
 - 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 that 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.

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.

D. **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.

E. **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

- **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.

patient groups on the impact of restrictions on care and on patient outcomes.

1. Given that economic step therapy policies add to the complexity and burden of health care 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

- 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 health care 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.

A. **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 non-medical step

- design and implementation criteria are met.
2. 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.
 - 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.
 3. 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 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 health care 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 socio-economic 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.
- therapy or switching policies.
- B. **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.
 - C. **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.
 - D. **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 hours 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.

- E. **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.
- F. **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.
- A. **Transparency of policies to consumers prior to health plan selection:** Individuals considering health plan enrollment should be able to easily find information related to coverage criteria, including prescriber qualifications, for drugs that they or family members are currently taking.
- B. **Transparency of policies to clinicians and patients during care:** Clinicians

- 1. In order to maintain broad access, restrictive qualifications for prescribers should be designed not to reduce utilization but used only when necessary to ensure appropriate patient selection, adherence to evidence-based guidelines, dosing, monitoring for side
 - Restrictions of coverage to specialty prescribers are reasonable when payers explicitly affirm one or more of the following justifications:
 - a) Accurate diagnosis and prescription require specialist training, with the risk that non-specialist clinicians would prescribe the medication for patients who may suffer harm or be unlikely to benefit.
 - b) Determination of the risks and benefits of treatment for individual patients requires specialist training due to potential for serious side

effects, and overall care coordination.

effects of therapy.

- c) Dosing, monitoring for side effects, and overall care coordination require specialist training to ensure safe and effective use of the medication.
- Requiring that non-specialist clinicians attest they are caring for the patient in consultation with a relevant specialist is a reasonable option when the condition is frequently treated in primary care settings but some elements of dosing, monitoring for side effects, and/or overall coordination of care would benefit from specialist input for many patients.

and patients should be able to rapidly determine whether there is a restriction on prescribing for any treatment. Insurers should provide ready assistance to primary care clinicians seeking connection with a relevant specialist for consultation as needed.

- C. **Reasonable flexibility:** Application of prescriber qualification restrictions should show flexibility if the requested treatment is for a serious condition for which there are no other treatment options.

Implementation of prescriber qualification restrictions 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 provider qualification restrictions are addressed.

- D. **Robust appeals procedures:** Appeals of prescriber qualification restrictions 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 hours of the request and for non-emergencies, 72 hours is a reasonable time.

- E. **Updating with new evidence:** Many drugs are initially prescribed primarily by specialists and then, as experience accumulates, primary care clinicians begin to become more competent in their use. Insurers should track the evolution of clinical practice to determine when prescriber qualifications may be broadened for drugs that were initially covered only for specialist prescribers. Reconsideration of the need for prescriber qualification restrictions should be performed annually at a minimum.
- F. **Evaluation of impact:** Insurers should regularly evaluate 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.

Appendix E. 2019 ICER Policy Summit Attendees

Representatives from the following companies and organizations attended ICER's 2019 Policy Summit, which was held from December 4-6, 2019 in San Diego, CA:

- Allergan
- America's Health Insurance Plans
- Alnylam Pharmaceuticals
- Anthem Blue Cross Blue Shield
- American College of Rheumatology
- AstraZeneca
- Biogen
- CVS/Caremark
- Blue Shield of California
- Editas Medicine
- Boehringer Ingelheim
- Express Scripts
- Familial Hypercholesterolemia Foundation
- Harvard Pilgrim Health Care
- Genentech
- Health Care Service Corporation
- GlaxoSmithKline
- Health Partners
- LEO Pharma
- Mallinckrodt
- Kaiser Permanente
- MedSavvy
- Merck
- Novartis
- National Pharmaceutical Council
- National Psoriasis Foundation
- Premera Blue Cross
- Prime Therapeutics
- Singh Healthcare Advisors
- Regeneron
- UnitedHealthcare
- Sanofi
- University of Chicago