

PIPC Patient Blog: Formulary Restrictions Devalue And Endanger The Lives Of Disabled People

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By Ari Ne'eman

As policymakers around the world seek to manage rising drug expenditures, people with disabilities find ourselves increasingly concerned by the potential harms that cost-cutting measures may bring. The growing fight between health care purchasers and drug manufacturers offers yet another instance where people with disabilities and chronic conditions may find themselves caught in the cross-fire.

CVS Caremark's [recently announced policy](#) allowing self-insured employers to exclude new drugs from their formularies based on a flawed cost-effectiveness measure has brought this conflict into the spotlight. We can better understand the threat this policy poses by examining the effects of the United Kingdom's use of the same measure in drug coverage decisions. It is also important to understand that this is not an isolated threat for disabled people – proposed changes to Medicare and Medicaid drug coverage rules also imminently risk endangering patients by introducing restrictive access policies that devalue disabled lives.

The Trouble With QALYs

CVS Caremark recently announced that the company will introduce new cost-effectiveness thresholds based on the concept of Quality Adjusted Life Years (QALYs). Self-insured employers contracting for Caremark's services as a Pharmaceutical Benefit Manager can choose to exclude new drugs from their formularies if they are launched at a price that exceeds \$100,000 per QALY. The measure – which has its [detractors](#) and [defenders](#) – has sparked significant backlash from the disability community.

QALYs are a measure of cost-effectiveness intended to quantify the extent to which a treatment

extends life and improves health. To measure the latter, a QALY analysis uses “[disability weights](#),” which reduce the value of a treatment’s life-extending properties if they do not also succeed in returning a person to perfect health. These disability weights are typically calculated by surveying members of the mostly non-disabled general public and asking them how much life they would be willing to sacrifice to avoid acquiring a disability.

In the words of Princeton bioethicist Peter Singer, advocating for the adoption of QALYs in a magazine piece entitled *Why We Must Ration Health Care*, “If...a year with quadriplegia is valued at only half as much as a year without it, then a treatment that extends the lives of people without disabilities will be seen as providing twice the value of one that extends, for a similar period, the lives of quadriplegics.” Such an approach has disability rights advocates justifiably worried.

CVS Caremark may counter that the company intends to use QALYs only to contain costs, not to discriminate. Unfortunately, the discriminatory nature of the QALY is built into its very structure. Cost-per-QALY calculations inherently privilege treatments that extend the lives of those who can be restored to perfect health, and disadvantage the many who seek life-extending treatments despite having a disability or chronic condition that is not curable.

This subjective valuing of non-disabled lives over disabled lives has historically prevented the adoption of the QALY by public payers in the United States. When Oregon attempted to introduce a QALY-based rationing system in 1992, the federal government [denied](#) them permission to proceed, citing the newly passed Americans with Disabilities Act and its non-discrimination mandate. Similarly, disability rights advocates successfully secured a prohibition on the use of QALYs in Medicare in the Affordable Care Act. Though not legally barred, large-scale, systematic use of QALYs in treatment coverage decisions by private payers is also unprecedented in the United States.

The United Kingdom’s [National Institute for Health and Care Excellence](#)(NICE), responsible for determining which new treatments will become available through the British National Health Service, provides a clear example of the perils of QALY-based systems. Like CVS Caremark’s plan, NICE’s system rests on set price-per-QALY benchmarks. NICE recently denied three groundbreaking, disease-modifying treatments for rare, complex conditions on these grounds: Orkambi for the most common form of cystic fibrosis, Spinraza for spinal muscular atrophy, and ocrelizumab for primary progressive multiple sclerosis.

All three drugs work by slowing irreversible organ damage and cell death. While they can and do improve current symptoms, their greatest promise is in halting or delaying disease progression. This has especially concerning implications when QALY-based systems are used to determine treatment availability. These determinations disadvantage patients who are already significantly affected. Specialty drugs may still be able to add years to these patients’ lives, but NICE and other QALY-based systems discount the value of each of these years. Their calculations treat each actual added year as more costly than the same year would be for a person in better health.

This judgment is not based on the perceptions of people with disabilities about their quality of life. Instead, funding bodies rely on surveys measuring the general public’s hypothetical

preferences between years of life spent in different states of illness and disability. A telephone survey that asks a member of the public if they would rather live ten years in a wheelchair or five years without one is not a sound basis on which to ground public policy.

While some QALY frameworks try to mitigate this [by surveying people with disabilities](#) themselves, this presents other problems. If people with disabilities choose to value their lives as equally worthy of life-sustaining care to that of non-disabled people, the QALY framework then is less likely to recommend coverage of drugs and other medical interventions that address functional impairment or otherwise mitigate the negative aspects of a condition. There is no inherent reason why life-extension and improved function have to be pitted against each other – the QALY system sets up an arbitrary choice that punishes disabled people for the natural desire to have access to life-sustaining treatment.

For now, the relatively limited use of QALYs in the United States means Americans are shielded from the worst of this system. However, this protection is growing thin. CVS has indicated that they will exempt from QALY-based formulary exclusions any drug that receives the FDA’s [“breakthrough therapy”](#) designation – a category that includes Spinraza and Orkambi. This measure is likely due to the legal and public relations challenges of using a discriminatory methodology to deny medications that are documented to provide substantial benefits over existing therapies for serious conditions. But CVS [has indicated](#) that the current policy represents a starting place – the company may reconsider that carve-out as the health care system gets accustomed to QALYs. These concerns are precisely why over 90 disability groups recently objected to the new policy in a [letter](#) organized by the Partnership to Improve Patient Care (a coalition for which I am proud to serve as an adviser).

Medicare And Medicaid Formulary Restrictions

CVS Caremark’s new policy comes from the private sector – but similarly concerning proposals are coming from the public sector. While the government’s use of QALYs is restricted in Medicare, the Trump Administration has proposed other measures that would attempt to moderate federal drug spending by restricting access for people with disabilities and chronic health conditions.

Last month, the Centers for Medicare and Medicaid Services (CMS) [announced](#) that the agency would grant unprecedented flexibility to Medicare Advantage insurance plans, which serve over 20 million people, to impose step therapy on certain drugs. This policy, a follow up to the [Trump Administration’s “American Patients First” blueprint](#), would allow insurers to require patients to try and [fail first](#) with cheaper medications before being able to access a more costly drug - even if the latter was prescribed to them by their physician.

Like QALY-based coverage decisions, step therapy is grounded in deciding which treatments are most valuable, on average. Unfortunately, the research used to inform “step therapy” decisions often excludes or underrepresents people with co-occurring conditions and disabilities, along with other marginalized groups. Such determinations often fail to acknowledge side effects and differences in effectiveness for populations that are far from average.

Efforts to impose formulary restrictions within Medicaid are also accelerating. Responding to

one state request, CMS [indicated that](#) the agency is open to providing “[flexibility to exclude specific drugs from coverage based on cost- effectiveness or other approved criteria](#).” Additionally, [President Trump’s FY2019 budget proposal](#) calls for a new five-state demonstration program to allow states to “make drug coverage decisions that meet state needs” by withdrawing from the Medicaid Drug Rebate Program, which requires Medicaid to cover a broad array of medications. Health policy experts [have rightly criticized this proposal](#), noting that if it produces cost-savings, “it would likely only be the result of states using the new authority to impose a closed formulary to unduly restrict access to needed high-cost drugs, including those with high clinical value.”

To be clear, utilization management tools should play a role in our health care system. Prior authorization requirements can help ensure that drugs with dangerous side effects are used in a targeted fashion for the populations most likely to receive clinical benefit. Data shows that psychotropic drugs are frequently used as a form of chemical restraint on [children in foster care](#) and [people of all ages with developmental and psychiatric disabilities](#). State Medicaid prior authorization requirements for atypical antipsychotics [show some promise](#) in reducing inappropriate use. There is likely room for expansion of this form of utilization management, when focused on protecting patients rather than saving insurers money.

But these proposed Medicare and Medicaid policy changes represent something very different. Instead of leveraging utilization management to improve the quality of care, they use it to cut costs by restricting access to medications necessary for many people with disabilities. Even where U.S. law restricts the ability of payers to engage in outright denials, formulary restrictions still threaten to choke people with disabilities in a sea of paperwork to access medications off the formulary. It is hardly fair or just to add to the extensive labor people with disabilities and chronic health conditions face in getting needs met, particularly for low-income Americans on Medicaid.

A Call To Action

Like efforts to grant states additional “flexibility” to impose work requirements and increase cost-sharing for Medicaid beneficiaries, giving states the ability to restrict access to medically appropriate medications would weaken Medicaid’s underlying purpose: to improve health and well-being. Rather than more measures to restrict access to care, we need a strong coalition fighting to ensure that public and private payers cover services that people with disabilities need to survive and thrive.

In the U.K. and other countries using QALYs to determine which treatments to fund, we see groups of people with disabilities, families, and providers focused around one specific condition, fighting against denials on a case-by-case basis. Because the most expensive specialty drugs are almost always for orphan diseases, these groups are generally small and have limited impact. A dialogue of scarcity and competition for limited resources undermines coalition building among patient groups. Each success is isolated. The next denial will be fought by a different small, isolated group of patients and allies.

We are not yet in this position in the U.S. We can choose alternative strategies to tackle the issue of high prescription drug costs. We do not have to choose a system that endangers and

devalues people with rare and complex conditions. We can form a powerful coalition among the small individual communities at risk now, before we are silo-ed by a system that treats us as competitors for scarce resources.

People with disabilities deserve better than to be held hostage in the growing fight between payers and pharmaceutical manufacturers. As important as moderating drug expenditures may be, we need our policymakers to pursue policies that can accomplish this without threatening the well-being of the most marginalized people in our health care system.