SUBMITTED ELECTRONICALLY

February 23, 2026

Mehmet Oz, MD, Administrator
Centers for Medicare & Medicaid Services
U.S. Department of Health and Human Services
7500 Security Blvd
Baltimore, MD 21244

Re: Guarding U.S. Medicare against Rising Drug Costs Model (GUARD)
CMS-5546-P

Dear Administrator Oz:

The Protecting Innovation in Rare Cancers (PIRC) coalition, together with the undersigned rare disease advocacy organizations, appreciates the opportunity to comment on the GUARD Model proposed under Section 1115A of the Social Security Act. We remain committed to working with the Administration as it strives to deliver on its goals of improving the health and lives of Medicare beneficiaries, including those living with rare cancers. For the reasons discussed below, however, our organizations cannot support CMS' implementation of the GUARD model.

PIRC is a coalition of rare cancer patient advocacy organizations representing individuals living with hematologic malignancies, sarcomas, neuro-oncologic conditions, rare gastrointestinal cancers, and other low-incidence malignancies. Our organizations also recognize that the healthcare landscape extends beyond science, clinical care, and patient support. Payers and their evolving coverage, payment, and care delivery mechanisms drive the access and innovation our patient communities rely on to enhance survival and maintain quality of life.

The Medicare program has historically provided reliable access to Part B cancer therapies administered for a medically accepted indication. Part D plans have presented more of a challenge – clinicians treating rare cancer patients invariably encounter prior authorization and documentation requirements, step therapy protocols, and restrictive formulary structures, even within the "protected classes." These processes can drive treatment delays

as well as switches from a treatment plan based on shared decision making to a therapy course determined by a patient's Part D plan based on financial considerations. CMS anticipates that the GUARD Model will accrue savings, in part, through changes in plan behaviors that reduce utilization of treatments subject to the model as well as care setting shifts from Part D to Part B. As more fully detailed below, we expect that our patient communities will not experience reduced drug costs. Instead, the GUARD Model – by design – would serve as an additional force impeding access to prescribed treatments.

Chronic lymphocytic leukemia (CLL) serves as an illustrative example of the impact Part D plan incentives can have on the treatments beneficiaries are able to receive. Over the past decade, targeted oral therapies such as Bruton's tyrosine kinase (BTK) inhibitors and the BCL2 inhibitor venetoclax have transformed the treatment landscape. CLL patients now have substantially more effective and better-tolerated treatment options compared to just years ago, when chemoimmunotherapy was the standard of care. Although most patients experience an initial response, CLL is a chronic, relapsing disease. Treatment selection must be individualized based on prior therapy, response history, discontinuation reasons, biomarker characteristics, comorbidities, patient preference, and therapeutic goals.

Until last year, beneficiaries struggled to afford the high out-of-pocket costs associated with continuous BYK inhibitor therapy. The out-of-pocket cap alleviated the financial toxicity patients face and has substantially reduced the likelihood that a Medicare beneficiary would be unable to start and maintain their treatment. Unfortunately, patients and clinicians have observed increasingly burdensome access barriers to prescribed therapies — particularly within the BTK inhibitor class covered under Part D. The reimbursement landscape is evolving due to Part D redesign and phased-in manufacturer discount obligations and will continue to shift as negotiated prices are implemented for Imbruvica and scheduled for Calquence.

Plans have already adapted to their increased liability for Brukinsa (due to the phased-in discount obligation) with aggressive prior authorization and step therapy protocols that often diverge from NCCN guidelines. Several Medicare Advantage and Part D plans limit new starts to Calquence or Imbruvica unless patients demonstrate intolerance or contraindications. At least one plan appears to require patients already stable on Brukinsa to switch unless they have failed both alternative agents. NCCN guidelines explicitly state that BTK inhibitors are not interchangeable and that selection should be made by the treating clinician based on patient-specific factors.

Against this already unstable backdrop, the GUARD Model proposes an additional layer of uncertainty in the form of additional pricing control mechanisms and benchmarking constraints. The impacts CLL patients have started to experience will not only increase but likely extend to most cancer and rare disease patients relying on Medicare.

The GUARD Model Tests the Limits of CMMI Authority and Departs from Section 1115A's Prioritization of Care Coordination and Delivery Innovation

Section 1115A authorizes CMS to test models that are expected to reduce expenditures "while preserving or enhancing the quality of care furnished to individuals." 42 U.S.C. § 1315a(a)(1). This conjunctive requirement is not satisfied where credible concerns exist regarding beneficiary access, cost exposure, or clinical outcomes.

The GUARD Model modifies manufacturer rebate obligations and compares Medicare net prices to international benchmarks. It is, fundamentally, a pricing recalibration mechanism. Section 1115A(b)(2)(B) directs CMS to prioritize models that improve care coordination, improve quality, and advance patient-centered delivery reform.

In contrast to the prioritized models under Section 115A, the GUARD Model does not alter care delivery structures, coordination mechanisms, or provider incentives. It operates almost exclusively at the pricing and rebate layer. Section 1115A does not authorize savings-first experimentation. It requires that models be expected to reduce expenditures while preserving or enhancing quality. Savings alone are not sufficient. Because the GUARD model's primary mechanism is the recalibration of manufacturer liability—rather than delivery system reform—and because anticipated savings may depend on plan behavior that affects utilization and access, the model risks exceeding the spirit, if not the letter, of Section 1115A's prioritization mandate.

Section 1115A Requires Preservation of Beneficiary Access and Quality — Not Merely Retrospective Monitoring

As noted above, the CMMI statute requires that savings be achieved while preserving or enhancing quality. The GUARD proposal explicitly acknowledges that CMS will evaluate "the ways in which Part D plan benefits may change" and "whether and to what extent there are impacts on beneficiary cost sharing." That language reflects CMS' recognition that plan design and beneficiary exposure may change. In rare cancers, the impact of these changes is not

theoretical. It is already occurring due to changes in Part D plan economics from the IRA's Part D redesign.

Patients and providers report:

- Step therapy protocols that force switches among Part D drugs as well as from Part D drugs to Part B alternatives
- Denial of prescribed agents in favor of plan-preferred alternatives
- Burdensome prior authorization delays
- Delays in receiving prescribed medications
- Forced treatment changes unrelated to clinical judgment.

When formulary and utilization management decisions replace shared decision making and drive which targeted therapy a patient receives, quality preservation is directly implicated and cannot be described as neutral or an improvement. As you are likely aware, in specialty oncology, utilization management, step therapy, and tier placement can effectively function as formidable access barriers. Where these beneficiary-facing consequences are foreseeable, yet not proactively mitigated within the model's design, the statutory threshold for CMMI's waiver authority is not satisfied.

Compression of Rebate Elasticity May Reshape Plan Behavior, Premiums, and Access

MedPAC has documented that manufacturer rebates materially influence Part D plan bids and premiums. Similarly, an Avalere study has confirmed what patients have conveyed - Part D redesign already materially shifts plan liability and impacts plan behaviors with respect to utilization management and formulary design.

Introducing GUARD into this environment may compress available price elasticity at the federal level, reducing plan-level negotiation flexibility. In a highly concentrated pharmacy benefit manager (PBM) market — where the top three PBMs control approximately 80 percent of prescription volume — structural shifts in rebate mechanics can reverberate quickly and broadly through formulary decisions

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Our patient communities have already experienced step therapy protocols that appear driven by financial preference rather than NCCN-aligned clinical criteria or other evidence-based rationales. Our patient communities are justifiably concerned that adding another pricing overlay risks intensifying those pressures.

Projected Savings Are Uncertain and May Be Offset by Premium Growth, Cost-Shifting, or Reduced Access

As CMS asserts, the GUARD Model is premised on expected federal savings from enhanced rebates due to Medicare when US prices exceed international benchmarks. Unfortunately, the magnitude of savings ultimately depends on reactive and/or proactive responses from manufacturers and plans, including increased use of utilization management to reduce access to prescribed, high-cost treatment options.

If manufacturer concession capacity is compressed at the federal level through GUARD rebates, plans may respond through higher premiums, tighter formularies, or increased cost-sharing. As beneficiaries have learned within the MDPNP, savings to Medicare do not necessarily equate to savings for beneficiaries. In fact, if premium growth accelerates or formularies narrow, costs from savings to the Medicare program may be simply transferred to beneficiaries rather than reduced.

The appropriate question on economic adjustments from GUARD is not only whether GUARD reduces federal outlays, but whether it:

- Preserves beneficiary access
- Avoids premium escalation
- Prevents increased cost-sharing burdens
- Maintains a sufficient set of standalone Part D plans for beneficiaries to have a meaningful set of coverage options
- Maintains alignment with disease-specific and/or NCCN guideline–based treatment selection.

Even if GUARD generates federal rebate savings, plan bids, rebate retention, and formulary design decisions will mediate whether beneficiaries experience relief or increased burden. Moreover, cancer patients – a population specifically targeted for model inclusion – are unlikely to benefit from reduced cost-sharing due to GUARD model savings. As we have found with the Medicare Drug Price Negotiation Program (MDPNP), the IRA's cap on out-of-pocket costs under Part D has made oral cancer drugs affordable. Given the relative high cost of these treatments, negotiated prices do not impact what patients pay for their treatments. It is therefore unlikely that incremental reductions under GUARD would meaningfully alter out-of-pocket costs for rare disease and cancer patients.

Moreover, since the GUARD Model relies on plan-level coverage responses as a means of achieving savings, our patient communities face a meaningful risk of harm from inclusion in the model. CMS' reliance on monitoring and evaluation rather than patient opt-outs and/or real-time resolution of access impediments makes those potential harms more durable and potentially irreversible with respect to outcomes for individuals with a rare disease or cancer diagnosis.

We firmly believe that models developed to accrue any savings for the Medicare program that arise from or are associated with reduced access or increased beneficiary cost-sharing fail to satisfy the statutory "preservation-of-quality" requirement.

GUARD's Reliance on International Benchmarks Risks Functional Incorporation of QALY-Based Valuation Frameworks

Beyond plan behavior and premium impacts, GUARD raises a structural valuation concern. Congress has repeatedly rejected use of QALY-based valuation in federal health programs. The Affordable Care Act prohibits use of cost-per-QALY thresholds in Medicare coverage and reimbursement decisions. Similarly, the Inflation Reduction Act's MDPNP provisions explicitly prohibit CMS from using QALY-based cost-effectiveness thresholds or evidence relying on QALY methodologies in determining maximum fair prices.

The GUARD Model incorporates international benchmarks derived from OECD countries, many of which use centralized health technology assessment frameworks that incorporate QALY-based valuation. Although CMS does not directly apply QALY thresholds, incorporating benchmark prices derived from QALY-constrained national systems risks functionally embedding those valuation frameworks into Medicare.

Layering GUARD onto MDPNP and Part D Redesign Compromises Evaluation Integrity

CLL serves as an example of the combination of policy and pricing changes that will coincide with implementation of the GUARD Model:

- Implementation of MFP for Imbruvica in 2026;
- Implementation of MFP for Calquence in 2026
- Part D redesign liability shifts
- Inflation rebate enforcement

- Ongoing PBM consolidation

If premiums rise, formularies tighten, or issuers exit the Part D market, will CMS be able to isolate whether the cause is GUARD, redesign, MFP, or market adaptation? When multiple contemporaneous reforms affect the same outcome variables — premiums, utilization management, formulary design — isolating the effects of GUARD becomes methodologically challenging and may reduce the reliability and interpretability of results.

If CMS determines to move forward with GUARD, it should first clearly articulate how it will isolate GUARD-specific effects and ensure reliability and interpretability of results.

Conclusion

The GUARD Model seeks to address legitimate fiscal concerns. However, introduced amid extensive policy transition and in the context of already observed formulary tightening and access burdens, it raises serious questions on statutory fit, model test validity, and CMS' ability to maintain beneficiary access and care quality.

Patients living with rare cancers rely on individualized treatment decisions guided by clinician expertise and NCCN guidelines — not by plan-level financial preference. Given the significant uncertainties associated with GUARD, we cannot support the model and urge CMS to withdraw its proposal. If, however, the Agency determines to move forward, we recommend that It exempt cancer patients and others for whom delays in care or coverage denials can have life-threatening consequences.

We appreciate your consideration and welcome the opportunity to collaborate with you and your team on policy changes that improve care and potentially reduce costs for Medicare beneficiaries, including cancer patients.

A Cure In Sight
Amyloidosis Support Groups
Association for Creatine Deficiencies
Cancer Support Community
Chondrosarcoma Foundation
CLL Society
Cutaneous Lymphoma Foundation
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