COUNCIL FOR AFFORDABLE
HEALTH COVERAGE

August 17, 2026

Dr. Mehmet Oz
Administrator
Centers for Medicare & Medicaid Services
U.S. Department of Health and Human Services
200 Independence Avenue, S.W.
Washington, DC 20201

RE: Medicare Program: Medicare Drug Price Negotiation Program and Medicare Prescription Drug Benefit Program Proposed Rule; CMS-4215-P; RIN 0938-AV90

Submitted electronically to https://www.regulations.gov/

Dear Administrator Oz,

Thank you for the opportunity to provide comments on the Centers for Medicare & Medicaid Services' (CMS) proposed rule entitled *Medicare Drug Price Negotiation Program and Medicare Prescription Drug Benefit Program* (CMS-4215-P).

The Council for Affordable Health Coverage (CAHC) has long supported lower drug costs, increased access to drug therapies, and continued innovation in the treatment and cure of diseases. CAHC (www.cahc.net) is a broad-based alliance with a singular focus: ensuring that all Americans have access to affordable health care. We are pro-patient, pro-competition, and pro-innovation. Our member organizations include employers, medical providers, patient groups, technology companies, and pharmaceutical manufacturers.

CAHC has commented extensively on CMS's implementation of the Medicare Drug Price Negotiation Program since its inception. As CMS transitions from implementing the program through guidance to establishing permanent regulations for initial price applicability year 2029 and subsequent years, it is particularly important that CMS establish clear statutory guardrails, preserve incentives for continued pharmaceutical innovation, and provide sufficient transparency for beneficiaries and other stakeholders to understand how the government makes decisions that have significant consequences for patient access and the health care market.

CAHC's principal concern with the proposed rule is that it would expand the definition of a qualifying single source drug beyond its statutory footing while leaving significant agency determinations unexplained. CAHC offers the following recommendations:

1. Withdraw the proposed expansion of the qualifying single source drug definition for certain fixed combination products and new formulations.
2. Revise the broader qualifying single-source drug policy to recognize meaningful distinctions among separately approved products.
3. Implement the expanded orphan drug exclusion in a manner that protects continued investment in rare disease treatments.
4. Strengthen transparency throughout drug selection, negotiation, and public stakeholder engagement.

I. CMS Should Revise Its QSSD Policy to Protect Clinically Meaningful Follow-On Innovation

CAHC remains concerned that CMS's interpretation of what constitutes a qualifying single source drug (QSSD) under section 1192 of the Social Security Act aggregates products in ways that exceed the statutory framework and undermine incentives for innovation.

CAHC has previously objected to CMS's practice of aggregating dosage forms, strengths, routes of administration, and formulations sharing the same active ingredient or active moiety, even when products are approved under different New Drug Applications (NDAs) or Biologics License Applications (BLAs) and serve different clinical purposes. In our comments[1] on the Initial Price Applicability Year 2028 Draft Guidance, CAHC urged CMS to recognize meaningful distinctions among separately approved products and cautioned against expanding its treatment of fixed-combination drugs.

CAHC recognizes that section 1192(d)(3)(B) requires aggregation "across dosage forms and strengths of the drug, including new formulations." However, that language should not be interpreted to permit CMS to disregard meaningful distinctions among separately approved products or undermine incentives for clinically valuable follow-on innovation. Products sharing an active ingredient or active moiety may differ significantly in their indications, safety profiles, formulations, delivery methods, dosing frequency, routes of administration, patient populations, or treatment settings.

These differences may reflect substantial additional research and investment. Pharmaceutical innovation does not end with a product's initial approval. Follow-on research may identify new indications, make therapies available to patients at earlier stages of disease, develop formulations appropriate for pediatric or other specialized populations, reduce administration burdens, improve adherence, address safety or tolerability issues, or allow treatment to occur in different care settings.

The proposed rule's treatment of certain fixed-combination products illustrates the consequences of an overly broad aggregation policy. CMS generally treats a distinct fixed-combination product as a separate QSSD. Under proposed § 429.125(b)(4)(i), however, CMS would create an exception when a fixed-combination product shares an active ingredient with another product from the same manufacturer and an added ingredient enables a different route of administration. CMS could therefore aggregate a newly approved product with an older product and use the older product's approval or licensure date in determining when the QSSD becomes eligible for negotiation.

CMS specifically discusses products that add an ingredient such as hyaluronidase, which can allow a therapy previously administered by infusion to be administered by subcutaneous injection. This type of innovation should not automatically be treated as minor or a technical reformulation. Converting an infused therapy to a subcutaneous treatment can shorten administration time, reduce burdens on patients and providers, and enable treatment in different care settings.

The distinction is important because CMS's aggregation decision also affects the statutory period before a product becomes eligible for negotiation. If CMS aggregates a later-approved

[1] https://cahc.net/cahc-urges-cms-to-address-policies-driving-up-health-costs/

formulation with an older product, the eligibility clock for the newer product would run from the older product's approval or licensure date rather than its own, making the newer product selectable for negotiation substantially earlier. A later-developed product could therefore lose a significant portion of the seven-year period for small-molecule drugs or eleven-year period for biological products that would otherwise follow its own approval or licensure.

This creates a potential disincentive to invest in precisely the types of follow-on research that can produce meaningful benefits for patients. A 2025 *Health Affairs* analysis of 184 oncology approvals found that 41.8 percent received at least one follow-on approval and 59.7 percent of follow-on indications moved treatment to an earlier disease stage.[2] IQVIA has similarly identified development involving new indications, populations, and formulations as an important component of pharmaceutical innovation.[3] These findings illustrate why CMS's Negotiation Program should not assume that clinically meaningful innovation ends with a product's initial approval.

CMS characterizes the proposed fixed-combination exception as "narrowly tailored" to new formulations that raise program-integrity concerns and indicates that it does not expect the policy to meaningfully affect incentives for innovation. However, that assurance appears only in the preamble. Preamble statements do not carry the binding force of codified regulatory text, and nothing in proposed § 429.125(b)(4)(i) limits the exception to the circumstances CMS describes. Although CMS discusses the policy primarily in the context of biologics, the regulatory text is not limited to biologics, and CMS acknowledges that the policy could extend to drugs approved under NDAs in the future.

Furthermore, the proposed rule does not establish sufficiently clear criteria for distinguishing a minor product modification from a clinically meaningful innovation. Adding an ingredient without producing a meaningful clinical difference is fundamentally different from developing a formulation that materially changes how, where, or for whom a therapy can be administered. CMS should not treat those circumstances identically.

Recommendation: CMS should withdraw proposed § 429.125(b)(4)(i) and retain its existing treatment of distinct fixed-combination products. At a minimum, CMS should establish clear limits that preserve incentives for clinically meaningful follow-on innovation. In determining whether products should be aggregated as a single QSSD, CMS should consider meaningful differences in route of administration, delivery method, clinical use, site of care, and patient population. CMS should not apply the proposed policy in a manner that treats significant improvements in how a therapy is administered, such as converting an infused therapy to a subcutaneous treatment, as indistinguishable from minor product modifications. If CMS nevertheless finalizes the exception, it should state these limits in the regulatory text rather than in preamble discussion and should also define the criteria by which it will distinguish a minor product modification from a clinically meaningful innovation.

[2] Philipson TJ, Zhao Q, Zhang D, Fujibayashi K, Johnson H. Follow-On Cancer Drugs Target Earlier Stages Than Initial Drugs: Implications Of The IRA. *Health Aff (Millwood)*. 2025;44(12):1448-1456. doi:10.1377/hlthaff.2025.00627.

[3] IQVIA Institute for Human Data Science. *Expanding Options for Emerging Biopharma in the U.S.: A Decade of Change*. Published October 29, 2025. https://www.iqvia.com/insights/the-iqvia-institute/reports-and-publications/reports/expanding-options-for-emerging-biopharma-in-the-us-a-decade-of-change.

II. CMS Should Implement the Expanded Orphan Drug Exclusion Consistent With Congress's Intent to Protect Rare Disease Innovation

CAHC appreciates the statutory changes made since our previous comments to expand the orphan drug exclusion under the Medicare Drug Price Negotiation Program.

In our 2025 comments, CAHC expressed concern that the original exclusion was too narrow because a drug could qualify only if it had a single orphan designation and was approved solely for that rare disease or condition. CAHC urged policymakers to allow therapies with more than one orphan indication to remain eligible for the exclusion and warned that the prior policy could discourage manufacturers from pursuing additional rare disease indications.

Congress subsequently amended section 1192 to expand the orphan drug exclusion and establish rules governing drugs that previously qualified for the exclusion. The proposed rule would codify those statutory changes and further clarify how CMS will determine the applicable seven- or eleven-year period when a drug ceases to qualify.

CAHC supports CMS's implementation of the expanded exclusion and believes the change better reflects the objectives of federal orphan drug policy.

Rare disease drug development presents unique scientific and economic challenges. Patient populations are small, clinical trials can be difficult to design and enroll, and manufacturers frequently pursue additional orphan indications only after gaining substantial knowledge from an initial development program. Public policy should encourage, rather than discourage, continued research into additional rare disease uses for existing therapies.

Recommendation: CAHC supports codification of the expanded orphan drug exclusion and urges CMS to interpret the statutory changes in a manner that preserves strong incentives for continued rare disease development. CMS should provide detailed examples addressing multiple orphan designations, later non-orphan approvals, withdrawn indications, changes in orphan designation status, and the date from which the applicable seven- or eleven-year period begins. CMS should also address how it will avoid disadvantaging manufacturers that pursued additional orphan indications in reliance on the prior exclusion, including through an appropriate transition period before a drug that ceases to qualify becomes selectable.

III. CMS Should Establish a More Transparent and Accountable Negotiation Program

CAHC appreciates that the proposed rule includes additional transparency measures, but more substantial improvements are warranted as CMS converts the Negotiation Program from a guidance-based process into a permanent regulatory framework.

Transparency has been a consistent concern for CAHC throughout implementation. Last year, we noted that stakeholders had little visibility into how CMS used public input, why particular drugs or therapeutic alternatives were treated as they were, how negotiation decisions were reached, or what analysis supported the final MFP. CAHC urged CMS to publish meaningful explanations of its decisions, release non-confidential economic analyses, improve transparency concerning therapeutic alternatives, and make stakeholder engagement more substantive.

The proposed rule represents progress in some areas. For initial price applicability year 2029 and subsequent years, CMS proposes to publish up to 30 of the highest-ranked negotiation-eligible drugs, including the drugs ultimately selected for negotiation, based on combined Part B and Part D expenditures. CMS also proposes to codify elements of the negotiation process and related public engagement that previously existed primarily through guidance.

CAHC supports greater disclosure, but CMS should go further. A program that permits the federal government to establish prices for some of the highest-expenditure therapies in Medicare should provide stakeholders with sufficient information to understand the basis for those decisions.

First, CMS should provide greater transparency regarding drug selection. Publishing ranked expenditure information is useful, but stakeholders should also be able to understand how CMS applied exclusions, aggregation policies, generic and biosimilar determinations, and other selection rules. Where a drug is selected or excluded based on an agency determination requiring judgment, CMS should provide a non-confidential explanation of that determination.

Second, CMS should provide significantly greater transparency regarding the negotiation itself. CAHC recognizes the need to protect proprietary and confidential commercial information. That obligation does not require the Agency to shield the analytical basis of its decisions from public scrutiny. CMS should explain the methodology it used, the factors that materially affected its analysis, and the reasons the final MFP differs from relevant benchmarks.

Third, CMS should make stakeholder engagement meaningful rather than merely procedural. Patient-focused events and opportunities for public input are valuable only if participants can understand how their input affected the Agency's decision-making. CMS should publish summaries of the issues raised through stakeholder engagement and explain, at an appropriate level of detail, how that evidence informed the negotiation process.

Fourth, CMS should disclose non-confidential economic and regulatory analyses used to assess the downstream consequences of the Negotiation Program. Those consequences include not only federal spending but also patient access, provider reimbursement, site-of-care shifts, market consolidation, investment in research and development, and incentives to develop new indications and formulations. As CAHC emphasized last year, transparency is essential to determining whether the program is achieving lower costs without producing unintended consequences for beneficiaries and the broader healthcare system.

Recommendation: CMS should strengthen the final rule by requiring regular publication of non-confidential information sufficient for stakeholders to understand how the Agency selects drugs and determines MFPs. At a minimum, CMS should:

- Publish a clear explanation of the basis for each drug-selection decision, including relevant QSSD, exclusion, generic, or biosimilar determinations;
- Provide sufficient information about the analytical methodology used to develop and negotiate each MFP to allow stakeholders to understand the principal factors driving the result;
- Explain which therapeutic alternatives were considered and the basis for determining their relevance;
- Publish summaries of stakeholder input and describe how that information affected CMS's analysis;

- Release non-confidential economic, actuarial, and regulatory impact analyses used by CMS or other components of HHS; and
- Preserve the ability of manufacturers and other participants to discuss non-confidential aspects of the process rather than imposing restrictions beyond those necessary to protect proprietary information.

Meaningful transparency will improve accountability, permit Congress and stakeholders to evaluate the program's effects, and strengthen confidence that negotiation decisions are grounded in consistent and objective standards.

CONCLUSION

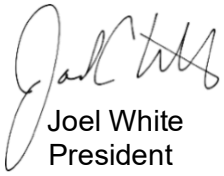
CAHC supports efforts to lower prescription drug costs and make health coverage more affordable for Medicare beneficiaries and other Americans. At the same time, affordability policies must be implemented in a manner that preserves patient access, encourages competition, and maintains incentives for continued innovation.

As CMS establishes permanent regulations governing the Medicare Drug Price Negotiation Program, the Agency should avoid expanding the definition of a qualifying single-source drug beyond the statutory boundaries established by Congress, recognize clinically meaningful distinctions among separately approved therapies, faithfully implement Congress's expanded protections for orphan drugs, and establish a negotiation process characterized by substantially greater transparency and accountability.

These objectives are complementary rather than conflicting. A sustainable negotiation program should lower costs while continuing to reward advances that improve treatment, expand access, and address unmet patient needs.

We appreciate the opportunity to submit these comments and welcome further dialogue regarding implementation of the Medicare Drug Price Negotiation Program.

Sincerely,



Joel White
President