



September 1, 2022

RE: Submitted via <http://www.regulations.gov>
Ms. Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
U.S. Department of Health and Human Services
7500 Security Boulevard
Baltimore, MD 21244

Re: Medicare and Medicaid Programs; CY 2023 Payment Policies Under the Physician Fee Schedule and Other Changes to Part B Payment Policies; Medicare Shared Savings Program Requirements; Medicare and Medicaid Provider Enrollment Policies, Including for Skilled Nursing Facilities; Conditions of Payment for Suppliers of Durable Medicaid Equipment, Prosthetics, Orthotics, and Supplies (DMEPOS); and Implementing Requirements for Manufacturers of Certain Single-Dose Container or Single-Use Package Drugs To Provide Refunds With Respect to Discarded Amounts. [CMS-1770-P]

Dear Administrator Brooks-LaSure:

The Rare Access Action Project, (RAAP) appreciates the opportunity to comment on the Centers for Medicare & Medicaid Services' (CMS) proposed payment updates to the calendar year 2023 Medicare Physician Fee Schedule (Proposed Rule).¹ RAAP appreciates CMS' numerous proposals to implement the discarded drug policy process but urges CMS to broaden its applicability of its "unique circumstances" authority and implement some procedural safeguards for manufacturers during the refund determination process.

RAAP is a registered 501(c)(4) non-profit organization that is a coalition of life sciences and patient stakeholders that explore creative policy solutions to address structural issues in access and coverage. Our priority is to help ensure rare

¹ 87 Fed. Reg 45,860 (July 29, 2022).



disease patients have access to the care and treatments they need and submits the following comments consistent with that objective.

I. Background: Rare Diseases and Orphan Products

The [Orphan Drug Designation Program](#) provides orphan status to drugs and biologics which are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than [200,000 people in the US](#), or that affect more than 200,000² persons but are not expected to recover the costs of developing and marketing a treatment drug.³ Rare diseases include more familiar conditions, such as cystic fibrosis, Lou Gehrig’s disease, and Tourette’s syndrome, as well as less familiar conditions, such as aromatic L-amino acid decarboxylase (AADC) deficiency, Duncan’s Syndrome, Madelung’s disease, and acromegaly/gigantism. These conditions are complex and often not well understood, which causes great challenges to the diagnosis and treatment as well as research efforts.

[Rare disease treatments](#) range from curing the disease, modifying how the disease functions, or treating the symptoms. Truly curative treatments are rare. Disease-modifying therapies target the underlying pathology of a disease to prevent it from worsening. Symptomatic treatments seek to temper symptoms or to maintain physical, emotional, and mental functioning.

[Only 5%](#) of rare diseases have a treatment approved by the Food and Drug Administration (FDA) and for [one-third of individuals](#) with a rare disease, it can take between one and five years to receive a proper diagnosis. Patients with rare diseases often seek treatment in clinics where the condition has never been seen before and have symptoms that are absent, masked, misunderstood, or confused, which often leads to delayed diagnosis further complicating the patient’s and family’s arduous journey. The rate of rare disease among Medicare beneficiaries is 11.5 percent, and they account for nearly 40 percent of rare disease healthcare spending (39 percent).⁴ As such, Medicare’s coverage and payment policies impact millions of patients daily.

² Department of Health and Human Services, Food and Drug Administration (FDA). Office of Orphan Drug Development. <https://www.fda.gov/about-fda/office-clinical-policy-and-programs/office-orphan-products-development>. Accessed March 13, 2021.

³ [Id.](#)

⁴ <https://healthpayerintelligence.com/news/rare-disease-healthcare-spending-tops-costly-chronic-diseases>



For those beneficiaries with treatments, the regimens can be complicated, vary amongst and between beneficiaries, and often require trial and error to achieve maximum clinical benefit. RAAP therefore appreciates CMS' willingness to engage the healthcare stakeholder community for feedback on "on whether there are other drugs with unique circumstances as described under section 1847A(h)(3)(B)(ii) of the Act that may warrant an increase in the applicable percentage"⁵ to hopefully apply a more flexible discarded drug policy to certain orphan drugs.

II. The Statute Requires CMS to Apply a Two-Step Process to Determine Discarded Drug

Section 90004 of the Infrastructure Investment and Jobs Act (Pub. L. 117-9, November 15, 2021) amended current law to require manufacturers to provide a refund to CMS for certain discarded amounts from a refundable single-dose container or single-use package drug. The refund amount is the amount of discarded drug that exceeds an applicable percentage, which is required to be at least 10 percent, of total charges for the drug in a given calendar quarter.⁶

RAAP believes that Congress established a clear definition of discarded drug in new Section 1847A(h)(1)(B), which is as follows:

"For purposes of subparagraph (A)(i), with respect to a refundable single-dose container or single-use package drug furnished during a quarter, the amount of such drug that was discarded shall be determined based on the amount of such drug that was **unused and discarded** for each drug on the date of service." (Emphases added)

RAAP therefore believes that to determine discarded drug CMS must establish that the drug was both unused and discarded. This is clearly a two-step process, requiring the Agency to further define what is unused. Basic rules of statutory construction do not allow CMS to use the words interchangeably, they must have different meanings.

As such, in establishing a difference RAAP urges CMS to recognize that active ingredient that is used to safely administer an appropriate dose without being

⁵ *Id.* at 46,062.

⁶ *Id.* at 46,056.



administered to the patient is considered used product and not subject for a refund. RAAP believes that Congress chose the word unused to imply that the liquid was not instrumental in assuring that a safe and effective dose was administered to the patient. In other words, if the liquid served a useful purpose this should not be considered for a refund. For example, there are small vial fills that contain active ingredient, beyond overfill, that is in the vial to assure that the appropriate dose is withdrawn and then administered to the patient consistent with FDA approved required dose. Specifically, the active ingredient serves a valuable clinical purpose that is required in order to safely administer the correct dose. This amount is in addition to overfill, which is not included in the labeled amount. RAAP knows that the FDA considers in its vial fill the amount of liquid required and used to express a needle and to account for dead volume in the transfer and so should CMS because this active ingredient served a useful purpose.

CMS recognizes the distinction of used for a clinical purpose versus unused by citing an example of a drug that is reconstituted with a hydrogel and administered via ureteral catheter or nephrostomy tube into the kidneys where there is substantial amount of reconstituted hydrogel that adheres to the vial wall during preparation. In this example, CMS proposes that the drug amount that adheres to the vial wall (and not able to be extracted from the vial) and must be discarded perhaps inappropriately leads to a higher percentage of discarded units billed with the JW modifier.⁷ In response, CMS proposes a 35 percent drug discarded percentage. RAAP supports this higher percentage and urges CMS to establish a policy that drug that is needed and used to safely administer other drug to patients is NOT considered unused and therefore not subject to a refund.

III. CMS Should Establish a Drug Specific Review Policy for Orphan Drugs

Orphan drug development presents several major challenges and obstacles, such as low disease prevalence, disease severity, small and heterogeneous patient populations, difficulties in patient recruitment, and limited knowledge of the natural history of disease, among others. These factors can lead to great variability in dosing regimens for certain drugs as physicians try to find combinations that provide maximum clinical benefit. In discussing its authority to increase the applicable percentage for any drug with unique circumstances, CMS states that “[w]e expect that for most drugs supplied in single-dose containers, the amount of drug indicated on the vial or container reflects the amount of drug that could

⁷ Id.



potentially be administered to a patient.”⁸ RAAP supports this conclusion for most drugs. For orphan drugs, however, this is not always true.

Starting with the Orphan Drug Act, Congress has long recognized the need to create appropriate commercial incentives for the development of drugs to treat patients living with a rare condition. This trend continued with the recent passage of the Inflation Reduction Act of 2022⁹ further demonstrating Congress’ intent to protect the commercial development of Orphan Drugs and subsequent patient access to those therapies. In continuance of Congress’ intent, RAAP urges CMS to establish a specific review policy for those orphan drugs that are exposed to large refunds under this provision for a possible higher discarded drug refund percentage. In addition to the criteria CMS mentions as part of the appeal process, this review process should focus on the feasibility of manufacturing alternative vial sizes considering the small patient population the therapy treats. RAAP believes this analysis will be used to balance patient access versus a large refund that could hamper that access given the small patient population.

IV. CMS Should Establish an Appeals Process Within its Dispute Resolution Process

RAAP appreciates that CMS proposes a dispute resolution process to aid in the successful implementation of this new policy. CMS details which information the manufacturer should include and how the Agency will work with manufacturer to resolve the dispute. RAAP urges CMS to finalize this dispute resolution process with the following additional processes. First, we urge CMS to confirm that the dispute resolution process will be confidential such that none of the issued reports during the dispute will be public because of the confidential proprietary commercial information contained in each report. Second, CMS should develop an appeals process that allows manufacturers to appeal a refund amount to a mutually agreed upon independent third party.

V. CMS Should Establish Clear Criteria and Definitions for When a Therapy Qualifies as a Unique Circumstance.

RAAP believes that CMS should establish some initial criteria that the Agency believes satisfies the unique circumstance criteria. Specifically, are there classes of products, modes of administration, or disease states that the Agency believes

⁸ *Id.*

⁹ Congress exempted from negotiation an orphan drug indication of an approved therapy.



categorically contain unique circumstances necessitating a higher applicable percentage? As mentioned above, RAAP believes that this applies to orphan drugs due to the uniquely treated patients. We urge CMS to include in its final rule a framework, with an opportunity for stakeholder feedback, that further guides industry on how CMS will apply its unique circumstances authority.

Second, CMS should establish a process for the manufacturer to engage CMS on the applicability of these criteria to any particular drug. Specifically, CMS should develop an application process for manufacturers to use to work with CMS to clearly understand whether their vial size, complex procedure or delivery mechanism qualifies as a unique circumstance. Like pass through applications in the hospital outpatient department, CMS can conduct this process which is then discussed in notice and rulemaking. This predictability and transparency will dramatically help manufacturers understand CMS' policy, help with vial development, and avoided unnecessary discarded drug.

VI. Issues Related to Average Sales Price

CMS does not publish all manufacturer submitted Average Sales Price (ASP) data on the ASP drug pricing file, "there are likely billing and payment codes payable under Medicare Part B that would meet the proposed definition of refundable single-dose container or single-use package drug that are not found on the ASP drug pricing file or the JW modifier data published on the CMS website."¹⁰ RAAP agrees that these drugs could be included in the discarded drug policy but urges CMS to then publish the ASPs of these drugs on the ASP drug pricing file and JW modifier data published on the CMS website. RAAP believes that if CMS collects a refund on discarded drug, it is only fair that the drug's JW modifier data and ASP also be published.

In addition, RAAP urges CMS to seek only a refund amount that serves as a refund and not as a mechanism to profit. CMS states that the refund amount is calculated as the product of the "total number of units of the billing and payment code for such drug that were discarded during such quarter; and the payment limit amount for the refundable single-dose container or single-use package drug."¹¹ CMS states that it plans on using 106 percent of the ASP as the payment limit amount. RAAP, however believes that if sequestration is in place the payment limit amount should be 104.3 percent of the ASP consistent with congressional intent of this being only a refund program.

¹⁰ Id. at 46,058.

¹¹ Id. at 46,061.



VII. Conclusion

Thank you for the opportunity to submit these recommendations to the Agency's Proposed Rule. We look forward to working with CMS to further develop policies that maximize access to therapies treating rare diseases. Please feel free to contact me at 202-631-5752 or by email at mike@rareaccessactionproject.com.

Sincerely,

Mike Eging