April 8, 2019

The Honorable Alex Azar
Secretary
Department of Health and Human Services
200 Independence Avenue SW, Room 600E
Washington, D.C. 20201

Re: Removal of Safe Harbor Protection for Rebates Involving Prescription Pharmaceuticals and Creation of New Safe Harbor Protection for Certain Point-of-Sale Reductions in Price on Prescription Pharmaceuticals and Certain Pharmacy Benefit Manager Service Fees Proposed Rule (84 Fed. Reg. 2340)

Dear Secretary Azar:

On behalf of the 25 to 30 million Americans with one of the over 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks the Department of Health and Human Services (HHS) for the opportunity to provide comments on its proposed rule titled "Removal of Safe Harbor Protection for Rebates Involving Prescription Pharmaceuticals and Creation of New Safe Harbor Protection for Certain Point-of-Sale Reductions in Price on Prescription Pharmaceuticals and Certain Pharmacy Benefit Manager Service Fees (Proposed Rule)."

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. For more than 30 years, NORD has voiced the needs of the rare disease community, advanced medical research, and provided crucial services to patients and families who need them most. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

In an effort to reduce pharmaceutical drug pricing and increase cost transparency, the Proposed Rule seeks to eliminate retrospective pharmacy benefit manager (PBM) rebates in the Medicare Part D and Medicaid managed care markets through changes to the discount safe harbor for the federal Anti-Kickback Statute (AKS). While NORD applauds HHS for tackling the complex issue of drug pricing and supports the intent of this proposal, we are concerned that the Proposed Rule may not materially decrease costs and, instead, may increase premiums for rare disease patients.

Rare Diseases and Orphan Drugs

Currently, over 7,000 rare diseases have been identified, yet over 90 percent of those diseases do not have therapies indicated for their disease that have been approved by the Food and Drug Administration (FDA). For the less than ten percent of rare diseases that do have an approved treatment, the therapies, also known as orphan drugs, in many cases, have no treatment alternatives and lack generic competition.[1] Consequently, it is unlikely that rebates are utilized for rare disease therapies to the same extent that they are for common disease therapies because, with fewer competing products, manufacturers have less of an incentive to offer a

[1] Orphan Drugs in the United States; Exclusivity, Pricing and Treated Populations. Report. IQVIA Institute for Human Data Science. 2018. https://www.iqvia.com/-/media/iqvia/pdfs/institute-reports/orphan-drugs-in-the-united-states-exclusivity-pricing-and-treatedpopulations.pdf.

rebate to secure placement on the formulary.[2] Additionally, within the context of Medicare, many rare disease therapies fall within the six protected classes, meaning they must be included on the formulary and, therefore, are similarly unlikely to be paired with rebates.[3]

The Proposed Rule's Impact on Rare Disease Patients

While NORD supports the overall intent of the rule, we are concerned that (1) the Proposed Rule could result in increased premiums and (2) overhauling pharmaceutical reimbursement methodologies without a clear understanding of the risks could produce unintended pricing and care consequences.[4,5] Further, any potential benefit of the proposal, in the form of manufacturers voluntarily lowering their list prices or offering discounts, is not guaranteed by the Proposed Rule.

Even if the Proposed Rule were to result in such benefits, they may be highly diluted for rare disease patients because, as stated above, the lack of competition and the overlap between orphan drugs and Medicare's six protected classes has likely resulted in the limited use of rebates in the rare disease space. HHS specifically acknowledges the effect of the six protected classes in the Proposed Rule, stating that "a beneficiary using high cost drugs in protected classes may be less likely to benefit from a reduced pharmacy purchase price, because manufacturers generally offer low or no rebates to plans for these drugs."[6]

Given that the Proposed Rule could lead to increased premiums from the altered discount safe harbor and that traditional market forces and rebate structures may not be applicable to orphan drugs, NORD is concerned that the risks of the Proposed Rule would outweigh any potential benefit for the rare disease population.

Rare disease patients already face significant financial hurdles in accessing the care they need. They cannot afford an increase in premiums, particularly without a proportional decrease in drug costs. NORD recognizes that it is difficult to predict manufacturer and plan behavior in response to this regulation and believes more research, study, and analysis is needed to prevent unintentional cost increases for rare disease patients. NORD is committed to working with HHS on this analysis but cautions against adoption of this rule until the consequences and benefits are better understood.

For questions regarding NORD or the above comments, please contact me at rsher@rarediseases.org, or (202) 545-3970.

Sincerely,

/s/ Rachel Sher

Rachel Sher
Vice President of Regulatory and Government Affairs

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[2] Removal of Safe Harbor Protection for Rebates Involving Prescription Pharmaceuticals and Creation of New Safe Harbor Protection for Certain Point-of-Sale Reductions in Price on Prescription Pharmaceuticals and Certain Pharmacy Benefit Manager Service Fees, 84 Fed. Reg. 2342 (Feb. 6, 2019).
[3] Ibid, 2358
[4] Ibid, 2356-2359
[5] Dea, Belazi, *Eliminating Drug Rebates Could Raise Insurance Premiums*, Bloomberg (Mar. 4, 2019), https://news.bloomberglaw.com/health-law-and-business/insight-eliminating-drug-rebates-could-raise-insurance-premiums
[6] Removal of Safe Harbor Protection for Rebates Involving Prescription Pharmaceuticals and Creation of New Safe Harbor Protection for Certain Point-of-Sale Reductions in Price on Prescription Pharmaceuticals and Certain Pharmacy Benefit Manager Service Fees, 84 Fed. Reg. 2358 (Feb. 6, 2019).