



May 9, 2016

The Honorable Andrew M. Slavitt, Acting Administrator
Centers for Medicare and Medicaid Services
Department of Health and Human Services
Hubert H. Humphrey Building, Room 445-G
200 Independence Avenue, SW
Washington, D.C. 20201

RE: Comment on CMS-1670-P (“Medicare Program: Part B Drug Payment Model”)

Dear Acting Administrator Slavitt,

On behalf of the 30 million Americans with one of the approximately 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) would like to thank the Centers for Medicare and Medicaid Services (CMS) for the opportunity to provide comments on the proposed rule titled, “Medicare Program: Part B Drug Payment Model.”

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

NORD’s central policy and advocacy mission is to foster the innovation, development, and delivery of life-changing and often life-saving therapies for rare disease patients. While we are pleased with the accelerating growth in orphan product development since the passage of the Orphan Drug Act, we remain vigilant in ensuring these therapies, once developed, are accessible and affordable for rare disease patients.

We would first like to thank CMS for its commitment to fostering a more efficient healthcare system by “spending our dollars more wisely for drugs paid under Part B, that is, a reduction in Medicare expenditures, while preserving or enhancing the quality of care provided to Medicare beneficiaries”.¹ We agree that value-based purchasing tools have the potential to allow public and private insurers to encourage the use of less costly and/or more effective treatments.

However, such efforts must not compromise patient access to physicians and therapies they rely on. We are concerned this proposed rule may have such an adverse impact on rare disease patients.

We are troubled by CMS’s process for implementing the rule as well as the structure of the rule itself. According to the proposed rule, 75 percent of all physicians and outpatient facilities that accept Medicare patients will face lower reimbursement rates and/or value based purchasing

(VBP) arrangements. The extensive scope of the demonstration hardly makes it seem like a demonstration at all. We are also disappointed by CMS's lack of outreach to the patient stakeholder community. With the patient community's assistance, CMS could collaboratively propose value based purchasing experiments that would not compromise patient health and wellbeing.

Second, CMS's rationale for proposing both Phase 1 and Phase 2 of this rule is to "strengthen the financial incentive for physicians to choose higher value drugs".² It is CMS's belief that "the revised pricing will remove any excess financial incentive to prescribe high cost drugs over lower cost ones when comparable low cost drugs are available".³

While we do not have any comments on the CMS's extrapolation of theoretical economic arguments that assert physicians are particularly profit driven, even if this is the case, this assertion generally is inapplicable to orphan drug prescribing. This is simply because orphan therapies usually do not have alternative therapies altogether, let alone cheaper alternatives. Of the nearly 200 physician-administered drugs that likely fall under Medicare Part B, over 60 percent of these drugs do not have an alternative treatment.⁴

Third, of the remaining physician administered therapies that do have an alternative, and thus fall under CMS's arguments, we have concerns regarding both phases that may limit access to vital therapies.

In regards to Phase 1, our position on what the appropriate rate of reimbursement for physicians should be is the reimbursement rate that affords rare disease patients access to the care they require. This includes finding care in their community in accessible and comfortable settings. It is already very difficult for rare disease patients to find physicians who can treat them due to the rarity and complexity of their disease.

We are concerned that by lowering reimbursement rates for physicians and outpatient facilities, many physicians and outpatient facilities may no longer offer care to our rare disease patients. These patients should not be forced to drive many miles to inpatient facilities to access necessary care. We simply ask CMS to be incredibly careful in moving forward with Phase 1 to ensure that rare disease patients do not lose access to the care they need.

We also request that CMS recognize and respect the unique characteristics of the rare disease community and the orphan products that treat them when crafting VBP experiments as part of Phase 2.

For example, reference pricing inherently assumes there are more than one product to choose from with either varying effectiveness or varying prices. This is simply not the case for the majority of orphan therapies as most do not have alternative therapies

We are also concerned about how CMS may define effectiveness in any given product. For diseases in which there are several therapies to choose from, the therapies may vary drastically in effectiveness and occurrence of adverse events among patients, and it is important to continue to allow patients to access the treatment that is most effective for them.

We encourage CMS to follow the example of the Food and Drug Administration's recent implementation of various patient-focused initiatives to better capture patient perspectives in drug development and review. Through initiatives such as the Patient-Focused Drug Development initiative, the FDA acknowledges that no one is better at identifying the effectiveness and importance of a drug than the patients themselves.

Patient populations cannot be treated as homogenous and thus all share the same risk-benefit formula. Patients widely differ on what symptoms they find most troublesome, and what adverse effects they are willing to endure. Patients may also respond quite differently to the same medication. This is particularly true with biologics and Biosimilars, many of which are administered under Part B.

All of these factors underscore the importance of ensuring patients are able to choose the drug that is appropriate for their particular needs. If CMS is to test VBP designs that reimburse differently based upon the effectiveness of the drug, we implore CMS to involve the affected patient populations thoroughly throughout the process.

Finally, we strongly support CMS's proposal to explore the elimination or discounting of patient cost-sharing for effective therapies. Orphan products, which are inherently more expensive due to the small populations they treat, are often accompanied by prohibitive patient cost-sharing. These orphan products are usually the most effective treatment for these patients by far, yet still face prohibitive tiering. By lowering or eliminating patient cost-sharing for these effective therapies, CMS would recognize their importance to the healthcare of thousands of rare disease patients.

NORD thanks CMS for the opportunity to comment, and we look forward to working with CMS on ensuring that rare disease patients receive the innovative treatments they need. For questions regarding NORD or the above comments, please contact me at mrinker@rarediseases.org or (202) 588-5700, ext. 102.

Sincerely,



Martha Rinker
Vice President of Public Policy

¹ Centers for Medicare and Medicaid Services, Proposed Rule, "Medicare Program: Part B Drug Payment Model" Federal Register 81, no. 48 (March 11, 2016): 13231,

² Centers for Medicare and Medicaid Services: 13232

³ Centers for Medicare and Medicaid Services: 13233

⁴ "Search Orphan Drug Designations and Approvals" Accessed March 17, 2016. https://www.accessdata.fda.gov/scripts/opdlisting/opd/OOPD_Results_2.cfm.