



December 31, 2018

The Honorable Seema Verma, 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

Attn: CMS-5528-ANPRM
P.O. Box 8013
Baltimore, MD 21244-8013

RE: Comment on CMS-2018-0132-0001 (“Medicare Program; International Pricing Index Model for Medicare Part B Drugs”)

Dear Administrator Verma,

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 advanced notice of proposed rulemaking titled, “Medicare Program; International Pricing Index Model for Medicare Part B Drugs.”

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. Since 1983, we have been committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

NORD strongly believes that all patients should have access to quality and affordable health care that is best suited to their medical needs. One of NORD’s responsibilities is to ensure that we represent rare disease patients each and every time a decision by the Federal Government can influence their access to care.

We commend CMS for seeking to improve access to critical therapies, and we encourage CMS to further explore areas in which affordable access to life-changing, even life-saving, therapies can be strengthened. CMS, and more broadly the Department of Health and Human Services (HHS), have already proposed several potentially beneficial improvements such as extending drug rebates to Medicare beneficiaries and ensuring Medicaid formularies include therapies approved via FDA’s accelerated approval pathway.

We want to be absolutely sure, however, that any attempt to increase access does not inadvertently do the opposite. We at NORD do not wish to discourage innovation in

reimbursement or outright reject efforts to make therapies more affordable, but we do wish to be absolutely certain that these policies will not make it harder for rare disease patients to acquire the therapies they need.

CMS's proposal to explore an "International Pricing Index Model for Medicare Part B Drugs" could, if implemented poorly, have a deleterious effect on our community. The magnitude of the effect of this proposal on our patients will also depend upon the scope of the demonstration project, namely on which therapies are included.

Further clarity from CMS on which therapies, and which indications on said therapies, will be included will be critical for us to ascertain the effects of the proposal on individuals with rare diseases. Even if CMS is to only include a handful of high utilization therapies within this demonstration (thus excluding the vast majority of orphan drugs), these very same high utilization therapies may be the only therapeutic option for certain rare disease communities whose conditions are one of many indicated uses for the therapy.

This is one of several considerations NORD believes CMS must consider when choosing to implement any of the proposals offered. Below are additional comments on how CMS can approach this proposal with rare disease patients in mind.

International Reference Pricing

NORD understands that CMS is concerned with discrepancies between what the United States and other countries pay for the same therapies. CMS's goal is laudable: patients in the United States are just as deserving of affordable, innovative therapies as any other patient in other similar countries.

We are concerned, however, that substantially altering the reimbursement for these therapies has the potential to unduly hurt innovation and limit access. Further, we are concerned that the addition of this restriction could potentially disincentivize manufacturers from offering their therapies within Medicare Part B or, conversely, incentivize them to increase their prices in the private sector to compensate for their loss. Were any of these scenarios to become reality, rare disease patients, not the Federal Government, would be harmed.

NORD encourages CMS to thoughtfully consider the second and third order effects of implementing this new reimbursement regime while keeping the needs of patients at the forefront. Were CMS to chart a path forward that would limit the rare disease community's access to innovative therapies, NORD would strongly oppose such efforts. We will carefully monitor CMS's continued development of this proposal in hopes that CMS does not go in that direction.

Addition of Private-Sector Vendors

While negotiation holds the potential to lower the price of drugs, NORD asks CMS to ensure that the addition of a third party to act as a middle man between physicians and manufacturers does not impede therapeutic access.

Private-sector vendors may not have the best interest of patients as their top priority as HHS adroitly points out in the “American Patients First” blueprint. If additional “middle men” are placed in between patients and their therapies, NORD is fearful that this policy could lead to fewer therapies available in Medicare Part B for patients in need.

NORD urges CMS to explore whether the addition of private-sector vendors could in any way lead to rare disease patients struggling to access their necessary therapies in Part B.

Changes to the Drug Add-On Payment

Again, NORD understands the desire to remove any potentially perverse incentives surrounding the purchase and reimbursement of therapies on the part of physicians and hospitals. We reiterate our comments from May 2016 that stated,

“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.”¹

While we do not have a position on the appropriate reimbursement scheme for physicians and institutions participating in the Medicare Part B program, we implore CMS to ensure any proposal does not disincentivize physicians to care for our patients and prescribe the very best treatment for their particular needs.

NORD thanks CMS once again for the opportunity to comment. We look forward to working with CMS to ensure that rare disease patients will continue to have access to the therapies they need. For questions regarding NORD or the above comments, please contact me at pmelmeyer@rarediseases.org or 202-545-3828.

Sincerely,



Paul Melmeyer
Director of Federal Policy

¹ https://rarediseases.org/wp-content/uploads/2014/11/NORD-Comments-on-Medicare-Program_-Part-B-Drug-Payment-Model-1.pdf