March 7, 2014

Centers for Medicare & Medicaid Services
Department of Health and Human Services
Attention: CMS-4159-P
P.O. Box 8013
Baltimore, Maryland 21244-8013

Re: CMS-4159-P: Medicare Program: Contract Year 2015 Policy and Technical Changes to the Medicare Advantage and the Medicare Prescription Drug Benefit Programs

Dear Sir or Madam:

On behalf of individuals with rare diseases who rely on the Medicare Advantage Program (Part C) and the Medicare Prescription Drug Benefit Program (Part D) for access to treatment, the National Organization for Rare Disorders (NORD) gratefully submits the following comments on the proposed rule regarding "Contract Year 2015 Policy and Technical Changes to the Medicare Advantage and the Medicare Prescription Drug Benefit Programs", published in the January 10, 2014, *Federal Register*.

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.

The following comments address the proposed changes to the "Drug Categories or Classes of Clinical Concern and Exemptions" that are contained within this proposed rule.

In this proposed rule, the Centers for Medicare and Medicaid Services (CMS) proposes a new interpretation of Section 3307 of the Affordable Care Act (ACA) which defines the scope of the protected classes within Parts C and D of the Medicare program.[1] This proposed interpretation applies a two-pronged test for determining whether a drug class should be included within the classes of clinical concern. This test reads as follows:

1. A typical beneficiary, who is initiating therapy must administer a drug within the category or class in less than 7 days or failure to do so will lead to hospitalization, incapacity, disability or death; and

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2. Other CMS formulary requirements are not sufficient to ensure the access to an appropriate range of therapies, either due to the diversity of disease or condition manifestations or the associated specificity or variability of drug therapies necessary to treat such manifestations.

After applying this test to the Part D drug classes, CMS deemed only anti-retrovirals, anti-convulsants, and anti-neoplastics as appropriately passing this test. Thus, CMS proposes the removal of the anti-depressant and immunosuppressant classes from the classes of clinical concern in 2015, and the removal of the anti-psychotic drug class in 2016.

While NORD is appreciative of CMS's efforts to strengthen the Medicare program, we are gravely concerned with the impact this rule change would have on access to these critical therapies within the rare disease community. We support the full withdrawal of this proposed change for the following reasons.

While most anti-depressants, anti-psychotics, and immunosuppressants are unlikely to be orphan drugs, many rare disease patients rely on off-label prescription drug use to treat their condition. There are nearly 7,000 rare diseases recognized in the United States, but only approximately 300 of these conditions have an FDA-approved treatment. Most patients with a rare disease rely on off-label prescriptions for treatment, many of which may fall within these three classes of clinical concern.

Second, many rare disease patients who rely on Medicare for drug coverage have chronic and debilitating conditions that often carry co-morbidities of depression and other psychological conditions that require anti-depressants or anti-psychotics. Many rare diseases, such as Alpha-1 Antitrypsin Deficiency, also often require transplants for continued survival, and reduced access to immunosuppressants could have devastating impacts if a transplant is rejected.

We are also very concerned that this rule change may cause an interruption in care. Under this proposal, plans may remove drugs within these classes from their formularies, forcing patients who depend on these drugs to find a new plan or file an appeal. This lack of stability in drug coverage would be quite damaging to these chronically ill patients, as conditions can deteriorate rapidly with an interruption in care.

Finally, the effectiveness of a drug's therapeutic effect, including for categories of drugs such as anti-depressants, anti-psychotics, and immunosuppressants, has been shown to vary by patient, context of administration and dosage, and it is absolutely essential that rare disease patients have access to most if not all drugs within these protected classes.[2]

CMS's states that there are strong protections in place for beneficiaries in the form of a "robust coverage determination and appeals process", thus rendering the protection of these drug classes no longer necessary. However, we believe the Part D appeals process is not sufficient to protect these vulnerable patients. According to the Medicare Rights Center, many beneficiaries are refused medication at the pharmacy counter without explanation. Many beneficiaries are also unaware of their right to appeal, and even those who are aware find the process quite arduous and confusing.[3]

CMS cites rising Medicare Part D program costs as a justification for this proposed change, positing that the removal of three protected classes will produce cost savings, and that existing patient protections are strong enough to protect beneficiaries affected by this change. We remain skeptical that cost savings would be achieved if this proposal is enacted, as savings gained within the Part D program may be offset by higher spending in the Medicare Parts A and B programs due to higher utilization of hospital and physician services as a result of changes in access to medicines as a consequence of the proposed rule. A November 2012 Congressional Budget Office (CBO) report recognizes that increased access to prescription drugs lowers utilization of costly hospital services.[4]

Even if cost-savings are realized within the Medicare program, NORD takes strong reservation to the premise of prioritizing cost savings over patients' access to treatments.

NORD supports full withdrawal of the proposed removal of anti-depressants, anti-psychotics, and immunosuppressants from the drug categories or classes of clinical concern, and requests CMS abandons these proposed changes.

We thank CMS for the opportunity to comment, and we look forward to working with CMS and its partners in ensuring access to these critical treatments within the rare disease community. For questions regarding NORD or the above comments, please contact Diane Dorman, Vice President of Public Policy, at ddorman@rarediseases.org or (202) 588-5700 ext. 102.

Sincerely,

Peter L. Saltonstall
NORD President & CEO

[1] 42 U.S. Code § 1395w–104

[2] *Ma, Quiang and Anthony Y. H. Lu, "*Pharmacogenetics, Pharmacogenomics, and Individualized Medicine." *Pharmacological Reviews;* June 2011, vol. 63, no. 2, 437-459.

[3] Medicare Rights Center. "Refused at the Pharmacy Counter: How to Improve Medicare Part D Appeals." Winter, 2013

[4] Congressional Budget Office (CBO). "Offsetting Effects of Prescription Drug Use on Medicare's Spending for Medical Services." November 29, 2012