



December 21, 2015

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-9937-P (“Patient Protection and Affordable Care Act; HHS Notice of Benefit and Payment Parameters for 2017: Proposed Rule”)

Dear Sir or Madam:

On behalf of the 30 million Americans with one of the approximately 7,000 known rare diseases, NORD would like to thank the Centers for Medicare and Medicaid Services (CMS) for the opportunity to provide comments on the proposed rule titled, “Patient Protection and Affordable Care Act; HHS Notice of Benefit and Payment Parameters for 2017.”

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 concerned about the growing use of discriminatory health insurance practices that often prohibit patients from accessing these therapies.

In its proposed rule, CMS addresses many of these challenges rare disease patients face. The following are our comments on those proposals

Parts 146 and 147 – Requirements for the Group Health Insurance Market and Health Insurance Reform Requirements for the Group and Individual Health Insurance Markets:

Continuity of Care: Continuity of care is incredibly important for rare disease patients. Physicians that are knowledgeable and able to treat particular rare diseases are unfortunately far and few between. CMS’s proposal to allow patients to continue treatment with a physician up to 90 days following the physician’s termination from the network without a cause is critical, and we thank CMS for this policy.

We ask that this continuity of care proposal extend to transition fills for patients switching to a new health plan, and require plans to provide 30 day transition fills for off-formulary medicines or medicines subject to utilization management.

Part 155 - Exchange Establishment Standards and Other Related Standards Under the Affordable Care Act:

Consumer Assistance Tools and Programs of an Exchange: NORD supports CMS’s efforts to strengthen the Navigator program, particularly the attempts to better serve neglected and underserved populations and the additional emphasis on post-enrollment services, including appealing adverse coverage decisions.

Rare disease patients require unique and specialized assistance when accessing health coverage. Rare disease patients are also more likely to require post-enrollment assistance, particularly in accessing therapies off-label. There are only approximately 475 orphan products to treat just over 350 rare diseases leaving the vast majority of rare disease patients to seek treatment from therapies that are indicated for other diseases. Because these treatments are accessed “off-label”, rare disease patients face a host of reimbursement difficulties in accessing these therapies from their insurance providers.

With greater assistance from navigators, rare disease patients will be able to access these life-saving therapies without the battle that often accompanies off-label access.

Rare disease patients also require a unique combination of care. Navigators can assist rare disease patients in understanding the differences between co-pays, co-insurance, deductibles, drug tiers and formularies, provider networks, and in-network versus out-of-network coverage. Rare disease patients need to know how much they will be paying for their care before they purchase a plan, and both navigators and consumer assisters play a critical role in ensuring transparency, clarity, and educated decision making in purchasing health insurance.

Part 156 – Health Insurance Issuer Standards Under the Affordable Care Act, Including Standards Related to Exchanges

Standardized Option: NORD thanks CMS for its attempts to streamline the patient experience in marketplace plans by developing standardized options for bronze, silver, and gold plans.

There are several aspects of the standardized options NORD supports, such as requiring prescription drug co-pays to apply pre-deductible in silver and gold plans. This policy mirrors most employer-offered plans (as is intended by the ACA) and is critical in ensuring patients are not required to pay their full deductible (which in ACA plans are very large and only growing) when accessing necessary medications. We thank CMS for this recognition, but we ask that this policy be extended to Bronze plans as well.

NORD asks that CMS better define what a “specialty drug” tier actually is, as insurers have increasingly used specialty tiers for any and all expensive medications regardless of the circumstances. While we understand insurers’ attempts to encourage use of less expensive alternatives by placing more expensive alternatives that offer little to no increased health benefits on specialty tiers, this situation rarely applies

to rare disease patients. There are rarely any alternatives for orphan therapies placed on specialty tiers, and there are rarely cheaper therapies for rare disease patients to choose from. By placing orphan drugs on specialty tiers, rare disease patients are left with only one option with prohibitive cost sharing. We ask CMS to better define what a specialty drug is, and structure standardized plan options to exclude orphan drugs from the specialty tier.

We thank CMS for its recognition that, “consumers often prefer copayments to coinsurance because the former are more transparent and make it easier for consumers to predict their out-of-pocket costs”. This recognition makes it all the more puzzling why CMS has chosen the coinsurance levels that it has for the standardized plans.

In this proposed rule, CMS proposes coinsurance levels ranging from 25 percent to 50 percent for bronze, silver, and gold plans. NORD strongly opposes this proposal, and implores CMS to reconsider.

Coinsurance has been extremely detrimental to patients with rare and chronic diseases, as expensive drugs and biologics that are placed on a plan’s formulary are often placed on the specialty tier, requiring the patient to pay upwards of 40 percent of the drug’s full cost. Many drugs that treat rare diseases are quite expensive – sometimes over \$100,000 annually. Covering this 40 percent coinsurance suggested by CMS for silver plans will likely max out a patient’s out-of-pocket maximum within the first visit to the pharmacy, burdening patients with a prohibitive bill that may lead patients to forgo treatment altogether.

These practices are discriminatory towards rare disease patients. By placing a rare disease patient’s only therapy on the specialty tier and thus requiring a coinsurance under CMS’s suggested plans, CMS is essentially endorsing insurance plans discriminating against rare disease patients by discouraging them from signing up for their plans in the first place. Section 1557 of the Affordable Care Act prohibits discrimination against patients with disabilities, and we believe such insurance design is discriminatory against rare disease patients.

Essential Health Benefits Package: Prescription Drug Benefits: NORD thanks CMS for its continuing efforts to ensure patients have access to medically necessary therapies that are not on a plan’s formulary. These efforts are particularly important to rare disease patients as orphan drugs are often left off formularies due to their small indicated populations. The vast majority of rare disease patients also must seek care off-label as there is no treatment indicated for their disease.

For these reasons, we thank CMS for its attempts to strengthen and expedite appeals processes for gaining access to off-formulary therapies. We agree that state regulation should precede federal regulations if their coverage appeals laws or regulations are more stringent than Federal rules. However, we ask that CMS clarify the intent of allowing state laws that “are in conflict” with Federal proposals to be exempt as well.

We join the “I Am Essential Coalition” in asking that CMS “clarifies that costs of non-formulary drugs would count toward annual patient cost limits, even if state regulations are applied to the exceptions process. Additionally, we believe costs incurred by patients who rely on off-formulary medications should be included in the patient’s annual maximum out of pocket amount, even when the drug is not obtained via the exemption process but the physician determines it is the most appropriate treatment”.

Network Adequacy Standards: We thank CMS for its efforts to ensure patients have “a [provider] network that is sufficient in number and types of providers to assure that all services will be accessible without unreasonable delay”. Again, ensuring access to physicians, particularly specialists, specialty facilities, and children’s hospitals is particularly important to rare disease patients.

Rare disease patients must see specialists that are knowledgeable in their disease, and often must visit hospitals or clinics that are specially equipped to provide care for their unique situation. If a rare disease patient’s specialist or hospital is removed from their insurance network, they will likely be unable to pay out-of-pocket for the services, and thus risk losing critical care and treatments altogether.

The exclusion of children’s hospitals in provider networks is one example of such practices occurring in marketplace plans. This is particularly troublesome to the rare disease community as the majority of rare disease patients are children with complex medical conditions.

We believe the proposals put forth are a step in the right direction, but we ask that CMS take one step further by requiring proof of network adequacy as a prerequisite to QHP certification.

Third Party Payment of Qualified Health Plan Premiums: NORD thanks CMS for its continued attention toward this very important access issue for the rare disease community. We appreciate CMS “considering whether we should expand the list of entities from who issuers are required to accept payment”. However, the longer CMS considers the issue, the more rare disease patients lose their treatments due to insurers denying charitable assistance.

In 1987, NORD established the first-ever patient assistance program for medications. Over the past 25 years, NORD has administered more than 380 patient assistance programs on behalf of over 1.3 million patients with the principle goal of ensuring fair and reasonable access to appropriate medical care and life-saving orphan therapies. No one understands the suffering and healthcare needs of our patients better than NORD, the organization specifically established to serve them. Without our programs, many of our patients would not have access to care.

NORD continues to be deeply troubled by CMS’s treatment of non-profit premium assistance programs, particularly those operated by charitable patient organizations such as NORD. With over thirty years of advocating for the rare disease community, CMS treatment of NORD’s efforts as problematic due to risk pool impacts is unconscionable. NORD’s attempts to help rare disease patients to access their medications should not be questioned using the same risk pooling rationale used to reject payments from hospitals and other for-profit providers.

Furthermore, rare disease patients and the third party premiums they receive from NORD and similar non-profit rare disease patient organizations are unlikely to have any impact on risk pooling due to the inherent rarity of these patients within the plan’s population.

CMS’s treatment of premium assistance programs also conflicts with the opinion of the Department of Health and Human Services Office of Inspector General (OIG). In a December 2013 opinion, the OIG explained the benefits of premium assistance programs, stating,

“By providing grants to pay for insurance premiums and certain expenses not covered by insurance, [organizations’] ability to influence how patients ultimately choose items and services payable...is minimized. Once patients have insurance coverage, they are able to select among the providers, practitioners, and suppliers of their choice. In this particular context, grants for payment of insurance premiums expand, rather than limit, beneficiaries’ freedom of choice”¹

In addition, CMS’s inclusion of cost-sharing within the preamble of this section is concerning. To quote the American Plasma User’s (APLUS) Coalition,

“While CMS intends to clarify that its rules surrounding premium assistance also apply to cost-sharing assistance in a positive way – plans must accept cost-sharing assistance from Ryan White programs, for example – we fear that plans will take this as authority to decline non-profit cost-sharing assistance. While the impact of this prohibition is less clear since any cost-sharing restrictions may be harder to implement, this would be extremely harmful to our patients. We urge CMS to clarify that QHPs should accept cost-sharing assistance from non-profits.”

CMS has recognized several times that charitable third party payments are allowable. CMS first clarified this matter in its February 7, 2014 guidance regarding third party payments of premiums for Qualified Health Plans (QHP) in the health insurance marketplaces, and later reaffirmed this position in a May 14, 2014 letter.”^{2, 3} NORD urges CMS to take this one step further by requiring QHPs to accept premium assistance payments from non-profits, particularly charitable non-profit rare disease patient organizations with an overarching mission of fostering the development of, and access to, innovative therapies.

Once again, thank you 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 Martha Rinker, Vice President, Public Policy at mrinker@rarediseases.org or (202) 588-5700, ext. 102.

Sincerely,



Peter L. Saltonstall
President and CEO

¹ Department of Health and Human Services Office of Inspector General (OIG), “OIG Advisory Opinion No. 13-19, December 2, 2013. <http://oig.hhs.gov/fraud/docs/advisoryopinions/2013/AdvOpn13-19.pdf>

² Centers for Medicare & Medicaid Studies (CMS), FAQ on Third Party Payments of Premiums for Qualified Health Plans in the Marketplaces. February 7, 2015. <https://www.cms.gov/CCIIO/Resources/Fact-Sheets-and-FAQs/Downloads/third-party-payments-of-premiums-for-qualified-health-plans-in-the-marketplaces-2-7-14.pdf>

³ HHS letter to the American Hospital Association dated May 21, 2014. <http://www.aha.org/content/14/140521-sebelius-umbdenstock.pdf>