July 24, 2019

The Honorable Chuck Grassley
Chairman
U.S. Senate Committee on Finance
Washington, D.C.

The Honorable Ron Wyden
Ranking Member
U.S. Senate Committee on Finance
Washington, D.C.

Dear Chairman Grassley and Ranking Member Wyden:

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) applauds the Committee for its efforts to drive meaningful, bipartisan drug pricing reform.

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 believes that all individuals with a rare disease should have access to quality and affordable health care that is best suited to their medical needs. NORD commends you for your hard work on this bipartisan Chairman's mark of the Prescription Drug Pricing Reduction Act (PDPRA) of 2019 (Chairman's mark) aimed at lowering costs for all patients.

NORD shares the Committee's goals that are clearly reflected in this draft legislation. People living with rare diseases must be able to afford the therapies that come to the market. The high cost of prescription drugs has a direct impact on the ability of patients to access lifesaving care. The small patient populations and medical complexity associated with rare diseases can lead to costly therapies, but it is vital that these therapies remain affordable and, therefore, accessible to rare disease patients. These therapies must also remain affordable so that the sustainability of the healthcare system as a whole is preserved.

As all levels of government and a variety of stakeholders look for legislative and regulatory tools to ensure that medicines are affordable, NORD has compiled a set of drug pricing principles to help guide NORD's engagement with these issues in a deliberate and transparent manner.[1] As the principles convey, NORD believes that it is critical to assure that a balance is struck between preserving incentives to innovate in the rare disease space and access to such innovative new therapies. NORD urges the Committee to remain vigilant about the fact that over 90 percent of rare diseases continue to lack a treatment approved by the Food and Drug Administration (FDA). Thus, rare disease patients depend on a vibrant pharmaceutical industry. Any efforts to address the high cost of medicines should not inhibit the innovation that has led to the kinds of orphan therapies that have made it to market or restrict access to those same therapies.

[1] The complete set of principles are attached to this letter and can also be found online at: https://rarediseases.org/nord-releases-principles-for-assessing-proposals-designed-to-lower-the-cost-of-prescription-drugs-in-the-united-states/

Like many other stakeholders, NORD is still evaluating the Chairman's mark with a particular focus on whether its policies adhere to the drug pricing principles described above. Based on our initial review, it appears that many of these principles are generally reflected in the Committee's proposal. However, we are concerned that some policies could inadvertently decrease patients' coverage and access, particularly for treatments for rare diseases. As this legislation moves forward, NORD urges the Committee to carefully consider the unique access and innovation issues impacting the rare disease community.

We appreciate the opportunity to share additional comments below on specific provisions in the description of the Chairman's mark. As we continue to evaluate the legislation, NORD will likely have additional, more detailed comments and will provide those to the Committee as they are developed.

Inclusion of Value of Coupons in Determination of Average Sales Price (Section 102)

NORD appreciates the necessity of ensuring that average sales price (ASP) provides as accurate an assessment as possible of what manufacturers receive for their product and agrees with the Committee's intent in incorporating the value of manufacturer assistance in the calculation of ASP.

Due to the innovative nature of many of the treatments for rare diseases and the small populations they are intended to treat, these therapies can be expensive. Thankfully, both charitable and manufacturer-based programs exist to help rare disease patients defray the cost. As noted above, addressing the high cost of drugs and other barriers to care is a priority for NORD. However, failing to support patients who are in need of immediate assistance to pay for their prescribed treatment by interfering with copay coupons prematurely could have a devastating impact on their health. As stakeholders work to lower prices, it is important that patients can continue to utilize existing mechanisms to afford critical therapies. NORD urges the Committee to ensure that patients are not inadvertently left without assistance to afford the therapies they need in this time of transition.

Biosimilars Policy (Sections 105, 107, & 128)

NORD supports policies to create incentives for the development of biosimilars. While FDA has approved 23 biosimilars to date, only a fraction is being marketed, and uptake has been very slow. This should be a concern for patients, providers, and the healthcare system overall. With improved access and usage, biosimilars could play a significant role in decreasing overall drug costs. A 2017 RAND Corporation study estimated that biosimilars could potentially result in $54 billion in health care cost savings over ten years.[2] The Chairman's mark recognizes the important role that biosimilars could play in increasing access and reducing drug costs and offers innovative policies to incentivize uptake of biosimilars. In particular, NORD is supportive of Section 105, which would increase the add-on payment for biosimilars to ASP plus 8 percent (of

[2] RAND Corporation, "Biosimilar Cost Savings in the United States; Initial Experience and Future Potential," (online at: https://www.rand.org/pubs/perspectives/PE264.html)

the reference product ASP) for five years, as well as the exemption of biosimilars from the Medicare inflation rebate provisions reflected in Sections 107 and 128.

Inflationary Rebate in Medicare Parts B and D (Sections 107 & 128)

NORD applauds the Committee's efforts to address price increases as reflected in the Chairman's mark in Sections 107 and 128. Unfortunately, rare disease patients have experienced the severe challenges associated with a therapy tripling in price overnight.

NORD agrees with the Committee's policy to permit exceptions to the inflationary rebate requirement when there are drug shortages. However, there may be additional situations in which similar exceptions are warranted. As stated in our principles, patient access is a top priority. To the extent that inflationary rebates could jeopardize patient access, NORD would be concerned about this policy.

Further, NORD is concerned that inflationary rebates could have the unintended effect of encouraging higher launch prices. In the interest of patient access and the sustainability of the health care system, NORD urges the Committee to address this possibility as the legislation moves forward.

Establishment of Maximum Add-on Payment (Section 110)

Under current law, the reimbursement system in Medicare Part B may have inadvertently created incentives for providers and hospitals to purchase the more expensive therapy, as opposed to less expensive alternatives. NORD, therefore, appreciates the intent of the Committee in Section 110's establishment of a cap for the add-on payment to average sales price (ASP).

In the case of orphan drugs, however, the need to address the possibility that incentives may have driven providers and hospitals toward more expensive therapies is largely inapplicable. Many, if not most, orphan drugs are single-source, with no therapeutic alternatives. Plus, as noted above, over 90 percent of rare diseases do not have any FDA-approved indicated treatments. Thus, add-on payments are much less likely to sway the prescribing practices of physicians and hospitals in this context because, in many cases, there is only one therapeutic option available, if there even is an option available. Rather, add-on payments in the rare disease space are key to enabling physicians and hospitals throughout the country to provide orphan therapies, which are often difficult and complicated to administer, to our patient populations. NORD urges the Committee to ensure that limits to add-on payments will not have the unintended effect of limiting patient access to necessary orphan drugs.

Medicare Part D Benefit Redesign (Section 121)

Within the rare disease community, many patients in Medicare Part D face serious challenges to accessing medications and paying the high out-of-pocket (OOP) costs, particularly if their drug(s) is on a specialty tier. Therefore, NORD is supportive of the concept of having an OOP cap for Medicare Part D beneficiaries, as proposed in the Chairman's mark. NORD also urges the Committee to consider a monthly OOP cap so that total OOP costs could be distributed more

evenly throughout the year. This type of relief could be helpful to Medicare beneficiaries who would reach the OOP cap almost immediately in the benefit year.

While NORD is supportive of the OOP cap policy in the Chairman's mark, we urge the Committee to carefully consider other aspects of the benefit redesign and whether these policies could inadvertently incentivize higher drug prices or result in formulary restrictions that prevent access to certain drugs.

Conflict of Interest (Sections 201, 202)

Pharmacy and therapeutics committees (P&T committees) and drug use review (DUR) boards play an important role in ensuring that coverage and payment decisions about covered outpatient drugs are based on sound science. These entities should make such decisions in an unbiased and conflict-free manner. NORD supports the intent of the Chairman's mark in addressing this goal.

However, NORD is concerned that the conflict of interest provisions in the Chairman's mark may be too restrictive in the context of evaluations of therapies necessary for the rare disease community. Of particular concern are Sections 201 and 202, which would require the P&T committee or DUR to include at least one practicing physician and one practicing pharmacist who are independent and free of manufacturer, Medicaid plan, and PBM conflicts of interest. That same P&T committee member would also be required to have expertise in the care of at least one Medicaid-specific beneficiary population. This requirement may not be feasible within the rare disease community and may preclude medical experts with specific knowledge about a particular rare disease from participating in these important P&T committees.

In many rare diseases, the pool of individuals who possess the requisite knowledge and experience about a particular rare disease is highly limited. Therefore, it is not unusual for an entire stakeholder community to be deemed "conflicted." The P&T committee and DUR are responsible for ensuring that Medicaid covered outpatient drugs prescriptions are appropriate, medically necessary, and unlikely to result in adverse reactions. We urge the Committee to consider the need for a more flexible conflict of interest provision when it comes to the rare disease community.

Risk-Sharing Value-based Agreements for Covered Outpatient Drugs under Medicaid (Section 208)

As the Committee notes, the science around gene therapies has progressed significantly in recent years. By some predictions, there are expected to be over 40 gene therapies on the market by the year 2030.[3] Many of these therapies are administered only once, yet they have the potential to save lives. This is an exciting day for rare disease patients because a majority of these medicines are expected to be indicated for orphan populations (under 200,000 individuals in the U.S.).

[3] MIT NewDigs, "Projections from the existing pipeline of cell and gene therapies: Launches and patient numbers," (online at: https://newdigs.mit.edu/sites/default/files/FoCUS%20Research%20Brief%202018F210v027.pdf).

The science demonstrating the safety and efficacy of these products has been years in the making and the manufacturing is complex. Consequently, these life-saving medicines come with a substantial price tag. The companies that have invested in this space deserve to be rewarded.

However, these medicines cannot fulfill their promise if patients, and the healthcare system overall, cannot afford to pay for them. Section 208 takes some helpful steps toward ensuring that states can employ innovative payment mechanisms to spread the cost to the state out over time and to ensure that only those therapies with demonstrable value according to patients are eligible. Section 208 also ensures that Medicaid can continue to access rebates that are so essential to the sustainability of Medicaid programs throughout the country. NORD supports the intent of Section 208 and looks forward to continuing to engage with the Committee on this important provision.

Modification of Maximum Rebate Amount under Medicaid Drug Rebate Program (Section 209)

NORD appreciates and supports the Committee's efforts to address inappropriate drug price increases. There have been far too many instances in which patients have suddenly become unable to afford vital therapies due to sudden and unexplained increases in cost. NORD is concerned, however, that raising the rebate cap in Medicaid could have the unintentional and negative consequence of limiting access. If manufacturers are forced to sell therapies at a loss, it is possible that rather than continue to do so, manufacturers will no longer supply their products to Medicaid programs. This could result in particularly bad outcomes for rare disease patients on Medicaid whose disease disproportionately impacts Medicaid beneficiaries, such as cystic fibrosis.[4] This policy could also have the unintended effect of incentivizing higher launch prices, which could hurt those individuals attempting to access therapies outside of Medicaid. NORD urges the Committee to ensure that patients will not ultimately lose more than they gain under this provision.

NORD appreciates the Committee's efforts to address rising drug prices and patient OOP costs. NORD stands ready to work with the Committee on this legislation as it moves forward. If you have any questions or need further information, please contact Rachel Sher, NORD's Vice-President of Policy and Regulatory Affairs at (202) 588-5700.

Sincerely,

Peter L. Saltonstall
President and CEO

[4] MACPAC, "Report to Congress on Medicaid and CHIP," (online at: https://www.macpac.gov/wp-content/uploads/2019/06/June-2019-Report-to-Congress-on-Medicaid-and-CHIP.pdf)