



June 25, 2019

The Honorable Lamar Alexander, Chairman  
Committee on Health, Education, Labor & Pensions  
United States Senate  
Washington, D.C. 20515

The Honorable Patty Murray, Ranking Member  
Committee on Health, Education, Labor & Pensions  
United States Senate  
Washington, D.C. 20515

Dear Chairman Alexander and Ranking Member Murray:

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) thanks you for advancing the *Lower Health Care Costs Act of 2019* (S. 1895) to markup in the Senate Committee on Health, Education, Labor and Pensions (HELP) on June 26, 2019.

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the over 270 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 strongly 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 legislation aimed at improving care and lowering costs for all patients. NORD submitted comments on June 5, 2019, on the initial draft legislation, which are attached for your reference. Below, we reiterate those comments and our support for the provisions that have remained in the introduced bill. We also provide additional comments on the provisions that have been newly included in the bill.

### **Title I: Ending Surprise Medical Bills:**

NORD applauds the Committee for taking this important step to prevent surprise medical bills from harming patients. Particularly for rare disease patients, emergencies can be frequent and bewildering. At these times, individuals do not have the time, or perhaps even the capacity, to ensure they are being treated at an in-network facility by an in-network provider. NORD supports the provisions contained within this legislation that neutralize the cost of surprise medical bills for patients.

### **Title II: Reducing the Prices of Prescription Drugs:**

Far too many individuals with rare diseases and their families struggle under the weight of the exorbitant costs to maintain their health. NORD continues to support the provisions of Title II that remain in the introduced legislation that are aimed at increasing patent transparency and lowering the cost of medicines by facilitating the entry of generics and biosimilars.

Additionally, NORD supports the inclusion within the Manager's Amendment of Section 214, which reflects the *Creating and Restoring Equal Access to Equivalent Samples (CREATES) Act of 2019* (S.

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340). The CREATES Act would give generic and biosimilar manufacturers a clear and efficient pathway to obtaining brand samples, ultimately allowing for earlier approvals. The bill would simultaneously ensure patient safety by requiring that the Food and Drug Administration (FDA) only authorize qualified manufacturers to receive these samples when the brand is subject to REMS requirements. The CREATES Act would also address another practice that delays generic and biosimilar competition—specifically, when brand companies deny generic and biosimilar access to an FDA-approved single, shared REMS program.

NORD is also supportive of Section 213, which would enable FDA to identify and compel generic manufacturers to update the labels of generic drugs for which the reference product has been discontinued. Granting FDA this new authority will help patients and providers obtain the most accurate labeling information for generics that have been unable to change their label as a result of the reference product's label remaining the same.

Finally, NORD is supportive of Section 210, which would require that all orphan therapies, regardless of their designation date, comply with FDA's clinical superiority standards. Without a clinical superiority requirement for all designations made prior to the *Food and Drug Administration Reauthorization Act's* (FDARA) enactment, these therapies can receive designation, approval, and Orphan Drug Act (ODA) incentives for identical products that have already been approved by FDA. This increases the incentive to generate “copy-cat” drugs that do not improve the lives of rare disease patients.

Furthermore, this allows therapies designated and approved prior to FDARA's enactment to receive successive designations, approvals, and exclusivity periods, thus, potentially blocking generic and biosimilar entry for many years. Without a clinical superiority requirement, companies could incrementally change their therapy in innumerable ways and still receive approvals and ODA benefits.

### **Title III: Improving Transparency in Health Care:**

NORD supports the aspects of this legislation that address the need for greater transparency within the health care delivery system. As stated in our previous comments, rare disease patients need to have readily available access to straightforward information, including what costs they will face, what providers are in-network, and what brokers they can, or cannot, trust to deliver unbiased advice. With additional, easily accessible information, patients will have greater control over their care and will be able to make decisions that better comply with their finance and health needs.

### **Title IV: Improving Public Health:**

NORD commends the Committee's actions on vaccine awareness and is supportive of these provisions. Vaccines are critical to creating the kind of herd immunity that many within the rare disease community rely on due to their compromised immune systems.

### **Title V: Improving the Exchange of Health Information:**

NORD supports the measures within this legislation to expand upon the Blue Button Initiative. As stated previously in our June 5, 2019, comments, greater access to information give rare disease patients more control over their health care and enables them to make the best decisions for their unique situations.

NORD thanks you for advancing this bipartisan legislation. We look forward to working with the Committee to ensuring lower costs and better care for rare disease patients by enacting these important provisions. For questions regarding NORD or the above comments, please contact me at [rsher@rarediseases.org](mailto:rsher@rarediseases.org) or 202-545-3970.

Sincerely,

/s/

Rachel Sher  
Vice President, Policy and Regulatory Affairs