



June 5, 2019

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

The Honorable Patty Murray, Ranking Member
United States Senate
Committee on Health, Education, Labor & Pensions
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 the opportunity to comment on your bipartisan draft legislation, the *Lower Health Care Costs Act of 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. Far too many individuals with rare diseases and their families struggle under the weight of the exorbitant costs to maintain their health. NORD commends the Committee on Health, Education, Labor & Pensions (HELP) on releasing this thoughtful and comprehensive draft aimed at addressing the high cost of health care for patients and is pleased to provide the following comments on the *Lower Health Care Costs Act of 2019*:

Title I: Ending Surprise Medical Bills

NORD applauds the Committee's efforts to eliminate surprise medical bills for patients. Rare disease patients in particular, unfortunately, often have experienced the stress of surprise medical bills as a variety of medical emergencies can accompany many of the over 7,000 known rare diseases. In times of emergency, 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, and it is unfair to expect as much.

All of these options will address the core problem of relieving patients of the burden of surprise medical bills. Rare disease patients should not be left with any undue financial burden nor lose access to necessary providers under any proposal to address these problems. Rare disease patients should also not bear any administrative burden in any proposal put forward. Finally, we ask that the Committee ensure that the same guarantee for in-network fees apply to any transfer that may result from patients opting for in-network facilities or providers once stabilized following an emergency admission pursuant to Section 102(a).

Title II: Reducing the Prices of Prescription Drugs

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NORD supports Sections 201 and 202, which would increase the transparency and accessibility of information on patents protecting drugs and biologics. This transparency is a key element of ensuring competition from generic drugs and biosimilars, which provide rare disease patients with more affordable alternatives to the medicines they need. Today, developers of generics and biosimilars face challenges in determining the scope and extent of patent protection on reference products. Greater transparency of this patent information will, therefore, ultimately foster earlier access to these products. NORD encourages the Committee to ensure that the Food and Drug Administration (FDA) has the requisite resources to successfully implement these provisions.

Section 203 would provide FDA with additional useful tools to address abuses of the agency's citizen petition process. Certain companies have filed meritless citizen petitions in an effort to delay competition by generic drugs. Section 203 would codify in significant part FDA's 2018 guidance¹ in an effort to stem these abuses. There have been previous legislative attempts to put an end to this practice.² NORD applauds the Committee for making another effort to address this long-standing concern.

Section 204 would clarify that biologics transitioning to the regulatory framework under Section 351 of the Public Health Service Act in March 2020, pursuant to the Biologics Price Competition and Innovation Act of 2009 (BPCIA),³ will not be eligible for an additional 12-year term of exclusivity by virtue of this transition. This provision will ensure that products that already had the opportunity to reap the benefits of Hatch-Waxman exclusivity are not also eligible for exclusivity under the BPCIA, thereby potentially blocking biosimilars. NORD believes this provision is an important step to take in helping foster a thriving biosimilars market for rare diseases.

Section 206 would create much-needed additional educational resources on biologics. Given their novelty and complexity, biological products remain a significant source of confusion among the rare disease community. Little is understood about the biosimilar pathway and how FDA assures the safety and effectiveness of these products. This education is critical among the rare disease community and their providers. Additional educational materials from FDA would be an important and helpful step toward addressing this problem. It is NORD's hope that greater understanding will lead to greater use.

NORD supports the inclusion of Section 207, which would help to remove unnecessary hurdles in FDA's review of biologics and biosimilar products and, therefore, ultimately speed access to these critical medicines. Under current law, biologics, like drugs, must comply with U.S. Pharmacopeia (USP) compendial standards or monographs. Biologics, and biosimilars, are far more complicated and are in need of a more nimble and flexible regulatory paradigm, which FDA has acknowledged.⁴ Specifically, FDA has indicated that "monographs for biological products may impede or delay innovative technology and present an additional, unnecessary burden on regulated industry."⁵ Importantly, Section 207 would allow FDA and sponsors to utilize USP monographs when they exist and are appropriate. The provision would merely permit FDA to exercise its scientific judgment about which monographs to

¹ FDA, Draft Guidance, *Citizen Petitions and Petitions for Stay of Action Subject to Section 505(q) of the Federal Food, Drug, and Cosmetic Act*, October 2018 (online at: <https://www.fda.gov/media/117884/download>)

² See, e.g., Section 914, FDA Amendments Act of 2007 (P.L. 110-85).

³ Biologics Price Competition and Innovation Act of 2009, Section 7002(e), PPACA, (P.L. 111-148)

⁴ FDA, Letter from Peter Marks, Director, Center for Biologics Evaluation and Research, and Janet Woodcock, Director, Center for Drug Evaluation and Research, to Ronald T. Piervincenzi, PhD, CEO, The United States Pharmacopeial Convention, March 28, 2018 (online at: <https://www.fda.gov/media/112103/download>).

⁵ *Id.*

apply and when. NORD hopes that relieving biological products from the need to comply with a blanket requirement on USP monographs will foster innovation and lead to greater use of biologics and biosimilars among rare disease patients.

Title III: Improving Transparency in Health Care

NORD is supportive of Sections 301, 303, 304, 305, 308, and 309, which, collectively, would utilize transparency to improve health care delivery. 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.

Section 301 would remove gag clauses that prevent the release of information regarding price and quality information related to health care services. Individuals with rare diseases should have access to information that allows them to determine the cost and quality of certain health care services, but gag clauses can prevent this goal. By prohibiting these clauses, patients will be permitted access to information on price and quality of services that can help them be better-informed consumers of health care. NORD supports this provision and its intention to cut costs for patients.

Section 303 would create a nongovernmental, nonprofit entity with the task of collecting and analyzing deidentified health care claims data with the goal of finding ways to cut costs and increase efficiency. NORD supports this endeavor and is excited about the positive, cost-cutting impact that the recommendations of this entity could have. We do, however, recommend that the Committee explicitly require that a patient representative be included among the Advisory Committee members.

Section 304 would require insurers to provide patients with updated, online directories detailing all in-network providers. This provision would also protect patients by guaranteeing in-network cost-sharing if a patient can demonstrate that the provider was listed as in-network by the health plan. Rare disease patients often report difficulties associated with determining what providers or facilities are in-network. Consequently, NORD supports this provision to protect and inform patients.

Section 305 would improve transparency and timing around health care billing practices. In order to plan, patients need to know which services they are being charged for and for how much. Patients also need the flexibility of 30 business days to be able to pay these bills. For these reasons, NORD supports this policy.

Section 308 would require disclosure of information critical to allowing plan sponsors and patients to make informed decisions pertaining to brokers. Similar to the ban on gag clauses, knowing whether brokers and consultants have received direct or indirect compensation for referral of services will help plan sponsors and patients to be informed consumers. NORD supports this level of transparency and the power that it bestows to patients.

Finally, Section 309 would obligate providers and health plans to provide cost-sharing estimates for any services within 48 hours of a request. Cost-sharing information is central to a patient's ability to financially plan and consciously shop, yet it is not always readily available. NORD commends the Committee for requiring this essential information sharing that will undoubtedly help patients to better plan for their health care expenses.

Title IV: Improving Public Health

NORD is supportive of Sections 401 and 402 as vaccines are particularly important to the rare disease community. Many of the diseases that are prevented with vaccines, or that could be prevented with vaccines, are rare diseases. Additionally, many within the rare disease community have a compromised immune system and, therefore, rely heavily on the herd immunity that is established when large groups of individuals are vaccinated against various diseases. NORD believes an awareness campaign and a dissemination of grants will help increase fluency around vaccinations and their importance to public health in a way that will lead to increased vaccination rates. For these reasons, we applaud the Committee for directing resources to this deserving cause.

Title V: Improving the Exchange of Health Information

NORD supports inclusion of Section 501, which would allow patients to have electronic access to the information necessary for them to make informed decisions regarding their care. Rare disease patients often struggle to acquire this information in a timely manner and could benefit considerably from having access to everything they need to plan through application programming interfaces. NORD applauds the HELP Committee for addressing this need by expanding the Blue Button Initiative and further empowering patients to successfully manage their health care.

NORD thanks you once again for the opportunity to comment. We look forward to working with the Committee to ensure that rare disease patients will have access to quality and affordable health care. For questions regarding NORD or the above comments, please contact our Vice President of Policy and Regulatory Affairs, Rachel Sher, at rsheer@rarediseases.org or 202-545-3970.

Sincerely,

A handwritten signature in dark ink, appearing to read "Peter L. Saltonstall", written in a cursive style.

Peter L. Saltonstall
President and CEO