NORD National Organization for Rare Disorders

Testimony In Support HB 5517: An Act Concerning Cost-Sharing for Prescription Drugs
Insurance and Real Estate Committee—March 10, 2016

Good afternoon Chairman Crisco, Chairman Menga, and members of the Committee. Thank you for the opportunity to testify in support of House Bill 5517 an act that would limit coinsurance, copayments, deductibles or other out-of-pocket expenses imposed on insured individuals for prescription drugs.

My name is Lesley Bennett. I am a Stamford resident, member and volunteer patient advocate with the National Organization for Rare Disorders (NORD)...and the mother of a daughter with a rare metabolic disorder. Although my husband and I have sizable out-of-pocket costs for our daughter's specialized medical food and supplements, our expenses pale in comparison to the co-insurance costs that many Connecticut rare disease patients and their families must pay for specialty tier medications. As NORD's Connecticut State Ambassador I am testifying today on behalf of the families in our state whose children require life-sustaining specialty tier medications for the treatment of rare disorders such as tyrosinemia, many rare blood cancers, primary immunodeficiency disorders, and hemophilia.

NORD is a national non-profit headquartered in Danbury Connecticut that for more than 30 years has been the voice of the Rare Disease Community and a leader in advocating for legislation (such as the Orphan Disease Drug Act of 1983) to help patients with rare disorders obtain access to therapies and needed services to improve the quality of their lives. In the US a rare disorder is defined as a medical condition affecting less than 200,000 Americans—some disorders are so rare they affect only a few hundred people worldwide. According to NIH, more than 7000 rare disorders affecting about 30 million Americans (~10% of the population) have been identified and more than half of those living with a rare disorder are children. Rare disorders can range from a medical condition that is a minor nuisance to one that is a debilitating, chronic and often life-threatening condition.

On February 25, 2016, NORD sponsored a Rare Disease Day Awareness event at the Legislative Office Building here in Hartford. Connecticut rare disease patients and their families had a chance tell legislators their stories to raise awareness of their disorders and issues important to our community. The President of the Connecticut Hemophilia Society discussed his family's struggles trying to pay the high out-of-pocket costs for his son's life -saving medication. Hemophilia is a genetic bleeding disorder that prevents the blood from clotting properly and hemophilia patients often develop bleeding in their joints that can result in debilitating joint damage if is not treated. Hemophilia affects about 20,000 Americans and these individuals require infusions of clotting factors that can cost more than $300,000 per year. Due to the high cost, these medications are placed in the specialty tier category by insurers and patients must pay a percentage of the

cost of the drug as a co-insurance payment—which can often be higher 33% of the actual drug cost. Yes, the ACA cap has helped but many families of children with hemophilia meet that ACA-cap (almost $13,000 in out-of-pocket expenses) by the end of January every year. Many middle income families in our state with children cannot afford to pay this amount leading to skipped patient doses, going completely without treatment, or lowering doses as families try to spread the medication out over a longer period of time in order to save money—often leading to devastating complications or costly hospitalizations. No parent should have to choose between paying for a child's life-sustaining medication versus paying the rent or buying food for the rest of the family..

Hemophilia families are not the only patients at the Rare Disease Day event who told stories of struggles with high out of packet costs for specialty tier medications. The family of a 16 year old girl girl with Primary Immunodeficiency disorder who requires daily infusions of immunoglobulin to prevent life threatening infections; the family of a 14-year-old boy with Tyrosinemia needing a specialty tier medication to prevent life-threatening liver and kidney damage; and the Dad of a child with a rare blood disorder needing a medication that costs more than $7000/month—all had similar stories to tell. These high out-of-pocket payments place too high a strain on many families in our state.

I understand that that the original intent of specialty tiers was to basically incentivize the average person to choose lower-cost medications to treat more common diseases or illnesses. However, there are no generic drugs or lower cost medications for patients with rare disorders—for most rare disorders there are no cures and very few effective treatments. For those conditions lucky enough to have an FDA-approved effective treatment, patients and their families are hit very hard by high out-of-pocket costs for medications that are life-saving!

NORD believes that HB5517 has the potential to benefit many rare disease patients by limiting the out-of-pocket costs for prescribed specialty tier medications. In 2015, the Leukemia and Lymphoma Society commissioned a study (MIlliman) looking at how the proposed changes would impact costs for the average commercial insurance plan—the results show that these changes are unlikely to adversely affect the plans since specialty tier medications are used by only small part of the overall patient pool (about 1 to 5% of patients according to the 2013 Employer Health Benefits Survey from the Henry Kaiser Foundation-Health Research & Educational Trust). I respectfully urge the committee to support for HB5517.

Thank you,

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