

Public Health Committee
Legislative Office Building—Room 3004
Hartford, CT 06106-1591

February 22, 2017

Testimony in Support Proposed House Bill 6009: An Act Establishing a Rare Disease Advisory Council
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Good morning Chairman Gerratana, Chairman Somers, Chairman Steinberg, Representative Srinivasen and esteemed members of the Committee. Thank you for the opportunity to testify in support of proposed HB6009 an act that would create an advisory council to make recommendations to the General Assembly on legislation concerning rare diseases and healthcare issues affecting this community.

My name is Lesley Bennett, I am a Stamford resident and the primary caregiver to a 27 year-old daughter with a rare metabolic disorder. For the last 25 years I have been actively advocating for patients, mostly children, with rare disorders on a local, state, and national level. Currently I am the volunteer NORD State Ambassador for the Connecticut Rare Action Network (CT-RAN)—a coalition patients and their families, advocates, caregivers, physicians, nurses, genetic counselors, therapists, researchers, biotechnology companies, and lawmakers concerned with helping the more than 350,000 Connecticut residents with a rare disorder. It is the belief of Connecticut RAN members that a permanent Rare Disease Advisory Council to the Legislature made up of Connecticut residents (patients, advocates, caregivers, researchers, healthcare providers, legislators and state agencies such as DPH, DDS, DmHAS) can be a key factor in helping the state develop a comprehensive public health care plan to improve the health of rare disease patients and lower the costs associated with managing this population.

In the US a rare disorder is defined as a medical condition that affects less than 200,000 people. According to NIH over 7000 separate rare diseases have been identified that affect approximately 30 million Americans (~10% of the US population)—more than half of those living with a rare disease are children. Dr Marshall Summar, Director of Genetics and Metabolism at Children's National in Washington DC, estimates that rare diseases account for ~40% of all pediatric healthcare costs since these medical conditions are complex and very difficult to manage—many families have trouble finding treatments or obtaining needed services, insurance coverage, or support since there are very few physicians who are trained to recognize or treat these unusual medical conditions. According to the Pharmaceutical Research and Manufacturers of America (PhRMA), 95% of identified rare diseases do not have a single effective, FDA-approved treatment. Since many rare diseases start in childhood (most have devastating consequences if not detected early), and ~80% of rare diseases are caused by a genetic variations that often lead to chronic, debilitating medical conditions that may become life-threatening in a crisis; health policy experts in Europe (EURODIS) and the US (NIH) warn that healthcare costs associated with managing rare disease patients are now an important public health priority issue.

My husband and I understand all too well the huge emotional, physical, psychological, social, and **financial burden** or stress that caring for a child or adult affected by a rare disease places on the whole family, our community, and the state! Despite extensive diagnostic testing our daughter's underlying condition eludes a specific diagnosis—but we are not alone. The first major challenge all rare disease patients face is obtaining an accurate diagnosis—for many it takes years to get a correct diagnosis since there are often many steps in the process when a patient's medical condition (such as our daughter's) is evolving, degenerative, or complicated by drug reactions or environmental factors. Since our daughter Kelly has had several life-threatening reactions to medications, John and I both understand that the lack of a correct diagnosis often places the patient's life in danger and can cause enormous waste in

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healthcare spending due to treatment delays, unnecessary hospitalizations or specialist visits, or the use of medications that are often inappropriate and may even be harmful for the patient.

In January 2015, due to the terrible experience that one of his constituents faced, Representative John Hampton introduced a bill (HB6580) to create a Rare Disease Advisory Council that **would advise the Department of Public Health** on ways to improve the health and quality of life of those affected by a rare disorder (Connecticut-RAN members can't thank Representative Hampton enough for introducing this bill) The original bill was approved by the Public Health Committee after a few modifications but ended up being tabled in April 2015 when DPH Commissioner Mullin objected to the Advisory Council due to budgetary issues at DPH. In June 2015, the Public Health Committee chairs decided to help the Rare Disease Community by adding an amendment to HB6987 (*An Act Concerning Revisions to Various Public Health Statutes*) that was passed and signed into law in June 2015. Under Section 35 of Public Act 15-242 the Rare Disease Task Force **shall (1) examine research, diagnoses, treatment and education relating to rare diseases, and (2) make recommendations for the establishment of a permanent group of experts to advise the Department of Public Health on rare diseases....and submit a report to the CGA Public Health Committee by the end of January 2016.**

When I was appointed to the Connecticut Rare Disease Task Force in 2015 (patient advocate and volunteer member from NORD), it was the hope of Connecticut rare disease patients and advocates (from organizations such as NORD, Rare Disease United, Rare New England, MitoAction, and UMDF who worked on the original legislation) that this Task Force would meet before the end of the 2015-2016 legislative session and provide the Connecticut General Assembly (CGA) with recommendations for a permanent Advisory Council. In July 2016, the chairs for the Task Force were finally named and enough appointments had been made so that a meeting of the Rare Disease Task Force could take place by October 2016 (60 days after the chairs were appointed—as called for in PL15-242). When a meeting did not take place by October or the end of December 2016, several CGA members suggested that the Connecticut Rare Disease Community may want to start over on this issue and have a new bill introduced—which is HB6009 submitted by Representative Kim Rose.

Two years ago, Connecticut rare disease advocates thought that a Rare Disease Council to advise the Department of Public Health on issues concerning rare diseases would be enough to help our community. We now realize that while DPH may be able to address public health issues related to education, test procedures, epidemiology codes, or patient registries; DPH is not able to help the Connecticut Rare Disease Community with *“legislative issues”* related to insurance coverage, limiting the use of biosimilars or the *“specialty tier”* designation for rare disease therapies; insurance coverage for medical foods/formulas; addressing school plans/education issues; transition to adult care issues; streamlining the addition of newborn screening tests; ensuring access to health insurance (now that ACA is being dismantled); examining costs associated with these disorders and the long-term care for these patients; developing registries; creating regional networks to share knowledge; identifying state research priorities; or improving access to services at DSS, DDS, DMHAS, OHCA, or SDE. HB6009 is not a duplicate of HB6580 and it does not duplicate the work of the current Task Force. HB 6009 differs from the current Rare Disease Task Force charge in that it would create an advisory body to make recommendations to the *General Assembly (not DPH)* on **legislation** concerning the rare disease issues related to public health, insurance, education, and human services committees. HB6009 would also remove any DPH objections to the Advisory Council based on the current CGA budget and I believe the presence of *“legislative”* chair will make the council more effective since meetings would occur on a regular scheduled basis .

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Thank you for your time and attention to this matter. If you need any further information or have questions, please contact me at lesley.bennett@rareaction.org.