State of New Hampshire

GENERAL COURT

MEMORANDUM

DATE: **December 1, 2021**

TO: **Honorable Sherman Packard, Speaker of the House**
Honorable Chuck Morse, President of the Senate
Honorable Paul C. Smith, House Clerk
Honorable Tammy L. Wright, Senate Clerk
Honorable Chris Sununu, Governor
Michael York, State Librarian

FROM: **Representative William Marsh, Chairman**

SUBJECT: **Annual Report of the Rare Disease Advisory Council.**
RSA 126-A (HB 237, Ch. 24:1) Laws of 2019)

Pursuant to RSA 126-A (HB 237, Chapter 24:1, Laws of 2019), enclosed please find the Annual Report of the Rare Disease Advisory Council.

If you have any questions or comments regarding this report, please do not hesitate to contact me.

I would like to thank those members of the council who participated. I would also like to acknowledge all those who testified before the council and assisted the council in our deliberation.

Enclosures

cc: Members of the committee

ANNUAL REPORT

Rare Disease Advisory Council

RSA 126-A (HB 237, Ch. 24:1) Laws of 2019

December 1, 2020

Committee Membership

Representative William Marsh, Chairman	Senator Cindy Rosenwald
Representative Gary Woods, Vice Chair	Dr. Sai Cherala
Dr. Angela Shepard	Dr. Mary Beth Dinulos
Dr. Richard Lafleur	Krista Gilbert
Dr. Laura Landerman Garber	Elizabeth Shannon
Dr. Elijah Stommel	

Council Charge and Purpose:

The advisory council shall:
(a) Advise the legislature and the department of health and human services on rare diseases in New Hampshire.
(b) Coordinate with other states' rare disease advisory bodies, community-based organizations, and other public and private organizations for the purpose of ensuring greater cooperation between state and federal activities encouraging research, diagnosis, and treatment of rare diseases. Federal agencies may include, but are not limited to, the National Institutes of Health, and the United States Food and Drug Administration.
(c) Explore existing data on rare diseases in New Hampshire collected by the department of health and human services.
(d) Encourage public awareness regarding rare diseases in New Hampshire.

Findings and Recommendations:

2021 became a reorganizational year for the Rare Disease Advisory Council when the ability to hold remote meetings under the covid-19 state of emergency ended. As Rare Disease patients can be immunocompromised, either from their disease or their treatment, resuming in person meetings has been problematic. As of this writing, we have been unable to convene a quorum, and our chair has drafted this report by collating input from members, and it has been approved unanimously by all members able to meet either in person or remotely.

Relevant to our charges above, the Council accomplished the following:

(a) Advise the legislature and the department of health and human services on rare diseases in New Hampshire.

We discussed relevant legislation, including HB600 relative to funding the newborn screening panel, which was signed into law. We discussed HB191, relative to prior authorizations and patient transfers under managed care group health insurance policies, which is retained in House Commerce. We discussed HB62, relative to continued in-network access to certain health care providers, which was tabled by the House. We discussed two LSRs for 2022 – one by Rep. Woods regarding an ALS registry, and a second by Rep. Marsh, allowing our council to hold remote meetings.

(b) Coordinate with other states' rare disease advisory bodies, community-based organizations, and other public and private organizations for the purpose of ensuring greater cooperation between state and federal activities encouraging research, diagnosis, and treatment of rare diseases. Federal agencies may include, but are not limited to, the National Institutes of Health, and the United States Food and Drug Administration.

Ms. Gilbert and Representative Woods participated in Project RDAC trainings. NORD's Project RDAC is designed to optimize existing RDACs and to increase the number of RDACs across the country.

The RDAC has continued to develop relationships with other organizations including the Council for Youth with Chronic Conditions, the New Hampshire Occupational Therapy Association, the NH Council on Developmental Disabilities, the NH Rare Disorders Association, the NH Rare Action Network, the Youth and Sudden Death Committee with participation in council meetings and communication.

(c) Explore existing data on rare diseases in New Hampshire collected by the department of health and human services.

The council continues to be contacted by rare disease patients about the lack of insurance coverage for prescription medications, including low dose naltrexone. The council gathered data regarding reimbursement for low dose naltrexone in New Hampshire by insurance providers. Coverage is complicated by compounding and off-label use issues. Low dose naltrexone is not covered by Medicare. It is not covered by private carriers without prior authorization. Few prior authorization requests have been completed in the last year. There is no mechanism in place to gather data about Medicaid reimbursement. Krista Gilbert and Dr. LaFleur did meet about this issue and we are hopeful they are addressing it without the need for legislation.

(d) Encourage public awareness regarding rare diseases in New Hampshire.

Dr. Bruce Vrooman, Section Chief, Center for Pain and Spine, Associate Professor of Anesthesiology, provided education during the January meeting regarding the indications for low

dose naltrexone and lack of insurance coverage. He would like to see a pilot study in NH that includes insurance coverage.

Ms. Gilbert has reached out to Mallinkrodt, a manufacturer of naltrexone, and provided them with information about the need for it to be available in low doses for patients. Additionally, she provided the company research articles supporting the use of LDN with varied diagnosis.

Dr. Shepard and Ms. Gilbert will work with the NH Rare Disorders Association to create and host an on-demand, on-line CME program for physicians describing the benefits, indications, and dosing strategies for low dose naltrexone.

The RDAC reviewed facebook pages and homepages of other RDACs. We do not feel that it is necessary for our group at this time to develop a facebook page for the RDAC or a homepage that is independent of the NH government webpage. Notification of meetings, agendas and minutes are posted on the government webpage.

Ms. Tracy Williams was a guest in January who shared her concerns about limited supports in New Hampshire for her adult brother who has a rare disorder and developmental disability. She also shared information about her desire for the development of a group home for adults with rare disorders/ developmental disabilities in the seacoast area.

There is a state-wide staffing shortage of direct support professionals and in-home support providers. This impacts the quality of life for people with rare disorders. It places limitations on the individuals and their families/caregivers in terms of their ability to work, to care for themselves, and to remain living in the community. COVID has further reduced the workforce. Many individuals and families have been faced with the difficult choice of leaving their job to care for their loved one or placing them in a facility.

Specific issues addressed by members:

ALS Database

Dr. Shepard inquired as to what kind of infrastructure might exist for multiple medical conditions and the possibilities of data-sharing, regional study groups vs Legislature, secondary to that there are few cases which might ease the data sharing idea. She noted that there is already some infrastructure in place for data-sharing.

Youth sudden death

Dr. Dinulos is section chief of Genetics and Child Development at DHMC and a member of the NH Sudden Death in the Young (SDY) committee. This committee reviews all sudden deaths in children and infants (SUID - Sudden Unexplained Infant Death) in the state of New Hampshire. Criteria include:

a. All cases must be residents of NH

b. Children are less than 20 years old and have sudden and unexpected death

c. Includes drownings in children older than age 5 years and drivers in motor vehicle accidents

In 2019 there were 124 child deaths (ages newborn to 21 years), and 62 of those cases were "natural" deaths. We would then expect that a subset of those cases may be SUDY.

Dr. Dinulos attended (virtually) the America College of Medical Genetics and Genomics (ACMG) annual meeting this year and report on a presentation from the Baltimore and Boston groups regarding incidence of genetic variants noted in their SDY/SUID populations. They reviewed 352 cases of SUDP (ages newborn to 11 years) from 2012-2020 and found that 11-28% of SUDP cases had an identifiable genetic variant that may account for, or add to, the child's unexpected death. Testing performed was either whole exome sequencing (WES) or large panel testing that would encompass cardiac, neurologic and systemic/syndromic genes. The majority of the mutations were in cardiac and neurologic genes. The number of mutations found in the SUDP cases were much greater than those found in a control cohort. They concluded that genetic evaluation should be a part of SUDP evaluation for several reasons. First, it may provide a diagnosis for the parents who have just lost a child. Second, it would allow for medical surveillance for at-risk surviving family members. And lastly, it would aide in recurrence risk counseling for the parents.

Dr. Dinulos is advocating for genetic testing in these children with unexpected death, including comprehensive cardiac panels and comprehensive epilepsy panels. Depending on numbers and cost, it may be that large panel testing is the most cost effective route at this time, but as technology changes, so will testing algorithms. Barriers to this testing include monies to cover the cost of the testing and informed consent from legal guardians. Dr. Dinulos has been in communication with the NH medical examiners regarding this topic and will continue the conversation with Dr. Weinberg and Dr. Duvall regarding this recent data from Boston. They are scheduled to meet virtually on September 20, 2021 to discuss this issue. Dr. Shepherd and Dr. Dinulos are also working offline regarding potential funding sources for this genetic testing.

Respectfully Submitted,

Representative William Marsh, Chairman

Attachments:
Minutes from 2021 meetings