Rare Disease Advisory Council
Meeting Minutes 11/15/19

Present: Rep. Marsh, Dr. Laura Landerman-Garber, Krista Gilbert, Dr. Angela Shephard
Called in: Robert Stoker, Dr. Mary Beth-Dinulos, Dr. Elijah Stommel
Absent: Sen. Rosenwald, Dr. Richard LaFleau, Rep Osborne, Dr. Sai Cherala
Also present: Nelia Gibson, Dreamer Hubbard, Nicolas Carano (NH Hospital Assn), Abby Rogers (DHHS), Mary Morse (Special Education), Meagan Lampier, Courtney Tanner (DHMC)

Rep. Marsh called the meeting to order at 3pm.

Guests and members present were introduced.

Minutes from previous two meetings were approved.

Robert Stoker updated on his research into pricing for a database. He also mentioned researching prescribed drugs for rare disease, particularly those that are prescribed for orphan diseases. He would be interested in finding out which drugs are prescribed off formulary. Looking at councils in other states and trying to connect – mentioned Kentucky.

Dr. Stommel updated that he had received files at EPIC through DHMC and is looking into what rare diseases are treated at DHMC. He will compile a list and report out next month on his progress.
Dr. Shepard asked what query he used – he used ICD codes from the NORD website.

Krista Gilbert had contacted the Connecticut disease councils about attending this meeting, as we requested last month. CT is not is a good state to do this as they are still organizing. She has suggested a member from the North Carolina council which is housed at UNC. She suggested that perhaps the call in could be arranged for the December meeting. We will make this 1st agenda item.

Dr. Shepard updated on step therapy legislation from the NHMS that she is tracking. Other bills look at increased costs, which in turn raise health insurance premiums, and impacts health care more broadly. The bills will allow NH to import certain high cost drugs from Canada as Canada has been able to negotiate better prices. There is an accessibility bill which will prohibit and insurance company from requiring or forcing a beneficiary change to medications for non- medical reasons (i.e cheaper options). There is a transparency bill that would require manufacturers to notify the state if they have raised the price of a medication more than a certain percentage over the past 12 months. Language is not publicly available yet.

Dr. Mary Beth-Dinulos updated on the YSD committee which looks at sudden death of youth in NH. She is interested in having the ability for the medical examiner or other lab to do genetic testing, such as NGS sequencing to test for unidentified cardiac or seizure disorders. Ideally for testing, the parents and child, or sibling would submit samples for genetic testing to rule out a de novo mutation found only in the deceased child.

Dr. Stommel updated that he had spoken with Alicia Fraser, who is the point person for the ALS registry in Massachusetts. The registry was started by Dr. Knorr 15 years ago. It was originally funded with $100,000 with a nurse coordinator position and costs $150,000 per year. MA has a larger population that NH, 7 million compared to 1.3 million. There was discussion of NH tagging on to either the MA registry or perhaps the national registry. Alicia Fraser suggested frequent outreach to

providers (neurologists) by telephone on a regular basis on ALS rating criteria and to make sure they are capturing all the cases they are trying to compare. The Agency of Toxic Substance and Disease Registry (ATSDR) has studied populations of ALS cases in defined areas. Dr. Stommel talked about how to collect and store the data, perhaps in Red Cap, or EPIC. He mentioned that DHMS with residents, fellows, other physicians, and researchers may be the best place to have the data and or registry due to the ability to provide the resources necessary to maintain a registry.

Krista Gilbert updated on outreach. She suggested that the council consider hosting listening sessions on rare disease across the state perhaps using the DHHS offices as a place to hold the listening sessions. She suggested other partners such as Partners in Health, NH Developmental Disabilities Council, Able NH, and ACT – another disabilities organization.

Nelia Gibson and Dreamer Hubbard from Manchester spoke about two members of their family with Huntington Disease, a debilitating condition that leads to progressive degeneration of nerve cells in the brain. They spoke about their issues relating to law enforcement misunderstanding their family members speech or uncontrolled movements which are symptoms of the condition. There was a suggestion of having the family members care a laminated card with a description of the disease and what symptoms may be noticeable to have better understanding that this is a result of the disease process and not subordination or disorderly behavior. Dr. Shepard suggested the NH Rare Disease Association could make such cards and will report back to us.

Mary Morse, an advocate and teacher of the blind, came to the council to represent special education. There is not a special education appointee listed in the membership for the council. She would like to attend the council meetings. As it is a public meeting, Rep. Marsh welcomed her to attend.

Meagan Lampier mentioned she had a family member with Moyamoya disease, who had recently required cerebral artery bypass surgery, but was not prepared to speak. Rep. Marsh mentioned his sister had a stroke in her 30s from this disease, and had done well after similar surgery performed in Pittsburgh PA. Laura Landerman-Garber also knew a patient with this disease.

Updates will continue the next time the council meets which will be on December 20th at 3pm at 29 Hazen Drive. Between now and the next meeting date Krista Gilbert will contact the UNC and the Rare Disease Advisory Council there to see if a representative from the council could call in.

Rep. Marsh thanks Abigail Rogers for taking these minutes.

Meeting adorned at 3:50pm

Respectfully submitted, Rep. Marsh Chair