**MASSACHUSETTS RARE DISEASE ADVISORY COUNCIL (RDAC)**

**STEERING COMMITTEE**

**Meeting Minutes – Approved February 27, 2025**

REMOTE MEETING:    Thursday December 19, 2024

**Meeting Time 10:30 am – 11:30 pm**

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**Dr Dylan Tierney** welcomed all to the meeting. He then conducted a roll call to establish a quorum.

**Roll Call**

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|  | Member | Present |
| 1 | Representative Jay Livingstone | x |
| 2 | Jenn McNary | x |
| 3 | Tai Pasquini | x |
| 4 | Dr. Ryan Thompson | Joined at 10:34 |
| 5 | Dr. Dylan Tierney | x |

A quorum was established so D Tierney brought the meeting to order at 10:33.

**D Tierney** then asked if all received the minutes from the last meeting. All stated yes and no one offered any edits or revisions. D Tierney asked if there was a motion to approve the minutes as presented.

**T Pasquini** made a motion to accept the minutes. **J Livingstone** seconded.

**D Tierney** conducted a roll call vote for meeting approval.

**Roll Call Vote to approve minutes from 10.31.24 Steering Committee**

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| ***Member*** | ***Approve*** |
| Representative Jay Livingstone | x |
| Jenn McNary | x |
| Tai Pasquini | x |
| Dr. Ryan Thompson | x |
| Dr. Dylan Tierney | x |

**Discuss priorities for 2025** D Tierney then asked the committee what we want to do about priorities for 2025.

Some of the priorities discussed in 2024 included:

* 1. **Medical Nutrition-** what do we want to do about medical nutrition?

**J McNary –** added that she would recommend that we look more into the topic.

**R Thompson –** stated that he agreed with J McNary. He stated that after review of the NORD report card on Medical Nutrition, he realized that the reason for the B grade seemed to be about insurance coverage. He suggested getting more information from Medicaid about what they pay for. Maybe we then review the criteria as a blanket approval may not be best either.

**J McNary –** suggested getting a speaker with expertise in medical nutrition and coverages for it. She suggested that she would find it hard to believe that someone would request medical nutrition if they didn’t’ need it.

**T Pasquini –** stated that she felt that there may be a grey line on the definition of medical nutrition. Maybe asking the insurance carriers for their definition of medical nutrition. She also thought that it may be a good idea to find out why people are denied coverage.

**J McNary-** agreed that looking into coverages from medical nutrition may be a good next step.

* 1. **The economic burden of rare disease in MA** (should we form a committee to structure and oversee the project?) **D Tierney** asked what people thought about pursuing the this.

**T Pasquini –** Suggested that we may want to look at many of these topics as an ACCESS issue. For example, when we talk about access to medical nutrition, we can also talk about access to specialty provider and resources, and access to healthcare through telehealth. She stated that it just seemed like many of the topics could be organized under an ACCESS header. Tai went on to talk about the burden of rare disease and is very interested in learning more about the burden. She also noted that this study may help flush out some of the access issues.

**J McNary –** stated that we should still identify the access issues by name. She would like to include access to medications. She wanted to add that homecare has been a very challenging access issue because there are not enough homecare personnel to help the people who need it.

**D Tierney –** expressed concern about taking on too much. How do we come up with meaningful and realistic recommendations. I want us to think about being clear about our priorities. The question is, what is the bandwidth for us to dig into these topics.

* 1. **Transition from pediatrics to adult care - D Tierney** asked the group what they thought about the topic of transition from pediatric care to adult care. We know that this is a priority that the commissioner mentioned so maybe this is a topic we can explore more. He asked what people thought about that?

**Rep Livingstone-** He felt that the topic of transition from pediatric to adult care for rare disease patients is a topic that may be limited in scope. Maybe this is a topic that we could provide some concrete recommendations. He then discussed that the economic burden is clearly a topic that we all can relate to . He would recommend pursuing this study.

**T Pasquini –** thinks that it would be helpful to learn from the community about the problematic issues for those with rare disease. She knows first-hand, that there are huge primary care shortages in Western Massachusetts. Could we look at what other groups are working on.

**D Tierney –** agreed that working in a vacuum is not helpful. He suggested that the policy workgroup may be able to do some investigation into this.

**R Thompson-** As Jenn talks about homecare coverages, that is a major issue for my patients. Although this is a huge issue, how do we take on such a huge issue. When we talk about pediatrics to adult care, transitions is a big issue.

One of the ideas we have touched on over the past year, maybe would be helpful to revisit for things like this. For example, an ombudsman, who could help the primary care doctor find placement or services that their patient needs. An example is when I have a patient that I’m having a hard time finding post-acute placement, we reach out to a state funded ombudsman. Her name is Mary Lou Gamache. She is amazing at working behind the scenes to help with placement. This is very specific but maybe a hotline or a case manager that has expertise in rare disease could be a good resource for people. This person could make the connections that people need. When there are issues that are really hard to solve, this could be a valuable resource for providers, patients and families.

**D Tierney –** thought that maybe this idea was put aside because we wanted to learn more from the community. Maybe we keep this on our priority list and we will look to get more information about how to do this.

The final issue is related forming a rare disease registry. The commissioner is interested in this topic so we may want to explore this topic also.

**T Pasquini** – This topic is huge for her. She is very interested in learning more about how the commissioner would approach this. She is not in favor of a patient reported registry, would not be a good idea. She has done a lot of work in this area and is most concerned about the approach.

**D Tierney** – asked if there was a way to narrow down the list. How many issues can we go more deeply on. We don’t have a lot of bandwidth.

**T Pasquini –** asked if we could leave all the topics on the list. She felt that it was important to keep the issues we identified as our priorities. Maybe we see if we can engage more members to do some of the work. She felt that three topics could be possible.

**D Tierney –** Stated that the three topics we have identified are

1. The economic burden of rare disease
2. Transition from pediatric to adult care
3. Access, medical nutrition etc.

**Rep Livingstone –** advocated that the economic burden will help us better understand access issues. He is in favor of pursuing economic burden as well as the transition from pediatric to adult care.

**R Thompson –** Agreed that focusing on the economic burden, transition of care from pediatrics to adult care as well as the registry. He suggested pulling together a roundtable or a group of experts in this area to better understand the details.

**D Tierney** stated in summary that we all agree to keep all the topics on our priority list however we will focus on medical nutrition, the economic burden, which may help us better understand the other access issues, and then work on the transition of care from pediatric to adult care for those with a rare disease.

We will look for opportunities to talk about all the issues we discussed.

**R Thompson** asked that we keep the ombudsman issue on the table. This may help everyone with all the issues.

**T Pasquini –** Asked if we keep all the topics on our list. If the commissioner wants to have a discussion about a registry, we should have that discussion.

**D Tierney -** stated that we were looking into getting a speaker to talk about the transition of care from pediatrics to adult care. Maybe we can look at getting a speaker to talk about 211 and also maybe look into the state position of an ombudsman that I talked about.

He then gave all an update on the submission of the annual report. She stated that the report was complete and was working along the submission process.

He then let everyone know that Dr. Olaf Bodamer has been asked to be part of the NBS Advisory Committee. He will be able to give the council updates on the work of the NBS Advisory.

**Rep Livingstone** summarized how we put forth recommendations. We first want to identify an issue. Next we highlight patient stories or research/data that show the issue, then we set forth a recommendation based on what others are doing, what evidence has shown effective.

He recommended a schedule of things. The next fiscal year starts in July. The budget usually comes out in January, followed by the legislature and ideally the final budget is set in June. If we want to do anything related to a budget, we should present our information in the first of the year.

Bills are usually filed in January. There are opportunities to file later than that but if it’s filed later than January 17, there is no guarantee that the topic will be brought up.

He then updated the steering committee on the following.

* + **Membership –** we are still waiting for appointments for fill the council. All nomination packets have been sent to the appointing body. One nominee, a rare disease patient, has passed away so we will be submitting a new recommendation for a person with a rare disease who is over 18. Maybe ask if there are any recommendations.
  + **Membership on the steering committee** – we still need 2 more members for the steering committee. We need a person living with a rare disease (was Michelle Rhee) and a clinician (was David Miller).
  + **Meeting schedule for 2025 –** Mary Lou will be sending a full year meeting schedule for 2025. Meetings are on the third or fourth Thursday depending on school vacations and holidays.
  + **Rare Disease Day in Massachusetts –** He let all know that he has been invited to speak on a panel with Rep Livingstone on Rare Disease Day.

He then asked for a motion to adjourn; T Pasquini made a motion to adjourn

Seconded by Rep Livingstone . All were in favor so the meeting was adjourned at 11:33am