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

**Meeting Minutes**

Approved September 15, 2022

REMOTE MEETING:    Thursday, July 28, 2022, 9:00-11:00 AM

Welcome – Dr. Dylan Tierney, Council Chair, welcomed everyone to the meeting.

Roll Call and Quorum- Dr. Tierney conducted a roll call vote to establish quorum.

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| --- | --- | --- | --- |
|  | **Council Member** | **Present** | **Time arrived** |
| 1 | Charlotte M. Boney, M.D. | no |  |
| 2 | Janis Creedon | X |  |
| 3 | Ms. Lisa Deck | x |  |
| 4 | Andrew A. Dwyer, PhD, FNP-BC, FNAP, FAAN | no | 9:10 |
| 5 | Michael R. Green, M.D., Ph.D. | X |  |
| 6 | Julie D. Gortze | X |  |
| 7 | Guadalupe Hayes-Mota | X |  |
| 8 | Lena Joseph | X |  |
| 9 | Representative Hannah Kane | X |  |
| 10 | Andrew A. Lane, MD, PhD | X |  |
| 11 | Representative Jay Livingstone | no | 9:10 |
| 12 | Jeff R. Livingstone, PhD | X |  |
| 13 | Diane Lucente, MS, LCGC | x |  |
| 14 | Alexsandra B. Mahady | X |  |
| 15 | Jenn McNary | no |  |
| 16 | David T. Miller, MD, PhD | X |  |
| 17 | Tai Pasquini, PhD, MPA (Sen. Bruce Tarr) | X |  |
| 18 | Shivang Patel, Pharm.D. | X |  |
| 19 | Asma Rashid, MS, CGC | X |  |
| 20 | Michele Rhee, MBA, MPH | X |  |
| 21 | Robert E. Schultz | no | 9:13 |
| 22 | Celia Segel | X |  |
| 23 | Michael Sherman, MD | X |  |
| 24 | Ms. Glenda E. Thomas | X |  |
| 25 | Ryan Thompson, MD, MPH | no | 9:15 |
| 26 | Dylan Tierney, MD, MPH | X |  |
| 27 | Ann Wessel, MS, RD, LDN | X |  |
| 28 | Ross Zafonte, DO | no | 9:30 |

A quorum was established, and Dr. Tierney called the meeting to order at 9:05

Approval of minutes from the last meeting (6/23/22).

Dr. Tierney asked if all had read the minutes and if anyone had any edits or corrections. No one responded. Dr. Tierney then asked if he could get a motion to accept the minutes as presented.

Dr. Sherman made a motion to vote on the acceptance of the minutes from 6/23/22.

Rep. Kane seconded the motion.

**VOTE** - Dr. Tierney conducted a roll call to accept the minutes as presented.

|  |  |  |
| --- | --- | --- |
|  | **Council Member** | **approve** |
| 1 | Charlotte M. Boney, M.D. | - |
| 2 | Janis Creedon | X |
| 3 | Ms. Lisa Deck | x |
| 4 | Andrew A. Dwyer, PhD, FNP-BC, FNAP, FAAN | x |
| 5 | Michael R. Green, M.D., Ph.D. | X |
| 6 | Julie D. Gortze | X |
| 7 | Guadalupe Hayes-Mota | X |
| 8 | Lena Joseph | X |
| 9 | Representative Hannah Kane | X |
| 10 | Andrew A. Lane, MD, PhD | x |
| 11 | Representative Jay Livingstone | x |
| 12 | Jeff R. Livingstone, PhD | x |
| 13 | Diane Lucente, MS, LCGC | x |
| 14 | Alexsandra B. Mahady | x |
| 15 | Jenn McNary | x |
| 16 | David T. Miller, MD, PhD | x |
| 17 | Tai Pasquini, PhD, MPA (Sen. Bruce Tarr) | x |
| 18 | Shivang Patel, Pharm.D. | x |
| 19 | Asma Rashid, MS, CGC | x |
| 20 | Michele Rhee, MBA, MPH | - |
| 21 | Robert E. Schultz | x |
| 22 | Celia Segel | x |
| 23 | Michael Sherman, MD | X |
| 25 | Ms. Glenda E. Thomas | X |
| 26 | Ryan Thompson, MD, MPH | X |
| 27 | Dylan Tierney, MD, MPH | x |
| 28 | Ann Wessel, MS, RD, LDN | X |
| 29 | Ross Zafonte, DO | - |

Dr. Tierney introduced today's speaker Glenda Thomas

**Glenda Thomas,** Council member, and patient advocate started her presentation by sharing her journey to a diagnosis of a rare disease. She stated that it took 2 years to receive a diagnosis of Myasthenia Gravis, also referred to this as MG.

Glenda explained that Myasthenia Gravis or MG is a chronic autoimmune, neuromuscular disease that causes weakness in the skeletal muscles. Symptoms worsen after periods of activity and improves after periods of rest. Muscles responsible for functions like breathing and moving parts of the body, including the arms and legs are most affected.

She stated that her rare disease has created significant disruption to her life. She can no longer work the job that she enjoyed and excelled at for so many years. She often needs to rely on others for help and to drive her to appointments when her disease prevents her from driving.

She stated that there are medications to treat MG, but they are often very expensive and not always covered by insurance. Her current treatment is helping but it's been a slow process. After 12 months of treatment, she started to see some improvement. Now 9 years later, she is still slowly improving and is thankful she has found a drug that helps.

Glenda discussed some of the challenges that people diagnosed with MG face. She stated that MG affects all ages however there are no clinical trials for children under age 16. Her biggest challenge has been finding a neurologist that understands her diagnosis.

One of the priorities she would like to see the council address is educating providers, both primary care and specialty providers about rare diseases.

She would like to see the council help patients and providers to better understand the rare diseases that affect Massachusetts residents.

Dr. Tierney thanked Glenda for her bravery to talk about her diagnosis. He stated that hearing from patients like her helps ground the council and aids all members to better understand why the council has important work to do.

Dr. Tierneyasked everyone to read the proposed mission statement. He explained that the proposed mission statement was approved by the steering committee on 7/14/22.

Dr. Tierney read the statement aloud;

“The Massachusetts Rare Disease Advisory Council’s mission is to provide guidance and recommendations on rare disease incidence, the status, and the needs of the rare disease community to the governor, the legislature, and the department of public health with the goal of improving the lives of people impacted by a rare disease in the commonwealth.”

He asked if there were any comments or questions.

Dr. Livingstone asked if it should include the rare disease community in the statement.

Dr. Tierney stated that the focus of the legislative language was for the council to provide guidance and recommendations to the governor, the legislature, and the department of public health. He stated that the public and rare disease community will be a recipient of the work of the council.

Dr. Lane asked if we should be making recommendations on other things besides incidence and the status of the rare disease community. He asked if it made sense to add language such as needs and burden of rare diseases to the statement.

Dr. Dwyer stated that there are often other forms of information besides incidence that would be helpful to know. Information about the things that impact the lives of those with rare diseases.

Rep. Kane explained that the mission statement should be broad and that once we get into the specific tasks and activities, we could focus on some of those specifics.

Dr. Tierney summarized the discussion and provided a modified version of the mission statement based on the discussion.

REVISED to:

“The Massachusetts Rare Disease Advisory Council’s mission is to provide guidance and recommendations on rare disease incidence and the status and needs of the rare disease community to the governor, the legislature, and the department of public health with the goal of improving the lives of people impacted by a rare disease in the commonwealth.”

Dr. Tierney asked what people thought of the statement as revised.

Dr. Pasquini agreed with the revision

Dr. Patel also agreed with the revision.

Dr. Tierney asked if there were any other thoughts before moving to a vote.

No response.

Dr. Tierney asked if there was a motion to move to vote on the revised mission statement.

Dr. Miller made a motion to accept the mission statement as revised.

Dr. Pasquini made a second.

Dr. Tierney conducted a roll call vote to accept the revised mission statement as presented.

**Roll Call VOTE**

|  |  |  |
| --- | --- | --- |
|  | **Council Member** | **approve** |
| 1 | Charlotte M. Boney, M.D. | - |
| 2 | Janis Creedon | X |
| 3 | Ms. Lisa Deck | X |
| 4 | Andrew A. Dwyer, PhD, FNP-BC, FNAP, FAAN | X |
| 5 | Michael R. Green, M.D., Ph.D. | x |
| 6 | Julie D. Gortze | x |
| 7 | Guadalupe Hayes-Mota | x |
| 8 | Lena Joseph | x |
| 9 | Representative Hannah Kane | x |
| 10 | Andrew A. Lane, MD, PhD | x |
| 11 | Representative Jay Livingstone | X |
| 12 | Jeff R. Livingstone, PhD | X |
| 13 | Diane Lucente, MS, LCGC | X |
| 14 | Alexsandra B. Mahady | X |
| 15 | Jenn McNary | x |
| 16 | David T. Miller, MD, PhD | X |
| 17 | Tai Pasquini, PhD, MPA (Sen. Bruce Tarr) | x |
| 18 | Shivang Patel, Pharm.D. | X |
| 19 | Asma Rashid, MS, CGC | X |
| 20 | Michele Rhee, MBA, MPH | - |
| 21 | Robert E. Schultz | X |
| 22 | Celia Segel | X |
| 23 | Michael Sherman, MD | X |
| 25 | Ms. Glenda E. Thomas | X |
| 26 | Ryan Thompson, MD, MPH | X |
| 27 | Dylan Tierney, MD, MPH | X |
| 28 | Ann Wessel, MS, RD, LDN | X |
| 29 | Ross Zafonte, DO | - |

**Subcommittees** - Dr. Tierney presented the steering committee proposal to add three new subcommittees. He reviewed the charges by the legislature and walked through the slides. He reviewed the specific charges for each subcommittee as voted on during the previous steering committee.

Dr. Tierney asked attendees to refer to the document titled RDAC Subcommittee Structure.

After review, Dr. Tierney asked if anyone had thoughts, questions, or comments.

Rep. Livingstone asked why the council would need funding.

Rep. Kane responded that there might be activities that require funding. Whether conducting a survey to inform the council or holding an event to educate the public, would take funding. She would expect the fundraising committee to work on a fundraising plan.

Dr. Tierney asked if the members were ready to vote on adding three new subcommittees.

Dr. Tierney asked if anyone could make a motion.

L. Deck made a motion to vote on accepting the addition of 3 new subcommittees.

Research, Advocacy and Policy, and Fundraising.

R. Shultz made a second.

Dr. Tierney conducted a roll call vote to approve the three new subcommittees as presented.

**Roll Call VOTE**

|  |  |  |
| --- | --- | --- |
|  | **Council Member** | **approve** |
| 1 | Charlotte M. Boney, M.D. | - |
| 2 | Janis Creedon | X |
| 3 | Ms. Lisa Deck | x |
| 4 | Andrew A. Dwyer, PhD, FNP-BC, FNAP, FAAN | x |
| 5 | Michael R. Green, M.D., Ph.D. | x |
| 6 | Julie D. Gortze | x |
| 7 | Guadalupe Hayes-Mota | x |
| 8 | Lena Joseph | X |
| 9 | Representative Hannah Kane | X |
| 10 | Andrew A. Lane, MD, PhD | X |
| 11 | Representative Jay Livingstone | x |
| 12 | Jeff R. Livingstone, PhD | X |
| 13 | Diane Lucente, MS, LCGC | x |
| 14 | Alexsandra B. Mahady | x |
| 15 | Jenn McNary | x |
| 16 | David T. Miller, MD, PhD | x |
| 17 | Tai Pasquini, PhD, MPA (Sen. Bruce Tarr) | x |
| 18 | Shivang Patel, Pharm.D. | x |
| 19 | Asma Rashid, MS, CGC | X |
| 20 | Michele Rhee, MBA, MPH | - |
| 21 | Robert E. Schultz | x |
| 22 | Celia Segel | x |
| 23 | Michael Sherman, MD | x |
| 25 | Ms. Glenda E. Thomas | x |
| 26 | Ryan Thompson, MD, MPH | x |
| 27 | Dylan Tierney, MD, MPH | X |
| 28 | Ann Wessel, MS, RD, LDN | X |
| 29 | Ross Zafonte, DO | - |

Dr. Tierney stated that the next agenda item was to discuss priorities for the council. He introduced Rep Kane to present about priorities and the intent of the legislation.

Rep. Kane presented two diagrams. (Diagram A and Diagram B) She explained that the first diagram was just an example to start the discussion. She identified three priorities based on past discussions of the council and formatted them into diagram A. She explained that we should think about priorities broadly and let the subcommittees work on drilling down into specific tasks or activities that would support the council's priorities.

She stated that we needed to think about priorities and activities that are achievable in a short period of time. We need to show progress as soon as possible. What can we do to improve the lives of those with rare diseases sooner than later?

Dr. Tierney asked everyone to think about Rep Kane’s presentation and start a list of priorities. He asked people to state a priority that they felt the council should focus on. He stated that he wanted the council to make a list, then the steering committee could review the list and come back to the full council with their thoughts on which ones to focus on in the next year.

A. Mahady stated that access to care was the biggest priority for her.

C. Segel stated that she wanted the council to provide support to patients and employees.

J. Creedon stated that as a caregiver, she had no protections from her employer to take care of her son, who has a rare disease. She had to leave her job as a nurse to care for her son. Her employer could not make accommodations for her because she was not the patient. She would like to see the council work on providing protections for caregivers.

C. Segel stated that we should have societal goals for protecting patients and caregivers. She also thought that ensuring access to appropriate medical care was also important.

Dr. Pasquini thought that the council should focus on access to care.

G. Thomas asked about getting data. She wondered how we could know what rare diseases are most common in Massachusetts. She stated that she felt the council needed at least a snapshot of the incidence of rare diseases in Massachusetts before setting specific priorities.

Dr. Tierney stated that he would see the research committee working on that. They could identify data sources to answer some of those questions.

L. Deck asked if the priorities could change or are we setting permanent priorities. She explained that the priorities might change from year to year. How would we deal with that?

Rep Kane stated that priorities might change, and that would be ok. She noted that the priorities should be broad so that new priorities could fit under the broader categories. She also asked the council to think about the legislative cycle. The legislature will be going into a 2-year cycle in January of 2023. If any issues may need legislative action, we need to form a timeline to fit within the legislative process. She suggested that we think about things that will have the most significant impact for the most people.

J. Gortze from Rare NE stated that her organization is working on identifying the rare disease incidence in all of NE. She suggested maybe working together to identify those numbers. She would introduce the people working on that or ask them to present at a meeting.

Dr. Tierney identified that working together with other rare disease organizations could be significant; that way, we don’t duplicate efforts and be more efficient without time.

L. Deck stated that she would like to see patient access to medical care as a priority. She spent 18 years trying to get a diagnosis. Access to the right providers is a challenge.

G. Hayes-Mota stated that we needed to think about patient support across the continuum of ages. When children are diagnosed with a rare disease, they seem to have resources that disappear when they become adults.

G. Thomas added that people and providers need a place to get information about a specific disease. We need to figure out a way to increase education and awareness for both the public and providers.

R. Shultz stated that any fundraising efforts should align with our priorities. He asked how we should work with other rare disease organizations. How do we know who the rare disease community stakeholders are? We should identify all the people and organizations that care about the rare disease community. He also stated that once we have information to share, how would we circulate or share this information with people? How do we get input from the rare disease community?

Dr. Tierney stated that it would be important to build a trusted network of people, organizations, industries, and businesses that care about the rare disease community.

Rep Kane stated that it would be possible to hold hearings or public forums to elicit comments and thoughts from the public. We could even have these sessions virtually if they were helpful.

Dr. Pasquini asked if we could build a non-siloed network. Often, we work in silos.

Dr. Tierney asked people to think about broad priorities for now. We will have time to get into a deeper discussion about the activities and outcomes for each priority.

C. Segel stated that we need to find “actionable” items to work on. We also don’t need to do this today. We have time to figure this out. We may need to know the incidence of rare diseases in Massachusetts first.

Dr. Tierney stated that the research and fundraising committees could work together on some of these things. The research committee could develop data collection and analysis projects and identify the financial needs to complete the work. The fundraising committee could work on identifying ways to fund the project.

Dr. Pasquini asked where we would get the data. Data is often challenging to get. We know that claims data, Medicaid data, etc., can be very challenging to obtain and interpret.

Dr. Dwyer suggested that other RDACs may be helpful in giving us best practices to access data and do epidemiologic studies to answer our questions.

C. Segel stated that Health Policy has access to some data. She would check into what data is available to the council and get back to the group.

Dr. Tierney thanked everyone for the productive discussion. He stated that the steering committee was scheduled to meet in August, and they would review the priorities discussed today. The steering committee will come back to the full council with their thoughts on which ones to focus on first.

He also stated that the steering committee would focus on operating procedures for the council during their August meeting. They would hopefully have a draft of the operating procedures for the full council meeting in September. Once that is voted on, the council would look to identify people for each subcommittee.

Dr. Tierney then reviewed the future meeting schedule. The frequency of meetings would increase so that work could get done more efficiently and effectively. He asked everyone to review the meeting schedule sent out with specific dates for each meeting. The full council would meet every other month starting in September. The steering committee and other subcommittees would meet every other month or as directed by the subcommittee chair. The subcommittees would report back to the steering committee and full council regularly.

He stated the next full council meeting would be on September 15th from 9-11 am. All council meetings would be held on the third Thursday of the month unless it fell on a holiday or school vacation week.

Dr. Tierney asked if there were any announcements.

No response.

Dr. Tierney asked if there was a motion to adjourn.

L. Deck made a motion to adjourn.

Dr. Pasquini made a second to the motion.

Dr. Tierney adjourned the meeting at 10:55.