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

**MEETING MINUTES-Approved 3.16.23**

REMOTE MEETING:    Thursday, January 19, 9:00-11:00 AM

**9:00 Dr. Dylan Tierney welcomed all to the RDAC full council meeting**

He made an announcement about Guest Participant Announcement

“The public is welcome to join any of the RDAC meetings. However, only guests that are on the agenda will be allowed to speak during the meeting. For all guests on this call, could you please make sure that your video is turned off and your audio is on mute.

Thank you for your interest in learning more about the work of the rare disease advisory council.

Dr, Tierney then conducted a **Roll Call to establish a quorum**

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| --- | --- | --- |
|  | **Council Member** | **Present** |
| 1 | Charlotte M. Boney, M.D | no |
| 2 | Janis Creedon | no |
| 3 | Lisa Deck | no |
| 4 | Andrew A. Dwyer, PhD, FNP-BC, FNAP, FAAN | no |
| 5 | Michael R. Green, M.D., Ph.D. | no |
| 6 | Julie D. Gortze, RN | X |
| 7 | Guadalupe Hayes-Mota, MBA, MS, MPA | X |
| 8 | Lena Joseph, RN, CPN | X |
| 9 | Representative Hannah Kane | X |
| 10 | Andrew A. Lane, MD, PhD | X |
| 11 | Representative Jay Livingstone | X |
| 12 | Jeff R. Livingstone, PhD | no |
| 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 | X |
| 21 | Robert E. Schultz, MBA | X |
| 22 | Celia Segel, MPP | X |
| 23 | Michael Sherman, MD | X |
| 24 | Glenda E. Thomas | X |
| 25 | Ryan Thompson, MD, MPH | X |
| 26 | Dylan Tierney, MD, MPH | X |
| 27 | Ann Wessel, MS, RD, LDN | X |
| 28 | Ross Zafonte, DO | X |

**Dr. Tierney** stated that there was a quorum and brought the meeting to order at 9:05.

**Dr. Tierney** asked if all had received and reviewed the meeting minutes from the last meeting.

He stated that C. Segel sent some revisions by email and he put the revisions on the screen. The last paragraph was revised to:

“The discussion focused on pay parity. Telehealth for mental health remains high, but for other conditions, the medical community has largely returned to see their patients in person. Dr. Tierney presented the concept of studying the relationship between telehealth and health outcomes.

***Celia Segel*** raised the issue of telehealth improving access for patients who need to frequently see many specialists to manage complex conditions, and need to travel long distances to see multiple different providers.”

**Dr. Tierney** asked if anyone had any other comments or corrections. No one responded.

**D. Miller** made a motion to vote to accept the minutes as revised.

**M. Sherman** made a second to the motion.

**Dr. Tierney conducted a ROLL CALL VOTE to Approve Minutes from 11.17.22**

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| --- | --- | --- |
|  | **Council Member** | **Approve** |
| 1 | Charlotte M. Boney, M.D | NA |
| 2 | Janis Creedon | NA |
| 3 | Lisa Deck | NA |
| 4 | Andrew A. Dwyer, PhD, FNP-BC, FNAP, FAAN | NA |
| 5 | Michael R. Green, M.D., Ph.D. | Yes |
| 6 | Julie D. Gortze, RN | Yes |
| 7 | Guadalupe Hayes-Mota, MBA, MS, MPA | Yes |
| 8 | Lena Joseph, RN, CPN | Yes |
| 9 | Representative Hannah Kane | Yes |
| 10 | Andrew A. Lane, MD, PhD | Yes |
| 11 | Representative Jay Livingstone | Yes |
| 12 | Jeff R. Livingstone, PhD | NA |
| 13 | Diane Lucente, MS, LCGC | Yes |
| 14 | Alexsandra B. Mahady | Yes |
| 15 | Jenn McNary | Yes |
| 16 | David T. Miller, MD, PhD | Yes |
| 17 | Tai Pasquini, PhD, MPA (Sen. Bruce Tarr) | Yes |
| 18 | Shivang Patel, Pharm.D. | Yes |
| 19 | Asma Rashid, MS, CGC | Yes |
| 20 | Michele Rhee, MBA, MPH | Yes |
| 21 | Robert E. Schultz, MBA | Yes |
| 22 | Celia Segel, MPP | Yes |
| 23 | Michael Sherman, MD | Yes |
| 24 | Glenda E. Thomas | Yes |
| 25 | Ryan Thompson, MD, MPH | Yes |
| 26 | Dylan Tierney, MD, MPH | Yes |
| 27 | Ann Wessel, MS, RD, LDN | Yes |
| 28 | Ross Zafonte, DO | Yes |
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The meeting minutes were approved.

**Dr. Tierney** Introduced Guest Speaker: Rich Pezzillo

Richard Pezzillo is the Executive Director of the New England Hemophilia Association (NEHA). Prior to joining NEHA, Richard worked in Washington, DC as the press secretary for Senator Sheldon Whitehouse, and then as the Communications and Marketing Director for the Hemophilia Federation of America (HFA). Richard is the former co-chair of the National Hemophilia Foundation’s (NHF) Youth Leadership Institute. He is the recipient of the Ryan White Meritorious Service Award, NHF Advocate of the Year Award, and a “40 under Forty” winner for the Association for Healthcare Philanthropy and Providence Business News. Richard graduated from Western Connecticut State University and currently resides in Rhode Island.

Rich thanked the RDAC for the invitation and the opportunity.

He proceeded first to let everyone know that he has hemophilia, which is a rare disease. He talked a little about what hemophilia is.

Rich stated that hemophilia was predominantly a male disease. 70% of cases are genetic and inherited on the x chromosome. The other 30% of cases are spontaneous, and there is no known cause. New England is unique, with so many hemophilia treatment centers. In Massachusetts, they are at UMass, Mass General, the Brigham, and Children’s Hospital.

Next, Rich touched on some of the misconceptions about hemophilia.

Most commonly, people think that hemophilia means you can bleed to death if you get a cut. People don’t realize that we bleed longer because we are missing the clotting protein, we don’t bleed faster. They also don’t realize that the most severe kind of bleeding is internal. If a hemophiliac has a major internal bleed, it can be life-threatening.

Today, the best practice for treatment is prophylactic therapy through IV infusions. There is now a subcutaneous injection available as of November 2022 for hemophilia B.

As with many rare diseases, the treatment for hemophilia is very individualized. What works for one person may not work for another. One patient may need weekly IV treatment s and another may be able to go monthly.

There are approximately 1,000 patients with hemophilia in Massachusetts.

Next, Rich touched on the cost of treatment for someone with hemophilia. The average annual cost of treating hemophilia is $350,000. Cost can be a heavy burden for people with hemophilia.

To better understand why this is, we need to look at the history of hemophilia.

In the 70s, before clotting protein infusions were invented, treatment involved real blood. During the AIDS epidemic, many people with hemophilia died because of this. In fact, the number of older people with hemophilia was greatly reduced because of this.

Rich went on to talk about the organization, New England Hemophilia Association, in which he is the executive director. The organization is based in Dedham, Massachusetts. It has been around since 1957. The organization focuses on education, retreats, and camps. It runs the largest hemophilia walk in the country. Advocacy is the heart of the organization.

To talk a little about our priorities, we advocate for policies and laws that support hemophiliacs and those with other bleeding disorders. One of the main challenges we face today is banning insurance companies from using accumulation adjusters. When an insurance company has

an accumulator adjustment program, it means that the patient is prevented from using any co-payment assistance from the pharmaceutical company that makes the medicine. These programs are often available for high-cost specialty drugs so that the cost is excluded from counting toward a patient's deductible or maximum out-of-pocket expenses. Many pharmaceutical manufacturers offer co-pay cards that help cover a patient's portion of drug costs.

What is an accumulator in health insurance?

An accumulator is a running total of money you've paid towards your out-of-pocket max for covered services. This includes any copays, coinsurance, and other health care costs, but not your monthly premium payments.

As of Fall 2022, **14 states have banned copay accumulators**: Arizona, Arkansas, Connecticut, Georgia, Illinois, Kentucky, Louisiana, Maine, North Carolina, Oklahoma, Tennessee, Virginia, Washington, and West Virginia, as well as Puerto Rico. We hope Massachusetts can be next.

Another issue we are seeing involves hemophilia but also other bleeding disorders. People with bleeding disorders have been denied access to substance use disorder clinics because they need IV medications. We did a national survey and 83% of providers have identified this as a national issue.

Rich then thanked the council for inviting him to speak. Dr. Tierney thanked Rich for his thoughtful and educational talk, then asked if anyone had any questions.

Dr. Tierney asked Rich if he knew how his organization had been able to build such a strong group.

Rich stated that some of it evolved after the federal law, the Ricky Ray Hemophilia Relief Act of 1998, was passed. This act provided for compassionate payments with regard to individuals with blood-clotting disorders, such as hemophilia, who contracted HIV due to contaminated antihemophilic factors, and for other purposes. The other reason, I think, is that this disease often involves families because it is predominantly passed on by genetics. The whole family is often involved in our advocacy efforts, and it passes on for generations.

Rich stated that he would work with the council in any effort that would help those with hemophilia or any other bleeding disorder.

**Dr. Tierney then gave an update on the Workgroups.** He let the council know that the workgroups have been formed. Everyone has been assigned to a workgroup. The chairs are set for each group. The chairs met last week, and since then, we have met with DPH legal counsel about whether or not the workgroups would fall under open meeting law. The conclusion is that, yes, the workgroups would need to follow the rules for open meeting law. Mary Lou will work with the chairs to make sure the meetings are posted appropriately. She will also work with the chairs to set the agenda, take the minutes and post all required documents according to the laws.

The chairs plan to meet with their members sometime in February and report back to the full council in march. Going forward the workgroups will mostly likely meet every other month until their goals are met.

**D. Miller** asked if it was possible for a few people to meet to discuss a specific topic relative to the workgroup. Dr. Tierney stated that DPH legal counsel was clear that the groups could not deliberate on a topic that the workgroup should hear. If it was a one-off discussion, it might be ok, but we should try to keep all the discussions to the workgroup sessions.

**G. Thomas** asked if there needed to be a certain number of people present to meet.

**Dr. Tierney** stated that the number wasn’t an issue, the groups could meet even if only 3 people were present.

**J. McNary** asked about holding an in-person meeting. Dr. Tierney stated that it would be more complicated, but it would be possible.

**Rep Kane** stated that there were many places available to hold an in-person meeting.

**J. Gortze** added that any in-person location needed to be ADA-compliant.

**WORKGROUPS**

**Workgroup 1 – Chair Dr. David Miller**

***Improve healthcare access and quality of care for people with rare diseases.***

**GOAL:** Determine the prevalence of rare diseases in Massachusetts.

**Workgroup 2 – Chair Jenn McNary**

***Advocate for and improve access to social supports and services for people impacted by rare diseases.***

**GOAL:** Develop a profile of rare disease social supports and services in Massachusetts

**Workgroup 3 – Chair Lena Joseph**

***Foster communication and collaboration to empower the rare disease community in Massachusetts.***

**GOAL:** Develop a profile of rare disease expert individuals, community-based organizations, voluntary organizations, healthcare providers, and any other public or private organizations with an interest in rare diseases in Massachusetts.

Next on the agenda is a presentation on

Determining the Prevalence of Rare Diseases in Massachusetts by council member,

**Tai Pasquini, PhD, MPA**

**Dr. Tierrney** introduced Tai:

**Tai Pasquini** is a rare disease patient advocate and researcher. Currently, she is the Research and Policy Director for Congenital Hyperinsulinism International (CHI), where she leads the HI Centers of Excellence program, the Collaborative Research Network, and helps facilitate drug access programs. She is also the Principal Investigator of the HI Global Registry. Tai also serves as a Regional Policy Taskforce Volunteer Co-Chair for the National Organization for Rare Disorders (NORD). Tai completed a Ph.D. in Health Policy from the University of Massachusetts Amherst, and her dissertation focused on issues of access and financing for rare disease patients and families. She holds a Master’s in Public Administration and a Bachelor of Arts in Interdisciplinary Studies from American University.

**Tia presented using slides (posted in meeting materials)**

Tai discussed the importance of an accurate prevalence number and some of the challenges to gathering this data. She discussed the definition of incidence and prevalence. The currently published numbers for rare disease prevalence in the US. She then reviewed methods for estimating prevalence in Massachusetts. She concluded her talk with considerations to think about as Massachusetts decides on how it will determine prevalence.

**Dr. Tierney** thanked Tai for her thoughtful presentation and asked if there were any questions.

**D. Miller** Thanked Tai and said that they hadn’t discussed her plan for the presentation, but she must have read his mind as he agreed with all of Tai’s discussion points.

He thinks that it will be hard to get an exact number. Many of the small numbers of disorders make up an overall majority. There are disorders that are considered “ultra-rare” How precise do we need to be?

**J. McNary** asked if we are considering people that come to Massachusetts for treatment. She stated that her son is dealing with this now. Because so many people come to our state for treatment, it sometimes affects Massachusetts residents' ability to get in to see a doctor when they need to.

**T. Pasquini** responded by saying that maybe we should include the whole ecosystem for reasons like this.

**R. Thompson** thanked Tia for her presentation and stated that we should compare our estimates against claims data. This may be a good way to see if we are close. We could also look at a specific disease that has a registry to measure against that.

**Rep Kane** added that she thought this was a great discussion. She agreed that getting an accurate prevalence number would be challenging, but we just need a good estimate. When we report our numbers, we should also report about the challenges of determining the prevalence number. We should also think about looking at data on private versus public payers. Are there disparities between access to care for those relying on public payers versus private payers?

We need to collect data that helps us better understand the challenges and barriers faced by those with a rare disease.

**Dr. Tierney** thanked everyone for a great conversation and asked everyone to think about future plans for the council. We discussed having a legislative briefing and public forums. What do people think about holding a legislative briefing? When should we do this?

**Rep Kane** suggested that we should hold it sooner than later. We could have a small briefing with a small group of legislators or even have a zoom meeting. We should share our vision, mission, and goals. We should share our ideas and think about doing something like this annually.

**Rep. Livingstone** agreed that we should do this annually. He felt that a zoom briefing might be the best way to do a briefing as it would most likely be viewed by more people.

**L. Joseph** asked if workgroups should meet before the briefing.

**J. McNary** asked if we should have a workplan before holding a briefing.

**Rep. Livingstone** thought that we didn’t need to wait for workgroups or a workplan. Educating the legislators on what has happened so far may be more helpful. Attendance will depend on interest.

**Rep Kane** stated that generally staffers and legislators would attend. We should think about making it informative and interactive.

She noted that Feb. 28th was Rare Disease Day at the statehouse. We could think about doing something then.

**Dr. Tierney** stated that he would reach out to Rep Kane and Rep Livingstone to make plans for the legislative briefing. He then asked about a public forum to gain information from the public? What is the group's thoughts on holding this type of meeting?

**J. McNary** suggested a “town hall” style meeting. The public could share their experiences.

**T. Pasquini** stated thatNORD holds these types of meetings. It would be helpful to have a virtual option as some people may still not be comfortable meeting in person.

**Dr. Tierney** suggested that we could have 1 or 2 in-person meetings and 1 virtual one.

**J. McNary** agreed that we should have both in-person and virtual options.

**Dr. Tierney** summarized the discussion.

I will talk with Rep Kane and Rep Livingstone to plan a legislative briefing in Feb. We will start to think about holding public “town halls” in the spring.

**Dr. Tierney** asked if there were any announcements.

**J. Gortze** let everyone know that Rare Disease Day was on Feb. 28th. The legislative day would have educational sessions, and there would be meetings with advocates and legislators. Should we think about having someone from the RDAC to speak on that day?

**Rep Kane** offered to speak with the planners and see how the RDAC could be represented on the schedule. She offered to work with J. Gortze

She suggested going to MassBio for information on the day. <https://www.massbio.org/events/listing/2023-rare-disease-day/>

**Dr. Tierney** let everyone know that the NEXT FULL COUNCIL MEETING would be on

March 16, 2023 9:00 am – 11:00 am

**T. Pasquini** made a motion to adjourn

**D. Miller** made a second

**Dr. Tierney** adjourned the meeting at 10:59