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

REMOTE MEETING:    Thursday, March 31, 2022, 9:00-11:00 AM

* **Welcome**

Dr. Tierney welcomed everyone to the third meeting of the MA RDAC. Explained the meetings will now be held using ZOOM. Reviewed the agenda.

* **Roll Call**

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| --- | --- | --- | --- | --- |
| **Council Member** | **March 31, 2022** | **Approve 12/9/21 minutes** | **Establish RDAC Steering Committee**  | **Committee Membership**  |
| Charlotte M. Boney, M.D | x | x | x | x |
|  Janis Creedon  |  |  |  |  |
| Ms. Lisa Deck  | x | x | x | x |
| Andrew A. Dwyer, PhD, FNP-BC, FNAP, FAAN | x | x | x | x |
| Michael R. Green, M.D., Ph.D.  | x | x | x | x |
|  Julie D. Gortze  | x | x | x | x |
| Guadalupe Hayes-Mota  | x | x | x | x |
| Representative Hannah Kane  | x | x | x | x |
| Andrew A. Lane, MD, PhD | x | x | x | x |
| Representative Jay Livingstone  | x | x | x | x |
|  Jeff R. Livingstone, PhD | x | x | x | x |
| Diane Lucente, MS, LCGC | x | x | x | x |
| Alexsandra B. Mahady  | x | x | x | x |
| Jenn McNary | x | x | needed to leave early  | needed to leave early  |
| David T. Miller, MD, PhD | x | x | x | x |
| Tai Pasquini, PhD, MPA  | x | x | x | x |
| Shivang Patel, Pharm.D.  | x | x | x | x |
| Asma Rashid, MS, CGC  | x | x | x | x |
| Michele Rhee, MBA, MPH | x | x | x | x |
| Robert E. Schultz | x | x | x | x |
| Celia Segel | x | X | x | x |
| Michael Sherman, MD | x | x | needed to leave early  | needed to leave early  |
| Senator Bruce Tarr | emailed - could not attend |  |  |  |
| Ms. Glenda E. Thomas  | x | x | x | x |
| Ryan Thompson, MD, MPH | x | x | x | x |
| Dylan Tierney, MD, MPH | x | x | x | x |
| Ann Wessel, MS, RD, LDN  | x | x | x | x |
|  Ross Zafonte, DO | x | x | x | x |

* **Vote: to approve 12/9/21 meeting minutes**

Ms. Rhee made one comment on the minutes to correct the spelling of her name.

All present members approved the minutes (see table above).

* **Speaker: Anna Legassie, Patient Advocate**

Dr. Dylan Tierney introduced Anna Legassie. Diagnosed with systemic juvenile idiopathic arthritis (SJIA) at age 11, Anna is a nationally recognized patient advocate regularly advising healthcare start-ups, pharmaceutical companies, and other organizations to incorporate patient values into healthcare design, technology, and policy reform. Anna serves on the Board of Directors of the Autoimmune Association, as co-chair of the Tufts Medical Center Patient and Family Advisory Council (PFAC), and on the patient advisory board of Clara Health. She previously served on the Massachusetts Leadership Board of the Arthritis Foundation and on the Foundation’s National Advocacy Committee.

Ms. Legassie thanked Dr. Tierney for the introduction. Ms. Legassie indicated she is familiar with several names of the Council members listed on ZOOM. Ms. Legassie said she was recently sick, which highlights some of the invisible issues of having a rare disease. Ms. Legassie described her early diagnosis and her much longer path to additional diagnoses. Ms. Legassie emphasized issues she sees as common in the rare disease community: missing symptom causes as clinicians are not familiar with the rare disease, dismissal of symptoms and lack of continuity of care when transitioning to new providers or leaving one age group (child to adolescent; adolescent to young adult). Almost 20 years passed before the diagnosis of cardiac problems. Ms. Legassie described rare disease diagnosis and long-term treatment as shifting sand. Ms. Legassie assumed her story/journey is very familiar to Council members. When a diagnosis was finally reached, viable treatment options were limited or nonexistent. MA is a state where medical innovation and access to care is prominent, but this does not mean anything for individuals living with a rare disease(s). Ms. Legassie said she is very encouraged by the formation of the MA RDAC and very happy to be speaking at an RDAC meeting. Ms. Legassie commented on the recent posting of rare disease report cards on the NORD website and feels some of the grades are not in line with the patient experience – which is why it is important to hear from people living with rare diseases. MA needs to work on “step therapy” for treatment, continue to allow access to telehealth, focus on insurance coverage for genetic testing (not just genetic testing for rare diseases), more clinicians equipped to identify and treat rare diseases.

Dr. Tierney thanked Ms. Legassie for sharing her story with the Council and highlighted the common theme of “dismissal.”

Dr. Livingstone commented that the medical community is poorly tooled to assess rare diseases vs. standard diagnosis.

Dr. Sherman thanked Ms. Legassie and reminded the group the focus should be on the patient.

Ms. Thomas also thanked Ms. Legassie and asked if she knew if there was any information on the timeline from a rare disease diagnosis to treatment and/or drug approval. Ms. Legassie did not. She commented that she feels where on lives (geographically) is a factor. Also, that many female patients get dismissed with symptoms being “in your head” as providers are not trained to address different symptoms in females, especially cardiac symptoms. Ms. Legassie emphasized the importance of maintaining access to telehealth, especially as institutions merge as it affects access to care across the state.

Dr. Thompson thanked Ms. Legassie for the honesty of her story. Dr. Thompson commented that continuity of care, or lack of, is a very real factor. He feels the current clinical system does not customize care very well for any unique situation. Dr. Thompson asked Ms. Legassie what would delivery of care look like for rare disease? What would be ideal? Ms. Legassie responded that the question is a great one. First, she feels social work services should be available to those who are most vulnerable. She can be her own advocate, but not everyone has that option/can do that. Rare disease patients need holistic approaches to care, covering all facets of life. For example, sometimes hospital admission for an individual with a rare disease is not doable. Care should be customized to address services outside of a hospital setting.

Ms. Segel thanked Ms. Legassie and indicated she has a young family member going through a journey to diagnosis and care. Ms. Segel added that her focus at the HPC is on prescription drugs, and she has concern over out-of-pocket drug costs. HPC is currently looking at telehealth services trying to get a sense of how telehealth is accessed across the state. Ms. Segel will share information on this a appropriate.

Dr. Tierney commented that the subject of telehealth keeps being mentioned.

Dr. Lane shared that his wife is a pediatric rheumatologist, and her experience is diagnosis is not necessarily a barrier, but appropriate evaluations and access to therapies is a problem. Insurers put pressure on clinicians to be sure a diagnosis exists. Dr. Lane asked what the RDAC can do to help with easing the path to treatment post diagnosis.

Dr. Dwyer commented there is an implicit bias in complex care, and it is critical for patients, and is important to maintain access to telehealth, especially for those residing a distance from large clinical institutions. This means access to clinical staff (including RNs) and other support services as well. Dr. Tierney asked if Dr. Dwyer could expand a bit. Dr. Dwyer responded that it is not just about getting a diagnosis, but what happens afterward. Patients need access to pharmacists, social workers, nurses, treatment education – and travel expenses are a real roadblock.

Dr. Pasquini thanked with Ms. Legassie. She feels it is hard to capture the burden of rare disease when the medical community has a myopic view of rare diseases. The focus is only on medical issues and ignores the rest of the individual’s life. Dr. Pasquini emphasized there should be a focus on life outside of the care setting. Also, made a comment that some assessment tools are old and have not been updated as new treatments are approved (ex: arthritis).

Dr. Tierney suggested the re-framing of the rare disease experience lead by individuals living rare diseases. This would help all – example: ramps help everyone with mobility issues not just individuals living a rare disease.

Ms. Mahady thanked Ms. Legassie and suggested having an individual with a rare disease at each meeting of the RDAC. She is willing to help find speakers. Also, Ms. Mahady agreed with all the comments on telehealth, as some patients have 200-300 appointments in a year, and it effects individual quality of life.

Ms. Legassie thanked the RDAC for having her and commented that the formation of the Council makes her optimistic.

* **Vote: to establish an RDAC Steering Committee**

Dr. Tierney reminded the Council the possible formation of a steering committee was discussed at the last meeting, but the Council needed to discuss and vote again, as the item was not on the agenda in accordance with Open Meeting Law rules. Dr. Tierney presented the slide below:

**Vote to establish RDAC Steering Committee**

The RDAC shall establish a Steering Committee to advise the chair on the following topics: 1) agenda setting recommendations for quarterly RDAC meetings; 2) identifying recommended meeting speakers; and 3) recommending Council priorities.

The steering committee shall consist of seven members who are current RDAC members, including

1. The RDAC chair
2. People living with a rare disease
3. Caregivers or parents of people living with a rare disease
4. The Legislature
5. Hospital administrators
6. Clinicians
7. Researchers

Steering Committee membership will be voted on by the full RDAC.  Steering Committee members shall serve on the Committee for the balance of their term as RDAC members.

A roll call vote was taken, and the Council voted to establish a Steering Committee (see table on page 1).

* **Vote: RDAC Steering Committee Membership**

Dr. Tierney reviewed the names of individuals nominated at the last meeting to serve on the Steering Committee and presented the slide below:

**Steering Committee Membership**

As of March 31, 2022, the following individuals will serve on the RDAC Steering Committee, for the balance of their present terms on the RDAC

RDAC Chair – Dr. Dylan Tierney

Person living with a rare disease – Michele Rhee

Caregiver or parent - Jenn McNary

Legislator – Representative Hannah Kane

Hospital administrator – Dr. Thompson

Clinician – Dr. Miller

Researcher – Dr. Livingstone

A roll call vote was taken, and the Council voted members onto the Steering Committee (see table on page 1).

Dr. Tierney explained that all RDAC Steering Committee meetings are subject to Open Meeting Law and will follow the same procedures as the full RDAC. A question was asked about the number of people necessary to have a Steering Committee meeting and the Open Meeting Law. Dr. Tierney responded that both the Steering Committee and the full Council must have a quorum to hold a meeting and must adhere to the Open Meeting Law. Dr. Livingstone reiterated that any communications between members is covered by the Open Meeting Law. Dr. Tierney said it is best to communicate at scheduled meetings. Can always email ideas to him directly – but not as an email discussion with multiple members.

* **Chairperson Updates**

Any documents including minutes produced by DPH for the RDAC are public and can only be commented on during scheduled meetings.

At the last meeting there was a suggestion to have an online calendar (RDAC webpage). This would not be easy to maintain and most DPH public bodies only list their own meetings. Members should always look at the NORD webpage as well as the Rare Action Network (these links are on the MA RDAC webpage). Non-live documents preferably links, can be posted as resources.

* **Speaker(s) for future meetings**

Next meeting June 23, 2022

Suggested topic: telehealth.

Suggested speakers: Dr. Tierney suggested Dr. Scott Strome of the Tennessee RDAC and Erica Barnes from Minnesota. Dr. Livingstone asked if regional or national resources exist. There was a suggestion to review the NORD website. Not aware of regional resources, but regional advocacy groups exist. Mr. Hayes-Mota suggested a speaker to address cost of treatments and office visits. Ms. Segel indicated she would investigate speakers for that topic. Ms. Deck suggested having a speaker’s bureau – a list of patient speakers. Ms. Rhee and Ms. Gortze indicated they would like to assist with developing that list. Dr. Tierney said that would be very helpful for the RDAC. Ms. Rhee added that a speaker focusing on the caregiver experience would also provide insight for the Council.

* **Member Announcements**

Ms. Deck announced that the New Hampshire Rare Disease Council is holding a meeting on May 20, 2022 @ 3pm. The Massachusetts RDAC is invited to join at the beginning for a Meet & Greet. Ms. Deck will forward information to Dr. Tierney.

#### Dr. Pasquini identified several state bills that have been filed - *An Act relative to ensuring treatment for genetic craniofacial conditions –* there are several versions, H1153, H1214,

#### H1208, S648.

* **Adjourn**

Meeting adjourned.