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

REMOTE MEETING:    Thursday, September 16, 2021, 9:00-11:00 AM

*The Council will have one regular meeting on a quarterly basis. These meetings are open to public attendance. The Council’s meetings are not hearings, nor do members of the public have a right to speak or address the Council.*

1. **Welcome**
2. **Review of RDAC statutory authority and law regarding open meetings, public records, and conflict of interest**
3. **Member Introductions**
4. **Future Scheduling**

**Members Present:**

Charlotte Boney; Lisa Deck; Andrew Dwyer; Julie Gortze; Guadalupe Hayes-Mota; Rep. Hannah Kane; Andrew Lane; Rep. Jay Livingstone; Jeff Livingstone; Diane Lucente; Alexsandra Mahady; Jenn McNary; David Miller; Tai Pasquini; Asma Rashid; Michele Rhee; Robert Schultz; Celia Segal; Glenda Thomas; Ryan Thompson; Dylan Tierney; Ann Wessel; Ross Zafonte.

Also in attendance: Thera Meehan, consultant to the Bureau of Infectious Disease and Laboratory Sciences, DPH; Lynn Squillace, Deputy GeneralCounsel, Office of the General Counsel, DPH.

1. **Welcome**

Dr. Dylan Tierney, Chair of the RDAC welcomed members to the first meeting of the MA RDAC. Dr. Tierney briefly reviewed the legislation that established the RDAC – Chapter 111 Section 241 (Section 26 of Chapter 260 of the Acts of 2020) and reviewed the definition of "Rare disease” in the statute as any disease that affects fewer than 200,000 people in the United States, has status as an orphan disease for research purposes or is known to be substantially under-diagnosed and unrecognized as a result of lack of adequate diagnostic and research information.

1. **Review of RDAC statutory authority and law regarding open meetings, public records, and conflict of interest**

Attorney Lynn Squillace, explained the Open Meeting Law (OML), a deliberation, and the definition of a quorum:

* The OML is designed to ensure transparency in the *deliberations* of public bodies.
* A *deliberation* is:
  + an oral or written communication, through any medium, *including electronic mail*,
  + between or among a *quorum* of a public body,
  + on any public business within its jurisdiction.
* A *deliberation* does not include:
  + distribution of a meeting agenda, scheduling or *procedural* information, or
  + reports or documents that may be discussed at a meeting, provided that no member of the public body expresses an opinion on matters within the body’s jurisdiction.
  + *NOTE: If a public body member sends an email to a quorum of the public body expressing an opinion on any matter that could come before that body, the communication violates the OML, even if no recipient responds.*
* A Quorum is defined as:
  + A simple majority of the members of a public body, unless otherwise provided in a general or special law, executive order, or other authorizing provision.  G.L. c. 30A, § 18.
  + As applied to the RDAC quorum equals 15 members (½ of 29 members + 1)

If a quorum of a public body wants to discuss public business within that body’s jurisdiction, they must do so during a properly posted meeting.

Ms. Squillace described best practice recommendations to avoid OML violations:

* Public body members must not engage in “serial deliberations”—a series of separate, independent conversations outside of a meeting among a quorum of the members regarding a topic within its jurisdiction.
* In order to avoid even the appearance of a potential OML violation, the AGO advises public body members to refrain from communications over email except for distributing meeting agenda, scheduling meetings and distributing documents created by nonmembers.

Ms. Squillace also reviewed Conflict of Interest Law:

* The Conflict of Interest (COI) law, M.G.L. c. 268A, is meant to prevent conflicts (and appearances of conflict) between a state employee’s private interests and his or her public duties.
* As statutory public body members, you are considered to be “special state employees” subject to the COI law.
* The COI law is complex; State Ethics Commission attorneys are available, through the “Attorney of the Day” program, to provide confidential advice/guidance on how the COI law applies to you in a particular situation. ***Contact Attorney of the Day @ (617) 371-9500***

the Public Records Law:

* Massachusetts Public Records Law, M.G.L. c. 66, provides that each person has a right of access to public information
* The Public Records Law broadly defines “public records” to include “all books, papers, maps, photographs, recorded tapes, financial statements, statistical tabulations, or other documentary materials or data, regardless of physical form or characteristics, made or received by any officer or employee” of any Massachusetts governmental entity, with some exceptions.

and provided the following resources:

* Conflict of Interest Law:

[https://](https://www.mass.gov/learn-more-about-conflicts-of-interest)www.mass.gov/laws-regulations-rulings-opinions-and-advisories

https[://www.mass.gov/learn-more-about-conflicts-of-interest](https://www.mass.gov/learn-more-about-conflicts-of-interest)

* Office of Attorney General, Open Meeting Law Website and Guide:

<https://www.mass.gov/files/documents/2017/09/25/2017%20Guide%20only.pdf>

<http://www.mass.gov/ago/government-resources/open-meeting-law/>

* Secretary of the Commonwealth, A Guide to the Massachusetts Public Records Law

<https://www.sec.state.ma.us/pre/prepdf/guide.pdf>

Dr. Tierney asked if there were any questions for Attorney Squillace and there were none.

* 1. **Member Introductions**

Dylan Tierney, MD, MPH, Chair (designee of the Commissioner of DPH) began the introduction portion of the meeting. Dr. Tierney is an infectious disease physician with a focus on tuberculosis, and the Associate Medical Director of the Bureau of Infectious Disease and Laboratory Sciences (BIDLS) at DPH. Dr. Tierney has a focus and interest in health equity. He asked each member to answer the following questions when introducing themselves:

1. What are the most important issues faced by people with rare diseases?
2. What would you like to see the Massachusetts RDAC achieve within the next year?

**Dr. Tierney**

What are the most important issues faced by people with rare diseases?

Continuity of care

What would you like to see the Massachusetts RDAC achieve within the next year?

Would like to identify an achievable list of priority actions for the Council, with input from community/advocacy groups.

**Charlotte Boney, MD, Chair, Department of Pediatrics, Baystate Medical Center. Practicing Pediatric Endocrinologist.**

What are the most important issues faced by people with rare diseases?

The vast majority of rare diseases in MA are found in children.

What would you like to see the Massachusetts RDAC achieve within the next year?

Focus on equity in care.

**Lisa Deck, Patient and Patient Advocate; state and federal level lobbyist; member of Rare New England.**

What are the most important issues faced by people with rare diseases?

Challenges to receiving a timely diagnosis; long-term journey to diagnosis, access to treatment and care.

What would you like to see the Massachusetts RDAC achieve within the next year?

Identify needs of the rare disease community in MA; identify incidence of certain rare diseases; develop an interactive RDAC website.

**Andrew Dwyer, Ph.D., FNP-BC, FNAP, FAAN. Assistant Professor Boston College; MGH Center for Reproductive Medicine. Focus on rare endocrine disorders.**

What are the most important issues faced by people with rare diseases?

Social justice and access to care.

What would you like to see the Massachusetts RDAC achieve within the next year?

Create a road map for individuals with rare diseases. Use technology to connect people.

**Julie Gortze, RN. Patient. Founder of Rare New England. Dedicated several years to getting legislation passed in MA. Very happy to have the first meeting of the Council. Sees so much potential for the Council to help the rare disease community.**

What are the most important issues faced by people with rare diseases?

Accessibility of care; affordability of treatment, particularly medication; patient and family isolation; support for school-based Individual Education Plans (IEPs)

What would you like to see the Massachusetts RDAC achieve within the next year?

Identify common challenges for the rare disease community and develop resource to address them; Raise general public/community awareness of rare diseases – similar to how cancer is recognizable.

**Guadalupe Hayes-Mota, MS, MBA, MPP. Patient; founder Healr Solutions.**

What are the most important issues faced by people with rare diseases?

Accessibility to treatment (cost is a barrier); finding treatment – 92% of rare diseases have none; maintaining independence as a rare disease patient.

What would you like to see the Massachusetts RDAC achieve within the next year?

Create an inventory of the needs of the rare disease community and develop a plan for solutions.

**Representative Hannah Kane, MA House of Representatives. Parent. Sponsor of legislation.**

What are the most important issues faced by people with rare diseases?

Understand the challenges of those in the rare disease community; look at different levels/systems to fix the challenges such as legislative and regulatory.

What would you like to see the Massachusetts RDAC achieve within the next year?

Council is set up for the long term and needs to make a difference for rare disease patients. Council should consider subcommittees to address issues. Shares Julie Gortze’s enthusiasm to be at the first Council meeting.

**Andrew Lane, MD, PhD, Oncologist, Dana Farber; Faculty, Harvard Medical School. Focus on leukemia and other blood disorders.**

What are the most important issues faced by people with rare diseases?

Access to expertise as well as access to standard therapies; lack of mechanisms for patients to find information, to find experts and to access clinical trials.

What would you like to see the Massachusetts RDAC achieve within the next year?

Want to learn from Council members; determine what is achievable without losing site of idealism.

**Representative Jay Livingstone, MA House of Representatives. Vice Chair of Health Care Financing Committee.**

What are the most important issues faced by people with rare diseases?

Identifying and understanding barriers to treatment, with a focus on equity.

What would you like to see the Massachusetts RDAC achieve within the next year?

Identify legislative or regulatory changes that will support the rare disease community.

**Jeff Livingstone, PhD, CEO Igia Pharmaceuticals. Long history with rare diseases (brother had a rare liver disease).**

What are the most important issues faced by people with rare diseases?

Supporting the family/parents/caretaker of rare disease patients.

What would you like to see the Massachusetts RDAC achieve within the next year?

Identify what advocacy groups in MA are doing; provide accurate information on rare diseases through social media platforms – accurate information access through social media is a problem for family/parents/caregivers.

**Diane Lucente, MS, LCGC. Genetic Counselor, MGH; clinical researcher; neurology.**

What are the most important issues faced by people with rare diseases?

Providers and the general community need education on rare diseases; patients need access to treatment including clinical trials.

What would you like to see the Massachusetts RDAC achieve within the next year?

Education, outreach, and awareness so patients do not feel isolated while trying to navigate the rare disease system of care.

**Alexsandra Mahady, Parent.**

What are the most important issues faced by people with rare diseases?

Spending so much time in a hospital/clinical setting, especially for young children.

Telehealth during the COVID-19 pandemic has been a godsend.

What would you like to see the Massachusetts RDAC achieve within the next year?

Help to make telehealth permanent for rare disease patients; will enable care and improve quality of life.

**Jennifer McNary. Parent.**

What are the most important issues faced by people with rare diseases?

Many children diagnosed with rare diseases are living longer. Services for young adults are lacking, such as higher learning, independent living, employment supports and access to mental health services.

What would you like to see the Massachusetts RDAC achieve within the next year?

Conduct a landscape assessment and identify reimbursement issues.

**David Miller, MD, PhD, Director, Neurofibromatosis Program, Boston Children’s Hospital; Assistant Professor, Harvard Medical School. Interest in advancing genetic technologies to end the genetic diagnosis odyssey most patients face.**

What are the most important issues faced by people with rare diseases?

Access and equity. The reimbursement landscape is fragmented – access to genetic testing should easily accessible and covered by health plans.

What would you like to see the Massachusetts RDAC achieve within the next year? Make telemedicine a permanent benefit for those with rare diseases. Children with rare diseases receive services other than clinical services (Early Intervention etc) – always leaving the house for medical care is very disruptive for ongoing services/supports.

Challenge is how to focus on issues that will impact the most people in the rare disease community.

**Tai Pasquini, PhD, MPA; Research and Policy Director at Congenital Hyperinsulinism International, UMASS Amherst; State Ambassador, Massachusetts RareAction Network.**

What are the most important issues faced by people with rare diseases?

Help/assistance navigating the hospital systems in MA. Not all medical professionals understand rare diseases and some patients are dismissed. There are problems with insurance coverage. Also, transition from Early Intervention to school systems is difficult.

What would you like to see the Massachusetts RDAC achieve within the next year?

Understand challenges of patients/families/care givers. Identify “low hanging fruit” as well as develop a long-term agenda.

**Asma Rashid, MS, CGC,** [**Division of Genetics and Genomics**](https://www.childrenshospital.org/centers-and-services/departments/genetics)**, Department of Pediatrics, Boston Children’s Hospital. Focus on informed consent for genetic testing.**

What are the most important issues faced by people with rare diseases?

Delays in accessing genetic testing causes delay in diagnosis, treatment, and management.

What would you like to see the Massachusetts RDAC achieve within the next year?

Identify barriers to detection; address inadequate health insurance coverage (procedures are not covered even when recommended by physician); make telehealth permanent.

**Michele Rhee, Vice President Patient Affairs and Advocacy, X4 Pharma. Patient.**

What are the most important issues faced by people with rare diseases?

Navigating the world as a rare disease patient – going to school or working full time is hard. As a patient, “charming” doctors is necessary to get more time with them. Coordinating multiple specialists is a full-time job.

What would you like to see the Massachusetts RDAC achieve within the next year?

Landscape assessment in MA. Address affordable testing and avoid step therapy. Prioritize what the Council can achieve.

**Robert Schultz, MBA. Managing Director, Massnex; Adjunct Professor Babson College, Northeastern University, MA College of Pharmacy and Health Sciences.**

What are the most important issues faced by people with rare diseases?

Understanding the role of caregivers. How can the family/caregiver unit be supported?

What would you like to see the Massachusetts RDAC achieve within the next year?

Awareness campaign – society does not really see rare disease, but rare disease impacts society. Identify individuals with a high net worth who have a personal connection to rare disease to use philanthropy to address issues.

**Celia Segel, MPP. Associate Director, Pharmaceutical Pricing and Policy, Health Policy Commission.**

What are the most important issues faced by people with rare diseases?

Current health care system in MA is unforgiving. This is the first time hearing the term “diagnostic journey.”

What would you like to see the Massachusetts RDAC achieve within the next year?

Sustain and encourage development of rare disease research and innovations. Describe the landscape of data sources exist in the state.

**Glenda Thomas. Patient. Advocate. Volunteer. MGFA New England Myasthenia Gravis Support Group Leader**

What are the most important issues faced by people with rare diseases?

Access to medical professionals is not the same across the state (particularly outside of the Boston area).

What would you like to see the Massachusetts RDAC achieve within the next year?

Make telehealth a permanent benefit for rare disease patients. Review federal policies concerning drugs/treatment for rare diseases.

**Ryan Thompson, MD, MA General Hospital. Focus on care continuum and complex care services.**

What are the most important issues faced by people with rare diseases?

Care delivery models do not necessarily support patients with rare diseases.

What would you like to see the Massachusetts RDAC achieve within the next year?

Figure out how to organize health care delivery to minimize suffering for patients with rare diseases.

**Ann Wessel, MS, RD, LDN. Nutrition Center, Boston Children’s Hospital Division of Gastroenterology, Hepatology and Nutrition.**

What are the most important issues faced by people with rare diseases?

Health insurance coverage. Access to medical nutrition services. Barriers to out-of-network care.

What would you like to see the Massachusetts RDAC achieve within the next year?

Lack of general knowledge of rare diseases by public, school systems, insurers. Identify and align with existing groups/resources.

**Ross Zafonte, DO, MA General Hospital, Brigham & Women’s Hospital and Spaulding Rehabilitation Hospital. Focus on long-term disability from rare disease.**

What are the most important issues faced by people with rare diseases?

Continuity of care; bias in physical access to care.

What would you like to see the Massachusetts RDAC achieve within the next year?

Identify best practices at the state level; identify commonalities in the system that are good and those that produce stress for patients and their families/caregivers.

Dr. Tierney commented on the expertise, experience, and passion each member exhibited during introductions. Dr. Tierney explained that the National Organization on Rare Disorders (NORD) is supporting all states across the country to establish rare disease advisory councils. Approximately 30 states have established a Rare Disease Advisory Council (RDAC) or are in the process of doing so. MA is unique in that the state has high quality medical care, leading bioscience technology and strong patient advocacy. The MA RDAC can innovate and show the way for other states. The MA RDAC was established to be a long-term council. We will need to establish short-term and long-term goals. The Council will meet on a quarterly basis.

Dr. Tierney summarized themes he heard from member introductions:

* Increase amount of information on rare disease in MA (prevalence of diseases)
* There are barriers to accessing diagnosis and treatment
* Telehealth has been very important to care for patients with rare diseases and should be made permanent
* Equity is important – access to diagnosis, treatment and care should be equitable across populations
* Awareness needs to be raised.

Dr. Tierney described the new RDAC website as a placeholder. Invites Council members to help by suggesting updates, links etc. Meetings will be held virtually for the time being. It will be great to eventually meet in person. Next meeting will be held in mid-December.

Dr. Tierney asked members to think about having meetings/listening sessions with individuals, groups who are not generally part of the conversation, or in the room. He also asked members to consider establishing working groups or subcommittees of members who share similar interests/expertise – groups could be topic focused or tool focused.

Dr. Tierney asked if any members had last comments.

Ms. Thomas indicated she is interested in knowing what information the state has on rare diseases – epidemiological data or just in general.

Ms. Rhee suggested looking to other states that have conducted surveys on rare diseases and said she would reach out to the state of PA.

Dr. Tierney suggested having a guest speaker at the next meeting – perhaps someone from NORD – to give an overview of rare disease activity in the US.

Ms. Deck also suggested having someone from a state run RDAC speak to MA about the pros and cons involved in getting an RDAC off the ground. Ms. Gortze agreed with this suggestion.

Ms. Pasquini said she is aware that the state of Minnesota is looking at the prevalence of rare disease in the state’s Medicaid system.

Dr. Thompson asked if the Council is interested in looking at what is happening with telehealth at the state and federal level, stating that patients with rare diseases and disabilities should have continued access to telehealth services and be able to stay at home.

Dr. Tierney indicated he was excited and humbled to finally meet council members, albeit virtually.

Dr. Tierney ended the meeting.