

Rare Disease Advisory Council
Strategic Priorities
Approved and voted September 15, 2022

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

People with rare diseases face many healthcare challenges. The “diagnostic odyssey” is a common experience, with repeated evaluations across multiple health systems until a diagnosis is made. Once diagnosed, it can be difficult to find a healthcare provider that has knowledge and expertise in a rare disease. In the long-term, management of rare diseases may falter because of the difficulty in establishing regular access to complex care and treatment. The Massachusetts RDAC will seek to identify barriers and advocate for changes that will improve access and quality of care to people with rare diseases to maximize their health to the fullest possible degree.

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

People with rare diseases and their caregivers often struggle with the social aspects of illness in addition to the medical demands. Social isolation, financial stress, and stigma are common. Children with rare diseases may struggle with their schooling experience. The Massachusetts RDAC will seek to improve awareness of and access to existing social supports, and services and identify needs for new social supports and services across the lifespan that can ease suffering and improve the quality of life for people impacted by rare diseases and their caregivers.

3. Foster communication and collaboration to empower the rare disease community in MA

The rare disease community comprises an ecosystem of passionate stakeholders who are working to improve the lives of people with rare diseases. The Massachusetts RDAC will seek to strengthen ongoing communications and collaborations between rare disease advisory bodies, community-based organizations, and public and private organizations including life sciences and healthcare sectors to ensure greater coordination regarding the research, diagnosis, and treatment of rare diseases.