



**150 YEARS**  
OF ADVANCING  
**PUBLIC  
HEALTH**

# Massachusetts Department of Public Health

## Rare Disease Advisory Council

### FULL COUNCIL MEETING

**September 15, 2022**

**9:00 am – 11:00 am**

# AGENDA

## **Welcome: Dr. Dylan Tierney, Council Chair**

- Roll Call
- VOTE Minutes from the last full council meeting on 7/28/22
- Rare Disease Speaker – Tasia Rechisky
- Discussion and VOTE on Council Priorities
- Discussion – RDAC Goals
- NOMINATIONS for Subcommittees
- Review and VOTE on Operating Procedures
- Review and VOTE on the 180-Day Report
- Adjourn

# Council Priorities

1. Improving healthcare access and quality of care for people with rare diseases.
2. Advocate for and improve access to social supports and services for people impacted by a rare disease.
3. Foster communication and collaboration to empower the rare disease community in Massachusetts.

# Priorities with Descriptors

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. 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.*

# Priorities with Descriptors

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. The Massachusetts RDAC will seek to improve awareness of and access to existing social supports and services that can ease suffering and improve quality of life for people impacted by rare diseases.*

# Priorities with Descriptors

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

*The rare disease community comprises an ecosystem of passionate stakeholders who are working to improve the lives of people with rare diseases. By necessity, these stakeholders are often singularly focused on impacting their disease of interest. There is potential to strengthen the rare disease community by fostering communication between these stakeholders across rare disease interests. The Massachusetts RDAC will seek to foster 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.*

# Creating tangible goals for each priority

**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.**

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

GOAL

Advocate for and improve access to social supports and services for people impacted by a rare disease.

GOAL

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

GOAL

# Creating Goals, Objectives and Activities

GOAL

Objective

Activity  
1

Activity  
2

Activity  
3

# Writing a meaningful GOAL

A **GOAL** is an inspirational statement about what you want to achieve.

- Broad, future-oriented statement that describes the expected effect
- Defines scope
- Provides a framework for objectives

EXAMPLE:

**Using existing publicly available records and information, develop a profile of the social and economic burden of rare diseases in Massachusetts. (*legislative language ii*)**

# EXAMPLE

## GOAL

Using existing publicly available records and information, develop a profile of the social and economic burden of rare diseases in Massachusetts.

## Objective

**Estimate the prevalence of rare diseases in Massachusetts**

## ACTIVITIES

1. Develop a methodology for measuring the prevalence of rare disease
2. Create a survey tool
3. Identify epidemiologic/statistical resources to assist in measuring the prevalence

# Creating SMART Objectives

## **SPECIFIC**

Who and what?

## **MEASURABLE**

By how much?

## **ACHIEVABLE**

How?

## **RELEVANT**

Why?

## **TIME-BOUND**

When?

By June 30, 2023, identify 100% of the publicly available records and information on the prevalence of rare diseases in Massachusetts using the NORD list of rare diseases by diagnosis.

# Creating Goals for each Priority

Improve healthcare access and quality of care for people with a rare disease.

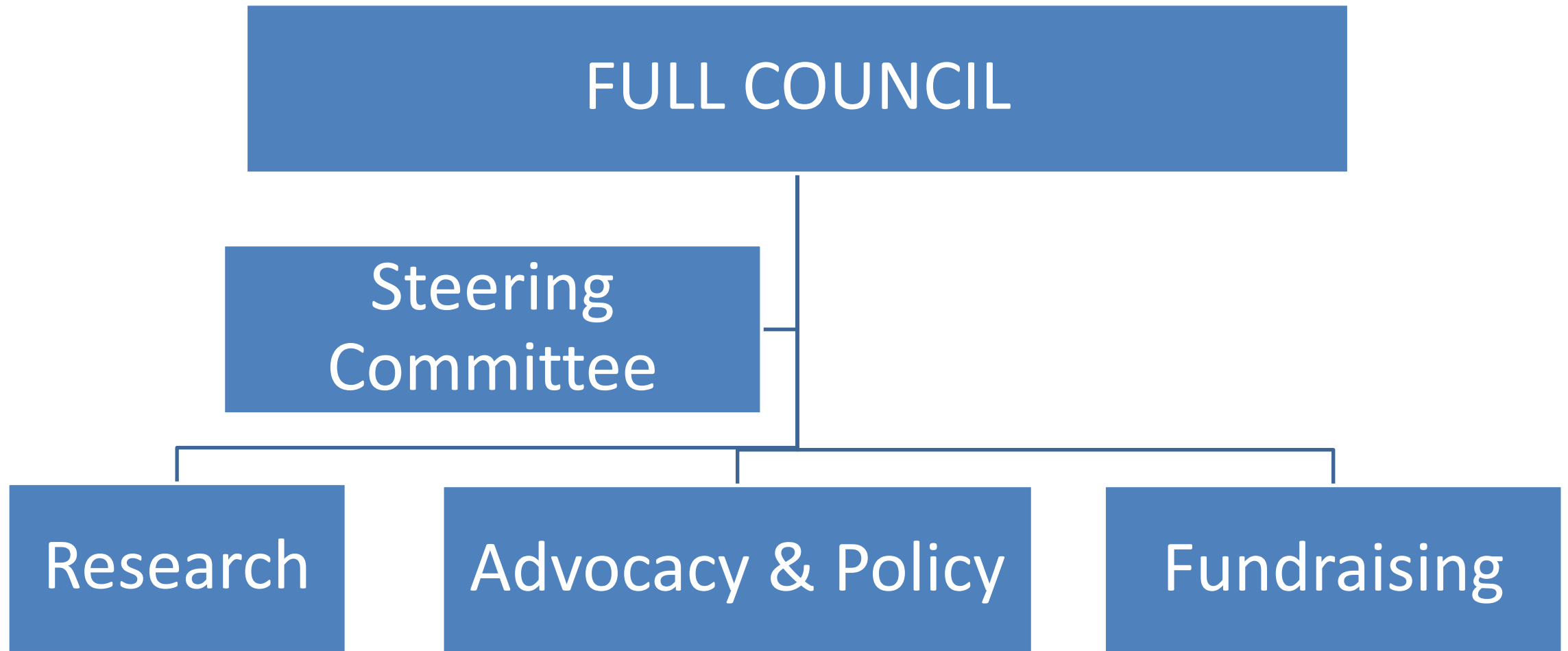
What would be a goal under this priority?

Example

1. Identify barriers to access quality healthcare for people with rare diseases on Massachusetts

What activities would need to be completed to reach this goal?

# NOMINATIONS for Subcommittees

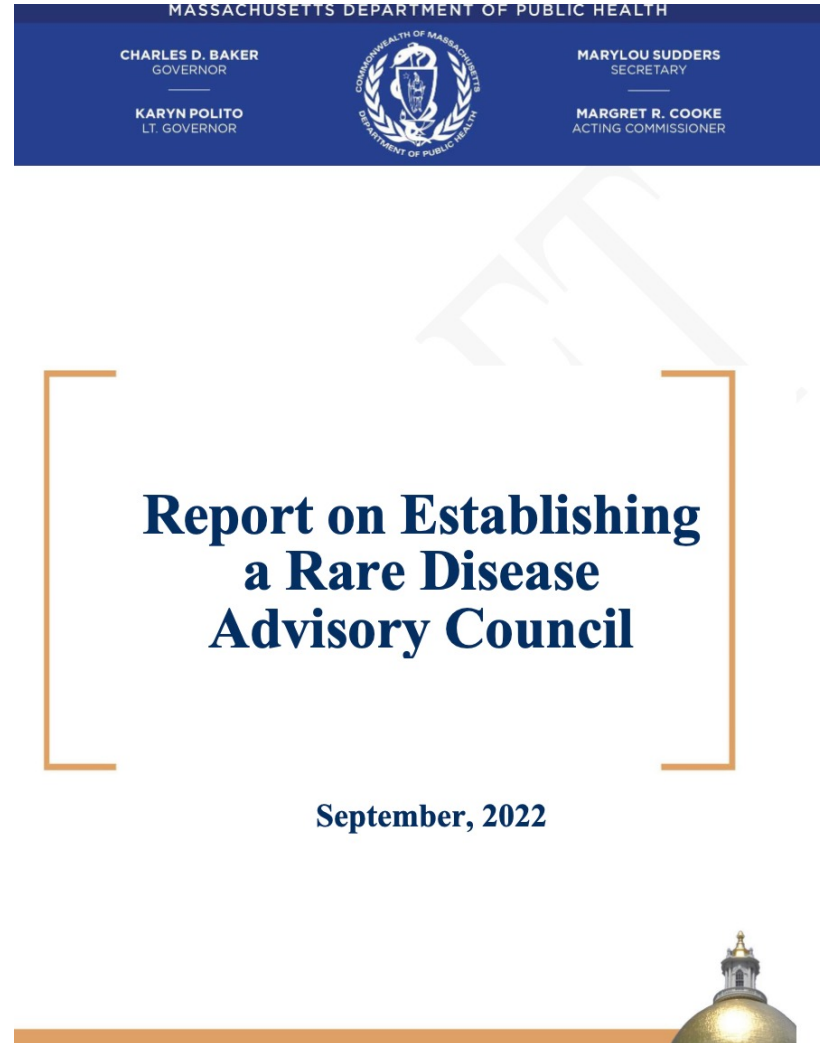


# OPERATING PROCEDURES

Review and VOTE on Operating Procedures for the RDAC



# Review and VOTE on 180-Day Report



# Adjourn

## **Next Full Council Meeting**

November 17, 2022

9:00 am – 11:00 am

# Adjourn

# Thank you

Dr. Dylan Tierney, RDAC Chair

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