



## ANNUAL REPORT FFY 2023

Summary of activities undertaken by the Council for FFY 2023

*Envisioning a world where every Minnesota citizen living with a rare disease has access to a timely diagnosis, comprehensive care, and an effective treatment.*

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## PILLARS OF FOCUS



Deepen our understanding of the collective needs of the rare disease community



Reduce the time to diagnosis



Increase coordination of care/improve transition of care



Accelerate rare disease research



## LETTER FROM THE CHAIR

Abbie Meyers, the founder of the National Organization for Rare Disorders (NORD) and mother to a child with a rare disease once said, “Families affected by rare diseases represent a medically disenfranchised population that falls through the cracks of every healthcare system in the world.” In 2019 and again in 2022 the Minnesota State Legislature heard the voice of the rare disease community when it asked for a stronger voice in the healthcare system by first establishing the Council and then by transitioning the Council to an executive branch state agency. At the time of its establishment, the Minnesota Rare Disease Advisory Council (MNRDAC) was only 1 of 3 such state Councils. Currently, 28 states have passed legislation for RDACs and many of these states look to Minnesota for leadership. The Minnesota Rare Disease Advisory Council is proud of our position as a national leader in operationalizing this nationwide effort to ensure that individuals with rare diseases are given equitable access to healthcare.



RDAC was active in 2023, seeking to operationalize our newly-formed agency, working with the Legislature to pass bills, improve policies, increase representation in state agencies, and build collaborations with community partners. Our engagement with policy makers ensured that legislators heard directly from individuals with rare diseases to better understand the impacts bills would have on their lived experience. Throughout 2023 we increased our collaboration with state agencies such as DHS and MDH to review policies and programming and provide advice on the unique considerations that must be incorporated into policy deliberations for the rare disease community. The Council held patient listening sessions and town halls and engaged in public speaking opportunities both within state and nationally.

Looking forward to 2024, the Council will continue to focus on broadening our impact. We will continue to be an expert resource for our policy makers, engage with the medical community to ensure that clinicians feel confident in providing care to the rare disease community, and equip the rare disease community to take a direct role in driving meaningful policy changes. While there is much to be done, the Minnesota Rare Disease Advisory Council is optimistic about the future for the rare disease community and is confident that the state of Minnesota will continue to be a national leader in finding innovative solutions to overcome the barriers to care encountered by people with rare diseases. .

Truly, we are stronger together.

Sincerely,

*Erica Barnes*

**Erica Barnes, MA, CCC-SLP**  
MNRDAC Executive Director

## INTRODUCTION

### Who is the Rare Disease Community?

The FDA defines a rare disease as a condition that affects fewer than 200,000 people in the US. There are over 7,000 rare diseases and the total number of Americans living with a rare disease is estimated at between 25-30 million. While each individual community may be small, collectively they represent a significant 8-10% of the population. Despite this large collective number, individuals with rare diseases encounter a number of inequities when seeking care in our health system such as

- Significant delay in receiving a diagnosis (7-8 years)
- Significant number of visits prior to diagnosis (8 different clinicians)
- Numerous misdiagnosis (2-3)
- Lack of effective treatments options (only 5% of rare diseases have an FDA approved treatment)

### The Minnesota Rare Disease Advisory Council: Who we are, how we got here

In 2019, recognizing that the current structures of the health system are inequitable for individuals with rare diseases, a grassroots group of patient advocates called on the state of Minnesota to pass legislation for the creation of a Rare Disease Advisory Council. The bill passed unanimously and was endorsed by 42 organizations across patient advocacy groups, hospital systems, medical associations, and industry. The Council was originally housed at the University of Minnesota where it received vital support to conduct needs assessment surveys. In 2022 new legislation was passed to transition the council to an executive branch state agency. Membership includes patient advocates, physicians and researchers, other related health professionals, industry, hospital administrators, and legislators.

The Minnesota Rare Disease Advisory Council is an executive branch, non-cabinet agency tasked with advocating for improved care for the rare disease community. The council is a cross-sector and geographically diverse group of governor-appointed council members. Our primary stakeholders are the Governor, Legislature, state agencies, rare disease patient organizations, and, foremost, citizens.

## AGENCY GOALS AND OBJECTIVES

### Be a comprehensive policy and information resource

- Legislature
- State agencies
- Governor's Office

### Support and equip the medical community

- Diagnostic support tools
- Increased awareness of healthcare disparities

### Empower the rare disease community

- Organizational maturation
- Connection to the medical and research community
- Increased support for advocacy, connection to policy makers

## 2023 MEMBERS

**David Tilstra, MD** | Hospital Administrator (Greater Minnesota)  
**Amy Gaviglio, MS CGC** | Genetic Counselor  
**Angela Cowen, LCSW** | Social Worker  
**Art Beisang, MD** | Physician (Pediatrics)  
**Sheldon Berkowitz, MD** | Physician  
**Barbara Joers** | Hospital Administrator  
**Dawn Stenstrom** | Industry  
**Jackie Foster, MPH, RN, OCN** | Rare Disease Non-profit  
**Abbie Miller, MD** | Health Plan Representative  
**Karl Nelsen, PA-C** | Rare Disease Patient/Caregiver  
**Kerry Hansen, RN** | Nurse  
**Kris Ann Schultz, MD** | Physician  
**Maureen Alderman** | Rare Disease Patient/Caregiver  
**Paul Orchard, MD** | Researcher  
**Rae Blaylark** | Rare Disease Patient/Caregiver  
**Soraya Beiraghi, DDS, MSD, MS, MSD** | Dentist

### LEGISLATIVE APPOINTEES (VOTING)

**Sen Julia Coleman** | Legislator  
**Sen Kelly Morrison, MD** | Legislator  
**Rep Tom Kelly** | Legislator  
**Rep Liz Reyer** | Legislator

### EX OFFICIO (NON-VOTING)

**Nicole Brown, RN** | Commissioner of Health designee  
**Nishitha Pillai, MD** | University of Minnesota Medical School  
**Lisa Schimmenti, MD** | Mayo Clinic



**Rare is not rare.**

## HOW WE MET OUR GOALS IN 2023

The following calendar provides an overview of the high-level activities of RDAC in 2023



### MONTHLY

- Council committees meet

Executive, Policy committee meet throughout the calendar year

### JANUARY - MAY

- Council executes on its legislative agenda

RDAC engages through meeting with legislators, providing public testimony, writing letters of support for coalition partners, and providing technical feedback on legislation and policies

### JANUARY

- Annual Report due
- “Project Reach,” University of Minnesota
- Quarterly full Council meeting

The Council begins 2023 strategic planning. Annual report due to the Legislature.

### FEBRUARY

- Rare Disease Day

RDAC celebrates this international day of recognition for the rare disease community

### MARCH

- Center for Orphan Drug Research, University of Minnesota Rare Disease Day Conference. “Rare: Stories of Dis-ease” documentary premier (written by Kevin Kling)

RDAC serves on the planning committee, participates in CODR program. Documentary of a collaboration that RDAC participated in premier at conference.

### APRIL

- Council Quarterly meeting
- New website was created

Council provides oversight and governance

### MAY

- Legislative session ends

Council initiates planning for debriefing the community, implementation planning commences for successfully passed legislation

### JUNE

- Patient listening session
- Strategic planning activities

RDAC hosts a listening session to both debrief the community on 2023 legislative outcomes and gather feedback for 2024 legislative priorities

### JULY

- FY23 end
- Council quarterly meeting

RDAC closes out FY23, Council continues oversight and governance work

### AUGUST

- “Rare at the Fair”

RDAC hosts a booth at the Minnesota State Fair for a day, meets individuals from 26 rare disease patient communities who share their lived experiences of rare disease

### SEPTEMBER

- “Grand Challenge” class lecture collaboration
- Integrated Gov Delivery platform and created newsletter

RDAC participates in class lectures as well as project guidance for a University of Minnesota undergraduate course focused on rare diseases

### OCTOBER

- Full Council strategic planning retreat
- NORD Breakthrough Summit presentations and federal legislative meetings

The Council participates in a retreat to train members and focus on strategic planning. RDAC staff and Council members attend the NORD Breakthrough Summit in Washington DC, meet with MN’s federal legislators to raise awareness of federal policy related to rare diseases.

### NOVEMBER

- Advocacy Day 2024 planning commences.
- 2024 policy agenda finalized

RDAC engages patient advocacy groups/ forms a coalition for Advocacy Day at the Hill 2024. Council approves 2024 legislative agenda

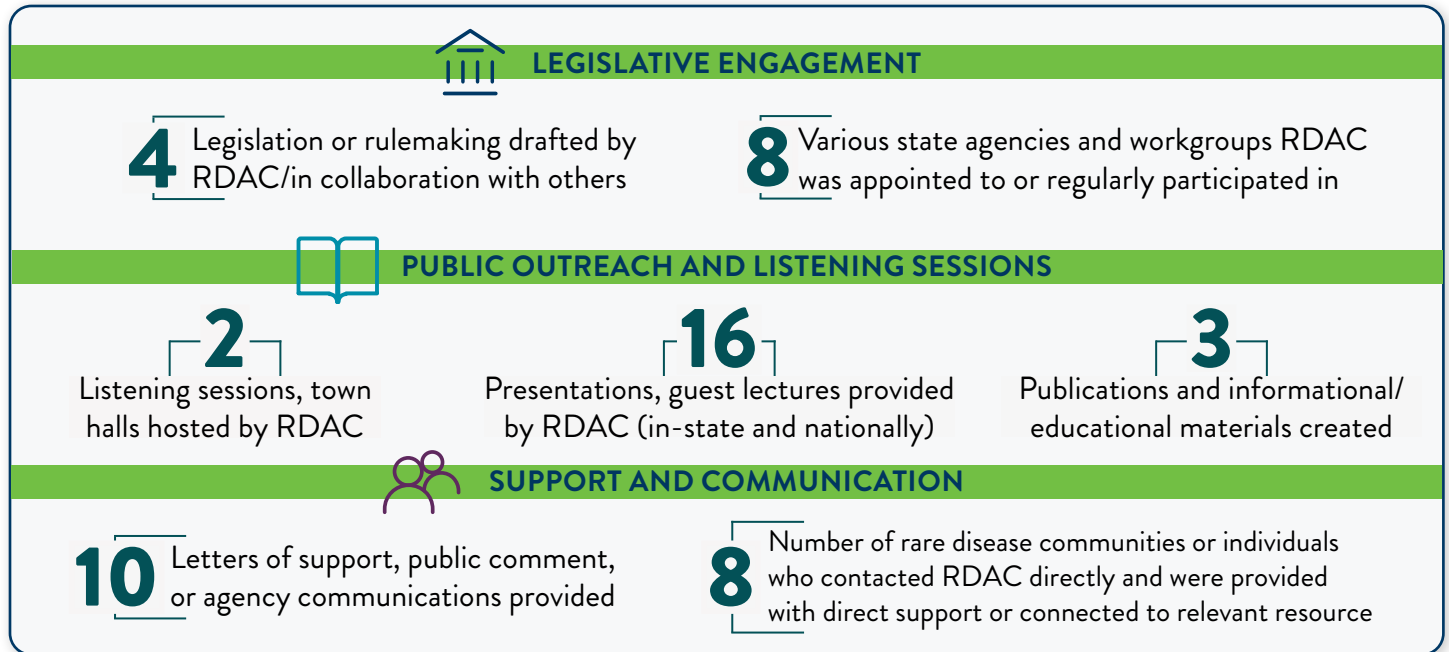
### DECEMBER

- Town Hall

RDAC hosts a town hall to educate and prepare the rare disease community for policy changes that will go in to effect in 2024

## HOW WE MET OUR GOALS IN 2023, continued

### Summary of success metrics



### Goal #1: Be a comprehensive policy resource

In 2023, the Council accomplished this goal both by successfully passing legislation as well as engaging with other state agencies to ensure that state policy takes into consideration the unique needs of the rare disease community. Below is a list of legislative accomplishments as well as committees and workgroups RDAC participated in 2023.

#### Agencies engaged:

Opioid Prescribing Improvement Program (DHS)	Ensure that patients with rare diseases and chronic pain have access to appropriate pain management
Sickle Cell Data Collection Workgroup (MDH)	Collect baseline data to determine ways to improve care for individuals diagnosed with sickle cell disease
Health Economics Program, All Payer Data Claims (DHS)	Establish prevalence data for rare diseases in Minnesota
Telehealth Workgroup, Center for Health Information Policy and Transformation (DHS)	Establish the unique needs of the rare disease community that telehealth addresses
Health Improvement and Benefit Design (DHS)	Establish criteria for the expansion of genetic testing to infants in the NICU with a suspected rare disease
Transition of Care Learning Collaborative (community partners and MDH)	Improve transition from pediatric to adult care for children and youth with rare diseases
Newborn Screening Committee (MDH)	Assist patient communities in advocating for the addition of their condition to the newborn screening panel
Drug Formulary Review Committee (DHS)	Provide input on unique considerations in drug development and access related to rare diseases



## HOW WE MET OUR GOALS IN 2023, continued

Legislation advocated for:

### NETWORK ACCESS BILL (HF384/SF1029)

The HHS Omnibus included this landmark legislation that allows individuals with a diagnosed rare disease (or a suspected rare disease defined by specific criteria) to receive care from a specialist with expertise in their rare disease without being assessed an out-of-network fee by health plans.

### DRUG FORMULARY COMMITTEE REFORM (HF1159/SF1129)

The HHS Omnibus contained language that makes significant positive modifications relevant for the rare disease community to the Drug Formulary Review Committee (the committee that oversees the state's preferred drug list). Reforms include the addition of the RDAC as a voting member, the addition of a physician with knowledge of rare diseases, and some improvements to access for specific medications.



### EXCESSIVE PRICE INCREASES BY MANUFACTURERS PROHIBITION TO GENERIC OR OFF-PATENT DRUGS (SF168/HF17) (PDAB) RDAC MEMBERSHIP

The Commerce Omnibus Bill included language to create a Prescription Drug Affordability Board (PDAB). The MN Rare Disease Advisory Council is included, serving in an advisory capacity.

### RDAC FUNDING BILL (HF988/SF705)

The final amount that the HHS omnibus appropriated to the MN Rare Disease Advisory Council is \$314,000 for FY23 and \$326,000 thereafter. While this is just over double the original budget, it does not include the amount requested to increase program capacity. The sunset was removed, meaning this is a base budget that does not expire in four years as did the original funding.

## HOW WE MET OUR GOALS IN 2023, continued

### Goal #2: Support and equip the medical community

In 2023 the Rare Disease Advisory Council provided education to a number of medical providers, researchers, and medical students to ensure that the medical community is better equipped to provide appropriate and meaningful care to the rare disease community. Below is a list of presentations and mentorships the Rare Disease Advisory Council participated in:

- Children’s Hospital of Philadelphia student forum lecture
- Center for Orphan Drug Research, University of Minnesota Rare Disease Day moderator, co-planner
- “Students for Rare” co-sponsor and guest presenter
- National Association of Health Data Organizations presentation (ICD-10 code project)
- Emerging Therapies Conference speaker- reinsurance market solutions for reimbursement challenges in rare disease treatment
- Children and Youth with Special Needs division/MDH retreat speaker
- U of MN Grand Challenge undergraduate course guest lecturer and student project mentor
- Democratic Campaign Conference panelist
- NORD speaker-RDAC panel
- NORD corporate Counsel meeting with Rep Reyer and Rep Murphy
- PACER Center presentation
- RDAC Massachusetts Council presenter
- RDAC Tennessee Council presenter
- National Conference of State Legislators Forecast conference presenter
- National Organization for Rare Disorders (NORD) policy forum presenter

### Goal #3: Support the rare disease community

It has been said that those who are closest to some of society’s most challenging problems are often the furthest away from the mechanisms to address the problems. The Rare Disease Advisory Council is dedicated to empowering the rare disease community so the community itself can provide direct input into policy changes and education aimed at improving care for the rare disease community. Below is a list of activities related to this goal:

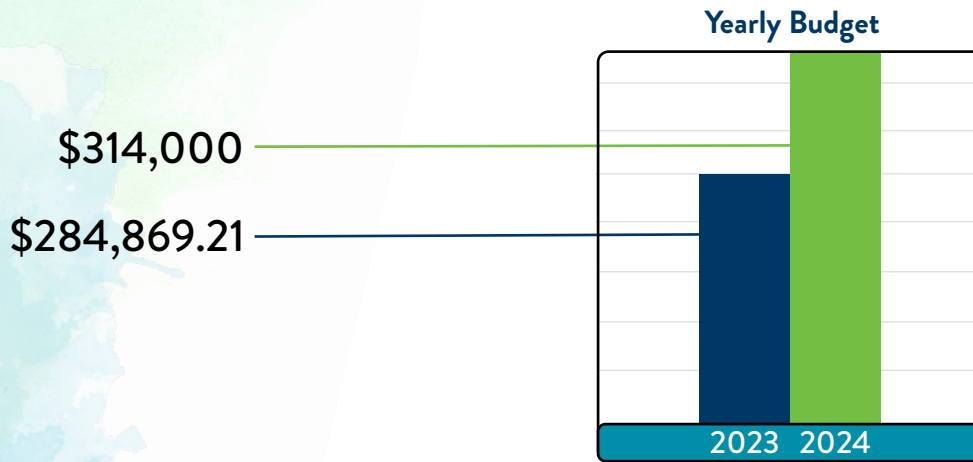
- “Project Reach” (U of Minnesota School of Public Health) guest lectures and mentorship provided
- Patient Advocacy Group Listening session related to identification of policy priorities convened
- Patient group convening session to assist with application to the newborn screening panel convened
- National Institute of Health Clinical Trial Readiness Consortia, Patient Advocacy Group Chair
- Patient Community Town Hall related to policy changes coming in 2024 held

The Minnesota Rare Disease Advisory Council was able to accomplish these goals thanks to the generous contributions of the **Minnesota Council on Disability** in providing us with mentorship and administrative support.

## 2023 FINANCIAL REPORT

### Agency budget and staffing

The Rare Disease Advisory Council began FY23 with a carried over appropriation of \$286,618 from the University of Minnesota that supported 1 full time employee (FTE). In March the Council was able to hire 1 additional staff to serve as operations manager. The Council began FY24 with a base operating budget of \$314,000.



### Budget expenditures FY23

