MINNESOTA
RARE DISEASE ADVISORY COUNCIL

ANNUAL REPORT 2025

Summary of activities undertaken by the
Minnesota Rare Disease Advisory Council in 2025

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

TABLE OF CONTENTS

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LETTER FROM THE CHAIR

Dear Legislators, Policy Makers, Community Members, and Friends,

We are honored and excited to present our annual report for 2025. This year's report will show that Minnesota is considered the leader and a vital resource in rare disease communities. The council staff have been engaged in numerous activities outlined in the report. Staff have been asked to lead or take part in discussions about the rare disease environment at conferences, lectures, town halls, and other public forums in Minnesota and across the nation. The Minnesota Rare Disease Advisory Council (MNRDAC) has aided in drafting legislation to support the early recognition of rare disease and to reduce the financial and bureaucracy burden for those who have rare disease. In the future, MNRDAC will continue to focus on reducing the cost burden of rare diseases so that more patients will be able to access the rapidly increasing number of diagnostics and treatments available. Thank you for your support of our work in this rapidly changing field.

David Tilstra

David Tilstra, MD, MBA CPE
Chair, Minnesota Rare Disease Advisory Council

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. Recent analysis estimates that there are over 10,000 rare diseases and the total number of Americans living with a rare disease is 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)[1]
- 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)
- A substantial collective indirect and direct cost burden even when compared to more prevalent conditions[2]

THE MINNESOTA RARE DISEASE ADVISORY COUNCIL

The Minnesota Rare Disease Advisory Council is an executive-branch, non-cabinet agency dedicated to representing the needs of the rare disease community in state government. We hold ourselves to a high standard of accountability and transparency. Through our use of the Results-Based Accountability (RBA) framework and adherence to transparent decision-making, we operate with integrity.

PILLARS OF FOCUS

1. **Deepening our understanding of the collective needs of the rare disease community**
2. **Reducing the time to diagnosis**
3. **Increasing coordination of care/improving transition of care**
4. **Accelerating rare disease research**

AGENCY GOALS AND OBJECTIVES

1. **Be a comprehensive policy and information resource**
    - Legislature
    - State agencies
    - Governor's Office

2. **Support and equip the medical community**
    - Diagnostic support tools
    - Increased awareness of healthcare disparities

3. **Empower the rare disease community**
    - Organizational maturation
    - Opportunities for connection and cross-disease partnerships
    - Connection to the medical and research community
    - Increased support for advocacy, connection to policy makers

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[1] ShireReport-1.pdf (globalgenes.org)

[2] NIH Study Suggests People with Rare Diseases Face Significantly Higher Health Care Costs | National Center for Advancing Translational Sciences

CURRENT MEMBERS

David Tilstra, MD, MBA CPE | Hospital Administrator (Chair)
Jackie Foster, MPH, RN, OCN | Rare Disease Non-profit (Vice-Chair)
Erica Barnes, MA-SLP | Minnesota Rare Disease Advisory Council Executive Director

Maureen Alderman | Rare Disease Patient/Caregiver
Art Beisang, MD | Physician (Pediatrics)
Rae Blaylark | Rare Disease Patient/Caregiver
Angela Cowen, LCSW | Social Worker
Dan Endreson | Health Plan Representative
Kerry Hansen, RN | Nurse
Barbara Joers | Hospital Administrator
Laura Lambert, PhD | Researcher
Elizabeth Lando-King | Rare Disease Patient/Caregiver
Alexandra Mohror, MS CGC | Genetic Counselor
Paul Orchard, MD | Physician
Paul Peterson | Dentist
Richard Schirber | Ad hoc member
Sofia Shrestha/Jeannine Conway | Pharmacist
Doug Smith, MD | Physician
Dawn Stenstrom | Industry

Legislative Appointees (Voting)

Sen Julia Coleman | Legislator
Sen Liz Boldon | Legislator
Rep Tom Murphy | Legislator
Rep Liz Reyer | Legislator

Ex Officio (Non-Voting)

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

Rare is not rare.

MINNESOTA
RARE DISEASE ADVISORY COUNCIL

HOW WE MET OUR GOALS IN 2025

HIGH-LEVEL ACTIVITIES IN 2025

MONTHLY

- Council and committees meet
- ECHO sessions

- Full Council and Executive/ Policy committees meet throughout the calendar year
- MNRDAC hosts monthly ECHO sessions to support clinicians in various aspects of rare disease care

JAN - MAY

- Council executes on its legislative agenda

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

JAN

- Annual report due
- Legislative Session Engagement
- Quarterly Full Council meeting

- Annual report due to the Legislature
- MNRDAC continues ECHO series (telementoring for providers caring for rare disease patients)
- MNRDAC initiates engagement for legislative priorities

FEB

- Rare Disease Day
- NIH funded Workshop
- Speaking Engagements (3)

- MNRDAC celebrates this international day of recognition for the rare disease community
- Facilitates advocacy workshop for NIH-funded consortia
- ECHO session # 4

MAR

- Center for Orphan Drug Research, University of Minnesota Rare Disease Day Conference
- Speaking Engagement (4)

- MNRDAC serves on the planning committee, participates in CODR program.

APR

- Advocacy Day at the Capitol
- Quarterly Full Council meeting
- Speaking Engagements (3)

- Advocates for rare diseases gather at State Capitol

MAY

- Legislative session ends
- Speaking Engagements (3)

- Legislative session ends, Council initiates planning for debriefing the community, implementation planning commences for successfully passed legislation

JUNE

- Strategic planning activities
- Speaking Engagements (1)

JULY

- FY25 end
- Speaking engagement (1)

- MNRDAC closes out FY25, Council engages in strategic planning for FY26

AUG

- "Community Forum/Legislative Debrief
- Rare at the Fair"

- MNRDAC hosts a forum to both debrief the community on 2025 legislative outcomes and gather feedback for 2026 legislative priorities (see Insights Report on website)
- MNRDAC hosts a booth at the Minnesota State Fair for a day, meets individuals who share their lived experiences of rare disease

SEPT

- "Grand Challenge" class lecture collaboration
- Speaking engagements (2)

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

OCT

- NORD Breakthrough Summit presentation
- Council Retreat
- Speaking engagements (3)

- MNRDAC staff attend the NORD Breakthrough Summit in Washington DC, presented to other state MNRDACs
- Council holds retreat, Results-Based Accountability Framework training initiated

NOV

- Hormel Institute Viral Manufacturing Translational Consortia Tour
- RBA goal setting

- MNRDAC organizes a tour of Hormel Institute's Viral Manufacturing Translational Consortia Tour in Austin, MN
- Council members and agency staff establish performance metrics based on RBA training

DEC

- Speaking Engagement (1)
- Policy Priorities 2026 finalized

- MNRDAC determines 2026 policy priorities

HOW WE MET OUR GOALS IN 2025, continued

SUMMARY OF SUCCESS METRICS

LEGISLATIVE ENGAGEMENT

4 | Legislation or rulemaking drafted by MNRDAC, in collaboration with others

8 | Agency committees, departments, workgroups MNRDAC regularly participated in or collaborated with on an initiative

PUBLIC OUTREACH AND LISTENING SESSIONS

3 | Listening sessions, town halls, community forums hosted by MNRDAC

18 | Presentations, guest lectures provided by MNRDAC (in-state and nationally)

5 | Publications (3) and informational/educational materials (2) created or contributed to

SUPPORT AND COMMUNICATION

7 | Letters of support requested by and provided to patient groups or medical communities

13 | Conference exhibitorship and outreach

5 | Number of constituents supported upon request from legislators

GOAL #1: BE A COMPREHENSIVE POLICY RESOURCE

In 2025, the MNRDAC 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 MNRDAC participated in in 2025

- **Sickle Cell Data Collection Workgroup (MDH)** | Collect baseline data to determine ways to improve care for individuals diagnosed with sickle cell disease, identify policies to improve care for individuals with SCD
- **Health Economics Program, All Payer Data Claims (DHS)** | Establish prevalence data for rare diseases in Minnesota, determine population eligibility for gene therapy
- **Department of Commerce** | Serve on the Prescription Drug Affordability Board (PDAB) to address affordability challenges
- **Transition of Care Learning Collaborative*** | Improve transition from pediatric to adult care for children and youth with rare diseases
- **Newborn Screening Committee (MDH)** | Identify ways to improve Minnesota's newborn screening program
- **State Medical Review Team (DHS)** | (*New in 2025*) Asset in implementation of expedited disability determination pathway for individuals with rare diseases
- **Drug Formulary Review Committee (DHS)** | Provide input on unique considerations in drug development and access related to rare diseases, facilitate public comment submission by rare disease patient communities
- **Care Coordination "Learning Journey" collaborative (MDH)** | (*New in 2025*) Assist the state of Minnesota in moving beyond program-level improvements toward a more transformative, cross-sector effort that clarifies Title V's role in an evolving environment, strengthens connectedness and cohesion among partners, and positions Minnesota to take collective action on care coordination

HOW WE MET OUR GOALS IN 2025, continued

LEGISLATION ADVOCATED FOR

Establishment of expedited disability determination pathway for children identified through newborn screening Passed, effective January 2025	Infants who receive a positive newborn screening result for a serious medical condition require timely access to care. An expedited pathway for disability determination based on a positive newborn screen was included as part of the Health and Human Services omnibus bill. The State of Minnesota expanded the expedited disability determination process to support faster access to Medical Assistance (MA) for these vulnerable patients.
Addition of Metachromatic Leukodystrophy to MN Newborn Screening Panel (supported) Passed, MLD added	Metachromatic leukodystrophy (MLD) is a devastating condition that is universally fatal unless identified and treated per-symptomatically. MNRDAC worked with the advocacy community, legislators, and the MN Department of Health to add MLD to the MN newborn screening panel. Nationally, the commitee assigned to add diseases to the Recommended Uniform Screening Panel (RUSP) was disbanded; however, MLD was added individually to the RUSP in December 2025.
Ban on copay accumulator programs Did not pass	Many specialty drugs for rare diseases are expensive and the overwhelming number of them do not have a generic equivalent. Cost sharing for these drugs is a significant burden to rare disease patients and MNRDAC supports a prohibition on programs that prohibit the use of patient assistance coupons to be counted towards the individuals' cost sharing totals.
MNRDAC Base Operations Funding Passed, full funding received	MNRDAC is grateful to the Legislature for providing full funding for the requested base operating budget identified.

HOW WE MET OUR GOALS IN 2025, continued

GOAL #2: SUPPORT AND EQUIP THE MEDICAL COMMUNITY

In 2025 the Rare Disease Advisory Council provided education to a number of medical providers, researchers, and students to ensure that the medical community is better equipped to provide appropriate and meaningful care to the rare disease community.

- University of Pennsylvania Engaged Scholars program
- GLIA Consortium (NIH Funded network)
- Center for Orphan Drug Research, University of Minnesota Rare Disease Day
- American College of Medical Genetics Conference
- Project Reach, University of Minnesota School of Public Health
- Newborn Screening Alliance Meeting
- United Leukodystrophy Foundation newborn screening session
- Fairview Pharmacy Symposium
- Hormel Institute Viral Manufacturing and Translation Consortium Workshop
- NORD Summit panel participant
- U of MN Grand Challenge class lecture and community workshop partner
- U of MN “Mini Medical School” panelist
- “Genomic Newborn Sequencing: A State Level Approach” webinar host, (*New in 2025*)

Throughout 2025 the MNRDAC continued its telementoring program. Project ECHO[3] is a widely used tele-education platform that connects primary care clinicians from across the state to rare disease specialists and experts. Sessions are held monthly and include a presentation on a relevant topic related to rare diseases and case review to increase provider competence in identification and referral for rare diseases. ECHO sessions addressed topics such as the use of genetic testing to diagnose rare diseases, the intersection of rare disease and mental health, and the implications of a positive newborn screen on a clinician’s practice. Following the completion of the first series, MNRDAC found the following:

- 162 unique individuals registered, a total of 81 unique attendees. This indicated robust attendance (average of 20.6 attendees per session) compared to other ECHOs in the state.
- Repeat participation. On average, each attendee joined 2.0 of the eight sessions. Nurses and physicians comprised the most consistent audience, accounting for between one- and two-thirds of attendees for any given session, though attendees included a wide range of clinical backgrounds and fields of expertise.
- Positive responses. Across all surveys, evaluations were very positive. Respondents rated the series as 4.6 out of 5 for “the ECHOs met their stated objectives and provided valuable information they could use in their organization or practice.”

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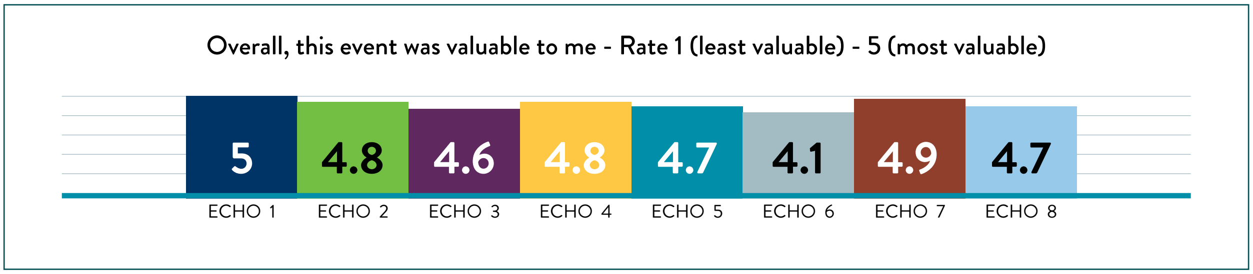
[3] Osei-Twum JA, Wiles B, Killackey T, Mahood Q, Lalloo C, Stinson JN. Impact of Project ECHO on Patient and Community Health Outcomes: A Scoping Review. Acad Med. 2022 Sep 1;97(9):1393-1402. doi: 10.1097/ACM.0000000000004749. Epub 2022 May 24. PMID: 35612913.

HOW WE MET OUR GOALS IN 2025, continued

GOAL #2: SUPPORT AND EQUIP THE MEDICAL COMMUNITY, continued

When broken down by session, responses were as follows:


Overall, this event was valuable to me - Rate 1 (least valuable) - 5 (most valuable)

Genomic Sequencing Education
Genomic sequencing in newborns (gNBS) holds enormous potential of early diagnosis and detection for individuals with rare diseases[4]. Reducing the diagnostic journey can allow for timely intervention and treatments as well as a reduction in healthcare utilization. At the same time, implementation into public health programs and clinical practice poses a number of challenges. MNRDAC is engaging internationally recognized experts to take a deeper look at the ethical and operational challenges incorporating this technology into clinical practice in a 3 part educational series spanning 2025-26 (recording can be found on the MNRDAC website). For presentations 1 and 2, investigators from the Guardian study –which was recently recognized by JAMA as one of the most impactful studies of 2025–will join us in presenting.

GOAL #3: SUPPORT THE RARE DISEASE COMMUNITY

The MNRDAC is committed to ensuring that the rare disease patient community is equipped and supported to engage in policy and research decisions that directly affect them. The MNRDAC continued some of the 2024 activities as well as expanded its efforts in 2025.

In 2025, MNRDAC launched a "Monthly Meetup" for patient advocacy leaders. The vast majority of patient advocacy groups for rare diseases are led and managed by people directly affected by rare diseases. Often the work is isolating and challenging with little in the way of training. Based on community feedback, MNRDAC has designed the meetups to provide mentorship, opportunities for collaboration, and mental health support for patient advocacy leaders in the state of Minnesota.

- "Project Reach" (University of Minnesota School of Public Health) guest lectures and mentorship provided
- "Students for Rare" co-sponsorship
- Host of patient advocacy group breakfast in collaboration with the Center for Orphan Drug Research (University of Minnesota)
- Rare Disease Day at the Hill attended by Patient Advocacy Groups (PAGs), individuals with rare diseases, and other stakeholders
- Patient Advocacy Group Listening session related to identification of policy priorities
- Pompe Community Town Hall- Medicaid changes panel
- National Institute of Health Clinical Trial Readiness Consortia, Patient Advocacy Group Chair
- Patient Community Advocacy workshop (in collaboration with EveryLife Foundation)

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[4] Ziegler A, Koval-Burt C, Kay DM, et al. Expanded Newborn Screening Using Genome Sequencing for Early Actionable Conditions. JAMA. 2025;333(3):232–240. doi:10.1001/jama.2024.19662

2024 FINANCIAL REPORT

AGENCY BUDGET AND STAFFING

While this annual report is based on a calendar year, the state budget operates on a July - June fiscal year. The Rare Disease Advisory Council began 2025 in the middle of a FY25 appropriation of $668,00. MNRDAC is currently operating on a FY26 budget appropriation of $674,000 and a carryover from FY25 of $20,000.
Below is a breakdown of FY25 spending and the FY26 budget:

	2025	**2026**	**2027**
Appropriation	668	674	679
Cancellations	-	-	-
Balance Forward In	9	7	-
Balance Forward Out	-	-	-
Transfers In	-	-	-
Staff (Full time and Consulting)			
Full Time - Salary	345	458	498
Overtime	-	2	-
Other Employee Cost	-	-	-
Consulting	60	60	-
Programs			
Project ECHO	100	-	-
Taskforces	59	-	-
Travel (in state)	4	3	4
Travel (out of state)	5	2	5
Operating Costs			
Space Rental and Utilities	3	4	4
Printing and Advertising	4	2	2
Prof-Tech Serv-Outside Vend	6	57	2
Communications	1	2	2
Employee Development/training...	1	2	2
State Agcy-Prov Prof-Tech Serv...	35	57	57
Rate-Based MNIT Services	4	4	8
Agency-Specific MNIT Services ...	1	2	4
Supplies	5	2	1
Equipment	-	-	1
Other Operating Costs	1	1	5
Equipment-Non Capital	-	1	7
Expenses Total	634	659	602
Balance brought forward	43	22	77

WHAT IS ON THE HORIZON

FUTURE DIRECTIONS FOR THE RARE DISEASE COMMUNITY

Since the establishment of the Minnesota Rare Disease Advisory Council as the 3rd in the nation in 2019, roughly 30 states have passed legislation forming councils. We are proud to be national leaders as stated by the National Organization for Rare Disorders (NORD) in a letter to the Governor's office:

"The MNRDAC is nationally recognized and is considered by NORD to be a model for the other 29 Rare Disease Advisory Groups that exist across the country. Under Erica Barnes' leadership as the Executive Director, the MNRDAC was one of the first Councils to be created and has provided guidance and mentorship to other state advocates throughout the process of Rare Disease Advisory Council creation and implementation. In addition, the legislative progress and policy influence that MNRDAC has achieved has set the standard for all other states. The Minnesota Legislature and Executive branch should be proud of the innovation and progress that is being done through their state agency and provide the funded vital to continue its great work."

MNRDAC is proud to live in a state that does not simply invite rare disease advocates to the policy table but is allowing the advocacy community to build the table through MNRDAC's work.

Additionally, the National Institutes of Health (NIH) announced that Minnesota was selected as one of just seven states to participate in a multi-million-dollar NIH grant exploring the feasibility of incorporating genetic newborn sequencing (gNBS) alongside traditional newborn screening. The initiative, known as BEACONS, is part of the NIH Venture Program and aims to evaluate how genomic sequencing could responsibly expand early detection and improve health outcomes for newborns. Erica Barnes will serve on the grant's Patient Advocacy Committee, contributing patient-centered perspectives to help guide ethical implementation, equity, and family engagement throughout the project.

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