

ANNUAL REPORT 2024



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

We are honored to present our annual report for 2024.

This year's achievements reflect our dedication to making Minnesota a leader in rare disease innovation and support. Our council has actively engaged in numerous initiatives, from legislative advocacy to community outreach, ensuring that the voices of the rare disease community are heard and their needs addressed. We have collaborated with various agencies and organizations to drive meaningful change and support the medical community in providing better care. Although our young agency has much more work to do, we are proud of our progress and look forward to even more progress in the coming years. As more rare conditions are recognized and more treatments emerge, there has never been a more critical time to address the complexities facing those with a rare disease.



Sincerely,

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. 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: A NATIONAL LEADER

The Minnesota Rare Disease Advisory Council (MNRDAC) is an executive branch, non-cabinet agency established as such in 2022 after being established at the University of Minnesota (U of M) in 2019. The MNRDAC 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.

Minnesota's Rare Disease Advisory Council was one of the first to be established and 27 states currently have a Rare Disease Advisory Council. The National Organization for Rare Disorders (NORD) considers MNRDAC to be the national model and the Minnesota Rare Disease Advisory Council has engaged in numerous speaking engagements and workshops to assist other states in operationalizing their own councils. We are proud of the priority that the State of Minnesota has placed on rare disease innovation and we aim to maintain our reputation as a national leader in systems improvement for the rare disease community.

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

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 | Pharmacist
Doug Smith, MD | Physician
Dawn Stenstrom | Industry

Legislative Appointees (Voting)

Sen Liz Boldon | Legislator
Sen Julia Coleman | 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.



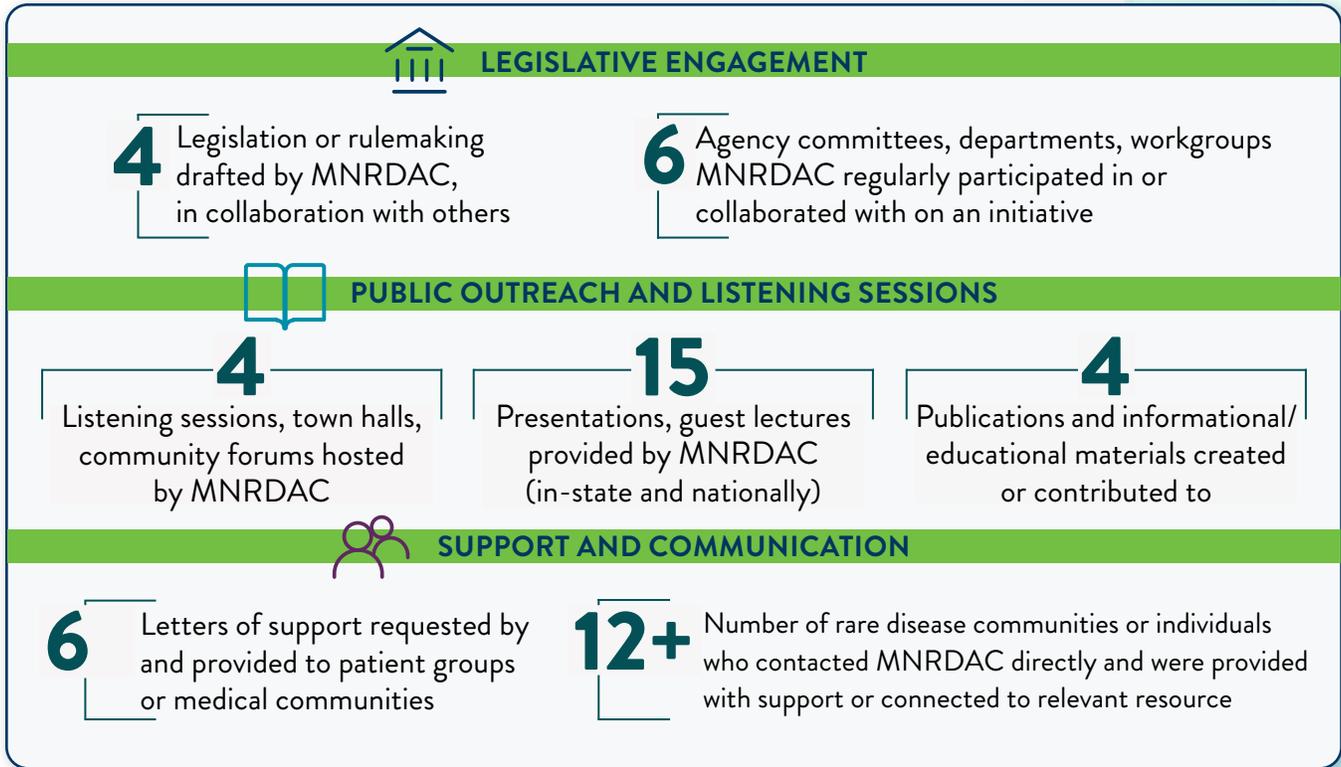
HOW WE MET OUR GOALS IN 2024

HIGH-LEVEL ACTIVITIES IN 2024

MONTHLY	<ul style="list-style-type: none"> MNRDAC and committees meet <p>Full Council and Executive/Policy committees meet throughout the calendar year</p>	JAN - MAY	<ul style="list-style-type: none"> MNRDAC executes on its legislative agenda <p>MNRDAC engages through meeting with legislators, providing public testimony, writing letters of support for coalition partners, and providing technical feedback on legislation and policies</p>
JAN	<ul style="list-style-type: none"> Annual Report due Educational preparation for legislative session <p>Gene Therapy listening and learning session hosted by MNRDAC</p>	FEB	<ul style="list-style-type: none"> Rare Disease Day Speaking Engagement <p>MNRDAC celebrates this international day of recognition for the rare disease community Co-hosts an advocacy training workshop for individuals with rare diseases in partnership with EveryLife Foundation</p>
MAR	<ul style="list-style-type: none"> Center for Orphan Drug Research, U of M Rare Disease Day Conference Rare Disease Day at the Capitol Quarterly Full Council meeting Speaking Engagement (4) 	APR	<ul style="list-style-type: none"> Speaking Engagement (1) <p>MNRDAC provides oversight and governance</p>
MAY	<ul style="list-style-type: none"> Legislative session ends Speaking Engagements (2) <p>MNRDAC initiates planning for debriefing the community, implementation planning commences for successfully passed legislation</p>	JUNE	<ul style="list-style-type: none"> Community Forum/Legislative Debrief Strategic planning activities Speaking Engagements (2) <p>MNRDAC hosts a forum to both debrief the community on 2024 legislative outcomes and gather feedback for 2025 legislative priorities (see Insights Report on website)</p>
JULY	<ul style="list-style-type: none"> FY24 end MNRDAC Retreat Speaking engagement (1) <p>MNRDAC closes out FY24, engages in strategic planning for FY25</p>	AUG	<ul style="list-style-type: none"> “Rare at the Fair” <p>MNRDAC hosts a booth at the Minnesota State Fair for a day, meets individuals who share their lived experiences of rare disease</p>
SEPT	<ul style="list-style-type: none"> “Grand Challenge” class lecture collaboration MNRDAC awarded the Eric Dick Policy Partnership Award by the Minnesota Medical Association for collaboration in 2024 Speaking and testifying engagements (2) <p>MNRDAC participates in class lectures and project guidance for a U of M undergraduate course focused on rare diseases</p>	OCT	<ul style="list-style-type: none"> NORD Breakthrough Summit presentation <p>MNRDAC staff and Council members attend the NORD Breakthrough Summit in Washington DC, presented to other state RDACs</p>
NOV	<ul style="list-style-type: none"> 2024 policy agenda finalized Project ECHO launch <p>MNRDAC approves 2024 legislative agenda MNRDAC launches an ECHO series to provide support for diagnosis to the medical community</p>	DEC	<ul style="list-style-type: none"> PAG town hall Gene Therapy taskforce launches <p>MNRDAC hosts a town hall to prepare the rare disease community for the upcoming legislative session MNRDAC launches a gene therapy taskforce to assist the State in clinical delivery of gene therapies for rare diseases</p>

HOW WE MET OUR GOALS IN 2024, continued

SUMMARY OF SUCCESS METRICS



GOAL #1: BE A COMPREHENSIVE POLICY RESOURCE

In 2024, the Minnesota Rare Disease Advisory 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 MNRDAC participated in 2024.

AGENCIES ENGAGED AND OBJECTIVES	
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 (publication pending)
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)	Assist patient communities in advocating for the addition of their condition to the newborn screening panel, engage with the committee to ensure
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
The Council for Minnesotans of African Heritage	Work in collaboration with the Council for Minnesotans of African Heritage to explore opportunities to address the needs of the community in an inclusive manner

*Community partners and MDH

HOW WE MET OUR GOALS IN 2024, continued

LEGISLATION ADVOCATED FOR

MNRDAC FUNDING (HF3841/SF3927)

As a new state agency, MNRDAC seeks to ensure that the base operating budget is sufficient to meet the needs of the agency. This additional single appropriation funding that is available through FY27 enables the MNRDAC to carry out its duties prescribed by state statutes and better serve the rare disease community. The legislative ask was a permanent increase to the base budget.

PASSED: Effective July 2024

UNBUNDLING GENE PRODUCTS FOR RARE DISEASES FROM CURRENT REIMBURSEMENTS TO HOSPITALS (SF4058/HF3664)

This bill provides separate reimbursement for biological products provided in the inpatient hospital setting as part of cell or gene therapy to treat rare diseases separate from the associated DRG. This increases access to emerging and innovative treatments, allows hospitals to provide gene therapy without significant financial loss, and allows payers to enter into value-based arrangements with manufacturers.

PASSED: Effective July 1st, 2025

EXPANDED COVERAGE OF RWGS FOR CRITICALLY ILL INFANTS WITH SUSPECTED RARE DISEASE (SF2445/ HF3330)

This bill requires commercial payers (including managed care organizations contracted with the State) to cover rapid whole genome sequencing (rWGS) for children under the age of 21 in the ICU, reducing time to diagnosis. Reimbursement must be separate from the DRG/capitated payment.

PASSED: Effective January 1, 2025

PRIOR AUTHORIZATION REFORM (SF3532/ HF3578)

This bill streamlines prior authorization and requires reporting related to how and when prior authorization is being used by payers. This improves timely access to treatments by reducing some prior authorization requirements, including treatment of a chronic condition.* This authorization does not expire unless the standard of treatment for that health condition changes.

PASSED: Effective January 1st, 2026

*A chronic health condition is defined as: a condition that is expected to last one year or more and:
(1) requires ongoing medical attention to effectively manage the condition or prevent an adverse health event; or
(2) limits one or more activities of daily living.

HOW WE MET OUR GOALS IN 2024, continued

GOAL #2: SUPPORT AND EQUIP THE MEDICAL COMMUNITY

In 2024 the Minnesota 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. Additionally, the MNRDAC increased its collaboration with the Minnesota Medical Association, becoming the recipients of the Eric Dick Advocacy partnership award. Below is a list of presentations and mentorships the Minnesota Rare Disease Advisory Council participated in:

- Mayo Clinic Rare Disease Day webinar
- University of Pennsylvania Engaged Scholars program
- Project Reach, University of Minnesota School of Public Health
- Center for Orphan Drug Research, University of Minnesota Rare Disease Day
- MDH-hosted CYSHN Local Public Health Annual Meeting
- MDH Community of Practice webinar
- North American Rare Disease Summit
- Congressional Briefing on Newborn Screening
- Mayo Precision Medicine Conference
- Sano Health: Ethical considerations for expanded genetic testing webinar
- NORD Summit panel participant
- University of Minnesota Grand Challenge class lecture and community workshop partner
- FDA public testimony on behalf of patient centered clinical trials



In November of 2024 the Minnesota Rare Disease Advisory Council launched its first clinician support tool due to an increased appropriation from the state in FY25 (FY25 appropriation details will be provided in the next annual report). Project ECHO is a widely-used tele-education platform that will connect 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 will continue into calendar year 2025 and address 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.

A promotional graphic for the Project ECHO tele-education series. It features a dark blue background with white and green text. The main headline reads "HAVE YOU EVER BEEN PUZZLED BY A PATIENT?". Below this, it says "Join the NEW Project ECHO Tele-Education Series: UNDIAGNOSED/RARE DISEASE PATIENTS". It is presented by The Minnesota Rare Disease Advisory Council and Stratis Health. There is a QR code on the left with the text "Scan the QR code to register." and "CE credits provided." at the bottom. On the right, there is a graphic of puzzle pieces, with one piece highlighted in dark blue. The Minnesota Rare Disease Advisory Council logo is in the bottom right corner.

HOW WE MET OUR GOALS IN 2024, continued

GOAL #3: SUPPORT THE RARE DISEASE COMMUNITY

The Minnesota Rare Disease Advisory Council 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. Far too often the communities who will be the most directly affected by policy decisions are afforded the least opportunity to influence those decisions.

- “Project Reach” (University of Minnesota School of Public Health) guest lectures and mentorship provided
- “Students for Rare” letter writing campaign and co-sponsorship of the club
- Host of patient advocacy group breakfast in collaboration with the Center for Orphan Drug Research (University of Minnesota)
- First ever Rare Disease Day at the Hill with multiple Patient Advocacy Groups (PAGs) participating as well as individuals with rare diseases
- Patient Advocacy Group Listening session related to identification of policy priorities
- Continued support of patient groups to facilitate the expansion of rare diseases to Minnesota’s newborn screening panel
- National Institute of Health Clinical Trial Readiness Consortia, Patient Advocacy Group Chair
- Patient Community Advocacy workshop (in collaboration with EveryLife Foundation): “Telling Your Story”
- FDA testimony following a listening session on the topic of patient perspectives for clinical trials for gene therapy

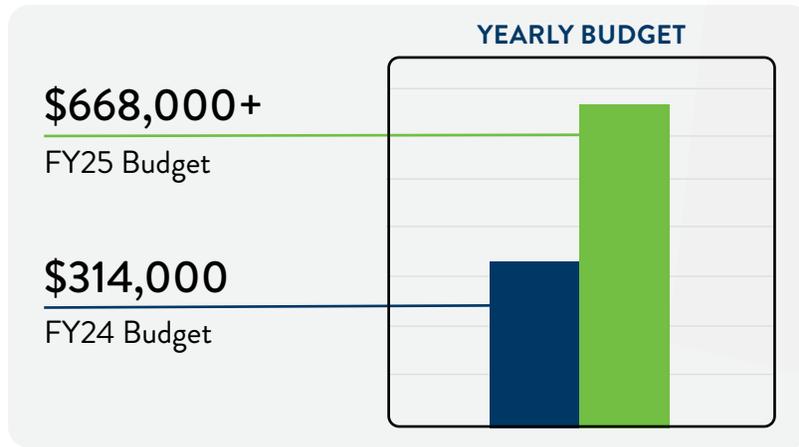


2024 FINANCIAL REPORT

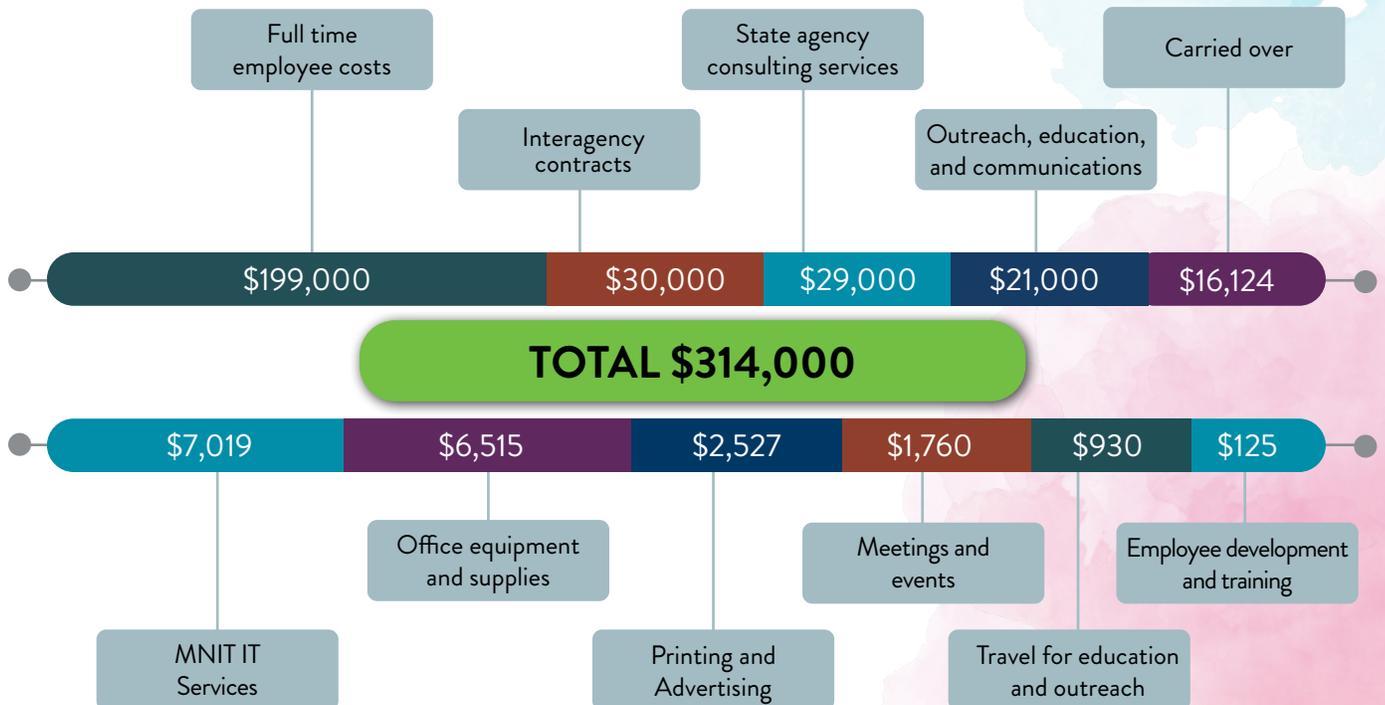
AGENCY BUDGET AND STAFFING

While this annual report is based on a calendar year, the state budget operates on a July–June fiscal year. The Minnesota Rare Disease Advisory Council began this reporting cycle with a FY24 appropriation from the Legislature of \$314,000. This amount supported 1.8 FTEs, some community outreach initiatives, resources to address policy changes, and medical community education. A FY24 budget breakdown is below.

The increased appropriation for FY25 allowed the MNRDAC to expand its programming for clinician support, collaborate on more publications, and expand our civic engagement activities in 2024. In addition to the programming dollars, the appropriation supported the hire of a policy manager and a programs/civic engagement manager, bringing MNRDAC staff to 4 FTEs. A detailed FY25 budget will be included in next year’s annual report.



BUDGET EXPENDITURES FY24



WHAT IS ON THE HORIZON

FUTURE DIRECTIONS FOR THE RARE DISEASE COMMUNITY

Research and development of treatments for rare diseases continues to accelerate at an unprecedented pace. An area of particular focus for the rare disease community is gene therapy as many of these therapies are being developed for rare diseases. As of November 21, 2024, the FDA has approved 32 gene therapies and an additional 1,013 are being developed for rare diseases. While this exponential growth in available treatments offers the state of Minnesota with opportunities to improve care for individuals with rare diseases, it also presents a number of challenges to existing processes and healthcare structures. It will be critical for the state of Minnesota to proactively find solutions to structural challenges to ensure that available treatments are accessible to every patient that needs them. The Minnesota Rare Disease Advisory Council will continue to be a resource for state agencies and policy makers and a convener of stakeholders to ensure that the state of Minnesota is the best place to live for individuals living with a rare disease.

POLICY FOCUS IN 2025

MNRDAC is committed to identifying policies through a deliberative and transparent process that takes into account community input and is centered on individuals with rare diseases. Throughout 2024 MNRDAC gathered community perspectives and identified the following policy priorities for 2025:

TOPIC	LEVEL OF ENGAGEMENT	INPUT
Base budget establishment	Lead	Previous MNRDAC Legislation
Drug Formulary Committee modifications	Lead	Previous MNRDAC Legislation, PAG feedback
Requirement that patient assistance programs be permitted to go toward patient cost sharing (copays and deductibles)	Lead	PAG priority
Medicaid eligibility based on newborn screening	Lead	Other stakeholder (medical community) priority
Access to FDA approved gene therapies	Support	Other stakeholder, PAG priority
Improved reimbursement for medical foods	Support	PAG priority
Smart 911 expansion	Support	PAG and other stakeholder priority

Click [here](#) for MNRDAC progress on these topics.

PAG = Patient Advocacy Group

The Minnesota Rare Disease Advisory Council extends its deepest gratitude to the Minnesota Council on Disability (MCD) for its invaluable strategic and operational support. MCD's dedication and willingness to go above and beyond are truly appreciated.

CONTACT US

The Council seeks to be a policy resource and assist policy makers as they identify solutions for improved care for rare diseases. Please reach out to us with any needs at info@mnraredisease.org



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