

# Healthcare access in rare disease: A PUBLIC HEALTH PRIORITY!

There are over 25 million patients affected by more than 10,000 rare diseases, yet healthcare access and quality of life are elusive to many rare disease patients.

The *2020 Rare Diseases Health Care Access Study* set out to learn more about rare disease patients' barriers to care, providing valuable quantitative information related to the rare disease community. In 2025, this study was further evaluated with a focus on qualitative information and published as: *"Lack" and "Finally": A Qualitative Analysis of Barriers and Facilitators in Rare Disease Healthcare*. Below summarizes both the qualitative and quantitative information from this study.



For more information on this study and the MNRDAC, please scan the QR code

## LOCAL CARE? ONLY FOR SOME

Participants living in outstate Minnesota were more likely to report traveling **over 60 miles** for rare disease care.

Rare disease care **60 MILES**  
Chronic disease care **2 MILES**



*"Unable to obtain quality specialized care in home area, must travel to specialty centers 4 hours away."*

## INVESTIGATIONAL DRUG ACCESS

**72%**  
MN adult  
respondents



**79%**  
MN pediatric  
respondents

reported delays or denials for use of an investigational treatment.

*"The insurance company will not approve any medication to treat my condition because there is no FDA-approved medication for Idiopathic Hypersomnia."*

## MULTIPLE PROVIDER APPOINTMENTS



37% of patients saw  
**4-10 PROVIDERS**  
prior to receiving a diagnosis.

*"Doctors kept telling me it was all in my head until I finally got a diagnosis years later."*

## DELAYED TIME TO DIAGNOSIS



**56%**

of rare disease patients waited over **one year and up to ten years** to receive a diagnosis.

*"Waiting years for a diagnosis left me feeling hopeless and unheard."*

## OUT OF NETWORK ACCESS

IN



**27%**  
pediatric

**19%**  
adult

Respondents reported being **initially denied** seeing a specialist due to that specialist being out of network.

OUT



*"My child's medication is \$500,000 per year, and losing my insurance plan keeps me up at night."*

## TRANSITION OF CARE



**44%**

of participants reported **problems** with transition from pediatric to adult care.

*"The shift from pediatric to adult care was overwhelming. I lost access to coordinated care, and my new doctors didn't communicate with my previous specialists."*