



DRAFT



**NVRDAC**

Rare Disease Advisory Council

# **EMPOWERING**

Nevadans Living with a

Rare Disease to

Improve Their Future...



# Table of Contents

To Be Determined  
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# Vision For The Future

Our primary objective is to ensure that the voices of the rare disease patient and the providers who care for them have their voices heard. My name is Annette Logan- Parker, I am the Founder & CEO of Cure for The Kids Foundation. I am honored to hold the position of Chair for the Nevada Rare Disease Advisory Council (NV-RDAC). I am committed to advancing the objectives of the council and fulfilling the duties assigned to us through SB-315 .

We are pleased to share the 2023-2024 Nevada Rare Disease Advisory Council's (NV-RDAC) official (and first ever) strategic plan. We believe that through intentional planning and focused efforts our advisory council can provide a positive impact for the people of Nevada living with a rare disease, as well as for those healthcare professionals working hard to care for them. The Nevada RDAC plans to give rare disease patients and providers a much-needed voice, and my goal is to make it easier for them to articulate their needs and concerns as they sit at the table with state officials.

For years Nevada, as well as many other states, have failed to adequately address rare disease issues. Primarily due to a basic lack of awareness of the patients' and the providers' challenges. The Nevada Rare Disease Advisory Council plans to change that. Rare diseases should be common knowledge.

Nevada's government officials and lawmakers have several powerful policy levers at their disposal. Their ability to create health policies, regulate insurance implementation, and oversee public health gives them the power to transform the lives of rare disease patients. By using these various means strategically and in concert; in complete collaboration with our state government officials, the NV- RDAC plans to advance a more holistic approach toward improving the health and well-being of the rare disease community. The Nevada Rare Disease Advisory Council holds a significant promise for solving some of the many the problems faced by thousands of Nevadans, and we will not rest until rare diseases get the attention they deserve.

This strategic plan is designed to serve as the initial framework for creating a collaborative approach to the establishment of a comprehensive plan for the management of rare disease in Nevada. Collaboration and support from numerous government, academic, public health, community-based and other private sector entities will be essential to its success.

Together we can make rare diseases a little less rare.

Annette Logan- Parker



To Learn More: Please join us for our regularly scheduled meetings. \*Meeting schedule- we meet 6 times a year - every other month on the First Friday of the month at 9:30 AM



Rare Disease  
Advisory Council  
- 2023 Meeting Schedule ([nv.gov](http://nv.gov))

**2023**

February  
April  
June  
August  
October  
December

**2024**

February  
April  
June  
August  
October  
December

\*For a list of current RDAC members, please see [www.NVRDAC.org](http://www.NVRDAC.org).





The NVRDAC's mission is to improve the quality of life and support care for Nevadans affected by rare diseases through collaboration, education, support, and advocacy. Our council seeks to advocate for Nevadans impacted by rare diseases as well as identify the overall impact these diseases have in our community.

# Introduction to Rare Diseases

In the United States, the Food and Drug Administration (FDA) defines a rare disease as any disease that affects fewer than 200,000 Americans. Our European Colleagues consider a disease rare when it affects less than 1 in 2,000 people. Regardless of the contrastingly different attitudes towards what constitutes a rare disease; these numbers at first glance may seem insignificant. However, considering that there are more than 7,000 known rare diseases which affect approximately 25 million people. This is roughly translated to about 10% of the U.S. population.

Rare diseases are far more common than the term "rare disease" implies. Rare diseases are a significant contributor of chronic illness, disability, and premature death in both children and adults. They take a disproportionate share of our health care dollars and generate an enormous administrative burden on the healthcare industry as a whole because of their complexity and the expense of, in most cases, inadequate medical interventions. A Rare Disease Advisory Council (RDAC) is an advisory body providing a platform for the rare disease community to have a stronger voice in state government. RDACs address the needs of rare patients and families by giving stakeholders an opportunity to make recommendations to state leaders on critical issues including the need to increased awareness, diagnostic tools and access to affordable treatments and cures.

In 2015, the first state advisory council on rare diseases was created in North Carolina. Since the inception of the first RDAC, several states have established their own RDACs, and the national trend is taking hold with rare disease stakeholders across the country diligently working to establish a mechanism to allow improved representation of the this historically underserved and often misunderstood population of people.



**The symbol for rare disease awareness is a black and white striped "Zebra" ribbon.**

**Rare disease advocates worldwide use a zebra stripe ribbon because of the well-known medical expression.**

The formation of the Nevada Rare Disease Advisory Council (“the Council”) was formed under SB 315 during the 2019 session of the Nevada Legislature. The council was assigned the following thirteen specific duties:

1. Perform a statistical and qualitative examination of the incidence, causes and economic burden of rare diseases in Nevada.
2. Receive and consider reports and testimony concerning rare diseases from persons, the Division, community-based organizations, providers of health care and other local and national organizations whose work relates to rare diseases.
3. Increase awareness of the burden caused by rare diseases in Nevada.
4. Identify evidence-based strategies to prevent and control rare diseases.
5. Determine the effect of delayed or inappropriate treatment on the quality of life for patients suffering from rare diseases and the economy of Nevada.
6. Study the effect of early treatment for rare disease on the quality of life for patients suffering from rare diseases, the provision of services to such patients and reimbursement for such services.
7. Increase awareness among providers of health care of the symptoms of and care for patients with rare diseases.
8. Evaluate the systems for delivery of treatment for rare diseases in place in Nevada and develop recommendations to increase the survival rates and quality of life of patients with rare diseases.
9. Determine effective methods of collecting data concerning case of rare diseases in Nevada for the purpose of conducting epidemiological studies of rare diseases.
10. Establish a comprehensive plan for the management of rare diseases in Nevada, which must include recommendations for the state and local health authorities, public and private organizations, businesses and potential sources of funding, and update the comprehensive plan as necessary.
11. Develop a registry of rare diseases diagnosed in Nevada to determine the genetic and environmental factors that contribute to such rare diseases; and
12. Compile an annual report that includes a summary of the council’s activities and any recommendations of the council for legislation or other policies.
13. A specialized license plate to support the Rare Disease Advisory Council and research and treatment for childhood cancer can be purchased through the Nevada DMV Specialized License Plates website.

**The expression comes from an old saying used in teaching medical students about how to think logically regarding the process of differentiating between two or more conditions that share similar signs or symptoms. When you hear hoof-beats, think of horses, not zebras.**

**In the case of rare diseases- it is zebra we are looking for and they are often extremely hard to identify in a world of horses. The Zebra print ribbon seems to represent the difficulty people with rare diseases often face when seeking a diagnosis.**

# Our History

The NV-RDAC was formed under SB 315 during the 2019 session of the Nevada Legislature. The council was assigned several specific duties that range from performing a statistical and qualitative examination of the incidence, causes, and economic burden of rare disease in Nevada; including the development of a registry of rare diseases diagnosed in Nevada to determine the genetic and environmental factors that contribute to such rare diseases. As well as responsibility of increasing awareness of the burden caused by rare disease; including improving awareness among health care providers of the symptoms and care required for patients with rare diseases. As well as many other extremely lofty assignments. Unfortunately, the state of Nevada did not provision their RDAC with the required resources to be effective in their efforts: apart from the Nevada DMV Specialized License Plate for childhood cancer initiatives – which has failed to raise a meaningful amount of resources to provide any level of significant financial assistance to the RDAC's assignments.



<https://dmvnev.com/platescharitable.htm>.

Although the RDAC enjoys extremely engaged and driven council members, as well as an immensely supportive team of NV- DHHS employees assigned to assist the RDAC; for the most part we have been unsuccessful at accomplishing the any of duties assigned to the council under SB 315. The RDAC's less than desirable outcomes are a direct result of limited resources coupled with the complexities of a volunteer start-up council and the absence of a well-defined strategy.





Even though a significant portion of the population has a rare disease, state policymakers typically lack in-depth knowledge about the rare disease community as a whole and the issues relevant to this community. This incomplete understanding contributes to common obstacles that rare disease patients face, such as delays in diagnosis, misdiagnosis, lack of treatment options, high out-of-pocket costs, and limited access to medical specialists.

In Nevada, SB315 (Senate Bill- 315) established the Nevada Rare Disease Advisory Council (NRS 439.5075 – 439.5077) in the 2019 Legislative Session:

Nevada was the 10th state to enact a Rare Disease Advisory Council- As of July 2022 there are now 24 states with established RDACs and 9 more with pending legislation.

- The NV RDAC was established to help bridge policymakers' gaps in knowledge, and we serve as an advising body and liaison between the rare disease community and state government. The council helps inform, evaluate, and offer recommendations on policies and issues relevant to the rare disease community.
- There are statutory requirements for RDAC members to ensure there is a balanced representation of interested parties

For example:

- Physicians and nurses who care for patients with rare diseases and Hospital administrators that provide services to people with rare disease, an employee of the Division of the Nevada Department of Health and Human Services
- Patients who have rare diseases and parents of children with rare diseases.
- Administrators of organizations from both northern and southern Nevada who provide services to patients suffering from rare diseases.

# NV-RDAC to Date:

The current Chair of the Nevada Rare Disease Council, Annette Logan-Parker, requested that RDAC council members participate in an anonymous survey to help her understand the primary objectives of the individuals who volunteer their time to the efforts of the RDAC, as well as to gain perspective of where the council members feel our limited resources should be used.

The information gathered through this exercise will ensure that the collective efforts of the NVRDAC are productive in action, as well as rewarding in experience. The secondary objective of the survey is to provide the new Chair with the information required to understand how to best lead the NV-RDAC through her term as Chair. The survey was conducted in an anonymous format, and orchestrated by Ashlyn Torres, Health Program Specialist I, from the Nevada Department of Health and Human Services, this document summarizes results of these efforts.

This strategic plan is based on these particular survey findings of the RDAC council.

The Nevada Rare Disease Advisory Council has been tasked with a few important deliverables, a few of them are as follows:

1. The development of a statewide registry of rare diseases diagnosed in Nevada to help the state better understand the prevalence of rare diseases as well as the burden of rare disease affecting the people of Nevada.
2. Evaluate the systems for delivery of treatment for rare diseases in place in Nevada and develop recommendations to increase the survival rates and quality of life of patients with rare diseases.
3. Establish a comprehensive plan for the management of rare diseases in Nevada, which must include recommendations for the state and local health authorities, public and private organizations, businesses, and potential sources of funding.

Since the inception of the Nevada RDAC- the council has been focusing on three primary categories of rare disease:

1. All Childhood Cancers
2. Inherited bleeding and clotting disorders- like hemophilia and Von Willebrand Disease
3. All rare diseases screened for through the state's newborn screening program- there are currently 46 conditions that all newborns are being screened for in Nevada- to name a few:
  - Sickle Cell Disease, Cystic Fibrosis, Primary Immune Disorders, and Metabolic Disorders,

The newborn screening process involves:

1. Checking (screening) babies at birth for certain conditions
2. Finding those few who might have one of the conditions, and
3. Helping to connect these babies with early care, treatment and/or the intervention they need to give them the best possible chance at a healthy life.
  - Many babies are born with rare diseases yet have no known family history of a rare diseases or have any known family members with symptoms of a rare disease- newborn screening is often the only way to detect these conditions before symptoms begin.

The NVRDAC's mission is to improve the quality of life and support care for Nevadans affected by rare diseases through collaboration, education, support, and advocacy. Our council seeks to advocate for Nevadans impacted by rare diseases as well as identify the overall impact these diseases have in our community.

We are excited to announce that the Nevada RDAC will rolling out a statewide Rare Disease Needs Assessment in the summer of 2023.

The RDAC is planning a statewide Rare Disease Needs Assessment – we will be seeking survey participants to help identify the extent of rare disease(s) in Nevada. These survey results will be used to improve quality of care and to advise state agencies on research, diagnosis, treatment, and educational needs relating to rare diseases.

The purpose of the Needs Assessment is to better understand the experiences of individuals, family members and caregivers of people with rare disease(s), especially with respects to accessing care and difficulties with navigating the healthcare system in Nevada.

All information obtained through the needs assessment will be anonymous and will remain confidential. No questions will require identifying information that will lead back to a participant. Survey findings will only be reported in the aggregate and be used to guide policies and services related to rare diseases in Nevada.

**Rare disease advocates worldwide use a zebra stripe ribbon because of the well-known medical expression.**

**The expression comes from an old saying used in teaching medical students about how to think logically regarding the process of differentiating between two or more conditions that share similar signs or symptoms.**

**'When you hear hoof-beats, think of horses, not zebras.'**

**For example, when someone develops a mild transient cough, a virus infection is the most logical and likely cause, and tuberculosis is a zebra.**

**In the case of rare diseases- it is zebra we are looking for and they are often extremely hard to identify in a world of horses. The Zebra print ribbon seems to represent the difficulty people with rare diseases often face when seeking a diagnosis.**

# Summary

The survey confirms that the members of the RDAC are not only engaged and willing to serve the state Nevada on behalf of people with rare disease, but they are also equally aligned in their personal and collective agendas to improve the overall landscape of rare disease management in Nevada; including access to high quality medical care, improving the knowledge base of the states healthcare providers, influencing positive change with payers who provide coverage for people with rare diseases, and educating the state's law makers to create new and improved regulations that positively impact people with rare disease(s) and the families who love them.

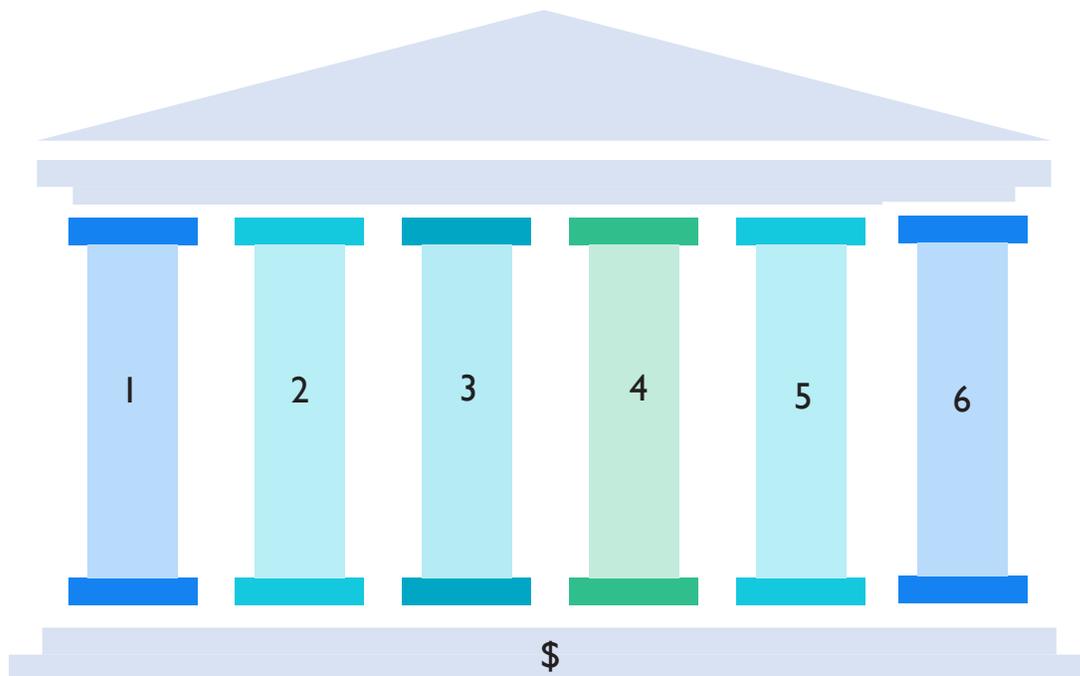
To establish a strategic action plan for the NV RDAC the survey listed the 13 duties assigned to the council by SB 315 and placed them into 5 groups and as a council we ranked them in order of priority based on our current capabilities. The survey has determined that the top priorities should be as follows, in the order of importance:

- 1) Nevada's Specific Data - Collection & Analysis of data
- 2) NV-RDAC Awareness & Education Campaigns
- 3) The Establishment of a Comprehensive Rare Disease Plan for Nevada
- 4) Health Equity and Disparities
- 5) Understanding RDACs
- 6) Understanding Nevada-Specific Burdens aka Needs Assessment



# Nevada RDAC Pillars of Success

- 1) Nevada's Specific Data - Collection & Analysis of data
- 2) NV-RDAC Awareness & Education Campaigns
- 3) The Establishment of a Comprehensive Rare Disease Plan for Nevada
- 4) Health Equity and Disparities
- 5) Understanding RDACs
- 6) Understanding Nevada-Specific Burdens aka Needs Assessment





# Taking The Plan Forw

Nevada Rare Disease Advisory Council Strategic Plan 2023– priorities moving into the next two years. We will continue faced with new challenges and opportunitie. This plan is n priorities, strategies, and objectives that allow us to collecti recognized, effective Rare Disease Advisory Council.

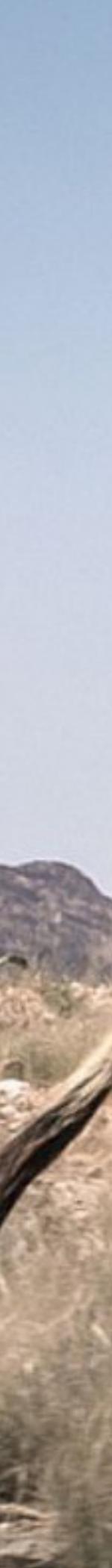
The next step is to determine subcommittess for the follow

- 1) Strategic Plan
- 2) Needs Assessment Sub-committee



# Our Strategies to Success

Next steps in strategy to be determined by the subcommittees.



# Our Approach to Nevada's Specific Data Collection & Analysis of Data

## AIM:

To Be Determined by the  
Sub-committee





## Objective A:

To Be Determined

## Objective B:

To Be Determined

## Objective C:

To Be Determined

# Our Approach to NV-RDAC Awareness & Education Campaigns:

## AIM:

To Be Determined by the  
Sub-committee

## While



## Mientras esperas...



*¿Eres tú o un ser querido?  
vivir con una enfermedad rara?*

***¡Queremos que tu voz sea escuchada!***

**TOMA EL CONFIDENCIAL  
NEVADA RAROENCUESTA DE  
ENFERMEDADES AHORA**

10 minutos de su tiempo ayudarán al  
Aviso de Enfermedades Raras de  
NevadaEl Consejo obtenga una mejor  
comprensión de las necesidades  
y cargas de Nevadenses que viven  
con una enfermedad rara.

**¡Es fácil!**

Simplemente escanee el código QR con  
un dispositivo móvil o visite [nvr dac.org/  
needs-assessment/](http://nvr dac.org/needs-assessment/)



Objective A:

To Be Determined

Objective B:

To Be Determined

Objective C:

To Be Determined

# Our Approach to The Establishment of a Comprehensive Rare Disease Plan for Nevada

## AIM:

To Be Determined by the  
Sub-committee





## Objective A:

To Be Determined

## Objective B:

To Be Determined

# Our Approach to Health Equity & Disparities

## AIM:

To Be Determined by the  
Sub-committee



## Objective A:

To Be Determined

## Objective B:

To Be Determined



## Objective C:

To be Determined

# Our Approach to Understanding RDACs

**AIM:**

To Be Determined by the  
Sub-committee





## Objective A:

To Be Determined

## Objective B:

To Be Determined

# Our Approach to Understanding Nevada-Specific Burdens aka Needs Assessment

## AIM:

To Be Determined by the  
Sub-committee



Objective A:

To Be Determined

Objective B:

To Be Determined



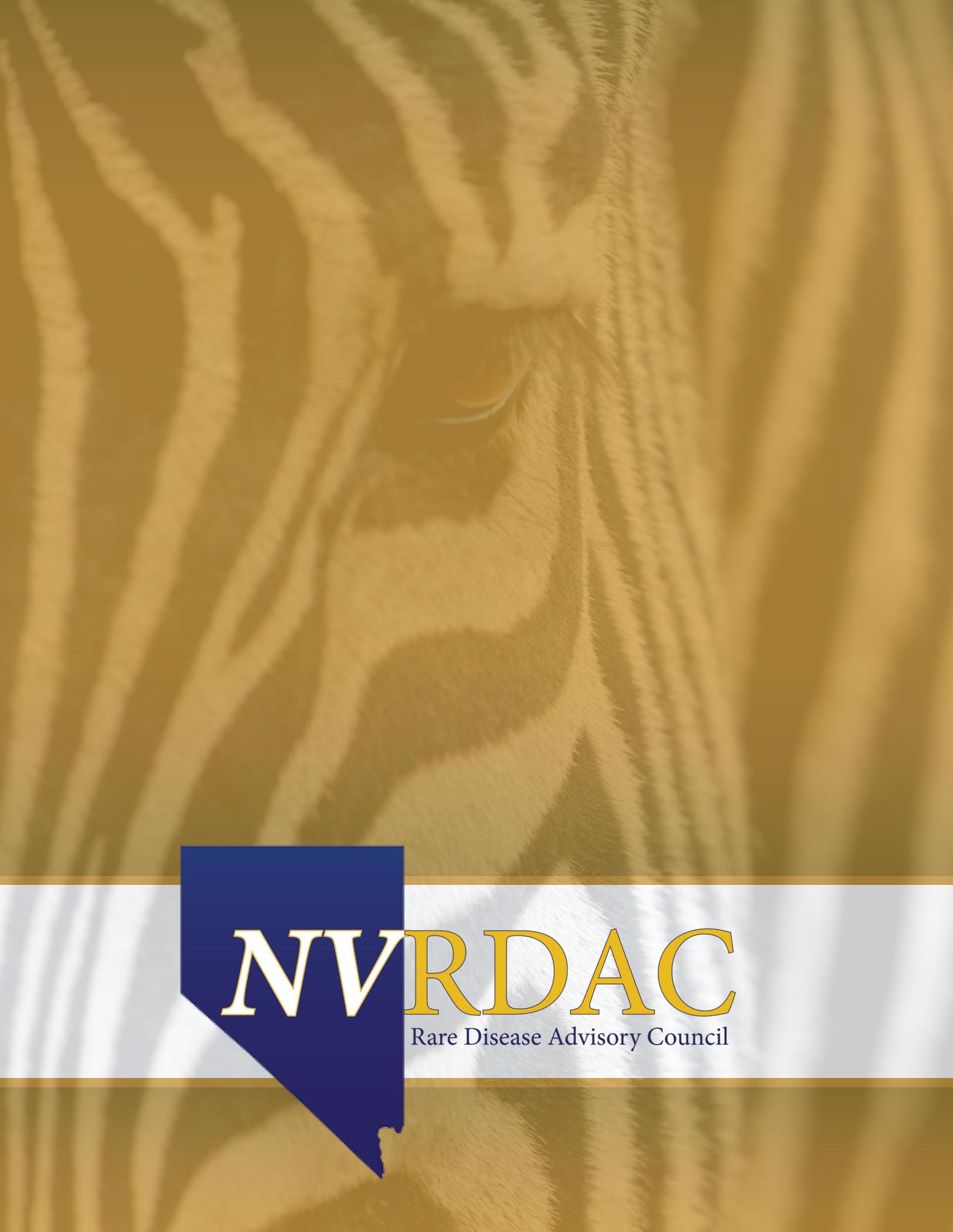
Objective C:

To Be Determined

# Rare Disease Statistics

- In the United States, a disease is considered “rare” if it affects fewer than 1 in 200,000 people.
- It is estimated that 25-30 million Americans have a rare disease- that is almost 1 in 10 people- and represents 400 million people worldwide.
- 50% of those affected by rare disease are children and 30% of children with a rare disease will not live to the age of 5.
- Rare diseases impact more people than cancer and AIDS combined.
- There are approximately 7,000 known rare disease.
- There are more than 500 types of rare cancers.
- All pediatric cancers are rare.
- Most rare diseases are genetic or have a genetic component.
- More than 90% of rare diseases do not have any FDA-approved treatment or associated research.





**NV**RDAC

Rare Disease Advisory Council