NEVADA RARE DISEASE ADVISORY COUNCIL (RDAC)

Welcome Packet

Nevada Department of Health and Human Services
Nevada Division Public and Behavioral Health

Table of Contents

Dear New NV-RDAC Council Member,

Welcome to the Nevada Rare Disease Advisory Council (NV RDAC)! On behalf of the entire council, I am thrilled to welcome you to our community of passionate advocates, professionals, and leaders dedicated to transforming the landscape of rare disease care and support in Nevada.

Your commitment to advancing rare disease awareness, advocacy, and healthcare aligns with the very heart of our mission. Together, we are building a future where every individual with a rare disease has access to comprehensive, compassionate, and high-quality care. Your unique expertise and insights will play an essential role in driving this vision forward, and I am confident that your contributions will make a lasting impact on our work.

As a council member, you'll join us in developing and implementing initiatives that span public health, education, research, and advocacy. From shaping public policy to supporting Nevada's families and healthcare providers, our collective efforts are making meaningful changes in the lives of those affected by rare diseases.

To help you get started, we've provided a welcome packet with valuable resources, including information on the council's strategic priorities, meeting schedule, and key contacts. Our team is here to support you in every way possible, so please don't hesitate to reach out with any questions or if there's anything you need.

Thank you for joining us in this vital work. I look forward to meeting you in person and to the valuable contributions you'll bring to NV RDAC. Together, we will continue to be a powerful voice and force for positive change, ensuring that Nevada remains at the forefront of rare disease advocacy and care.

Warm regards,
Annette Logan-Parker
Chair, Nevada Rare Disease Advisory Council

Rare Disease Advisory Council (RDAC) Meeting Schedule

RDAC (Main Council) Meeting Schedule:

Per February 13, 2023, RDAC meeting, the Council approved to meet on the first Friday of the month every 2 months effective since April 7, 2023, at 9:30 AM PST (on even numbered months) with a 2-hour block as a placeholder, and the duration of the meetings depends on the volume of discussion and public comment. Below is every month that the Council meets.

February, first Friday of the month @ 9:30AM PST

April, first Friday of the month @ 9:30AM PST

June, first Friday of the month @ 9:30AM PST

August, first Friday of the month @ 9:30AM PST

October, first Friday of the month @ 9:30AM PST

December, first Friday of the month @ 9:30AM PST

RDAC Subcommittee Meeting Schedule:

Per the August 4, 2023, Rare Disease Advisory Council meeting, the Strategic Plan Subcommittee was created to meet on the first Friday of the month every 2 months, when the RDAC Board is not meeting beginning on Friday, November 3rd at 9:30 AM PST. Any members of the main Council are welcome to attend the Subcommittee meetings as members of the public. These attendees would not be considered members of the Subcommittee and thus would not be able to take action on the agenda. These attendees are welcome to participate via public comment and other avenues available to the members of public attending meetings subject to Nevada Open Meeting Law. The 2-hour block is a placeholder, and the duration of the meetings depends on the volume of discussion and public comment. Below is every month that the Subcommittee meets.

January, first Friday of the month @ 9:30AM PST

March, first Friday of the month @ 9:30AM PST

May, first Friday of the month @ 9:30AM PST

July, first Friday of the month @ 9:30AM PST

September, first Friday of the month @ 9:30AM PST

November, first Friday of the month @ 9:30AM PST

Attendance expectations/requirements:

In accordance with RDAC bylaws, *Article IV: Membership, Section 5 Removal of a Member, Subsection 3: unexcused absence from two consecutive meetings of the Council.* As stated above RDAC (Main Council) and RDAC Subcommittee approved to meet every two months on the first Friday of the month. We understand that conflicts can arise, and if unable to attend, the Council requests that members please let Chair Logan-Parker (alogan@cure4thekids.org) and RDAC Support Staff, Ashlyn Torrez (atorrez@health.nv.gov) know prior to the scheduled meeting date if unable to attend via email to be counted as an excused absence.

Informational Websites

- **Nevada Revised Statues: Chapter 439 – Administration of Public Health, Section 5075 Rare Disease Advisory Council & Section 5077 Rare Disease Advisory Council Duties**
    - https://www.leg.state.nv.us/nrs/nrs-439.html#NRS439Sec5075
    - https://www.leg.state.nv.us/nrs/nrs-439.html#NRS439Sec5077
- **Open Meeting Law Portal**
    - https://ag.nv.gov/About/Governmental_Affairs/OML/
- **Division of Public and Behavioral Health (DPBH) Home webpage**
    - https://dpbh.nv.gov/
- **Division of Public and Behavioral Health – Rare Disease Advisory Council**
    - https://dpbh.nv.gov/Boards/RDAC/RDAC/
- **Nevada Rare Disease Advisory Council Website (ran by Chair Logan-Parker's Team)**
    - https://nvrdac.org/
- **Nevada DPBH – Office of State Epidemiology (OSE)**
    - https://nvose.org/
- **Nevada DPBH OSE – Rare Diseases Registries Program**
    - https://nvose.org/programs/rare-disease/
- **National Organization for Rare Disorders (NORD)**
    - https://rarediseases.org/

RARE DISEASE ADVISORY COUNCIL

NRS 439.5075 Creation; membership; vacancies; compensation; Chair; meetings.

1. The Rare Disease Advisory Council is hereby created within the Department of Health and Human Services. The Council consists of:

(a) The Chair of the State of Nevada Advisory Council on Palliative Care and Quality of Life created by NRS 232.4855 or his or her designee; and

(b) The following members appointed by the Director:

(1) Not more than three physicians who practice in the area of cardiology, emergency care, neurology, oncology, orthopedics, pediatrics or primary care and provide care to patients with rare diseases;

(2) Two registered nurses who provide care to patients with rare diseases;

(3) Not more than two administrators of hospitals that provide care to patients with rare diseases or their designees;

(4) One representative of the Division who provides education concerning rare diseases or the management of chronic conditions;

(5) The employee of the Division who is responsible for epidemiology services;

(6) Two persons over 18 years of age who have suffered from or currently suffer from a rare disease;

(7) Two parents or guardians who each have experience caring for a child with a rare disease;

(8) One representative of an organization dedicated to providing services to patients suffering from rare diseases in northern Nevada; and

(9) One representative of an organization dedicated to providing services to patients suffering from rare diseases in southern Nevada.

2. The Council may, by affirmative vote of a majority of its members, request the Director to appoint to the Council additional members who have expertise on issues studied by the Council. Such members serve for a period determined by the Council.

3. A vacancy in the membership of the Council must be filled in the same manner as the initial appointment.

4. The members of the Council serve without compensation and are not entitled to the per diem and travel expenses provided for state officers and employees generally.

5. Each member of the Council who is an officer or employee of this State or a political subdivision of this State must be relieved from his or her duties without loss of regular compensation so that the officer or employee may prepare for and attend meetings of the Council and perform any work necessary to carry out the duties of the Council in the most timely manner practicable. A state agency or political subdivision of this State shall not require an officer or employee who is a member of the Council to make up the time the officer or employee is absent from work to carry out duties as a member of the Council or use annual leave or compensatory time for the absence.

6. The Department shall provide such administrative support to the Council as is necessary to carry out the duties of the Council.

7. The Council shall:

(a) Elect a Chair from among its members; and

(b) Meet at least once every 3 months at the times and places specified by a call of the Chair and may meet at such further times as deemed necessary by the Chair.

(Added to NRS by 2019, 1476)

NRS 439.5077 Duties.

1. The Rare Disease Advisory Council created by NRS 439.5075 shall:

(a) Perform a statistical and qualitative examination of the incidence, causes and economic burden of rare diseases in this State;

(b) 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;

(c) Increase awareness of the burden caused by rare diseases in this State;

(d) Identify evidence-based strategies to prevent and control rare diseases;

(e) Determine the effect of delayed or inappropriate treatment on the quality of life for patients suffering from rare diseases and the economy of this State;

(f) Study the effect of early treatment for rare diseases on the quality of life for patients suffering from rare diseases, the provision of services to such patients and reimbursement for such services;

(g) Increase awareness among providers of health care of the symptoms of and care for patients with rare diseases;

(h) Evaluate the systems for delivery of treatment for rare diseases in place in this State and develop recommendations to increase the survival rates and quality of life of patients with rare diseases;

(i) Determine effective methods of collecting data concerning cases of rare diseases in this State for the purpose of conducting epidemiological studies of rare diseases in this State;

(j) Establish a comprehensive plan for the management of rare diseases in this State, which must include, without limitation, recommendations for the Department, the Division, local health districts, public and private organizations, businesses and potential sources of funding, and update the comprehensive plan as necessary; and

(k) Develop a registry of rare diseases diagnosed in this State to determine the genetic and environmental factors that contribute to such rare diseases.

2. The Council shall compile an annual report which must include, without limitation, a summary of the activities of the Council and any recommendations of the Council for legislation or other policies. The Council shall:

(a) Post the report on an Internet website maintained by the Department; and

(b) Submit the report to the Department, the Governor and the Director of the Legislative Counsel Bureau for transmittal to:

(1) In even-numbered years, the next regular session of the Legislature; and

(2) In odd-numbered years, the Joint Interim Standing Committee on Health and Human Services.

3. As used in this section, "provider of health care" has the meaning ascribed to it in NRS 629.031.

(Added to NRS by 2019, 1477)

THE RARE DISEASE ADVISORY COUNCIL BYLAWS
Approved by Council 06 November 2020

ARTICLE 1: NAME AND DEFINITIONS

1.1 The name of this group shall be the Rare Disease Advisory Council, hereinafter referred to as the "Council".

1.2 The term "Department" refers to the Department of Health and Human Services.

1.3 The term "Director" refers to the Director of the Department of Health and Human Services.

1.4 The term "Division" refers to the State of Nevada Division of Public and Behavioral Health.

1.5 The term "NRS" refers to the Nevada Revised Statutes.

ARTICLE 2: AUTHORITY

2.1 The Council is authorized under NRS 439.5075 – NRS 439.5077.

2.2 The Council is considered advisory in nature and makes recommendations to the Nevada Division of Public and Behavioral Health and the Nevada Department of Health and Human Services

ARTICLE 3: PURPOSE AND FUNCTION

3.1 In accordance with NRS 439.5077, the Council shall carry out the provisions, inclusive, of this act:

3.1.1 Perform a statistical and qualitative examination of the incidence, causes and economic burden of rare diseases in this State;

3.1.2 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;

3.1.3 Increase awareness of the burden caused by rare diseases in this State;

3.1.4 Identify evidence-based strategies to prevent and control rare diseases;

3.1.5 Determine the effect of delayed or inappropriate treatment on the quality of life for patients suffering from rare diseases and the economy of this State;

3.1.6 Study the effect of early treatment for rare diseases on the quality of life for patients suffering from rare diseases, the provision of services to such patients and reimbursement for such services;

3.1.7 Increase awareness among providers of health care of the symptoms of and care for patients with rare diseases;
3.1.8 Evaluate the systems for delivery of treatment for rare diseases in place in this State and develop recommendations to increase the survival rates and quality of life of patients with rare diseases;

3.1.9 Determine effective methods of collecting data concerning cases of rare diseases in this State for the purpose of conducting epidemiological studies of rare diseases in this State;

3.1.10 Establish a comprehensive plan for the management of rare diseases in this State, which must include, without limitation, recommendations for the Department, the Division, local health districts, public and private organizations, businesses and potential sources of funding, and update the comprehensive plan as necessary; and

3.1.11 Develop a registry of rare diseases diagnosed in this State to determine the genetic and environmental factors that contribute to such rare diseases.

3.2 The Council shall compile an annual report which must include, without limitation, a summary of the activities of the Council and any recommendations of the Council for legislation or other policies. The Council shall:

3.2.1 Post the report on an Internet website maintained by the Department; And

3.2.2 Submit the report to the Department, the Governor and the Director of the Legislative Counsel Bureau for transmittal to:
(a) In even-numbered years, the next regular session of the Legislature; and
(b) In odd-numbered years, the Legislative Committee on Health Care.

ARTICLE 4: MEMBERSHIP

4.1 According to NRS 439.5075, the Council shall consist of:

4.1.1 The Chair of the State of Nevada Advisory Council on Palliative Care and Quality of Life created by NRS 232.4855 or his or her designee; and

4.1.2 The following members appointed by the Director:

(a) Not more than three physicians who practice in the area of cardiology, emergency care, neurology, oncology, orthopedics, pediatrics or primary care and provide care to patients with rare diseases;
(b) Two registered nurses who provide care to patients with rare diseases;
(c) Not more than two administrators of hospitals that provide care to patients with rare diseases or their designees;
(d) One representative of the Division who provides education concerning rare diseases or the management of chronic conditions;
(e) The employee of the Division who is responsible for epidemiology services;
(f) Two persons over 18 years of age who have suffered from or currently suffer from a rare disease;
(g) Two parents or guardians who each have experience caring for a child with a rare disease;
(h) One representative of an organization dedicated to providing services to patients suffering from rare diseases in northern Nevada; and
(i) One representative of an organization dedicated to providing services to patients suffering from rare diseases in southern Nevada.

4.2 The term for each member of the Council is three years from the appointment date and members shall serve at the pleasure of the Director.

4.3 The Council may, by affirmative vote of a majority of its members, request the Director to appoint to the Council additional members who have expertise on issues studied by the Council. Such members serve for a period determined by the Council.

4.4 A vacancy in the membership of the Council must be filled in the same manner as the initial appointment.

4.5 Removal of a Member:

4.5.1 A member of the Council may be removed by a majority vote of Council members, and with approval of the Director

4.5.2 Reasons include malfeasance in office or neglect of duty; and

4.5.3 Unexcused absence from two consecutive meetings of the Council.

ARTICLE 5: VOTING

5.1 A majority of the voting members of the Council, eight, constitutes a quorum to transact all business, and a majority of those voting members present, physically or via telecommunications, must concur in any decision.

5.2 Each appointed Council member shall have one vote.

5.3 A concurrence of at least a majority of the members of the Council shall be required on all questions.

5.4 Members may abstain.

5.5 The Chair will count and announce the results of any vote.

5.6 Proxy for a Member: Should the Chair of the State of Nevada Advisory Council on Palliative Care and Quality of Life be unable to attend a meeting, he/she may designate a representative who has knowledge of the Council's activities to attend in his/her place, who shall have all the rights and privileges of the member while acting in his/her behalf.

ARTICLE 6: OFFICERS/NOMINATIONS/ELECTIONS

6.1 Council officers shall be elected from among its members, and include a Chair as required by the NRS, and a Vice Chair per vote of the Council.

6.1.1 The Chair and Vice Chair shall be elected at the first meeting of even numbered State fiscal years by a majority vote of all Council members.

6.1.2 Other Council officers may include a Secretary, and Treasurer, and may include others as deemed necessary by a majority vote of the Council.

6.1.3 The Council's Chair shall open and close the floor for nominations.

6.1.4 The Chair, or their designee, shall call roll for the purposes of each member casting their vote and recording the votes.

6.1.5 In accordance with the Nevada Open Meeting Law all nominations and votes shall be provided orally.

6.1.6 Only members present during the meeting (includes teleconference attendance) may nominate and vote for officers.

6.2 Terms of office. The term of office of the Chair is two years.

6.2.1 The Chair may serve any number of consecutive terms.

6.3 Vacancies. When a vacancy occurs in the office of Chair, either by reason of his/her having left the Council or office; a new Chair is elected to fill out the unexpired term.

6.3.1 This shall not constitute a full term for the purposes of 6.2.

6.4 Duties

6.4.1 The Chair shall preside at all meetings of the Council.

6.4.2 If appointed, the Vice Chair shall act for and in behalf of the Chair in all cases of his/her absence.

ARTICLE 7: COMPENSATION (NRS 439.5075)

7.1 The members of the Council serve without compensation and are not entitled to the per diem and travel expenses provided for state officers and employees generally.

7.2 Each member of the Council who is an officer or employee of this State or a political subdivision of this State must be relieved from his or her duties without loss of regular compensation so that the officer or employee may prepare for and attend meetings of the Council and perform any work necessary to carry out the duties of the Council in the most timely manner practicable. A state agency or political subdivision of this State shall not require an officer or employee who is a member of the Council to make up the time the officer or employee is absent from work to carry out duties as a member of the Council or use annual leave or compensatory time for the absence.

ARTICLE 8: STAFFING/SUPPORT

8.1 The Department shall provide such administrative support to the Council as is necessary to carry out the duties of the Council.

8.2 Staff to the Council shall be provided by the Division for purposes of secretarial, research, and other needs.

.

ARTICLE 9: MEETINGS

9.1 The Council shall meet at least once every 3 months at the times and places specified by a call of the Chair and may meet at such further times as deemed necessary by the Chair.

9.2 Agenda items may be submitted in writing no later than 15 working days before the meeting by Division employees and/or Council members.

9.3 Meetings will generally follow parliamentary procedure as contained in Robert's Rules of Order insofar as they do not conflict with the Nevada Revised Statute and these bylaws.

9.4 Meetings shall be open to the public and conducted in accordance with NRS 241, known as Nevada's "Open Meeting Law".

ARTICLE 10: MINUTES

10.1 A member of the Council, or a support staff member designated by the Division, shall act as a recording secretary for the purpose of taking minutes at each meeting.

10.2 Minutes of the previous meeting will be transcribed and made available for inspection by the public within 30-working days after the adjournment of the meeting.

10.2.1 The recording secretary will transcribe minutes from tape recording, notes taken at meeting and from agenda exhibits.

10.2.2 After draft of minutes is prepared, it will be submitted to the Chair for approval.

10.2.3 After approval, minutes will be finalized and included in meeting materials for members.

10.2.4 Final approval of the minutes will be done by the Council at its next meeting.

10.3 Minutes of each meeting of the Council are considered public record and will be maintained in the Administrative Office of the Division.

10.4 Copies of the minutes of the previous meeting will be made available to Committee members prior to the next meeting.

ARTICLE 11: AMENDMENTS

11.1 The Bylaws may be amended or changed at any regular meeting by a majority of the voting members who are present at the meeting of the Council, if the proposed amendment or change was submitted, in writing, to members of the Council and the Chair, at least 14 days before the meeting.

NVRDAC

Rare Disease Advisory Council

ANNUAL REPORT 2023

www.nvrdac.org

Table of Contents

Community Awareness Efforts

Conclusions and Recommendations

A Letter from the Chair

In this comprehensive report, the Nevada Rare Disease Advisory Council (NV-RDAC) sheds light on critical issues affecting individuals living with rare diseases as well as many issues facing the broader healthcare landscape in Nevada.

Throughout the report, NV-RDAC outlines pressing concerns, including the underfunding of both Nevada's Medicaid Program and the Department of Health and Human Services (DHHS) data collection efforts, which significantly impact the rare disease community. Medicaid, originally designed as a safety net, has become a lifeline for rare disease individuals facing physical limitations and high healthcare costs. Nevada has consistently fallen behind in national data collection and reporting efforts in key areas of rare disease reporting. DHHS is severely under-resourced in this area, posing a considerable challenge to their efforts to enhance data quality and meet the necessary requirements for inclusion in national benchmarking.

Accurate and comprehensive data collection is crucial for understanding the prevalence, impact, and treatment outcomes of rare diseases in Nevada. Without sufficient funding for data collection efforts, the state risks being unable to adequately address the unique needs of individuals living with rare diseases. The lack of robust data can hinder policymakers and healthcare providers in making informed decisions and allocating resources effectively. Moreover, the absence of published data inhibits healthcare facilities in Nevada from being recognized for federal grants, further compounding the challenges faced by the rare disease community.

Nevada now has the opportunity to address these challenges through tailored funding strategies for Medicaid beneficiaries with rare diseases and DHHS data collection efforts. Adequate funding for rare diseases can alleviate the financial burdens associated with seeking qualified care, empower healthcare providers to do more, and improve data quality. By increasing Nevada's Medicaid budget and implementing distinct reimbursement rates for rare diseases, including high-cost drug reimbursement, the state can significantly improve care access and address the shortage of specialized physicians. Simultaneously, allocating resources to DHHS for enhanced data collection and analysis will provide essential insights into the rare disease landscape, supporting evidence-based policymaking and resource allocation.

The NV-RDAC requests your consideration of these recommendations, emphasizing the need for adequate funding and tailored reimbursement rates to support individuals living with rare diseases in Nevada. Additionally, we urge the allocation of resources to bolster DHHS data collection efforts, ensuring that we have the necessary information to make informed decisions, improve the lives of those affected by rare diseases in our state, and enhance healthcare facilities' eligibility for federal grants.

Sincerely,

Annette Logan-Parker

Annette Logan-Parker

On Behalf of the Council:

Annette Logan-Parker (Chair)
Gina Glass (Vice-Chair)
Veneta Lepera, BSN, RN
Dr. Nik Abdul Rashid, M.D.
Amber Federizo, DNP, APRN, FNP-BC
Dr. Susana Sorrentino, M.D.
FACMGG, FAAP
Valerie Porter, DNP, APRN, AG-ACNP-BC, MBA
Jennifer Millet, DNP, RN
Dr. Ihsan Azzam, M.D., PhD
Naja Bagner
Linetta Barnes, BSN, RN
Kimberly Palma-Ortega
Shirley Folkins-Roberts
Paul Niedermeyer
Dr. Craig Vincze, PhD
Christina Thielst, LFACHE, MHA
Dr. Sumit Gupta, M.D.

Special Acknowledgement:
The council would like to recognize the contributions of our support team from the Nevada Department of Health and Human Services:
Ashlyn Torrez, Pierron Tackes, Kayla Samuels, Lindsey Kinsinger

The council would like to recognize the contributions of our support team from Cure 4 The Kids Foundation:
Amber Williams, Mitch Koulouris, Jaromy Russo, Jamey Felsing

A Year of Remarkable Progress

The Nevada Rare Disease Advisory Council (NV-RDAC) has made significant strides over the past year, and we are proud to present our accomplishments in the 2023 Annual Report. This report reflects the deliberate planning and unwavering dedication of the council throughout the year. In 2023, NV-RDAC embarked on an ambitious mission to enhance its effectiveness and efficiency, not only to improve the rare disease landscape for affected individuals in Nevada but also to foster increased communication with state lawmakers to bring about lasting change for our state.

In a world where the challenges faced by rare disease patients and providers have often been overshadowed by a fundamental lack of awareness, NV-RDAC is steadfast in its commitment to bring these issues to the forefront. By bridging the gap between patients, providers, and state lawmakers, our council aims to transform rare diseases into common knowledge, ensuring they receive the attention and resources they so urgently require.

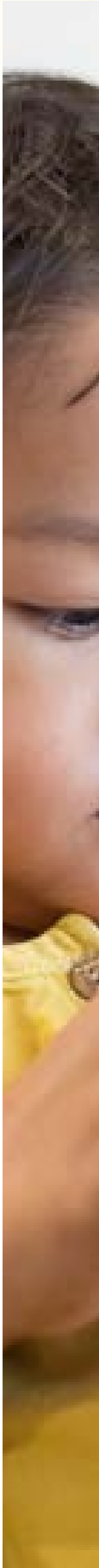
Nevada's government officials and lawmakers possess the tools to enact significant policy changes. Their expertise in health policy creation, insurance regulation, and public health oversight has the potential to revolutionize the rare disease landscape. NV-RDAC is wholeheartedly dedicated to collaborating closely with these officials to effectively leverage these tools and advance a more inclusive approach to rare disease management in our state.

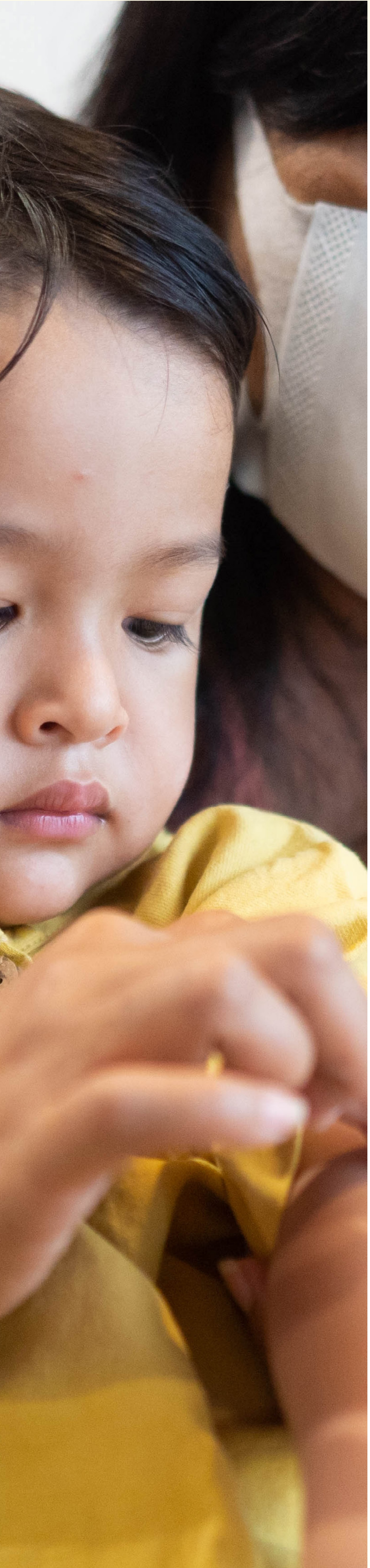
This 2023 Annual Report of NV-RDAC highlights a collaborative effort between the council and the state, marking the commencement of a unified journey toward comprehensive rare disease management in Nevada. The success of NV-RDAC's ambitious plans relies on collective support and collaboration from diverse sectors, including government, public health, and community-based organizations.

Our goal is to elevate the recognition of rare diseases as a pressing issue, ensuring that every voice is heard and that every challenge is met with empathy, understanding, and robust support. Together, we strive to make a difference and bring about meaningful change in the lives of those affected by rare diseases.

Mission

The Nevada Rare Disease Advisory Council'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

In the United States, a rare disease is defined by the Food and Drug Administration (FDA) as any ailment that affects fewer than 200,000 Americans. Conversely, our European counterparts classify a disease as rare when it afflicts less than 1 in 2,000 people. At first glance, these figures might appear inconsequential. However, it's essential to recognize that there are over 7,000 identified rare diseases impacting approximately 25 million individuals. This translates to roughly 10% of the U.S. population.

Rare diseases are far more prevalent than the term "rare disease" suggests. They constitute a significant source of chronic illness, disability, and premature mortality in both children and adults. Rare diseases consume a disproportionate share of our healthcare resources and place an immense administrative burden on the healthcare industry due to their intricacy and the substantial cost of often insufficient medical interventions.

A Rare Disease Advisory Council (RDAC) serves as an advisory body that provides a platform for the rare disease community to have a more influential voice in state government affairs. RDACs address the needs of patients and families grappling with rare diseases by affording stakeholders the opportunity to propose recommendations to state leaders on critical issues. These issues encompass the necessity for heightened awareness, improved diagnostic tools, and increased access to affordable treatments and cures.

The inaugural State Advisory Council on rare diseases was established in North Carolina in 2015. Since the inception of this pioneering RDAC, several states have instituted their own RDACs, contributing to a growing national trend. Rare disease advocates throughout the country are diligently working to establish mechanisms that enhance the representation of this historically underserved and often misunderstood population.

The Nevada Rare Disease Advisory Council (NV-RDAC) came into existence through NRS 439.5075 during the 2019 session of the Nevada Legislature. This legislative measure vested the council with a variety of distinct obligations, demonstrating its dedication to tackling the unique issues presented by rare diseases in Nevada. One of these responsibilities includes the yearly creation of a report that provides a concise summary of the council's actions, along with any suggested recommendations for legislative or policy improvements. This document marks the third consecutive annual report prepared by the NV-RDAC.

National Ranking In Rare Disease Support

Since 2015, the National Organization of Rare Disorders (NORD) has been engaged in its State Report Card project, aimed at assessing the effectiveness of states in meeting the requirements of their residents with rare diseases. While the report card evaluates states across eight specific and pertinent policy and access areas, it's crucial to emphasize that this grading criteria, although valuable, is not all-encompassing. Numerous significant factors affecting the lives of rare disease patients may exist, even if they are not reflected in this ranking.

Nevada's Current Report Card

Nevada's Report Card for Rare Disease Support showed limited fluctuations between 2022 and 2023, with two noteworthy score adjustments. Specifically, in the category of medical nutrition, the state's coverage of disorders declined from an "A" rating to a "C" rating. In the newborn screening category, the utilization of dried blood spots increased from a "D" rating to a "B" rating. It's important to note that all other factors have remained consistent since the initial NV-RDAC annual report submitted for the 2021 calendar year, with both prescription drug out-of-pocket costs and step therapy aka fail first remaining at an "F" rating.

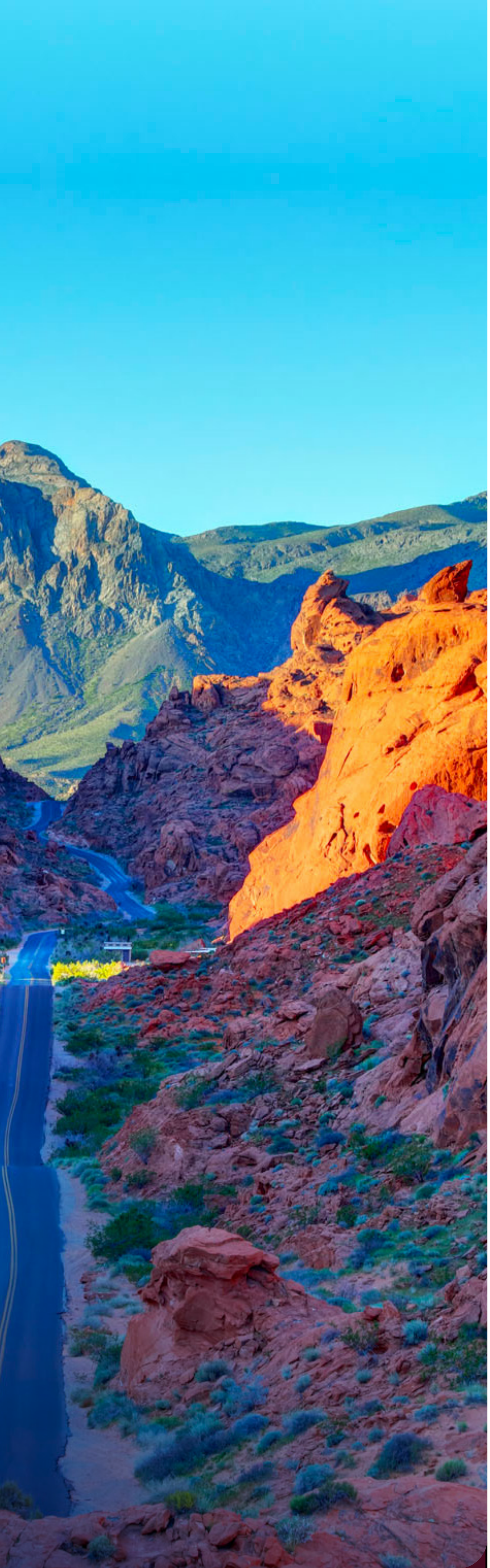
- SB 194 did pass in June 2023- and may not be reflected in the current NORD rating yet.

This bill revises provisions relating to step therapy protocols under very limited circumstances.

Despite continuous efforts to engage with various government agencies, including Medicaid, to address issues related to prescription drugs and step therapy, as well as the numerous barriers hindering access to care for many Nevadans with rare diseases, we find ourselves perpetually stuck in an unsatisfactory rating on critical matters.

It's important to note that the council does not currently possess advisory authority over prescription formularies or other access-related issues concerning rare diseases. Consequently, we are unable to provide guidance on therapy standards of care or pathways to reduce costs in this regard.

The council has consistently conveyed its concerns regarding the outdated Medicaid Service Manuals for rare disease conditions, which promote prescription patterns misaligned with current best practices. Furthermore, it has been underscored that national Pharmacy Benefit Managers (PBMs), including entities like Magellan, create exceedingly challenging situations for medical providers. These situations lead to costly administrative burdens as they fail to adequately address the distinct and unique needs of the Nevada healthcare landscape.



We appreciate that Nevada, like any state, faces its own set of challenges. However, we urge the Governor's office to recognize that patients with rare diseases present fundamentally distinct challenges compared to those with more common diseases. In Nevada, these challenges are exacerbated by outdated policies, insufficient reimbursement models (billing guides), uninformed access protocols, and a lack of effective collaboration among various stakeholders. To make substantial progress in Nevada and fulfill the vital objectives outlined in NRS 439.5077, we must prioritize improved collaboration as a foundational step.

In addition to the unfavorable report card Nevada receives from the National Organization for Rare Diseases ("NORD"), it is important to note that Nevada consistently falls short of meeting the minimum publication requirements set by the United States Cancer Statistics ("USCS") for data recognition in the CDC's national cancer registry. This holds particular relevance for the NV-RDAC and this annual report since childhood cancer falls under the classification of rare diseases.

This means that Nevada's data consistently fails to meet the fundamental standards required for inclusion in the United States Cancer Statistics (USCS) data. As part of the annual release of USCS data, the National Program of Cancer Registries (NPCR) conducts an evaluation of data provided by each supported central cancer registry. This evaluation is based on the NPCR's established criteria, which encompass data completeness, timeliness, and quality. Only registry data that meets these basic standards are deemed eligible for inclusion in USCS data products.

Nevada consistently falls short of the criteria required for inclusion in this significant national statistical profile. This shortfall has far-reaching implications, particularly as it restricts the state's physician-scientists and researchers from accessing vital federal cancer research funding, both in the realm of adult and childhood cancer. This limitation hampers the state's ability to contribute to and benefit from crucial advancements in cancer research, ultimately impacting the well-being of its residents.

The Chair of the NV-RDAC has actively engaged with the Nevada Central Cancer Registry on numerous occasions to tackle this vital issue. While the team at the Nevada Central Cancer Registry is enthusiastic about meeting the standards established by the National Program of Cancer Registries (NPCR), it is evident that their program is severely under-resourced. This resource deficiency poses a considerable challenge to their efforts to enhance data quality and meet the requirements necessary for inclusion in national cancer research initiatives.

Nevada is facing challenges in its Sickle Cell Data Collection efforts as well. In 2019, Nevada established the Sickle Cell Registry (SCR) through Assembly Bill 254 (AB 254). The SCR's primary objective is to gather data on the incidence (number of newly diagnosed cases) and prevalence (number of cases living in Nevada) of sickle cell disease and its variants.

Under Nevada law, healthcare facilities, healthcare providers, and medical laboratories are mandated to submit data to the state for the Sickle Cell Registry through a standardized reporting form. This process mirrors the one currently in place for the cancer registry. However, despite the law being enacted four years ago, both the electronic reporting forms and the paper reporting forms are still in the process of being developed and implemented. Like the state's central cancer registry, the sickle cell registry is facing significant resource limitations, which have hindered its ability to operate effectively and efficiently.

In summary, Nevada's report card for rare disease support falls short, and prospects for improvement remain dim due to the persistent pattern of inadequate resourcing for crucial initiatives established through legislative processes. We earnestly implore the leaders and lawmakers of Nevada to translate their commitment into action by champion the development of a comprehensive statewide action plan that explicitly addresses the unique needs of individuals living with rare diseases, their families, and the dedicated healthcare professionals who care for them.

It is imperative that Nevada initiate a coordinated and statewide strategy that systematically allocates the essential resources required for compliance with existing and new legislation. This approach stands as the primary avenue through which Nevada can exert its influence and enact the critical policy changes necessary to adequately support individuals living with rare diseases within our state.

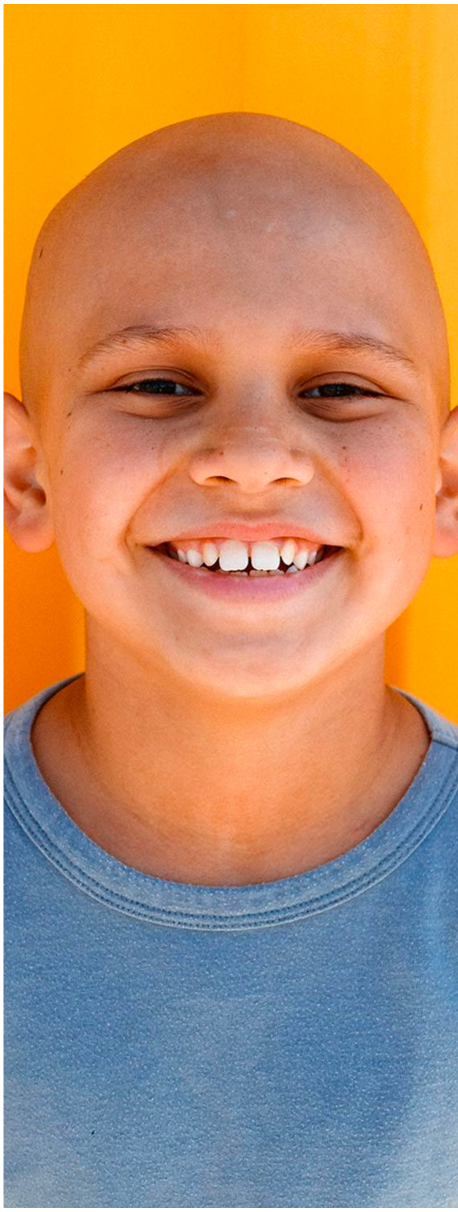
Current Rare Diseases Being Focus on by the NV-RDAC

The NV-RDAC voted in December of 2020, that their initial focus would be on three primary categories of rare diseases:

1. All Childhood Cancers
2. Factor deficiencies and inherited platelet disorders
3. Newborn screening conditions- currently, the newborn screening in Nevada screens for 46 conditions

2023 NV-RDAC Activities

In a year marked by commendable progress, the Nevada Rare Disease Advisory Council (NV-RDAC) has devoted significant efforts to bring about meaningful change for the rare disease community in Nevada.



Medicaid Study on the Rare Disease Service Delivery Models and an Analysis of the Issues Unique to Nevada

At the close of 2022, the Nevada Rare Disease Advisory Council (NVRDAC) achieved a significant milestone through successful advocacy for the state of Nevada's participation in a groundbreaking and essential study. Through continuous discussions and collaboration with the leadership team at the Nevada Medicaid office, the Nevada State Interim Finance Committee approved a generous grant of $250,000 to conduct a comprehensive study specifically focused on Rare Disease Service Delivery Models unique to Nevada.

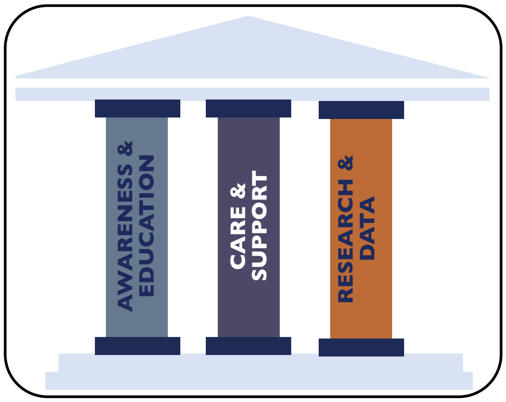
Throughout the early months of 2023, the members of the NV-RDAC collaborated closely with the contracted study coordinators to ensure that a diverse range of stakeholders were actively engaged in the process. The primary objective of this study is to facilitate an in-depth analysis, shedding light on the unique challenges that are often overlooked within the context of Nevada. It is anticipated that this study will yield valuable insights that will serve as a roadmap for enhancing rare disease service delivery within the state.

The substantial involvement of NV-RDAC members underscores the council's unwavering commitment to advocating for the needs and concerns of the rare disease community. Their active participation ensures that the voices of individuals affected by rare diseases are effectively heard and considered in the realms of legislative and policy-making decisions. **The NV-RDAC eagerly awaited the release of this report.**

Implementation of Additional NV-RDAC Regularly Scheduled Meetings.

In a bid to enhance progress in 2023, the NV-RDAC made the strategic decision to incorporate two additional meetings into their annual schedule. This proactive adjustment aims to promote more consistent, timely, and efficient communication among the diverse Council members throughout the year. The increased frequency of communication is vital for the swift and effective implementation of decisions, enabling the NV-RDAC to respond more promptly and effectively to the evolving needs and challenges faced by the rare disease community in Nevada.

NV-RDAC Council Member Survey



The Nevada Rare Disease Council conducted a member survey to gather insights into how they should allocate their limited resources. The survey results affirmed that NV-RDAC members are not only deeply committed to serving the state of Nevada on behalf of individuals with rare diseases, but they also share a common vision for enhancing the overall landscape of rare disease management in Nevada. This vision encompasses various critical aspects, including:

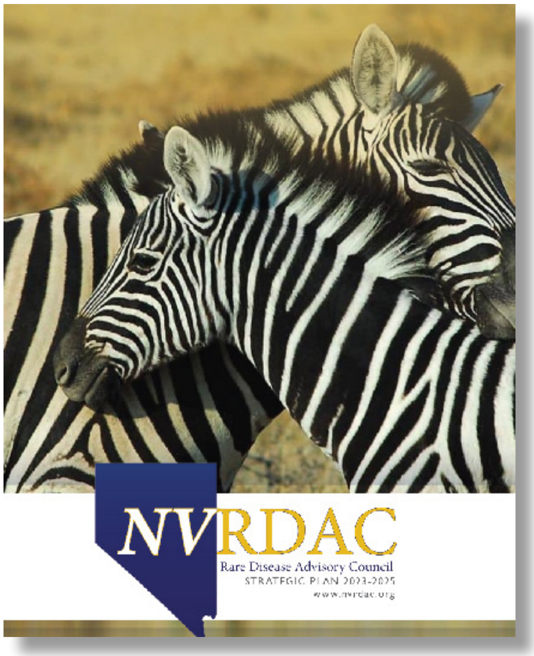
1. **Access to High-Quality Medical Care:** Ensuring individuals with rare diseases have access to top-notch medical care.
2. **Improving Healthcare Provider Knowledge:** Enhancing the knowledge and awareness of healthcare providers in the state regarding rare diseases.
3. **Advocating for Positive Payer Coverage:** Advocating for positive changes in insurance coverage for individuals with rare diseases.
4. **Influencing Regulatory Reform:** Educating state lawmakers and advocating for improved regulations that positively impact individuals with rare diseases and their families.

To effectively assess their priorities and resource allocation, the NV-RDAC members utilized this survey tool that categorized their 13 assigned duties into six distinct categories. Subsequently, they ranked these categories in order of importance based on their current capabilities. The survey results led to the following prioritized areas:

1. **Nevada-Specific Data Collection & Analysis:** Collecting and analyzing data specific to Nevada's rare disease landscape to inform decision-making and resource allocation.
2. **NV-RDAC Awareness & Education Campaigns:** Launching campaigns to raise awareness and educate the public and healthcare professionals about rare diseases.
3. **Establishment of a Comprehensive Rare Disease Plan for Nevada:** Developing a comprehensive plan that addresses the unique needs of individuals with rare diseases in Nevada.
4. **Health Equity and Disparities that Impact People with Rare Diseases:** Addressing disparities and inequities in healthcare access and outcomes for individuals with rare diseases.
5. **Leveraging the National Rare Disease Advisory Council for Efficiencies and Effectiveness:** Collaborating with the national council to leverage resources and expertise for more efficient and effective rare disease management.
6. **Establishment of a Statewide Rare Disease Needs Assessment:** Conducting a comprehensive assessment of the needs of individuals with rare diseases across the state.

These priorities, as determined by the NV-RDAC members, serve as the cornerstone of the first NV-RDAC strategic plan. They embody the collective vision and unwavering commitment of the council members to effecting substantial improvements in the lives of individuals impacted by rare diseases in Nevada.

The Drafting of the First-ever NV-RDAC Strategic Plan


NVRDAC
Rare Disease Advisory Council
STRATEGIC PLAN 2023-2025
www.nvrdac.org

Scan the QR code
or click to view
the 2023-2024
Strategic Plan

This strategic plan signifies a significant milestone as it represents the inaugural statewide coordinated effort by the NV-RDAC to address rare diseases in Nevada. Recognizing the intricate nature of rare diseases, the substantial unmet needs, and the urgency associated with these conditions, it is imperative that we embark on a path of systematic reform.

Rare diseases encompass a wide array of conditions, yet they also exhibit numerous commonalities. Informed by extensive stakeholder consultations conducted through the 2022 council member survey, this strategic plan places a strong emphasis on these shared aspects. It authentically captures the perspectives of the rare disease sector and outlines the initial, comprehensive, collaborative, and data-informed approach essential to achieving the optimal health and well-being outcomes for individuals living with rare diseases in Nevada. The survey tool employed by NV-RDAC council members thoughtfully categorized their 13 designated duties into six distinct areas, which are consolidated under the following three primary foundational pillars:

1. **Awareness and Education**
2. **Care and Support**
3. **Research and Data**

These three pillars serve as the guiding framework for our strategic endeavors, underpinning our commitment to enhancing the rare disease landscape in Nevada. While each pillar is presented separately in this plan, it is vital to recognize their interconnectedness, as they operate in harmony to drive positive change and progress for individuals with rare diseases in the state.

The NV-RDAC's strategic plan plays a pivotal role in aligning the council's activities for the upcoming three years. As we move forward, the council is bound to grow and evolve, encountering new challenges and opportunities that will require our attention. Nevertheless, the pillars outlined in this plan will serve as the steadfast foundation of our collective efforts, guiding us with clear goals and objectives.

It is worth noting that this Strategic Plan garnered unanimous approval from our council members this year, underscoring its importance and relevance. It establishes a clear and focused roadmap for our future initiatives, ensuring that we remain steadfast in our commitment to addressing rare diseases in Nevada. These three pillars are derived from the objectives outlined in NRS 439.5077, which provides a solid legislative framework for the NV-RDAC's mission and purpose.

Awareness & Education Campaign and Rare Needs Assessment

Scheduled for its official launch on World Rare Disease Day in 2024, the 'While You Wait' campaign initiated by NV-RDAC marks a momentous step in addressing the critical need for awareness and education regarding rare diseases in the state of Nevada. This campaign is strategically positioned to serve as a cornerstone in raising awareness and educating the public about this urgent issue. Its launch on World Rare Disease Day underscores the importance of this initiative and its potential to make a substantial impact in the realm of rare diseases within the state.

This campaign has been thoughtfully designed to have a far-reaching impact, as it will be prominently displayed in healthcare provider offices throughout the state, ensuring extensive exposure and influence. The campaign's collateral materials serve a dual purpose: not only do they communicate the urgent need to amplify the voices of individuals living with rare diseases in Nevada, but they also empower individuals to take collective action.

Each piece of collateral contains a QR code that links to a Comprehensive Statewide Rare Disease Needs Assessment. This user-friendly digital platform has been meticulously structured to gather firsthand information from Nevadans regarding various aspects of access to and barriers in healthcare. This assessment goes beyond surface-level questions, providing individuals with the opportunity to share their personal journeys, diverse experiences, and the specific challenges they encounter in dealing with rare diseases in Nevada. The sharing of these real-life stories is fundamental in constructing a comprehensive and nuanced understanding of the state's rare disease landscape.

The utilization of the REDcap platform to house the data collected from the assessment underscores the campaign's commitment to precision, security, and effectiveness. This platform will adeptly capture and securely store the data collected through the campaign. Long-term data collection and analysis are essential in providing continuous and evolving insights into the needs and challenges of the rare disease community. This ensures that strategies, interventions, and policies remain pertinent, responsive, and effective over time.

The 'While You Wait' campaign comprises a suite of collateral materials available in both English and Spanish, designed to raise awareness about NV-RDAC. These materials will be distributed to all relevant provider offices statewide with the intention of providing awareness and educational resources to a diverse range of Nevadans while they wait in their healthcare provider's office. These materials are suitable for a wide variety of audiences and are designed to cater to different medical specialties.

Due to the NV-RDAC's insufficient funding allocation from the state through NRS 439.5075, this critical statewide data collection initiative has been made possible through the generous financial support provided by the Cure 4 The Kids Foundation. Their contribution has been instrumental in enabling the council to carry out this important effort, ensuring that it can effectively address the needs of individuals with rare diseases in Nevada

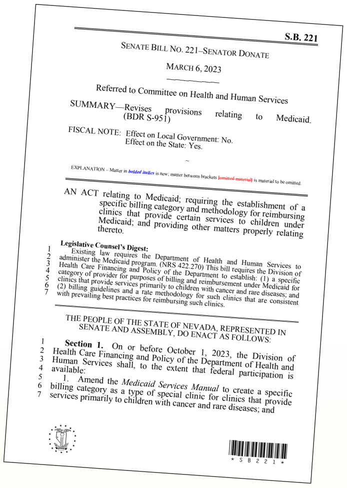
Legislation Monitoring

In 2023, the NV-RDAC undertook a diligent and meticulous monitoring of several bills to ensure that each piece of legislation resonated with and supported the well-being and multifaceted needs of the rare disease community. The council was actively engaged in closely tracking the progress, amendments, and implications of these bills, with a steadfast commitment to advocating for provisions and modifications that prioritize and address the concerns and requirements of rare disease patients and their healthcare providers. The Senate and Assembly bills included:

- **AB471**: This bill appropriates funds to the State Public Works Division of the Department of Administration for the replacement of computer hardware and associated software. Passed on June 12, 2023.
- **AB155:** It establishes provisions related to biomarker testing. Passed on June 13, 2023.
- **SB194**: This bill revises provisions relating to step therapy protocols. Passed on June 1, 2023.
- **SB221**: It revises provisions related to Medicaid. Passed on July 17, 2023.
- **SB255:** This bill makes various changes relating to services provided for the treatment of diseases that predominantly affect children. Failed on April 15, 2023.

Senate Bill 221 holds exceptional significance for the residents of Nevada living with rare diseases. This legislation duly acknowledges the vital role played by unique specialty clinics dedicated to caring for pediatric cancer and rare diseases. SB221 goes a step further by establishing a dedicated Nevada Medicaid billing guideline and rate methodology meticulously tailored to accommodate the specialized services these clinics offer.

The passage of SB221 stands as a momentous achievement for healthcare providers and the states' Medicaid program, as well as patients throughout Nevada. This legislation ensures that specialty clinics, often entrusted with the provision of highly specialized and costly care, receive reimbursement rates that accurately reflect the intricacies and expenses associated with the care they provide. This, in turn, facilitates these clinics in maintaining their capacity to deliver high-quality care to patients grappling with serious and rare conditions. **Although, SB221 serves as a resounding victory for the rare disease community in the state, the bill still falls short, as it failed to address the issue of high-cost drugs and only impact the professional services provided.**



S.B. 221

SENATE BILL NO. 221–SENATOR DONATE

MARCH 6, 2023

Referred to Committee on Health and Human Services

SUMMARY—Revises provisions relating to Medicaid. (BDR S-951)

FISCAL NOTE: Effect on Local Government: No.
Effect on the State: Yes.

~

EXPLANATION – Matter in ***bolded italics*** is new; matter between brackets ~~[omitted material]~~ is material to be omitted.

AN ACT relating to Medicaid; requiring the establishment of a specific billing category and methodology for reimbursing clinics that provide certain services to children under Medicaid; and providing other matters properly relating thereto.

Legislative Counsel's Digest:

Existing law requires the Department of Health and Human Services to administer the Medicaid program. (NRS 422.270) This bill requires the Division of Health Care Financing and Policy of the Department to establish: (1) a specific category of provider for purposes of billing and reimbursement under Medicaid for clinics that provide services primarily to children with cancer and rare diseases; and (2) billing guidelines and a rate methodology for such clinics that are consistent with prevailing best practices for reimbursing such clinics.

THE PEOPLE OF THE STATE OF NEVADA, REPRESENTED IN SENATE AND ASSEMBLY, DO ENACT AS FOLLOWS:

Section 1. On or before October 1, 2023, the Division of Health Care Financing and Policy of the Department of Health and Human Services shall, to the extent that federal participation is available:

1. Amend the *Medicaid Services Manual* to create a specific billing category as a type of special clinic for clinics that provide services primarily to children with cancer and rare diseases; and

Building Relationships

The NV-RDAC remains steadfast in advancing its agenda, dedicated to catalyzing substantial positive changes in Nevada's healthcare landscape. The council's proactive engagements have led to high-level discussions with a multitude of key stakeholders in the rare disease field. These discussions have included organizations such as NORD, Medical Home Portal, and Fertility Advocates, among others. (Please refer to the meeting minutes for a comprehensive list of presenters.) These conversations have ignited collaborative efforts and enriched the resources and support available to rare disease patients and healthcare providers across the state. Resulting in the council actively collaborating with the state of Nevada to identify opportunities for streamlining data collection processes. Through ongoing dialogues with both the vital records and the cancer registry teams within the Department of Health and Human Services, the council has identified gaps in the current processes and has initiated efforts to enhance their efficiency. This initiative is primarily driven by the overarching goal of first optimizing childhood cancer registry and then sickle cell efforts in Nevada.

In a highly significant partnership, the NV-RDAC has partnered with Cure 4 The Kids Foundation, the largest provider of medical care for both childhood cancer and sickle cell patients in Nevada. This collaboration has resulted in Cure 4 The Kids Foundation making the commitment to investment in the development of an electronic medical record interface with the state's central cancer registry. This interface will be active in early 2024.

This collaborative initiative is driven by the shared goal of ensuring that, going forward, childhood cancer statistics for Nevada not only align with the criteria set by the National Program of Cancer Registries (NPCR) for inclusion in the United States Cancer Statistics (USCS) annual reporting but are also collected and managed efficiently through technological solutions, reducing the reliance on manual labor. This is of particular importance given the resource and staffing challenges faced by the Nevada central cancer registry.

This collaborative effort represents a significant leap forward in enhancing the accuracy and efficiency of data collection and reporting for childhood cancer statistics in Nevada. Furthermore, this same technology will be employed in the future for sickle cell registry efforts, further strengthening the state's data collection capabilities for these critical healthcare areas.

Community Awareness

The NV-RDAC Website

The establishment of the Nevada Rare Disease Advisory Council (NV-RDAC) webpage at www.nvrdac.com, generously funded and supported by Cure 4 The Kids Foundation, represents a major leap forward in harnessing information and communication technology to address the needs of individuals living with rare diseases in Nevada.. In today's modern world, access to current and reliable data is crucial for informed decision-making and support, especially for those facing rare diseases.

Nevada's unique geographic landscape, with its large landmass and predominantly rural and frontier counties, underscores the importance of having an online platform like the NV-RDAC website. Such a platform serves as a vital means of communication and information dissemination, enabling the council to reach both patients with rare diseases and the medical providers caring for them, regardless of their location within the state.

This website will grow to serve as a hub for sharing essential resources, educational materials, updates on legislative initiatives, and a means for rare disease communities in rural areas to connect and access support. It plays a pivotal role in bridging geographic gaps and ensuring that individuals living with rare diseases in all parts of Nevada have equitable access to information, resources, and support networks.

Social Media Platforms established for the NV-RDAC

Leveraging social media for rare disease education and awareness campaigns is an excellent strategy employed by the NV-RDAC. Social media platforms provide a powerful means to connect with a wide audience and disseminate crucial information efficiently. The use of social media allows for the rapid spread of awareness and education, reaching numerous people in a relatively short amount of time.

The increasing trend nationally in using digital media as a platform for raising awareness about rare diseases aligns with the NV-RDAC's efforts to harness the potential of social media. Establishing platforms like a Facebook page and LinkedIn for the NV-RDAC is a proactive step in engaging with the community, sharing resources, and keeping the public informed about the issues facing individuals living with rare diseases in Nevada.

By utilizing social media, the NV-RDAC can expand its reach, engage with a diverse audience, and contribute to the broader national trend of increasing awareness and understanding of rare diseases, ultimately working towards improved support and care for those affected by these conditions within the state. The NV-RDAC social media platforms are voluntarily managed by the digital and social media director at Cure 4 The Kids Foundation.

Public Relations and Media
In 2023, members of the NV-RDAC's showcased their unwavering commitment to the rare disease community by prominently being showcased on local news channels, social media platforms, and in print for World Rare Disease Day 2023. This media coverage served as a potent platform for the council to raise awareness and educate the local community about the critical issues, challenges, and needs faced by individuals with rare diseases.

The news coverage effectively spotlighted the council's initiatives, strategic plans, and its steadfast dedication to improving the landscape of rare disease care within the state. This heightened visibility not only shed light on the importance of addressing rare diseases but also fostered a greater understanding and support from the community at large.

Conclusions & Recommendations:

The establishment of the Nevada Rare Disease Advisory Council (NV-RDAC) in 2019 during the Nevada Legislature marked a significant milestone for the state. At that time, Nevada became the 11th state in the United States to officially adopt an RDAC through legislation. Nevada is technically considered an early adopter, which highlights Nevada's commitment to addressing the unique challenges posed by rare diseases within the state.

The NV-RDAC's challenges underscore the critical importance of aligning the volume and complexity of duties assigned with the necessary resources to fulfill their essential role effectively. In this context, the generous financial support provided by Cure 4 The Kids Foundation has proven to be indispensable for the council's operations. Without this external support, the NV-RDAC would face significant limitations in its ability to carry out its mission effectively.

When addressing intricate and pressing healthcare issues like rare diseases, it is paramount to ensure that advisory councils have the requisite resources, including funding, personnel, and infrastructure, to meet their objectives and make meaningful improvements in the lives of individuals affected by these conditions. This situation serves as a poignant reminder of the vital role played by investments in initiatives aimed at comprehensively addressing critical healthcare challenges, ultimately benefiting both patients and the broader community.

NV-RDAC Summary of Progress on Duties assigned by NRS 439.5077

Despite the challenges outlined throughout this document that might suggest limitations on the RDAC's ability to make progress, the Nevada Rare Disease Advisory Council (NV-RDAC) has achieved significant strides in addressing the tasks assigned to them through NRS 439.5077. Here is a summary of their effectiveness in fulfilling their assigned duties:

1) **Statistical Examination of Rare Diseases:** The council has initiated efforts to establish data collection systems (childhood cancer and sickle cell registry support, and the statewide needs assessment) so that in the future Nevada will be capable of examining the incidence, causes, and economic burden of rare diseases in the state. Continued progress in data collection and analysis will be dependent on adequate funding.

2) **Receiving Reports and Testimony:** The NV-RDAC has actively engaged with various stakeholders and organizations, including community-based groups, healthcare providers, and individuals affected by rare diseases, to ensure significant participation in the Medicaid Study (Mercer Study) that, based on information from Medicaid leadership is schedule for publication in December 2023. The result of the study will be key in determining the NV-RDAC next steps.

3) **Increasing Awareness:** The council has demonstrated a commitment to raising awareness about rare diseases through initiatives like the 'While You Wait' campaign and social media presence, as well live television appearances and print article about members of the NV-RDAC.

4) **Identifying Prevention Strategies:** It is anticipated that efforts to identify evidence-based strategies for preventing and controlling rare diseases may be improved through the efforts of SB 221. It is early to determine the true impact of SB 221. As of the date of this report the implantation plan has not been shared with the NV-RDAC.

5) **Effect of Delayed Treatment:** Through the statewide needs assessment launching in February 2024, the NV-RDAC will be collecting the data required to study the effect of delayed or inappropriate treatment on the quality of life for rare disease patients and the economy of Nevada. However, it is already a well-known fact worldwide that the lengthy diagnosis odyssey experienced by people with rare disease results in both delayed diagnosis and delayed treatment.

6) **Effect of Early Treatment:** The endeavor to comprehend the impact of early treatment on patient outcomes and service provision may appear redundant for Nevada, given that the medical profession has globally documented the importance of early intervention across all disease states. The members of the NV-RDAC are pleased to offer published data that substantiates the significance of early intervention. This data will be incorporated into the ongoing strategic plan status updates to reinforce the importance of timely action.

7) Awareness Among Healthcare Providers: Efforts are being actively implemented to enhance awareness among healthcare providers regarding the symptoms and care required for rare disease patients. The rollout of the "While You Wait" campaign includes educational information designed for provider offices that will be invited to participate in the campaign. This dual-purpose campaign serves to introduce both the needs assessment and the ongoing work of the NV-RDAC, along with providing guidance on how to engage with the NV-RDAC for information and collaboration.

8) Evaluating Treatment Systems: The upcoming Medicaid study is expected to shed light on the limited healthcare resources in Nevada, especially in rural areas. The NV-RDAC is eager to assess the study's results, as they anticipate it will confirm what the council members already recognize - that Nevada is both a Medically Underserved Area and a Health Professional Shortage Area. This situation is further compounded by the absence of a comprehensive, academic-based children's hospital within the community. Therefore, given this unique context, there is a pressing need for an innovative approach to rare disease care that differentiates it from the standard practices observed in other communities across the country. The NV-RDAC is keen to leverage the insights gained from the Mercer Study to advocate for enhanced healthcare resources and tailored solutions to address the specific healthcare challenges faced by Nevada's residents, particularly those affected by rare diseases.

9) Data Collection Methods: The NV-RDAC and Cure 4 The Kids Foundation are partnering to enhance data collection methods for rare diseases in Nevada, particularly childhood cancer and sickle cell patients. This undertaking is hindered by the significant time and financial investments required. The primary challenge lies in the limited resources available to the NV-RDAC and the Nevada Department of Health and Human Services, which impedes the state's ability to effectively collect and use relevant data.

10) Comprehensive Management Plan: The Nevada Rare Disease Advisory Council (NV-RDAC) has released its 2023-2025 Strategic Plan, with the goal of benefiting rare disease patients and healthcare professionals in the state. Historically, rare diseases have been neglected due to a lack of awareness about the difficulties faced by those affected. The NV-RDAC intends to address this issue by increasing awareness and advocating for greater recognition of rare diseases. This strategic plan serves as the foundational framework for developing a comprehensive management plan for rare diseases in Nevada, and it can be accessed by the public at www.nvrdac.org.

11) Rare Disease Registry: The NV-RDAC plans to establish a Rare Disease Registry using the data collected through the needs assessment set to commence in February 2024. This initiative is designed to collect data on rare diseases diagnosed within the state. However, it is anticipated that several years will be required for the data to accumulate sufficiently to enable the identification of specific genetic and environmental factors related to rare diseases in Nevada.

12) **Annual Report:** The council has provided the required annual report summarizing its activities and making recommendations for legislation or policy changes- this is the third such report.

13) **Specialized License Plate:** A specialized license plate supporting the NV-RDAC, and childhood cancer research and treatment is offered by the Nevada DMV. However, this funding source has not generated sufficient funds to have a substantial impact on the organization's efforts.

(I) NOTE:

(a) **As of October 12, 2023, the DMV Specialized License Plate Account holds a balance of $39,091 earmarked for the NV-RDAC.** No funds from this account have been utilized thus far.

(i) All expenses incurred by the NV-RDAC up to this point have been covered by Cure 4 The Kids Foundation. These expenses encompass various areas, including the creation and maintenance of the website domain and platform, creative expenses related to the NV-RDAC branding guide, development of the strategic plan, and the establishment and construction of the statewide needs assessment. This also includes the provision of the designated Redcap server and the "While You Wait" campaign. The in-kind contribution to the NV-RDAC from C4K is estimated to be valued at $50,000.00.

The Cost of Poorly Managing Rare Disease

The cost of poorly managing rare diseases for a state can be substantial and multifaceted and is known to be substantial in terms of both economic and human impact. While it is challenging to provide an exact figure as it varies depending on several factors, here are some of the nationally recognized costs associated with inadequate management of rare diseases:

1. Healthcare Costs: Individuals with rare diseases often require specialized medical care, including costly treatments, medications, and frequent hospitalizations. Poor management of these conditions, exacerbated by outdated policies resulting in unnecessary administrative burdens, has been demonstrated to result in significantly increased healthcare expenditures. Additionally, the lengthy and often costly diagnostic odyssey that individuals with rare diseases experience, which can span several years, adds to the overall economic burden of managing rare diseases. Streamlining policies, administrative processes, and improving diagnostic pathways can significantly help mitigate these costs and enhance the overall management of rare diseases.

2. Loss of Productivity: Rare diseases often lead to significant disability, particularly when diagnosis is delayed due to the extended time required for the diagnostic process. This delay not only affects the individuals with rare diseases but also their parents and caregivers, preventing them from working and making contributions to the economy. Consequently, there is a loss of productivity at both the individual and societal levels. Early diagnosis and timely intervention are crucial in mitigating this loss of productivity and enhancing the economic well-being of individuals and families affected by rare diseases.

3. Increased Hospitalizations: Inadequate management of rare diseases, often stemming from outdated utilization review and access rules, frequently leads to preventable complications and hospitalizations. This not only drives up healthcare costs but also places a significant burden on the healthcare system as a whole. Revising and modernizing utilization review and access policies can be instrumental in reducing unnecessary hospitalizations and improving the overall management of rare diseases.

4. **Long-Term Care:** Certain rare diseases necessitate lifelong care and support, resulting in long-term care costs that can be financially burdensome for families, individuals, and the state's resources. These ongoing care requirements can pose challenges in terms of both financial sustainability and access to necessary services. Addressing the long-term care needs of individuals with rare diseases is vital to ensure their well-being and alleviate the economic strain on affected families and the state's healthcare system.

5. **Lower Quality of Life:** Inadequately managed rare diseases can significantly lower an individual's quality of life, causing physical and psychological suffering not only for the affected individuals but also for their family members. This added emotional and mental burden can exacerbate existing mental healthcare challenges, further underscoring the importance of effective rare disease management. Improving the management of rare diseases is not only essential for physical health but also for enhancing the overall well-being and mental health of those affected and their families.

6. **Systemic Ineffective Management and Missed Research Opportunities:** Systemic ineffective management of rare diseases obstructs access to and progress in research and innovation within the field of rare diseases. This hindrance can lead to missed opportunities for discovering new treatments and therapies that could significantly benefit individuals with rare conditions. Timely and effective management, along with dedicated research efforts, is essential to advance our understanding of rare diseases and develop innovative solutions that can enhance the quality of life for affected individuals. Encouraging research and innovation is vital for addressing the unique challenges posed by rare diseases and for unlocking potential breakthroughs in their treatment and care.

7. **Societal Costs:** Rare diseases entail significant societal costs, encompassing the emotional toll on families and caregivers, as well as the broader social and economic impacts of individuals unable to fully participate in society. These costs extend beyond healthcare and encompass the well-being and productivity of affected individuals and their support networks. Addressing rare diseases effectively not only mitigates the emotional burden on families but also fosters a more inclusive and economically productive society, where individuals with rare conditions can contribute to their fullest potential.

8. **Public Health Costs**: Neglecting the overhaul of rare diseases management poses significant public health challenges, including situations that often necessitate frequent public health responses, adding additional administrative burdens and demanding expertise. Failing to stay current with best practices in the diagnosis and treatment of rare diseases effectively can strain public health resources and disrupt overall health system operations. This emphasizes the importance of proactive management and surveillance to prevent and mitigate the overuse of already strained public health departments associated with rare diseases. Keeping up to date with advancements in rare disease management is crucial for both individual and public health and can help alleviate the burdens on public health systems.

9. **Legal and Ethical Costs:** Legal and ethical issues often arise if individuals with rare diseases are denied access to necessary diagnostic tests, treatment or medical care or experience discrimination. These issues create legal challenges and associated costs, including litigation and regulatory actions. Ensuring equitable access to the things people with rare diseases need and addressing discrimination in the management of rare diseases is not only a matter of ethical responsibility but also a means of preventing legal disputes and the associated financial burdens on both affected individuals and the healthcare system.

Poorly managing rare diseases can indeed result in various economic, social, and health-related costs for a state. Implementing adequate funding and effective strategies to address rare diseases, as mandated by legislation like NRS 439.5077, is crucial not only for enhancing the quality of life for affected individuals but also for mitigating the economic and societal burdens associated with these conditions.

Recommendations

The Nevada Rare Disease Advisory Council (NV-RDAC) has made significant progress in various areas. However, addressing rare diseases is an ongoing and complex challenge. The effectiveness of the NV-RDAC will continue to evolve as they work on their responsibilities, collaborate with stakeholders, and aim to improve the quality of life for individuals affected by rare diseases in Nevada.

The primary objective of the Nevada Rare Disease Advisory Council (NV-RDAC) is to address the intricate challenges posed by rare diseases in the state and work towards improving the lives of those affected. This mission involves conducting research, raising awareness through campaigns, and contributing to policy development. To accomplish these goals, close collaboration with the state and financial support are essential.

Key recommendations include:

1. **Allocate Adequate Funding:** The state's budget needs to allocate appropriate and feasible funding to sustain the necessary staffing for the creation and execution of a comprehensive rare disease strategy across Nevada. Sufficient funding is imperative for the successful realization of these initiatives.

2. **Policy Reforms and Innovative Funding for Rare Disease Care in Nevada:** Proactively engage with and motivate Nevada legislators to spearhead advocacy efforts at the state level. These initiatives should emphasize a dual approach, encompassing policy reforms and innovative state-funded initiatives, with the goal of improving insurance coverage, expanding access to advanced diagnostic tests and progressive treatments, and enhancing the spectrum of support services accessible to individuals dealing with rare diseases in the state.

3. **Consider Outsourcing Rare Disease Data Collection and Analysis:** Acknowledging the shortcomings in the existing data collection processes, it is advisable to allocate resources towards outsourcing the improvement of data collection and analysis methods. This outsourcing initiative should prioritize obtaining accurate and detailed data regarding the prevalence of rare diseases, the effectiveness of treatments, and the experiences of patients in Nevada. Collaborating with external experts can provide the necessary expertise and resources essential for enhancing data quality and analysis.

    A) As part of this initiative, it is advisable to review and potentially amend the current Nevada legislation that defines the Rare Disease Advisory Council's data collection responsibilities, as well as those governing the Sickle Cell Disease and Lupus Registries. Such a revision can ensure that the legal framework harmonizes effectively with the outsourcing efforts, simplifying the process of collecting and managing valuable rare disease data. Additionally, this revision aims to eliminate redundancy in efforts and leverage economies of scale to enhance the efficiency of disease data collection in Nevada.

B) As of now, the NV-RDAC relies exclusively on state-specific rare disease data provided by C4K. Click HERE (or scan the QR Code) to view a three-year overview of rare disease cases treated at Cure 4 The Kids Foundation over the past continuous 36 months. This data pertains exclusively to the three primaryrare disease categories that the NV-RDAC is currently concentrating on.

4. **Bill Draft Request (BDR):** The Council respectfully requests, for the second consecutive year, authorization to present one Bill Draft Request (BDR) during each legislative session in the future. This ongoing authorization is considered crucial for the Nevada Rare Disease Advisory Council (RDAC) to effectively pursue and implement meaningful changes at the legislative level, which are essential for achieving its objectives.

5. **Increased Engagement with DHHS:** We respectfully request heightened engagement from the Department of Health and Human Services (DHHS), specifically with the Administrators of state Medicaid Programs. The ramifications of insufficiently addressing rare diseases within a state can be profound and multifaceted, encompassing significant economic and human impacts. The precise quantification of these impacts remains challenging due to their dependency on various factors, and the members of the Rare Diseases Advisory Committee (RDAC) believe that closer collaboration is imperative.

6. **Address Inadequate Reimbursement to Specialty Providers:** To effectively tackle the widespread shortage of specialized physicians and facilities in Nevada that are treating individuals with rare diseases, it is imperative to address the systemic issue of inadequate reimbursement. This problem arises from financial disincentives, burdensome student debt, and the considerable operational costs associated with delivering highly specialized medical services. This scarcity of specialists has led to geographical disparities in healthcare access, compromised service quality, and adverse public health consequences for the state of Nevada. To mitigate these challenges, potential solutions encompass recalibrating reimbursement rates, implementing loan forgiveness programs for sub-specialists, advocating for telemedicine, investing in workforce development, and offering incentives to specialists in underserved regions, all aimed at guaranteeing equitable access to specialized care.

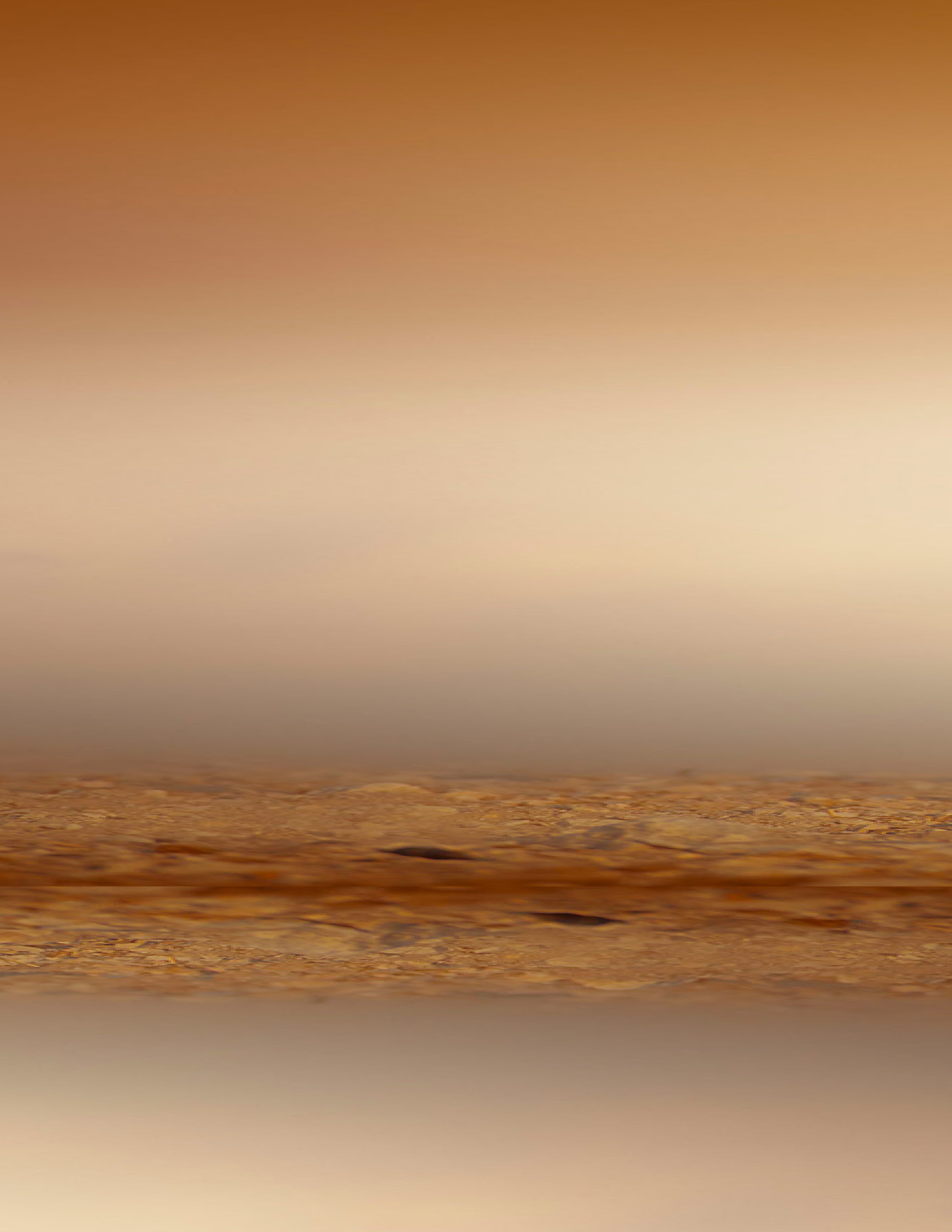
A) It is advisable to exclude rare disease providers, especially those eligible under the new provider type established through SB 221, from the Physician Administered Drug (PAD) fee schedule. It is widely recognized that patients living with rare diseases often require expensive medications, and the accessibility of these drugs is increasingly challenging. For instance, as of July 2023, the sudden adjustment in outpatient drug reimbursement rates due to the introduction of the Nevada Physician Administered Drug (PAD) fee schedule has had a significant and adverse impact on healthcare providers. This alteration has placed substantial strain on these providers, exacerbating their financial losses while delivering care to Medicaid beneficiaries. Specialized physicians, in particular, who offer essential yet costly medical treatments for individuals with rare diseases, have been disproportionately affected.

B) **Although SB 221 did offer some financial relief on the medical services side of the equation, it did not address the broader issue of reimbursing high-cost drugs.** The state's cost-saving initiative, the Physician Administered Drug (PAD) fee schedule, has driven drug reimbursement rates to an unprecedented low. This situation is likely to disincentivize specialty physicians from considering relocation to Nevada to practice. The adverse impact of the Physician Administered Drug (PAD) fee schedule, which diminishes drug reimbursement rates, creates an additional barrier for these specialized healthcare providers. As they grapple with financial challenges related to delivering care to individuals with rare diseases, the attractiveness of practicing in Nevada is diminishing, exacerbating the increased shortage of specialized medical expertise in the state.

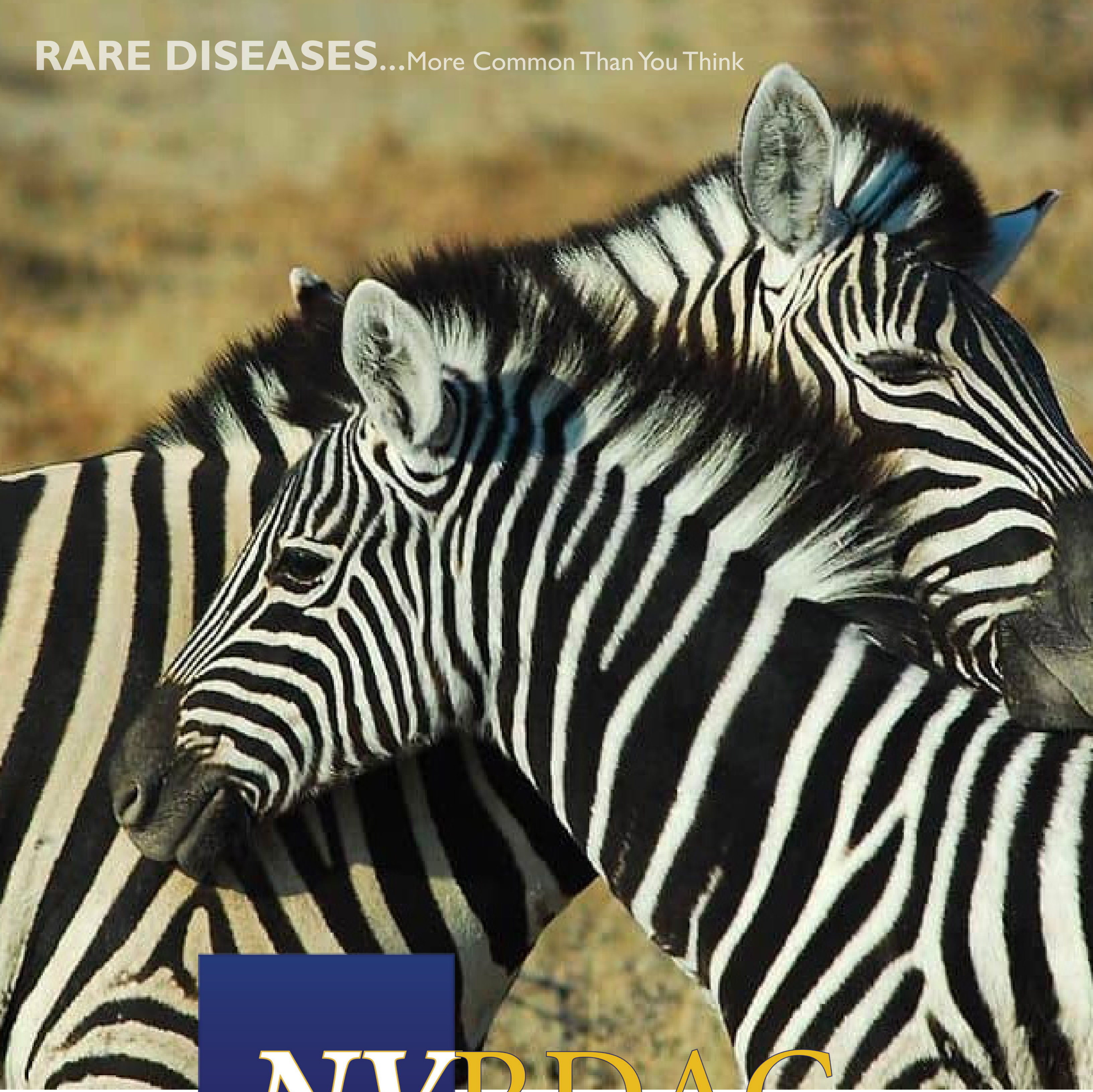
C. In the absence of a 340B Drug Discount Program for these providers; the alternative drug fee schedule for providers qualifying for the new provider type established through SB 221 should be 130% of the published Wholesale Acquisition Cost (WAC), which is the manufacturer's list price for a prescription drug when sold to wholesalers, pharmacies, or other healthcare providers. It represents the price at which the manufacturer makes the drug available to purchasers before any discounts, rebates, or other reductions in price are applied. In essence, it is the baseline price set by the pharmaceutical manufacturer for their product.

By implementing these recommendations, Nevada can take significant steps toward better addressing the needs of its rare disease community, ensuring adequate funding, policy support, and improved data collection and analysis to enhance the quality of care and support for those affected by rare diseases in the state.

Submitted Respectfully by Annette Logan-Parker, NV-RDAC Chair



RARE DISEASES…More Common Than You Think



NVRDAC

Rare Disease Advisory Council

STRATEGIC PLAN 2023-2025

www.nvrdac.org

EMPOWERING

Nevadans Living with a

Rare Disease to

Improve Their Future...

Table of Contents

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 current chair of the Nevada Rare Disease Advisory Council (NV-RDAC) and the Founder & CEO of Cure 4 The Kids Foundation. I am honored to hold the position and am committed to advancing the objectives of the council and fulfilling the duties assigned to us through SB-315 .

The Nevada Rare Disease Advisory Council is pleased to share our 2023-2025 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 NV-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 NV-RDAC 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 (February, April, June, August, October, and December) on the first Friday of the month at 9:30 AM (*Time subject to change, please confirm using the QR code below.)

Rare Disease Advisory Council
Meeting Schedule (nv.gov)

For a list of current RDAC members, please see www.NVRDAC.org.

MISSION

The Nevada Rare Disease Advisory Council'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.

What is a RARE DISEASE?

In the late 1940s, Dr. Theodore Woodward, professor at the University of Maryland School of Medicine, instructed his medical interns: “When you hear hoofbeats, think of horses, not zebras.” *Rare Disease Report*™ wants to encourage physicians and patients to always keep an eye out for the zebras, too, though. The rare disease community is always growing, but these uncommon conditions can sometimes be difficult to identify. It’s always important to embrace your stripes.

These **5 FACTS** are a good place to start.

FACT 1

<200,000

In the United States, a disease or disorder is defined as “rare” when it affects fewer than 200,000 Americans at any given time.

FACT 2

Common symptoms can mask underlying rare diseases and lead to misdiagnosis and treatment delay.

FACT 3

50%

of rare diseases affect children.

FACT 4

The lack of scientific knowledge and quality information on a rare disease often delays a correct diagnosis and can result in heavy burdens, both social and financial, on patients and their families.

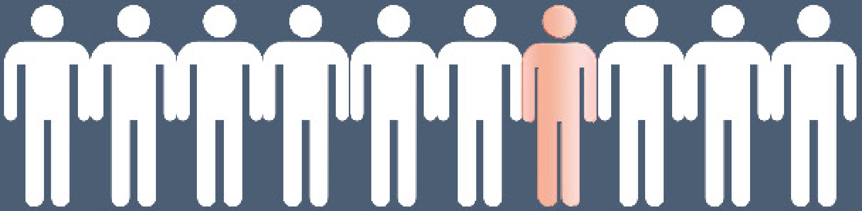
FACT 5

80%

of rare diseases have identified genetic origins, while others result from infections (bacterial or viral), allergies, and environmental causes or are degenerative and proliferative.

RARE DISEASES ARE MORE COMMON THAT YOU THINK!

1 in 10



Americans has a rare disease **+ 30 MILLION PEOPLE**

Globally, **50% of those living with a rare disease are CHILDREN**

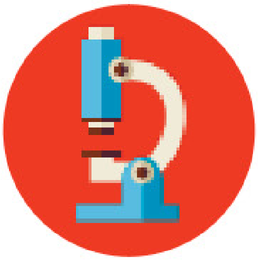
If all people affected by a rare disease lived in one country, this would make up the world's third-most populous nation - equivalent to the total US population

This means more than 300 million people around the world have a rare diseases - approximately the same number of monthyly users of Instagram

80%

of Rare Diseases are Genetically Based

Many Rare Diseases result in premature death of infants and young children or are FATAL in early adulthood



Families & private foundations provide about 3% of ALL medical research funding for Rare Diseases in the US

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 patients and families struggling with rare diseases 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 the zebra stripe ribbon because of the well-known medical expression.

The formation of the Nevada Rare Disease Advisory Council “the Council” was formed in 2019 session of the Nevada Legislature. The council was assigned the following 13 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
3. persons, the Division, community-based organizations, providers of health care and other local and national organizations whose work relates to rare diseases.
4. Increase awareness of the burden caused by rare diseases in Nevada.
5. Identify evidence-based strategies to prevent and control rare diseases.
6. Determine the effect of delayed or inappropriate treatment on the quality of life for patients suffering from rare diseases and the economy of Nevada.
7. 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.
8. Increase awareness among providers of health care of the symptoms of and care for patients with rare diseases.
9. 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.
10. Determine effective methods of collecting data concerning case of rare diseases in Nevada for the purpose of conducting epidemiological studies of rare diseases.
11. 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.
12. Develop a registry of rare diseases diagnosed in Nevada to determine the genetic and environmental factors that contribute to such rare diseases; and
13. Compile an annual report that includes a summary of the council’s activities and any recommendations of the council for legislation or other policies.
14. 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 a 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

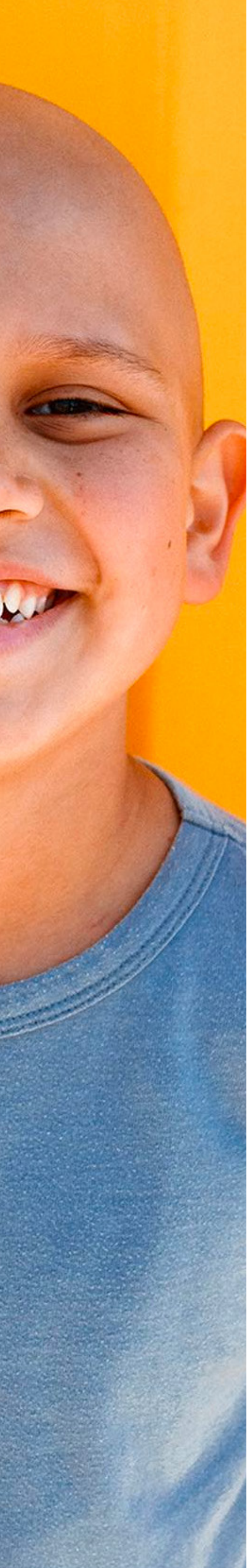
The NV-RDAC was formed under SB-315 during the 2019 session of the Nevada Legislature. The council was assigned several specific duties that range from performing a statistical and qualitative examination of the incidence, causes, and economic burden of rare disease in Nevada; including the development 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 the 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.

Unfortunately, as of yet, the state of Nevada has not provisioned their RDAC with the required resources to be effective in their efforts. We are hopeful that the Nevada DMV Specialized License Plate for childhood cancer initiatives will eventually grow to become a significant resource for the RDAC. For more information, please scan the QR code to the right to learn more about the license plate sales.

Scan to View
or click HERE

	Total Fees	
Numbering	Initial	Renewal
Sequential	$61	$30
Personalized	$96	$50

- Sequential plates are in stock at DMV offices.
- **Order personalized plates online**, or
- Use the **Application (SP 66)** **(PDF)** for in-person, mail or fax orders.



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.

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 NV-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.

If you are interested in being considered for a future Council Member appointment to the RDAC, contact Ashlyn Torrez by phone 775-447-0263, by email at atorrez@health.nv.gov, or by mail at: 4150 Technology Way, Ste. 300, Carson City, NV 89706.

Please submit a letter of interest and a resume or curriculum vitae. Materials submitted will be forwarded to the DHHS director or consideration for possible appointment.

Scan to View
or click HERE

NV-RDAC To Date:

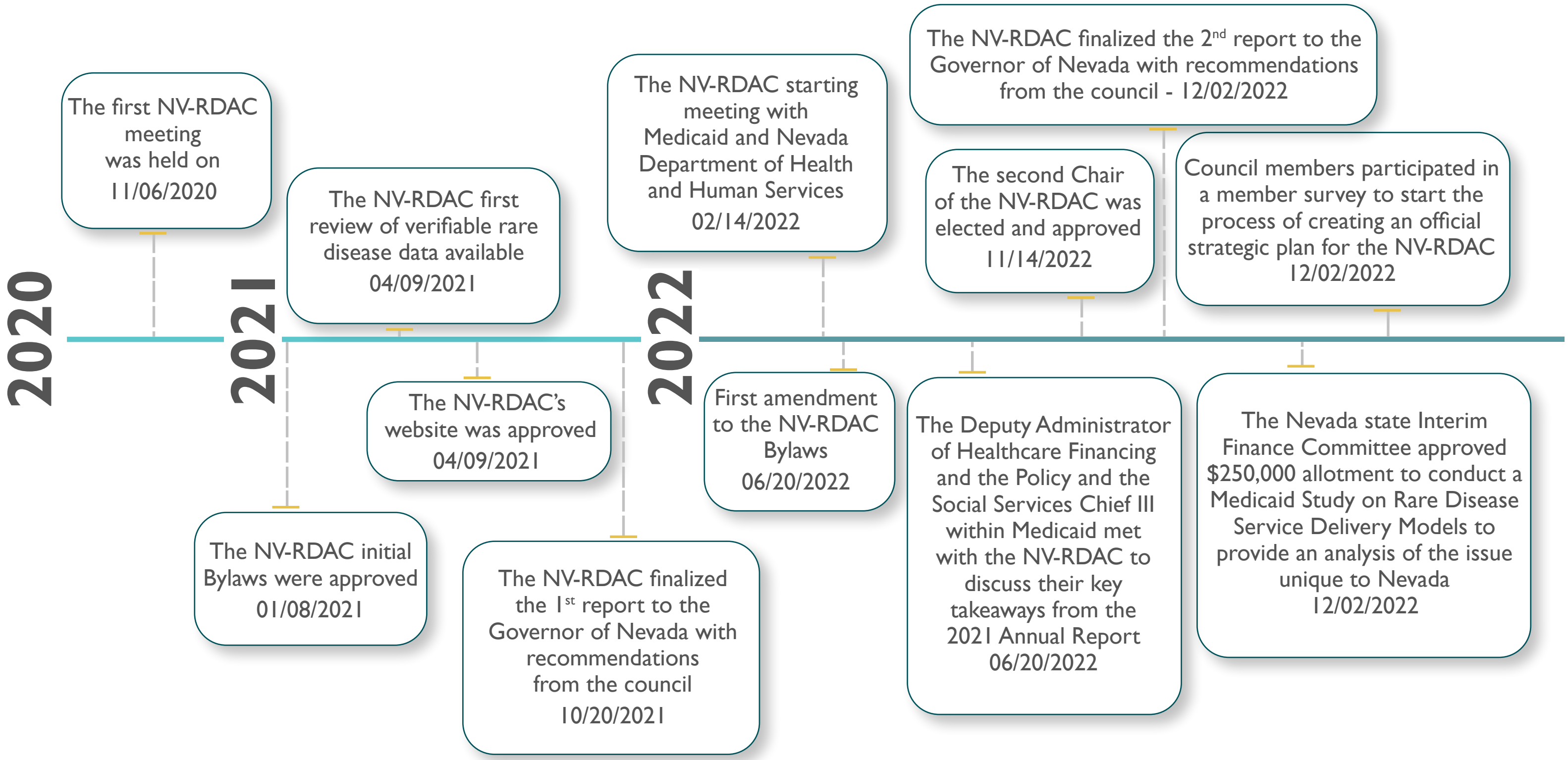
The Nevada Rare Disease Advisory Council has been tasked with several important deliverables, a few of them are as follows:

1. The development of a statewide registry of rare diseases diagnosed in Nevada to help the state better understand the prevalence of are 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.

NV-RDAC Timeline



2020

The first NV-RDAC meeting was held on 11/06/2020

2021

The NV-RDAC initial Bylaws were approved 01/08/2021

The NV-RDAC first review of verifiable rare disease data available 04/09/2021

The NV-RDAC's website was approved 04/09/2021

The NV-RDAC finalized the 1st report to the Governor of Nevada with recommendations from the council 10/20/2021

2022

The NV-RDAC starting meeting with Medicaid and Nevada Department of Health and Human Services 02/14/2022

First amendment to the NV-RDAC Bylaws 06/20/2022

The Deputy Administrator of Healthcare Financing and the Policy and the Social Services Chief III within Medicaid met with the NV-RDAC to discuss their key takeaways from the 2021 Annual Report 06/20/2022

The second Chair of the NV-RDAC was elected and approved 11/14/2022

The NV-RDAC finalized the 2nd report to the Governor of Nevada with recommendations from the council - 12/02/2022

Council members participated in a member survey to start the process of creating an official strategic plan for the NV-RDAC 12/02/2022

The Nevada state Interim Finance Committee approved $250,000 allotment to conduct a Medicaid Study on Rare Disease Service Delivery Models to provide an analysis of the issue unique to Nevada 12/02/2022

Current Rare Disease Areas of Focus

The NV-RDAC voted in December of 2020, that their initial focus would be on three primary categories of rare diseases:

1. All Childhood Cancers
2. Factor deficiencies and inherited platelet disorders
3. Newborn screening conditions- currently, the newborn screening in Nevada screens for 46 conditions

Scan to View
or click HERE

2023

Council members began the process of drafting the st NV-RDAC Strategic Plan
02/13/2023

The NV-RDAC added 2 additional meetings to the annual meeting schedule
02/13/2023

The NV-RDAC approved the development of a statewide Rare Disease Needs Assessment
02/13/2023

AB471, AB155, & SB194 - Currently being monitored by the NV-RDAC
03/2023

The NV-RDAC introduced their initial Awareness & Education 'While You Wait' campaign
02/13/2023

2023 World Rare Disease Day The NV-RDAC was showcased on the local news for the first time
02/28/2023

SB221, SB255, & SB194 - Introduced to the Health and Human Services Commitee and currently being monitored by the NV-RDAC
03/2023

Approval of the final questions for the NV-RDAC Needs Assessment and the platform to be used will be REDcap
04/07/2023

Council members approved the 1st draft of NV-RDAC Strategic Plan
06/02/2023

Preview and anticipated approval of the Comprehensive Statewide Rare Disease Needs Assessment Platform
08/04/2023

The NV-RDAC previewed the Awareness & Education 'While You Wait' campaign collateral
10/06/2023

Draft the NV-RDAC 3rd report for the Governor of Nevada with recommendations from the council
12/02/2023

2024

Launching the Comprehensive Statewide Rare Disease Needs Assessment
01/2024

Launching the Awareness & Education 'While You Wait' campaign statewide
01/2024

Anticipated

Preparing for this Strategic Plan

The Nevada Rare Disease Council members participated in an anonymous survey to gain perspectives on where their limited resources should be applied. The survey confirmed that the members of the NV-RDAC are not only engaged and willing to serve the state of Nevada on behalf of people with rare diseases, but they are also equally aligned in their personal and collective agendas to improve the overall landscape of rare disease management in Nevada. This includes 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 lawmakers to create new and improved regulations that positively impact people with rare diseases and the families who love them.

The survey instrument that the NV-RDAC council members used placed the 13 duties assigned to them into 6 different categories. The council members then ranked them in order of priority based on their current capabilities. The survey determined that the top priorities should be as follows, in the order of importance:

1) Nevada-Specific Data - Collection & Analysis
2) NV-RDAC Awareness & Education Campaigns
3) The Establishment of a Comprehensive Rare Disease Plan for Nevada
4) Health Equity and Disparities that Impact people with Rare Diseases
5) Leveraging the National Rare Disease Advisory Council for Efficiencies and Effectiveness
6) The Establishment of a Statewide Rare Disease Needs Assessment

Scan the QR code to the right to view the summary of findings of the survey in the 02/13/2023 Meeting Minute attachments

Scan to View
or click HERE

Moving The Plan Forward

This strategic plan is the first statewide coordinated effort to address rare diseases in Nevada. Due to the great complexity of rare disease, the significant unmet needs, and the critical urgency associated with rare diseases, systematic reform is required.

While there are many rare diseases, they share countless commonalities. Informed by extensive stakeholder consultation through the 2022 council member survey, this strategic plan addresses this common ground. It represents the views of the rare disease sector and outlines the initial comprehensive, collaborative, and data-informed approach required to achieve the best possible health and well-being outcomes for people living with rare diseases in Nevada.

The NV-RDAC strategic plan is built upon the following 3 Pillars

- **Awareness and Education**
- **Care and Support**
- **Research and Data**

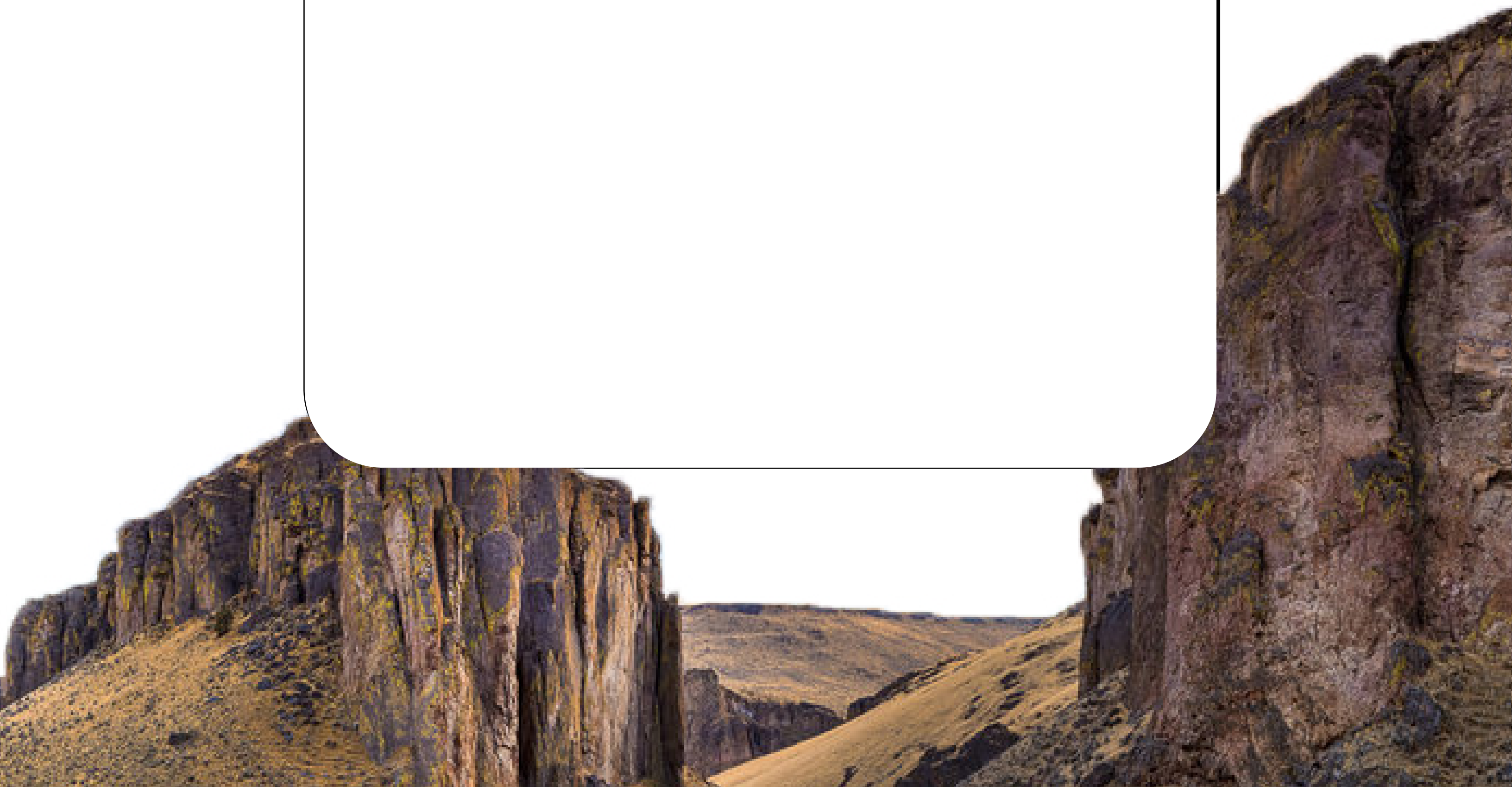
These pillars are easily recognizable to people living with rare diseases as well as the healthcare professionals providing services for them. For the purpose of this strategic plan, each pillar is presented seperately, yet is important to recognize that they are all interrelated and go hand-in-hand. As such, it is critical for us to address the priorities of each individual pillar while recognizing that reform and change to one pillar will create change and momentum in the others.

The importance of statewide leadership and coordination in rare diseases cannot be underestimated. It is going to require collaborative relationships, multi-stakeholder involvement and engagement, as well as shared governance and oversight.

This NV-RDAC strategic plan is critical in aligning the council's activities moving into the next three years. The council will continue to grow and evolve. We will be faced with new challenges and opportunities that will take priority. However, the pillars of this plan will remain the foundation of our collective efforts moving forward with clear goals and objectives.

NV-RDAC Pillars of Success

AWARENESS & EDUCATION	CARE & SUPPORT	RESEARCH & DATA
• Create and Develop NV-RDAC's Awareness & Education Campaign	• Establish continuity of care for people living with a rare disease in Nevada through the creation of a comprehensive rare disease plan for the state.	• Develop a Nevada-Specific Data Collection & Analysis Platform
• Leverage the network of Rare Disease Advisory Councils for Efficiencies and Effectiveness	• Address Health Equity and Disparities that Impact People with Rare Diseases	• Launch a Comprehensive Statewide Rare Disease Needs Assessment



1 Our Approach to **Awareness & Education**

What are we hoping to achieve?

1. Increase every Nevadan's awareness of rare diseases and education on the resources available.

2. Ensure Nevadans living with a rare disease have access to information and education that empowers them to be active participants in their rare disease journey.

3. Develop a statewide rare disease workforce strategy that responds to current and future demands; including the impact of genomics and artificial intelligence.

Rare Disease, Genomics, and the Future...We need to Get Ready!

The Diagnostic Odyssey- A search for answers that can last years and involve many different tests and consultations with clinicians across multiple specialties. Even if a diagnosis is reached, there is often no treatment. Genomics can offer a long-awaited diagnosis that can bring some certainty and understanding where before there was none. Over 80% of rare diseases currently have a known genetic origin.

Fortunately, there is a great opportunity to understand the cause of each rare disease and to provide an accurate diagnosis for each patient. Genomics plays an important role with a notable impact on public health. Many emerging technologies appear to play an essential role in diagnosis and for patients who remain undiagnosed. This future looks bright- as long as state policy can keep up with science.

Whole genome sequencing from a single blood test can pick up 31% more cases of rare genetic disorders than standard tests, shortening the expensive and frustrating "Diagnostic Odyssey" [1]. The faster, cheaper generation of genomic data is driving the integration of genomics into all healthcare specialties. It is likely that within the next decade, many healthcare professionals will be using genomics to diagnose and manage their patients- this is expected to be the case with rare diseases.

Nevada is not prepared for this genomic based future. In order to keep up with scientific progress in the field of rare diseases, there are **three major actions** that we must take in Nevada:

1. Increased awareness of the state's actual incidence and burden of rare diseases
2. Accessible and equitable genetic testing for all Nevada's- new genomic technologies are transforming healthcare.
3. State policy that requires Medicaid and commercial insurance coverage for genetic testing; particularly for children - as 70% of rare disease start in childhood.

Increased awareness of various rare diseases would help clinicians quickly recognize symptoms and pursue a diagnosis. Free or low-cost genetic tests would eliminate the need to rule out multiple conditions and get a quicker diagnosis,. This could allow patients to access medications or investigational products, and, hopefully, slow or halt disease progression. Insurance coverage would help patients access potentially life-saving therapies.

Why is this Important?

Increased awareness and education at the individual and community level is important. It is common for people to be diagnosed with a rare disease that they and their family members never even knew existed. Due to the volume of rare diseases, it can be challenging to find a healthcare provider who is sufficiently experienced in the care of patients with rare diseases. Yet, the people diagnosed with a rare disease and the healthcare professionals providing the care are reliant on services and information for both the care and support of these diseases. Lack of awareness of rare diseases often contribute to people feeling isolated, misunderstood, and extremely frustrated. This lack of awareness also results in delays in diagnosis and treatment, potentially missing important opportunities for early intervention and improved outcomes [2].

While awareness is important, with over 7,000 known different rare diseases, it is impossible for any individual; including healthcare professionals to be aware of them all. Awareness activities must be supported by systematic identification, classification, and a prioritized response to rare diseases and undiagnosed rare diseases [3]. Therefore, it stands to reason, that people would not need to know everything about all rare diseases, but rather would need to know how to locate relevant information as it is needed. Rare disease data collection and use is further explored in Pillar 3.

Education about rare diseases needs to empower people living with a rare disease to become active participants in their rare disease journey. Education efforts need to appreciate the fact that people living with a rare disease are constantly learning, collaborating, and ultimately teaching each other, as well as healthcare professionals, about the nuances of these particular diseases.

There is also a serious need for concentrated education of the healthcare workforce that supports people living with rare diseases. We need to focus on increasing the healthcare industry's capacity to meet (and eventually exceed) the care and support requirements of people living with rare diseases. While this may apply to the entire healthcare workforce, there are certain segments of the workforce such as those providing mental health services, with known and urgent awareness and education needs.

1 Our Priorities and Actions
Awareness & Education

Priority One: **Create and Develop NV-RDAC's Awareness & Education Campaign**

Actions:

1. To create and launch our 'While You Wait' campaign (in English and Spanish). This campaign is a suite of collateral materials to raise awareness of the NV-RDAC to be sent out to all relevant provider offices statewide. The intention is to provide awareness and educational materials to a variety of Nevadans while they are waiting in their healthcare provider's office. This material will be available for a wide variety of audiences and appropriate for a range of specialties. This also allows us to seek participants for the Rare Disease Needs Assessment, which will link Pillars 1 and 3.
2. Create a Media Campaign using a variety of social media channels (Facebook, Instagram, and Linkedin) as well as an in-kind/sponsored traditional news and print campaign (magazines and newspaper) to bring awareness of the NV-RDAC, the important work we are doing, and how to support the NV-RDAC initiatives.

3. Develop an awareness and education program that empowers frontline heatlhcare professionals to provide culturally safe and appropriate medical care to support all Nevadans living with a rare disease. While also Identifing existing gaps in the workforce that provide medical services to people living with rare diseases and outline a path towards a sustainable workforce, taking into account the impact of genomics.
4. Expand the capabilities of the NV-RDAC website. In order to develop and promote the use of an easily accessable multi-purpose digital repository for both healthcare professionals and people living with rare diseases.
    - Healthcare professionals: In order to support these healthcare professionals to consider a rare disease diagnosis when people present with unexplainable symptoms.
    - People with rare diseases: In order to support them and their families to identify, not only appropriate info but also who are the professionals in the state of Nevada who can treat them.
5. Promote through education and awareness efforts the specific needs of people with a life-limiting rare disease and the importance of providing thoughtful and appropriate end-of-life care and support services.

Priority Two: **Leverage the network of Rare Disease Advisory Councils for Efficiencies and Effectiveness**

Actions:

1. Ensure collaboration and consultation occurs between Nevada state policy & lawmakers with rare disease stakeholders including education providers, healthcare providers, payers, and community-based nonprofit organizations.

2. Capitalize on the existing multi-purpose digital resources already available nationally and internationally in order to bridge the available information on the NV-RDAC website. Ensuring the ease of access of information to both the people living with rare diseases as well as the healthcare workforce providing them with medical services.

3. Build on existing activities of local and national rare disease organizations to raise awareness of care and support services available to people living with rare diseases in Nevada and identify gaps and opportunities for improvement.

4. Strengthen the connection with state lawmakers and both the local rare disease community and the healthcare professionals providing care to them. Improve communication on a range of issues that people with rare diseases face in Nevada.

5. Survey all existing RDACS and NORD to gain an understanding on how they are addressing genomics and artificial intelligence in order to determine next steps for the NV-RDAC.

Artificial Intelligence in Healthcare... What to Expect

The complexity and rise of data in healthcare mean that artificial intelligence (AI) will increasingly be applied within the field, this application is likely to impact rare disease management in significant ways. Several types of AI are already being employed by payers and providers of care, and life sciences companies. The key categories of applications involve diagnosis and treatment recommendations, patient engagement and adherence, and administrative activities.

The members of the NV-RDAC believe that AI has an important role to play in the healthcare offerings of the future. In the form of machine learning, it is the primary capability behind the development of precision medicine; widely agreed to be sorely needed in the advancement of care- specifically in the field of rare diseases. It is true that early efforts at providing diagnosis and treatment recommendations have proven challenging, yet we do expect that AI will ultimately master that domain. Given the rapid advances in AI for imaging analysis, it seems likely that most radiology and pathology images will be examined at some point by a machine. Among the many challenges we face in the rare disease community; some of the most difficult things to navigate included how rare diseases often camouflage themselves as other common diseases, coupled with a systemic lack of access to data or other clinicians the patients see, and a lack of familiarity with extremely uncommon conditions. AI can now reveal patterns across huge amounts of data that are too subtle or volumes for people to detect and could therefore be a real game changer in these areas of challenge.

We believe that the most significant challenge to AI in the healthcare domain is not whether the technologies will be capable enough to be useful, but rather ensuring their adoption in daily clinical practice is done in an ethical and thoughtful manner. For widespread adoption to take place, AI systems must be approved by regulators, integrated with EHR systems, standardized to a sufficient degree that similar products work in a similar fashion, taught to clinicians, paid for by public or private payer organizations, and updated over time in the field. These challenges will ultimately need to be overcome, but they will take much longer to do so than it will take for the technologies themselves to mature. As a result, we must get ready for a new reality in healthcare.

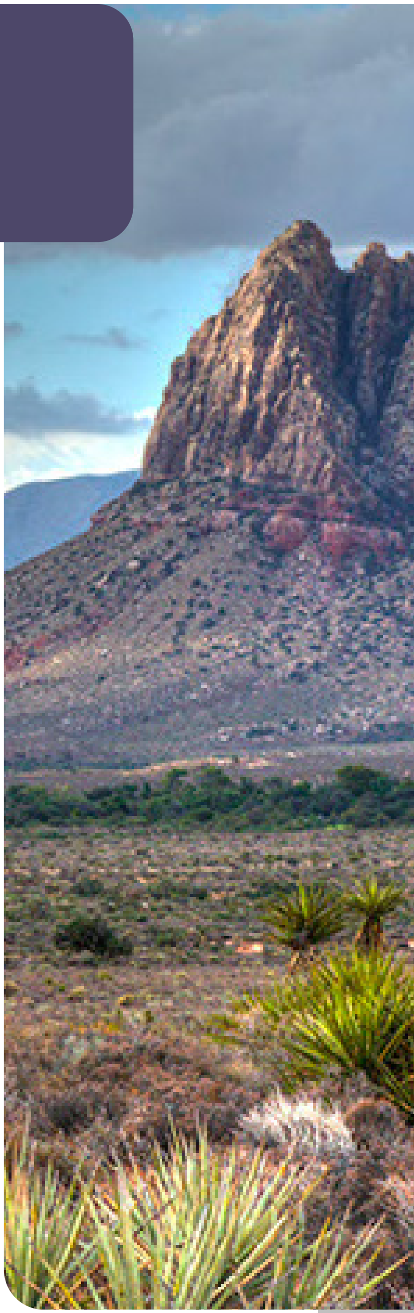
Why is this Important?

The objective of using technology, i.e., AI in healthcare is to improve outcomes and quality of care, and we need to remember that this may not be strictly aligned with the statistical performance of any given AI model. There is no doubt that AI will have widespread ramifications that revolutionize the practice of medicine, transforming the patient experience and physicians' daily routines. Yet, we do run the risk of over-reliance on metrics in diagnostic AI that may deepen hidden biases embedded within the dataset and exaggerate unequal health outcomes based on socio-economic factors- which could devastate the diagnostic process for people with rare diseases. Balancing the benefits and the risks of AI in the diagnoses of rare diseases needs to be at the forefront of this important conversation.

The NV-RDAC recommends that we work together to get ready for AI in the rare disease arena by focusing on the following key elements:

- Artificial intelligence, although a powerful and exciting technology, does create a novel set of ethical challenges that must be identified and mitigated.
- Ensuring that the healthcare AI security and privacy measures become a priority within the state of Nevada.

2 Our Approach to Care & Support



What are we hoping to achieve?

1. Influence improved care and support that is integrated and appropriate for all Nevadans living with a rare disease.

2. Set the standard that diagnosis of a rare disease in Nevada is timely and accurate while ensuring Nevadans have equitable access to qualified healthcare and the best treatment options.

3. Create awareness and educational opportunities that inspire integration of mental health, and social & emotional well-being into rare disease care and support.

Why is this Important?

Early diagnosis enables the best clinical care, treatment options, access to services, peer support, increased reproductive confidence and access to participation in clinical trials. Yet, diagnostic delay and misdiagnosis is common in rare diseases.

It has been reported that at large number of people living with a rare disease worldwide are impacted by a diagnostic delay of more than five years, while almost half have received at least one misdiagnosis [4]. Currently, Nevada-specific statistics in diagnostic delay and misdiagnosis are unknown. It is believed by the members of the NV-RDAC that it is highly likely that Nevadans experience the same delay and misdiagnosis as seen around the world. Rare diseases data collection and use is explored further in Pillar 3.

Significant delays in diagnosis as well as experiencing misdiagnosis have physical, psychological, emotional and financial costs for the person and family living with a rare disease. There is a severe need for rare disease care and support to be less fragmented and more integrated. The medical care and community support required for people living with a rare disease needs to be both personal and family-centered. People with a rare disease often require large interdisciplinary teams of doctors, nurses and allied health professionals who work in different settings (primary care, hospital, emergency departments, community allied health) to manage the multiple medical problems and disabilities experienced. Their health needs can change throughout the course of their lifetime, and these changes can be rapid and critical. Additionally, they often have complex support needs that extend beyond healthcare that include disability, social/welfare, mental health, education,

employment and housing. It is not just the person living with a rare disease who needs support. Their family and the healthcare professionals also have high and significant support needs.

Rare disease care and support should meet the needs of patients and the healthcare professionals while taking into account local contextual factors, such as existing services and structures; resources, including funding and workforce; workforce expertise, and the preferences of people living with a rare disease and their families and providers.

Living with a rare disease does not only affect a person's health; it impacts every facet of their life, including education, employment, as well as mental & physical health. For example, it is currently difficult for people living with a rare disease to navigate their way through the health and disability systems as there is a lack of clear referral pathways and clinical coordination.

Valuable care and support are provided by tax-exempt, rare disease organizations both nationally and locally. This includes peer support, the provision of information, access to resources, and individual and systemic advocacy. Many rare disease organizations are run by people living with their own rare disease challenges, which can leave them vulnerable and affect their ability to offer services over the longrun (including awareness, education, care, support and research). The state of Nevada has an opportunity to work directly with the NV-RDAC to identify soluations that could result in the stabilization of this fragile yet critically important support system, that people living with a rare diseases access.

There are limited treatment options for rare diseases, and even when a treatment does exist, financial support may not be available in Nevada and thus accessibility may be limited. Reimbursement of health technologies for rare diseases, using models designed for more common diseases, is challenging as smaller patient numbers impact cost effectiveness, and there is often less clinical evidence available due to the challenges of conducting large-scale clinical trials. This highlights the importance of alternative approaches to both identifying and funding the best possible treatment options.

There are many examples of an approved medicine (for a more common condition) that demonstrate benefits for rare diseases. However, due to small numbers, it is not always commercially viable for companies to seek reimbursement for a rare disease indication. Without government reimbursement, many rare disease medicines are unaffordable. As many rare diseases are progressive, time is often critical, making timely and equitable reimbursement essential for people living with a rare disease so that they may benefit from new and transformative treatment options.

2 Our Priorities and Actions
Care & Support

Priority One: Establish continuity of care for people living with a rare disease in Nevada through the creation of a comprehensive rare disease plan for the state.

NV-RDAC is focused on establishing a comprehensive statewide strategy to deliver safe, sustainable, world-class medical care for people living with a rare disease in Nevada. The goal is for all Nevadans to receive the correct diagnosis and the most current and medically appropriate treatment provided by a team of highly qualified and skilled professionals. All while ensuring access (if appropriate) to participation in any rare disease clinical trial, for which they are eligible.

Actions:

1. Educate Nevada lawmakers on the importance of building a broad range of care and support services that are responsive to the unique needs of people living with a rare disease.

2. Influence Medicaid and commercial payers in Nevada to use published best practices for the standardization of available care and support for people living with rare diseases. Ensuring these standards of care and support are integrated throughout the entire healthcare delivery system.

3. Work to reduce fragmented care, ensuring policy addresses the full range of needs of people living in Nevada with a rare disease; including healthcare, disability, housing and education.

4. Increase awareness among healthcare professionals, payers (Medicaid and commercial), as well as rare disease community-based organizations of the importance of palliative care in rare disease management. Palliative care can achieve a wide range of objectives for people and their families living with a rare disease; improving both the quality of life and thoughtful and appropriate end-of-life care.

The Right **Diagnosis**
\+ The Right **Treatment**
\+ The Right **Team**
\+ The Right **Environment** = **CONTINUITY OF CARE**

Priority Two: **Address Health Equity and Disparities that Impact People with Rare Diseases**

80% of rare diseases are genetically based, yet access to genetic testing and counseling is extremely limited and often completely unavailable to people with limited resources. The developement of a statewide standard of practice that ensures the timely access to appropriate genetic testing and genetic counseling, will result in healthcare professionals being able to quickly deliver high-quality care to many people with rare diseases.

The Diagnostic Odyssey which translates into the relentless persuit of diagnostic answers can take several years and often involves many different tests through consultations with clinicians across multiple specialties. This historically accepted practice is not only incredibly frustrating to people seeking diagnosis with rare diseases and the healthcare professionals that treat them, it is also unnecessary, extremely expensive, and leads to chronic financial toxicity.

Financial toxicity in healthcare, particularly in the rare disease sector has become a serious national topic and Nevada has not escaped the conversation. There is a common misconception that financial toxicity is simply the high cost of care, but it's more than that. Most families are pushed to the edge and often beyond their financial limits, and this still does not convey the full expenses that result from living with a rare disease.

Actions:

1. Influence the state of Nevada's policy to adequetely support people living with a rare disease to have timely and equitable access to both diagnostic opportunities as well as the most current scientifically-proven treatment options for their rare disease.

2. Work with lawmakers in the state of Nevada and payers including Medicaid and commercial to build a equitable and timely delivery of medically-appropriate and scientifically-proven services such as genetic testing (diagnostics) and gene therapies (treatments) as well as genetic counseling to Nevadans who are suspected of having a rare disease or those with an increased chance.

3. Encourage the state of Nevada to develop policy that ensures equitable access to appropiate genetic diagnostic testing, genetic counseling, and the resulting healthcare interventions for all Nevadans living with a rare disease or seeking answers for undiagnosed medical conditions.

4. Identify the statewide specific roots of financial toxicity for patients and families living with rare diseases in Nevada.

The single most important driver for the right diagnosis, right treatment, right team, and right environment **is defining and setting statewide standards of care** to achieve the very best clinical outcomes, while putting the needs of the patient and their families at the center of all decisions.

3 Our Approach to Research & Data

What are we hoping to achieve?

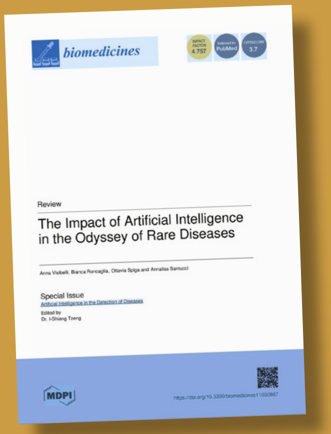
1. Establish coordinated and collaborative data collection to facilitate the monitoring and cumulative knowledge of rare diseases in Nevada, informing policy makers, payers, and healthcare professionals statewide.

2. Develop a statewide research strategy for rare diseases to foster, support, and drive all types of research for rare diseases while systematically addressing the identified gaps in care and support.

3. Influence the state of Nevada to recognize the unique needs of patients living with rare diseases using research and data to educate our lawmakers on the level of reform that is required for our state to adequetely ensure Nevadans have equitable access to qualified healthcare and the best treatment options.

4. Research the impact of artificial intelligence in the diagnostic odyssey of rare diseases in Nevada and include in the NV-RDAC annual report a set of recommendations for state officials to consider.

The Impact of Artificial Intelligence in the Odyssey of Rare Diseases



biomedicines

IMPACT FACTOR 4.757

Indexed in: PubMed

CITESCORE 3.7

Review

The Impact of Artificial Intelligence in the Odyssey of Rare Diseases

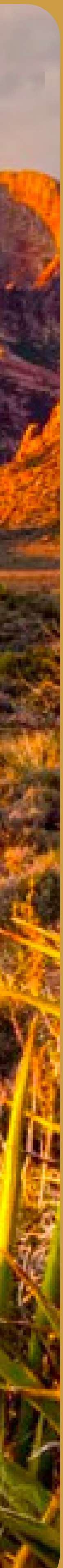
Anna Visibelli, Bianca Roncaglia, Ottavia Spiga and Annalisa Santucci

Special Issue
Artificial Intelligence in the Detection of Diseases
Edited by
Dr. I-Shiang Tzeng

MDPI

https://doi.org/10.3390/biomedicines11030887

Scan QR code
to view



Why is this Important?

In Nevada, data for most rare diseases is not captured in either health information systems or registries and there is no coordinated strategy to collect, measure, build and translate the data that does exist. The NV-RDAC has identified the need for a statewide, coordinated, and systematic approach to the collection and use of rare disease data, including registries. Such an approach will enable monitoring and the accumulation of knowledge about rare diseases to inform clinical practice, research and health policy planning.

For key decision-makers at all levels, greater knowledge of rare diseases can facilitate more responsive and appropriate services for people living with a rare disease. The NV-RDAC is eager to lead significant positive change in this space.

For many rare diseases, there are a number of barriers to effective research and often no active research programs at all. One of the biggest challenges is that rare diseases at the individual disease level have small patient numbers and are often very complex. When looking at rare diseases collectively, we can learn alot on how to improve the care and support that people living with rare diseases need. This is further explored in Pillar 2. Depending on the specific rare disease, research priorities can be different. For example, while funding for translational research may be important for some, other rare diseases are not yet in the position to be prioritized in translational research leaving unmet needs for basic discovery initiatives or investment into data collection and genomics.

For many people living with a rare disease, participation in a clinical trial may be the only way to access treatment. Estimates are as high as 90% of people living with a rare disease are interested in joining a patient registry, in recognition of the key role that registries play in linking people living with a rare disease with clinical trials for new treatment options [5].

The translation of rare disease research into clinical settings, while currently hampered, is vital. This two-way relationship benefits from active participation by patients, their families and their healthcare providers, and patient advocacy groups to ensure the best possible outcomes for people living with a rare disease.

There is an understanding in the rare disease community that, while research may not lead to better outcomes for people currently living with a rare disease, participation in research may drive change for future generations. This is supported by outcomes of the Rare Barometer survey undertaken in February 2018 by EURORDIS, Rare Diseases Europe [6].

Research into rare diseases must address existing gaps and the coordination of research projects must be prioritised. Improving policy settings and statewide collaborations, will help to drive strong research and innovation for all rare diseases in Nevada. Research into rare diseases needs to inform evidence-based policy across all systems, extending beyond healthcare to incorporate disability, social/welfare, mental health, education, employment and housing.

3 Our Priorities and Actions
Research & Data

Priority One: **Develop a Nevada-Specific Data Collection & Analysis Platform**

There is an urgent need for the expansion of rare disease expertise and further development of evidence-based rare disease care. iLmited data is a common feature in rare diseases. This is heightened by poor quality, disjointed collection methods and the ineffective use of data for rare diseases. Such limitations are evident across a range of areas, from health system classification to research. Research, monitoring and ongoing evaluation are critical in rare diseases because, ultimately, if we are not counting rare diseases, people living with rare diseases do not count.

Actions:

1. Establish a statewide community of stakeholders focused on rare disease data collection and research.

2. Improve rare disease data collection efforts to include a statewide approach to collecting data on the 3 focus groups of rare diseases. Childhood cancer, bleeding and NB screening.

3. Influence the state to undertake broad epidemiological surveillance of rare disease to support decisionmakers and lawmakers to access the information they need to inform the health and well-being of Nevada's living with rare disease.

4. Develop a statewide registry for rare diseases to foster, support and drive all types of research for rare disease.

5. Support collaborative research into rare disease in Nevada, and nationally, by investigating and promoting options that enable Nevadan's living with a rare disease to participate in clinical trials and other research activities.

Priority Two: **Launch a Comprehensive Statewide Rare Disease Needs Assessment**

The NV-RDAC is seeking survey participants to gain further information from individuals and their families and caretakers living with rare disease in the state of Nevada. The primary objective of this needs assessment is to gather insights and perspectives directly from Nevada residents to increase our understanding on the barriers to care. The findings of the needs assessment will advise on quality of care, or lack thereof, educational needs, diagnosis, and other factors related to living with a rare disease. We are anticipating on collecting data from the needs assessment for 24 months starting in January of 2024.

Actions:

1. Researched our peer groups and existing RDACs across the country to develop a series of appropriate questions for our needs assessment.

2. Building, through RedCap, the needs assessment survey instrument. The needs assessment is a collection of questions aimed toward data collections focused on gaps in care and opportunities for statewide improvement. Throught this data collection, we are intertwining initiatives in Pillars 3 and Pillar 1.

3. Launching the first comprehensive needs assessment survey to be available in provider offices as well as other available platforms statewide. Anticipated launch date is January 2024.

4. Showcasing our findings on an annual basis through our required NR-RDAC Annual Report to the Governor's office.

5. Champion transformative healthcare policy development through publishing our findings for public review. While also ensuring all Nevada lawmakers, Nevada Medicaid leadership, and relevant commercial payers understand how to use this information to not only improve the lives of Nevadans living with rare diseases but to also understand and address the health inequities and disparities that impact this patient population.

Summary & Call to Action

We face many challenges in the state of Nevada when it comes to the fight against rare diseases. One of the more significant challenges is the lack of statewide coordination. There are many different organizations and individuals supporting these individuals and their families within the state. Even with all the effort and good work being done for them, there is essentially little coordinated effort between southern and northern Nevada. Working together as a united front would allow us to harness our collective efforts and capitalize on our combined strengths and resources to bring a significant amount of influence to our state leaders and lawmakers to help us advance the fight against these terrible diseases.

This strategic plan is our call to action. In this document, we have taken you through the NV-RDAC's current initiatives and outlined our focus through 2025. This document provides an outline of the minimum requirements to bring the state of Nevada to acceptable standards for the care and support to the people living with rare diseases.

Our plea to the leaders and lawmakers of Nevada is to support a meaningful statewide action plan that addresses the unique needs of people living with rare diseases, thier families, and the heathcare professionals that care for them. A coordinated effort and statewide strategy is the only way for Nevada to influence the type of policy change required to meet the needs of the people living with a rare disease.