



NVRDAC

Rare Disease Advisory Council

ANNUAL REPORT 2023

www.nvrdac.org

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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 SB-315 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

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 SB 315, 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 members of the NV-RDAC collectively decided to focus initial efforts on three primary categories of rare diseases:

1. Childhood Cancers of All Types
2. Factor Deficiencies and Inherited Platelet Disorders
3. Newborn Screening Conditions - At present, Nevada's newborn screening program covers 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.

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

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 Senate Bill 315 (SB315), which provides a solid legislative framework for the NV-RDAC's mission and purpose.

Awareness & Education Campaign and Rare Disease 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 SB 315, 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.
- **SB 194:** Revises provisions relating to step therapy protocols. Passed on June 1, 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. Ultimately, SB221 serves as a resounding victory for the rare disease community in the state, significantly enhancing their access to the care and support they urgently need.

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 Efforts

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.

Conclusion and 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 SB 315

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 SB 315. 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.

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, and 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 SB 315, 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 to Governor Lombardo and the state of Nevada include:

1. **Allocate Adequate Funding:** Ensure that appropriate and realistic funding is allocated to support the staffing required for the development and implementation of a comprehensive statewide rare disease strategy in Nevada. Adequate funding is essential for the success of these initiatives.

2. **Advocate for Policy Changes:** Actively engage with and encourage Nevada lawmakers to champion advocacy efforts at the state level. These efforts should focus on bringing about policy changes that improve insurance coverage, enhance access to new and novel diagnostic testing and progressive treatment options, as well as expand support services for individuals living with rare diseases in the state.
3. **Outsource Rare Disease Data Collection and Analysis:** Recognizing deficiencies in the current data collection efforts, allocate resources to outsource the enhancement of data collection and analysis methods. This outsourcing should prioritize obtaining precise information about rare disease prevalence, treatment effectiveness, and the experiences of patients in Nevada. Outsourcing can bring the expertise and resources needed to improve data quality and analysis.

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

