



Rare Disease Advisory Council

Legislative Report

July 1, 2022

Joseph A. Ladapo, MD, PhD

State Surgeon General

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Executive Summary

The Florida Department of Health (FDOH), Division of Community Health Promotion is pleased to present the inaugural annual legislative report of the Rare Disease Advisory Council (RDAC) in accordance with section 381.99, Florida Statutes.

In June 2021, the RDAC was created adjunct to the FDOH. The RDAC is composed of representatives from state agencies, health care providers, researchers, advocacy groups, insurance and pharmaceutical industries, as well as individuals with rare diseases and caregivers of individuals with rare diseases. Council members hold a shared vision: to improve health outcomes for individuals residing in Florida who have rare diseases.

This inaugural report describes the accomplishments of the RDAC over the past year and the council's recommendations. This report will also help guide the RDAC and direct the council's operations in the years to come. The development of council recommendations was assigned to the council's three subcommittees: the Academic Research Institutions Subcommittee, the Health Care Providers Subcommittee, and the State Agencies Subcommittee. The subcommittees have developed their recommendations through consensus during this planning year. Each recommendation is linked to a set of ambitious, yet achievable, objectives. The objectives are set to be accomplished over the next three years.

Mission:

To protect, promote & improve the health of all people in Florida through integrated state, county & community efforts.



Ron DeSantis
Governor

Joseph A. Ladapo, MD, PhD
State Surgeon General

Vision: To be the Healthiest State in the Nation

July 1, 2022

On behalf of the members of the Florida Rare Disease Advisory Council (RDAC), it is my pleasure to submit the first annual legislative report as outlined in section 381.99, Florida Statutes. This inaugural report summarizes the collaborative efforts of the RDAC over the past year and outlines the recommendations developed by the council on ways to improve health outcomes for Floridians who have a rare disease.

While each rare disease is different, the obstacles faced by individuals living with a rare disease and their families are common. The RDAC seeks to identify barriers and address the unique needs of the rare disease community by improving access to care, education, and other essential resources. Additionally, the RDAC seeks to encourage research on rare diseases in Florida.

Sincerely,

Melissa Jordan, MS, MPH
Chair, Rare Disease Advisory Council
Assistant Deputy Secretary for Health
Florida Department of Health

Council Membership

A representative of the Office of Insurance Regulation:

David Altmaier
Insurance Commissioner
Florida Office of Insurance Regulation

A physician who is licensed under Chapter 458 or Chapter 459, Florida Statutes, and practicing in this state with experience in treating rare diseases:

Barry Byrne, MD, PhD
Director, Powell Gene Therapy Center
Associate Chair, Department of Pediatrics
Professor, Pediatrics and Molecular Genetics & Microbiology
University of Florida

A representative of the Agency for Health Care Administration:

Ann Dalton, MM
Bureau Chief, Bureau of Medicaid Policy
Florida Agency for Healthcare Administration

A representative of organizations in this state which provide care or other support to individuals with rare diseases:

Anita Davis, PT, DPT, FNCP, CNPT
Physical Therapist
Brooks Rehabilitation

A representative of organizations in this state which provide care or other support to individuals with rare diseases:

Rebekah Dorr
Director of Clinical Patient Advocacy
Myasthenia Gravis Hope Foundation

An individual who is, or was previously, a caregiver for individuals with a rare disease:

Zana Dupee, JD
Caregiver

Council Membership

A representative of the biotechnology industry:

Jonathan Hawayek, MBA
Head of State Government Affairs
Spark Therapeutics, Inc.

A representative of the Department of Education:

Kathryn Hebda, MM - Council Vice Chair
Chancellor, Florida College System
Florida Department of Education

A representative of the Department of Health:

Melissa Jordan, MS, MPH - Council Chair
Assistant Deputy Secretary for Health
Florida Department of Health

A representative of health insurance companies:

Scott McClelland, PharmD
Vice President, Commercial and
Specialty Pharmacy Programs and
Health Care Solutions
Florida Blue

An individual who is 18 years of age or older who has a rare disease:

Jessica O'Reilly, JD
Self-Advocate

A representative from an academic research institution in Florida, which receives grant funding for research regarding rare diseases:

Divya Patel, DO, MBA
Program Director, Interstitial Lung Disease
Program Director, Sarcoidosis
Associate Professor of Medicine
University of Florida

An individual who is 18 years of age or older who has a rare disease:

India Steinbaugh, MPH
Self-Advocate

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Jennifer Sutherland
Caregiver

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Mustafa Tekin, MD
Division Director, Clinical and Translational Genetics
University of Miami

A physician who is licensed under Chapter 458 or Chapter 459, Florida Statutes, and practicing in this state with experience in treating rare diseases:

Rajan Wadhawan, MD, MMM
Senior Executive Officer, AdventHealth
for Children and AdventHealth for Women

An individual who is, or was previously, a caregiver for individuals with a rare disease:

Vacant

An individual who is, or was previously, a caregiver for individuals with a rare disease:

Vacant

A pharmacist who is licensed and practicing in this state who has experience with drugs that are used in the treatment of rare diseases:

Vacant

One geneticist practicing in this state:

Vacant

One registered nurse or advanced practice registered nurse who is licensed and practicing in this state with experience in treating rare diseases:

Vacant

One hospital administrator from a hospital in this state which provides care to individuals diagnosed with rare diseases:

Vacant

Introduction

The Orphan Drug Act defines a rare disease as a disease or condition that affects fewer than 200,000 individuals in the United States.¹ According to the National Institutes of Health, the total number of Americans living with rare diseases is estimated to be between 25 to 30 million individuals, or approximately 10 percent of the total national population.² This estimate has been used by the rare disease community for several decades to highlight that while individual diseases may be rare, the total number of individuals living with rare diseases is large.

Rare diseases include genetic disorders, infectious diseases, cancers, and other various pediatric and adult conditions.² Rare disease can affect anyone at any point within the lifespan. Rare diseases can be acute or chronic and are characterized by the variation of symptoms not only displayed from one rare disease to another, but also among individuals who are diagnosed with the same disease. Although the features of specific rare diseases can differ in a myriad of ways, the effects on life and functioning are often similar for affected individuals and their families. Individuals who live with rare diseases may face reduced quality of life due to the lack or loss of autonomy caused by chronic, progressive, degenerative, and life-threatening aspects of many rare diseases.

Rare diseases have many different causes. It is believed that 80 percent or more of rare diseases are genetic.³ For genetic rare diseases, genetic testing is often the only way a definite diagnosis can be made. Research shows genetic rare diseases are directly caused by a variant, or change, in a gene or chromosome. Variants can be inherited from a parent or occur during an individual's lifetime.⁴ There are a number of rare diseases that do not have a known genetic cause. These include some types of infections, autoimmune diseases, certain forms of cancer, diseases caused by exposure to toxins or other environmental factors, and some conditions which have multiple possible causes.⁵ For specific rare diseases that have been named and characterized for decades, investigators still have not determined a cause. However, many rare diseases with reasonably well understood causes and mechanisms still lack effective treatments or preventive strategies.

1) H.R.5238 - 97th Congress (1981-1982): Orphan Drug Act. (1983, January 4). Retrieved from <https://www.congress.gov/bill/97th-congress/house-bill/5238>

2) U.S. Department of Health and Human Services. (2021, January 26).

FAQs About Rare Diseases. Genetic and Rare Diseases Information Center. Retrieved from <https://rarediseases.info.nih.gov/about>

3) Rare Genetic Diseases. Genome.gov. (2018, April 13). Retrieved from <https://www.genome.gov/dna-day/15-ways/rare-genetic-diseases>

4) U.S. National Library of Medicine. (n.d.). What is a gene variant and how do variants occur? MedlinePlus Genetics. MedlinePlus. Retrieved from <https://medlineplus.gov/genetics/understanding/mutationsanddisorders/genemutation/?msclkid=ba654498cfd11ec9fb750b3c3c476aa>

5) Field, M. J., & Boat, T. F. (2010). Profile of Rare Diseases. In *Rare Diseases and Orphan Products: Accelerating Research and Development*. essay, National Academies Press. Retrieved from <https://www.ncbi.nlm.nih.gov/books/NBK56184/?msclkid=6d5bce28d07c11ec85146bea249c2fa3>.

Introduction

Rare diseases present fundamentally different challenges from those of more common diseases. Individuals with undiagnosed genetic diseases often face an uncertain and unpredictable journey, referred to as a "diagnostic odyssey." For one-third of individuals with rare diseases, getting an accurate diagnosis can take one to five years.⁶ Individuals with rare diseases often seek treatment in health care settings where their condition has never been seen before. Many health care providers are often unfamiliar with the multitude of symptoms that can be associated with rare diseases. Additionally, absent, masked, misunderstood, or confusing symptoms can contribute to a delayed diagnosis.

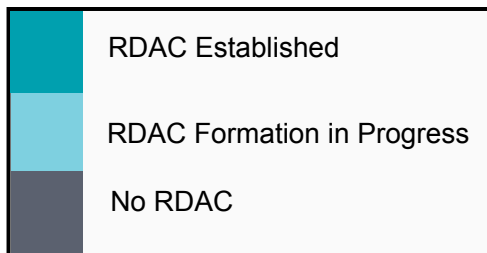
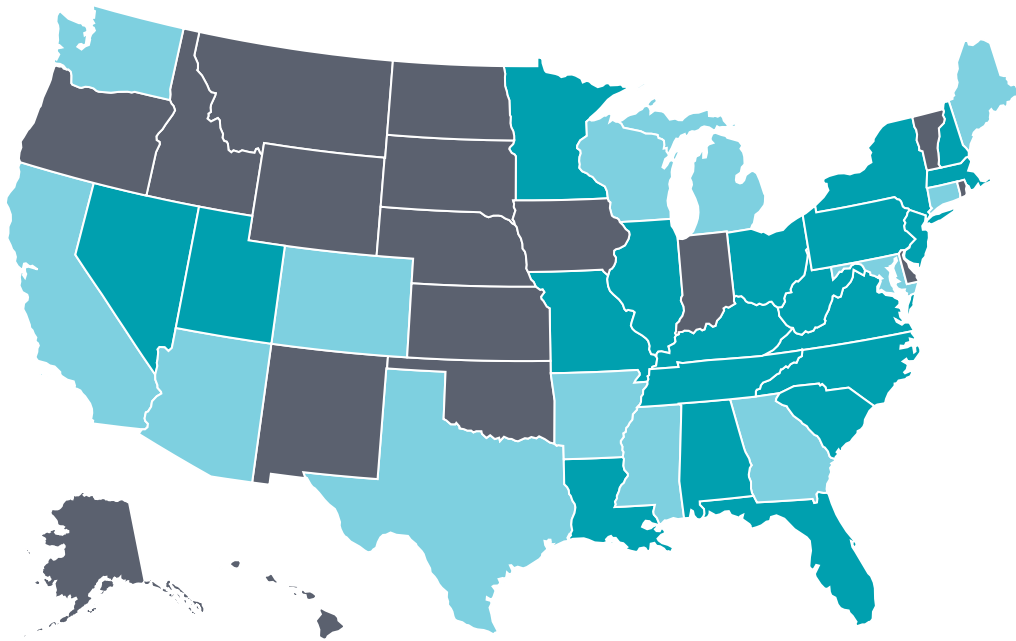
Achieving a timely and accurate diagnosis ensures that individuals living with rare diseases can receive the clinical care and counseling appropriate for their disease. A diagnosis also removes a significant psychological weight from those who have been struggling with a long "diagnostic odyssey." It allows individuals and their families affected by rare diseases the opportunity to connect with a rare disease community.

In the past decade, Florida has had considerable advancements in medicine and new treatment options for rare diseases. For example, the only drugs for Leber Congenital Amaurosis and Spinal Muscular Atrophy approved by the Food and Drug Administration were developed in Florida in 2017 and 2019, respectively. The RDAC wants to accelerate this progress. The RDACs hope is that the state of Florida will continue to be on the cutting edge of research protocols and treatments for Floridians living with rare diseases.

6) FDA.(2018, March 1). Orphan Products: Hope for People with Rare Diseases. U.S. Food and Drug Administration. Retrieved from <https://www.fda.gov/drugs/information-consumers-and-patients-drugs/orphan-products-hope-people-rare-diseases>

Background

On June 21, 2021, Senate Bill 272 was signed into law by Governor Ron DeSantis to establish section 381.99, Florida Statutes — Rare Disease Advisory Council. Florida became the 19th state to pass legislation creating an RDAC. Nationally, there are a total of 21 established RDACs, with several other states seeking to establish an RDAC in the future.⁷



Source: NORD, Project RDAC

7) *RDACs by State*. NORD (National Organization for Rare Disorders). (2022, January 25). Retrieved from <https://rarediseases.org/projectrdac/rdacs-by-state/>

Functions of the RDAC

As outlined in section 381.99 (4)(a) through (d), Florida Statutes, the advisory council shall:



Consult with experts on rare diseases and solicit public comment to assist in developing recommendations on improving the treatment of rare diseases in this state.



Develop recommended strategies for academic research institutions in this state to facilitate continued research on rare diseases.

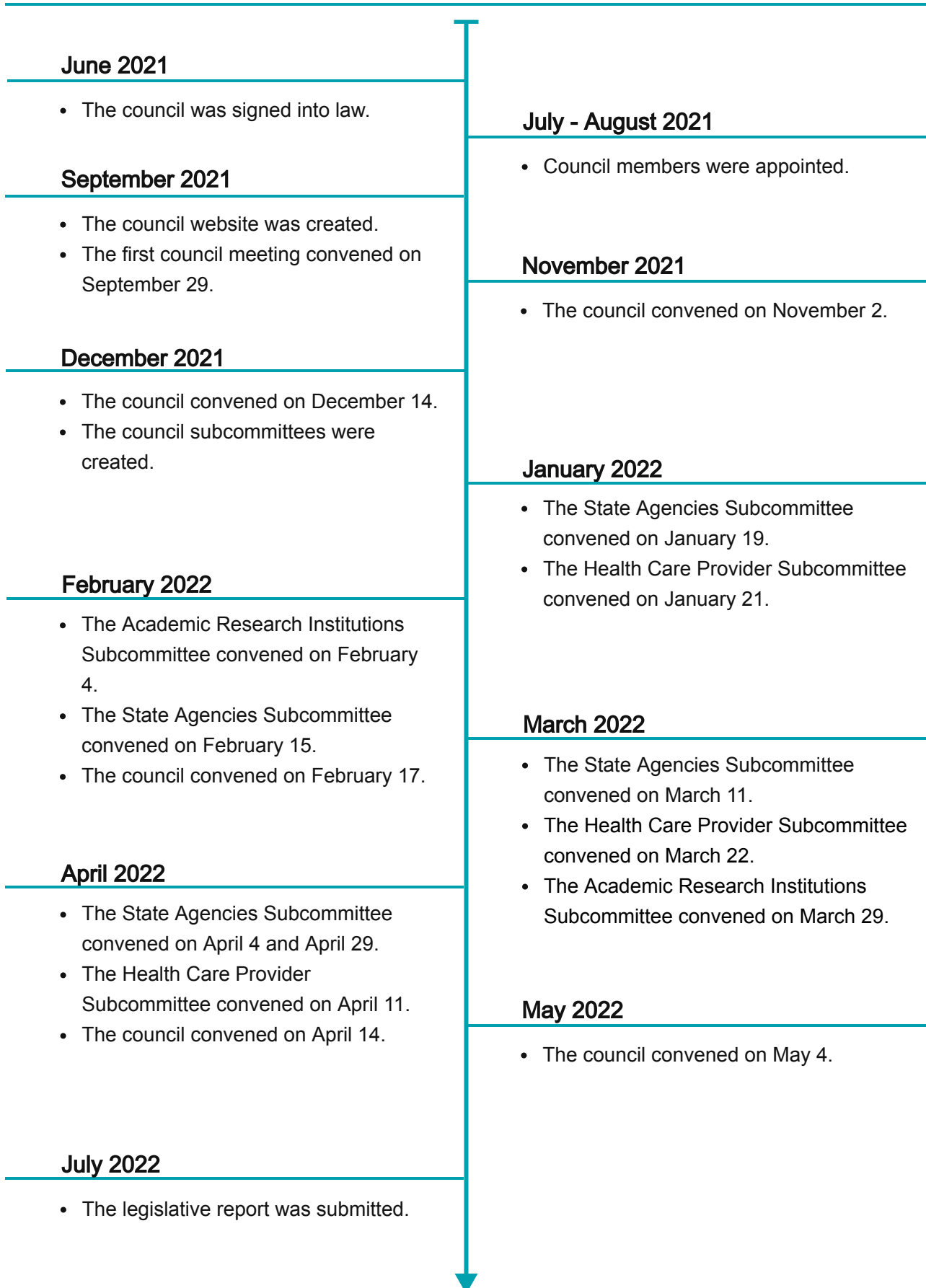


Develop recommended strategies for health care providers to be informed on how to more effectively recognize and diagnose rare diseases in order to effectively treat patients. The advisory council shall provide such strategies to the FDOH for publication on the Department's website.



Provide input and feedback in writing to the FDOH, the Medicaid program, and other state agencies on matters that affect individuals who have been diagnosed with rare diseases, including, but not limited to, pandemic or natural disaster preparedness and response.

Planning Year Timeline



Fiscal Year 2021-2022 Accomplishments

Assembly of the Council

Sixteen of the twenty-two council positions were appointed by the Governor, the Speaker of the Florida House of Representatives, and the President of the Florida Senate.

Development of the RDAC Website

The RDAC website was created to provide an overview of the council as well as information on membership, meetings, reports, and publications.

Establishment of Council Meeting Operations and Procedures

Since September, 2021, the full council has convened six times.

Formation of Subcommittees

The RDAC formed three subcommittees: the Academic Research Institutions Subcommittee, the Health Care Providers Subcommittee, and the State Agencies Subcommittee in order to address the requirements under section 381.99 (4)(b), (4)(c), and (4)(d), Florida Statutes (see Appendix for subcommittee composition). Since their formation, the Academic Research Institutions Subcommittee has met two times, the Health Care Providers Subcommittee has met three times, and the State Agencies Subcommittee has met five times.

Consultation with Experts on Rare Diseases

The council has consulted with national and state experts on rare diseases. This includes presentations from guest speakers during council and subcommittee meetings.

Solicitation of Public Comment

All meetings of the council and subcommittees were publicly posted in the Florida Administrative Register and FDOH website. The council received public comment that was considered in the development of the recommendations.

Development of Recommendations

The council completed development of recommendations over the past year. Subsequent objectives to accomplish the recommendations begin in Fiscal Year 2022-2023 and conclude in Fiscal Year 2025-2026.

Recommended Strategies for Academic Research Institutions

Recommendation 1: Support research institutions in Florida.

Year 1 Objectives

Objective 1.1: Identify the components that constitute a best practice "rare disease research institution."

Objective 1.2: Elicit feedback on developed components for a best practice "rare disease research institution."

Year 2 Objective

Objective 2.1: Centralize developed components of a best practice "rare disease research institution" on a publicly available website.

Year 3 Objective

Objective 3.1: Identify and promote best practices established by rare disease research institutions on a publicly available website.

Recommended Strategies for Academic Research Institutions

Recommendation 2: Promote availability of research institutions to individuals with rare diseases.

Year 1 Objective

Objective 1.1: Promote expanded access to genome sequencing for all individuals with rare diseases.

Year 2 Objective

Objective 2.1: Establish a maximum waiting period to promote timely access to research clinical trials for individuals with rare diseases.

Year 3 Objectives

Objective 3.1: Develop a database of research institutions that are available within a geographic location (ZIP code, city, region) on specific rare diseases.

Objective 3.2: Centralize resources on a publicly available website.

Objective 3.3: Create a community of practice amongst research institutions and individuals with rare diseases who are looking to be involved in research studies.

Recommended Strategies for Academic Research Institutions

Recommendation 3: Collaborate with state and national experts on complex rare disease cases.

Year 1 Objective

Objective 1.1: Identify state and national rare disease experts available to consult with health care providers on complex rare disease cases.

Year 2 Objective

Objective 2.1: Explore the availability and use of technology for consultation between health care providers and rare disease experts.

Year 3 Objective

Objective 3.1: Centralize consultation resources for health care providers on a publicly available website.

Recommended Strategies for Health Care Providers

Recommendation 1: Promote education on rare diseases to health care providers.

Year 1 Objectives

Objective 1.1: Identify existing educational resources, including continuing education courses, for health care providers on various topics related to rare diseases.

Objective 1.2: Identify educational resources on patient-centered care for health care providers.

Objective 1.3: Identify educational resources on holistic health care practices for health care providers.

Objective 1.4: Centralize educational resources on a publicly available website.

Year 2 Objectives

Objective 2.1: Evaluate rare disease curriculum taught in medical and nursing schools, allied medical programs, and public health programs, to identify gaps.

Objective 2.2: Identify additional curriculum to meet identified gaps in education on rare diseases.

Objective 2.3: Educate medical and nursing schools, allied medical programs, and public health programs, on the benefits of education on rare diseases as a part of their curriculum.

Year 3 Objective

Objective 3.1: Distribute educational materials to participating medical and nursing schools, allied medical programs, and public health programs.

Recommended Strategies for Health Care Providers

Recommendation 2: Promote advancements in the process of achieving a diagnosis for rare diseases.

Year 1 Objective

Objective 1.1: Identify screening tools for health care providers on rare diseases.

Year 2 Objective

Objective 2.1: Compile screening tools for rare diseases for health care providers and centralize resources on a publicly available website.

Year 3 Objectives

Objective 3.1: Develop an infographic for health care providers upon suspicion of a rare disease through the process of referral, diagnosis, and treatment.

Objective 3.2: Develop an infographic for individuals with rare diseases and their families that identifies the rare disease diagnosis process of referral, diagnosis, and treatment.

Objective 3.3: Centralize the developed infographics on a publicly available website.

Recommended Strategies for Health Care Providers

Recommendation 3: Promote advancements in the process of achieving treatments for rare diseases.

Year 1 Objectives

Objective 1.1: Identify current medical practices and specialists who accept both pediatric and adult individuals with rare diseases.

Objective 1.2: Identify barriers and challenges to medical practices and specialists accepting both pediatric and adult individuals with rare diseases.

Objective 1.3: Develop a list of resources for individuals with rare diseases on how to self-advocate during interactions with health care providers.

Year 2 Objectives

Objective 2.1: Survey individuals with rare diseases to identify barriers in access to care for testing and treatment.

Objective 2.2: Promote the inclusion of advocates, case managers, and interdisciplinary care teams to assist in facilitation during interactions with health care providers.

Objective 2.3: Support the development of transition practices within medical facilities and specialist providers to transfer care from pediatric to adult specialists.

Objective 2.4: Identify how rare diseases affect an individual long-term, such as pain management, household needs, work place, lifestyle adjustments, and overall quality of life.

Year 3 Objectives

Objective 3.1: Identify or develop a list of resources for individuals with rare diseases for mental and emotional support.

Objective 3.2: Develop a list of resources for individuals with rare diseases, and their families, including but not limited to, patient advocacy groups that support the rare disease community.

Objective 3.3: Publicly post a list of resources for individuals with rare diseases for mental and emotional support.

Recommended Strategies for State Agencies

Recommendation 1: Identify data needs necessary to understand the population with rare diseases in Florida.

Year 1 Objectives

Objective 1.1: Define incidence of rare disease in Florida.

Objective 1.2: Develop a list of rare diseases with an overlay of distribution across the state, beginning with the top 50 most prevalent rare diseases nationally.

Objective 1.3: Develop a list of International Classification of Diseases, Tenth Edition codes for conditions covered by the Florida Newborn Screening Program.

Objective 1.4: Identify the business rules for data extraction and research questions, including legal review.

Objective 1.5: Develop a list of resources with an illustrated network of the varying systems of care that serve individuals with rare diseases such as public services, support organizations, educational support systems, employment services, etc.

Year 2 Objectives

Objective 2.1: Develop Florida specific data from national rare diseases using the current available databases in Florida.

Objective 2.2: Define prevalence of rare disease in Florida.

Year 3 Objectives

Objective 3.1: Develop a list of rare diseases with an overlay of distribution across the state, using the top 500 most prevalent rare diseases nationally.

Objective 3.2: Determine where Florida falls in comparison to other states regarding rates of rare disease.

Recommended Strategies for State Agencies

Recommendation 2: Identify areas of improvement for FDOH on rare diseases.

Year 1 Objectives

Objective 1.1: Identify FDOH programs that serve the population with rare diseases.

Objective 1.2: Identify the population with rare diseases FDOH serves.

Objective 1.3: Develop a plan to enhance public-private partnerships.

Objective 1.4: Develop a plan for interagency data sharing agreements on data about the population with rare diseases.

Year 2 Objectives

Objective 2.1: Develop a needs assessment survey on the population with rare diseases FDOH serves.

Objective 2.2: Research and develop additional evidence-based programs to address barriers and gaps in services.

Objective 2.3: Publish data on rare diseases through FDOH data portals, such as FLHealthCHARTS.

Year 3 Objectives

Objective 3.1: Make recommendations for program improvement.

Objective 3.2: Research and develop training for FDOH staff to better serve the population with rare diseases.

Objective 3.3: Develop an evaluation plan.

Objective 3.4: Implement program improvements to better serve the population with rare diseases.

Objective 3.5: Implement FDOH staff training.

Objective 3.6: Implement evaluation plan. Use the evaluation results to make recommendations to improve program processes.

Recommended Strategies for State Agencies

Recommendation 3: Identify areas of improvement for the Agency for Health Care Administration (Agency) on rare diseases.

Year 1 Objectives

Objective 1.1: Identify Medicaid data that can be used to determine prevalence of rare diseases in Florida.

Objective 1.2: Identify Medicaid programs that serve the population with rare diseases.

Objective 1.3: Identify the population with rare diseases in Florida Medicaid serves and assess its needs.

Objective 1.4: Develop a plan to enhance public-private partnerships.

Year 2 Objectives

Objective 2.1: Identify how current surveys are being utilized to collect feedback from Medicaid recipients with rare diseases in relation to receipt of Medicaid services.

Objective 2.2: Research potential opportunities to improve Medicaid programs or services and address barriers and gaps.

Year 3 Objectives

Objective 3.1: Make recommendations for Medicaid program improvement.

Objective 3.2: Develop training for Agency staff to better serve Medicaid recipients with rare diseases.

Objective 3.3: Develop an evaluation plan.

Objective 3.4: Implement Medicaid service improvements to better serve the Medicaid recipients with rare diseases.

Objective 3.5: Implement Agency staff training.

Objective 3.6: Implement evaluation plan. Use the evaluation results to make recommendations to improve Medicaid program processes.

Recommended Strategies for State Agencies

Recommendation 4: Identify areas of improvement for the Florida Department of Education (FDOE) on rare diseases.

Year 1 Objectives

Objective 1.1: Identify FDOE programs that serve the population with rare diseases and identify any barriers or gaps in services.

Objective 1.2: Develop a resource guide to educate public and private school staff, students with rare diseases, and families on available resources and supports such as Individualized Educational Plans, 504 Plans, and scholarships offered.

Year 2 Objectives

Objective 2.1: Make recommendations for program improvement.

Objective 2.2: Develop a communication plan to include distribution of available resources and supports to meet the needs of students with rare diseases and their families.

Year 3 Objectives

Objective 3.1: Train FDOE staff on recommendations for improvement to better serve the population with rare diseases.

Objective 3.2: Implement outreach and engagement plan to public and private school staff, students with rare diseases, and families on available resources and supports.

Objective 3.3: Develop an evaluation plan.

Objective 3.4: Implement program improvements to better serve students with rare diseases and their families.

Objective 3.5: Implement evaluation plan. Use the evaluation results to make recommendations to improve programs.

Recommended Strategies for State Agencies

Recommendation 5: Identify areas of improvement for the Florida Office of Insurance Regulation (OIR) on rare diseases.

Year 1 Objectives

Objective 1.1: OIR will determine what data on rare diseases currently exist through commercial health insurance plans.

Objective 1.2: Identify the population with rare diseases OIR serves and assess its needs.

Objective 1.3: Identify if current surveys are being utilized to collect feedback from health insurance recipients with rare diseases.

Objective 1.4: Identify OIR programs that serve the population with rare diseases.

Objective 1.5: Develop a plan to enhance public-private partnerships.

Objective 1.6: Evaluate requirements for insurance benefits not to be discriminatory against insurance recipients with rare diseases.

Year 2 Objectives

Objective 2.1: Research potential opportunities to improve health insurance programs or services and address barriers and gaps.

Year 3 Objectives

Objective 3.1: Make recommendations for health insurance program improvement.

Objective 3.2: Develop training for OIR staff to better serve health insurance recipients with rare diseases.

Objective 3.3: Develop an evaluation plan.

Objective 3.4: Implement health insurance program improvements to better serve recipients with rare diseases.

Objective 3.5: Implement OIR staff training.

Objective 3.6: Implement evaluation plan. Use the evaluation results to make recommendations to improve health insurance program processes.

Recommended Strategies for State Agencies

Recommendation 6: Identify areas of improvement for disaster preparedness and pandemic response for individuals with rare diseases in Florida.

Year 1 Objectives

Objective 1.1: Develop partnership with the FDOH Division of Emergency Preparedness and Community Support, State Emergency Operations Center and local emergency shelter administrators on the needs of individuals with rare diseases in Florida.

Objective 1.2: Complete a needs assessment of the population with rare diseases in Florida for natural disaster preparedness and pandemic response.

Year 2 Objective

Objective 2.1: Identify local community resources individuals with rare diseases may contact in the event of an emergency for assistance.

Year 3 Objectives

Objective 3.1: Develop educational and training materials on the needs of individuals with rare diseases for emergency shelter coordinators and staff.

Objective 3.2: Develop educational materials for individuals with rare diseases on emergency preparedness planning to be shared with partnering agencies, such as information on the Florida Special Needs Shelter Registry, the special 30-day medication refill pursuant to section 456.0275(2), Florida Statutes, and a checklist for individuals with rare diseases that highlight specific items they may need during a disaster. Distribute these materials to physicians, pharmacies, and support organizations to share with individuals and their families.

Objective 3.3: Develop an evaluation plan.

Objective 3.4: Implement outreach and engagement plan to include distribution of educational and training materials and training programs on the needs of individuals with rare diseases for emergency shelter coordinators and staff.

Recommended Strategies for State Agencies

Objective 3.5: Implement an outreach and engagement plan to include distribution of educational and training materials and training programs on the needs of individuals with rare diseases for other professionals who serve individuals with rare diseases during emergency operations and with partnering agencies, such as information on the Florida Special Needs Shelter Registry, the special 30-day medication refill pursuant to section 456.0275(2), Florida Statutes, and a checklist for individuals with rare diseases that highlight specific items they may need during a disaster.

Objective 3.6: Implement evaluation. Use the evaluation results to make recommendations to improve program processes.

Conclusion

In conclusion, the RDAC was established and convened to develop recommendations and objectives to improve supports and services to individuals with rare diseases in Florida. The council met regularly and has effectively developed a multitude of recommendations for research institutions; health care providers; and state agencies that include the Florida Department of Health, the Agency for Health Care Administration, Florida Department of Education, and the Florida Office of Insurance Regulation. Recommendations were outlined as a three-year plan. The council looks forward to implementation of the recommendations and objectives presented in this inaugural report. A primary next step of implementation is to identify the population of individuals living with rare diseases in Florida. This is just the beginning of a long, but rewarding, journey to ensure Floridians with rare diseases have access to supports and services that will help them lead full and healthy lives.

Appendix

Academic Research Institutions Subcommittee Members

A physician who is licensed under chapter 458 or chapter 459 and practicing in this state with experience in treating rare diseases:

Barry Byrne, MD, PhD
Director, Powell Gene Therapy Center
Associate Chair, Department of Pediatrics
Professor, Pediatrics and Molecular Genetics & Microbiology
University of Florida

A representative from an academic research institution in Florida, which receives grant funding for research regarding rare diseases:

Divya Patel, DO, MBA
Program Director, Interstitial Lung Disease
Program Director, Sarcoidosis
Associate Professor of Medicine
University of Florida

A representative from an academic research institution in this state which receives grant funding for research regarding rare diseases:

Mustafa Tekin, MD
Division Director, Clinical and Translational Genetics
University of Miami

A physician who is licensed under chapter 458 or chapter 459 and practicing in this state with experience in treating rare diseases:

Rajan Wadhawan, MD, MMM
Senior Executive Officer
AdventHealth for Children
AdventHealth for Women

Appendix

Health Care Provider Subcommittee Members

A representative of organizations in this state which provide care or other support to individuals with rare diseases:

Anita Davis, PT, DPT, FNCP, CNPT
Physical Therapist
Brooks Rehabilitation

A representative of organizations in this state which provide care or other support to individuals with rare diseases:

Rebekah Dorr
Director of Clinical Patient Advocacy
Myasthenia Gravis Hope Foundation

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Caregiver

An individual who is 18 years of age or older who has a rare disease:

Jessica O'Reilly, JD
Self-Advocate

An individual who is 18 years of age or older who has a rare disease:

India Steinbaugh, MPH
Self-Advocate

Appendix

State Agencies Subcommittee Members

A representative of the Office of Insurance Regulation:

David Altmaier
Insurance Commissioner
Florida Office of Insurance Regulation

A representative of the Agency for Health Care Administration:

Ann Dalton, MM
Bureau Chief, Bureau of Medicaid Policy
Florida Agency for Healthcare Administration

A representative of the Department of Education:

Kathryn Hebda, MM - Council Vice Chair
Chancellor, Florida College System
Florida Department of Education

A representative of the biotechnology industry:

Jonathan Hawayek, MBA
Head of State Government Affairs
Spark Therapeutics, Inc.

A representative of the Department of Health:

Melissa Jordan, MS, MPH - Council Chair
Assistant Deputy Secretary for Health
Florida Department of Health

A representative of health insurance companies:

Scott McClelland, PharmD
Vice President, Commercial and
Specialty Pharmacy Programs and
Health Care Solutions
Florida Blue

An individual who is, or was previously, a caregiver for individuals with a rare disease:

Jennifer Sutherland
Caregiver