Legislative Report on the Louisiana Rare Disease Advisory Council

Report Prepared in Response to ACT 321 (HB 460)
of the 2021 Regular Legislative Session

Reflects activities of State Fiscal Year (SFY) 2023

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Introduction

A rare disease is a disease or condition that affects fewer than 200,000 people in the United States.[1] Throughout the country, about one in 10 people is diagnosed with one of the 7,000 known rare diseases. Patients, caregivers, families, and providers experience many obstacles in diagnosis and clinical care due to the lack of awareness and in-depth understanding of rare diseases. The symptoms associated with rare diseases vary considerably depending on the specific condition, and individuals with the same disease may have different clinical presentations. Rare diseases are sometimes present in individuals even though some symptoms may not appear until later in life. However, these diseases are often chronic, life-threatening genetic disorders.

The Louisiana Rare Disease Advisory Council (LA RDAC) is a statutorily mandated advisory group that is charged with serving in a resource capacity to any public and private agency located in this state that provides services for a person who has been diagnosed with a rare disease. The LA RDAC is administered through the Louisiana Department of Health (LDH or the Department) Office of Public Health (OPH) Bureau of Family Health (BFH), which provides staff support and policy expertise to assist the group with carrying out its charge. Per Louisiana R.S. 40:1122.1, the charges of the LA RDAC are:

1) To provide input and feedback to the Department and any other state agency on matters that affect a person who has been diagnosed with a rare disease, including but not limited to all of the following:
    a. Rare diseases in general, the severity of rare diseases, and unmet medical needs associated with rare diseases.
    b. Research, education, diagnosis, and treatment relating to rare diseases within Louisiana.
    c. The demographics and clinical description of patient populations.
    d. Timely access to screening, care, insurance or Medicaid coverage, specialists, and other needed services for a patient diagnosed with a rare disease.
    e. The impact that coverage, cost-sharing, tiering, and any other utilization management procedures have on providing treatment and services to a patient diagnosed with a rare disease.
    f. Pandemic preparedness and response and its impact on a person living with a rare disease.
2) To provide expert and clinical advice to the board in its review of treatments for a rare disease. The treatments may include drugs or biological products emerging from fields of personalized medicine and non-inheritable gene editing therapeutics.
3) To provide a report to the governor, the Legislature of Louisiana, LDH, and any other relevant agency of both of the following:
    a. Any findings, activities, and progress of the advisory council pursuant to provisions of Paragraphs (1) and (2) of this Subsection.
    b. Any recommendations for addressing the needs of a person living with a rare disease in this state.

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[1] *Public 97-414 97th Congress an Act - Food and Drug Administration. www.fda.gov/media/99546/download.*

The LA RDAC comprises 12 appointed members consisting of representatives from diverse organizations and entities that receive or provide services for individuals with a rare disease or condition (see Appendix A for the list of members). This report summarizes the activities, progress, and findings from the LA RDAC State Fiscal Year 2023 (SFY 23).

Activities, Progress, and Plans

The LA RDAC was established in law as a part of the 2021 Regular Session of the Louisiana Legislature. Over the past two years, the LA RDAC has been in the formative stages of development. Work has included identification and confirmation of members, drafting operating procedures, and promoting awareness of this newly forming group. In SFY 23, the advisory council held two meetings (see Appendix B for meeting dates and notes). In addition, BFH established and hired public health's first-ever dedicated role to support key commissions charged with advancing the systems of care for individuals with rare and heritable conditions. Having this dedicated role will be integral to the work of the LA RDAC and ensuring regular quarterly meetings and advancement of the council's mission. The activities and progress with the operations of the council and the charges of the council are summarized below.

Activities and Progress Related to Council Operations

A key formative task accomplished in SFY 23 was the development of procedures to define the expectations and responsibilities of appointed members. The guidelines encompass the authority, purpose, and function of the council along with processes related to voting, officer selection, and meeting schedule. These guidelines are under review by the RDAC and will be voted on when a quorum, defined a majority of council members, is present.

During SFY 23, the primary operational focus of the council was to review the implementation toolkit and webinar resources from the National Organization for Rare Disorders (NORD) and Project Rare Disease Advisory Council (RDAC) in order to inform the development of the LA RDAC. Project RDAC is a national initiative to ensure all states have impactful, community-engaged, high-functioning councils. Reviewing these resources was instrumental in the LA RDAC beginning to establish operational guidelines and a work plan to carry out the council's charges.

The vision of the council is to improve the quality of life for all those affected by rare diseases in Louisiana.
Its mission is to provide advice on research, diagnosis, treatment, and education related to rare diseases.

Activities and Progress Related the Charges of the LA RDAC

Provide recommendations to state agencies related to individuals diagnosed with rare diseases
Similar to RDACs nationally, the LA RDAC is responsible for researching patient populations in the state; assessing access to coverage, care, and unmet needs; and promoting awareness and education. Furthermore, the LA RDAC is responsible for identifying and addressing needs related to emergency preparedness and response. In SFY 23, the LA RDAC initiated work to identify the population and needs. The council also began to identify approaches to promote provider education and awareness generally.

Understanding the populations in Louisiana and their needs. The number of individuals living with a rare disease in Louisiana remains unknown due to the lack of established methodology for identifying the incidence (the number of new cases that develop in a specified time period) and prevalence (the proportion of a population who have a specific characteristic in a specified time period) of rare diseases. Data are essential to understanding who is affected and to illuminate the actions needed to support their care and quality of life.

Establishing a system for estimating the number of people living with rare diseases in Louisiana is one of the most significant priorities for the LA RDAC. In SFY 23, BFH staff initiated efforts to obtain data about diagnoses and hospitalizations associated with rare conditions. In SFY 24, the LA RDAC plans to develop approaches to quantify and monitor rare diseases, and the number of people affected, by utilizing available health information (i.e. ICD-10 and ICD-11 disease codes) through public health, Medicaid, Medicare, major hospital systems, and managed care organizations. The aim is to establish a reliable and replicable method for estimating the population and evaluating access to specialty care, therapies, supports, insurance, and disability benefits for those affected by rare disorders. BFH staff will work with OPH's Bureau of Health Informatics and LDH Medicaid to conduct preliminary analyses to assess the conditions represented in hospital discharge and Medicaid claims data.

To better quantify and understand the needs of the rare disease community, the LA RDAC will survey people affected by rare disorders as well as the healthcare providers who serve this population to gain more information. The surveys to community members will illuminate the complexity and magnitude of challenges associated with the currently available resources, programs, and support. The surveys of healthcare providers will help determine the support providers need in order to deliver high-quality medical services to individuals with rare diseases. These assessments are expected to be distributed through rare disease support organizations, major hospital systems, and social media. BFH staff will assist with the development and dissemination of the survey and with analysis.

Education and resources for providers. The LA RDAC discussed various ways to provide support to the medical community regarding rare disease resources. Topics included implementing a compendium of organizations that provide resources to individuals with rare disorders and establishing the Extension for Community Healthcare Outcomes (ECHO) model to inform community practitioners in virtual settings on how to treat patients with rare conditions. These activities will be explored further in SFY 24.

Education and resources for state leaders and policy makers. One of the functions of the LA RDAC is to educate elected officials, state leaders, and state government officials on rare disease research, education, diagnosis, and treatment. To serve as a unified voice for the rare disease community, the LA RDAC plans to engage these various constituencies in SFY 24 to share challenges experienced by individuals with rare conditions, especially challenges related to legislation and policies affecting people with rare diseases. In SFY 24, the LA RDAC will ensure an equal representation of all appropriate perspectives by assessing the membership. BFH staff will assist the LA RDAC in conducting outreach to constituencies whose perspective is needed to inform the work and efforts of the council.

Education and resources for the public, policy makers, providers, and affected individuals and families. The legislation that established the LA RDAC brought attention to the issues related to rare diseases and conditions. In SFY 23, members discussed the need to ensure that the issues, work, and recommendations of the LA RDAC are visible and accessible. Members proposed establishing a dedicated webpage to serve as a platform to post information about the current priorities and activities of the council. They also discussed the benefit of the site serving as a centralized resource for rare disease awareness, as well as a space to host reports, the results of needs assessments, and data such as hospitalization utilization patterns. In SFY 24, the LA RDAC will consult with BFH’s communications team to create content for the site.

Provide expert and clinical advice to the Drug Utilization Board in its review of treatments
The authorizing statute for the LA RDAC charges the council with working with the LDH Drug Utilization Board to provide expertise and clinical advice. Work related to this charge was not initiated in SFY 23, but will be pursued in SFY 24.

Conclusion

The LA RDAC was established to improve the quality of life for individuals with rare diseases through improvements in medical care coordination, research, legislation, and advocacy. With the objectives identified during the inaugural year, the LA RDAC is now focused on implementing positive changes by conducting needs assessments, engaging public and private partners, obtaining data to inform actions, and making policy recommendations. This report summarizes the Louisiana Rare Disease Advisory Council’s SFY 2023 activities and outlines strategies the LA RDAC will undertake in SFY 2024 to strengthen the systems of care and support for individuals living with a rare disease in Louisiana.

Appendix A: LA RDAC Membership List & Meeting Attendees

LA RDAC Membership List

Member's Name	Role	Selection Method
Jonathan James Hope for Hemophilia	Appointed by President of the Senate	President of the Senate
Kelly Rouse, MS, BCBA, LBA, Vice-Chair Rouse Consultants, LLC	Appointed by the Speaker of the House of Representatives	Speaker of the House of Representatives
Vacant	Representative from the Louisiana Department of Health	Governor
Ross Klingsberg, MD Tulane University School of Medicine	Representative from Academic Research Institution	Governor
Jennifer Borrillo Louisiana Center for Bleeding and Clotting Disorders at Tulane University School of Medicine	Representative from Academic Research Institution	Governor
Deiadra Garrett, MD Our Lady of Lourdes Physician Group Pediatric General Surgery Center	Physician with Rare Disease expertise	Governor
Hans Andersson, MD Hayward Genetics Center at Tulane University School of Medicine	Physician with Rare Disease expertise	Governor
Meredith Allain, MD Ochsner Clinic Foundation	Geneticist	Governor
Carolyn Moore, FNP-BC, MHA Department of Corrections	Registered Nurse or Advance Practice Registered Nurse with Rare Disease expertise	Governor
Libby Airhart, Chair Acadian Ambulance	Individual or caregiver of individual with a Rare Disease diagnosis (18+)	Governor
Marissa Benchea Beam Feel Good	Individual or caregiver of individual with a Rare Disease diagnosis (18+)	Governor
Lauren Williams CureGRIN	Representative of a Rare Disease patient organization	Governor

Appendix B: LA RDAC Meeting Schedule & Agendas

Below are the LA RDAC quarterly meeting dates with agendas for each meeting linked:

- July 5, 2022 | Meeting Agenda
- October 11, 2022 |Meeting Agenda

Appendix C: Revised Statute 40:1122.1

§1122.1. Louisiana Rare Disease Advisory Council
A. For the purpose of this Part, the following terms have the meanings ascribed to them:
(1) "Board" means the Drug Utilization Review board established by the Louisiana Department of Health.
(2) "Department" means the Louisiana Department of Health.
(3) "Rare disease" means any disease or condition that affects fewer than two hundred thousand persons in the United States. Rare disease shall also mean sickle cell disease and sarcoidosis.

B. (1) The Louisiana Rare Disease Advisory Council is hereby created within the Louisiana Department of Health.
(2) The Louisiana Rare Disease Advisory Council hereby referred to as the "advisory council" shall serve only in a resource capacity to any public and private agency located in this state that provide services for a person who has been diagnosed with a rare disease.
(3) The advisory council shall be composed of the following members:
(a) One member appointed by the president of the Senate.
(b) One member appointed by the speaker of the House of Representatives.
(c) Members appointed by the governor as follows:
(i) One representative from the department.
(ii) Two representatives from academic research institutions in this state that conduct rare disease research.
(iii) Two physicians who are licensed and practicing in this state with experience in researching, diagnosing, or treating rare diseases.
(iv) One geneticist who is licensed and practicing in this state.
(v) One registered nurse or advanced practice registered nurse who is licensed and practicing in this state and has experience treating rare diseases.
(vi) Two residents of this state who are eighteen years of age or older and who have either been diagnosed with a rare disease or is a caregiver for a person who has been diagnosed with a rare disease.
(vii) One representative of a rare disease patient organization operating in this state.
(4) The governor shall determine who serves as the chair and vice chair of the advisory council.
(5) To the extent practicable, every organization or entity that provides a nomination to the advisory council shall strive for diversity in its appointment on the basis of race, ethnicity, sex, professional or educational background, and geographic residency.

C. The advisory council shall hold its initial meeting no later than October 1, 2021. The advisory council shall meet at least quarterly in a calendar year and at any other time, as it deems necessary.

D. The purposes of the advisory council include all of the following:
(1) To provide input and feedback to the department and any other state agency on matters that affect a person who has been diagnosed with a rare disease, including but not limited to all of the following:
(a) Pandemic preparedness and response and its impact on a person living with a rare disease.
(b) Research, education, diagnosis, and treatment relating to rare diseases within this state.
(c) Rare diseases in general, the severity of rare diseases, and unmet medical needs associated with rare diseases.
(d) The demographics and clinical description of patient populations.
(e) Timely access to screening, care, insurance or Medicaid coverage, specialists, and other needed services for a patient who has been diagnosed with a rare disease.

(f) The impact that coverage, cost-sharing, tiering, and any other utilization management procedure has on providing treatment and services to a patient who has been diagnosed with a rare disease.
(2) To provide expert and clinical advice to the board in its review of treatments for a rare disease. The treatments may include drugs or biological products emerging from fields of personalized medicine and non-inheritable gene editing therapeutics.
(3) To provide a report to the governor, the Legislature of Louisiana, the department, and any other relevant agency of both of the following:
(a) Any findings, activities, and progress of the advisory council pursuant to the provisions of Paragraphs (1) and (2) of this Subsection.
(b) Any recommendations for addressing the needs of a person living with a rare disease in this state.

E. The advisory council shall not have authority on any matter relating to the department or the board.

F. Nothing in this Section shall require the board to follow the recommendations of the advisory council.

G. Nothing in this Section shall require the advisory council, the board, or any state agency to consult with a person on any matter or be required to meet with any specific expert or stakeholder.
H. An advisory council member shall not receive any compensation for serving on the advisory council.
Acts 2021, No. 321, §2.

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