

2021 Report to the Governor and General Assembly

Rare Disease Council

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Executive Summary

The Rare Disease Council (RDC) is established in the Code of Virginia [§32.1-73.14] to advise the Governor and General Assembly on rare diseases. The Council is required to report to the Governor and General Assembly by October 1st of each year the following:

1. Activities and recommendations of the Council
2. Status of funding available to the Council, including grants applied for and received by the Council

Council members have been identified as outlined in the law. Formal appointments of council members by the Governor are in progress as of this report submission. The first Rare Disease Council meeting was held on Monday, September 27, 2021. This document serves as the required 2021 annual report.

Introduction—What is a rare disease?

According to the National Institutes of Health's (NIH) Genetic and Rare Diseases Information Center, a rare disease is defined as a condition that affects fewer than 200,000 people in the United States, and there are an estimated 7,000 known rare diseases.¹ The NIH adopted this definition from the Rare Diseases Act of 2002.² Per a fact sheet published by the National Organization for Rare Disorders, it is estimated that there are nearly 30 million, or one in ten, Americans living with a rare disease.³

Background

The RDC was established during the 2021 General Assembly Special Session I as a result of HB1995. The purpose of the Council is as follows:

- Advise the Governor and the General Assembly on the needs of individuals with rare diseases in Virginia;
- Identify the challenges that such individuals face, including delays in obtaining a diagnosis or the receipt of a misdiagnosis, shortages of medical specialists who can provide treatment, and lack of access to therapies and medication used to treat rare diseases;
- Fund research related to rare diseases and the development of new treatments for rare diseases; and
- Fund supports for persons with rare diseases in the Commonwealth.

The membership of the Council must comply with the specifics set out in the Code of Virginia, Chapter 2 of Title 32.1, Article 19, sections 32.1-73.14 through 32.1-73.17. The Council consists of 21 members, including eighteen non-legislative members and three ex-officio

members. The eighteen non-legislative members are citizens of the Commonwealth and include the following representation: one representative from an academic research institution in the Commonwealth that receives any grant funding for rare disease research; one geneticist licensed and currently practicing in the Commonwealth; one registered nurse or advanced practice registered nurse licensed and currently practicing in the Commonwealth, with experience in treating rare diseases; two physicians with expertise in rare diseases who are licensed and currently practicing medicine in the Commonwealth; one hospital administrator, or his/her designee, from a hospital in the Commonwealth that provides care to persons diagnosed with rare diseases; two persons who are 18 years of age or older who have been diagnosed with a rare disease; two caregivers of persons with a rare disease; two representatives of rare disease patient organizations operating in the Commonwealth; one licensed pharmacist with experience with drugs used to treat rare diseases; one representative from the biopharmaceutical industry; one representative from health plan companies; and one member from the scientific community who is engaged in rare disease research, which may include a medical researcher with experience conducting research on rare diseases. The Governor appoints two of the eighteen non-legislative members to serve as chairman and vice chairman. The Commissioner of Health, the Director of the Department of Medical Assistance Services, and the Superintendent of Public Instruction, or their designees, shall serve ex officio with nonvoting privileges. Staff to the Rare Disease Council is provided by the Virginia Department of Health (VDH).

Status Report on Council Activities

VDH staff completed activities to identify potential members of the Council and to plan the first meeting within 90 days of the Council's effective date of July 1, 2021, as required by the

law. Member nominations were solicited from stakeholder organizations, and VDH provided the nominations to the Office of the Secretary of the Commonwealth. Council appointments for the eighteen non-legislative members, including the chair and co-chair, were finalized in late September 2021. The three ex officio member designees were identified by the Commissioner of Health, the Director of the Department of Medical Assistance Services, and the Superintendent of Public Instruction. The VDH Office of Family Health Services' Birth Defects Surveillance Coordinator is overseeing programmatic support for the Council, and a RDC Coordinator was contracted to support the Council and associated activities.

The RDC's first meeting was convened via a virtual format on Monday, September 27, 2021. The meeting was facilitated by VDH staff, and the agenda included a welcome and introduction of all members and staff, as well as establishing governance for the group. The draft bylaws were sent electronically to council members prior to the meeting. VDH staff provided a detailed review of HB 1995 and the draft bylaws. Jana Monaco, Vice Chair, provided a presentation on advocacy and priority policy issues that directly affect those with rare diseases. The presentation was followed by an open discussion among council members regarding the Council's first-year priorities and objectives. VDH staff will conduct a survey of council members to collect baseline data in order to determine the Council's priorities and what is financially feasible to attain in the first year.

References

¹ *FAQs About Rare Disease*. National Institute of Health. (2021, January 26). <https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases>.

² Rare Diseases Act of 2002. 42 U.S.C. §281 (2002)280. Retrieved September 20, 2021 from https://www.congress.gov/107/plaws/publ280/PLAW-107publ280.pdf?_cf_chl_jschl_tk_=pmd_GRnKbmpqPXwmJBJvpS3AgdjPcmOOQSRnWPaBuiPwwk4-1632166412-0-gqNtZGzNAjujcnBszQil.

³ National Organization for Rare Disorders. *Rare Disease Facts*. Retrieved September 20, 2021 from <https://rarediseases.org/wp-content/uploads/2019/02/nord-rareinsights-rd-facts-2019.pdf>