

SENATE BILL REPORT

SB 5064

As of February 10, 2025

Title: An act relating to creating an advisory council on rare diseases.

Brief Description: Creating an advisory council on rare diseases.

Sponsors: Senators Liias, Holy, Stanford, Wagoner, Lovick, Gildon, Cleveland, Cortes, Fortunato, Harris, Nobles, Riccelli and Warnick.

Brief History:

Committee Activity: Health & Long-Term Care: 1/23/25, 1/30/25 [DPS-WM].
Ways & Means: 2/10/25.

Brief Summary of First Substitute Bill

- Creates an advisory council on rare diseases within the University of Washington's School of Medicine to advise the Secretary of Health on research, diagnosis, treatment, and education related to rare diseases.

SENATE COMMITTEE ON HEALTH & LONG-TERM CARE

Majority Report: That Substitute Senate Bill No. 5064 be substituted therefor, and the substitute bill do pass and be referred to Committee on Ways & Means.

Signed by Senators Cleveland, Chair; Orwall, Vice Chair; Muzzall, Ranking Member; Bateman, Chapman, Christian, Harris, Holy, Riccelli, Robinson and Slatter.

Staff: Julie Tran (786-7283)

SENATE COMMITTEE ON WAYS & MEANS

Staff: Michele Alishahi (786-7433)

This analysis was prepared by non-partisan legislative staff for the use of legislative members in their deliberations. This analysis is not part of the legislation nor does it constitute a statement of legislative intent.

Background: The United States Congress, in the Orphan Drug Act of 1983, defines a rare disease as any disease or condition which affects less than 200,000 people in the United States. Rare diseases include genetic conditions, infections, some rare cancers, and autoimmune diseases. It is estimated that there are over 10,000 rare diseases, and rare diseases are estimated to affect more than 30 million people in the United States.

Summary of Bill (First Substitute): An advisory council on rare diseases (council) is established within the University of Washington's School of Medicine to advise the Secretary of Health on research, diagnosis, treatment, and education related to rare diseases. The council must submit annual reports on the council's work and other findings and recommendations to the Governor and Legislature beginning December 1, 2026.

"Rare disease" means a disease that affects fewer than 200,000 people in the United States.

Duties of the Advisory Council on Rare Diseases. The council must advise on and coordinate efforts to study the incidence and prevalence of rare diseases in the state and the status of the rare disease community. This work includes examining issues regarding quality of care outcomes and cost effectiveness of the earlier diagnoses and treatment for rare diseases and also, access to available treatment and services to people with rare diseases in the state.

The council's duties include:

- identifying effective research-based strategies that have been developed to help diagnose, treat, and prevent rare diseases;
- making recommendations on how to better educate and inform medical providers, schools, and public health districts around the diagnosis, treatment, and access to care for people living with a rare disease; and
- developing effective strategies to raise the public awareness of rare diseases in the state.

The council must make recommendations on establishing a website for people living with a rare disease, their families, and their caregivers, or those seeking diagnosis to access, to access a centralized, public accessible repository of information and resources available to patients in the state.

The council must serve only in an advisory role and may not supersede the authority of local health jurisdictions when responding to a communicable rare disease outbreak.

Membership of the Council. The Secretary of Health must appoint 12 voting members for three-year terms to the council including:

- four clinicians and researchers specializing in rare diseases;
- four individuals, who are Washington residents, representing patients;
- the Department of Health's Children and Youth with Special Health Care Needs Program Director, or their designee;

- a representative from the Health Care Authority; and
- two representatives from the life sciences industry focusing on developing therapeutic products for rare disease patients, research efforts related to those endeavors, or have a demonstrable understanding of the path to the commercialization of those products.

The council must be selected to ensure representation from both the west and east side of the state. A detailed list of requirements for specific qualifying voting members can be found in the bill.

The council must self-select a chair and the chair must convene the advisory council's first meeting within the first six months of the council's creation. After the first meeting, the advisory council must meet at least quarterly and the council must meet upon the call of the chair or upon the request of the majority of members.

EFFECT OF CHANGES MADE BY HEALTH & LONG-TERM CARE COMMITTEE (First Substitute):

- Requires the four individuals representing patients on the advisory council on rare diseases to be Washington residents.

Appropriation: The bill contains a section or sections to limit implementation to the availability of amounts appropriated for that specific purpose.

Fiscal Note: Available.

Creates Committee/Commission/Task Force that includes Legislative members: No.

Effective Date: Ninety days after adjournment of session in which bill is passed.

Staff Summary of Public Testimony on Original Bill (Health & Long-Term Care):

The committee recommended a different version of the bill than what was heard. PRO: A majority of rare diseases have a genetic component. It is within UW Medical Center's expertise to host the Rare Disease Advisory Council. This bill will add Washington to the list of states who have an advisory council on rare diseases. Advocates are fighting to improve the health care outcomes for rare disease patients all across the state. This bill highlights the awareness, visibility, and treatment needs for rare disease patients as its frustrating to get a diagnosis and access treatments. There is a need for information to inform policy. This bill will help develop that data which will inform policies on this issue. It will also help families that are beginning their diagnostic odyssey but it will also help families with kids who are growing up. This bill is crafted from a patient-centric perspective and centers the experiences of people experiencing rare disease and making sure they are at the center of the work moving forward. There is work being done with advocates relating to the focus on treatment costs.

OTHER: There are two amendment suggestions that include requiring all four patient representatives be Washington State residents and requesting the council consider the access, funding challenges, and funding solutions for the very high cost of rare disease treatments.

Persons Testifying (Health & Long-Term Care): PRO: Senator Marko Liias, Prime Sponsor; Gail Jarvik, University of Washington Medical Center; Whitney Stohr, Kids Rise Fund; Lindsey Viscarra, National Organization for Rare Disorders; Sarah Tompkins, NW Rare Disease Coalition.

OTHER: Stephanie Simpson, Bleeding Disorder Foundation of Washington.

Persons Signed In To Testify But Not Testifying (Health & Long-Term Care): No one.

Staff Summary of Public Testimony (Ways & Means): PRO: When you think of something rare, you think low numbers. In reality if it's one in ten and two-thirds are kids, and you apply it to Washington's 2020 census, that equates to about 520,000 kids and 260,000 adults. Advisory councils on rare diseases vary in composition, size, housing agency, and duties, but they all have the common goal of giving rare disease patients and their families a unified voice in state governments. They also help provide crucial information to policy makers that can help deliver diagnosis and treatment faster and reduce the diagnostic odyssey as much as possible and save money to the health care system as a whole and to the state.

Families navigating their diseases face immense challenges, especially in finding medical professionals who truly understand their diagnosis. Many families travel out of state to find appropriate care, incurring overwhelming costs, including travel, lodging, and medical expenses. The council's collection of data on rare diseases would attract critical funding, inform public policy, and guide efforts to establish clinics and allocate resources where they are needed most.

Persons Testifying (Ways & Means): PRO: Mary McDirmid, NW Rare Disease Coalition; Lindsey Viscarra, National Organization for Rare Disorders; Michelle O'Dell.

Persons Signed In To Testify But Not Testifying (Ways & Means): No one.