Washington State
House of Representatives
Office of Program Research

BILL ANALYSIS

Health Care & Wellness Committee

HB 1238

Brief Description: Creating an advisory council on rare diseases.

Sponsors: Representatives Low and Davis.

Brief Summary of Bill

- Creates an advisory council on rare diseases to provide information and recommendations to the Secretary of Health, the Governor, and the Legislature.

Hearing Date: 1/22/25

Staff: Emily Poole (786-7106).

Background:

The federal Orphan Drug Act defines a rare disease or condition as any disease or condition that affects fewer than 200,000 people in the United States or affects more than 200,000 people in the United States but for which there is no reasonable expectation that the cost of developing and making available a drug for the disease or condition will be recovered from sales of such a drug. Rare diseases include genetic conditions, infections, some rare cancers, and autoimmune diseases. The Food and Drug Administration estimates that there may be more than 7,000 rare diseases affecting more than 30 million people in the United States.

Summary of Bill:

An advisory council on rare diseases (council) is established within the University of Washington School of Medicine to advise the Secretary of Health (Secretary) on research, diagnosis, treatment, and education related to rare diseases.

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"Rare disease" is defined to mean a disease that affects fewer than 200,000 people in the United States.

Membership and Staff Support.

The Secretary is required to appoint 12 voting members to the council, including:

- four clinicians and researchers specializing in rare diseases, including at least one representative from a pediatric hospital, at least one representative from a hospital or academic research institution east of the Cascade mountains, and at least one representative from a hospital or academic research institution west of the Cascade mountains, from the following professions:
  - a physician with experience researching, diagnosing, or treating rare diseases;
  - a registered nurse, advanced practice registered nurse, genetic counselor, or social worker with experience with patients with rare diseases;
  - a medical researcher with experience conducting rare disease research; and
  - a representative from an academic research institution that receives grant funding for rare disease research;
- four individuals representing patients, including:
  - two patients living with a rare disease;
  - a rare disease patient caregiver; and
  - a representative from a rare disease foundation or patient advocacy group;
- the director of the Department of Health's Children and Youth with Special Health Care Needs Program, or their designee;
- a representative from the Health Care Authority; and
- two representatives from the life sciences industry.

Voting members must be appointed to serve a three-year term, and a vacancy on the council must be filled in the same manner as the original appointment. The council must self-select a chair. Subject to amounts appropriated, the University of Washington must provide staff support to the council, including physician leadership.

Council Duties.

The council is required to:

- act as the advisory body to the Secretary on research, diagnosis, treatment, and education related to rare diseases;
- advise on and coordinate efforts for the study of incidence and prevalence of rare diseases in the state and the status of the rare disease community;
- identify effective research-based strategies that have been developed to help diagnose, treat, and prevent rare diseases;
- make recommendations on how to better inform medical providers, schools, and public health districts regarding rare diseases;
- develop effective strategies to raise public awareness; and
- make recommendations on establishing a website for people living with a rare disease, their families, and their caregivers, as well as people seeking diagnosis.

Beginning December 1, 2026, and every biennium thereafter, the council must submit a report to the Governor and the Legislature: (1) detailing the work of the council, (2) identifying opportunities to support programs that directly impact the lives of patients, and (3) providing recommendations for statutory changes to the structure and duties of the council.

Appropriation: None.

Fiscal Note: Requested on January 14, 2025.

Effective Date: The bill takes effect 90 days after adjournment of the session in which the bill is passed.