HOUSE BILL REPORT
HB 1238

As Reported by House Committee On:
Health Care & Wellness

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

Brief Description: Creating an advisory council on rare diseases.

Sponsors: Representatives Low and Davis.

Brief History:
Committee Activity:
Health Care & Wellness: 1/22/25, 1/24/25 [DPS].

Brief Summary of Substitute Bill

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

HOUSE COMMITTEE ON HEALTH CARE & WELLNESS

Majority Report: The substitute bill be substituted therefor and the substitute bill do pass. Signed by 18 members: Representatives Bronoske, Chair; Lekanoff, Vice Chair; Schmick, Ranking Minority Member; Caldier, Assistant Ranking Minority Member; Marshall, Assistant Ranking Minority Member; Davis, Engell, Low, Macri, Manjarrez, Obras, Parshley, Shavers, Simmons, Stonier, Stuebe, Thai and Tharinger.

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

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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 Substitute 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.

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

<u>Membership and Staff Support</u>.
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, who are Washington residents, 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.

Substitute Bill Compared to Original Bill:

The substitute bill requires the four individuals representing patients on the council to be Washington residents.

Appropriation: None.

Fiscal Note: Available.

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

Staff Summary of Public Testimony:

(In support) People with rare diseases face a lack of information and resources, and it can take years, and a lot of financial resources, to obtain a diagnosis. This bill has the potential to bridge gaps in access for people with rare diseases, facilitate earlier diagnoses, and boost awareness. Many other states have similar advisory councils, which help to give patients with rare diseases a voice in state government. The council will help reduce the fiscal burden on states of caring for patients with rare diseases, because it will help pave the way for more efficient care. The University of Washington has the expertise to support this council. There should be an amendment to specify that the members of the council must be Washington residents.

(Opposed) None.

(Other) Facing a rare disease can be a frustrating and lonely experience. The bill should require the patient representatives of the council to be Washington residents. The bill should also require the council to consider the very high cost of, and potential funding solutions for, rare disease treatment.

Persons Testifying: (In support) Representative Sam Low, prime sponsor; Fuki Marie Hisama MD, University of Washington School of Medicine; Sarah Tompkins; Lindsey Viscarra, National Organization for Rare Disorders (NORD); and Lindsey Topping-Schuetz, NW Rare Disease Coalition.

(Other) Stephanie Simpson, Bleeding Disorder Foundation of Washington.

Persons Signed In To Testify But Not Testifying: None.