

JAN 24 2019

A BILL FOR AN ACT

RELATING TO THE RARE DISEASE ADVISORY BOARD.

BE IT ENACTED BY THE LEGISLATURE OF THE STATE OF HAWAII:

1 SECTION 1. The legislature finds that a rare disease is a
2 disease that affects fewer than two hundred thousand people.
3 There are seven thousand known rare diseases affecting
4 approximately thirty thousand people in the United States.
5 While the exact cause for many of these rare diseases remains
6 unknown, eighty per cent of rare diseases are genetic diseases
7 that can be linked to mutations in a single gene or in multiple
8 genes. Genetic disease can be passed down from generation to
9 generation, explaining why certain rare diseases run in
10 families. It is estimated that about half of all rare diseases
11 affect children.

12 The legislature also finds that a person suffering with a
13 rare disease in Hawaii faces a wide range of challenges,
14 including: delays in obtaining a diagnosis, misdiagnosis,
15 shortage of medical specialists familiar with rare diseases,
16 prohibitive treatment costs, and the inability to access
17 therapies and medications that are used by doctors to treat rare



1 diseases but have not been approved by the federal Food and Drug
2 Administration for that specific purpose. In recent years,
3 considerable progress has been made in developing diagnostic
4 tools and treatment protocols for rare diseases, and in
5 discovering ways to prevent the diseases. However, much more
6 remains to be done in the areas of rare disease research and the
7 search for new therapeutics.

8 Therefore, the purpose of this Act is to establish a
9 statewide rare disease advisory board to educate medical
10 professionals, government agencies, and the public about rare
11 diseases, and to encourage the funding of research in the
12 development of new treatments for rare diseases.

13 SECTION 2. Chapter 321, Hawaii Revised Statutes, is
14 amended by adding a new section to part I to be appropriately
15 designated and to read as follows:

16 "§321- Rare disease advisory board. (a) There is
17 established within the department of health a rare disease
18 advisory board for the purpose of:

19 (1) Coordinating statewide efforts for the study of the
20 incidence of rare diseases in Hawaii and the status of
21 the community of individuals with a rare disease;



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- 1 (2) Acting as the advisory body on rare diseases to the
2 legislature and state departments and agencies that
3 serve persons with rare diseases; and
- 4 (3) Coordinating the performance of the advisory board's
5 duties with other state rare disease advisory bodies,
6 community-based organizations, other public and
7 private organizations, and federal agencies such as
8 the National Institutes of Health and Food and Drug
9 Administration, to ensure cooperation between state
10 and federal governments in activities regarding rare
11 diseases.
- 12 (b) The advisory board shall consist of:
- 13 (1) Directors or ex officio members of the department of
14 health and other state agencies concerned with the
15 provision of care to persons with rare diseases; and
- 16 (2) The following public members, who shall be appointed
17 by the director:
- 18 (A) Two physicians licensed to practice in Hawaii who
19 have expertise in treating patients with rare
20 diseases, one of whom shall be a pediatrician who
21 provides care to children with rare diseases;



- 1 (B) A registered nurse licensed in Hawaii who has
- 2 expertise in providing care to patients with rare
- 3 diseases;
- 4 (C) Two representatives of hospitals in Hawaii;
- 5 (D) A representative of the health insurance
- 6 industry;
- 7 (E) A representative of the biopharmaceutical
- 8 industry;
- 9 (F) A representative of the scientific community who
- 10 is engaged in rare disease research;
- 11 (G) Two parents of a child with a rare disease;
- 12 (H) Two persons with a rare disease; and
- 13 (I) Two patient organizations.

14 The advisory board may advise the department of health on
15 additional at-large appointments that may be necessary to carry
16 out the advisory board's duties. At-large appointments to the
17 advisory board may serve on an ad hoc basis. Vacancies in
18 membership shall be filled in the same manner as the original
19 appointments. Members of the board shall serve without
20 compensation but shall be reimbursed for actual expenses,

1 including travel expenses necessary for the performance of their
2 duties.

3 The advisory board shall meet at least three times
4 annually.

5 The advisory board shall organize as soon as practicable
6 after the appointment of its members, and shall select a
7 chairperson and vice-chairperson from among its members. The
8 chairperson shall appoint a secretary who does not need to be a
9 member of the advisory board.

10 The advisory board may avail itself of the services of the
11 employees of any state, county, or municipal department, board,
12 bureau, commission, or agency, as required and as available.
13 The department of health may provide staff services to the
14 advisory board.

15 (c) The advisory board shall:

16 (1) Research and determine the most appropriate method to
17 collect rare disease data and information concerning
18 patients with rare diseases through conducting surveys
19 of rare disease patients; provided that surveys are
20 subject to all applicable privacy laws and
21 protections;



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- 1 (2) Ensure that data collection and patient surveys are
2 carried out in a way that is coordinated and
3 interoperable with similar research being conducted at
4 the state and federal levels;
- 5 (3) Research and identify priorities relating to the
6 quality and cost-effectiveness of, and access to,
7 treatment and services provided to persons with rare
8 diseases in Hawaii and develop policy recommendations;
- 9 (4) Identify best practices for rare disease care from
10 other states and nationally to improve rare disease
11 care in Hawaii; and
- 12 (5) Develop effective strategies to raise public awareness
13 of rare diseases in Hawaii.
- 14 (d) The advisory board shall report to the legislature on
15 existing sources of funding that may be used to finance the
16 formation and operation of the advisory board. The advisory
17 board shall apply for federal and local grant money that may be
18 available for programs related to rare diseases. The advisory
19 board shall report to the department of health and the
20 legislature biennially on its findings and recommendations.



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1 (e) For the purposes of this section, "rare disease" means
2 a condition that affects fewer than two hundred thousand people
3 in the United States."

4 SECTION 3. New statutory material is underscored.

5 SECTION 4. This Act shall take effect upon its approval.

6

INTRODUCED BY: *Rosalyn H Baker*
By Request



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Report Title:

Rare Disease Advisory Board; Department of Health; Rare Diseases

Description:

Establishes the rare disease advisory board within the department of health to provide education about rare diseases and encourage the funding of research in the development of new treatments for rare diseases. Describes duties of the advisory board, members of the board, and the board's obligation to coordinate with local and national groups conducting similar research and advocacy for persons with rare diseases.

The summary description of legislation appearing on this page is for informational purposes only and is not legislation or evidence of legislative intent.

