

JAN 20 2017

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 in the United
2 States, a rare disease is a condition that affects fewer than
3 two hundred thousand people. A rare disease is sometimes called
4 an orphan disease. There are seven thousand known rare diseases
5 affecting approximately thirty million men, women, and children
6 in the United States. About half of all rare diseases affect
7 children.

8 The legislature further finds that the exact cause for many
9 rare diseases remains unknown. However, eighty per cent of rare
10 diseases are genetic in origin and can be linked to mutations in
11 a single gene or in multiple genes. Such diseases are referred
12 to as genetic diseases and can be passed down through
13 generations in families.

14 The legislature also finds that a person suffering with a
15 rare disease in Hawaii faces a wide range of challenges,
16 including: delays in obtaining a diagnosis, misdiagnosis,
17 shortage of medical specialists familiar with rare diseases,



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

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

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

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



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

(2) Acting as the advisory body on rare diseases to the legislature and state departments and agencies that serve persons with rare diseases; and

(3) Coordinating the performance of the advisory board's duties with other state rare disease advisory bodies, community-based organizations, other public and private organizations, and federal agencies such as the National Institutes of Health and the Food and Drug Administration, to ensure cooperation between state and federal governments in activities regarding rare diseases.

(b) The advisory board shall consist of:

(1) Directors or ex officio members of the department of health and other state agencies concerned with the provision of care to persons with rare diseases; and

(2) The following public members, who shall be appointed by the director:



S.B. NO. 127

(A) Two physicians licensed to practice in Hawaii who have expertise in treating patients with rare diseases, one of whom shall be a pediatrician who provides care to children with rare diseases;

(B) A registered professional nurse licensed in Hawaii who has expertise in providing care to patients with rare diseases;

(C) Two representatives of hospitals in Hawaii;

(D) A representative of the health insurance industry;

(E) A representative of the biopharmaceutical industry;

(F) A representative of the scientific community who is engaged in rare disease research;

(G) Two parents of a child with a rare disease;

(H) Two persons with a rare disease; and

(I) Two patient organizations.

The advisory board may advise the department of health on additional at-large appointments that may be necessary to carry out the advisory board's duties. At-large appointments to the advisory board may serve on an ad hoc basis. Vacancies in



1 membership shall be filled in the same manner as the original
2 appointments. Public members of the board shall serve without
3 compensation.

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

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

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

16 (c) The advisory board shall:

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



1 subject to all applicable privacy laws and
2 protections;

3 (2) Ensure that data collection and patient surveys are
4 carried out in a way that is coordinated and
5 interoperable with similar research being conducted at
6 the state and federal levels;

7 (3) Research and identify priorities relating to the
8 quality and cost-effectiveness of, and access to,
9 treatment and services provided to persons with rare
10 diseases in Hawaii and develop policy recommendations;

11 (4) Identify best practices for rare disease care from
12 other states and nationally to improve rare disease
13 care in Hawaii; and

14 (5) Develop effective strategies to raise public awareness
15 of rare diseases in Hawaii.

16 (d) The advisory board shall report to the legislature on
17 existing sources of funding that may be used to finance the
18 formation and operation of the advisory board. The advisory
19 board shall apply for federal and local grant money that may be
20 available for programs related to rare diseases. The advisory



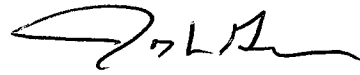
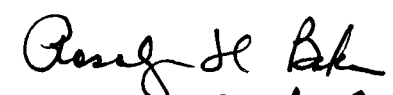
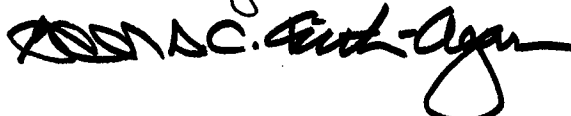
1 board shall report to the department of health and the
2 legislature biennially on its findings and recommendations.

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

6 SECTION 3. New statutory material is underscored.

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

8
INTRODUCED BY:



S.B. NO. 727

Report Title:

Rare Disease Advisory Board

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.

