

[Home](#)[Bill Information](#)[California Law](#)[Publications](#)[Other Resources](#)[My Subscriptions](#)[My Favorites](#)**SB-247 Rare Disease Advisory Council.** (2021-2022)

SHARE THIS:



Date Published: 09/07/2021 09:00 PM

ENROLLED SEPTEMBER 07, 2021

PASSED IN SENATE JUNE 01, 2021

PASSED IN ASSEMBLY SEPTEMBER 02, 2021

AMENDED IN SENATE MAY 20, 2021

AMENDED IN SENATE MARCH 01, 2021

CALIFORNIA LEGISLATURE— 2021–2022 REGULAR SESSION

SENATE BILL**NO. 247****Introduced by Senator Eggman****(Coauthors: Senators Bates, Grove, Hurtado, Laird, Newman, and Rubio)****(Coauthors: Assembly Members Fong and Levine)****January 22, 2021**

An act to add Part 4.6 (commencing with Section 124965) to Division 106 of the Health and Safety Code, relating to rare diseases.

LEGISLATIVE COUNSEL'S DIGEST

SB 247, Eggman. Rare Disease Advisory Council.

Existing law establishes the California Health and Human Services Agency, which includes the State Department of Public Health, among other state departments charged with the administration of health, social, and other human services. Under existing law, the State Department of Public Health has authority over various programs promoting public health, including genetic disease testing and newborn screenings.

This bill would establish the Rare Disease Advisory Council within the California Health and Human Services Agency.

This bill would specify the purposes of the Rare Disease Advisory Council, including, among others, coordinating statewide efforts for the study of the incidence of rare diseases within the state, and acting as the advisory body on rare diseases to the Legislature and state and private agencies that provide services to persons with rare diseases. Under the bill, the duties of the advisory council would include, among others, adopting implementing regulations, researching and determining the most appropriate method to collect data on rare diseases, and identifying best practices for rare disease care.

The bill would specify the composition of the advisory council, including members appointed by the Secretary of California Health and Human Services and the State Public Health Officer. Before any appointments are made, the bill would require the agency to research and report to the Legislature on existing sources of funding that may be used to finance the formation and operation of

the advisory council. The bill would require the advisory council to apply for, and accept, any grant of funds from the federal government, private foundations, or other sources. The bill would prohibit the advisory council from accepting funds from the employer of any sitting council member.

The bill would require the advisory council to meet at least 3 times annually and to report to the agency and the Legislature every 2 years on its activities, findings, and recommendations.

Vote: majority Appropriation: no Fiscal Committee: yes Local Program: no

THE PEOPLE OF THE STATE OF CALIFORNIA DO ENACT AS FOLLOWS:

SECTION 1. The Legislature finds and declares all of the following:

(a) Under federal law, including the Orphan Drug Act of 1983 and its amendments and the Rare Diseases Act of 2002, a rare disease is generally defined as a disease that affects fewer than 200,000 persons in the United States. Rare diseases are sometimes called orphan diseases. There are about 7,000 known rare diseases affecting approximately 30,000,000 men, women, and children in the United States.

(b) The exact cause for many rare diseases remains unknown. However, 80 percent of rare diseases are genetic in origin and can be linked to mutations in a single gene or in multiple genes. Those diseases are referred to as genetic diseases. A genetic disease can be passed down from generation to generation, explaining why certain rare diseases run in families. It is also estimated that about one-half of all rare diseases affect children.

(c) A person suffering from a rare disease in this state faces a wide range of challenges, including, but not limited to, delays in obtaining a diagnosis, a misdiagnosis, a shortage of medical specialists familiar with and who can provide treatment for rare diseases, the prohibitive costs of treatment, and the inability to access therapies and medication that are used by physicians to treat rare diseases but that have not been approved by the United States Food and Drug Administration for that specific purpose.

(d) In recent years, researchers have made considerable progress in developing diagnostic tools and treatment protocols for, and in discovering ways to prevent, a variety of rare diseases. However, much more remains to be done in the areas of rare disease research and the search for, and development of, new therapeutics.

(e) It is, therefore, an appropriate public policy for this state to establish an advisory body whose membership would be comprised of persons with suitable qualifications in the field of rare diseases, including persons living with rare diseases, to educate medical professionals, government agencies, and the public about rare diseases as an important public health issue, and to encourage and fund research in the development of new treatments for rare diseases.

SEC. 2. Part 4.6 (commencing with Section 124965) is added to Division 106 of the Health and Safety Code, to read:

PART 4.6. Rare Disease Advisory Council

124965. (a) There is hereby established, within the California Health and Human Services Agency, the Rare Disease Advisory Council.

(b) For purposes of this part, the following terms have the following meanings:

(1) "Advisory council" means the Rare Disease Advisory Council, unless otherwise specified.

(2) "Rare disease" has the same meaning as provided in Section 360bb of Title 21 of the United States Code, or its successor.

124965.1. (a) The advisory council shall consist of the following members:

(1) Directors, or ex officio members, within the California Health and Human Services Agency and other state agencies concerned with the provision of care to persons with rare diseases, to be appointed by the Secretary of California Health and Human Services.

(2) The chair of the Medi-Cal Drug Use Review Board, or the chair's designee, who shall serve as an ex officio, nonvoting member.

(3) Public members, to be appointed by the State Public Health Officer, who shall include all of the following:

(A) Two physicians and surgeons licensed to practice in this state 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 to practice in this state who has expertise in providing care to patients with rare diseases.
- (C) A representative of the hospital administration in this state who treats rare disease patients.
- (D) A representative of outpatient health care administration in this state who treats rare disease patients.
- (E) A representative of the health care coverage industry.
- (F) A representative of the biopharmaceutical industry.
- (G) A representative of the scientific community who is engaged in rare disease research or is a rare disease geneticist or genetic counselor.
- (H) Five individuals who are either a rare disease patient or family member of a rare disease patient. Neither group shall hold more than three seats at one time.
- (I) A medical social worker who works with rare disease patients.
- (J) Two patient advocacy organizations that operate within this state.
- (b) On or after the date on which the advisory council is first convened, the advisory council may advise the California Health and Human Services Agency on additional at-large appointments to the advisory council that may be necessary to carry out its duties. At-large appointments to the advisory council may serve on an ad hoc basis.
- (c) Members of the advisory council shall serve without compensation. However, public members appointed pursuant to paragraph (3) of subdivision (a) may be reimbursed for travel and other miscellaneous expenses necessary to perform their duties within the limits of funds made available to the advisory council for its purposes.
- (d) The advisory council shall organize as soon as practicable after the appointment of its members and shall select a chairperson and vice chairperson from among its members. The chairperson shall appoint a secretary who need not be a member of the advisory council.
- (e) The advisory council shall meet periodically, but at least three times annually. The advisory council may call to its assistance, and avail itself of the services of, the employees of any state, county, or municipal department, board, bureau, commission, or agency as it may require and as may be available to it for its purposes, if those employees elect to participate.
- (f) The term of each member of the advisory council is three years, to be staggered so that approximately one-third of the appointments expire each year. Vacancies in the membership of the advisory council shall be filled in the same manner as provided for in the original appointments.
- (g) No single disease population shall have more than two representatives on the council.
- (h) Each member of the advisory council shall annually sign a conflict of interest statement disclosing any economic or other relationship with an entity that could influence the member's decisions. At least 20 percent of the council's members shall not have a conflict of interest with respect to an insurer, pharmaceutical benefits manager, or pharmaceutical manufacturer.

124965.2. (a) The following are the purposes of the advisory council:

- (1) Coordinating statewide efforts for the study of the incidence of rare diseases within this state and the status of the rare disease community.
- (2) Acting as the advisory body on rare diseases to the Legislature, and state departments, agencies, commissions, and authorities, and private agencies, that provide services to, or that are charged with the care of, persons with rare diseases.
- (3) Serving as an expert advisory committee to the Medi-Cal Drug Use Review Board, providing necessary consultation to the board when the board makes recommendations or determinations regarding beneficiary access to drugs or biological products for rare diseases, or when the board itself determines that it lacks the specific scientific, medical, or technical expertise necessary for the proper performance of its responsibilities and such necessary expertise can be provided by experts outside the board. As used in this section, "beneficiary access" means developing prior authorization and reauthorization criteria for a rare disease drug, including placement on a preferred drug list or a formulary, as well as payment, cost sharing, drug utilization review, or medication therapy management.
- (4) (A) Coordinating the performance of the advisory council's duties with any other state entities acting as advisory bodies on rare diseases, community-based organizations, and other public or private organizations, for the purpose of ensuring greater cooperation between state and federal activities regarding the research, diagnosis, and treatment of rare diseases.

(B) Federal agencies with which the advisory council may seek to coordinate include, but are not limited to, the National Institutes of Health and the United States Food and Drug Administration.

(C) The coordination described in subparagraph (A) shall require, when appropriate, both of the following:

(i) Disseminating the advisory council's research, identified best practices, and policy recommendations.

(ii) Utilizing common research collection and dissemination procedures.

(b) The following are the duties of the advisory council:

(1) Research and determine the most appropriate method to collect data on rare diseases, and information concerning patients, as the advisory council deems necessary and appropriate by conducting thorough and complete surveys of rare diseases diagnosed in this state, subject to all applicable privacy laws and protections. The advisory council shall ensure that the duties described in this paragraph are carried out in a manner that is coordinated and interoperable with similar research being conducted at the state and federal levels.

(2) Research and identify priorities relating to the quality and cost-effectiveness of, and access to, diagnosis, treatment, and services provided to persons with rare diseases in this state, and develop policy recommendations on those issues.

(3) Identify best practices for rare disease care from other states and at the national level that will improve rare disease care in this state.

(4) Develop effective strategies to raise public awareness of rare diseases in this state.

(5) Determine the best methods for the creation of a rare disease fund housed in the Department of Treasury to be used to aid the council in fulfilling their duties.

(6) Adopt regulations for the implementation of this part.

124965.3. (a) Before any appointment to the advisory council is made pursuant to Section 124965.1, the California Health and Human Services Agency shall research and report to the Legislature on existing sources of funding that may be used to finance the formation and operation of the advisory council.

(b) The advisory council shall apply for, and accept, any grant of funds from the federal government, private foundations, or other sources, that may be available for programs related to rare diseases. The council shall not accept any funds from the employer of a currently seated council member.

(c) The advisory council shall report to the California Health and Human Services Agency, and to the Legislature, every two years on the activities of the advisory council and its findings and recommendations on issues relating to the quality and cost-effectiveness of, and access to, treatment and services provided to persons with rare diseases in this state.

(d) Any reports submitted to the Legislature pursuant to this section shall be submitted in compliance with Section 9795 of the Government Code.