SENATE COMMITTEE ON HEALTH
Senator Dr. Richard Pan, Chair

BILL NO: SB 247
AUTHOR: Eggman
VERSION: January 22, 2021
HEARING DATE: March 10, 2021
CONSULTANT: Kimberly Chen

SUBJECT: Rare Disease Ombudsperson and Rare Disease Advisory Council

SUMMARY: Establishes the Office of the Rare Disease Ombudsperson (Ombudsperson) and the Rare Disease Advisory Council (Council) within the California Health and Human Services Agency (CHHSA). Establishes the duties and responsibilities of the Ombudsperson and Council. Establishes requirements for the membership of the Council and the manner in which members are appointed. Requires CHHSA to report on existing funding sources to support the operation of the Council prior to appointing the Council. Requires the Council to report to CHHSA the activities of the Council and its findings and recommendations on issues relating to persons with rare diseases, as specified.

Existing law:
1) Establishes in state government the California Health and Human Services Agency (CHHSA) and within CHHSA, among other departments, offices and councils, the Department of Health Care Services (DHCS) and the California Department of Public Health (CDPH). [GOV §12800, 12803]

2) Establishes in state government the State Public Health Officer, who serves as the director of CDPH. [HSC §131005]

3) Establishes the California Children's Services Program (CCS), administered by the DHCS, under which individuals under the age of 21, who have specified health conditions and meet financial requirements, are eligible to receive medically necessary services and treatments. [HSC §123800, et seq.]

4) Establishes the Genetically Handicapped Persons Program (GHPP), administered by the DHCS, under which individuals who have specified genetic conditions are eligible to receive medical care and social support services. [HSC §125125 - 125191]

5) Establishes the Hereditary Disorders Act, under which CDPH administers the Genetic Disease Screening Program (GDSP), which provides a statewide program for newborn and prenatal testing for genetic disorders and tracking of birth defects. Requires GDSP to provide genetic screenings and follow up services for persons who have the screening, as specified. [HSC §124975-125119.5]

This bill:
1) Establishes Ombudsperson within CHHSA. Requires the Governor, with recommendations by the CHHSA, to appoint the Rare Disease Ombudsperson for a four-year term. Requires a vacancy be filled within 60 days and in the same manner as the appointment was made.

2) Requires the Ombudsperson to serve at the pleasure of the Governor. Authorizes the Governor to remove the Ombudsperson for just cause upon recommendation the Secretary of CHHSA or a majority of the Council.

3) Requires the Ombudsperson to do all of the following:

   a) Advocate for the needs of persons diagnosed with rare diseases within state departments, public agencies, or health and social services agencies, as appropriate;
   b) Receive and refer complaints that are made by, or on behalf of, rare disease patients to the appropriate state departments, public agencies, or health and social services agencies that may investigate and resolve those complaints;
   c) Perform other duties related to rare diseases as determined necessary by the Ombudsperson, the Council, and CHHSA; and,
   d) Create, develop, and maintain a resource guide for rare disease patients to be housed at CHHSA and to be made available to the public.

4) Establishes the Council within the CHHSA. Requires the Council to consist of the following members:

   a) Directors, or ex officio members, within the CHHSA and other state agencies concerned with the provision of care to persons with rare diseases, appointed by the Secretary of CHHA;
   b) The Ombudsperson;
   c) Chair of the Medi-Cal Drug Use Review Board, or the chair's designee, who will serve as an ex officio, nonvoting member; and,
   d) Public members, appointed by the State Public Health Officer, who include all of the following:
      i) Two physicians and surgeons licensed to practice in this state who have expertise in treating patients with rare diseases, one of whom is a pediatrician who provides care to children with rare diseases;
      ii) A registered professional nurse licensed to practice in this state who has expertise in providing care to patients with rare disease;
      iii) Two representatives of hospitals in this state who treat rare disease patients;
      iv) A representative of the health care coverage industry;
      v) A representative of the biopharmaceutical industry;
      vi) A representative of the scientific community who is engaged in rare disease research or is a geneticist;
      vii) Five individuals who are either a rare disease patient or family member of a rare disease patient. Prohibits either group from holding more than three seats at one time;
      viii) A medical social worker who works with rare disease patients; and,
      ix) Two patient advocacy organizations that operate within this state.

5) Authorizes the Council, on or after its first meeting, to advise the CHHSA on additional at-large appointments to the advisory council, which shall serve on the Council on an ad-hoc basis.

6) Requires the term of each member to be three years and to be staggered so that approximately one-third of the appointments expire each year. Requires vacancies to be filled in the same manner as the original appointments.

7) Prohibits any single disease population from having more than two representatives on the Council. Requires each member of the Council to annually sign a conflict of interest statement disclosing any economic or other relationship with an entity that could influence the member's decisions. Prohibits at least 20% of the Council's members from having a conflict of interest with an insurer, pharmaceutical benefits manager, or pharmaceutical manufacturer.

8) Requires the members of the Council to serve without compensation. Authorizes public members to be reimbursed for travel and other expenses necessary to perform their duties within the limits of funds made available to the Council.

9) Requires the Council to organize as soon as practicable after the appointment of its members and to select a chairperson and vice chairperson. Requires the chairperson to appoint a secretary who need not be a member of the council. Requires the council to meet at least three times annually. Authorizes the council to request and receive assistance from the employees of any state, county, or municipal department, board, bureau, commission, or agency, who volunteers to participate.

10) Requires the Council's purpose and duties to include the following:

    a) Coordinate statewide efforts for the study of the incidence of rare diseases within this state and the status of the rare disease community;
    b) Serve 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.;
    c) Coordinate with any other state entities that act as advisory bodies on rare diseases, community-based organizations, and other public or private organizations, in order to ensure greater cooperation of state and federal activities on the research, diagnosis, and treatment of rare diseases. Authorizes the Council to coordinate with federal agencies, including but not limited to, the National Institutes of Health and the United States Food and Drug Administration. Authorizes coordination to include both disseminating the council's research, best practices, and policy recommendations and utilizing common research collection and dissemination procedures;
    d) Research and determine the most appropriate method to collect data on rare diseases, and information concerning patients by conducting surveys of rare diseases diagnosed in this state, subject to all applicable privacy laws and protections and in a manner that interoperable with similar state and federal research;
    e) 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, and develop policy recommendations;
    f) Identify best practices for rare disease care from other states and at the national level that will improve rare disease care;
    g) Develop effective strategies to raise public awareness of rare diseases; and,
    h) Determine the best methods for the creation of a rare disease fund housed in the Department of Treasury to be used by Council and the Ombudsperson to fulfill their duties.

11) Defines "beneficiary access" to mean 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.

12) Requires the Council, with assistance from the Ombudsperson, to adopt regulations to implement this bill.

13) Requires CHHSA to research and report to the Legislature on existing sources of funding that may be used to finance the formation and operation of the Council prior to appointing the Council. Requires the Council to 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.

14) Prohibits the Council from accepting any funds from the employer of any current Council member.

15) Requires the Council to report to CHHSA and to the Legislature, every two years on the activities of the 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. Requires any reports submitted to the Legislature to be in compliance with the requirements of existing law.

16) Makes legislative findings and declarations regarding rare diseases and the need for an advisory body and ombudsperson to support 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.

FISCAL EFFECT: This bill has not been analyzed by a fiscal committee.

COMMENTS:

1) *Author's statement.* According to the author, the rare disease community is one of the most underserved communities in health world. Very few people outside the community have any knowledge of the maintenance, struggles, and barriers rare disease patient face every day. The Council and Ombudsperson envisioned in this bill could make important progress towards addressing the issues faced by rare disease patients through recommendations on disease representation, specialist physician directories, understanding financial impacts to patients and the state, and communicating what private and public resources may be available to patients and their families across the state. The Council and Ombudsperson give rare disease patients a much needed departmental voice to advocate and strategically plan for the future of rare disease. As has been adopted in states like Pennsylvania, North Carolina, and Connecticut, a council can use a diverse group of experts to craft recommendations for the state on how best to address these issues faced by the nearly four million Californians with rare diseases and their caregivers.

2) *Rare diseases.* According to the National Human Genome Institute, a rare disease is a condition that affects fewer than 200,000 people in the United States. There are more than 6,800 rare diseases. Altogether, rare diseases affect an estimated 25 million to 30 million Americans. Examples of rare diseases include life-threatening and physically or mentally

disabling conditions such as Huntington disease, spina bifida, fragile X syndrome, Guillain-Barré syndrome, Crohn disease, cystic fibrosis, and Duchenne muscular dystrophy. According to a 2016 essay published in Preventing Chronic Disease: Public Health Research, Practice, and Policy, rare disease patients are few and scattered across populations and many rare diseases have a long list of characteristics that present serious challenges for public health practitioners. Among rare diseases it is common to find that: a) diagnoses are difficult and delayed; b) case definitions for surveillance are usually lacking; c) International Classification of Diseases (ICD) codes for record keeping are poorly defined or not assigned; d) underlying molecular or physiologic mechanisms are unknown; e) specialized and coordinated medical care is in short supply, and treatments can be complex; g standards of care for treatment and rehabilitation are not evidence-based because health research is necessarily done at small scale; f) longitudinal data collections are scarce; g) the development of new medications and treatments can be fragmented and slow; h) screening strategies lack efficiency; and, i) scope and capacity of most registries and databases are limited. The knowledge of most rare diseases is so insufficient that they are also known as orphan diseases (and their treatments known as orphan drugs) because of their failure to attract the interest of researchers, medical specialists, drug makers, and policy makers.

The essay recommends five goals for a comprehensive public health approach to rare diseases and its potential impact on affected populations. The goals include: a) defining and examining the impact of the disease; b) developing common surveillance practices; c) improving knowledge about the disease; d) support health care systems in treating the diseases; and, e) reducing the impact of the diseases on patients, their relatives and caregivers, and society in general.

3) *State programs related to rare diseases.* DHCS and CDPH operate state programs that provide services and screenings for individuals with certain diseases and health problems, which include some rare diseases. These programs include the CCS program, GHPP, and GDSP.

   a) *CCS.* CCS is a state-only health program for children, up to the age of 21, with certain diseases or health problems, including bleeding disorders, cystic fibrosis, Duchenne muscular dystrophy, and other conditions. CCS requires applicants to meet additional requirements, such as income limits or expected out-of-pocket medical expenses. CCS provides coverage for a variety of medical visits, medical case management, and care at sickle cell disease centers.

   b) *GHPP*. GHPP is a state-only health program that provides care for adults, aged 21 years and older, with certain genetic diseases, including rare blood diseases, cystic fibrosis, diseases of brain, nerves and metabolism, and other diseases. There is no income limit; however, applicants may be required to pay an annual enrollment fee, which is based on income and family size. The GHPP program benefits include coverage for hospital stays, outpatient medical care, pharmaceutical services, surgeries, nutritional products, durable medical equipment, care at a Special Care Center, and other services. Individuals with private health insurance or Medicare coverage may apply for GHPP but the program will only cover limited services.

   c) *GDSP*. CDPH administers the GDSP, which screens newborns and pregnant women for genetic and congenital disorders. GDSP operates two primary screening programs: the Newborn Screening (NSB) program and the Prenatal Screening (PNS) program.

Established in 1966, NSB provides screenings for every child born in California and includes screenings for more than 80 separate disorders. GDSP conducts screening, clinical, and surveillance activities statewide through public-private partnerships. The program contracts with five regional laboratories for initial screening activities and other private laboratories for confirmatory testing. GDSP also contracts with major medical centers, regional offices, and community organizations for diagnostic and follow-up services.

4) *Federal Orphan Drug Act (ODA)*. Under the ODA, the Federal Drug Administration (FDA) can grant special status to a drug or biological product to treat a rare disease or condition. This status, known as orphan designation, qualifies the sponsor of the drug for various development incentives of the ODA, including tax credits for qualified clinical testing. The Office of Orphan Products Development (OOPD) administers the program that provides orphan drug status to drugs and biologics. According to the FDA, this designation program has successfully enabled the development and marketing of over 600 drugs and biologic products for rare diseases since 1983. In contrast, fewer than 10 such products supported by industry came to market between 1973 and 1983. The OOPD also administers a number of voucher and grant programs to support the development and use of drugs treating rare diseases.

5) *Prior legislation.* AB 2283 (Eggman of 2020) was identical to this bill. *AB 2283 was not heard by the Assembly Health Committee.*

   AB 1016 (Maienschein of 2019) would have established the Rare Disease Advisory Council. *AB 1016 was held on the Assembly Appropriations suspense file.*

   ACR 28 (Gipson of 2019) would have recognized September 2020 as Sickle Cell Disease Awareness Month and encourages the Legislature to appropriate funds for research, treatment, monitoring, education, and outreach related to the disease. *ACR 28 was not heard by the Senate Health Committee.*

6) *Support*. National Organization for Rare Disorders (NORD) and a number of organizations in a coalition letter write in support of this bill. They state that creating the Council and Ombudsperson will give rare disease patients a unified voice in California state government. They argue that the Council will be a valuable advisory body to elected officials and other state leaders on rare disease research, beneficiary access to treatments, and best practices for the care of those with rare diseases. They state this bill is needed because it will allow the Council and Ombudsperson to directly engage with a diverse group of stakeholders interested in identifying and solving pressing challenges for people with rare diseases.

SUPPORT AND OPPOSITION:

Support: Americans for Cures
American Kidney Fund
ALS Association, Golden West Chapter
Axis Advocacy
Be The Match/ National Marrow Donor Program
Biocom
CA Action Link for Rare Diseases
Cystic Fibrosis Research, Inc.
FAIR Foundation

Immune Deficiency Foundation
The Leukemia & Lymphoma Society
Liver Coalition of San Diego
Lymphedema Advocacy Group
MiOra
Neuromuscular Disease Foundation
National Organization for Rare Disorders
Necrotizing Enterocolitis (NEC) Society
Sick Cells
The Wall Las Memorias
Five Individuals

Oppose: None received

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