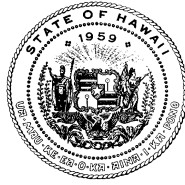


SCR 37

Measure Title: URGING THE ESTABLISHMENT OF A RARE DISEASE TASK FORCE.
Report Title: Rare Disease Task Force
Description:
Companion:
Package: None
Current Referral: CPH, WAM
Introducer(s): GREEN, Ruderman, Taniguchi



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Testimony in OPPOSITION to S.C.R. 37
URGING THE ESTABLISHMENT OF A RARE DISEASE TASK FORCE

ROSALYN H. BAKER, CHAIR
SENATE COMMITTEE ON COMMERCE, CONSUMER PROTECTION, AND HEALTH
Hearing Date: March 15, 2017 Room Number: 229

1 **Fiscal Implications:** The Department will need resources to staff the task force. It is unlikely
2 that the Department will be able to obtain additional federal or private funding for rare disease
3 activities, since most funding opportunities require a minimal threshold of affected individuals to
4 qualify to compete for the funding. Hawaii's population is too small to qualify.

5 **Department Testimony:** This concurrent resolution urges the Department to establish a Rare
6 Disease Task Force to investigate and make recommendations on the issues relating to quality,
7 cost effectiveness, and accessibility of treatment and services to persons with rare diseases in the
8 Hawaii. The Department recognizes that the challenges facing individuals and families with rare
9 diseases in Hawaii are similar to those faced across the nation, since specialists who treat or
10 research rare disorders are scarce.

11 As there is often only one expert in the county or world that works on a rare disease, it would be
12 difficult for a state task force to have enough expertise about rare diseases and accurate and up-
13 to-date information to investigate and make recommendations about treatment, quality of
14 services, and accessibility of services for the many rare diseases.

15 Instead of creating a state task force on rare disease, it would be more effective for individuals or
16 families affected with rare diseases to work with the National Organization of Rare Diseases
17 (NORD) or disease specific advocacy groups. Working with these national and international
18 organizations will allow individuals and families in Hawaii to have a greater voice in advocating
19 for resources, research, and access to treatment if known. It will also allow for more
20 coordination of efforts and support among affected individuals and families since most funding

1 for research or development of resources is not available unless a large pool of affected
2 individuals is identified.

3 Hawaii individuals/families with Medicaid coverage may contact their QUEST health plan or
4 Medicaid ombudsman about questions and concerns about treatment, quality, or accessing
5 services. Parents or caregivers of children with special health care needs may contact the
6 Hilopa‘a Family to Family Health Information for information or assistance in navigating the
7 health care system. The Special Parent Information Network (Hawaii) is a parent to parent
8 organization in Hawaii that provides information, support and referral to parents of children and
9 young adults with disabilities and the professionals who serve them; its website includes a list of
10 Hawaii support groups and links to national resources. The NORD website has a list of
11 organizations and resources for patients and families affected by rare diseases.

12 Thank you for the opportunity to testify.