

**ASSEMBLY, No. 2528**

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**STATE OF NEW JERSEY**

**218th LEGISLATURE**

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INTRODUCED FEBRUARY 1, 2018

**Sponsored by:**

**Assemblyman RONALD S. DANCER**

**District 12 (Burlington, Middlesex, Monmouth and Ocean)**

**SYNOPSIS**

Establishes the New Jersey Rare Disease Advisory Council.

**CURRENT VERSION OF TEXT**

As introduced.



1 AN ACT establishing the New Jersey Rare Disease Advisory  
2 Council and supplementing Title 26 of the Revised Statutes.

3  
4 **BE IT ENACTED** *by the Senate and General Assembly of the State*  
5 *of New Jersey:*

6  
7 1. The Legislature finds and declares:

8 a. A rare disease is defined as a disease that affects fewer than  
9 20,000 people. Rare diseases are sometimes called orphan diseases.  
10 There are 7,000 rare diseases affecting approximately 25 to 30  
11 million Americans;

12 b. The exact cause for many rare diseases remains unknown.  
13 However, 80% of rare diseases are genetic in origin and can be  
14 linked to mutations in a single gene or in multiple genes. Such  
15 diseases are referred to as genetic diseases. Genetic disease can be  
16 passed down from generation to generation, explaining why certain  
17 rare diseases run in families. It is also estimated that about half of  
18 all rare diseases affect children;

19 c. Environmental factors such as diet, smoking, or exposure to  
20 chemicals also play a part in causing rare diseases. These factors  
21 may directly cause, or interact with, genetic factors that increase the  
22 severity of rare disease;

23 d. Rare diseases can be chronic, progressive, debilitating,  
24 disabling, severe, and life threatening. A person suffering with a  
25 rare disease faces a wide range of challenges, including, but not  
26 limited to: delays in obtaining a diagnosis; being misdiagnosed; a  
27 shortage of medical specialists who are familiar with, and can  
28 provide treatment for, rare diseases; the prohibitive cost of  
29 treatment; and the inability to access therapies and medication that  
30 are used by doctors to treat rare diseases but have not been  
31 approved by the Federal Food and Drug Administration for that  
32 specific purpose;

33 e. In recent years, researchers have made considerable progress  
34 in developing diagnostic tools and treatment protocols for, and in  
35 discovering ways to prevent a variety of, rare diseases. However,  
36 much more remains to be done in the areas of rare disease research  
37 and the search for new therapeutics; and

38 f. It is therefore an appropriate public policy for the State of  
39 New Jersey to establish an advisory body, whose membership  
40 would be comprised of persons with suitable qualifications for this  
41 purpose, to examine the issues that affect persons with rare  
42 diseases, to educate medical professionals, government agencies,  
43 and the public about rare diseases as an important public health  
44 issue, and to encourage and fund research in the development of  
45 new treatments for rare diseases.

46  
47 2. There is established the New Jersey Rare Disease Advisory  
48 Council in the Department of Health.

1       a. The advisory council shall consist of 16 members as follows:

2       (1) the Commissioners of Health, Human Services, Children and  
3 Families, and Environmental Protection, or their designees, as ex  
4 officio members; and

5       (2) twelve public members to be appointed by the Governor,  
6 who shall include: two physicians licensed to practice in this State  
7 who have expertise in treating patients with rare diseases, one of  
8 whom shall be a pediatrician who provides care to children with  
9 rare diseases; a registered professional nurse licensed in this State  
10 who has expertise in providing care to patients with rare diseases; a  
11 patient advocate employed by a general hospital in this State; two  
12 representatives of general hospitals in this State, one of whom shall  
13 represent a religiously-affiliated hospital; a representative of the  
14 New Jersey Office on Minority and Multicultural Health; a  
15 representative of the health insurance industry, a representative of  
16 the pharmaceutical industry; a representative of the scientific  
17 community who is engaged in rare disease research; a parent of a  
18 child with a rare disease; and a person with a rare disease.

19       b. Vacancies in the membership of the advisory council shall  
20 be filled in the same manner provided for the original appointments.  
21 The public members of the council shall serve without  
22 compensation but may be reimbursed for traveling and other  
23 miscellaneous expenses necessary to perform their duties within the  
24 limits of funds made available to the council for its purposes.

25       c. The advisory council shall organize as soon as practicable  
26 after the appointment of its members and shall select a chairperson  
27 and vice-chairperson from among its members. The chairperson  
28 shall appoint a secretary who need not be a member of the council.

29       d. The advisory council may meet at the call of its chair and  
30 hold hearings at the times and in the places it deems appropriate and  
31 necessary to fulfill its charge. The council shall be entitled to call  
32 to its assistance, and avail itself of the services of the employees of,  
33 any State, county, or municipal department, board, bureau,  
34 commission, or agency as it may require and as may be available to  
35 it for its purposes.

36       e. The Department of Health shall provide staff services to the  
37 advisory council.

38

39       3. The purpose of the advisory council shall be to:

40       a. act as the advisory body on rare diseases to the Legislature  
41 and State departments, agencies, commissions, authorities, and  
42 private agencies that provide services to, or are charged with the  
43 care of, persons with rare diseases;

44       b. assist the Commissioner of Health in establishing and  
45 implementing a rare disease registry program to serve as a single  
46 repository of information on all rare diseases diagnosed in this  
47 State;

1 c. conduct a thorough and comprehensive study of all issues  
2 relating to the quality and cost-effectiveness of, and access to,  
3 treatment and services provided to persons with rare diseases in this  
4 State, and to develop policy recommendations on those issues;

5 d. identify effective research-based strategies that have been  
6 developed to help diagnose, treat, and prevent rare diseases; and

7 e. develop effective strategies to raise public awareness of rare  
8 diseases in this State.

9  
10 4. The advisory council shall apply for, and accept, any grant  
11 of money from the federal government, private foundations, or  
12 other sources, which may be available for programs related to rare  
13 diseases.

14  
15 5. The advisory council shall report to the Governor and,  
16 pursuant to section 2 of P.L.1991, c.164 (C.52:14-19.1), to the  
17 Legislature, no later than December 31st of each year, on the  
18 activities of the advisory council and its findings and  
19 recommendations on issues relating to the quality and cost-  
20 effectiveness of, and access to treatment and services to, persons  
21 with rare diseases in this State.

22  
23 6. The Commissioner of Health, pursuant to the  
24 "Administrative Procedures Act," P.L.1968, c.410 (C.52:14B-1 et  
25 seq.) shall adopt rules and regulations necessary to effectuate the  
26 purposes of this act.

27  
28 7. This act shall take effect immediately.

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31 STATEMENT

32  
33 This bill establishes the 16-member New Jersey Rare Disease  
34 Advisory Council in the Department of Health.

35 The membership of the advisory council will consist of: the  
36 Commissioners of Health, Human Services, Children and Families,  
37 and Environmental Protection, or their designees, as ex officio  
38 members; and twelve public members to be appointed by the  
39 Governor, including two physicians licensed to practice in this State  
40 who have expertise in treating patients with rare diseases, one of  
41 whom is a pediatrician providing care to children with rare diseases;  
42 a registered professional nurse licensed in this State who has  
43 expertise in providing care to patients with rare diseases; two  
44 representatives of general hospitals in this State, one of whom  
45 represents a religiously-affiliated hospital; a patient advocate  
46 employed by a general hospital in this State; a representative of the  
47 New Jersey Office on Minority and Multicultural Health, the health  
48 insurance industry, the pharmaceutical industry, and the scientific

1 community who is engaged in rare disease research; a parent of a  
2 child with a rare disease; and a person with a rare disease.

3 The purpose of the advisory council will be to: act as the  
4 advisory body on rare diseases to the Legislature and State  
5 departments, agencies, commissions, authorities, and private  
6 agencies that provide services to, or are charged with the care of,  
7 persons with rare diseases; assist the Commissioner of Health in  
8 establishing and implementing a rare disease registry program in  
9 this State; conduct a thorough and comprehensive study of all issues  
10 relating to the quality and cost-effectiveness of, and access to  
11 treatment and services provided for, persons with rare diseases in  
12 this State, and to develop policy recommendations on those issues;  
13 identify effective research-based strategies that have been  
14 developed to help diagnose, treat, and prevent rare diseases; and  
15 develop effective strategies to raise public awareness of rare  
16 diseases in this State.

17 Finally, the bill requires the advisory council to annually report  
18 to the Governor and the Legislature on its findings and  
19 recommendations on issues relating to the quality and cost-  
20 effectiveness of, and access to, treatment and services provided to  
21 persons with rare diseases in this State.

22 This bill is similar to H-7094, the "Rare Disease Community  
23 Support, Resource Coordination and Quality of Life Act of 2014,"  
24 that was adopted by the state of Rhode Island earlier this year.