

**House File 287 - Introduced**

HOUSE FILE 287

BY BODEN

**A BILL FOR**

1 An Act creating a rare disease advisory council.

2 BE IT ENACTED BY THE GENERAL ASSEMBLY OF THE STATE OF IOWA:

1 Section 1. FINDINGS.

2 1. A rare disease, sometimes called an orphan disease,  
3 is defined as a disease that affects fewer than two hundred  
4 thousand people in the United States.

5 2. There are seven thousand known rare diseases affecting  
6 approximately twenty-five to thirty million adults and children  
7 in the United States.

8 3. While the exact cause for many rare diseases remains  
9 unknown, many rare diseases are genetic in origin and can be  
10 linked to mutations in a single gene or in multiple genes which  
11 can be passed down from generation to generation.

12 4. People with rare diseases face many challenges,  
13 including delays in obtaining a diagnosis or misdiagnosis,  
14 shortages of medical specialists who can provide treatment, and  
15 lack of affordable access to therapies and medication used to  
16 treat rare diseases.

17 5. A state rare disease advisory council composed of  
18 qualified professionals and persons living with rare diseases  
19 could educate medical professionals, government agencies,  
20 legislators, and the public about rare diseases as an important  
21 public health issue and encourage and secure funding for  
22 research for the development of new treatments for rare  
23 diseases.

24 Sec. 2. NEW SECTION. 135.133 Rare disease advisory council  
25 — membership — duties.

26 1. A rare disease advisory council is created within the  
27 department for the purpose of advising the department and  
28 other state agencies, the general assembly, and the public on  
29 research, diagnosis, and treatment efforts related to rare  
30 diseases in this state.

31 2. The council shall consist of the following members:

32 a. The director of health and human services, or the  
33 director's designee.

34 b. The Medicaid director, or the director's designee.

35 c. The commissioner of insurance, or the commissioner's

1 designee.

2 *d.* The chairperson of the congenital and inherited disorders  
3 advisory committee, or the chairperson's designee.

4 *e.* All of the following appointed by the director:

5 (1) A representative from an academic research institution  
6 in the state that receives grant funding for rare disease  
7 research.

8 (2) A geneticist licensed and practicing in the state.

9 (3) A registered nurse or an advanced registered nurse  
10 practitioner licensed and practicing in the state with  
11 experience in treating rare diseases.

12 (4) A physician practicing in the state with experience in  
13 treating rare diseases.

14 (5) A hospital administrator, or the administrator's  
15 designee, from a hospital in the state that provides care to  
16 persons diagnosed with a rare disease.

17 (6) At least two persons diagnosed with a rare disease.

18 (7) At least two caregivers of persons diagnosed with a rare  
19 disease.

20 (8) A representative of a rare disease patient organization  
21 that operates in the state.

22 (9) A pharmacist with experience dispensing drugs used to  
23 treat rare diseases.

24 (10) A representative of the biopharmaceutical industry.

25 (11) A representative of an insurance company.

26 (12) A member of the scientific community who is engaged  
27 in rare disease research including but not limited to a  
28 medical researcher with experience conducting research in rare  
29 diseases.

30 3. *a.* The members selected by the director shall serve  
31 two-year terms. A vacancy on the council shall be filled in  
32 the same manner as the original appointment. The council shall  
33 elect a chairperson every two years. A majority of the members  
34 of the council constitute a quorum and a quorum is required for  
35 official action of the council.

1     *b.* The council shall meet on a monthly basis for the  
2 first six months following creation of the council, and on a  
3 quarterly basis thereafter.

4     *c.* The council members appointed by the director shall be  
5 reimbursed for their actual and necessary expenses incurred  
6 while engaged in the performance of official duties. Members  
7 may also be eligible for compensation as provided in section  
8 7E.6.

9     4. The council shall do all of the following:

10    *a.* Convene public hearings, make inquiries, and solicit  
11 comments from the general public to assist the council in  
12 surveying the needs of rare disease patients, caregivers, and  
13 providers in the state.

14    *b.* Consult with experts on rare diseases to develop policy  
15 recommendations to improve patient access to and the quality of  
16 rare disease specialists, affordable and comprehensive health  
17 care coverage, relevant diagnostics, timely treatment, and  
18 other services.

19    *c.* Research and make recommendations to state agencies and  
20 insurers that provide services and coverage to persons with a  
21 rare disease on the impact of coverage, cost-sharing, tiering,  
22 or other utilization management procedures on the provision of  
23 treatment and services.

24    *d.* Research and identify priorities related to treatments  
25 and services provided to persons with a rare disease in  
26 the state and develop policy recommendations that include  
27 safeguards against discrimination for these populations,  
28 including in disaster and public health emergency-related  
29 planning.

30    *e.* Evaluate and make recommendations to improve the newborn  
31 screening program.

32    *f.* Evaluate and make recommendations to improve Medicaid  
33 coverage of treatment and medications for persons with a rare  
34 disease.

35    *g.* Publish a list of existing, publicly accessible resources

1 on research, diagnosis, treatment, and education relating to  
2 rare diseases in the state on the department's internet site.

3 *h.* Identify areas of unmet needs for research and to inform  
4 the work of the council.

5 *i.* Establish resources for academic institutions, state  
6 agencies, health care professionals, and other entities to  
7 provide a basis for training on rare diseases in the state.

8 *j.* Identify and distribute educational resources for health  
9 care providers to foster recognition and optimize treatment of  
10 rare diseases in the state.

11 *k.* Research and identify best practices to reduce health  
12 disparities and achieve health equity in research, diagnosis,  
13 and treatment of rare diseases in the state.

14 *l.* Establish best practices and protocols to use during a  
15 state of emergency to aid persons with a rare disease.

16 *m.* Coordinate with rare disease and related entities,  
17 community-based organizations, and other public and private  
18 organizations in performing its duties to ensure greater  
19 cooperation between this state, other states, and the federal  
20 government regarding the research, diagnosis, and treatment of  
21 rare diseases.

22 5. The council may solicit funds and accept donations,  
23 gifts, and bequests approved by the council in accordance with  
24 the duties of the council.

25 6. The council shall submit an annual report to the governor  
26 and the general assembly, and publish the annual report on the  
27 department's internet site, that includes all of the following:

28 *a.* A summary of the activities and progress of the council  
29 in carrying out the council's duties in the previous year.

30 *b.* An update on the status of funding sought and received,  
31 the use of the funds, and any remaining balances.

32 *c.* Provide recommendations to address the needs of people  
33 living with a rare disease in the state.

34

EXPLANATION

35 The inclusion of this explanation does not constitute agreement with

1           the explanation's substance by the members of the general assembly.

2       This bill creates a rare disease advisory council within the  
3 department of health and human services (HHS) for the purpose  
4 of advising HHS and other state agencies, the general assembly,  
5 and the public on research, diagnosis, and treatment efforts  
6 related to rare diseases in the state. The bill specifies the  
7 members of the council and the procedures for the functioning  
8 of the council.

9       The bill specifies the duties of the council including:  
10 convening public hearings, making inquiries, and soliciting  
11 comments from the general public to assist the council in  
12 surveying the needs of persons with a rare disease, caregivers,  
13 and providers in the state; consulting with experts on rare  
14 diseases to develop policy recommendations to improve patient  
15 access to and the quality of rare disease specialists,  
16 affordable and comprehensive health care coverage, relevant  
17 diagnostics, timely treatment, and other services; researching  
18 and making recommendations to state agencies and insurers that  
19 provide services and coverage to persons with a rare disease  
20 on the impact of coverage, cost-sharing, tiering, or other  
21 utilization management procedures on the provision of treatment  
22 and services; researching and identifying priorities related to  
23 treatments and services provided to persons with a rare disease  
24 in the state and develop policy recommendations that include  
25 safeguards against discrimination for these populations,  
26 including in disaster and public health emergency-related  
27 planning; evaluating and making recommendations to improve  
28 the newborn screening program; evaluating and making  
29 recommendations to improve Medicaid coverage of treatment and  
30 medications for persons with a rare disease; publishing a  
31 list of existing, publicly accessible resources on research,  
32 diagnosis, treatment, and education relating to the rare  
33 diseases in the state on the HHS internet site; identifying  
34 areas of unmet needs for research and to inform the work of  
35 the council; establishing resources for academic institutions,

1 state agencies, health care professionals, and other entities  
2 to provide a basis for training on rare diseases in the state;  
3 identifying and distributing educational resources for health  
4 care providers to foster recognition and optimize treatment  
5 of rare diseases in the state; researching and identifying  
6 best practices to reduce health disparities and achieve health  
7 equity in research, diagnosis, and treatment of rare diseases  
8 in the state; establishing best practices and protocols to use  
9 during a state of emergency to aid persons with a rare disease;  
10 and coordinating with rare disease and related entities,  
11 community-based organizations, and other public and private  
12 organizations in performing its duties to ensure greater  
13 cooperation between this state, other states, and the federal  
14 government regarding the research, diagnosis, and treatment of  
15 rare diseases.

16 The council may solicit funds and accept donations, gifts,  
17 and bequests approved by the council in accordance with the  
18 duties of the council.

19 The council shall submit an annual report to the governor and  
20 the general assembly, and publish the annual report on the HHS  
21 internet site, summarizing its activities for the prior year,  
22 updating the status of funding, and providing recommendations  
23 to address the needs of persons living with a rare disease in  
24 the state.