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THE GENERAL ASSEMBLY OF PENNSYLVANIA

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HOUSE BILL

No. 239 Session of  
2017

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INTRODUCED BY TOEPEL, LONGIETTI, BAKER, V. BROWN, D. COSTA,  
HEFFLEY, MURT, O'BRIEN, SCHLOSSBERG, SIMMONS, TOPPER, WARD,  
PHILLIPS-HILL, CORBIN AND KORTZ, FEBRUARY 1, 2017

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SENATOR BAKER, HEALTH AND HUMAN SERVICES, IN SENATE, AS AMENDED,  
JUNE 13, 2017

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AN ACT

1 Establishing the Rare Disease Advisory Council and providing for  
2 its powers and duties; and providing for duties of the  
3 Department of Health, the Insurance Department, the  
4 Department of Human Services and the Department of Education.

5 The General Assembly of the Commonwealth of Pennsylvania  
6 hereby enacts as follows:

7 Section 1. Short title.

8 This act shall be known and may be cited as the Rare Disease  
9 Advisory Council Act.

10 Section 2. Definitions.

11 The following words and phrases when used in this act shall  
12 have the meanings given to them in this section unless the  
13 context clearly indicates otherwise:

14 "Advisory council." The Rare Disease Advisory Council  
15 established in section 3.

16 "Department." The Department of Health of the Commonwealth.

17 "Rare disease." A disease or condition that affects fewer

1 than 200,000 individuals living in the United States.

2 "Secretary." The Secretary of Health of the Commonwealth.

3 Section 3. Establishment and membership.

4 (a) Establishment.--The Rare Disease Advisory Council is  
5 established.

6 (b) Members.--The advisory council shall consist of the  
7 following members:

8 (1) The secretary or a designee authorized to act on  
9 behalf of the secretary.

10 (2) The heads of State agencies that may be concerned  
11 with the provision of care to persons with rare diseases or  
12 designees authorized to act on behalf of the agency heads. At  
13 a minimum, members under this paragraph shall include the  
14 Secretary of Human Services and the Secretary of Education or  
15 their respective designees.

16 (3) The Insurance Commissioner or a designee authorized  
17 to act on behalf of the Insurance Commissioner.

18 (4) Public members to be appointed by the secretary, who  
19 shall include:

20 (i) Three physicians licensed to practice in this  
21 Commonwealth who have expertise in the diagnosis and  
22 management of patients with rare diseases, one of whom  
23 shall be a pediatrician who provides care to children  
24 with rare diseases.

25 (ii) Two registered professional nurses or nurse  
26 practitioners licensed in this Commonwealth who have  
27 expertise in providing care to patients with rare  
28 diseases.

29 (iii) An epidemiologist licensed in this  
30 Commonwealth who has expertise in the incidence,

1 prevalence and control of rare diseases.

2 (iv) Two representatives of hospitals in this  
3 Commonwealth, one of which must be a research hospital.

4 (v) Two representatives of the health insurance  
5 industry.

6 (vi) Two representatives involved in the  
7 biopharmaceutical industry.

8 (vii) Two representatives of the scientific  
9 community who are engaged in rare disease research.

10 (viii) Two parents, each of whom has a child with a  
11 rare disease, not of the same family.

12 (ix) Two individuals with rare diseases.

13 (x) Representatives of two rare disease-specific  
14 patient organizations that operate within this  
15 Commonwealth.

16 (c) Additional members.--The advisory council may at any  
17 time advise the department on additional at-large appointments  
18 to the advisory council that may be necessary to carry out its  
19 duties. At-large appointees to the advisory council may serve on  
20 an ad hoc basis.

21 (d) Vacancies.--Vacancies in the membership of the advisory  
22 council shall be filled in the same manner as provided for the  
23 original appointments.

24 (e) Appointment of public members and chairperson.--The  
25 secretary shall appoint the public members of the advisory  
26 council and a chairperson of the advisory council within 60 days  
27 of the effective date of this section.

28 (f) Compensation.--The public members of the advisory  
29 council shall serve without compensation but may be reimbursed  
30 for travel and other miscellaneous expenses necessary to perform

1 their duties within the limits of funds made available to the  
2 advisory council for its purposes. Reimbursement for expenses  
3 incurred by public members of the advisory council shall be  
4 provided by the department according to standard criteria  
5 currently in existence.

6 (g) Meetings.--The advisory council shall meet periodically,  
7 at a minimum of three times each year. A member may participate  
8 in a meeting by teleconference. The initial meeting of the  
9 advisory council shall be held within 90 days of the effective  
10 date of this section.

11 (h) Assistance of other government agencies.--The advisory  
12 council shall be entitled to call to its assistance and avail  
13 itself of the services of the employees of any State or local  
14 government agency as it may require and as may be available to  
15 it for its purposes.

16 (i) Staff.--The department may provide staff services to the  
17 advisory council, including providing a secretary to the  
18 advisory council.

19 Section 4. Purpose and duties.

20 (a) Purpose.--The purpose of the advisory council is to:

21 (1) Coordinate Statewide efforts for the study of the  
22 incidence and prevalence of rare diseases within this  
23 Commonwealth and the status of the rare disease community.

24 (2) Act as the advisory body on rare diseases to the  
25 General Assembly and to all relevant State and private  
26 agencies that provide services to, or are charged with the  
27 care of, individuals with rare diseases.

28 (3) Coordinate the performance of the advisory council's  
29 duties with those of other rare disease advisory bodies and  
30 community-based organizations and other public and private

1 organizations within this Commonwealth for the purpose of  
2 ensuring greater cooperation between the entities within this  
3 Commonwealth and Federal agencies regarding the research,  
4 diagnosis and treatment of rare diseases. For purposes of  
5 this paragraph, Federal agencies may include, but are not  
6 limited to, the National Institutes of Health and the Food  
7 and Drug Administration. The coordination shall require, when  
8 appropriate:

9 (i) Disseminating the outcomes of the advisory  
10 council's research, identified best practices and policy  
11 recommendations.

12 (ii) Utilizing common research collection and  
13 dissemination procedures.

14 (b) Duties.--The duties of the advisory council are to:

15 (1) Research and determine the most appropriate method  
16 to collect rare disease data, and information concerning  
17 individuals with rare diseases, as the advisory council deems  
18 necessary and appropriate to conduct comprehensive surveys of  
19 rare diseases diagnosed in this Commonwealth, subject to all  
20 applicable privacy laws and protections.

21 (2) Ensure that the duties described in subsection (a)  
22 (1) are carried out in a manner that is coordinated and  
23 interoperable with other similar research being conducted at  
24 the Federal and State level.

25 (3) Research and identify priorities relating to the  
26 quality and cost effectiveness of and access to treatment and  
27 other services provided to individuals with rare diseases in  
28 this Commonwealth and develop policy recommendations on those  
29 issues.

30 (4) Identify best practices in other states and at the

1 national level for rare disease management, monitoring and  
2 surveillance, education, detection, diagnosis, information  
3 and care to improve these functions and capabilities relative  
4 to rare diseases within this Commonwealth.

5 (5) Develop effective strategies to raise public  
6 awareness of rare diseases within this Commonwealth.

7 (6) Coordinate the development of a task force,  
8 comprised of members of the advisory council and other  
9 individuals as may be necessary and appropriate to facilitate  
10 the development and publication of the report specified in  
11 section 6(b).

12 (c) Construction.--Nothing in this act shall be construed to  
13 prohibit the advisory council from issuing interim reports or  
14 taking interim actions should these seem appropriate.

#### 15 Section 5. Funding.

16 (a) Report to General Assembly.--Prior to appointing members  
17 of the advisory council, the department shall research and  
18 report to the General Assembly within 30 days of the effective  
19 date of this section existing sources of funding that may be  
20 used to finance the formation and operation of the advisory  
21 council.

22 (b) Grant and other funding.--The advisory council shall  
23 apply for, and is authorized to accept, a grant of money from  
24 the Federal Government, a private foundation or any other source  
25 which may be available for programs related to rare diseases.

#### 26 Section 6. Reports.

27 (a) Preliminary report.--Within 12 months of the effective  
28 date of this section, the advisory council shall deliver to the  
29 ~~Public Health and Welfare~~ HEALTH AND HUMAN SERVICES Committee of <--  
30 the Senate and the Health Committee of the House of

1 Representatives a preliminary report on the work of the advisory  
2 council. The report shall be made available to the public. The  
3 report shall include a summary of the accomplishments of the  
4 advisory council since its formation and the status of its work  
5 in preparation of the report required under subsection (b).

6 (b) Report.--Within two years of the effective date of this  
7 section, the advisory council shall deliver to the ~~Public Health~~ <--  
8 ~~and Welfare~~ HEALTH AND HUMAN SERVICES Committee of the Senate <--  
9 and the Health Committee of the House of Representatives a  
10 comprehensive report. The report shall be made available to the  
11 public. The report shall include the following:

12 (1) The incidence and prevalence of rare diseases within  
13 this Commonwealth.

14 (2) The needs of the rare disease community within this  
15 Commonwealth and the actions necessary and feasible to  
16 address those needs.

17 (c) Biennial reports.--The advisory council shall report  
18 biennially to the department, the ~~Public Health and Welfare~~ <--  
19 HEALTH AND HUMAN SERVICES Committee of the Senate and the Health <--  
20 Committee of the House of Representatives on the activities of  
21 the advisory council and its findings and recommendations on  
22 issues relating to the management, monitoring and surveillance,  
23 education, detection, diagnosis, information and care regarding  
24 rare diseases in this Commonwealth, including the quality and  
25 cost effectiveness of care, access to treatment and other  
26 relevant services for individuals affected by rare diseases.

27 Section 7. Expiration.

28 This act shall expire June 30, 2025.

29 Section 8. Effective date.

30 This act shall take effect immediately.