
THE GENERAL ASSEMBLY OF PENNSYLVANIA

HOUSE BILL

No. 239 Session of
2017

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

REFERRED TO COMMITTEE ON HEALTH, FEBRUARY 1, 2017

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

1 "Secretary." The Secretary of Health of the Commonwealth.
2 Section 3. Establishment and membership.

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

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

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

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

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

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

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

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

28 (iii) An epidemiologist licensed in this
29 Commonwealth who has expertise in the incidence,
30 prevalence and control of rare diseases.

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

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

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

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

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

11 (ix) Two individuals with rare diseases.

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

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

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

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

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

1 advisory council for its purposes. Reimbursement for expenses
2 incurred by public members of the advisory council shall be
3 provided by the department according to standard criteria
4 currently in existence.

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

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

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

18 Section 4. Purpose and duties.

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

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

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

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

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

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

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

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

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

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

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

29 (4) Identify best practices in other states and at the
30 national level for rare disease management, monitoring and

1 surveillance, education, detection, diagnosis, information
2 and care to improve these functions and capabilities relative
3 to rare diseases within this Commonwealth.

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

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

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

14 Section 5. Funding.

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

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

25 Section 6. Reports.

26 (a) Preliminary report.--Within 12 months of the effective
27 date of this section, the advisory council shall deliver to the
28 Public Health and Welfare Committee of the Senate and the Health
29 Committee of the House of Representatives a preliminary report
30 on the work of the advisory council. The report shall be made

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

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

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

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

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

26 Section 7. Expiration.

27 This act shall expire June 30, 2025.

28 Section 8. Effective date.

29 This act shall take effect immediately.