

No. 2017-14

AN ACT

HB 239

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

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

Section 1. Short title.

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

Section 2. Definitions.

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

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

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

"Rare disease." A disease or condition that affects fewer than 200,000 individuals living in the United States.

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

Section 3. Establishment and membership.

(a) Establishment.—The Rare Disease Advisory Council is established.

(b) Members.—The advisory council shall consist of the following members:

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

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

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

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

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

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

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

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

(v) Two representatives of the health insurance industry.

(vi) Two representatives involved in the biopharmaceutical industry.

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

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

(ix) Two individuals with rare diseases.

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

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

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

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

(f) Compensation.—The public members of the advisory council shall serve without compensation but may be reimbursed for travel and other miscellaneous expenses necessary to perform their duties within the limits of funds made available to the advisory council for its purposes. Reimbursement for expenses incurred by public members of the advisory council shall be provided by the department according to standard criteria currently in existence.

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

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

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

Section 4. Purpose and duties.

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

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

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

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

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

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

(ii) Utilizing common research collection and dissemination procedures.

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

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

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

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

(4) Identify best practices in other states and at the national level for rare disease management, monitoring and surveillance, education, detection, diagnosis, information and care to improve these functions and capabilities relative to rare diseases within this Commonwealth.

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

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

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

Section 5. Funding.

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

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

Section 6. Reports.

(a) Preliminary report.—Within 12 months of the effective date of this section, the advisory council shall deliver to the Health and Human Services

Committee of the Senate and the Health Committee of the House of Representatives a preliminary report on the work of the advisory council. The report shall be made available to the public. The report shall include a summary of the accomplishments of the advisory council since its formation and the status of its work in preparation of the report required under subsection (b).

(b) Report.—Within two years of the effective date of this section, the advisory council shall deliver to the Health and Human Services Committee of the Senate and the Health Committee of the House of Representatives a comprehensive report. The report shall be made available to the public. The report shall include the following:

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

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

(c) Biennial reports.—The advisory council shall report biennially to the department, the Health and Human Services Committee of the Senate and the Health Committee of the House of Representatives on the activities of the advisory council and its findings and recommendations on issues relating to the management, monitoring and surveillance, education, detection, diagnosis, information and care regarding rare diseases in this Commonwealth, including the quality and cost effectiveness of care, access to treatment and other relevant services for individuals affected by rare diseases.

Section 7. Expiration.

This act shall expire June 30, 2025.

Section 8. Effective date.

This act shall take effect immediately.

APPROVED—The 7th day of July, A.D. 2017

TOM WOLF