

HOUSE No. 4268

The Commonwealth of Massachusetts

In the One Hundred and Ninety-First General Court
(2019-2020)

An Act to create a Massachusetts rare disease advisory council.

Be it enacted by the Senate and House of Representatives in General Court assembled, and by the authority of the same, as follows:

1 SECTION 1. Chapter 111 of the General Laws is hereby amended by adding the
2 following section:-

3 Section 238. (a) For the purposes of this section the following words shall, unless the
4 context clearly requires otherwise, have the following meanings:-

5 "Rare disease", any disease that affects fewer than 200,000 people in the United States,
6 has status as an orphan disease for research purposes or is known to be substantially under-
7 diagnosed and unrecognized as a result of lack of adequate diagnostic and research information.

8 "Rare disease care", the academic research of a rare disease or the medical treatment of
9 individuals diagnosed with a rare disease.

10 (b) There is hereby established the rare disease advisory council consisting of the
11 following 28 members: the commissioner, or a designee who shall serve as chair; the executive
12 director, or a designee, of the Massachusetts health policy commission; 2 members of the state

senate, or their designee, 1 of whom shall be appointed by the senate president and 1 appointed by the minority leader; 2 members of the house of representatives, or their designee, 1 of whom shall be appointed by the speaker of the house and 1 appointed by the minority leader and 21 persons to be appointed by the governor, 2 of whom shall be from academic research institutions that receive grant funding for rare diseases research; 2 of whom shall be physicians licensed and practicing in the state with experience researching, diagnosing or treating rare diseases; 1 of whom shall be a geneticist licensed and practicing in the state; 1 of whom shall be a registered nurse or advanced practice registered nurse licensed and practicing in the commonwealth with experience treating rare diseases; 2 of whom shall be hospital administrators, or their designee, from hospitals in the commonwealth that provide care to persons diagnosed with a rare disease, 1 of whom shall represent a hospital in which the scope of service focuses on rare diseases of pediatric patients; 3 of whom shall be representatives of rare disease patient organizations that operate in the commonwealth; 1 of whom shall be a pharmacist with experience with drugs used to treat rare diseases; 2 of whom shall be a representative of the biotechnology and scientific community who is engaged in rare disease research, including, but not limited to, a medical researcher with experience conducting research on rare diseases; 1 of whom shall be a representative of a health plan or accountable care organization certified by the health policy commission; 1 of whom shall be a genetic counselor with experience providing services to persons diagnosed with a rare disease; 1 of whom shall be a representative from a rehabilitation facility that provides rare disease care; 1 of whom shall be a dietician licensed and practicing in the state with experience administering dietary therapies to those with rare diseases; 2 of whom shall be persons age 18 or older who have a rare disease; and 1 of whom shall be a caregiver of a person with a rare disease.

(c) Each member of the rare disease advisory council shall serve for a term of 3 years and shall serve until their successors have been appointed. The advisory council shall meet periodically no fewer than 4 times annually, with members able to participate in any meeting by teleconference. The members of the advisory council shall serve without compensation. The commissioner shall provide the advisory council with suitable accommodations for its meetings and the department shall further provide administrative support to assist the advisory council.

(d) The rare disease advisory council shall advise the governor, the general court and the department on the incidence of rare disease within the commonwealth and the status of the rare disease community. To achieve its purpose, the advisory council shall:

(i) coordinate the performance of the rare disease advisory council's duties with those of other rare disease advisory bodies, community-based organizations and other public and private organizations within the state for the purpose of ensuring greater cooperation regarding the research, diagnosis and treatment of rare diseases. The coordination shall require, when appropriate: (1) disseminating the outcomes of the advisory council's research, identified best practices and policy recommendations; and (2) utilizing common research collection and dissemination procedures;

(ii) using existing publicly available records and information, undertake a statistical and qualitative examination of the prevalence and causes of rare disease to develop a profile of the social and economic burden of rare disease in the commonwealth;

(iii) receive and consider reports and testimony from expert individuals, the department, community-based organizations, voluntary health organizations, healthcare providers and other public and private organizations recognized as having expertise in rare disease care, to learn

about their contributions to rare disease care and possibilities for the improvement of rare disease care in the commonwealth;

(iv) develop methods to publicize the profile of the social and economic burden of rare disease in the commonwealth to ensure that the public and healthcare providers are sufficiently informed of the most effective strategies for recognizing and treating rare disease;

(v) determine the human impact and economic implications of early treatment of rare diseases versus delayed or inappropriate treatment of rare disease as it pertains to the quality of care, the quality of patients' and their families' lives, and the economic burdens; including insurance reimbursements, rehabilitation, hospitalization and related services on patients, families and the commonwealth;

(vi) evaluate the current system of rare disease treatment and available public resources to develop recommendations to increase rare disease survival rates, improve quality of life and prevent and control risks of co-morbidities for rare disease, based on available scientific evidence;

(vii) research and determine the most appropriate method for the commonwealth to collect rare disease data, including a database of all rare diseases identified in the commonwealth along with known best practices for care of said diseases and such additional information concerning these cases as the advisory committee deems necessary and appropriate to conduct thorough and complete epidemiological surveys of rare diseases, subject to all applicable privacy laws and protections;

(viii) examine the feasibility of developing a rare disease information and patient support network in the commonwealth to aid in determining any genetic or environmental contributors to rare diseases; and

(ix) develop and maintain a comprehensive rare disease plan for the commonwealth, utilizing any information and materials received or developed by the advisory council pursuant to this subsection, and which shall include information specifically directed toward the general public, state and local officials, state agencies, private organizations and associations, and businesses and industries.

(e) The advisory council may apply for, and accept, any grants of money from the federal government, private foundations, or any other source which may be available for programs related to rare diseases or to advance the mission of the advisory council.

(f) On or before December 31st of each calendar year, the advisory council shall file a report with the clerks of the house of representatives and the senate and the executive office for administration and finance, which shall include, but is not limited to: (i) a summary of the current state of comprehensive rare disease plan for the commonwealth; (ii) those actions taken and progress made toward achieving implementation of the comprehensive rare disease plan; (iii) an accounting of all funds received by the council, and the source of those funds; (iv) an accounting of all funds expended by the council; and (v) to the extent practicable, an estimate of any cost savings on the part of individuals and the commonwealth that will occur upon full implementation of the comprehensive rare disease plan and accompanying programs.

SECTION 2. Prior to appointing the members of the rare disease advisory council established in this act, the governor or the secretary of the executive office of health and human

100 services shall research and report to the general court, within 30 days of the effective date of this
101 act, existing sources of funding that may be used to finance the formation and operation of the
102 advisory council.

103 SECTION 3. On or before 180 days following the effective date of this act, the rare
104 disease advisory council shall provide a preliminary report to the governor, the department of
105 public health and to the general court, by filing the same with the clerks of the house of
106 representatives and the senate. The preliminary report shall include, but is not limited to, an
107 estimate the financial, informational and other resources needed to achieve the goals and duties
108 of the advisory council.