

By: Senator(s) Moran, Wiggins, Dearing,
McMahan

To: Public Health and
Welfare; Appropriations

COMMITTEE SUBSTITUTE
FOR
SENATE BILL NO. 2463

1 AN ACT TO ESTABLISH A MISSISSIPPI RARE DISEASE ADVISORY
2 COUNCIL WITHIN THE STATE DEPARTMENT OF HEALTH TO EDUCATE MEDICAL
3 PROFESSIONALS, GOVERNMENTAL AGENCIES AND THE PUBLIC ABOUT RARE
4 DISEASES AND TO FUND RESEARCH IN THE DEVELOPMENT OF TREATMENTS FOR
5 RARE DISEASES; TO PROVIDE FOR THE MEMBERSHIP OF THE COUNCIL AND
6 PRESCRIBE ITS DUTIES AND RESPONSIBILITIES; TO PROVIDE THAT THE
7 COUNCIL SHALL APPLY FOR AND EXPEND GRANTS FROM THE FEDERAL
8 GOVERNMENT OR PRIVATE SOURCES FOR FUNDING THE ACTIVITIES OF THE
9 COUNCIL; AND FOR RELATED PURPOSES.

10 BE IT ENACTED BY THE LEGISLATURE OF THE STATE OF MISSISSIPPI:

11 **SECTION 1.** Findings. The Legislature finds and declares:

12 (a) A rare disease is defined as a disease that affects
13 fewer than two hundred thousand (200,000) people in the United
14 States. Rare diseases are sometimes called orphan diseases.
15 There are seven thousand (7,000) known rare diseases affecting
16 approximately thirty million (30,000,000) men, women and children
17 in the United States;

18 (b) The exact cause for many rare diseases remains
19 unknown. However, eighty percent (80%) of rare diseases are
20 genetic in origin and can be linked to mutations in a single gene
21 or in multiple genes. Such diseases are referred to as genetic



22 diseases. Genetic disease can be passed down from generation to
23 generation, explaining why certain rare diseases run in families.
24 It is also estimated that about half of all rare diseases affect
25 children;

26 (c) A person suffering with a rare disease in
27 Mississippi faces a wide range of challenges, including, but not
28 limited to: delays in obtaining a diagnosis; misdiagnosis;
29 shortage of medical specialists familiar with, and can provide
30 treatment for, rare diseases; prohibitive cost of treatment; and
31 the inability to access therapies and medication that are used by
32 doctors to treat rare diseases but have not been approved by the
33 Federal Food and Drug Administration (FDA) for that specific
34 purpose;

35 (d) In recent years, researchers have made considerable
36 progress in developing diagnostic tools and treatment protocols
37 for, and in discovering ways to prevent a variety of, rare
38 diseases. However, much more remains to be done in the areas of
39 rare disease research and the search for and development of new
40 therapeutics; and

41 (e) It is, therefore, an appropriate public policy for
42 Mississippi to establish an advisory body, whose membership would
43 be comprised of persons with suitable qualifications for this
44 purpose, including persons living with rare diseases, to educate
45 medical professionals, government agencies, and the public about
46 rare diseases as an important public health issue, and to



47 encourage and fund research in the development of new treatments
48 for rare diseases.

49 **SECTION 2. Establishment of a Rare Disease Advisory Council.**

50 (1) There is established the Mississippi Rare Disease Advisory
51 Council (council) in the Mississippi Department of Health.

52 (2) The advisory council shall consist of members as
53 follows:

54 (a) Employees of the Mississippi Department of Health
55 and other state agencies concerned with the provision of care to
56 persons with rare diseases appointed by the Executive Officer of
57 the State Department of Health; and

58 (b) Public members to be appointed by the Executive
59 Director of the State Department of Health, who shall include:

60 (i) Two (2) physicians licensed to practice in
61 Mississippi who have expertise in treating patients with rare
62 diseases, one of whom shall be a pediatrician who provides care to
63 children with rare diseases;

64 (ii) A registered professional nurse licensed in
65 Mississippi who has expertise in providing care to patients with
66 rare diseases;

67 (iii) Two (2) representatives of hospitals in
68 Mississippi;

69 (iv) A representative of the health insurance
70 industry;



71 (v) A representative of the biopharmaceutical
72 industry;

73 (vi) A representative of the scientific community
74 who is engaged in rare disease research;

75 (vii) Two (2) parents of a child with a rare
76 disease;

77 (viii) Two (2) persons with a rare disease;

78 (ix) Two (2) patient organizations that operate
79 within Mississippi.

80 (3) Upon or after the Advisory Council is first convened,
81 the council may advise the Executive Director of the State
82 Department of Health on additional at-large appointments to the
83 council that may be necessary to carry out its duties. At-large
84 appointments to the council may serve on an ad-hoc basis.

85 (4) Members shall serve for four-year terms and may be
86 reappointed. Vacancies in the membership of the Advisory Council
87 shall be filled in the same manner provided for the original
88 appointments. The public members of the council shall serve
89 without compensation but may be reimbursed for travel and other
90 miscellaneous expenses necessary to perform their duties within
91 the limits of funds made available to the council for its
92 purposes.

93 (5) The council shall organize as soon as practicable after
94 the appointment of its members and shall select a chairperson and



95 vice chairperson from among its members. The chairperson shall
96 appoint a secretary who need not be a member of the council.

97 (6) The council shall meet periodically, but at least three
98 (3) times annually. The council shall be entitled to call to its
99 assistance, and avail itself of the services of the employees of,
100 any state, county, or municipal department, board, bureau,
101 commission, or agency as it may require and as may be available to
102 it for its purposes.

103 (7) The State Department of Health may provide staff
104 services to the advisory council.

105 **SECTION 3. Purpose and duties.** (1) The purpose of the
106 Council shall be to:

107 (a) Coordinate statewide efforts for the study of the
108 incidence of rare disease within Mississippi and the status of the
109 rare disease community;

110 (b) Act as the advisory body on rare diseases to the
111 Legislature and state departments, agencies, commissions,
112 authorities, and private agencies that provide services to, or are
113 charged with the care of, persons with rare diseases; and

114 (c) Coordinate the performance of the council's duties
115 with other state rare disease advisory bodies, community-based
116 organizations, and other public and private organizations for the
117 purpose of ensuring greater cooperation between state and federal
118 activities regarding the research, diagnosis, and treatment of
119 rare diseases. Federal agencies may include, but are not



120 exclusive to, the U.S. National Institutes of Health (NIH) and the
121 U.S. Food and Drug Administration (FDA). Such coordination shall
122 require, when appropriate:

123 (i) Disseminating the council's research,
124 identified best practices, and policy recommendations; and

125 (ii) The utilization of common research collection
126 and dissemination procedures.

127 (2) The duties of the council shall be to:

128 (a) Research and determine the most appropriate method
129 to collect rare disease data, and such information concerning
130 these patients as the council deems necessary and appropriate to
131 conduct thorough and complete surveys of rare disease diagnosed in
132 Mississippi, subject to all applicable privacy laws and
133 protection.

134 (b) The council shall ensure that the duties described
135 in paragraph (a) are carried out in a manner that is coordinated
136 and interoperable with similar research being conducted at the
137 state and federal level.

138 (c) Research and identify priorities relating to the
139 quality and cost-effectiveness of, and access to, treatment and
140 services provided to persons with rare diseases in Mississippi,
141 and develop policy recommendations on those issues.

142 (d) Identify best practices for rare disease care from
143 other states and at the national level that will improve rare
144 disease care in Mississippi.



145 (e) Develop effective strategies to raise public
146 awareness of rare diseases in Mississippi.

147 **SECTION 4. Funding and reporting.** (1) Prior to appointing
148 members of the council pursuant to Section 2 of this act, the
149 State Department of Health shall research and report to the
150 Legislature on existing sources of funding that may be used to
151 finance the formation and operation of the council.

152 (2) The Advisory Council shall apply for, and accept, any
153 grant of money from the federal government, private foundations,
154 or other sources, which may be available for programs related to
155 rare diseases.

156 (3) The council shall report to the State Department of
157 Health and to the Legislature biennially on the activities of the
158 advisory council and its findings and recommendations on issues
159 relating to the quality and cost-effectiveness of, and access to
160 treatment and services to, persons with rare diseases in
161 Mississippi.

162 **SECTION 5.** This act shall take effect and be in force from
163 and after July 1, 2018.

