Legislative Analysis

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RARE DISEASE ADVISORY COUNCIL

House Bill 4167 (H-3) as reported from committee
Sponsor: Rep. Jason Morgan
Committee: Health Policy
Complete to 3-21-23

BRIEF SUMMARY: House Bill 4167 would create a new advisory council to advise the Department of Health and Human Services (DHHS) and state agencies on research, diagnosis, and treatment efforts related to rare diseases in Michigan; establish the composition of the council; provide authority to the council for certain tasks; and require the council to fulfill certain duties, including an annual report to the legislature on its activities, findings, and recommendations.

FISCAL IMPACT: The bill would have a minimal fiscal impact on DHHS, as the members of the Rare Disease Advisory Council would not receive compensation for service but could be reimbursed for actual expenses. Additionally, the FY 2022-23 DHHS budget appropriates $70,000 GF/GP for a rare disease advisory council through boilerplate section 1240.

THE APPARENT PROBLEM:

According to the Genetic and Rare Diseases Information Center in the National Institutes of Health (NIH), there are approximately 10,000 known rare diseases, with about one in ten, or 30 million, US residents afflicted.[1] About 80% of rare diseases are thought to have a genetic origin, and most have no known cure or treatment option. Because the number of individuals who have a particular disease is so low, funding is often lacking for research and for developing therapeutic and pharmacologic treatments for that disease. Moreover, a physician may not recognize that a patient has a rare disease or may lack the knowledge to recognize the symptoms. As a result, many patients with rare diseases spend years going from one doctor to another, undergoing expensive tests (sometimes multiple times), before obtaining a diagnosis. A delayed diagnosis not only has an impact on a patient's prognosis, but also on the patient's quality of life over their lifetime.

Because of the impact on the lives of individuals with rare diseases, their families and communities, and the health care system, at least 24 states have enacted legislation to create rare disease advisory councils (RDACs) that can advise policymakers regarding issues such as health insurance, research into therapies, development of new drugs or expanding the use of drugs currently in the marketplace, and various supports needed for those with rare diseases. It has been suggested that Michigan create an RDAC as well, to help identify policies that may lead to better outcomes for the state and those with a rare disease.

THE CONTENT OF THE BILL:

House Bill 4167 would add two sections to the Public Health Code to create the Rare Disease Advisory Council in the Department of Health and Human Services. The council would exist

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[1] https://rarediseases.info.nih.gov/about

for the purpose of advising DHHS and other state agencies on research, diagnosis, and treatment efforts related to ***rare diseases*** in Michigan.

> ***Rare disease*** would mean a disease that affects fewer than 200,000 individuals in the United States.[2]

Council members would include the DHHS director, or their designee, and all of the following members appointed by the DHHS director:

- A licensed physician (either an M.D. or a D.O.) who has experience treating patients with rare diseases.
- A registered professional nurse who has expertise in providing care to patients with rare diseases.
- An epidemiologist who practices in Michigan and has experience in the incidence, prevalence, and control of rare diseases.
- An individual who represents a hospital located in Michigan and an individual from a different institution who engages in rare disease research.
- An individual who represents a health insurer.
- An individual who represents a health maintenance organization.
- An individual who represents the biopharmaceutical industry in Michigan.
- Two parents or guardians of children with a rare disease. (Each parent or guardian would have to represent a different family.)
- Three individuals, each with a different rare disease.
- Two individuals who represent two different rare disease patient organizations in Michigan.
- A medical ethicist who practices in Michigan.
- A representative from the national pharmaceutical industry or a pharmaceutical company working in the area of rare diseases.
- A licensed genetic counselor who is familiar with rare diseases.

Additionally, upon recommendation from the council, the DHHS director could appoint additional members that the director considers necessary or appropriate.

The initial members would have to be appointed within 90 days of the bill's effective date. Members would serve for four-year terms or until appointment of a successor, whichever is later, and vacancies would be filled in the same way as that position was filled originally. The DHHS director could remove members for incompetence, dereliction of duty, malfeasance, misfeasance, or nonfeasance in office, or any other good cause.

After the DHHS director called the initial meeting, members would elect a chairperson and could elect other officers. The council would meet at least quarterly thereafter. Meetings would have to be held in compliance with the Open Meetings Act, and council documents would have to be made available to the public in compliance with the Freedom of Information Act (FOIA). Members would serve without compensation but could be reimbursed for necessary travel expenses for attendance.

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[2] This definition of *rare disease* is similar to that in the federal Orphan Drug Act.

The council could do one or more of the following:

- Subject to appropriation, apply for and accept grants and gifts from government, nonprofit, and private sources. (DHHS would provide assistance in applying as the DHHS director determined necessary and appropriate.)
- Hold public hearings and make inquiries and receive comments from the general public to assist the council in developing recommendations.
- Consult with experts on rare diseases to assist in developing recommendations.

The council would have to do all of the following:

- Research and identify priorities relating to the cost-effectiveness of and access to treatments and services provided to individuals with rare diseases in Michigan and develop policy recommendations aimed at preventing discrimination against those individuals with respect to the cost-effectiveness of and access to treatments and services and other related issues.
- Identify best practices for rare diseases from other states and at the national level that could improve the education, care, and treatment of, and services and supports provided to, adults and minors with rare diseases in Michigan.
- Coordinate with other rare disease advisory bodies and organizations in performing its duties to ensure greater cooperation between Michigan, other states, and the federal government regarding the research, diagnosis, and treatment of rare diseases, by disseminating the council's research, findings, and recommendations when appropriate.
- Serve as an advisory body on rare diseases to the legislature, the governor, DHHS, and other state agencies.
- Research and make recommendations to the legislature and DHHS on the most appropriate method to collect data on rare diseases.
- Provide information or advice on rare diseases to DHHS or the governor, as the director or governor considers necessary.
- By December 21, 2023, assist DHHS in developing a publicly accessible webpage on the department's website on rare disease resources. The council also would have to annually review and recommend changes to the webpage. The webpage would have to include at least all of the following:
    - A list of rare diseases that mostly affect geographically disadvantaged communities.
    - Links to websites on rare diseases.
    - Resources on rare diseases that the council and DHHS consider useful to those with rare diseases and the general public.
- Investigate rare diseases from the list described above and make recommendations to the legislature on the diseases.
- Beginning December 21, 2023, and by December 21 of every other year thereafter, submit a report to the legislature on its activities, findings, and recommendations.

Any findings or recommendations made by the council under the bill would have to be based on medical or scientific evidence.

The bill would take effect 90 days after its enactment.

Proposed MCL 333.5135 and 333.5135a

BACKGROUND:

The bill is a reintroduction of HB 5465 of the 2019-20 legislative session and HB 4654 of the 2021-22 legislative session, the latter of which was passed by the House of Representatives.

ARGUMENTS:

For:

Michigan has a diverse geography, from large urban centers such as Detroit and Grand Rapids, to remote towns in the Upper Peninsula that are far from hospitals able to provide specialized care. The needs of an individual with a rare disease in Ann Arbor, for instance, with its proximity to the University of Michigan hospital system and Veteran's Administration Hospital, may be vastly different from an individual with the same disease in a small town in the Keweenaw Peninsula or in a town that has a low median family income. Further, because many rare diseases have a genetic component, Michigan may have pockets where multiple individuals have the same rare disease.

In addition, some rare diseases are extremely expensive to treat. Some pay hundreds of thousands of dollars a year for medicine to stay alive. A transplant can cost over a million dollars. Lost days of work have an impact on the local and state economy, not just a family's income. Uncompensated medical care faced by hospitals and medical providers have an impact on all of health care. Court decisions have affected how approved drugs can be marketed, and to whom, cutting some off from medicines known to help them. Treatment for a particular disease may be available only from a specific rare disease center of excellence,[3] which could be located across the county and might not covered by an individual's health plan, including Medicaid and Medicare.

A rare disease advisory council could identify needs of Michigan residents with rare diseases that could result in better access to health care specific to the state, effect better state and federal policies affecting access to and the delivery of care for rare diseases, spur and coordinate research into diseases affecting residents, identify revenue sources for development of more effective therapies, disseminate information about specific rare diseases as it becomes available, and develop community supports for individuals and families facing rare diseases. Over time, the work of a council might could not only improve the lives of those with a rare disease, but also lead to reducing costs to the state.

Further, it was noted in House committee testimony that Michigan already has a model for the proposed council: the Community Values Advisory Board (CVAB) for Michigan BioTrust for Health, a DHHS program that oversees Michigan's newborn screening and stored blood spot program and their use in research. The CVAB provides guidance on ethical issues such as acceptable uses of the dried blood spots.

Against:

Some concern was voiced as to the number of appointees to the advisory council, as too many members can render a body unwieldy and ineffective.

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[3] NORD Rare Disease Centers of Excellence website offers information to the public on finding a RDCE, clinical trials and research studies, and support organizations, among other information. See https://rarediseases.org/rare-disease-centers-of-excellence/

Response:

The committee adopted a substitute that trimmed the number of members of the rare disease advisory council to 18. Reportedly, early proposals had as many as 26 members and allowed the governor to appoint additional members as they considered necessary. Because of the nature of rare diseases, the similarities and differences in needs of those affected by rare diseases, and the types of medical, pharmacological, and research professionals needed to offer insight, it would also not be possible to obtain the expertise necessary to find viable solutions and policy recommendations if the council represented a narrower demographic and body of knowledge.

POSITIONS:

Representatives of the following entities testified in support of the bill (3-9-23):

- National Organization for Rare Disorders
- MichBio
- The Bonnell Foundation: Living with Cystic Fibrosis

The following entities indicated support for the bill:

- Michigan Council for Maternal & Child Health (3-15-23)
- Michigan Association for Local Public Health (3-15-23)
- Boehringer Ingelheim USA Corporation (3-9-23)
- Michigan Health and Hospital Association (3-9-23)
- Cystic Fibrosis Foundation (3-9-23)
- BIO—Biotechnology Innovation Organization (3-9-23)
- Novartis (3-9-23)
- PTC (3-9-23)
- The Hemophilia Foundation (3-9-23)

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