May 13, 2021

Bronna Kahle
Chair, Health Policy Committee
Michigan House of Representatives
P.O. Box 30014
Lansing, MI 48909-7514

Dear Rep. Kahle,

On behalf of the biosciences industry in Michigan, and its many biotechnology and pharmaceutical companies engaged in research and manufacturing of life-saving therapeutics, MichBio supports passage of HB 4654, but only with revision. Generally, the legislation to establish a Rare Disease Review Council (RDAC) within the Department of Health and Human Services (DHHS) would give a strong voice to the rare diseases community in Michigan.

Michigan's bio-industry is committed to the identification, treatment and cure of rare disorders through the development of novel therapies and drugs, education, advocacy and patient support. Our companies span a range of therapeutic areas, just as rare diseases are present across a broad spectrum of medical conditions.

The RDAC will give rare disease patients a unified voice in our state government by providing them a forum to make recommendations about pressing health care issues. Coupled with MDHHS' nationally leading Newborn Screening Program, i.e., the Michigan BioTrust for Health, the RDAC would give the state a compelling ability to improve knowledge, awareness and management of rare diseases in Michigan, and bring together various stakeholders in the healthcare ecosystem to improve public policy regarding rare diseases. As Chair, and longtime member, of the Michigan BioTrust for Health Community Advisory Board (CVAB), I know that any outcomes of a well designed RDAC will be a great aid for patients and their families.

However, HB 4654 as introduced, warrants revision. MichBio recommends that RDAC membership be expanded to ensure the widest possible measure of stakeholder inclusiveness. For instance, the Council should include:

- At least three patients or caregivers to individuals living with a rare disease
- One researcher actively engaged in rare disease research
- One physician with experience in diagnosing and treating rare diseases
- One registered nurse with experience providing care to patients with rare diseases

- One licensed genetic counselor practicing in the area of pediatric genetics
- One DHHS/DCH representative, preferably with linkage to the Michigan BioTrust for Health and the Newborn Screening Program (e.g., Genomics and Newborn Screening Research Coordinator or Newborn Screening Program Epidemiologist)
- One representative each (total of 2) from a national and state biopharma industry company or association engaged in the rare diseases space
- One representative from a national rare disease foundation operating in Michigan
- Two representatives from Michigan-based rare disease advocacy organizations
- One representative from the Michigan hospital association
- One representative from Michigan health insurers
- One representative from an academic or clinical institution with experience in health economics, especially Medicaid and insurance as related to rare diseases
- One representative from a commission or constituency on minority/underrepresented community health
- Representatives appointed by the Governor and Michigan Legislature

At a minimum, the RDAC would be a group of 17 members appointed via the Director of the Michigan DHHS (except in the case of the Executive Office and Legislature), and should operate in an independent, apolitical and advisory manner under the auspices of the agency.

Such broad representation has proven to be an excellent model for the 14-member CVAB since its inception in 2009. Patient concerns, emanating from a cross-section of socio-economic, cultural and clinical condition perspectives, have been deliberated by the CVAB to address issues related to ethics, transparency, education, and equity. Given the breadth and diversity of the over 7000 rare diseases identified to date, it's critical that the makeup of the RDAC be empowered similarly. My experience with the CVAB is that a too small a body with limited breadth in expertise or representation needed to address patient issues sufficiently will prove to be less impactful in the long run and defeat its purpose.

Additionally, clarity must be provided regarding the legal ability for the RDAC to seek grant support. As envisioned, the Council would be housed under the auspices of the MDHHS/DCH (as is the CVAB), and thus the agency would be the fiduciary and grant applicant. The RDAC could have oversight on funding priorities and advise DHHS on pursuing appropriate funding for approved purposes that meet the Council's mission and requirements per statute. However, we doubt that RDAC would have the legal right to seek funding on its own, as it would not be a duly formed and registered, charitable non-profit nor a formal unit of DHHS (again similar to the CVAB). The bill language should clarify this.

MichBio suggests that an appropriate model legislation is one recently enacted by the State of Ohio (HB 412), though there are others that offer a comprehensive RDAC structure. Similarly, a successful example of an advisory body housed within MDHHS and focused on meeting patient and public health needs, one should look no further than the Michigan BioTrust for Health CVAB.

Thank you for the opportunity to comment on HB 4654; we reserve the right to provide further comments and possible amendments.

We look forward to working with you, Rep. Clemente and other co-sponsors, the Health Policy Committee, as well as rare disease community stakeholders to establish a RDAC that will advance the interests of the Michigan's rare disease patients in a robust and sustainable manner.

Sincerely,

Stephen Rapundalo, PhD
President and CEO

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