



6/1/2020

Re: Ohio House Bill 412 - establish Rare Disease Advisory Council

Chairman Burke, Vice Chairman Dr. Huffman, Ranking Member Antonio and Members of the Committee:

As the statewide organization representing more than 300 entities employing over 100,000 individuals in Ohio's bioscience community, BioOhio submits this testimony in support of HB 412, to establish an Ohio Rare Disease Council.

Our members are curing debilitating pediatric diseases, creating less invasive surgical tools, programming highly accurate diagnostic imaging techniques, improving provider processes to reduce patient costs, personalizing medication delivery for individual patients, and much more. Patients are at the heart of the bioscience industry, which is why we support HB 412.

Currently there are approximately 7,000 rare diseases. By establishing a Rare Disease Advisory Council in Ohio, it will give rare disease patients, their families and providers an instrument to share important insight and make recommendations to elected officials and administration leaders on health care issues relative to rare diseases.

Falling under Rare Disease is Ohio's nationwide strength in Gene & Cell Therapy.

Anchored by the Research Institute at Nationwide Children's Hospital and The Ohio State University, both in Columbus, the state of Ohio has become fertile and successful ground for the rapidly evolving gene therapy industry. World-class researchers, high-tech facilities, executive management & regulatory expertise and a growing investor base fuel an ecosystem that is creating startups and attracting both entrepreneurs and international investment to highly regarded healthcare hubs built around the Cleveland Clinic in Northeast Ohio, Cincinnati Children's hospital in Southwest Ohio, and Columbus.

Consider the following aspects of Ohio's growing gene therapy ecosystem and success:

- Nearly \$10 billion of investment in gene therapy startups spinning out of Nationwide Children's Hospital since 2018.
- Ohio is tied for 4th among states in number of identified gene therapy startups and companies.

1275 Kinnear Road, Columbus, OH 43212  
Telephone # (614) 675-3686, Fax # (614) 675-3687  
[www.bioohio.com](http://www.bioohio.com)

- Its hospitals have some of the most advanced research and development facilities in the nation and attract top researchers from around the world.
- This ecosystem attracts established companies and attracted them from across the country. Abeona Therapeutics (Epidermolysis Bullosa, Sanfilippo Syndrome and Batten Disease), Milo Biotechnology (increasing muscle strength/improving patients quality of life with muscular dystrophy), Avexis (Spinal Muscular Atrophy), Myonex (Duchenne's), Celenex (Batten disease), Aruvant Sciences (hematological conditions) and others are either now publicly traded companies or purchased by large pharmaceutical companies, with products in the market, serving patients.
- In 2019 Sarepta Therapeutics not just purchased Myonex but are finishing building their \$100M global gene therapy research & development center in Columbus.
- Cincinnati Children's and Nationwide Children's Hospital are 2 of the top 7 pediatric research facilities in the U.S.
- This statewide network also includes the National Center for Regenerative Medicine (NCRM)-see below
- **Clinical Trial Data - Cincinnati Children's Hospital Medical Center is The National Coordinating Center for Rare Disease and is facilitating studies involving thousands of scientists across the U.S.—and 22 other nations—by collecting, organizing, sharing, and analyzing exploding amounts of “Big Data” about rare diseases, including cell and gene therapies.**
- The talent pipeline is clearly in Ohio to support the state's growing gene therapy sector due to this ecosystem.
- A relatively low cost of living and high quality of life.
- In early 2020, Nationwide Children's spun out Andelyn Biosciences, for manufacturing of early-stage gene therapy products for Phase I and II clinical trials, providing commercial-scale Good Manufacturing Practices (cGMP) clinical manufacturing for gene therapies. Multiple gene therapy drugs, several of which are in the market have been clinically proven.
- Not just industry support but also State of Ohio support is clearly present with the 2020 announcement of InnovateOhio's Gene & Cell Therapy Work Group as a state priority.

This statewide network also includes the National Center for Regenerative Medicine (NCRM), a designated national Center of Excellence since 2007) in Ohio's strongest density of healthcare R&D on Case Western Reserve University's campus, next to the Cleveland Clinic and University Hospitals, in partnership with The Ohio State University in Columbus. It has deep expertise (120 principal investigators), biomanufacturing experience (14 active therapies in the labs), and a statewide network including healthcare innovators (e.g. Cincinnati Children's and Nationwide Children's Hospital (Columbus) ranked respectively #3 & #7 nationally, numerous startups and corporations (e.g. STERIS, Cardinal Health, Athersys and Charles River Labs).


The ability to effectively run clinical trials interests many pharma and device companies as 16% of all U.S. clinical trials are conducted at Ohio institutions, including 3,000 annual trials at The Ohio State University alone.

Finally, Ohio's workforce talent is a critical differentiator as close to 1 million people are employed in bioscience, healthcare, and related industries. Annually, Ohio students earn ~30,000 degrees in STEM fields, exceeding totals from comparable states, such as North Carolina, at four-year institutions and workforce-focused schools such as Cuyahoga Community College (Cleveland), which has five degree and workforce certificate programs dedicated to the pharma/biotech industry, Sinclair Community College (Dayton), and please check out other Education/Talent resources at BioOhio. <https://www.bioohio.com/education/>;  
<https://www.bioohio.com/talent/>

HB 412 creates a unique collaborative council with a wide variety of perspectives including patients, healthcare providers, insurers, the manufacturing industry, and our bioscience researchers. This educational resource will communicate with stakeholders about the ways in which rare disease patients interact with our health care system.

BioOhio supports measures that benefit Ohio patients. We urge the General Assembly's support in enacting HB 412 and providing the opportunity to advise Ohio's key policy makers on the research, diagnosis, treatment, and education related to rare diseases.

For further questions, please do not hesitate to contact me at [jlewis@bioohio.com](mailto:jlewis@bioohio.com).  
Sincerely,



John F. Lewis Jr.  
President & CEO  
BioOhio