

September 16, 2014



Hon. Gail Haines
Chair, Health Policy Committee
Michigan House of Representatives
PO Box 30036
Lansing, MI 48909

Dear Rep. Haines,

On behalf of the Michigan BioSciences Industry Association (MichBio) we respectfully write to you in regard to Senate Bill 991 sponsored by Sen. Pappageorge, passed by the Senate, and referred to the House Health Policy Committee.

The proposed legislation will only raise false hope for already desperate patients and create the illusion of "right to try". It will not allow access to investigational new drugs a priori. Instead it will create a public furor over an inability to deliver on hollow promises, as well as harm the reputation of Michigan's biopharma companies and the industry.

Let's be very clear...the biosciences industry would like nothing more than to provide quick access to new therapies and devices for patients in need. Indeed, it is our industry's mission. As such we value the intent behind the proposed legislation.

SB 991, as proposed here, will be either wholly unconstitutional or at least unconstitutional in the majority of possible applications. That is because the U.S. Food & Drug Administration (FDA) has exclusive jurisdiction over the regulation of drugs via Congress's constitutional authority to regulate interstate commerce. State laws (i.e., as proposed in this current legislation) that impinge on that exclusivity are pre-empted. Federal drug laws regulate not only the approval process for new drugs, but also the misbranding and adulteration of drugs, both of which could come into play with the provision of an unapproved drug to a patient.

Proponents of "right to try" legislation say that such laws are needed since the FDA takes too long with drug reviews and approvals. However, the FDA is mandated by Congress to carefully review each new drug application based on the peer-reviewed merit of its scientific and medical data following stringent regulations to ensure safety and efficacy.

Moreover, a majority decision in 2007 by the U.S. Court of Appeals for the District of Columbia Circuit affirmed that terminally ill patients have no constitutional right to unapproved drugs.

Does this mean that drug development of investigational new drugs cannot be accelerated or that patients can't seek earlier access to them? The short answer is no. Indeed, SB 991 reflects many aspects of the FDA's Expanded Access Programs, of which the industry is supportive.

In the FDA's evaluation of requests made through the Expanded Access Programs they must determine, based on the information available, that the potential benefit justifies the potential risk; and, that those risks are not unreasonable in the context of the disease or condition being treated. Additional considerations of the FDA are that providing access will not interfere with the development of the drug or device, and that the patient cannot receive treatment through any other protocol (e.g., clinical study). The FDA continues to offer updates and new guidance documents on the EAP, the latest of which was released in May of 2013¹. These updates and clarifications demonstrate the FDA's focus on the importance of patient access, especially for patients and groups of patients facing serious and life-threatening diseases

¹ <http://www.fda.gov/downloads/Drugs/.../Guidances/UCM351261.pdf%E2%80%8E>

or conditions. In fact, the FDA receives hundreds of applications every year from drug companies to supply drugs to individuals before final approval and agrees to nearly all of them.

Together with other state/national partners and federal policymakers, MichBio has worked in concert with the FDA to create and improve mechanisms for patient access. The provision, Expanded Access to Unapproved Therapies and Diagnostics (EAP) in Section 561² of the Federal Food, Drug and Cosmetic Act (FFDCA)³, authorizes the use of investigational drugs and devices for patients with serious or immediately life-threatening diseases or conditions who lack other therapeutic options.

Notwithstanding FDA regulations and initiatives, it should be pointed out that several other factors may make it unlikely that new investigational drugs could be made available to terminally ill patients. For instance, drug supply is one such concern. Typically only enough is manufactured to support approved clinical trials and "on-demand" manufacturing would not be a simple, short nor cost-effective process. Second, the management of any emergency program by a manufacturer may present a challenge and financial burden. This would be a significant issue to small biotechnology companies, of which we have many in Michigan, with little resources to support such a program. This would not only jeopardize their path to commercialization, but have economic impacts for Michigan's economy and long-term sustainability of the state's bio-industry.

Furthermore, and contrary to what you might hear otherwise, it is important to underscore that Phase I clinical trials of investigational drugs are too limited to assure complete safety. They are merely a preliminary indication of confidence, but one still fraught with much medical threat. Terminally ill patients could face additional perils – unanticipated pain and torment, quicker time to death, and a lesser quality of life for the time remaining. While they may be willing to accept such risks, biopharma manufacturers and healthcare providers may not wish to be party to such suffering based on ethical, medical and legal grounds.

The bioscience industry in Michigan employs over 42,000 within 1700+ companies across the state. Many of these companies are mandated to follow FDA rules and regulations, including the parameters within the EAP. The relationship between industry and the FDA has an essential balance, where FDA oversight on safety and effectiveness is critical in bringing new treatments to patients, as well as ensuring that clinical trial data is not compromised. The FDA is a critical partner in our companies' efforts to bring safe and effective treatment options to patients. Without a strong and effective FDA, we cannot have a robust and competitive industry.

Our companies will be extremely wary of funning afoul of the FDA when considering making available investigational new drugs to terminally ill patients. The potential risks to clinical development and market access, as well as to accepted standards of care will simply be too high.

Thus, we have serious concerns with any approach to make investigational medicines available that seeks to bypass the oversight of the FDA and clinical trial process.

By passing this legislation, the House will only create the illusion of hope where little surety exists. This would not be in the best interest of patients and public health in Michigan.

Sincerely yours,



Stephen Rapundalo, PhD
President and CEO

² <http://www.gpo.gov/fdsys/pkg/USCODE-2010-title21/pdf/USCODE-2010-title21-chap9-subchapV-partE-sec360bbb.pdf>

³ <http://www.fda.gov/RegulatoryInformation/Legislation/FederalFoodDrugandCosmeticAct/FDCA/default.htm>