



July 14, 2014

Hon. Jim Marleau
Chairman, Health Policy Committee
Michigan State Senate
State Capitol
Lansing, MI

Dear Senator Marleau,

On behalf of the Michigan BioSciences Industry Association (MichBio) we respectfully write to you in regard to Senate Bill 991 sponsored by Sen. Pappageorge and other esteemed legislators.

Providing access to new therapies and devices for patients is of the utmost importance for our industry. As such we value the intent behind SB 991 to provide access for patients to novel, life-saving treatments.

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 561¹ of the Federal Food, Drug and Cosmetic Act (FFDCA)², authorizes the use of investigational drugs and devices for patients with serious or immediately life-threatening diseases or conditions who lack other therapeutic options.

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 2013³. 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 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.

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. As you

¹ <http://www.gpo.gov/fdsys/pkg/USCODE-2010-title21/pdf/USCODE-2010-title21-chap9-subchapV-partE-sec360bbb.pdf>

² <http://www.fda.gov/RegulatoryInformation/Legislation/FederalFoodDrugandCosmeticActFDCA/default.htm>

³ <http://www.fda.gov/downloads/Drugs/.../Guidances/UCM351261.pdf%E2%80%8E>

know, the bioscience industry in Michigan employs over 42,000 within 600+ 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.

SB 991 reflects many aspects of the FDA's Expanded Access Programs, of which the industry is supportive. It should be noted that the regulations and actions administered by the FDA will likely preempt SB 991, making the legislation difficult if not impossible to implement. In this regard, SB 991 could provide patients with false hope on the impact state legislation could have on access to novel therapies and devices since much of the legislation will be preempted by federal law.

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 nor short process. Second, the management of any emergency program by a manufacturer may present a challenge and burden, particularly to small biotechnology companies with little resources to support such a program. Moreover, Phase I clinical trials of investigational drugs are too limited to assure complete safety and thus terminally ill patients could face additional risks.

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. This would not be in the best interest of patients and public health.

The industry as a whole understands the significant need for patients with life-threatening, serious diseases and conditions to have access to investigational therapies and devices that could better their health outcome. Indeed efforts like the 21st Century Cures that are currently being led by Rep. Fred Upton and other federal policymakers seek to identify additional ways to speed up the approval of new treatments and access by patients. To that end, we will continue to work alongside the FDA to ensure patients will have access to new and investigational therapies in the most efficient and effective manner.

We stand ready to provide you with additional resources and information on the FDA and the Expanded Access Programs. We are also happy to provide you with connections to experts in these areas who would be able to discuss this with you further.

Sincerely yours,

A handwritten signature in black ink that reads "Stephen Rapundalo". The signature is written in a cursive, flowing style.

Stephen Rapundalo, PhD
President and CEO