The James
THE OHIO STATE UNIVERSITY
COMPREHENSIVE CANCER CENTER

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The Honorable Shannon Jones
Chair, Senate Health and Human Services Committee
Senate Building
1 Capitol Square, 2nd Floor
Columbus, Ohio 43215

Dear Senator Jones:

On behalf of the Ohio State University Comprehensive Cancer Center – James Cancer Hospital and Solove Research Institute (OSUCCC-James), thank you for the opportunity to submit written comments on the U.S. FDA Drugs-Terminally Ill legislation (House Bill 290), otherwise known as the "Right to Try" bill.

We commend Representatives Sprague and Anielski for introducing H.B. 290. The bill would provide patients with terminal conditions access to experimental therapies that have gone through Phase I clinical trials, if approved by their physician and access is granted by the manufacturer. As one of the nation's premier institutions for cancer research and advancing patient care, we understand the desire to find new ways to treat patients who have received a life-threatening diagnosis.

The OSUCCC – James has more than 500 open clinical trials at any given time, with some of the world's latest discoveries available to clinical trial patients. We are one of only four comprehensive cancer centers funded by the National Cancer Institute (NCI) to conduct phase I and phase II clinical trials. These trials go only to centers that demonstrate an exemplary capacity for research and clinical care, the expertise to deliver the latest in treatments, and the infrastructure to interpret and track treatment results.

Additionally, Ohio State has nearly 300 cancer researchers dedicated to understanding what makes each patient's cancer grow, move, metastasize or reoccur. Because of the OSUCCC – James' NCI phase I and II approvals, these experts can move research discoveries into clinical trials and make them available to patients sooner.

Clinical trials are key to developing new methods to prevent, detect and treat cancer. It is through clinical trials that researchers can determine whether new treatments are safe and effective and work better than current treatments. When a patient takes part in a clinical trial, he or she adds to our knowledge about cancer and help improve cancer care. The OSUCCC-James has a tremendous track record for participation in trials. Compared to the national average of 12 percent among Comprehensive Cancer Centers, 23 percent of our patients enroll in trials.

Because participation in clinical trials is so important for the patient and future treatments, we have worked to make sure that it is financially viable for participants. All care associated with the trial itself is provided at no cost

to the patient. In addition, we worked to pass both federal and state legislation to ensure that routine care costs are remain covered by insurance when a patient participates in a trial.

Unfortunately, there are times when participation in a clinical trial is not possible for a patient, particularly if a patient does not meet the approved research protocol. For such cases, the federal Food and Drug Administration (FDA) has created a "compassionate use" authorization process to allow treatment with a new, unapproved drug when no other treatments are available. Last year, the FDA simplified this process to ease access to unapproved drugs and devices in such circumstances. The current FDA process has been carefully crafted to put patients first, and the administrative burden and approval process times have been greatly reduced. It takes about an hour to complete required paperwork, and approval can be obtained in as little as 24 hours if needed on an emergency basis.

We support and currently utilize this process as needed, for approximately 10 cancer patients per year. This process, similar to that outlined in H.B. 290, allows the manufacturer to determine whether to make the drug available. Drug companies may only have enough available for use in a clinical trial and may or may not make it available. Some will supply it for free, but others charge patients. Most insurance companies will not pay for investigational drugs in these cases. And there may be other costs, such as the clinic's cost of giving the drug and monitoring one's response that might not be covered by insurance. Nonetheless, it is the right option in some circumstances.

While H.B. 290 proposes to create a similar process outside FDA approval, providing access to an investigational drug, product, or device after a Phase I clinical trial, without participating in an ongoing clinical trial, represents a risk to patients. We appreciate the willingness Senator Hite and Representative Sprague to work with us to mitigate these risks.

Phase I clinical trials are first-in-human trials, involve a small sample size of patients (15 – 30), and help determine toxicity and side effects. Such trials do not establish efficacy or safety for the patient. The evidence of safety and effectiveness is determined through later-phase clinical trials. Accessing early-stage experimental drugs could result in someone dying faster than they otherwise would, or result in significant suffering through side effects of the drug. While the majority of new therapies (63.2 percent) will reach a Phase II clinical trial, overall only 9.6 percent of therapies progress from a Phase I trial to reach final approval.[1]

Federal rules help ensure that clinical trials are safe. A patient's rights and safety are protected through informed consent, and the research protocol is approved and monitored by an Institutional Review Board (IRB). While H.B. 290 includes a written consent requirement, we thought additional transparency was needed for the patient to understand the full risks and responsibilities associated with accessing an investigational product outside of a clinical trial.

[1] Thomas, David W., Burns, J, Audette, J., Carroll, A., Dow-Hydelund, C., Hay, M. (2016). Clinical Development Success Rates 2016-2015. Retrieved from https://www.bio.org/sites/default/files/Clinical%20Development%20Success%20Rates%202006-2015%20-%20BIO,%20Biomedtracker,%20Amplion%202016.pdf

Amendment 2991 offered by Senator Hite would strengthen this informed consent in several ways:

1. It requires the physician to use a template created by the Ohio State Medical Board to ensure there is consistent information provided to patients across the state.
2. It adds that there is no proof of efficacy of the treatment, in addition to existing bill language that requires best and worst outcomes to be specified.
3. It specifies that the patient's insurance plan is not required to provide coverage for the treatment or associated costs.
4. It includes in the consent that there is no cause of action against the provider or manufacturer for use of the investigational drug, product or device. The bill stipulates this elsewhere, but it is important the patient receive such information.

While we support offering options to patients, we also do not want to draw attention and resources away from efforts to develop effective treatments. Advancements in new treatments only can be achieved through the multi-step clinical trials process, which is conducted in a systematic way to get information regarding the benefits of a new therapy. Patients may choose to get the drug through this new mechanism rather than participate in a trial, potentially delaying research and new therapies. We appreciate the willingness of Sen. Hite and the sponsors to include language that would clarify that a patient should first enroll in a clinical trial to treat his or her terminal condition, if the patient is eligible for enrollment and it is available within 100 miles. Other states have included this clinical trial enrollment threshold in their Right to Try laws. This provision also is included in amendment 2991 expected to be offered by Sen. Hite.

Finally, we thank Senator Hite for including language that specifies that the bill does not create a claim against a physician or hospital that does **not** recommend this treatment option. There may be circumstances in which, in a physician's clinical judgement, accessing such an unapproved therapy is not in the patient's best interest.

Thank you for considering our thoughts on this legislation and we urge support for Senator Hite's amendment. If these provisions are included in the bill, we recommend support for the legislation.

Sincerely,

Jennifer K. Carlson
Associate Vice President for External Relations and Advocacy