The Cancer Support Community (CSC) is the largest direct provider of social and emotional support services for people impacted by cancer, and the largest nonprofit employer of psychosocial oncology professionals in the United States. As such, CSC has a unique understanding of the cancer patient experience. Each year, CSC serves more than one million people affected by cancer through its network of over 40 licensed affiliates, more than 120 satellite locations, and a dynamic online community of individuals receiving social support services. Overall, we deliver more than $40 million in free, personalized services each year to individuals and families affected by cancer nationwide and internationally.

**Background**
As an organization, CSC is dedicated to ensuring that all patients have access to high quality, appropriate, comprehensive, and affordable care. Individuals have the right to decide what type of care they do or do not receive and they should be empowered to make well-informed decisions, in collaboration with their loved ones and healthcare team, as to what type of treatments they choose. Patients should have access to therapies that have been proven to be safe and effective, as well as opportunities to gain access to investigational therapies through expanded access or “compassionate use” pathways should they wish to do so. Unfortunately, there are times when there are not many treatment options available for individuals, especially toward the end of life. When this is the case, organizations like CSC offer resources and support to help patients process information about their treatment options so that they may make informed decisions about their care. CSC advocates for services and resources to empower patients to be their own best self-advocates and to access all available treatment options within the constraints of the law. Under the current regulatory system, the U.S. Food and Drug Administration (FDA) approves 99.7% of the submitted expanded access requests for investigational new drugs and protocols (Jarrow et al., 2016).

The following principles guide the work of CSC in regards to this issue:

**Principles**

1. **Patients Must Maintain Self-Determination.**
   CSC supports patient self-determination. Patients should have the ability to make their own decisions about the type of treatment they chose to receive or not receive. Before making treatment decisions, patients should have the opportunity and resources necessary to make well-informed decisions based on the risks and benefits of a therapy. Based on a
patient’s own personal values, he or she should be able to select a treatment that is most likely to help them achieve their desired outcomes.

2. **Patients Must Be Informed About Safety Risks.**

Patients must be informed about any risks associated with treatments obtained outside of the FDA’s regulatory system. The FDA is responsible for protecting the public health by assuring the safety, efficacy and security of drugs, biological products, and medical devices while advancing the public health by working to speed innovations that make medicines safer and more effective (FDA, n.d.-a). The current regulatory system protects patients from undue harm and anytime an individual accesses a therapy outside of this regulatory approval system, he or she could be put at risk.

A product is not deemed safe and effective until it has completed the regulatory approval process. Anytime an individual accesses an unapproved, investigational agent he or she is at risk of harm. Individuals accessing drugs through expanded access programs may be different (by disease type, disease stage, patient demographics, etc.) than individuals in the controlled clinical trials for those same products. Therefore, it is possible that the product will respond differently than in the clinical trial.

All patients, whether participating in a clinical trial or taking an unapproved product through expanded access, should have the right to be protected by clinical research subject protections. Individuals in expanded access programs are not in clinical trials and therefore are not afforded these protections. As a result, these patients are at risk of undue harm, and may not be fully aware of these risks. There is also an ethical responsibility to protect patients from misguided expectations—just because a drug is working for individuals in a controlled clinical trial does not mean it will work for individuals outside of that trial in different circumstances. Allowing patients to try any unproven therapy may put the patient at risk for physical and emotional harm.

3. **The Integrity of Data Collection Must Not Be Altered.**

Right to try efforts should not circumvent clinical trial protocols, including the collection of adverse event and drug safety information. When individuals receive a product through expanded access, their experiences are not captured as part of the clinical trial. Adverse events that individuals may experience are not captured in real-time or in as much detail as they would be if enrolled in a trial. This data may be useful for the ultimate evaluation of the product yet because individuals receiving a drug through expanded access are not enrolled in clinical trials, the findings will not be captured. Any changes to or divergence from the current expanded access program should include robust data collection mechanisms.

4. **The Drug Development Infrastructure Must Not Be Negatively Impacted.**

CSC does not support the loosening of existing rules and regulations that reduce the role of the FDA in determining access to investigational therapies. Bypassing the current expanded access system will not increase patient access to effective treatments and may create unintended consequences. Lowering the standards and regulatory oversight of access to investigational products may put patients at risk as products will not need
confirmed safety or effectiveness as proven by the clinical trials process. Additionally, lowering the integrity of the clinical trial process by granting access for individuals not enrolled in trials could reduce incentives for patients to participate in clinical trials. This could have an overall negative effect on the drug development paradigm given that currently less than 5% of eligible participants enroll in cancer clinical trials (Unger et al., 2016).

5. **Treatment Supplies Must Not Be Put In Jeopardy.**
   Expanded access programs are part of the biopharmaceutical industry’s commitment to patients, however participation in such programs must not jeopardize the supply of a drug needed for patients enrolled in ongoing clinical trials nor delay the start of new clinical trials. During the development of new drugs, supplies for clinical trials are manufactured on a scale commensurate with the investigational drug’s stage of development and the supply may only be sufficient for the patients enrolled in the clinical trial. In addition to the amount of the drug on hand, there are also limited manufacturing capabilities until a product receives full approval. Manufacturers may not have enough product to meet the demand of patients who are willing to try unapproved therapies, and providing access to these individuals limits the amount that is available for those who are involved in the trial, potentially slowing down the trial.

6. **Improvements to the Current Regulatory System Must Be Ongoing.**
   The FDA has begun to implement efficiencies to their expanded access program such as reviewing applications for expanded access within 24 hours and requiring only one Institutional Review Board (IRB) to respond favorably to a prescribing physician’s request to secure early access to a product. Thanks to the 21st Century Cures Act of 2016, drug manufacturers are required to publically share information on their early access programs via their website or other public-facing avenues and must include contact information for interested patients.

**Definitions**

**Expanded Access:** Expanded access, sometimes called "compassionate use," is the use outside of a clinical trial of an investigational medical product (i.e. one that has not been approved by the FDA (FDA, n.d.-b). To qualify for the Expanded Access program, a patient’s treating physician must determine that the probable risk to the patient from the investigational drug is not greater than the probable risk from the patient’s disease or condition. Once the physician makes this determination, and the patient and physician together decide that it is appropriate to pursue this treatment option, the physician approaches the pharmaceutical company to obtain agreement from the sponsor/company that it will provide the drug being sought. If the company agrees, the physician then submits the request to FDA. Key protections are included for patients receiving experimental treatments through the expanded access program (FDA, n.d.-c).

**Right to Try:** Legislative attempts to allow patients with life-threatening diseases to bypass the FDA in receiving experimental drug treatments would not mandate that drug manufacturers provide access to unapproved therapies, but it would allow drug manufacturers to provide access to unapproved therapies outside of a clinical trial without the approval of the FDA.
References


