



July 23, 2019

Chairperson James Falahee
c/o Michigan Department of Community Health Certificate of Need (CON) Program
Policy Section
South Grand Building
333 S. Grand Ave.
Lansing, MI 48933
MDHHS-ConWebTeam@michigan.gov

Re: Proposed New Standard for Immune Effector Cell Therapy (IECT) Services

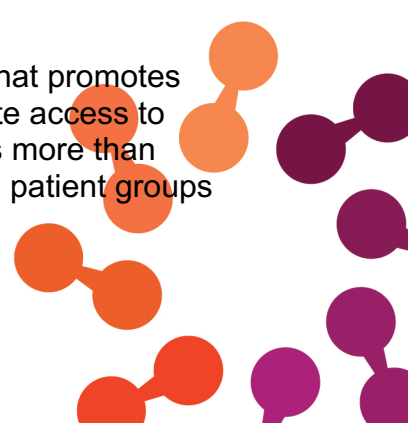
Dear Chairman Falahee and the CON Commission,

The Alliance for Regenerative Medicine (ARM) appreciates the opportunity to comment on the creation of a new standard for Immune Effector Cell Therapy (IECT) Services, which includes the administration of CAR T therapies. If implemented, this new standard would require any facility to receive both the Commission's approval and a third-party accreditation from the Foundation for the Accreditation of Cellular Therapy (FACT) in order to deliver these transformative therapies to patients in Michigan. Each facility has three years to receive both the Commission's approval and FACT accreditation.

ARM understands that this new process was developed to ensure quality of care and promote the safe administration of this new and innovative class of therapies. ARM, however, respectfully disagrees with the premise behind this new accreditation program because the manufacturing process for CAR T therapies and the current manufacturer site accreditation process already ensures safe administration and does not require a significant capital investment. As such, a new standard is not necessary. Specifically, the specialized re-engineering of a patient's T cells are manufactured off-site by the manufacturer of the CAR T and each manufacturer already accredits each facility via a FDA REMS requirement.

Therefore, ARM asks that you rescind the recommendation to create a new standard for IECT that will be an onerous barrier to access, create an unnecessary financial burden on health care facilities across the state, and limit the sites of care from offering cellular therapies to patients.

ARM is an international multi-stakeholder advocacy organization that promotes legislative, regulatory, and reimbursement initiatives necessary to facilitate access to life-giving advances in regenerative medicine worldwide. ARM comprises more than 300 leading life sciences companies, research institutions, investors, and patient groups



that represent the regenerative medicine and advanced therapies community. ARM takes the lead on the sector's most pressing and significant issues, fostering research, development, investment, and commercialization of transformational treatments and cures for patients worldwide. As of year-end 2018, ARM estimates there are 906 regenerative medicine and advanced therapies developers worldwide sponsoring 1,028 clinical trials across dozens of indications, including oncology, cardiovascular, central nervous system, musculoskeletal, metabolic disorders, ophthalmological disorders, and more.¹

A subset of these clinical trials focuses on the power of chimeric antigen receptor (CAR T) therapies. These therapies are the first in a wave of new and exciting advanced therapies and technologies that are the next frontier in the fight against some of humankind's most devastating diseases and disorders. CAR T therapy is a type of treatment in which a patient's T cells (a type of immune system cell) are changed in the laboratory so they will attack cancer cells. T cells are taken from a patient's blood, as it flows through a tube to an apheresis machine, which removes the white blood cells, including the T cells, and sends the rest of the blood back to the patient. Then, the gene for a special receptor called a chimeric antigen receptor (CAR) is inserted into the T cells in the laboratory. Millions of the CAR T cells are grown in the laboratory and then given to the patient by infusion. The CAR T cells are able to bind to an antigen on the cancer cells and kill them.² ARM is currently tracking the outcomes of the approximately 158 ongoing clinical trials using the CAR T technology in a variety of stages of cancer and cancer types. ARM believes that this new and promising technology provides the possibility that most future treatments for many types of cancer at its many stages will focus on using the power of the patient's immune system to fight their particular disease.

What's critical about all the technologies represented by ARM, including CAR T, immunotherapy, and cell and gene therapy, is that many of the products are transformative – they provide a durable therapeutic benefit or even a cure with a single administration of the therapy. For patients suffering from a diverse array of serious and costly conditions, many without current therapeutic option, this field represents enormous potential and hope.

1. Creating a New Standard for IECT is Outside the Scope and Jurisdiction of the Michigan Certificate of Need (CON) Program

ARM believes that the jurisdiction to create a new standard to regulate CAR T cell therapy is outside the intent and scope of the Michigan CON Commission because the CON program is intended to control health care costs while ensuring safety and access by restricting health care facilities from unnecessary and expensive capital investments to serve their patient populations. These factors do not exist for a CAR T administration.

¹ <https://alliancerm.org/publication/2018-annual-report/>

² <https://www.cancer.gov/publications/dictionaries/cancer-terms/def/CAR-T-cell-therapy>

Given current federal regulatory oversight of the manufacturing process, the new standard is unnecessary. Cell therapy products, such as those identified in the proposed language, can only be marketed if they receive FDA approval. By definition, that ensures that the products have undergone the scientifically rigorous FDA review process and have demonstrated safety. In addition, under current FDA rules, the agency has the authority to use Risk Evaluation and Mitigation Strategies (REMS) and other required programs to limit where patients can receive therapies – particularly new cell therapies. In other words, the safety of CAR T therapies is determined by the FDA and the FDA can regulate the site of care for administration. Finally, the significant capital investment required to create a CAR T therapy is absorbed by the manufacturer at a manufacturer's FDA regulated facility. The provider is not manufacturing the CAR T therapy such that neither objective of the CON program applies to CAR T therapies.

ARM urges the Commission to reject the new proposed standard because the scope of the CON program does not apply to CAR T therapies. Rather, ARM believes the FDA is best suited to regulate and determine the safety conditions for administering this new classes of therapies.

2. Proposal to Require Third Party Accreditation Will Limit Access to CAR T cell therapy for Michiganders

As stated above, the safety criteria are an important component of what a facility must demonstrate to deliver CAR T cell therapy and manage the associated adverse events. In their review and approval, the FDA requires comprehensive safety criteria and standards for any facilities to provide CAR T cell therapy through REMS. Additionally, the federal government is currently reviewing coverage and access for CAR T cell therapy, which may include additional provider site requirements. While we are encouraged by the discussion and interest of the Michigan CON Commission to provide CAR T therapy to patients, we believe it is premature for the Commission to approve any recommendation that could limit patient access by requiring additional onerous and costly approvals and third-party accreditation on sites of care that go beyond what the federal government requires, which may discourage or prevent sites of care from providing CAR T therapy to patients.

ARM believes that the proposed changes will negatively impact patient access to current and potentially new therapies. Based upon this new proposed standard, it seems that all of these facilities would now need to apply for a CON, which will likely disrupt access to current and future marketed therapies.

For example, based on current regulatory requirements and the burdensome process of obtaining a CON, many facilities will not be certified and their patients will have to seek care elsewhere. This will create a significant disruption in care, deterioration of treatment, and is likely to limit patient access to treatments that patients are currently receiving.

In addition, by restricting the number of facilities that a patient could receive a cell therapy treatment, the proposal specifically limits patient access to new transformative cell therapies. We have heard from many patients suffering from cancer about how these products represent their last and best chance for survival. To deliberately limit access to care for these patients would be, at best, unjust.

For a patient who receives CAR T therapy, the medically appropriate selection of administration as an inpatient or outpatient, in a transplant or non-transplant center, will depend on their treating provider's clinical judgment of the patient's clinical circumstances and the safety-related labeling provisions for the relevant CAR T therapy. With over 80 percent of cancer patients currently being treated in the community setting, it is imperative to ensure patient access to these new and transformative therapies, a provider's medical judgment and the patient's individual medical needs should determine the appropriate site of care. A new standard for IECT will prematurely limit the sites of care that can and will provide CAR T cell therapy services.

3. The Proposed Changes are Likely to Increase Medical Costs

By limiting the sites that can provide these potential lifesaving therapies, the sites gain significant leverage against payers for network participation. The CON sites will likely be able to dictate payment rates to Michigan health plans because they will be the only providers of these lifesaving therapies. These increased costs to the health plans could result in higher premiums for all patients. ARM believes that the new proposed standard will harm actual patient access to currently available and new and innovative therapies while also harming access due to the likely increased financial burden.

Conclusion

ARM believes it is unnecessary to require additional onerous and costly approvals and accreditations on any facilities that go beyond what the federal government requires, which may discourage or prevent sites from providing CAR-T cell therapy to patients.

ARM therefore urges the Michigan Certificate of Need Commission to take into consideration our comments and reject the proposed new standard for IECT. ARM looks forward to working with you to create policies that afford appropriate and equal access to innovative therapies. If you have any questions or need any additional information, please do not hesitate to contact me at rfalb@alliancerm.org

Sincerely,



Robert Falb
Director, U.S. Policy and Advocacy