

# 2023 HILL DAY

## ASTCT URGES CONGRESS TO SUPPORT PATIENT ACCESS TO CELL THERAPIES

1. By assisting with FDA drug shortages
2. Supporting the Donor Leave Act (H.R 3024)
3. Requiring state Medicaid programs that authorize out-of-state care, to accept active Medicare enrollment to make payment
4. Increased funding for the National Institutes of Health (NIH)

## BACKGROUND ON THE AMERICAN SOCIETY FOR TRANSPLANTATION AND CELLULAR THERAPY (ASTCT)

The ASTCT is a professional membership association of more than 3,700 physicians, scientists, and other health care professionals promoting blood and marrow transplantation and cellular therapy through research, education, scholarly publication, and clinical standards. Our Society's clinical teams have been instrumental in developing and implementing clinical care standards and advancing cellular therapy science, including participation in trials that led to current Food and Drug Administration (FDA) approvals for chimeric antigen receptor T-cell (CAR-T) therapy and hematopoietic stem cell-based gene therapies for genetic immune system and blood disorders.

For more than 25 years, ASTCT members have focused on innovation in the treatment of hematologic malignancies, hematologic disorders, and other immune system diseases. ASTCT members very much rely on team care for the complex cancers and other disorders requiring hematopoietic stem cell transplants (HSCTs) and newer cell therapies like CAR-T.

## WHAT ARE CELL AND GENE THERAPIES?

**Cell therapies, of which CAR-T therapy is one type,** are innovative, personalized, and life-saving immunotherapy for patients with cancers and other acquired and inherited conditions. Often these therapies are for patients who have exhausted all other therapies. Since 2017, the Food and Drug Administration (FDA) has approved 6 CAR-T products for multiple indications.

**Gene therapies are innovative, personalized, treatments often for rare inherited disorders.** There have been multiple gene therapies approved, with several more expected in the next 1-2 years. ASTCT members focus on hematopoietic stem cell (HSC) gene therapies, which used gene addition or gene editing to correct blood disorders and other metabolic and/or autoimmune disorders. HSC gene therapies are delivered via autologous stem cell transplants.



# HOW CONGRESS CAN HELP

## Drug Shortages

### The Problem:

According to a National Comprehensive Cancer Network (NCCN) survey, 93% of cancer centers reported a shortage of carboplatin and 70% report shortages for cisplatin. Additionally, transplant physicians and pharmacists use a drug called fludarabine for both stem cell transplant patients and CAR-T therapy patients, which is also in short supply. Currently in oncology, there are potential curative therapies for these diseases and the delays and shortages from the FDA is delaying curative treatment for patients.

The ASTCT in partnership with the National Marrow Donor Program (NMDP) wrote a letter to the FDA in June 2022 regarding the impact the shortages have had on therapies such as fludarabine. Fludarabine is a therapy within transplant conditioning that is the standard for our immunocompromised patient population. With the shortages continuation, transplant centers have been forced to move away from fludarabine-based regimens and use alternative drugs (i.e. cladribine or clofarabine) which are significantly less studied with less supporting evidence of use and effectiveness. The continued shortage is forcing centers to use non-FDA approved lymphodepleting regimens that may negatively impact the success of a possibly life-saving CAR-T therapy.

We requested the FDA take immediate action on the critical shortages. There remains uncertainty with supply and no timeframe for the return of availability of these therapies. As a result, the ASTCT published a list of FAQs advising centers on administration and conservation of currently supplies.

### Proposed Solutions:

We request that Congress reach out to the FDA on our behalf asking for a timeframe for the return of availability of therapies and in what ways transplant centers can get therapies through their suppliers in a timely manner.

## Medicaid Barriers

### The Problem:

ASTCT members provide critically important and potentially lifesaving therapies to patients with blood cancers and disorders. ASTCT member physicians are highly specialized clinicians that work with specifically trained clinical and administrative teams within a limited number of hospitals that have demonstrated their ability to safely administer the treatments needed by individuals with blood cancers and disorders. Due to this specialization, many parts of the country are without a local care team that can administer CAR-T, HSCT and/or HSC gene therapy and patients often have to travel significant distances – frequently across state lines – to receive the care they need. Geographic limitations are directly correlated with reduced overall survival due to

lack of access to curative cell and gene therapies.

Medicaid patients with authorization from their home states requiring travel to a specialized facility for their care face an additional barrier beyond that of other patients. While their home state Medicaid program or managed Medicaid plan has authorized the treating in an out-of-state hospital, that hospital and specialized clinicians are required to enroll with the patient's home (out-of-state) Medicare program, a process that is bureaucratic, extremely complex, time-consuming (often taking months to years) and costly to the hospital and clinicians. Some state Medicaid programs do not allow enrollment until each facility and clinician has a threshold of unpaid claims – all while having pre-authorized the services. However, these hospitals and clinicians are all enrolled with Medicare and have valid licensure and credentials.

A patient receiving cell or gene therapy for a blood cancer or disorder will interact with dozens of physicians and other treating clinicians during his or her treatment episode. Due to Medicaid enrollment rules, each individual clinician and hospital that provides care to the patient during an extended episode of care must have completed the enrollment process – a task that is close to impossible as hospitals cannot reasonably project the exact staffing during a future visit and/or what complications may arise, necessitating intervention or treatment from additional clinicians. As a result of this bureaucratic barrier, many facilities and clinicians do not accept out-of-state Medicaid patients even when the state has prior authorized the services thus creating a barrier to access.

### Proposed Solutions:

We request that Congress make a federal mandate for the federal-state Medicaid programs and managed Medicaid plans that when out-of-state treatment is authorized, then the state and plan accept active Medicare enrollment as sufficient to remit Medicaid payment for authorized services to the clinicians and facilities.

## NIH Funding

Currently, the Senate Labor-H Appropriations bill increases funding for the NIH by \$943 million over FY 2023 making the total funding \$47.8 billion. The bill outlines a target increase for cancer research at \$60 million.

***We support any increase in funding for the NIH because research is critical to development of innovative and curative therapies for oncology patients.***

