Medicaid Patient Access to Gene & Cell Therapies

ASGCT Meeting with MACPAC
March 2021
ASGCT Overview

**Professional membership organization for gene & cell therapy**
- 4500 + Members
- Majority: researchers and students
- Majority: gene therapy, including genetically-modified cell therapy
- Established 25 years ago
- Publishes the *Molecular Therapy* family of journals

**Mission**
To advance knowledge, awareness, and education leading to the discovery and clinical application of genetic and cellular therapies to alleviate human disease
The Science of Gene & Cell Therapy

• Gene therapy is the introduction, removal or change in genetic material in the cells of a patient.
  • Gene addition: DNA or RNA is transferred into the target cell using a vector, often a virus with the viral genes removed.
  • Gene editing is a type of gene therapy that removes, disrupts, or corrects faulty elements of DNA within a gene.

• Cell therapy is the transfer of living cells into a patient (e.g., blood transfusion)

• Some therapies are both gene and cell therapies (e.g., CAR T-cell therapy)

www.patienteducation.asgct.org
Unique Value and Benefits of Gene Therapy

Addresses underlying cause
- Improve quality of life by altering the disease state rather than repeatedly treating or managing symptoms
- Intended to be single administration with durable, potentially curative effect

Often for rare diseases
- Many rare diseases have great unmet medical need (no alternative treatments or inadequate treatment options)
- Many rare diseases that are rapidly progressing, severely debilitating or life-threatening
- 75% of rare diseases affect children

Long-term savings
- Average lifetime cost for hemophilia patients $21 million - $99 million, depending on type of prophylaxis treatment\(^1\)
- Cumulative lifetime healthcare fees for a patient with sickle cell disease who lives to age 50 can be as high as $9 million\(^2\)

Pipeline of Gene and Cell Therapies

Over 25 years, ASGCT members have made scientific discoveries leading to today’s current robust pipeline of potentially curative therapies to alleviate human disease

• Currently more than 1100 active and recruiting gene and cell therapy clinical trials in the US\(^1\)
• Includes those for gene therapy candidates for sickle cell disease, for which the majority of the population is covered by Medicaid\(^2,3\)
• By 2030, more than 60 gene and cell therapy FDA-approvals are expected\(^4\)

---

\(^1\) [www.asgct.org/clinicaltrials](http://www.asgct.org/clinicaltrials)


ASGCT Policy Priority

**Patient access** to safe, effective gene therapies

- Regulatory policy for efficient development
- Coverage of approved gene therapy products
- Sufficient reimbursement levels to providers
- New payment models for unique gene therapies

*These policy positions do not imply endorsement of any individual pricing decisions.*
Regulatory Policy: Accelerated Approval

Accelerated approval

- Serious or life-threatening disease or conditions only
- Products approved based on effects on a surrogate or intermediate endpoint that is “reasonably likely to predict clinical benefit” earlier than “irreversible morbidity of mortality”
- Approval considers “the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments.”
- Post-approval confirmatory studies may be required by FDA

By nature, gene therapies often provide strong mechanistic surrogate endpoints (e.g., restoring protein levels)

ASGCT positions

- Products receiving accelerated approval are FDA-approved
- The FDA framework for oversight of medical products robustly addresses safety and efficacy
- Expedited pathways facilitate timely access to products including gene therapies that may positively affect the lives of patients with serious, potentially fatal diseases
Input to MACPAC TAP Proposals

Payment and Coverage of High-Cost Specialty Drugs

• Differential rebates for accelerated approval drugs
  o Manufacturers of drugs approved under the accelerated approval pathway would pay a higher rebate to states until the manufacturer completes any FDA mandated post-marketing studies.
  o ASGCT is concerned this proposal could negatively affect patients through
    o Decreasing investments in products that can show benefit through surrogate endpoints
    o Disincentivizing the use of the accelerated approval pathway
    o Increasing the time to market for therapies
    o Exacerbating misunderstandings about the definition of accelerated approval

• New benefit for cell and gene therapies
  o ASGCT appreciates the thoughtful consideration of this challenging issue.
  o Aspects of this proposal could potentially enhance patient access, e.g., an increased federal match for reimbursement of these products.
  o We would welcome opportunities to further engage on this topic.
Upcoming ASGCT Programs

• Emerging Issues in Market Access Workshop 
  May 10, 2021, 3-7 pm ET (Virtual)

• ASGCT 24th Annual Meeting Sessions
  May 11 -14, 2021 (Virtual)
  - Payment Policies for Non-Policy Specialists: Joining the Conversation
  - Issues in Gene Therapy: Considerations for Efficient Development and Access

• ASGCT Policy Summit
  September 22 -24, 2021
  Washington, DC