Medicaid Patient Access to Gene & Cell Therapies

ASGCT Meeting with MACPAC
March 2021

American Society of Gene + Cell Therapy

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

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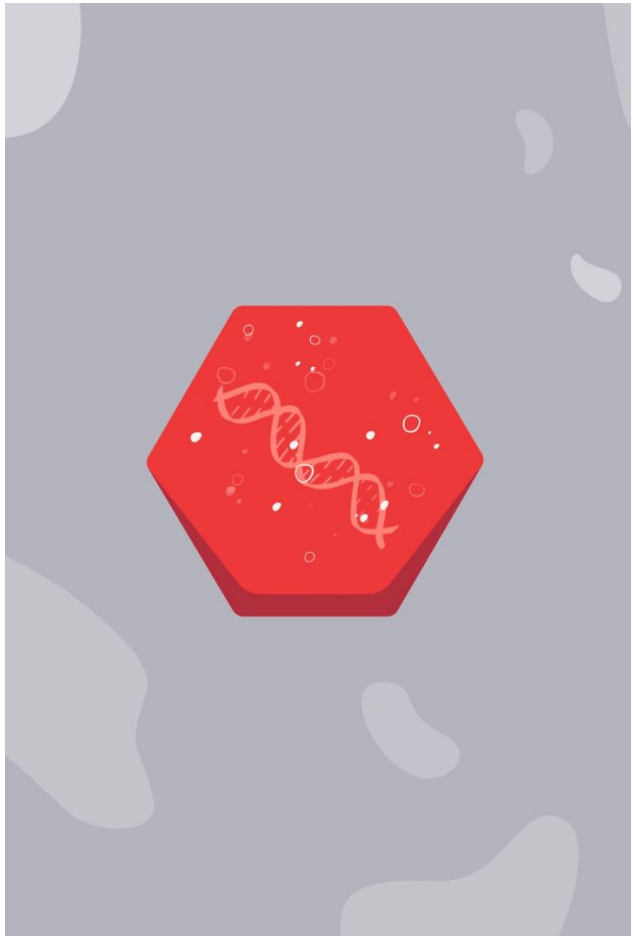
The Science of Gene & Cell Therapy

- **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

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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

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]

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

[1]Pearson, S. (2018). Can We Determine "Value-based" Prices for Gene Therapies? Institute for Clinical and Economic Review presentation, September 24, 2018, ASGCT Value Forum, Washington, DC.
[2]Ballas, S.K. (2009). The cost of health care for patients with sickle cell disease. *Am J Hematol 84*(6):320-2.

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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

[2]https://www.cdc.gov/ncbddd/hemoglobinopathies/data-reports/2018-summer/index.html

[3]Steiner, C., Miller, J. (2006). HCUP Statistical Brief, no. 21. https://www.hcup-us.ahrq.gov/reports/statbriefs/sb21.jsp

[4]Massachusetts Institute of Technology NEWDIGS FoCUS Project. (2020). Updated projection of US durable cell and gene therapies product-indication approvals based on December 2019 development pipeline.

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ASGCT Policy Priority

Patient access to safe, effective gene therapies

These policy positions do not imply endorsement of any individual pricing decisions.

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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

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Input to MACPAC TAP Proposals

Payment and Coverage of High-Cost Specialty Drugs

- Differential rebates for accelerated approval drugs
  - 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.
  - ASGCT is concerned this proposal could negatively affect patients through
    - Decreasing investments in products that can show benefit through surrogate endpoints
    - Disincentivizing the use of the accelerated approval pathway
    - Increasing the time to market for therapies
    - Exacerbating misunderstandings about the definition of accelerated approval

- New benefit for cell and gene therapies
  - ASGCT appreciates the thoughtful consideration of this challenging issue.
  - Aspects of this proposal could potentially enhance patient access, e.g., an increased federal match for reimbursement of these products.
  - 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

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