

Policy Considerations to Advance Gene Therapies for Sickle Cell Disease

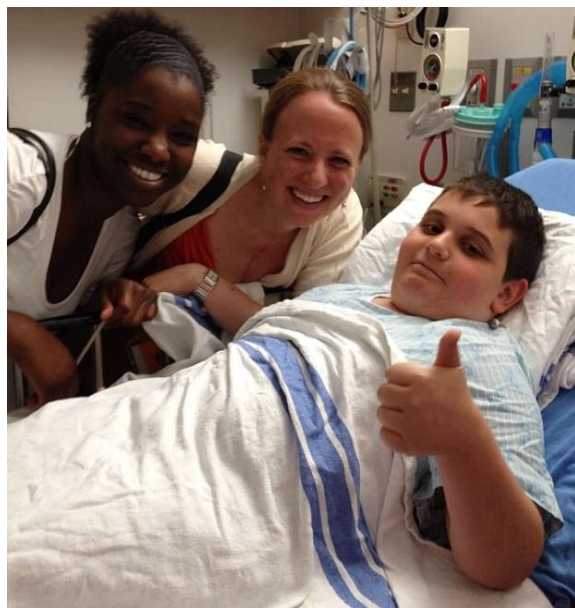
Francesca Cook, MPH

ASGCT Government Relations Committee Member
Senior Director, Pricing & Market Access, REGENXBIO

Advancing knowledge, awareness,
and education of gene and cell therapy



Gene and cell therapies are starting to transform lives with approved treatments and trials



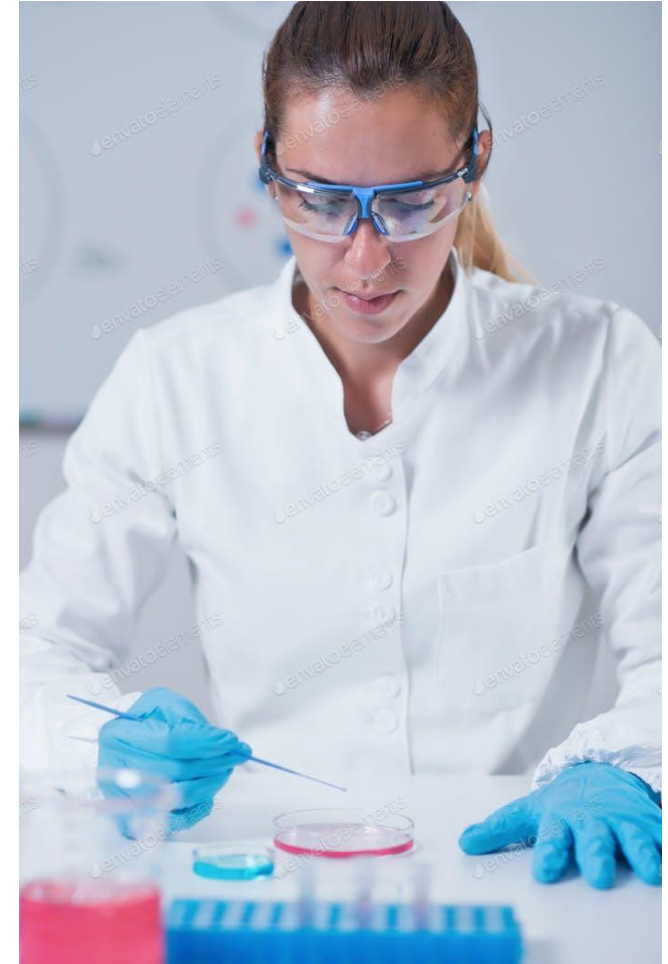
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asgct.org/clinicaltrials

Title	Phase	Modalities	Interventions	Location
Stem Cell Gene Therapy for Sickle Cell Disease	1/2	Gene Therapy, Stem Cell Therapy, Viral Vector	CD34+ cells transduced with lentiviral vector to express β AS3-FB	USA - CA - Los Angeles
A Safety and Efficacy Study Evaluating CTX001 in Subjects With Severe Sickle Cell Disease	1/2	Gene Therapy, Stem Cell Therapy	autologous CRISPR-Cas9 Modified CD34+ hSPCs	View Locations ▾
A Study to Assess the Safety, Tolerability, and Efficacy of BIVV003 for Autologous Hematopoietic Stem Cell Transplantation in Patients With Severe Sickle Cell Disease	1/2	Gene Therapy, Immunosuppressive Agents, Non-Viral Vector, Stem Cell Therapy	CD34+ progenitor cells transfected with ZFN mRNA, plerixafor, busulfan	View Locations ▾
Gene Transfer for Patients With Sickle Cell Disease	1/2	Gene Therapy, Viral Vector	lentivirus vector containing fetal hemoglobin gene	USA - OH - Cincinnati
A Study Evaluating the Safety and Efficacy of the LentiGlobin BB305 Drug Product in Severe Sickle Cell Disease	1/2	Gene Therapy, Immunomodulatory Agent, Stem Cell Therapy, Viral Vector	cells with lentiviral vector encoding human betaA-T87Q-globin, plerixafor	View Locations ▾
Gene Transfer for Sickle Cell Disease	1	Gene Therapy, Stem Cell Therapy, Viral Vector	CD34+ stem cells transduced with lentiviral vector targeting BCL11a	USA - MA - Boston

Support Robust NIH Research Funding

- Funds basic research that underpins gene therapies and clinical trials
- NHLBI is currently funding the Cure Sickle Cell Initiative, which aims to accelerate the development of genetic therapies to cure SCD within the next 5 – 10 years
- ASGCT has requested the Senate -
 - Appropriate at least a \$2 billion increase in NIH funding for FY2020, as proposed by the House
 - Appropriate the \$8 million authorized by 21st Century Cures for FY2020 for the Regenerative Medicine Innovation Project



Reauthorize Newborn Screening



- The Newborn Screening Saves Lives Act is set to expire in September 2019
- Authorizes funding for states' screening programs and educational efforts
- All 50 states screen for sickle cell disease
- H.R. 2507 was introduced in the House by Reps. Roybal-Allard and Simpson
- Support reauthorization efforts led by the Energy and Commerce and HELP Committees

Thinking Ahead: Approvals of Gene Therapy for Sickle Cell Disease

To maximize patient access to approved gene and genetically-modified cell therapies ASGCT:

- Supports coverage and maximal reimbursement levels for approved therapies
- Does not endorse any specific gene therapy pricing decisions



Why Access is Crucial: Unique Value of Gene Therapy for SCD

- **High efficacy, single administration**
- **Reduces or eliminates need for other costly treatments**



The total lifetime costs of treating sickle cell disease through current standards of care is extremely high

“If this gene therapy works, I won’t have to take off work every month for blood transfusions or deal with the daily pain. It would improve my life in ways that are hard to even imagine right now.”

-Woman enrolled in a gene therapy trial for SCD

Coverage & Reimbursement Issues for Approved Gene Therapies



- Upfront costs for single administration will be challenging for payers, especially Medicaid programs and smaller commercial payers
- Recent Medicare actions for approved cell therapies raise concerns about system readiness (insufficient Inpatient Prospective Payment System reimbursement levels to providers)
- State Medicaid reimbursement practices vary, with some states reimbursing providers primarily for services, in a bundled payment, which is often insufficient to cover both services and the therapeutic product

Coverage & Reimbursement Solutions to Prepare for the Approval of SCD Therapies

- Enable novel payment models (outcomes-based payment and payment over time) by exempting such arrangements for gene and cell therapies from barriers—Medicaid best price requirements, Stark Law, and Anti-Kickback Statute
- For Medicare in the inpatient setting: Reform NTAP levels and formulas, and collect accurate cost and payment data from providers of cell and gene therapies.





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