Study Synopsis

Design

The Centers for Medicare & Medicaid Services (CMS) Sickle Cell Disease (SCD) Cell and Gene Therapy (CGT) Access Model study will use long term follow-up data of Medicaid-eligible patients who receive, or plan to receive, gene therapy for Sickle Cell Disease. CIBMTR will collect a standard set of clinical data and Patient-Reported Outcomes (PRO) data, establish a study master Model ID to support matching between CMS data sources and deliver clinical data and PRO data to CMS. A provider who submits a claim for administration of a State-Selected Model Drug must be a member of the CMS-designated patient registry (e.g. CIBMTR) for the Model and seek patient consent for a CMS-specified study such as this one. More information about the CMS CGT Access Model can be found here: Cell and Gene Therapy (CGT) Access Model | CMS

CIBMTR will collect clinical data and PRO data using standard CIBMTR processes. SCD patients eligible to participate will be recruited to the study at the time of a request for product manufacture. Recruitment flyers will be available at centers to share with eligible patients that provide information about why patients are being asked to participate, the type of clinical data and PRO data to be collected, what types of questions to expect to be asked on PRO surveys, and how they will be compensated. Patients who agree to participate will sign the standard CIBMTR Research Database consent forms and centers will register the patient in FormsNet, CIBMTR's clinical data collection systems. Centers will be asked complete a Study Eligibility form to enroll the patient into the study. There will be a QR code on the flyer that leads to a PRO page on the CIBMTR website with more information about CIBMTR PRO data collection: https://cibmtr.org/CIBMTR/Data-Operations/Protocols-Consent-Forms/PRO-Data-Collection-Protocol

Participants

Participants are defined by type and role and include:

- US states participating and not participating in the CMS CGT Medicaid Access Model.
    - Patients receiving treatment in non-participating states will be used as a comparator group to patients receiving treatment in a participating state.
- Centers certified to perform SCD gene therapies.
- The location of the center is not tied to state participation per se. Participating states will likely approve treatment at in-state centers, but a state must allow out-of-state treatment when needed. Some states might not have an in-state treatment center.
- Patients meeting the following inclusion and exclusion criteria:
    - **Inclusion**
        - Gene therapy recipients, age 12 and older at the time of infusion
        - Medicaid and Medicaid-expansion Children's Health Insurance Program (CHIP) Beneficiaries is the primary payor for the gene therapy

- Gene therapy recipients who consent to participation in the CIBMTR Research Database Protocol
- PRO specific eligibility for this study includes patient age at year 12, or any parent/guardian who has a child aged 12 or older, and who has received or will receive a gene therapy infusion, who speaks and reads in English or Spanish, and has consented to the CIBMTR Research Database Protocol and has agreed to be contracted by the CIBMTR for PRO research.

- **Exclusion**
  - Gene therapy recipients for whom Medicaid or Medicaid-expansion CHIP is not the primary payor for the gene therapy.
  - Patients who receive gene therapy treatment paid for by State Medicaid agencies that do not participate in the CGT Access Model may be compared to patients who receive gene therapy treatment paid for by a participating State Medicaid agency

Recruitment

CIBMTR will collaborate with CMS and participating centers to identify patients where a center has requested and/or received Medicaid pre-authorization for gene therapy or at the time of request to manufacture the product. CIBMTR data collection infrastructure allows centers to initiate CIBMTR database registration and consent at the time of their request for cell manufacturing or at the time of pre-authorization approval. Patients will be enrolled by centers into the study through the CIBMTR Form 2554: CMS Study Eligibility Form.

Data Collection

Clinical Data Forms and PRO Measures and data collection timepoints for each are provided below.

CIBMTR Clinical Data collection forms and timepoints

Number	Name	Pre-TX	Baseline/Day 0	Day 100	Day 180	Annually
2804	CIBMTR Research ID Assignment	•				
2814	Indication (2 forms)	•				
2554	CMS Registration	•				
2820	Recipient Contact Info	•				
2400	Pre-TX Essential Data		•			
2402	Disease Classification	•				
2000	Baseline Data, CRF		•			
2003	Gene Therapy Product		•			
2030	SCD Pre-TX	•				
2100	Post-TX Follow-Up, CRF			•	•	•
2103	Gene Therapy Persistence		•	•	•	•
2130	SCD Post-TX			•	•	•

- Abbreviations: CRF, Comprehensive Report Form; SCD, sickle cell disease, TX, treatment.

PRO measures and data collection timepoints

Measures	Pre-TX	Day 30	Day 100	Day 180	Annually
PROMIS domains					
Anxiety domain	•	•	•	•	•
Depression domain	•	•	•	•	•
Pain interference	•	•	•	•	•
Fatigue	•	•	•	•	•
Cognitive function	•	•	•	•	•
Ability to participate in social roles and activities	•	•	•	•	•
Global Health	•	•	•	•	•
ASCQ-ME					
Pain episodes	•	•	•	•	•
COST-FACIT version 2	•			•	•
Socio-demographics	•				•

Reimbursement

Center incentive - The budget includes an incentive payment of $1,000 per center for a portion of the centers anticipated to participate in the study.

Clinical forms reimbursement - The budget includes support for Forms Reimbursement. NMDP will administer the reimbursement program. Comprehensive Report Forms and CTED forms are reimbursed according to the following schedule below. TED forms, which capture federally mandated data for the SCTOD, are not reimbursed.

No.	Description	Payment
2000 2003	Recipient baseline form, plus disease-specific inserts Gene therapy product form	$135 (all forms required)
2100	100 days post-TX data, plus any required disease-specific inserts	$110
2100	6 months to 2 years post-TX data, plus any required disease-specific inserts	$110
2100	Yearly follow-up for greater than 2 years post-TX data, plus any required disease-specific inserts	$110
2900	Recipient death data	$15

PRO reimbursement

Patients or their caregivers (one or the other, depending on who is completing the survey) of both the study and comparisons groups will receive a $25 gift card for a completed baseline PRO survey and a $10 gift card for each subsequently completed survey through the duration of the funding (February 2028).