

FIRST IN A SERIES

## Emerging market solutions for financing and reimbursement of gene therapies for sickle cell disease: Why do payment innovation?

In a landmark decision, the FDA approved two cell and gene therapies (CGTs) to treat sickle cell disease (SCD) on December 8, 2023<sup>1</sup>. These approvals mark not only hope for SCD patients for transformed quality of life but also represent CGTs for the largest population condition to date. At list prices over \$2 million, healthcare payers seek new solutions to finance and reimburse CGTs like those for SCD.

### RESEARCH BRIEF

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CGTs were initially launched for ultra-rare conditions with less than 1,000 patients. SCD impacts approximately 100,000 Americans<sup>2</sup>, creating unique challenges for the healthcare payer system.

Payment innovation is needed for CGTs due to performance uncertainty, actuarial risk, and payment timing/budget impact. SCD treatments have all these factors but with some nuances that create urgency for creating solutions that work for all stakeholders.

### WHY DO WE NEED PAYMENT INNOVATION FOR SCD?

For five reasons:

- Disease complexity and treatment costs
- Prevalence
- Budget impact
- Geographic and payer density
- Equity and health disparities

SCD is an inherited blood disorder, most common in African American populations, with an incidence rate of 1 in every 365 African-American births<sup>3</sup>. This severe, painful, and progressively debilitating genetic condition alters the structure and function of red blood cells, leading to organ damage and shortened lifespan<sup>4</sup>. Pain is the hallmark feature of SCD, developing from vaso-occlusive events (VOEs) or vaso-occlusive crisis (VOC), where sickled red blood cells block blood flow, resulting in severe pain as affected tissues start to die<sup>5</sup>. Repeated VOEs, VOCs, chronic hemolytic anemia, and persistent vasculopathy can lead to progressive damage to organs such as the brain, eyes, heart, lungs, liver, and kidneys. These chronic complications are the primary causes of morbidity and premature

### Key takeaways

Sickle cell disease (SCD) has the potential to be the largest population durable cell and gene therapy (CGT) to date

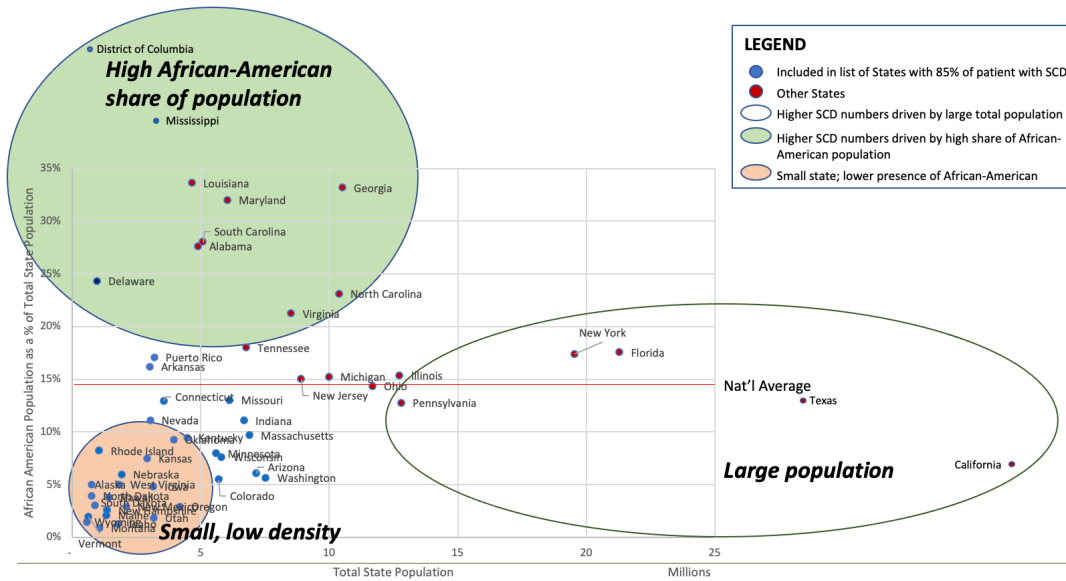
Impending CGTs for SCD create a sense of urgency for innovative payment designs

Geographic concentration and payer mix unique to SCD will disproportionately strain state Medicaid systems.

mortality in adults with SCD, with an average life expectancy of 52.6 years.<sup>6</sup>

Higher disease prevalence and SCD treatment costs of \$2-3 million per patient create concerns about the budget impact and the timing of payments. One budget impact analysis for SCD on the Medicaid program estimated mean first-year costs of \$29.96 million per state Medicaid program for states with the highest prevalence of SCD. Additionally, the cost of care involved in administering a gene therapy such as those in the near-term pipeline is complex (i.e., requiring an inpatient treatment at a Center of Excellence, and multiple stays), and one where multiple other therapy options either compete or may be used in conjunction with the cell therapy. This combination is more analogous to CAR-T's challenges than those facing CGT therapies for monogenic, often pediatric, and much smaller orphan or ultra-orphan populations.

SCD is also costly. Average nonelderly lifetime treatment costs for SCD are estimated at \$1.7 million for those with commercial insurance<sup>7</sup>. Although lifetime cost estimates for those with



**Figure 1.**  
**States Separate into 3 Prevalence Archetypes**

Source: <https://data.census.gov/cedsci/table?q=race&d=ACS%201-Year%20Estimates%20Detailed%20Tables&tid=ACSDT1Y2018.B02003&vintage=2018>, NEWDIGS analysis

public insurance are not available, other studies have found that those with Medicaid have more VOCs and other acute complications, likely resulting in higher costs.<sup>8</sup>

Adding additional strain to the system is that SCD has a concentrated population distribution by payer segment and geography. Medicaid covers a high proportion of affected patients, and 85% of patients with SCD are in 17 states.<sup>a</sup> Even within these 17 states, the prevalence may be experienced differently, with some states seeing a higher share. In contrast, others see a larger number but a similar population fraction to other states (Figure 1). At least initially, SCD patients are expected to have a payer mix skewed to public payers, as therapies target the more severe adult patients experiencing in-patient hospital visits from VOCs. These patients are expected to skew more towards Medicaid and Medicare (disability) than Commercial insurance coverage: roughly 50% Medicaid and 25% Medicare.<sup>9</sup>

Health disparities and limited resources among individuals with SCD, particularly in low-income and rural communities, create unique challenges that can lead to limited access to specialized treatment at SCD centers and worse outcomes. Less than 70% of US doctors accept new Medicaid patients, further exacerbating the challenges.<sup>10</sup> Without finding ways to pay for SCD CGTs, equity only lessens.

### MORE SLOW TIDE THAN TSUNAMI

Despite these challenges, the uptake of CGTs for SCD is more likely to be a slow tide and not the tsunami feared. There is considerable uncertainty around treatment efficacy. Efficacy in clinical trials has been measured as a reduction in VOEs and VOCs by adding more good hemoglobin to SCD patients. Current CGTs do not repair existing damage; they only reduce or prevent additional complications. Additionally, not all SCD patients meet

the criteria for CGTs; both approved therapies were approved for patients 12 and older and with a history of severe VOCs or VOEs. Reticence to use new therapies is also anticipated by African American and Hispanic SCD patients, given the historical racism and inequities faced by them.<sup>11</sup> Access to treatment Centers of Excellence, travel, and other financial constraints, combined with the arduous nature of the CGT treatment itself, will also reduce demand.<sup>12</sup>

However, given the promise of CGTs to help people living with SCD and the rich pipeline of CGTs to treat SCD and more prevalent conditions, the need for innovative payment approaches that are practical and accepted by the market has never been greater.

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<sup>a</sup>Alabama, California, Delaware, District of Columbia, Georgia, Illinois, Louisiana, Maryland, Michigan, Mississippi, New York, North Carolina, Pennsylvania, South Carolina, Tennessee, Texas, Virginia

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## ABOUT NEWDIGS FOCUS

The FoCUS project was launched in 2016 in response to the need for new financing and reimbursement approaches that facilitate access to new potentially curative therapies within a value-based framework. The program vision is to collaboratively address the need for new, innovative financing and reimbursement models for durable therapies in the US that ensure patient access and sustainability for all stakeholders by designing and piloting new value-based financing and reimbursement models for curative therapies, leading to system-wide, implementable precision financing.

The New Drug Development Paradigms Initiative (NEWDIGS) is an international “think and do tank” dedicated to delivering more value faster to patients in ways that work for all stakeholders. NEWDIGS designs, evaluates, and initiates advancements that are too complex and cross-cutting to be addressed by a single organization or market sector. Its members include global leaders from patient advocacy, payer organizations, biopharmaceutical companies, regulatory agencies, clinical care, academic research, and investment firms. For more information, <https://newdigs.tuftsmedicalcenter.org/>.