CATALYST IMPLEMENTATION BRIEF

Understanding access issues for new therapies

This brief outlines several major barriers to patient access for novel cell and gene therapies. It also describes how providers, payers, and developers may overcome these barriers, with a focus on addressing these challenges for the new sickle cell disease (SCD) therapies, Casgevy and Lyfgenia. For more details on these access barriers and potential solutions to address these barriers, see the NEWDIGS FoCUS Paying for Cures Toolkit and this white paper.

Category	Barriers to access	Overcoming barriers
How patients become eligible for treatment	• For some diseases, there may be limited access to medical specialists and testing that lead patients to diagnosis and make them eligible for treatment. • Patients must be educated about new therapies and pathways to receive those therapies as they become available.	• For Casgevy and Lyfgenia, eligibility is determined by patients' vaso-occlusive events. Eligibility for other diseases, particularly rare diseases, may be more complex, due to challenges like limited availability of specialized testing. • SCD medical specialists and advocacy organizations can educate patients about new therapies. Advocacy organizations may also share payers' coverage policies and help patients to prepare for the treatment journey, empowering patients to advocate for themselves throughout the process.

Where patients are treated	• Due to the complexity and specialization of cell and gene therapies, patients require treatment at a certified Center of Excellence.
	• Patients may need to seek out new providers or travel—perhaps even across state borders—to access treatment, and may require financial support for their travel.
		• Developers' standards for SCD gene therapy treatment require an organization to have an accredited bone marrow transplant center on site, provide sickle cell care, and operate an apheresis treatment center. As of March 2024, there are 12 authorized treatment centers for Casgevey and 27 for Lyfgenia.
		• Options for covering out-of-state providers:
		  • For commercial payers, a single case agreement;
		  • For Medicaid FFS and managed care, methodologies may include DRGs; Per diems; Carve-outs; Episodes of care;
		  • For Medicare, reimbursement is set nationally by CMS.

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Treatment duration and follow-up	• A first payer may approve treatment, but the patient may change mid-treatment to a payer that does not cover the therapy. Patient mobility also complicates longer term outcomes monitoring, impacting innovative contract opportunities. • Patients and caregivers require medical leave, which, based on their employer, may not span the full duration of treatment. • Safety and outcomes data are integral to performance-based agreements as well as FDA compliance.	• Treatment with either of the gene therapies for sickle cell disease is a long and complex process that may take up to a year or more before administration, followed by years of follow up; payers, developers, providers, and COEs should all coordinate care. Vertex Conects and mybluebird support offer patient support for Casgevy and Lyfgenia, respectively. • The Family and Medical Leave Act protects patients and caregivers at large companies by ensuring 12 weeks of leave per year. Short and long-term disability policies offer another option. • Stakeholders must identify key outcomes and track patients over time. Bluebird bio offers a 3-year performance agreement for Lygenia, using claims-based outcomes. Vertex plans to offer an outcomes-based contract for Casgevy but has not yet publicly shared details about the agreement.
How providers get paid for treatment	• No ASP value is reported in the first three months of sales, as CMS uses those early data to set the value. • Existing inpatient DRGs will not include the cost of CGTs. This may be partially addressed by Medicare New Technology Add-on Payments, but these payments often significantly lag the therapy launch, are not approved, and only provide partial coverage for specific time periods.	• AMP for Casgevy and Lyfgenia will initially approximate list price less the Medicaid standard rebate of 23%. The inclusion of VBC discounts in the calculation of ASP will become a factor only after a performance failure results in a commercial rebate. • Medicaid FFS and Medicaid managed care may include the following reimbursement methodologies: MS-DRG, APR-DRG, Per diems, Carve-outs, Episodes of care. • A Medicare FFS inpatient stay is reimbursed using an MS-DRG and may be eligible to receive NTAP and an outlier payment, if applicable. NTAP application request for approval has been submitted for both Casgevy and Lyfogenia and if approved, would be effective Jan. 1, 2025.
How payers afford to pay for treatment	• Payers are concerned by the larger potential candidate population for SCD gene therapy. At least initially, SCD patients are expected to have a payer mix skewed to public payers, as therapies target the more severely impacted adult patient.	• Actuarial and performance risks and payment timing are critical components in assessing payment solutions for SCD therapies. • Various financial and value based models are needed to meet the varied needs of providers. • Stop-loss and reinsurance term modifications are needed with CGTs. Carrier-specific innovations may exist to help address SCD gene therapy coverage. • CMS's CGT Access Model will test voluntary multi-state outcomes-based agreements between manufacturers and state Medicaid programs with the goal of increasing access to CGTs beginning in 2025. The initial model will address SCD.