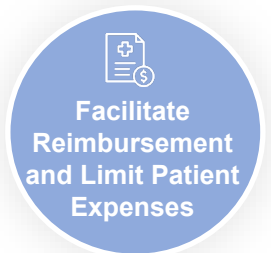


Real Life™

CIRM Access and Affordability Working Group Roadmap
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CIRM
CALIFORNIA'S STEM CELL AGENCY

Roadmap to Access & Affordability Strategies - Examples



Patient Support Services	Engagement with Payers	Creation and/or endorsement of new State policy for C&GT	Alpha Clinics and CCCE
Alpha Site Coordination and Alignment	Performance Based Models	Treatment across state lines	Patient and post-marketing Registries, RWE, HEOR
Patient Navigator at CCCEs	Introduce New Payer Mechanisms for Orphan Products	Inpatient vs. Outpatient reimbursement	Accelerate Advocacy at community level; Medical Education
Coverage Analysis, Insurance Support, Co-Pay Assistance, Access and Appeals	Risk Pools/Coalition Partners	Align to Gov Plan on Office of Health and Affordability	State and Federal authorized treatment centers

Last year there were ~500 unique cell and gene products in Phase 1, 2, and 3 trials

- FDA will decide on 13 more CGTs in 2023 and three will be decided in Europe.¹
- An additional 50 to 75 CGTs are expected to be approved in the U.S. by 2030.²

Nevertheless, manufacturers developing CGTs face commercialization challenges vs. traditional biopharmaceutical products. Factors affecting scalability of CGTs and the patient and healthcare professional experiences include:

- Supply chain and manufacturing challenges
- Healthcare infrastructure barriers
- Treatment delivery problems
- Patient economic burdens

1. Regenerative Medicine: The Pipeline Momentum Builds. Alliance for Regenerative Medicine. September 2022. <https://alliancerm.org/sector-report/h1-2022-report/>

2. Cell and Gene Therapies: Looking Ahead to 2022. Milken Institute. <https://milkeninstitute.org/report/cell-gene-therapies-2022>

A survey of U.S. CAR-T treatment centers asked if inadequate CAR-T reimbursement is resulting in fewer patients having access to these therapies.

Almost 90% of 22 surveyed treatment centers report earning a profit or at least breaking even on CAR-T administration. Additionally, 75% of these centers are profiting or breaking even on inpatient Medicare patients.

On average, ~15-20% of cases are outpatient and many centers are considering or developing infrastructure to enable future outpatient CAR-T administration; however, outpatient CAR-T administration does not always improve financial outcomes.

Treatment centers value the support of manufacturers and request future assistance, such as robust patient assistance programs to help cover lodging, transportation and other necessary expenses.

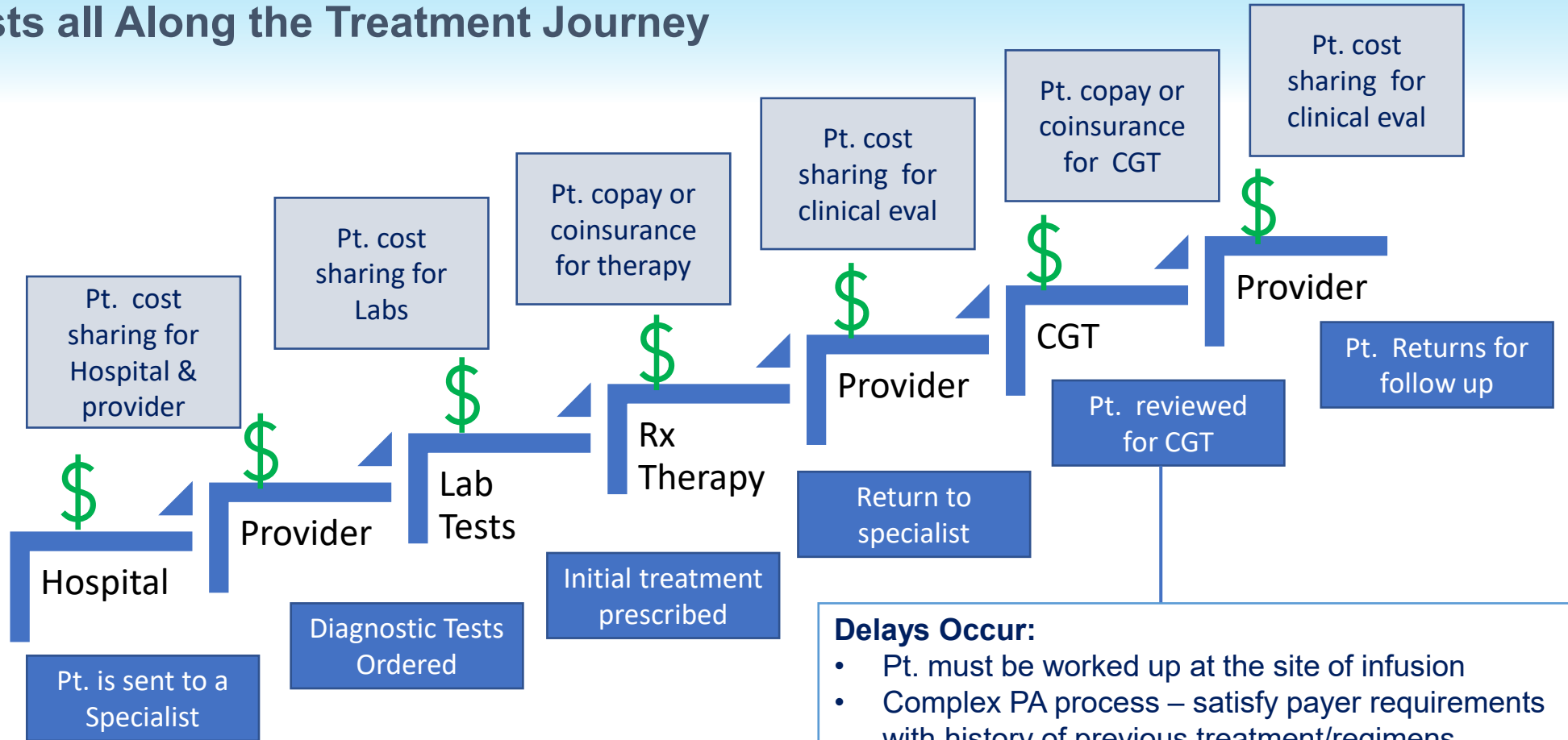
Improving effective access and reimbursement support could accelerate expansion of treatment centers beyond the current top tier centers.

McDonald M, Bourjolly B, Shah S. CAR-T reimbursement in the US: ZS separates myths from reality. Oct. 25, 2022. Available: <https://www.zs.com/insights/car-t-reimbursement-in-the-us-zs-separates-myth-from-reality>.

A Complex Care Experience of Patients/Caregivers Incurring Costs all Along the Treatment Journey

Additional OOP Costs

- Travel
- Hotel
- Meals
- Childcare
- Loss of work & income due to post operative support



Pt. presents with symptoms

Hospital

Provider

Lab Tests

Rx Therapy

Provider

CGT

Provider

Pt. reviewed for CGT

Return to specialist

Initial treatment prescribed

Diagnostic Tests Ordered

Pt. is sent to a Specialist

Payers are open to newer models of reimbursement for cell and gene therapy¹

- **Traditional models:** Most payers currently use traditional techniques, such as formulary or utilization management tools (step therapy, prior authorization), to manage cell and gene therapies.
- **Future state:** The majority of payers plan to leverage reinsurance as well as increase their use of alternative payment mechanisms or value-/outcomes-based contracting
 - Annuity-based payment
 - Outcomes-based payment
 - Outcomes-based rebate
 - Outcomes-based annuity

Theoretical Payer Options with States

- Government-managed pooling mechanism funded by carrier assessments
- Governments directly involved in ensuring specific risks
- Governments and private industry stakeholders (employers, manufacturers, payers) work together toward solutions
- Governments provide funds that private insurers pay back over time

CAR-T Therapies: Shifting From In-Patient to Out-Patient

Out-patient administration is the future of CAR-T treatment, but may remain a long-term objective due to competing near-term priorities and significant investments required

To Minimize Costs & Enable Scale:¹

- Many are considering or developing infrastructure to further enable outpatient CAR-T administration to increase capacity and offset inpatient losses with outpatient profits
- Patient populations will expand as more CRTs with improved toxicity profiles are introduced
- Outpatient treatment may become the mainstay for CAR-T cell practices, requiring programs to build robust infrastructures and workflows supporting outpatient management of CAR T cell patients, with admission occurring only when clinically necessary

1. CAR-T reimbursement in the US: ZS separates myths from reality, Oct. 25, 2022. Available: <https://www.zs.com/insights/car-t-reimbursement-in-the-us-zs-separates-myth-from-reality>

Thank you