CIRM Access Plan Requirements

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CIRM
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Agenda

1. Background
   i. CIRM Access Plan Requirements
   ii. Access Plans implementation within CIRM Programs

2. Access Plan evaluation of CIRM funded Commercial therapies
   i. Blue Ridge Patient Assistance Programs (PAP) Benchmarking Findings
   ii. Gaps, Questions, and Policy Considerations

3. Generalized Timeline for Access Plan Development

CIRM Access Plan Requirements

Access Plan Requirement

- A Commercializing Entity selling a Drug must submit a plan to CIRM to afford access to Californians "with no other means" to purchase the Drug.
- Drugs developed with CIRM funds must be sold in California with public funds at the benchmark price in the California Discount Prescription Drug Program (or successor program).
- "no other means" = Those without prescription drug benefits and with family incomes < 300% of the federal poverty level.

Timing

- The Commercializing Entity must submit their Access Plan to CIRM **within 10 business days** after FDA approval (extensions possible).
- Waiver option available through ICOC.

Access Plan must:

- Be consistent with Industry-standards at the time of commercialization.
- Account for:
  - drug market size and
  - company resources.
- Burden on company to establish the plan satisfies these requirements.

CIRM Access Plan Review Process

Plan Approval by CIRM

- Plan is subject to approval by CIRM after a “public hearing” that “provides for receipt of public comment.”

- **Public Hearing**: Includes public comments (written, oral).

- **Timeline**:
  - Non-confidential portions posted online.
  - 7-business-day public comment period.
  - Decision rendered **within 5 business days** after comments close.
  - CIRM approval shall not be unreasonably withheld and Access Plans shall not exceed industry standards at the time of commercialization.

- **Submit to CIRM within 10 business days of FDA approval**
  - Extension possible up to 30 days

CIRM Access Plan Review Process

- **Access Plan must:**
    - Align with industry standards
    - Reflect entity's resources
    - Be approved by CIRM after public hearing

Waiver Petitions to ICOC

- **Timing**: Petitions must be submitted within 10 days of FDA approval (extensions possible).
- **Process**: The ICOC may waive the Access Plan requirement if, after a public hearing, the ICOC determines that in the absence of a waiver the development and delivery of the Drug will be unreasonably hindered, or that the waiver will provide significant benefits that equal or exceed the benefits that would otherwise flow to the state pursuant to the Plan.

CIRM Access Plan Review Process

Confidential Treatment of Access Plan

- Propositions 71 and 14 protect from disclosure any documents containing or reflecting confidential intellectual property or work product.
- To the extent a company feels certain information or components of the Plan constitute confidential intellectual property or work product, the company should identify that information as such upon submission of the Plan and explain why it should be protected from disclosure under applicable law.
- CIRM will review the identified information and evaluate whether it meets the definition of protected records as provided in Propositions 71 and 14 and the Public Records Act and ensure confidential material is not shared publicly.
- Non-compliant requests for confidentiality can be withdrawn and resubmitted.

ICOC may review proprietary materials in closed session

- Requirements do not preempt stricter state/federal obligations

Complementary Function with Programmatic Efforts

Programs (AAWG approval Apr 2025)

- Developed and implemented Affordability and Accessibility Checklist in PDEV and CLIN2 programs/review
  - Builds discipline in access planning via checklists, rubrics, milestones
  - Creates transparent and consistent A&A expectations across programs
  - Prepares awardees for access plan obligations before commercialization

Legal (AAWG Sept 2025)

- Provide background for CIRM review of Awardee Access Plans
  - Defines real-world models of PAPs in cell & gene therapies
  - Identifies logistical, financial, and compliance supports components
  - Benchmarking will guide CIRM review Access Plans in conjunction with Proposition 14 standards

CIRM is building an ecosystem that ensures awardees deliver Access Plans that are (1) compliant with statutory requirements, (2) aligned with industry PAP benchmarks, and (3) sustainable, equitable, and enforceable.

GENERALIZED SUMMARY OF THE CONTENTS OF CGTX PATIENT ASSISTANCE PROGRAMS (PAPS)

BLUE RIDGE LIFE SCIENCES

Eight Key Attributes of an Access Plan

- Program Eligibility Criteria
- Drug access support & Care Navigation
- Financial assistance (drug cost, copay, caregiver support)
- Logistical Support and Coordination (cold chain, travel, lodging, childcare)
- Ancillary HCP wrap-around services (education/training, adherence, safety monitoring)
- Program Accessibility and Efficiency
- Equity-orientated program elements
- Post-treatment Support

Program Eligibility Criteria

- Medical Necessity
  - Because of high-costs associated with CGTx, there may be additional verification steps for Prior Authorizations
- Prescription Requirements
  - Documented diagnosis by doctor
- Residency/Citizenship
  - Most require that patients be U.S. citizen or legal resident
  - Some only require US residency
- Insurance Status
  - Many PAPs exclude Medicare, Medicaid, VA, TRICARE patients
- Financial Eligibility
  - Income level: Usually a percentage of the Federal Poverty Level (FPL), often below 200–600% of the FPL

Access Support & Care Navigation

- CGTx PAPs include dedicated support from access professionals to assist patients in navigating the complex payer landscape
  - Assist with insurance verification, prior authorizations, denials, and appeals
  - Use tools help providers assess patient eligibility to obtain free drug or co-payment assistance
  - Support extends beyond treatment (adherence, milestone monitoring)

- Because CGTx's are limited to authorized centers, dedicated support programs help patients find and schedule care at these sites.

Financial & Logistical Support

Financial assistance programs often cover drug costs and may also support other direct medical or indirect out-of-pocket (OOP) expenses for patients with demonstrated need.

Financial Support

- Direct costs: drug coverage, administration fees
- Co-Pay Assistance
- Medical service and Facility Fees

Logistical Support

- Transportation and Lodging
- Caregiver and Family Support
- Referrals

HCP and Post-treatment Support

HCP Support

- Provider Training
- Development of support tools and other resources

Post- Treatment Support

- CGTx PAPs provide ongoing support through check-ins, adherence tracking, and milestone monitoring to ensure continuous, compliant care

Program Accessibility and Equity-Oriented Elements

Program Accessibility

- Provider awareness low: ~50% of patients unaware of PAPs
- Time to first contact
- Turnaround time for support initiation
- Medium of contact (phone, email, online portal)

Equity-Oriented Elements

- Availability of multilingual support
- Access for remote populations

Outsourcing to Third-Party Service Providers

- ≈ 88% of manufacturers outsource at least some PAP services
- Hybrid models dominate (~80%): blend internal and external services
- Outsourcing addresses compliance/resource gaps, esp. for smaller biotechs
- Commonly outsourced: reimbursement, financial assistance, travel/logistics
- At the end of the Appendix is a list of Hub Service Providers which Awardees can consider

Gaps & Questions

- Administration models: in-house vs hybrid vs fully outsourced
- Eligibility transparency: which supports apply?
- Scope: behavioral health, fertility, non-IV therapies coverage?
- How to address knowledge gap with patients?
- Evolution challenge: balancing comprehensive support with cost/scalability

Next Steps

1. Delineate review process and resources
    i. Develop tool & checklist for scoring submitted Access Plans
    ii. Identify and onboard consultant experts for review
2. Delineate engagement and communication plan for awardees close to BLA

Appendix

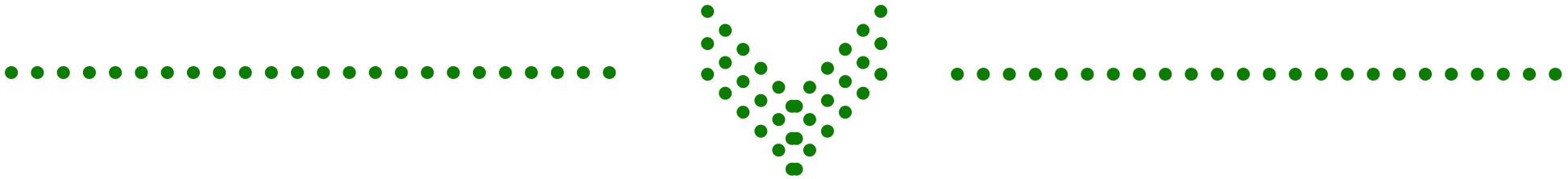
CGTx Patient Assistance Programs (PAPs) Research prepared by Blue Ridge Life Sciences

BlueRidge
LIFE SCIENCES

CGTx Patient Assistance Programs (PAPs)

Objective

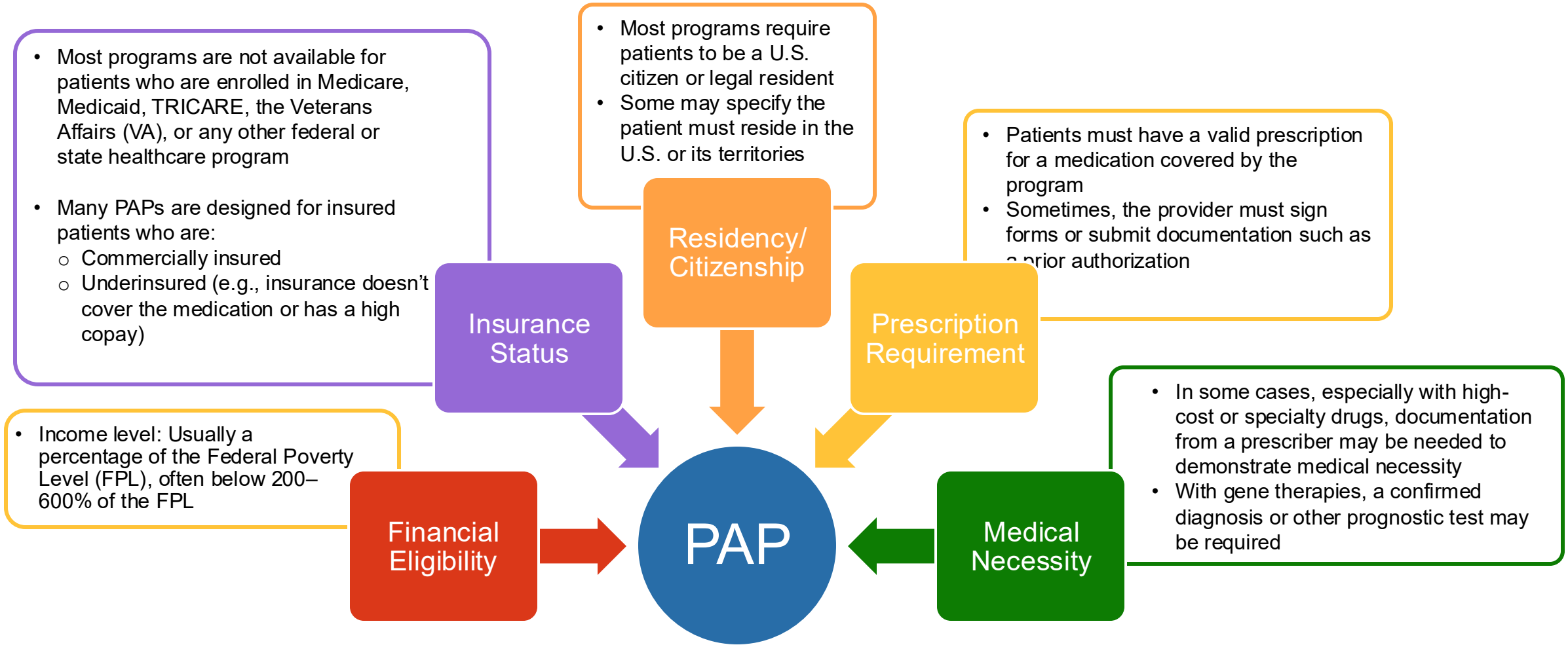
Provide industry benchmarks for Patient Assistance Plans (PAPs) for cell and gene therapies



- Detail industry standards for access and patient assistance plans for marketed cell and gene therapies
  - Conduct secondary research into current patient assistance programs
  - Conduct primary research to validate and inform patient assistance programs
- CIRM may use the benchmarks/standards to review access plans and make recommendations regarding funding of potential grantees

Secondary Research into Current PAPs

In general, across all therapy types, PAPs share several eligibility criteria for patients requesting financial support for treatment



Source: 1) Home | Pfizer RxPathways ; 2) JanssenCarePath, 3) What is Lilly Cares | Lilly Cares, 4) Poverty Guidelines | ASPE, 5) Krystal Connect™ - Personalized Patient Support Program, 6) Home | AutolusAssist™

8 key attributes of PAPs from diagnosis to post-treatment were identified from the review of included CGTx

- We searched materials available on the PAP websites and other grey literature of 14 selected FDA-approved cell and gene therapies of distinct drug manufacturers in April 2025 to describe their characteristics across the following 8 domains:

1. **Program eligibility criteria**
   - Including patient financial eligibility thresholds, and access for underinsured or uninsured populations
2. **Drug access support & Care Navigation**
   - Insurance navigation benefits verification, prior authorization assistance, and appeals support
3. **Financial assistance**
   - Free drug programs
   - Copay assistance, including caps (if any)
   - Travel and lodging for patients and caregiver(s)
4. **Logistical support**
   - Treatment center locating and referrals
   - Scheduling support
   - Travel and lodging coordination
5. **HCP Support**
   - Provider training and resources
   - Logistical support
6. **Program accessibility and efficiency**
   - Time to first contact
   - Turnaround time for support initiation
   - Medium of contact (phone, email, online portal)
7. **Equity-orientated program elements**
   - Availability of multilingual support
   - Access for remote populations
8. **Post-treatment support**
   - Clinical support
   - Financial support for follow-up care
   - Patient education on survivorship or ongoing health need

14 PAPs of FDA-approved CGTx spanning CAR-T, Lenti, AAV, and other gene therapies, each from distinct manufacturers, were reviewed

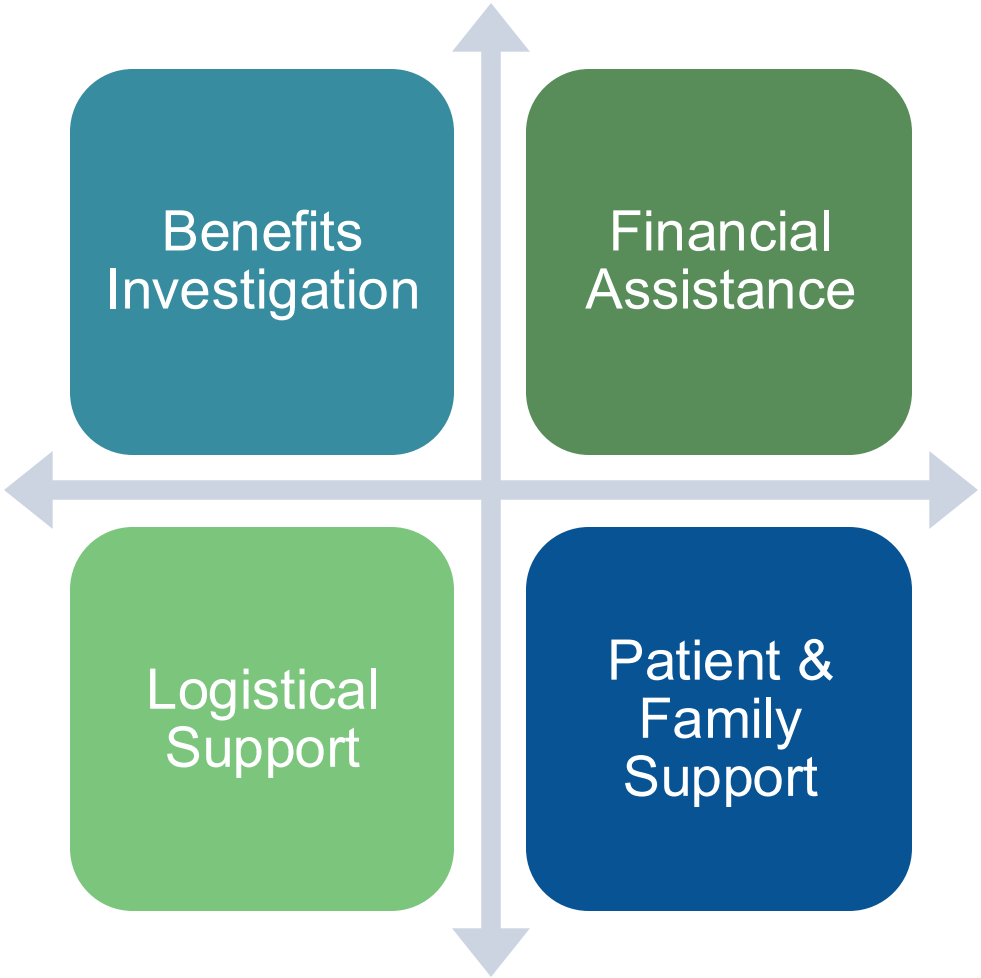
Trade Name (proper name)	CGTx Type	Manufacturer	Market Cap ($USD)	Therapeutic Area(s)	FDA Approval Year
Abecma (idecabtagene vicleucal)	CAR-T	Bristol Myers Squibb	101.07 B	Relapsed/refractory multiple myeloma	2021
Adstilladrin (nadofaragene fibradenovec)	Other GTx	Ferring Pharmaceuticals	N/A	Bacillus Calmette-Guérin (BCG)-unresponsive non-muscle invasive bladder cancer (NMIBC)	2022
Aucatzyl (obecabtagene autolecel)	CAR-T	Autolus Therapeutics	304.45 M	Relapsed/refractory B-cell acute lymphoblastic leukemia (ALL)	2024
Carvykti (Ciltacabtagene autoleucel)	CAR-T	Janssen Biotech, Inc	352.02 B	Relapsed/refractory multiple myeloma	2022
Casgevy (exagamglogene autotemcel)	Other GTx (CRISPR-Cas9 gene editing)	Vertex/CRISPR Therapeutics	118.01 B	Sick cell disease (SCD); transfusion-dependent β-thalassemia (TDT)	2023 (SCD) 2024 (TDT)
Kymriah (Tisagenlecleucel)	CAR-T	Novartis	181.20 B	B-cell ALL; relapsed or refractory large B-cell lymphoma; elapsed or refractory follicular lymphoma	2017 (ALL) 2018 (large B-cell lymphoma) 2022 (follicular lymphoma)
Lenmeldy (Atidarsagene autotemcel)	Lenti	Orchard Therapeutics (Kyowa Kirin)	756.82 M	Metachromatic leukodystrophy (MLD)	2024
Luxturna (Voretigene neparvovec)	AAV	Spark Therapeutics (Roche)	225.74 B CHF = 188.11 B USD (Roche)	RPE65 mutation-associated retinal dystrophy	2017
Roctavian (Valoctocogene roxaparvovec)	AAV	BioMarin Pharmaceutical	10.88 B	Hemophilia A	2022
Tecartus (Brexucabtagene autoleucel)	AAV	Kite Pharma (Gilead)	125.64 B (Gilead)	mantle cell lymphoma (MCL); ALL	2020
Tecelra (afamitresgene autoleucel)	Other GTx (TCR-T)	Adaptimmune Therapeutics	56.83 M	Synovial sarcoma	2024
Vyjuvek (Beremagene geperpavec)	Other GTx (topical HSV-1 vector)	Krystal Biotech	4.54 B	Dystophic epidermolysis bullosa	2023
Zolgensma (Onasemnogene abeparvovec)	AAV	Novartis Gene Therapies, Inc.	203.28 B	Spinal muscular atrophy (SMA)	2019
Zynteglo (Betibeglogene autotemcel)	Lenti	bluebird bio	45.83 M	Beta-thalassemia, cerebral adrenoleukodystrophy	2022

Source: FDA Approved Cellular and Gene Therapy Products (last updated: 03/06/2025); Google Finance (accessed April 9, 2025 and April 10, 2025)

CGTx PAPs include dedicated support from access professionals to assist patients in navigating the complex payer landscape through benefits investigation

Access services help patients, caregivers, and providers navigate a complex payer landscape, offering tailored assistance with benefits verification, prior authorizations, and appeals

Because CGTx are often limited to authorized or certified treatment centers, support programs play a vital role in helping patients locate and coordinate appointments with designated sites of care



Assess eligibility for support to obtain free drug or co-payment assistance, travel and lodging coverage, as well as referrals to charitable foundation to reduce both direct and indirect costs of care

Extended support beyond the point of administration, offering proactive check-ins, adherence tracking, and milestone monitoring to ensure continuity of care and compliance with pre- and post-treatment requirements

CGTx PAPs include dedicated support from access professionals to assist patients in navigating the complex payer landscape through benefits investigation

Case Manager Collects Insurance Information

Perform Benefits Verification

Check for Prior Authorization

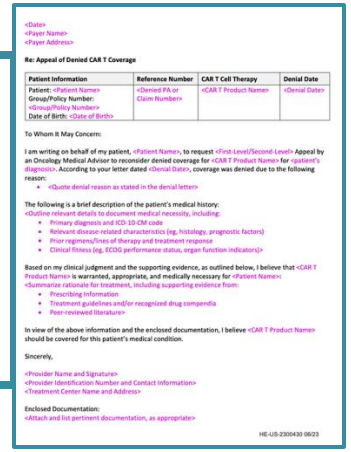
Appeals Support (if needed)

Temporary access solutions pending insurance

Cell Therapy 360 is offered by BMS

Many PAPs offer eligibility checkers and benefit verification tools to assist healthcare providers quickly assess a patient's eligibility for a medication

If a therapy is initially denied by the insurance, PAPs often: provide template letters for appeals, and guide providers on crafting letters of medical necessity



Programs with financial assistance often include the cost of drug, but may also include other direct medical or indirect patient out-of-pocket (OOP) costs with demonstrated need

Direct Costs

Drug Cost

- Most programs offer drug product at no cost to insured or underinsured eligible patients
- Duration of coverage may be limited (e.g. up to 12 months or through the calendar year)

Copay Assistance

- Most programs offer copay assistance to commercially insured eligible patents to help reduce or cover out-of-pocket costs for eligible patients

Medical Services & Facility Fee

- Administration of medication and other medical services including hospitalization, diagnostics, and laboratory testing are often not included

Annual Cap

- Some programs have an annual cap on financial support (e.g. $15,000/year)

Indirect Costs

Transportation and Lodging

- Some PAP for CGTx that require administration at authorized treatment centres and/or by specialty trained providers may provide stipends or reimbursement for travel and temporary lodging for patients and caregivers; however, additional restrictions and eligibility requirements may apply

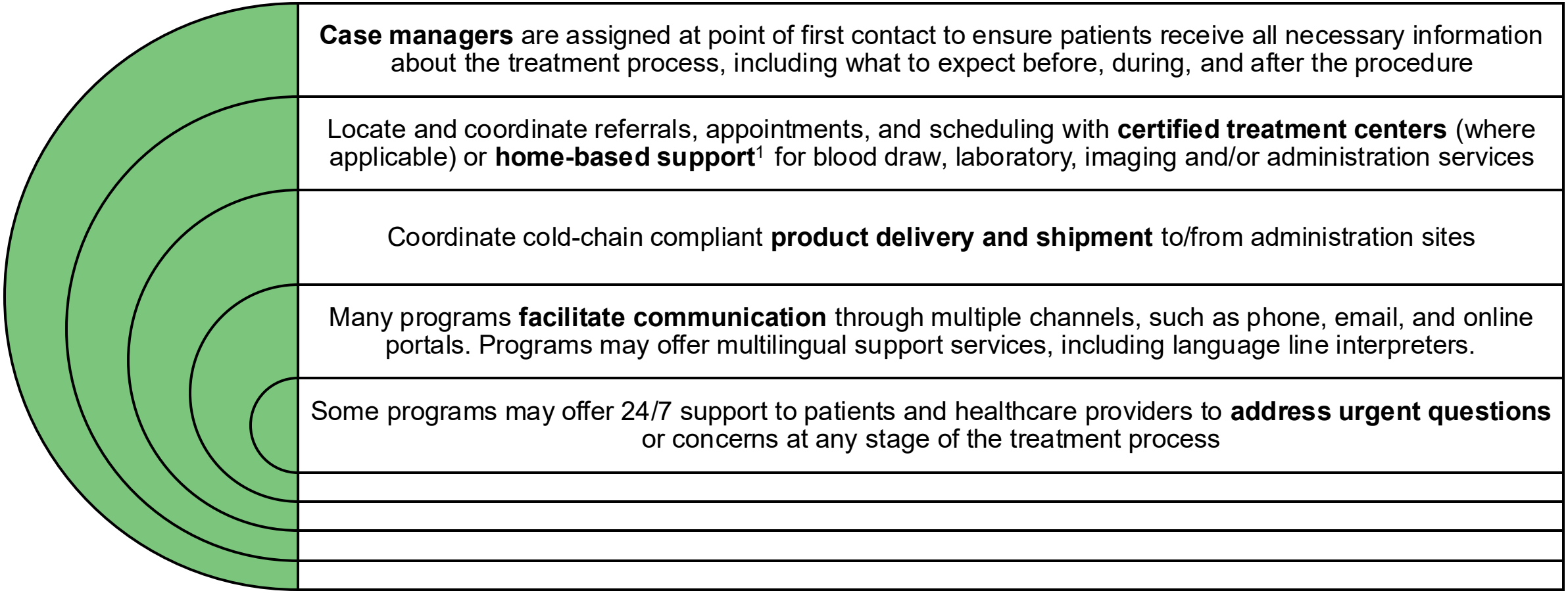
Caregiver and Family Support

- Some PAPs may reimburse for childcare or provide logistical support for caregivers to accompany the patient
- PAPs may not offer wage replacement or additional benefits for parents of children undergoing treatment

Referral to Non-Profit Foundations

- PAPs may refer patients to independent nonprofit foundations who may help with OOP costs

Many CGTx PAPs emphasize end-to-end personalized logistical support from enrollment to access with timely, defined points of contact with dedicated case managers



[1] PAPs for non-intravenous CGTx may have more flexible provider and site requirements including delivery by community health specialist, outpatient centers, or home (e.g. LUXTURNA administered by trained retinal specialist, ADSTILADRIN for intravesical administration by a urologist, VYJUVEK administered topically via home care services)

PAPs commonly partner with third-party services to address specialized patient and caregiver needs that extend beyond the treatment itself

Milestone Monitoring & Outcomes Tracking

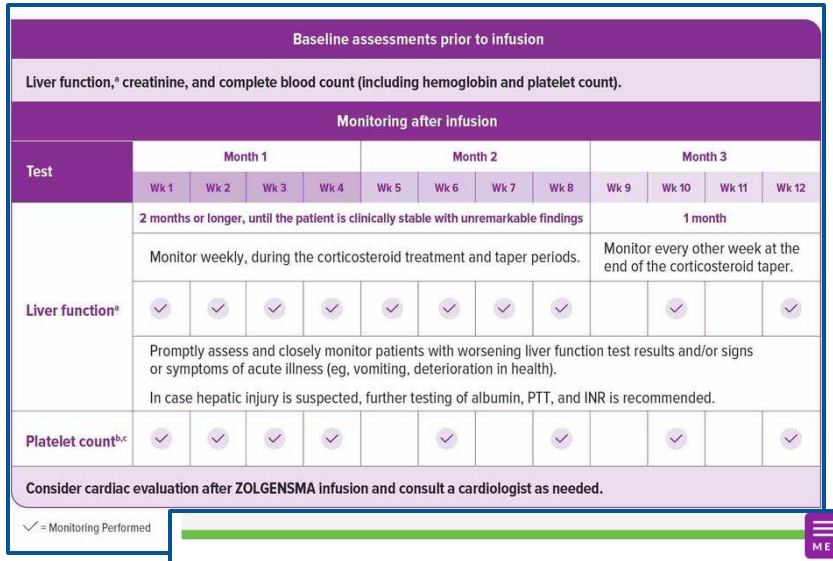
- Safety assessments
- Laboratory and imaging tests (including genetic tests, where applicable

Adherence and Compliance Support

- Medication reminder for pre- or post-treatment medication
- Support for REMS (Risk Evaluation and Mitigation Strategy) documentation

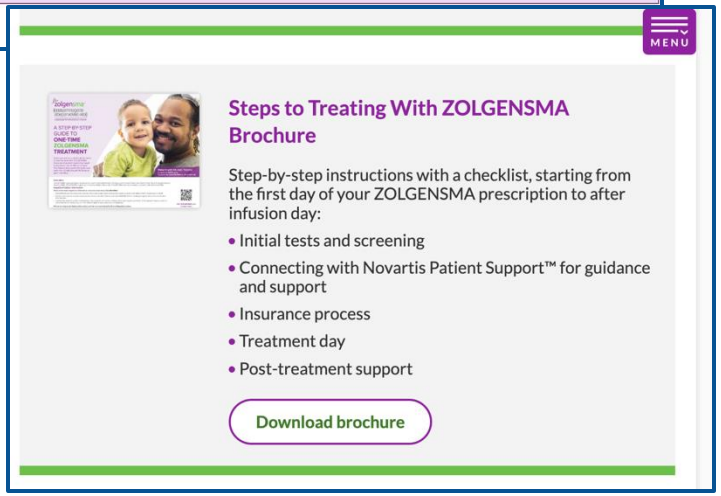
Caregiver and Family Support Services

- 24/7 nurse hotline for urgent questions
- Emotional support service like counseling or peer mentoring
- Access to patient and caregiver support groups



Baseline assessments prior to infusion												
Liver function,[a] creatinine, and complete blood count (including hemoglobin and platelet count).												
Monitoring after infusion												
Test	Month 1				Month 2				Month 3			
	Wk 1	Wk 2	Wk 3	Wk 4	Wk 5	Wk 6	Wk 7	Wk 8	Wk 9	Wk 10	Wk 11	Wk 12
Liver function[a]	2 months or longer, until the patient is clinically stable with unremarkable findings								1 month			
	Monitor weekly, during the corticosteroid treatment and taper periods.								Monitor every other week at the end of the corticosteroid taper.			
	✓	✓	✓	✓	✓	✓	✓	✓		✓		✓
	Promptly assess and closely monitor patients with worsening liver function test results and/or signs or symptoms of acute illness (eg, vomiting, deterioration in health). In case hepatic injury is suspected, further testing of albumin, PTT, and INR is recommended.											
Platelet count[b,c]	✓	✓	✓	✓		✓		✓		✓		✓
Consider cardiac evaluation after ZOLGENSMA infusion and consult a cardiologist as needed.												

✓ = Monitoring Performed



Centers for Medicare and Medicaid Innovation launched the Cell and Gene Therapy Access Model in January 2024 - sickle cell disease is the first indication

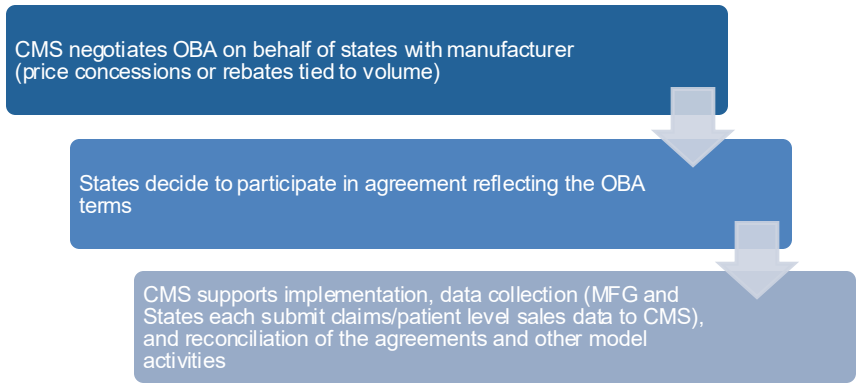
Goal

- Improve health outcomes in the Medicaid population
- Increase access specifically to transformative cell and gene therapies
- Lower health care use and costs for some of the nation's most vulnerable populations
- Make it easier for states to pay for these therapies that usually are high cost

Model Population

- Beneficiaries where Medicaid is the primary payer and Medicaid expansion Children's Health Insurance Program (CHIP) beneficiaries ("Title XIX beneficiaries") in fee-for-service and Medicaid managed care

Outcomes Based Agreement



- Test innovative payment and service delivery models to reduce costs and preserve/improve QoL
- Delivery may address gaps in care. In SCD these include fertility programs, equitable access, multi-disciplinary and comprehensive care
- Increase QoL from better access to CGTx

Funding

- State Medicaid programs will cover the cost of gene therapy (at a discounted price tied to specific outcomes)
- CMS will offer optional funding with increased reimbursement to states that enhance equitable access to cell and gene therapies and promote comprehensive care for Medicaid beneficiaries with SCD
- Manufacturers will provide rebates and discounts depending on volume
- Because the SCD CGTx requires myeloablative chemotherapy, OBAs may include provisions for costs of fertility preservation services
- Optional funding may be available to participating states that engage in activities to increase equitable access to CGTx, supporting ancillary services such as travel expenses, case management, and behavioral health services.

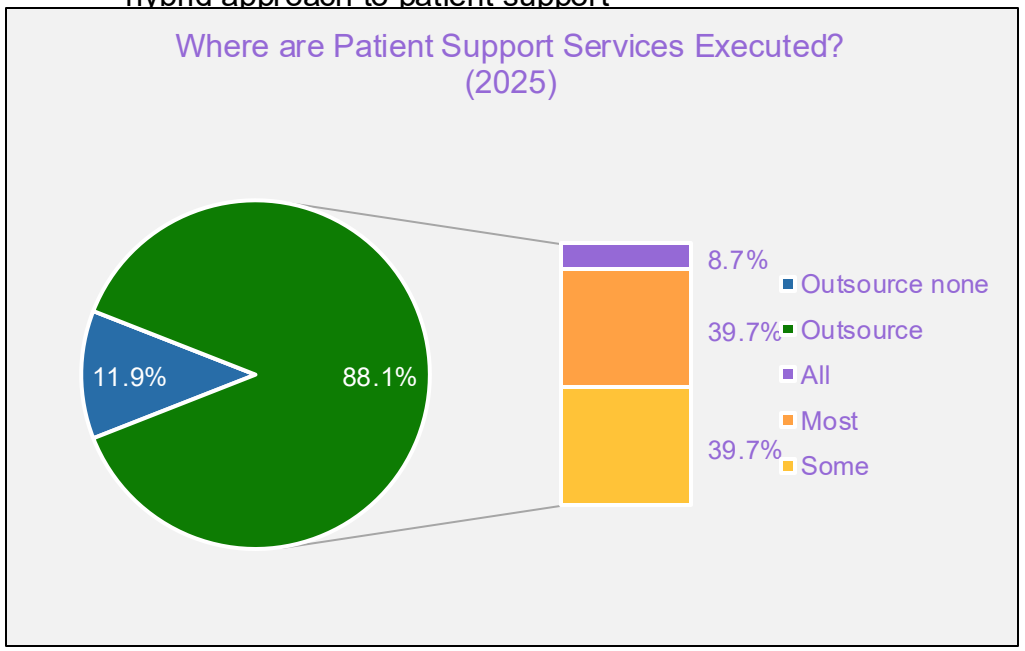
Initial focus of CGTx Access Model: Sickle Cell

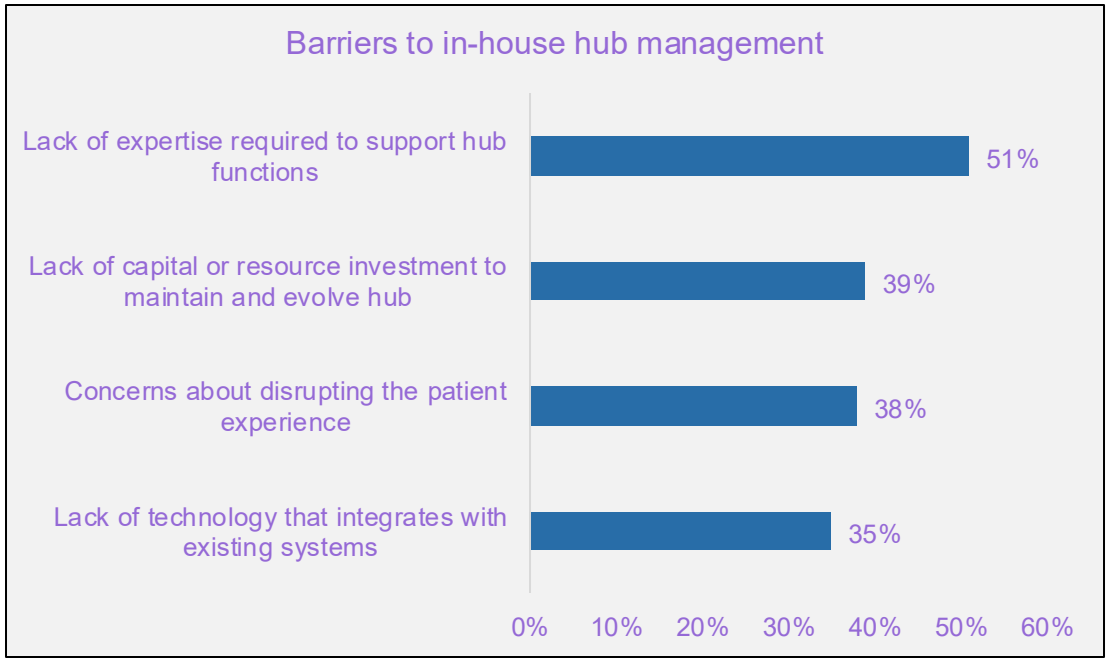
- High unmet need
- Rare disease (100,000+ in US)
- More than 90% are non-Hispanic Black or African American; 3%–9% are Hispanic or Latino
- Life span >20 years shorter than average
- Many people with SCD do not receive the recommended healthcare screenings and treatments
- 50% to 60% of people living with SCD are enrolled in Medicaid.
- Hospitalizations and other health episodes related to SCD cost the health system almost $3 billion per year
- Both Vertex and Bluebird are participating
- California also applied to participate

Source: 1) Data and Statistics on Sickle Cell Disease | Sickle Cell Disease (SCD) | CDC, 2) Biden-Harris Administration Announces Action to Increase Access to Sickle Cell Disease Treatments | CMS, 3) Cell and Gene T) Access Model | CMS

Patient assistance programs in general are mostly outsourced because of lack of competency, limited resources and fear of negatively impacting the patient experience

- According to a Cardinal Health survey in 2025, the vast majority (88%) of pharma companies will outsource some, most or all patient service programs
  - Hybrid models represent almost 80% of all patient assistance programs
  - Our research indicate gene therapies and other advanced treatments with small patient populations appear to use a hybrid approach to patient support
- There is a strong preference for external support, likely due to constraints like core competency in patient services within internal teams and financial and technical resources
  - An outsourced hub helps to streamline administration by managing the backend services, (e.g. reimbursement support, financial assistance qualification, travel and lodging etc.) while allowing the manufacturer more ownership of the patient journey

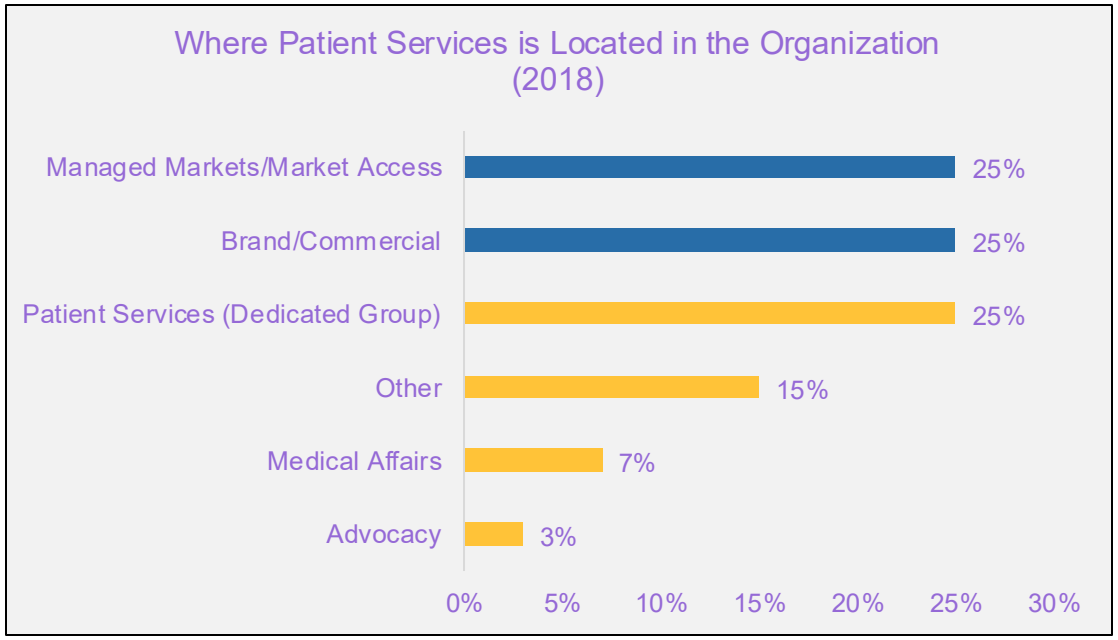


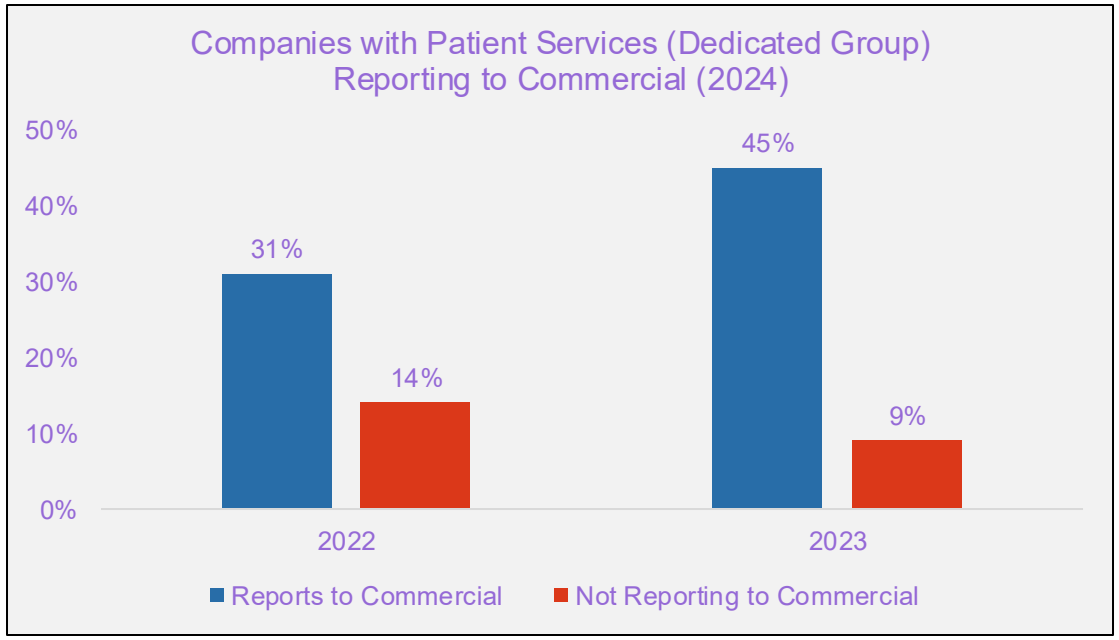


Source: Cardinal Health 2025. Industry survey: Hybrid models and the value of individual hub functions

Most companies structure the PAPs within the commercial organization

- Patient services may be located in different functional areas, but most commonly in brand/commercial, managed markets/market access or a dedicated patient services group
- There has been an evolution in what department patient services is part of
- In recent years PAPs predominantly reside within the commercial organization





*Other: Both commercial/ managed markets or corporate responsibility

Source: IntouchPOV_PharmaPatientSupportProgramsWhatDoestheFutureHold.pdf; 2024-02_Reprint_Helio-Seventh-Annual-Survey_001.pdf

Primary Research Closing Gaps

Gaps/Questions

- For CGTx companies, how is the PAP administered? (Internal; third party; Hybrid)
  - For hybrid models, what functions are typically done in house? By the third party?
  - What department does the PAP fall under?
  - How customized is each outsourced service within PAP administration?
- What are the typical patient eligibility requirements?
  - Do requirements apply to financial support only?
  - What details are provided to qualify/be eligible for financial support?
  - Are CGTx patients who do not qualify for financial support eligible for other support services?
- Do commercial payers include CMMI type support?
  - Fertility support services?
  - Behavioral support services?
- Are there differences in the PAP programs for CAR-T cell therapy vs other gene therapies (including non-IV routes)?
  - Are financial assistance programs different?
- How do you see these programs evolving?
- When should a manufacturer begin the work of planning their PAP?
- What are some providers of HUB services you would recommend?

Feedback from industry expert

Implementation and management of PAPs differ by company size and expertise

- Large/Medium sized well capitalized companies will implement a hybrid approach so they can control the patient experience, and close connection with payers and treatment centers
- Small biotech companies outsource the hub due to high costs, lack of experience and compliance challenges regarding financial support and scalability
- PAPs ownership varies and may be managed by a patient experience department, market access, customer experience group or commercial/technical operations
- PAPs will need to be specific to CGTx therapies (the general PAPs for CVD, GLP1, etc) will not suffice.
  - Case management is critical for CGTx.
  - About 20% of PAPs will need customization if working with a CGTx-experienced vendor

Patient (financial) eligibility requirements vary depending of disease area

- Some are more transparent than others (i.e. disclosing financial eligibility criteria)
- Collected by the care manager who does the onboarding, triage
- Best practice is to have a co-pay option as a safety net

CMMI type support depends on the specific therapy

- SCD is the first example where there is need for fertility and other support
- Because of the complexity of these disease states, companies are challenged by what to cover vs not and where to draw the line

PAP evolution

- Comprehensive patient support is needed
- Potential access barriers from provider may be coming
- Improve patient awareness of PAPs – 50% of pts are not aware of the PAP option

Recommendations for PAP

Patient Assistance Program – Industry Standard Benchmark Elements

Process for developing PAP

A PAP for companies developing CGTx should include a case manager who can help navigate patient benefits, treatment logistics and help assess financial needs

Patient Assistance Program – Industry Standard Benchmark Elements

Case Manager/ Navigator

Benefit investigation/ verification

- Assist with navigating the ongoing complexities of the reimbursement process
- Ensure documentation is in place for seamless access to treatment
  - US residency
  - Diagnosis, tests, prescription
  - Insurance status including:
    - Commercial insurance and coverage
    - Prior authorization process
    - Support with appeals

Logistical Support

- Ensure a seamless patient journey by relieving bottlenecks and pain points
- Coordinate cold-chain compliant product delivery and shipment to/from administration sites
- Assistance with travel plans and coordination, lodging, childcare, etc for
  - Patients and caregivers, when applicable

Financial Support

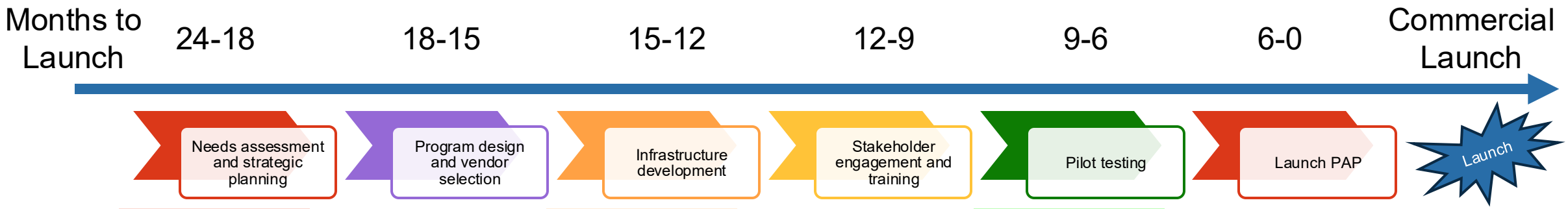
- Assess patient eligibility with demonstrated financial need
- Copay assistance (safety net)
- Coverage for drug
- Potential coverage for wrap-around services (administration, follow-up care , if required, etc.)
- Assess need to cover caregiver costs (travel, lodging, meals, childcare)

Ancillary/Other Support

- 24/7 assistance
  - Different modes of contact (phone, email, online portal)
  - Patient education and treatment expectations
- Referral to non-profit foundations and mentors
  - Emotional support service like counseling or peer mentoring
  - Access to patient and caregiver support groups
- Adherence and compliance support
- Safety monitoring

PAPs for CGTx's is a complex, multi-phase process that should begin well in advance of commercial launch, typically 18 - 24 months prior

Generalized timeline for developing a patient assistance program for cell and gene therapies*



Goal: Define the scope, objectives, and KPIs of the PAP

Activities:
- Assess the therapy's clinical profile, target population, and anticipated access barriers
- Conduct stakeholder interviews (patients, caregivers, providers, and payers)
- Benchmark against existing PAPs in rare disease and CGT spaces

Goal: Build a scalable, compliant, and patient-centric support model

Activities:
- Design core services: benefits verification, prior authorization support, financial assistance (copay, free drug), travel/lodging support, caregiver support, childcare support, and patient education
- Select and contract with a hub services provider or specialty pharmacy

Goal: Ensure operational readiness and interoperability

Activities:
- Develop IT systems for case management, data tracking, and reporting
- Create SOPs, training materials, and patient-facing resources
- Integrate with manufacturing and logistics systems (especially critical for autologous therapies)

Goal: Build awareness, disseminate and ensure seamless coordination

Activities:
- Train internal teams, hub staff, and field personnel
- Educate providers and treatment centers on program services
- Begin payer engagement to align on reimbursement pathways

Goal: Validate program functionality and make improvements

Activities:
- Pilot the PAP with early access or expanded access patients
- Test workflows, identify gaps, and refine processes

Goal: Ensure high patient satisfaction and access success

Activities:
- Go live with full PAP services at commercial launch
- Monitor performance metrics and patient feedback
- Adjust services based on real-world experience and evolving needs

***Note: Although CGTs have many similarities they are also very unique and therefore may require specific attention that is not captured in this generalized summary process**

Sources: Six key considerations for cell and gene therapy patient support programs, FierceBiotec.com; Cell and Gene Therapy (CGT) Access Model, CMS.gov; Keys to improving patient access and support services for cell and gene therapies, Amerisourcebergen.com

There are several potential options for partners with experience with cell and gene therapy PAPs

For **cell and gene therapies (CGTx's)**, the best hub service providers are those with deep expertise in managing the unique logistical, regulatory, and patient engagement challenges of these advanced therapies. Based on secondary research, these are some of the **hub service providers with expertise in CGT PAP support**

Company	Web-link
Cardinal Health Sonexus™	https://www.cardinalhealth.com/en/services/manufacturer/biopharmaceutical/patient-access-and-adherence.html
Cencora (formerly AmerisourceBergen)	https://www.cencora.com/human-health/our-capabilities/patient-access-and-adherence-support
Eversana	https://www.eversana.com/patient-support-services/
InspiroGene (McKesson)	https://www.mckesson.com/our-stories/expanding-access-to-cell-and-gene-therapy-inspirogene/
Orsini	https://www.orsini.com/therapeutic-areas/cell-gene-therapy/
Propharma	https://www.propharmagroup.com/thought-leadership/patient-support-services-in-cell-gene-therapies-cagts