

FoCUS Toolkit general education: The patient role in gene therapy financing

April 2023



NEW DIGS



The FoCUS Project has developed this toolkit as a resource for understanding the challenges of delivering and financing an advanced durable therapy.

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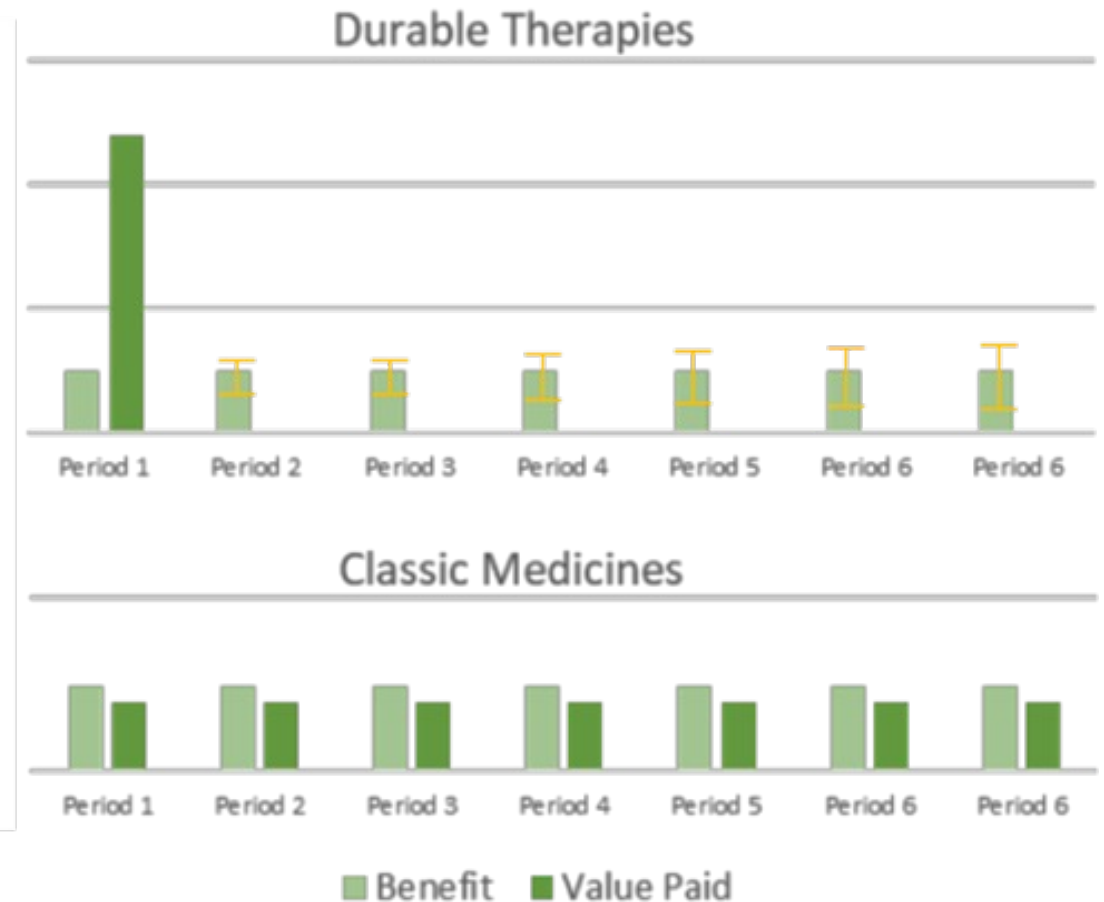
Why do cell and gene therapies need new payment models and what does that mean for patients?



Payment versus benefit

- Durable therapies are paid for up-front, but the benefits are delivered over time.
- Chronic treatment reimbursement traditionally happens in parallel with the delivery of the treatment and treatment benefit.
- Payers are attempting to address the disconnect between payment and value with new financial models.

Durable Therapies Distill Payments Upfront





Financial Impact:

Traditional therapy payments are ineffective arrangements for covering the cost of a one-time treatment for gene or cell therapy. New financial models help solve for some of the issues of traditional reimbursement. However, the models also change the patient role in therapy payment. Patient related issues to be addressed by new financing models include:

Payment
regardless of real-
world performance
certainty

Patient mobility

Provider network
capabilities and
coordination.



What does this mean for patients?



Cell and gene therapy payment challenge:

Payment
regardless of real-
world performance
certainty

Issue: Clinical uncertainties remain at the time of cell and gene therapy drug approvals and payers are concerned about paying for treatments that do not work as expected.

Scenario:



Small clinical trials



Potential variation in
FDA label from clinical
trials



Real world
outcomes TBD



Performance uncertainty implications for patients

Payers will potentially establish performance based financial agreements, requiring outcome tracking over time.

Patients need to know:

What outcomes are tracked?

How long will they be tracked?

What's required of the patient to support tracking?



Cell and gene therapy payment challenge:

Patient mobility

Issue: Outcomes based contracts will potentially require patient outcome data over multiple years. However, patients change payers over time.

Scenario:



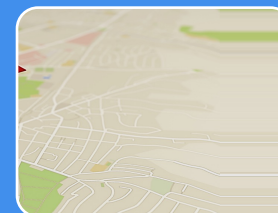
Plan A

Treatment



Plan A

1 year post
treatment



Plan B

2 years post
treatment



Patient mobility implications for patients

Changing health plan coverage may require continued follow-up and sharing of information with previous insurer.

- ❖ What outcomes are tracked?
- ❖ Can you change providers supporting tracking?
- ❖ Privacy concerns/HIPPA consents



Cell and gene therapy payment challenge:

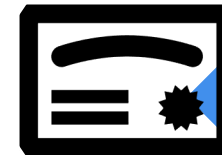
Provider network capabilities

Issue: Providers qualified to administer cell and gene therapy may not be contracted with payer and potentially located outside of payer's geographic coverage area.

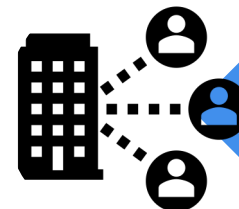
Scenario:



Treatment may be available only from a Center of Excellence



Current network providers may not be certified to deliver treatment.



Delivery of care may require coordination and care management to support financial contracts.



Provider network implications for patients

Treatment may call for care from a new provider and/or may require travel to a provider location away from home.

- ❖ Will I be able to maintain local care under my current doctor?
- ❖ How do I coordinate with current provider(s)?
- ❖ Will I have to travel for follow up care?



Patient solutions needed for new financial models

Elements of existing solutions:

- ❖ Developer Patient Assistance Programs (PAP)
- ❖ Developer access support programs
- ❖ Charitable organizations
- ❖ Provider discounts/charity care

Future solution requirements:

- ❖ Outcome metrics with minimal data collection burden to patients
- ❖ Data consents aligned with HIPAA requirements
- ❖ Enhanced supports for patient navigation of care and financial resources.



SUMMARY

Financial challenges of payment for gene and cell therapies are being addressed with payment and care models that may change engagement requirements of the patient. FoCUS recommends that you understand your role in how your care is financed. Ask questions. Suggest solutions that support your needs.