

FoCUS Toolkit general education: Overview of the cell and gene therapy pipeline

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NEW DIGS



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

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Disruption of Biopharma Payment System

The market of cell and gene therapies is poised to disrupt how we pay for biopharmaceutical care based on:

- Potential 'cures' for conditions with small numbers of patients
- High prices with payment 'up front'
- Performance uncertainty



Disruption Challenges

Potential 'cures' for conditions with small numbers of patients.

- Developers need to recover development costs and profits from limited numbers of patients.

High prices with payment 'up front'.

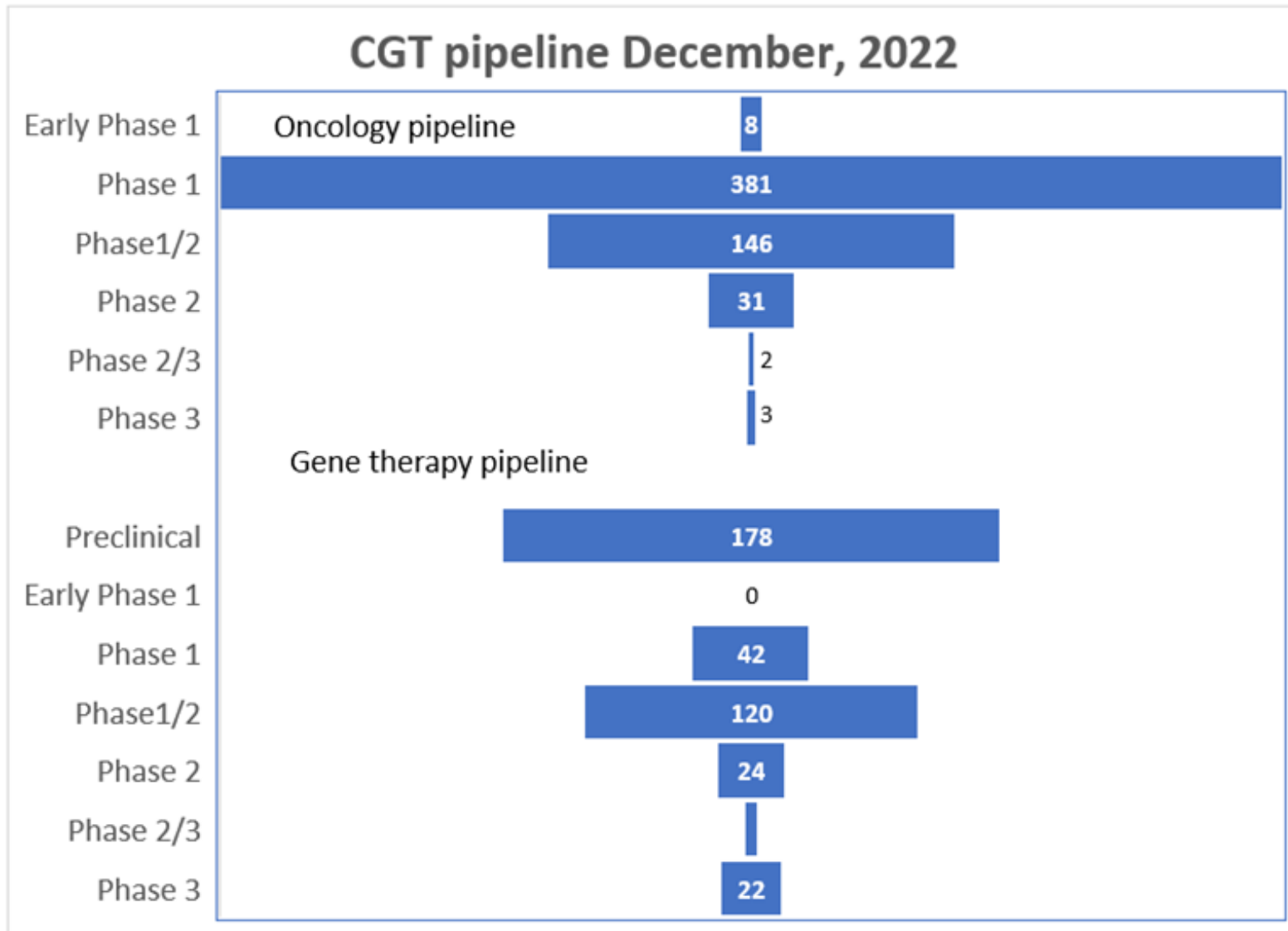
- Payers are faced with high, one-time payments when curative treatments replace chronic treatments.

Performance uncertainty

- Product approvals with limited trial size, long term clinical outcomes still being evaluated, changes in FDA label.



Expectation: 60+ product indications by 2032

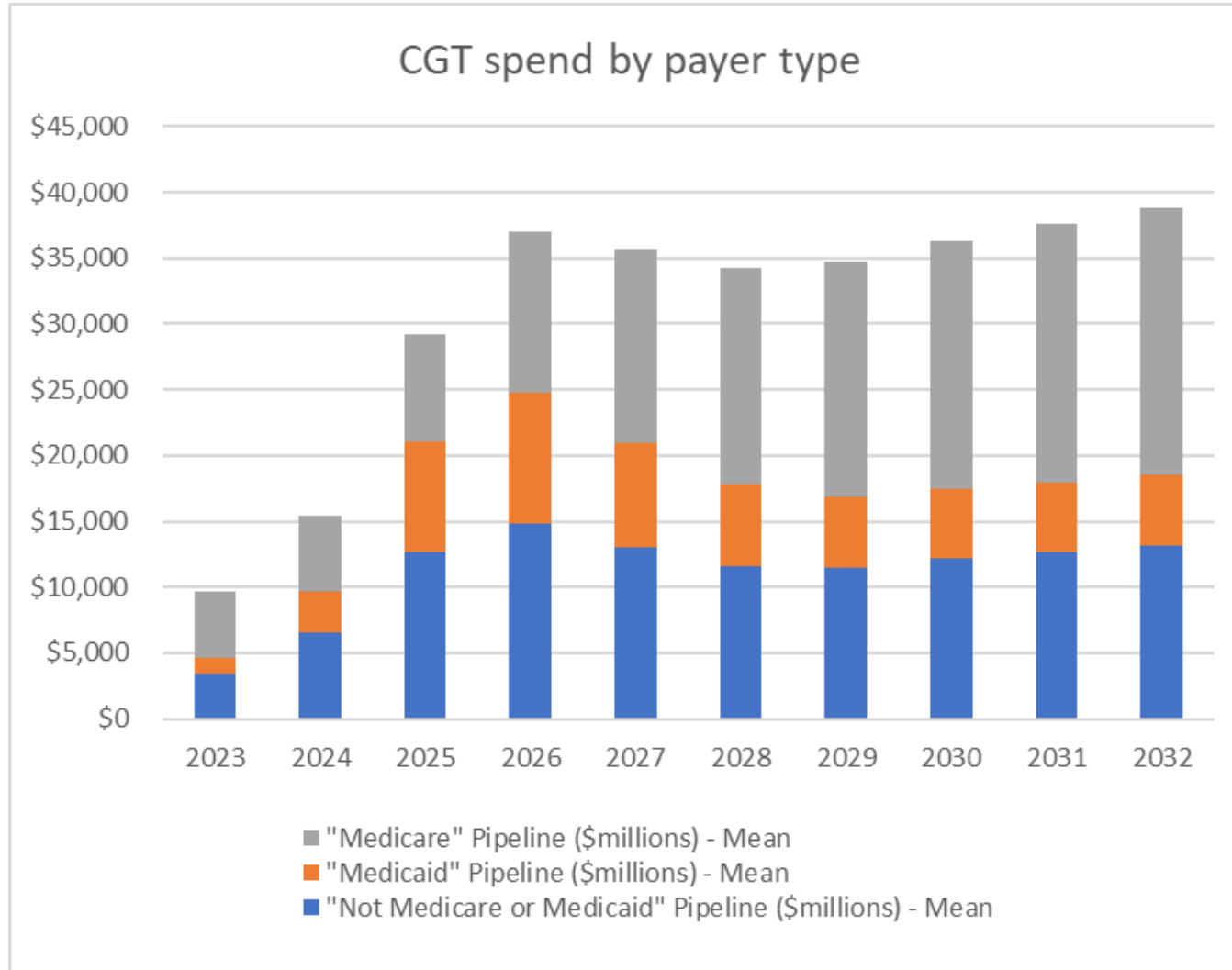


Pipeline product indications:

- ❖ 72% for oncology patients
- ❖ 22% for orphan, non-oncology indications
- ❖ 6% for larger therapeutic areas such as cardiovascular conditions



Growth in spend for CGT

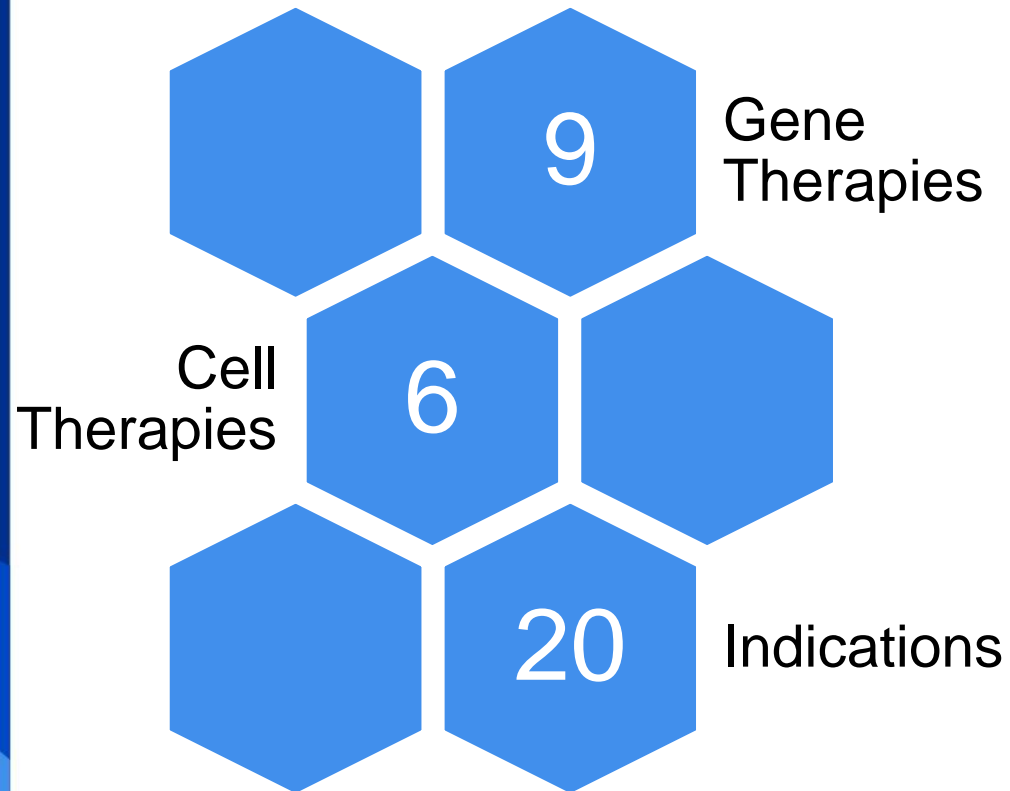


- ❖ Large oncology pipeline has its greatest impact on Medicare payers.
- ❖ Treatment for prevalent diseases peaks from 2025-2027 for Medicaid and Not-Medicaid or Medicare payers.



Pipeline Production

Already approved



Anticipated approvals

Rare diseases

- AADC Deficiency

Oncology

- B-Cell leukemia
- Lymphoma

Ophthalmological conditions

Neurological conditions

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