SEAC | Southeastern Actuaries Conference

Being Part of Something that Counts

The Future of Gene Therapy – Innovative Treatments and Funding Challenges

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Daniel Moore, FSA, MAAA, CERA

Daniel Moore is a Vice President and Principal with Lewis & Ellis, LLC (L&E). Since 2015, Daniel has assisted clients with designing, pricing, and filing A&H products including supplemental health, indemnity, student health, AD&D, and ACA Exchange products. Daniel assists clients with long-term care (LTC) needs including rate filings, rate review for state DOIs, LTC pricing, LTC projection modeling, and LTC litigation support. Daniel serves as an auditor for public welfare systems for Veteran's Affairs. He has reviewed MA and Part D plans for CMS Bid Desk review, and assisted clients with MA pricing work. He has also assisted CMS with Medicaid actuarial certification rate review. Daniel has reviewed a variety of experience, rate increase, and new filings for products for the state of Kentucky and has assisted in the review of individual and small group ACA filings in Maryland and the District of Columbia. Daniel has helped local governments with GASB 75 reporting. Prior to working as a consultant with L&E, Daniel spent a decade performing and teaching cello. He has worked in insurance sales, having spent one year as a licensed insurance agent selling health insurance.

What could Genetic Therapy do for Cystic Fibrosis?

- Three decades ago, the average person with CF would live to age 30, now age 50 is typical with symptom treatment
- Current treatment: Trikafta
  - Modulator Drug (pathogenetic therapy)
  - Estimated to increase median survival age for CF by 9.2 years
- Several gene therapy clinical trials underway for CF
  - Challenges in delivery of functional gene delivery to necessary cells
  - Need to overcome immune response
- Hope for etiotropic therapy in the near future

https://www.ncbi.nlm.nih.gov/pmc/articles/PMC10395777/

Cell & Gene Therapy (C&GT) Overview

Cell Therapy

Transfer of live cells into the body

Gene Therapy

Add new genes or edit / remove existing genes

By the Numbers

>2X

Expected increase in cell & gene therapy approvals by end of ‘25*

$4.25M

Current highest-priced gene therapy for ONE patient

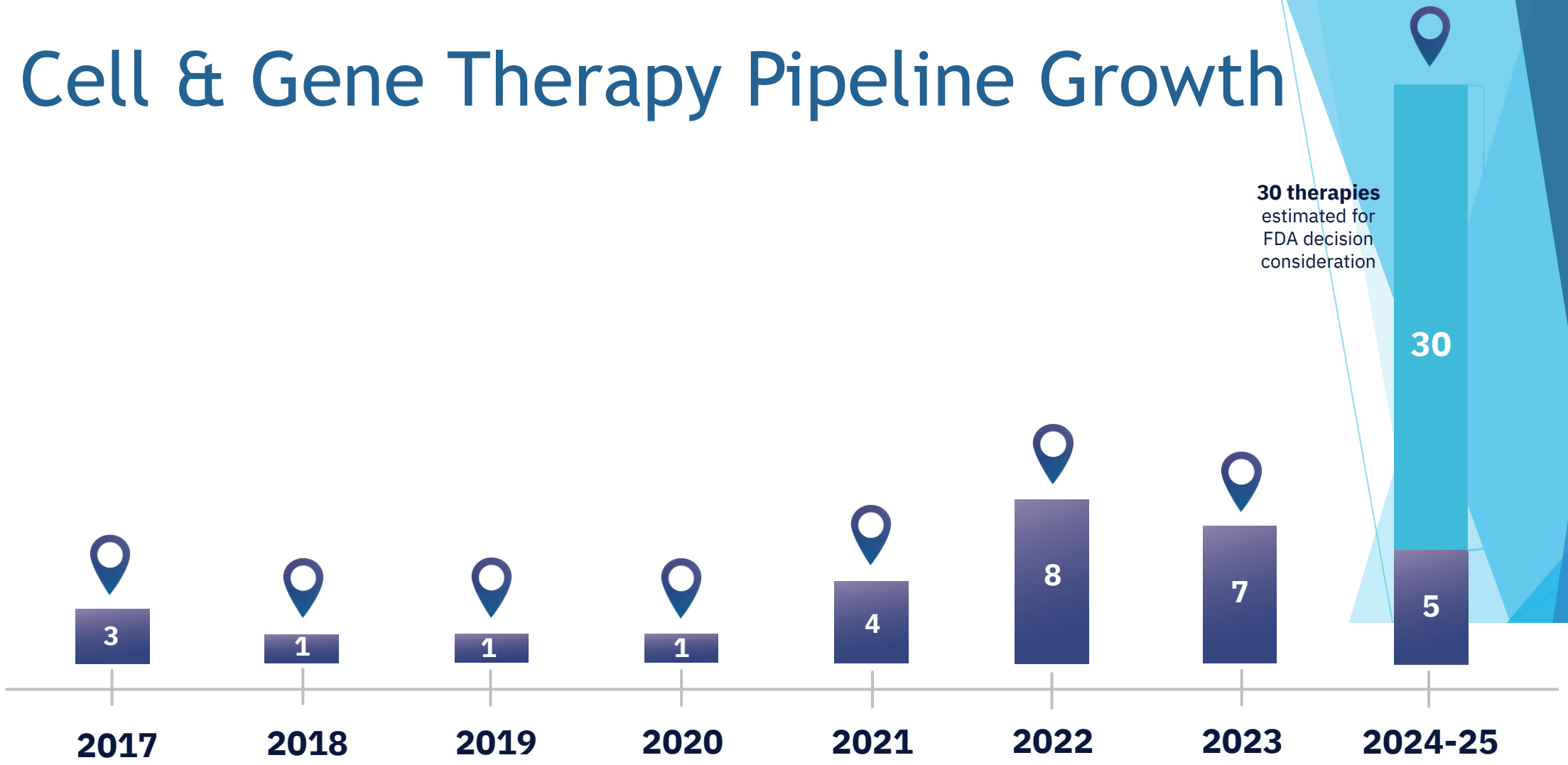
$13 PMPM

Expected CGT total cost of care spend by 2026, up from $4 - $5 PMPM today*

**Based on ETS estimates from forecast, for commercial population and total cost of care*

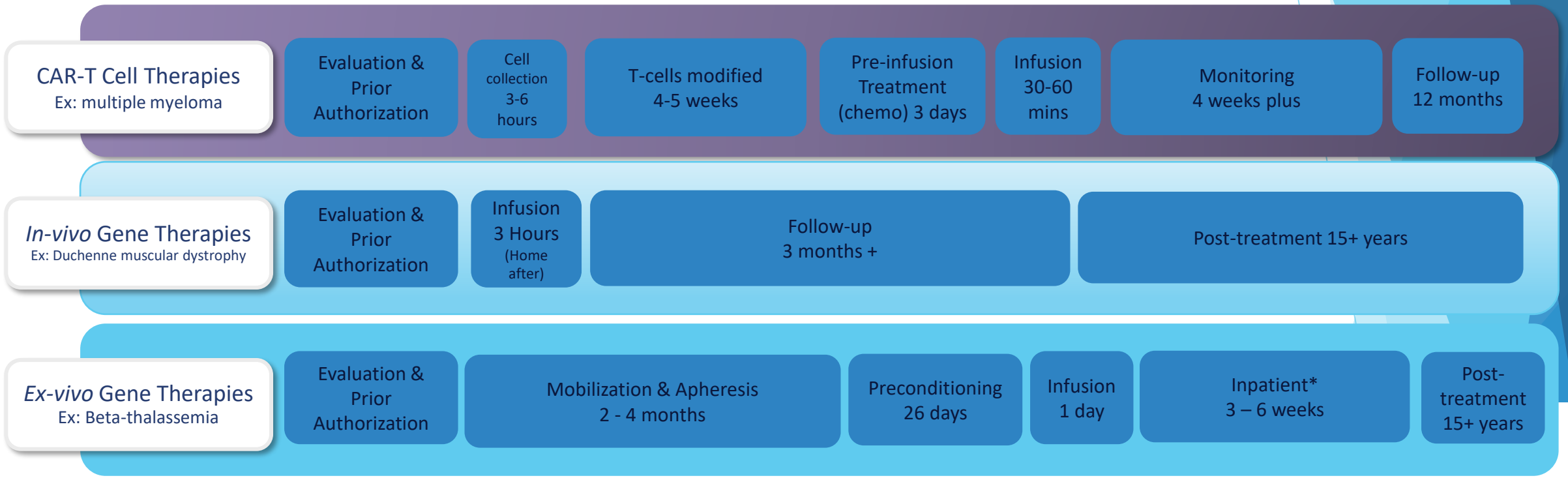
Cell & Gene Therapy Pipeline Growth

30 therapies estimated for FDA decision consideration



*US Food and Drug Administration (FDA) Approvals - Prescription Drug User Fee Act (PDUFA)

Understanding the complexities of cell & gene therapy treatment pathways, billing events, experience needs



IMPORTANT: these are examples and estimates for illustrative purposes only. For actual timeframes and treatment plans, please visit the manufacturer websites and content.

US FDA Approved Therapies with Condition, Date & Cost*

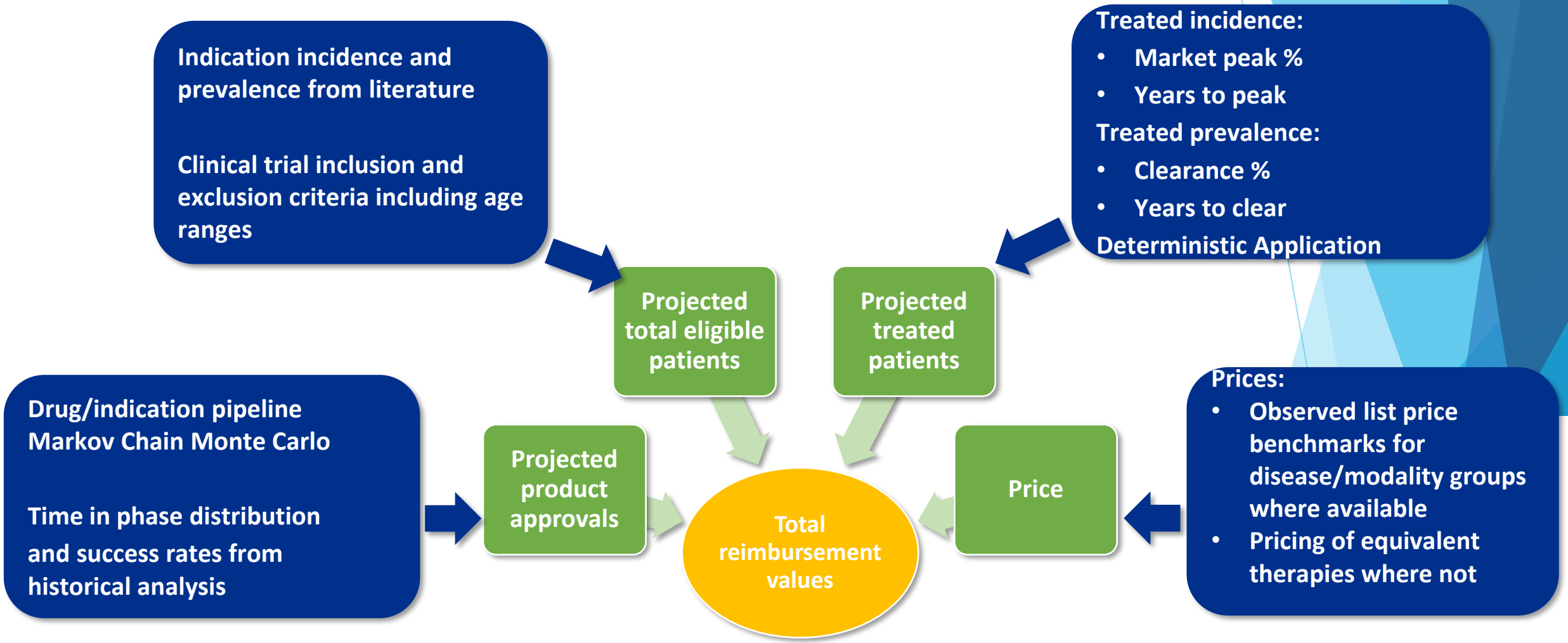
Therapy Brand Name	Condition(s)	Approval Date	Therapy List Cost†
Cell Therapies - *Chimeric antigen receptor (CAR) T-cell*			
Kymriah®	Acute lymphoblastic leukemia	August 2017	$581,895
Yescarta®	Diffuse large B-cell lymphoma, follicular lymphoma	October 2017	$462,000
Kymriah	Diffuse large B-cell lymphoma	May 2018	$456,941
Tecartus®	Mantle cell lymphoma	July 2020	$462,000
Breyanzi®	Diffuse large B-cell lymphoma, follicular lymphoma	February 2021; May 2024	$487,477
Tecartus®	Acute lymphoblastic leukemia	October 2021	$462,000
Kymriah	Follicular lymphoma	May 2022	$456,941
Breyanzi	Chronic lymphocytic leukemia or small lymphocytic lymphoma	March 2024	$487,477
Abecma®	Multiple myeloma	March 2021; April 2024	$498,408
Carvykti™	Multiple myeloma	February 2022; April 2024	$522,055
Cell Therapies - *Tumor-infiltrating lymphocytes (TIL)*			
Amtagvi™	Metastatic melanoma	February 2024	$515,000
Cell Therapies - *Other*			
Rethymic®	Congenital athymia	October 2021	$2,729,500
Omisirge®	Hematologic malignancies (Blood cancers)	April 2023	$338,000
Lantidra®	Diabetes Type 1	June 2023	Not available
Gene Therapies (*in vivo*)			
Luxturna®	Biallelic *RPE65* mutation associated retinal dystrophy	December 2017	$456,875 per eye ($913,750 both eyes)
Zolgensma®	Spinal muscular atrophy	May 2019	$2,322,044
Hemgenix®	Hemophilia B	November 2022	$3,500,000
Adstiladrin®	Bladder cancer	December 2022	$60,000 per instillation
Elevidys®	Duchenne muscular dystrophy	June 2023	$3,200,000
Roctavian®	Hemophilia A	June 2023	$2,900,000
Beqvez™	Hemophilia B	April 2024	$3,500,000
Gene Therapies (*ex vivo*)			
Zynteglo®	Transfusion-dependent beta-thalassemia	August 2022	$2,800,000
Skysona®	Cerebral adrenoleukodystrophy	September 2022	$3,000,000
Casgevy™	Sickle cell disease	December 2023	$2,200,000
Lyfgenia™	Sickle cell disease	December 2023	$3,100,000
Casgevy™	Transfusion-dependent beta-thalassemia	January 2024	$2,200,000
Lenmeldy™	Metachromatic leukodystrophy	March 2024	$4,250,000
Gene Therapies - *Topical*			
Vyjuvek™	Dominant and recessive dystrophic epidermolysis bullosa	May 2023	$631,000** ($900,000 maximum†)

Please note that list cost for cell & gene therapies does not include any of the care needed to deliver the therapy, such as costs before and after delivery. Administration and associated hospitalizations can range from $300,000 to $800,000.

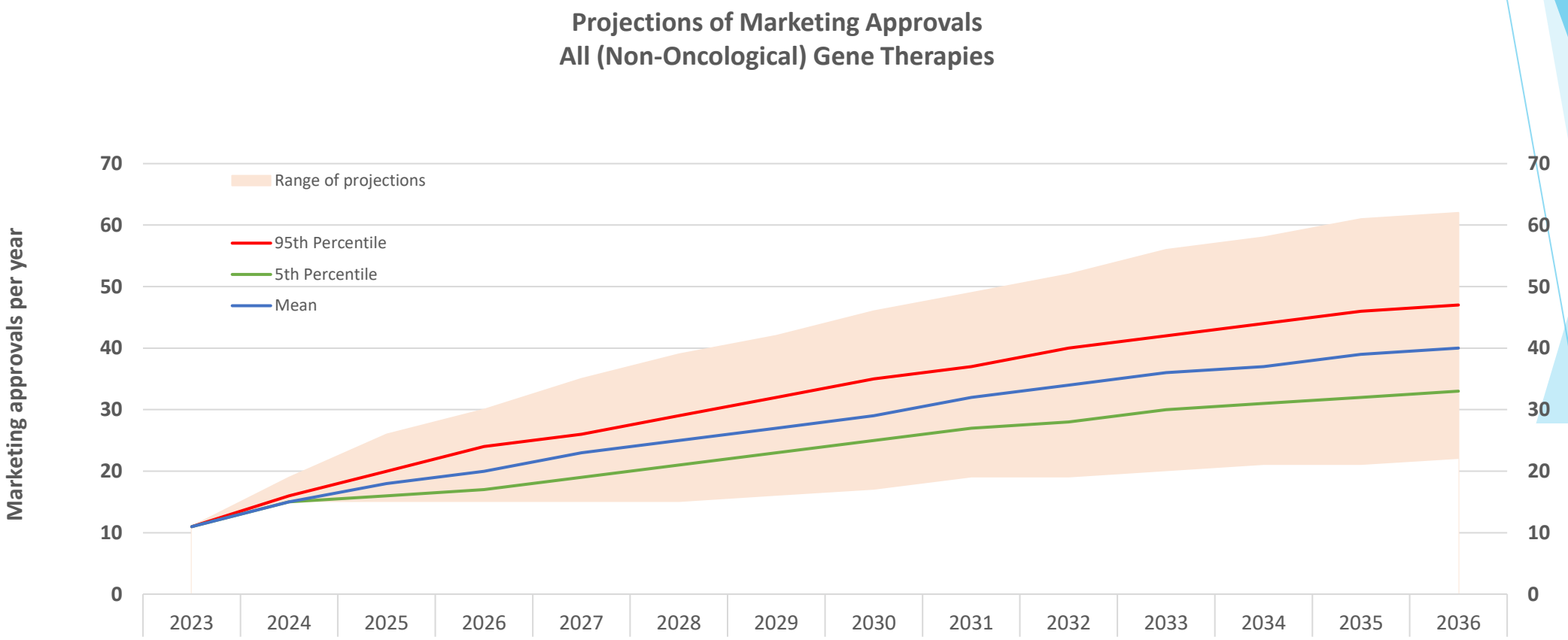
*US Food and Drug Administration (FDA) approved; Prices as of May 2024, and subject to change.

**Expected average annual cost per patient after induction and based on per vial cost of $24,250 for 26 weeks; commercial members capped at $900,000 with manufacturer terms.

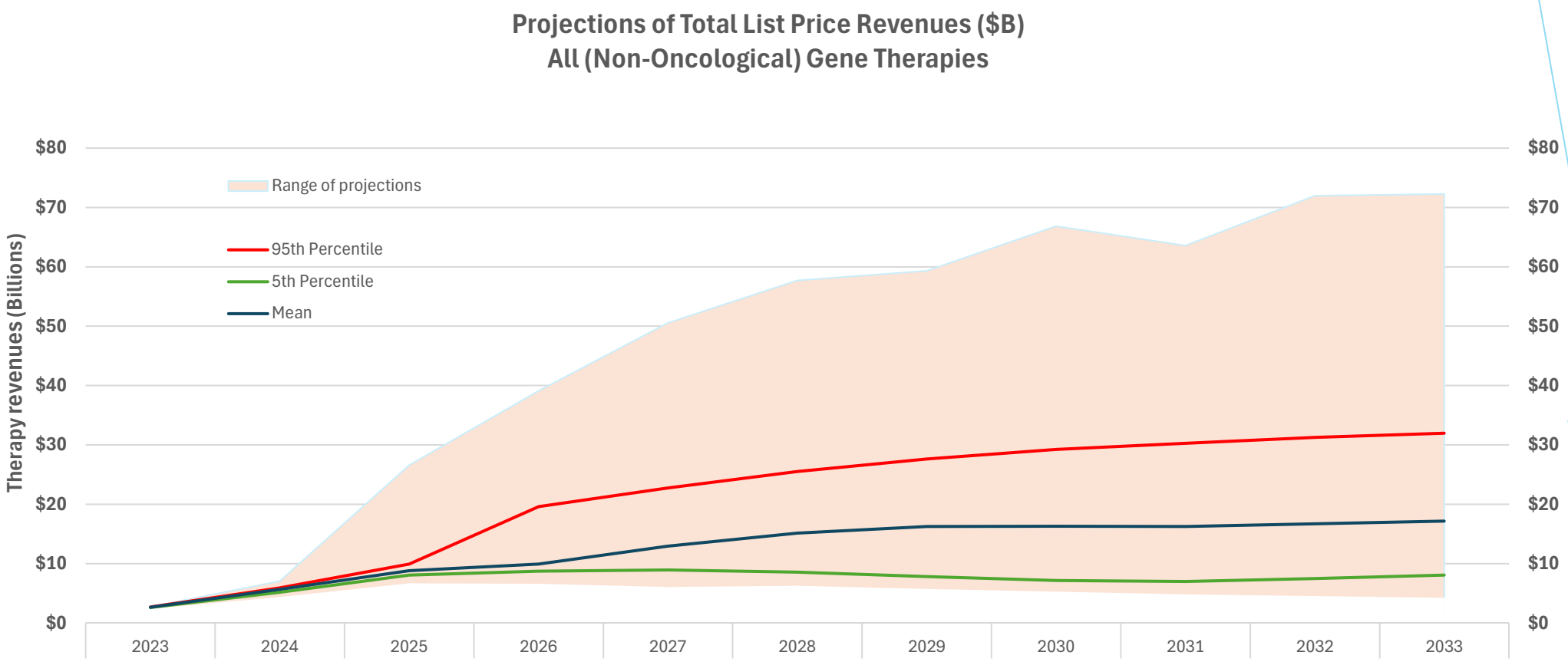
Pipeline Analysis Model



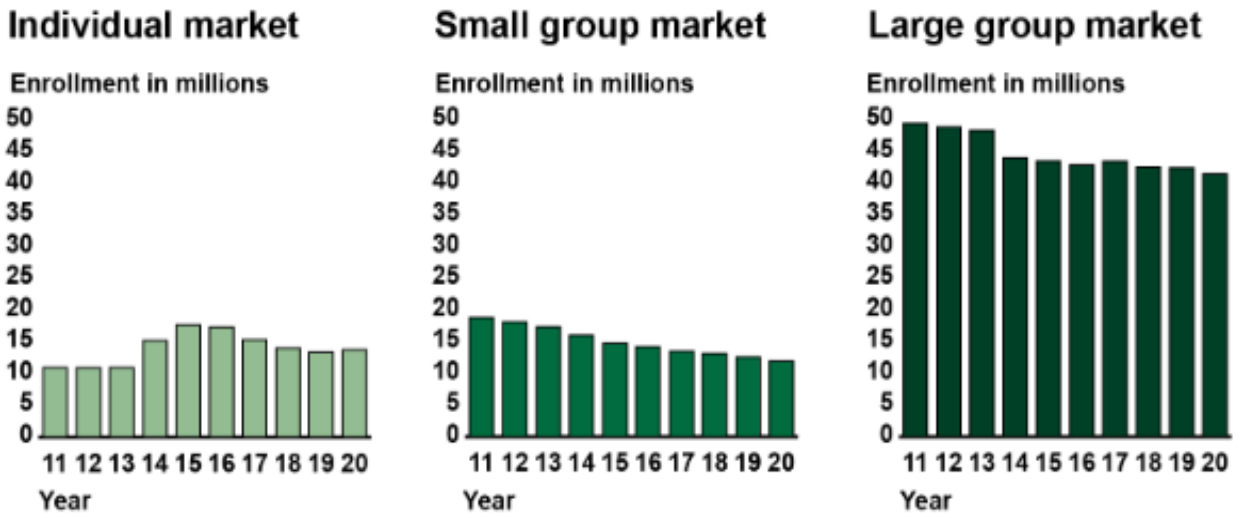
Projections of Cumulative FDA Approvals



Projections of List Price Revenues



Gene Therapy Drug Access & Funding



Source: GAO analysis of data from the Centers for Medicare & Medicaid Services (CMS). | GAO-23-105672

Questions?

$13 PMPM

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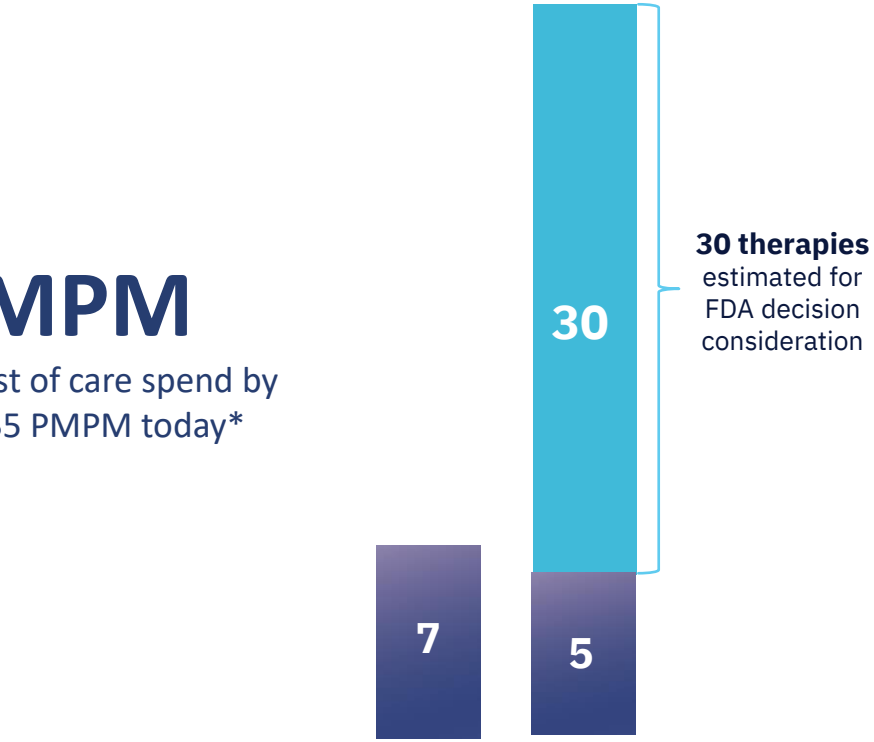

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