

AMWINS™

# Gene Therapy





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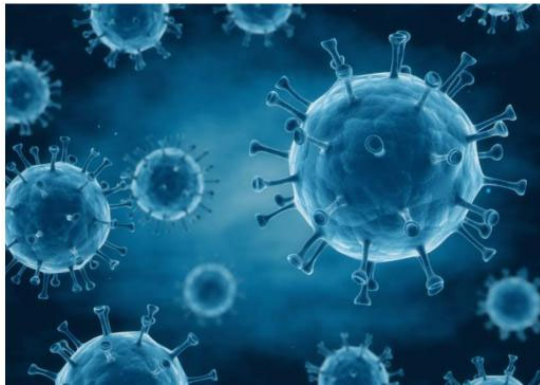
# Cell Therapy vs. Gene Therapy

## Cell Therapy:

- Transferring modified cells into the body

Autologous - Patients Cells

Allogeneic - Donor Cells

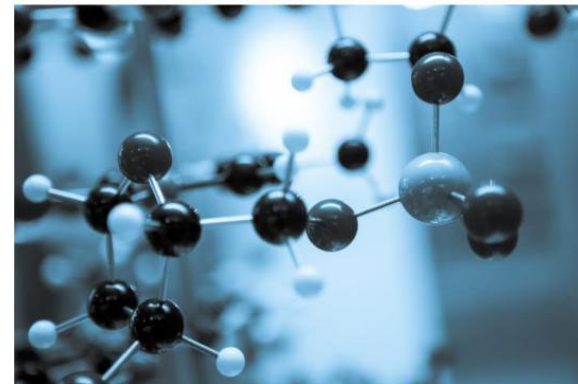


## Gene Therapy:

- Gene Addition
- Altering or removing existing genes

Ex Vivo – Genetically modified outside of the body

In Vivo – Genetic material directly inserted into the body





# Current Landscape



## Gene therapy landscape

- **33** cellular and gene therapy in market today
  - **23** cellular treatments
  - **10** gene therapy treatments
  - Another **7 - 10** treatments predicted to be approved by end of 2024
- Gene therapy treatments range in price from **\$850,000 - \$4.25M**





# Current Landscape (gene therapy)

- **Luxturna**
  - Spark Therapeutics; December 2017
  - Treats: Leber Congenital Amaurosis (LCA)
  - Cost: **\$850,000** (425k per eye)
  - Incidence: 1,000 to 2,000 patients in US
- **Zolgensma**
  - Novartis Gene Therapies, Inc.; May 2019
  - Treats: Spinal Muscular Atrophy (SMA)
  - Types 1 & 2 (Children ages 2 and under)
  - Cost: **\$2,100,000**
  - Incidence: 1 out of 10,000 births
- **Zynteglo**
  - BlueBird Bio, Inc.; August 2022
  - Treats: Beta thalassemia
  - Cost: **\$2,800,000**
  - Incidence: 1,500 prospective patients in US
- **Skysona**
  - BlueBird Bio, Inc.; September 2022
  - Treats: Cerebral Adrenoleukodystrophy
  - Cost: **\$3,000,000**
  - Incidence: 40 cases per year in the US
- **Hemgenix**
  - CSL Behring; November 2022
  - Treats: Hemophilia B
  - Cost: **\$3,500,000**
  - Incidence: 1 in 25,000 male births
- **Elevidys**
  - Sarepta; June 2023 **FAILS ENDPOINT TRIAL**
  - Treats: Duchenne Muscular Dystrophy
  - Cost: **\$3,500,000**
  - Incidence: 6 out of 100,000
- **Roctavian**
  - BioMarin; June 2023
  - Treats: Hemophilia A
  - Cost: **\$2,900,000**
  - Incidence: 12 out of 100,000 males
- **Vyjuvek**
  - Krystal Biotech, Inc.; May 2023
  - Treats: Dystrophic Epidermolysis Bullosa
  - Cost: **\$1,300,000** over 25 weeks
  - Incidence: 3.3 out of 1,000,000 in US
- **Casgevy**
  - Vertex Pharmaceuticals; December 2023
  - Treats: Sickle Cell (ages 12+)
  - Cost: **\$2,200,000**
  - Incidence: 100,000 in US
- **Lyfgenia**
  - BlueBird Bio, Inc. ; December 2023
  - Treats: Sickle Cell (ages 12+)
  - Cost: **\$3,100,000**
  - Incidence: 100,000 in US
- **Lenmeldy**
  - Orchard Therapeutics; **JUST APPROVED**
  - Metachromatic leukodystrophy
  - Cost: **\$4,250,000**
  - Incidence: Ultra-rare



## Current Landscape (common cell therapy's)

- **Kymriah**
  - Novartis; August 2017
  - Treats: ALL and Non-Hodgkin lymphoma
  - Cost: \$447,000-\$570,000
  - Incidence: Rare
- **Yescarta**
  - Kite Pharma, Inc.; April 2022
  - Large B-cell Non-Hodgkin lymphoma
  - Cost: \$478,000
  - Incidence: Rare
- **Tecartus**
  - Kite Pharma, Inc.; October 2021
  - Treats: Relapsed mantle cell lymphoma
  - Cost: \$478,000
  - Incidence: Ultra-rare
- **Breyanzi**
  - Bristol-Myers Squibb, Co.; **JUST APPROVED**
  - Treats: LBCL, CLL & SLL
  - Cost: \$492,000
  - Incidence: Rare
- **Abecma**
  - Bristol-Myers Squibb, Co.; March 2021
  - Treats: Multiple myeloma
  - Cost: \$503,000
  - Incidence: Rare
- **Carvykti**
  - Janssen Biotech, Inc.; February 2022
  - Treats: Multiple Myeloma
  - Cost: \$500,000
  - Incidence: Rare
- **Adstiladrin**
  - Ferring Pharm.; December 2022
  - Treats: High-grade bladder cancer
  - Cost: \$290,000
  - Incidence: Rare
- **Imlygic**
  - BioVex-Amgen, Inc.; October 2015
  - Treats: Melanoma
  - Cost: \$65,000
  - Incidence: Rare
- **Lantidra**
  - CellTrans Inc.; June 2023
  - Treats: Type 1 diabetes
  - Cost: \$300,000
  - Incidence: Rare
- **Provenge**
  - Dendreon Pharma.; April 2010
  - Treats: Advanced prostate cancer
  - Cost: \$65,000
  - Incidence: Ultra-rare



## Current Landscape (what's in the pipeline?)

- **Fidanacogene elaparvovec**
  - Estimated Approval: 2024
  - Treats: Hemophilia B in adults
  - Cost: TBD
  - Incidence: Ultra-rare
- **Fordadistrogene movaparvovec**
  - Estimated Approval: 2024
  - Treats: DMD
  - Cost: TBD
  - Incidence: Ultra-rare
- **Olenasuflogene relduparvovec**
  - Estimated Approval: 2024
  - Treats: Lysosomal storage disorders
  - Cost: TBD
  - Incidence: Ultra-Rare
- **Lumevoq**
  - Estimated Approval: 2024
  - Treats: LHON
  - Cost: TBD
  - Incidence: Ultra-rare
- **OTL-103**
  - Estimated Approval: 2024
  - Treats: Wiskott-Aldrich syndrome
  - Cost: TBD
  - Incidence: Ultra-rare
- **Pariglasgene brecaparvovec**
  - Estimated Approval: 2024
  - Treats: Von Gierke disease
  - Cost: TBD
  - Incidence: Ultra-rare
- **Botaretigene sparoparvovec**
  - Estimated Approval: 2024
  - Treats: X-linked retinitis pigmentosa
  - Cost: TBD
  - Incidence: Rare
- **Generx**
  - Estimated Approval: 2024
  - Treats: Myocardial ischemia due to CAD
  - Cost: TBD
  - Incidence: 1.7% of population
- **Giroctocogene fitelparvovec**
  - Estimated Approval: 2024-2025
  - Treats: Hemophilia A in adults
  - Cost: TBD
  - Incidence: Ultra-rare





# Employer Risk

## Expected Impact

ETS Estimates*	2020	2023	2026
Available Therapies	5	31	50
Qualified Patients	7	40	120
Therapy Costs**	~\$7M	~\$51M	~\$150M
Treatment PEPM	\$1.23	\$7.65	\$22.05
TCOC**	~12M	~\$65M	~\$190M
TCOC PEPM	\$1.62	\$9.72	<b>\$28.80</b>

\*Emerging Therapy Solutions provides data insights to health plans in order to maximize value for people in need of the newest complex and costly therapies. Including both gene and cell therapy treatments. [About Emerging Therapy Solutions \(ETS\) and LifeTrac Network \(emergingtherapies.com\)](https://emergingtherapies.com)

\*\*Above represents impact simulation for a million-member U.S. health plan, based on longitudinal data from over 90 million lives. TCOC – Total Cost of Care; PEPM – Per Member Per Month; Assumes 1.8 contract size



# Gene Therapy: To cover or not to cover?



## Why might employers consider not covering gene therapy?

- **Stop Loss Implications**

- Premium increases, lesser liability, exclusions, plan document ambiguity



- **Compliance Risks**

- Disabilities-based discrimination claim (e.g., extenuating circumstance exceptions)
- HIPAA Violation (e.g., mid-year changes due to impending claim)



- **Public Relations Risk**

- Negative publicity, child of an executive needs the life-saving treatment





# Why might employers consider covering gene therapy?

- **No Other Alternatives**

- Often, there are simply no alternatives to gene therapy. Gene therapies treat previously untreatable or difficult-to-treat conditions



- **Severe Disability**

- Patients who are candidates for gene therapies can suffer from severe disability and/or painful early death without these treatments



- **Curative**

- Some gene therapies may be curative, and cost associated with the ongoing treatment of disease symptoms may be reduced. This isn't universal to all gene therapies.



- **Recommended by ICER**

- Institute for Clinical and Economic Review (ICER) is the gold standard in the U.S. for assessing the value of prescription drugs and medical treatments. Of the currently approved gene therapies, ICER has concluded that the therapies demonstrate positive value to society but the pricing often exceeds the maximum value identified.





# Industry Solutions



# \*Industry Solution Models

1. ASO Carrier PEPM 2. Carveout Gene Therapy Carrier PEPM 3. Embedded Stop Loss Carrier

Consideration	Embarc ASO-Carrier Model	Berkshire Carveout Model	Berkley Embedded SL Model	Voya Embedded SL Model
Therapies Covered	Zolgensma, Luxturna, Skysona, Zynteglo, Hemgenix, Elevidys & Roctavian	Zolgensma, Luxturna, Skysona, Zynteglo, Spinraza	Zolgensma, Luxturna, Skysona, Zynteglo, Hemgenix, Elevidys & Roctavian	Zolgensma, Luxturna, Skysona, Zynteglo, Hemgenix, Elevidys & Roctavian
First-Dollar Coverage to Plan Sponsor	NO; 500K ISL	YES; Reimburses employer their ISL	NO; Subject to employer ISL w/ step-down for ETS.	NO; Subject to employer ISL
Portable	NO; unless over 20k ee's & bare on stop loss	YES; underlying stop loss must be placed via Stealth-Amwins A&H	NO	NO
Center of Excellence or Network Requirement	YES	NO	YES; Emerging Therapy Solutions for ISL step-down	NO
Available on a stand-alone basis	NO; unless over 20k ee's & bare on stop loss	NO; underlying stop loss must be placed via Stealth-Amwins A&H	NO	NO
Cost of Program	\$3 PEPM	\$1.99 PEPM	Included in underlying stop loss premiums	Included in underlying stop loss premiums

*\*Program disbursements are subject to coverage terms and exclusions.*



# Amwins-Stealth: Who we are



## We're Amwins

We're the largest specialty wholesale insurance distributor in the U.S.\* and we're devoted to your success.

With **\$26B in annual premium** and **6,200 employees worldwide**, when you partner with us, you can count on:



Market knowledge and access to close tough deals. Our scale and clout with **1,500+ markets and MGAs** are yours to tap into.



A global network of specialists and solutions who handle more than **1,500,000 submissions** each year.



A data-driven placement strategy, complemented by proprietary products and creative solutions



Our 150-year vision driving our decision-making. We're your partner for the long haul.

*\*Ranking from Business Insurance, September 2020*





## Stealth: Amwins Subsidiary

**#1**

Stop loss  
wholesale broker

**\$1.5B**

Premium placement

**2,300**

Employer groups

**37**

Carriers/markets

17 locations across the country



With focused expertise and a complete, unbiased commitment to providing quality, turnkey stop-loss solutions, since 2009 Stealth has made **self-funding a more viable option that drives value for employer groups of all sizes.**



# Why Stealth?



Carrier Partnerships



Independent Experts

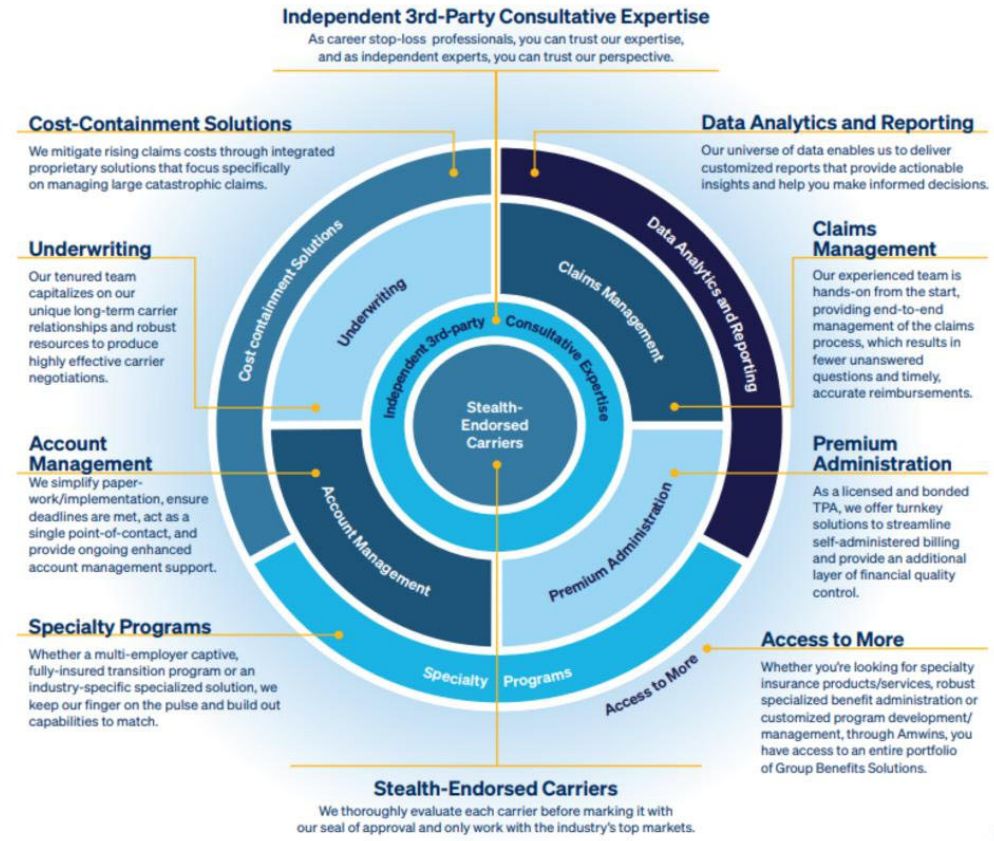


Comprehensive solution



Access to more

**With Stealth, you can expect so much more than just a quote.**





**THANK YOU!**