Washington State Health Care Authority

Emerging Therapies Workgroup – Funding Options

February 19, 2020 1-4pm
The Conference Center at SeaTac

Attendees:					
☐	Cody Gillenwater	☐	Stephanie Simpson	☐	Jean-Baptiste Roullet
☐	Carly Rodriguez	☐	Melissa Tribelhorn	☐	Sean Sullivan
☐	Bruce Wilson	☐	Jonathan Espenschied	☐	Monica Thakar
☐	Shawn Akavan	☐	Armen Khatchatourian	☐	Donna Sullivan
☐	Petra Eichelsdoerfer	☐	Thomas May	☐	Robyn Williams
☐	Shea Wilson	☐	Rebecca Owen	☐	Judy Zerzan
☐	Kerrie Fowler	☐	Yusuf Rashid	☐	Mike Bonetto
☐	Foxy Williams	☐	Meghan Gallagher	☐	Danielle Walters
Main Outcome: Potential funding and payment options for HCA to purchase emerging threapies.					

No	Agenda Items	Time	Lead	Summary Meeting Notes
1.	Welcome Meeting summary/ expectations	10	Mike Bonetto	
2.	State Medicaid budget overview (impact of high cost drugs and pipeline – policy trade-offs)	15	Robyn Williams, OFM	
3.	• HCA HCV Elimination- greater population solution • Overview of other States centralized purchasing	20	Donna Sullivan, HCA	
4.	Summary of funding options	40	Sean Sullivan, UW Carly Rodriguez, Moda Health	
5.	BREAK	10		
6.	Assessing the value of treatments and sustainable payment model	20	Meghan Gallagher, Bluebird Bio	
7.	Pharma questions: In our shoes what would you do? How would you address problem?	20	Manufacturers	
8.	Workgroup- viable options?	40	Workgroup	
9.	Next steps, next meeting	5	Mike Bonetto	

	Action Items/Decisions				
#	Action Item	Assigned To:	Date Assigned:	Date Due:	Status

Washington State Health Care Authority

Emerging Therapies Workgroup Charter

I. Background

During the 2019 legislative session, House Bill (HB) 1869 was introduced which would have created an emerging therapies workgroup to analyze prospective and emerging therapies and their impacts on patients in Washington State. The WA Health Care Authority (HCA) appreciates the high level of interest in these new therapies from the medical community and patient groups and has established this workgroup in order to gather important feedback and insight from key stakeholders.

This charter shall expire on June 30, 2020 or when HCA determines that the charter has been fulfilled.

II. Scope

The Emerging Therapies Workgroup is charged with providing input to HCA on the following topics:

- Long-term funding for emerging treatments.
- Potential funding options between manufacturers and the state, such as value-based purchasing and financing options.
- Different payment options between the state and managed care organizations.
- Quality oversight and outcome tracking of providers and facilities administering the emerging therapies.
- Management of patients eligible for emerging therapies with consideration of the benefit and cost to the overall state budget.
- Potential improvements to health outcomes and quality of life for patients and concerns about safety and efficacy.
- Potential long-term savings or expenditures to the state.
- Metrics that could be used to measure the fiscal and health impacts of emerging therapies.

HCA staff will provide workgroup members materials in advance of scheduled meetings in order to ensure adequate review time and meaningful input.

The workgroup will seek input from other subject matter experts as necessary.

The workgroup will prioritize three to five key immediate action steps based on its analysis and discussion.

The workgroup will make recommendations to HCA leadership. Final decision-making authority resides with HCA.

III. Schedule

The workgroup will hold four meetings over 12 months between June 1, 2019, and June 30, 2020. The first meeting is June 18th, 2019, and will be held in Spokane, Washington. The second meeting will be held on a day to be decided in late October 2019 at HCA in Olympia, 2/19/2020 and 4/15/2020 will be held at the Conference Center at SeaTac Airport.

Topics to be covered:

June 2019 Meeting | LEVEL SETTING

- Overview of new therapies coming to market
- Private sector perspective on managing emerging therapies
- HCA/Medicaid perspective on managing emerging therapies
- Current financing

October 2019 Meeting | CASE EXAMPLES

- Patient decision aids
- Patient experience
- Patient advocates present

February 2020 Meeting | FUNDING OPTIONS

- Long-term funding for emerging therapies
- Potential funding options between manufacturers and the state
- Different payment options between the state and managed care organizations

April 2020 Meeting | QUALITY OVERSIGHT

- Potential improvements and harms to health outcomes and quality of life for patients
- Quality oversight and outcome tracking of providers and facilities administering emerging therapies
- Metrics that could be used to measure the fiscal and health impacts of emerging therapies
- Potential long-term savings and expenditures to the state

IV. Work Group Membership

Workgroup members will be appointed by HCA and include representatives from various medical fields, along with representatives from managed care organizations and patient advocacy organization.

Chair: Judy Zerzan, MD, MPH – Chief Medical Officer: HCA

V. Staff Resources

Leta Evaskus – NW Prescription Drug Consortium Operations Manager: HCA

Leta.Evaskus@hca.wa.gov

206-521-2029

Mike Bonetto – Facilitator: Center for Evidence-based Policy, OHSU

mbonetto@tenfoldhealth.com

541-678-3204

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Emerging Therapies Workgroup Roster

Employee and Retiree Benefit (ERB) Plans

- Cody Gillenwater, MD, Regence, Associate Medical Director
- Carly Rodriguez, PharmD, Moda Health, Pharmacy Director, Clinical Innovation
- Bruce Wilson, MD, Kaiser P&T Committee Chair

Medicaid Managed Care Organizations (MCO)

- Shawn Akavan, MD, Amerigroup
- Petra Eichelsdoerfer, RPh, MS, ND, UnitedHealthCare, Pharmacist Account Manager
- Kerrie Fowler, PharmD, Coordinated Care, Senior Pharmacy Director
- Yusuf Rashid, RPh, Community Health Plan of Washington, Vice President of Pharmacy and Vendor Relationship Management
- Shea Wilson, PharmD, Molina Healthcare of Washington

Patient Advocates

- Stephanie Simpson, Bleeding Disorder Foundation of WA
- Melissa Tribelhorn, NW Parkinson's Foundation, Executive Director
- Foxy Williams, Seattle Sickle Cell Task Force

Subject Matter Experts (SME)

- Jonathan Espenschied, MD, WA State University, Associate Dean, GME and CME, Physician
- Armen Khatchatourian, PharmD, MBA, HEOR, OptumRx, Senior Director, Industry Relations & Formulary Consulting, Pharmacist
- Thomas May, PhD, WA State University, Medical Ethicist
- Rebecca Owen, FSA MAAA, HCA Solutions, Pharmacy Actuary
- Jean-Baptiste Roullet, PhD, WA State University, Rare Disease Researcher
- Sean Sullivan, PhD, University of WA, Dean, School of Pharmacy, Healthcare Economics
- Monica Thakar, MD, Fred Hutchinson, Gene-based Therapy

WA State Agencies Representatives

- Donna Sullivan, PharmD, MS, Health Care Authority, Chief Pharmacy Officer
- Robyn Williams, Office of Financial Management, Budget Analyst
- Judy Zerzan, MD, MPH, Health Care Authority, Chief Medical Director

February 19, 2020

Medicaid Budget Overview

Robyn Williams

OFM
OFFICE OF FINANCIAL MANAGEMENT

19-21 Enacted Budget

HCA total budget ~$5.8 Billion GFS ($21.2 Billion total)

- Medicaid Physical Health ~$4.6 Billion GFS ($17.7 Billion total)
  - Managed Care Rates ~$2.5 Billion GFS ($8.7 Billion total)
  - Drugs ~$1.0 Billion GFS ($3.3 Billion total)
- Community Behavioral Health ~$1.2 Billion GFS ($3.2 Billion total)
- PEBB/SEBB ~$222 Million total
- Health Benefit Exchange ~$12 Million GFS ($121 Million Total)

Medicaid Budgeting Process

Expenditures

Actual expenditures collected for time period. Feeds into both Managed Care rate setting and utilization forecast.

Managed Care Rates

Medicaid Managed Care rates are updated annually. Prior period actual expenditures used in actuarial analysis to set future rates.

Forecast

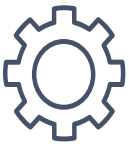
Forecast is updated twice annually. Includes updates to trends based on actual expenditures and projections based on new rates.

Budget

The governor proposes his budget in December using fall forecast. The legislature enacts budget using February forecast.

Timing Challenges

Expenditures



Managed Care Rates

CY 2020 rates use data from CY 2018

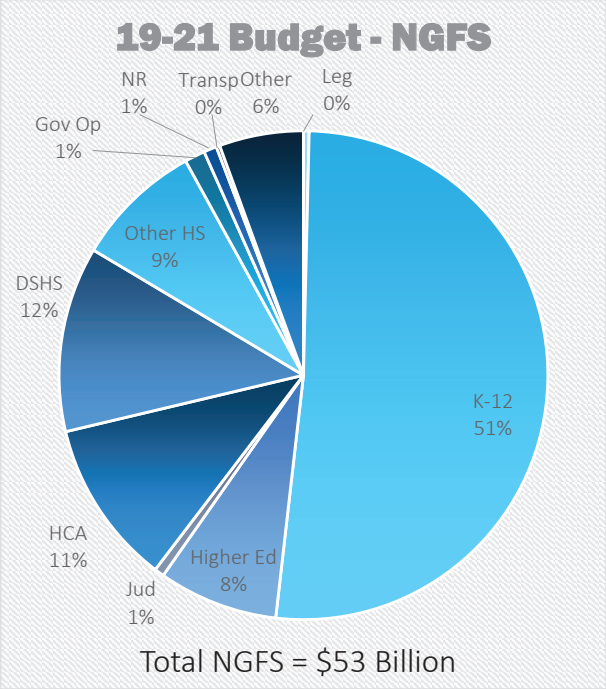
Forecast

February FC uses data through July 2019

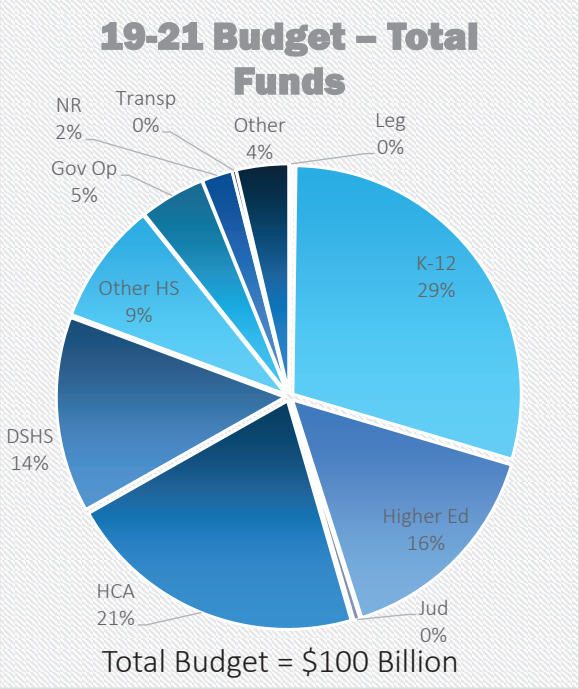
Budget

2020 Supplemental will update budgets FY20-FY21 (July 2019 – June 2021)

State Budget Overview



Total NGFS = $53 Billion



Total Budget = $100 Billion

FOR MORE INFORMATION:

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ofm.wa.gov

OFM
OFFICE OF FINANCIAL MANAGEMENT

State Strategies to Address Access and Cost

Donna L. Sullivan, PharmD, MS
Chief Pharmacy Officer

Washington State Health Care Authority

State Strategies to Balance Access and Cost

- Pharmacy Benefit Management
- Multi-Agency Purchasing
- Alternative Payment Approaches
- Affordability Approaches

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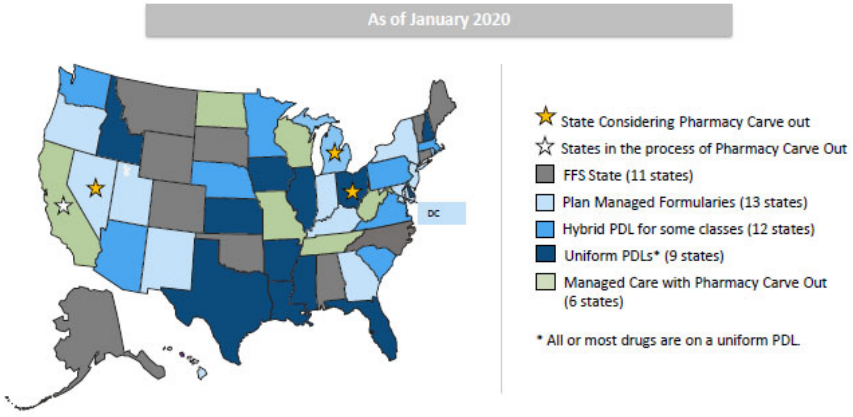
Medicaid Pharmacy Benefit Management



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Multi-Agency Purchasing

- States are looking to leverage purchasing power across agencies (Medicaid, state employees, corrections, and others)
- Attempts to increase market leverage by aggregating covered lives of more than one state program
- Notable examples:
  - California
  - Massachusetts
  - Oregon
  - Washington
  - Many others exploring possible pathways

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Alternative Payment Approaches

- Outcomes-based arrangements – Link payment to an agreed up on performance metric
  - Colorado
  - Michigan
  - Oklahoma
- Finance-based arrangements – Link payment to price/volume agreements, market share or utilization
  - Louisiana
  - Washington

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Affordability Approaches

- Strategies that recognize access needs while also addressing budget constraints
- Approaches to date”
  - Spending caps or thresholds (New York and Massachussets)
  - Affordability boards or councils (Maryland and Maine)
    - Rate-setting or ceiling prices

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Other Strategies

- Importation
- Manufacturing
- Price Transparency

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Questions?

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Assessing the Value and Affordability of Gene/Cell Therapy

Sean D. Sullivan, PhD
Professor and Dean
School of Pharmacy

UNIVERSITY of WASHINGTON

Topics

- **Why gene and cell therapies are different and why this matters for health systems?**
- How has ICER evaluated the clinical and economic value of gene and cell therapies?
- Emerging Financing Models for High Cost Treatments

Why are they different?

- Non-traditional PhRMA disease targets.
  - Small populations, rare and ultra rare disease, genetic targets, incurable or treatment resistant cancers
- FDA shift: Open to clinical research studies that
  - Do not follow the traditional model of two placebo-controlled, RCTs
  - Reliance on biomarkers (e.g., ORR) rather than traditional outcomes
  - Encourage early market introduction (faster access for patients)
- Might CURE cancers and genetic disorders or reset the immune-system to fight neurologic conditions for which we have few options (AD, MS, MD, PD)

Why does it matter to health systems?

- May soon have treatments that cure devastating diseases
  - Impact on population health might be substantial
  - Impact on families and caregivers may be significant
- The prices are astronomical
  - Costs = price * quantity – so the budget impact may be modest
  - We have the first $2.0+ million dollar therapeutic – a single dose gene therapy for SMD Type 1
- The cumulative impact on insurance premiums is not trivial and affordability of health insurance for small to medium size groups is at risk.

Topics

> Why gene and cell therapies are different and why this matters for health systems?

> **How has ICER evaluated the clinical and economic value of gene and cell therapies?**

> Emerging Financing Models for High Cost Treatments

ICER's Aim:
Fair price, Fair access, Future innovation

- **Vision**
  - A health system that engages with all stakeholders to use evidence to sustain innovation while guaranteeing all patients access to affordable high-value care
- **Mission**
  - To analyze evidence on the benefits and costs of health system interventions with the input of all stakeholders
  - To catalyze stakeholder and public engagement in discussions regarding the application of evidence to medical care, pricing, and insurance coverage policy
  - To improve the generation, interpretation, and application of evidence throughout the health system

ICER's Value-based Price Benchmarks (2018-2019)

Drug category	Minimum Recommended Discount*
Luxturna for childhood blindness	50%
Kymriah (CAR-T) for ALL	No discount
Yescarta (CAR-T) for NHL	No discount
Hemlibra for hemophilia A	Cost-saving
Cystic Fibrosis	72%
CGRPs for migraine prevention	25%
Elagolix for endometriosis	No discount

Drug category	Minimum Recommended Discount*
Apalutamide for prostate cancer	No discount
Psoriasis IL-23s and Cimzia	57%
Inotersen, patisiran (amyloidosis)	90%
Hereditary Angioedema	28%
Opioid Use Disorder (new agents)	53%
Eosinophilic asthma biologics	62%
Spinraza	83%

** Discount from launch list price (or post-rebate price if in the market) needed to achieve $150,000/QALY, the highest standard threshold for cost-effectiveness.*

ANALYTIC APPROACH OF ICER IN SMA

Interventions

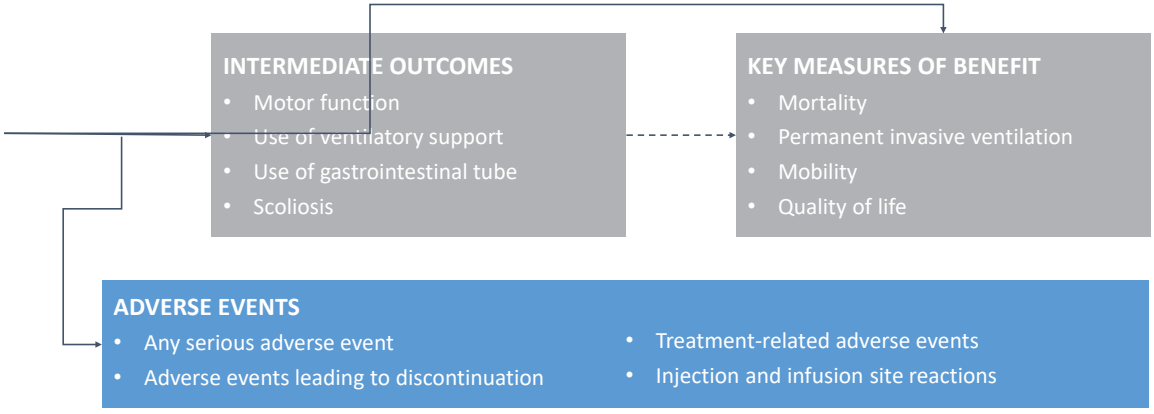
- Onasemnogene abeparvovec
- Nusinersen

Comparators

- Supportive care

Population

Infants, children, and adults with SMA



- ICER Spinraza® and Zolgensma® for Spinal Muscular Atrophy: Effectiveness and Value (May 2019): Page 9. https://icer-review.org/wp-content/uploads/2018/07/ICER_SMA_Final_Evidence_Report_052419.pdf. Last accessed 22nd August 2019

ICER EVIDENCE RATINGS FOR SMA TREATMENTS

The New England CEPAC voted **unanimously to confirm** the adequacy of onasemnogene abeparvovec evidence vs. best supportive care

Although some Council members recognized the smaller evidence base of onasemnogene abeparvovec in comparison to that of nusinersen, overall, they argued that the magnitude of clinical benefit was persuasive.
(ICER SMA Final Evidence Report Pg. 106)

The Council voted **unanimously** that the **evidence was inadequate** to distinguish the net health benefit between nusinersen and onasemnogene abeparvovec

Council members noted the lack of head-to-head studies and that the existing trials were not comparable.
(Pg. 106)

Differences in trial populations related to age at treatment initiation and disease duration limit our ability to adequately distinguish the net health benefit of onasemnogene abeparvovec versus nusinersen for infantile onset SMA.
(Pg. 47)

Population	Nusinersen	Onasemnogene abeparvovec	Ability to distinguish?
Type 0 SMA	I*	I*	I§
Type I SMA	A	A	I
Type II/III SMA	B+	I*	I§
Type IV SMA	I*	I*	I*
Presymptomatic SMA	B*	I*	I§

I - insufficient
* No studies (e.g RCTs, observational) identified.
§ Comparison is based on lack of available evidence for onasemnogene abeparvovec

- BSC, best supportive care; ICER (value), Incremental Cost Effectiveness Ratio; ICER (organization), Institute for Clinical and Economic Review;
- QALY, quality adjusted life year; SMA, spinal muscular atrophy, CEPAC, Comparative Effectiveness Public Advisory **Council**
- ICER Spinraza® and Zolgensma® for Spinal Muscular Atrophy: Effectiveness and Value (May 2019): https://icer-review.org/wp-content/uploads/2018/07/ICER_SMA_Final_Evidence_Report_052419.pdf. Last accessed 22nd August 2019

ICER – ECONOMIC EVALUATION OF THREE TREATMENT OPTIONS IN SMA TYPE I

US thresholds are $100,000 – $150,000 per QALY, however for rare diseases ICER notes that payers may consider thresholds up to $500,000 per QALY

At a $2 million placeholder price, onasemnogene abeparvovec has a substantially more favorable ICER *(value)* than nusinersen

	Best Supportive Care (BSC)	Nusinersen	Onasemnogene abeparvovec	Nusinersen vs. BSC	Onasemnogene abeparvovec vs. BSC
Total Costs	$789,000	$3,884,000	$3,657,000	$3,095,000	$2,684,000
Total Life Years (LY) Gained	2.40	7.64	18.17	5.24	15.77
Total QALYs Gained	0.46	3.24	12.23	2.78	11.77
ICER (Cost/QALY)	--	--	--	$1,112,000	$243,000

- BSC, best supportive care; ICER (value), Incremental Cost Effectiveness Ratio; ICER (organization), Institute for Clinical and Economic Review;
- QALY, quality adjusted life year; SMA, spinal muscular atrophy
- ICER Spinraza® and Zolgensma® for Spinal Muscular Atrophy: Effectiveness and Value (May 2019): https://icer-review.org/wp-content/uploads/2018/07/ICER_SMA_Final_Evidence_Report_052419.pdf. Last accessed 22nd August 2019

THEORETICAL COST EFFECTIVENESS OF ONASEMNOGENE ABEPARVOVEC IN PRE-SYMPTOMATIC SMA

In a hypothetical pre-symptomatic SMA scenario analysis, onasemnogene abeparvovec (i.e. Drug X) had a cost per QALY of approximately $157,000 with a $2M placeholder price

This hypothetical was a scenario analysis, not a base case analysis

ICER assumed Drug X had the one-time costs of onasemnogene abeparvovec with the unrelated healthcare costs, QALYs and LYs associated with nusinersen in presymptomatic SMA patients

	BSC	Drug X*	Drug X vs. BSC
Total Costs	$801,000	$3,264,000	$1,417,000
Total Life Years Gained	9.51	26.58	12.12
Total QALYs Gained	6.25	21.94	10.22
ICER (Cost/QALY)	--	--	$157,000

In contrast, the ICER for nusinersen versus BSC in the pre-symptomatic population was $709,000 per QALY gained

- BSC, best supportive care; ICER (value), Incremental Cost Effectiveness Ratio; ICER (organization), Institute for Clinical and Economic Review;
- QALY, quality adjusted life year; Lys life year; SMA, spinal muscular atrophy
- ICER Spinraza® and Zolgensma® for Spinal Muscular Atrophy: Effectiveness and Value (May 2019): https://icer-review.org/wp-content/uploads/2018/07/ICER_SMA_Final_Evidence_Report_052419.pdf. Last accessed 22nd August 2019

THREE MAIN CHALLENGES IN ESTIMATING THE VALUE OF GENE THERAPIES

Aspects of value not captured by QALYs

Social and ethical aspects of the implications of discounting

Uncertainty around the future costs and benefits of GT

- GT, gene therapy; QALY, quality adjusted life year; SMA, spinal muscular atrophy
- Drummond M et al. Value in Health. 2019;22(6):661-668

Potential problem with a diagnostic to identify a genetic disorder in cancer – an economics perspective

- When testing for a genetic mutation, if the prevalence of that mutation varies widely across tumor types, clinical epidemiology tells us that for a given level of sensitivity and specificity, the predictive value of a test will be lower for tumors where the prevalence of the mutation is low.

- Without rigorous, standardized and validated testing approaches, high proportions of patients may be mislabeled as having the genetic alteration and treated accordingly, with attendant adverse consequences and costs for the individual and the health care system.

An Example

- Consider a hypothetical single-arm basket trial of patients with lung, prostate, and thyroid cancer who have Mutation A (M-A). All are treated with a targeted agent. The agent shows activity across all tumor types and is FDA approved, with a tumor-agnostic label. The prevalence of the mutation varies widely: from 1% in lung cancer to 20% in thyroid cancer.

- A companion diagnostic test is developed to identify patients with M-A. The test is 99% sensitive and 95% specific for the mutation. The table on the next slide shows the prevalence of the mutation among the tumors of interest, and the positive predictive value of the test for each tumor type.

Table. Positive predictive value ranges for a genomic test with 99% sensitivity and 95% specificity for mutation M-A at varying levels of mutation prevalence across tumor types.

Cancer type	Nonsmall Cell Lung	Prostate	Thyroid
Prevalence of Mutation A	**1%**	**10%**	**20%**
Population	1,000	1,000	1,000
Mutation	10	100	200
No mutation	990	900	800
True Positives (TP)	10	99	198
False Positives (FP)	50	45	40
TP + FP	60	144	238
PPV	**17%**	**69%**	**83%**

The positive predictive value for the diagnostic test after accounting for the underlying prevalence of disease ranges from 17% for lung to 83% for thyroid cancer. In other words, 83% of positive test results are wrong for lung cancer patients and 17% of positive results are wrong for thyroid cancer patients.

Ref: Ramsey SD, Shankaran V and Sullivan SD. JCO June 24, 2019

Topics

- Why gene and cell therapies are different and why this matters for health systems?
- How has ICER evaluated the clinical and economic value of gene and cell therapies?
- **Emerging Financing Models for High Cost Treatments**

INNOVATIVE PAYMENT MODELS FOR GT and CT

Annuity
Payments are spread out and made over a defined period of time, from months to years

Direct payment
Payer purchases the gene therapy directly from the manufacturer

Consumer mortgage
A loan taken out directly by the patient

Expanded risk pools
Dedicated fund where several stakeholders allocate a proportion of their premiums or budget toward an expanded group of patients who are eligible for gene therapy

Discounts and revenue caps
Therapy price is reduced by a percentage if total revenue exceeds a certain target

Patient assistance/subsidy
Financial aid for patients for their expenses related to gene therapy

Re-insurance
Catastrophic insurance coverage purchased for gene therapy

Supplier credit
Gene therapy is purchased by a third-party financial entity that negotiates payment with payer

Pay for performance
Innovator companies are paid for their gene therapies based on mutually agreed upon timed measures and achievements of clinical performance or outcomes in the real world

- GT, gene therapy
- Adapted from: https://pharmaintelligence.informa.com/~/media/informa-shop-window/pharma/whitepapers/dmhc-gene-therapy-whitepaper.pdf

THANK YOU

SCHOOL OF PHARMACY
UNIVERSITY of WASHINGTON

The Payer Perspective on Gene and Cell Therapies

Carly Rodriguez, PharmD, FAMCP
Pharmacy Director, Clinical Innovation

Moda Health

moda

What are cellular and gene therapies? [1,2]

Definitions

- **Cellular therapy:** transfers whole, live cells (modified or unmodified) to produce an immune or other biological response
  - Cellular immunotherapies
  - Cancer vaccines
  - Autologous and allogeneic cells (e.g. hematopoietic and embryonic stem cells)
- **Gene therapy:** uses genetic material to modify or manipulate the expression of a gene, or alters the biological properties of living cells for therapeutic use

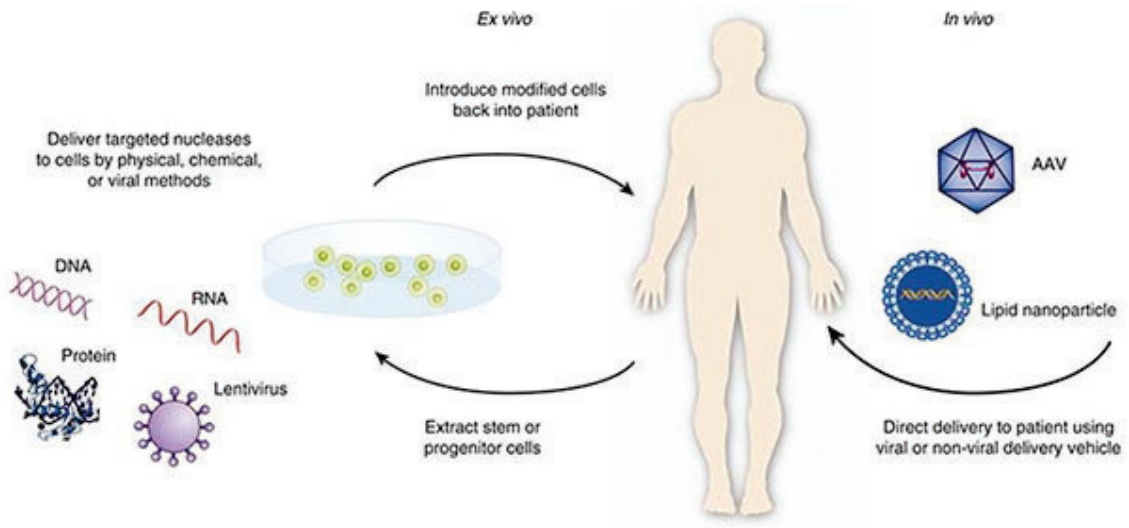
1. U.S. Food & Drug Administration. Center for Biologics Evaluation and Research (CBER): Cellular & Gene Therapy Products. Available from: https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products.
2. American Society of Cell & Gene Therapy. Gene Therapy vs. Cell Therapy. Available from: https://annualmeeting.asgct.org/about_gene_therapy/genevscell.php.

Comparing Gene-based Therapies [1]

- **Gene replacement therapy:** gives cells a new, working copy of a missing or non-working gene
  - Uses a vector to deliver gene to specific cells
- **Gene editing:** inserts, removes, changes, or replaces specific pieces of a person's existing DNA
  - CRISP-R
- **CAR-T cell therapy:** modifies a person's own immune cells to recognize and fight cancer cells inside the body
  - Used with gene-based therapies (i.e. CAR gene)

1. AveXis, Inc. Explore Gene Therapy. Get to Know the Different Types of Gene-Based Therapies. Available from: https://www.exploregenetherapy.com/how-gene-replacement-therapy-is-different

Gene-based Therapy Methods[1]



1. U.S. Food & Drug Administration. Center for Biologics Evaluation and Research (CBER). Cellular & Gene Therapy Products: What is Gene Therapy? Available from: https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/what-gene-therapy.

Current marketplace & emerging therapies

moda

Cellular therapies

Currently approved products

Hematopoietic Progenitor Cells (HPC), Cord Blood	• Unrelated donor stem cell transplantation • Eight products available; Treatment-center developed
Chimeric Antigen Receptor T cells (CAR-T)	• tisagenlecleucel (Kymriah) – B-cell precursor ALL and B-cell lymphoma • axicabtagene ciloleucel (Yescarta) – B-cell lymphoma
Immunotherapy	• talimogene laherparapvec (Imlygic) – recurrent melanoma • sipuleucel-T (Provenge) – castration-resistance prostate cancer
Other	• Gintuit (keratinocytes + fibroblasts) – topical dental wound treatment • azficel-T (Laviv) – improvement of nasolabial folds (“smile lines”) • MACI (autologous cultured chondrocytes) - knee repair

Cellular therapies

Pipeline

Drug	Manufacturer	Category	Indication	Route
aglatimagene besadenovec	Advantagene	Immunotherapy	Prostate cancer	Ultrasound-guided injection
idecabtagene vicleucel	Bluebird Bio / Celgene	CAR-T	Multiple myeloma	IV
lisocabtagene maraleucel	Celgene	CAR-T	DLBCL	IV

Gene therapies

Currently approved products

voretigene neparvovec-rzyl (Luxturna)

- Approved December 2017
- Treatment of bi-allelic RPE65 mutation-associated retinal dystrophy

onasemnogene abeparvovec-xioi (Zolgensma)

- Approved May 2019
- Treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the survival motor neuron 1 (SMN1) gene

Gene therapies

Pipeline

Drug	Manufacturer	Indication	Route	Anticipated Approval
valoctocogene roxaparvovec	BioMarin	Hemophilia A	IV	2020
DTX-201	Ultragenyx/ Bayer	Hemophilia A	IV	2022+
giroctocogene fitelparvovec	Sangamo/ Pfizer	Hemophilia A	IV	2022+
SPK-8011	Spark	Hemophilia A	IV	2022+
TAK-754	Takeda	Hemophilia A	IV	2022+
etranacogene dezaparvovec	uniQure	Hemophilia B	IV	2020
fidanacogene elaparvovec	Spark/Pfizer	Hemophilia B	IV	2021

Gene therapies

Pipeline - Continued

Drug	Manufacturer	Indication	Route	Anticipated Approval
eladocagene exuparvovec	PTC Therapeutics	AADC Deficiency	Intracerebral injection	2020
beta beglogene darolentivec	Bluebird Bio	Beta thalassemia	IV	2020
elivaldogene tavalentivec	Bluebird Bio	Cerebral ALD	Intracerebral infusion	2020
PF-06939926	Pfizer	DMD	IV	2021
SRP-9001	Serepta	DMD	IV	2021
nadofaragene firadenovec	Ferring	Bladder cancer	Intravesical	2021

Payer strategies

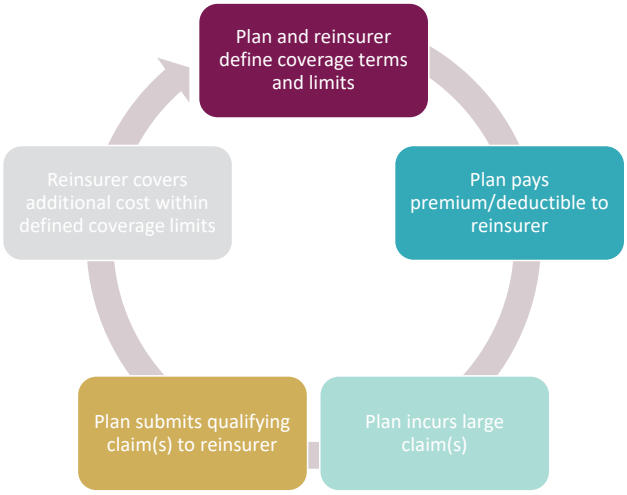
Managing market entry, access, and cost

moda

Fundamentals

- Prior authorization:
  - Member qualification where evidence has demonstrated safety and efficacy
    - May differ from FDA label
  - Manage pre- and post-treatment options
    - Example: Spinraza before or after Zolgensma
  - One-time treatment per lifetime
  - In-house management vs. contracted vendors
- Billing and coding management
  - Interdepartmental communication
  - Example: 1 billion vector units

Stop-Loss / Reinsurance Programs



Pay Over Time – Zolgensma [1,2]

- List price: $2.125 million
- Pay over time model:
  - $425,000 per year x 5 years
  - May be coupled with outcomes-based agreements
- Challenges:
  - Lump some payment advantageous when stop loss/re-insurance in play
  - Significant financial risk remains on the plan

1. Medscape. FDA OKs First Gene Therapy for Spinal Muscular Atrophy. May 24, 2019. Available from: https://www.medscape.com/viewarticle/913505.
2. Novartis. AveXis Announces Innovative Zolgensma® Gene Therapy Access Program for US Payers and Families. May 24, 2019. https://www.novartis.com/news/media-releases/avexis-announces-innovative-zolgensma-gene-therapy-access-programs-us-payers-and-families.

Cigna/ESI/eviCore – Embarc Benefit Protection [1]

- Pre-paid program that covers the cost of Luxturna and Zolgensma
  - More products may be added later
- Eliminates out-of-pocket payments for employers and unions
- Few details known:
  - Available to health plans, payers, employers, etc.
  - In-network gene therapy provider
  - PMPM rate paid to eviCore to participate in the gene therapy network
    - Rumored to be ~ $1.00 PMPM regardless of covered lives

1. Reuters Health News. Cigna rolls out new plan to fully cover multi-million dollar gene therapies. September 5, 2019. Available from: https://www.reuters.com/article/us-cigna-gene-therapy-idUSKCN1VQ1GA.
2. HealthPayer Intelligence. Cigna uses industry consolidation to increase access to gene therapy. September 9, 2019. Available from: https://healthpayerintelligence.com/news/cigna-uses-industry-consolidation-to-increase-access-to-gene-therapy.

Embarc Benefit Protection - Challenges

- May not make financial sense depending on plan size
  - If $1.00 PMPM rumor is true
  - Buy-and-bill advantageous for some gene therapy scenarios
- Unclear what utilization management requirements exist
  - Must follow ESI prior authorization guidelines?
  - What if therapy is approved by an Independent Review Organization?

Outcomes-Based Arrangements [1,2]

- Manufacturer backs clinical trial performance of therapy
  - Defined outcomes
  - Rebate or incremental rebate if/when defined outcome occurs
- Advantages:
  - Partially addresses unknowns in efficacy, durability of response
- Challenges:
  - Substantial financial risk still remains on the plan
  - Unknown implications for future generations
  - Operational challenges: member turnover, provider-controlled requirements

1. Spark Therapeutics. Spark Therapeutics Announces First-of-their-kind Programs to Improve Patient Access to LUXTURNA™ (voretigene neparvovec-rzyl), a One-time Gene Therapy Treatment. January 3, 2018. Available from: https://www.reuters.com/article/us-cigna-gene-therapy-idUSKCN1VQ1GA.
2. Managed Healthcare Executive. Increasing Access to Gene Therapies Requires Innovative Payment Approaches. September 23, 2019. https://www.managedhealthcareexecutive.com/pharma-forecast-report/increasing-access-gene-therapies-requires-innovative-payment-approaches.

Preferred Treatment Sites [1]

- Manufacturers may contract with select treatment centers that can provide therapy or perform procedure
- Payers may contract with a subset of those treatment centers
- Advantages:
  - May have advantageous reimbursement terms
  - Consistent quality of service and care
    - Multi-disciplinary support team
    - Clinical trial experience
- Challenges:
  - Patient and family travel
  - Network disruption

1. Spark Therapeutics. Generation Patient Services. Available from: https://mysparkgeneration.com/hcp-support.html#TreatmentCenters.

Questions?

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Washington Health Care Authority Emerging Therapies Workgroup

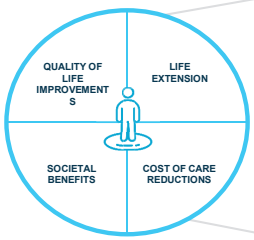
February 19, 2020

Meghan Gallagher and Danielle Walters

NASDAQ: BLUE

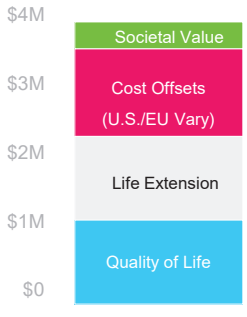
Value for money and price-setting
-the proposed bluebird shared value approach

THE VALUE AT WHICH TREATMENT IS COST EFFECTIVE* (NOT PRICE)

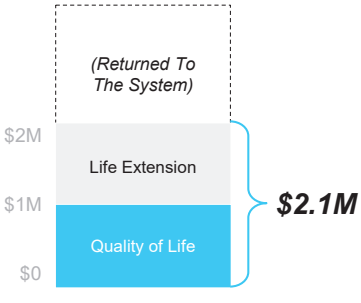


- Clinical Trial Data
- Outcomes Research
- Real World Evidence
- Economic Modeling

Traditional
All Inclusive Calculation



LentiGlobin
Intrinsic Value



LentiGlobin pricing for the EU and US will not exceed the products' intrinsic value.

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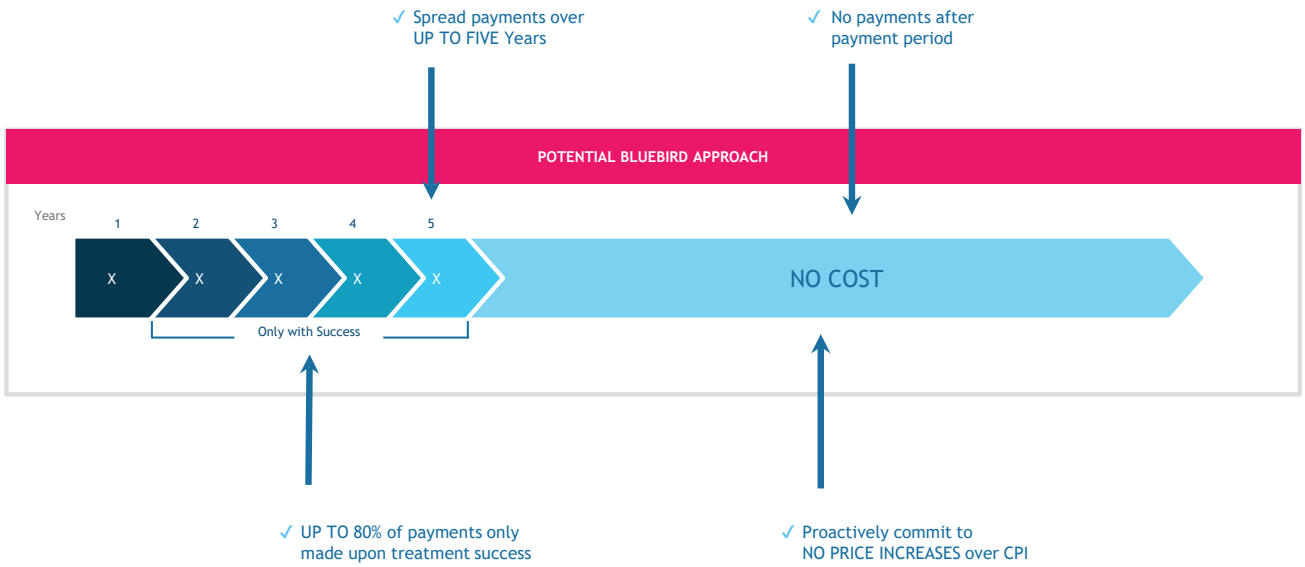
**We have quantified the impact on patient quality of life, survival, treatment cost and society using established modeling techniques*

Our proposed pricing principles

	OBJECTIVE	STRATEGIC APPROACH
1	FAIR VALUE RECOGNITION	✓ Lifetime cost effectiveness time frame ✓ Base value only on patient QOL and Life Extension
2	SHARED RISK	✓ Pay ONLY IF the treatment works ✓ Put UP TO 80% of the price at risk based on success
3	PER PATIENT AFFORDABILITY	✓ Spread payments over UP TO A FIVE YEAR period ✓ NO PRICE INCREASES above CPI
4	HEALTH SYSTEM AFFORDABILITY	✓ NO COST after payment period (vs. for life) ✓ Recode system to catalyze change

Potential bluebird bio approach: sharing risk and value with the system



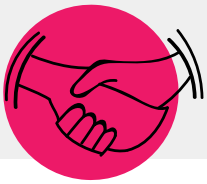
thank you

recode the status quo

A BOLD AND BALANCED PATH TO A SUSTAINABLE PAYMENT MODEL FOR GENE THERAPIES

It is bluebird bio's goal to create a sustainable model to pay for one-time gene therapies so that patients, health systems and society can realize the therapy's potential lifelong value.

we are committed to value-based payments over time, focused on direct clinical benefit for the patient.



We propose **sharing risk with health care systems** to create a sustainable model.

We are willing to put as much as **80 percent** of the price at risk.

After an initial payment of 20 percent, annual milestone payments would be made *only if* the treatment works, defined by **easy-to-measure outcomes** that assess meaningful patient benefit.

A one-time treatment intended to have lifelong benefit, annual milestone payments would be **capped at 5 years** of equal installments.

Fair value tied to direct patient benefit: **living longer** and **quality of life improvements.** Savings associated with treatment prior to therapy would be returned to health systems and society.

this model will help achieve a goal shared by both bluebird and health care systems:

MAXIMIZING PATIENT ACCESS & SYSTEM AFFORDABILITY

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