



Emerging Therapies Workgroup Overview

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Dr. Judy Zerzan, Medical Director, HCA

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

Workgroup's Scope

- ▶ Workgroup members were appointed by HCA and included representatives from various medical fields, along with representatives from managed care organizations and patient advocacy organization.
- ▶ Workgroup was charged with helping HCA identify concerns, ethics, and perspectives around emerging therapies.
- ▶ Issues related to Emerging Therapies:
 - ▶ Long-term funding
 - ▶ Quality oversight and outcome tracking
 - ▶ Management of patients eligible for emerging therapies
 - ▶ Potential improvements to health outcomes and quality of life
 - ▶ Potential long-term savings or expenditures to the state
 - ▶ Metrics that could be used to measure the fiscal and health impacts

Meeting Schedule

June 18, 2019	<ul style="list-style-type: none"> • Overview of new therapies coming to market • Private sector perspective on managing emerging therapies • HCA/Medicaid perspective on managing emerging therapies • Current financing 	Invitation only
October 18, 2019	<ul style="list-style-type: none"> • Patient decision aids • Patient experience • Patient advocates present 	Invitation only
December 18, 2019	<ul style="list-style-type: none"> • Summary of the workgroup's progress so far • Stakeholder questions and comments 	Open public meeting
February 19, 2020	<ul style="list-style-type: none"> • Long-term funding for emerging therapies • Potential funding options between manufacturers and the state • Different payment options between the state and managed care organizations 	Open public meeting
April 15, 2020	<ul style="list-style-type: none"> • 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 	Invitation only
June 22, 2020	<ul style="list-style-type: none"> • Options for drug coverage policy makers when there is a lack of evidence • Tracking outcomes: Patient registry, provider registry 	Invitation only
November 3, 2020	<ul style="list-style-type: none"> • Summary of the workgroup's meeting topics and discussions • Stakeholder questions and comments 	Open public meeting

Recap of Workgroup Topics

Growth in Specialty Drugs

2008

About 10 years ago, specialty medicines accounted for **24.7%** of total pharmacy spending

2017

Today, they contribute to **46.5%** of total pharmacy spending, but only **~2% of prescriptions**

Pipeline

▶ Breakthrough specialty drugs

- Certain types of cancer
- Cystic Fibrosis
- Duchenne Muscular Dystrophy
- Spinal Muscular Atrophy
- Nonalcoholic Steatohepatitis
- Blindness (neovascular age-related macular degeneration)
- Hemophilia
- Alzheimer's disease
- Certain neurologic diseases

Pipeline (cont.)

▶ Of the drugs now in Phase III trials...

- 60% are specialty drugs
- 33% are orphan drugs
- 13% are considered breakthrough therapies
- Only 8% are biosimilars

▶ Of the applications submitted to the FDA...

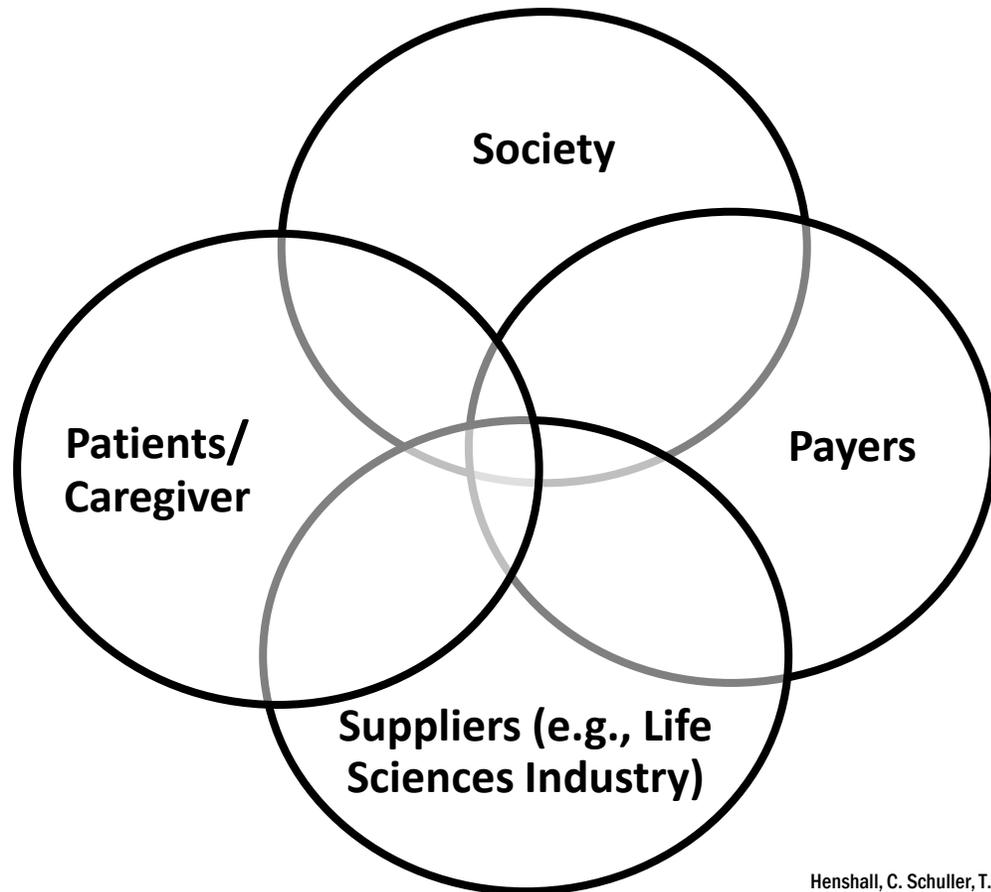
- 25% of new drug applications submitted to the FDA have been granted “priority review”

Current Approaches to Determining Coverage

Similarities between Commercial Insurers and WA Health Care Authority:

- ▶ Evaluation of evidence
- ▶ Budget/Financial impact analysis
- ▶ Development of clinical criteria
- ▶ Tracking outcomes

Decisions are Complex and Affected by Perspective



- ▶ What the decision-makers value and how they value it is a complex inter-relationship of what matters within the organization and who they serve.
- ▶ The decision-making criteria are shared though elements of it may be weighted or valued differently.

Henshall, C. Schuller, T., **Health Technology Assessment, Value-based Decision Making and Innovation.**, International Journal of Technology Assessment in Health Care, 29:4 (2013), 353-359.

Workgroup's Discussion

▶ Cost, Quality & Access

- ▶ What are the levers we have?
- ▶ Reinsurance options with orphan drugs
- ▶ Best price implications
- ▶ Societal impact
- ▶ Outcome-based contracts
- ▶ Long term side-effects
- ▶ Cure vs. maintenance (patient perspective -- disease by disease)
- ▶ Patient decision aids
- ▶ Outcome registry
- ▶ Provider education
- ▶ Informed consent
- ▶ Alternative treatments

Ethical Considerations for Emerging Therapies

- ▶ Health payer policy decisions
- ▶ Personal patient decisions
- ▶ Medical necessity
- ▶ Process of health care decision-making
- ▶ Benefits & harms of treatment

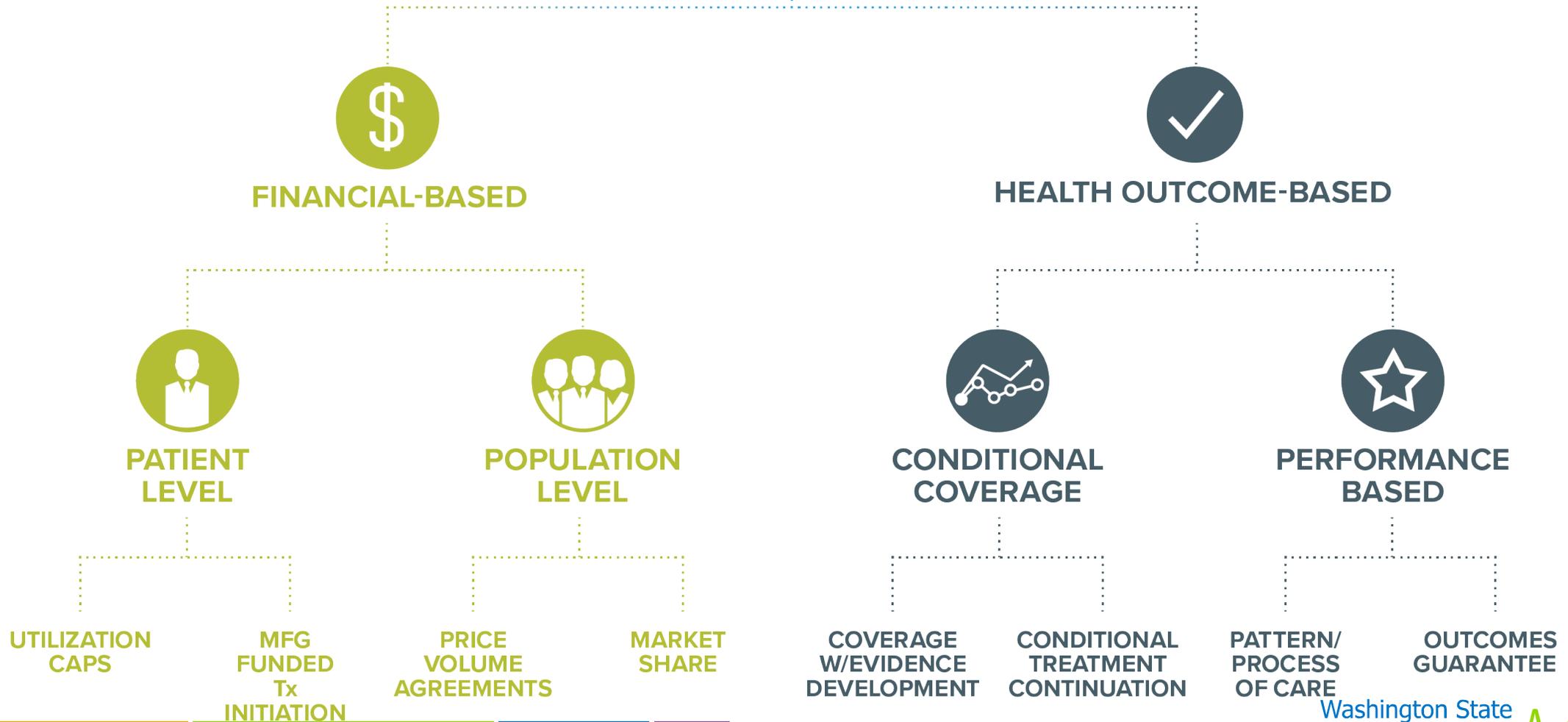
State Strategies to Balance Access and Cost

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

Funding Options

- When are APMs appropriate?
- When to carve out?
- Durability of response
- Stop-Loss / Reinsurance
- Pay-Over-Time / Annuity
- Outcome based models
- Public health emergency – loss of patent protection
- Discounts and revenue caps (France)
- Coverage based on evidence development (Medicare)
- International Price Referencing
- Understanding opportunities, challenges and unintended consequences – next meeting
- Reactionary – rear view mirror...need to incorporate future trends
- Patient knows the framework..
- Rare disease pool

ALTERNATIVE PURCHASING MODELS



Frameworks for Using Evidence in Policymaking



Source: Jacobs JA, Jones E, Gabella BA, Spring B, Brownson RC. Tools for Implementing an Evidence-Based Approach in Public Health Practice. *Prev Chronic Dis* 2012;9:110324.

Options for policy makers when evidence is poor

- ▶ Cover for FDA indications and get best rebate possible
- ▶ Base medical necessity on characteristics of study population and inclusion or exclusion criteria from available studies
- ▶ Conditional continuation coverage
- ▶ Negotiate price based on volume or exclusivity
- ▶ Negotiate price based on outcomes
- ▶ Require data for coverage with subsequent tailoring of coverage criteria

Tracking Outcomes of Emerging Therapies

- ▶ Randomized trials are the gold standard for testing new therapies in medicine
- ▶ They are usually performed in relatively narrow populations under strict management conditions
- ▶ Monitoring how these therapies are implemented in the real world and their consequences (both intended and unintended) for patient outcomes and for health care resources is a crucial and largely underappreciated issue.
- ▶ Data registries and quality improvement programs can fill this gap
- ▶ Stepped-wedge (cluster randomized) trials – could be conducted in the context of a quality collaborative like OB COAP

Workgroup Recommendations

- ▶ Patient perspective:
 - ▶ Patient decision aids
 - ▶ Informed consent
- ▶ Measurements for health impact:
 - ▶ Outcome registry
 - ▶ Cure vs. maintenance
 - ▶ Long term side-effects
- ▶ State payment options:
 - ▶ Reinsurance options with orphan drugs
 - ▶ Best price implications
 - ▶ Outcome based contracts
 - ▶ Coverage based on evidence development

Questions & Comments

- ▶ Email: HCAEmergingTherapies@hca.wa.gov
- ▶ Website: <https://www.hca.wa.gov/about-hca/clinical-collaboration-and-initiatives/emerging-therapies-workgroup>