Emerging Therapies
Workgroup Overview

December 18, 2019
Judy Zerzan, Medical Director
Workgroup’s Scope

- Workgroup is 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
<table>
<thead>
<tr>
<th>Date</th>
<th>Topics</th>
<th>Access</th>
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<tbody>
<tr>
<td>June 18, 2019</td>
<td>- Overview of new therapies coming to market&lt;br&gt;- Private sector perspective on managing emerging therapies&lt;br&gt;- HCA/Medicaid perspective on managing emerging therapies&lt;br&gt;- Current financing</td>
<td>Invitation only</td>
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<tr>
<td>October 18, 2019</td>
<td>- Patient decision aids&lt;br&gt;- Patient experience&lt;br&gt;- Patient advocates present</td>
<td>Invitation only</td>
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<tr>
<td>December 18, 2019</td>
<td>- Summary of the workgroup's progress so far&lt;br&gt;- Stakeholder questions and comments</td>
<td>Open public meeting</td>
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<td>February 19, 2020</td>
<td>- Long-term funding for emerging therapies&lt;br&gt;- Potential funding options between manufacturers and the state&lt;br&gt;- Different payment options between the state and managed care organizations</td>
<td>Open public meeting</td>
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<td>April 15, 2020</td>
<td>- Potential improvements and harms to health outcomes and quality of life for patients&lt;br&gt;- Quality oversight and outcome tracking of providers and facilities administering emerging therapies&lt;br&gt;- Metrics that could be used to measure the fiscal and health impacts of emerging therapies&lt;br&gt;- Potential long-term savings and expenditures to the state</td>
<td>Invitation only</td>
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<tr>
<td>June 9, 2020</td>
<td>- Summary of the workgroup's progress&lt;br&gt;- Stakeholder questions and feedback&lt;br&gt;- Next steps</td>
<td>Open public meeting</td>
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Recap of June 18th Meeting
About 10 years ago, specialty medicines accounted for 24.7% of total pharmacy spending. 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.

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
Recap of October 18th Meeting
Patient Advocate Presentations

- Parkinson’s Disease
- Hemophilia
- Sickle Cell

- Per your specialty, what drugs are in the pipeline that your organization is aware of and tracking?
- How does (or would) your organization define a cure?
- How does your organization educate patients/families about treatment options?
Perspectives on Educating & Guiding Patients to Treatment Decisions

Pediatric cancer specialist
- What are some of the biggest challenges and best practices from a physician perspective when educating and guiding families on treatment decisions for children?
- How do you talk to patients about therapies that have uncertain benefits?
- How much information do you need to know about an emerging therapy before you will discuss it with a patient?

Oncologist specialized in blood and marrow (stem cell)
- What are some of the biggest challenges and best practices from a physician perspective when educating and guiding adult patients on their own treatment decisions?
- How do you talk to patients about therapies that have uncertain benefits?
- How much information do you need to know about an emerging therapy before you will discuss it with a patient?

Patient decision aids
- What are the best practices when using PDAs?
- What is shared decision making?
Ethical Considerations for Emerging Therapies

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

- Glad Pharma will be represented. Multiple drug view points useful.
- FDA approval, how benfitting are drugs?
- Cost containment.
- Access, equity.
- Visibility
- Funding option challenges
- Senator Murray’s office- funding
- How do we ensure all stakeholders continue to be part of conversation?
- Pharma says pay over 5 yrs. Payers will not be part of.
- Reinsurance, risk corridor
- Cost to one versus savings to another
- Bills in legislation
- Education, shared decision making
Questions & Comments

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