Emerging Therapies Workgroup

June 18, 2019

Review of Agenda
Welcome and Introductions
Evolution of the US Health Care System

- WWII spurred systematic health care
  - Health care was provided for soldiers and their families (TriCare)
  - Employers offered health care coverage as compensation for workers’ frozen wages

- Employment Based Private Insurance - 1945
  - WWII wage and price controls prevent wage increases but allows for fringe benefits
  - Health care benefits became integral for attracting and retaining employees

- Taft-Hartly Act (1947): Health benefits declared a condition of employment for which labor was entitled to negotiate
Private Health Insurance

- Health insurance that can be purchased from private insurance companies to provide coverage for healthcare expenses

- Options for purchasing private health insurance:
  - Many employers offer health insurance as part of benefit package (fully insured/self-funded)
  - Self-employed individuals must pay entire cost of their health insurance
  - Insurance Exchanges implemented as a part of ACA (2008)

- National vs Regional Health plans
  - National Health Plans – Cover millions of lives and have a footprint across the US: United Healthcare, Anthem, Humana, Cigna, etc.
  - Regional Health Plans – May be small to medium in size in terms of no. of lives covered and are confined to a geographical region – State Blue Cross Blue Shield

Public Health Insurance

- The employment model does not cover the elderly or the poor

- Medicaid – Federal/State Partnership
  - State managed health coverage for people with low income
  - Dual eligibility with Medicare
  - Covered outpatient drug program (1990)

- Medicare
  - Federally funded coverage for the elderly and disabled
  - Part-A: Hospital insurance – “Free” (1965)
  - Part-B: Physician services – Federal taxes and beneficiary premiums (1965)
  - Part-C: Medicare Advantage – may or may not include prescription drugs (1997)

- Department of Defense

- Veterans Affairs provides vital services to more than 23.4 million American veterans
“Follow the Dollar”

A complex set of financial relationships ties together the distribution and financing components shown in the diagram to the right.

Pharmaceuticals make their way from manufacturers to distributors to retailers to patients (shown in blue).

The organizations involved in financing pharmaceuticals are shown in gray. These organizations include PBMs and public and private health insurance plans.

Manufacturers provide a series of cash payments to health plans, PBMs, and distributors in the form of rebates and chargebacks as a result of complex pricing arrangements across the industry.

Specialty drugs are the major driver of Rx drug costs in the United States…

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.
...and the pipeline shows no sign of slowing

![Bar chart showing the number of specialty drugs from 2008 to 2020.]

**20th Century Cures Act of 2016 spurs innovation**

- Facilitates development and approval of genetically targeted and variant protein targeted drugs for treatment of rare diseases
- Modified the FDA approval process
  - Expedites process by which new drugs and devices are approved
  - Allows submission of “real world” evidence such as registries, observational studies, insurance claims data, and anecdotal data.
- Breakthrough specialty drugs may be available as early as 2022 that treat:
  - Certain types of cancer
  - Blindness (neovascular age-related macular degeneration)
  - Hemophilia
  - Alzheimer’s disease
  - Certain neurologic diseases

Specialty drugs bring exciting innovation...

A Cure for Hemophilia within Reach

The New York Times

They Thought Hemophilia Was a ‘Lifelong Thing.’
They May Be Wrong.

Experimental gene therapies have yielded promising results in early trials. But the drugs have left some patients worried that success will not last.

...and enormous price tags

Can Novartis charge $4 million for a one-time drug?

STAT

A life-saving gene drug could hold families hostage with a $4 million price tag

MIT Technology Review
Specialty drugs continue to grow and influence the clinical and financial landscape

Of those drugs 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”

Drug Coverage Decision Making

- Before any decisions on coverage, access and reimbursement will be made, the drug must be approved by the Food and Drug Administration (FDA).
  - Until a drug is approved by the FDA, it cannot legally be given to anyone except through an approved clinical trial or through a drug manufacturer’s compassionate-use program approved by the FDA.
- The FDA approves a drug for certain conditions or indications. Those indications are noted on the label to be included with the drug.
  - The FDA approves the language in the label; that is, the FDA approves every word written on the label.
- Regardless of the approved indications a doctor can prescribe a medication for any use
- Health plans can decide to cover or not cover drugs for use off label
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.


Emerging Therapies Pipeline
High Cost Emerging Therapies

Jessica Daw, PharmD, MBA
UPMC Health Plan
&
Terri Levien, PharmD
Drug Information Center
Washington State University

Orphan Drugs in US

- Any disease, disorder, illness or condition affecting fewer than 200,000 people is considered rare (in US)
  - 1 in 10 Americans has a rare disease
  - Rare disease is often misdiagnosed or undiagnosed
  - 7,000 rare diseases exist
  - 80% of rare diseases are genetically based
  - ONLY 5% of rare diseases have treatments
- Mean drug cost for top 100 orphan drugs: $150,854

Orphan Drug Designations

Cumulative Count of Orphan Drug Designations

- Cumulative count of orphan drug designations from 2010 to 2018
- Blue line represents US, green line represents EU, and purple line represents Japan.
- 35 of 59 (58%) of drug approvals in US were orphan in 2018


Orphan Drug Sales

Orphan Sales as a % of Worldwide Prescription Sales

- Orphan Drugs
- Non-orphan Drugs
- Projected 2024

High Impact Drugs

- **SMA Gene Therapy**: $2M per member
- **Hemophilia Gene Therapies**: ~$2M per member
- **Givosiran**: Acute Prophryia $300K PMPY
- **Post-Partum Depression**: $36K PMPY
- **Peanut Allergy**: $5K PMPY
- **NASH**: $12-80K PMPY
- **Hemophilia Gene Therapies**: ~$2M per member
- **Vitrakvi**: NKTR gene $400K PMPY
- **DMD**: ATTR-CM $225K PMPY
- **CF Triple-therapy**: $350K PMPY
- **Tafamidis**: $250K PMPY
- **Vitrakvi**: NKTR gene $400K PMPY
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**Plan Prevalence**

PMPY = per member per year

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Orphan Diseases

**Cystic Fibrosis**

<table>
<thead>
<tr>
<th>Drug Name</th>
<th>Mechanism of Action</th>
<th>Manufacturer</th>
<th>Indication</th>
<th>Route</th>
<th>Anticipated Approval</th>
</tr>
</thead>
<tbody>
<tr>
<td>VX-659/tezacaftor/ivacaftor</td>
<td>CFTR protein correctors: VX-659, tezacaftor, CFTR protein potentiator: Ivacaftor</td>
<td>Vertex</td>
<td>CF with one F508del mutation and one minimal function mutation, CF with two F508del mutations</td>
<td>Oral</td>
<td>2020</td>
</tr>
</tbody>
</table>

**Duchenne Muscular Dystrophy (DMD)**

<table>
<thead>
<tr>
<th>Drug Name</th>
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<th>Route</th>
<th>Anticipated Approval</th>
</tr>
</thead>
<tbody>
<tr>
<td>Golodirsen</td>
<td>Antisense oligonucleotide; allows exon 53 to be skipped, providing altered messenger RNA (mRNA), which in turn produces a shortened but functional version of dystrophin</td>
<td>Sarepta Therapeutics</td>
<td>Treatment of patients with Duchenne muscular dystrophy (DMD) with genetic mutations subject to skipping exon 53 of the DMD gene</td>
<td>IV</td>
<td>Q3 2019</td>
</tr>
</tbody>
</table>
### Cystic Fibrosis Pipeline - VX-659/tezacaftor/ivacaftor

<table>
<thead>
<tr>
<th>Prevalence</th>
<th>Cost</th>
<th>Clinical Outcomes</th>
</tr>
</thead>
<tbody>
<tr>
<td>~30,000 Americans with CF</td>
<td>New starts and Switching Drug: $350,000+ per year per patient</td>
<td>• Improvement in ppFEV₁ at week 4 from baseline</td>
</tr>
</tbody>
</table>

~60% eligible for treatment with currently available drugs


Clinical Outcomes:

- Improvement in ppFEV₁ at week 4 from baseline

### DMD Pipeline - Golodirsen

<table>
<thead>
<tr>
<th>Prevalence</th>
<th>Cost</th>
<th>Clinical Outcomes</th>
</tr>
</thead>
</table>
| ~10,000 American males | New starts $250,000+ per year per patient | • Increase in skipping exon 53  
• Increase in mean dystrophin protein |


Clinical Outcomes:

- Increase in skipping exon 53
- Increase in mean dystrophin protein

Gene Therapy

Spinal Muscular Atrophy

<table>
<thead>
<tr>
<th>Drug Name</th>
<th>Mechanism of Action</th>
<th>Manufacturer</th>
<th>Indication</th>
<th>Route</th>
<th>Approval</th>
</tr>
</thead>
<tbody>
<tr>
<td>Onasemnogene abeparvovec-xioi AVXS-101, Zolgensma</td>
<td>Gene therapy using an adeno-associated virus vector (AAV9) containing the SMN1 transgene</td>
<td>Novartis</td>
<td>Treatment of children less than 2 years old with spinal muscular atrophy (SMA) Type 1</td>
<td>IV x 1</td>
<td>May 24, 2019</td>
</tr>
</tbody>
</table>

SMA Gene Therapy - Onasemnogene abeparvovec-xioi (Zolgensma, Novartis)

**Prevalence**

~35,000 Americans with SMA

**Cost**

$2.125 million for 1x IV infusion

**Clinical Outcomes**

- Increase in motor function improvement from baseline (CHOP-INTEND)
- Survival


Disease is caused by a known genetic mutation

- Monogenic: single defective gene responsible for disease
- Polygenic: multiple genes involved

Disease with no effective treatment

Disease for which current therapy involves long-term administration of an expensive therapeutic agent or an invasive procedure

Disease that has failed to improve or is resistant to conventional therapy

### Gene Therapy Targets

<table>
<thead>
<tr>
<th>Drug Name</th>
<th>Mechanism of Action</th>
<th>Manufacturer</th>
<th>Indication</th>
<th>Route</th>
<th>Anticipated Approval</th>
</tr>
</thead>
<tbody>
<tr>
<td>Eptacog beta activated</td>
<td>rFVila</td>
<td>LFB S.A.</td>
<td>Hemophilia A &amp; B w/ inhibitors</td>
<td>IV</td>
<td>2019</td>
</tr>
<tr>
<td>Fitusiran</td>
<td>siRNA</td>
<td>Alnylam/Sanoﬁ</td>
<td>Hemophilia A and B</td>
<td>SC</td>
<td>2020</td>
</tr>
<tr>
<td>Valoctocogene roxaparvovec</td>
<td>Gene therapy</td>
<td>BioMarin</td>
<td>Hemophilia A</td>
<td>IV</td>
<td>2020</td>
</tr>
<tr>
<td>AMT-061</td>
<td>Gene therapy</td>
<td>uniQure</td>
<td>Hemophilia B</td>
<td>IV</td>
<td>2020</td>
</tr>
<tr>
<td>Fidanacogene elaparvovec</td>
<td>Gene therapy</td>
<td>Spark/Pfizer</td>
<td>Hemophilia B</td>
<td>IV</td>
<td>2021</td>
</tr>
<tr>
<td>SB-525</td>
<td>Gene therapy</td>
<td>Sangamo/Pfizer</td>
<td>Hemophilia A</td>
<td>IV</td>
<td>2022</td>
</tr>
<tr>
<td>SHP-654</td>
<td>Gene therapy</td>
<td>Takeda</td>
<td>Hemophilia A</td>
<td>IV</td>
<td>2022</td>
</tr>
<tr>
<td>SPK-8011</td>
<td>Gene therapy</td>
<td>Spark</td>
<td>Hemophilia A</td>
<td>IV</td>
<td>2022</td>
</tr>
<tr>
<td>FLT 180a</td>
<td>Gene therapy</td>
<td>Freeline</td>
<td>Hemophilia B</td>
<td>IV</td>
<td>TBD</td>
</tr>
<tr>
<td>AMT-180</td>
<td>Gene therapy</td>
<td>uniQure</td>
<td>Hemophilia A</td>
<td>IV</td>
<td>TBD</td>
</tr>
<tr>
<td>SB-FIX</td>
<td>Gene therapy</td>
<td>Sangamo</td>
<td>Hemophilia B</td>
<td>IV</td>
<td>TBD</td>
</tr>
<tr>
<td>SPK-8016</td>
<td>Gene therapy</td>
<td>Spark</td>
<td>Hemophilia A with inhibitors</td>
<td>IV</td>
<td>TBD</td>
</tr>
<tr>
<td>Marzeptacog alfa</td>
<td>rFVila</td>
<td>Catalyst</td>
<td>Hemophilia A &amp; B w/ inhibitors</td>
<td>SC</td>
<td>TBD</td>
</tr>
</tbody>
</table>
Hemophilia Gene Therapy

Prevalence

20,000 Americans with hemophilia

Cost

$2 million for 1x IV infusion

Clinical Outcomes

- Factor activity
- Factor utilization
- Bleeding episodes
- Patient reported outcomes
- Safety


Proposed gene therapy cost based on cost of other gene therapy and investor estimates.

Hemophilia Gene Therapy

Results of valoctocogene roxaparvovec phase 1-2 dose escalation study

Annualized Bleeding Rate

Use of Factor XIII Concentrate

Participant No.

1 Yr before Vector Infusion  After Vector Infusion

# Gene Therapy Pipeline

<table>
<thead>
<tr>
<th>Drug Name/Manufacturer</th>
<th>Mechanism of Action</th>
<th>Indication</th>
<th>Potential Population</th>
<th>Anticipated Approval</th>
</tr>
</thead>
<tbody>
<tr>
<td>SRP-9001/Sarepta</td>
<td>Gene Therapy</td>
<td>Duchenne muscular dystrophy</td>
<td>3 per 10,000 male births</td>
<td>TBD</td>
</tr>
<tr>
<td>Elivaldogene tavalentivec (Lenti-D)/bluebird bio</td>
<td>Autologous gene therapy</td>
<td>Cerebral adrenoleukodystrophy</td>
<td>1 per 20,000-50,000 births</td>
<td>2020</td>
</tr>
<tr>
<td>LYS-SAF302/Lysogene/Sarepta</td>
<td>Gene therapy</td>
<td>Mucopolysaccharidosis IIIA (Sanfilippo Syndrome)</td>
<td>1 per 100,000 births</td>
<td>2022</td>
</tr>
<tr>
<td>GS010/GenSight Biologics</td>
<td>Gene therapy</td>
<td>Leber Hereditary optic neuropathy</td>
<td>1 in 50,000 people</td>
<td>2020</td>
</tr>
<tr>
<td>QR-110/ProQR</td>
<td>Gene therapy</td>
<td>Leber congenital amaurosis 10</td>
<td>2-3 per 100,000 newborns</td>
<td>2021</td>
</tr>
<tr>
<td>NSR-REP1/Nightstar</td>
<td>Gene therapy</td>
<td>Choroideremia</td>
<td>1 in 50,000-100,000 people</td>
<td>2021</td>
</tr>
</tbody>
</table>

National Organization for Rare Disorders (NORD). Rare Disease Database. [https://rarediseases.org](https://rarediseases.org)

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# Gene Therapy Pipeline

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</tr>
</thead>
<tbody>
<tr>
<td>Betibeglogene darolentivec (Bb305, Zynteglo)/bluebird bio</td>
<td>Autologous gene therapy</td>
<td>Beta-thalassemia, transfusion-dependent; sickle cell disease</td>
<td>1 in 100,000; + 100,000 in US with sickle cell disease</td>
<td>2020</td>
</tr>
<tr>
<td>MB-107/Mustang Bio</td>
<td>Autologous gene therapy</td>
<td>X-linked severe combined immunodeficiency (XSCID)</td>
<td>1 in 50,000-100,000 births</td>
<td>2021</td>
</tr>
<tr>
<td>FCX-007/Fibrocell</td>
<td>Autologous gene therapy, local inj</td>
<td>Recessive dystrophic epidermolysis bullosa</td>
<td>Severe form: 1 per million newborns</td>
<td>2021</td>
</tr>
<tr>
<td>EB-101/Abeona</td>
<td>Autologous gene therapy, local inj</td>
<td>Recessive dystrophic epidermolysis bullosa</td>
<td>Severe form: 1 per million newborns</td>
<td>TBD</td>
</tr>
<tr>
<td>Bercolagene telserpavec/Krystal Biotech</td>
<td>Autologous gene therapy, topical</td>
<td>Recessive dystrophic epidermolysis bullosa</td>
<td>Severe form: 1 per million newborns</td>
<td>TBD</td>
</tr>
</tbody>
</table>

National Organization for Rare Disorders (NORD). Rare Disease Database. [https://rarediseases.org](https://rarediseases.org)
Nonalcoholic steatohepatitis (NASH)

### Prevalence
- 3-12% of adults in United States
- 1.5-6.45% of the general population (worldwide)

### Cost
- ~$12K - $80K
- Global market may reach $22 million by 2025

### Clinical Outcomes
- Resolution of NASH
- Improvement in Fibrosis

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**Partial NASH Pipeline**

<table>
<thead>
<tr>
<th>Drug Name</th>
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<th>Manufacturer</th>
<th>Route</th>
<th>Anticipated Approval</th>
</tr>
</thead>
<tbody>
<tr>
<td>Obeticholic acid</td>
<td>Farnesoid X Receptor (FXR) Agonist</td>
<td>Intercept</td>
<td>PO</td>
<td>2019</td>
</tr>
<tr>
<td>Elafibranor</td>
<td>Dual peroxisome proliferator-activated alpha and delta receptor agonist (PPARα/δ agonist)</td>
<td>Genfit</td>
<td>PO</td>
<td>2020</td>
</tr>
<tr>
<td>Cenicriviroc</td>
<td>Dual chemokine receptor 5 (CCR5) and chemokine receptor 2 (CCR2) antagonist</td>
<td>Allergan</td>
<td>PO</td>
<td>2020</td>
</tr>
<tr>
<td>Semaglutide</td>
<td>Glucagon-like peptide-1 (GLP-1) analog</td>
<td>Novo Nordisk</td>
<td>SC</td>
<td>2020</td>
</tr>
<tr>
<td>Cilofexor (GS-9674)</td>
<td>Synthetic non-steroidal FXR agonist</td>
<td>Gilead</td>
<td>PO</td>
<td>2021</td>
</tr>
<tr>
<td>Cilofexor/Firsocostat (GS-9674/GS-0976)</td>
<td>Non-steroidal FXR agonist and acetyl-CoA carboxylase (ACC) inhibitor</td>
<td>Gilead</td>
<td>PO</td>
<td>2021</td>
</tr>
<tr>
<td>MGL-3196</td>
<td>Thyroid hormone receptor (THR) β-selective agonist</td>
<td>Madrigal</td>
<td>PO</td>
<td>2021</td>
</tr>
<tr>
<td>Aramchol arachidyl amido cholanoic acid</td>
<td>Fatty acid bile acid conjugate; Stearoyl-CoA Desaturase-1 Modulator (SCD-1)</td>
<td>Galmed</td>
<td>PO</td>
<td>2021</td>
</tr>
</tbody>
</table>

### NASH Pipeline

<table>
<thead>
<tr>
<th>Drug Name</th>
<th>Obeticholic Acid</th>
<th>Elafibranor</th>
<th>Cenicriviroc</th>
</tr>
</thead>
<tbody>
<tr>
<td>NASH Resolution</td>
<td>✗</td>
<td>✓/✗</td>
<td>✗</td>
</tr>
<tr>
<td>Improvement in Fibrosis</td>
<td>✓</td>
<td>✓/✗</td>
<td>✓</td>
</tr>
<tr>
<td>Safety Concerns</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
</tbody>
</table>


### Tumor Agnostic Therapies

**Pembrolizumab - (Keytruda)**

Immunotherapy - Adult and pediatric patients with unresectable or metastatic, microsatellite instability-high (MSI-H) or mismatch repair deficient

- Solid tumors that have progressed following prior treatment and who have no satisfactory alternative treatment options

**Larotrectinib (Vitrakvi)**

Adult and pediatric patients with solid tumors:

- Have a neurotrophic receptor tyrosine kinase (NTRK) gene fusion without a known acquired resistance mutation
- Are metastatic or where surgical resection is likely to result in severe morbidity, and
- Have no satisfactory alternative treatments or that have progressed following treatment

**Loxo-195**

Is being investigated to address potential mechanisms of acquired resistance that may emerge in patients receiving Vitrakvi or other multi-kinase inhibitors with anti-TRK activity
# U.S. Approved Biosimilars

<table>
<thead>
<tr>
<th>Remicade (infliximab)</th>
<th>Neulasta (pegfilgrastim)</th>
<th>Humira (adalimumab)</th>
<th>Herceptin (trastuzumab)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Inflectra</strong>&lt;sup&gt;*&lt;/sup&gt; - Pfizer/Celltrion</td>
<td><strong>Fulphila</strong>&lt;sup&gt;*&lt;/sup&gt; - Mylan/Biocon</td>
<td><strong>Amjevita</strong> - Amgen</td>
<td><strong>Ogivi</strong> - Mylan/Biocon</td>
</tr>
<tr>
<td><strong>Ixifi</strong> - Pfizer</td>
<td><strong>Udenyca</strong>&lt;sup&gt;*&lt;/sup&gt; - Coherus</td>
<td><strong>Cyltezo</strong> - BI</td>
<td><strong>Herzuma</strong> - Celltrion/Teva</td>
</tr>
<tr>
<td><strong>Renflexis</strong>&lt;sup&gt;*&lt;/sup&gt; - Merck</td>
<td><strong>Hyrimoz</strong> - Sandoz</td>
<td><strong>Ontruzant</strong> - Merck/Samsung</td>
<td><strong>Trazimera</strong> - Pfizer</td>
</tr>
</tbody>
</table>

- **Bevacizumab (Pfizer)** - Avastin (Genentech) - June 2019
- **Adalimumab (Samsung Bioepis)** - Humira (AbbVie) - July 2019
- **Rituximab (Pfizer)** - Rituxan (Genentech) - July 2019
- **Filgrastim (Tanvex BioPharma)** - Neupogen (Amgen) - August 2019
- **Infliximab (Amgen)** - Remicade (Janssen) - October 2019
- **Filgrastim (Adello Biologic)** - Neupogen (Amgen) - 2019
- **Filgrastim (Apotex/Intas)** - Neupogen (Amgen) - 2019
- **Pegfilgrastim (Apotex/Intas)** - Neulasta (Amgen) - 2019
- **Pegfilgrastim (Sandoz)** - Neulasta (Amgen) - 2019

* Indicates commercially available in the U.S.

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* Indicates commercially available in the U.S.
Considerations

- Affordability versus Value
- Value of a Cure
- Uncertainty around long-term benefits
- Genetic Markers in Oncology
- High Cost, Low Value
- Access/Regulatory issues

Perspective on current management of emerging therapies
Drug Coverage Considerations

A Commercial Health Plan Perspective

Carly Rodriguez, PharmD, FAMCP
Pharmacy Director, Clinical Innovation
Moda Health Plan

Drug coverage considerations - commercial

- Certificate of Coverage (“member handbook”) specifies what is and is not covered
- Who defines this?
  - Fully insured groups and individuals: sponsoring health plan
  - Self-insured/ASO groups: group
  - State laws and insurance divisions may also define
- Pertinent language:
  - Medically necessary
  - Experimental & Investigational
  - Exclusions
Clinical evidence review

Sources
- Clinical trials
- Systematic review
- ICER
- Guidelines
- Manufacturer data

Methods & assessment
- Assess quality of evidence: Delfini
  - High, moderate, low quality
- Synthesize evidence: ICER
  - Clinical benefit: meaningful, comparable, inferior or unknown

Additional considerations

- Disease characteristics
- Treatment alternatives
- Expert opinion
- Administration considerations
- Disease characteristics
Clinical criteria development

- Evidence-based criteria
  - Study protocols: dosing, frequency, duration, concomitant therapies
  - Inclusion/exclusion criteria: indication, age, co-morbidities
  - Prior therapy & alternatives: step therapy implications

- Other influences
  - FDA-approved label
    - Supported by evidence?
  - Treatment guidelines
    - Supported by evidence?

P&T committee

- Independent group of practicing providers not employed by the health plan
  - Physicians, pharmacists, nurses, economists, other
  - Variety of practice settings and specialties represented

- Role
  - Develop and maintain formularies
  - Establish clinical criteria
  - Direct drug utilization review activities

- Health plan pharmacists present evidence and propose recommendations, but only P&T Committee members have decision-making authority
Financial evaluation

- Goal: drive to lowest net cost option *when clinically appropriate*
- Evaluated in context of P&T decision
  - Formulary decision
  - Clinical criteria

Final coverage policy

Clinical evaluation + P&T decision + Financial evaluation = Coverage policy
Tracking outcomes

- Cost & utilization review
- Prior authorization data
- Claims analyses
- Clinical data
- Outcomes-based agreement results

Drug Coverage Considerations

A Medicaid and State Purchaser Perspective

Donna L. Sullivan, PharmD, MS
Chief Pharmacy Officer
Clinical Quality & Care Transformation
How HCA determines coverage status of new drug including emerging therapies

Medicaid must cover drugs
- That are included in the Medicaid Drug Rebate Program; and
- For their FDA approved or medically accepted indications; and
- That are medically necessary

Employee & Retiree Benefits plans must cover drugs that are
- FDA approved
- Medically Necessary
- New drugs and emerging therapies must be reviewed by the PEB Board (budget proviso)

Perform critical evaluation of the evidence

- Drug Effectiveness Review Project (DERP)
- Medicaid Evidenced-Based Decision Making (MED)
- Institute for Cost Effective Review (ICER)
- Clinical staff
Determine the strength of the evidence

- Analysis of Evidence
- Information about outcomes
- Value Judgments


Perform Budget Impact Analysis

- Identify the target population
- Estimate number of patients in our programs
  - Claims data
  - National and local prevalence data
- Estimate the cost of the new therapy
- Determine impact on WA programs
- Notify authorizing environment when necessary
  - HCA leadership
  - Governor’s office
  - Office financial management
  - Legislative members
Develop clinical criteria

- Develop standard clinical policies for therapies based on:
  - Strength of the evidence
  - Efficacy, effectiveness, and safety
  - Impacts on health outcomes
  - Indications for use
  - Alternative therapies

- For therapies with poor quality evidence determine medical necessity on a case by case basis

How HCA develops clinical policies

- HCA Clinical staff develop draft policies:
  - Reviewed with Medicaid MCO clinicians
  - Reviewed by internal Coverage Parameters workgroup
  - Draft policies are reviewed, edited, and approved by the Drug Utilization Review Board in open public meetings

- Employee & Retiree Benefit Plans:
  - Uniform Medical Plan - clinical policies are reviewed, edited, and approved by the Pharmacy & Therapeutics committee of the benefit administrators
    - Regence develops policies for drugs covered under the Medical Benefit
    - Moda develops policies for drugs covered under the pharmacy benefit
  - Fully insured plans develop their own clinical policies.
High-cost drug policy

- High-cost drug definition
  - Covered outpatient drug and physician administered drugs
  - Traditional drug, orphan drug, gene therapy
  - Expected annual cost per patient is ≥ $100,000 per year
- HCA will work with representatives from Medicaid managed care plans (MCO) to:
  - Identify high-cost drugs
  - Develop clinical policies
  - Carve new high-cost drugs out of MCO rates when it:
    - Is indicated for a disease previously untreated with drugs.
    - Has a different mechanism of action than existing high-cost drugs that are not carved out, costs 50% more than existing high-cost drugs, and was given breakthrough designation by the Food and Drug Administration.
    - Has the same mechanism of action as current high-cost drugs that are already carved out of the MCO rate.

Reimbursement for high cost drugs

- Maybe covered under pharmacy or medical benefit
- Prior authorization is required on all high cost drugs
- Provider/pharmacy must submit invoice along with authorization request
  - Pharmacy – reimburse acquisition cost + dispensing fee
  - Medical – reimbursement acquisition cost + EAPG payment
- Drugs covered through the medical benefit are carved out of the “Enhanced Ambulatory Patient Group (EAPG) bundled payment