Emerging Therapies

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Definitions

- “Emerging therapy” means a new drug with a novel mechanism of action including gene therapy
- “Orphan drug” means a drug that treats a condition that affects less than 200,000 individuals in the U.S.

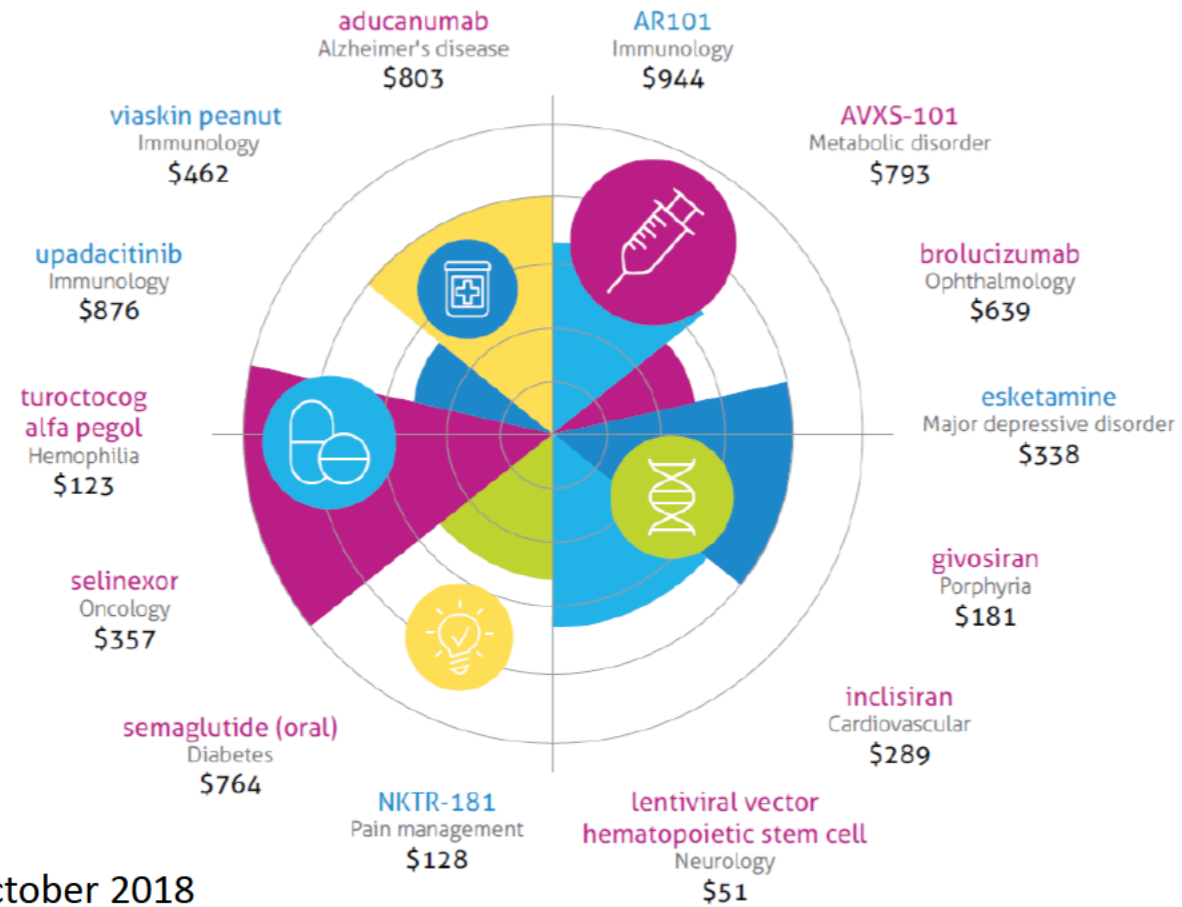
20th Century Cures Act of 2016 spurs innovation

- Modified FDA approval process
  - Expedites process by which new drugs and devices approved
  - Allows submission of “real world” evidence such as observational studies, insurance claims data, and anecdotal data
- Facilitates development and approval of genetically targeted and variant protein targeted drugs for treatment of rare diseases
- 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

Emerging therapies 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"

Source: Magellan, MRx Pipeline October 2018

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How HCA determines coverage status of new drugs–including emerging therapies

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

How HCA develops clinical policies for emerging therapies

- Identify new drugs or new indications approved by FDA
- Perform a critical evaluation of the available evidence
  - Drug Effectiveness Review Project (DERP)
  - Medicaid Evidenced-Based Decision Making (MED)
  - Clinical staff
- Determine the strength of the evidence
- Develop standard clinical policies for therapies based on:
  - Strength of 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

- Medicaid
  - HCA Clinical staff develop draft policies
  - Draft policies reviewed, edited, and approved by the Drug Utilization Review Board in open public meetings
  - Working toward standardized policies across Medicaid programs
- Employee & Retiree Benefits Plans
  - Uniform Medical Plan – clinical policies 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 own clinical policies

Medicaid is creating a high-cost drug policy

- High-cost drug definition
  - Covered outpatient drug
  - Traditional drug, orphan drug, gene therapy
  - Expected annual cost per patient is ≥ $100,000 per year
- HCA will work with Medicaid managed care plans (MCO) representatives 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 new mechanism of action than existing high-cost drugs, 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.

Questions?

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