AHIP
America's Health Insurance Plans

Rare Disease Scientific Workshop

Reimbursement of Innovative Therapies

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Our Mission

America's Health Insurance Plans and its members create and accelerate positive change and innovation across the health care system for consumers through market-based solutions and public-private partnerships that advance affordability, value, access and well-being.

A Balancing Act

- Insurers recognize and applaud the accomplishments of the drug industry in developing important therapeutic advancements in the treatment of devastating rare diseases.
  - Pompe's Disease; Hyperammonemia; Granulomatous Disease; Gaucher Disease; CAPS; Farbry's and the other 500-plus orphan drugs available today.

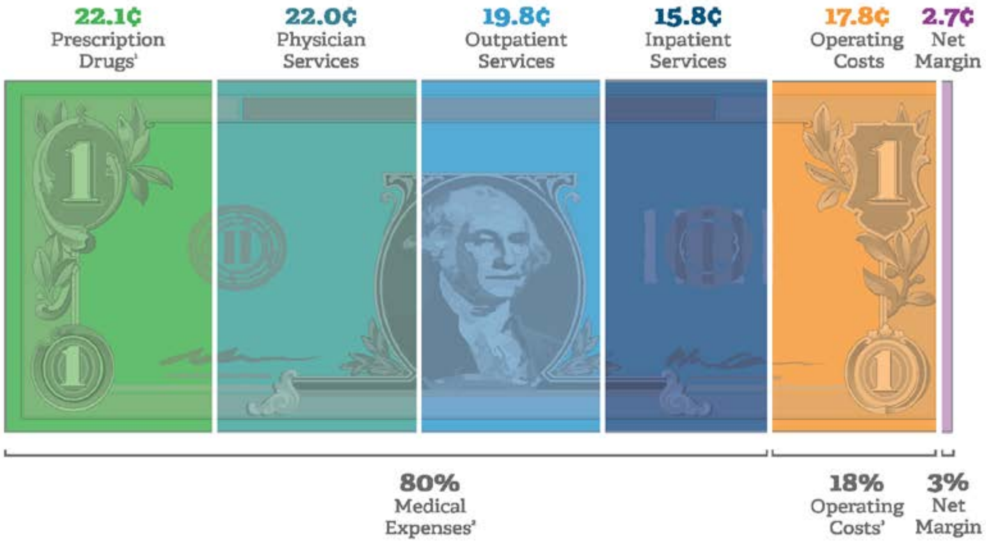
- However, insurers struggle with how to pay for these treatments that routinely cost between $100-500K per patient per year.[1]
  - Ultimately, these costs lead to increased premiums and costs for employers, taxpayers and individual consumers.

1. The Center for Policy and Research. *High-Priced Drugs: Estimates of Annual Per-Patient Expenditures for 150 Specialty Medications*. April 2016.

Medications are the top spending category

Where Does Your Premium Dollar Go?

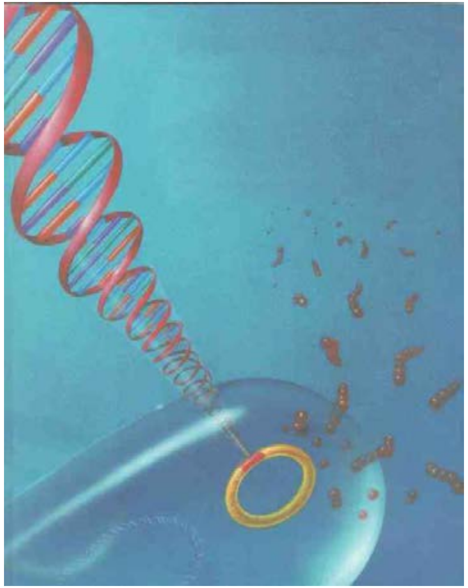
Health care costs are too high for too many. Your premium, or how much you pay for your health insurance each month, covers the costs of providing your insurance as well as the medical care you might receive – everything from prescription drugs and doctors' visits to health improvement programs and customer service. Here is a visual breakdown of where your premium dollar really goes.



Note: Values exceed 100% due to rounding
Source: Data sources and methodology are referenced in more detail at http://www.ahip.org/health-care-dollar
1 Prescription drug costs include outpatient, physician- and self-administered medications – but not those administered in inpatient settings
2 Medical expenses as identified in this research differs from Medical Loss Ratio as defined by the Affordable Care Act.
3 Operating costs include consumer-centric activities such as communicating with members, running customer service operations, quality reviews, and data analysis, among other activities.

A Balancing Act

- In the face of steadily-rising drug costs, however, the goal of balancing access and affordability has become increasingly challenging.
  - ➢ Drug spending is projected to reach about $600B by 2021(IMS-Quintiles) – This is unsustainable. [1]
  - ➢ 48% of 150 specialty drugs (including orphan drugs) cost over $100K per patient per year (AHIP). [2]
- Orphan drugs have, traditionally, been some of the most expensive specialty drugs available and the numbers are increasing.
  - ➢ 42% of new drug approvals were for orphan drugs in 2015; 41% in 2016 (FDA). [3,4]



1. The QuintilesIMS Institute. *Medicines Use and Spending in the U.S.: A Review of 2015 and Outlook to 2021*. May 2017.
2. The Center for Policy and Research. *High-Priced Drugs: Estimates of Annual Per-Patient Expenditures for 150 Specialty Medications*. April 2016.
3. The United States Food and Drug Administration. *CY2015 CDER New Molecular Entity (NME) Drug & Original BLA Calendar Year Approvals.* 31 Dec 2015.
4. The United States Food and Drug Administration. *CY2016 CDER New Molecular Entity (NME) Drug & Original BLA Calendar Year Approvals*. 31 Dec 2016.

A Balancing Act

- The growing number of orphan drugs coupled with the use of these agents in more prevalent, non-orphan disease states has recently forced a reexamination of the potential abuses of The Orphan Drug Act. [1,2]

- Still, the payer community recognizes the tremendous success of the ODA as evidenced by the steadily growing number of treatment options for rare diseases where virtually none existed before the Act.

  - Although some 6 – 7,000 rare diseases remain without any treatment options.

- AHIP and its member health plans stand ready to work with all stakeholders to identify effective market-based solutions.

  - Solutions that strike the appropriate balance between access and affordability.

1. Mukherjee, S. *An FDA Program Incentivizing Rare Disease Drugs Will Be Investigated for Abuses*. 22 March 2017.
2. Tribble, SJ. *GAO To Launch Investigation of FDA's Orphan Drug Program*. 21 March 2017.

Market-based Solutions

- Real Competition:
  - ➢ Establish a robust biosimilars market.
  - ➢ Reduce rules and “red tape” to generic market-entry.
  - ➢ Improve upon the Orphan Drug Act.

- Open & Honest Pricing:
  - ➢ Require publication of drug prices, true R&D costs, and justifications of price increases.
  - ➢ Examine and address the impacts of drug coupons, copay cards and 3rd party programs.
  - ➢ Evaluate the impacts of Direct-To-Consumer advertising.
- Deliver Value to Patients:
  - ➢ Inform patients of the cost-effectiveness and value of drug treatments.
  - ➢ Expand value-based formulary programs.
  - ➢ Reduce existing statutory and/or regulatory barriers to VBP strategies.

Discussion Q & A