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.
• 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.

Medications are the top spending category
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

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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