

Emerging Commercialization Strategies for Addressing the Needs of Ultra-Orphan Patients

Accelerate Product Launch and Profitability by Building Regulatory Requirements into Distribution

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Conference Presentation

The New Administration and the Impact on Orphan Drug Development

The Good, Bad and the Ugly

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Outline

- Health reform status update—the latest from each chamber and the administration.
- NORD initiatives, concerns and priorities.
 - Follow-on biologics
 - Comparative Effectiveness
 - Advocacy strategy
- Health insurance reform as an improvement to access and affect on the bottom line.

Follow-on Biologics-Interchangeability

- Similar language has passed both the House and the Senate.
- To determine similarity, application shall include:
 - Analytical studies that demonstrate that the biological product is highly similar to the reference product;
 - Animal studies; and,
 - A clinical study or studies that are sufficient to demonstrate safety purity and potency in 1 or more appropriate conditions of use for which the reference product is licensed and intended to be used. (Secretary can decide that one of these elements is not necessary)
- In addition, the FOB must utilize the same mechanism of action, treat the same condition or conditions, be administered via the same route of administration, and meets manufacturing standards.
- Secretary shall determine the biological product to be similar if the Secretary determines that the application shows that the product is biosimilar, can be expected to produce the same clinical result as the reference product, and can be switched with reference product during course of treatment.


Follow-on Biologics Other Provisions

- 12 years of exclusivity
 - No extra time for orphan indications
 - Extra 6 months for pediatric
- Labeling and packaging must bear a name that uniquely identifies the biological product and distinguishes it from the reference product.
- Do these provisions survive?

Comparative Effectiveness

- Many sources of research; AHRQ, NIH, IOM and Secretary's office.
- Funding provided by stimulus package.
- NIH is in the process of reviewing grant applications.
- AHRQ is establishing a process, first defined terms and is now soliciting grant applications for evidence generation.
- Still has a role in health reform—will the information be used to make coverage decisions?
- Issues for orphan treatments:
 - What are the comparators?
 - Overall percentage of health spend is minimal.
 - Will send the wrong message to public and investors.
 - However, does this establish price control mechanism?

Insurance Reform

- House/ Senate have passed legislation with key provisions for accessing therapies that treat rare disorders.
 - Elimination of pre-existing conditions---individuals cannot be excluded from a qualified health benefits plan based on “the condition being present before the date of enrollment for such coverage, whether or not any medical advice, diagnosis, care, or treatment was recommended or received before such date.”
 - Guaranteed issue and renewal of policy.
 - Benefit design requires network adequacy, no cost sharing for preventative services, limits annual cost sharing to \$5k for individual and \$10K per family, and seeks to use co-payments instead of co-insurance.
 - Many incentives to provide primary care and increase workforce to deliver care.
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Changes to the Medicaid Rebate Program and 340B

- Increase the base Medicaid rebate percentage from 15.1% to 22.1% or 23%
- Apply "Additional Rebate" for original dosage forms/strengths to New forms/strengths.
- Expansion of Medicaid rebate to Medicaid managed care utilization.
- Expands the number of "covered entities" and site of service (inpatient) eligible to receive 340B ceiling price.
- Other: removal of prompt pay from average sales price calculation.