By definition, rare diseases affect relatively few individual patients, but as a group they encompass up to 8,000 recognized rare diseases affecting approximately 10% of the population. The general lack of effective therapies and consequent low threshold for new product approval, engaged physicians and patient communities, as well as regulatory incentives, including expanded market exclusivity for companies investing in orphan diseases, all underscore the substantial commercial opportunities in this space. Historically, payers have tolerated high price points for orphan disease therapies owing to low patient numbers and very high unmet need. However, increased scrutiny of high prices for specialty drugs in the could be driving payers to impose onerous reimbursement restrictions just as a wave of innovative disease-modifying treatment options for debilitating rare diseases are reaching the market.

Questions Answered in This Report:

- Revolutionary disease-modifying treatments for rare diseases have become available in the United States in recent years. How familiar are U.S. payers with rare diseases and orphan drugs? How many beneficiaries with rare diseases are covered under payers’ health plans? How many orphan drugs are covered? What are cost-containment measures typically in place for orphan drugs?

- A growing number of orphan drugs is nearing the U.S. market. What is current and expected future cost burden of orphan drugs? What is the budgetary impact? At what yearly cost does an orphan drug become a budget disruptor?

- New cystic fibrosis (CF) treatments have become available in recent years and more are in the pipeline. How is current CF therapy Kalydeco (Vertex Pharmaceuticals) reimbursed? What is the level of coverage, price points, and restriction expected for future CF therapies?

- Duchenne muscular dystrophy (DMD) treatments are near completion of pivotal testing or are under regulatory review. Expected to be priced at a very high premium, how do payers expect to reimburse for emerging DMD treatments?

- Therapies targeting the motor symptoms of Huntington’s disease (HD) are under development at a time when generics are expected to launch shortly to current orphan treatment Xenazine
(Lundbeck), targeting HD chorea. How will current and emerging HD treatments fare in the face of generic competitors?

- Two new treatments for idiopathic pulmonary fibrosis (IPF) have recently become available in the United States—Esbriet (Roche/Genentech) and Ofev (Boehringer Ingelheim). How are the two new therapies covered? Is one preferred over the other, and if yes, why? Would payers reimburse for combination treatment?

- Therapies delivered via IV infusion or injection dominate the myelodysplastic syndromes treatment algorithm. How will an emerging oral formulation fare in terms of reimbursement?

Scope:
Market covered: United States.
Primary research: Survey of 43 U.S. MCO MDs/PDs and interviews with 4 MCO PDs.

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