

Rare Diseases Category

The Rare Diseases category includes medications that are used in patients with a disease state prevalence of fewer than 20,000 in the United States and in which the drug cost is \$150,000 or more for the average weight and dosing. The Rare Diseases category may help with the prediction and alignment of payment when high-cost medications are included under the risk-adjusted capitation payment. However, these cases can be so infrequent and so high cost that risk adjustment model may not precisely account for these high-cost outliers. Many states have alternative risk mitigation strategies to better account for high-cost drugs and high-cost individuals. This includes specific high costs drugs carved out of MCO coverage, high-cost drug risk pools, high-cost member risk corridors, and reinsurance. These other risk mitigation mechanisms more precisely account for these high-cost instances. For example, one state has employed state-specific cost weights and includes the Rare Diseases category to function in combination with their high-cost drug risk pool. This creates a balance of prospectively accounting for high-cost drug variation between health plan and retrospectively adjusting payments based on actual observed utilization during the period.

The evaluation of appropriate levels of risk and potential risk mitigation mechanisms is a requirement of the actuary to evaluate and an item for Medicaid agencies to evaluate and consider. Users of the CDPS+Rx Model should assess whether the state's Medicaid program includes a risk pool, risk corridor, kick reimbursement, or other risk-sharing arrangements specifically for drugs classified under the Rare Diseases indicator. For instance, if the state has a risk pool for high-cost drugs, the Rare Diseases component of the CDPS+Rx model might inadvertently double-count the impact of this existing risk pool. Likewise, if the state provides kick reimbursement for a drug that falls under the Rare Diseases category, users should consider removing that drug from the list of NDCs associated with Rare Diseases. Conversely, if there are no existing risk mitigations specific to drugs classified under the Rare Diseases indicator, then the user may consider the Rare Diseases category fully appropriate to use.