RTI (h)(s)™

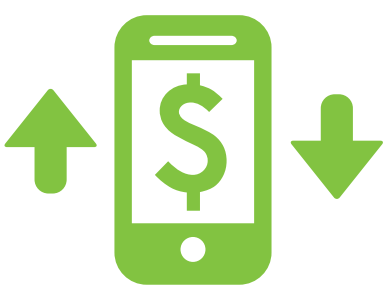
Health Solutions

Knowledgebase

Key questions orphan drug developers need to ask

Understand the challenges and opportunities for supporting P&MA of orphan drugs worldwide

What do payers want?



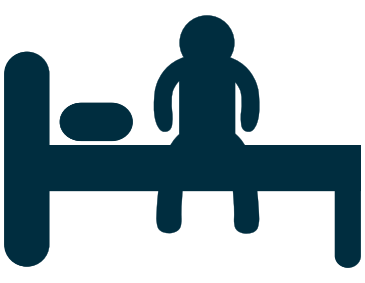
Payers are **seeking more value-based information** to better inform decision making in the evaluation of new health technologies for rare diseases.

Why are there limitations in the evidence base?

Low patient numbers and **heterogeneous populations** increase the difficulty of conducting clinical trials, and many trials have **short follow-up**.

The natural history of the disease may not be well understood and treatment options do not exist. **Consensus on clinical endpoints can be divisive.**

What keeps payers up at night?

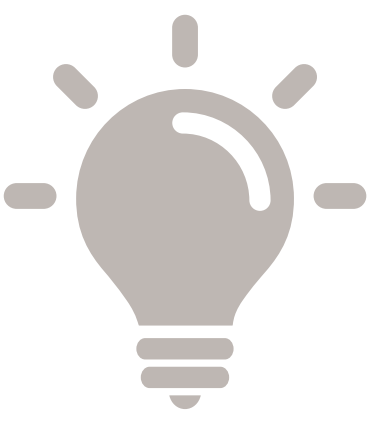


Benefits of new technologies may not be captured in traditional cost/QALY, thus **reducing uncertainty and bridging the clinical evidence with other robust data will be critical**.

What might happen?

Payers could pass more risk on to both patients and prescribers in an effort to manage budget constraints.

How can you solve the problem?



Early engagement with payers is critical

Understand the **unmet needs** and what it means to their population and their budget.

Manage the budget impact: **clearly define the patient population, clearly define treatment start/stop rules, with supporting evidence**.

Provide relevant **patient-centric information** to add to the evidence base and for prescriber pull-through.

Package the evidence for **clear and consistent messaging** for all audiences

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