A ‘Fair’ Price For Rare Disease Cures: Payers, Physicians, Patients Weigh In
A ‘Fair’ Price For Rare Disease Cures: Payers, Physicians, Patients Weigh In

By Cathy Kelly

INDUSTRY-FUNDED SURVEY BY CHARLES RIVER ASSOCIATES explores diverse perceptions about pricing for high-cost cell treatments – including curative cell and gene therapy – among various stakeholder groups.

There’s a wide disparity in the prices that US payers, physicians and patients consider to be “fair” for one-time curative cell and gene therapies for rare disease, according to a recent survey conducted by Charles River Associates VPs Andrew Parece and Matthew Majewski et al.

Physicians estimate an average of $852,000 is fair for curative treatments, while payers believe an average of $693,000 is fair and patients point to a price of $381,000. The researchers categorize as fair those specific prices that respondents identified as “somewhat fair” or “very fair.”

Findings from the survey, released on 27 May, reflect responses from 50 pharmacy and medical directors from large insurers, health plans and pharmacy benefit managers, 164 physicians who treat rare diseases and cancer, and 149 patients and family members or caregivers of patients with rare diseases and cancers. The study will be submitted for publication, the authors said.

The research was funded by several pharmaceutical and biotechnology manufacturers. It “breaks new ground by providing an understanding of the key factors that drive stakeholders’ perceptions of whether prices are considered fair” for high-cost innovative drugs, Parece and Majewski explained in an email.

“Perceptions of fairness in general are not currently considered by many stakeholders when considering how to manage or utilize a product but may become more relevant as the concept is brought to light.”

“It also enables a comparison across the research cohorts – payers, physicians and patients – regarding which factors are of particular importance to each group,” they add. “Perceptions of fairness in general are not currently considered by many stakeholders when considering how to manage or utilize a product but may become more relevant as the concept is brought to light.”

Attitudes Toward Prices For Rare Disease, Cancer Treatment

In addition to exploring attitudes towards prices for one-time curative treatments, the researchers asked stakeholders about prices for chronic treatments for rare disease and drugs to treat rare cancers.

The annual price of therapies for rare chronic conditions that is viewed as fair is three to four times lower than
the price deemed acceptable for curative therapies on average, the study found. (See chart on page 4.)

Researchers defined a curative therapy as the first approved for a condition that affects only 1,500 adults per year and that completely and permanently cures the disease and eliminates symptoms with a one-time treatment. A chronic therapy for rare disease was defined as the first approved for a condition that affects 1,500 adults annually and that completely halts disease progression and eliminates symptoms when taken regularly, such as once a month.

Payers and physicians were closer on the price they considered fair for chronic therapies for rare disease, indicating annual prices of $242,000 and $212,000, respectively, were appropriate. Patients indicated a price of $125,000 per year is fair for such treatments.

For therapies used to treat rare cancers, the average price considered to be fair was $296,000 per year for physicians, $208,000 per years for payers and $140,000 for patients. The researchers defined those drugs as the first approved to treat a type of cancer that affects 1,500 adults per year who are not expected to survive past six months without treatment. The drug would be able to extend survival by an additional 12 months (for a total of 18 months).

For all three types of treatments, patients gravitated toward prices that were about half as much as those endorsed by payers or physicians. The study does not address why stakeholders felt the way they did about pricing. However, the lower prices favored by patients relative to payers and physicians may reflect the fact that patients are less aware of what drugs actually cost because they have been shielded by insurance coverage.

Shifts In Parameters Like Patient Population, Age

The researchers also varied the treatment scenarios to see if perception of price fairness was affected by: 1) whether a condition is severe and/or life threatening; 2) the size of the patient population (1,500 vs. 200 vs. 50,000); and 3) whether the condition affects adults or children.

They found such factors could make a big difference. For example, 72% of payers indicated that the price that they considered to be fair for a chronic rare disease treatment affecting 1,500 patients would no longer be fair if the patient population is expanded to 50,000, and that a similar percentage of payers indicated that the price that they considered to be fair for curative treatments affecting 1,500 patients should be lower if the patient population was expanded to 50,000.

In contrast, around 68% of payers and 70% of physicians would consider prices above $242,000 and $125,000 to be fair if the chronic condition affected only 200 patients a year.

Across the three groups, a similar proportion of respondents indicate that a higher price for chronic treatment of a rare disease would be fair if the condition affects pediatric (versus adult) patients: 50% of payers, 55% of physicians and 44% of patients.

For rare cancer treatments, the researchers asked respondents whether they would change their assessment of price fairness if a treatment did not extend survival but only improved quality of life.

Eighty-two percent of payers said the previously identified fair price for a rare cancer drug would no longer be fair in that scenario. And 69% and 49% of physicians and patients felt previously supported prices would no longer be fair.

The researchers asked payers and physicians whether pricing is fair for currently marketed treatments in the three categories and found alignment between perceptions of fairness for the agents in the study and similar treatments already on the market.

Published online 5 June 2020
Stakeholders Identify Fair Prices For Chronic Drugs For Rare Disease, Curative Treatments In Survey

Chronic rare disease treatments defined in survey as halting disease progression and eliminating symptoms when taken regularly. Curative therapy defined as one-time treatments that completely, permanently eliminate disease. Average annual prices in thousands of dollars.

![Bar chart showing prices for curative and chronic treatments]

Source: Charles River Associates study on pricing for rare disease and curative therapies.