

BUDGETARY IMPACT OF ULTRA RARE DISEASES IN BRAZIL A REVIEW OF DECISIONS FROM THE PAST FIVE YEARS

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OBJECTIVES

In Brazil, rare diseases affect up to 65 per 100,000 people, and ultra-rare diseases impact no more than 1 per 50,000. The National Committee for Health Technology Incorporation (CONITEC) evaluates treatments for these diseases for the Unified Health System (SUS) based on efficacy, safety, and cost effectiveness. This study examines the budget impact of incorporated and non-incorporated technologies, identifying patterns influencing incorporation decisions.

METHODS

This study analyzed technology incorporation decisions for rare and ultra-rare diseases in the SUS over the past five years (2019-2024), focusing on budgetary impact. Publicly available data from CONITEC were evaluated using descriptive statistics, considering total quantity, mean, standard deviation, median, and quartiles (25% and 75%). The analysis included both incorporated and non-incorporated technologies, providing a comprehensive view of the economic factors influencing these decisions.

RESULTS

Public documents were evaluated, identifying 45 technologies divided into non-incorporated (n=23) and incorporated (n=22) groups. Nonincorporated technologies had a higher mean budgetary impact (\$99,387,000 vs. \$68,990,000), while the standard deviation was greater in incorporated technologies (\$121,949,000 vs. \$96,515,000). Quartile analysis revealed cost differences: the first quartile for non-incorporated technologies was \$21,520,000, over twice the \$9,375,000 of incorporated ones. The median for non-incorporated technologies was \$59,812,000, 68% higher than \$35,468,000 for incorporated ones. In the third quartile, non-incorporated technologies reached \$149,360,000, more than double \$67,370,000 for incorporated ones. Dominant incremental cost-effectiveness ratios appeared exclusively among incorporated technologies. These distributions are illustrated in Figures 1 and 2.

Figure 1. Budget impact distribution for **non-incorporated** Technologies for rare diseases in Brazil (n: 23)

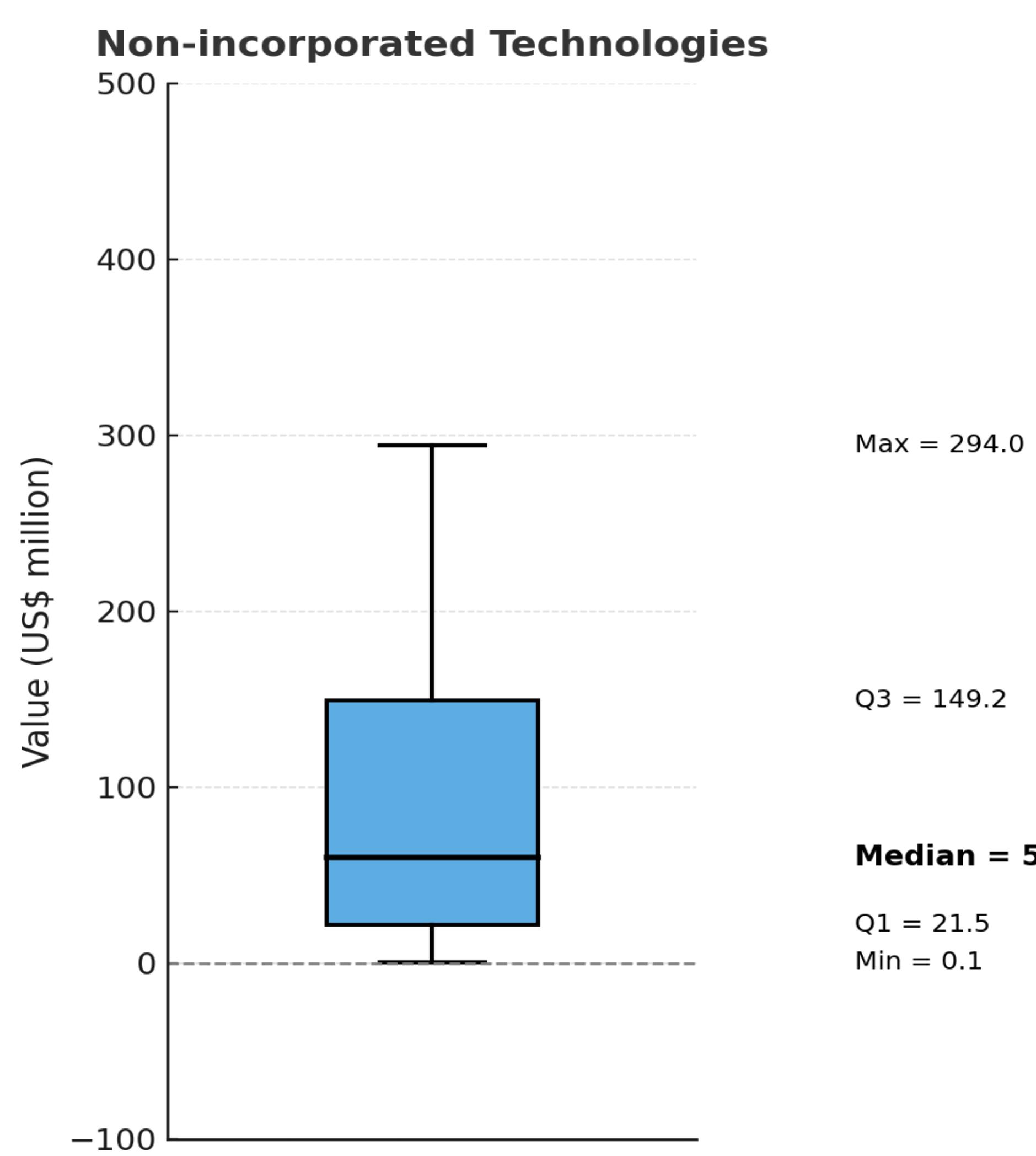
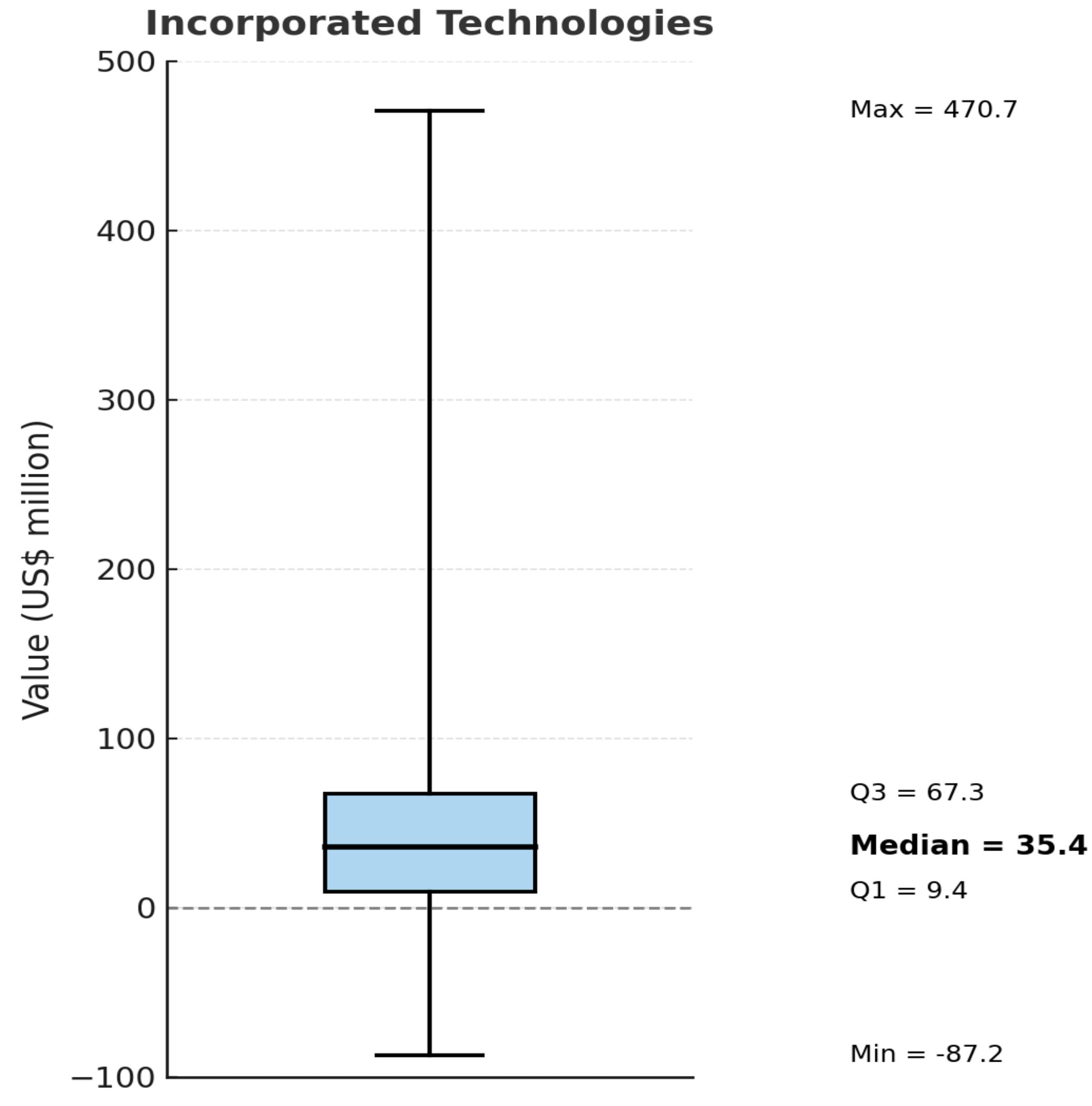


Figure 2. Budget impact distribution for **incorporated** Technologies for rare diseases in Brazil (n: 22)



CONCLUSION

Non-incorporated technologies show higher budgetary impact and cost distribution, highlighting cost as a key factor in non-incorporation for rare diseases in Brazil. Higher cost variability among incorporated technologies suggests diverse cost profiles, while dominant incremental cost-effectiveness ratios exclusively in incorporated technologies emphasize the importance of cost-benefit analysis in decision-making.