

The Cost-Effectiveness of Eteplirsen vs. Standard of Care in Delaying the Progression of Duchenne Muscular Dystrophy

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INTRODUCTION

Duchenne muscular dystrophy (DMD) is a severe progressive genetic disorder characterized by mutation of the DMD gene on the X chromosome affecting **1 in 3500 males worldwide.** ^{1,2,5}

The **standard of care** for DMD includes **corticosteroids** but with **limited long-term benefits.** ³

Eteplirsen is a novel **antisense oligonucleotide** therapy indicated for DMD patients with a mutation amenable to **exon 51 skipping** which further **delays disease progression.** ^{4,5}

Objective: The aim of this study was to analyze the cost-effectiveness of eteplirsen vs. SoC

METHODS

Base Case Scenario

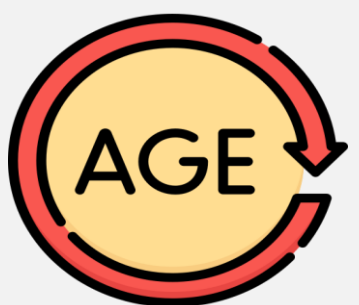
Markov state-transition model was developed using **TreeAge Pro** to simulate health state transitions.



U.S Societal Perspective (direct medical / non-medical and caregiver costs)



Cohort of male patients diagnosed with DMD



5 years of age at diagnosis

Treatment Initiation

Eteplirsen
\$ 300,000/yr

VS.

Prednisolone
\$15,000 / yr



Key assumptions adopted⁵

1-year average progression rate period for each stage



Median life expectancy of 25 years

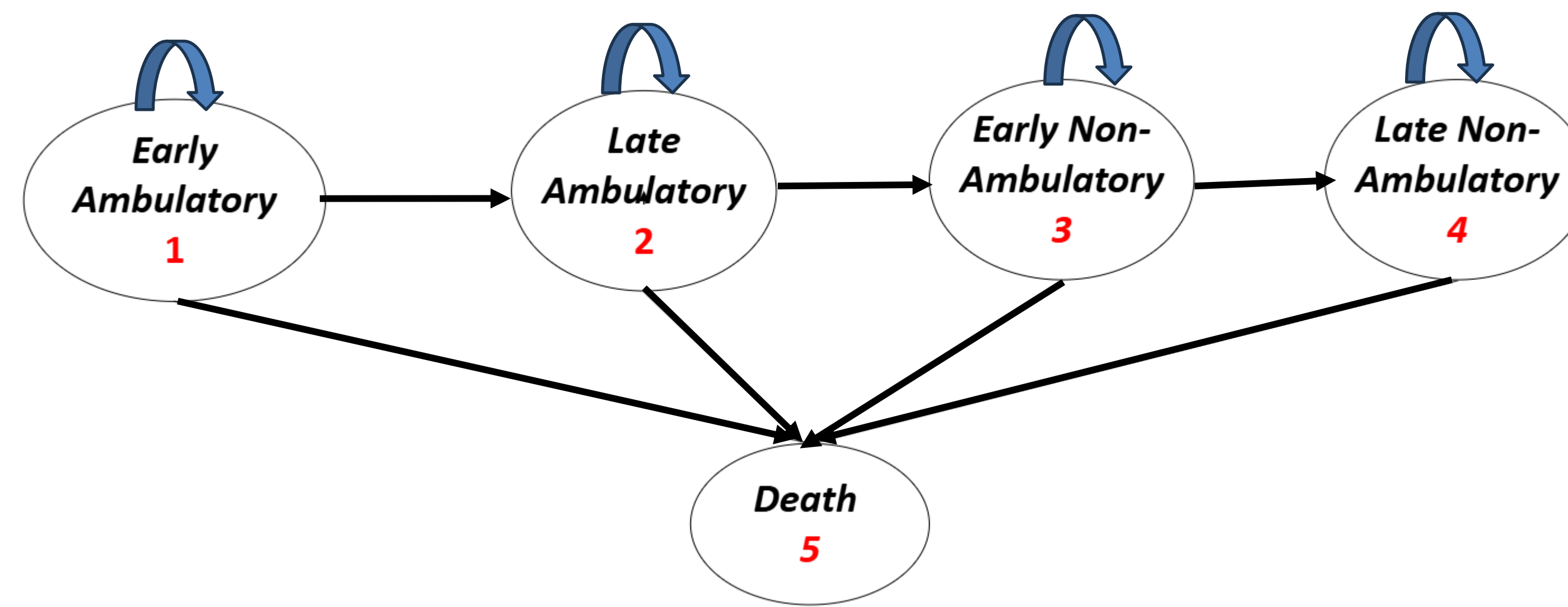
25% Progression rate probability was assumed for scenarios involving hypothetical treatment efficacy.

9% Annual mortality risk for both treatments

SoC reflect typical disease progression with a 1-year average progression rate per stage

Eteplirsen P (staying in each stage) and P(transition probabilities)

Figure 1. Progression of Duchenne Muscular Dystrophy (DMD) through Ambulatory and Non-Ambulatory Stages



†Footnote: Death state (5) is an absorbing state which reflects the terminal nature of DMD progression as patients move through increasingly severe stages. (1)Early Ambulatory – Individuals can walk independently but start experiencing muscle weakness affecting endurance. (2) Late Ambulatory – Mobility declines further, often requiring assistance or aids, though individuals can still walk with support. (3)Early Non-Ambulatory – Walking ability is lost, and individuals require a wheelchair and support for daily activities. (4) Late Non-Ambulatory – Severe muscle weakness impacts respiratory and cardiac functions, requiring intensive, full-time care.

RESULTS



Incremental Costs
4,784,113.27 (USD)



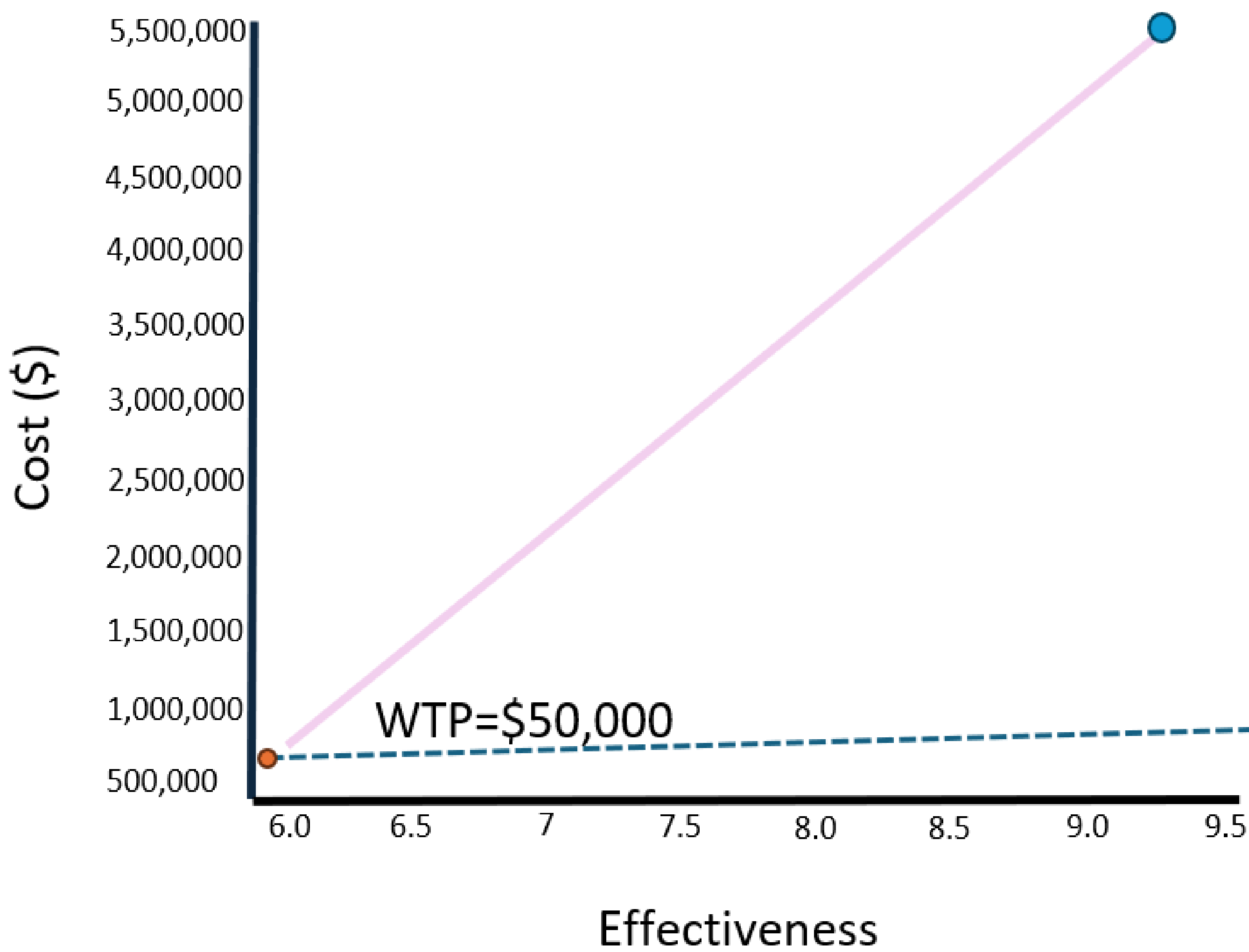
Incremental QALYs
3.24



Incremental Cost per QALY Gained
\$ 1,476,578 / QALY

Eteplirsen demonstrated enhanced effectiveness across **all stages of the disease** when compared to SoC, resulting in a **greater cumulative number of QALYs.**

Figure 2. Cost - Effectiveness Analysis



Note: The ICER for eteplirsen was \$1,476,578 per QALY, which is significantly higher than the WTP threshold. Eteplirsen is shown to be more expensive but demonstrates greater effectiveness compared to the standard of care (prednisolone), with an ICER of 1481150. The cost-effectiveness significantly exceeds the willingness-to-pay threshold of \$50,000.

Abbreviations:

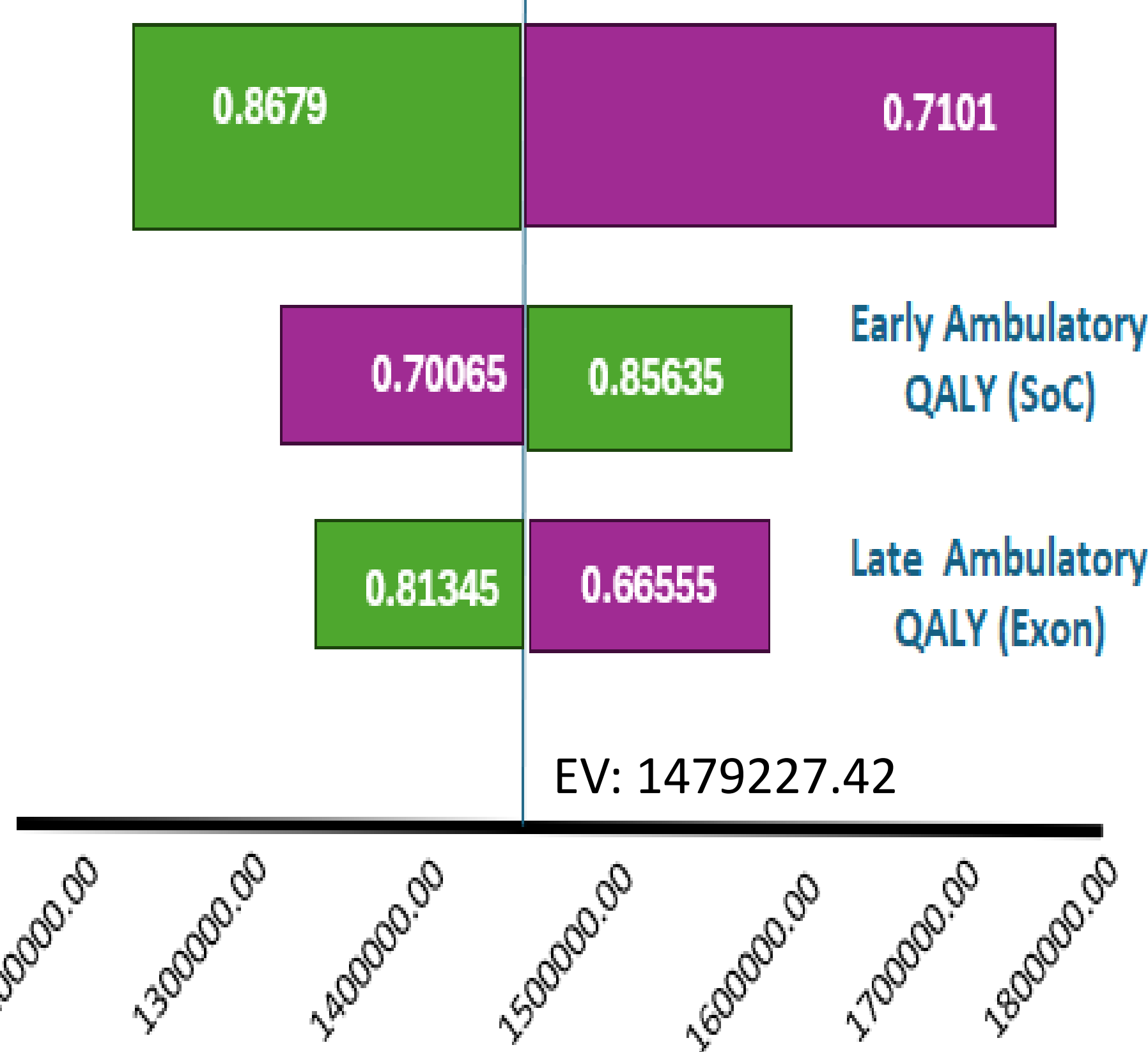
DMD (Duchenne Muscular Dystrophy); **SoC** (Standard of Care); **QALY** (Quality-Adjusted Life Year); **ICER** (Incremental Cost-Effectiveness Ratio); **WTP** (Willingness-To-Pay); **EA** (Early Ambulatory); **LA** (Late Ambulatory); **yr** (Year); **USD** (United States Dollar)

Disclosures:

There are no conflicts of interest to disclose. This work was funded by Rutgers University, independent of any external sponsors or funds.

Figure 3 . Probabilistic Sensitivity Analysis

Tornado Diagram ICER: Eteplirsen vs. SoC



Note: The sensitivity analysis determined QALYs in the early ambulatory (EA) phase of eteplirsen treatment to be the most influential variable.

DISCUSSION

- Given that the ICER is significantly higher than the WTP threshold, Eteplirsen is **not cost-effective** when compared to the current standard of care.
- Ensuring precision of QALY measurements is critical for accuracy of the model, as discerned by the sensitivity analysis.
- Thus, subsequent studies may choose to focus on the efficacy of Eteplirsen in the early ambulatory phase specifically. However, Eteplirsen serves a very limited patient population (13% of DMD patients).
- Additionally, it only has results indicating a delay of the progression of the disease rather than a cure for DMD.
- Further research is needed into the disease state of DMD and other therapies as a whole, considering the high emotional and financial burden on patients and their caregivers.

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