Decision Modeling for Public Health

Training provided by DARTH Workgroup

CDC Steven M. Tseutch Prevention Effectiveness Fellowship

The DARTH Workgroup

- The Decision Analysis in R for Technologies in Health (DARTH) Workgroup is a multi-institutional collaboration of researchers
- Aim to **expand knowledge** in decision analysis, **increase accessibiliy** of decision modeling, and **empower** others to construct R-based decision models

For more information

http://www.darthworkgroup.com/

DARTH Instructors

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Assisted by:

- Alan Yang, Hospital for Sick Children
- Margo Wheatley, University of Minnesota
- Tanvi Chiddarwar, University of Minnesota
- Zongbo Li, University of Minnesota

DARTH Publications

An Overview of R in Health Decision Sciences

Hawre Jalal, MD, PhD, Petros Pechlivanoglou, MSc, PhD, Eline Krijkamp, MSc, Fernando Alarid-Escudero, MSc, Eva Enns, MS, PhD, M. G. Myriam Hunink, MD, PhD

Microsimulation Modeling for Health Decision Sciences Using R: A Tutorial

Eline M. Krijkamp, Fernando Alarid-Escudero, Eva A. Enns, Hawre J. Jalal, M. G. Myriam Hunink, and Petros Pechlivanoglou

MDM Medical Decision Making

Medical Decision Making
2018, Vol. 38(3) 400–422

DOI: 10.1177/0272989X18754513
journals.sagepub.com/home/mdm
SAGE

Brief Report

MDM Medical Decision Making

Medical Decision Making
1–7

DOI: 10.1177/0272989X19893973

A Multidimensional Array Representation of State-Transition Model Dynamics

Eline M. Krijkamp*, Fernando Alarid-Escudero*, Eva A. Enns, Petros Pechlivanoglou, M.G. Myriam Hunink, Alan Yang, and Hawre J. Jalal

PharmacoEconomics (2019) 37:1329–1339
https://doi.org/10.1007/s40273-019-00837-x

PRACTICAL APPLICATION

Check for updates

A Need for Change! A Coding Framework for Improving Transparency in Decision Modeling

Fernando Alarid-Escudero[1] · Eline M. Krijkamp[2] · Petros Pechlivanoglou[3] · Hawre Jalal[4] · Szu-Yu Zoe Kao[5] · Alan Yang[6] · Eva A. Enns[5]

arXiv.org > stat > arXiv:2001.07824

Statistics > Applications

[Submitted on 22 Jan 2020 (v1), last revised 19 Aug 2021 (this version, v3)]

An Introductory Tutorial on Cohort State-Transition Models in R Using a Cost-Effectiveness Analysis Example

Fernando Alarid-Escudero, Eline M. Krijkamp, Eva A. Enns, Alan Yang, M.G. Myriam Hunink, Petros Pechlivanoglou, Hawre Jalal

DARTH Workshops

SMDM Society for Medical Decision Making

Better Health through Better Decisions.

40TH ANNUAL NORTH AMERICAN MEETING

October 13 - October 17, 2018 | Montreal, QC, Canada

AM6: Hands-on Model Calibration in R

PM5: Survival Analysis in Decision Modeling

PM7: Sensitivity Analysis and Value of Information Analysis Using Regression Metamodeling

PM8: Microsimulation Modeling in R

SickKids®

Advanced **Decision Modeling in R:** A 3-day workshop

When: April 1st, 2nd, & 3rd, 2020

Where: The Hospital for Sick Children, Toronto, Canada

SCHOOL OF PUBLIC HEALTH

UNIVERSITY OF MINNESOTA

Cost-Effectiveness and Decision Modeling in R: A 3-day course

When: July 9th, 10th & 11th, 2019

Where: University of Minnesota, Minneapolis, MN

Day 1: Introduction to cost-effectiveness and decision trees in R

Day 2: Markov models and probabilistic sensitivity analysis in R

Day 3: Microsimulation modeling and model calibration in R

Workshop Overview

Part I: Foundations

- 4-day series
- Topic: ***Concepts and examples*** of decision modeling and model-based cost-effectiveness analysis for public health applications
- Includes opportunities for fellows to discuss and workshop *model design* for fellowship projects

Part II: Hands-on Modeling

- 4-day series (Dec. 5-8)
- Topic: ***Implementation*** of decision analytic models in the R programming language
- Includes opportunity to *implement models* in R relevant to fellowship projects

Workshop Overview

- Workshop website:

https://decision-modeling-cdc-2022-23.netlify.app/

- Getting help

Contact

alan.yang@sickkids.ca

Slack workspace

slack

Launching CDC Decision Modeling for Public Health 2022-23

Introduction to Decision Modeling

Decision Modeling for Public Health

November 14, 2022

Overview of Decision Analysis

What is Decision Analysis?

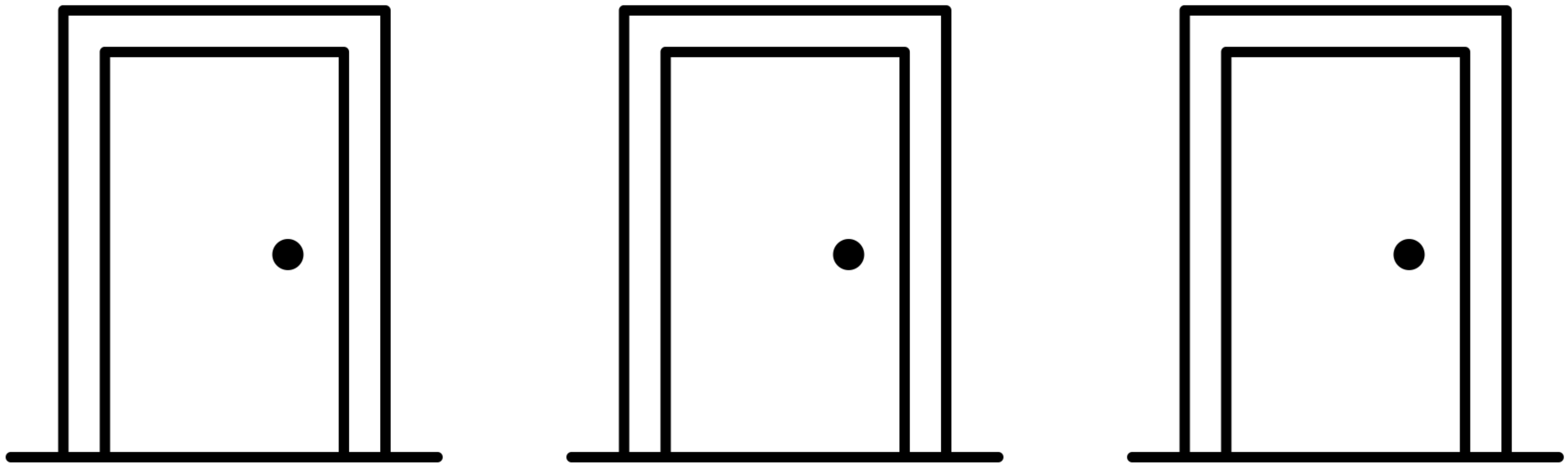
- Explicit, quantitative and systematic approach to decision making under *uncertainty*
- Identify, measure, and value the consequences of different alternatives as well as the uncertainty that exists when the decision needs to be made
- Help structure the analyst's thinking and facilitate the communication of assumptions
- Provide a structural framework for synthesizing data from disparate sources and allows for *extrapolation*
- Elements are incorporated into a mathematical *model* which is used to estimate the outcomes of different options or interventions

Decision Analysis

Option 1

Option 2

Option 3



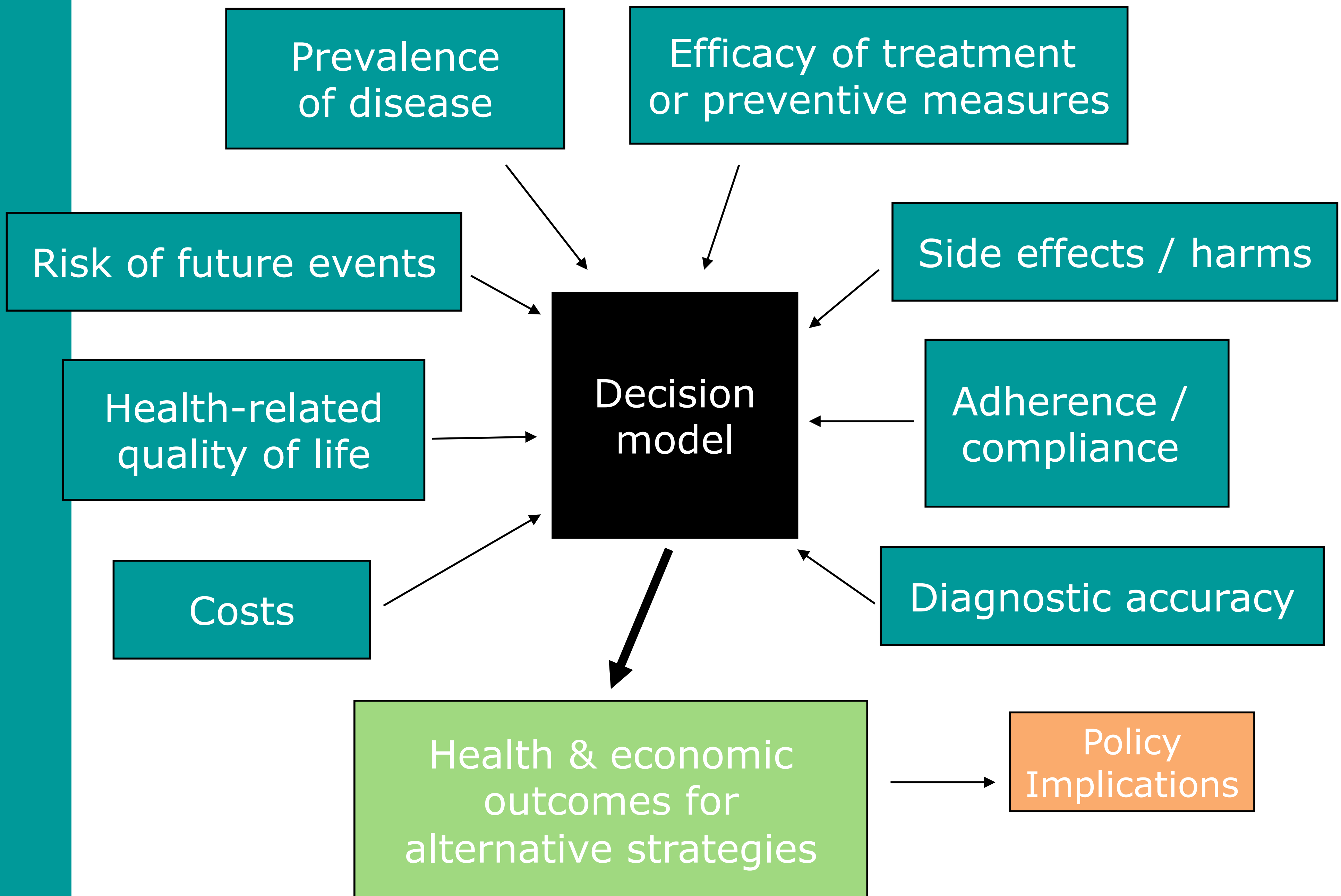
Created by Icons Bazaar
from Noun Project

Benefits?

Harms?

Cost?

Created by Vectors Point
from Noun Project


Prevalence of disease
Efficacy of treatment or preventive measures
Risk of future events
Side effects / harms
Decision model
Health-related quality of life
Adherence / compliance
Costs
Diagnostic accuracy
Health & economic outcomes for alternative strategies
Policy Implications

Model Types

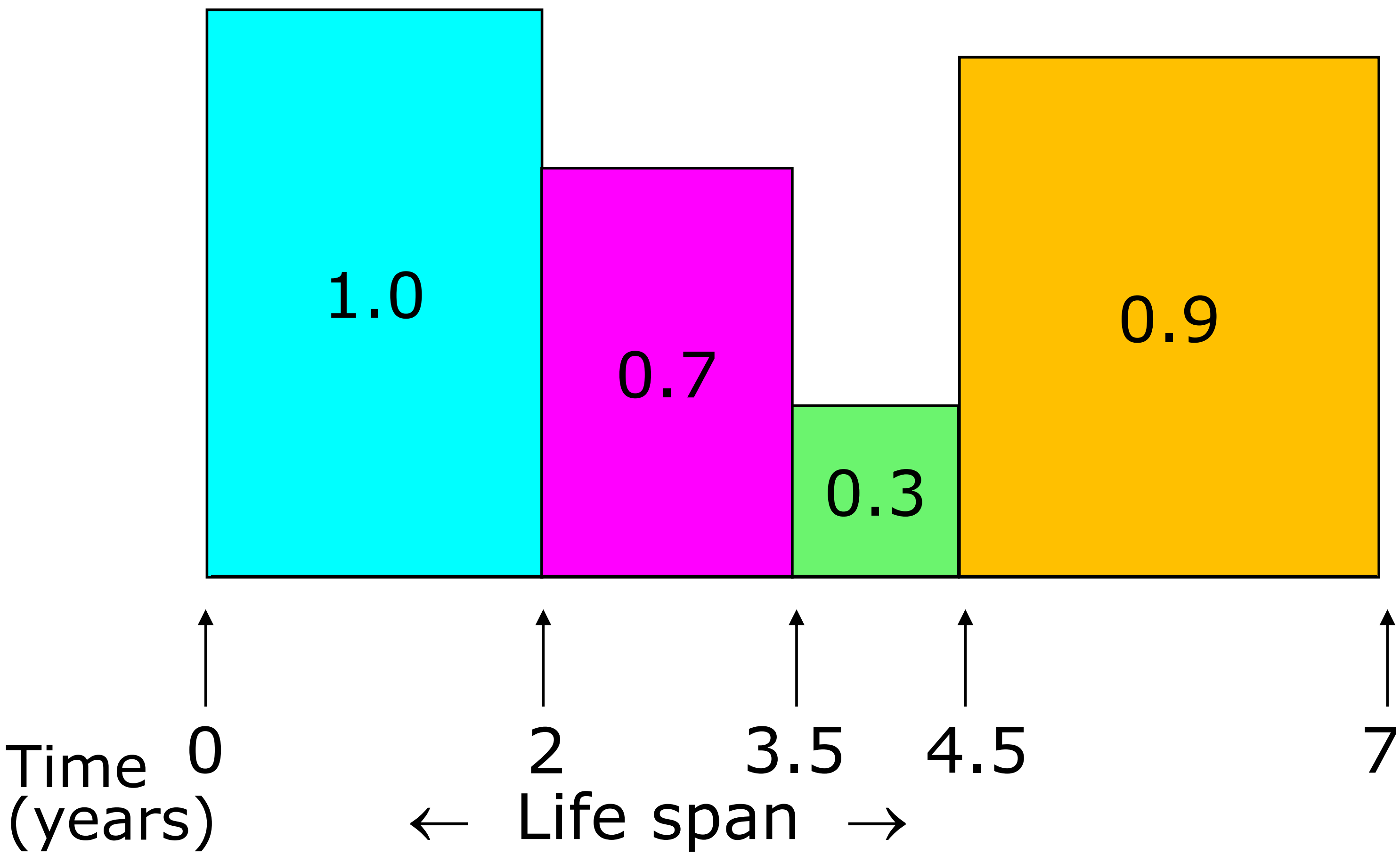
- Decision trees
  - Schematic representation of uncertain events/consequences of different alternatives
- Cohort state transition models
  - Dynamic model that captures disease progression and other events
  - Simulates a homogeneous cohort
  - Usually deterministic
- Individual-based models / microsimulation
  - Stochastic dynamic model
  - Simulates individuals
  - Most flexible model type

Analysis Outcomes

Health Outcomes

- Disease-specific
  - Intermediate clinical markers
  - Cases averted
  - Events averted
- Generic
  - Lives saved / deaths averted
  - Life-years gained
  - Quality-adjusted life-years (QALYs) gained

Quality-Adjusted Life-Years



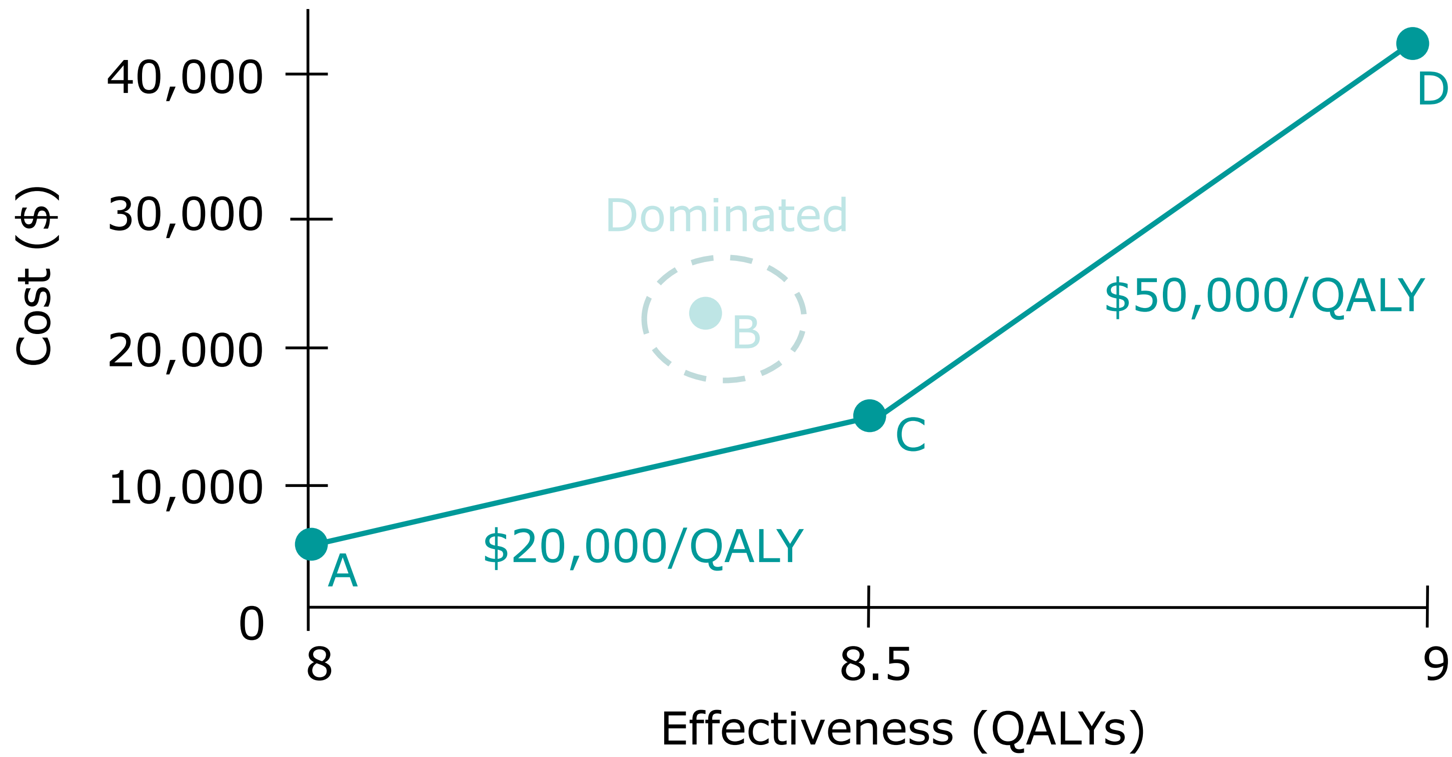
$$\text{QALYs} = (2)(1) + (1.5)(.7) + (1)(.3) + (2.5)(.9) = 5.6$$

Costs

- Formal healthcare sector
  - Facilities and resources
  - Drugs and devices
  - Personnel time
- Informal healthcare sector
  - Patient time
  - Unpaid caregiver time
  - Transportation costs
- Non-healthcare sector
  - Legal or criminal justice
  - Education
  - Housing

Cost-Effectiveness Analysis

- Subset of decision analytic questions where the objective is to balance costs and health benefits
- Defined willingness-to-pay per unit of health benefit (also called cost-effectiveness threshold)



Strengths and Challenges of Decision Modeling

Strengths of Modeling

- Clarifies decision-making
- Can use data from different sources
- Allows explicit and systematic characterization of uncertainty
- Extrapolates short-term observations into long-term outcomes
  - Can translate intermediate endpoints into life-years or QALYs gained
- Encourages “what if” analyses

Challenges of Modeling

- Validation issues
  - Model may be incorrectly specified (wrong structure)
  - Data to inform input parameter values may be lacking or of poor quality
- Not all decision considerations lend themselves well to modeling
- Communication issues
  - Transparency
  - Trust

Case Study Discussion

Eskander 2021:

To Ban or Not to Ban Tanning Bed Use for Minors

- Study question, design, perspective
- Outcome measures
- Main takeaways
- General impressions

Decision Trees

Decision Modeling for Public Health

November 14, 2022

Decision Tree (a type of model)

- Schematic representation of all of the important outcomes of a decision (e.g., clinical, economic, non-health sectors)
- Used to combine knowledge about decision problem from many sources
- Computes *average outcomes* (e.g., costs, events, QALYs) from decisions

Components of a decision tree

1. The alternative strategies of a decision making process

2. The events that follow from application of any of these strategies and their probabilities

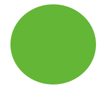
3. The outcomes (for an individual, a cohort or a population)

Structure of a decision tree

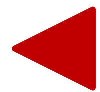
- A tree consists of 3 different types of *nodes* connected via *branches*:

1. A decision tree starts with a *decision node*, which represents the choices a decision maker has between mutually exclusive strategies

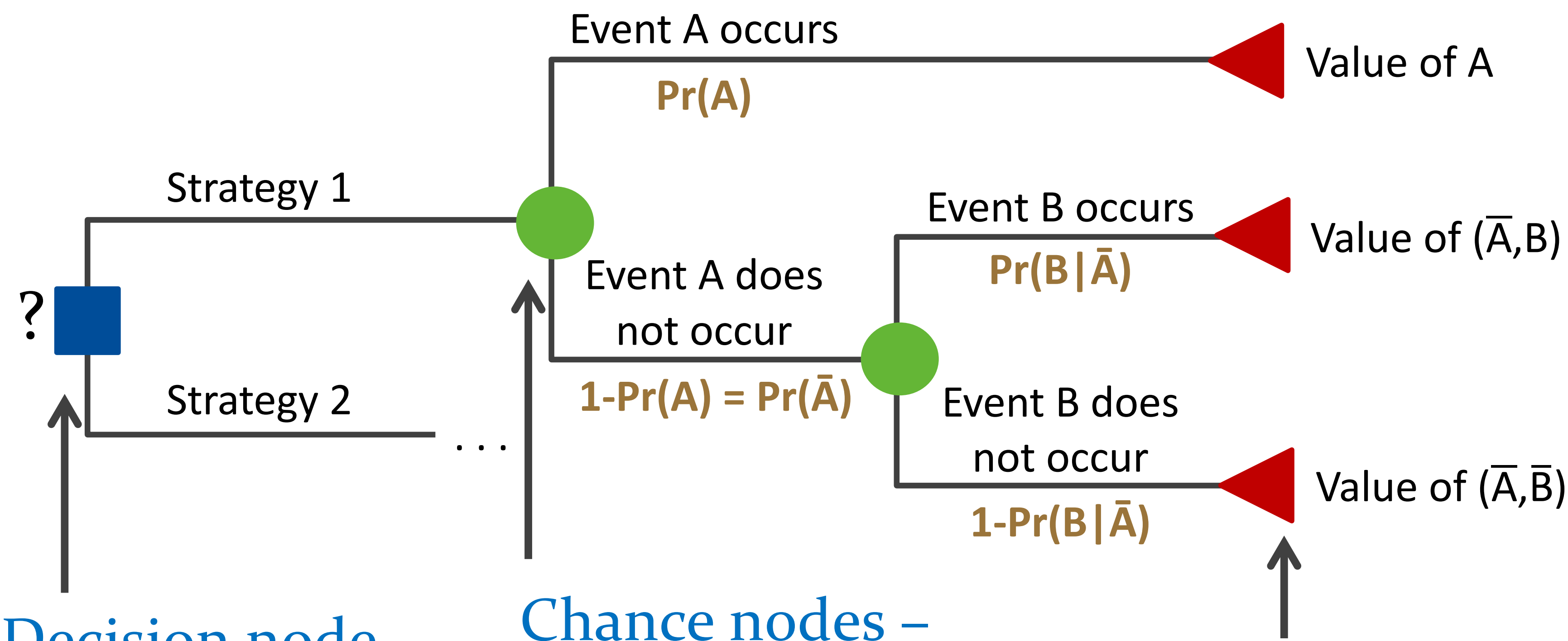
2. A *chance node* represents possible events that could occur following a decision or a previous event. We include probabilities of these events in the tree.



3. A *terminal node* represents end points of each complete branch and the outcome associated with it.



Structure of a decision tree



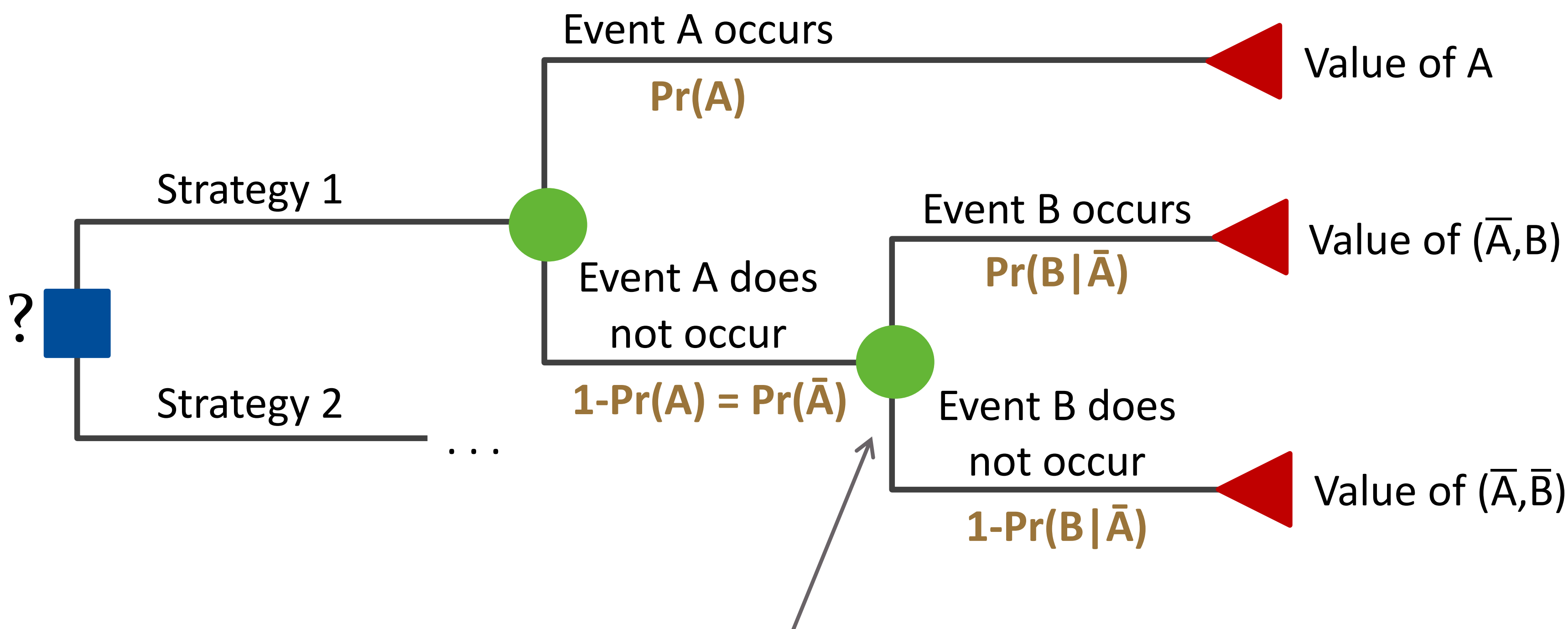
Decision node – list all relevant alternatives

Chance nodes – specify probabilities of uncertain events (must sum to 1)

Terminal nodes – specify the valuation of the event path

Evaluate a decision tree

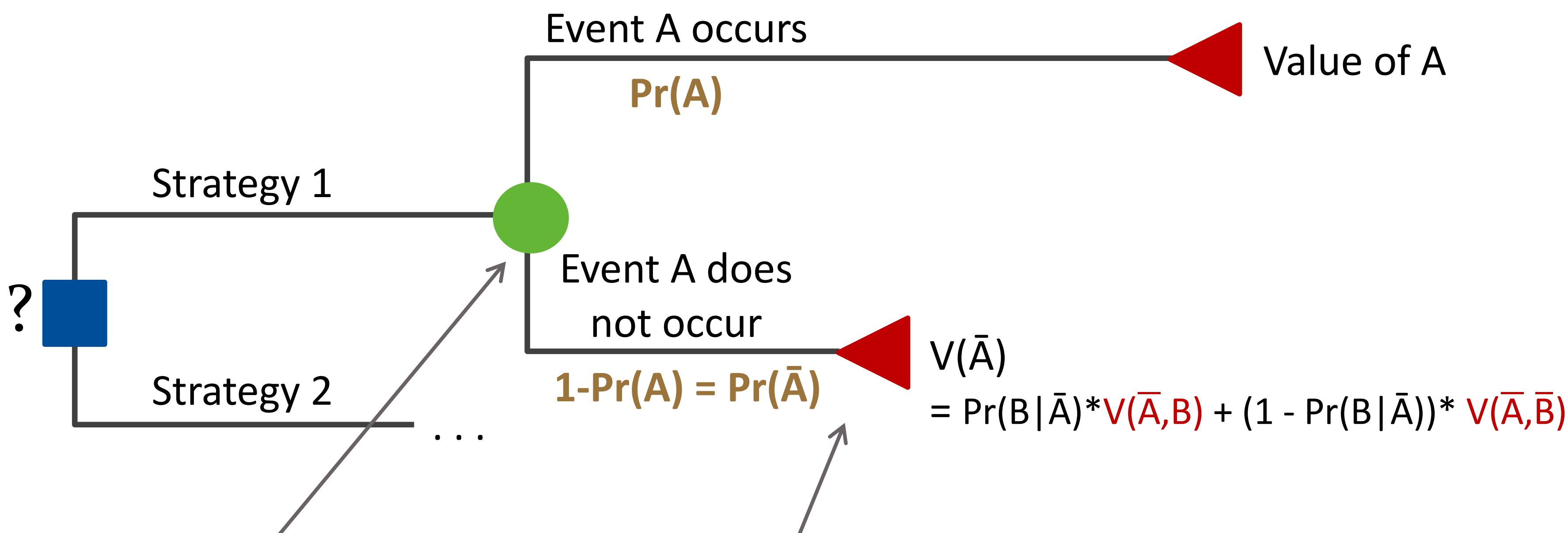
"Roll back" the tree



$$E[V(\bar{A})] \;=\; Pr(B|\bar{A})*V(\bar{A},B) + (1 - Pr(B|\bar{A}))*V(\bar{A},\bar{B})$$

Evaluate a decision tree

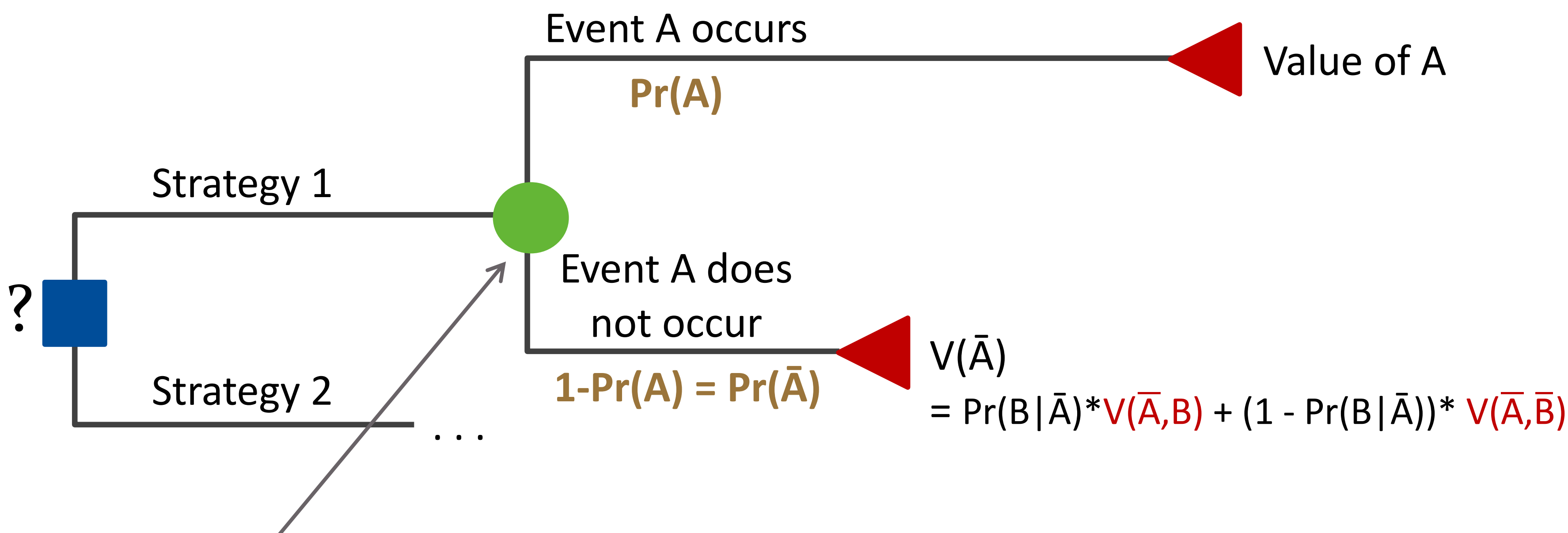
“Roll back” the tree



E[Strategy 1] = Pr(A)*V(A) + (1-Pr(A))* V(Ā)

Evaluate a decision tree

"Roll back" the tree



$$\begin{aligned} E[\text{Strategy 1}] &= Pr(A)*V(A) + (1-Pr(A))*V(\bar{A}) \\ &= Pr(A)*V(A) + (1-Pr(A))*[Pr(B|\bar{A})*V(\bar{A},B) + (1-Pr(B|\bar{A}))*V(\bar{A},\bar{B})] \end{aligned}$$

Simple Decision Tree Example

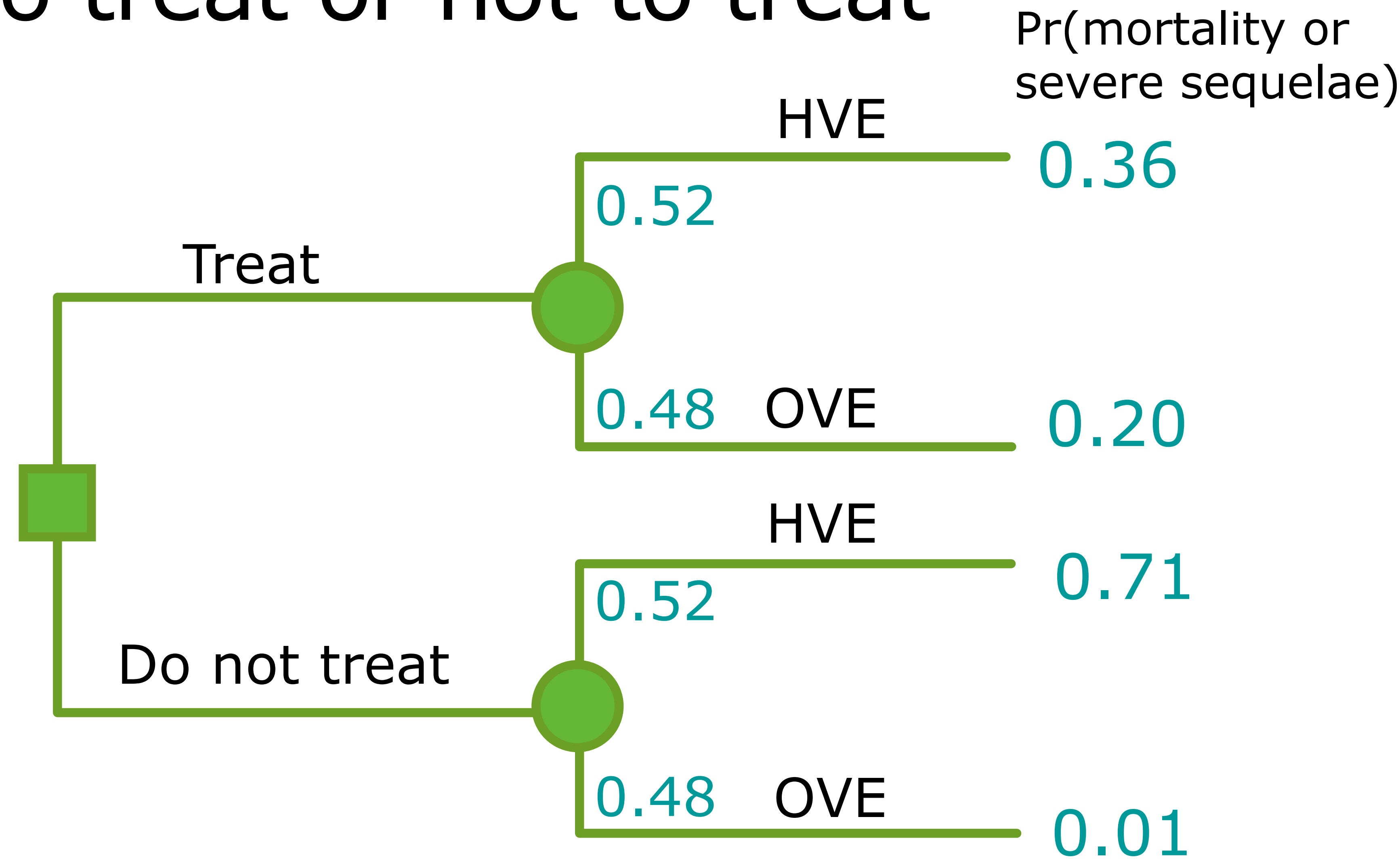
Decision Tree Example

- Viral encephalitis can be caused by herpes virus (HVE) or other viruses (OVE); Pr(HVE) = 52%.
- Untreated HVE leads to death or severe sequelae in 71%; for OVE the figure is 1%.
- A drug, vidarabine, decreases mortality or severe sequelae due to HVE from 71% down to 36%.
- Side effects cause an increase in mortality among OVE patients treated with vidarabine from 1% to 20%.

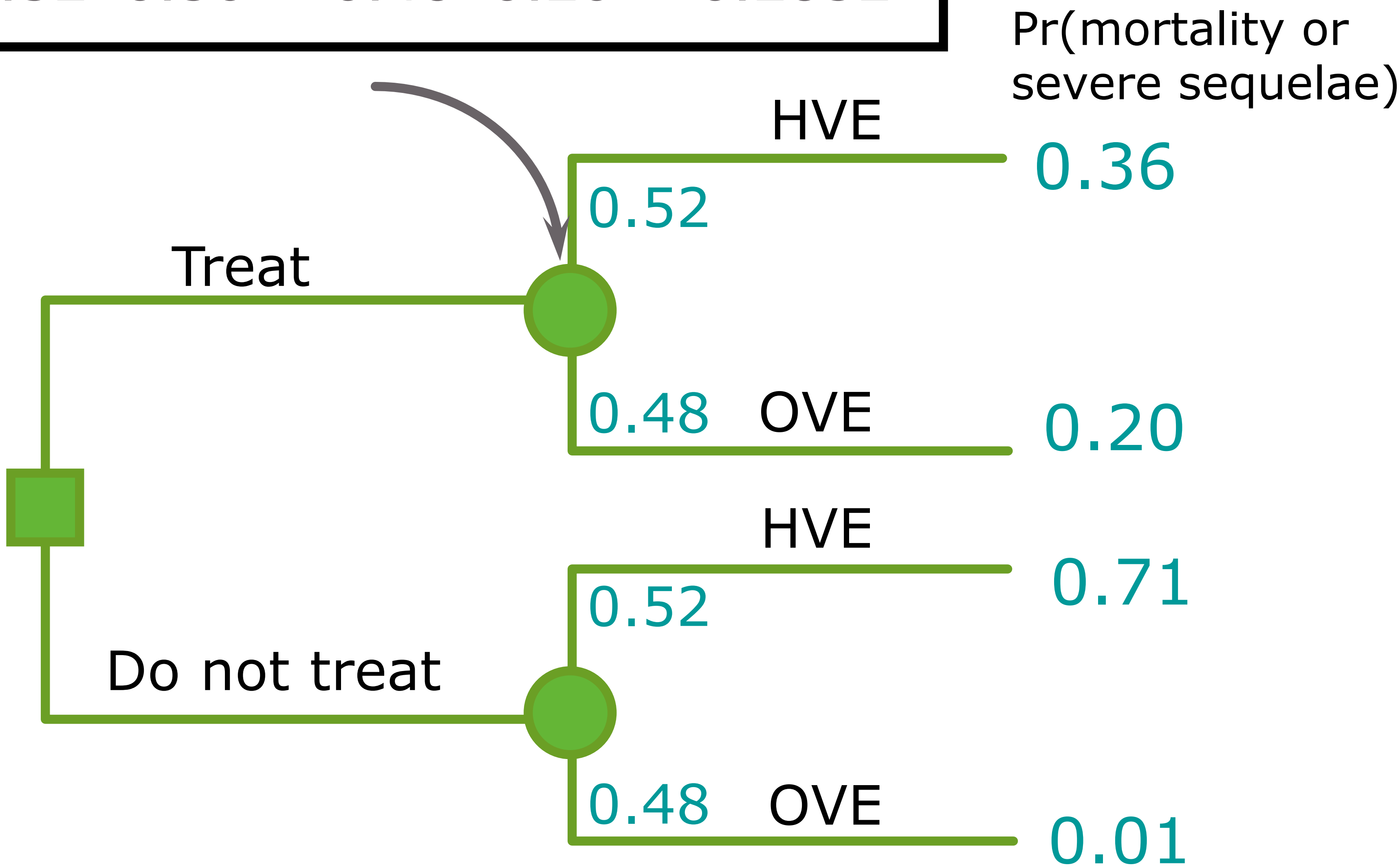
Summarize Model Parameters

Variable	Variable Name	Value
Prevalence of HVE	p_HVE	0.52
Probability of complications (death or sequelae) without treatment		
HVE	p_HVE_comp	0.71
OVE	p_OVE_comp	0.01
Probability of complications (death or sequelae) with vidarabine treatment		
HVE	p_HVE_comp_tx	0.36
OVE	p_OVE_comp_tx	0.20

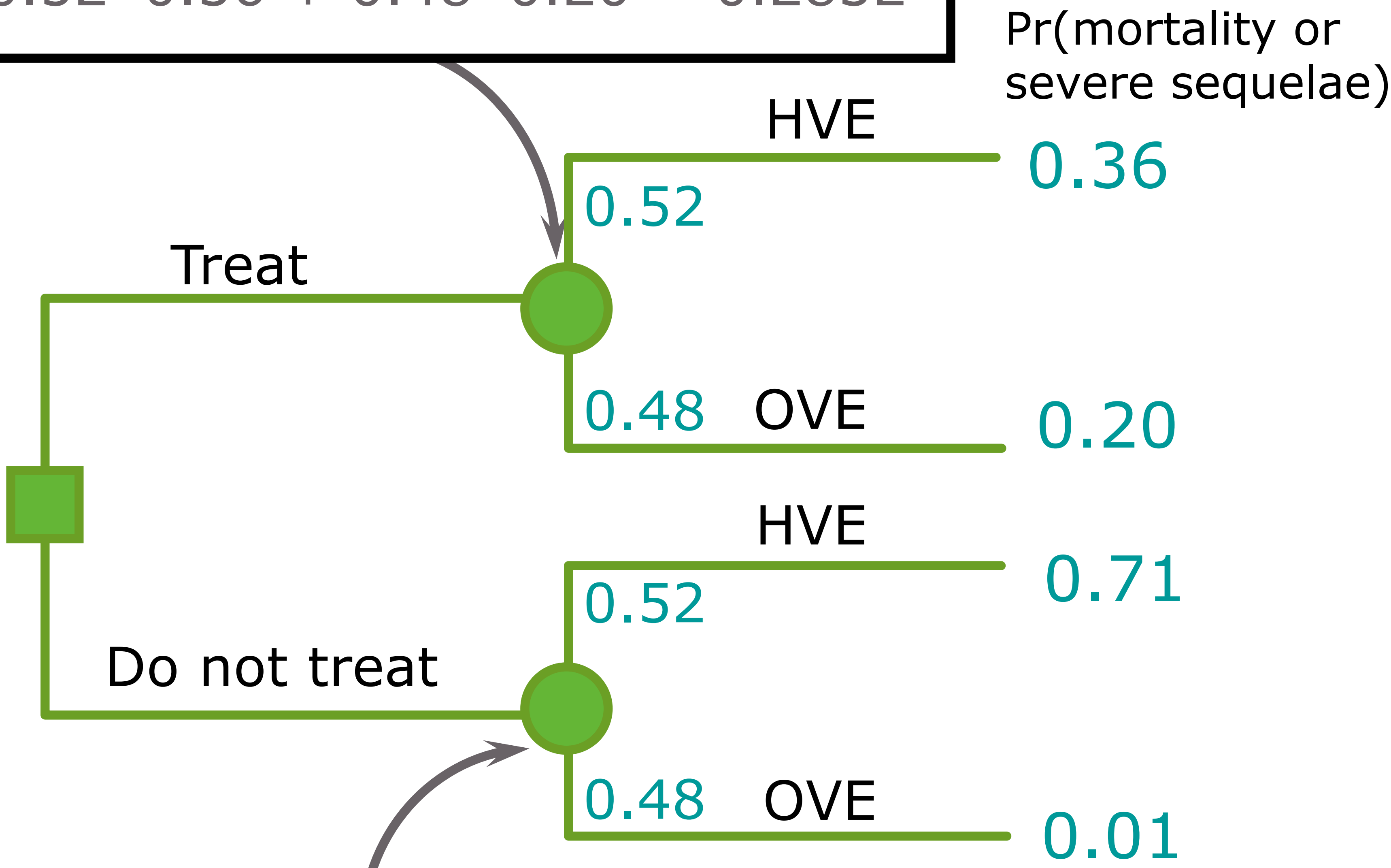
To treat or not to treat



$$0.52*0.36 + 0.48*0.20 = 0.2832$$

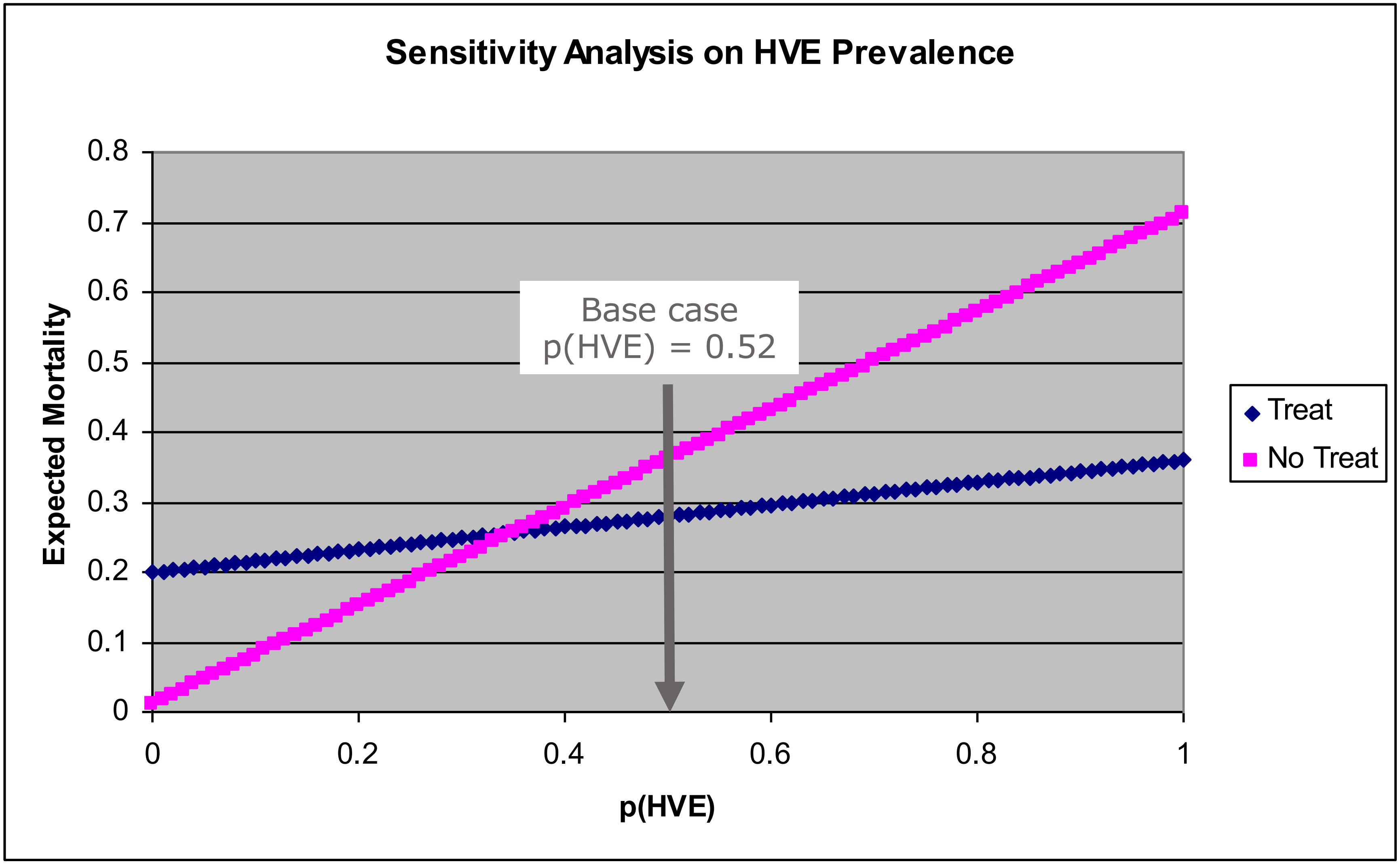


$0.52*0.36 + 0.48*0.20 = 0.2832$

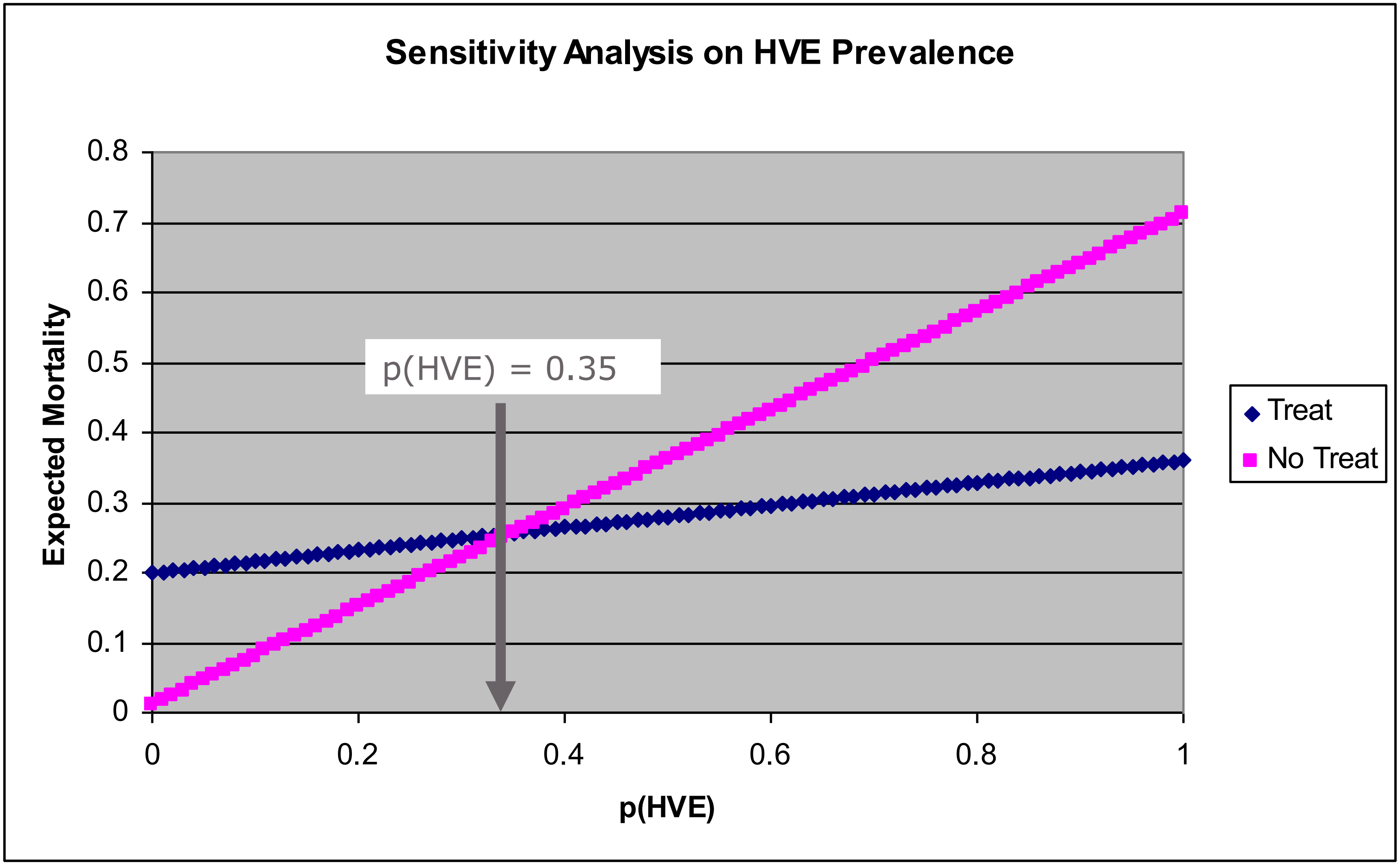


$0.52*0.71 + 0.48*0.01 = 0.3740$

One-Way Sensitivity Analysis



Threshold Analysis

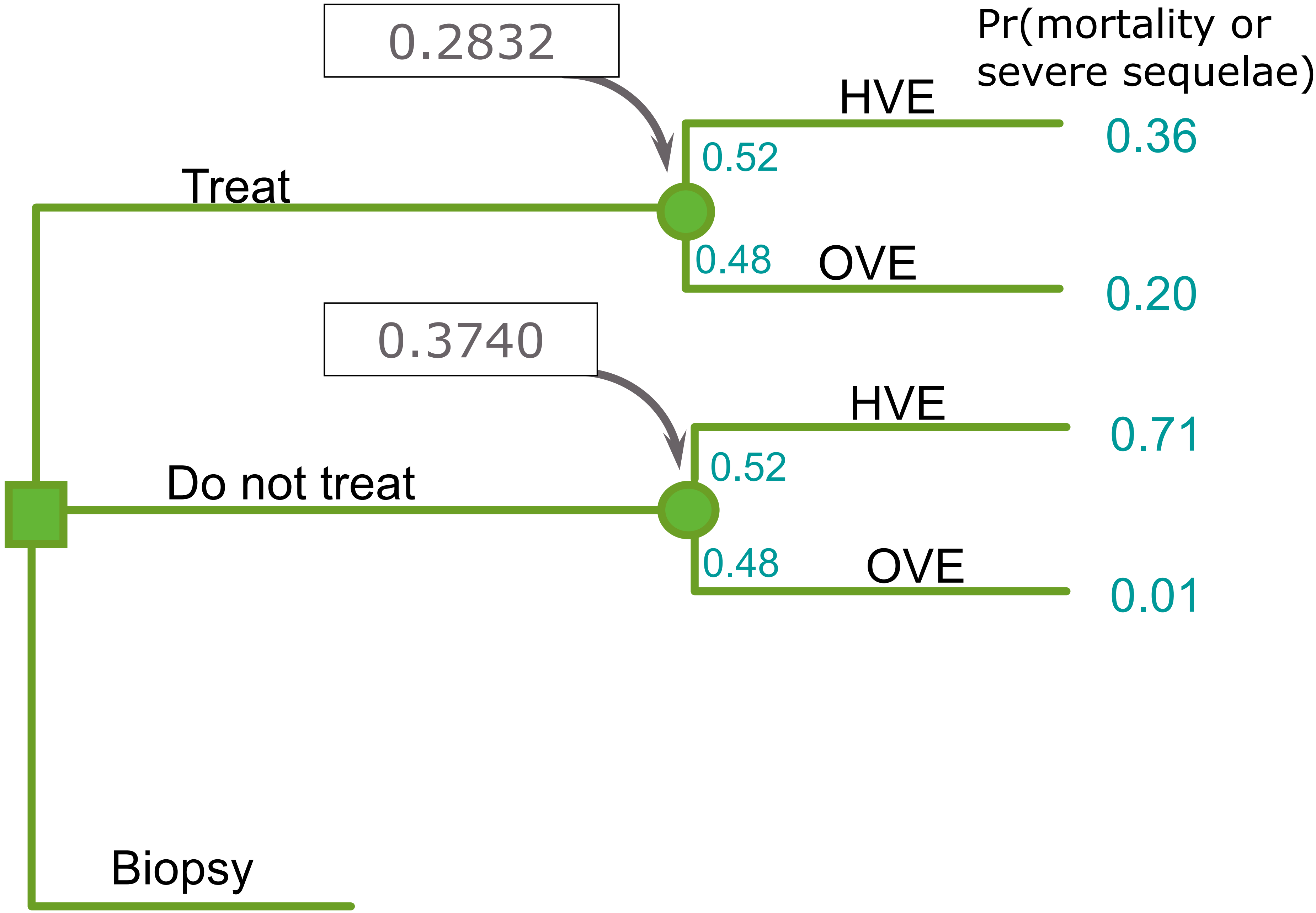


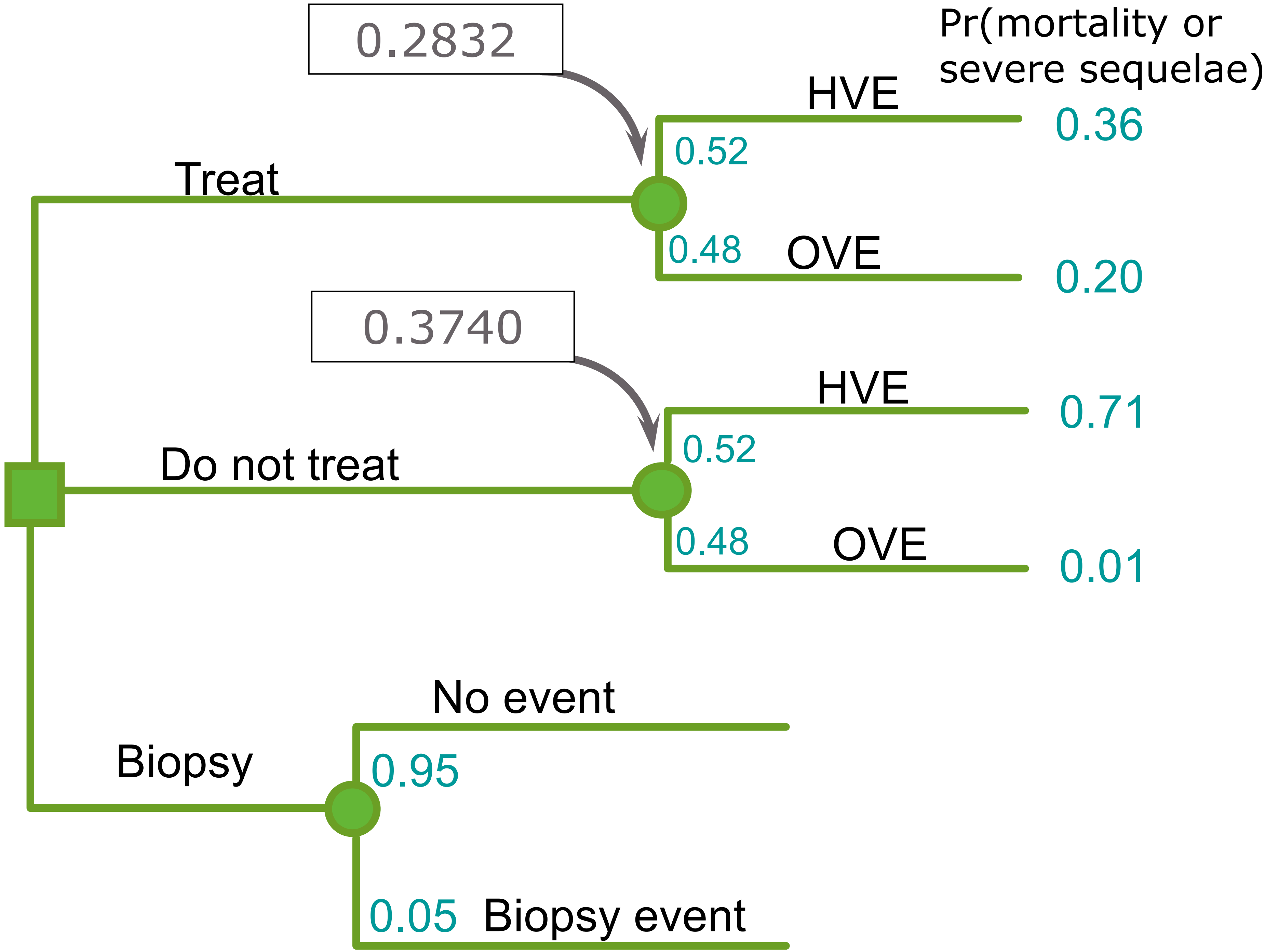
There is a third option

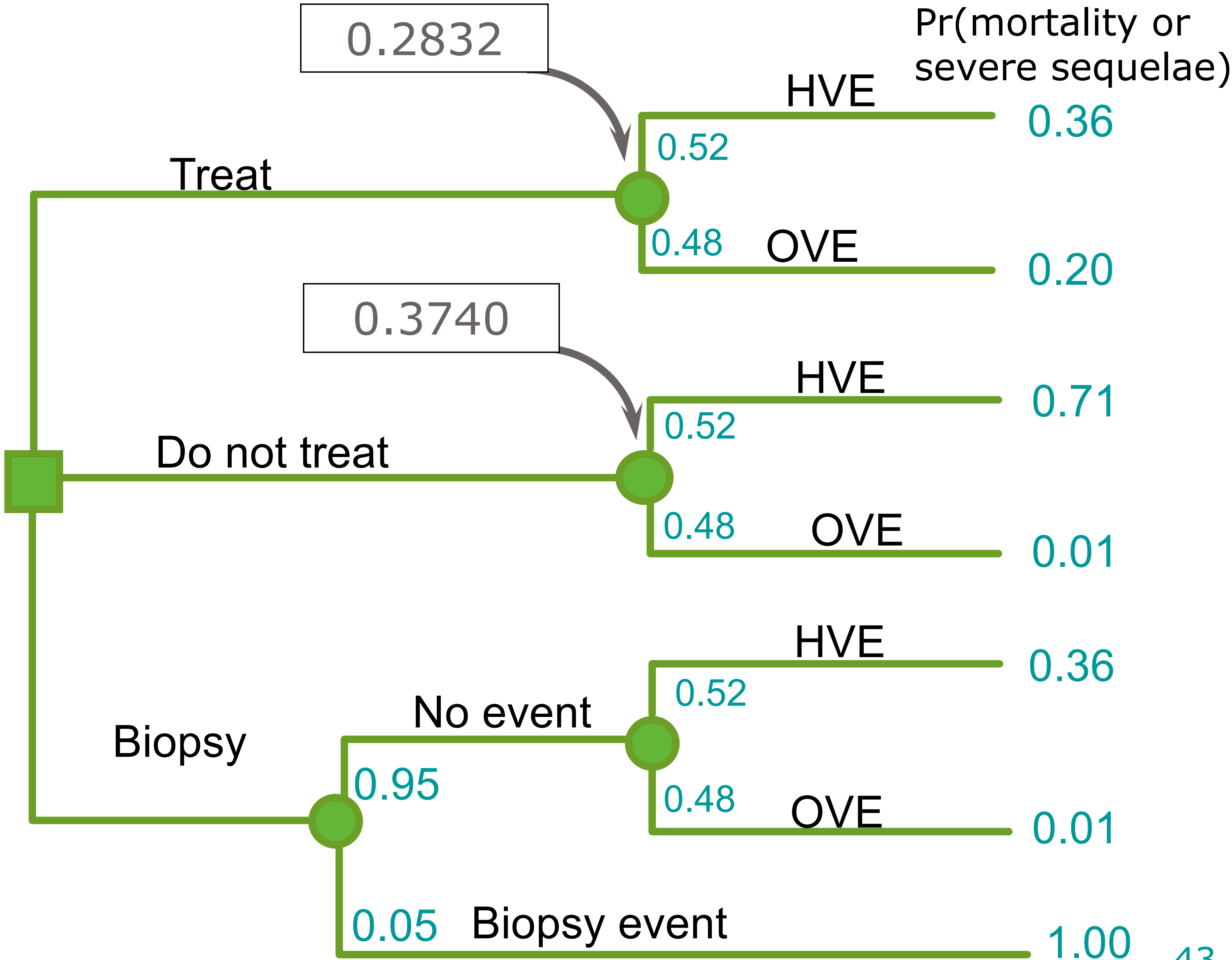
It is possible to obtain a definitive diagnosis by means of brain biopsy, but this procedure itself carries a rate of mortality or severe sequelae of 5%.

Add one more parameter

Variable	Variable Name	Value
Prevalence of HVE	p_HVE	0.52
Probability of complications (death or sequelae) without treatment		
HVE	p_HVE_comp	0.71
OVE	p_OVE_comp	0.01
Probability of complications (death or sequelae) with vidarabine treatment		
HVE	p_HVE_comp_tx	0.36
OVE	p_OVE_comp_tx	0.20
Probability of complications due to brain biopsy	p_biopsy_comp	0.05


0.2832
Pr(mortality or severe sequelae)
HVE
0.36
0.52
Treat
0.48
OVE
0.20
0.3740
HVE
0.71
0.52
Do not treat
0.48
OVE
0.01
Biopsy


0.2832
Pr(mortality or severe sequelae)
HVE
0.36
0.52
Treat
0.48
OVE
0.20
0.3740
HVE
0.71
0.52
Do not treat
0.48
OVE
0.01
No event
0.95
Biopsy
0.05
Biopsy event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OVE
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HVE
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No event
Biopsy
0.95
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OVE
0.01
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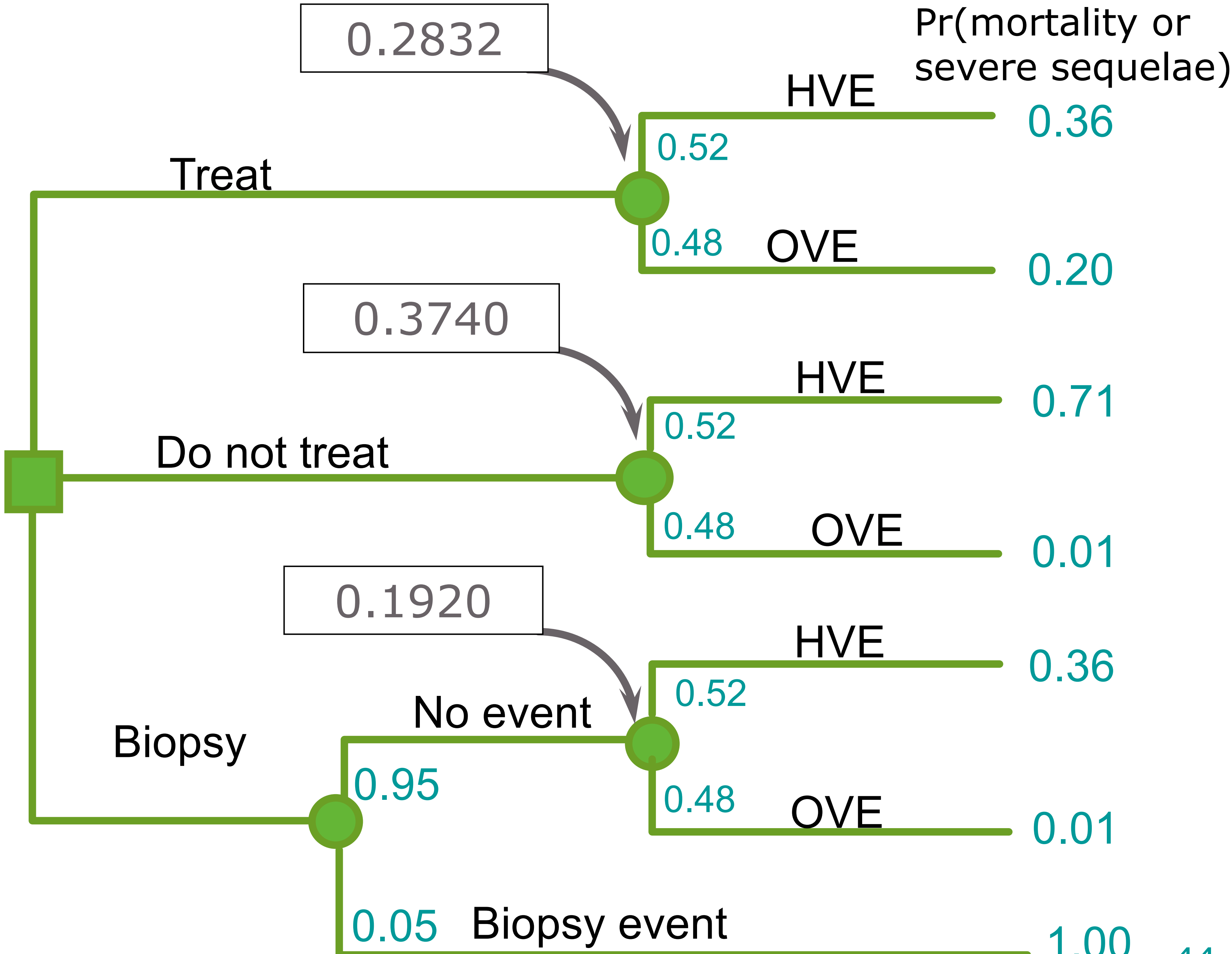
Biopsy event
1.00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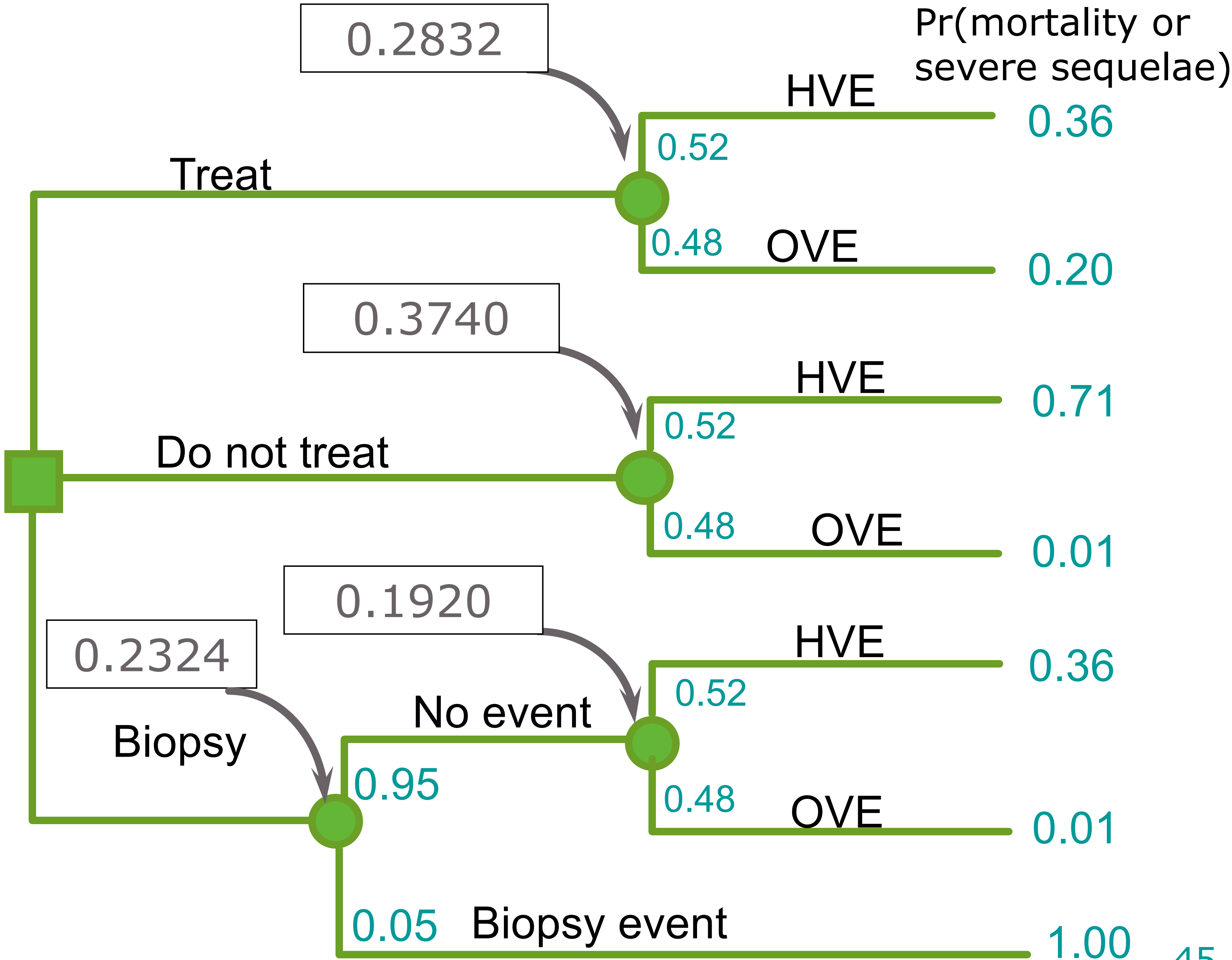
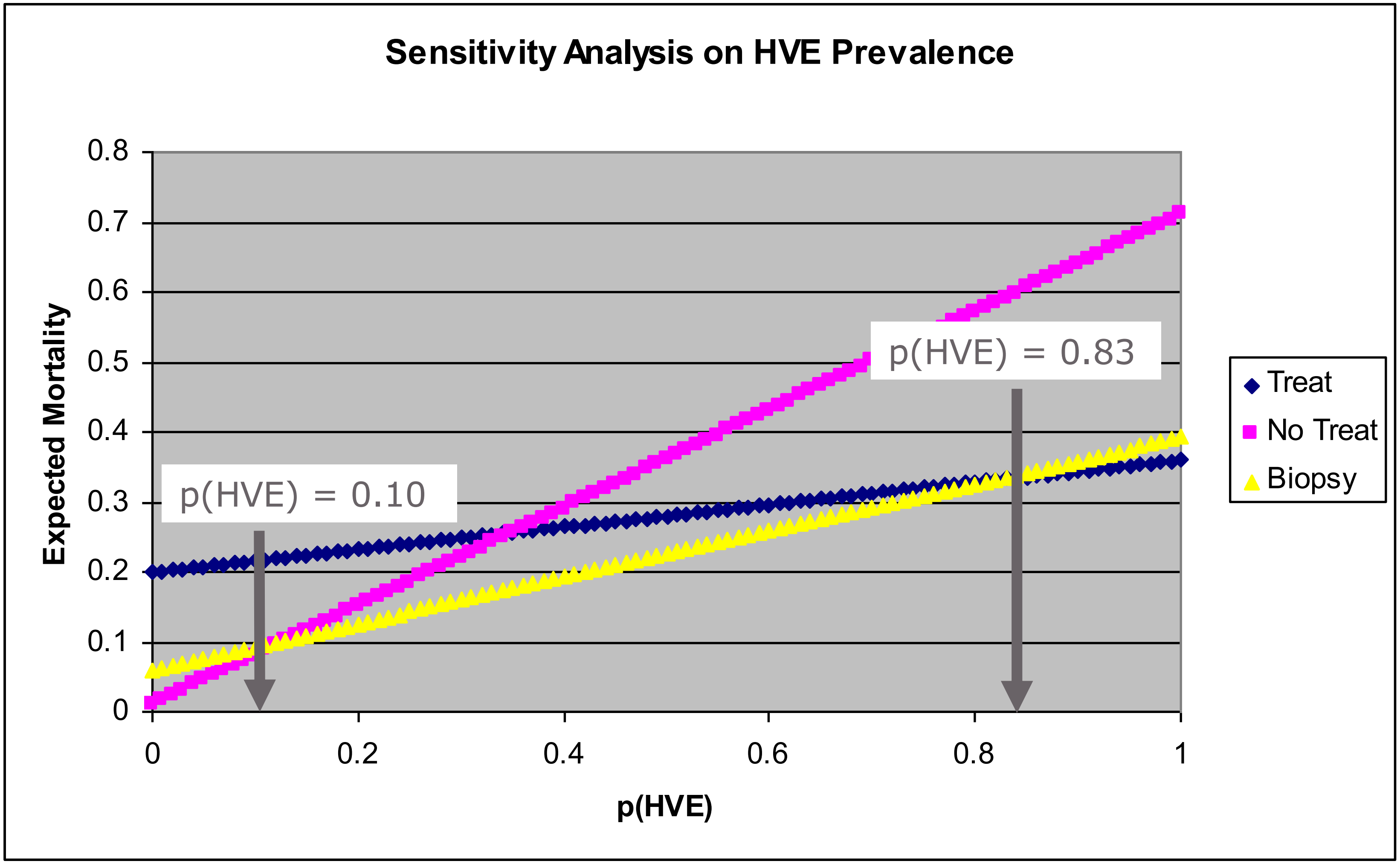
OVE
0.48
0.01
Biopsy event
0.05
1.00


Pr(mortality or severe sequelae)
0.2832
HVE
0.36
0.52
Treat
0.48
OVE
0.20
0.3740
HVE
0.71
0.52
Do not treat
0.48
OVE
0.01
0.1920
0.2324
HVE
0.36
0.52
No event
Biopsy
0.95
0.48
OVE
0.01
0.05
Biopsy event
1.00

One-Way Sensitivity Analysis



Decision Tree CEA:

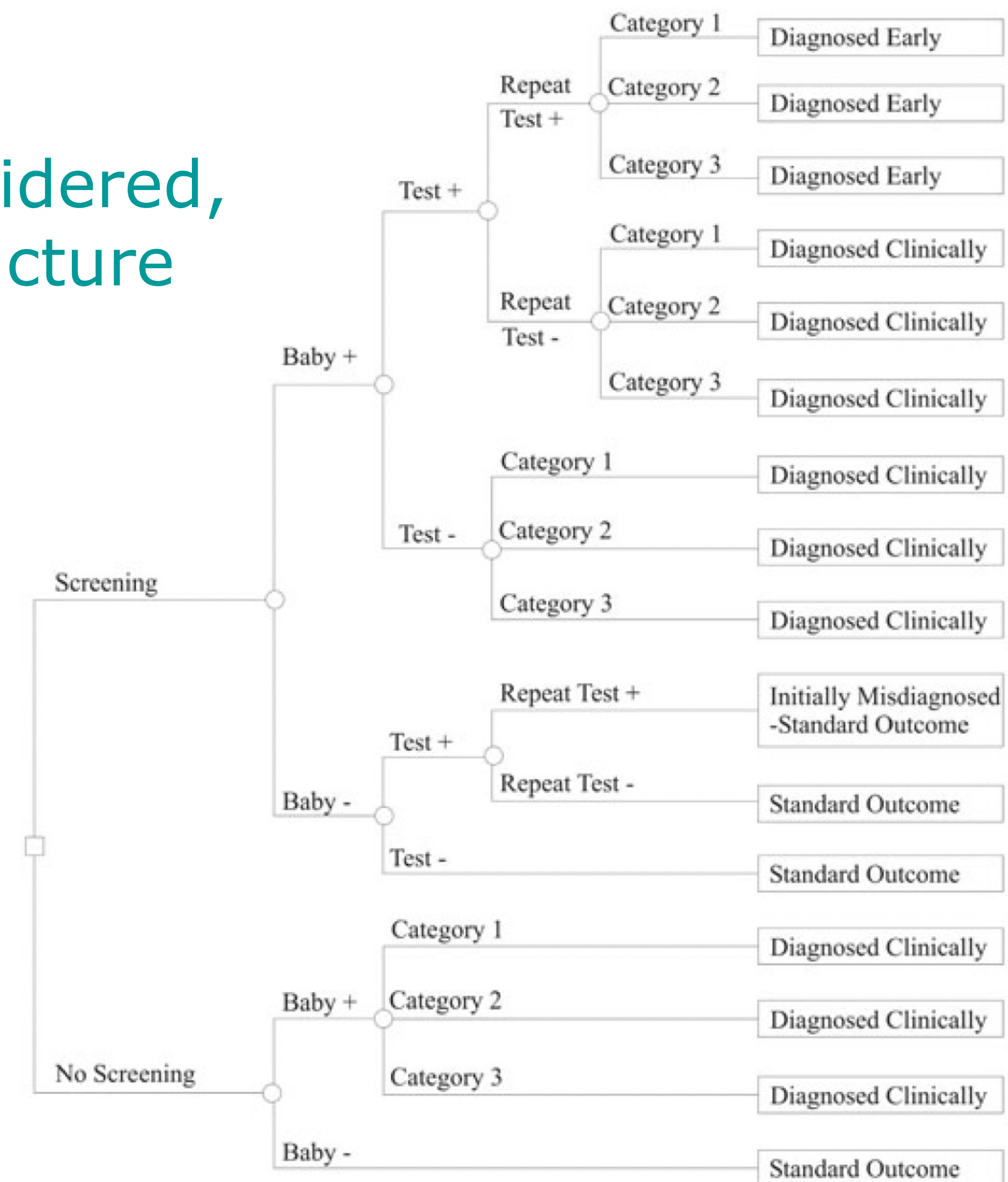
Newborn screening for metabolic disorders

Study Context and Design

- Cost-effectiveness analysis of new technology for newborns metabolic disorders screening
  - Screening identifies newborns before clinical symptoms manifest
  - Some screening already in place, but new technology ("tandem mass spectrometry") allows multiple disorders to be screened for at same time
- Population: Newborns born in Ontario, Canada
- Health outcomes: Life-years (LY)
- Costs: screening, diagnosis, treatment, education and social services
- Time horizon: Lifetime

Decision Tree Structure

Many different disorders considered, same tree structure



Results

- Evaluated CEA of switching from existing to new screening technology for PKU only
  - High cost per LY gained!
- Evaluated CEA of using new screening technology for other metabolic disorders (individually)
  - Also high cost per LY gained!
- Evaluated CEA of ”bundled” screenings
  - Leverages the advantage of the new screening technology
  - ICER as low as ~$65,000 (CAD) per LY
  - Fixed costs are spread across multiple disorders, making bundled ICERs more favorable

DARTH Workgroup

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4 Harvard T.H. Chan School of Public Health, Boston, USA
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6 The Hospital for Sick Children, Toronto and University of Toronto, Toronto ON, Canada

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