

Decision Analytic Evaluation of Newborn Screening Tests

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Variables to Consider (Besides Quality of Evidence)

- Prevalence of the condition
- Severity of the condition
- Quality of the test
 - Sensitivity & specificity
- Benefits of treatment
 - Decreased mortality & morbidity
- Harms of screening
 - False positives

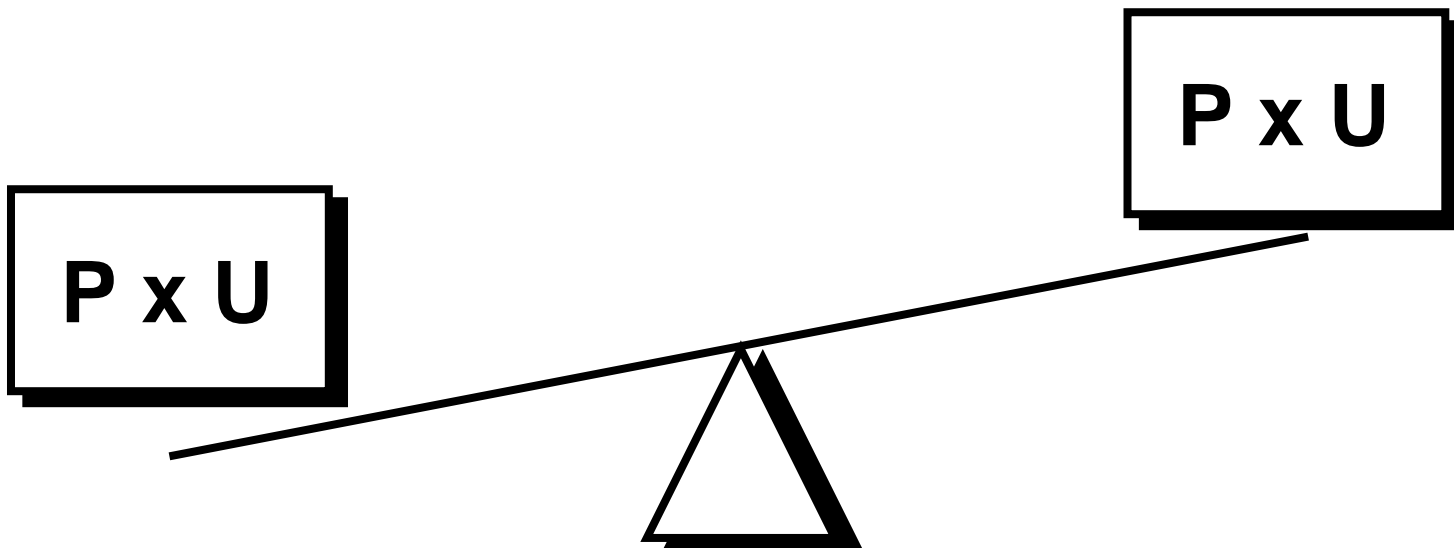
How to combine them all???

What is Decision Analysis?

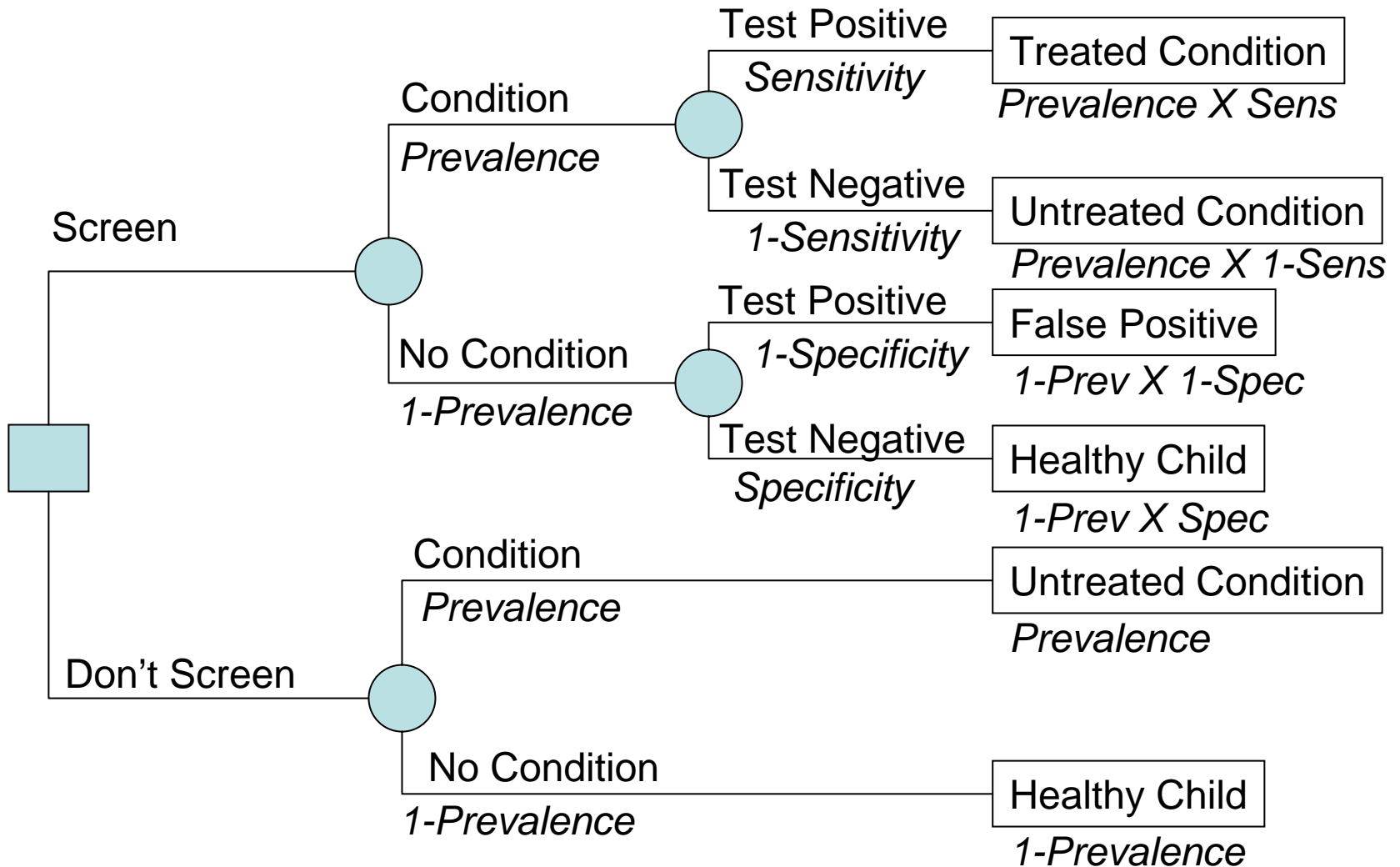
The product of probability x utility

Probability is the likelihood of something happening

Utility is how you feel about something happening



Decision Trees



- Prevalence = 1 in 10,000 = 0.001
- Sensitivity = 95% = 0.95
- Specificity = 99% = 0.99

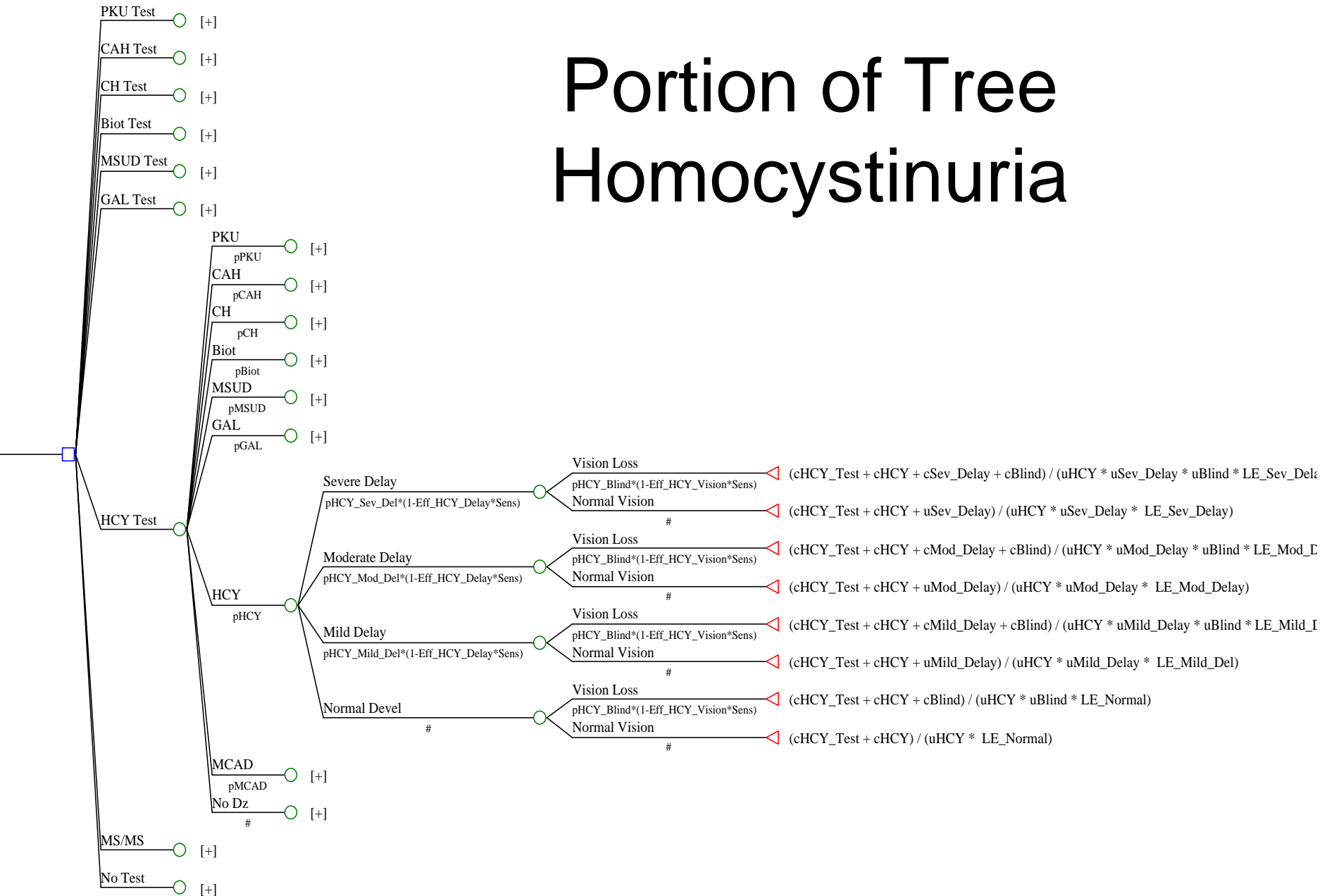
- Screening:
 - $0.0001 \times 0.95 = 0.000095$ treated cases
 - $0.0001 \times 0.05 = 0.000005$ untreated cases
 - $0.9999 \times 0.01 = 0.01$ false positives
 - $0.9999 \times 0.99 = 0.99$ healthy children

- No Screening
 - 0.0001 untreated cases
 - 0.9999 healthy children

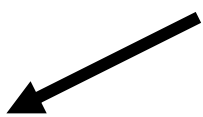
Results in 100,000 Babies

Outcome	Screen	No Screen
Treated Cases	10	0
Untreated Cases	<1	10
False Positives	1000	0
Healthy Children	98,990	99,990

Portion of Tree Homocystinuria

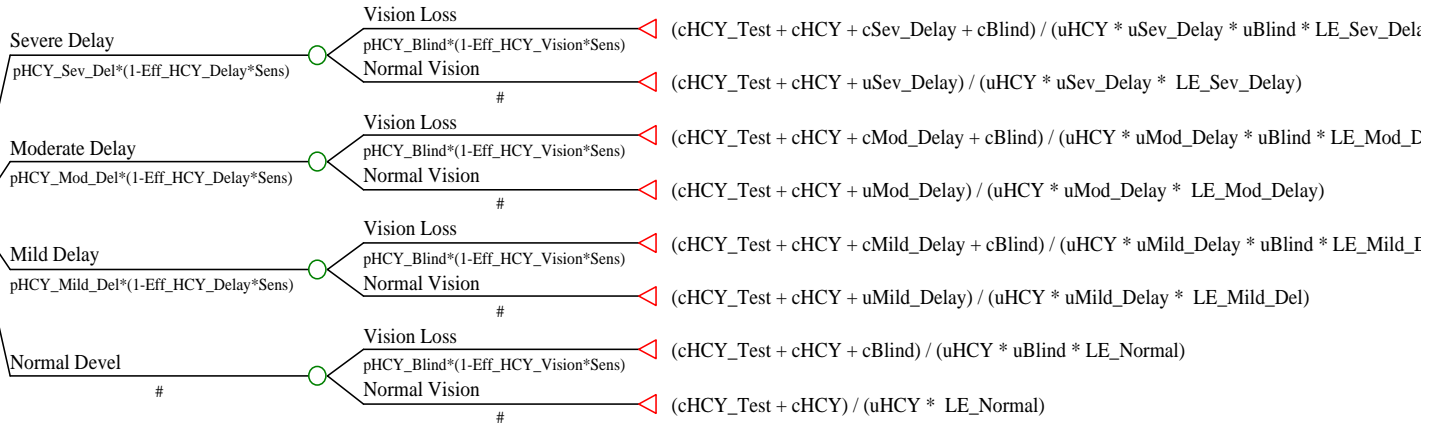


Tests



- PKU Test [○] [+]
- CAH Test [○] [+]
- CH Test [○] [+]
- Biot Test [○] [+]
- MSUD Test [○] [+]
- GAL Test [○] [+]
- HCY Test [○] #
- MCAD [○] #
- pMCAD [○] #
- No Dz [○] #
- MS/MS [○] [+]
- No Test [○] #

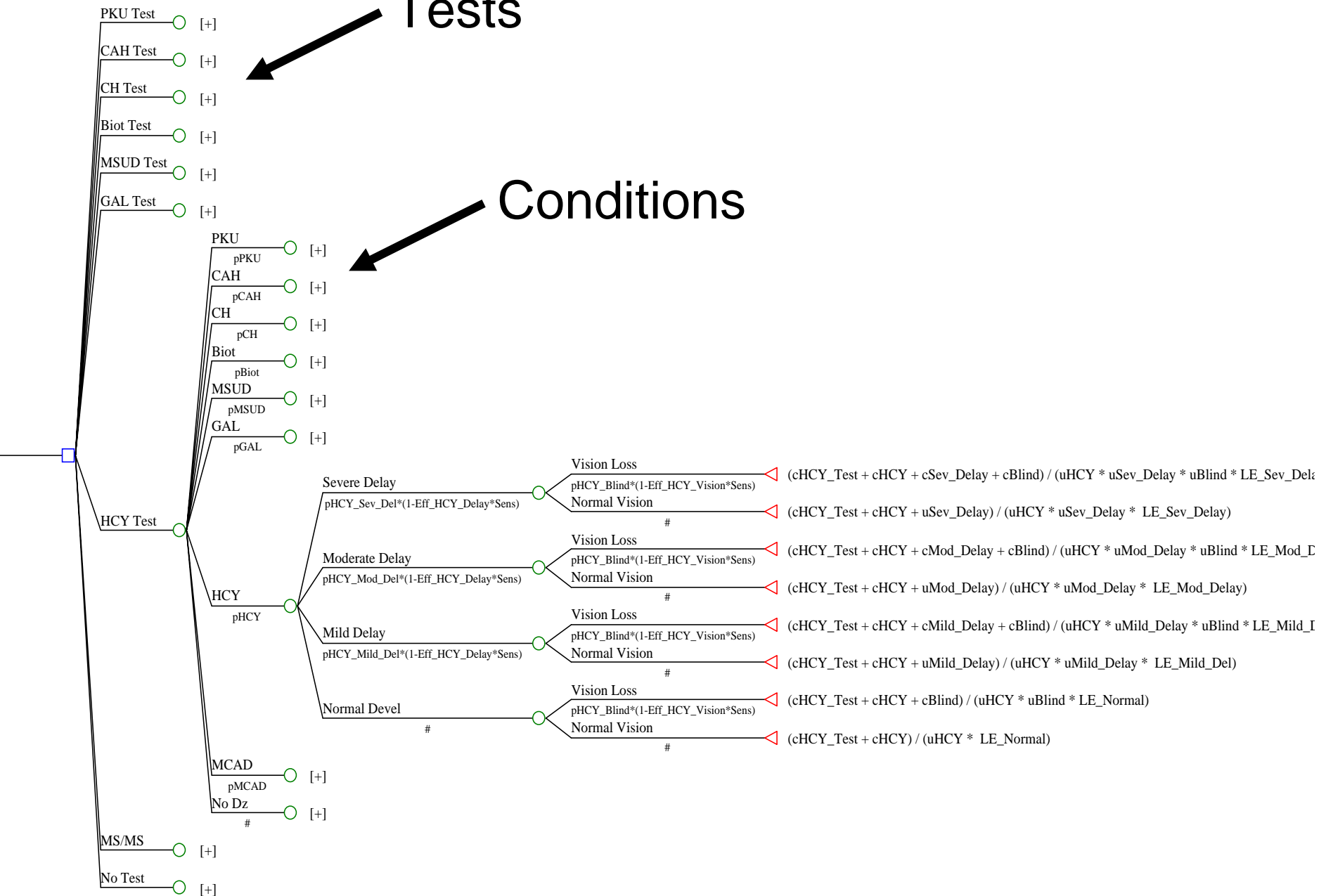
- PKU [○] [+]
- pPKU [○] [+]
- CAH [○] [+]
- pCAH [○] [+]
- CH [○] [+]
- pCH [○] [+]
- Biot [○] [+]
- pBiot [○] [+]
- MSUD [○] [+]
- pMSUD [○] [+]
- GAL [○] [+]
- pGAL [○] [+]



- MCAD [○] #
- pMCAD [○] #
- No Dz [○] #

Tests

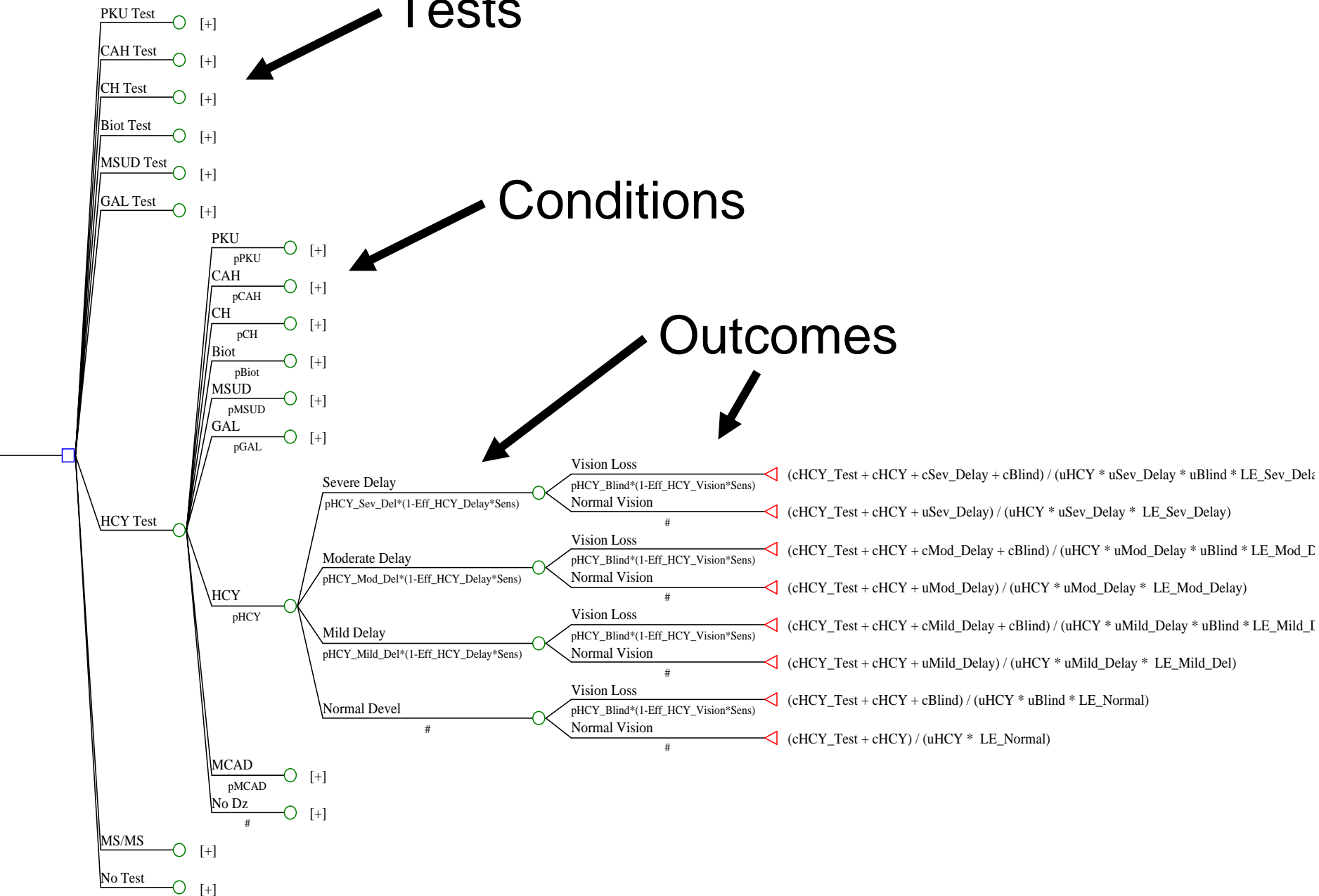
Conditions



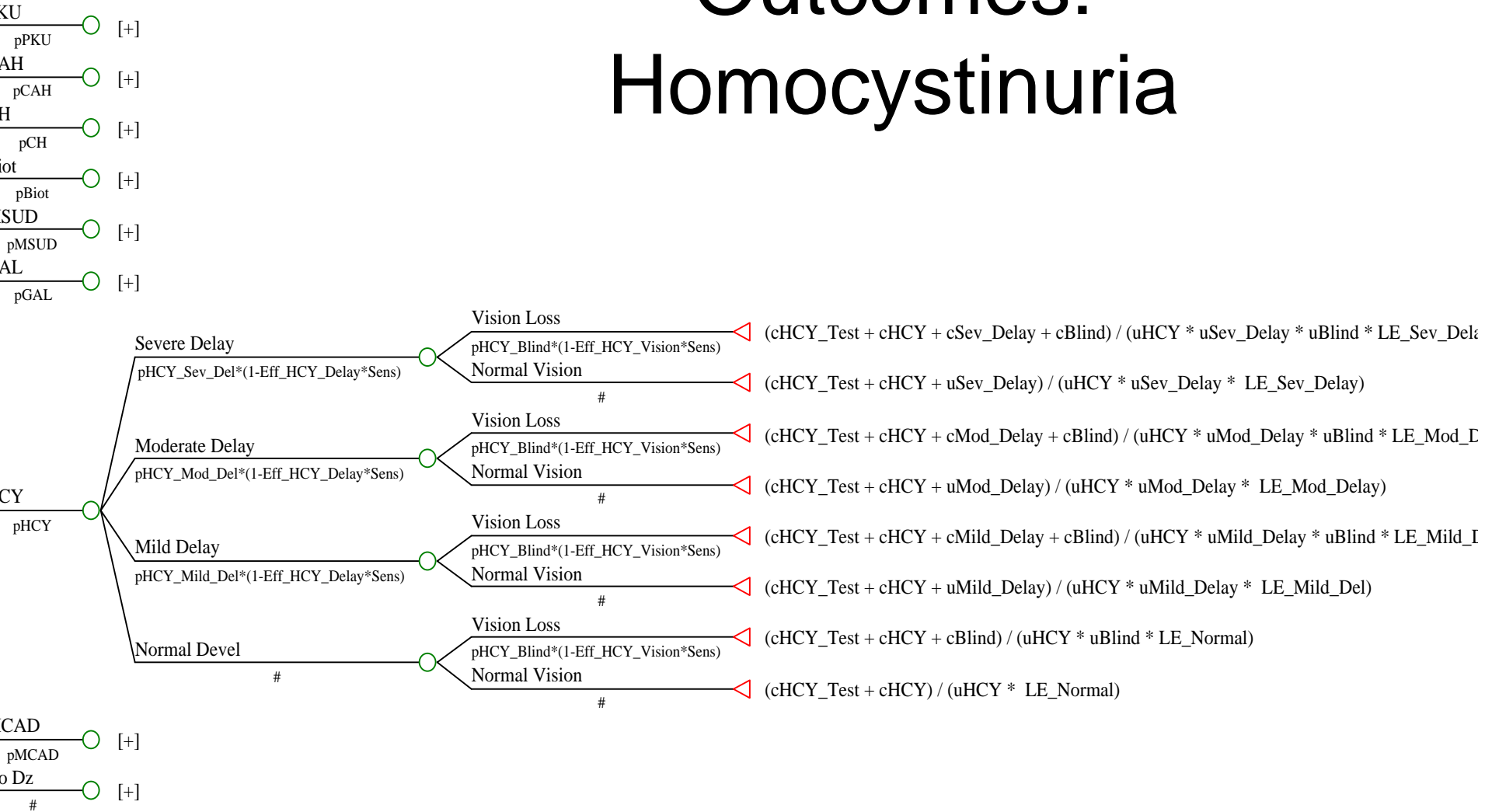
Tests

Conditions

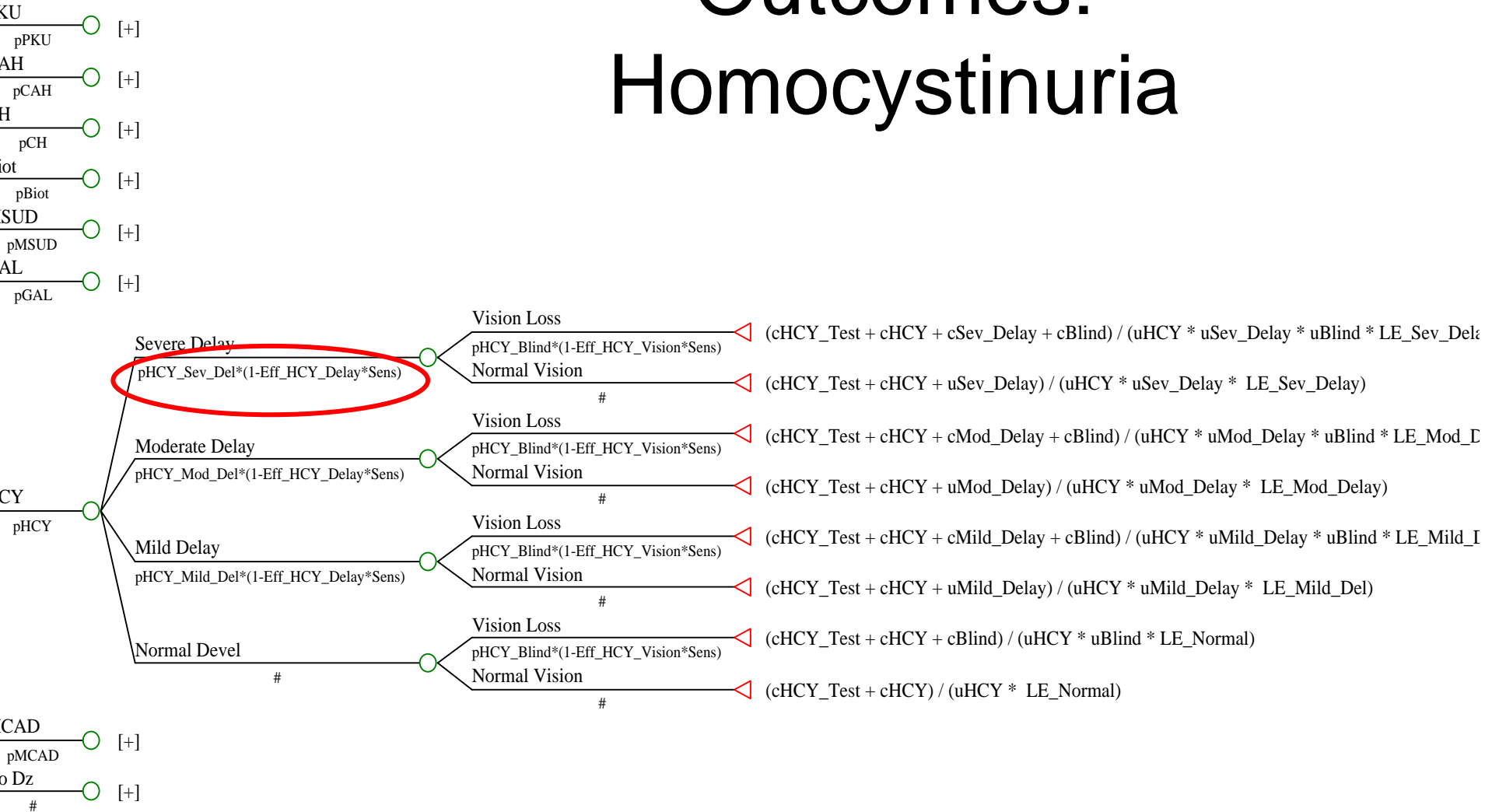
Outcomes



Outcomes: Homocystinuria

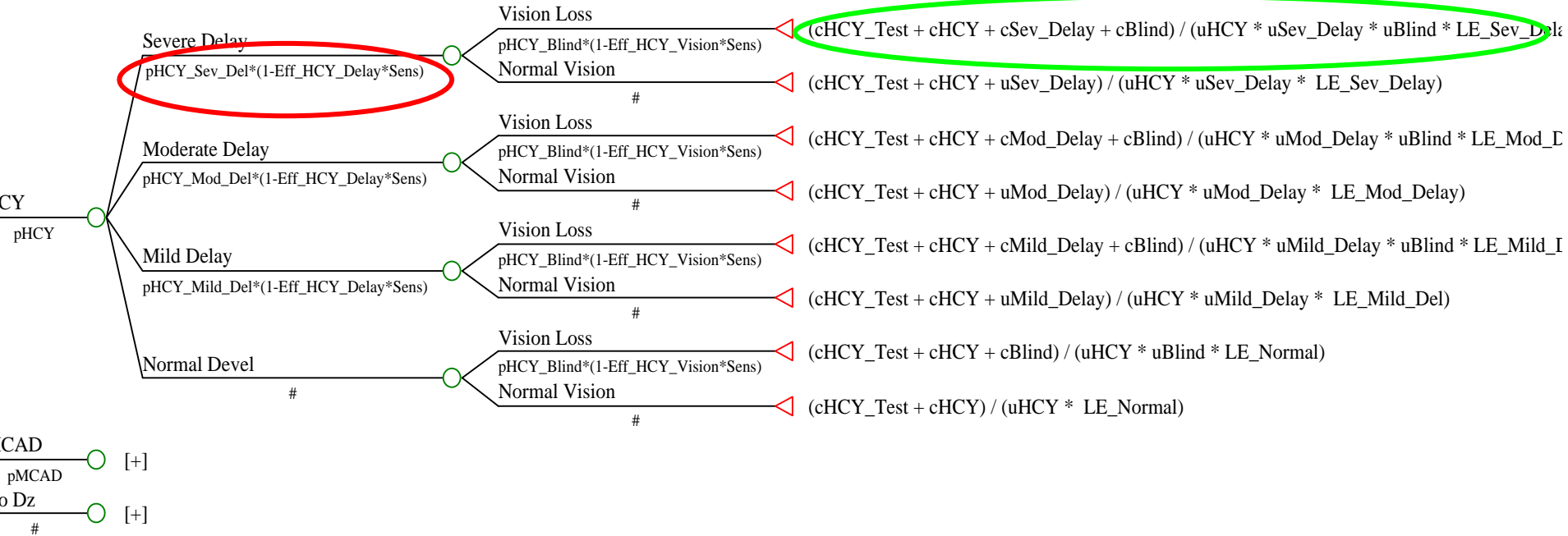


Outcomes: Homocystinuria



Outcomes: Homocystinuria

- KU [○] [+]
- pPKU [○] [+]
- AH [○] [+]
- pCAH [○] [+]
- H [○] [+]
- pCH [○] [+]
- iot [○] [+]
- pBiot [○] [+]
- SUD [○] [+]
- pMSUD [○] [+]
- AL [○] [+]
- pGAL [○] [+]

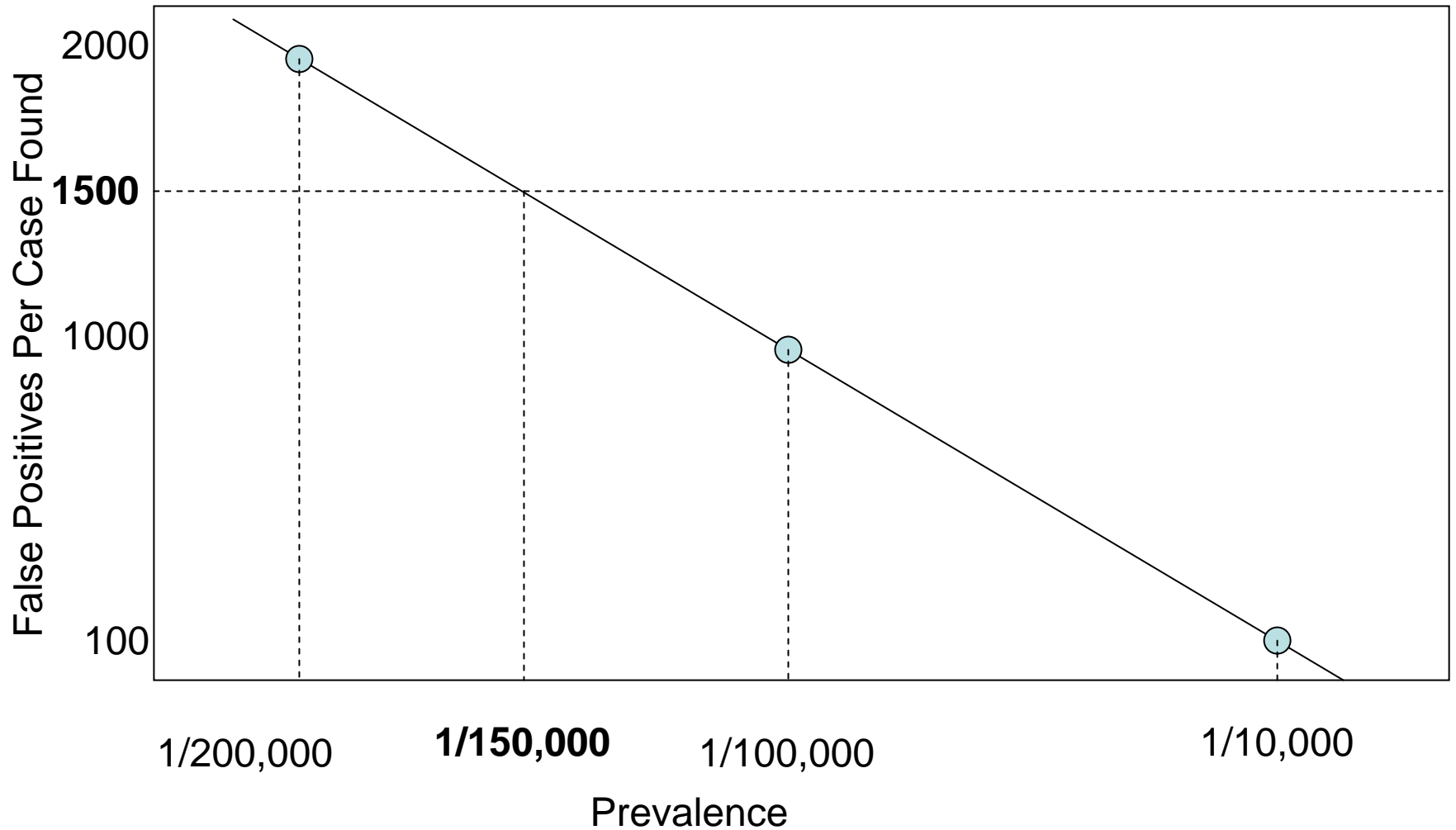


Value of an Outcome

$LE(\textit{Outcome}) \times U(\textit{Outcome})$

Cost

Sensitivity Analysis



Conclusion

- Decision analytic framework
 - Compares benefits and harms
 - Makes trade-offs explicit
- Mechanism for exploring potential value in an environment with scant evidence