Cost-Effectiveness of Alternative Therapies for Type 1 Diabetes Mellitus

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Background

- Diabetes Mellitus is a chronic disease characterized by the body's inability to produce or respond properly to insulin, a hormone used to convert blood glucose into energy
- Cause is a mystery, although both genetics and environmental factors (obesity, lack of exercise) appear to play a role

Two Main Types of Diabetes

- Type 1
  - auto-immune disease
  - body does not produce any insulin
  - most often occurring in children and young adults
  - must take daily insulin injections to stay alive
  - accounts for 5-10% of diabetes
- Type 2
  - metabolic disorder
  - body unable to make enough, or properly use, insulin
  - most common form of diabetes
  - accounts for 90-95% of diabetes

Health Complications

- Microvascular
  - Eye (Retinopathy) ➡ Blindness
  - Nerve (Neuropathy) ➡ Nerve Disease, Amputations, Impotence
  - Kidney (Nephropathy) ➡ Kidney Disease, End Stage Renal Disease
- Macrovascular ➡ Heart Disease
  ➡ Stroke

Burden of Diabetes

- 16 million people have diabetes in the U.S.
- 6th leading cause of death among diseases
- Accounts for 1 out of 7 health care dollars.

- Type 1 diabetes affects only 5-10% of the 16 million people with diabetes,

  but…

- Accounts for 30% of the total costs...

Treatment of Type 1 Diabetes

Conventional Therapy	Intensive Therapy
one or two daily injections of insulin	three or more times daily injections of insulin or external pump
daily self-monitoring of urine or blood glucose	self-monitoring of glucose at least four times per day
education about diet and exercise	a diet and exercise plan
usually not include daily adjustments in the insulin dosage	the insulin dosage is adjusted according to the results of blood glucose (food intake and exercise)

Diabetes Control & Complications Trial

- 10-year multi-center, randomized clinical trial
- Intensive therapy reduces risk for developing:
  - Retinopathy by 76%
  - Nephropathy by 50%
  - Neuropathy by 60%
  - Cardiovascular disease by 35%
- Intensive therapy slows the progression of retinopathy by 54%

Purpose of Study

- Evaluate the cost-effectiveness of alternative treatment strategies for type 1 diabetes

How?

- Model progression of type 1 diabetes
- Model costs associated with health states
- Model impact on quality of life of individuals

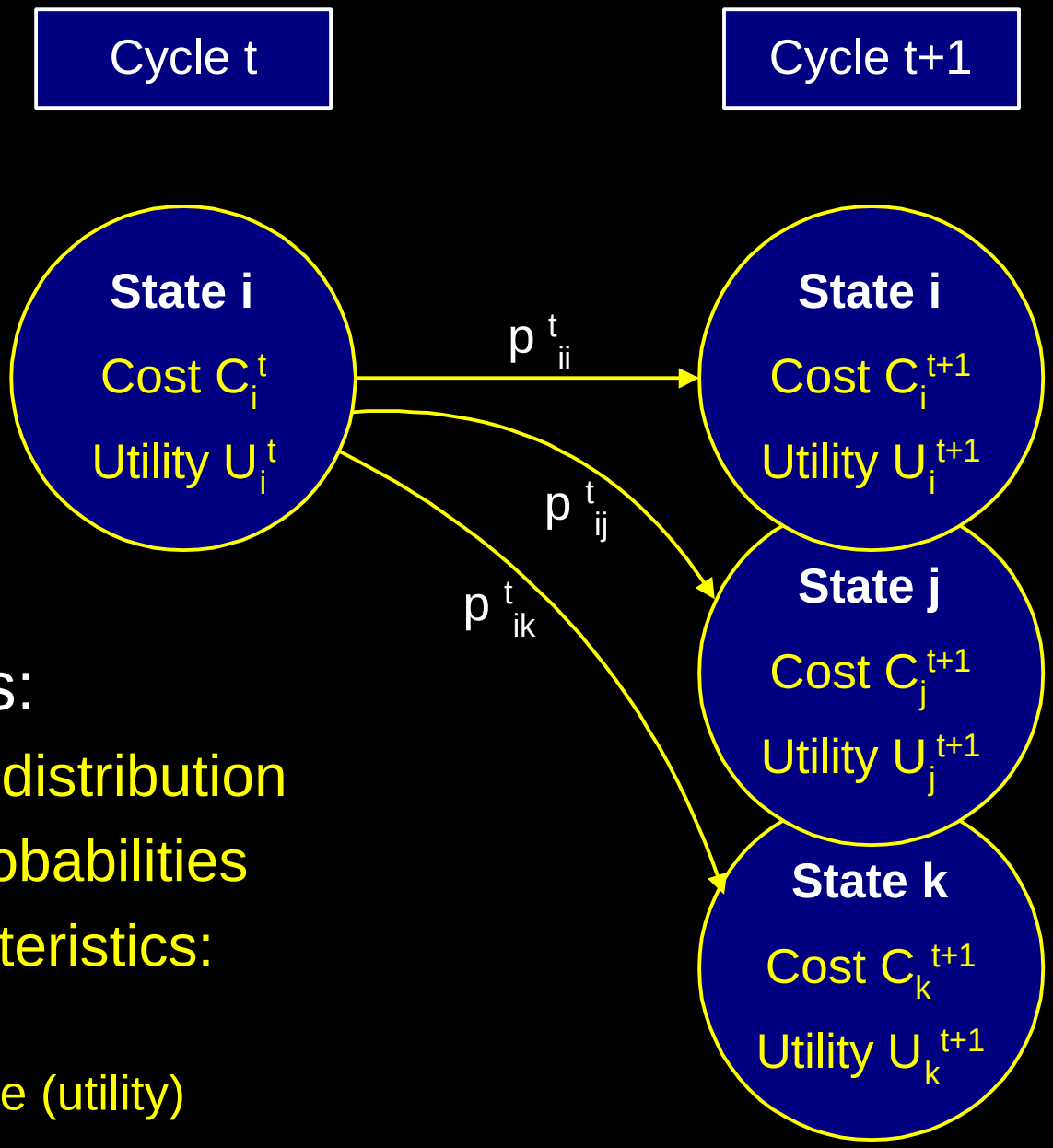
- Put pieces together and apply to:
  - a population of interest (Wisconsin)
  - for a certain period of time

Observations

- Type 1 DM is a chronic disease
  - no cure, “strict” progression from state to state
- Death is a terminal (absorbing) state

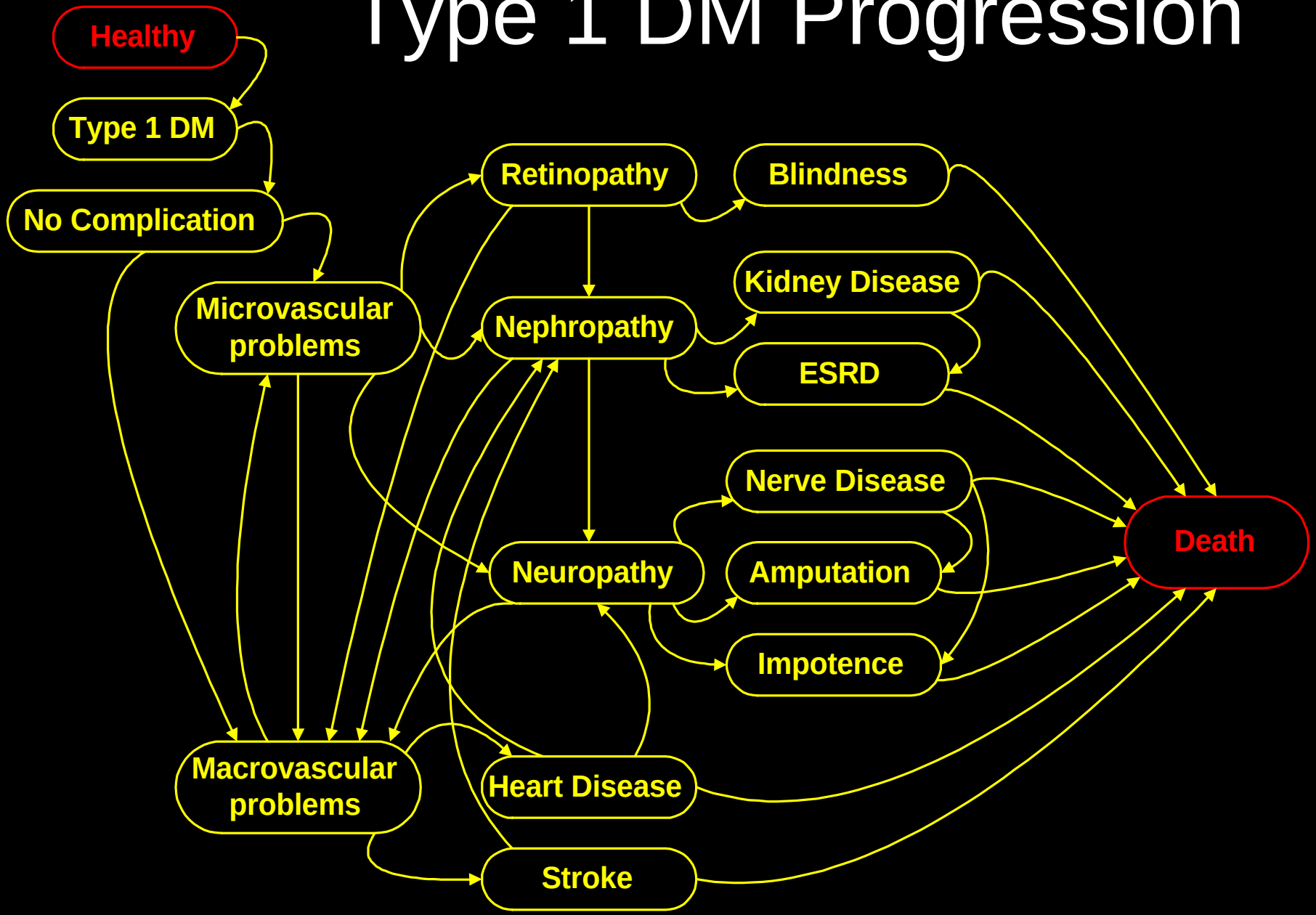
- Could be modeled as a **Markov** process

Markov Model



- Key elements:
  - initial states distribution
  - transition probabilities
  - state characteristics:
    - cost
    - quality of life (utility)

Type 1 DM Progression



Reduction of Health States

Healthy	➡	Healthy
No complication	➡	Type 1 DM without complication
Retinopathy	➡	Type 1 DM with retinopathy alone
Neuropathy +	➡	Type 1 DM with neuropathy alone or neuropathy and retinopathy
Nephropathy +	➡	Type 1 DM with nephropathy alone or with any other complications
Dead	➡	Dead

Data Needs

(Cycle Time = 1 year)

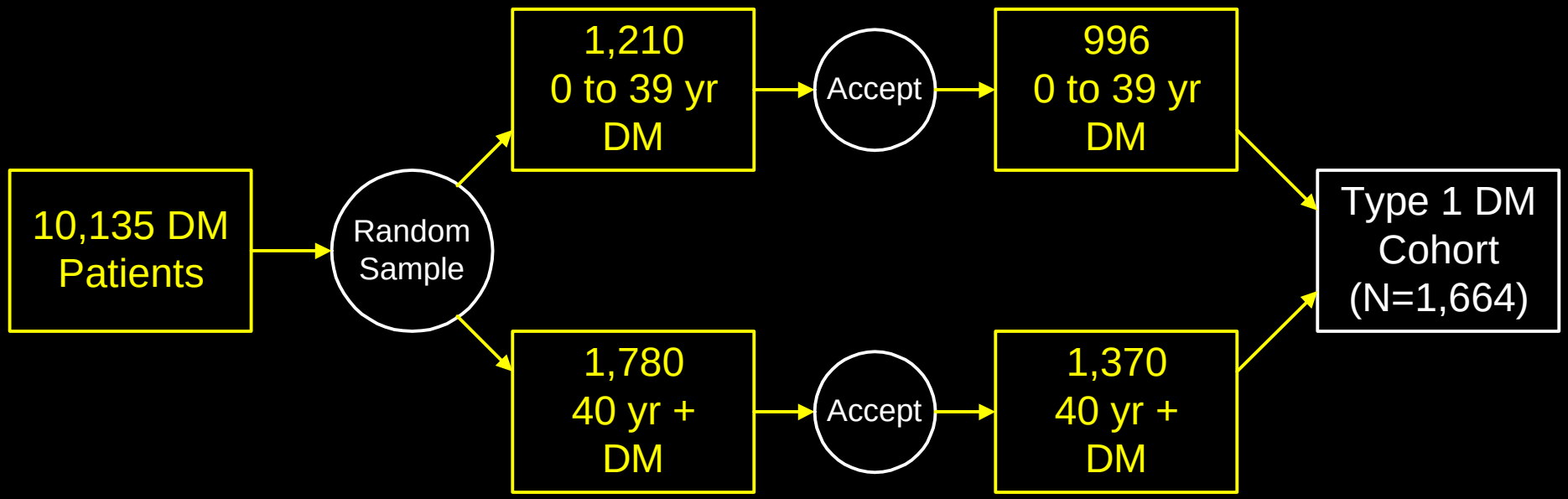
- Initial states distribution
  - prevalence of Type 1 DM
- Transition probabilities
  - incidence of Type 1 DM
  - incidence of complications
  - mortality
- Costs
  - direct costs
    - medical
    - etc.
  - indirect costs
- Utilities
  - quality of life

Primary Sources of Data

prevalence	➡	Diabetes Care (1995)
incidence	➡	Am. J. Epidemiology (1993) WESDR (1978, 1982, 1988, 1993) DCCT (1997, 1998)
mortality	➡	CDC Vital Statistics (1991-1994) WESDR (1978, 1982, 1988, 1993)
medical costs	➡	Local HMO (1994-1995)
utilities	➡	SF-36 Assessment (1997)

WESDR

- Wisconsin Epidemiologic Study of Diabetic Retinopathy
- Prospective cohort followed over time since 1978



Utility Data

- Measure of Health Related Quality of Life (HRQOL):
- Quality Adjusted Life Years (QALY)
  - 1 year in perfect health produces greater “utility” than 1 year in a diseased state,

    hence:

  - Assign a factor λ_S between 0 (death) and 1 (perfect health) to each diseased state ***S*** so that the utility of ***X*** years in diseased state ***S*** is equal to $\lambda_S \times X$

Quality Adjusted Life Years

- One commonly used scale for estimating λ_s :
  - *Quality of Well-Being scale* (QWB)
- However:
  - no QWB scores available for Type 1 DM-specific health states
  - primary data collection for QWB is resource- and time-intensive

Predict QWB scores from SF-36

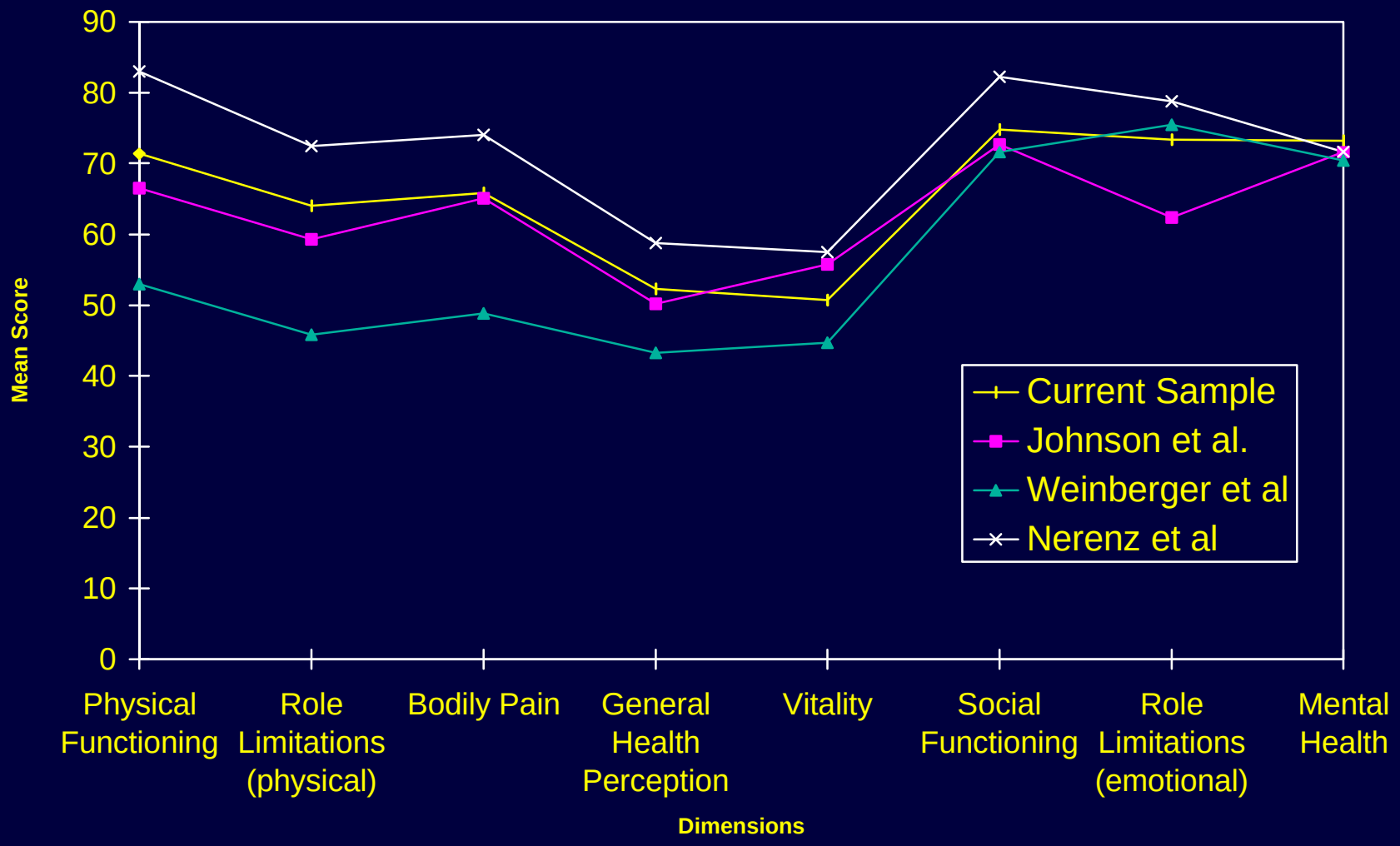
- Administer SF-36 health survey to Type 1 DM patients:
  - self-administered
  - relatively inexpensive
  - produces 8 scales profiling HRQOL of an individual
- Link SF-36 scores to QWB:
  - Use empirical regression equation (Fryback et al., 1997)

(Predicted) QWB Score = *f* (SF-36 scores)

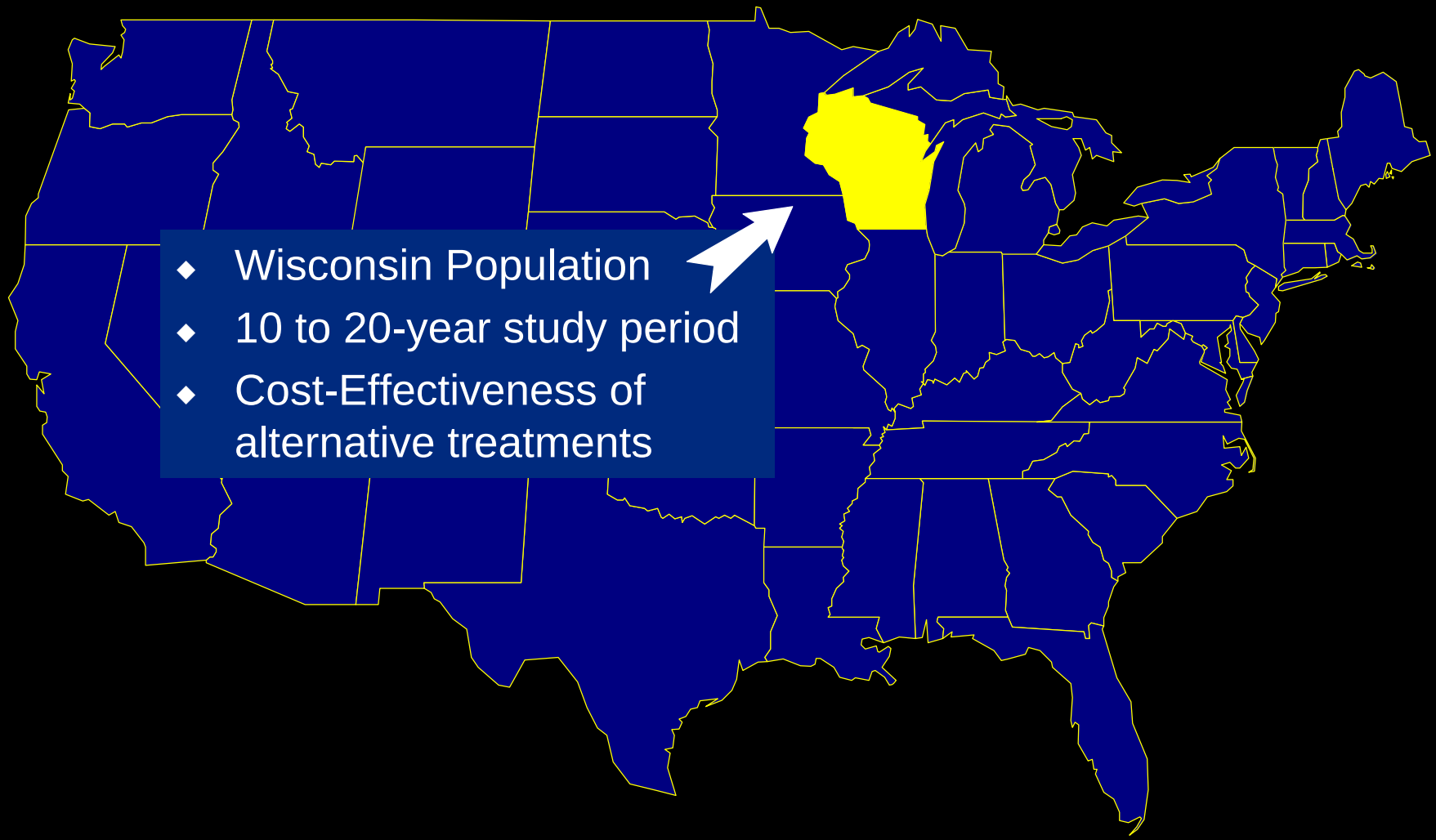
SF-36 Scales

PF	➡	physical functioning
RP	➡	role function as limited by physical problems
BP	➡	bodily pain
GH	➡	general health perceptions
VT	➡	vitality (energy/fatigue)
SF	➡	social function
RE	➡	role function as limited by emotional problems
MH	➡	mental health

SF-36 Health Profile of Diabetic Patients



Application of Model



- Wisconsin Population
- 10 to 20-year study period
- Cost-Effectiveness of alternative treatments

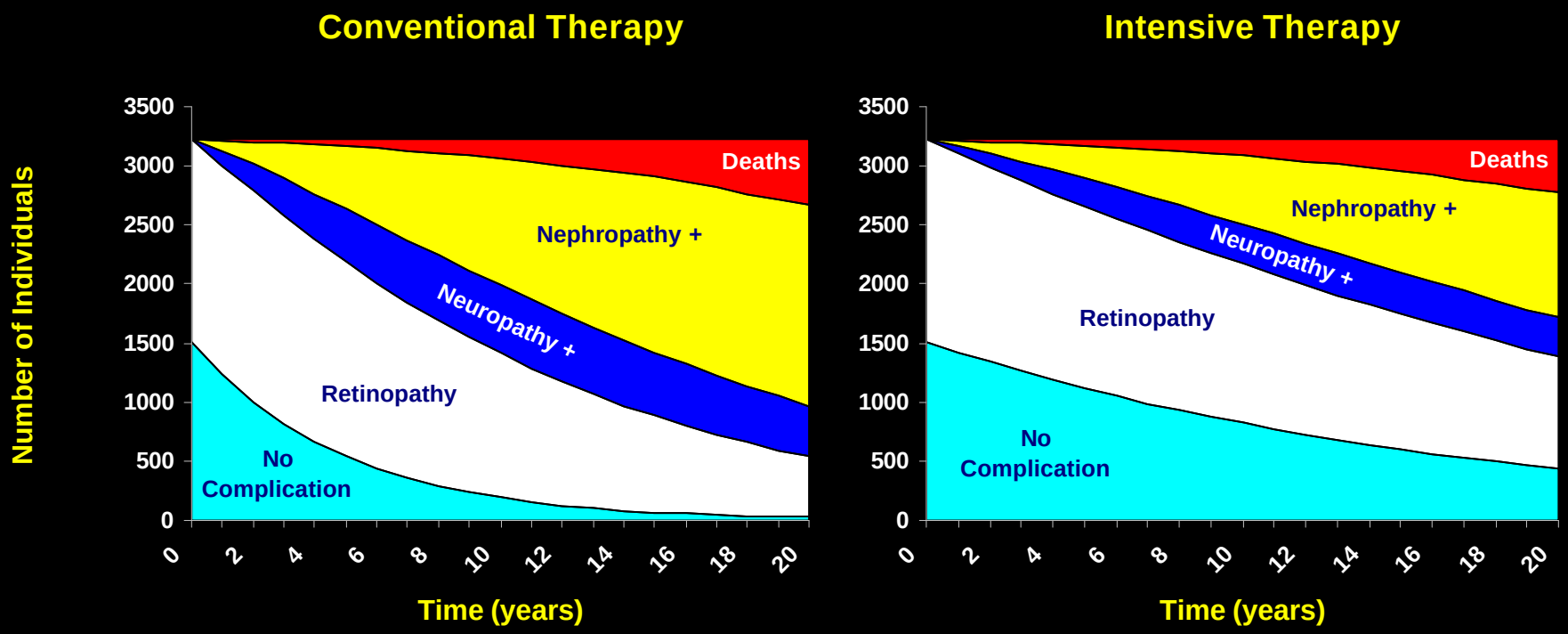
Conventional vs. Intensive Treatments

- Impact on disease progression
- Impact on costs
- Impact on quality of life

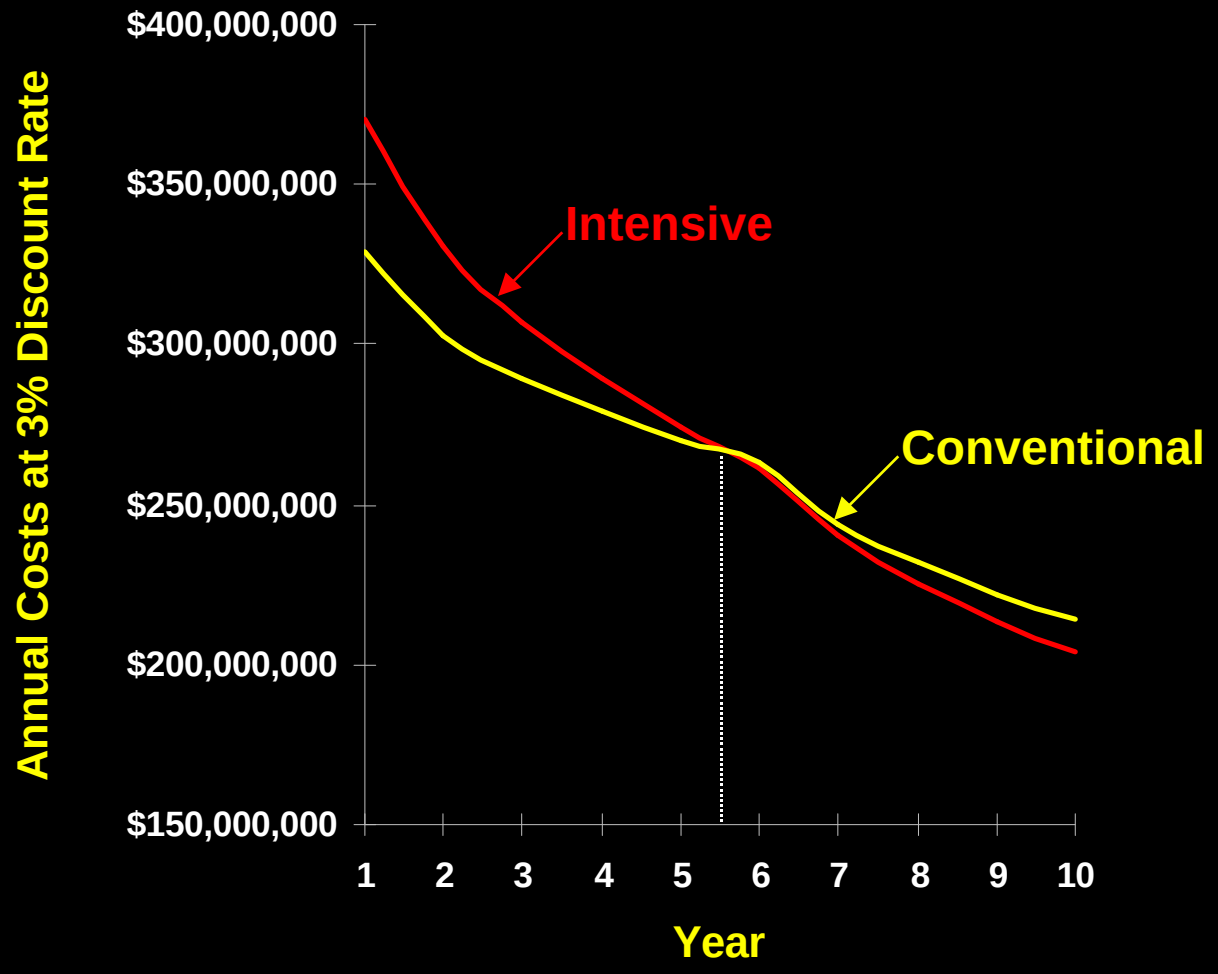
- Cost-Effectiveness

Disease Progression Comparison

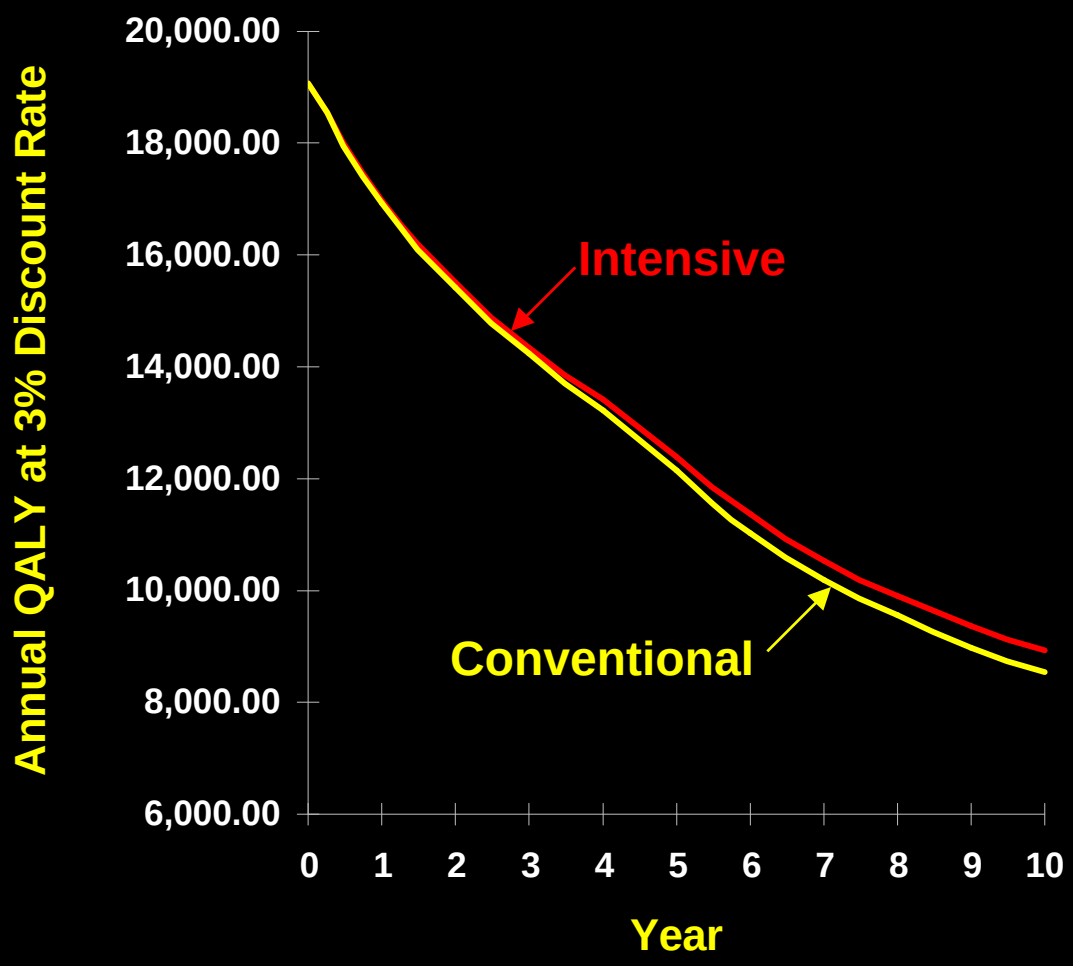
Type 1 DM patients age 10-39 starting at year 0
without complication or with retinopathy alone



Annual Cost Comparison



QALY Comparison



Incremental Cost-Effectiveness Table

10-39 year-old followed for 20 years

Therapy	*Cost Differential Assumption*	*Incremental Costs (millions)*	*Incremental QALYs*	*Cost-Effectiveness Ratio*
Intensive (50% effective)	$3,000	$111.9	328	$341,189 / QALY
	$1,500	$6.4		$19,512 / QALY
	$1,000	***-$28.8***		savings
Intensive (100% effective)	$3,000	$0.3	581	$534 / QALY
	$1,500	***-$105.6***		savings
	$1,000	***-$140.8***		savings

Conclusions

- According to this model, an intensive therapy program with
  - extra costs of $1500 or less per patient-yr
  - effectiveness of 50% or more
- will...
  - improve health states
  - save lives, and
  - save $ on direct costs

Limitations

- Modeling assumptions
  - Simplified health states
  - Discrete timing
  - Strict progression
  - Memoryless assumption
- Add/improve data
  - Macrovascular complications
  - Better and more cost data (e.g., indirect costs)
  - Better QALY data
- Applicability to other populations