

Good Practices for High Quality Decision Analytic Models

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Workshop Session Plan

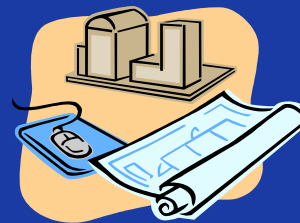
- ▲ What is decision-analytic modeling?
- ▲ When it is appropriate to use modeling techniques?
- _____
- ▲ What modeling standards/guidelines exist?
- ▲ How do you translate information into decisions?
- _____
- ▲ Summary of key points / findings

Role of Decision-Analytic Modeling in Healthcare Decision-Making

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What is a Decision Analytic Model?

- ▲ A decision analytic model:
 - ◆ Set of calculations laid out in a logical sequence
 - ◆ Informs a decision process – it is not purely to arrive 'perfect' scientific answers
 - ◆ Modeling should be used as a decision aid
 - ◆ Its goal should be to provide the decision-maker with information that can allow them to judge



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What is a Decision Analysis Model?

- ▲ Models are used when exact information/data is not available
- ▲ Decision modeling ...
 - ◆ Synthesizes information from multiple sources
 - **Sources:** RCTs, observational studies, claims data, expert opinion, preference studies...
 - ◆ Estimates clinical and economic consequences
 - **Costs:** drug, treatment, adverse events
 - **Outcomes:** life years saved, avoided secondary events
 - ◆ Acts as a conceptual framework to bring the data together

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When to Use Decision-Analytic Modeling?

- ▲ Examples of when a model is appropriate:
 - ◆ **Treatment selection** – To examine a large number of potential treatment / management options which may vary by setting
 - ◆ **Patient selection** – To extrapolate to a broader patient population than used in available studies
 - ◆ **Time periods** – To extrapolate to a longer-term treatment time horizon
 - ◆ **Uncertain evidence base** – To consider variation in effect size, inadequate power, confounding variables, or data sources
 - ◆ **Flexibility** - To develop analyses to cover alternative healthcare settings / country-specific analysis
 - ◆ **Timing and cost** – Decision modeling can be performed at a relatively low cost and results can be obtained quicker than primary data-collection studies

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Challenges of Decision-Analytic Modeling

- ▲ Economic evaluation is a standard requirement for many reimbursement / review systems
 - ◆ National Institute of Clinical Excellence (NICE) – United Kingdom
 - ◆ Canadian Coordinating Office for Health Technology Assessment (CCOHTA)
 - ◆ Pharmaceutical Benefits Advisory Committee (PBAC) - Australia
 - ◆ Academy of Managed Care Pharmacy (AMCP) – United States
- ▲ Result: Modeling is accepted as a valid analytical approach to performing economic evaluations
- ▲ *"It may be necessary to carry out an appraisal before the best quality outcome data are available. In these circumstances modeling is appropriate to adapt the best available data to the problem being addressed...." (NICE 2001)*

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Advantages and Challenges of Modeling

Advantages

- ▲ Represent a complex reality
- ▲ Estimate performance under projected conditions
- ▲ Minimize data collection

Challenges

- ▲ Lack of standardization/quality control in the development and reporting of models
- ▲ Potential for 'hidden' assumptions
- ▲ Variation in quality of data
- ▲ Lack of transparency makes it difficult to check
- ▲ Difficulty in communicating results in an effective and balanced way to a non-modeling audience

In general these challenges are applicable for other types of analyses (i.e., prospective studies, RCTs, etc)

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Guidelines

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Standards/Guidelines on Healthcare Modeling

Governmental (Reimbursement)

- NICE (Submission)
- AMCP (Format v2)
- Canada
- Australia
- Netherlands

Evaluation Checklists (Peer)

- BMJ (Drummond)
- NEJM
- Gold papers

Journal/Academic

- Sonnenberg et al, 1994
- Sheffield Workshop
- Sculpher et al, 2000
- McCabe et al, 2000

ISPOR Task Force -
Good Research Practices
in Modeling Studies

Milton Weinstien. *Value in Health* 2003 (6)1 9-17

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Guidelines: Structural Form

- ▲ Relevance to decision-maker
 - Perspective: Who is the decision-maker?
- ▲ Relevance to decision
 - Complexity and scope should have clear rationale and be relevant to the decision making perspective
- ▲ Relevance to disease
 - Health state definitions and linkages hold clinical validity in the face of current understanding
 - Solid grounding in disease theory through clinical concurrence



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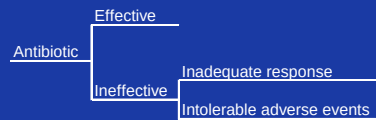
Guidelines: Structural Form

- ▲ Full acknowledgement of assumptions
- ▲ Time horizon reflects and captures major clinical events and costs related to the disease or treatment
- ▲ Appropriate treatment comparators
 - Include treatment options of immediate interest
 - Consider treatment options reflecting novel treatments and extremes
- ▲ Appropriate level of model memory
 - Variations in patient history, sub-groups, and attributes must be included if they have a logical and expected impact on event rates and resource use
 - Age-adjusted mortality
 - Increased risk of CHD as patient ages

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Guidelines: Structural Form

- ▲ Appropriate methodology
 - Clear justification for model methodology and health states
 - Decision tree
 - Markov
 - Simulation



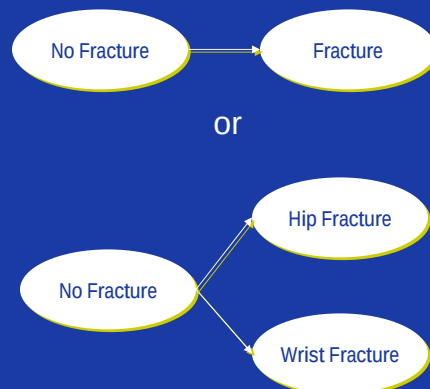
- ▲ Transparency: calculations are clearly presented
- ▲ Strike a balance between model structure and data availability



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Guidelines: Data Selection

- ▲ Model should include clinical, economic, and outcomes data that have relevance to the decision at hand



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Guidelines: Data Selection

- ▲ Evidence must show that consideration of all data and their values have been made



- Full details on the importance of a parameter and its value
- Appropriate ranges and distributions representing data uncertainty
- Clear acknowledgement where expert opinion is used

Nuijten MJ. The selection of data sources for use in modeling studies. *Pharmacoeconomics*. 1998 Mar;13(3):305-16.

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Guidelines: Data Preparation

- ▲ Data is seldom available 'off the shelf' and 'ready to go'
- ▲ Data often requires adaptation, translation, or mapping to other value scales
- ▲ Transparent presentation on data preparation methods



- Calculation of baseline risks
- Calculation of relative risks
- Calculation of transition probabilities
- Survival statistical modeling (weibull, exponential etc)
- Costs
- Cost inflation
- Cost and outcome discounting

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Guidelines: Validation

We can not formally 'test' the quality and validity of a model in its true sense.

Validation approaches

- ◆ Quality assurance 'debugging' plan
- ◆ Run head-to-head comparisons of structures, inputs, and outputs with other models in same area
- ◆ Direct comparison of model predictions to a known set of independent outcome or resource data
- ◆ Examination of uncertainty in model inputs and structure for sensitivity analyses



*'Putting the model
under the microscope'*

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Documentation

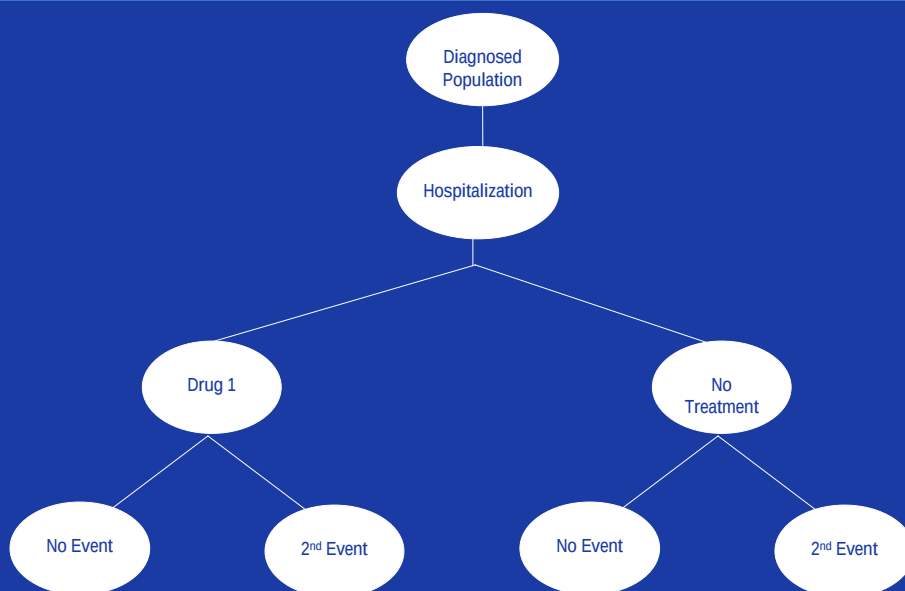
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Documentation

- ▲ Internal model documentation
 - ◆ Summary / introduction page to provide overview
 - ◆ Model schematic to discuss flow of data through model
 - ◆ Discussion of model calculations provided
 - All calculations are clearly identified
 - Discussion of models to allow a lay person to understand
 - ◆ Help buttons
- ▲ Accompanying documentation
 - ◆ User guide
 - ◆ FAQ's
 - ◆ One-page "cheat sheet"
 - ◆ Discussion points (scenario-based examples)

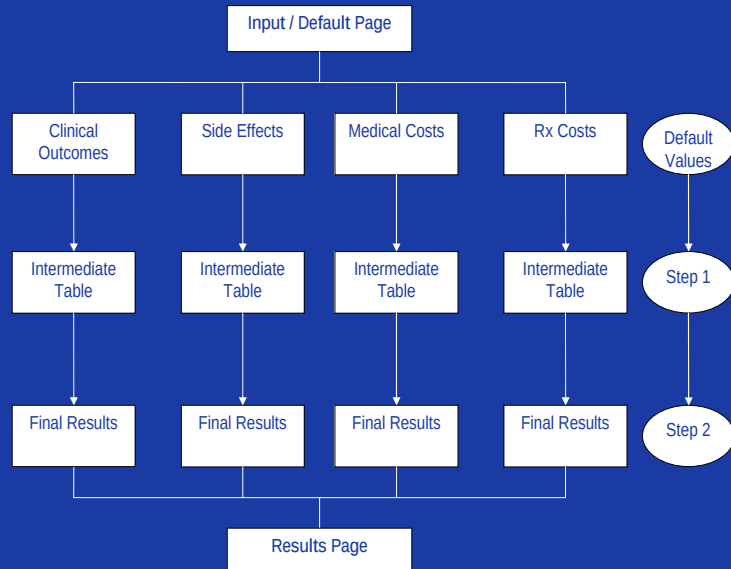
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Documentation - Model Schematic



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Documentation – Default to Results



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Documentation – Default / Input Data

Default Data and Input Data Page			
Category	Sub-Category	Default Data	Data used in Model
Health Plan Data	Number of Member lives	1,000,000	1,000,000
	Incidence Rate of Disease	4.6%	4.6%
	% Hospitalized	25.0%	25.0%
	% of population +65	25.0%	25.0%
Pharmacy cost data	AWP Price (\$) - 30 day	\$95.00	\$95.00
	Co-Pay	\$7.50	\$7.50
	Dispensing Fee	\$3.00	\$3.00

Table 2: Outcome Data: Drug 1			
Category	Sub-Category	Default Data	Data used in Model
All	Primary Event Rate	10.5%	10.5%
	2nd Event Rate	2.5%	2.5%
Under 65	Primary Event Rate	9.0%	9.0%
	2nd Event Rate	3.5%	3.5%
Over 65	Primary Event Rate	12.3%	12.3%
	2nd Event Rate	4.0%	4.0%

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Documentation – Calculations

Category	Value
Analysis Type	PMPM
Total Membership (N)	1,000,000
Member Months	9,000,000
Diagnosed Patients (Total)	11,500

Patient Rx Therapy	Number of Patients
Scenario 1	5,750
Scenario 2	11,500

Drug therapy Data	Cost (\$)
AWP Price (\$) - 30 day	\$95.00
Co-Pay (\$)	\$7.50
Dispensing Fee (\$)	\$5.00

Data Link:
This is a link
from the
[Default page](#)

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Documentation – Calculations

Calculation: Adjusted Drug Cost	
AWP Price (\$) - 30 day	\$45.00
Minus co-pay	\$37.50
Plus Dispensing Fee (Total)	\$42.50

Intermediate Table 1: Calculation of Avg 30-day script cost adjusting for co-pay and dispensing fee

COST CATEGORY	Drug therapy Data		
	Scenario 1	Scenario 2	DIFFERENCE
TOTAL PLAN COSTS	\$2,587,500.00	\$5,175,000.00	\$2,587,500.00
AVG/PT	\$225.00	\$450.00	\$225.00
COST PER MEMBER	\$2.59	\$5.18	\$2.59
COST PMPM	\$0.29	\$0.58	\$0.29

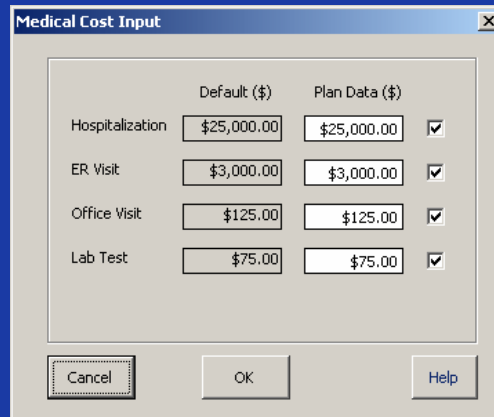
Intermediate Table 2: Shows Cost information by 4 methods
 1) [Total Plan Costs](#)
 2) [Avg/PT](#): Total plan Costs ÷ Number of Patients
 3) [Cost Per Member](#): Total plan Costs ÷ Number of Plan Members
 4) [Cost PMPM](#): Total plan Costs ÷ Number of Plan Members Months

COST CATEGORY	Drug therapy Data		
	Scenario 1	Scenario 2	DIFFERENCE
COST PMPM	\$0.29	\$0.58	\$0.29

Final Results: Data to be used in model results based on user selection. This Data is linked to [Results Page](#).

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Documentation – Input and Help



A screenshot of a 'Medical Cost Input' dialog box. The dialog has a title bar with a close button. It contains a table with two columns: 'Default (\$)' and 'Plan Data (\$)'. The rows are 'Hospitalization', 'ER Visit', 'Office Visit', and 'Lab Test'. Each row has input fields for both columns and a checkbox. The 'Plan Data' column values are all the same as the 'Default' values. At the bottom are 'Cancel', 'OK', and 'Help' buttons.

	Default (\$)	Plan Data (\$)	
Hospitalization	\$25,000.00	\$25,000.00	☒
ER Visit	\$3,000.00	\$3,000.00	☒
Office Visit	\$125.00	\$125.00	☒
Lab Test	\$75.00	\$75.00	☒

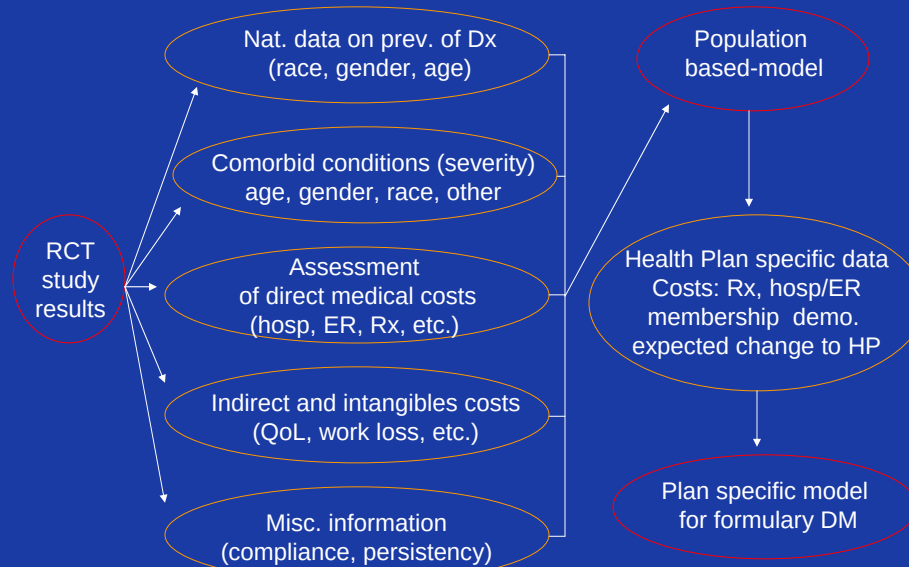
Buttons: Cancel, OK, Help

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Transforming Information into Decisions:
Making models that are relevant to health plans

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Transforming Information into Decisions



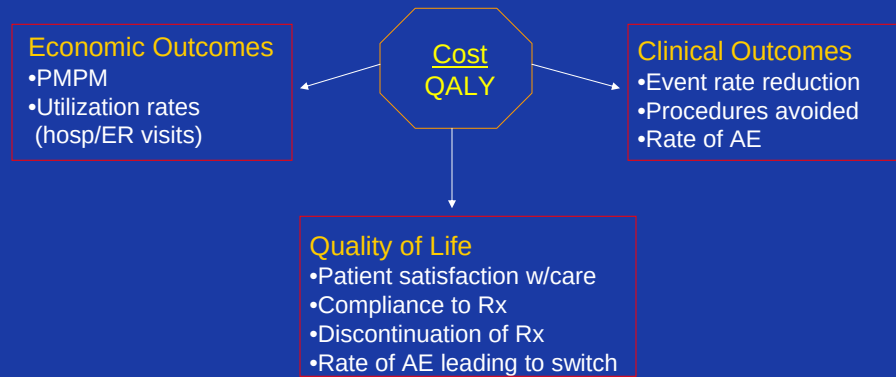
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Transforming Information into Decisions

- ▲ What additional information is needed to make a formulary decision?
 - ◆ Model results to “real world” setting
 - Age, gender and race
 - Patient severity (co-morbid conditions)
 - Prevalence of disease(s)
 - Compliance to drug therapy
 - Enrollment/dis-enrollment patterns
 - ◆ Economic assessment of reductions in “real world” events
 - ◆ Translate information into “health plan” language

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Transforming Information into Decisions



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Transforming Information into Decisions

The screenshot shows a dialog box titled **Results** with the following options:

- Analysis Type 1**
 - ☒ Cost
 - ☐ Utilization
- Disease / Category**
 - All (selected in dropdown)
- Analysis Type 2**
 - ☐ Total
 - ☐ Average per Member
 - ☐ PMPM
 - ☐ DX Population

Buttons at the bottom: Cancel, OK, Help.

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Summary

Modeling should be used as a decision aid

- ▲ Assist in making healthcare decisions by bringing together all key influencing factors into consideration
- ▲ Present elements of the decision to be made in a focused, credible, structured, and transparent manner
- ▲ Assist in prioritizing and handling uncertainty in key outcomes

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Summary

- ▲ Achieving a model that will be considered by peers to be a 'good' model is about finding right balance in scientific and decision credibility.

▲ 'Technical Elements'

- ▲ Clinical credibility
- ▲ Disease theory
- ▲ Level of data collection
- ▲ Model complexity

▲ 'Artistic Impression'

- ▲ Clarity to decision maker
- ▲ Transparency
- ▲ Appropriate to decision
- ▲ Responsive to decision timing



*Ice Dancing
Analogy.....*

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