Università Bocconi
CERGAS
Center for Research on Health and Social Care Management

SDA Bocconi
SCHOOL OF MANAGEMENT

SURROGACY ANALYSIS IN HTA CLINICAL AND COST EFFECTIVENESS ANALYSES: HOW TO STATISTICALLY VALIDATE YOUR MODELS

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ISPOR Europe

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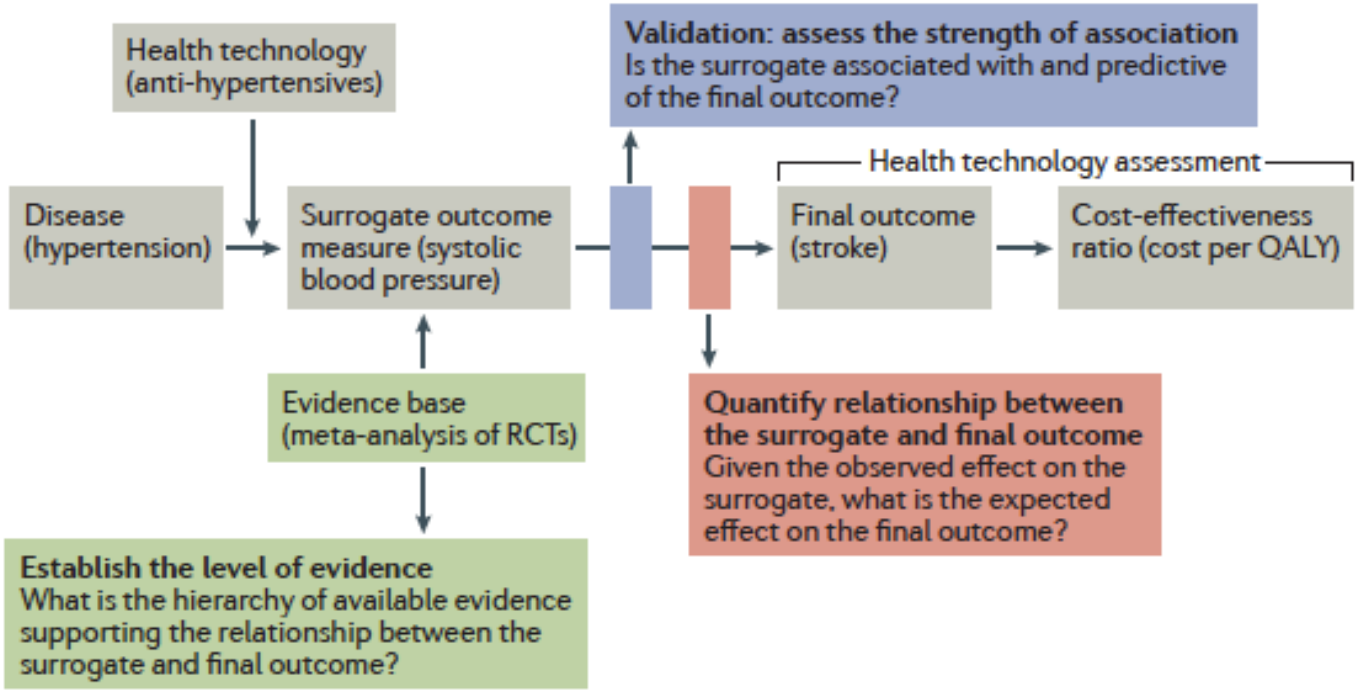
BACKGROUND

- Health technology assessment (HTA) agencies and payers are increasingly faced with reliance on evidence based on **surrogate outcomes**, thus increasing decision uncertainty
- Surrogate outcomes inevitably inform **decision analytic models,** however the methodological approaches adopted to account for their statistical validation are unclear



Ciani O, Buyse M, Drummond M, Rasi G, Saad ED, Taylor RS. Use of surrogate end points in healthcare policy: a proposal for adoption of a validation framework. Nat Rev Drug Discov. 2016 Jul;15(7):516.

OBJECTIVES

1) To introduce the concept of surrogacy and provide an overview of statistical methods to assess surrogacy
2) To illustrate the assumed relationships between surrogate and final patient relevant outcomes and the related treatment effects predicted using risk equations, multi-state models and multivariate models across a variety of different HTA submissions
3) To discuss the gap between ideal and current level of implementation of evidence threshold for establishing validity of surrogate outcomes

- *To start a multidisciplinary discussion between **statisticians** and **economic modelers** on how best practices for surrogate statistical validation could inform decision analytic modeling in HTA*

OUR SPEAKERS

Marc Buyse, ScD
Founder and Chief Scientific Officer, International Drug Development Institute (IDDI) and Associate Professor of Biostatistics, University of Hasselt, Hasselt, Belgium

Bart Heeg, PhD
Vice President, Cytel Rotterdam, the Netherlands

Mario Ouwens, PhD
Senior Statistical Science Director, Group Director, Payer and Medical Evidence Statistics, AstraZeneca, Göteborg, Sweden

It's Time for a Poll!

On a scale from 1 (not important at all) to 5 (absolutely critical), what is the role of RCT evidence for modeling immature or unavailable overall survival (OS)?

- 1 (not important at all)
- 2
- 3
- 4
- 5 (absolutely critical)

Advance to next slide for the poll

It's Time for a Poll!

Please rank the following options in terms of frequency of use for modelling immature or unavailable overall survival (OS)

- Directly extrapolating immature survival data from the trial
- Risk equations linking overall survival to a biomarker/intermediate outcome
- Simulating link between biomarker/intermediate outcome and OS through a (semi-)Markov model
- Predicting treatment effects on OS based on treatment effect on biomarker/intermediate outcome (surrogacy analysis)
- Waiting for when more mature OS data become available

Advance to next slide for the poll

ISPOR TASKFORCE

- This group is working to developing a proposal for an ISPOR Taskforce on
  - *Statistical Evaluation of Surrogate Outcomes - Emerging Good Practices for decision analytic models*

Do get in touch if you're interested!

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THANK YOU

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