

Both?

IP15: REDUCTION OF BIAS OR A BURDEN? THE USE OF INDIVIDUAL-PATIENT MODELS FOR SUBMISSION TO HTA AUTHORITIES

J. Jaime Caro MDCM FRCPC FACP

Professor (adj), Medicine, Epidemiology and Biostatistics, McGill University, Montreal, Canada

Professor in practice, London School of Economics and Political Science, London, UK

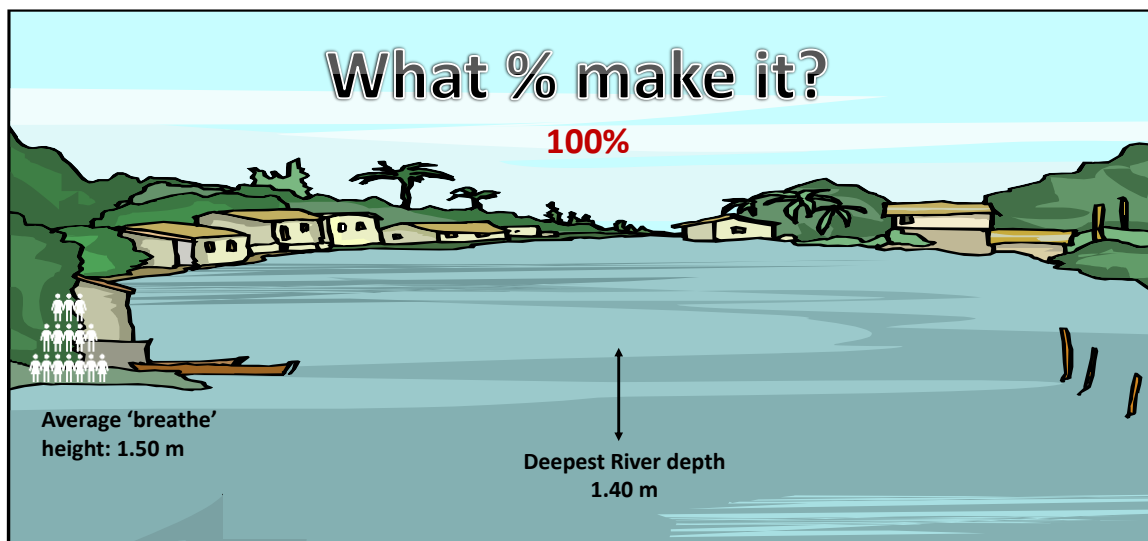
Chief Scientist, Evidera



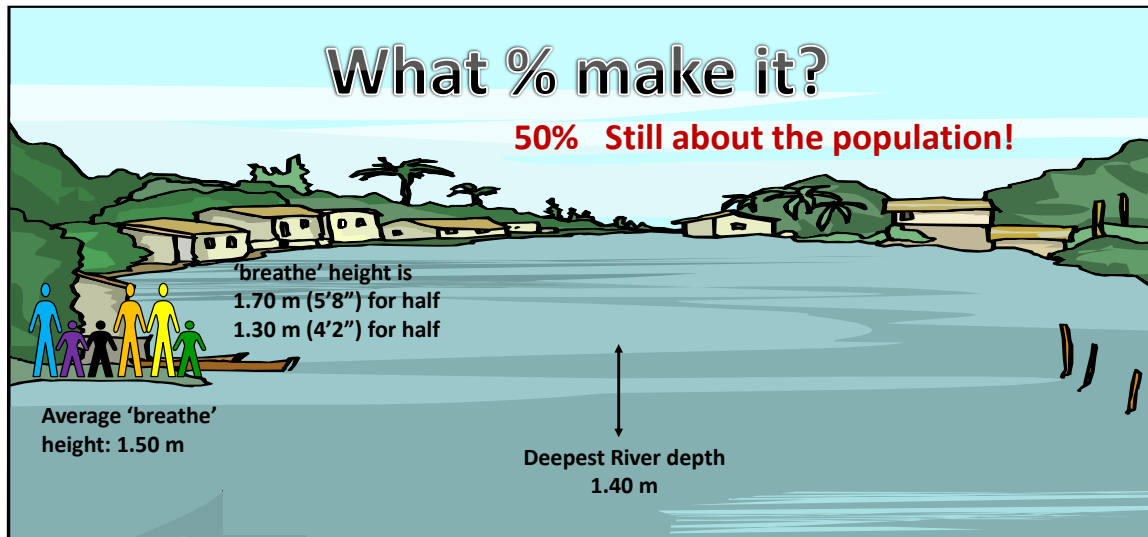
© 2019 J Caro. All Rights Reserved.



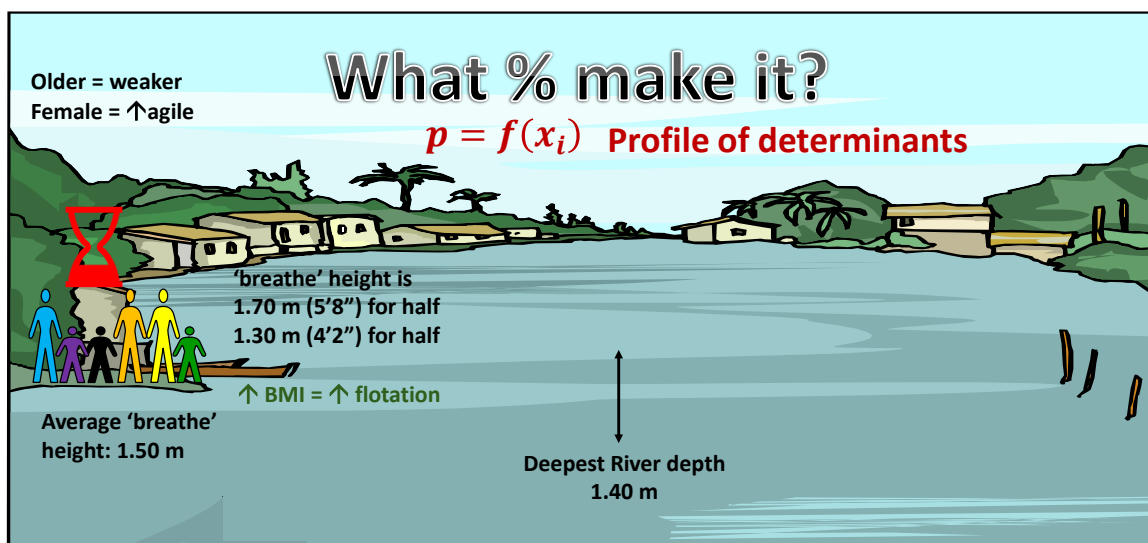
An “individual” model is NOT about individuals



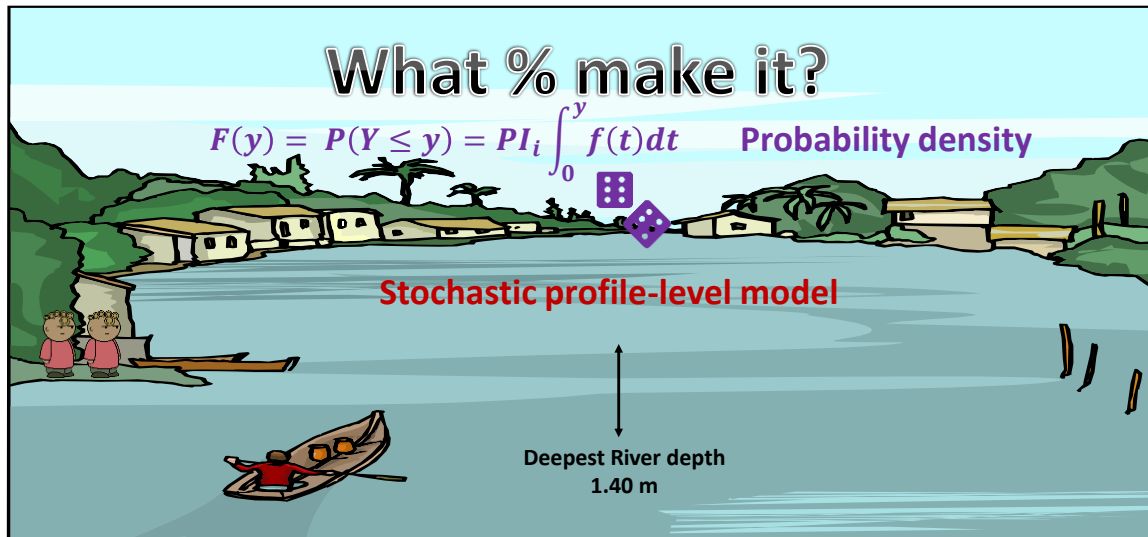
An “individual” model is NOT about individuals



An “individual” model is NOT about individuals



Identical “individuals” may have a different experience



Requirements for profile-level stochastic (“individual”) simulation

Knowledge of the

- Determinants of the course
- Probability density function – the distribution, typically of event times
- Profiles in the population

Data & equations

- On the determinants and their effects
 - ◆ At baseline
 - ◆ Over time
 - ◆ Dependent on treatment
- On the best fitting distributions
- On the profiles

Software to implement.



Challenges in the design and implementation

◆ Defining profiles

- Data from real patients
 - ◆ Confidentiality?
 - ◆ Representativeness
- Sampling from distributions
 - ◆ Correlation
 - ◆ Unrealistic profiles
- Both

◆ Analyses (PSA)

- Gazillions of calculations!
- Execution time

◆ Sampling event times

- Deriving quantile functions
 - ◆ AFT vs PH forms
 - ◆ Each *\$^%!! stats package parameterizes functions and names parameters in its own way
- Conditional or sequential sampling
- Random number generation and cloning

◆ Validation.

Conclusion

“Individual-patient” models should be the default approach in our field. Only in rare cases is a pure cohort approach appropriate