Norwegian Medical Products Agency

Hemoglobinopathies HTA Challenges

Anja Schiel, PhD, Special Adviser,
Lead Methodologist in Regulatory and Pharmacoeconomic Statistics

- Scientific Advice Working Party member
- Methodology Working Party member
- HTA-CG MPG/JSC SG member

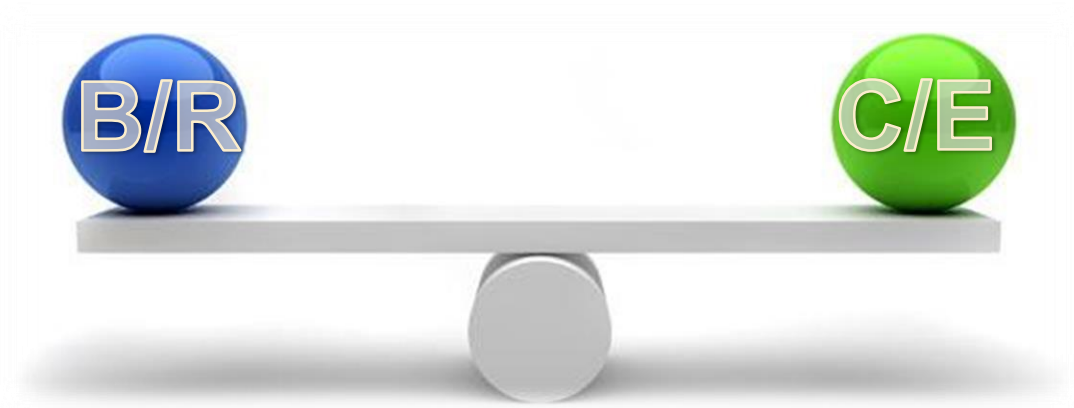
Disclaimer

The views expressed are those of the presenter and should not be understood or quoted as being made on behalf of:

- Norwegian Medical Products Agency (NOMA)
- The European Medicines Agency (EMA) or its scientific committees
- The HTAR Coordination Group (HTAR CG)

The Difference between B/R and C/E

- Health economic models predict the future based on available data from different sources

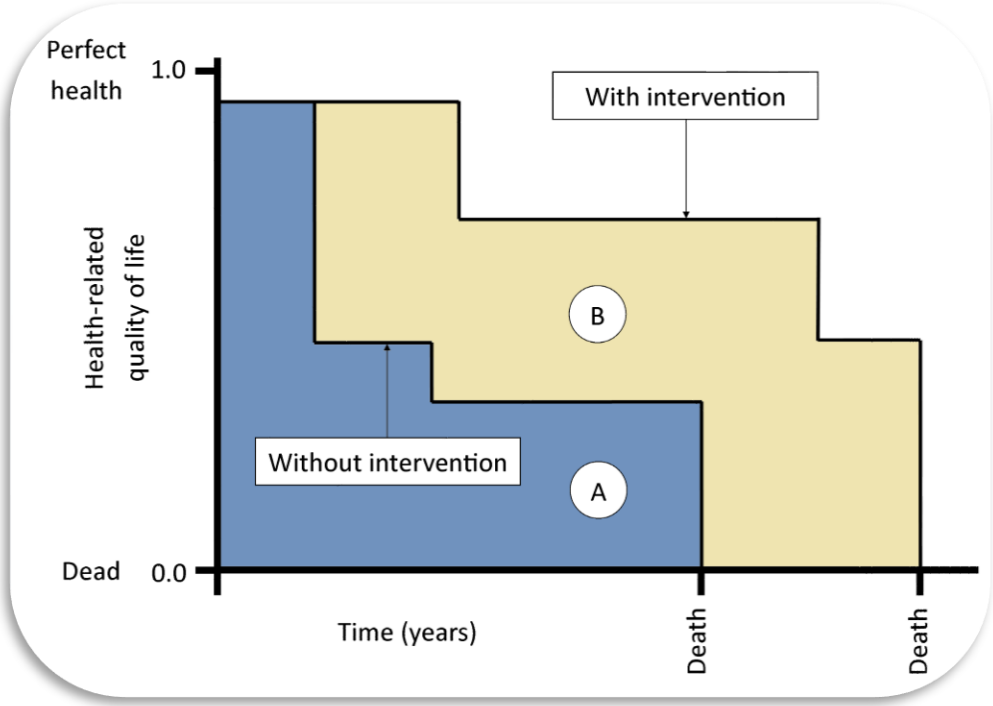


- All models are wrong; some models are useful

George E. P. Box; Norman R. Draper (1987)

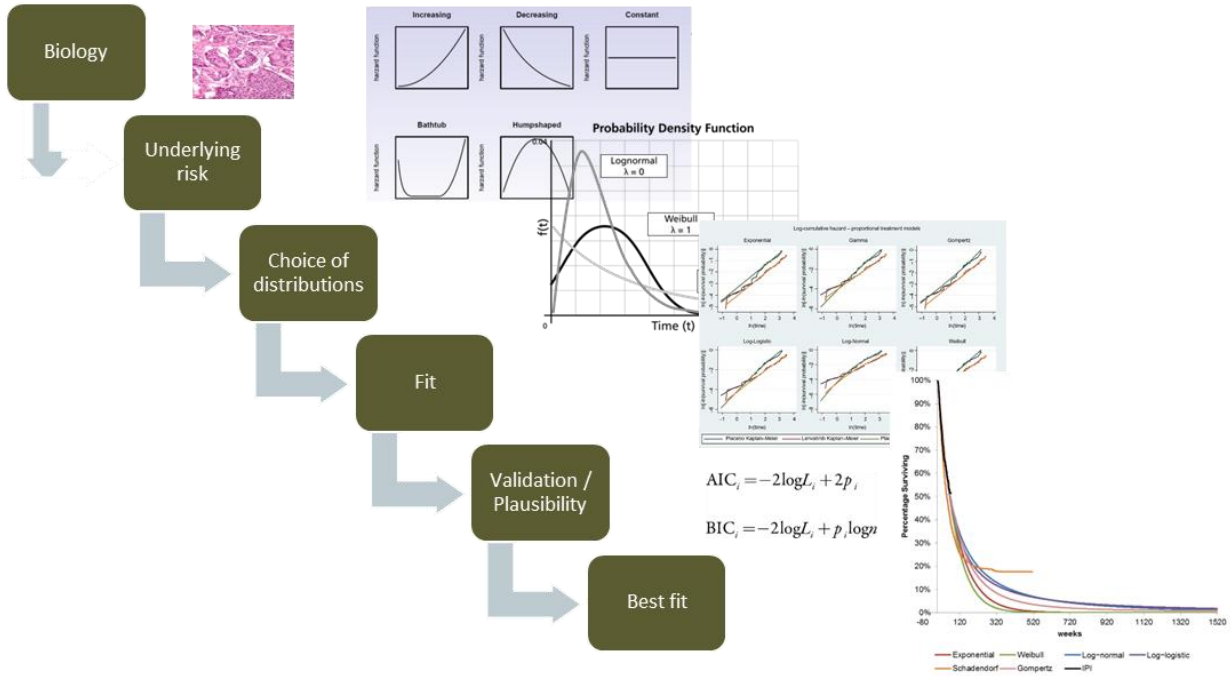
The principle of QALY's

- Many HTABs do Health Economic assessments based on cost-utility analyses (CUA), not all.
- The quality-adjusted life year (QALY) is a generic measure of disease burden, including both the **quality** and the quantity of life lived. (Wikipedia)



The principle of QALY's

- NoMA does Health Economic assessments based on cost-utility analyses (CUA)
- The quality-adjusted life year (QALY) is a generic measure of disease burden, including both the **quality** and the quantity of life lived. (Wikipedia)
- Unless we have a study that would cover the entire length of the natural course of the disease, we need to extrapolate beyond the observation in the trial.



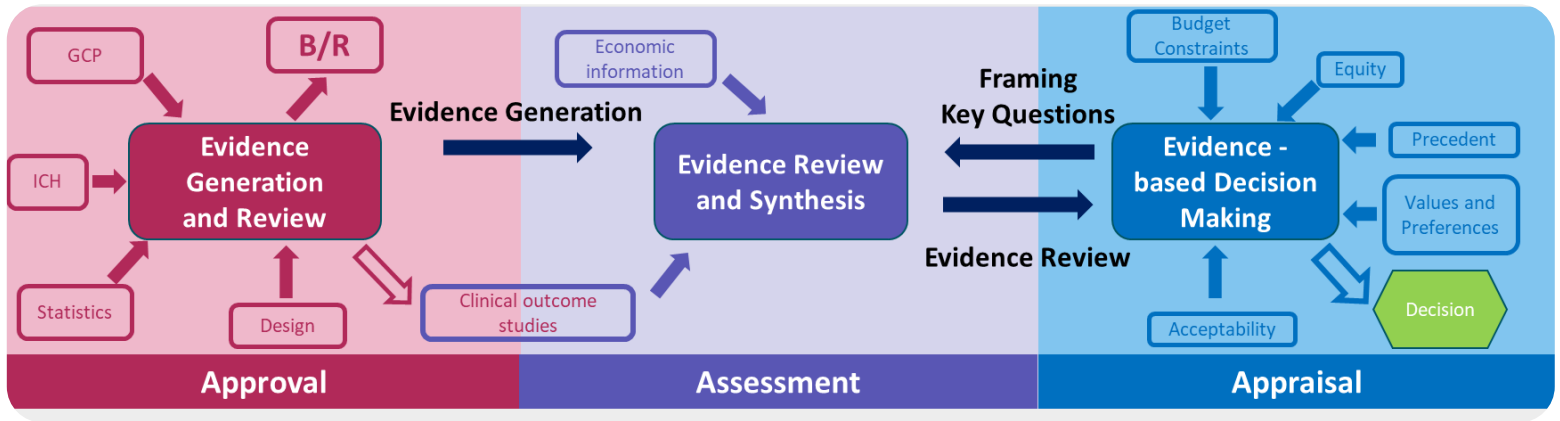
The principle of QALY's

- NoMA does Health Economic assessments based on cost-utility analyses (CUA)
- The quality-adjusted life year (QALY) is a generic measure of disease burden, including both the **quality** and the quantity of life lived. (Wikipedia)
- Unless we have a study that would cover the entire length of the natural course of the disease, we need to extrapolate beyond the observation in the trial.
- We create what is called a counterfactual reality

How do you want it - the crystal mumbo jumbo or statistical probability?

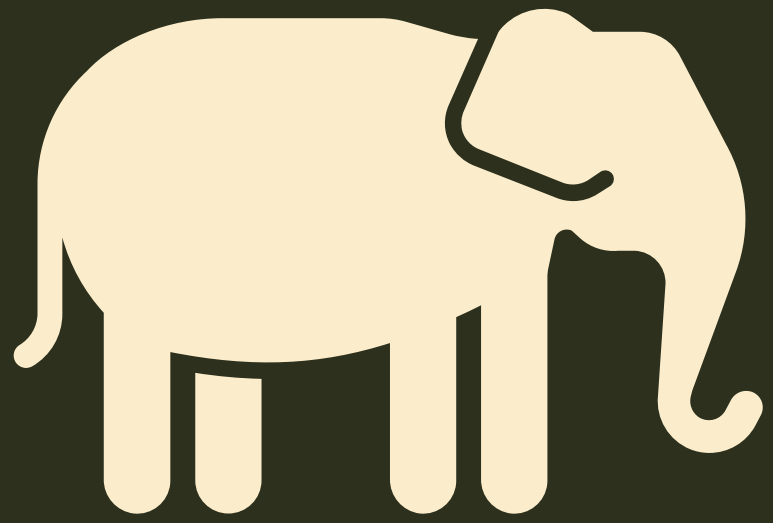
Welfare Economics

- A Pareto improvement if it leaves **everyone** in a society better-off (or at least as well-off as they were before) -> assumption is made for the entire society
- A Kaldor–Hicks improvement if those that are made better off could **hypothetically compensate** those that are made **worse off** and lead to a Pareto-improving outcome. -> there are losers!
- Health care systems are too complex to allow identification of the losers -> we make decisions under uncertainty when it comes to the compensation part.



Adapted from Teutsch, S.; Berger, M. (2005) 'Evidence synthesis and evidence-based decision making: Related but distinct processes. *Medical Decision Making*, pp 487-489

What is the elephant in the room then?



If one-time is as good as chronic treatment

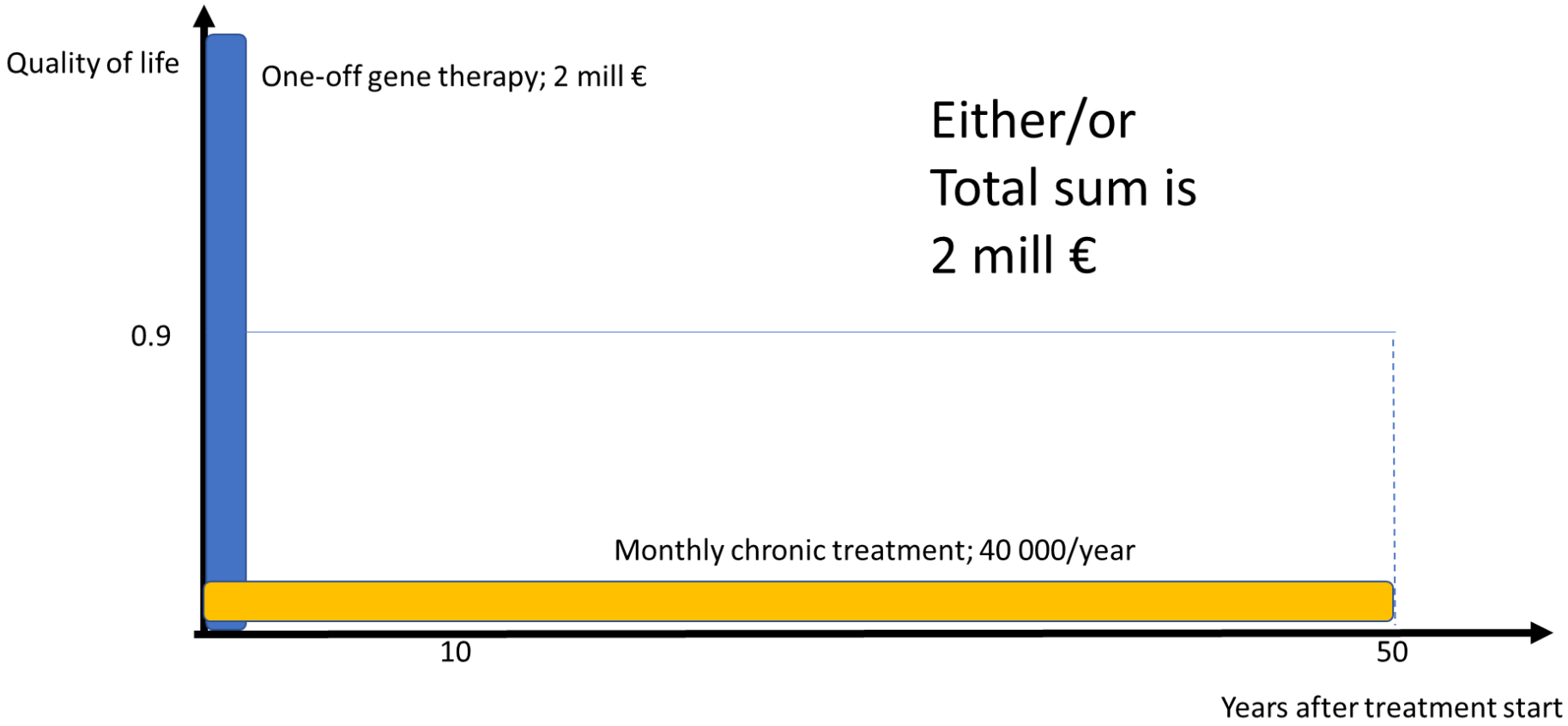


Figure modified from ‘Hur ska vi utvärdera och hur skavi betala?’, 2021; TLV Sweden

But if not, the irreversibility phenomenon kicks in

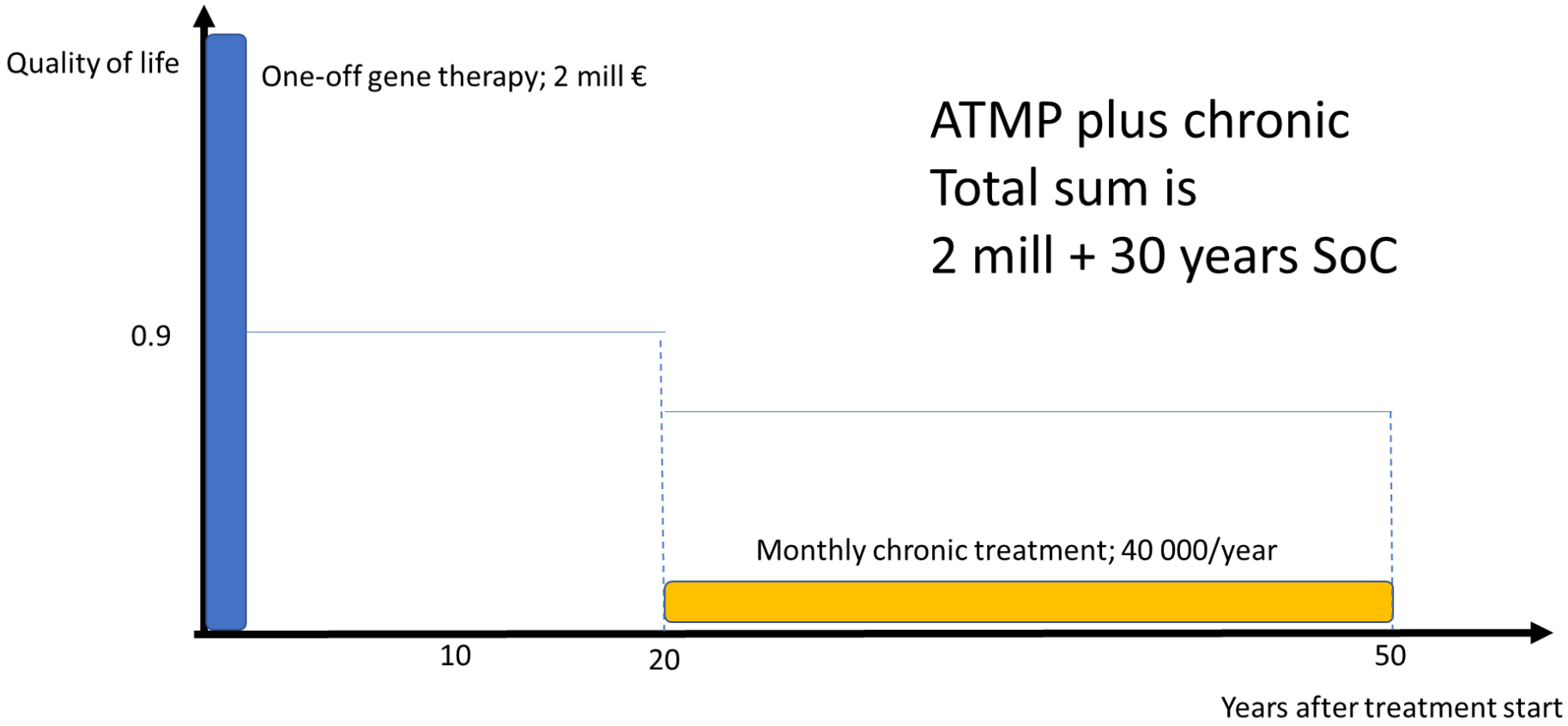
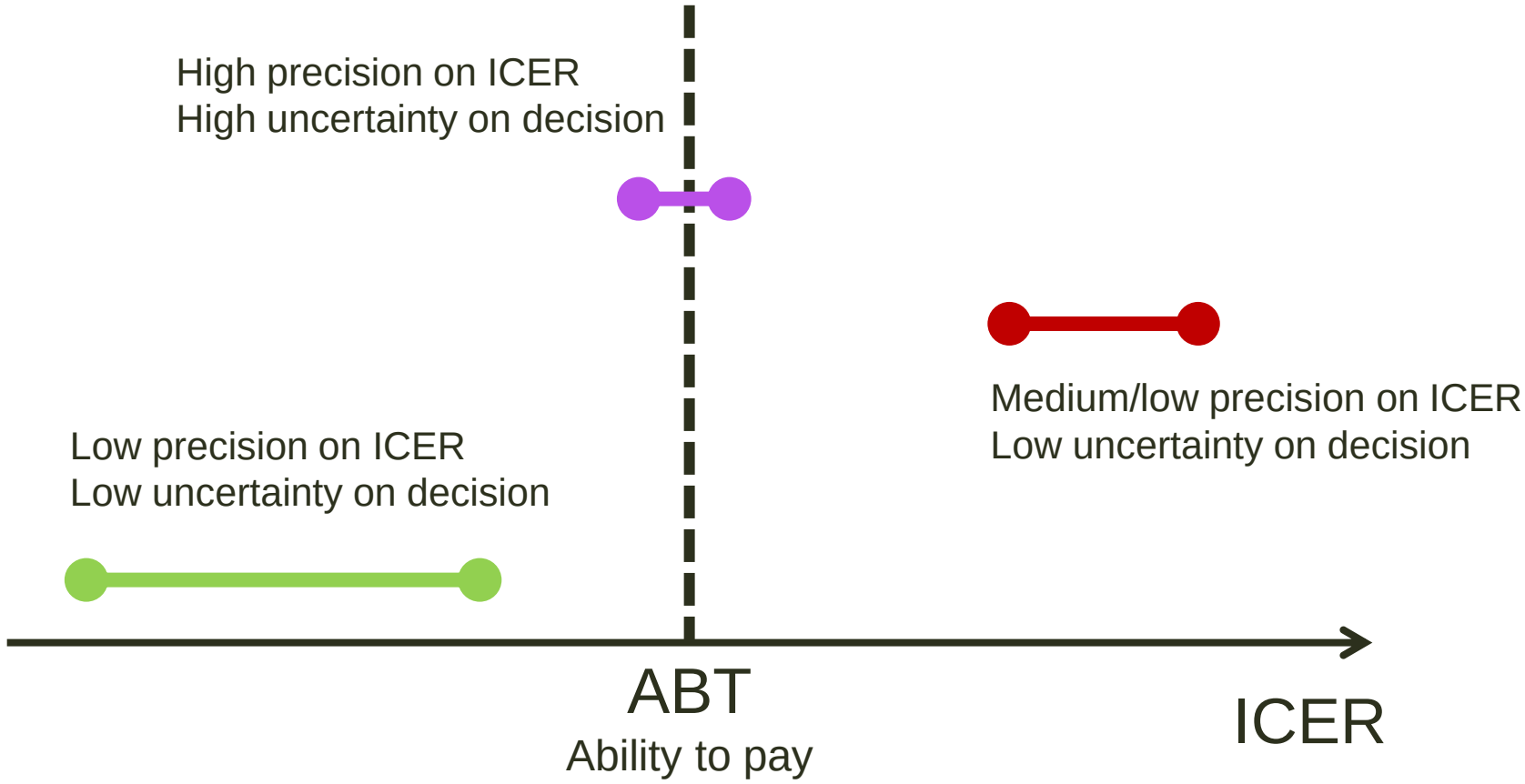


Figure modified from 'Hur ska vi utvärdera och hur skavi betala?', 2021; TLV Sweden

Uncertainty of decision is a sum of all uncertainties



dmp.no

helsenorge.no

Direktoratet for medisinske produkter

Norwegian Medical Products Agency