

HEOR Excellence



Uncertainty in health care evaluations – an industry perspective

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Issue statement for this presentation

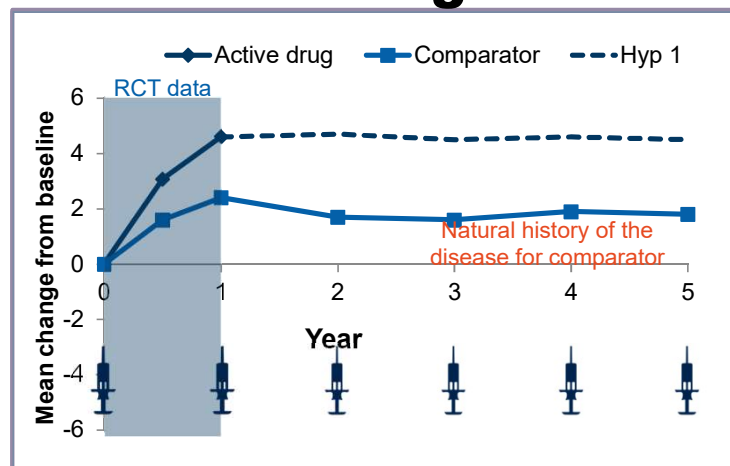
Health economists have **little (or no)** role to play assessing **ATMP uncertainties** for **long-term effectiveness**

ATMP: advanced therapy medicinal product (cell or gene therapy)

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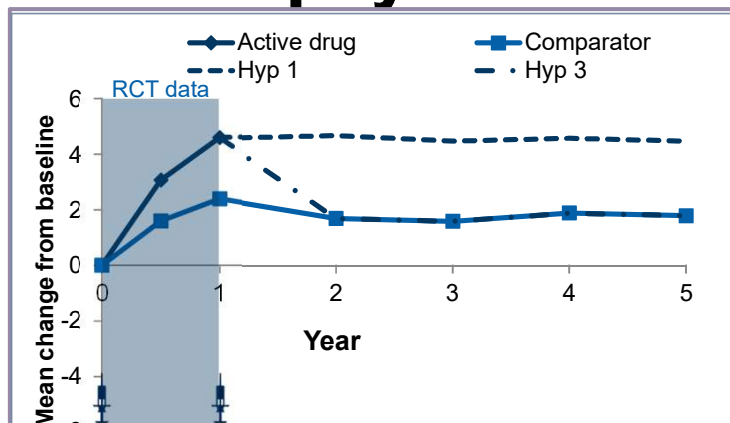
Modeling the lifelong impact of a non-DMT can be a challenge...



DMT: disease modifying therapy

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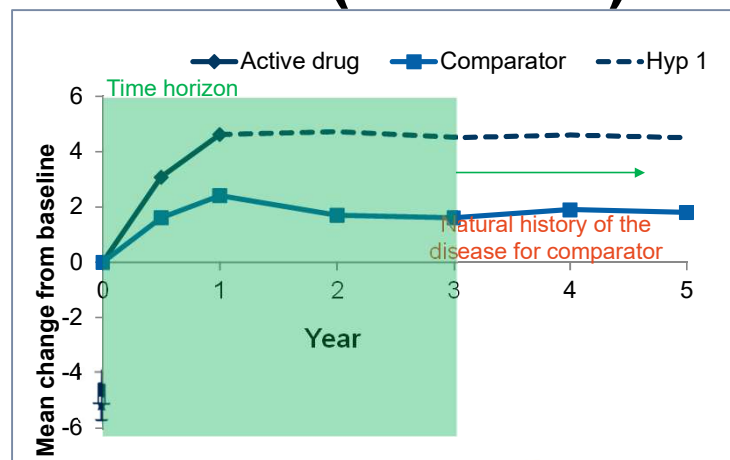
However, for non-DMT, health economists can play with # of doses



As a result, the long-term efficacy assumptions do not impact dramatically the ICER (or the cost/injection)

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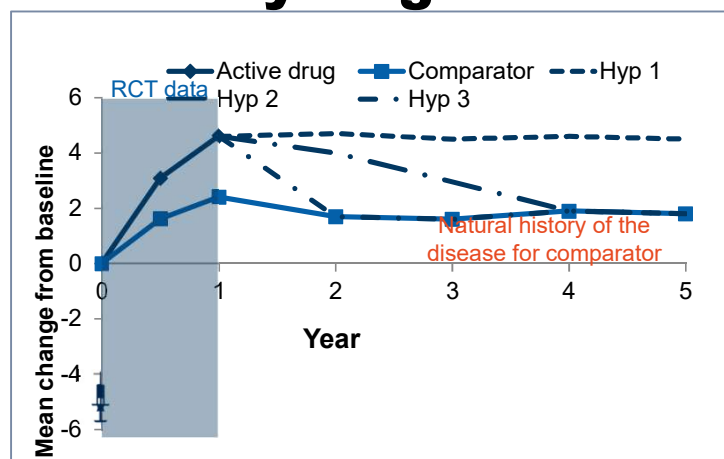
However, for ATMP, time horizon impacts the ICER (and EJP*)



*EJP: economically justifiable price

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As well as long-term effectiveness (with sufficiently-long time horizon)



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But concretely, what is the impact of time horizon?

- Assumptions*

- Cure of a fatal disease of a 5 year-old child who would die in 10 years with standard Rx
- 50 incremental QALY linearly distributed over 60 years
- WTP threshold of \$100,000/QALY
- Discount rate of 0

- Economically justifiable price as a function of the time horizon

Time horizon	QALY gained	Cost-offset	Economically justifiable price	% vs. lifetime horizon
Lifetime	50	\$4MM	\$9MM	100%
20 years	16.7	\$4MM	\$5.7MM	63%
10 years	8.3	\$4MM	\$4.8MM	54%
5 years	4.2	\$2MM	\$2.4MM	27%

Adapted from Steven D Pearson. Valuing a Cure: Are New Approaches Needed? ISPOR. Barcelona, Spain. Nov 13 2018.

⁸ Available at https://www.ispor.org/docs/default-source/presentations/88571pdf.pdf?sfvrsn=39cc55a7_0 (accessed Dec 13 2018)

What did we learn from the ATMP illustration? A limited role for the HE

- Time horizon impacts dramatically the EJP
 - If a manufacturer prices a drug with a lifetime time horizon in mind then it will never be cost-effective if the HTA only recognizes 5 or 10 years of value
- We don't need any health-economics modeling to reach that conclusion!
- Is the HTA or the manufacturer right when considering different TH?
 - Difficult to say *a priori*
 - In practice, 5 to 7 years data on phase I patients might be available
 - The extrapolation should be a scientific and not a HE consideration
- If only a short time horizon is considered for ATMP, why develop ATMP?
 - If patient pool is very small, the business case to develop an ATMP might disappear

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Conclusions

- **Some** but **not all** uncertainties can be solved by health economists methods
- In fact, some methods are likely **irrelevant** for ATMP
 - Traditional sensitivity analyses (waning, shorter time horizon) are uninformative
 - Need to go beyond a pure check-list approach
 - Importance of scientific input (e.g. on risk of gene silencing)

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