

# Round Table Discussion

## *The use of external controls in regulatory decision making*

EFSPI 10th Statistical Leaders Meeting  
July 4, 2019

# Background

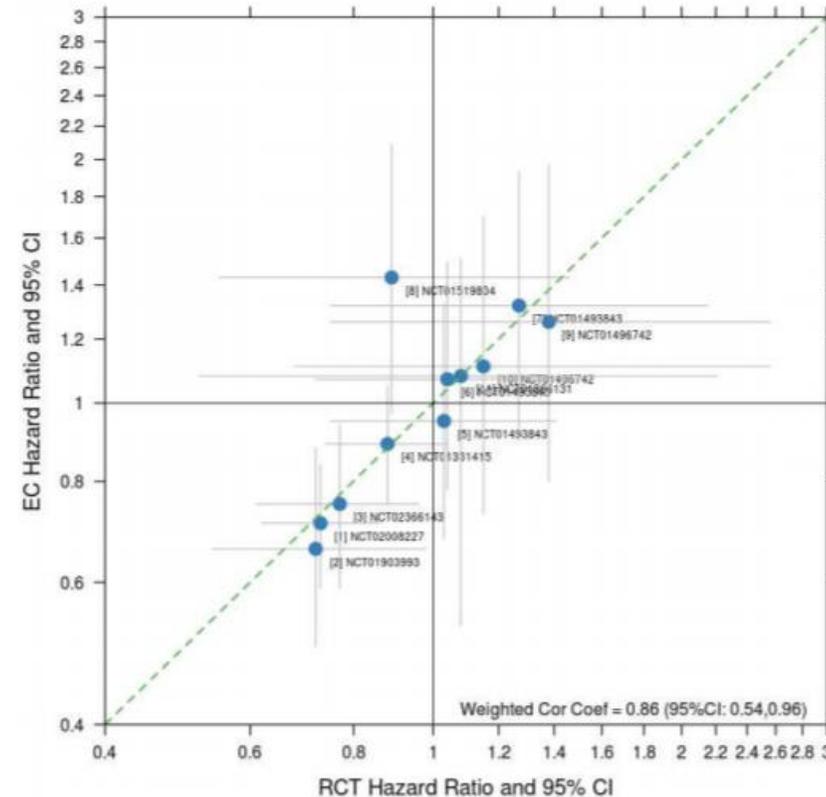
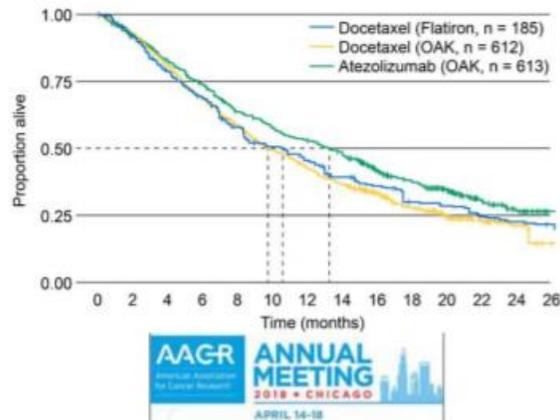
- With digitization of HR we have more and more access to good quality data external to clinical trials
- Key question:  
With modern techniques (e.g. propensity score) can we make meaningful use out of such data to replace randomized controls by controls external to the study?
- There are workups which show that external controls could replace randomized controls and there are examples which show that it does not work
- There are examples where HAs accepted single arm studies with external controls and other examples where they did not
- Fact is that there is a “tradition” (oncology, RD) where single arm data can be accepted

# Example: Validation by replication in lung cancer

## Treatment effects with EC



- Trials results replicated:
  - Treatment effect estimates, except for one trial
  - Conclusions from statistical tests ( $H_0: \log HR = 0$ )



# 40 mins. Discussion

- Is this becoming a Hype in your company ?
- How much is your department engaged in this topic ?
- Is this an important topic for yourself and why ?
- What do we need to do to solve/implement it?

# Summary for 5–7 mins. plenary feedback

- Is this an important topic for yourself and why?
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- What do we need to do to solve/implement it?
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