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Perspective from EMA

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BBS Seminar: Statistical challenges in the clinical development of CAR-T cell therapies
March 22nd 2021

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Introduction

- CAR-T therapies are a fundamental change in the treatment paradigm
  - "All the problems together at once""

- It is particularly important to carefully think through the actual question/s of interest and how to incorporate it/them in the study

- The level of evidence needed for an MAA is not different for traditional and advanced therapies

When coming for Scientific Advice…

- Carefully describe the question/s of interest, study desing, estimand and statistical methdology

- (And make sure they all fit together…)

- For a complex regimen, more than one measure may be needed to capture the treatment effect

- Most importantly: justify and explain your proposal

- For CAR-T, this is particularly important because traditional design features and endpoint definitions included in guidelines and publications for an indication may not apply

When submitting you MAA…

- Hopefully, you have followed the Scientific Advice!

- Interpret the results according to the estimands concept

- Walk us through the totality of evidence that has been generated to support the MAA

- Explain the rationale for the figures included in the SPC and discuss their statistical robustness

- For a complex therapy, more than one estimate may be needed

Focus on the most relevant scientific question