

DATA MANAGEMENT • BIOSTATISTICS • PROGRAMMING • MEDICAL WRITING • PHARMACOVIGILANCE



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Oncology Efficacy Endpoints

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Things we will cover



- Purpose of endpoints
- RECIST
 - Measurements
 - Outcomes
 - Calculations
- Other endpoints

Purpose of end points



- Track if cancer gets better or worse
- A comparable measure when comparing treatments

RECIST



Response Evaluation Criteria In Solid Tumours

A method for measuring cancer.

Lesions

- Target lesions
- Non target lesions
- New lesions



Responses



- Complete response
- Partial response
- Stable disease
- Progressive disease
- Confirmed responses

Methods of measurement



- Computed tomography (CT)
- Spiral CT
- MRI
- X-ray - Not recommended
- Consistency
 - within a subject
 - across study

Non measureable disease



- Too small
 - 😊
- Too big
 - ☹️
- Has been radiated
 - Lesion becomes non evaluable which may lead to subject being censored

Visit windows



- Measurements to be taken at predictable and comparable time points.

Missing data & censoring

- Missed visits
- Missing values
- Radiated lessions
- Solutions
 - Scaling
 - PD



RECIST versions



- Version 1.0 and version 1.1
- Main differences
 - Number of target lesions reduced
 - Absolute increase added to PD
 - Guidance added for lymph nodes, bone lesions and other tumours which can behave differently to typical tumours.

Endpoints



- Many are based on numbers/responses from RECIST
- Some use questionnaires and/or safety data

Survival data



- Overall survival (OS)
 - Time to death
- Progression free survival (PFS)
 - Time to disease (RECIST) progression
- Are PFS and OS correlated?
 - Can this be used to simplify/shorten studies?

Disease control



- Compare treatments at a preselected time point
 - i.e. take RECIST response at 24 weeks and summarize the data to compare the treatments.

Best response



- Best overall RECIST response (BoR)
 - i.e. take the best response each subject has during treatment then summarize for comparison

Quality of life



- Questionnaires
 - All have pain, impact on life, how subject feels
 - Some are specific to cancer type
 - i.e. shortness of breath for lung cancer.
- Adverse events
- Cost of health care

Conclusion



- RECIST gives us a clear way to track and compare tumour burden for cancer patients.
- When discussing endpoints and treatment options it is worth considering all the different factors to find out what is important to the subject.
 - i.e. length of life, reduced tumour burden or better quality life

References/Further reading



- <https://www.eortc.be/Recist/documents/RECISTGuidelines.pdf>
- <http://www.eortc.org/investigators-area/recist>
- <http://www.recist.com/recist-comparative/01.html>
- <http://web.quanticate.com/overview-of-the-challenges-for-oncology-studies>
- <http://imaging.cancer.gov/clinicaltrials>
- <http://www.facit.org/FACITOrg/Questionnaires>
- <http://info.quanticate.com/bid/69353/Considering-the-Ethics-of-Oncology-Clinical-Trials>



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Any Questions?