EMWA workshop

Clinical study reports in oncology

Workshop leaders: Thomas M Schindler, Kirsten Herbach (Boehringer Ingelheim Pharma)

Homework

(Time required: about 2.5 h)

Please read the EMA "Guideline on the evaluation of anticancer medicinal products in man", Sections 4 to 6
http://www.ema.europa.eu/docs/en_GB/document_library/Scientific_guideline/2013/01/WC500137128.pdf

In addition, you may want to look at the following links:

- NCI homepage (www.cancer.gov)
- FDA Guidance for Industry "Clinical Trial Endpoints for the Approval of Cancer Drugs and Biologics" (2007) (https://www.fda.gov/downloads/Drugs/Guidances/ucm071590.pdf)
- Davis C, Naci H, Gurpinar E, Poplavska E, Pinto A, Aggarwal A (2017): Availability of evidence of benefits on overall survival and quality of life of cancer drugs approved by European Medicines Agency: retrospective cohort study of drug approvals 2009-13, BMJ 359 (http://www.bmj.com/content/bmj/359/bmj.j4530.full.pdf)
- Technology Quarterly "Targeting tumours", The Economist, September 2017 (https://www.economist.com/technology-quarterly/2017-09-16/treating-cancer)

Please answer the following questions in some depth:

1. Conventionally, phase I trials in oncology are not performed in healthy volunteers but in patients with cancer. What are, in your opinion, reasons for this?
2. What is usually investigated in phase I oncology trials? List at least 3 items and give explanations for each item.
3. The design of phase I oncology trials was developed in the era of cytotoxic drugs. With the advent of non-cytotoxic drugs, certain design features of phase I trials are being challenged. Please give examples (at least 2) and provide some justification for choosing these examples.
4. What is the evidence for the usefulness of accelerated approval pathways for new oncology drugs?

Please send your answers in a word or pdf file to kirsten.herbach@boehringer-ingelheim.com and thomas.schindler@boehringer-ingelheim.com. Please return your homework **by 19 April 2019** to receive credit for the workshop.