

Assessment of your knowledge

(a) Answer the following questions to assess your command on terminology, facts, concepts, and theories learned in this chapter:

1. Explain the Medical Devices and classification in Classes I-IIa-IIb-III.
2. Explain the IMDD-investigational medical device dossier in terms of its content.
3. In what year did the new Medical Devices Regulation (MDR) go into effect?
4. What is the biggest change/benefit in the new MDR compared to the previous regime?
5. To whom/which organization does the manufacturer of medical devices report to?
6. Which products are understood as ATMPs and why?
7. When was the new ATMP regulation introduced? By what organization?
8. Difference between sponsor and principal investigator of a clinical study; who is liable in case of major Serious Adverse Events, including fatalities?
9. Explain the distinction between efficacy and effectiveness of a new therapy.
10. What documents need to be prepared/signed of before a clinical trial can be initiated?
11. Since double blinding a trial intervention is not always feasible, which stakeholders should be consulted at the study design phase?
12. Explain the difference between a directive and a regulation.

(b) Answer the following questions to assess your ability to apply the concepts and theories learned in this chapter in real life, clinical, and scientific situations.

1. Discuss choice of appropriate preclinical animal models for joint surface repair
2. Discuss choice of preclinical animal models for the treatment of large bone defects
3. Discuss/define the timelines for time to market of a novel bone defect healing strategy with a medical device versus an ATMP with as example:
 - a. a bioceramic only
 - b. a bioceramic coated with a growth factor
 - c. a bioceramic with growth factor and expanded bone marrow stromal cells seeded on it.
4. Clinical trial design:
 - a. Why is it important to compare novel treatment strategies to the standard-of-care?
 - b. What if the standard-of-care has never been investigated properly versus placebo control?
 - c. What consideration should be given to defining the control?
5. What do we mean by unmet medical need? How do you assess the unmet medical need?
6. Discuss the importance of manufacturing and scaling of a novel device or ATMP
7. What are the required characterizations of the product/cells (ATMPs/HCTPs) intended for the treatment that should be evaluated to ensure that the final product can be manufactured robustly and reproducibly?
8. Can you comment on challenges encountered in study design/or conduct for ATMPs regarding choice of outcome measures and subject care and follow-up?
9. What kind of monitoring and audits are warranted, and why?
10. What items should be included in the informed consent? what is the importance of the informed consent process? and how would you document this process?
11. Please discuss short and long-term efficacy based on examples.