Clinical development checklist

- Apply the same manufacturing process to the materials used in clinical studies as has been used in pivotal non-clinical studies
- Route of administration should be similar in non-clinical and clinical studies
- Investigate the feasibility of the route of administration
- Standardize the administration procedure
- Demonstrate that the drug reaches the site of action
- Investigate pharmacokinetic characteristics
- Determine the optimal dose regimen
- Analyse the dose-response relationship
- Demonstrate the mechanism of action
- Investigate the off-target effects
- Demonstrate efficacy
- Investigate safety and tolerability
- Fit the study population with the therapeutic indication and target population of the product
- Include clinical endpoints relevant for the therapeutic indication and target population in pivotal trials
- Distinguish the effects of concomitant medication from the ATMP effect
- Determine sources of variability in drug response
- Update the risk profile according to the risk based approach
- Collect relevant data for the environmental risk assessment
- Integrate post-authorisation studies in the development plan
- Ask for Scientific Advice