EMA'S GUIDE ON ADVANCED THERAPY MEDICINAL PRODUCTS

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