Fragen zur Neurosensorik-Vorlesung vom 18.10.10 zum Thema:
"Restoration of cone vision in the Cnga3-/- mouse model of congenital complete lack of cone photoreceptor function using AAV-mediated gene replacement"

1. **Question:**
   What is a gene replacement therapy?

2. **Question:**
   Name three requirements for successful gene therapy!

3. **Question:**
   Briefly describe the principle of *ex vivo* gene therapy. Which is the preferred vector used? To which tissues is *ex vivo* gene therapy limited today?

4. **Question:**
   Why is the eye an ideal target for the application of gene replacement therapy? Provide four reasons!

5. **Question:**
   Gene therapies for two eye diseases are currently at the stage of clinical trials. Name them and briefly describe their characteristics!

6. **Question:**
   Describe lentiviruses on the basis of three characteristics! What are the advantages and disadvantages of lentiviral vector systems for gene therapy approaches?

7. **Question:**
   For the gene therapy of which human diseases are lentivirus-based vector systems applied? Provide three examples!

8. **Question:**
   Describe adeno-associated viruses (AAV) on the basis of three characteristics! What are the advantages and disadvantages of recombinant AAV vector systems for gene therapy?

9. **Question:**
   Briefly describe the eye disease 'Achromatopsia'. Defects in which genes are causing it?

10. **Question:**
    Briefly describe a successful murine gene therapy approach for 'Achromatopsia'. Which approach of gene application and which vector system are used? Which mouse model (knock out) is treated? How is the success evaluated?