GGSB PRELIM QUESTION # 2

Question:
As a concept, gene therapy has been around for decades. But it has only recently emerged as an effective therapeutic modality with a number of drugs getting approved in the last few years. With gene therapy finally moving beyond the proof-of-concept stage, the field is poised to flourish, with many previously incurable diseases now expected to have complete cures in the coming years.

Please describe the major classes of diseases targeted by gene therapy either as approved treatments or at the research stage. Please explain why these diseases are particularly conducive to gene therapy. Conversely, please describe what types of diseases are not conducive to gene therapy at present and why.

What are the major limitations and challenges in the effective use of gene therapy?

Lentivirus (LV) and adeno-associated virus (AAV) are currently the most popular gene therapy vectors. Please describe the pros and cons of LV and AAV, and what types of diseases they are each suitable for.

References:

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2. Wang D, Tai PWL, Gao G. Adeno-associated virus vector as a platform for gene therapy delivery. Nat Rev Drug Discov. 18:358 (2019) https://www.nature.com/articles/s41573-019-0012-9

3. Cavazzana M1,2,3,4, Bushman FD5, Miccio A6,7, André-Schmutz I8,6, Six E. Gene therapy targeting haematopoietic stem cells for inherited diseases: progress and challenges. Nat Rev Drug Discov. 18:447 (2019). https://www.ncbi.nlm.nih.gov/pubmed/30858502