2. Long-term correction by gene therapy, but **T-ALL** in 40% of patients.

- **Lipid metabolism disorders:**
  1. AAV mediated delivery of lipoprotein lipase (LPL) gene for the treatment of LPL deficient (**LPLD**) recently licensed in the EU as the first gene therapy product in the west.

- **Haemophilia:**
  1. Prolonged blood clotting times, hence life threatening external and internal bleeding.
  2. AAV mediated introduction of the therapeutic gene into the liver of patients results in normal clotting times.
  3. **The therapeutic effect is transient** – gene corrected cells are recognized and thus destroyed by the immune system.

- **Other genetic disorders:**
  1. Clinical trials in progress for various genetic disorders, including congenital blindness, lysosomal storage disease, muscular dystrophy, neuromuscular disorders, sickle cell anaemia, and beta-thalassemia.

- **Cancer:**
  1. Multiple gene therapy strategies for a variety of cancers, including **suicide gene therapy,** oncolytic virotherapy, anti-angiogenesis, and therapeutic gene vaccines.
  2. 2/3 of all gene therapy trials are for cancer and many of these are entering advanced stages of development.
  3. Two products have been licensed in China since the mid-2000S and now also the first product (gene therapy) in the US is for cancer.

- **Neurodegenerative diseases:**
  1. Recent progress in gene therapy has allowed for novel treatments of neurodegenerative diseases such as Parkinson's Disease and Huntington's Disease.

- **Other acquired diseases:**
  1. Gene therapy based strategies have also been applied to the treatment of other acquired disorders such as viral infections (i.e. influenza, HIV, hepatitis), heart disease, diabetes, to name a few.
  2. HIV:
  - Bone transplants from a donor with the **CXCR5 mutation** leaves the patient HIV-free and the T cells are no longer infectable by the virus.
  - This is done via CRISPR, whereby the mutation is introduced to stop HIV entry into the T cells