**Target identification**
- C9ORF72 protein levels
- Abolish RNA expansions
- Increase RNA binding proteins
- Identify other genes or epigenetic factors influence  
  C9ORF72 phenotype

**Lead identification**
- Expansion binding oligonucleotides
- C9ORF72 elevating drugs
- Drugs repurposed from ALS trials based on  
  C9ORF72 genotyping

**Preclinical Efficacy**
- C9ORF72 knockout
- Hexanucleotide expansion transgenic flies, worms, mice
- IPS cells derived neurons from c9FTD/ALS patients

**Preclinical Safety**
- Phase 2 trial
- Biomarker(s) of C9ORF72 effects

**Human safety**
- Phase 3 trial
- Inclusion based on genotype
- Possibility of prevention trial in asymptomatic gene carriers

**Human target engagement**
- Regulatory
- Orphan status
- Possible extended indication in sporadic, other genetic FTLD-TDP cases

**Human Efficacy**
- Human engagement
  Approval