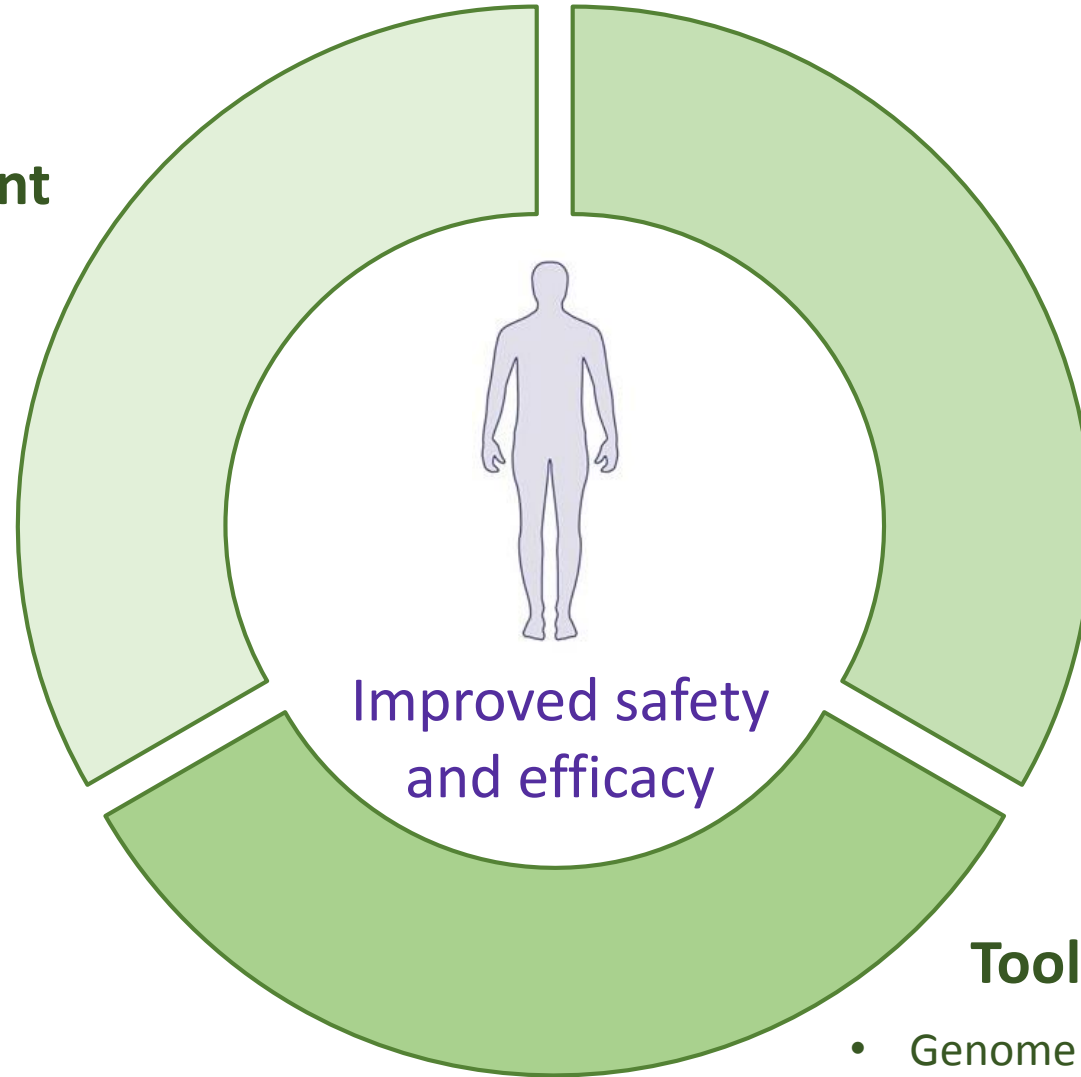


## Accessibility of treatment

- Expanding number of conditions treatable by gene therapy
- Automation of process
- Cryopreservation of product to allow distribution to wider centers



## Understanding

- Increasing evidence of safety and efficacy from Phase I/II clinical trials
- Improved integration site analysis

## Tools

- Genome editing platforms (ZFNs, TALENs, CRISPR/Cas9) to allow precise correction
- AAV mediated homologous recombination
- Alternative vectors such as alpharetroviruses