

FIG. 1

Packaging cell line based production system

More rAAV vector genome per cell produce
more rAAV vectors

Deploy gene amplification systems (DHFR/GS
Genes) to achieve high copy number

Knock out DHFR and GS gene
Knock in rAAV Genome (rAAV hFix)
Select rAAV positive stable clones (MTX/MST)
Gene amplification
rAAV Production

FIG. 2

Packaging cell line based production system

- A novel parental cell lines (DHFR^{-/-}; GS^{-/-}) created.
- Clones containing multi-thousand rAAV-genomes isolated.
- rAAV productivity is comparable to current RB productivity
- Cell clones are stable on vector production

