



Chromosome therapy: rescue of ring chromosomes by cellular reprogramming



Approximately 1 in 500 newborn infants are born with chromosomal abnormalities that include trisomies, translocations, large deletions and duplications. There is currently no therapeutic approach for correcting such chromosomal aberrations in vivo or in vitro. Surprisingly, when we attempted to produce induced pluripotent stem cell (iPSC) models from patients that contained ring chromosomes, the ring chromosomes were eliminated and replaced by a duplicated normal copy of the aberrant chromosome (Bershteyn et al. 2014, Nature 506:99). This finding suggested a potential therapeutic strategy to correct large-scale chromosomal aberrations. We hypothesized that a chromosome with a large aberration could be corrected by producing a ring chromosome from the aberrant chromosome in iPSCs, which would then be eliminated and replaced by a normal chromosome. We are currently testing this hypothesis. If successful, we will have a generalizable system of "chromosome therapy" for the correction of large chromosomal aberrations by the induction of ring chromosomes through genome editing followed by duplication of the normal chromosome.

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