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Human artificial chromosome (HAC) vector with a conditional centromere for correction of genetic deficiencies in human cells

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Human artificial chromosome HAC-based vectors offer a promising system for delivery and expression of full-length human genes of any size. HACs avoid the limited cloning capacity, lack of copy number control and insertional mutagenesis due to integration into host chromosomes that plague viral vectors. We previously described a synthetic HAC that can be easily eliminated from cell populations by inactivation of its conditional kinetochore. This HAC has a unique gene acceptor site. Here, we demonstrate the utility of the synthetic HAC for delivery of full-length genes and correction of genetic deficiencies in human cells. We also show that phenotypes arising from stable gene expression from the HAC can be reversed when cells are "cured" of the HAC by inactivating its kinetochore in proliferating cell populations. Our results demonstrate the benefit of the combining TAR gene cloning technology with the HAC system for gene transfer and expression studies.