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SMaRT correction

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A technique called SMaRT (spliceosome-mediated RNA *Trans*-splicing) has been developed to generate functionally corrected mRNA transcripts and proteins in patients with genetic diseases. In the January issue of Nature Biotechnology, Liu *et al.* describe the use of SMaRT technology to correct endogenous ΔF508 mutations in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene (*Nature Biotechnology* 2002, **20:**47-52). They constructed recombinant adenoviral vectors containing CFTR exons 10-24 and a *trans*-splicing domain. These could partially restore CFTR chloride ion conductance in ΔF508 airway epithelial cells *in vitro*. The SMaRT vectors resulted in the expression of corrected CFTR mRNA and protein. Liu *et al.* also demonstrated functional correction *in vivo* using a human bronchial xenograft model. These results show the potential feasibility of using SMaRT technology to correct, rather than replace, defective genes.

References

1. Spliceosome-mediated RNA trans-splicing as a tool for gene therapy.

2. *Nature Biotechnology*, [http://biotech.nature.com]