Gene therapy revised: SMaRT

The term SMaRT™ stands for "Spliceosome-Mediated RNA Trans-splicing." This technique targets and repairs the messenger RNA (mRNA) transcripts copied from the mutated gene. SMaRT™ works as follows:

A complementary RNA strand is generated that pairs specifically with the intron next to the mutated segment of mRNA. The RNA strand also contains the corrected version of the mutated sequence. When the RNA strand binds to the mRNA, it prevents the spliceosome from including the mutated segment in the final, spliced RNA product. Since the correct exon is now present, the repaired RNA will now be made into a functional protein (see figure below).

Questions:
1. Does this technique get rid of the mutated copy of the gene?
2. Does this technique repair the mutated gene? Why or why not?
3. Is this technique likely to have a permanent effect on the patient, or will it have to be repeated?
4. Why might this technique be better than some of the other gene therapy approaches?