Research provides clues to improving safety of gene therapy

National Institutes of Health researchers have uncovered a key factor in understanding the elevated cancer risk associated with gene therapy.

Toxic side effects actually are rarely observed by researchers who have designed gene therapies using an adeno-associated virus (AAV) as a vector to deliver the corrected gene to a specific point in the cell's DNA.

But one prior study did find an association between AAV and the occurrence of liver cancer.

In many mice that developed liver cancer, the AAV vector targeted a region of the mouse genome called Rian, near a gene called Mir341 that codes for a microRNA molecule. MicroRNAs are small, non-coding RNA molecules involved in the regulation of gene expression. When the AAV was inserted near Mir341, the vector caused elevated expression of the gene, which the researchers believe contributed to the occurrence of liver cancer in the mice. The authors note that Mir341 is found in the mouse genome, however, it is not present in humans.

When the researchers used an alternate AAV vector to deliver the corrected gene in a study of just 10 mice, that vector did not insert where it would elevate the expression of nearby genes and it did not cause liver cancer. The researchers found that this modification was a safer gene therapy.

Read full, original article: NIH researchers tackle thorny side of gene therapy