NIP AND TUCK... EMBRACE THE FUTURE

EDITORIAL

I wish to start 2018 by discussing gene editing which, following last year's remarkable advances, is rumoured to continue to make a star appearance on stage. Gene editing basically rewrites DNA, disabling target genes, correcting harmful mutations and changing the activity of specific genes.

This technology has already been used successfully in the agricultural industry, in that gene editing is faster and more precise than conventional genetic modification, with the added advantage of avoiding the addition of genes from other organisms [this has fuelled the backlash against GM crops]. Seedless tomatoes and gluten-free wheat are some of the results of gene editing.

However, this editorial will discuss the notion of gene editing within the realm of medicine. We have already read about its relation to cystic fibrosis and sickle cell anaemia. However, gene editing has also been recently used to treat infant acute lymphoblastic leukaemia[1] as well as to increase resistance to HIV infection.[2] The cornerstone for this technology is Crispr-Cas9. Crispr stands for '*Clustered Regularly Interspaced Short Palindromic Repeats*' and Cas9 stands for '*Crispr-associated protein-9 nuclease*'. As you may well have envisaged, CRISPR-Cas9 consists of two key molecules. The first component is the Cas9 enzyme. In simple terms, this acts as a pair of 'molecular scissors' that cuts the two strands of DNA at a specific location in the genome so that specific sections of DNA can then be added or removed. The second component is gRNA [guide RNA] which consists of a small piece of pre-designed RNA sequence [approximately 20 bases long] located within a longer RNA scaffold. The scaffold part binds to DNA and the pre-designed sequence 'guides' Cas9 to the right part of the genome. This esnures that the Cas9 enzyme cuts at the right point in the genome. Different enzymes can also be used instead of Cas9, such as Crispr-Cpf1 [*Clustered Regularly Interspaced Short Palindromic Repeats from Prevotella and Francisella 1*].

A question logically crops us … how do the gene editing molecules effectively arrive at the site of action? There are three main methods, viral carriers [lentiviral, adenoviral and adenovirus-associated viral vectors], non-viral carriers [cationic lipid-based vectors] and physical method [electroporation]. These words may seem extracted from a Star Trek episode. However, interestingly, only last year scientists published the results of a proof-of-principle experiment showing that gene-editing [with viral carriers] can effectively be used to prevent muscle wasting in Duchenne muscular dystrophy.[3] Electroporation has, on the other hand, been used to produce micro-holes in embryos to deliver such gene editing molecules ...

More in the next issue…

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