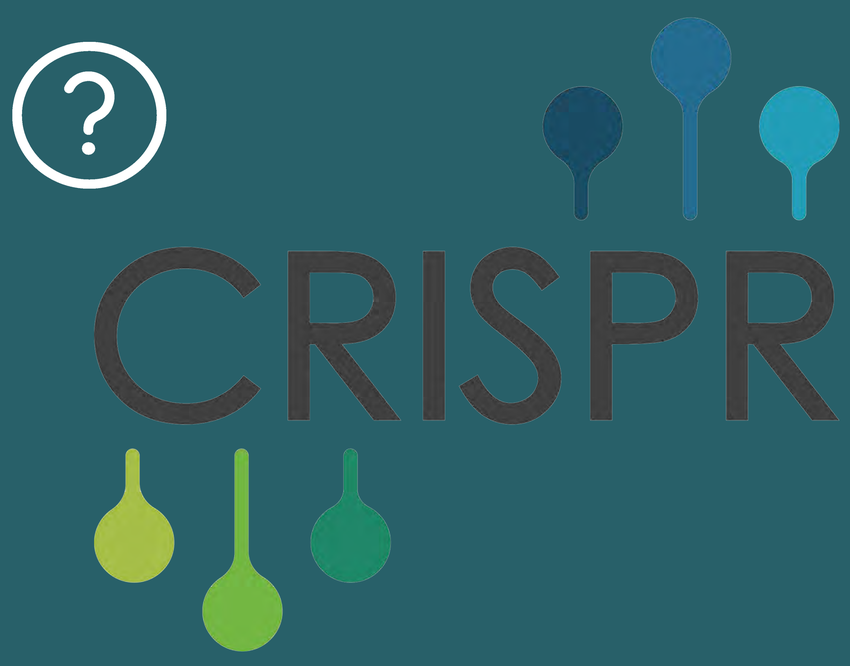
POSSIBILITIES OF CRISPR



"CRISPR": clusters of regularly interspaced short palindromic repeats. A special region of DNA with two distinct characteristics: the presence of nucleotide repeats and spacers.

CRISPR-Cas9 was adapted from CRISPR technologies in bacteria; via the natural defense mechanisms of bacteria and archaea.

Here bacteria capture invading virus through DNA snippets or CRISPR arrays. These are then made to memorize invading viruses for the bacteria. If these viruses come back the bacteria will use Cas9 or something similar to snip the DNA, disabling the virus.

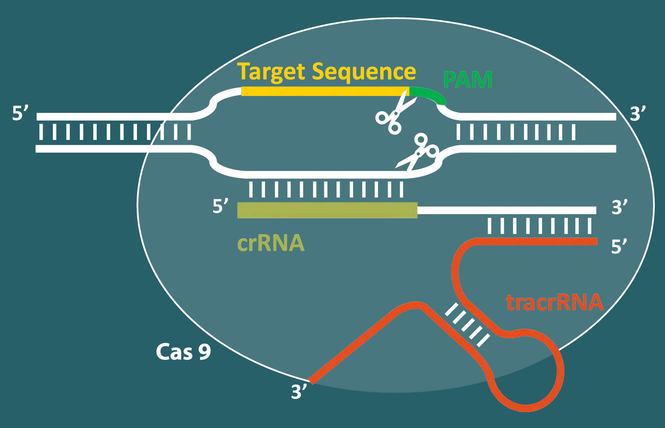
Vidyasagar, A (2018, April 21). What Is CRISPR? Retrieved April 2, 2020 from https://www.livescience.com/58790-crispr-explained.html

RESEARCH

GENOME EDITING

A group of technologies that allow genetic material to be

at specific locations in the genome, changing the organism's DNA



1. Create a small segment of RNA containing a short guiding sequence that binds to a targeted sequence of genome DNA, Cas9 enzyme.
2. Modified RNA is used for recognizing the new DNA sequence; cutting off the rest of the DNA.
3. The new DNA used to study the cell's repair machinery by add/delete/change the genetic material; making custom DNA sequences.

Testing

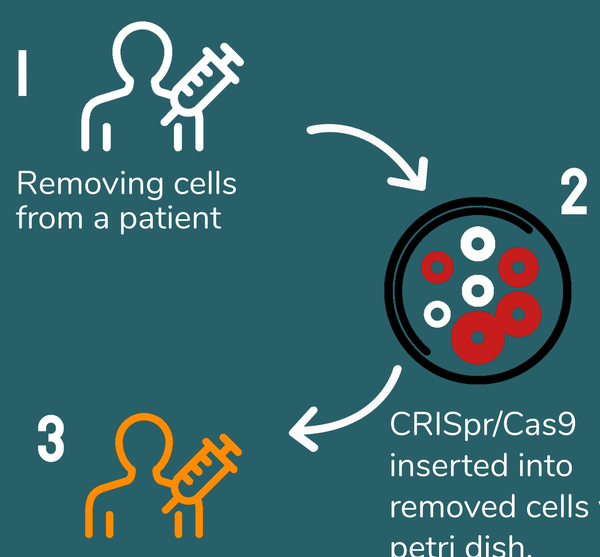
Safety

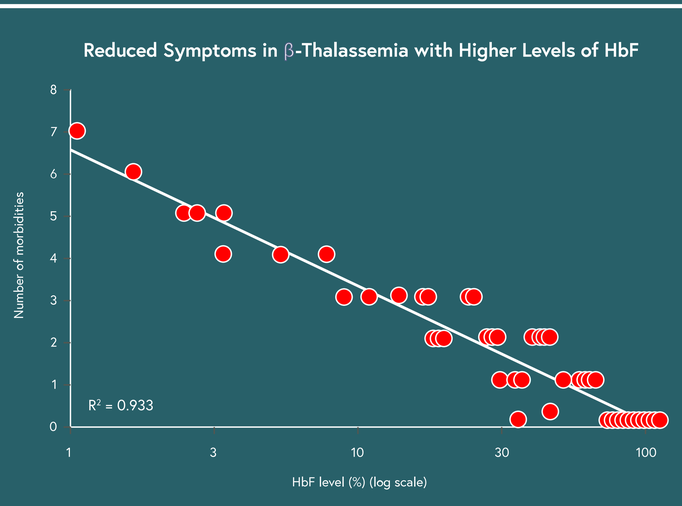
Research on Animal/Cell Models

Exploration in the prevention, treatment of human diseases; like cystic fibrosis or sickle cell.

Holds promise to treating/preventing complex diseases like HIV or cancer.

Ex Vivo





Studying the treatment β-thalassemia, SCD by creating them to increase levels of fetal hemoglobin (HbF), a naturally-occuring form of hemoglobin present in all people before birth.

HbF can substitute for the diseased hemoglogin in β-thalassemia and SCD patients, reducing or eliminating symptoms.

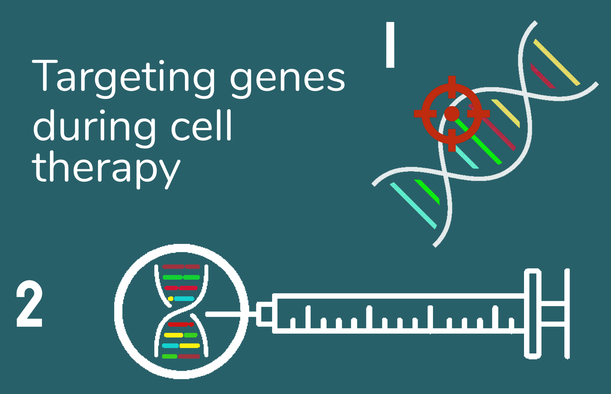
Cell therapy with CTX001 is isoloating a patient's blood stem cells, editing them with CRISPR/Cas9 to increase HbF expression, then returning the edited cells to the patient. Over time the belief is these editied blood

stem cells will generate red blood cells with increased levels of HbF, which may reduce or eliminate patient's symptoms.

VERTEX

2017 signed an agreement to co-develop, commercialize the program

IN VINO



Non-Viral: Focused on LPNs targeting the liver. Encapsualte messanger RNA enconding Cas9, guife RNA, a donor DNA template, into LPNs to train into the iver.

Viral: Focused on other organ systems using AAV vectors. They can deliver DNA encoding for Cas9/guide RNA into specifc body tissues.

For LNP Technology

CUREVAC the RNA people®

For mRNA to support liver targeted research

stridebio

Aiming to engineer novel AAV vectors that target separate tissue types