

Check your understanding



1 Why did we use nanoparticles?

Answer

First, it solves the delivery problem for peptides and nucleic acids! The donor DNA and PNA would get rapidly broken down or eliminated before it reached the site it needs to get to. In addition, it is a low-risk procedure. We can inject them into the developing circulatory system of the fetus through the placenta. This reduces the odds of unintentionally aborting the pregnancy.

2 What could go wrong if our editor molecule wasn't specific enough and edited other spots in the genome?

Answer

That mouse could then have two genetic diseases rather than just one! Once the DNA is edited, there is no "undo" button.

3 Look at Figure 2. Why do you think we checked twice, even though we got the results we wanted at 6 weeks?

Answer

We want to know that our edited cells keep making hemoglobin. If we harmed the cells in some way when we edited them, they might stop growing. We want a permanent cure!

4 Our method could theoretically be used for any disorder caused by one bad gene. Can you think of a disease where our editing method would not be helpful?

Answer

Diseases that aren't caused by a genetic disorder! That includes any illness that a vaccine prevents (flu, measles, tuberculosis, etc.) as well as acute conditions like hypothermia or broken bones. Any disease where 100% correction of the cells with the mutated gene is necessary to restore health. For example, cancer: if we only un-mutate 99% of the cancer cells, that patient still has cancer!
Any disease in cells that aren't dividing. If there isn't a reason for the cell to make much of the DNA repair enzyme, there isn't going to be much of it around to do the editing for us.

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5 Why did we use biodegradable nanoparticles?

Answer Just like in nature, all things brought into a body must be taken back out of it. Otherwise, we leave "molecular trash!" By using a biodegradable material our nanoparticles broke down to smaller molecules the body could use - the molecular equivalent of compost.

6 Some people consider genetic editing unethical. All powerful tools can be used for good or for bad. If we can genetically edit away a disease, do you think we should?

Answer This is a pretty open-ended question. There's so much debate out there about genetic editing, especially on human beings.
Pros: ending needless suffering, helping kids grow up happily healthy, reducing medical costs over the lifetime of the child, etc.
Cons: any error meaning the child's death or a second new permanent mutation, the method possibly being available only to the wealthy rather than those who need it most, the technique being used to try to enhance a baby ("designer babies," "super babies") who do not have any genetic diseases, etc.