



1. How does gene therapy differ from other types of treatments?
2. Why did it take Corey's parents, and the other parents in the book, months to realize that their children had medical problems?
3. What can a parent do when she knows that something is wrong with her child, but health care professionals do not take her seriously?
4. Imagine your young child is diagnosed with a rare inherited disease. List ways to learn about the disease, find clinical trials, and raise funds.
5. What factors would you consider in deciding whether your very young child should have an experimental treatment?
6. Informed consent guidelines from the Food and Drug Administration (FDA) state that age seven is old enough for a child to give consent to undergo an experimental procedure. Do you think that this age is an appropriate cut-off? Why or why not?
7. Paul Gelsinger, whose eighteen-year-old son Jesse died in a gene therapy experiment (Chapter 4-6), sent the author this e-mail after reading *The Forever Fix*:

I am glad that gene therapy is making headway. I have always felt (maybe even known) that it was a viable technology. I hope that it pans out as you hope, but please, oh please, don't make the same mistake I did and believe everything you hear or see. I am thankful that there is a great deal more honesty and oversight in the field now as a result of what happened to Jesse. . . . I guess that's his real legacy. Feel free to tell anyone that I am glad there have been some breakthroughs and that I hope many more follow.

Would you be able to be as forgiving as Paul Gelsinger? What did he mean by the mistake of believing everything? That is, which particular facts were misconstrued or miscommunicated in Jesse's sad case?

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8. Lori Sames, Hannah's mom, is reaching out to all the families she can find whose children have giant axonal neuropathy (GAN). Participation of families is important in research to develop tests and treatments, but some families prefer to keep to themselves. To what extent should an activist parent like Lori try to convince hesitant families to join the group seeking a treatment?
9. How can social media help families with rare diseases?
10. What complementary therapies might ease the daily lives of children with certain inherited conditions? How can complementary therapies help or hinder the progress of FDA-sanctioned clinical trials?
11. Gene therapy for HIV/AIDS is safe and appears to be working, after nearly a dozen years in three clinical trials. Yet drugs (anti-retrovirals) have been very successful in controlling the infection. What are the pros and cons of a "forever fix" for HIV/AIDS? How does the situation for HIV/AIDS differ from that of a rare disease such as giant axonal neuropathy?
12. What criteria should researchers use to select diseases to approach with gene therapy? Knowledge of mechanism? Prevalence? Whether a famous person has it? How involved the parents are? Possibility of translation to more common conditions?
13. Why were the first gene therapies done on very rare diseases?
14. Did reading the book alter your view of the use of animals in biomedical research?
15. Why won't the gene therapies described in the book be passed to the children of the treated patients?
16. Which do you think is more dangerous to use in a gene therapy trial to deliver the genes, adenovirus or HIV? Why?
17. Under what circumstances would you have or allow your child to have gene therapy?

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