

Can a Genetic Disease Be Cured? A Mother's Dilemma

by

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It had been a particularly troublesome day for Kim. Her older son, Johnny, nearly fell off the toilet earlier in the day. Fortunately, Kim had heard the telltale sounds of him struggling to keep his balance and had rushed to the bathroom just in time to avert a disaster.

Twelve-year-old Johnny was home from school because of a slight cold, and he had probably passed the virus on to her younger son, Mark. As an ex-nurse married to a physician, Kim knew that this was a mere nuisance for normal children. But her kids weren't normal.

The phone call confirmed her suspicion. Around noon Mrs. Fletcher, her younger son's teacher, had called from school to tell Kim that Mark was coming down with a hacking cough and should be taken home. Mrs. Fletcher had taught for many years at a special school for handicapped children and had a special sensitivity to the needs of her students as well as their parents. Rather than have Mark come home at four in an adapted wheelchair van, Kim made plans with Mrs. Fletcher to pick him up at one o'clock.

"In the winter months, children always seem to come down with one cold after another. It's part of growing up," thought Kim as she automatically began to prepare to get Mark. "But my two kids have muscular dystrophy. A simple cold could very well prove fatal!"

Kim began to get a bit worked up as she thought more about the phone call from Mrs. Fletcher, who as a rule rarely called home unless it was really necessary. Kim wondered how bad the cough was. In spite of Kim's preoccupation with Mark, she immediately telephoned a neighbor to look in on Johnny while she was out. Seconds later she called her husband at his office.

Todd greeted his wife Kim on the phone and talked while chewing his sandwich. "Kim, did you read the research articles I left on the kitchen table this morning? One of the articles reviews Dr. Hidi's work where he injected normal myoblasts into muscles of mice weakened by genetic nerve degeneration. There's some good news and some bad news. The good news is that he found that the treatment was safe, and it could prolong the running ability of the mice when placed in a treadmill. But here's the bad news: At a recent scientific meeting there were a number of very vocal critics of his research."

Shuffling through copies of the same articles on his desk, Todd continued, "Kim, one thing the scientists were arguing about is that Dr. Hidi's mice were not a model of inherited muscular dystrophy but nerve degeneration. Apparently, these mice do not have the same disorder as Johnny and Mark. Furthermore, at the same meeting, there was a big discussion as to whether or not the body's immune system would reject

the injected myoblasts. I don't know if it's professional envy or a debate over what is good science, but Dr. Hidi certainly seems to be on the hot seat!"

Somewhat distracted, Kim responded, "Er ... no, I haven't had a chance to look at the papers yet. Things have been a little hectic around here. Mark's teacher just called and said he appears to be coming down with a cold. I think that tickle he felt in his throat this morning was more than just a tickle. I'm going to pick him up in a few minutes."

"Anyone there at the school to give you a hand with Mark?" asked Todd.

Kim sighed, "No, but I'll be okay. My back isn't hurting today. I'll be careful and take my time getting Mark into the car. I just wish it wasn't snowing right now, that's all."

Todd spoke quietly and with as much support as he could muster. "Well, please take it slow and easy. Okay?" After a moment, he added, "Kim, what do you want to do with Dr. Hidi? They want a decision on the boys real soon."

Feeling somewhat pressured by the question, Kim answered, "Todd, I just don't know. Maybe next year ... maybe just Johnny. I just don't know. At first the treatment looked like a good idea ... then all this stuff in the papers about Dr. Hidi. He isn't even a medical doctor. I just don't know. I can't talk about it now. I've got to get Johnny into bed and pick up Mark. We can talk about it tonight. Bye."

Ten minutes later, with Johnny secure in bed, Kim was on her way through the kitchen with coat and overshoes on. The phone began to ring as she reached the garage door. "Please, not now," Kim muttered out loud as she spun around to answer the phone. "Hello?"

"Mrs. Davis? Hi, this is Henrietta Conklin calling to find out whether you've made a decision about the boys joining the treatment group. I'm sorry to call you at home like this, but as I mentioned in my letter, we need to know by today because of all the necessary preparations." Henrietta could sense that this was not a good time to call.

Kim answered, "Err ... no ... we haven't decided yet."

With understanding that comes from years of caring for a seriously disabled child herself, and yet with a need to move things along, Henrietta continued, "Listen, as you know, we're all in this boat together. We all love our sons, but this may be our only chance at some real treatment. Listen, Mrs. Davis, sleep on it, talk to your husband and let me know one way or the other tomorrow."

As the gravity and urgency of the impending decision began to sink in, Kim asked, "What's this I recently read about Dr. Hidi leaving the university and moving his clinic to an office building? Will there be a problem in getting treatment?"

"Absolutely not," said Henrietta. But she felt at this stage maybe she ought to explain and qualify a few things for Kim. After all, Kim had two sons with the disease, and she had contributed financially to Dr. Hidi's research efforts. Henrietta had just finished up a major letter-writing campaign to solicit financial as well as political support for Dr. Hidi's clinical treatment trials.

Henrietta explained to Kim that Dr. Hidi was the victim of a political plot carried on by a major, privately funded research organization. She reaffirmed that Dr. Hidi was the first scientist in the world to inject healthy muscle cells into the weakened muscles of a mouse with an inherited nerve disorder. The treatment

was found to greatly delay weakness in this particular type of mouse strain. Dr. Hidi was eager to try this mode of therapy on humans with muscular dystrophy. He needed her sons.

Kim knew that this was the routine procedure for the Food and Drug Administration. She had a synopsis of the FDA procedure somewhere on her desk. Dr. Hidi was going by the book. After getting approval from his university's Institutional Review Board, Dr. Hidi and his research collaborators had begun a Phase I human trial in which a small number of healthy volunteers were treated with normal healthy muscle cells. Since Dr. Hidi had seen no serious side-effects from the injections in this Phase I study, he wanted to quickly expand the study to Phase II, when for the first time dystrophic patients could be added to the treatment population.

Henrietta again told Kim that it was for this stage in the protocol that her two sons were needed. But there was more than medicine going on. Politics were involved.

Sensing that Kim was listening intently, Henrietta continued by describing how some of Dr. Hidi's competitors were jealous of his success. Some were trying to block his Phase II trials by openly criticizing his experimentation in the hopes of preventing him from publishing his results or gaining financial support from granting agencies.

"Kim, there is no treatment for our sons," Henrietta said with emphasis. "Dr. Hidi is the only one who has offered us help. We need to stand by him and his courageous work. The only reason Dr. Hidi left the university was that he was unfairly treated. It's all political!"

With her head buzzing, Kim added, "Yes, and at the expense of our children." Kim had to get going and explained to Henrietta her pressing circumstances with Johnny at home and Mark needing to be picked up at school. She promised to phone Henrietta the next day with her answer.

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