

Lesson 12 Science 10

Gene Therapy Worksheet

Read the following article taken from the Prince George Citizen (March 21, 2019) and answer the following questions.

Questions

1. What is the name of the disease that Josh McQuillin suffered from?

2. Due to this disorder, what could Josh eat? Why?

3. What treatment did Josh receive? How was this achieved?

4. Was the treatment successful? How did the doctors know this?

BREAKTHROUGH GENE THERAPY BENEFITS PRINCE GEORGE MAN

97/16 NEWS SERVICE

A big sushi meal would have once made Josh McQuillin gravely ill, but the Prince George man can now gorge on one of his favourite foods worry-free thanks to a breakthrough clinical trial for his rare genetic disorder.

McQuillin was 12 when he was diagnosed with urea cycle disorder, a life-threatening condition that causes ammonia to build up in the body and can put a person in a coma.

He had to strictly limit how much protein he ate and took expensive medication several times a day. He could never be too far from a hospital, which made it hard to travel abroad or join friends backcountry camping.

"Now I can eat as much protein as I want. I'm eating differently, sleeping differently, exercising differently," McQuillin, 30, said during a monitoring appointment at Calgary's Foothills Medical Centre on Thursday.

"I've gained a bit of weight. I've never had to fight weight gain before, which is kind of funny. I've always been underweight my whole entire life."

The genes needed to process ammonia were delivered to McQuillin's liver intravenously. A virus, modified to be harmless, was used as a transmitter. It's believed McQuillin is the first Canadian to receive gene replacement this way.

Only three other people in the world have undergone similar treatment.

McQuillin said he felt the results two weeks after the one-time injection.

Anael Khan with the University of Calgary's Cumming School of Medicine is leading the trial. He also treated McQuillin in Ontario when he first got sick as a boy.

Khan recalled telling McQuillin's parents years ago that he wasn't sure their son would survive.



97/16 news service photo

Josh McQuillin of Prince George undergoes tests at the University of Calgary's clinical trials unit in Calgary on Thursday.

"Since he's had this therapy, his ammonia has not gone high, despite him eating whatever amount of protein he wants. It's a massive change," said Khan. "We're very excited – especially for rare genetic diseases, DNA diseases – that we don't have to tell the parents that stuff anymore."

Khan said the treatment is being studied for other genetic diseases involving the liver such as hemophilia. Alberta Health Services has set aside beds in Foothills hospital's intensive

care unit for clinical trial patients. That's important, because it's often not known whether an experimental treatment will have serious adverse effects, said Christopher Doig, a medical director in intensive care for the agency's Calgary zone.

"They can get it in a very safe way where they can be very closely watched, very closely monitored. At the same time, we're not using resources taking away from other patients."

McQuillin said he's looking forward to going on a road trip in the United Kingdom this spring without having to worry about his medication or whether the nearest hospital can treat his condition. He can also rest easier when on his forestry job, which once required painstaking meal planning for trips into the bush.

"Everything's 100 per cent good to go for now," he said.

"I guess my only concern or fear is they don't know really how long it will last. But it's definitely exciting."