

Scenario 5

Helping boys with muscular dystrophy

Scientists at 'We Will Get You Moving' Inc. (Get Moving Inc.) have recently discovered a treatment that may offer new hope for boys with muscular dystrophy.

Muscular dystrophy makes muscles waste away. This is because the gene for a protein called dystrophin is altered and leads to death of muscle fibres. The gene is on the X chromosome, which means boys are affected. Muscular dystrophy is very serious, with most affected boys requiring wheelchairs by their teenage years and do not usually survive beyond their early twenties. As yet, there is no effective treatment or cure for the disease.

Scientists at Get Moving Inc. have discovered a chemical (UtX) which is usually present only in very low levels in muscle cells. High levels of UtX result in increased activity of the Ut gene. The Ut gene produces a muscle protein called utrophin that is similar in structure and function to dystrophin. Increased utrophin has been shown to compensate for the lack of dystrophin in mice with muscular dystrophy.

Scientists reason that if UtX is supplied to muscle it will improve the health of those with muscular dystrophy and reduce the muscle wasting. Researchers have now submitted an application to their Human Research Ethics Committee and G-TRAP (Gene-Related Therapies Advisory Panel) to conduct clinical trials using UtX on boys with muscular dystrophy. As there is no effective treatment now, they argue that clinical trials should start immediately as this treatment is the only hope for those with muscular dystrophy.

The \$100 million in funding required for these trials will be provided by the pharmaceutical giant, DrugsRU Corporation. They want to develop new technologies that will produce UtX and similar products in large amounts for commercial sale. It is possible that the treatment could also be used on healthy individuals to enhance sporting ability, increase muscle bulk and improve cardiac output. This could have a significant impact on major sporting events, such as the Olympic Games and professional sporting competitions, by increasing the ability of athletes beyond current limits. UtX occurs naturally in the body and is degraded after 28 days, hence, it would be virtually impossible to detect in athletes using it in training programs. Possible side-effects of increased utrophin production in healthy mice have not yet been investigated.

Issue

Should clinical trials of UtX for boys with muscular dystrophy be approved?

Outline your response as a member of the ethics committee reviewing this application?

Questions

Here are some questions for you to consider in responding to the issue.

1. Who is paying for the research? Is there a bias?
2. What are the potential long term effects?
3. Who will benefit?
4. Who could be harmed in the research?
5. Should sporting groups have access to the technology?
6. Who will have access to the new technology?
7. Have you examined the four principles of bioethics?