2021 PSBR High School Essay Contest
Finalist

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Every day, science is rapidly changing to bring about positive changes in the world. Discoveries from nanotechnologies to medical advances are changing the way people live their lives. Medical research and advancements in immunotherapy are curing diseases that people died from in the past. New therapeutics such as antibiotics, vitamins, and prescription drugs are saving lives. These new medicines go through rigorous testing to ensure their ability to work properly. Drug trials are the most important step in making a successful new drug. Mice are usually used in the first step of testing a new drug. This may arise ethical controversy for animal rights activists but when done correctly it is very beneficial for medical research. Mice are critically important for testing prescription drugs that would benefit humans because mice are genetically similar to humans. Mice reproduce quickly and only live for a short period of time so, their full life span can be examined by scientists. Mice and other animals are extremely important in the development of all medicine ("Why Animal Research?"). They played a critical role in the development of Spinraza which is a cure for spinal muscular atrophy (The Jackson Laboratory).

Spinal muscular atrophy (SMA) is a disease caused by a mutation in the SMN1 gene. This gene holds SMN protein which lets nerve cells live. The SMN1 gene also contains a second gene called SMN2. The SMN2 protein is the body's backup if the SMN gene is faulty (Figueiredo). The severity of SMA depends on the number of copies of the SMN2 gene. If the body has two or fewer SMN2 genes, then the disease develops earlier in life and is more severe. If the body contains three or more SMN2 genes with failing SMN1 genes, then the disease will develop later in life and be less severe. If these SMN and SMN2 proteins are not found at all, the SMA patient can become a paraplegic and lose control over all muscles in their body (The Jackson Laboratory). Infants with spinal muscular atrophy have a shortened life span that leads to death as toddlers or young adults. Their death is usually caused by respiratory failure (Center for Drug Evaluation and Research).

Spinal muscular atrophy is an inherited disease. There are four different types of SMA. They are Type 1, Type 2, Type 3, and Type 4. The different levels of SMA vary in degrees of severity from Type 1, which is the deadliest, to Type 4 which is the least invasive. For a long time, there was no cure for SMA until Spinraza was discovered in 2011 (The Jackson Laboratory).

Spinraza is a drug that fixes SMN2's ability to produce a strong SMN protein. It has two chemical modifiers that help to relieve symptoms of the patient and extend their life expectancy (Figueiredo). Spinraza was the first drug approved to be injected into children with SMA. Spinraza went through many animal trials, before being used with humans. If it were not

for animal trials when Spinraza was being developed, then its creation would not have been possible. Mice were used in the trial because pups (baby mice) can be born with SMA. These pups were injected with Spinraza and scientists saw instant improvement. They were able to survive and meet fundamental development goals (The Jackson Laboratory). After these trials were successful, Spinraza went to human trials. The first human trial was a group of 121 infants that received Spinraza. The first trial showed that Spinraza was successful in preventing the progression of SMA. During the trial, it was found that the Spinraza worked more effectively the earlier it was given to the child. Due to the success of this trial, Spinraza was approved to treat SMA in children worldwide (Center for Drug Evaluation and Research).

The development of Spinraza is very close to my heart because my family friends had a child diagnosed with SMA. The mother of the family recently became pregnant with twins. As the twins developed in her womb, the doctors noticed from genetic testing that one of the twins had spinal muscular atrophy. Normally, this child would have had a shortened life span, but thanks to the modern advances in medicine, the child was able to receive a Spinraza injection upon birth. Due to this, the two children have been able to grow up together without any physical limitations. This family was able to avoid the horrific loss of a child due to the development of Spinraza. Thanks to the work of scientists, biomedical research, animal trials, and human trials, this family and my family can say that this child is alive and will never suffer from SMA.

Works Cited

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