Research Assessment #6

Date: November 6, 2020

Subject: Hemophilia Evaluation and Treatment

APA Citation:

Furie, Bruce, Limentani, Steven A., and Rosenfield, Cathy G., "A Practical Guide to the Evaluation and Treatment of Hemophilia" (2017).
https://ashpublications.org/crawlprevention/governor?content=%2fblood%2farticle%2f84%2f1%2f3%2f172499%2fA-practical-guide-to-the-evaluation-and-treatment

Assessment:

Hemophilia is a common disease that many people who have blood related conditions suffer from. Hemophilia occurs when a persons' blood does not have coagulation, or in other words it does not have the necessary proteins needed to clot blood. Whenever there is an area that is bleeding, due to lacerations or head trauma, among other causes, people with hemophilia bleed a lot. Because of this, people with hemophilia can suffer with intensive bleeding if the bleeding is not stopped. Some medical advancements in treatments for hemophilia that have been extremely helpful in recent years are bone marrow transplants (BMT) and transfusion medicine (Bruce, Limentani, Rosenfield).

Another treatment option that was discussed in the article was initial therapy using factor VIII or factor IX (Bruce, Limentani, Rosenfield). I thought that this treatment was especially interesting because these factors that cause hemophilia in the first place is what is being used to directly treat hemophilia. For example, " Hemophilia A is caused by the deficiency of factor VIII, and hemophilia B is caused by the deficiency of factor IX" (Bruce, Limentani, Rosenfield). This treatment is unique in that it straightaway addresses the problem, and it is almost as if the treatment is replenishing the supply of the factors that cause the deficiencies and hemophilia.

Another part of the article talked briefly about inhibitors. I remember learning about angiogenesis inhibitors, which help stop the growth of a tumor because of the inhibitors that stop the blood vessel production and growth. This causes the tumor growth to be suppressed. I thought that this may be similar to how inhibitors work, but in hemophiliacs. However, I surprisingly found that these inhibitors of factor VI11 or factor IX, "Particularly a high-titer inhibitor associated with an anamnestic response, greatly complicate treatment" (Bruce, Limentani, Rosenfield). From this I learned that there is still a long way to go in developing effective treatments that do not interfere with other medical issues at hand.

Next, I learned about the treatment using purified factor IX or FFP, which could help treat patients with hemophilia B. However, the downside to this was that the treatments could cause "myocardial infarctions" (Bruce, Limentani, Rosenfield). At first, I was really astonished to learn that this would interfere with treatment, and even cause "myocardial infarctions" (Bruce, Limentani, Rosenfield), but then I realized that since the purified factor IX has to do with red blood cells, which carry oxygen to different parts of the body, it would actually make sense that myocardial infections could be a side effect of this treatment. In order to prevent this from happening as much as possible, it is of utmost importance that these treatments are administered carefully and accurately.

Lastly, there was a section discussing gene therapy, which was a part of prophylactic therapy which would treat hemophilia. This gene therapy could be used in the future to evaluate a person's necessities based on what their hemophilia is like. Currently, there are tests for evaluating the " factor VIII or factor IX gene as it is introduced via infection with retroviral vectors or transfected with plasmids" (Bruce, Limentani, Rosenfield). Gene therapy also holds the most promise because it is more cost-effective in comparison to other medicines. Hopefully, as this medicine is tested soon, it can get to the people that need it.