Akshaya Saravanan

Karene Goff

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Cancer Treatments in Hematology and Evaluating Which Treatments Are the Best Suited Towards Each Patient

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Introduction

Purpose of Researching Treatments

Research in cancer treatments is vital in providing optimal patient care. As cancer is a very resilient illness, it is important to continue fighting it as much as possible. It is not easy finding new treatments, because often cancer changes in the ways it affects various patients. A common condition in hematology is leukemia. According to page 1 of the article "Methods for determining a prognosis for survival for a patient with leukaemia" there are four main types of leukemia, "Myelogenous and lymphocytic/lymphoblastic leukemia," (Pendino, Grange, Lillehaug, Aloysius, Knappskog, 1). These four different types of leukemias have different effects in patients, including the severity, which could be acute or chronic (Pendino, Grange, Lillehaug, Aloysius, Knappskog, 1). Scientists are working to find drugs for types of cancer. Treatments such as chemotherapy, which is commonly used to treat a lot of types of cancer, may not be the best option for certain patients. (Pendino, Grange, Lillehaug, Aloysius, Knappskog, 1).

People tend to have different reactions to certain types of medicines or therapy, and understanding whether it may or may not affect a certain person is very important. By researching how these treatments affect patients, understanding what kind of treatments a patient could use based on their health concerns, and then providing a cancer treatment plan, we can provide better care for patients.

Treatment Evaluation Plans

Looking at sample patient data is one way that might help determine how certain treatments have affected patients. Then, we can look for similarities between the patient data and

other patients, and see how the similarities might have a correlation with the treatment that was given to these patients.

Research on Hematology Conditions

Hematology is the study of blood-related disorders, including blood cancers. Some conditions in hematology are leukemia, lymphocytopenia, hemolytic anemia, and thrombocytosis. Other blood coagulation, or clotting, disorders include hemophilia A, disseminated intravascular coagulation disorder, thrombocytopenia, Von Willebrand's disease, and hemophilia C (Odle, Coagulation Disorders). Hemophilia is a condition in which blood does not clot properly. Blood clotting is essential because it stops blood from continuously flowing out of a cut or wound. However, people who do not have coagulants which help with clotting tend to have much more blood flow before the blood finally clots. (Furie, Limentani, Rosenfield).

These disorders can be hereditary, acquired, or congenital. They can be caused by a deficiency in a certain factor. For example, in thrombocytopenia, there is a decreased production of platelets (Haggerty, Maureen). Because these disorders are caused by various factors, it may be a better idea to see how they could be prevented in the first place. However, this is not an easy prospect because in these hematologic disorders, especially the ones related to genetics, it could be very hard to prevent a genetic disorder. Now, there are more developments in genetic testing which may help find if a person might have a certain genetic hematologic disorder, but so far there haven't been any technological advancements that have been developed that show any signs of preventing disorders that are hereditary. (Odle, Hemolytic Anemia).

On the other hand, there are treatments for these conditions. Although disorder prevention is another topic, treatment is available for a lot of these hematologic conditions.

Research on Treatments in Hematology

Most treatment options consist of some type of specific drug that is used to alleviate symptoms, or sometimes even boost the immune system. Immunotherapy is a type of therapy that is used for cancer patients along with or in the place of chemotherapy and radiotherapy (Garant). In immunotherapy, patients are usually given a drug that will help boost the immune system, so that it can fight cancer.

Hemophilia and Von Willebrand's disease is treated with a drug called DDAVP, or desmopressin. In Von Willebrand's disease, DDAVP "functions by increasing the amount of factor VIII and vWF in the bloodstream" (Andres, Frey, 1). Cryoprecipitate, which is similar to DDAVP, is a blood product which contains a "concentrated amount of vWF" (Andres, Frey, 1). In considering these treatment options, there are specific patients that these are recommended to, because of some side effects. Aout 80% of people who have type 1 Von Willebrand's disease respond to treatment, and since some people with type 2 Von Willebrand's respond to it, it can be used to treat that as well. Patients with type 2B, however "should not be treated with DDAVP since it can induce dangerous platelet clumping," and people with "Type 3 Von Willebrand's should not be treated with DDAVP since it doesn't increase the level of vWF" (Andres, Frey, 1).

Another treatment that can be used is blood products, such as blood, platelet, or plasma transfusions (Odle, Coagulation Disorders). Any kind of blood product transfusion is a very tricky process because there has to be an exact match that goes with the patient's blood type. Heparin is another treatment that is usually used as a last resort, because of how controversial it has been in treating patients with DIC, or disseminated intravascular coagulation. It can be used

to stop hemorrhaging, which is why it is typically given as a last resort, if no other treatments have helped. In secondary acquired thrombocytopenia, the symptoms are usually treated first. However, corticosteroids or immune globulin can be used to improve platelet function and production (Odle, Coagulation Disorders).

Another treatment that has been used for treating types of cancer but could be useful in treating hematologic cancers and disorders is OMICS medicine. OMICS medicine pertains to biological studies, such as genomics. There is still a lot of research that needs to be done on OMICS medicine, especially in relation to hematologic cancers, but an interesting thing is that it is a personalized type of medicine, because doctors may be able to find out a root cause of a disorder using OMICS technology, which could then be used in finding a treatment for it. An example of using OMICS technology would be in studying genomics, and focusing on the gene factors of a patient. A tumor biomarker test can be developed to find a cancer mutation in cells. This might be especially helpful if a patient's cancer is metastatic, or has spread to other parts of the body (Hayes).

Evaluations of Patient Data

According to Genentech, data from Hemlibra, a treatment in the form an an injection that is used to treat some types of hemophilia, "20/35, or 56% of young adults and adults who took Hemlibra once a week had 0 bleeds," and "21/35, or 60% or people who took Hemlibra once every two weeks had 0 bleeds" (Genentech, 1). This was conducted using an injection that did not have factor VIII inhibitors. Factor VIII inhibitors act similarly to antibodies that help with blood coagulation. There was a Hemlibra injection that had factor VIII inhibitors, and in that trial, "22/35 or 60% of people taking Hemlibra once a week had 0 bleeds" (Genentech, 1).

Another statistic pertaining to children is: "50/65, or 77% of children taking Hemlibra once a week had 0 bleeds" and children are within the age groups of less than 12 years of age, while young adults are 12-18 years, and adults are 18 years or older (Genentech, 1).

Patient Data in Finding Treatment Options

These studies that were conducted are related to using an injection that either contained or did not contain factor VIII inhibitors, along with other factors included in the injection.

Sometimes, hemophilia is directly treated with factor VIII inhibitors to directly aid in coagulation (Furie, Limentani, Rosenfield).. The studies done on Hemlibra show that compared to people who just used factor VIII inhibitors, the people who used Hemlibra were much more likely to be treated successfully. There was a more than 56% success rate in people who used Hemlibra whereas individuals who solely used factor VIII inhibitors had a 0% rate of successful treatment. This indicates that other drugs similar to Hemlibra, which directly treat blood coagulation, may be much more helpful in comparison to a treatment solely with factor VIII inhibitors (Genentech).

One other factor to look at is the dosage and frequency of Hemlibra that people took. In young adults and adults who took Hemlibra with factor VIII inhibitors once a week, 56% had 0 bleeds, while in young adults and adults who took Hemlibra with factor VIII inhibitors once every two weeks, 60% had 0 bleeds (Genentech). This is a small increase in the success rate of Hemlibra, but it indicates that a gap in treatment, such as taking it once every two weeks instead of once every week, may be more effective. More studies could be conducted to see if this hypothesis might be accurate.

In OMICS medicine that has been used to develop precision medicine, there have been some uses in hemato-oncology. On the other hand, it has been more widely used in treating other types of cancers. One way that OMICS medicine is used is in single-gene testing. Single-gene testing is referred to as "using sequencing limited to coding gene mutations" (Epstein, Lin, 1). This is used in two types of gene categories: "cancer-causing oncogenes that cause cell growth and anti-cancer tumor suppressor genes" (Epstein, Lin, 1). Using this helps develop DNA repair genes, which are used to repair the mutations. As stated before, there is much more research that needs to be done in proving the effectiveness of this in people with hematologic cancers. Seeing how it has been used in certain types of cancer in suppressing tumor growth, and finding gene mutations, and because it is a personalized type of medicine, these factors make it a potentially viable option for a lot of people. (Hayes).

From these findings, in patients with hemophilia A, the best recommendations for treatment would be DDAVP in mild cases, and drugs such as and similar to Hemlibra (with or without factor VIII inhibitors, depending on a person's coagulation characteristics), in more severe cases of hemophilia A (Genentech). In people with Von Willebrand's disease, DDAVP may be helpful, as well as potentially using transfusion of VIII factors to replace the VIII factors already present (Odle, Coagulation Disorders). Plasma and cryoprecipitate, a part of plasma that is used because of its blood clotting properties, can also be used to treat Von Willebrand's (Andres, Frey). In DIC, or disseminated intravascular coagulation, blood transfusions of platelets and plasma can be used to increase platelet count. Heparin can also be used as a final resort to stop hemorrhage, if nothing else has worked. (Odle, Coagulation Disorders). In hemophilia C patients, there are two categories: factor XI and factor VII, and between both of those, if factor XI or factor VII cannot be used, plasma transfusions or experimental OMICS medicine could be

helpful (Furie, Limentani, Rosenfield). In some other types of blood disorders, a general treatment that can be used is corticosteroids, which typically help in reducing inflammation, but have been used to increase platelet production (Odle, Coagulation Disorders). Finally experimental OMICS could possibly be used for many of these blood disorders, specifically those that are genetic or hereditary (Hayes).

Works Cited

Andres, Lisa Maria, and Rebecca J. Frey. "Von Willebrand Disease." *The Gale Encyclopedia of Medicine*, edited by Laurie J. Fundukian, 4th ed., vol. 6: T-Z, Organizations, Glossary, Indexes, Gale, 2011, pp. 4616-4620. *Gale eBooks*, https://link.gale.com/apps/doc/CX1919601845/GVRL?u=j043905001&sid=GVRL&xid=3f5 13e66. Accessed 12 Dec. 2020.

Epstein, Lin, Advances in Physiology: "Cancer and the omics revolution" Volume 46, No.4, 2017 Pages 189-193 (2017).

https://www.racgp.org.au/afp/2017/april/cancer-and-the-omics-revolution/

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

Garant, Immunotherapy: "Interview with Dr. Garant" (2019).

https://docs.google.com/document/d/1iiLvLMqIwvs-HhKIFEk_SpT3goceqpjqBnUCdt6J
M7c/edit?usp=sharing

Genentech, Aim for zero: Most people on HEMLIBRA didn't have any bleeds that needed treatment with additional therapy (2020).

https://www.hemlibra.com/patient/about-hemlibra/how-hemlibra-may-help.html?c=hea-1 65155f5f4c&gclid=cc4ec11d34dd1d255e9d9ec9ccbe2c4a&gclsrc=3p.ds

Haggerty, Maureen. "Thrombocytosis." *The Gale Encyclopedia of Medicine*, edited by Jacqueline L. Longe, 6th ed., vol. 8, Gale, 2020, pp. 5110-5112. *Gale In Context: Science*, https://link.gale.com/apps/doc/CX7986601869/GPS?u=j043905001&sid=GPS&xid=2022da 04. Accessed 12 Dec. 2020.

Hayes, D.F. OMICS-based personalized oncology: if it is worth doing, it is worth doing well!. BMC Med 11, 221 (2013).

https://doi.org/10.1186/1741-7015-11-221

- Odle, Teresa. "Coagulation Disorders." *The Gale Encyclopedia of Medicine*, edited by Jacqueline L. Longe, 6th ed., vol. 2, Gale, 2020, pp. 1225-1229. *Gale In Context: Science*, https://link.gale.com/apps/doc/CX7986600438/GPS?u=j043905001&sid=GPS&xid=b9b4ac e4. Accessed 12 Dec. 2020.
- Odle, Teresa. "Hemolytic Anemia." *The Gale Encyclopedia of Medicine*, edited by Jacqueline L. Longe, 6th ed., vol. 4, Gale, 2020, pp. 2422-2423. *Gale In Context: Science*, https://link.gale.com/apps/doc/CX7986600878/GPS?u=j043905001&sid=GPS&xid=cb6a17 4c. Accessed 12 Dec. 2020.

Pendino, Frederic, Grange, Pierre De La, Lillehaug, Johan R., Aloysius, Thomas, Knappskog, Stian. "US20130217590A1 - Methods for Determining a Prognosis for Survival for a Patient with Leukaemia." Google Patents, Google,

https://patents.google.com/patent/US20130217590A1/en