News from ASH: Advances in Bleeding Disorders
ASH Conference Coverage
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Introduction

Andrew Schorr:
Hello. Welcome to our live broadcast direct from the American Society of Hematology meeting. I’m Andrew Schorr. We are in San Francisco. We are in this huge convention center, and this is where hematologists, and oncologists, researchers from around the world get together, and this is where studies, often groundbreaking studies, are presented and sometimes just updates, and sometimes the information that’s helpful to your physician is not just what happens in the main hallway, but it’s when they get together with their colleagues and talk about clinical practice, and the way it can be improved.

We’re going to talk now about bleeding disorders, and we have with us Dr. Craig Kessler, and he is at the Georgetown University Medical Center in Washington D.C. Dr. Kessler thank you for being on Patient Power.

Dr. Kessler:
Thank you very much for the invitation.

Andrew Schorr:
Okay there is a lot of cancer news that comes out here, but also as hematologists get together, you discuss bleeding disorders. Families touched by hemophilia and Von Willebrand, if I get it right, this is a lifelong concern.

Dr. Kessler:
Definitely.

Andrew Schorr:
Is there anything you’ve been discussing that you’d like to update people about now?

Dr. Kessler:
Well I think one of the interesting things about this meeting is that there is a large amount of visibility on some of the new trends and treatment as far as cancers are concerned, but in actuality in parallel at this meeting there has been an explosion of new ideas and new products for the treatment of Von Willebrand disease and hemophilia, and
in fact Von Willebrand disease is the most common inherited bleeding disorder that we know of. It affects up to two percent of the population, and so when you come to a meeting like this sometimes when you are dealing in bleeding and clotting disorders and all of the attention in the lay press is on cancer, people forget that there is a very large population that is affected by these bleeding disorders.

So at this meeting it’s been very exciting because in the area of hemophilia, which is a bleeding disorder which is hereditary, a bleeding disorder primarily in males, but also there are variants of hemophilia that affect women. There are now new approaches to the treatment of this disease.

In the past we have always been concerned about how do you stop the clotting. Now we are looking at how do you use these new products that are coming onto the market to make the quality of life of the patients better and maybe even the survival time of the patients much better, and how do we avoid complications, and how do we treat the complications that occur?

So at this meeting we are now beginning to see quality of life issues come to the forefront in hemophilia. This is a bleeding disorder where patients have spontaneous bleeds. They damage their joints, they can have bleeds into critical areas of the body, and now we’re looking at programs in which there is a prophylaxis treatment regimen which has now been proven in the literature and in clinical trials to prolong the use of the joints, to prevent damage to the joints, to prevent the brain bleeds that occur in the patients. So that I would predict that in the next five years if the insurance system in the United States allows us to implement this into the typical population it would be my hope that you would not even be able to recognize that an individual has hemophilia walking down the street.

Cost Issues for Treatment and Clinical Trials

Andrew Schorr:
Tell us a little more about this prophylaxis. How does it work? Because there may be somebody that says, ‘Oh, I know all about it,’ but there may be other people who say, ‘That doesn’t happen where I am.’

Dr. Kessler:
Well essentially individuals when they are born with these bleeding disorders, they have a deficiency of the clotting factor proteins in their bloodstreams, and up until recently we weren’t really sure what the minimum amount of clotting factor that was absolutely necessary to prevent spontaneous bleeding, that is bleeding without trauma. Now we’ve been able to develop regimens, protocols where we’ve tested and found out that individuals can prevent spontaneous bleeds from occurring typically if their factor-8 levels, the clotting factor-8 or clotting factor-9 levels are low as around three to five percent of normal. Typically we run 50 to 150 percent levels. If you can get down to three to five then you will not have spontaneous bleeds.
So knowing this piece of information now we can treat individuals maybe twice a week or three times a week with clotting factor and maintain that trough level above three to five percent, and they will not have any spontaneous bleeds, and therefore they will preserve joint function and joint structure.

**Andrew Schorr:**
Now you mentioned the insurance companies. We are talking about very expensive conditions, but ones that are all too prevalent, but yet you are developing strategies to help people live better, live longer. So how can you help people with those battles? Where are we now with industry, with medical centers and trying to help the medical directors, where even where people don’t have insurance, so that they can get the care they need and deserve?

**Dr. Kessler:**
Well I think it is a several-pronged answer, and it’s very critical that you ask it.

**Andrew Schorr:**
And you’re in Washington D.C. which is sort of action central for this.

**Dr. Kessler:**
Well it is action central all over the country now, particularly when we have such economic complications in our country. Well I think that all medicine is going to be reimbursed in the future on an evidence-based program. That is if you can show that a little money spent up front actually saves a large amount of money through the years of a patient’s health care then I think that insurance companies and our system of healthcare provided in the government will allow us to use these innovative, but more expensive, upfront treatments.

There is no question that there is an upfront cost, but the long term savings has been proven now, and I think that as these data emerge I think it’s critical that individuals who have these diseases are amenable to participating in clinical trials which will eventually provide the information that the government and insurance companies want to prove that these upfront extra costs will mean savings in the long run.

Unfortunately, as in cancer in the United States and bleeding disorders there is a paltry number of citizens who choose to participate in clinical trials. We really need to turn this around. As you can see at this meeting at ASH most of the large clinical trials that are being reported with great findings have been European because in Europe up to 50 percent of all of their citizens participate in clinical trials; in the United States less than 20 percent.
Andrew Schorr:
I’m going to tell you a little story. So I’m a leukemia survivor, chronic lymphocytic leukemia which has made news here because there have been phase-III European trials. And guess what? It validated the treatment I received in a phase two single center trial eight years ago. So I’ve benefited. So I want people to listen. Look, I got what now they are saying at this meeting in leukemia today’s medicine standard of care worldwide, I got it eight years ago. Now it doesn’t always work out that way. You know it worked out well for me, and you go into it with your eyes open, but the only way we can help Dr. Kessler and his colleagues in bleeding disorders is by participating. So please consider that.

Now you are at a major academic medical center Georgetown. Is this where these trials tend to be, at the major academic medical centers?

Dr. Kessler:
Well there are several networks of clinical trial centers around the country. Most of the more expensive treatments and the difficult to monitor treatments are usually in academic medical centers because the monitoring of the patients, the kinds of tests that are necessary, care of the patient if a complication occurs are all centered in one center.

There are some community based clinical trials as well, but those are mainly not the cutting edge. Those are what we call phase-IV trials. That is usually post licensing by the FDA. Studies in which the FDA is requesting longitudinal surveillance of any population that receives a particular new modality of treatment to make sure that over the long term that there aren’t any side effects that did not show up in the very short term trials in the academic centers.

Andrew Schorr:
Alright. We are going to take a break in a second. If you listen to this as a replay you can listen again and again. In a week or two we’ll add a transcript, and tell people you know who are affected by these not uncommon conditions. We’re going to take a quick break. We will be right back.

New Developments and Research

Andrew Schorr:
And we are back live, and we are visiting with Dr. Craig Kessler who is a hematologist at the Georgetown University Medical Center in Washington D.C. We are talking about bleeding disorders. So Dr. Kessler we’ve talked about insurance, and we talked about prophylaxis. Now I wandered the exhibit hall, and there are some big companies with different, sometimes competing products that are related to helping people with bleeding disorders, and I’m sure there is research going on. So help us understand where we are now with the tools to be used for people with bleeding disorders, and also where research is headed.
Dr. Kessler:
You know at this meeting perhaps what wasn’t presented is even more exciting than what was presented because at the meeting it was announced just the other day that the first patient, the first human patient, with Von Willebrand disease received a new drug of purified Von Willebrand clotting factor which was genetically engineered. This is taking the knowledge from the laboratory to the bedside in a major, major way. This is a very complicated molecule, and only through many years of research have we now been able to get to the point where this product has passed through animal trials, and now the first human individual with Von Willebrand disease, which as I said is the most common bleeding disorder that we know of on an inherited basis.

So now we are looking at a whole new era of how to use genetically engineered products in a disease for which we were always relying on plasma-based products with all of the complications associated with blood transfusions. So this is just an amazing story. It happened after all of the deadlines were due for abstracts, but there is tremendous excitement in this field right now.

In addition there are, in the hemophilia area, genetically engineered proteins which have been tweaked to extend the natural circulating survival of these proteins so that again, in a prophylaxis manner, right now hemophilia patients have to treat themselves two to three times a week with infusions and injections into their veins of these clotting factors. Now we’re seeing that by tweaking the normal human molecule and adding carbohydrates or changing some of the molecular structure you can take this normal protein and have it circulate in the plasma for up to a week so that we are now getting to the point that we will be seeing a dramatic change in the quality of life of our patients.

Andrew Schorr:
Okay. Let’s just paint a picture for just a minute. So whether it’s next year or a meeting five years from now or not even the meeting, somebody living with one of these bleeding disorders, tell me how their life might be different in the therapies they give themselves or have administered, and the way they feel. Just tell me what you can envision based on what you’re seeing.

Dr. Kessler:
Well I think what is going to happen is the following. If you see adult hemophilia patients walking around now, many of them have joint disease. Many of them have had other structural bleeds which have caused them to have pain and damage to those structures throughout life. I would predict that in the next five or so years that all young hemophilia patients when they are born will be placed on some kind of a prophylaxis measure regimen, maybe after their first bleed or so and then they will be on this regimen for life, and with an extended survival time, circulating survival time product, they may only have to inject themselves maybe once a week, whereas now they have to inject three times a week. So that if they are on a prophylaxis program and a young child is going to go play
baseball or play some sport, they have to pre-treat themselves. It may be that we are getting to the point now that their activities of living will be indistinguishable from a normal individual. It’s just going to turn the whole quality of life of our patients into a new realm.

Andrew Schorr:
And for parents of younger children this gives them a great deal of hope.

Dr. Kessler:
Definitely.

Andrew Schorr:
Well I wish you all the best. You and your colleagues working on this and industry partnering with you, and also urge people to consider being in clinical trials so they can; everybody’s part of a community working ahead to make it better.

We’ve been visiting with Dr. Craig Kessler who is a hematologist and a specialist in bleeding disorders at the Georgetown Medical Center in Washington D.C., and I appreciate them and the American Society of Hematology making you available to us. Dr. Kessler all the best.

Dr. Kessler:
Thank you very much.

Andrew Schorr:
Well this is what we do on Patient Power. The replay will be posted very shortly on www.patientpower.info, and we’ll work to have it spread far and wide, and if you have questions you can always send them in or suggest other programs to www.questions@patientpower.info. I’m Andrew Schorr, and as I always like to say knowledge can be the best medicine of all. Thanks for joining us.