Transcript Details
This is a transcript of an educational program. Details about the program and additional media formats for the program are accessible by visiting: https://reachmd.com/programs/clinicians-roundtable/working-towards-implementing-gene-therapy-for-hemophilia/12864/

ReachMD
www.reachmd.com
info@reachmd.com
(866) 423-7849

Working Towards Implementing Gene Therapy for Hemophilia

Announcer:
You're listening to *Clinician's Roundtable* on ReachMD, and this episode is sponsored by CSL Behring Medical Affairs. Before we begin, please note that gene therapy for hemophilia B is currently under clinical investigation and is not approved by any regulatory authority for therapeutic use. And now, here's your host, Dr. Charles Turck.

Dr. Turck:
Welcome to *Clinician's Roundtable* on ReachMD. I'm Dr. Charles Turck, and joining me today to review practical lessons learned from implementing gene therapy for hemophilia are Dr. Steven Pipe and Dr. Giancarlo Castaman. Dr. Pipe is the Laurence A. Boxer Research Professor of Pediatrics and Professor of Pathology at the University of Michigan. Dr. Pipe, welcome to the program.

Dr. Pipe:
Thank you for having me.

Dr. Turck:
And Dr. Giancarlo Castaman is the head of the Center for Bleeding Disorders and Coagulation in the Department of Oncology at Careggi University Hospital in Florence, Italy. Dr. Castaman, thanks for being here today.

Dr. Castaman:
Thank you, too, for inviting me.

Dr. Turck:
Let's begin with some background. Starting with you, Dr. Pipe, can you tell us what led to the need to establish a gene therapy clinic for hemophilia?

Dr. Pipe:
I think it really stemmed from the learnings that we drew from the clinical trial experience of doing gene therapy over several years. We noted that there was really a quite a long process to talk to patients ahead of time and help them understand what the whole process of gene therapy was to make sure that we were bringing patients who were going to be suitable for this platform of therapy. We had to work with our investigational research pharmacies. This was a new platform, a new product. There were some biologic containment issues that we had to deal with at the university level. We were using our clinical research centers, and we also identified that we really needed dedicated research nurses and coordinators to make sure that we did this really well. So we're going to take those learnings from the clinical trials, and we're going to try and implement that into our clinical experience going forward.

Dr. Turck:
And based on your experience, what are some process and site-specific considerations we should keep in mind when one establishes a gene therapy clinic?

Dr. Pipe:
Well I think certainly, this needs to be done in specialized and experienced hemophilia treatment centers. Patient education that's required to bring this to the clinic is already there at the centers. We have lifetime experience and engagement with these patients over a number of years, and they're going to have the confidence to participate in this new platform of therapy based on having that engagement, which the hemophilia treatment centers have been looking after them for a long time. There are going to be some changes because we're going to be switching from our investigational research pharmacies to our clinical pharmacies, so there's new teams that have to learn about how to handle these products, and we're going to be changing to our clinical nurses and our clinical coordinators in

helping shepherd patients all the way through the process for this treatment.

Dr. Turck:
So now that we've heard Dr. Pipe's experiences, let's turn to you, Dr. Castaman, and review key data from medical societies, like the American Thrombosis and Hemostasis Network, the American Society of Hematology, the European Association for Hemophilia and Allied Disorders, and the European Hemophilia Consortium. Dr. Castaman, what do those societies tell us about implementing a gene therapy clinic?

Dr. Castaman:
Well, there is a huge effort by the societies, but also from the patient association, to implement carefully the gene therapy in the clinical practice. So some societies also release a survey to make the clinicians aware about the fact that this kind of treatment could be really available in the future and, from this point of view, they emphasize the fact that there is really a need to have very specialized centers, possibly already having experience in the clinical trial with gene therapy, to set a hub-and-spoke framework to make this novel treatment available.

Dr. Turck:
For those just tuning in, you're listening to *Clinician's Roundtable* on ReachMD. I'm Dr. Charles Turck, and today I'm speaking with Dr. Steven Pipe and Dr. Giancarlo Castaman about what we're learning as we start to implement gene therapy for hemophilia patients. So once a gene therapy clinic is up and running, Dr. Castaman, who makes up the multidisciplinary care team, and how do they divide the responsibilities?

Dr. Castaman:
So I think that there is a key person leading the multidisciplinary team, usually a hematologist or pediatrician, according to the special attention to patients with bleeding disorders. And then usually, there is a possibility to convene and to discuss several cases according to what is the eligible candidates, the limitation, the possible discussion of drawbacks, or possibility to include the patients, and how to follow this patient. This is a very important issue and we should also be aware that this kind of treatment should also consider it by the regulatory authorities in the sense that they should establish infusion centers and hub centers to make this treatment really useful and completely safe also for the patient. This is an important aspect.

Dr. Turck:
And now let's turn our attention to the stakeholders at play here, because as we know, coordination and communication between the physicians, manufacturers, and patients can be complex. If we come back to you, Dr. Pipe, how do we go about improving those interactions?

Dr. Pipe:
Well if you think about it, patients with hemophilia spend a lifetime infusing treatment products to manage their hemophilia. And they have established a significant amount of trust in those products and in the manufacturers who make those products. And at the physician and care coordination level, we have a set of trust between the manufacturers and the patients so that we are prescribing and recommending these therapies. The companies that have been developing gene therapy, in many cases, are new to the field of hemophilia. They're not going to be familiar to the treatment centers or to the patients. And so, we are responsible to build up the trust between all of those stakeholders to communicate all the preclinical and clinical trial work that went into establishing the safety and efficacy of these products, and then ensuring that we are enhancing the trust with the patients in this transformative therapy. The second point I think I would make here is that there's going to be a significant amount of care coordination, from patient selection, walking them all the way through the steps of the gene therapy process, and then following them over time, to ensure good outcomes. And the expertise and that care coordination of the treatment center I think needs to be compensated adequately, and so there's an enhanced relationship we need to establish with the manufacturers and the payers to ensure that that happens.

Dr. Turck:
Before we close, let's look ahead for a moment. Dr. Pipe, how do you think the development and implementation of gene therapy clinics will improve care for patients with hemophilia?

Dr. Pipe:
Well, I've seen this in the clinical trial setting that this really can be truly transformative for patients. To have a single treatment event and then, within a very few weeks, they're no longer requiring regular prophylactic therapy with their previous product, and then to see over time that these patients just no longer really have to think about their hemophilia. We still have questions about the durability of that clinical outcome, but what we've seen in the clinical trial process now over multiple years of follow-up is that this really is truly transformative for the vast majority of patients. And I think that's a really exciting therapy to have really come to our clinics now and give patients an option for something that could really open up new possibilities for how they live their lives in the future.

Dr. Turck:
Same question to you, Dr. Castaman. Moving forward, how do you think gene therapy clinics will impact outcomes for patients with hemophilia?

Dr. Castaman:
Well, I totally agree with Dr. Pipe's statements because it is truly a new era in the field of hemophilia, and how our patients are so impressed about the fact that they do not need any regular infusion and that they are able to live a normal life without being so concerned about the risk of bleeding. So this is incredibly new, and we have to work close on this because some patients have some psychological difficulties in accepting the fact that they are no longer affected by hemophilia, so it is an important aspect that we also have to consider, but from the clinical point of view, this really is so outstanding and amazing to see that the patients do not need that any longer prior treatment with the usual concentrate replacement treatment.

Dr. Turck:
Well, that brings us to the end of today's program, so I'd really like to thank the two of you for sharing the insights you gained while starting up gene therapy clinics for patients with hemophilia. Dr. Pipe, Dr. Castaman, it was great speaking with you both today.

Dr. Pipe:
Thank you, my pleasure.

Dr. Castaman:
Thank you, my pleasure too.

Announcer:
This episode of *Clinician's Roundtable is* sponsored by CSL Behring Medical Affairs. To access this episode and others from this series, visit ReachMD.com/Clinician's Roundtable, where you can Be Part of the Knowledge. Thanks for listening!