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## UC San Diego Health Joins National Clinical Trial on Hemophilia B Gene Therapy

### 69-year-old hemophilia sufferer joins trial after lifetime of spontaneous bleeding

The Hemophilia and Thrombosis Treatment Center at UC San Diego Health has joined a nationwide clinical trial testing a potential gene therapy that may one day provide a better and long-lasting treatment for people with hemophilia B.

The Phase I/II open-label trial is sponsored by Baxalta Incorporated and will assess the safety and optimal dosing level of an investigational blood clotting factor IX gene therapy treatment. The drug, BAX 335, is designed to enable a hemophilia B patient's own liver to achieve stable factor IX activity over an extended period following a single dose.



*People with hemophilia can experience spontaneous internal bleeding, primarily into the joints, which can last for days unless controlled by medication. Pictured: a sufferer with swelling from an internal bleed into the knee. Photo courtesy of C. Campos-MacDonald.*

Hemophilia is a potentially life-threatening bleeding disorder caused by a lack of clotting proteins. Hemophilia comes in two primary types. In hemophilia A, the more common type, people lack clotting factor VIII, and in hemophilia B, clotting factor IX is deficient.

“UC San Diego Health is excited to participate in testing this new therapeutic approach in patients suffering from hemophilia,” said Annette von Drygalski, MD, PharmD, associate clinical professor of medicine at UC San Diego School of Medicine, and director of the Hemophilia and Thrombosis Treatment Center. “Our center is dedicated to improving the lives of patients with both forms of the disease.”

UC San Diego Health currently serves 150 adult hemophilia patients. Another 250 children with hemophilia are treated at Rady Children's Hospital – San Diego. An estimated 400,000 people worldwide have hemophilia, a genetic disorder which strikes almost exclusively in males.

Patients with this disease who have a cut or injury experience excessive bleeding. While bleeding from external cuts can generally be controlled with compresses, the bigger issue for hemophilia sufferers is spontaneous internal bleeding.

The center has one participant involved in the trial, who is also their oldest patient.

“These patients experience sudden and unexplained internal bleeding that can last for days unless controlled with clotting factor preparations,” said von Drygalski. These “bleeds,” as they are known, occur mainly into the knees, ankles and elbows and can cause extreme swelling, pain and damage to joints and internal organs. Worse yet, such bleeds can strike the brain and lead to early death.

“Our participant is approaching 70 and is on crutches with severely debilitating joint disease from multiple bleedings,” said von Drygalski. “He knows he may not personally benefit from the trial but wants to contribute to science and make life better for the young generation to follow.”

With treatment advances, which include several weekly infusions of plasma-derived or recombinant clotting factor concentrates, most hemophilia sufferers can now live normal life spans with less pain and orthopedic damage. Even so, von Drygalski said many sufferers may have limitations as they age. “The infusions control some, but not all of their bleeding,” she explained. “So as they get older, they may have severe joint disease, painful walking or impaired range of motion.”

“When I was a child in the 1940s, we had no medicine for hemophilia,” said Gary O., clinical trial participant. “My parents used cold pressure and ice packs to minimize the trauma,” he said, referring to the swelling and pain from bleeding that could last for several days. “In my early years, I was bedridden 30 to 40 percent of the time with some kind of hemophilia problem.”

Gary said things improved over the years as knowledge of the disease grew and medications became available. He managed to attend college and became a computer consultant. He also survived the early 1980s, when about half the nation’s hemophilia sufferers became infected with HIV through contaminated blood products, and thousands died. Through it all, Gary has maintained his health to the best of his ability and is happy to participate in the trial.

Enrollment is open for patients with severe or moderately severe Hemophilia B, age 18 and older. For more information about the UC San Diego Health treatment center or the trial, call 858-657-6028.

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