Better Health, Brighter Future

August 20, 2020

TAK-754 and TAK-748 Clinical Studies

Dear Sharon Meyers, President & CEO, Hemophilia Federation of America

As one of our partners we work closely with, we are writing today to share an important update with you on Takeda's gene therapy in hematology. Takeda has recently made the decision to suspend screening and further enrollment in the TAK-754 and TAK-748 clinical studies, effective immediately.

As you may know , TAK-754 is an AAV8 gene therapy for the treatment of hemophilia A currently in Phase 1/2 clinical development, while TAK-748 is an AAV8 gene therapy for the treatment of hemophilia B in Phase 1/2 study start-up (no subject dosed as of today). In order to determine the most appropriate way forward for both TAK-754 and TAK-748, we are currently assessing the interim data from the ongoing Phase 1/2 study of TAK-754 in hemophilia A, together with the overall current gene therapy landscape in hemophilia. We will communicate more details on these results to the community in due time once our assessment has been finalized. It is important to note that this decision is not a result of any safety concerns with TAK-754 and TAK-748.

We have informed those investigators who are working directly on these studies. Our priority first and foremost, is to ensure the safety and wellbeing of patients. Those who are enrolled will continue to be under close clinical observation according to study protocol, and those who were planned to be enrolled into these studies will be informed and transitioned smoothly.

We would like to emphasize that we remain committed to advancing gene therapies and very much believe in their potential. Our strategy is focused on delivering novel and transformative medicines to patients, and we will continue to work with our gene therapy platforms with the aim of delivering treatments in the future that can help make a meaningful difference in those living with hemophilia and other rare hematological diseases. We hope to continue the dialogue with your organization to help us inform that innovative focus.

We are proud to be a long-standing leader in bleeding disorders, and of our relationship with the Hemophilia

Federation of America, which is grounded in our common goal to improve standards of care for the bleeding disorders community. Takeda's vision for a bleed-free world continues to fuel our passion each and every day to make a difference in the lives of those affected by bleeding disorders and working together we hope to achieve it.

Thank you for your continued support. If you have any questions, please contact Rodney Dickson, Sr. Director, Therapeutic Policy and Advocacy, Rare Disease, U.S. Public Affairs (Rodney.Dickson@Takeda.com).

Regards,

Cheryl Schwartz
Senior Vice President,
Rare Disease Business Unit