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Published on *Sanofi Genzyme News* (https://news.sanofigenzyme.com) on 7/7/19 8:00 am EDT

Positive Data from Completed Phase 1/2a study of BIVV001 Presented at ISTH 2019

Release Date:
Sunday, July 7, 2019 8:00 am EDT

Terms:

Dateline City:
Cambridge, MA

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Positive data from the completed Phase 1/2a EXTEN-A study of BIVV001 (rFVIIIFc-VWF-XTEN) showed a single 65 IU/kg dose of BIVV001 achieved average factor activity levels of 17% at seven days post infusion and significantly extended the half-life of factor VIII to 43 hours. These data were presented today at an oral session of the XXVII Congress of the International Society on Thrombosis and Haemostasis (ISTH 2019) taking place in Melbourne, Australia.

"These data reinforce the potential for BIVV001 to address an area of significant unmet need for people with hemophilia A; optimal protection from bleeds with longer prophylactic dosing intervals," said Mouhamed Gueye, PharmD, MBA, Head of Global Medical Affairs, Rare Blood Disorders, Sanofi Genzyme. "Factor replacement therapy is a foundational component of hemophilia care and these early clinical results support the continued development of BIVV001."

BIVV001 is the first and only investigational von Willebrand (VWF)-independent factor VIII therapy that is designed to extend protection from bleeds with prophylaxis dosing of once weekly for people with hemophilia A. Data presented today from the completed Phase 1/2a EXTEN-A trial show that a single dose of BIVV001 resulted in an extended half-life 3- to 4-fold higher than conventional recombinant FVIII therapy (rFVIII) with sustained high factor activity levels.

Factor activity levels refer to the amount of factor VIII in a person's blood, and they determine the severity of a person's disease. Participants in the EXTEN-A trial have severe hemophilia A (factor levels of <1%). Moderate hemophilia A is characterized by factor levels of 1-5%, and mild hemophilia A is from 6-49%.[i]

EXTEN-A is an open-label, multicenter study that evaluated the safety, tolerability and pharmacokinetics of BIVV001 in both a 25 IU/kg dose and 65 IU/kg dose cohort of subjects aged 18-65 years with severe hemophilia A. In the trial, subjects received a single dose of rFVIII followed, after a washout period, by either a single 25 IU/kg or 65 IU/kg dose of BIVV001. Primary endpoints include occurrence of adverse events and development of inhibitors. Key findings presented at ISTH include:

- In the 65 IU/kg dose (n=8) cohort, a single dose of BIVV001 achieved a FVIII half-life of 43 hours, a significant increase from the 13-hour half-life observed with rFVIII in this study.
- Average factor VIII activity for subjects in the 65 IU/kg cohort was 38% at five days, and 17% at seven days post infusion of BIVV001.
- In the 25 IU/kg cohort (n=6), a single dose of BIVV001 achieved a FVIII half-life of 38 hours, significantly higher than the 9-hour half-life observed with rFVIII in this study, with factor activity levels >5% at seven days.
- BIVV001 was generally well tolerated with no development of inhibitors. No adverse events of allergic reaction or anaphylaxis or treatment-related adverse events were reported.

The results of the EXTEN-A study support further development of BIVV001. BIVV001 was recently awarded Orphan Drug status by the European Commission.

About BIVV001

BIVV001 (rFVIIIFc-VWF-XTEN) is a novel and investigational recombinant factor VIII therapy that is designed to extend protection from bleeds with prophylaxis dosing of once weekly for people with hemophilia A. BIVV001 builds on the company's innovative Fc fusion technology by adding a region of von Willebrand factor and XTEN polypeptides to potentially extend its time in circulation. BIVV001 was granted orphan drug designation by the Food and Drug Administration in August 2017 and the European Commission in June 2019. BIVV001 has not been approved by the FDA, EMA or any other regulatory authority for any indication and no conclusions can or should be drawn regarding the safety or effectiveness of this investigational therapeutic.

About Hemophilia A

Hemophilia is a rare, genetic disorder in which the ability of a person's blood to clot is impaired. Hemophilia A occurs in about one in 5,000 male births annually, and more rarely in females. The World Federation of Hemophilia estimates that approximately 158,000 people are currently diagnosed with hemophilia A worldwide[ii].

People with hemophilia A may experience significant bleeding episodes, some of which can be life-threatening. Prophylactic infusions of factor VIII can temporarily replace clotting factors that are needed to help control bleeding and prevent new bleeding episodes.[iii] The World Federation of Hemophilia (WFH) recommends prophylaxis as the optimal therapy to help

prevent bleeding in patients with hemophilia.[iv]

[i] National Hemophilia Foundation, Hemophilia A. Available at https://www.hemophilia.org/Bleeding-Disorders/Types-of-Bleeding-Disorders/Hemophilia-A . Accessed May 17, 2018

[ii] World Federation of Hemophilia, Annual Global Survey 2016, published in June 29, 2019. Available at: http://www.wfh.org/en/data-collection

[iii] World Federation of Hemophilia. About Bleeding Disorders – Frequently Asked Questions. Available at: http://www.wfh.org/en/page.aspx?pid=637#Difference_A_B. Accessed on: June 29, 2019

[iv] Guideline for the management of hemophilia, World Federation of Hemophilia, 2nd edition, http://www1.wfh.org/publications/files/pdf-1472.pdf. Accessed on June 29, 2019

Language:
English
