Session: Novel hemophilia treatment, clinical trials I

OC 40.1 - A Novel Extended half-life factor VIII Fc fusion protein FRSW107 for severe hemophilia A: A multicentre, open-label, single-arm, phase 3 study and its open-label extension

Monday, June 24, 2024 14:45 – 15:00 ICT Room: Plenary Hall

Presenting Author(s)

Feng Xue, MD

Chief physician
State Key Laboratory of Experimental Hematology, National Clinical Research Center for Blood Diseases, Haihe Laboratory of Cell Ecosystem, Institute of Hematology & Blood Diseases Hospital, Chinese Academy of Medical Sciences & Peking Union Medical College, Tianjin Key Laboratory of Gene Therapy for Blood Diseases, CAMS Key Laboratory of Gene Therapy for Blood Diseases, Tianjin 300020, China
Tianjin, Tianjin, China (People's Republic)

Co-Author(s)

Hu Zhou, MD

Chief Physician
Department of Hematology, The Affiliated Cancer Hospital of Zhengzhou University & Henan Cancer Hospital
Zhengzhou, Henan, China (People's Republic)

YC

Yun Chen

professor
Jinan central hospital, Shandong, P.R. China
Jinan, Shandong, China (People's Republic)

Jing Sun, MD

No. 1838 North Guangzhou Avenue, Guangzhou, China
Department of Hematology, Nanfang Hospital, Southern Medical University
Guangzhou, Guangdong, China (People's Republic)

CJ

Chenghao Jin

professor
Jiangxi Provincial People's Hospital, Jiangxi, P.R. China
Jiangxi, Jiangxi, China (People's Republic)

PL

Pingchong Lei

professor
Henan provincial People's Hospital, Henan, P.R. China
zhenzhou, Henan, China (People's Republic)

ZL

Zhenyu Li

professor
The Affiliated Hospital of Xuzhou Medical College, Jiangsu, P.R. China
Xuzhou, Jiangsu, China (People's Republic)

YX

Yaming Xi

professor
The First Hospital of Lanzhou University, Gansu, P.R. China
Lanzhou, Gansu, China (People's Republic)

XZ

Xiaojing Zeng

professor
The Affiliated Hospital of Guizhou Medical University, Guizhou, P.R. China
Guizhou, Jiangxi, China (People's Republic)

ZC

Zhongguang Cui

professor
The Affiliated Hospital of Qingdao University, Shandong, P.R. China
Qingdao, Shandong, China (People's Republic)

XZ

Xielan Zhao

professor
Xiangya Hospital of Central South University, Hunan, P.R. China
changsha, Hunan, China (People's Republic)

LY

Linhua Yang

professor
The Second Hospital of Shanxi Medical University, Shanxi, P.R. China
Shanxi, Shanxi, China (People's Republic)

YY

Yi Yang

professor
Chongqing University Three Gorges Hospital, Chongqing, P.R. China
Chongqing, Chongqing, China (People's Republic)

BT

Baolin Tang

Doctor
Anhui Provincial Hospital, Anhui, P.R. China
Hefei, Anhui, China (People's Republic)

AS

Aizong Shen

professor
Anhui Provincial Hospital, Anhui, P.R. China
Hefei, Anhui, China (People's Republic)

Runhui Wu, PhD, MD

Professor
Department of Hematology, Beijing Children's Hospital, Capital Medical University, National Center for Children's Health, Beijing, 100045, China
Beijing, Beijing, China (People's Republic)

XW

Xiaolin Wang

professor
Capital Medical University affiliated Beijing Children's Hospital, Beijing, P.R. China
Beijing, Beijing, China (People's Republic)

Ling Liu

LL
professor
The Second Affiliated Hospital of Guangzhou Medical University, Guangzhou, P.R. China
Guangzhou, Guangdong, China (People's Republic)

JI
Jiwei Iou
professor
Gensciences Inc.,Nantong, P.R. China
Nantong, Jiangsu, China (People's Republic)

Renchi Yang, MD
Head of Thrombosis and Hemostasis Center
Institute of Hematology and Blood Diseases Hospital, Chinese Academy of Medical Sciences and Peking Union Medical College
Tianjin, Hebei, China (People's Republic)

Background: Hemophilia A is a rare hereditary disease caused by a deficiency of coagulation factor VIII (FVIII).

Aims: This multicentre, open-label, single-arm phase 3 trial and its open-label extension aimed to assess the efficacy and safety of a novel extended half-life factor VIII (FVIII) Fc fusion protein FRSW107 as prophylactic and on-demand treatment for severe hemophilia A.

Methods: Between October 9, 2020 and June 26, 2022, adolescents and adults with severe haemophilia A (FVIII activities < 1 IU/dL) without FVIII inhibitors received intravenously FRSW107 50 IU/kg Q3D for 50 exposure days and at least six months for prophylaxis or FRSW107 30 to 50 IU/kg for six months for on-demand therapy. The primary outcomes were the annualized bleeding rate (ABR), annualized joint bleeding rate (AJBR) and number of target joints.

Results: Eighty-three patients received prophylaxis and 36 received on-demand treatment; 101 entered the extension period. FRSW107 had a mean elimination half-life of 20.1±4.7 h and a mean incremental recovery of 2.1±0.5 IU/dL/IU/kg. By exposure day 100, 53 (63.9%) patients in the prophylaxis group had zero bleed. The mean ABR was 1.5 ± 3.8 events (95% CI, 1.0-2.3), with a 95.3% reduction from baseline ($p < 0.0001$). The mean AJBR was 1.2±3.5 events (95% CI, 0.8-1.9), with a 95.8% reduction from baseline ($p < 0.0001$). The mean number of target joints was 0.1±0.3 (95% CI, 0.0-0.1), representing a 96.9% reduction from baseline ($p < 0.0001$). Treatment-related adverse events occurred in 19 (16.0%) patients but caused no treatment interruption, discontinuation, withdrawal or death.

Conclusion(s): FRSW107 was well tolerated and efficacious in the prophylactic and episodic treatment of bleeding events in previously treated adolescents and adults with severe hemophilia A.