Innovation all for the patients

CHUGAI

Roche A member of the Roche group

Data on the use of Chugai's Hemlibra in Hemophilia A Children with or without Inhibitors were Published in Journals

- Data from a Japanese clinical study HOHOEMI conducted by Chugai with hemophilia A children without inhibitors were published in Haemophilia online
- Data from a global clinical study HAVEN 2 conducted with hemophilia A children with inhibitors were published in Blood online

TOKYO, October 18, 2019 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced that two data on the use of Chugai's hemophilia A treatment Hemlibra® (emicizumab) in children were published in journals.

"Conventional standard treatment for hemophilia A requires intravenous injections multiple times a week, which can be a treatment hurdle especially for children with difficult vascular access. Hemlibra can be administered subcutaneously in a longer dosing interval. We are very pleased that data on the use of Hemlibra in children were published in the international journals," said Chugai's Executive Vice President, Co-Head of Project & Lifecycle Management Unit, Dr. Yasushi Ito. "The HOHOEMI study that we conducted in Japan is the first clinical study which examined the use of Hemlibra in hemophilia A children without inhibitors. We will continue undertaking clinical studies with exceptional science so that patients and healthcare professionals may use Chugai's products with more confidence."

[Overview of publication]

1. HOHOEMI study: a Japanese phase III study conducted by Chugai

- Journal: Haemophilia online (published on September 12, 2019)
- Title: A multicentre, open−label study of emicizumab given every 2 or 4 weeks in children with severe haemophilia A without inhibitors
- URL: https://doi.org/10.1111/hae.13848
- Overview of the study:
  - A Japanese study investigating the efficacy, safety and pharmacokinetics of once every two weeks or every four weeks subcutaneous administration of Hemlibra prophylaxis in children with hemophilia A without inhibitors including infants previously untreated with FVIII therapy.
  - Annualized bleeding rates for treated bleeding events (95% CI) were 1.3 (0.6; 2.9) in those receiving Hemlibra every two weeks (n=6), and 0.7 (0.2; 2.6) in those receiving Hemlibra every four weeks (n=7).
  - The most frequently reported adverse events were contusion (76.9%), nasopharyngitis (38.5%), and excoriation and fall (30.8%).

CHUGAI PHARMACEUTICAL CO., LTD. Corporate Communications Dept.
1-1, Nihonbashi-Muromachi 2-chome, Chuo-ku Tokyo, 103-8324 Japan
Media Relations Group TEL:+81-(0)3-3273-0881 E-mail:pr@chugai-pharm.co.jp
Investor Relations Group TEL:+81-(0)3-3273-0554 E-mail:ir@chugai-pharm.co.jp

2. HAVEN 2 study: a global phase III study

- Journal: Blood online (published on October 10, 2019)
- Title: A multicenter, open-label, phase 3 study of emicizumab prophylaxis in children with hemophilia A with inhibitors
- URL: https://doi.org/10.1182/blood.2019001869
- Overview of the study:
  - A global study evaluating the efficacy, safety and pharmacokinetics of once weekly, every two weeks or every four weeks subcutaneous administration of Hemlibra prophylaxis in children with hemophilia A with factor VIII inhibitors.
  - Annualized bleeding rates for treated bleeding events (95% CI) were 0.3 (0.17; 0.50) in those receiving Hemlibra once weekly (n=65), 0.2 (0.03; 1.72) in those receiving Hemlibra every two weeks (n=10) and 2.2 (0.69; 6.81) in those receiving Hemlibra every four weeks (n=10).
  - The most common adverse events in enrolled 88 children were nasopharyngitis (37.5%) and injection site reactions (29.5%).

###