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%).
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](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%).

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