Hemlibra (emicizumab) from Roche approved for the prophylactic therapy of people with haemophilia A

- The first medication to provide significant reduction of treated bleeding compared to previous factor VIII prophylaxis in intra-patient comparisons
- The only prophylaxis which can be administered subcutaneously and in multiple application forms (weekly, every 2 or 4 weeks)
- Efficacy and safety were demonstrated within the largest clinical study program for haemophilia A

Basel, 28 August 2019 - Roche (SIX: RO, ROG; OTCQX: RHHBY) announced today that the Swiss Agency for Therapeutic Products (Swissmedic) had granted approval for Hemlibra® for the prophylactic therapy of people with severe haemophilia A without factor VIII inhibitors.

Hemlibra can be used in all age groups. It was shown in two of the largest pivotal clinical studies in haemophilia A without factor VIII inhibitors that Hemlibra provides a significant and clinically relevant reduction of the bleeding after once weekly subcutaneous administration in comparison to previous treatment with factor VIII products. This effect was seen in adults, adolescents and children. The subcutaneous therapy with the bispecific antibody emicizumab (Hemlibra) already had marketing authorisation in Switzerland since 6 November 2018 for patients with haemophilia A with so-called inhibitors (antibodies against factor VIII).

Haemophilia is a serious, inherited blood coagulation disorder which can cause uncontrolled and often spontaneous bleeding. Apart from an increased risk from external injuries, people with haemophilia A often suffer spontaneous internal bleeding in the muscles and joints, particularly in the knees, elbows and ankles. The new therapy has the potential to prevent these bleedings.

"The prevention of bleeding is a particular challenge for people with haemophilia A. Previously, they typically needed intravenous injections of products several times a week, which can be especially hard for young children and their families," says Marios Ntinis, Director of the Neurosciences, Autoimmune & Rare Diseases Business Unit of Roche Pharma Schweiz. "The intra-patient comparison showed that Hemlibra reduces the frequency of bleedings in comparison to the products available at the time."

Haemophilia A is caused by a deficiency of factor VIII. This disrupts the coagulation of the blood. Around 600 people are affected in Switzerland. Treatment with Hemlibra differs fundamentally from the previous substitution therapy with factor VIII products. Owing to its long half-life, Hemlibra can be administered at various application intervals (weekly, every two or four weeks). As a monoclonal, bispecific antibody, emicizumab (Hemlibra) mimics the function of factor VIII: it binds to both activated factor IXa and factor X, largely restoring coagulation.
Simpler handling means a better quality of life
Hemlibra provides people with haemophilia A without inhibitors a new treatment option which offers more effective bleeding control and easier handling. As well as reducing bleeding, another advantage is the ease of use: The new active substance is injected subcutaneously into the fat tissues, for example in the thigh or abdomen. Children and adolescents will profit from this in particular, as the current intravenous therapy is often highly stressful for them and their parents. Study results with intra-patient comparisons support the advances which can be made with the new therapeutic approach in the treatment of haemophilia A: In comparison to a therapy provided when required, the weekly prophylactic treatment with Hemlibra reduced the number of bleedings requiring treatment by 96 per cent; twice weekly treatment reduced this by 97 per cent. The number of internal joint haemorrhages requiring treatment was reduced by 95 per cent. A four-weekly therapy also achieved similar good results. Similar significant reductions had initially been demonstrated for patients with inhibitors.


The study data:
On 27 August 2019, Swissmedic approved hemlibra for the prophylactic treatment of people with severe hemophilia A (congenital factor VIII deficiency, FVIII < 1%) without factor VIII inhibitors. Hemlibra has been approved for people with hemophilia A with Factor VIII inhibitors since 6 November 2018. Hemlibra can be used in all age groups.

The results from the HAVEN 3 and HAVEN 4 clinical studies were pivotal for the decision to grant marketing authorisation. In the HAVEN 3 study, adults and adolescents from 12 years of age with haemophilia A without factor VIII inhibitors received Hemlibra prophylaxis weekly or every two weeks. In comparison to patients who received no prophylaxis, the incidence of bleedings requiring treatment over one year (annualised bleeding rate, ABR) was reduced significantly by 96% for weekly administration (rate ratio [RR]=0.04; p<0.0001) or by 97% when administered every two weeks (RR=0.03; p<0.0001). An intra-individual comparison showed the advantage of Hemlibra compared to standard factor VIII prophylaxis: after switching to Hemlibra, study participants had a significantly lower ABR (68% reduction) (RR=0.32; p<0.0001).

In the single-arm Phase III HAVEN 4 study, four-weekly Hemlibra prophylaxis also achieved highly clinically relevant bleeding control in adults and adolescents from 12 years of age with haemophilia A with factor VIII inhibitors (n=5) and without factor VIII inhibitors (n=36). No bleeding occurred during the observation period in 56% of participants (95% CI: 39.7-71.5).

The most frequent adverse reactions occurring in ≥10% of participants in both studies with Hemlibra included injection site reactions, headaches and arthralgia (joint pain).
The bispecific monoclonal antibody was well tolerated in both HAVEN 3 and HAVEN 4. The USA’s health authority (FDA) had already classified Hemlibra as a therapy breakthrough for routine prophylaxis for patients with haemophilia A without inhibitors against factor VIII in May 2018 and granted marketing authorisation on 4 October. In the European Union, Hemlibra has had marketing authorisation since February 2018 for the prophylactic therapy of haemophilia A with inhibitors against factor VIII and since March 2019 also for people with severe haemophilia A without factor VIII inhibitors.

**About Hemlibra (emicizumab)**

Hemlibra is a bispecific factor IXa- and factor X-directed antibody. It is designed to bring together factor IXa and factor X, proteins required to activate the natural coagulation cascade and restore the blood clotting process for people with haemophilia A. Hemlibra is a prophylactic (preventative) treatment that can be administered by an injection of a ready-to-use solution under the skin (subcutaneously) once-weekly, every two weeks or every four weeks. Hemlibra was created by Chugai Pharmaceutical Co., Ltd. and is being co-developed globally by Chugai, Roche and Genentech.

**About Roche**

Roche is a global pioneer in pharmaceuticals and diagnostics focused on advancing science to improve people’s lives. The combined strengths of pharmaceuticals and diagnostics under one roof have made Roche the leader in personalised healthcare – a strategy that aims to fit the right treatment to each patient in the best way possible.

Roche is the world’s largest biotech company, with truly differentiated medicines in oncology, immunology, infectious diseases, ophthalmology and diseases of the central nervous system. Roche is also the world leader in in vitro diagnostics and tissue-based cancer diagnostics, and a frontrunner in diabetes management. Founded in 1896, Roche continues to search for better ways to prevent, diagnose and treat diseases and make a sustainable contribution to society. The company also aims to improve patient access to medical innovations by working with all relevant stakeholders. More than thirty medicines developed by Roche are included in the World Health Organization Model Lists of Essential Medicines, among them life-saving antibiotics, antimalarials and cancer medicines. Moreover, for the tenth consecutive year, Roche has been recognised as the most sustainable company in the Pharmaceuticals Industry by the Dow Jones Sustainability Indices (DJSI).

The Roche Group, headquartered in Basel, Switzerland, is active in over 100 countries and in 2018 employed about 94,000 people worldwide. In 2018, Roche invested CHF 11 billion in R&D and posted sales of CHF 56.8 billion. Genentech, in the United States, is a wholly owned member of the Roche Group. Roche is the majority shareholder in Chugai Pharmaceutical, Japan. For more information, please visit [www.roche.com](http://www.roche.com).

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References

Roche Group Media Relations
Phone: +41 61 688 8888 / e-mail: media.relations@roche.com
- Nicolas Dunant (Head)
- Patrick Barth
- Ulrike Engels-Lange
- Karsten Kleine
- Barbara von Schnurbein