European Commission approves Roche’s MabThera (rituximab) for a rare autoimmune disease

- MabThera is the first biologic treatment approved for moderate to severe cases of the rare autoimmune disease pemphigus vulgaris (PV), and the first major advancement in the treatment of the disease in more than 60 years.
- PV is a rare and potentially life-threatening blistering condition which can cause severe pain and disfigurement.
- MabThera is now approved in Europe to treat four autoimmune diseases.

Basel, 15 March 2019 - Roche (SIX: RO, ROG; OTCQX: RHHBY) today announced that the European Commission has approved MabThera® (rituximab) for the treatment of adults with moderate to severe pemphigus vulgaris (PV), a rare condition characterised by progressive painful blistering of the skin and/or mucous membranes. Extensive blistering can lead to serious, life-threatening fluid loss, infection and/or death.

MabThera is the first biologic therapy approved by the European Commission for PV and the first major advancement in the treatment of the disease in more than 60 years. Following approval by the US Food and Drug Administration (FDA) in June 2018 and today’s decision, MabThera is now approved to treat four autoimmune diseases in the US and Europe.

“We’re pleased to bring the first biologic medicine to the more than 50,000 people in Europe suffering from pemphigus vulgaris,” said Sandra Horning, MD, Roche’s Chief Medical Officer and Head of Global Product Development. “This MabThera approval provides a much needed new treatment that has been shown to provide higher remission rates than corticosteroids alone, which can cause debilitating side effects.”

The European approval is based on data from the phase III Ritux 3 trial, a Roche-supported randomised controlled study, conducted in France, which evaluated MabThera plus a tapering regimen of oral corticosteroids (CS) compared to a standard dose of CS alone, as a first-line treatment in patients with newly diagnosed moderate to severe pemphigus. The primary endpoint of the study was complete remission at month 24 without the use of CS for two or more months. The study demonstrated that 89.5% of people with PV treated with MabThera, in combination with short-term oral CS treatment, achieved complete remission without the use of CS for two or more months, compared to 27.8% of people with PV receiving CS alone, the current standard of care. The results of the Ritux 3 trial were published in The Lancet in March 2017.

The Roche-sponsored phase III multicentre, randomised, double-blind PEMPHIX study, evaluating the efficacy and safety of MabThera compared with mycophenolate mofetil (MMF), an immunosuppressant, in patients with moderate to severe PV, is ongoing.
Recently, an international panel of experts, the International Bullous Disease Group, published new recommendations on the diagnosis and management of pemphigus in the Journal of the American Academy of Dermatology, and recommended the use of an anti-CD20 monoclonal antibody, such as MabThera combined with a tapering regimen of oral CS, as a first-line therapy option for moderate to severe pemphigus.[6]

**About pemphigus vulgaris**
Pemphigus vulgaris is an autoimmune, blistering disease, occurring within the epidermis, affecting the skin and mucous membranes.[1] It is the most common type of a group of autoimmune disorders collectively called pemphigus.[2] It is estimated that around three in every 100,000 people are diagnosed with this disease globally.[7]

**About the Ritux 3 trial**
Ritux 3 is a Roche-supported phase III, prospective, multicentre, parallel-group, open-label randomised trial (NCT00784589), conducted in France by the French Study Group on Autoimmune Bullous Diseases. It was designed to evaluate MabThera plus a tapering regimen of oral corticosteroid (CS) treatment compared to a standard dose of CS monotherapy as a first-line treatment in patients with newly diagnosed moderate to severe pemphigus.[4] The primary endpoint of the study was complete remission at month 24 without the use of corticosteroids for two or more months.

**About the PEMPHIX study**
A phase III, randomised, double-blind, double-dummy, active-comparator, parallel-arm multicentre study (PEMPHIX, NCT02383589), designed to evaluate the efficacy and safety of MabThera compared with mycophenolate mofetil (MMF), an immunosuppressant, in patients with moderate to severe active pemphigus vulgaris requiring 60-120 mg/day oral prednisone (or equivalent).[5]

**About MabThera/Rituxan**
MabThera (Rituxan in the US) in combination with methotrexate is indicated for the treatment of adults with severe active rheumatoid arthritis (RA) who have had an inadequate response or intolerance to other disease-modifying anti-rheumatic drugs (DMARD) including one or more tumour necrosis factor (TNF) inhibitor therapies. MabThera/Rituxan, in combination with glucocorticoids, is indicated for the treatment of adults with severe, active granulomatosis with polyangiitis (Wegener’s, GPA) and microscopic polyangiitis (MPA). People with serious infections should not receive MabThera/Rituxan. It is not known if MabThera/Rituxan is safe or effective in children.

**About Roche in rheumatology and beyond**
For more than 50 years, Roche has followed the science to pioneer medicines for immune-mediated rheumatic diseases. First-in-class anti-IL-6 receptor therapy Actemra/RoActemra (tocilizumab) has treated more than one million people with debilitating conditions, such as rheumatoid arthritis (RA), polyarticular and systemic juvenile idiopathic arthritis, giant cell arteritis and chimeric antigen receptor T-cell-induced cytokine release syndrome. MabThera/Rituxan (rituximab), which targets CD20, has significant clinical and real-world experience treating rheumatic conditions including RA, granulomatosis with polyangiitis (GPA) and microscopic polyangiitis (MPA). Roche aims to provide solutions for people that need new treatments
most, particularly those with severe or life-threatening conditions and limited treatment options. Our pipeline consists of treatments designed to target immune pathways including a glycoengineered type II anti-CD20 antibody in lupus nephritis.

**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. 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.

All trademarks used or mentioned in this release are protected by law.

**References**


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