Media Release

FDA approves Roche's Rituxan (rituximab) in children with two rare blood vessel disorders

- **Rituxan is the first and only FDA-approved treatment for paediatric patients 2 years of age and older living with granulomatosis with polyangiitis or microscopic polyangiitis**
- **These two forms of vasculitis in children are rare and associated with severe, potentially life-threatening symptoms**
- **This approval is the first paediatric indication for MabThera/Rituxan**

Basel, 30 September 2019 - Roche (SIX: RO, ROG; OTCQX: RHHBY) today announced that the U.S. Food and Drug Administration (FDA) has approved Rituxan® (rituximab), in combination with glucocorticoids, for the treatment of granulomatosis with polyangiitis (GPA) and microscopic polyangiitis (MPA) in paediatric patients 2 years of age and older. GPA and MPA are rare, potentially life-threatening diseases affecting small and medium sized blood vessels.

"Rituxan is now approved as the first and only medicine for paediatric patients living with GPA and MPA, two potentially life-threatening blood vessel disorders which are rare in children," said Sandra Horning, M.D., Roche's Chief Medical Officer and Head of Global Product Development. "Today's approval is a result of our ongoing commitment to working with the FDA to develop medicines for paediatric patients with rare diseases where there is a serious unmet need."

The approval is based on data from the PePRS study, a Phase IIa, global, open-label, multicentre single-arm study investigating the safety, pharmacokinetics, exploratory efficacy and pharmacodynamic outcomes of intravenous MabThera/Rituxan in 25 patients with active GPA or MPA between 6 and 17 years of age. Treatment with four weekly infusions of MabThera/Rituxan in combination with a tapering course of oral glucocorticoids was assessed in newly diagnosed or relapsing active GPA or MPA paediatric patients. Of the 25 patients in the study, 19 had GPA and 6 had MPA at baseline. Efficacy was an exploratory endpoint and primarily assessed using the Paediatric Vasculitis Activity Score (PVAS). Efficacy assessment showed that 56% of patients achieved PVAS remission by month 6, 92% by month 12, and 100% of patients achieved remission by month 18. The safety profile of MabThera/Rituxan in patients with paediatric GPA and MPA was consistent in type, nature and severity with the known safety profile of MabThera/Rituxan in adult patients with GPA and MPA, rheumatoid arthritis and pemphigus vulgaris.

The FDA previously granted Priority Review to Rituxan for the treatment of GPA and MPA in paediatric patients. In 2011, Rituxan became the first and only therapy approved by the FDA for the treatment of adults with these two rare forms of vasculitis. MabThera/Rituxan is currently indicated for the treatment of four autoimmune conditions and since 2006 more than 900,000 people have been treated with MabThera/Rituxan for autoimmune conditions worldwide. MabThera/Rituxan is not indicated in children less than 2 years of age with GPA or MPA, or in children with conditions outside of GPA and MPA.

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About Granulomatosis with Polyangiitis and Microscopic Polyangiitis
Granulomatosis with polyangiitis (GPA) (formerly known as Wegener's Granulomatosis) and microscopic polyangiitis (MPA) are two types of ANCA-associated vasculitis (AAV). AAV is a form of vasculitis, or blood vessel inflammation, that primarily affects small blood vessels. In general, GPA and MPA both affect the small blood vessels of the kidneys, lungs, sinuses, and a variety of other organs, but the diseases may affect each person differently. Both GPA and MPA are considered rare diseases, with an estimated prevalence globally of 23 to 160 cases per million in the population. Cases of paediatric onset GPA and MPA are even more rare and are associated with severe, potentially life-threatening symptoms.

About MabThera/Rituxan in Immunology
MabThera (Rituxan in the US) is indicated for the treatment of four autoimmune conditions. MabThera/Rituxan in combination with glucocorticoids, is indicated for the treatment of granulomatosis with polyangiitis (Wegener's granulomatosis, GPA) and microscopic polyangiitis (MPA) in adults. Rituxan, in combination with glucocorticoids, is also indicated for the treatment of granulomatosis with polyangiitis (GPA) and microscopic polyangiitis (MPA) in paediatric patients 2 years of age and older.

MabThera/Rituxan 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 is indicated for the treatment of adults with moderate to severe pemphigus vulgaris (PV). People with serious infections should not receive MabThera/Rituxan.

MabThera/Rituxan is not indicated in children less than 2 years of age with GPA or MPA, or in children with conditions outside of GPA and MPA.

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), microscopic polyangiitis (MPA) and pemphigus vulgaris (PV). 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. 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 eleventh consecutive year, Roche has been recognised as one of the most sustainable companies 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.

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