Basel, 10 June 2015

Roche receives U.S. FDA breakthrough therapy designation for ACTEMRA®/RoACTEMRA® in systemic sclerosis, and will present new study results at EULAR 2015

- ACTEMRA/RoACTEMRA monotherapy and combination treatment regimens almost double sustained remission rates in people with early rheumatoid arthritis¹
- Five year sustained efficacy of ACTEMRA/RoACTEMRA demonstrated in children with systemic juvenile idiopathic arthritis²
- Global Phase 3 clinical trial initiated in systemic sclerosis, a potentially fatal disease with limited treatment options³⁴

Roche (SIX: RO, ROG; OTCQX: RHHBY) today announced that the U.S. Food and Drug Administration (FDA) has granted breakthrough therapy designation (BTD) status to ACTEMRA®/RoACTEMRA® (tocilizumab) for systemic sclerosis (SSc). This designation is designed to expedite the development and review of medicines intended to treat serious diseases, and to help ensure patients have access to them as soon as possible. Roche also initiated a global Phase 3 study in SSc (NCT02453256).

In addition, new data from the U-ACT-EARLY and TENDER studies in patients with early rheumatoid arthritis (RA)¹ and systemic juvenile idiopathic arthritis (sJIA)², respectively, as well as results from the Phase 2 faSScinate study in SSc³ will be presented this week at the annual congress of the European League Against Rheumatism (EULAR 2015), Rome, 10 to 13 June.

“Close to 500,000 people worldwide have benefited from treatment with ACTEMRA/RoACTEMRA since initial approval over a decade ago. The breadth of our study results at EULAR, ranging from arthritis in adults and children to a rare inflammatory disorder, underscores our commitment to helping people with debilitating autoimmune diseases,” said Sandra Horning, M.D., Roche’s Head of Global Product Development and Chief Medical Officer. “These new data further demonstrate the efficacy and safety of ACTEMRA/RoACTEMRA in multiple diseases, including use as a single therapy in early RA.”
ACTEMRA/RoACTEMRA in early RA

ACTEMRA/RoACTEMRA has been proven to help people with RA protect against damage to their joints and have a better quality of life.\textsuperscript{6,7} Clinical research has shown that effective treatment during the early phase of the disease may prevent irreversible damage to joints and long-term disability.\textsuperscript{8,9} In patients who were diagnosed less than a year prior to study enrolment with no history of any previous disease-modifying therapy, ACTEMRA/RoACTEMRA nearly doubled sustained remission (SR) rates with comparable results as monotherapy and combination. SR rates were 84% for ACTEMRA/RoACTEMRA monotherapy, 86% for ACTEMRA/RoACTEMRA + methotrexate (MTX), and 44% for MTX alone.\textsuperscript{1} Median time to SR was seen in just over two months: 9.9 weeks for ACTEMRA/RoACTEMRA + MTX and 12.7 weeks for ACTEMRA/RoACTEMRA monotherapy (MTX alone results were not measurable).\textsuperscript{1} The safety profile was comparable with previously reported data.\textsuperscript{1} Full results from the U-ACT-EARLY study will be presented as an oral presentation at EULAR 2015 (OP0033; presentation date: Thursday, 11 June, 2015).\textsuperscript{1}

ACTEMRA/RoACTEMRA in systemic juvenile idiopathic arthritis (sJIA)

Juvenile idiopathic arthritis (JIA) affects approximately 100 in every 100,000 children,\textsuperscript{10} of which sJIA – a rare and severe form of childhood arthritis – accounts for 10 to 20 percent of all cases.\textsuperscript{11} The Phase 3 TENDER study demonstrated that 97% of patients achieved 30% improvement in their disease symptoms (JIA ACR30) and 64% achieved 90% improvement (JIA ACR90).\textsuperscript{2} ACTEMRA/RoACTEMRA’s efficacy was maintained through week 260 (4.9 years) with no change in the observed safety profile.\textsuperscript{2} Full results will be presented as a poster presentation at EULAR 2015 (abstract number: THU0508; presentation date: Thursday, 11 June, 2015).\textsuperscript{2} ACTEMRA/RoACTEMRA is the only agent approved for the treatment of both sJIA and polyarticular juvenile idiopathic arthritis (pJIA) in patients two years and older.\textsuperscript{12}

ACTEMRA/RoACTEMRA in systemic sclerosis (SSc)

SSc is a rare, chronic disorder characterised by blood vessel abnormalities, as well as degenerative changes and scarring in the skin, joints, and internal organs.\textsuperscript{13} The incidence of SSc is difficult to measure but is estimated to affect approximately 2.5 million people worldwide, and has the highest mortality of any rheumatic disease.\textsuperscript{3,14} FDA BTD status for ACTEMRA/RoACTEMRA was granted based on data from the Phase 2 faSScinate study. 48 week data from faSScinate will be presented as an oral presentation at EULAR 2015 (OP0054; presentation date: Thursday, 11 June, 2015).\textsuperscript{5} While the primary endpoint of improvement in
skin thickening at 24 weeks, as assessed by Rodnan skin score, was not met a meaningful trend was observed. In this second part of the study, there was continued improvement in skin thickening between weeks 24 and 48. The overall adverse event profile between both groups was comparable. The extent and severity of skin thickening correlates to disease worsening, increased disability and decreased survival. Based on these Phase 2 results and the unmet need in patients with SSc, for which there are no approved disease modifying therapeutic options, Roche initiated a global Phase 3 multicentre, randomised, double-blind, placebo-controlled study (NCT02453256).

About RoACTEMRA®/ACTEMRA® (tocilizumab)

ACTEMRA/RoACTEMRA is the first anti-IL-6 receptor biologic approved in intravenous (IV) and subcutaneous formulations, for the treatment of adult patients with moderate to severe active RA. ACTEMRA/RoACTEMRA can be used alone or with MTX in adults who are intolerant to, or have failed to respond to other anti-rheumatic medications. In the most recent update to the EULAR RA management guidelines, ACTEMRA/RoACTEMRA is highlighted as the only biologic that has been repeatedly demonstrated to be superior as a monotherapy over MTX or other conventional disease-modifying anti-rheumatic drugs (DMARDs). ACTEMRA/RoACTEMRA IV formulation is approved in most major countries for polyarticular juvenile idiopathic arthritis (pJIA) or systemic juvenile idiopathic arthritis (sJIA) in children two years of age and older. In Europe, ACTEMRA/RoACTEMRA is also approved for use in patients with severe, active and progressive RA (early RA) who previously have not been treated with MTX. ACTEMRA/RoACTEMRA is part of a co-development agreement with Chugai Pharmaceutical Co., Ltd and has been approved in Japan since April 2005. ACTEMRA/RoACTEMRA is approved in more than 100 countries worldwide.

About Roche in Immunology

The Roche Group’s immunology medicines include rheumatoid arthritis treatments MabThera®/Rituxan® (rituximab) and ACTEMRA®/RoACTEMRA® (tocilizumab), XOLAIR® (omalizumab) in asthma, Pulmozyme® (dornase alfa) for cystic fibrosis and Esbriet® (pirfenidone) for idiopathic pulmonary fibrosis. In addition, MabThera is approved for the treatment of certain types of small-vessel vasculitis. Roche’s late-stage pipeline includes etrolizumab, which is being studied in ulcerative colitis, and lebrikizumab for severe asthma.
About Roche

Headquartered in Basel, Switzerland, Roche is a leader in research-focused healthcare with combined strengths in pharmaceuticals and diagnostics. Roche is the world’s largest biotech company, with truly differentiated medicines in oncology, immunology, infectious diseases, ophthalmology and neuroscience.

Roche is also the world leader in in vitro diagnostics and tissue-based cancer diagnostics, and a frontrunner in diabetes management. Roche’s personalised healthcare strategy aims at providing medicines and diagnostics that enable tangible improvements in the health, quality of life and survival of patients. Founded in 1896, Roche has been making important contributions to global health for more than a century. Twenty-four medicines developed by Roche are included in the World Health Organization Model Lists of Essential Medicines, among them life-saving antibiotics, antimalarials and chemotherapy. In 2014, the Roche Group employed 88,500 people worldwide, invested 8.9 billion Swiss francs in R&D and posted sales of 47.5 billion Swiss francs. 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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References


2 De Benedetti F, et al. Safety and Efficacy of Tocilizumab in Patients With Systemic Juvenile Idiopathic Arthritis: 5-Year Data From TENDER, a Phase 3 Clinical Trial. Poster presentation at EULAR, 2015. Poster number: THU0508. Presentation date: Thursday, 11 June, 2015. Presentation time: 12:00-1:45PM.


