FDA grants breakthrough therapy designation for Roche’s Actemra/RoActemra in giant cell arteritis, a form of vasculitis

- Roche recently announced positive results from a Phase III GCA clinical trial
- Fourteenth Breakthrough Therapy designation for Roche medicines
- There have been no therapies approved for GCA in more than 50 years

Roche (SIX: RO, ROG; OTCQX: RHHBY), announced today that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation status to Actemra/RoActemra® (tocilizumab) for giant cell arteritis (GCA), a chronic, potentially life-threatening autoimmune condition. The disease is caused by inflammation of large and medium-sized arteries, most often in the head, but also in the aorta and its branches.¹

“The FDA Breakthrough Therapy designation for GCA underscores our continued commitment to explore Actemra/RoActemra in autoimmune diseases with significant unmet need,” said Sandra Horning, M.D., Chief Medical Officer and head of Global Product Development. “We are looking forward to working with the FDA in the hope of making Actemra/RoActemra available to people with GCA, a condition for which there has been no approved treatment in more than 50 years.”

Breakthrough designation is intended to expedite the development and review of medicines with early evidence of potential clinical benefit in serious diseases and to help ensure that patients receive access to medicines as soon as possible. This is the fourteenth Breakthrough Therapy Designation granted to Roche since 2013, and the second for Actemra/RoActemra.

In June this year, Roche announced the positive outcome of the Phase III GiACTA study evaluating Actemra/RoActemra in people with GCA. Results showed that Actemra/RoActemra, initially combined with a six month steroid (glucocorticoid) regimen, more effectively sustained remission through one year compared to a six or 12 month steroid-only regimen in people with GCA. Full data will be presented at an upcoming medical meeting in 2016.
**About Giant Cell Arteritis**

Giant cell arteritis (GCA) - also known as temporal arteritis (TA) - is a potentially life-threatening autoimmune condition. GCA has a global impact and usually affects those above the age of 50, and the disease is two-to-three-times more likely to affect women than men.\(^1\) GCA is often difficult to diagnose because of the wide and variable spectrum of signs and symptoms. GCA can cause severe headaches, jaw pain and visual symptoms and if untreated, can lead to blindness, aortic aneurysm or stroke.\(^1\) Treatment to date for people with GCA has been limited to high-dose steroids that play a role as an effective ‘emergency’ treatment option to prevent damage such as vision loss. However, steroids do not always maintain long-term disease control (flare-free remission) and are often associated with severe side effects.\(^2,3,4\) Due to the variety of symptoms, complexity of the disease and its complications, people with GCA are often seen by several physicians including rheumatologists, neurologists and ophthalmologists.

**About Actemra/RoActemra**

Actemra/RoActemra is the only approved anti-IL-6 receptor biologic, available in both intravenous (IV) and subcutaneous formulations, for the treatment of adult patients with moderate to severe active rheumatoid arthritis (RA). Actemra/RoActemra can be used alone or with methotrexate (MTX) in adults who are intolerant to, or have failed to respond to, other anti-rheumatic medications. In the most recent update to the European League Against Rheumatism (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 antirheumatic drugs (DMARDs). The extensive Actemra/RoActemra RA IV clinical development program included five Phase III clinical studies and enrolled more than 4,000 people with RA in 41 countries. The Actemra/RoActemra RA subcutaneous clinical development program included two Phase III clinical studies and enrolled more than 1,800 people with RA in 33 countries. Actemra/RoActemra IV formulation is approved in most major countries for polyarticular juvenile idiopathic arthritis (pJIA) and 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 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 115 countries worldwide.

Actemra/RoActemra is also being investigated in a global Phase III multicentre, randomised, double-blind, placebo-controlled study (NCT02453256) for patients with systemic sclerosis (SSc). Actemra/ RoActemra
was granted Breakthrough Therapy Designation for SSc by the US Food and Drug Administration (FDA) in June 2015.

**About Roche**

Roche is a global pioneer in pharmaceuticals and diagnostics focused on advancing science to improve people’s lives.

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. 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.

Founded in 1896, Roche continues to search for better ways to prevent, diagnose and treat diseases and make a sustainable contribution to society. Twenty-nine 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. Roche has been recognised as the Group Leader in sustainability within the Pharmaceuticals, Biotechnology & Life Sciences Industry eight years in a row by the Dow Jones Sustainability Indices.

The Roche Group, headquartered in Basel, Switzerland, is active in over 100 countries and in 2015 employed more than 91,700 people worldwide. In 2015, Roche invested CHF 9.3 billion in R&D and posted sales of CHF 48.1 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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**Additional information**

- Roche in Oncology: [www.roche.com/media/media_backgrounder/media_oncology.htm](http://www.roche.com/media/media_backgrounder/media_oncology.htm)

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References