Global pharmaceutical company GlaxoSmithKline is developing a treatment for rheumatoid arthritis and osteoarthritis, based on discoveries made by University of Melbourne researchers. The company is nearing completion of three Phase II clinical trials, after sublicensing the development and commercialisation rights from German biotechnology company MorphoSys, which licensed them from the University of Melbourne.

The compound acts on the immune system to limit the inflammation that causes the joint damage and pain that characterise these diseases. Studies show that it may also have applications for other chronic inflammatory conditions, such as multiple sclerosis and some lung diseases.

Key points
- GlaxoSmithKline nearing completion of Phase II clinical trials for drug to treat hand osteoarthritis and rheumatoid arthritis
- Osteoarthritis and rheumatoid arthritis are the two most common forms of arthritis
- Based on antibody technology developed by Professor John Hamilton and Associate Professor Andrew Cook of the Faculty of Medicine, Dentistry and Health Sciences

GlaxoSmithKline
Developing treatments for rheumatoid arthritis and osteoarthritis

The outcome
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The need
Osteoarthritis and rheumatoid arthritis are the two most common forms of arthritis. It has been estimated that half of the world’s population aged 65 years or older has osteoarthritis, while 1–2 per cent of people of all ages have rheumatoid arthritis.

Osteoarthritis is a degenerative disease affecting specific joints, while rheumatoid arthritis is an auto-immune disease that can affect the entire body. Although they differ in origin, their effects are similar: inflammation leads to cartilage and bone damage, and the subsequent swelling and stiffness cause pain. The associated loss of movement can reduce quality of life and increase the risk of obesity and cardiovascular disease.

Current therapies treat the symptoms rather than curing or preventing the diseases.
The science

Professor John Hamilton, Associate Professor Andrew Cook and colleagues from the Faculty of Medicine, Dentistry and Health Sciences focused their research on a specific type of protein involved in the inflammatory immune response.

The protein, granulocyte-macrophage colony-stimulating factor (GM-CSF), stimulates the production of different cell types and then activates those cells to produce pro-inflammatory compounds.

After inducing arthritis in mice, the team found that mice without GM-CSF had less joint deformation, cartilage damage and inflammation than mice with GM-CSF. By using an antibody that inactivates GM-CSF, the researchers successfully prevented its pro-inflammatory activity in mice.

Together with Professor Gary Anderson, now director of the University’s Lung Health Research Centre, the team have also demonstrated an effect of the anti-GM-CSF antibody in lung disease.

Technology development history

The researchers patented a method of treatment for their antibody technology for inflammation in the USA in 2000. Several pharmaceutical companies expressed an interest in the technology. In 2005, the University partnered with MorphoSys, which had extensive experience in developing human monoclonal antibodies.

MorphoSys obtained exclusive rights to use GM-CSF inhibitors to treat inflammatory diseases, in return for providing the University of Melbourne with an upfront payment, milestone payments linked to clinical development, and a percentage royalty on net sales in the USA. In 2008, Professor Hamilton and Associate Professor Andrew Cook filed additional patents covering the use of the anti-GM-CSF antibody to treat osteoarthritis and pain. This technology was assigned to MorphoSys under similar licensing terms.

MorphoSys successfully completed a Phase I clinical trial in healthy volunteers with the anti-GM-CSF antibody (which they named MOR103) and a Phase Ib trial in individuals with multiple sclerosis.

A Phase I/II trial in patients with rheumatoid arthritis demonstrated that MOR103 is the first antibody against GM-CSF to safely and effectively treat this disease. (Phase I clinical trials are designed to test the safety, side effects, dose and formulation of a particular treatment, while Phase II clinical trials are designed to evaluate its effect.)

In 2013, MorphoSys sublicensed the technology to GlaxoSmithKline for an upfront payment of €22.5 million, milestone payments of up to €423 million, and double-digit royalties on net sales.

GlaxoSmithKline assumed responsibility for the development and commercialisation of MOR103, which they renamed GSK3196165. After reformulating the technology, the company launched three Phase II clinical trials in hand osteoarthritis and rheumatoid arthritis, expected to be completed in 2018.

Players, publications and patents

**Company:** GlaxoSmithKline, MorphoSys

**Researchers:** Professor John Hamilton, Professor Gary Anderson, Associate Professor Andrew Cook

**Patents and key publications:**


Half the world’s population aged 65 and over has osteoarthritis. Picture: Shutterstock