

MS drug candidate also shows promise for ulcerative colitis

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Positive new clinical data were released today on a drug candidate for ulcerative colitis that was first discovered and synthesized at The Scripps Research Institute (TSRI).

According to results released today from a Phase 2 study of 199 patients with active, moderate to severe disease, the [drug candidate](#) RPC1063 has potential to significantly improve the treatment paradigm for [ulcerative colitis](#) patients. The latest results show that, after eight weeks of treatment with a 1 mg dose of RPC1063, 16.4 percent of patients were in clinical remission, as compared to 6.2 percent of patients on placebo.

"We are delighted that RPC1063 is showing promise for ulcerative colitis patients in addition to its already significant efficacy and safety data in [multiple sclerosis](#)," said TSRI Professor Hugh Rosen, who together with Professor Ed Roberts led the team that discovered RPC-1063. "Research carried out at TSRI since 2002 has led to the discovery of fundamental mechanisms that can be modulated for potential treatments of a variety of autoimmune diseases including ulcerative colitis and multiple sclerosis, and the unique multidisciplinary environment in chemistry and biology at TSRI allowed this progression to clinical trials."

The clinical trial, sponsored by Receptos, Inc., the San Diego biotechnology company now developing the drug, also showed that RPC1063 was generally well tolerated.

Ulcerative colitis is a chronic condition that involves inflammation and sores in the inner lining of the digestive tract. Ulcerative colitis is an [inflammatory bowel disease](#), which, along with Crohn's disease, affects more than one million people nationwide, according to the U.S. Centers for Disease Control and Prevention. Some people have mild disease, while others are affected with life-threatening complications.

While existing medications for ulcerative colitis do help some patients, 23 to 45 percent of ulcerative colitis sufferers progress and eventually require surgical removal of all or part of the colon, according to the Crohn's and Colitis Foundation of America.

The drug candidate RPC1063 was derived from a screening "hit" from the National Institutes of Health molecular library at Scripps Florida's Molecular Screening Center, using assay technology from the Rosen lab in La Jolla. The Roberts and Rosen labs then developed significant medicinal chemistry to turn that hit into a validated lead, and then ultimately a drug candidate.

TSRI then licensed the compound to Receptos, which is developing RPC1063 for approval by the U.S. Food and Drug Administration.

The latest results come from Receptos's multi-national, multi-center, double-blind, randomized, placebo-controlled study investigating the effect of two active doses of RPC1063 versus placebo for the treatment of moderately to severely active ulcerative colitis. For more information on the results, see the [press release](#) from Receptos.

In light of the current positive results, Receptos plans to initiate a Phase 3 trial of RPC1063 for ulcerative colitis, as well as a Phase 2 study of the drug candidate for Crohn's disease.

The mechanism of RPC1063 (Sphingosine 1-Phosphate Receptor

modulation) may also be significant in the treatment of other [autoimmune diseases](#). Receptos is also currently evaluating the drug candidate in a Phase 3 study for the treatment of multiple sclerosis.

Provided by The Scripps Research Institute

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