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Receptos might not fly solo for much longer

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There are often perfectly logical reasons why biotech assets remain unpartnered into the late stages of development: lack of efficacy, a me-too mechanistic approach or a dodgy patent position, for instance.

None of these seems to apply to Receptos's RPC1063, which yesterday generated phase II ulcerative colitis data that had some analysts touting it as a possible best-in-class agent. If the finer details of the result hold up, a lucrative deal could be in the offing, and Leerink has already speculated on a $3bn takeout of the company.

This could be possible in the current bull market. After the RPC1063 data from the Touchstone trial were released last night Receptos surged 35% in the aftermarket, opening at $93, equivalent to a market cap of around $2.5bn.

Biotech watchers were keeping a close eye on RPC1063 given its sole developer status – something *EP Vantage* highlighted in a recent analysis of the sector's most valueable unpartnered assets (*Still unpartnered, but now worth a lot more, September 22, 2014*). However, the more closely watched indication was multiple sclerosis, in which RPC1063 is in phase III.

Ulcerative colitis thus adds a second string to its bow. Of particular note is the higher 1mg dose of RPC1063, which hit statistically significant and clinically relevant increases versus placebo in the primary endpoint – remission at week eight – and all three secondary endpoints.

A 0.5mg dose showed numerical increases versus placebo but failed to hit statistical significance – supporting a dose response effect. The company said 1mg seemed like the right dose to take into phase III, and analysts speculated that the phase II data were good enough to make Touchstone a pivotal study, meaning that just one positive phase III result could now suffice for approval.

As good as biologicals

The ulcerative colitis market is dominated by biologicals, which are injected or infused, of which the most recent entrant is Takeda's Entyvio.

RPC1063, a sphingosine-1-phosphate 1 receptor modulator, seems at least as good – with the added benefit of being dosed orally. The 1mg group resulted in eight-week remission of 16.4% versus 6.2% for placebo ($p<0.05$), which compares favourably with phase III data for Humira (16.5% vs 9.3%), Simponi (17.8% vs 6.4%) and Entyvio (16.9% vs 5.4%).

This gives RPC1063 its second phase II hit in four months; in June positive topline data in multiple sclerosis looked like giving Novartis's Gilenya a run for its money. Gilenya acts in a similar way, modulating the sphingosine-1-phosphate receptor.

Leerink said the two indications fitted nicely into pricing models; Entyvio, the new ulcerative colitis benchmark, costs $50,000 per patient per year, while recently launched multiple sclerosis drugs have price tags of about $60,000.

What is to stop Receptos now doing a lucrative licensing deal, or even being bought out? One obvious point is that stellar phase II data are rarely replicated in the tougher phase III setting, and partners might want to see longer-term remission. Receptos owes the Scripps Research Institute a 2% royalty on RPC1063, though this should not impede partnering.

There is a competitive threat; numerous compounds acting on the sphingosine-1-phosphate receptor are in development, including Novartis's siponimod and Merck & Co's ceralifimod. Interestingly, however, very few have been tested in ulcerative colitis, and Kyorin's KRP-203 was dropped in this setting.

There is nothing to stop Novartis investigating Gilenya in ulcerative colitis, though presumably some dose adjustment would be needed. That said, Receptos has a clear first-mover advantage, and few biotechs can boast two excellent phase II readouts.

Receptos has over $360m of cash in the bank so no doubt it will push hard in partnering talks, but it would be

surprising to see RPC1063 still unpartnered this time next year.

Study	Design	Trial ID
Touchstone	199 patients, 0.5mg, 1.0mg or placebo, double-blind	NCT01647516

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