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Receptos (RCPT - OUTPERFORM): RPC1063 Update Offers Further Evidence of Distinction as a Potential Best-in-Class S1P1R Modulator, In Our View; Reiterate OUTPERFORM and $23 FV

Price: $18.50 **Fair Value Estimate: $23**

- **Receptos provided an update on RPC1063 which includes thorough QT study results and Special Protocol Assessment (SPAs) agreements with the FDA.** As previously disclosed, the company has SPAs with the FDA for the planned Phase 3 portion of the Phase 2/3 Radiance study as well as a second confirmatory Phase 3 study of RPC1063 in relapsing multiple sclerosis (RMS). Although SPAs are not binding contracts, we believe they do significantly reduce the regulatory risk associated with potential RPC1063 approval.

- **We believe the TQT results continue to differentiate RPC1063 as a best-in-class S1P1R modulator given its benign effect on cardiac safety.** The company completed a thorough QT/QTc (TQT) study that enrolled 124 subjects with 62 subjects randomized to receive RPC1063 at an intended therapeutic dose (1 mg/day) and at a supra-therapeutic dose (2 mg/day), and 62 subjects randomized to placebo. The dosage of RPC1063 was titrated from 0.25 mg to 2.0 mg over 14 days. As previously disclosed, the primary objective of the study was met as top-line results showed no meaningful QT effect at both the therapeutic and supra-therapeutic doses. We note that supra-therapeutic (1.25 or 2.0 mg) doses of Gilenya resulted in QTc prolongation, as expected, in a TCT study; however, no clinically relevant findings have been observed in treated patients, to our knowledge. Additionally, the TQT study was designed to assess RPC1063's effect on cardiac safety. Results showed that the dose titration regimen being implemented in all ongoing and planned trials appears to attenuate the first dose heart rate effect seen with Gilenya. Overall, we believe these data support our view that RPC1063 has pharmacological properties that may improve safety over Gilenya.

- **We project cash runway through major clinical catalysts in 2014 and project full-year profitability in 2019.** The company ended Q1 2013 with about $20 million in cash, investments, and equivalents. With financing from the initial public offering (IPO), management guided to runway into H2 2015, which includes anticipated mid-2014 releases of transforming top-line Phase 2 results testing RPC1063 treatment of RMS and IBD/UC. With a commercial partner on board for RPC1063 in RMS and IBD, we project full-year profitability in 2019 after launching RPC1063 in RMS in late 2018, in IBD in 2019 as well as RPC4046 launch in 2019.

- **We reiterate our OUTPERFORM rating and $23 fair value.** We calculate RCPT's fair value based on the sum of a 30% annual discount and a 1x-10x premium range on our net peak annual sales estimate for each product and indication in the clinic to reflect risk.

Investment Thesis

Receptos, located in San Diego, CA, is an emerging biopharmaceutical company developing first-in-class and best-in-class drug candidates for large market opportunities and rare diseases. The company's lead product, RPC1063, is a sphingosine 1-phosphate (S1P1R) receptor modulator being developed as an orally-dosed treatment candidate being tested in a Phase 2/3 clinical trial for relapsing multiple sclerosis (RMS) and in a Phase 2 trial for inflammatory bowel disease (IBD). The second treatment candidate, RPC4046, is an anti-IL13 monoclonal antibody being developed as a potential treatment for an allergic/immune orphan disease called Eosinophilic Esophagitis (EoE). We believe clinical risk is lower than normal as RPC1063 has the same disease target as Novartis's approved RMS treatment Gilenya, but has a better safety profile and best-in-class potential. RPC4046 offers an orphan drug opportunity for Receptos to develop its own sales force. We believe execution risk is lower than normal as we consider management to have higher-than-normal knowledge and experience in the pharmaceutical industry—especially in multiple sclerosis. The CEO was successful at not only developing daclizumab, but also increasing value for FACET and making it an acquisition target for ABT. Additionally, we view the rest of the management team as being top tier. Receptos ended Q1:13 with about $20 million in cash and along with the IPO funding of about $72.8 million (excluding shoe), management projects runway into H2 2015, which includes top-line results from the ongoing Phase 2/3 trial testing RPC1063 treatment of RMS as well as IBD in mid-2014. We anticipate RPC1063 is likely to achieve clinical success and regulatory approval and could reach gross peak annual worldwide sales of over $2 billion for RMS and over $850 million for IBD. We also project RPC4046 treatment of EoE could reach over $1 billion in gross peak annual worldwide sales with premium orphan drug pricing.

Figure 1: Anticipate Milestones (*our estimates) – Transforming in 2014

Q2:13	Q1 FINANCIALS (MID-JUNE)
H2:13	COMPLETE ENROLLMENT PHASE 2 RADIANCE
Q4:13	180 DAY UNLOCK (11/5/13; 11,826,464 SHARES)
YE:13/Q1:14	RPC 1063 RMS PHASE 3 RADIANCE INITIATION WITH SPA
Q1:14	RPC 4046 EOE PHASE 2 INITIATION
MID:14	RPC 1063 RMS PHASE 2 RADIANCE DATA RELEASE
MID:14	RPC 1063 UC PHASE 2 TOUCHSTONE DATA RELEASE
H2:15	RPC 4046 EOE TOPLINE PHASE 2 DATA RELEASE

Source: Company data, Wedbush Securities, Inc.

Risks to Attainment of Our Fair Value

Clinical Risk: We believe clinical risk is low in 2013, but likely to increase in 2014 with release of Phase 2 clinical results. Receptos is a developmental stage emerging pharmaceutical company which has completed Phase 1 and is conducting a Phase 2 trial for their lead product candidate, RPC1063 for the treatment of relapsing multiple sclerosis (RMS) with top-line results expected in mid-2014. As will all clinical candidates, RPC1063 is susceptible to inherent risks of failure at any stage of drug development, which may include unexpected adverse events; however, the S1P1 target has been validated by Novartis' GILENYA™ and RPC1063 appears to have a better safety profile. The company is also developing RPC1063 as a treatment candidate for inflammatory bowel disease (IBD) which is currently in a Phase 2 clinical trial with initial results expected in mid-2014. A second clinical candidate, RPC4046 is being developed as a treatment candidate for Eosinophilic Esophagitis (EoE) and is expected to start Phase 2 in 2014. Because the company is not expected to release initial top line results from mid-to-late stage clinical candidates, we do not believe clinical risk to our fair value is high in 2013.

Regulatory Risk: We consider regulatory risk to be low in 2013; however; in general, we believe if RPC1063 successfully completes clinical development, we believe regulatory risk is likely to be lower than average. That the FDA approved Novartis' GILENYA™ in 2011 despite safety issues including potential mortality upon initial dosing due to cardiovascular adverse events, suggests to us that a safer drug candidate with a similar efficacy profile is also likely to obtain approval. Receptos has never obtained marketing approval for a drug candidate and we do not anticipate NDA filing for the lead drug candidate (RPC1063) until 2017. Upon completion of regulatory review, if the FDA requires additional studies or data, the resulting increased costs and delays in the marketing approval would likely increase financing risk. Even after conducting such trials and submitting new data, the FDA may find these to be insufficient or may not agree with the analysis and still may not approve the NDA. Any delay in obtaining, or an inability to obtain, marketing approvals would increase financing risk by delaying commercialization as well as potential profitability. Regulatory risk can involve turnover in regulatory

decision-makers, which can change policy and approval criteria after the trial is conducted. Agency statisticians may choose a different analytical process than was conducted in the NDA and conclude that the trials failed to achieve statistical efficacy. Changes in standard-of-care occurring while the trial is ongoing may also result in the design being found to be obsolete during regulatory review. Even if a product is approved, the designated patient population may be much smaller than expected, which could limit sales potential. Post-approval clinical studies may be required as well as limits on sales and marketing practices and materials. If unexpected adverse effects emerge the drug can be withdrawn from the market. Regulatory requirements also vary among different countries and may result in requirements for additional clinical trials.

Manufacturing Risk: We consider manufacturing risk to be low in 2013, but higher than normal for the future as Receptos lacks manufacturing capability and plans to continue relying on third parties to supply its product candidates. In addition, the company does not have any executed agreements for long-term commercial supply for any of its drug candidates, but plan to do so for RPC1063 prior to commercial launch. For RPC4046, AbbVie has agreed to manufacture enough for preclinical and clinical trials and may continue to or may choose to engage a third party following the planned Phase 2 results in EoE, after which, AbbVie may choose to execute an option to collaborate with Receptos for RPC4046 development and commercialization. Multiple improvements to the manufacturing process for RPC4046 have been made and a comparability assessment of the material used in the completed Phase 1 study versus the new process must be filed prior to the initiation of the Phase 2 in EoE.

Commercialization Risk: We consider commercialization risk to be low in 2013, but higher than average in general due to Receptos's small size and development stage. Receptos's business model is to develop and commercialize clinical candidates; however, for small development-stage companies, we view commercialization risk in general as higher than normal until/unless the company partners commercialization with an appropriate larger pharmaceutical company—especially for large indications such as multiple sclerosis. We anticipate Receptos is likely to partner commercial activities for large markets globally. For rare diseases such as EoE, the company may hire a small specialty sales force for the US, but we anticipate the company will partner commercialization for primary care globally as well as for all physicians outside the US. We consider this commercial plan to be optimal for leveraging potential profits from sales for a small company.

Competition Risk: We view competition risk as low in 2013 but, in general, higher than average unless Receptos partners with an appropriate global pharmaceutical company for commercialization. In general, we believe a small development-stage emerging pharmaceutical company with limited resources has higher-than-average competition risk. In the situation with RPC1063, while we believe large pharmaceutical companies with large marketing budgets, such as Novartis and Biogen-Idec may counter-detail RPC1063 after potential launch in late 2018, if its emerging profile of equal efficacy to GILENYA™, but improved safety while maintaining once-daily oral dosing is maintained through clinical development, we believe physicians treating MS patients are likely to prefer it over the currently approved oral therapies. In addition, physicians treating MS have commented that twice-daily dosing such as for Biogen-Idec's Tecfidera™ may have reduced real-world efficacy as their patients may forget to take the evening dose.

Intellectual Property Risk: We consider intellectual property risk to be low in 2013 and, in general, as the company has an exclusive license for the RPC1063 composition of matter patent which expires in May 2029 and could be extended into 2032. In addition, intellectual property protection for RPC4046 also has a long runway with expiration in 2028 and may be extended up to 5 years.

Financing Risk: We consider financing risk to be low in 2013, but likely to increase in H2 2014 as runway following the IPO financing lasts into H2 2015.

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I, Liana Moussatos, Ph.D., Richard Lau, certify that the views expressed in this report accurately reflect my personal opinion and that I have not and will not, directly or indirectly, receive compensation or other payments in connection with my specific recommendations or views contained in this report.

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Company	Disclosure
Receptos	1,3,5,7

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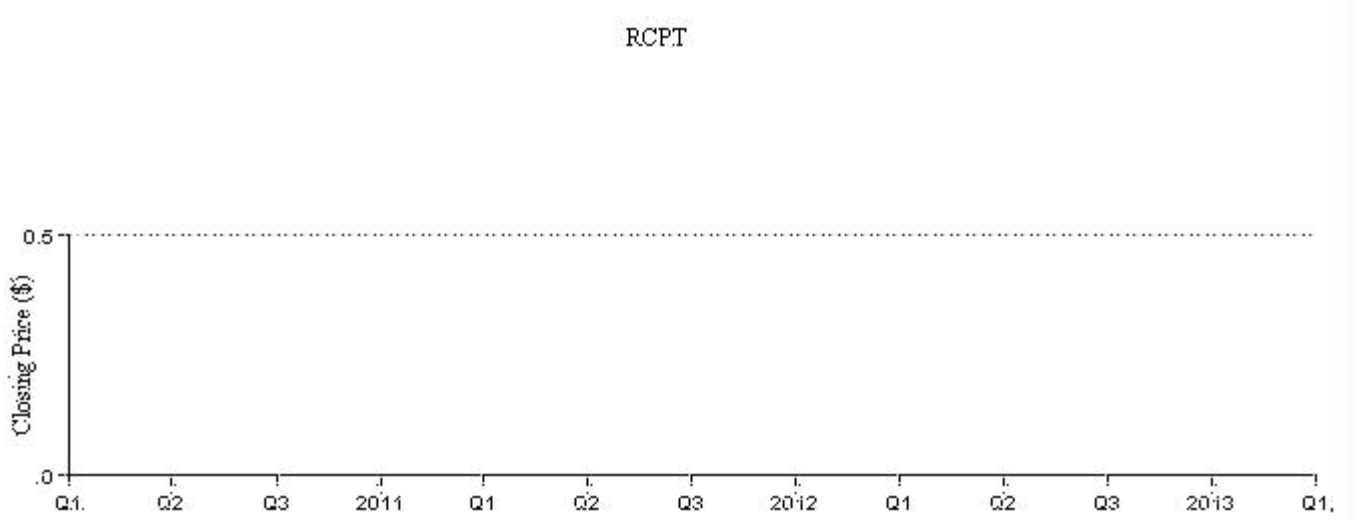

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