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## Receptos (RCPT - OUTPERFORM): Additional Safety and PK/PD Analyses at AAN Support RPC1063's Superior Profile in Our View. Reiterate OUTPERFORM and \$59 PT.

Price: \$34.37

12-Month Price Target: \$59

- **Receptos presented additional safety analyses for RPC1063 at the American Academy of Neurology Annual Meeting (AAN April 26-May 3, 2014 Philadelphia).** The company previously disclosed positive safety results from a thorough QT study and additional analyses were presented at AAN. In addition, additional safety & PK/PD data around the metabolism of RPC1063 further supports its potentially superior safety profile (in our view) compared with Gilenya.
- **RPC1063 shows consistent metabolite biotransformation and predictable drug exposure time-dependent steady state lymphocyte reduction across subjects in Phase I study.** In a poster presentation that will be presented at AAN (American Academy of Neurology (AAN) Annual Meeting, Philadelphia, April 26-May 3) titled "Pharmacokinetics and Pharmacodynamics of RPC1063 and Its Metabolites in Healthy Adult Volunteers, Receptos' investigators will show that RPC1063 is consistently and predictably metabolized into therapeutic equivalent RP101988 and RP101075 metabolites (total plasma AUC were 39% for RPC1063, 49% for RP101988 and 11% for RP101075). RP1063 metabolites have similar  $T_{max}$  (6-8hrs) and elimination half-life (19-22hrs). Additionally, RP1063 and metabolites show no inhibition of CYP450 enzymes or the hERG channel, similar binding profile to plasma proteins and have been qualified in chronic GLP toxicology studies. Moreover, the reduction in steady state lymphocyte levels, measured using a biomarker, correlated with RPC1063, RP101988 and RP101075 exposure time. These data highlight the superior safety profile for RP1063 and further differentiate RPC1063 as a best-in-class S1P1R inhibitor.
- **RPC1063 shows no increase in QTc prolongation based on pre-specified criteria.** In a second poster presentation that will be presented at AAN titled "Absence of a Relevant Effect on Cardiac Repolarization In a QT/QTc (TQT) Study of RPC1063, A Novel , Selective S1P1 Receptor Agonist, In Healthy Adults Volunteers" Receptos' investigators will show RPC1063 QTc change from baseline was <10ms at both 1mg and 2mg doses, meeting a pre-specified criteria. Similar to previous data (reported on in May 6<sup>th</sup>, 2013 note) dose titration regimen ( 0.25mg to 2mg) were well tolerated, tempered the first dose heart rate effect and minimized decrease in heart rate. RPC1063 and control group had similar AEs, which included site reaction, orthostatic hypotension, dizziness, musculoskeletal chest pain and constipation. No SAEs were recorded.

Figure 1: ANTICIPATED MILESTONES (\*Our Estimates)

Timing	Milestone	Estimated Probability	Estimated Upside/Downside
H1:14	RPC 4046 EOE File IND for Ph2 initiation in 2014	--	--
Mid:14	RPC 1063 RMS Ph2 RADIANCE data release	80:20	+5-25%/-10-35%
Mid:14	RPC 1063 UC Ph2 TOUCHSTONE data release	75:20	+5-25%/-5-25%
H2:15	RPC 4046 EOE Topline Ph2 data release	50:50	+10-25%/-5-25%

Source: Company data, Wedbush Securities, Inc.

- **Next:** In addition to release of top-line results from RADIUS mid-year, we also anticipate release of results from TOUCHSTONE in Q3. We believe TOUCHSTONE is also likely to be positive due to Novartis previously' validating S1PR in Phase 2 for UC with a follow-on to Gilenya and preclinical / Phase 1 clinical results for RPC1063 showing a reduction in peripheral lymphocyte count and beneficial preclinical histology changes presented in three posters (search <http://ddw.scientificposters.com/epsSearchDDW.cfm> for #TU1616, #SA1221, #SA122) during Digestive Disease Week (DDW May 18-21, 2013 Orlando). Presuming a commercial partner, we project full-year profitability in 2019 after launching RPC1063 in RMS/IBD in Q4:18/Q1:19, respectively. However, if RPC1063 has positive results in both Phase 2s mid-year, we anticipate a potential acquisition could occur in H2 2015.
- **We reiterate our OUTPERFORM rating and \$59 price target.** We calculate RCPT's 12-month price target using a 365 day projection of our current 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. Due to our 30% annual discount, our 12-month price target is higher than our current fair value.

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**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. In addition, we view the rest of the management team as being top tier. Receptos ended 2013 with about \$69.5 million in cash and raised about \$110 million net in a follow-on offering in January 2014. We project runway into mid-2017 which includes top-line results from the ongoing Phase 2 trial testing RPC1063 treatment of RMS as well as IBD/UC 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 \$4 billion for RMS and over \$950 million for IBD/UC. We also project RPC4046 treatment of EOE could reach over \$1 billion in gross peak annual worldwide sales with premium orphan drug pricing and the oral GLP-1 candidate could reach gross peak WW sales of over \$5 billion. If successful in Phase 2, we believe any of these candidates are likely to attract a partner and could trigger RCPT's acquisition.*

## **RISKS TO THE ATTAINMENT OF OUR FAIR VALUE**

**Clinical Risk:** We believe clinical risk is 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 initiate clinical testing in 2014.

**Regulatory Risk:** We consider regulatory risk to be low in 2014; 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 2014, 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 2014, but higher than average in general due to Receptos' small size and development stage. Receptos' 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 2014 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 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:** Receptos ended 2013 with \$69.5 million in cash and raised about \$102.1 million on January 9, 2014 and exercised overallotments on January 14, 2014 raising gross proceeds of about \$110 million. With this financing we project cash runway (with a partner for the RMS & UC Phase 3 programs) into mid-2017 (not including the \$25MM remainder in MidCap Financial venture debt). Since Receptos just conducted a financing, we consider financing risk to be low in 2014.

### Analyst Certification

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Company	Disclosure
Receptos	1,3,4,5

### Research Disclosure Legend

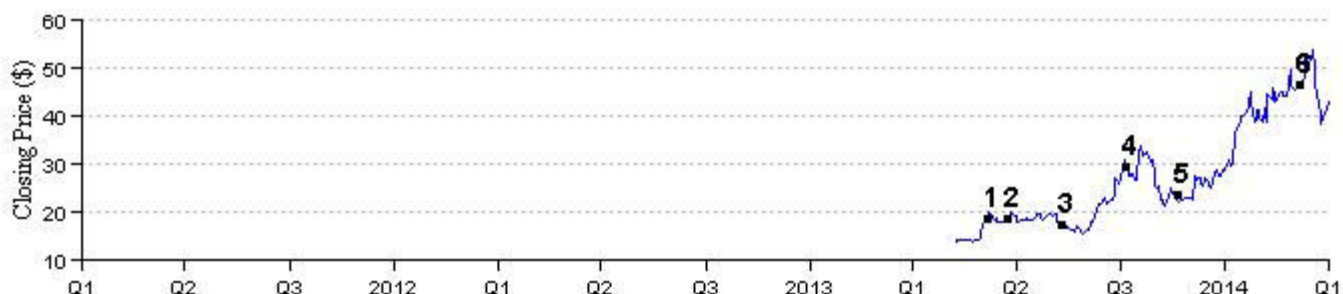
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# RCPT

1) 06/03/13	2) 06/21/13	3) 08/07/13	4) 10/02/13	5) 11/18/13	6) 03/05/14
OUTPERFORM \$23	OUTPERFORM \$24	OUTPERFORM \$25	OUTPERFORM \$26	OUTPERFORM \$44	OUTPERFORM \$59



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