

## Receptos (RCPT)

**Initiating Coverage Of RCPT With An OUTPERFORM Rating And \$23 Fair Value: We See A Blockbuster Pipeline, Solid Management, And Acquisition Potential**

- **Receptos is an emerging pharmaceutical company developing potential blockbuster treatments for two large markets and a rare disease related to immune system disorders.** The company has two clinical candidates, RPC1063 and RPC4046. RPC1063 is being tested as a potential best-in-class treatment for relapsing multiple sclerosis (RMS) and a potential first-in-class treatment for inflammatory bowel disease (IBD). RPC4046 being tested as a first-in-class treatment for a rare disease called Eosinophilic Esophagitis (EoE).
- **Management's track record reduces execution risk, in our view.** Faheem Hasnain, CEO previously developed daclizumab for RMS as CEO of FACET, which was acquired by Abbott (NYSE: ABT) in April 2010. Not only does he have a great track record developing MS drug candidates, but he has surrounded himself with an impressive management team which we believe reduces execution risk.
- **We consider Intellectual property to be strong due to composition of matter patent protection with potential for extended runway.** RPC1063 has CoM protection into 2029, with the potential to extend to 2032. RPC4046 has CoM protection into 2028, with the potential to extend up to five years. With launches in 2018-2019, we see at least 9-10 years' post-launch protection.
- **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.
- **At about \$16 per share, we consider RCPT to be at an attractive valuation compared with our \$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.

June 3, 2013

Price  
**\$15.87**

Rating  
**OUTPERFORM**

Fair Value Estimate  
**\$23**

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### Company Information

Shares Outst (M)	17.6
Market Cap (M)	\$255
52-Wk Range	\$13.00 - \$15.16
Book Value/sh	\$0.78
Cash/sh	\$1.06
Enterprise Value (M)	\$235
LT Debt/Cap %	0.00

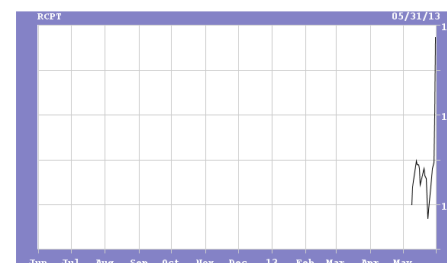
### Company Description

Receptos is developing first- and best-in-class treatments for immune disorders. The lead candidate, RPC1063, is being developed as a safer S1P1 modulator versus GILENYA(TM) for multiple sclerosis as well as for IBD.

FYE Dec	2012E	2013E			2014E		
REV (M)	ACTUAL	CURR.	PREV.	CONS.	CURR.	PREV.	CONS.
Q1 Mar	\$2.2A	\$0.6E		N/AE	\$0.2E		N/AE
Q2 Jun	2.2A	0.6E		N/AE	0.2E		N/AE
Q3 Sep	2.2A	0.6E		N/AE	0.2E		N/AE
Q4 Dec	2.2E	0.6E		N/AE	0.2E		N/AE
Year*	\$8.6E	\$2.2E		N/AE	\$0.7E		N/AE
Change	--	--			--		
	2012E	2013E			2014E		
EPS	ACTUAL	CURR.	PREV.	CONS.	CURR.	PREV.	CONS.
Q1 Mar	(\$0.53)A	(\$0.36)E		N/AE	(\$0.55)E		N/AE
Q2 Jun	(0.53)A	(0.40)E		N/AE	(0.60)E		N/AE
Q3 Sep	(0.53)A	(0.43)E		N/AE	(0.63)E		N/AE
Q4 Dec	(0.53)E	(0.48)E		N/AE	(0.65)E		N/AE
Year*	(\$2.12)E	(\$1.67)E		N/AE	(\$2.44)E		N/AE
P/E	NMx	NMx			NMx		
Change	--	--			--		

Consensus estimates are from Thomson First Call.

\* Numbers may not add up due to rounding.



Source: Thomson Reuters

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## INVESTMENT THESIS

### COMPANY SUMMARY

**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.

### KEY POINTS

- Receptos is an emerging biopharmaceutical company developing a potential blockbuster treatment for two large markets and a second candidate to treat rare diseases related to immune system disorders.** The company has two clinical candidates, RPC1063 and RPC4046. RPC1063 is being tested as a potential best-in-class treatment for relapsing multiple sclerosis (RMS) and a potential first-in-class treatment for inflammatory bowel disease (IBD). RPC4046 being tested as a first-in-class treatment for a rare disease called Eosinophilic Esophagitis (EoE).
- With two drug candidates for large markets and a rare disease, we project Receptos has a multi-billion dollar pipeline and is likely to become an acquisition target.** We project RPC1063 for RMS launch in late 2018 and calculate peak annual sales could reach over \$2 billion. With a 2019 launch, we project peak annual sales for IBD could reach over \$850 million. For RPC4046 treatment of EoE, we project a 2019 launch and with premium pricing for an orphan drug, we project peak annual sales could reach over \$1 billion. With two potential blockbuster drug candidates, we believe Receptos is likely to become an acquisition target.
- 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 the anticipated mid-2014 release 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 believe clinical and commercial risks are reduced for RPC1063 and that it has potential to be best-in-class for RMS and first-in-class for IBD.** RPC1063 modulates sphingosine 1-phosphate 1 receptor (S1P1R) which controls the movement of inflammatory white blood cells and was established as a validated drug target for RMS by Novartis's Gilenya—the first once-daily, orally-dosed treatment for MS. Despite safety issues, we believe Gilenya's efficacy and oral delivery resulted in it achieving over \$1 billion in sales in 2012—which was only its second full year of launch. RPC1063 also has the orally-delivered once-daily attributes, but has pharmacological properties which may improve safety over Gilenya—while maintaining the same high level of efficacy—making it potentially best-in-class. The company has an ongoing Phase 2/3 trial with data release expected in mid-2014. For inflammatory bowel disease (IBD), the company is conducting a Phase 2 trial in ulcerative colitis (UC) with Phase 2 results also expected in mid-2014.
- RPC4046 provides Receptos with a first-in-class orphan drug development and commercialization opportunity.** RPC4046 is a monoclonal antibody targeting interleukin-18 (IL-13) which is involved in a rare allergic/immune disorder disease called Eosinophilic Esophagitis (EoE). Receptos plans to initiate a Phase 2 trial in H1 2014.
- Management's experience and past success reduces execution risk, in our view.** Faheem Hasnain is CEO and previously developed daclizumab for RMS as CEO of FACET, which was acquired by Abbott (NYSE: ABT) in April 2010. Not only does he have a great track record developing MS drug candidates, but he has surrounded himself with an impressive management team, which we believe reduce execution risk.
- We consider intellectual property to be strong due to composition of matter patent protection with potential for extended runway.** RPC1063 has CoM protection into 2029, with the potential to extend to 2032. RPC4046 has CoM

protection into 2028, with the potential to extend up to five years. With launches in 2018-2019, we see at least 9-10 years post-launch protection.

8. **In our view, RCPT at \$16 per share or about \$280 million market cap is at an attractive valuation compared with our fair value of \$23 per share or about \$400 million market cap.** 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, we calculate RCPT's current fair value at about \$400 million market capitalization or about \$23 per share—approximately 47% upside from the current \$16. With two potential blockbuster drug candidates, we believe Receptos is likely to become an acquisition target. We project a current take-out value at about \$500 million market cap or \$30 per share for RCPT. We consider peer comparables to include companies with oral treatments for multiple sclerosis such as Novartis (NVS:NYSE; not rated), Genzyme (SNY:NYSE; not rated) and Biogen-Idec (BIIB:Nasdaq; not rated) as well as companies in mid-stage clinical development for treatment of multiple large markets and orphan diseases such as Intercept (ICPT:Nasdaq; OUTPERFORM) and Lexicon Pharmaceuticals (LXRX:Nasdaq; OUTPERFORM).

**FIGURE 1: PIPELINE—BEST-IN-CLASS AND FIRST-IN-CLASS**

Product	Pre-Clinical	Phase 1	Phase 2	Phase 3	NDA Review	Launch
<b>RPC1063</b> Relapsing Multiple Sclerosis (RMS) Orally-delivered S1P1 Receptor Modulator	√	√	Ph2/3 ongoing Data mid:14		2018	YE 2018
<b>RPC1063</b> Ulcerative Colitis (UC) Orally-delivered S1P1 Receptor Modulator	√	√	Ongoing Data mid:14	Start 2015*	2018	2019
<b>RPC4046</b> Eosinophilic Esophagitis (EoE) Anti-IL13 Monoclonal Antibody	√	√	Start 2014	Start 2015*	2018	2019
<b>GLP-1R allosteric modulator</b> Type 2 Diabetes	Ongoing					

\*Wedbush estimates; Sources: Receptos and Wedbush Pacgrow Life Sciences

**We believe clinical and commercial risks are reduced for RPC1063 and that it has potential to be best-in-class and achieve multi-billion dollars in annual sales.** RPC1063 modulates sphingosine 1-phosphate 1 receptor (S1P1R), which controls the movement of inflammatory white blood cells and was established as a validated drug target for RMS by Novartis' Gilenya—the first once-daily, orally-dosed treatment for MS. Despite safety issues, we believe Gilenya's efficacy and oral delivery resulted in its achieving over \$1 billion in sales in 2012—which was only its second full year of launch. RPC1063 also has the orally-delivered once-daily attributes but has pharmacological properties which may improve safety over Gilenya—while maintaining the same high level of efficacy—making it potentially best-in-class.

RPC1063 is currently being tested in a Phase 2/3 clinical trial to treat relapsing multiple sclerosis (RMS) with top-line results expected in mid-2014. With successful clinical development and regulatory approval, we project launch in late 2018 and the potential to become best-in-class and achieve gross peak annual sales of over \$2 billion for RMS.

RPC1063 is also being tested in a Phase 2 trial for UC with results also expected in mid-2014. For IBD, we anticipate RPC1063 could become first-in-class and project launch in 2019 with peak annual sales potentially reaching over \$850 million.

**RPC4046 provides Receptos with an orphan drug and commercialization opportunity.** RPC4046 is a monoclonal antibody targeting interleukin-13 (IL-13) which is involved in a rare allergic/immune disorder disease called Eosinophilic Esophagitis (EoE). Receptos plans to initiate a Phase 2 trial in H1 2014.

For RPC4046, we anticipate a first-in-class launch in EoE in 2019 and the potential to reach over \$1 billion in peak annual sales with orphan drug premium pricing. In addition, the small patient population for a rare disease potentially allows Receptos to develop its own sales force.

**FIGURE 2: MODEL—WE PROJECT FULL-YEAR PROFITABILITY IN 2019**

Receptos, Inc. (NASDAQ: RCPT)						Wedbush Pac Grow Life Sciences					
Historical and Projected Income Statement (In thousands except per share data)						Liana Moussatos, Ph.D. Richard Lau					
	2012A	2013E				2014E	2015E	2016E	2017E	2018E	2019E
	FY:12A	Q1	Q2	Q3	Q4	FY:13E	FY:14E	FY:15E	FY:16E	FY:17E	FY:18E
Gross Sales											
RPC1063	-	-	-	-	-	-	-	-	-	\$ 7,215	\$ 229,723
RMS	-	-	-	-	-	-	-	-	-	7,215	175,010
IBD	-	-	-	-	-	-	-	-	-	-	54,713
RPC4046	-	-	-	-	-	-	-	-	-	\$ -	\$ 46,242
EoE	-	-	-	-	-	-	-	-	-	-	46,242
<b>Total Gross Sales</b>	<b>\$ -</b>	<b>\$ -</b>	<b>\$ -</b>	<b>\$ -</b>	<b>\$ -</b>	<b>\$ -</b>	<b>\$ -</b>	<b>\$ -</b>	<b>\$ -</b>	<b>\$ 7,215</b>	<b>\$ 275,965</b>
<b>Revenues:</b>										72%	70%
Net Product Sales	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ -	\$ 3,572	\$ 159,382
RPC1063	-	-	-	-	-	-	-	-	-	\$ 3,572	\$ 113,140
Grant Revenue	-	-	-	-	-	-	-	-	-	-	-
Collaborative Revenue	8,647	556	556	556	556	2,225	700	1,463	1,463	1,208	1,350
<b>Total Net Revenues</b>	<b>\$ 8,647</b>	<b>\$ 556</b>	<b>\$ 556</b>	<b>\$ 556</b>	<b>\$ 556</b>	<b>\$ 2,225</b>	<b>\$ 700</b>	<b>\$ 1,463</b>	<b>\$ 1,463</b>	<b>\$ 1,208</b>	<b>\$ 4,949</b>
<b>Cost and Expenses:</b>											
Cost of Goods	-	-	-	-	-	-	-	-	-	-	11,561
R&D	22,927	6,305	6,935	7,629	8,392	29,261	40,928	34,643	40,527	47,411	64,886
(S)G&A	3,430	892	936	993	1,062	3,883	5,050	5,972	8,875	21,638	43,182
Other	174,990	-	-	-	-	-	-	-	-	-	-
<b>Total Operating Expenses</b>	<b>\$ 106,977</b>	<b>\$ 7,197</b>	<b>\$ 7,872</b>	<b>\$ 8,622</b>	<b>\$ 9,454</b>	<b>\$ 33,144</b>	<b>\$ 45,978</b>	<b>\$ 40,615</b>	<b>\$ 49,402</b>	<b>\$ 69,050</b>	<b>\$ 119,628</b>
Operating Income (Loss)	(98,330)	(6,640)	(7,316)	(8,065)	(8,898)	(30,919)	(45,278)	(39,152)	(47,940)	(67,841)	(41,104)
Net Interest Income (Expense)/Other Income	-	8	32	50	44	133	109	2	(106)	(247)	(560)
Other Income (Expense)	-	-	-	-	-	-	-	-	-	-	-
<b>Income Before Income Taxes</b>	<b>\$ (98,330)</b>	<b>\$ (6,633)</b>	<b>\$ (7,283)</b>	<b>\$ (8,016)</b>	<b>\$ (8,854)</b>	<b>\$ (30,786)</b>	<b>\$ (45,169)</b>	<b>\$ (39,151)</b>	<b>\$ (48,046)</b>	<b>\$ (68,088)</b>	<b>\$ (40,544)</b>
Provision (Benefit) for Income Taxes	-	-	-	-	-	-	-	-	-	-	-
<b>Net Income (Loss)</b>	<b>\$ (98,330)</b>	<b>\$ (6,633)</b>	<b>\$ (7,283)</b>	<b>\$ (8,016)</b>	<b>\$ (8,854)</b>	<b>\$ (30,786)</b>	<b>\$ (45,169)</b>	<b>\$ (39,151)</b>	<b>\$ (48,046)</b>	<b>\$ (68,088)</b>	<b>\$ (40,544)</b>
<b>EPS (GAAP, Taxed, Diluted)</b>	<b>(\$10.22)</b>	<b>(\$0.37)</b>	<b>(\$0.41)</b>	<b>(\$0.45)</b>	<b>(\$0.50)</b>	<b>(\$1.72)</b>	<b>(\$2.52)</b>	<b>(\$2.17)</b>	<b>(\$2.65)</b>	<b>(\$3.73)</b>	<b>(\$4.79)</b>
Weighted Shares Outstanding (Basic and Diluted)	9,620	17,810	17,835	17,860	17,885	17,847	17,947	18,047	18,147	18,247	18,347
Total Shares Outstanding (Diluted)	9,620	18,342	18,367	18,392	18,417	18,379	18,479	18,579	18,679	18,779	18,879
Cash	\$5,427	\$19,430	\$83,590	\$75,018	\$65,608	\$65,608	\$19,739	(\$19,412)	(\$67,458)	(\$135,546)	(\$223,716)
Net Cash per share	\$0.56	\$1.09	\$4.69	\$4.20	\$3.67	\$3.68	\$1.10	(\$1.08)	(\$3.72)	(\$7.43)	(\$12.19)
Annual (Burn)/Generation	(\$5,909)					\$60,181	(\$45,869)	(\$39,151)	(\$48,046)	(\$68,088)	(\$88,170)

Source: Wedbush Pac Grow Life Sciences

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. 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. For both RPC1063 and RPC4046, we have modeled that Receptos will have a commercial partner and receive royalties outside of the US and a 50:50 co-promote in the US. Our model projections presume that RPC1063 and RPC4046 are approved by the FDA on the first round of NDA review. Cash runway includes the anticipated mid-2014 release of transforming top-line Phase 2 results testing RPC1063 treatment of RMS and IBD/UC.

**FIGURE 3: ANTICIPATED 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

Sources: Receptos and Wedbush Pac Grow Life Sciences



**FIGURE 4: RECEPTOS MANAGEMENT—QUALITY AND EXPERIENCE REDUCES EXECUTION RISK, IN OUR VIEW**

Name	Position and Past Experience
Faheem Hasnain	<b>President, Chief Executive Officer and Director.</b> Mr. Hasnain has served as President, CEO and a director of Receptos since November 2010. Prior to joining Receptos, Mr. Hasnain was the president and chief executive officer and a director of Facet Biotech Corporation (NASDAQ: FACT), a biology-driven antibody company with a focus in multiple sclerosis and oncology. He held that position from December 2008 until the company's acquisition by Abbott Laboratories in April 2010. Previously, Mr. Hasnain was president, chief executive officer and a director of PDL BioPharma, Inc. (NASDAQ: PDLI) from October 2008 until Facet Biotech was spun off from PDL BioPharma in December 2008. From October 2004 to September 2008, Mr. Hasnain served at Biogen Idec Inc., a biotechnology company specializing in neurological disorders, autoimmune disorders and cancer, most recently as executive vice president in charge of the oncology/rheumatology strategic business unit. Prior to Biogen Idec, Mr. Hasnain held roles with Bristol-Myers Squibb, where he was president of the Oncology Therapeutics Network, and for 14 years at GlaxoSmithKline and its predecessor organizations. Mr. Hasnain was appointed chairman of the board of Ambit Biosciences Corporation in November 2010 and serves on the board of directors of Somaxon Pharmaceuticals (NASDAQ: SOMX) and Aragon Pharmaceuticals. He has been chairman of the board of Sente, Inc. since 2008 and served as a member of the board of directors of Tercica, Inc. Mr. Hasnain received a B.H.K. and B.Ed. from the University of Windsor Ontario in Canada.
Graham Cooper	<b>Chief Financial Officer.</b> Mr. Cooper has served as Chief Financial Officer of Receptos since February 2013. During 2012, he was the Executive Vice President, Finance and Chief Financial Officer of Geron Corporation (NASDAQ: GERN), an oncology-focused biopharmaceutical company. From 2006 to 2011, Mr. Cooper held the position of Senior Vice President, Chief Financial Officer and Treasurer of Orexigen Therapeutics (NASDAQ: OREX), a biotechnology company focused on obesity. From 1999 to 2006, he held positions of increasing responsibility at Deutsche Bank Securities including Director, Health Care Investment Banking. From August 1992 to January 1995, Mr. Cooper was an accountant at Deloitte & Touche, where he earned his C.P.A. He received a B.A. in Economics from the University of California at Berkeley and an M.B.A. from the Stanford Graduate School of Business.
Marcus F. Boehm, Ph.D.	<b>Chief Technology Officer and Co-Founder.</b> Dr. Boehm has served as Chief Technology Officer of Receptos since May 2009 and is a co-founder. He brings extensive medicinal chemistry, drug discovery and development expertise in the fields of oncology, inflammation and metabolic diseases. From 2006 to 2009, Dr. Boehm held the position of Senior Director of Chemistry at Biogen Idec with responsibility for multiple medicinal chemistry programs and head of chemistry for the San Diego site. Dr. Boehm formerly served as Vice President of Chemistry at Conformia Therapeutics until its \$250 million acquisition by Biogen Idec in 2006. While at Conformia, he directed chemistry efforts resulting in the discovery of CNF1010 and CNF2024, Hsp90 inhibitors in the clinic for oncology indications. Prior to joining Conformia, Dr. Boehm held various positions with progressing responsibility in medicinal chemistry at Ligand Pharmaceuticals. There he successfully led chemistry efforts on multiple intracellular receptor (IRs) programs resulting in the discoveries of Panretin®, Targretin® and LGD1550 retinoids. Dr. Boehm has over 100 patents and publications in the fields of intracellular receptors, Hsp90 and kinase chemistry. He received a B.A. in Chemistry from the University of California, San Diego, a Ph.D. in Chemistry from State University of New York Stony Brook and completed a National Institutes of Health Postdoctoral Fellowship at Columbia University.
Sheila Gujrathi, M.D.	<b>Chief Medical Officer.</b> Dr. Gujrathi has served as Chief Medical Officer of Receptos since April 2011. She joined Receptos from Bristol-Myers Squibb where she was Vice President of the Global Clinical Development Group in Immunology from 2008 to 2011. In that role, she was responsible for late-stage clinical development and global regulatory submissions for Orencia® (both intravenous and subcutaneous formulations), Nulojix® in solid organ transplant, and other clinical immunology assets being developed in rheumatoid arthritis, systemic lupus erythematosus, inflammatory bowel disease, solid organ transplant, scleroderma, and other immunology indications. In addition, she provided strategic direction for the Immunoscience franchise at BMS for both the discovery and early development internal pipeline as well as external business development opportunities where she was involved in licensing agreements and/or acquisitions of several immunology and fibrosis compounds both in the discovery and clinical stages of development. Prior to joining BMS, Dr. Gujrathi was a management consultant at McKinsey & Company from 2002 to 2008 in the healthcare practice where she provided strategic advice on a variety of projects in the healthcare and pharmaceutical industry. From 1999 to 2002, she served at Genentech where she held roles of increasing responsibility in the Immunology, Tissue Growth and Repair clinical development group. She worked on a number of clinical programs in the immunology area including Xolair® in IgE-mediated asthma, Rituximab® and ocrelizumab in multiple autoimmune disorders, Raptiva®, and other early development compounds.

	where she was involved in filing INDs and initiating early clinical trials. At Genentech, she also held the role of Avastin® Franchise Team Leader helping to develop strategies in multiple oncology indications. She received her B.S. with highest distinction in Biomedical Engineering and M.D. from Northwestern University in their accelerated Honors Program in Medical Education. She completed her Internal Medicine Internship and Residency at Brigham and Women's Hospital, Harvard Medical School and is board certified in internal medicine. She received additional training at UCSF and Stanford in their Allergy and Immunology Fellowship Program.
Robert J. Peach, Ph.D.	<b>Chief Scientific Officer and Co-Founder.</b> Dr. Peach has served as Chief Scientific Officer of Receptos since May 2009 and is a Co-Founder. Dr. Peach came to Receptos with 19 years of pharmaceutical and biotechnology company experience, successfully discovering and developing drugs in the areas of autoimmunity/inflammation and oncology. In his most recent position from 2000 to 2007, Dr. Peach was Senior Director of Oncology Discovery at Biogen Idec where he had responsibility for several bicoastal research programs, two of which have entered clinical trials. Dr. Peach was also responsible for leading technical due diligence efforts that lead to a 3-compound co-development deal with Protein Design Labs, acquisition of Conforma Therapeutics, and a venture investment in Globelimmune including a subsequent appointment to their Scientific Advisory Board. Prior to joining Biogen Idec, Dr. Peach held several research positions with increasing responsibility at Bristol-Myers Squibb from 1991 to 2000. During that time he played an integral role in the research and development of Orencia®, now registered for the treatment of rheumatoid arthritis. He also led a program that discovered and developed belatacept (Nulojix®), a drug recently approved for the treatment of solid organ transplant rejection. Dr. Peach has authored over 60 peer-reviewed publications and 11 patents. He received his B.S. and M.S. (1st class honors) from the University of Canterbury and a Ph.D. in Biochemistry from the University of Otago, New Zealand.
Chrysa Mineo	<b>Vice President, Corporate Development.</b> Ms. Mineo has served as Vice President, Corporate Development of Receptos since July 2009. Ms. Mineo came to Receptos with over 20 years of experience in the biotechnology industry. In her most recent position, from 1997 to 2009 Ms. Mineo was Senior Director of Business Development for Neurocrine Biosciences, where she spent 12 years specializing in both world-wide and regional development and commercialization collaborations for Neurocrine's GPCR directed therapeutic candidates, predominantly in the areas of CNS, endocrinology, and metabolism. She also led technical due diligence and negotiations for several successfully in-licensed candidates in both preclinical and clinical development. Ms. Mineo established multiple technology collaborations for Neurocrine to support the acceleration of drug discovery efforts in the GPCR field, including in the areas of receptor screening, computational chemistry, and GPCR targeted screening libraries. Prior to Neurocrine, Ms. Mineo served in various capacities in research, marketing, and business development for such companies as Amgen, DNAX Research Institute, Schering Plough, and Baxter Biotech. She began her career in 1987 with Amgen as a member of a cellular biology team investigating the therapeutic role of Neupogen. Ms. Mineo holds a B.S. in zoology from the University of California, Davis and received her M.B.A. from Duke University's Fuqua School of Business.
James R. Schmidt, CPA	<b>Vice President, Finance and Administration.</b> Mr. Schmidt has served as Vice President, Finance and Administration of Receptos since May 2009. He had extensive finance and operations experience prior to joining Receptos. He was formerly Senior Director of Finance and Operations at Conforma Therapeutics from 2001 to the successful acquisition by Biogen Idec in 2006 where he assisted in the transition and integration of the companies. Prior to that, from 1986 to 2001 Mr. Schmidt served in various financial and operational roles including Chief Financial Officer for Kent SeaTech Corporation, Controller for Medical Imaging Centers of America, Inc., MCA, Inc./MCA Concerts, Inc. and Manager of Accounting - Retirement Inns of America, Inc. He has managed the finances, human resources, information technology and facilities of companies from start-up to maturity. He started his career with Coopers & Lybrand and received his B.S. in Accounting and Corporate Finance from Drake University in Des Moines, Iowa.

Sources: Receptos and Wedbush Pacgrow Life Sciences

**Execution risk is reduced by management's quality and experience.** Faheem Hasnain is CEO and previously developed daclizumab for RMS as CEO of FACET which was acquired by Abbott (NYSE: ABT) in April 2010. Not only does he have a great track record developing MS drug candidates, but he has also surrounded himself with an impressive management team which we believe reduces execution risk.

**FIGURE 5: INTELLECTUAL PROPERTY—LONG RUNWAY FOR COMPOSITION OF MATTER PROTECTION**

Number (Status)	Title	Priority/Filing Date	Source
<b>RPC1063</b>			
WO09/151529 (converted) US2010/0010001 (pending) Plus expedited U.S. cases (allowed)	NOVEL MODULATORS OF SPHINGOSINE PHOSPHATE RECEPTORS	May 2009	Exclusive License from TSRI
WO2011/060392 (converted) US2011/0172202 (allowed)	SELECTIVE SPHINGOSINE 1 PHOSPHATE RECEPTOR MODULATORS AND METHODS OF CHIRAL SYNTHESIS	Nov 2010	Receptos
WO2011/060389 (converted) US2011/0178056 (allowed)	SELECTIVE SPHINGOSINE 1 PHOSPHATE RECEPTOR MODULATORS AND METHODS OF CHIRAL SYNTHESIS	Nov 2010	Receptos
WO2011/06039 (converted) US2011/0183953 (pending)	SPHINGOSINE 1 PHOSPHATE RECEPTOR MODULATORS	Nov 2010	Receptos
WO2012/158550 (pending)	SELECTIVE HETEROCYCLIC SPHINGOSINE 1 PHOSPHATE RECEPTOR MODULATORS	May 2012	Receptos
<b>RPC4046</b>			
US 7,915,388 US13/043,293 (pending)*	INTERLEUKIN-13 BINDING PROTEINS	Sep 2006	Development and License Option from AbbVie

\*Australia, Canada, China, Europe, Japan, Mexico, Norway, Korea, Russia, Costa Rica and PCT application  
Sources: Receptos and Wedbush Pacgrow Life Sciences

**We consider intellectual property to be strong due to having a long runway for Composition of Matter protection.**

**RPC1063: Composition of Matter expiration in 2029 may be extendable to 2032.** Receptos's intellectual property rights for RPC1063 include composition of matter and method of use protection from two issued US and seven pending US and foreign patents and applications exclusively licensed from The Scripps Research Institute (TSRI) patents. Protection lasts through May 2029 and is potentially extendable to 2032 with a 2018 launch and three-year patent term extension. Receptos owns additional IP for RPC1063 metabolites (US) and alternate scaffold for potential back-ups (US) expiring after November 2030. Foreign patent applications include a pending Patent Cooperation Treaty (PCT) application as well as applications pending or patents issued in Canada, Europe, Japan, Australia, Mexico, Eurasia, South Korea, China, New Zealand, Malaysia, Philippines, Singapore, Brazil, India, Israel, and South Africa. Presuming RPC1063 achieves regulatory approval, the term of the composition of matter patent in the US (if issued) may be extended for 3-5 additional years under the provisions of the Drug Price Competition and Patent Term Restoration Act of 1984 (Hatch-Waxman Act). Similar extensions may be possible in foreign countries after regulatory approval. Overall, the other patents expire from 2030 to 2032.

**RPC4046: The earliest expiration for RPC4046 composition of matter is 2028 and may be eligible for up to five years extension.** Intellectual property rights for RPC4046 are covered through a development and license option from AbbVie and include an issued US patent (US7915388) and a pending US patent (US13/043.293) as well as pending patents in Europe, Japan, China, Canada, Australia, Mexico, Norway, Korea, Russia, Costa Rica, and a PCT application.

The patent portfolio for RPC4046, which is in-licensed from AbbVie, contains issued patent and pending patent applications for compositions of matter and certain methods of use. In addition to the issued US patent and pending US patent application, there are corresponding foreign pending patent applications in Europe, Japan, China, Canada, Australia, Mexico, Norway, Korea, Russia, and Costa Rica. We expect the issued composition of matter patent in the US to expire in 2028. With regulatory approval, it is possible for the term of the composition of matter patent in the US to be extended for up to five years under the Hatch-Waxman Act.



Receptos's use of RPC4046 intellectual property is limited by AbbVie's consent and whether AbbVie exercises its option for a global collaboration. In this case, Receptos would instead receive an exclusive worldwide license to RPC4046 and AbbVie retains the decision to pursue an extension. If issued, in certain foreign countries pending patent applications are expected to expire in 2027 and may be extended with AbbVie's permission upon regulatory approval.

## EARLY STAGE PROGRAMS

**GLP-1R PAMs: Composition of matter expiration is from 2031-2032.** Intellectual property protection for the glucagon-like peptide-1 receptor (GLP-1R) positive allosteric modulators (PAMs) contains patent applications for compositions of matter for chemical scaffold and methods of use. These include two pending US and ex-US applications. If issued, the US and ex-US composition of matter patents expire from 2031-2032, but could be extended up to 5 years for Hatch-Waxman if FDA approved.

**GPCR program: Composition of matter expiration is from 2028-2032.** The glycoprotein coupled receptor (GPCR) program was in-licensed from The Scripps Research Institute (TSRI) and includes patents for structure determination as well as methods and compositions for crystals of GPCRs. The license covers a patent and patent applications for methods and compositions for high-resolution crystals of GPCRs. Receptos has exclusive commercial license rights from TSRI to a US patent, two pending US patent applications, a PCT application and foreign patent applications in Canada, Europe and Japan related to GPCR structure determination. The patent and applications are expected to expire from 2028 to 2032.

## VALUATION

**FIGURE 6: WEDBUSH RCPT FAIR VALUE--\$400 MILLION MARKET CAPITALIZATION**

RCPT Product Pipeline Valuation		Eligible # Patients	Pricing \$ / Patient / Year	Gross Peak Sales WW (\$000)	Net Peak Revs (\$000)	Revs Year	Peak Penetration	Multiple	Launch	Discount Rate	MktCap Fair Value (\$000)	Stock Fair Value
Product	Indication											
RPC1063	RMS	910,714	\$22,896	\$2,324,492	\$991,012	2022	12%	5	12/18/2018	30%	\$304,627	\$17.30
RPC1063	IBD	375,000	\$22,896	\$852,670	\$354,768	2023	12%	5	1/15/2019	30%	\$106,849	\$6.07
RPC4046	EoE	257,250	\$43,803	\$1,563,202	\$82,108	2023	12%	4	6/15/2019	30%	\$17,722	\$1.01
We use multiples to account for clinical and regulatory risk at various stages of development.			Total Peak Revs:	\$4,740,364	\$1,427,889			5/31/13				
1x: in preclinical testing	6x: passed Phase 2 / in Phase 3 / positive Phase 3											
2x: passed preclinical	8: regulatory review											
3x: IND filing accepted	9: approved											
4x: In Phase 1	10: launched											
5x: In Phase 2												
Late Stage Products Fair Value									Stock	MktCap	Upside Potential	
Current Quarter's Est Net Cash (000):									\$5.17	\$95,314		
Total Technology Value									\$24.38	\$429,198	54%	
Total RCPT Value:									\$29.55	\$524,512	88%	
Current RCPT Value:									\$15.87	\$279,386		

Sources: Receptos and Wedbush Pacgrow Life Sciences

In our view, RCPT at \$16 per share or about \$280 million market cap is at an attractive valuation compared with our fair value of \$23 per share or about \$400 million market cap. 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, we calculate RCPT's current fair value at about \$400 million market capitalization or about \$23 per share compared with the current \$16 per share or approximately \$280 million market cap, with approximately 47% upside to our fair value estimate.

We consider peer comparables to include companies with oral treatments for multiple sclerosis such as Novartis (NVS:NYSE; not rated), Genzyme (SNY:NYSE; not rated) and Biogen-Idec (BIIB:Nasdaq; not rated) as well as companies in mid-stage clinical development for treatment of multiple large markets and orphan diseases such as Intercept (ICPT:Nasdaq; OUTPERFORM) and Lexicon Pharmaceuticals (LXRX:Nasdaq; OUTPERFORM).

**With two drug candidates for large markets and a rare disease, we project Receptos has a multi-billion dollar pipeline and is likely to become an acquisition target.** We project RPC1063 for RMS launch in late 2018 and calculate peak annual sales could reach over \$2 billion. With a 2019 launch, we project peak annual sales for IBD could reach over \$850 million. For RPC4046 treatment of EoE, we project a 2019 launch and with premium pricing for an orphan drug, we project peak annual sales could reach over \$1 billion. With two potential blockbuster drug candidates, we believe Receptos is likely to become an acquisition target. We project a current take-out value at about \$500 million market cap, or about \$30 per share for RCPT.

## RISKS TO ATTAINMENT OF OUR FAIR VALUE INCLUDE:

**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.

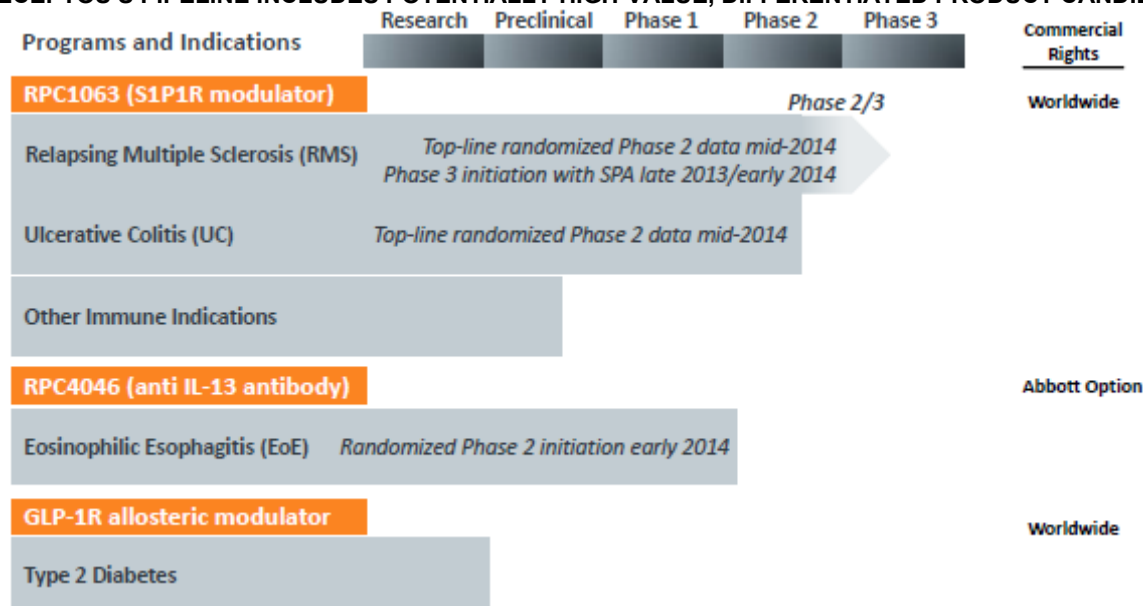
**Commercialization Risk:** We consider commercialization risk to be low in 2013, but higher than average in general due to Receptos's small size and development stage. Receptos's business model is to develop and commercialize clinical candidates; however, for small development-stage companies, we view 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.

FIGURE 7. RECEPTOS'S PIPELINE INCLUDES POTENTIALLY HIGH-VALUE, DIFFERENTIATED PRODUCT CANDIDATES



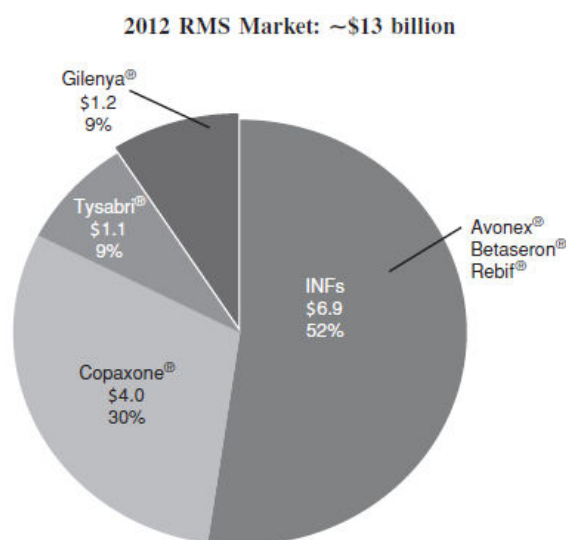
Source: Receptos

#### RPC1063 (S1P1R MODULATOR) IN RELAPSING MULTIPLE SCLEROSIS (RMS)

**Relapsing multiple sclerosis (RMS) is the most frequent clinical presentation of multiple sclerosis (MS).** MS is a chronic central nervous system disorder that affects the brain, spinal cord, and optic nerves. RMS is characterized by recurrent acute exacerbations of neurological symptoms followed by periods of recovery with clinical stability between relapses. About half of relapses result in incomplete recovery of function leaving permanent damage that accumulates over time. The autoimmune disease is driven by lymphocytes that attack surrounding nerve cells which results in nerve damage. Symptoms include neurological dysfunction, numbness, difficulty walking, visual loss, lack of coordination, and muscle weakness. The majority of RMS patients are diagnosed between the ages of 20 and 40 (peak 29-30) with a higher prevalence in females than males (2 to 1). The life span for MS patients is typically shortened by about 10 years. There are an estimated 500,000 RMS patients worldwide

**In 2012, the branded RMS market was estimated to be about \$13 billion worldwide.** Sales were comprised mainly of interferons (Avonex, Betaseron, and Rebif), Copaxone, Gilenya, Tysabri, and Aubagio which accounted for \$6.9 billion, \$4 billion, \$1.2 billion, \$1.1 billion and \$9 million in sales, respectively (Figure 8). Furthermore, the market is expected to grow to \$16 billion by 2018.

FIGURE 8. BRANDED RMS MARKET



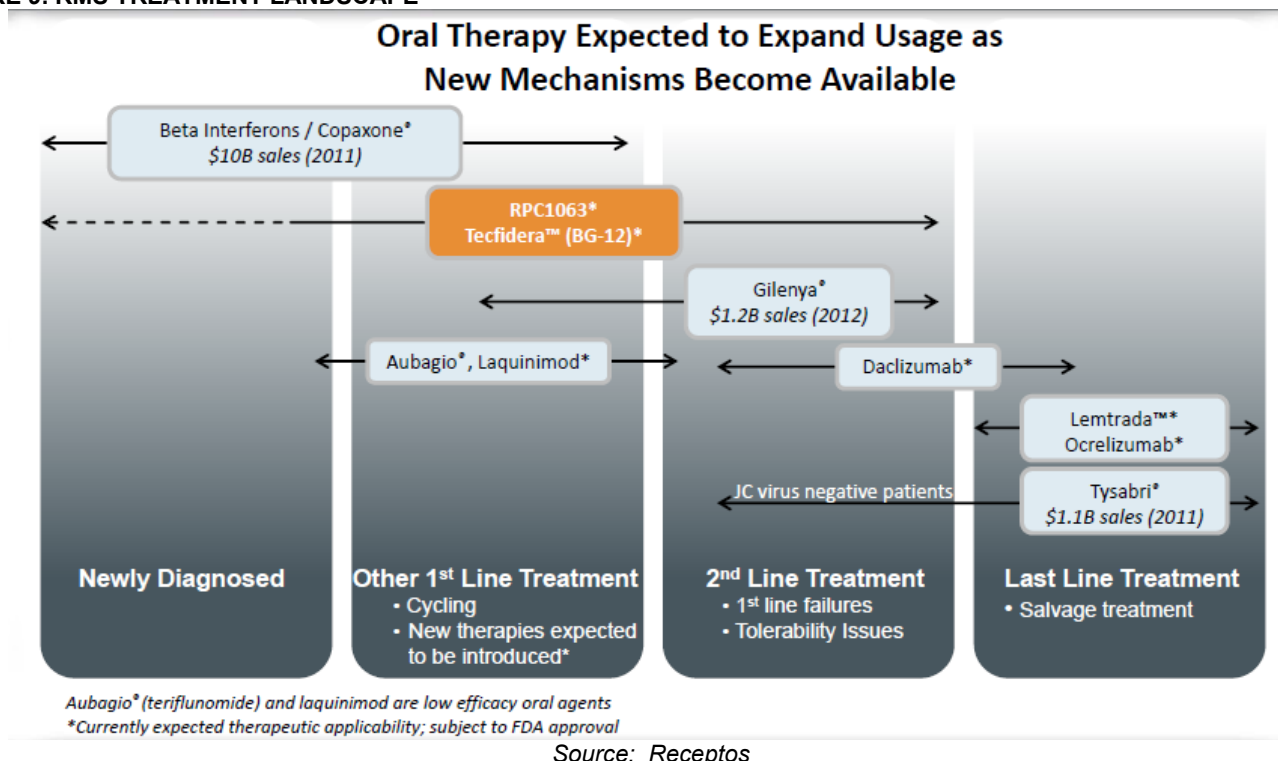
Note: Aubagio® sales were \$9M for 2012

(\$ in billions)

**Although there are multiple treatment options available for RMS, there remains a large unmet need for safer, more convenient yet efficacious drugs.** Collectively, Avonex, Betaseron, Copaxone, and Rebif are often referred to as the ABCRs and are usually used as first line treatment. Newly diagnosed RMS patients typically cycle through the ABCRs for as long as they remain controlled and responsive to therapy. In terms of efficacy, ABCR therapy results in about a 30% reduction in ARR. However, the interferon-betas are associated with flu-like side effects and require weekly to every-other day injections. Copaxone is not associated with flu-like symptoms, but requires daily injections. Inevitably, patients become unresponsive to ABCR therapy and their disease progresses. These second-line patients have been the primary market for Gilenya so far; however, we believe future safer oral medications, such as RPC1063, could also penetrate first-line patients. We note that Tysabri which has shown good efficacy (ARR reduction of 67%) is usually reserved for last line salvage treatment due to the risk of serious side effects including the potentially fatal disease, progressive multifocal leukoencephalopathy (PML).

Source: Receptos

FIGURE 9. RMS TREATMENT LANDSCAPE



**There are currently three approved oral therapies including Gilenya, which sold \$1.2 billion in its second year (2012) of launch.** Gilenya (Novartis) is a non-selective S1PR modulator that hits four of the five receptors: S1P1R, S1P3R, S1P4R, and S1P5R. The 0.5 mg once-daily oral dose has shown ARR reductions of 54 – 60% versus placebo and 52% versus Avonex. However, due to the non-selective nature of Gilenya, it is associated with significant risks, most notably, slowing of the heart rate (bradyarrhythmia), atrioventricular (AV) block, infection, macular edema, respiratory effects, hepatic effects, fetal risk, blood pressure effects and immune system effects. As we describe in detail below, we believe RPC1063 has the potential to significantly improve upon the safety profile of Gilenya, while maintaining similar efficacy.

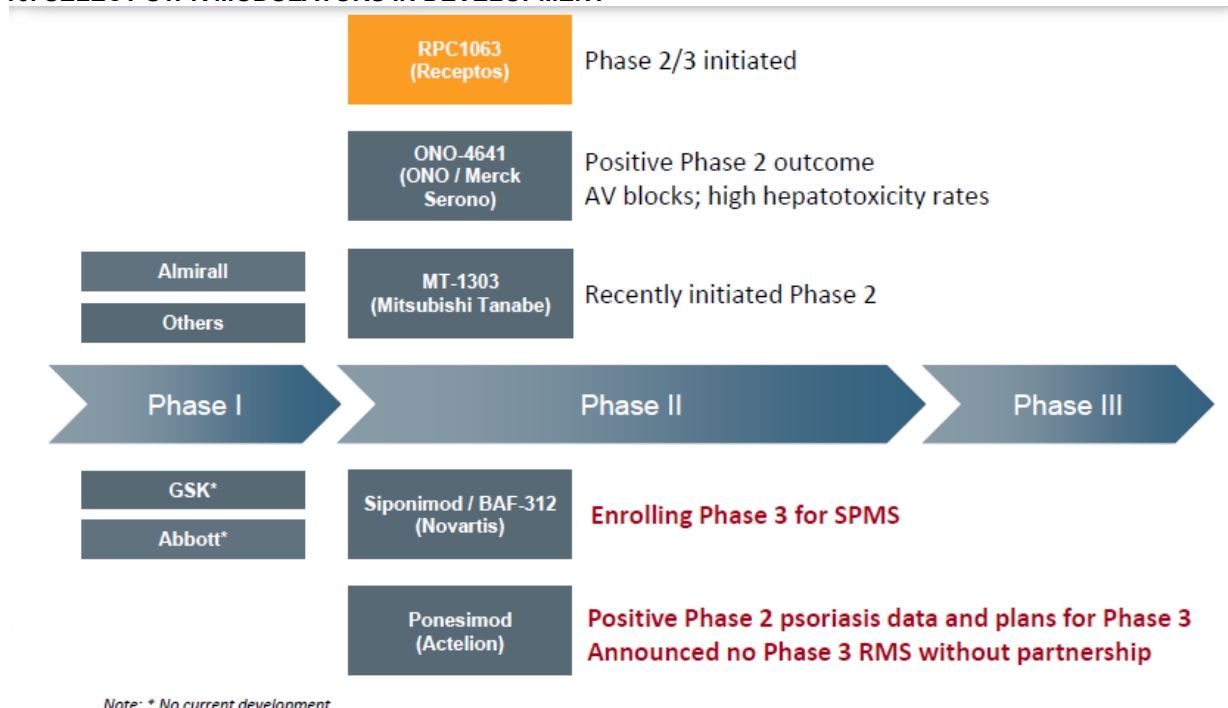
In September 2012, Aubagio (Genzyme) was approved as a first line treatment for RMS. In terms of efficacy, Aubagio is an immunomodulator that has shown ARR reductions of 31% versus placebo. In terms of safety, Aubagio carries a black box warning for both hepatotoxicity and teratogenicity, and common side effects include risk of liver enzyme increases, hair loss, diarrhea, influenza, nausea and paresthesia. We believe sales of Aubagio (\$9 million in 2012) have been lackluster due mainly to its low efficacy.

On March 27, 2013, Tecfidera (formerly BG-12; Biogen Idec) was approved for the treatment of RMS. Tecfidera is a dimethyl fumarate compound that does not work through S1PR modulation. In Phase 3 trials, twice daily Tecfidera showed ARR reductions of 44% and 53% versus placebo. Furthermore, reduction in GdE lesions were 73% and 57% compared to placebo. Common adverse events include flushing (40%), abdominal pain (18%), diarrhea (14%), nausea (12%), and vomiting (9%).

**There could be an additional four new products on the market by 2018; however, we do not believe any are that competitive with RPC1063.** Regulatory filings have been submitted for Lemtrada (injectable; Sanofi) and laquinimod (oral; Teva). In addition, there are two Phase 3 drug candidates that could be approved by 2018: daclizumab (injectable; Biogen Idec/AbbVie) and ocrelizumab (IV infused therapy; Genentech). To our knowledge, laquinimod, the only oral agent among these drug candidates, has shown rather modest efficacy results to date. Furthermore, we believe the new injectable and infused therapies will likely be used after oral agents such as RPC1063.

**We believe the number of companies pursuing improved S1PR modulators validates the need for a safer Gilenya. Importantly however, RPC1063 appears to have the best profile to date, in our view.** Other S1PR modulators in development include ONO-4641 (Ono/Merck), MT-1303 (Mitsubishi Tanabe), Siponimod (Novartis), and Ponesimod (Actelion). Figure 10 provides a comparison and status of S1PR modulators in development.

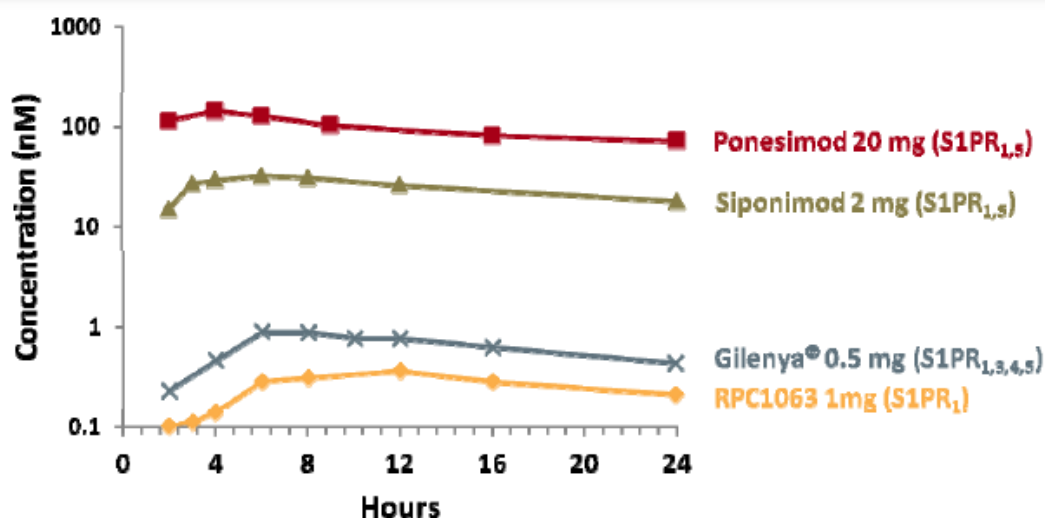
FIGURE 10. SELECT S1PR MODULATORS IN DEVELOPMENT



Source: Receptos

We believe the intrinsic properties of RPC1063 are likely to lead to a better safety profile while maintaining similar efficacy compared to Gilenya. At doses resulting in about 70% lymphocyte count reduction, RPC1063 appears to have the lowest peak plasma concentration ( $C_{max}$ ) and total drug exposure (AUC), less rapid absorption ( $T_{max}$ ), and higher volume of distribution compared to Gilenya, ponesimod and siponimod (Figure 11). We believe these beneficial properties of RPC1063 are likely to confer a more favorable safety profile.

FIGURE 11. PHARMACEUTIC PROPERTIES OF SELECT S1PR MODULATORS



Source: Receptos

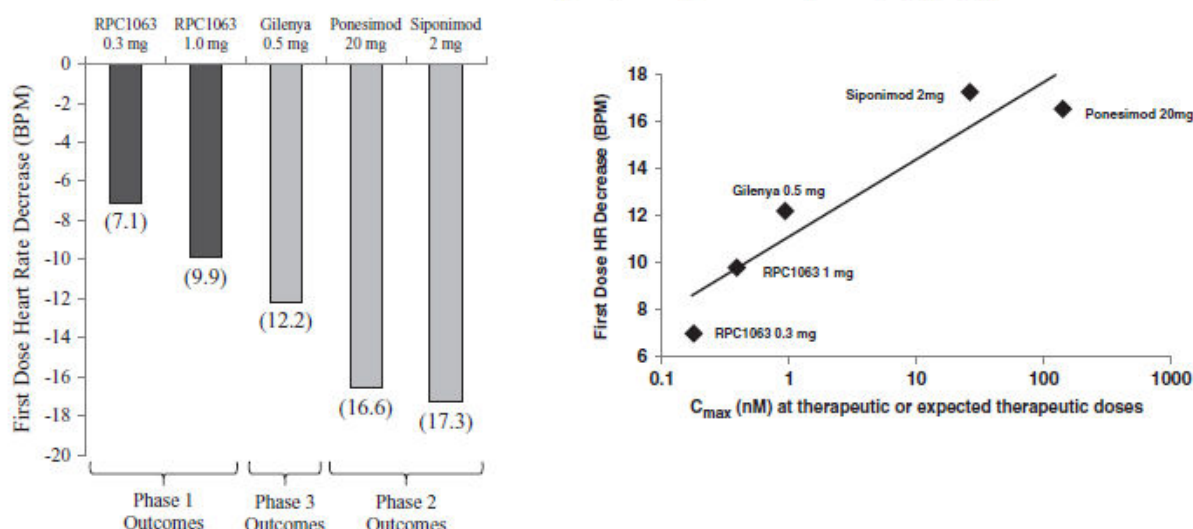
We believe RPC1063 has the potential to significantly improve upon the cardiac safety profile of Gilenya. Upon initial treatment, S1PR modulators cause a dose-dependent transient drop in heart rate. As such, the prescribing information for Gilenya requires six hours of cardiac monitoring upon first dose to observe patients for potential cardiovascular side effects such as bradyarrhythmia and AV blocks. As illustrated in Figure 12, the first dose heart rate decrease is correlated to the  $C_{max}$  of the drug, with RPC1063 outperforming the other S1PR modulators. In addition, Receptos will utilize a dose titration strategy to further minimize the first dose heart rate effect. Despite having a dampened heart rate effect compared to Gilenya, we currently anticipate RPC1063 will still carry the requirement for



first dose heart rate monitoring given it is likely a drug-class effect. However, we believe this requirement could eventually be lifted by the FDA once RPC1063's safety profile has been established on the market, similar to what was seen with Gilead's Letairis.

**FIGURE 12. FIRST DOSE HEART RATE DECREASE**

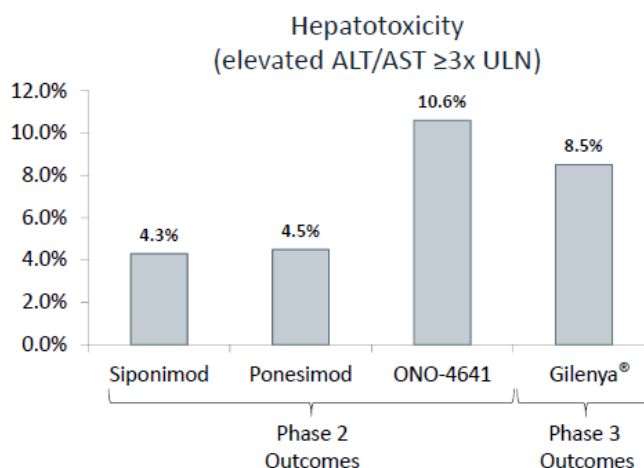
**Observed First Dose Heart Rate (HR) Decrease at Approximately 70% Lymphocyte Count Reduction For Gilenya® and other S1PR Modulators in Development (without dose titration)**  
*RPC1063 Has Lowest First Dose Impact, Likely Driven by PK ( $C_{max}/T_{max}$ )*



Source: Receptos

**We are optimistic that RPC1063 could result in reduced hepatotoxicity compared to Gilenya as well as other S1PR modulators.** In the preclinical toxicology program for RPC1063, there was no hepatotoxicity signals observed for up to nine months at doses up to 150-200-fold above the pharmacologically relevant dose. Furthermore, there was no evidence of hepatotoxicity seen in the Phase 1 study. By comparison, the Gilenya prescribing information notes that 8% of patients experience liver enzyme elevations  $\geq 3x$  the upper limit of normal. In addition, siponimod, ponesimod, and ONO-4641 all have shown some increase in liver enzymes (Figure 13).

**FIGURE 13. HEPATOXICITY PROFILE**



RPC1063 to date no elevated liver transaminases observed either preclinically (9-month dosing) or in Phase 1 healthy volunteer study (28-day dosing)

Source: Receptos

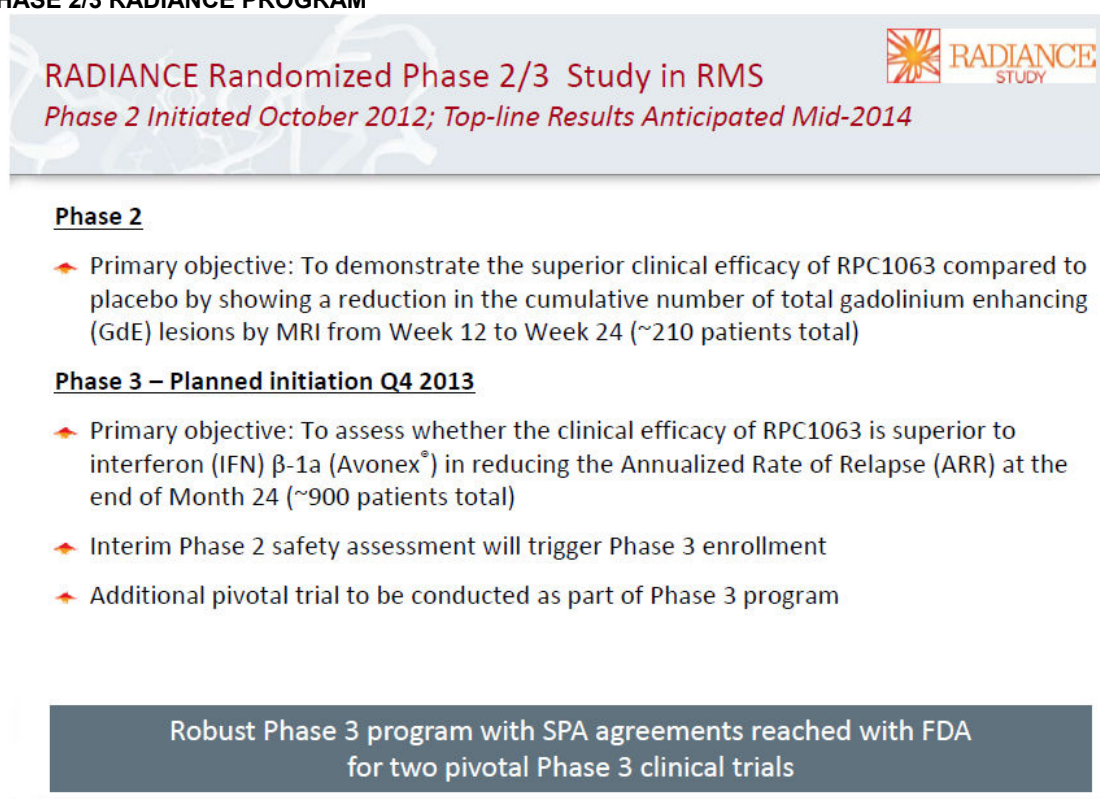
## Phase 2/3 RADIANCE Program

The company is currently enrolling the Phase 2 portion of a randomized Phase 2/3 study. It expects to complete enrollment in H2:13 and initiate the Phase 3 portion in late 2013 or early 2014. Top-line data from the Phase 2 portion is anticipated in mid-2014. In addition to the Phase 3 portion of RADIANCE, the company will also conduct an additional Phase 3 pivotal trial. We note that SPA agreements have been reached with the FDA for the two Phase 3 trials.

The Phase 2 portion of RADIANCE is a randomized, double-blind, placebo-controlled study designed to enroll up to 210 RMS patients. The trial will compare doses of 0.5 mg and 1.0 mg of RPC1063 against placebo. The primary endpoint is reduction in the cumulative number of total GdE lesions as measured by MRI from week 12 to week 24. We note that the doses were chosen based on exposure-response modeling done by the company. The 0.5 mg and 1.0 mg doses are projected to lead to approximately 50% and 70% lymphocyte count reductions, respectively.

The Phase 3 portion of RADIANCE is a randomized, double-blind, double-dummy, active-control study designed to enroll up to 900 patients. The trial will compare doses of 0.5 mg and 1.0 mg of RPC1063 against Avonex (30 µg intramuscular injection). The primary endpoint is reduction in annualized relapse rate (ARR) at month 24 (superiority vs. Avonex).

### FIGURE 14. PHASE 2/3 RADIANCE PROGRAM



Source: Receptos

## Phase 1 Clinical Data

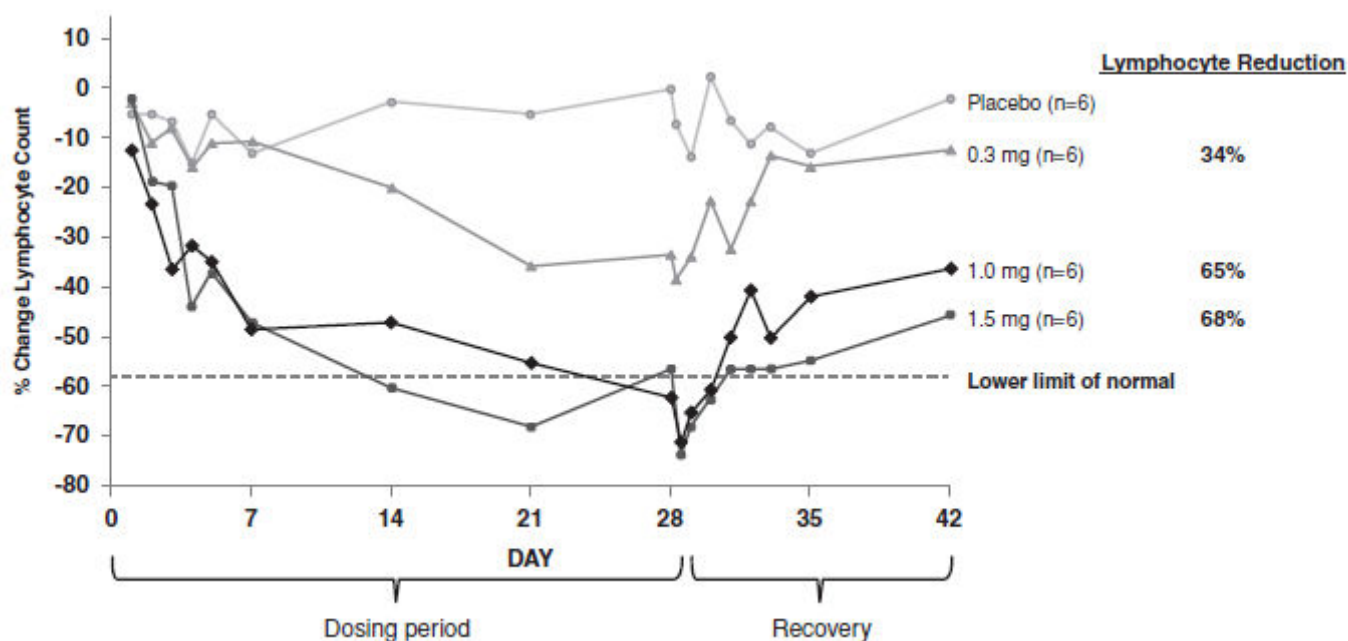
We believe the Phase 1 trial design was particularly robust as it included multiple doses as well as an important pharmacodynamic marker measurement. RPCS-001 was a single-center, randomized, double-blind, placebo-controlled, single and multiple dose escalation Phase 1 study. The primary goals of the study were to evaluate safety, tolerability, pharmacokinetic (PK) and pharmacodynamic (PD) effects of RPC1063 administered orally to 88 healthy adult volunteers (68 patients received RPC1063). The trial consisted of four parts: A) RPC1063 single doses of 0.3 to 3.0 mg; B) RPC1063 multiple doses of 0.3 to 2.0 mg for seven days; C) RPC1063 multiple doses of 0.3 to 1.5 mg for 28 days; and D) RPC1063 multiple daily doses in a dose titration regimen from 0.3 to 2.0 mg for 10 days.

In our view, no major red flags were raised by the Phase 1 results. Overall, the study was successful in demonstrating that RPC1063 treatment resulted in good safety and tolerability, linear PK, and dose-dependent reduction in lymphocyte count. Below, we detail the key findings that may differentiate RPC1063 versus other S1P1R modulators as well as lead to a successful Phase 2/3 study.

**RPC1063 treatment resulted in lymphocyte count reduction on par with other S1P1R modulators. However, lymphocyte recovery occurred more rapidly, which theoretically, should allow for better management of infections and retreatment decisions – an important distinction versus Gilenya.** There was a dose-dependent reduction in lymphocyte count following multiple dose administration for 28 days, with lymphocyte count continuing to decrease throughout the RPC1063 treatment period and appearing to reach steady state by day 28. The median decreases in lymphocyte count on day 28 were 34%, 65%, and 68% with dose levels of 0.3 mg, 1.0 mg, and 1.5 mg, respectively (Figure 15). We view these levels of reduction as promising given similar results produced by other S1PR modulators have been predictive of positive clinical outcomes.

Upon completion of the 28-day dosing period, circulating lymphocyte levels returned to above lower limit of normal within three days in all patients (Figure 15). These findings are likely attributable to the short half-life of RPC1063 (~19 hours) and is an important differentiating factor versus Gilenya (~168 hours). Of note, in Gilenya-treated patients, it can take four to eight weeks for lymphocyte counts to return to the normal range. From a treatment standpoint, a faster lymphocyte recovery time is desirable because it allows physicians to better manage adverse events as well as restart treatment sooner. We believe this will be one of the key selling points for RPC1063 to distinguish it as a potential best-in-class S1P1R modulator.

**FIGURE 15. PHASE 1 LYMPHOCYTE COUNT REDUCTION**



Sources: Receptos

**Lymphocyte count reduction is a pharmacodynamic marker that is predictive of positive clinical outcomes as measured by reduction in total gadolinium enhancing (GdE) lesions and annualized relapse rate (ARR). Therefore, we see a high probability of success in the ongoing Phase 2/3 study based on the Phase 1 results as well as data from other S1PR modulators.** Studies of other S1PR modulators, including Gilenya, have shown that lymphocyte count reductions in the range of 50 – 70% are correlated with clinical efficacy as measured by reduction in GdE lesions (MRI measurement/Phase 2 primary endpoint). Furthermore, lymphocyte count reductions in the range of 60 – 70% are correlated to longer-term clinical outcomes as measured by ARR (Phase 3 primary endpoint). Therefore, we believe the 65% and 68% (1.0 mg and 1.5 mg doses, respectively) reductions in lymphocyte count seen in the Phase 1 trial significantly de-risks the ongoing Phase 2/3 study.

FIGURE 16. CORRELATION BETWEEN LYMPHOCYTE COUNT REDUCTION AND CLINICAL OUTCOMES

	Gilenya® vs Pbo		Ono-4641 versus Placebo		Ponesimod versus Placebo		Siponimod versus Placebo	
	0.5mg	1.25mg	0.1 mg	0.15 mg	20 mg	40 mg	2 mg	10 mg
Lymphocyte Count Reduction	~70%	~75%	~60%	~65%	~65%	~70%	~70%	>70%
Reduction in Cumulative Number of Gd+ T1 Lesions (as measured by MRI)	80%	80%	90%	89%	83%	77%	76%	85%
Reduction in Annualized Relapse Rate (ARR)	54%	55%	70%	39%*	21%*	52%	66%	48%*
	Phase 3 Outcomes		Phase 2 Outcomes					

\* not significant

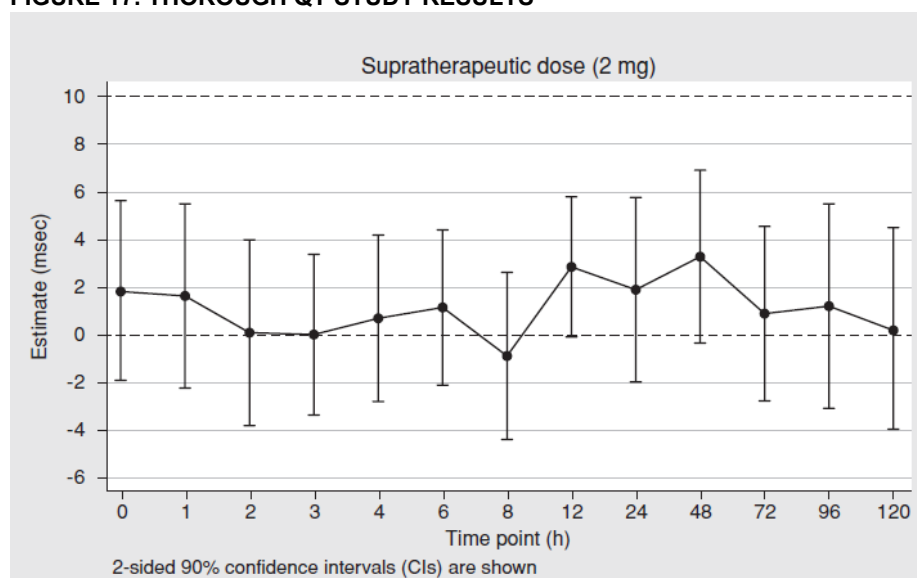
note: Phase 2 studies not powered to show significant reduction in ARR outcomes

Source: Receptos

## Phase 1 Safety Results

The Phase 1 safety findings were largely consistent with the biology of S1P1R modulation with no major surprises, in our view. The overall adverse event rate in RPC1063 treated patients was 75% compared to 70.8% in placebo treated patients. The most frequent (>5%) adverse events were local contact dermatitis as a result of reaction to medical adhesive used to apply electrocardiogram leads (42.6% RPC1063 vs. 50% placebo), headache (13.2% RPC1063 vs. 12.5% placebo), and sleepiness (8.8% RPC1063 vs. 4.2% placebo). There was one Grade 2 serious adverse event that was deemed to be a pre-existing condition and unrelated to RPC1063. There were no Grade 3 or higher adverse events and no dose-limiting toxicities in Phase 1.

FIGURE 17. THOROUGH QT STUDY RESULTS



A thorough QT study showed no relevant QT prolongation at both therapeutic and supra-therapeutic doses. 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. 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. Of note, 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, to our knowledge.

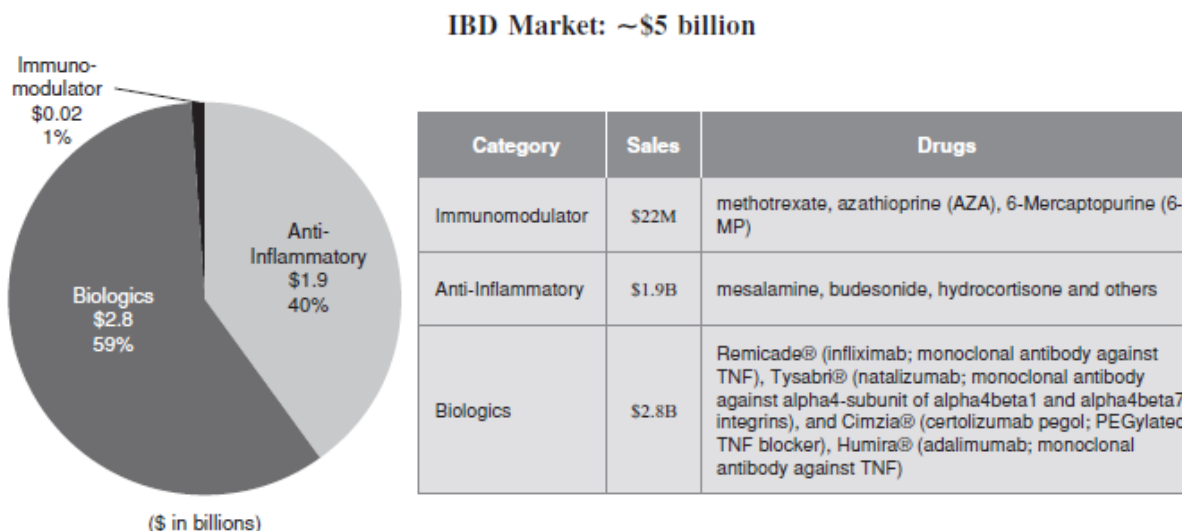
Source: Receptos



## RPC1063 AS A TREATMENT CANDIDATE FOR INFLAMMATORY BOWEL DISEASE

The current IBD market is estimated to be about \$5 billion worldwide. The worldwide prevalence of IBD is estimated at about 2.5 million people with UC representing about 1.5 million of the patient population. The current treatment landscape is dominated by biologics (~\$2.8 billion annual sales), anti-inflammatory agents (~\$1.9 billion annual sales), and immunomodulators (~\$22 million annual sales).

FIGURE 18. IBD MARKET OPPORTUNITY

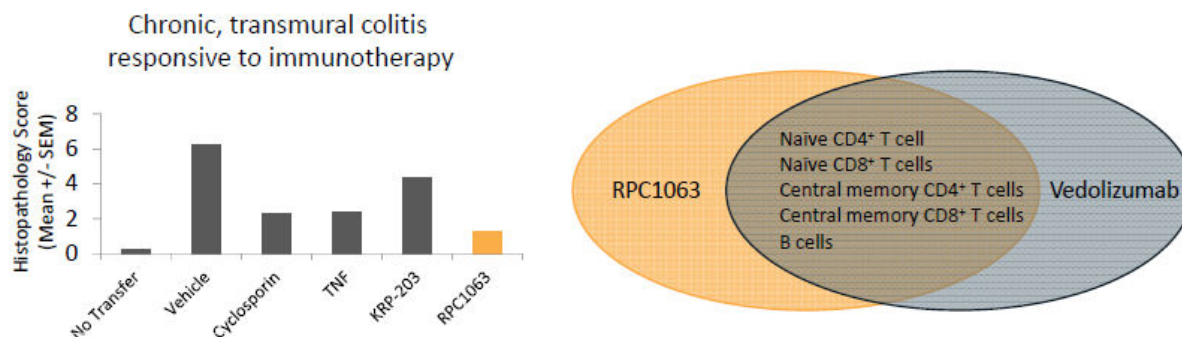


Source: Receptos

There are two main disease states of IBD: **Ulcerative Colitis (UC)** and **Crohn's Disease (CD)**. UC is a chronic inflammatory disorder of the GI tract that primarily affects the colon (the large intestine). Symptoms are mainly gastrointestinal in nature and include diarrhea, rectal bleeding, and weight loss. The disease is characterized by periods of remissions and exacerbations. About 20% of adults with UC undergo a colectomy within 10 years of diagnosis. CD is also a chronic inflammatory disorder of the GI tract. However, whereas UC is limited to the colon, CD most commonly affects the end of the small bowel (the ileum) and the beginning of the colon, but may also affect any part of the GI tract. Symptoms include diarrhea, blood in the stool, abdominal pain, and weight loss.

The company is initially focusing on UC in order to mitigate development risk. Although we believe success in UC could ultimately also lead to success in CD, there are several advantages to pursuing UC first. 1) There is a greater unmet need for new agents in UC. 2) There are better diagnostic tests and measurements of disease activity in UC versus CD. 3) The primary efficacy endpoint for UC trials is more objective as compared to CD trials.

FIGURE 19. SCIENTIFIC RATIONALE FOR RPC1063 USE IN IBD



Source: Receptos

We believe there is sound scientific rationale for the use of S1P1R modulation of lymphocyte trafficking for the treatment of inflammatory bowel disease. Preclinical efficacy of RPC1063 has been established across multiple IBD disease models including acute, chronic, preventive, and therapeutic. Furthermore, clinical proof of concept has been demonstrated by other lymphocyte trafficking agents such as Tysabri (approved) and vedolizumab (Phase 3 data). More specifically, vedolizumab has shown clinical



efficacy in UC and impacts largely the same lymphocyte population (naïve CD4+ and CD 8+ T cells, central memory CD4+ and CD8+ T cells, and B cells) as RPC1063 (Figure 19). While we believe the other lymphocyte trafficking inhibitors have validated the mechanism, RPC1063 could offer a more convenient oral dosage form with similar efficacy to vedolizumab (IV alpha-4-beta-7 integrin antibody inhibitor).

**Early preclinical and clinical studies of RPC1063 recently presented at Digestive Disease Week (DDW, May 18-21, 2013 Orlando) 2013 showed therapeutic efficacy in animal models of IBD including UC and CD as well as promising PK/PD characteristics in healthy volunteers.** Preclinical in vitro pharmacology testing showed RPC1063 to be a potent S1P1R agonist with an EC<sub>50</sub> of 0.16nM. Additionally, RPC1063 showed 269-fold selectivity for S1P1R and greater than 20,000-fold selectivity over S1P2R, S1P3R and S1P4R. In mouse models of IBD, RPC1063 significantly reduced inflammation comparable to anti-TNF-α and cyclosporine, two commonly prescribed treatments. In addition, RPC1063 demonstrated reduction of Th1, Th2, and inflammatory cytokines and chemokines in a mouse model of CD. Overall, RPC1063 was shown to reduce the majority of elevated cytokines with TNF-α, MCP-1, MIP-1β, MIP-2, LIF, IL-6, IL-12p40, Eotaxin, IP-10 and IL-13 reaching statistical significance. Furthermore, clinical testing in healthy volunteers showed a favorable therapeutic window. The PK data showed dose proportionality with low variability, a 19.3 hour half-life, and a delayed Tmax, low Cmax and a low peak-to-trough plasma concentration ratio.

#### **Ongoing Phase 2 TOUCHSTONE Trial in Ulcerative Colitis (UC)**

**The company is currently enrolling patients into the Phase 2 trial with top-line results expected in mid-2014.** The Phase 2 study is a multi-national, multi-center, double-blind, randomized, placebo-controlled study designed to enroll about 180 patients with moderately to severely active UC in North America, Europe and Asia-Pacific. The trial will compare doses of 0.5 mg and 1.0 mg of RPC1063 against placebo. The primary endpoint is percentage of patients achieving induction of clinical remission at eight weeks of treatment. Secondary endpoints include clinical response, clinical remission, mucosal healing, and safety and tolerability at weeks eight and 32.

**If the Phase 2 TOUCHSTONE results are compelling, the trial could be considered a pivotal Phase 3 trial.** The TOUCHSTONE trial design, including endpoints and statistical analysis, is consistent with a Phase 3 study approach. As a result, the FDA has indicated it could be considered as one of the necessary Phase 3 studies if the results are clinically and statistically persuasive; however, it is not being conducted under a SPA. Typically, a Phase 3 registration program in UC requires two Phase 3 studies for induction of clinical remission and one Phase 3 study for maintenance of clinical remission. The company believes that it may be able to submit an NDA to the FDA as early as 2018. It would also submit an MAA to the EMA pending input from European regulatory authorities.

#### **RPC4046 (ANTI-IL-13 MONOCLONAL ANTIBODY) AS A TREATMENT FOR EOSINOPHILIC ESOPHAGITIS (EOE)**

**Receptos in-licensed RPC4046 from AbbVie in October 2012.** Under the terms of the agreement, Receptos is responsible for all Phase 2 development costs in EoE and AbbVie has the option to opt-in once the Phase 2 study is complete. If AbbVie exercises its option, the companies will split further global development costs. Furthermore, in the US, there will be a 50/50 co-promote and profit split, while ex-US Receptos will receive a double-digit royalty on net sales. If AbbVie does not exercise its option, Receptos will retain global rights and will owe AbbVie a double digit royalty on worldwide net sales. AbbVie has a second option to collaborate if data from a Phase 2 study supports development in an additional indication. If AbbVie exercises its second option, they would be required to reimburse Receptos for all development costs incurred after completion of the Phase 2 EoE study and the collaboration would be a 50/50 profit split worldwide.

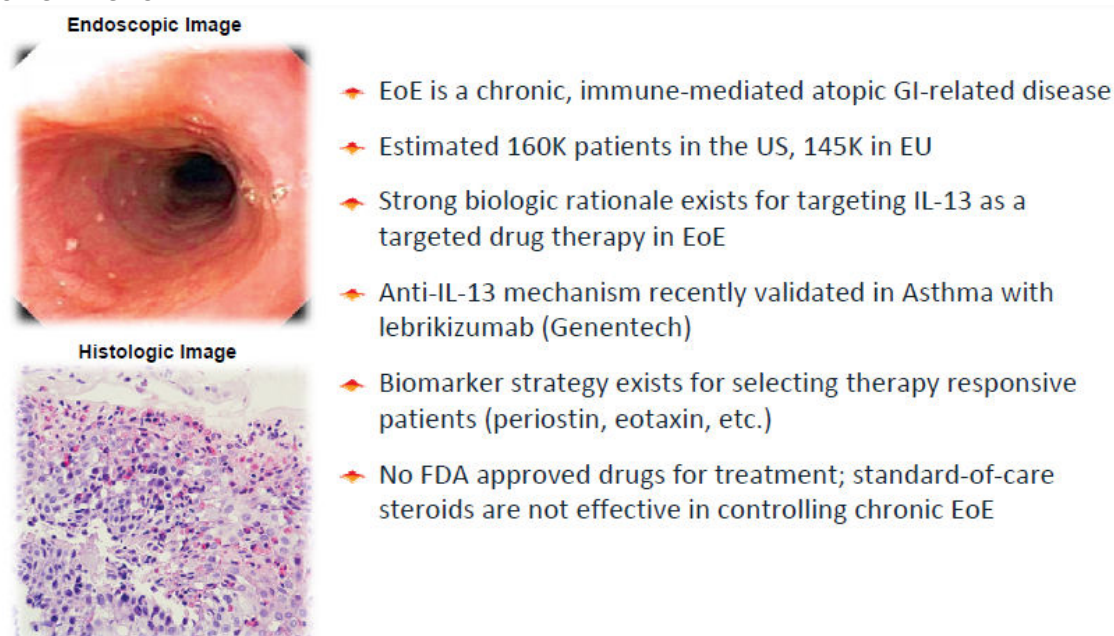
**AbbVie previously completed a Phase 1 study that showed both intravenous (IV) and subcutaneous (SC) formulations of RPC4046 were well tolerated.** The Phase 1 study was conducted in both healthy subjects and patients with mild to moderate asthma (biology of anti-IL-13 therapy suggests it may also be effective in treating asthma). Receptos plans to use an initial IV loading dose followed by SC injections in future EoE trials.

**Receptos plans to initiate a Phase 2 study testing RPC4046 for the treatment of EoE in early 2014.** The company plans to request a pre-IND meeting with the FDA by the end of 2013 and subsequently file an IND in H1:14. Assuming they are able to initiate a Phase 2 trial in early 2014, top-line results could be available in H2:15. The company currently anticipates the Phase 2 study will be a randomized, double-blind, placebo-controlled, parallel enrollment, multicenter trial in 90 patients with active EoE as measured by endoscopic, histologic, and clinical assessment. The trial will evaluate two doses of RPC4046 with the primary objective being clinical efficacy as determined by histological improvement at 12 weeks, while total treatment duration will be 16 weeks. Secondary endpoints will include a comparison of patients with a histological response and remission, improvement in swallowing difficulty, safety, and pharmacodynamic and biomarker measures.

**EoE is an orphan disease with a prevalence of about 160,000 patients in the US and 145,000 patients in the EU.** EoE is a chronic, allergic inflammatory condition of the esophagus that is characterized by swallowing difficulty, food impaction, and other disease effects. Although there are no currently approved drugs for the treatment of EoE, the majority of patients are treated with topical steroids. However, steroids have short-lived duration of efficacy (~4 months), and are associated with a number of side effects such as fungal infection and esophageal candidiasis.

**There is strong biological rationale for the anti-IL-13 antibody mechanism in the treatment of EoE.** Preclinical and clinical studies of EoE have shown it to be an allergic-associated disease characterized by infiltration into the esophagus by a type of white blood cell (eosinophil). IL-13 contributes to localized allergic immune responses, recruitment of proinflammatory cells and tissue remodeling including fibrosis, all of which are features of EoE. Furthermore, two genes, eotaxin-3 and periostin, that are over-expressed in patients with EoE are induced by IL-13, and may represent important diagnostic biomarkers for therapy.

**FIGURE 20. EOE SNAPSHOT**



Source: Receptos

## GLP-1R PAM AS A TREATMENT FOR DIABETES

In addition to receiving income from collaborations (ongoing collaboration with Ono Pharmaceutical Co., Ltd., a completed partnership with Ortho-McNeil-Janssen Pharmaceuticals, Inc. and a completed collaboration with Eli Lilly and Company), Receptos has used its proprietary glycoprotein coupled receptor (GPCR) structure-based drug design technology platform to discover potentially best-in-class drug discovery candidates including orally-dosed, small molecule, glucagon-like peptide-1 receptor (GLP-1R) positive allosteric modulators (PAMs) in preclinical development for the treatment of Type 2 Diabetes (T2D). These allosteric modulators bind to alternate sites on the receptor to which the natural ligand binds to enhance to activity between the receptor and its natural ligand.

Activation of the GLP-1 receptor with peptide modulators has resulted in a lucrative class of treatments for T2D; however, the currently marketed GLP-1R peptide agonists are dosed as subcutaneous injections. We believe that an orally-dosed equivalent therapeutic could achieve multi-billion dollars in sales. Due to the early preclinical status of the program, we have not included its potential value in our model or valuation for RCPT.

## Covered public companies mentioned in this report

Company	Ticker	Price (close 5/31/13)	Fair Value	Rating
Intercept Pharmaceuticals	ICPT	\$33.34	\$65	OUTPERFORM
Lexicon Pharmaceuticals	LXRX	\$2.38	\$3	OUTPERFORM

## Analyst Biography

Liana Moussatos joined Wedbush from Pacific Growth Equities where she was a Senior Research Analyst. Prior to that she came from UBS Global Asset Management where she was Director and portfolio manager of the UBS Global Biotech Funds for five years. Previously, Liana was with Bristol-Meyers Squibb where she was a manager in University and Government Licensing, External Science and Technology and she also worked with Sloan-Kettering Cancer Institute in the Office of Industrial Affairs and the National Cancer Institute in the Office of Technology Development. Liana received a BS in Entomology and a MS in Zoology and Biochemistry from Clemson University. She also earned a Ph.D. in Plant Pathology from the University of California, Davis and completed a postdoctoral research fellowship in Cellular and Molecular Physiology at the Yale School of Medicine.

## Analyst Certification

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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## Wedbush Equity Research Disclosures as of June 3, 2013

Company	Disclosure
Receptos	1,3,5,7
Intercept Pharmaceuticals	1,3,4,5,7
Lexicon Pharmaceuticals	1,3,4,5,7

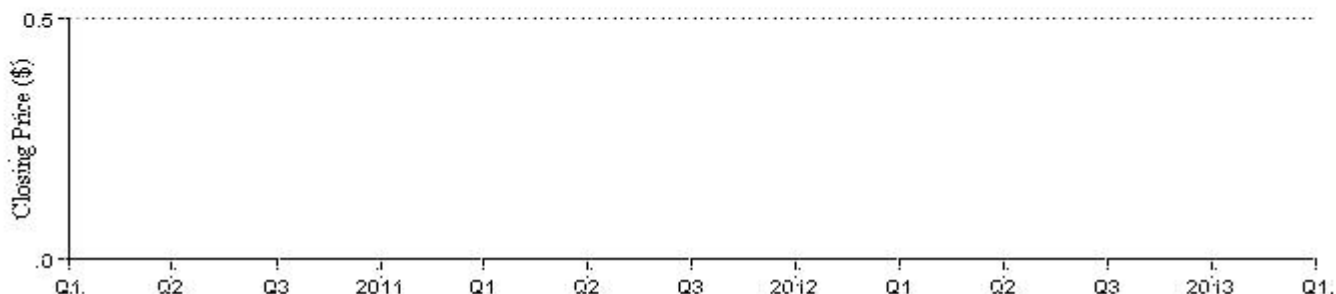
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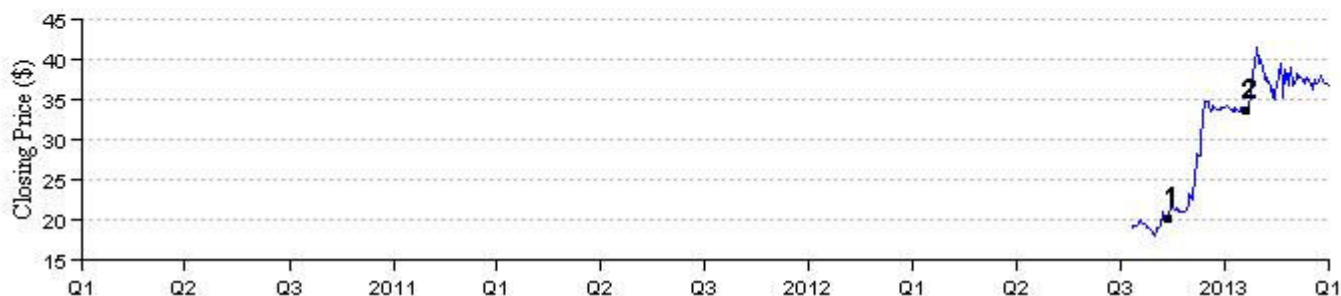
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RCPT



ICPT

1) 11/08/12	2) 01/16/13
OUTPERFORM \$25	OUTPERFORM \$46



LXRX

1) 04/25/11
OUTPERFORM \$3



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