

Reason for report:

PROPRIETARY INSIGHTS

## RECEPTOS, INC.

**Increasing Positive Bias for RPC-1063 in UC Drives New \$75 PT; Reit. OP**

• **Bottom Line:** We are increasing our price target on top pick RCPT to \$75 (from \$60) after increasing our probability of success (POS) estimate for RPC-1063 ("1063") in Ulcerative Colitis (UC) to the mid-30s percentage (from ~30%). After multiple MEDACorp KOL due diligence calls, we believe the 1063 Phase II blinded placebo adjusted induction clinical response delta is trending above Vedolizumab's ("Vedo") respective Phase III GEMINI-1 results. Additionally, while Vedolizumab is gaining differentiation vs. moderate to severe UC standard of care (SOC) anti-TNFs on the basis of safety, 1063 could be further differentiated on this point, in our view. While clinical remission is the ultimate key endpoint, we believe clinical response is an important surrogate. As a reminder, top-line 1063 Phase II data in Multiple Sclerosis (MS) is expected mid-14 (est July) and in UC right after in 3Q14. We believe RPCT represents an increasingly attractive risk-reward opportunity further accentuated by recent broad market turbulence. We reiterate our Outperform (OP) rating.

• **We believe 1063's Phase II blinded placebo-adjusted induction clinical response delta is trending above the 22% delta observed in Vedolizumab's pivotal Phase III GEMINI-1 trial.** Key is that the Vedo trial did not use a Centralized Reading Process by Endoscopy (CRPE) to screen appropriate patients while the 1063 Phase II does. As a result, we should expect the 1063 trial to have: (1) a higher screen failure rate vs. Vedo; (2) lower induction placebo clinical response rate vs. Vedo (~26%); and (3) lower 1063 induction clinical response rate needed to achieve the same 22% Vedo delta in GEMINI-1. Importantly, a Mesalamine Phase III UC trial (pp. 3-4) in mild-moderate UC (vs. mod-severe w/Vedo, 1063) shows use of a CRPE decreased placebo clinical response rate by ~10%. This drives our induction clinical response rate estimate of ~16% for placebo and ~38% for 1063 (delta of 22%) needed in the Phase II 1063 trial to match GEMINI-1 data.

• **We believe 1063's emerging attractive safety profile could also significantly differentiate it vs. Vedo and anti-TNFs, which in turn could drive earlier use and penetration.** While anti-TNFs are the established SOC for refractory mod-severe UC patients, the community is very interested in newer therapies with an improved safety profile given anti-TNFs are associated with high infection rates and long-term sequelae. Vedo is already garnering community enthusiasm given a better adverse event (AE) profile but comes with the hypothetical risk of progressive multifocal leukoencephalopathy (PML), similar to Tysabri. Based on 1063 maintaining a competitive profile vs. Gilenya at its interim Phase II review of 250 MS patients out to three months, we anticipate a better AE profile vs. Gilenya and potential for unremarkable safety in the small 180-patient 1063 UC Phase II trial. Gilenya's main AE is First-Dose-Heart-Rate-Monitoring (FDHRM) which may be more attractive than potential PML risk with Vedo.

Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2013A	\$1.5	\$1.2	\$1.1	\$0.8	\$4.6	(\$5.46)	(\$0.98)	(\$0.88)	(\$0.86)	(\$4.23)	NM
2014E	0.0	0.0	0.0	0.0	0.0	(\$0.91)	(\$1.02)	(\$1.39)	(\$1.53)	(\$4.86)	NM
2015E	--	--	--	--	0.0	--	--	--	--	(\$6.19)	NM

Source: Company Information and Leerink Partners LLC Research  
Revenues in \$000s.

### Key Stats:

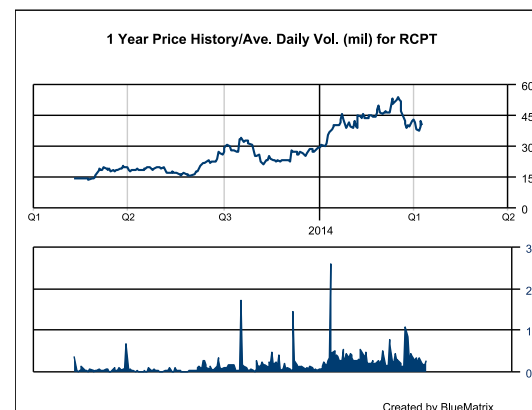
(NASDAQ:RCPT)

**S&P 600 Health Care Index:** 1,233.19  
**Price:** \$40.22  
**Price Target:** \$75.00 from \$60.00  
**Methodology:**

DCF analysis

52 Week High: \$55.00  
 52 Week Low: \$13.00  
 Shares Outstanding (mil): 22.2  
 Market Capitalization (mil): \$892.9  
 Book Value/Share: \$0.21  
 Cash Per Share: \$8.11  
 Dividend (ann): \$0.00  
 Dividend Yield: 0.0%

*General: Cash per share is pro forma for Jan-14 financing and assumes full exercise of over-allotment.*



## INVESTMENT THESIS

**We rate RCPT Outperform.** We believe RCPT shares are poised to appreciate near/longer term driven by clinical progress and commercialization of lead compound RPC-1063. Compared to other S1P1 compounds, RPC-1063 is earlier stage but emerging as “best in class.” In 2014, RCPT plans to sign an RPC-1063 partnership with large pharma, announce RPC-1063 Phase II data in relapsing MS (RMS) and Ulcerative Colitis (UC), and start two pivotal Phase III RMS trials. We modified our model by increasing probability adjusted RPC1063 revenues from UC to the mid-30s percentage from 30%. We currently assume a mid-30 percentage probability of approval for RPC-1063 in RMS in 2018. The 2Q13 MEDACorp MS Survey suggests that if approved in 2018, RPC-1063 would take significant market share from Gilenya (~58%), Tecfidera (~13%), and Tysabri (15%) that could be worth \$1.2B in U.S. revenue in 2019E. Core RPC-1063 Intellectual Property (IP) expires in 2029 but Gilenya (NVS) currently goes off patent in 2019. Assuming generic pricing starts in 2020, we model peak risk adjusted RPC-1063 WW revenues of ~\$770M (or \$2.2B non-risk adjusted) which leads to our base case NPV calculation of \$1.7B (previously \$1.4B), including cash, based only on approval and use in RMS.

### Change in Model

We modified our model based on our MEDACorp KOLs diligence. We increased probability of success of RPC1063 in UC to the mid-30s percentage from ~30%.

### Milestones

Product	Partner	Indication	Phase	Timing	Milestone
RPC-1063 (S1P1)	Proprietary	Relapsing MS	Phase III	2014	Partnership announcement
				2H14	Initiate 2 <sup>nd</sup> pivotal Phase III RMS trial (with SPA)
				Mid-2014	Phase II data of 1 <sup>st</sup> pivotal (RPC01-201)
				2017	2nd pivotal Phase III RMS trial data
				YE17	NDA submission
		Ulcerative Colitis (UC)	Phase II	2H18	FDA Approval
				1H14	Complete trial enrollment
				3Q14	Phase II UC trial data (might serve as 1 of 2 pivotals)
				2015	Initiate pivotal trial (possibly maintenance)
RPC-4046 (IL-13)	ABBV	Eosinophilic Esophagitis (EoE)	Phase II	2018	Possible NDA submission
				4Q13/1Q14	Submit IND
				1H14	Initiate Phase II data
				2H15	Phase II trial data

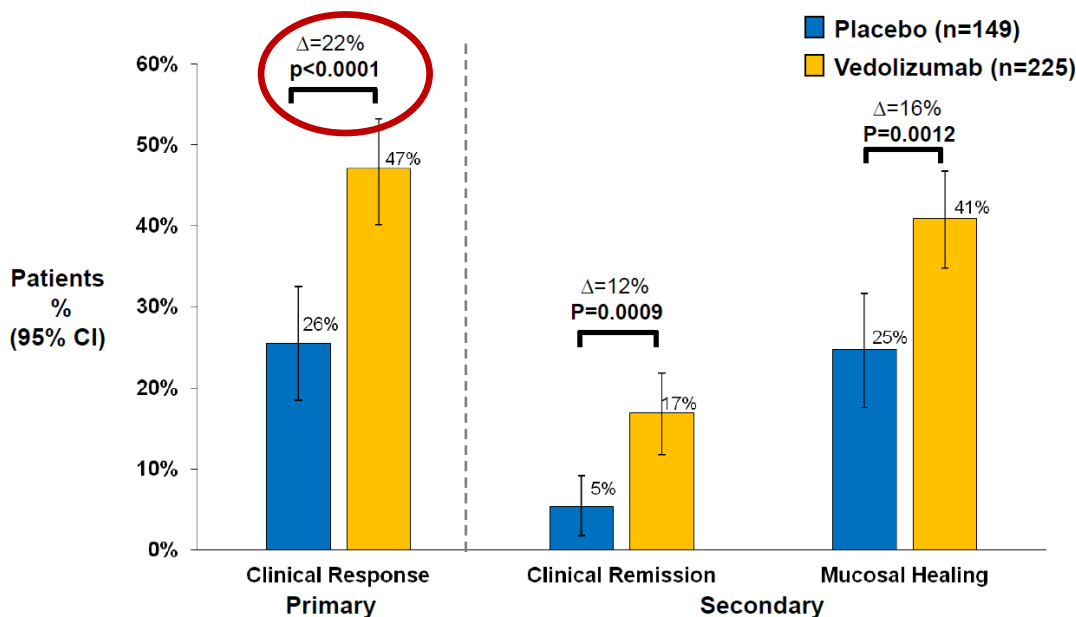
Source: Company Reports, Leerink Partners estimates

### Induction Efficacy (Clinical and Remission) Rates in Ulcerative Colitis (UC):

Induction Efficacy Results GEMINI 1 Study of Vedolizumab in UC			
	<u>N</u>	<u>Response Rate</u>	<u>N (# of Responders)</u>
Placebo	149	26%	39
Vedolizumab	225	47%	106
Overall	374	39%	144
<b>Delta (Vedo - PBO)</b>		<b>22%</b>	

Source: NEJM Vedolizumab publication of GEMINI-1

## UC (C13006): Statistical Significance for All Induction Phase Endpoints



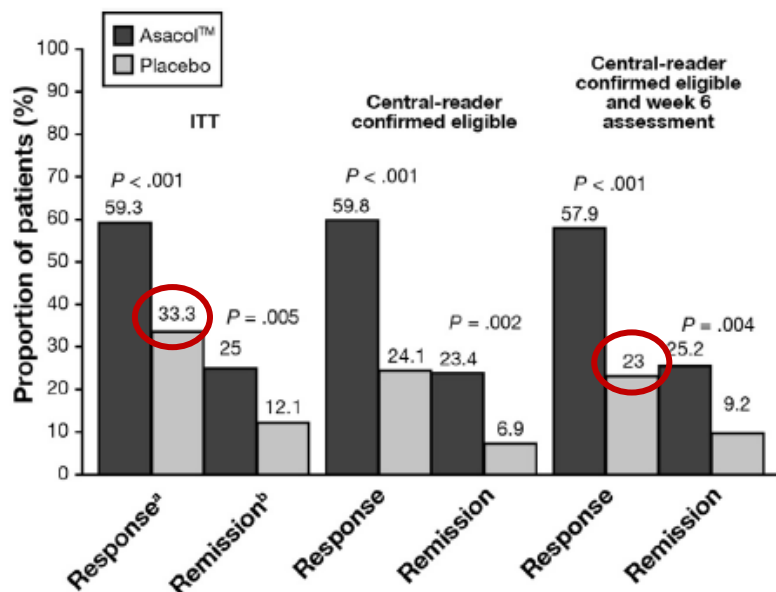
Source: Takeda presentation to FDA.

### Hypothetical Induction Efficacy Results in UC Assuming the Use of a Central Reader and Same Delta as GEMINI 1

	<u>Response Rate</u>
Placebo	16%
Treatment (Txt)	38%
<b>Delta (Txt - PBO)</b>	<b>22%</b>

Source: Takeda presentation to FDA. Leerink Partners estimates – assumes placebo rate decreases by ~10% (33.3%-23% from following efficacy diagram) due to central reading.

### Effect of Central Reading on Placebo Rate in a Randomized Controlled Trial of Mesalamine for Ulcerative Colitis:



Source: Gastroenterology July 2013 - The Role of Centralized Reading of Endoscopy in a Randomized Controlled Trial of Mesalamine for Ulcerative Colitis.

## VALUATION

We calculate a new \$75 (previously \$60) DCF price target for RCPT in the next 12 months based on a discounted cash flow (DCF) analysis. Based on MEDACorp KOL feedback, we are now increasing our probability of success of RPC1063 in Ulcerative Colitis (UC) to mid-30s percentage from 30% and assume launch in 2019. We only penetrate into fourth-line UC patients. We assigned a mid 30% probability of success for RPC-1063 in the MS setting, assuming launch in 2018. We apply a discount rate of 11% and a terminal growth rate of 1% which translates to a ~10x terminal multiple which we believe is comparable to biotechnology companies in a similar development stage. The 2Q13 MEDACorp MS Survey suggests that if approved in 2018, RPC-1063 would take significant market share from Gilenya (~58%), Tecfidera (~13%) and Tysabri (15%) that could be worth \$1.2B in U.S. revenue in 2019E. Core RPC-1063 Intellectual Property (IP) expires in 2029, but Gilenya (NVS) currently goes off patent in 2019. Assuming generic pricing starts in 2020, we model peak risk adjusted RPC-1063 WW revenues of ~\$770M (\$2.2B non-risk adjusted) which leads to our base case NPV calculation of \$1.7B including cash, based on approval and use in RMS and UC.

## RISKS TO VALUATION

An investment in RCPT is fundamentally a high-risk, high-reward investment, in our opinion. RCPT may face significant clinical, regulatory, and commercial risks for pipeline products. Most important is risk associated with potential failure of RPC-1063 (Relapse Remitting Multiple Sclerosis) to obtain regulatory approvals and capture market share in the MS treatment paradigm. RPC-1063 is also the earliest among other S1P receptor modulators. There is also risk that evolving therapeutic landscapes could render RCPT pipeline compounds non-competitive or less valuable once approved.

	RCPT P&L (\$000s, except per share data)																
	2013A	1Q14E	2Q14E	3Q14E	4Q14E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
Revenues																	
RPC1063 WW Revenue in MS										\$316,680	\$1,088,253	\$569,241	\$768,848	\$1,003,905	\$1,257,093	\$1,470,700	\$1,664,873
<i>Probability of Success</i>										35%	35%	35%	35%	35%	35%	35%	35%
Risk Adjusted RPC1063 WW Revenue in MS										\$110,838	\$380,889	\$199,234	\$269,097	\$351,367	\$439,983	\$514,745	\$582,706
RPC1063 U.S. Revenue in UC										-	\$81,377	\$194,873	\$292,476	\$460,913	\$484,235	\$508,738	\$534,480
<i>Probability of Success</i>										35%	35%	35%	35%	35%	35%	35%	35%
Risk Adjusted RPC1063 U.S. Revenue in UC										-	\$28,482	\$68,205	\$102,367	\$161,320	\$169,482	\$178,058	\$187,068
RPC4046																	
Collaborative Revenue	\$4,641	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Total Revenue	\$4,641	-	-	-	-	-	-	-	-	\$110,838	\$409,371	\$267,440	\$371,464	\$512,686	\$609,465	\$692,803	\$769,774
Costs and Expenses																	
Probability Adjusted COGS	-	-	-	-	-	-	-	-	-	\$11,084	\$40,937	\$26,744	\$37,146	\$41,015	\$48,757	\$55,424	\$61,582
R&D	\$43,585	\$16,630	\$18,950	\$27,250	\$30,340	\$93,170	\$139,755	\$174,694	\$195,657	\$100,250	\$80,000	\$81,600	\$83,232	\$84,897	\$86,595	\$88,326	\$90,093
SG&A (Risk Adjusted from Time of RPC1063 Launch)	\$8,949	\$3,400	\$3,600	\$3,800	\$4,000	\$14,800	\$15,984	\$17,263	\$53,514	\$43,000	\$68,800	\$75,680	\$80,978	\$85,836	\$90,986	\$96,446	\$102,232
Total Costs and Expenses	\$52,534	\$20,030	\$22,550	\$31,050	\$34,340	\$107,970	\$155,739	\$191,956	\$249,171	\$154,334	\$189,737	\$184,024	\$201,356	\$211,748	\$226,338	\$240,196	\$253,907
Operating Income (EBIT)	(\$47,893)	(\$20,030)	(\$22,550)	(\$31,050)	(\$34,340)	(\$107,970)	(\$155,739)	(\$191,956)	(\$249,171)	(\$43,496)	\$219,634	\$83,416	\$170,108	\$300,939	\$383,127	\$452,607	\$515,866
<i>Y/Y growth</i>																	
Income Before Taxes	(\$50,376)	(\$20,214)	(\$22,734)	(\$31,234)	(\$34,524)	(\$108,705)	(\$157,504)	(\$193,721)	(\$250,266)	(\$43,496)	\$219,634	\$83,416	\$170,108	\$300,939	\$383,127	\$452,607	\$515,866
Provision for Taxes						-	-	-	-	-	-	-	-	-	106,030	153,886	175,395
Net income	(\$50,376)	(\$20,214)	(\$22,734)	(\$31,234)	(\$34,524)	(\$108,705)	(\$157,504)	(\$193,721)	(\$250,266)	(\$43,496)	\$219,634	\$83,416	\$170,108	\$300,939	\$277,097	\$298,721	\$340,472
EPS (LPS) Basic	(\$4.23)	(\$0.91)	(\$1.02)	(\$1.39)	(\$1.53)	(\$4.86)	(\$6.19)	(\$6.94)	(\$8.29)	(\$1.43)	\$7.13	\$2.68	\$5.41	\$9.48	\$8.64	\$9.23	\$10.41
<i>Y/Y growth</i>																	
Basic Shares* (000)	11,916	22,168	22,278	22,390	22,502	22,360	25,441	27,917	30,197	30,499	30,804	31,112	31,423	31,737	32,054	32,375	32,699

Source: Leerink Partners and company reports.

DCF Calculation
Discount rate
Terminal Growth Rate
Valuation
Valuation / Share

Source: Leerink Partners estimates.

RCPT DCF Valuation / Share Sensitivity Analysis						
		Discount Rate				
		9.0%	10.0%	11.0%	12.0%	13.0%
Terminal Growth Rate	0.0%	\$104	\$86	\$71	\$60	\$50
	1.0%	\$112	\$92	\$75	\$63	\$52
	2.0%	\$123	\$99	\$80	\$66	\$55
	3.0%	\$137	\$108	\$87	\$71	\$58
	4.0%	\$157	\$120	\$95	\$76	\$62

Source: Leerink Partners estimates.

## Disclosures Appendix

### Analyst Certification

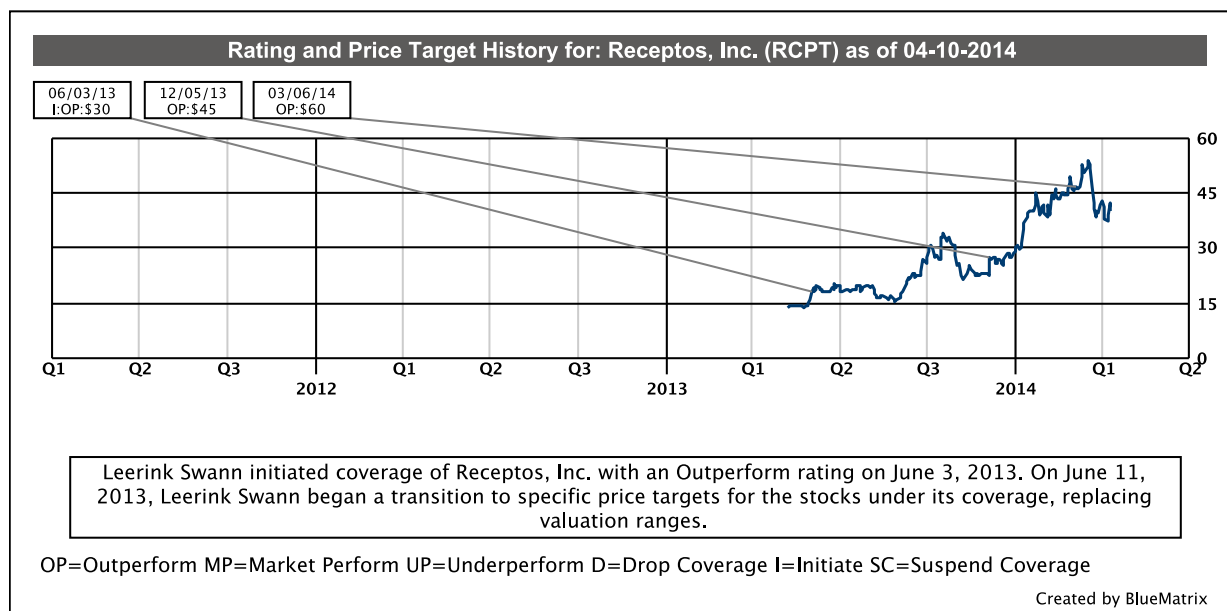
I, Marko Kozul, M.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

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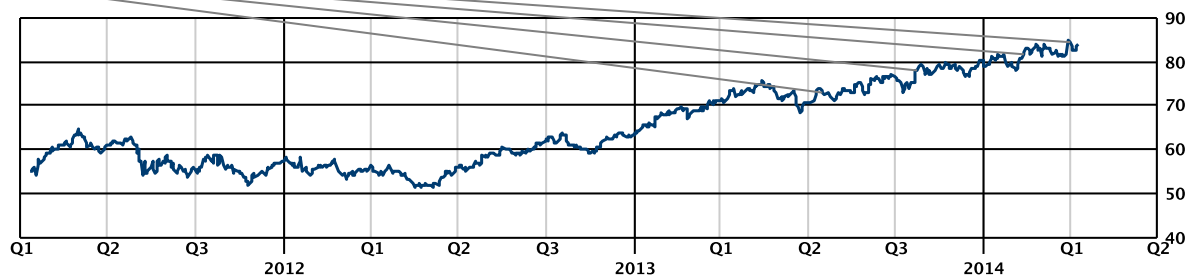
### Rating and Price Target History for: Novartis AG (NVS) as of 04-10-2014

07/17/13  
OP:\$80

10/23/13  
OP:\$83

02/12/14  
OP:\$88

04/02/14  
OP:\$93



Leerink Swann initiated coverage of NVS with an Outperform rating on November 9, 2010. On June 11, 2013, Leerink Swann began a transition to specific price targets for the stocks under its coverage, replacing valuation ranges.

OP=Outperform MP=Market Perform UP=Underperform D=Drop Coverage I=Initiate SC=Suspend Coverage

Created by BlueMatrix



Distribution of Ratings/Investment Banking Services (IB) as of 03/31/14				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OP]	131	68.23	46	35.11
HOLD [MP]	61	31.77	3	4.92
SELL [UP]	0	0.00	0	0.00

## Explanation of Ratings

**Outperform (Buy):** We expect this stock to outperform its benchmark over the next 12 months.

**Market Perform (Hold/Neutral):** We expect this stock to perform in line with its benchmark over the next 12 months.

**Underperform (Sell):** We expect this stock to underperform its benchmark over the next 12 months. The degree of outperformance or underperformance required to warrant an Outperform or an Underperform rating should be commensurate with the risk profile of the company.

For the purposes of these definitions the relevant benchmark will be the S&P 600® Health Care Index for issuers with a market capitalization of less than \$2 billion and the S&P 500® Health Care Index for issuers with a market capitalization over \$2 billion.

## Important Disclosures

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MEDACorp is a network of healthcare professionals, attorneys, physicians, key opinion leaders and other specialists accessed by Leerink and it provides information used by its analysts in preparing research.

**In the past 12 months, the Firm has received compensation for providing investment banking services to Receptos, Inc. .**

**Leerink Partners LLC makes a market in Receptos, Inc.**

**Leerink Partners LLC is willing to sell to, or buy from, clients the common stock of Novartis AG on a principal basis.**

**In the past 12 months, an affiliate of the Firm, Leerink Swann Consulting LLC, has received compensation for providing non-securities services to: Novartis AG.**

**Leerink Partners LLC has acted as a co-manager for a public offering of Receptos, Inc. in the past 12 months.**

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