**OUTPERFORM** 

Reason for report:

Marko Kozul, M.D. (415) 905-7221 Marko.Kozul@Leerink.com

Irene Lau

Irene.Lau@Leerink.com

(415) 905-7256

**ESTIMATE CHANGE** 



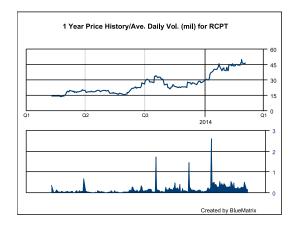
## RECEPTOS, INC.

Two Mid-14 Catalysts; Adding 1063 UC Revenues to Model Drives New \$60 PT

- Bottom Line: Based on a variety of emerging signals and anecdotes, we believe RPC-1063 ("1063") is at least demonstrating a signal in Ulcerative Colitis (UC) worth adding to our model with a low probability of success (POS) ~ 30% (vs. mid-30% for MS). Topline 1063 Phase II data in Multiple Sclerosis (MS) is expected mid-14 and in UC slightly after in 3Q14. A benign interim 1063 safety analysis in a larger MS trial should also be somewhat de-risking to potential safety in the US setting. Despite this interim Phase II MS analysis and unless the final topline data representing treatment from month 3-6 were to demonstrate a surprising safety issue, we believe even at current levels RPCT represents an attractive risk reward opportunity with 2 back-to-back major near-term catalysts with significant upside potential. We reiterate an Outperform (OP) rating and new \$60PT.
- · Based on a significant number of Phase II TOUCHDOWN UC induction patients moving into the maintenance phase and ~50% advancing into the open label extension (OLE) trial (on blinded basis) and no major Data Monitoring Committee (DMC) adverse events (AEs) reported, we believe a positive signal is emerging that at least justifies UC revenue inclusion in our model. As a reminder, this placebo-controlled double blinded randomized global clinical trial began ~YE12, evaluating 2 dose levels (0.5mg and 1mg) vs. placebo in 180 patients. It includes a saturation period and 8-week treatment period with superiority design and primary endpoint of clinical remission. The design approximates a typical Phase III and there is upside potential if it yields positive results and FDA signs off on it serving as 1 of 2 pivotal trials. Induction phase patients demonstrating clinical improvement can advance into a maintenance trial and both induction and maintenance patients can at any time enroll into the OLE trial. On the 4Q13 EPS call, management indicated that the proportion of patients crossing over into the maintenance phase is meeting their expectations from a clinical improvement perspective and this seems consistent anecdotally with the effective established treatments. Previously, the company has indicated that ~50% of induction/maintenance phase patients are enrolling into the OLE. In terms of safety, the 250-patient interim analysis from the Phase II MS trial should provide confidence the safety profile is relatively benign. Topline Phase II UC data is rapidly approaching in 3Q14.
- 4Q13 earnings were benign and inclusion of 1063 US revenue in UC adds \$15 to our valuation. We now include risk-adjusted 1063 US revenue in or model derived from UC using a probability of success (POS) of ~30%. This leads to probability adjusted peak sales in 2029E is \$287M (unadjusted \$955M). Our model includes penetration only into 4th-line UC patients with upside from earlier use. RCPT had YE13 proforma cash of ~\$180M (\$8.11/share) assuming full exercise of overallotment

S&P 600 Health Care Index:	1,321.30
Price:	\$46.49
Price Target:	\$60.00 from \$45.00
Methodology:	DCF analysis
52 Week High:	\$50.48
52 Week Low:	\$13.00
Shares Outstanding (mil):	22.2
Market Capitalization (mil):	\$1,030.6
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.



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 - New	0.0	0.0	0.0	0.0	0.0	(\$0.91)	(\$1.02)	(\$1.39)	(\$1.53)	(\$4.86)	NM
2014E - Old					0.0					(\$2.79)	NM
2015E					0.0					(\$6.19)	NM

Source: Company Information and Leerink Partners LLC Research

Revenues in \$000s. Basic shares for '12, PF for 1Q13.



### **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 including probability adjusted RPC1063 revenues from UC at 30%. Peak adjusted revenue in 2029E from UC is \$287M (\$955M unadjusted). We currently assume a mid 30% 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 ~\$747M, previously ~\$640M, (or \$2.1B non-risk adjusted) which leads to our base case NPV calculation of \$787M (previously \$520M), including cash, based only on approval and use in RMS. Core RPC-1063 Intellectual Property (IP) expires in 2029 but Gilenya (NVS) currently goes off patent in 2019.

#### Change in Estimates

We modified our model based on earnings reported 3.5.14 and the inclusion of probability adjusted revenue from RPC1063 in UC. We also increased R&D estimates from 2014-2017 due to the cost of running two Phase III trials in MS and one pivotal in UC. As a result, our 2014E EPS changes from (\$2.79) to (\$4.86).

### Milestones

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

Source: Company Reports, Leerink Partners estimates



### **VALUATION**

We calculate a new \$60 (previously \$45) DCF price target for RCPT in the next 12 months based on a discounted cash flow (DCF) analysis. We now include probability adjusted RPC1063 revenue from Ulcerative Colitis (UC) in the U.S. only at 30% and assume launch in 2019. We only penetrate into fourth-line UC patients. We now include probability adjusted RPC1063 revenue from Ulcerative Colitis (UC) in the U.S. only at 30% and assume launch in 2019. We only penetrate into fourth-line UC patients. Peak probability adjusted UC revenue in 2029E is \$287M (\$955M unadjusted). 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 ~\$747M (\$2.1B non-risk adjusted) which leads to our base case NPV calculation of \$1.3B 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.

RECEPTOS, INC. March 6, 2014

	RCPT P&L (\$000s, except per share data)																				
	1Q13A	2Q13A	3Q13A	4Q13A	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
Probability of Success														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	\$645,647	\$712,233	\$783,904
Probability of Success														30%	30%	30%	30%	30%	30%	30%	30%
Risk Adjusted RPC1063 U.S. Revenue in UC														-	\$24,413	\$58,462	\$87,743	\$138,274	\$193,694	\$213,670	\$235,171
RPC4046																					
Collaborative Revenue	\$1,488	\$1,238	\$1,142	\$773	\$4,641		-														
Total Revenue	\$1,488	\$1,238	\$1,142	\$773	\$4,641	-	-	-	-	-	-	-	-	\$110,838	\$405,302	\$257,696	\$356,840	\$489,641	\$633,677	\$728,415	\$817,877
Costs and Expenses																					
Probability Adjusted COGS	-	-	-	-	-	-	-	-		-	-	-	-	\$11,084	\$40,530	\$25,770	\$35,684	\$39,171	\$50,694	\$58,273	\$65,430
R&D	\$8,020	\$9,441	\$13,500	\$12,624	\$43,585	\$16,630	\$18,950	\$27,250	\$30,340	\$93,170	\$139,755	\$174,694	\$195,657	\$100,250	\$75,000	\$76,500	\$78,030	\$79,591	\$81,182	\$82,806	\$84,462
SG&A (Risk Adjusted from Time of RPC1063 Launch)	\$1,062	\$1,589	\$3,050	\$3,248	\$8,949	\$3,400	\$3,600	\$3,800	\$4,000	\$14,800	\$15,984	\$17,263	\$53,514	\$43,000	\$65,800	\$69,090	\$72,545	\$76,172	\$79,980	\$83,979	\$88,178
Total Costs and Expenses	\$9,082	\$11,030	\$16,550	\$15,872	\$52,534	\$20,030	\$22,550	\$31,050	\$34,340	\$107,970	\$155,739	\$191,956	\$249,171	\$154,334	\$181,330	\$171,360	\$186,258	\$194,934	\$211,857	\$225,059	\$238,071
Operating Income (EBIT)	(\$7,594)	(\$9,792)	(\$15,408)	(\$15,099)	(\$47,893)	(\$20,030)	(\$22,550)	(\$31,050)	(\$34,340)	(\$107,970)	(\$155,739)	(\$191,956)	(\$249,171)	(\$43,496)	\$223,972	\$86,337	\$170,581	\$294,707	\$421,820	\$503,356	\$579,806
Y/Y growth			]																		
Income Before Taxes	(\$9,649)	(\$9,918)	(\$15,565)	(\$15,244)	(\$50,376)	(\$20,214)	(\$22,734)	(\$31,234)	(\$34,524)	(\$108,705)	(\$157,504)	(\$193,721)	(\$250,266)	(\$43,496)	\$223,972	\$86,337	\$170,581	\$294,707	\$421,820	\$503,356	\$579,806
Provision for Taxes										-	-	-	-			-	-	-	119,696	171,141	197,134
Net income	(\$9,649)	(\$9,918)	(\$15,565)	(\$15,244)	(\$50,376)	(\$20,214)	(\$22,734)	(\$31,234)	(\$34,524)	(\$108,705)	(\$157,504)	(\$193,721)	(\$250,266)	(\$43,496)	\$223,972	\$86,337	\$170,581	\$294,707	\$302,124	\$332,215	\$382,672
EPS (LPS) Basic	(\$5.46)	(\$0.98)	(\$0.88)	(\$0.86)	(\$4.23)	(\$0.91)	(\$1.02)	(\$1.39)	(\$1.53)	(\$4.86)	(\$6.19)	(\$6.94)	(\$8.29)	(\$1.43)	\$7.27	\$2.78	\$5.43	\$9.29	\$9.43	\$10.26	\$11.70
Y/Y growth																					
Basic Shares* (000)	1,767	10,151	17,715	17,806	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 Calcuation

Discount rate	11%
Terminal Growth Rate	1%
Valuation	\$1,346,293
Valuation / Share	\$60

Source: Leerink Partners estimates.

RCPT DCF Valuation / Share Sensitivity Analysis											
	_	Discount Rate									
		9.0%	10.0%	11.0%	12.0%	13.0%					
	0.0%	\$82	\$68	\$57	\$48	\$40					
Terminal Growth Rate	1.0%	\$88	\$72	\$60	\$50	\$42					
irowt	2.0%	\$96	\$77	\$63	\$53	\$44					
inal G	3.0%	\$106	\$84	\$68	\$56	\$46					
Term	4.0%	\$120	\$93	\$74	\$60	\$49					
Source: Leerink Pai	rtners estimat	es.									



# 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.

March 6, 2014

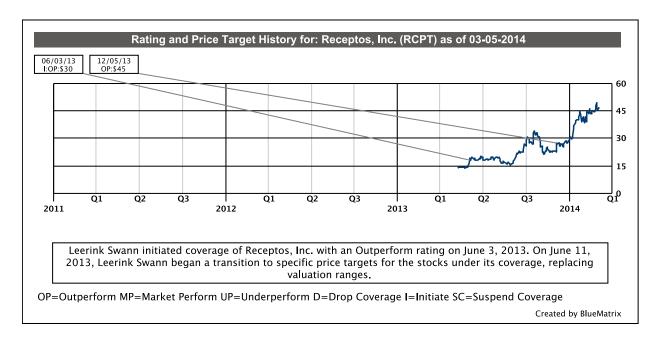
### **Valuation**

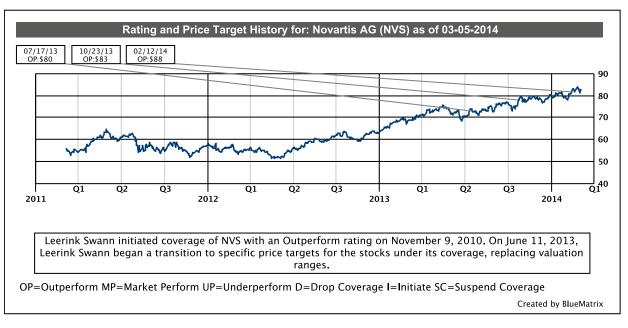
We calculate a new \$60 (previously \$45) DCF price target for RCPT in the next 12 months based on a discounted cash flow (DCF) analysis. We now include probability adjusted RPC1063 revenue from Ulcerative Colitis (UC) in the U.S. only at 30% and assume launch in 2019. We only penetrate into fourth-line UC patients. We now include probability adjusted RPC1063 revenue from Ulcerative Colitis (UC) in the U.S. only at 30% and assume launch in 2019. We only penetrate into fourth-line UC patients. Peak probability adjusted UC revenue in 2029E is \$287M (\$955M unadjusted). 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 ~\$747M (\$2.1B non-risk adjusted) which leads to our base case NPV calculation of \$1.3B 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.







RECEPTOS, INC. March 6, 2014



Distribution of Ratings/Investment Banking Services (IB) as of 12/31/13 IB Ser								
Rating	Count	Percent	Count	Percent				
BUY [OP]	118	64.50	30	25.00				
HOLD [MP]	65	35.50	2	3.00				
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.

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

<u>Underperform (Sell):</u> 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**

This information (including, but not limited to, prices, quotes and statistics) has been obtained from sources that we believe reliable, but we do not represent that it is accurate or complete and it should not be relied upon as such. All information is subject to change without notice. This is provided for information purposes only and should not be regarded as an offer to sell or as a solicitation of an offer to buy any product to which this information relates. The Firm, its officers, directors, employees, proprietary accounts and affiliates may have a position, long or short, in the securities referred to in this report, and/or other related securities, and from time to time may increase or decrease the position or express a view that is contrary to that contained in this report. The Firm's salespeople, traders and other professionals may provide oral or written market commentary or trading strategies that are contrary to opinions expressed in this report. The Firm's proprietary accounts may make investment decisions that are inconsistent with the opinions expressed in this report. The past performance of securities does not guarantee or predict future performance. Transaction strategies described herein may not be suitable for all investors. Additional information is available upon request by contacting the Editorial Department at One Federal Street, 37th Floor, Boston, MA 02110.

Like all Firm employees, analysts receive compensation that is impacted by, among other factors, overall firm profitability, which includes revenues from, among other business units, Institutional Equities, and Investment Banking. Analysts, however, are not compensated for a specific investment banking services transaction.

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.

Leerink Consulting LLC, an affiliate of Leerink Partners, is a provider of evidence-based strategy and consulting to the healthcare industry.

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.

©2014 Leerink Partners LLC. All rights reserved. This document may not be reproduced or circulated without our written authority.

	Leerink Partners LLC	Fauity Research	h
	Eccilik i artifers EEO	Equity Research	
Director of Equity Research	John L. Sullivan, CFA	(617) 918-4875	john.sullivan@leerink.com
Associate Director of Research	Alice C. Avanian, CFA	(617) 918-4544	alice.avanian@leerink.com
Healthcare Strategy	John L. Sullivan, CFA	(617) 918-4875	john.sullivan@leerink.com
<b></b>	Alice C. Avanian, CFA	(617) 918-4544	alice.avanian@leerink.com
Dietechnology	Haward Liona Dh D	(647) 040 4057	howard liang@loovink.com
Biotechnology	Howard Liang, Ph.D.	(617) 918-4857	howard.liang@leerink.com
	Joseph P. Schwartz	(617) 918-4575	joseph.schwartz@leerink.com
	Marko Kozul, M.D.	(415) 905-7221	marko.kozul@leerink.com
	Michael Schmidt, Ph.D.	(617) 918-4588	michael.schmidt@leerink.com
	Jonathan Chang, Ph.D.	(617) 918-4015	jonathan.chang@leerink.com
	Irene Lau	(415) 905-7256	irene.lau@leerink.com
	Paul Matteis	(617) 918-4585	paul.matteis@leerink.com
	Gena Wang, Ph.D., CFA	(212) 277-6073	gena.wang@leerink.com
	Richard Goss	(617) 918-4059	richard.goss@leerink.com
Life Science Tools	Dan Leonard	(212) 277-6116	dan.leonard@leerink.com
and Diagnostics	Justin Bowers, CFA	(212) 277-6066	justin.bowers@leerink.com
Pharmaceuticals/Major	Seamus Fernandez	(617) 918-4011	seamus.fernandez@leerink.com
	Ario Arabi	(617) 918-4568	ario.arabi@leerink.com
Specialty Pharmaceuticals,	Jason M. Gerberry, JD	(617) 918-4549	jason.gerberry@leerink.com
Generics	Christopher W. Kuehnle, JD	(617) 918-4851	chris.kuehnle@leerink.com
Medical Devices, Cardiology &	Danielle Antalffy	(212) 277-6044	danielle.antalffy@leerink.com
Orthopedics	Richard Newitter	(212) 277-6088	richard.newitter@leerink.com
	Robert Marcus, CFA	(212) 277-6084	robert.marcus@leerink.com
	Ravi Misra	(212) 277-6049	ravi.misra@leerink.com
Healthcare Services	Ana Gupte, Ph.D.	(212) 277-6040	ana.gupte@leerink.com
ricaltricale Services	Alla Gupte, I II.D.	(212) 277-0040	ana.gupte@ieemink.com
Healthcare Technology	David Larsen, CFA	(617) 918-4502	david.larsen@leerink.com
& Distribution	Christopher Abbott	(617) 918-4010	chris.abbott@leerink.com
Sr. Editor/Supervisory Analyst	Mary Ellen Eagan, CFA	(617) 918-4837	maryellen.eagan@leerink.com
	-	(017) 310-4037	
Supervisory Analysts	Robert Egan		bob.egan@leerink.com
	Amy N. Sonne		amy.sonne@leerink.com

**New York** 299 Park Avenue, 21<sup>st</sup> floor New York, NY 10171 (888) 778-1653 Boston One Federal Street, 37<sup>th</sup> Floor Boston, MA 02110 (800) 808-7525

San Francisco 201 Spear Street, 16<sup>th</sup> Floor San Francisco, CA 94105 (800) 778-1164