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OUTPERFORM

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

LEERINK

Reason for report:

COMPANY UPDATE

ACCELERON PHARMA

Robust EHA Datasets in Both MDS & β-Thal Drive Our New $57 PT; Reit. OP

• **Bottom Line:** EHA data for Sotatercept (SOT) and ACE-536 in MDS (myelodysplastic syndrome) and β-Thal. were very encouraging in both Transfusion Dependent (TD) and Non-Transfusion Dependent (NTD) patients, driving our increased probability of success (POS) for these compounds in MDS to ~40% (from ~30%) and in β-Thal to ~50% (from ~40%). This in turn drives our new $57 price target (from $52). Looking ahead, XLRN has many 2H14 catalysts to drive the shares higher, including: 1) SOT ESRD data, including bone mineral density details, at ASN-14 (Nov. 11-16); 2) maturing SOT and ACE-536 MDS/β-Thal. datasets at ASH-14 (Dec. 6-9); 3) preliminary data from new initiatives such as response rates from the Dalantercept-Sorafenib combo in HCC.

• **New ACE-536 MDS data at EHA demonstrate impressive responses in both NTD/TD patients at doses ≥0.7.5mg/kg and the ability to dose higher given only Grade 1-2 (G1-2) toxicity up to 1.33mg/kg.** Key is that no serious adverse events (SAEs) and only G1-2 tox were observed in dosing cohorts up to 1.33mg/kg, suggesting the possibility of benefiting from further efficacy in the 1.75mg/kg cohort. In terms of efficacy, data are available for 21 patients (5 NTD, 16 TD). For the TD patients, ~38% (6/16) experienced ≥50% reduction in transfused units during an 8-week interval on ACE-536 (doses 0.25-1.33mg/kg) after failing ESAs, Revlimid, or both. These TD data are particularly strong given a mean baseline transfusion burden of 6.3 units suggesting a relatively heavily transfused cohort of patients. For NTD patients, 60% (3/5) treated at 0.75mg/kg were either ESA refractory or non-responders and they experienced maximum Hgb increases of 1.6, 1.9 and 3.3g/dL (impressive) despite a modest mean Hgb of 9g/dL. For the 0.75mg/kg cohort, data demonstrate meaningful, and more than doubling, transient reticulocyte increases. At ASH-14, we hope to see additional dose escalation data, more granular reticulocyte details and sustained hematocrit levels without tachyphylaxis as well as data for SOT in this indication.

• **New ACE-536 β-Thal. EHA data are also impressive for both TD and NTD patients, again in the context of favorable safety with no SAEs.** Data were presented for 24 patients (20 NTD, 4 TD) with NTD baseline data suggesting patients were β-Thal-intermedia (rather than major) with mean Hgb of 8.2g/dL. In terms of safety, no drug SAEs were reported to date and toxicity in the 0.8mgkg cohort (highest currently available) showed only G2 toxicities, suggesting the potential to continue dose escalation. For the 4 TD patients (1 at 0.6 and 3 at 0.8mg/kg), 100% experienced reductions in transfusion burden (-78.5%, -66.7%, -66.7% and -69.8%). Key is that on top of chelation therapy these 4 patients also demonstrated reductions in serum ferritin levels (-39.7%, -27.5%, -59.5% and -42.7%), which is key to improving their quality of life and their long-term survival. For NTD patients, efficacy was dose proportional with 100% experiencing a ≥1.0g/dL increase by the 0.6mg/kg cohort and 33% experiencing a ≥2.0g/dL increase at 0.8mg/kg.

Key Stats:	**(NASDAQ:XLRN)**
S&P 600 Health Care Index:	**1,282.26**
Price:	**$33.88**
Price Target:	$57.00 from $52.00
Methodology:	DCF analysis; 10% discount rate; 1% terminal growth rate
52 Week High:	$57.89
52 Week Low:	$15.00
Shares Outstanding (mil):	30.3
Market Capitalization (mil):	$1,026.6
Book Value/Share:	$1.32
Cash Per Share:	$7.06
Dividend (ann):	$0.00
Dividend Yield:	0.0%
Cash Per Share:	

Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2013A	$15.0	$26.4	$4.3	$11.5	$57.2	$0.13	$0.64	($0.66)	($0.64)	($4.15)	NM
2014E	$3.3A	0.0	$30.0	0.0	$33.3	($0.30)A	($0.55)	$0.39	($0.60)	($1.06)	NM
2015E	--	--	--	--	0.0	--	--	--	--	($2.29)	NM

Source: Company Information and Leerink Partners LLC Research
Revenue in MM; GAAP EPS presented.

INVESTMENT THESIS

We rate XLRN Outperform. We believe XLRN shares are poised to appreciate near/longer term driven by progress with CELG-partnered compounds Sotatercept/ACE-536 and proprietary Dalantercept (ACE-041). XLRN has multiple significant data read-out catalysts during almost every quarter until YE14. Pivotal catalysts through 2014: (1) Maturing Sotatercept Phase II Part-1 data (0.7mg/kg cohort+BMD/Vasc Calcificat) at American Society of Nephrology (ASN) Nov. 11-16, 2014; (2) Maturing Sotatercept and ACE-536 Phase II MDS and β-Thal. (4 trials) data at ASH in 4Q14; (3) initiate pivotal MDS and/or β-Thal. trials in early-15; (4) Preliminary Dalantercept Phase II randomized Part-2 renal cell carcinoma (RCC) data in 2H15. MEDACorp KOLs are very bullish and encouraged by emerging pipeline data and science. We assume probability of success in the ~40% range for Sotatercept/ACE-536 in MDS, 50% for β-Thal., 20% in end-stage renal disease (ESRD) patients on hemodialysis, and low 30% range for Dalantercept in 2nd-line RCC.

Milestones

Product	Partner	Indication	Phase	Timing	Milestone
ACE-536	CELG	MDS + β-Thal.	Ph. II	**4Q14**	**Final Phase II in MDS and β-Thal. data**
				YE14/early-15	Initiate Phase III trial for MDS and/or β-Thal.
				2018	Approval and launch
Sotatercept (ACE-011)				**4Q14**	**Final Phase II in MDS + β-Thal. data**
				2018	Approval and launch
				YE14/early-15	Initiate Phase III trial for MDS and/or β-Thal.
	CELG	ESRD	Ph. II	**11.11-16.14**	**Phase II Part-1 data (0.7mg/kg cohort+BMD/Vasc Calcificat)**
Dalantercept (ACE-041)	Proprietary	Oncology	Ph. II	2Q14	Initiate Phase II Dal-sorafenib combo trial in HCC
				YE14	Preliminary Phase II Dal-sorafenib combo RR data in HCC
				2H15	**Phase II RCC Part-2 data**
				2014	Phase II data in SCCHN
				2018	Approval and launch in RCC
ACE-083		Muscle	PC	2H14	Advance ACE-083 into clinic for Muscle Loss
New TGF-β Candidates		Fibrosis	PC	2015	Advance Fibrosis (i.e., PAH) candidate into clinic

Source: Company reports, Leerink Partners estimates

VALUATION

Our new $57 (from $52) 12-month price target of XLRN shares reflects probability-adjusted royalty revenue at 20% from end-stage renal disease (ESRD) patients on hemodialysis. Our valuation is based on a discounted cash flow analysis. We believe XLRN shares are poised to appreciate near/longer term driven by progress with CELG-partnered compounds Sotatercept/ACE-536 and proprietary Dalantercept (ACE-041). We apply a discount rate of 10% and a terminal growth rate of 1%, which translates to an 11x terminal multiple, which we believe is comparable to biotechnology companies in a similar development stage.

RISKS TO VALUATION

An investment in XLRN is fundamentally a high-risk, high-reward investment, in our opinion. XLRN may face significant clinical, regulatory, and commercial risks for pipeline products. Most important is clinical risk for Phase II Sotatercept and ACE-536 trials in MDS (myelodysplastic syndromes) and β-Thal. as well as Dalantercept/Axitinib in RCC. There is also competitive risk from emerging MDS, β-Thal., and RCC therapies. Finally, XLRN may face financing risk beyond 1H15.

XLRN P&L ($000s, except per share data)	2013A	1Q14A	2Q14E	3Q14E	4Q14E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E
Revenues																		
Sotatercept/ACE-536 WW Revenue in MDS to CELG										$66,089	$141,589	$227,505	$324,937	$435,091	$559,283	$698,955	$855,680	$1,031,180
Probability of Success										*40%*	*40%*	*40%*	*40%*	*40%*	*40%*	*40%*	*40%*	*40%*
Risk Adjusted Sotatercept/ACE-536 WW Revenue										$26,436	$56,635	$91,002	$129,975	$174,036	$223,713	$279,582	$342,272	$412,472
Risk Adjusted Sotatercept/ACE-536 WW Royalties in MDS										**$5,287**	**$11,893**	**$20,020**	**$29,894**	**$40,028**	**$53,691**	**$69,895**	**$85,568**	**$103,118**
Sotatercept/ACE-536 WW Revenue in NTD β-Thal. to CELG										$3,659	$60,642	$127,092	$226,546	$339,098	$466,097	$609,020	$767,222	$904,639
Probability of Success										*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*
Risk Adjusted Sotatercept/ACE-536 WW Revenue in NTD β-Thal.										$1,830	$30,321	$63,546	$113,273	$169,549	$233,049	$304,510	$383,611	$452,320
Risk Adjusted Sotatercept/ACE-536 WW Royalties in NTD β-Thal.										**$366**	**$6,064**	**$13,345**	**$24,920**	**$37,301**	**$53,601**	**$73,082**	**$92,067**	**$113,080**
Dalantercept WW Revenue in 2nd-line RCC										$68,061	$131,647	$210,325	$298,864	$398,173	$509,233	$633,101	$770,918	$877,863
Probability of Success										*32%*	*32%*	*32%*	*32%*	*32%*	*32%*	*32%*	*32%*	*32%*
Risk Adjusted Dalantercept WW Revenue in 2nd-line RCC										**$21,780**	**$42,127**	**$67,304**	**$95,637**	**$127,415**	**$162,954**	**$202,592**	**$246,694**	**$280,916**
Sotatercept US Revenue in ESRD Patients on Hemodialysis												$301,866	$819,107	$1,418,781	$1,915,379	$2,064,199	$2,220,226	$2,383,756
Probability of Success												*20%*	*20%*	*20%*	*20%*	*20%*	*20%*	*20%*
Risk Adjusted Sotatercept US Revenue in ESRD Patients on Hemodialysis												$60,373	$163,821	$283,756	$383,076	$412,840	$444,045	$476,751
Risk Adjusted Sotatercept US Royalties in ESRD Patients on Hemodialysis												**$12,075**	**$36,041**	**$65,264**	**$91,938**	**$103,210**	**$111,011**	**$119,188**
Collaboration Revenue	$57,231	$3,307	-	$30,000	-	$33,307	-	$40,000	$30,000	$28,000	$10,000	-	$10,000	-	$10,000	$10,000	$10,000	$10,000
Total Revenue	**$57,231**	**$3,307**	**-**	**$30,000**	**-**	**$33,307**	**-**	**$40,000**	**$30,000**	**$55,433**	**$70,085**	**$112,744**	**$196,492**	**$270,009**	**$372,185**	**$458,780**	**$545,340**	**$626,302**
Costs and Expenses																		
Probability Adjusted Dalantercept COGS	-					-	-	-	-	$3,267	$6,319	$10,096	$9,564	$12,742	$16,295	$20,259	$24,669	$28,092
Research and Development	$36,051	$11,765	$12,200	$12,500	$13,280	$49,745	$55,217	$61,291	$68,033	$34,016	$35,717	$37,503	$39,378	$41,347	$43,415	$45,585	$47,864	$50,258
SG&A (Risk Adjusted from Time of Dalantercept Launch)	$14,227	$3,750	$4,500	$5,100	$5,200	$18,550	$20,405	$22,446	$24,690	$31,090	$34,199	$36,935	$38,782	$40,721	$42,757	$44,895	$47,139	$49,496
Total Costs and Expenses	**$50,278**	**$15,515**	**$16,700**	**$17,600**	**$18,480**	**$68,295**	**$75,622**	**$83,736**	**$92,723**	**$68,373**	**$76,235**	**$84,534**	**$87,724**	**$94,810**	**$102,467**	**$110,739**	**$119,673**	**$127,846**
Operating Income (EBIT)	**$6,952**	**($12,208)**	**($16,700)**	**$12,400**	**($18,480)**	**($34,988)**	**($75,622)**	**($43,736)**	**($62,723)**	**($12,941)**	**($6,151)**	**$28,210**	**$108,768**	**$175,199**	**$269,718**	**$348,041**	**$425,666**	**$498,456**
Y/Y growth																		
Other Income (Expenses)	($27,710)	$3,088				-	-	-	-	-	-	-	-	-	-	-	-	-
Interest Income	$20																	
Interest Expense	($1,161)		($378)	($298)	($219)	($895)	($521)	-	-	-	-	-	-	-	-	-	-	-
Income Before Taxes	**($21,898)**	**($9,120)**	**($17,078)**	**$12,102**	**($18,699)**	**($32,795)**	**($76,143)**	**($43,736)**	**($62,723)**	**($12,941)**	**($6,151)**	**$28,210**	**$108,768**	**$175,199**	**$269,718**	**$348,041**	**$425,666**	**$498,456**
Provision for Taxes						-	-	-	-	-	-	-	-	-	53,417	118,334	144,727	169,475
Tax Rate						0%	0%	0%	0%	0%	0%	0%	0%	0%	20%	34%	34%	34%
Net income	**($21,898)**	**($9,120)**	**($17,078)**	**$12,102**	**($18,699)**	**($32,795)**	**($76,143)**	**($43,736)**	**($62,723)**	**($12,941)**	**($6,151)**	**$28,210**	**$108,768**	**$175,199**	**$216,301**	**$229,707**	**$280,940**	**$328,981**
Net income (loss) applicable to common stockholders—diluted	**($39,003)**	**($9,120)**	**($17,078)**	**$12,102**	**($18,699)**	**($32,795)**	**($76,143)**	**($43,736)**	**($62,723)**	**($12,941)**	**($6,151)**	**$28,210**	**$108,768**	**$175,199**	**$216,301**	**$229,707**	**$280,940**	**$328,981**
Change in fair value of warrants	$1,500																	
EPS (LPS) Basic	**($4.15)**	**($0.30)**	**($0.55)**	**$0.39**	**($0.60)**	**($1.06)**	**($2.29)**	**($1.30)**	**($1.85)**	**($0.38)**	**($0.18)**	**$0.81**	**$3.08**	**$4.92**	**$6.01**	**$6.32**	**$7.65**	**$8.87**
Basic Shares (000)	**9,407**	**30,321**	**31,140**	**31,171**	**31,202**	**30,931**	**33,241**	**33,573**	**33,909**	**34,248**	**34,590**	**34,936**	**35,286**	**35,639**	**35,995**	**36,355**	**36,719**	**37,086**

Source: Leerink Partners estimates and company reports.
NTD=non-transfusion dependent.

DCF Calcuation

Discount rate	*10%*
Terminal Growth Rate	*1%*
Valuation ($M)	$1,835
Valuation / Share	**$57**

Source: Leerink Partners estimates.

XLRN DCF Valuation/Share Sensitivity Analysis

Terminal Growth Rate \ Discount Rate	8.0%	9.0%	10.0%	11.0%	12.0%
0.0%	$75	$63	$53	$45	$39
1.0%	$84	$69	**$57**	$48	$42
2.0%	$94	$76	$62	$52	$44
3.0%	$109	$85	$69	$57	$47
4.0%	$132	$99	$77	$62	$52

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.

Valuation

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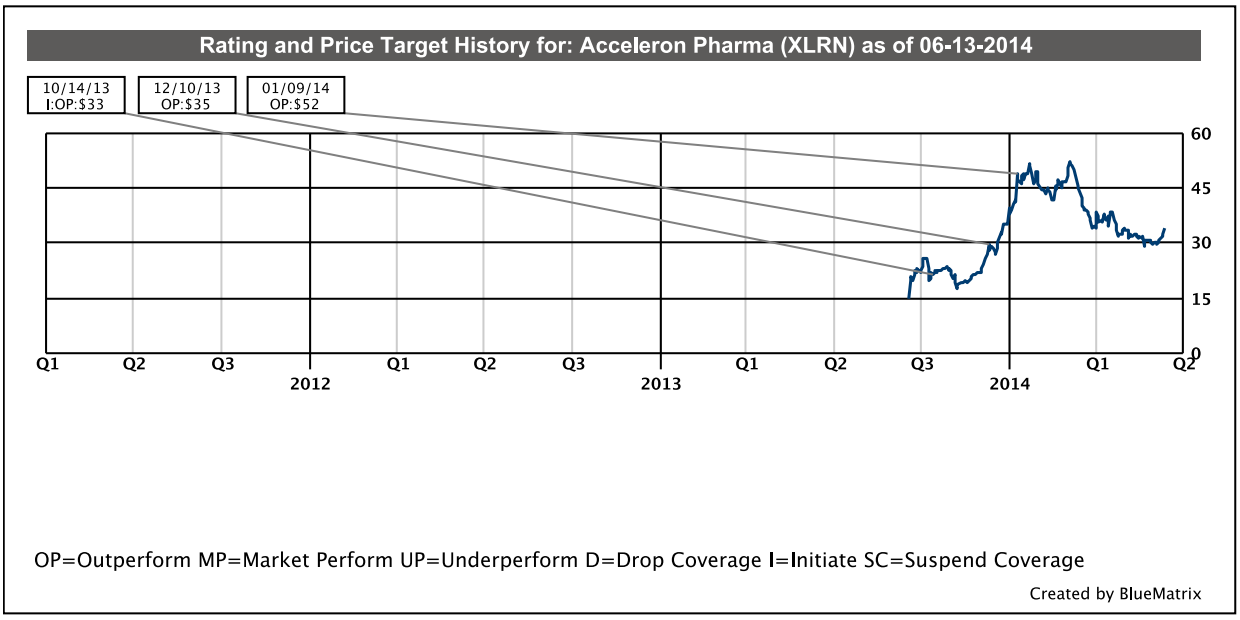

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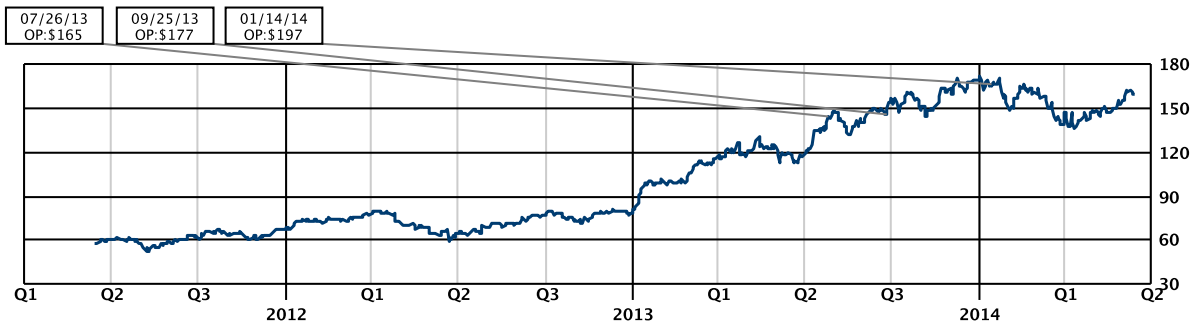

Leerink Swann initiated coverage of CELG with an Outperform rating on February 7, 2003. On June 11, 2013, Leerink Swann began a transition to specific price targets for the stocks under its coverage, replacing valuation ranges.

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				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	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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Leerink Partners LLC makes a market in Acceleron Pharma and Celgene, Inc.

Leerink Partners LLC has acted as the manager for a public offering of Acceleron Pharma in the past 12 months.

Leerink Partners LLC 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
	Alice C. Avanian, CFA	(617) 918-4544	alice.avanian@leerink.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
	Gena Wang, Ph.D., CFA	(212) 277-6073	gena.wang@leerink.com
	Jonathan Chang, Ph.D.	(617) 918-4015	jonathan.chang@leerink.com
	Paul Matteis	(617) 918-4585	paul.matteis@leerink.com
	Richard Goss	(617) 918-4059	richard.goss@leerink.com
Life Science Tools and Diagnostics	**Dan Leonard**	(212) 277-6116	dan.leonard@leerink.com
	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
	Aneesh Kapur	(617) 918-4576	aneesh.kapur@leerink.com
Specialty Pharmaceuticals	**Jason M. Gerberry, JD**	(617) 918-4549	jason.gerberry@leerink.com
Medical Devices, Cardiology & Orthopedics	**Danielle Antalffy**	(212) 277-6044	danielle.antalffy@leerink.com
	Richard Newitter	(212) 277-6088	richard.newitter@leerink.com
	Ravi Misra	(212) 277-6049	ravi.misra@leerink.com
Healthcare Services	**Ana Gupte, Ph.D.**	(212) 277-6040	ana.gupte@leerink.com
Healthcare Technology & Distribution	**David Larsen, CFA**	(617) 918-4502	david.larsen@leerink.com
	Christopher Abbott	(617) 918-4010	chris.abbott@leerink.com
Sr. Editor/Supervisory Analyst	**Mary Ellen Eagan, CFA**	(617) 918-4837	maryellen.eagan@leerink.com
Supervisory Analysts	Robert Egan		bob.egan@leerink.com
	Amy N. Sonne		amy.sonne@leerink.com
Editorial	Cristina Diaz-Dickson	(617) 918-4548	cristina.diaz-dickson@leerink.com
Research Assistant	Carmen Augustine	(212) 277-6012	carmen.augustine@leerink.com

New York
299 Park Avenue, 21st floor
New York, NY 10171
(888) 778-1653

Boston
One Federal Street, 37th Floor
Boston, MA 02110
(800) 808-7525

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