OUTPERFORM

Reason for report: **INITIATION**

Marko Kozul, M.D.

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

Irene Lau

(415) 905-7256



Kev Stats:

LEERINK SWANN

RECEPTOS, INC.

Emerging RPC-1063 Best in Class S1P Drives Our OP Rating & \$30 **Valuation**

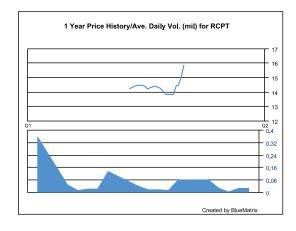
- Bottom Line: Our research and findings from the 2Q13 MEDACorp Multiple Sclerosis (MS) Survey suggest attractive revenue potential for RPC-1063 in Relapsing MS (RMS) with upside (NPV \$221M) provided by potential in Ulcerative Colitis (UC). RPC-4046 is a 2nd pipeline candidate that provides pipeline risk diversification and longer term upside. Multiple catalysts over the next 12 months should drive RCPT including potential: 1) 4Q13 ECTRIMS RPC-1063 data; 2) 1H14 RPC-1063 large pharma partner; 3) RPC-1063 Phase II data in RMS in mid-14; 4) RPC-1063 Phase II data in UC in mid-14, and 5) 1Q14 start of a pivotal Phase III RMS trial. We initiate coverage of RCPT with an Outperform rating & \$30 DCF valuation.
- RPC-1063 is early but emerging as the "Best in Class" S1P receptor modulator in the same class as NVS's (OP) Gilenya. Compared to Gilenya and other development stage S1Ps, RPC-1063 appears differentiated based on: 1) more rapid lymphocyte recovery (lower risk for infections), 2) improved cardiovascular (CV) profile (clean QTc, less bradycardia), and; 3) reduced hepatotoxicity (no LFTs so far).
- Gilenya predictions in the 2Q13 MEDACorp MS Survey reflecting continued overall growth (especially in EU) and meaningful utilization in secondary progressive MS (SPMS) patients reinforce potential for RPC-1063. This survey predicts 3-year (to mid-16) Gilenya market share growth to 9% (or ~\$1B) in US and higher to 12% (~\$1.4B) in EU. This represents an initial and attractive revenue base into which RPC-1063 could grow.
- RPC-1063 in the 2Q13 MEDACorp MS Survey generated significant participant enthusiasm reflected by substantial interest in switching patients to RPC from Gilenya (~58%), Tecfidera (~13%), and Tysabri (~15%) assuming approval in 2018. We estimate this switching could potentially generate ~\$1.2B in US revenue in 2019, the year after potential approval.
- SPMS could be a Win-Win opportunity for RPC-1063 driven either by a potential void that could be created by failure of Tysabri in the Phase III ASCEND trial (2015) or positive results of the Siponimod Phase III EXPAND trial (2016). Current Tysabri use in SPMS is predicted at ~41% of total use (and revenue) and penetrates 18%/14% of the SPMS market in US/EU. We calculate WW 2017 Tysabri SPMS revenue will amount to \$878M (of \$2.1B). Based on our research, we are biased that the ASCEND trial will fail in 2015 leading to decreased Tysabri SPMS revenue worth ~\$830M in FY16-20. We believe this void could be made up by Gilenya and other S1Ps such as Siponimod (NVS) that is the current focus of the EXPAND trial with data ~2016. If EXPAND is positive. this could accelerate RPC-1063 revenues if approved in 2018 for RMS.

(NASDAQ:RCPT)

HEALTHCARE EQUITY RESEARCH

S&P 600 Health Care Index:	981.46
Price:	\$15.87
Price Target:	\$30.00
52 Week High:	\$16.00
52 Week Low:	\$13.00
Shares Outstanding (mil):	17.6
Market Capitalization (mil):	\$279.3
Book Value/Share:	\$0.27
Cash Per Share:	\$4.59
Dividend (ann):	NA
Valuation:	~\$30 on DCF analysis
Cash Per Share: Cash per sha	are includes ~\$68M net

proceeds from IPO in May 2013.



Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2012A					\$8.6					(\$2.12)	NM
2013E	\$0.5	\$0.5	\$0.5	\$0.5	\$1.9	(\$0.88)	(\$0.51)	(\$0.54)	(\$0.83)	(\$2.76)	NM
2014E					\$0.6					(\$2.16)	NM

Source: Company Information and Leerink Swann LLC Research

Revenues in \$000s; EPS on basic shares in 2012; 1Q13E PF for IPO priced 5/8/13.



Table of Contents

Initiatio	on of Coverage	3
	Investment Thesis	5
	Upcoming Milestones	6
	RPC-1063 Product Profile	7
	Valuation	17
	Financial Model	19
	Management	20
	Multiple Sclerosis (MS) U.S. Market Model	22
MS Su	rvey Analysis	23
RPC-1	063 Clinical Trials	30
Graphs	s of MEDACorp MS Survey Analysis	33
MEDA	Corp MS Survey	81



LEERINK SWANN

The Healthcare Investment Bank

Receptos (RCPT): Initiation of Coverage with OP & \$30 Valuation

Marko Kozul, M.D. Research Analyst, Managing Director (415) 905-7221 marko.kozul@leerink.com Irene Lau Research Associate (415) 905-7256 irene.lau@leerink.com

Receptos (NASDAQ: RCPT) Company Overview



- Mid stage biopharmaceutical company focused on developing and commercializing immunology drugs with a lead candidate RPC-1063 for Multiple Sclerosis (MS) and also Ulcerative Colitis (UC)
- RPC-1063 (lead program) is an oral S1P1 receptor modulator similar to approved MS therapy Gilenya (NVS)
- Compared to other S1P1 candidates, RPC-1063 is earlier stage but also emerging as the "best in class" based on differentiation that includes: 1) rapid lymphocyte recovery;
 improved cardiovascular (CV) profile; 3) reduced hepatotoxicity; 4) other selective advantages
- RCPT has an ongoing Phase II Relapsing MS (RMS) trial and Phase II UC trial that will read out mid-2014; Phase III trials in MS both with Special Protocol Assessments (SPA) are planned for 4Q13/1Q14 and 2H14
- Based on emerging, strong and differentiated Phase I RMS data to date for RPC-1063, a known class of therapeutic (S1P1), we estimate likelihood of FDA approval at ~30% and model first sales in 2018E
- RPC-4046 (2nd lead program), a monoclonal antibody vs. IL-13 for Eosinophilic Esophagitis (EoE), provides pipeline risk diversification and longer term upside

Receptos (RCPT): Investment Thesis



- RCPT shares 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"
- 2014 pivotal year: Plan to sign an RPC-1063 partnership with large pharma, announce RPC-1063 Phase II data in relapsing MS (RMS) and UC and start 2 pivotal Phase III RMS trials
- We currently assume a 30% probability of approval for RPC-1063 in RMS in 2018.
- ➤ 2Q13 MEDACorp 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 2019
- 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 ~\$640M (or \$2.1B non-risk adjusted) which leads to our base case NPV calculation of \$520M (including cash), based only on approval and use in RMS
- Positive RPC-1063 data in UC could lead to upside in our base case NPV of \$211M by adding risk-adjusted peak sales up to \$300M in 2029

Upcoming Milestones



Product	Partner	Indication	Phase	Est. Timing	Milestone
				4Q13/1Q14	Initiate Phase III portion of 1st pivotal (RPC01-201), (with SPA)
				4Q13	ECTRIMS updates (i.e., TQTc data)
				2014	Partnership announcement
		Relapsing MS	Phase III	2H14	Initiate 2 nd pivotal Phase III RMS trial (with SPA)
		Trelapsing Mo	i nase iii	Mid-2014	Phase II data of 1st pivotal (RPC01-201) trial
RPC-1063	Proprietary			2017	2nd pivotal Phase III RMS trial data
(S1P1)				YE17	NDA submission
				2H18	FDA Approval
		Ulcerative	Phase II	1H14	Complete trial enrollment
				3Q14	Phase II UC trial data (might serve as 1 of 2 pivotal trials)
		Colitis (UC)		2015	Initiate pivotal trial (possibly maintenance)
				2018	Possible NDA submission
DDC 4046		Eosinophilic		4Q13/1Q14	Submit IND
RPC-4046 (IL-13)	ABBV	Esophagitis	Phase II	1H14	Initiate Phase II data
(12-13)		(EoE)		2H15	Phase II trial data

RPC-1063 Product Profile



- RPC-1063 is an oral S1P1 receptor modulator with similar Method of Action (MOA) to approved MS therapy Gilenya (NVS)
- Other S1P1 receptor modulators: ONO-4641 (SEO), Siponimod/BAF-312 (NVS) and Ponesimod (ATLN)
- To date, RPC-1063 has been evaluated in 130 subjects in Phase I trials and appears to have equal efficacy while demonstrating increasingly important safety differentiation from Gilenya and other development stage S1P1 receptor modulators
- In early clinical development, efficacy for S1P1 compounds is measured by pharmacodynamic (PD) markers such as lymphocyte reductions which correlate with standard Phase II end points measuring reductions in Mean Number of Gd+1 T1 Lesions by MRI and Phase III end points of reductions in Annualized Relapse Rates (ARR)

RPC-1063 Key Differentiations/Advantages



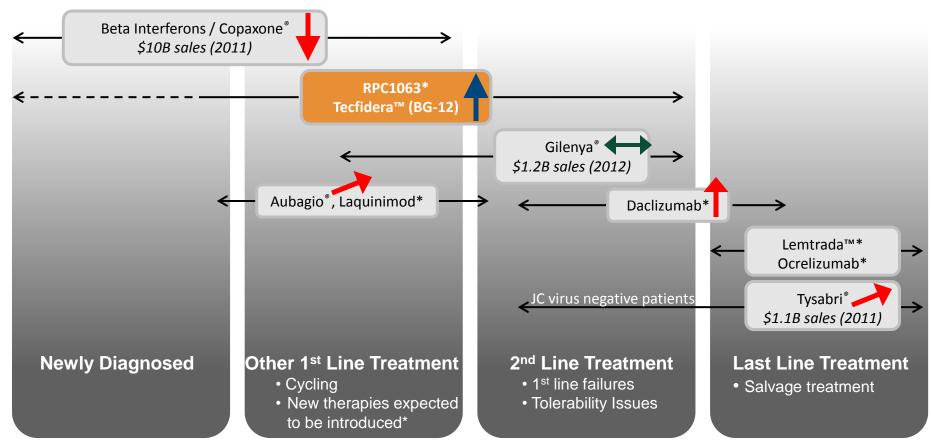
Emerging RPC-1063 Differ	entiation vs. Competing S1P1 Receptor Modulators:	
Differentiator:	RPC-1063 Comment:	Impact:
Lymphocyte Reductions during treatment:	RPC-1063 reduces lymphocytes by 50-70% which is in-line with the other competing S1P1 compounds:	RPC-1063 should demonstrate efficacy in- line with Gilenya and other development stage S1P1 candidates
Rapid Lymphocyte Recovery:	 RPC-1063's shortest half-live → More rapid lymphocyte recovery: RPC-1063: T1/2=19hrs; 3 day recovery Gilenya: T1/2=168hrs; 4-8 week recovery Siponimod: T1/2=30hrs; 1week recovery Ponesimod: T1/2=30hrs; 3-6 day recovery ONO: TBA 	RPC-1063 should have a lower risk for opportunistic infections
Potential Improved Cardiovascular (CV) profile:	First Dose Heart Rate Monitoring (FDHRM): Standard for Gilenya and appears to be class effect Improved with RPC-1063 vs. all others	Assume RPC-1063 will need FDHRM so lack of it would lead to significant upside
Potential for Reduced Hepatotoxicity:	Liver Function Test (LFTs) >3x Upper Limit Normal (ULN) RPC-1063: None (over 28 days dosing trial) Gilenya: 8% Siponimod: 4.3% Ponesimod: 2.8-4.5% ONO: 5.9-14.2%	RPC-1063 has Minimal/No impact on Liver
Potential for other Advantages (TQT, Fibrosis):	Thorough QT Study (TQTc) Trial Result: RPC-1063: NO QTc prolongation Gilenya: Yes QTc prolongation Other S1P1 candidates: Unavailable	RPC-1063 has NO QTc prolongation → NO potential to cause heart arrhythmias

Source: Company Reports, Leerink Swann.

RPC-1063 Positioning in MS Market



Oral Therapy Expected to Expand Usage as New Mechanisms Become Available



Source: Company Reports, 2Q13 MEDACorp MS Survey and Leerink Swann Estimates.

Highlights from Leerink Swann Biopharma MS Survey in 2Q13



- MEDACorp 95-neurologist (45 US, 40 EU and 10 Canadian) survey assessed current and future trends in the treatment of multiple sclerosis (MS) through end of 2015
- Tecfidera: Forecast to become the market leader among new relapsing multiple sclerosis (RMS) patients with 16% share within 3 years
- ➤ ABCRs : Tecfidera growth would drive rapid declines in ABCR (Avonex, Betaseron, Copaxone, Rebif) share, especially Avonex and Copaxone
- ➢ Gilenya ←→ : Will be challenged by Tecfidera in North America but continue to do well in the EU
- Aubagio, Lemtrada >> : Growth would be very modest
- Tysabri : Will be challenged by Tecfidera in North America but continue to do well in the EU
- ➤ Daclizumab, Ocrelizumab : Not a major focus of survey given approval in out years (2015+) but MEDACorp KOLs previously estimated Daclizumab could take market share from all MS therapies except orals i.e., Tecfidera, RPC-1063, etc Source: Company Reports, 2Q13 MEDACorp MS Survey and Leerink Swann Estimates.

RPC-1063 Pivotal Phase III RMS Trial Designs



- RCPT has negotiated Special protocol Assessments (SPAs) for its 2 RMS pivotal Phase III trials
- 1st Phase III RMS Trial RADIANCE (start 4Q13/1Q14): Compare superiority of RPC-1063 vs. Avonex (BIIB)

RADIANCE 1st Phase	III Pivotal RPC-1063 Relapsing Multiple Sclerosis (RMS) Trial (RPC01-201):							
Purpose:	Determine whether RPC-1063 is superior to Avonex in relapsing multiple sclerosis (RMS).							
# pts:	N>900 (>1,100 in Phase II + Phase III)							
Design:	Interventional randomized, safety/efficacy, parallel assignment, double blind (subject, caregiver,							
	investigator, outcomes assessor) treatment study							
SPA: Yes								
Trial Arms:	Arm A: 0.5mg RPC-1063 oral daily (QD) for 2+ years							
	Arm B: 1mg RPC-1063 oral daily (QD) for 2+ years							
	Arm C: Avonex 30µg intramuscular (IM) weekly for 2+ years							
Primary End Point:	Assess if RPC-1063 is superior to Avonex in reducing Annual Rate of Relapse (ARR) over 2 yrs							
Start:	4Q13/1Q14							
End:	2017							
Sponsors:	Receptos							
Clin.Trials.	NCT01628393							
Gov.ID	RPC01-201							

Source: clinicaltrials.gov

RPC-1063 Pivotal Phase III RMS Trial Designs (Cont'd)



- Second Phase III RMS Trial: Current plan is to compare superiority of RPC-1063 vs. Avonex (BIIB) but other options are Tecfidera (BIIB) and Avonex (BIIB)
- Choice of comparator in second Phase III RMS trial may benefit commercial positioning of RPC-1063
- Upside Possible Related to Choice of second Phase III RMS Comparator: Choice of Tecfidera as comparator would be aggressive and risky but also significantly improve commercial potential

RPC-1063 Opportunity in Ulcerative Colitis (UC)



- Inflammatory Bowel Disease (IBD) includes both Ulcerative Colitis and Crohn's Disease
- Current therapies for IBD include immunomodulators (methotrexate, etc), anti-inflammatory drugs (mesalazine, budesonide, hydrocortisone, etc) and biologics (Remicade, Humira, Tysabri, Cimzia)
- There are ~795K US patients suffering from UC Biologics are used as a fourth line treatment (~120K patients)
- Remicade and Humira combined sales in UC surpass \$700M in 2012
- Not included in our model but based on the above, we conservatively estimate a peak sales of \$300M in UC, or \$211M in NPV

Gilenya Intellectual Property



Gilenya has 3 issued patents listed on the Orange Book:

Patent Number	Description	Expiration Date
5,604,229	Drug substance patent (a seminal patent)	2/18/2019 (Hatch Waxman Extension Granted)
8,324,283	Solid pharmaceutical compositions comprising a SIP receptor agonist and a sugar alcohol	3/29/2026
6,004,565	Compositions and methods of using compositions with accelerated lymphocyte recovery immunosuppressive properties	9/23/2017

Source: Orange book.

Model Assumptions



Pricing

- On par with Gilenya in 2018 and 2019
- Generic pricing beyond 2020 at 1/3 of branded price
- Year-over-year price increase of 3% from 2013 and beyond
- EU pricing assumed at 70% of US pricing; no price increase
- Probability of Success (At Current Stage)
 - Assumes 30% probability of success on WW RPC-1063 MS revs
- Discount Rate of 11% and Terminal Growth Rate of 1% (translates to a 10x terminal multiple)

Key Sources of Future Upside



- ➤ RPC-1063 RMS Clinical Data: Continued RPC-1063 clinical differentiation demonstrating "best in class" S1P1 receptor modulator that could lead to increased penetration vs. our current estimates.
- ➤ **Gilenya Intellectual Property (IP):** Successful prolongation of Gilenya IP beyond its current 2019 expiration would allow RCPT to maintain branded pricing and compete against branded Gilenya rather than lower priced generics.
- S1P1 Growth in SPMS: An underappreciated emerging theme is use of Gilenya in Secondary progressive MS (SPMS). Modeling RPC-1063 use in SPMS would lead to upside and could be driven by: 1) growth in off-label use of Gilenya in SPMS; 2) Tysabri failure in the Phase III ASCEND SPMS trial in 2015; 3) positive data for other S1P1 candidates in SPMS (i.e. Siponimod-NVS).
- > RPC-1063 UC Clinical Data: We currently do not include any UC derived revenue in our model and we estimate positive data could lead to upside in our base case NPV of \$211M by adding risk adjusted peak sales up to \$300M in 2029.
- > RPC-4046 EoE Clinical Data: RPC-4046 provides longer term pipeline risk diversification and upside.

RCPT DCF Valuation



	RCPT Cash Flow (\$000s, except per share data)																	
	2012A	1Q13E	2Q13E	3Q13E	4Q13E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
Cash Flow from Operating Activities																		
Net Loss	(\$17,710)	(\$7,417)	(\$8,917)	(\$9,667)	(\$14,867)	(\$40,867)	(\$50,006)	(\$85,703)	(\$109,696)	(\$145,350)	(\$79,504)	\$106,785	(\$35,401)	(\$1,387)	\$48,716	\$103,443	\$150,167	\$198,179
Adj to Reconcile losses to net cash in Op Act																		
Depreciation and amortization expense	\$656	\$250	\$250	\$250	\$250	\$1,000	\$2,000	\$2,000	\$2,000	\$2,000	\$2,000	\$2,000	\$2,000	\$2,000	\$2,000	\$2,000	\$2,000	\$2,000
Deferred revenue	(\$2,228)	-	-	-	- 1	-	-	-	-	-	-	-	-	-	-	-	-	-
Stock-based compensation	220	\$66	\$78	\$102	\$230	\$856	\$1,266	\$2,571	\$3,291	\$4,361	\$4,808	\$5,975	\$6,320	\$6,668	\$6,939	\$7,203	\$8,465	\$8,682
Deferred rent	27				- 1													
Changes in operating assets and liabilities:																		
Prepaid expenses and other assets	(278)				- 1													
Accounts payable and accrued expenses	679																	
Accrued payroll	216				- 1													
Net cash used in operating activities	(18,418)	(7,101)	(8,588)	(9,315)	(14,386)	(39,011)	(46,739)	(81,132)	(104,405)	(138,990)	(72,696)	114,760	(27,082)	7,282	57,654	112,646	160,632	208,861
Cash flows from investing activities					- 1													
Purchases of property and equipment	(\$214)	(\$54)	(\$54)	(\$54)	(\$54)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)
Net cash used in investing activities	(\$214)	(\$54)	(\$54)	(\$54)	(\$54)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)	(\$214)
Free Cash Flow	(\$18,632)	(\$7,154)	(\$8,642)	(\$9,369)	(\$14,440)	(\$39,225)	(\$46,953)	(\$81,346)	(\$104,619)	(\$139,204)	(\$72,910)	\$114,546	(\$27,296)	\$7,068	\$57,440	\$112,432	\$160,418	\$208,647

Source: Leerink Swann estimates and company reports.

DCF Calcuation

201 Gallouation	
Discount rate	11%
Terminal Growth Rate	1%
NPV FCF	\$473,400
Valuation / Share	\$30

Source: Leerink Swann estimates.

RCPT DCF Valuation Sensitivity Analysis (\$M)											
	<u>Di:</u>	scount Rate	•								
		9.0%	10.0%	11.0%	12.0%	13.0%					
Φ	0.0%	\$45	\$35	\$27	\$21	\$16					
h Rat	1.0%	\$49	\$38	\$30	\$23	\$18					
srowt	2.0%	\$55	\$42	\$32	\$25	\$19					
Terminal Growth Rate	3.0%	\$63	\$47	\$36	\$27	\$21					
Term	4.0%	\$74	\$54	\$40	\$30	\$23					

Source: Leerink Swann estimates.

Valuation and Risks to Valuation



Valuation:

RCPT shares are poised to appreciate near and longer term driven by clinical progress and the commercialization of lead compound RPC-1063. We apply a 12-month valuation of RCPT shares of ~\$30 a share based on a discounted cash flow analysis. 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.

Risk 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 Income Statement



Assumes generic pricing in 2020

								cept per sha										
	2012A	1Q13E	2Q13E	3Q13E	4Q13E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
Revenues																		
RPC1063 WW Revenue											\$316,680	\$1,088,253	\$569,24	\$768,848	\$1,003,905	\$1,257,093	\$1,470,700	\$1,664,873
Risk Adjusted RPC1063 WW Revenue											\$95,004	\$326,476	\$170,772	\$230,654	\$301,171	\$377,128	\$441,210	\$499,462
RPC4046																		
Ono	\$6,088	\$483	\$483	\$483	\$483	\$1,933	\$644											
Eli Lilly	\$2,480	-	-	-	-	-	-	-	-	-	-	-	_ -	-	-	-	-	-
Ortho McNeil Janssen	\$79												<u> </u>					
Collaborative Revenue	\$8,647	\$483	\$483	\$483	\$483	\$1,933	\$644	-	-	-	-	-		-	-	-	-	
Total Revenue	\$8,647	\$483	\$483	\$483	\$483	\$1,933	\$644	-	-	-	\$95,004	\$326,476	\$170,772	\$230,654	\$301,171	\$377,128	\$441,210	\$499,462
Costs and Expenses																		
Probability Adjusted COGS	_	_	_	_	_	_	_	_		_	\$14,251	\$48,971	\$25,616	\$41,518	\$54,211	\$67,883	\$79,418	\$74,919
R&D	\$22,927	\$6,500	\$7,700	\$8,250	\$13,250	\$35,700	\$42,840	\$77,112	\$100,246	\$112,275	\$121.257	\$127,320	\$133,686	\$140,370	\$144.581	\$148,919	\$151.897	\$154,935
SG&A (Risk Adjusted from Time of RPC1063 Launch)	\$3,430	\$1,400	\$1,700	\$1,900	\$2,100	\$7,100	\$7,810	\$8,591	\$9,450	\$33,075	\$39,000	\$43,400	\$46,872	\$50,153	\$53,664	\$56,884	\$59,728	\$62,117
Total Costs and Expenses	\$26,357	\$7.900	\$9.400	\$10,150	\$15,350	\$42,800	\$50,650	\$85,703	\$109,696	\$145,350	\$174,508	\$219,691	\$206,174	\$232,041	\$252,456	\$273,685	\$291,043	\$291,971
Total Goots and Expenses	Ψ20,001	ψ1,500	ψ3,400	φ10,100	ψ10,000	ψ-12,000	ψου,οοο	ψου, εου	ψ105,050	ψ140,000	ψ17-4,000	Ψ213,031	Ψ200,174	Ψ 2 0 2 ,041	Ψ202,400	Ψ210,000	Ψ251,045	\$231,371
Operating Income (EBIT)	(\$17,710)	(\$7,417)	(\$8,917)	(\$9,667)	(\$14,867)	(\$40,867)	(\$50,006)	(\$85,703)	(\$109,696)	(\$145,350)	(\$79,504)	\$106,785	(\$35,401)	(\$1,387)	\$48,716	\$103,443	\$150,167	\$207,491
Y/Y growth																		
Income Before Taxes	(\$17,710)	(\$7,417)	(\$8,917)	(\$9,667)	(\$14,867)	(\$40,867)	(\$50,006)	(\$85,703)	(\$109,696)	(\$145,350)	(\$79,504)	\$106,785	(\$35,401)	(\$1,387)	\$48,716	\$103,443	\$150.167	\$207,491
Provision for Taxes	(, , ,		(, ,	(,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,	(, ,,	(, ,,,,,,,	-	-	-	-	-	-	-	-	-	-	-	9,312
Net income	(\$17,710)	(\$7,417)	(\$8,917)	(\$9,667)	(\$14,867)	(\$40,867)	(\$50,006)	(\$85,703)	(\$109,696)	(\$145,350)	(\$79,504)	\$106,785	(\$35,401)	(\$1,387)	\$48,716	\$103,443	\$150,167	\$198,179
EPS (LPS) Basic	(\$2.12)	(\$0.88)		(\$0.54)	(\$0.83)	(\$2.76)	(\$2,16)	(\$2.57)	(\$2.66)		(\$1.89)		(\$0.83)	(\$0.03)		\$2.34	\$3,37	\$4.40
Y/Y growth	,,,	,,,,,,,	,,,,,,	,,,,,,	,,,,,,,,	(*=,	(*=::0)	(+=)	(4-100)	(401.10)	(4)	7	(40.00)	(40.00)	****	, , , , , , , , , , , , , , , , , , ,	.,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,	
, , g. s. a.																		
Basic Shares* (000)	8,367	8,451	17,605	17,781	17,958	18,138	23,138	33,369	41,203	41,615	42,031	42,452	42,876	43,305	43,738	44,175	44,617	45,063

Source: Leerink Swann estimates and company reports.

^{*} Basic shares for 2012A and 1Q13E are pro forma for IPO priced on 5/8/13.

Management



- Faheem Hasnain (President, CEO and director since Nov-2010) Prior to joining RCPT, Mr. Hasnain was the President and CEO and a director of Facet Biotech, 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, CEO and a director of PDL BioPharma 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, most recently as Executive Vice President in charge of the oncology/rheumatology strategic business unit.
- Graham Cooper (CFO). Prior to joining RCPT, during 2012, Mr. Cooper was the EVP, Finance and CFO of Geron Corporation, a biopharmaceutical company focused on cancer therapies. From 2006 until 2011, Mr. Cooper served as SVP, CFO and Treasurer of Orexigen Therapeutics, a biotechnology company focused on obesity. From 1999 to 2006, Mr. Cooper held positions of increasing responsibility including Director, Health Care Investment Banking at Deutsche Bank Securities.
- Marcus Boehm, Ph.D. (CTO since October 2011) Dr. Boehm served as RCPT's VP of Chemistry from May 2009 to October 2011, and he is a Co-Founder. From 2007 to 2009, Dr. Boehm served as the VP of Chemistry for Apoptos, which we acquired in May 2009. From 2006 to 2007, Dr. Boehm held the position of Senior Director of Chemistry at Biogen Idec with responsibility for multiple medicinal chemistry programs and served as the head of chemistry for the San Diego site. Dr. Boehm formerly served as VP of Chemistry at Conforma Therapeutics until its acquisition by Biogen Idec in 2006.
- Sheila Gujrathi, M.D. (CMO since June 2011) She joined RCPT from Bristol-Myers Squibb where she was VP of the Global Clinical Research Group in Immunology from 2008 to 2011. Prior to joining BMS, Dr. Gujrathi worked at Genentech where she held roles of increasing responsibility in the Immunology, Tissue Growth and Repair clinical development group from 2002 to 2008. From 1999 to 2002, Dr. Gujrathi was a management consultant at McKinsey & Company in the healthcare practice where she provided strategic advice on a variety of projects in the healthcare and pharmaceutical industry.

Receptos: Risks



- > RPC-1063
 - Clinical risk for Phase II RMS and UC Trials
 - Longer term treatment (beyond 28 days) resulting in unforeseen toxicities
 - Competitive risk from other S1P1 candidates and other novel drugs entering the MS and UC market
 - Financing risk

MS Market Model



US Sales	2013E	2014E	2015E	2016E	2017E
MS patients, US (000s)	448	457	466	476	485
% y/y grow th	2%	2%	2%	2%	2%
% MS Patient that are Compliant	43%	43%	43%	43%	43%
Compliant MS patients, US (000s)	191	194	198	202	206
% y/y grow th	2%	2%	2%	2%	2%
% Market Share					
Avonex	20.4%	18.0%	15.5%	12.0%	10.1%
Betaseron	9.0%	8.0%	7.0%	6.0%	5.0%
Rebif	15.0%	14.0%	14.0%	13.5%	13.0%
Copaxone (incl. 3x-w eekly)	30.0%	27.0%	23.5%	14.7%	9.9%
Tecfidera	4.0%	11.0%	16.5%	21.0%	24.0%
Tysabri	11.4%	11.7%	11.8%	11.8%	11.7%
Gilenya	8.6%	8.2%	8.0%	8.0%	7.9%
Aubagio	1.5%	2.1%	2.6%	3.1%	3.5%
Other orals (laquinimod, novel S1P1)	0.0%	0.0%	0.0%	0.0%	0.9%
ABCR generics (Copaxone in 2016)				7.0%	10.0%
Other biologics	0.0%	0.0%	1.0%	3.0%	4.0%
TOTAL	100%	100%	100%	100%	100%
US pricing - per Patient/Year (000s)					
Avonex	47	48	50	51	53
Betaseron	42	44	45	46	48
Rebif	47	49	50	52	53
Copaxone	52	55	57	57	57
Tecfidera	54	56	57	59	61
Tysabri	48	50	51	53	54
Gilenya	56	57	58	59	60
Aubagio	42	42	43	44	45
Other orals	56	57	58	59	60
Other biologics	42	44	45	46	48
U.S. Revenue (\$000s)					
Avonex	\$1,825	\$1,692	\$1,531	\$1,245	\$1,100
Betaseron	\$728	\$680	\$625	\$563	\$493
Rebif	\$1,347	\$1,321	\$1,388	\$1,406	\$1,423
Copaxone	\$2,977	\$2,867	\$2,673	\$1,701	\$1,173
Tecfidera	\$411	\$1,189	\$1,874	\$2,505	\$3,008
Tysabri	\$1,052	\$1,133	\$1,206	\$1,265	\$1,314
Gilenya	\$915	\$900	\$915	\$950	\$985
Aubagio	\$120	\$175	\$225	\$275	\$325
Other orals (laquinimod)	\$3	\$2	\$6	(\$4)	\$106
Other biologics	\$0	\$0	\$89	\$282	\$394
TOTAL US MS Revenue (\$MM)	\$9,379	\$9,960	\$10,531	\$10,188	\$10,322
% y/y growth	11%	6%	6%	-3%	1%
Source:Leerink Swann Estimates.					

2Q13 MEDACorp MS Survey:

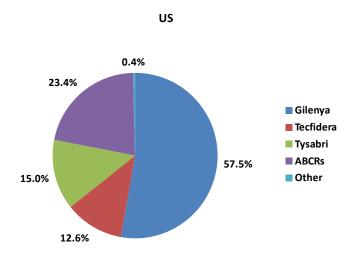
Participants & Sample Size (Pages 34-40): Participating in this 2Q13 survey were 95 neurologists (45 U.S., 40 EU and 10 Canadian). None of these participants could be investigators in ongoing clinical trials (vs. the 10.15.12 survey that could). Overall, the survey captured 25,443 U.S. and 15,035 EU total MS patients who we believe represent 6% and 2.1% of all patients in these geographies. In terms of patients with Relapsing MS (RMS), the survey captured 10,185 U.S. and 4,258 EU patients who we believe equate to ~4% and ~1% of all RMS patients in these geographies. Important to understanding the context of survey participant responses is their prediction of underlying annual growth rate for RMS. In the U.S. and EU participants estimate a 12% and 15% annual RMS growth rate, respectively. For patients with Secondary progressive MS (SPMS), the survey captured 2,936 U.S. and 1,569 EU patients. According to BIIB, ~35% of all MS patients have SPMS and, based on this assumption, we believe our sample size equates to ~2% and ~1% of all SPMS patients in these geographies. For SPMS, participants estimate a U.S. and EU annual growth rate of ~11% and 9%, respectively.

Novel MS Therapeutic Agents (Pages 52-56): Participants were very familiar (>50%) with Tecfidera (BIIB), Lemtrada (SNY), Aubagio (SNY), Laquinimod (TEVA), and somewhat familiar (≤50%) with Daclizumab (BIIB-ABBV) and Ocrelizumab (BIIB-Roche). They were very unfamiliar (<18%) with the class of emerging S1P receptor modulators with a mechanism of action (MOA) similar to Gilenya that includes RPC-163 (RCPT), Siponimod (NVS), Ponesimod (ATLN), and ONO-4641 (SEO).

Watch-out Longer Term for Emerging S1P Receptor Modulators (Page 79-80): Despite low current survey participant familiarity (<18%) with the emerging class of S1P receptor modulators, multiple candidates are advancing through clinical trials that could lead to approvals in the 206-2018 timeframe. We provided survey participants with a general product profile for an emerging "best in class" S1P receptor modulator and compared its emerging clinical data to the Gilenya FDA approval label (Page 47). If approved in 2018, the survey predicts this "best in class" S1P receptor modulator could take meaningful market share for all classes of currently marketed MS therapies (Page 48). Based on 2019 Leerink Swann projections for Tecfidera, Gilenya and Tysabri, we calculate this best in class compound would hypothetically garner \$1.2B in U.S. revenue if survey predictions are directly applied.

	2Q13 MS Survey U.S. :	LS 2019 Estimate:	Implied Revenue Potential:
Gilenya:	57.5%	\$1.1B	\$632M
Tecfidera:	12.6%	\$2.7B	\$340M
Tysabri:	15.0%	\$1.4B	\$210M
			Total: ~\$1.2B

Source: MEDACorp Survey, "Trends in Multiple Sclerosis (MS) Treatment, May 2013



Source: MEDACorp Survey and Leerink Swann Estimates.

3-Year MS Landscape Predictions from mid-13 to mid-16 (Pages 52-56): The survey predicts generous declines in use of all ABCR therapies over the next 3 years with a concurrent rise in use of novel MS therapies across all geographies. In aggregate, Avonex (A) use would decline from 22% currently to 12% (-10%), Betaseron (B) from 13% to 10% (-3%), Copaxone (C) from 22% to 14% (-7%) and Rebif (R) from 18% to 11% (-7%) over this period. Meanwhile, novel therapy use would grow with Tecfidera the most (+15%) and Tysabri the least (+2%). In aggregate Gilenya, Laquinimod, Aubagio and Lemtrada are expected to grow modestly by +3-4%. In the U.S., Tecfidera predicted growth is more pronounced (+19%) while Lemtrada is least robust (+2%). Interestingly in the EU, Tecfidera predicted growth is less pronounced (+9%) while Gilenya is more pronounced (+5%) even marginally surpassing total Tecfidera penetration EU mid-2016 by +2% (12% vs. 10%).

	2Q13 Survey (May-20	13):	4Q12 Survey (Oct-2012):		
Aggregate	Peak Share:	3 Year Growth:	Peak Share:	3 Year Growth:	
Tecfidera:	16% (U.S. 21% vs. EU 10%)	+15%	15% (U.S. 17% vs. EU 13%)	15%	
Tysabri:	9% (U.S. 10% vs. EU 9%)	+3%	10% (U.S. 10% vs. EU 12%)	+2%	
Gilenya:	10% (U.S. 9% vs. EU 12%)	+4%	10% (U.S. 7% vs. EU 13%)	+6%	

Source: MEDACorp Survey, "Trends in Multiple Sclerosis (MS) Treatment, May 2013

Stable Disease MS Patients (Page 57): The survey predicts ~33% of stable disease patients are expected to switch to a new therapy over the next 3-years (mid-2016). In aggregate, 14% may switch at 6 months, 22% after 18 months and ~30% after 3 years, suggesting fairly linear growth in switching to a new therapy during this period.

Treatment Naïve MS Patients (Pages 58-61): The survey predicts Tecfidera will take greatest market share for treatment naïve MS patients. In aggregate, Tecfidera is expected to growth to a total 22% market share while for Gilenya this would be 9%, Aubagio 7%, Tysabri 6%, and

Laquinimod 4%. In the U.S. the Tecfidera trend is more pronounced with total predicted at 29% while in the EU it is less pronounced with 13%. For Gilenya, the U.S. trend is less robust achieving 9% share while it is more pronounced in the EU to 11%.

Aggregate	2Q13 Survey (May-2013):	4Q12 Survey (Oct-2012):
Aggregate	Peak Share:	Peak Share:
Tecfidera:	22% (U.S. 29% vs. EU 13%)	20% (U.S. 24% vs. EU 14%)
Gilenya:	9% (U.S. 9% vs. EU 11%)	11% (U.S. 8% vs. EU 15%)

Source: MEDACorp Survey, "Trends in Multiple Sclerosis (MS) Treatment, May 2013

"Warehousing" of Patients and Early Tecfidera Experience (Pages 62-65): The survey predicts 3-5% of patients were (are) being "warehoused" in preparation for approval of Tecfidera. In the U.S. this was ~4% of patients while in the EU it is closer to ~5%. In the first 5-6 weeks of the U.S. launch, participants wrote Tecfidera for ~2.6% (266/10,185) of all their RMS patients with ~30% (78/266) filling their prescription. During this period, ~87% of U.S. physicians indicated they had not experienced insurer reimbursement pushback that would limit prescribing of this drug. Results suggest the greatest sources of patients coming to Tecfidera therapy are from Avonex (24%), Copaxone (21%) and treatment naïve patients (19%).

Tecfidera

3%

12%

19%

Avonex

Betaseron

Copaxone

Rebif

Tysabri

Gilenya

U.S. (n=21): Previous Status of Current RMS Patients on Tecfidera

Source: MEDACorp Survey, "Trends in Multiple Sclerosis (MS) Treatment, May 2013

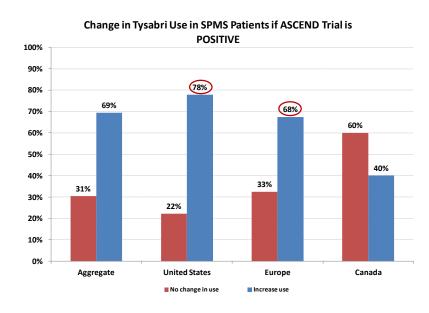
U.S. Physician Satisfaction with Newer MS Treatment Options (Pages 66-69): In the U.S., Gilenya (3.8) fared slightly better than Tecfidera (3.6) in terms of overall satisfaction despite scoring lowest in treatment initiation logistics (2.9). Gilenya scored toward the top in the categories of drug tolerability (3.9) and frequency of patient complaints (3.8). Copaxone score highest in terms of treatment initiation logistics (4.4), physician practice support (4.3), drug tolerability (4.0) and patient reimbursement support (3.9). Despite only recent availability on the market in the U.S., Tecfidera apparently scored toward the top in terms of physician practice support (4.0) but at the lower end in terms of patient reimbursement support (3.3).

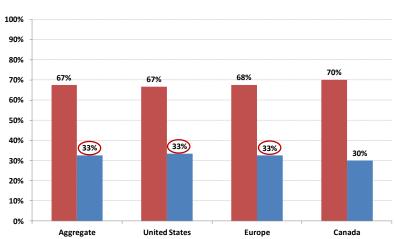
Copaxone Predictions (Pages 70-72): Assuming approval of Copaxone dosed 3x/week (vs. current daily formulation), U.S. physicians predict switching slightly >50% of current Copaxone patients to the new formulation. Physicians in Europe (~42%) and Canada (24%) predict lower switching rates. If generic Copaxone were to be approved it would take most share (6-8%) from Interferons with only marginal erosion to Gilenya, Tecfidera or Aubagio. More than half of physicians believe it would be unethical to require that an AB-rated Copaxone be used as front-line therapy.

SPMS Treatment Experience (Pages 73-76): While Interferons appear to be the mainstay of SPMS therapy, Tysabri and Gilenya are also used to a meaningful degree. In the U.S. and EU, Interferons represent 41% & 43% of all treatments for SPMS patients while Tysabri represents 18% &14% and Gilenya 10% & 8%, respectively. Around 65% believe Tysabri provides some benefit including stable/slowing of progression (27%), a general benefit (23%) and reducing relapses (15%). On the other hand, around half of physicians believe Gilenya provides some degree of benefit to SPMS patients including stable/slowing of progression (32%), a general benefit (12%) and reducing relapses (4%).

Tysabri Revenue Estimates in SPMS: Given meaningful SPMS contribution to overall Tysabri revenues, we believe it is important to quantify sales specific to this indication. The 2Q13 survey predicts Tysabri use in SPMS is ~41% of its total use in MS (RMS is ~59%). Based on our FY13-17E Tysabri WW projections of \$1.9B, \$2B, \$2.1B, \$2.2B and \$2.4B, we estimate SPMS use would represent \$767M, \$823M, \$878M, \$928M and \$973M during these years.

Impact on Revenue from Tysabri Phase III ASCEND Trial data in 2015 (Pages 77-78): We were surprised to find that survey results suggest a positive ASCEND result would lead the majority of U.S. (78%) and EU (68%) physicians to prescribe more Tysabri while a negative outcome would cause only 33% to prescribe less Tysabri.





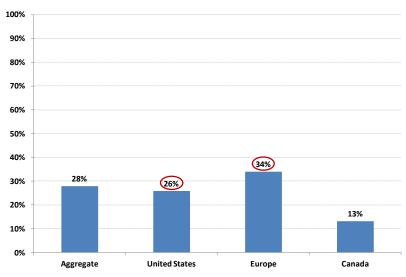
Change in Tysabri use in SPMS Patients if ASCEND Trial is NEGATIVE

Source: MEDACorp Survey, "Trends in Multiple Sclerosis (MS) Treatment, May 2013

Decrease in use

We believe this disproportionate impact to positive/negative data is related to where Tysabri is currently being used in SPMS. Previously MEDACorp KOLs suggested that the majority of their Tysabri use in SPMS occurred in patients who continue to experience relapsing disease despite an SPMS diagnosis. According to the National MS Society, in SPMS the disease continues to progress whether or not patients experience relapses and over time most people experience fewer inflammatory relapses or none at all. According to this survey (Page 73), in the U.S. and EU ~26% and 34% of SPMS patients continue to have relapses after diagnosis. Thus, survey results suggest a positive ASCEND Trial would encourage significantly more Tysabri utilization and penetration into the SPMS patients without relapses (i.e., 74% and 66% of all SPMS patients) while a negative result would likely only diminish use in SPMS patients that continue to experience relapses (26% and 34%).

% SPMS Patients Continue to have Relapses



Source: MEDACorp Survey, "Trends in Multiple Sclerosis (MS) Treatment, May 2013

Our Bias Is for a Negative ASCEND Trial Outcome But Risk/Reward Actually Appears Positive: Based on multiple MEDACorp KOL interviews, our bias is for a negative ASCEND trial result in 2015 when data are reported. KOLs describe the main risk of this trial being enrollment criteria specifying that patients must have SPMS disease for a lengthy minimum period of time. The prevailing hypothesis is that patients with this longevity of disease may be "untreatable" similar to multiple failures experienced in the Alzheimer's Disease (AD) setting in later stage patients. Given this diligence, we estimate only a 35% chance of success for the ASCEND Trial (65% chance of failure). The sensitivity analysis below is meant to illustrate the potential impact to future Tysabri revenues for a positive and negative ASCEND Trial outcome.

Tysabri WW Revenue (\$M) Sensitivity Analysis on Positive ASCEND Trial					
_	%Increase in Tysabri Use in SPMS Patients			ents	
Year	0%	50%	100%	150%	200%
2016	\$2,248	\$2,610	\$2,972	\$3,334	\$3,696
2017	\$2,355	\$2,734	\$3,113	\$3,493	\$3,872
2018	\$2,452	\$2,847	\$3,242	\$3,637	\$4,032
2019	\$2,532	\$2,940	\$3,347	\$3,755	\$4,163
2020	\$2,599	\$3,017	\$3,436	\$3,855	\$4,273
Total 2016 to 2020 Revenue Incremental Revenue	\$12,185	\$14,148 \$1,963	\$16,111 \$3,925	\$18,073 \$5,888	\$20,036 \$7,851

Note: Assumes Positive ASCEND Trial Results and 78% of Physicians Increase Tysabri Use in SPMS.

Tysabri WW Revenue (\$M) Sensitivity Analysis on Negative ASCEND Trial					
_	%Increase in Tysabri Use in SPMS Patients				
Year	0%	-50%	-100%	-150%	-200%
2016	\$2,248	\$2,095	\$1,941	\$1,788	\$1,635
2017	\$2,355	\$2,194	\$2,034	\$1,873	\$1,713
2018	\$2,452	\$2,285	\$2,118	\$1,951	\$1,784
2019	\$2,532	\$2,359	\$2,187	\$2,014	\$1,842
2020	\$2,599	\$2,422	\$2,245	\$2,068	\$1,890
Total 2016 to 2020 Revenue	\$12,185	\$11,355	\$10,525	\$9,694	\$8,864
Incremental Revenue		(\$830)	(\$1,661)	(\$2,491)	(\$3,321)

Note: Assumes Negative ASCEND Trial Results and 33% of Physicians Decrease Tysabri Use in SPMS.

Source: MEDACorp Survey and Leerink Swann Estimates.

RPC-1063 Clinical Trials

Phase I RPC-1	063 Thorough QT Study (TQTc) Trial:		
Purpose:	Further characterizing cardiac safety profile of RPC1063 through a Phase I TQT trial		
# Patients:	■ N=124		
	Randomized 1:1		
	Positive Control with Moxifloxacin 400mg		
Background:	Gilenya:		
	 TQTc Trial: At supra-therapeutic doses of 1.25 or 2.5mg (vs. recommended 0.5mg dose) Gilenya at steady- state when a negative effect on HR was still present, was reported to result in mild but statistically significant prolongation of QTc interval 		
	MS clinical database: Includes no clinically relevant prolongation of QT interval, but patients at risk for QT prolongation were NOT included in Gilenya MS studies		
	 RPC-1063 TQTc Trial: Also includes HR assessment as additional objective on potential for improved RPC1063 cardiac safety profile 		
	 DT: Utilizes same dose titration regimen for RPC-1063 as in Phase II and planned Phase III trials up to supra-therapeutic dose of 2mg in order to reach steady state dosing and obtain more clinical experience utilizing clinical dose titration regimen 		
	 Additional Monitoring: Incorporated additional detailed HR monitoring with continuous 24-hour ambulatory electrocardiogram (ECG) monitoring in-house on day before dosing, first day of dosing, and each day of dose escalation during dose titration regimen to assess changes in HR and risk of any cardiac adverse events (AEs) 		
Trial Arms:	Doses up 2mg (titrated from 0.25mg to 2mg over 14 days)		
Primary End	Assess whether exposure to therapeutic (1mg) or supratherapeutic (2mg) doses of RPC1063 in healthy		
Point:	male and female subjects increases the mean corrected QT interval (known as QTc interval) vs. placebo		

Source: Company Reports, Leerink Swann LLC estimates

Phase I RPC	C-1063 Healthy Volunteer Trial:
Purpose:	Determine RPC1063 safety/tolerability as oral single and multiple daily (QD) doses in healthy subjects and pharmacokinetic (PK), pharmacodynamic (PD) and effect of dose titration of RPC1063 on heart rate (HR) changes
# pts:	N=88 (68 on PRC-1063)
	Consisted of cohorts each with 8 subjects assigned to RPC1063 (N=6) or placebo (N=2):
	Single ascending dose (SAD) cohorts
	Multiple ascending dose cohorts (MAD) for 7 days (MAD-7 days)
	MAD for 28 days (MAD-28)
	Dose titration (DT) cohort
Design:	First-in-human, randomized, double-blind, placebo-controlled, single/multiple ascending dose study of RPC1063
Trial	Doses in ranged from 0.3mg → 3mg:
Arms:	SAD: RPC1063 given as single oral dose (0.3, 1, 2, or 3mg).
	MAD-7: RPC1063 given as oral daily doses (0.3, 1, 2mg) for 7 days
	MAD-28: RPC1063 given as oral daily doses (0.3, 1, or 1.5mg) for 28 days
	DT : RPC1063 given in dose titration regimen with doses increasing from 0.3mg → 2mg over 8 days
Primary	Safety/tolerability assessed by collection/analysis of:
End Point:	Adverse Events (AEs), vital signs, clinical laboratory, electrocardiogram, pulmonary function test
	(PFT), chest x-ray, ophthalmologic exam, and continuous 24 hour cardiac monitoring (telemetry and Holter)
	Pharmacodynamic (PD) evaluated by measuring:
	circulating lymphocytes + flow cytometric analysis on lymphocyte subsets
Sponsors:	RCPT
2	any Panarta Lagrink Swann LLC actimates

Phase I RPC-	1063 Healthy Volunteer Trial Data (ECTRIMS-2012):
General:	 RPC1063 was well tolerated; AEs generally similar in placebo and RPC-1063 treatment groups
	 Higher percentage of subjects treated RPC-1063 had at least 1 AE vs. placebo (76.5% vs. 62.5%)
Efficacy:	Pharmacodynamics (PD):
,	Median reduction in lymphocyte count after 28 days dosing:
	 34% at RPC1063 0.3mg
	■ 65% at RPC1063 1mg
	■ 68% at RPC1063 1.5mg
	 Recovery of lymphocyte counts to normal range occurred within 48-72 hours for all subjects in 1 +
	1.5mg MAD-28 dose cohorts
Safety:	 All AEs mild or moderate with no severe AEs observed + no dose limiting toxicities (DLT) reported
	Most common AEs with RPC-1063: Headache (9), somnolence (6), nausea (6), dizziness (5), fatigue (4),
	abdominal pain (3), pruritus (3).
	 Most common treatment-emergent AEs (TEAEs) occurring in >1 subject grouped by system organ
	class:
	Adverse Events of Special Interest:
	Cardiac:
	■ 1 MAD-28 subject on 1.5mg: had 2nd-degree Mobitz type-1 atrioventricular block on Day 1 of dosing →
	discontinued; event resolved without medical intervention.
	 In 3mg SAD group: 2 subjects experienced 2 second sinus pause during periods of bradycardia, and 1
	subject experienced intermittent bradycardia. These AEs identified on telemetry, were asymptomatic and
	resolved without medical intervention.
	Pulmonary:
	2 subjects in 2mg MAD-7 had mild asymptomatic PFT abnormalities → resolved without medical intervention
	Target Effects of Special Interest:
	• HTN: No clinically significant changes in BP observed. Dose related change in systolic BP observed on Day 1
	which coincided temporally with lowest HR
	Liver: No drug-related effects observed on liver function tests (ALT, AST and bilirubin).
	Ophthalmology: No apparent drug related effects observed on ophthalmologic exams
	Lymphocytes: Robust, dose-dependent reduction of circulating lymphocytes observed: Streets on Libert Bate (LB):
	Effects on Heart Rate (HR):
	Transient, dose-related decrease in HR observed Createst reduction in LIP absorbed during 1st day of RPC 1063 design a generally assured within 1st 6hrs.
	 Greatest reduction in HR observed during 1st day of RPC-1063 dosing + generally occurred within 1st 6hrs Mean placebo adjusted change from baseline in 1st 6hrs following RPC-1063 dosing:
	■ -7.1 (BPM) at RPC-1063 0.3mg
	9.1 (BPM) at RPC-1063 0.3mg
	11.5 (BPM) RPC-1063 2mg
	23 (BPM) at RPC-1063 3mg
	 RPC-1063 treatment effect on HR attenuated over 1st week of dosing, with smaller effects observed starting
	on Day 2 and HR returning towards pre-dose values by Day 7, demonstrating possible tachyphylaxis.
	Lowest mean hourly HR seen during 1st day of dosing
	Effects of Dose Titration:
	Hypothesis: Gradual titration of RPC-1063 dose over several days may mitigate vs. larger reductions in HR
	 Smaller decreases in HR observed when RPC-1063 dose titrated to 1mg (Day 6) and 2mg (Day 8) vs. HR
	decreases observed on Day-1 of 1mg and 2mg fixed dose groups
	 Lymphocyte counts decreased to same extent with dose titration (>65% decrease vs. baseline) vs. fixed
	dosing
Conclusion:	 RPC-1063 generally well tolerated with dose-dependent effects observed on number of clinical parameters.
	 Use of dose titration regimen further attenuated HR effects observed on 1st dose of RPC1063
	Robust pharmacodynamic (PD) effect of peripheral lymphocyte count reduction observed at all doses.
	Lymphocytes rapidly recover to normal range upon dosing cessation.
	■ Based on favorable safety and PD profile → 0.5mg and 1mg will be carried forward in MS program

RADIANCE	Phase II Portion of RPC-1063 Relapsing Multiple Sclerosis (RMS) Trial (RPC01-201):	
Purpose:	Determine whether RPC-1063 is effective in treatment of relapsing multiple sclerosis (RMS)	
# pts:	N=210	
Design:	Interventional randomized, safety/efficacy, parallel assignment, double blind (subject, caregiver, investigator,	
	outcomes assessor) treatment study	
Trial	Arm A: 0.5mg RPC-1063 oral daily (QD) for 24 weeks	
Arms:	Arm B: 1mg RPC-1063 oral daily (QD) for 24 weeks	
	Arm C: Placebo oral for 24 weeks	
Primary	Total number (#) of new GdE lesions, assessed on brain MRIs [Week 12 → 24]	
End Point:		
Status:	Ongoing (expect to complete enrollment in 2H13)	
Start:	October 2012 (FPI 10.22.12)	
Interim:	Yes and this will trigger beginning enrollment for 1st Phase III trial	
End:	mid-14	
Sponsors:	Receptos	
Clin.Trials.	NCT01628393	
Gov.ID	RPC01-201	

Source: Company Reports, Leerink Swann LLC estimates

RADIANCE ²	Ist Phase III Pivotal RPC-1063 Relapsing Multiple Sclerosis (RMS) Trial (RPC01-201):
Purpose:	Determine whether RPC-1063 is superior to Avonex in relapsing multiple sclerosis (RMS).
# pts:	N>900 (>1,100 in Phase II + Phase III)
Design:	Interventional randomized, safety/efficacy, parallel assignment, double blind (subject, caregiver, investigator, outcomes assessor) treatment study
SPA:	Yes
Trial	Arm A: 0.5mg RPC-1063 oral daily (QD) for 2+ years
Arms:	Arm B: 1mg RPC-1063 oral daily (QD) for 2+ years
	Arm C: Avonex 30µg intramuscular (IM) weekly for 2+ years
Primary	Assess if RPC-1063 is superior to Avonex in reducing Annual Rate of Relapse (ARR) over 2 years
End Point:	
Start:	4Q13/1Q14
End:	2017
Sponsors:	RCPT
Clin.Trials.	NCT01628393
Gov.ID	RPC01-201



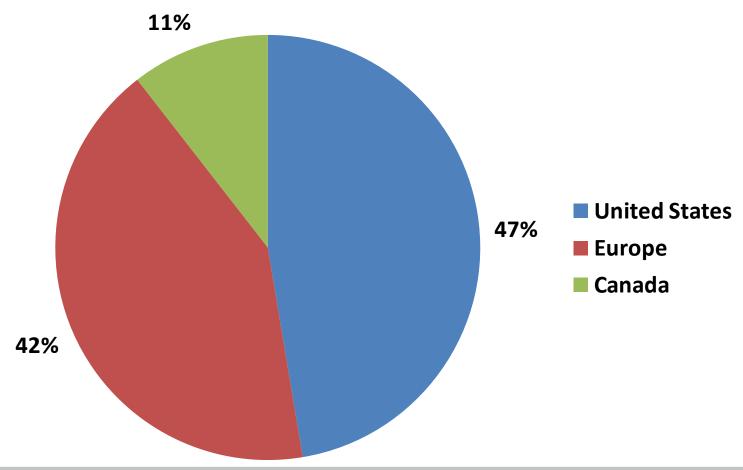
The Healthcare Investment Bank

MEDACorp Survey: Trends in Multiple Sclerosis (MS) Treatment

95 Specialists Responded; 45 in the U.S.



Physical Location

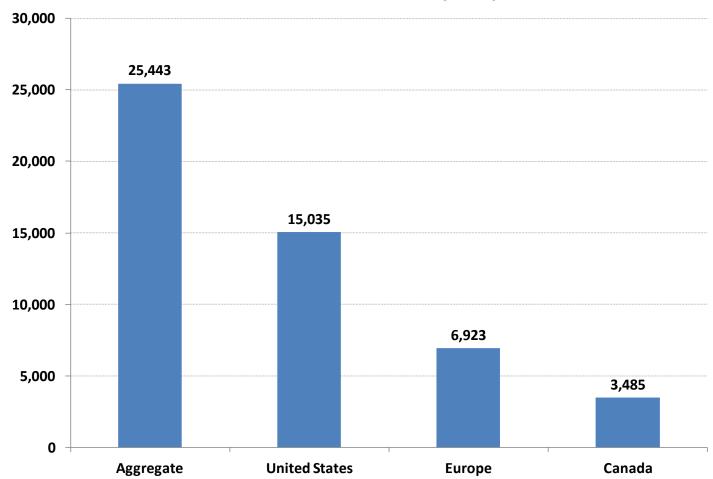


Source: MEDACorp Survey, "Trends in Multiple Sclerosis (MS) Treatment," May 2013

MEDACorp Survey Captured 25,443 MS Patients



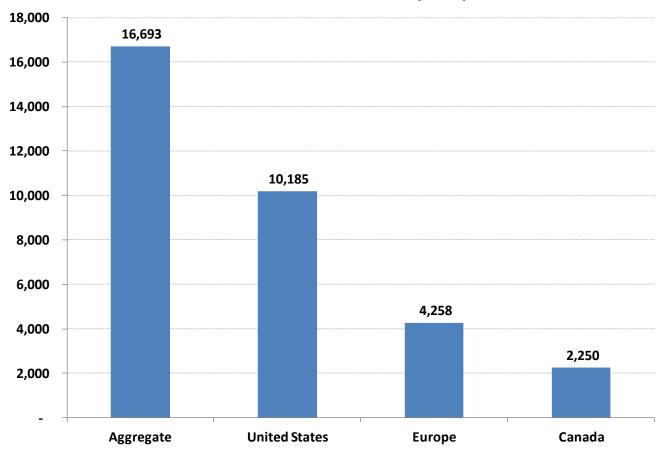
Total # of MS Patients Treated by Respondents



The Survey Captured 16,693 RMS Patients



Total # of RMS Patients Treated by Respondents

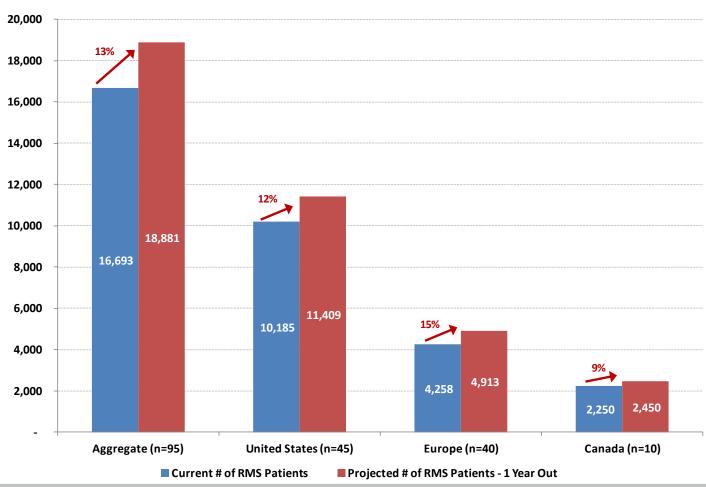


RMS: Relapse Remitting form of Multiple Sclerosis Source: MEDACorp Survey, "Trends in Multiple Sclerosis (MS) Treatment," May 2013

Double-Digit Growth of RMS Patient Pool Likely Overestimates Actual Market Growth



Estimated Annual Growth Rate for RMS Patients

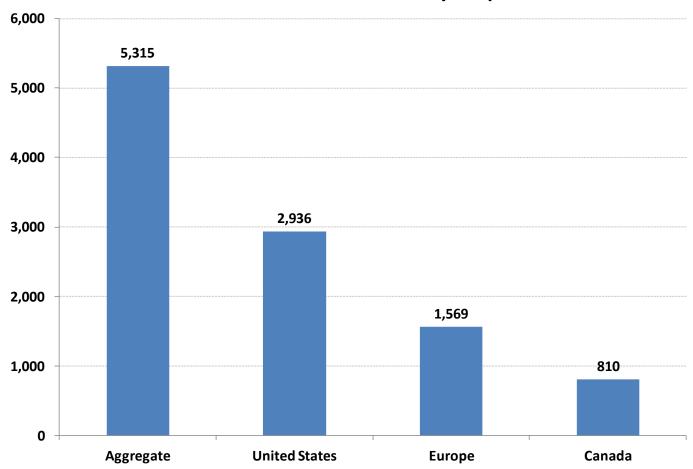


37

The Survey Captured 5,315 SPMS Patients



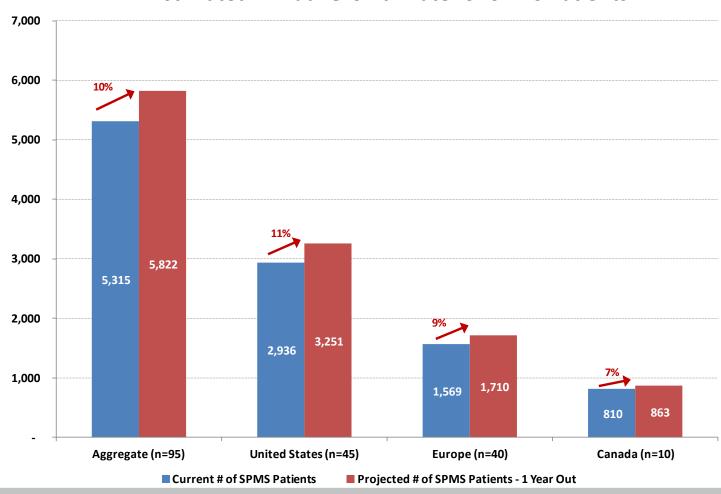
Total # of SPMS Patients Treated by Respondents



Estimated Pace of Growth of SPMS Patient Pool Strong but Likely Overestimated



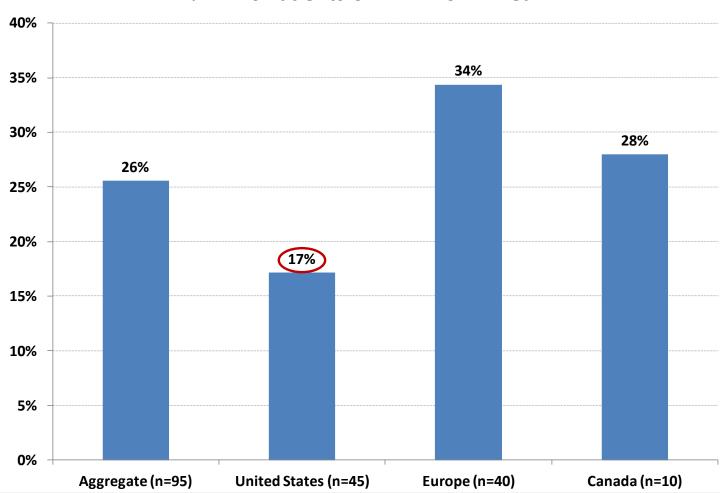
Estimated Annual Growth Rate for SPMS Patients



U.S. Patients on a DMD for < 1 Year May Reflect High Penetration & Stable Disease

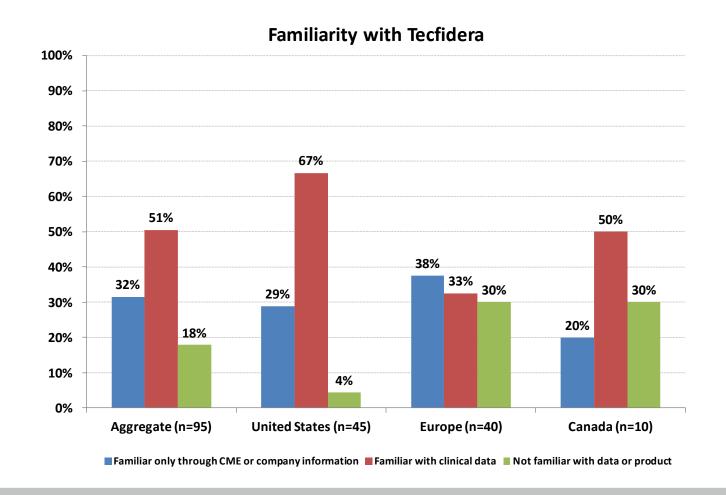


% RMS Patients on DMD for <1 Year



No Surprise, Highest Awareness of BIIB's (OP) Tecfidera is in U.S. (Approved March 2013)

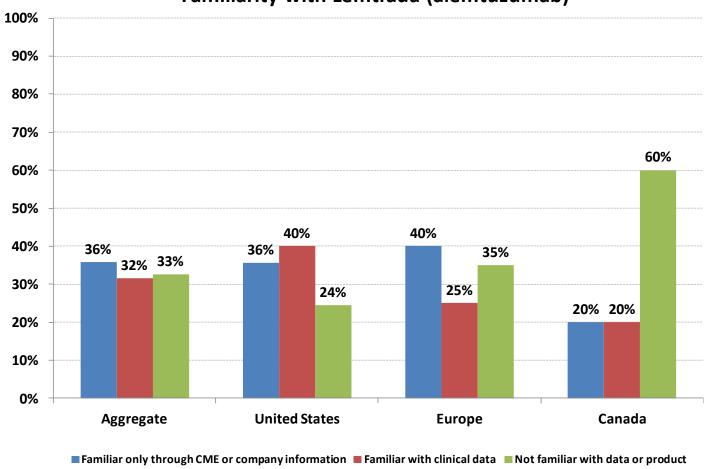




Relatively Balanced Global Awareness of SNY's (OP) Lemtrada...

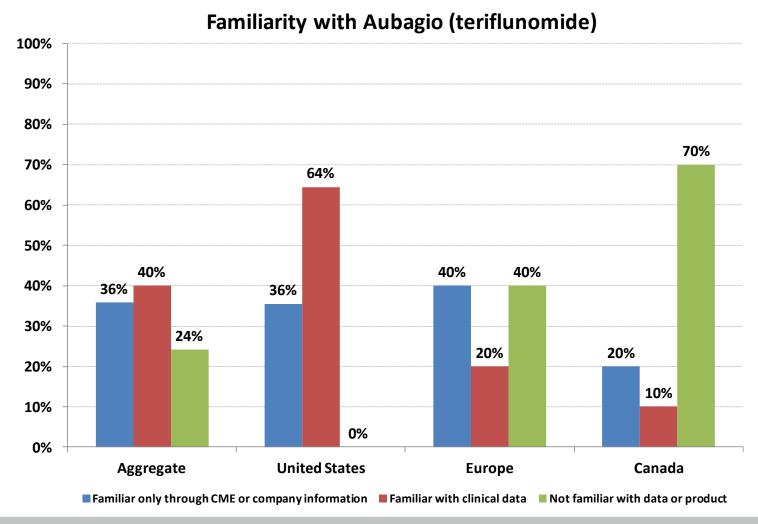


Familiarity with Lemtrada (alemtuzumab)



Most U.S. Physicians are Familiar with SNY's Aubagio

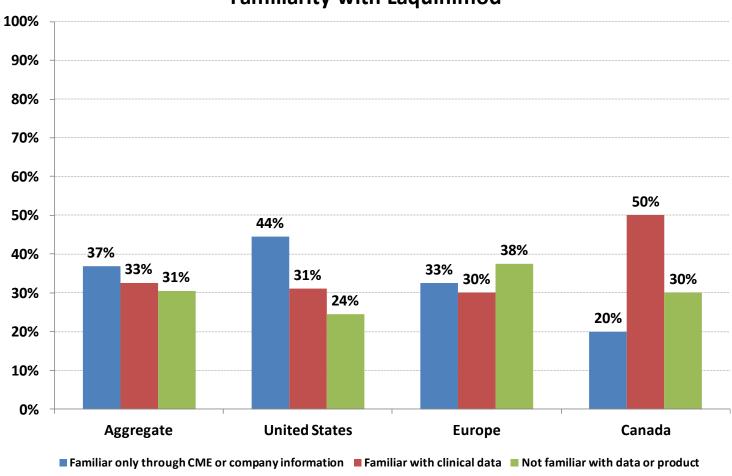




Relatively Balanced Global Awareness of TEVA's (MP) Laquinimod...

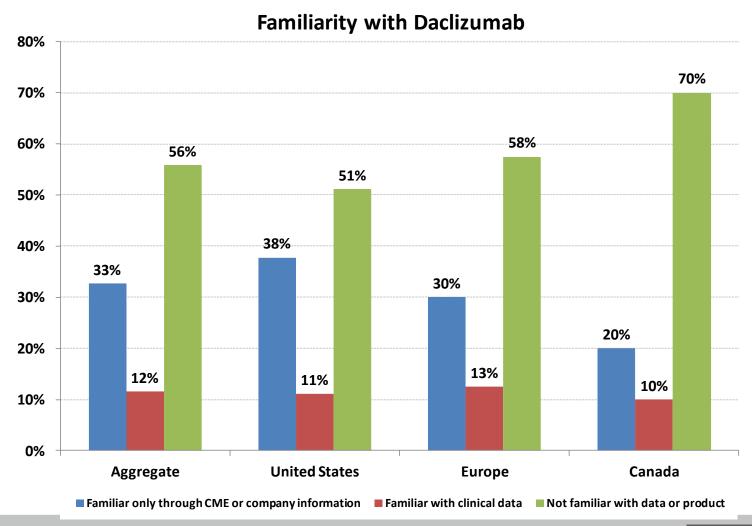


Familiarity with Laquinimod



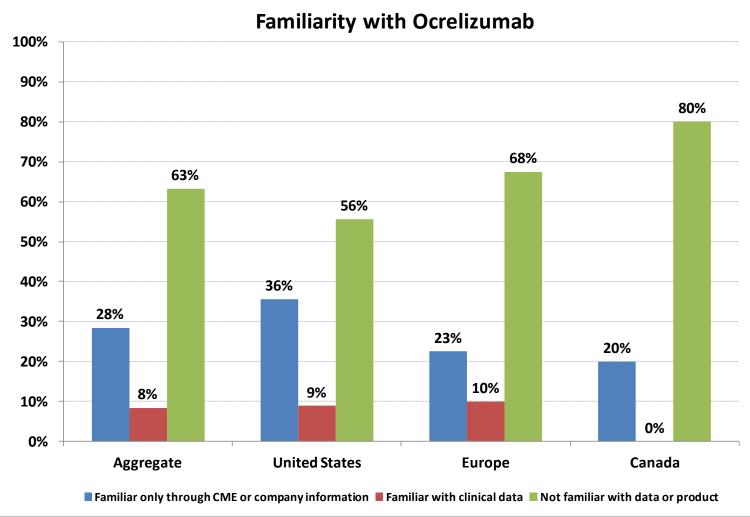
Unsurprisingly Low Global Awareness of BIIB (OP) & ABBVs' Daclizumab Given Companies Have Not Yet Been Highlighting It





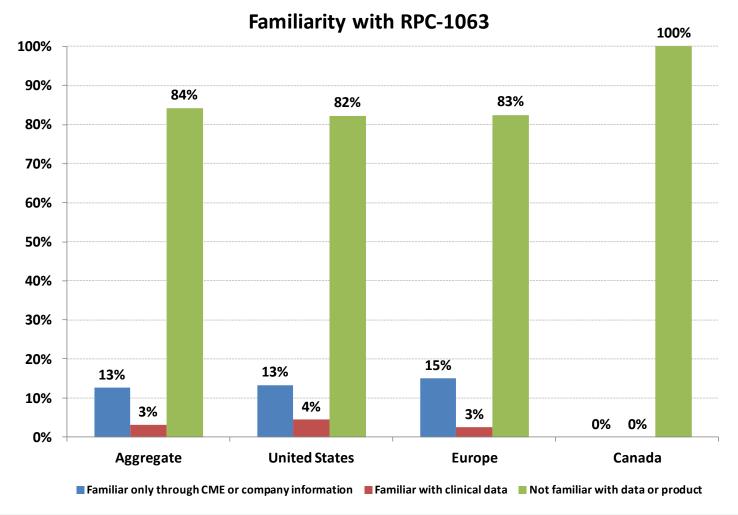
Unsurprising Low Global Awareness of BIIB (OP) & Roche's Ocrelizumab Given Companies Have Not Yet Been Highlighting It





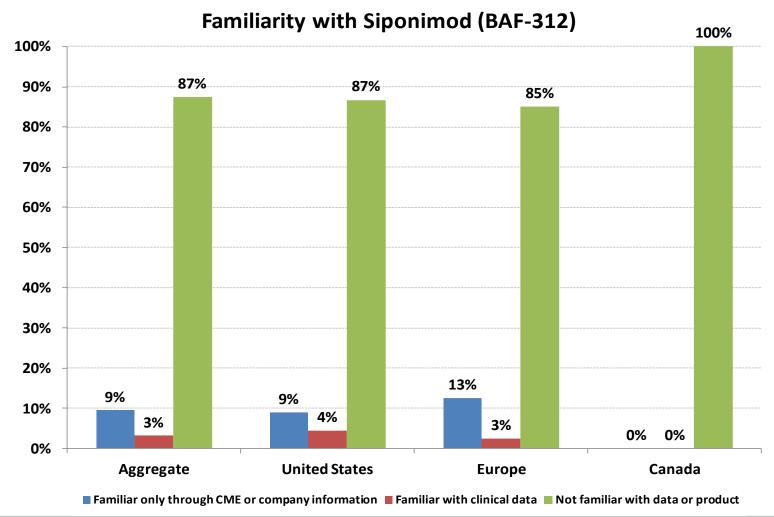
Low Global Awareness of S1P Receptor Modulators such as RCPT's RPC-1063





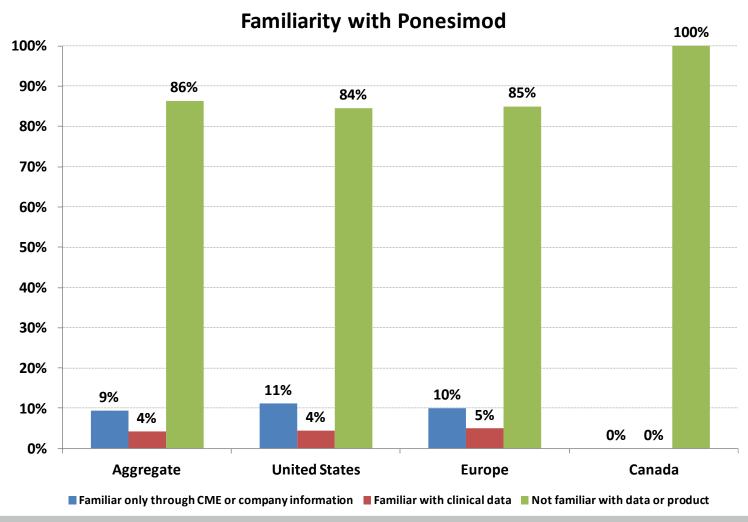
Low Global Awareness of S1P Receptor Modulators such as NVS's (OP) Siponimod





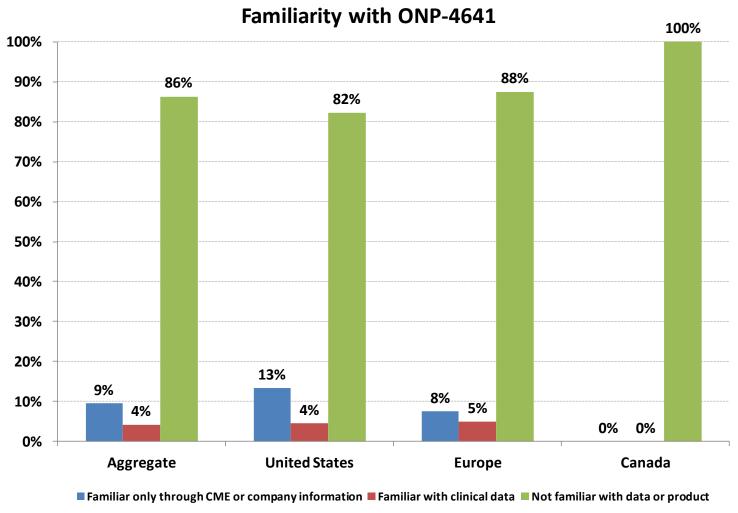
Low Global Awareness of S1P Receptor Modulators such as ATLN's Ponesimod





Low Global Awareness of S1P Receptor Modulators such as SEO's ONO-4641

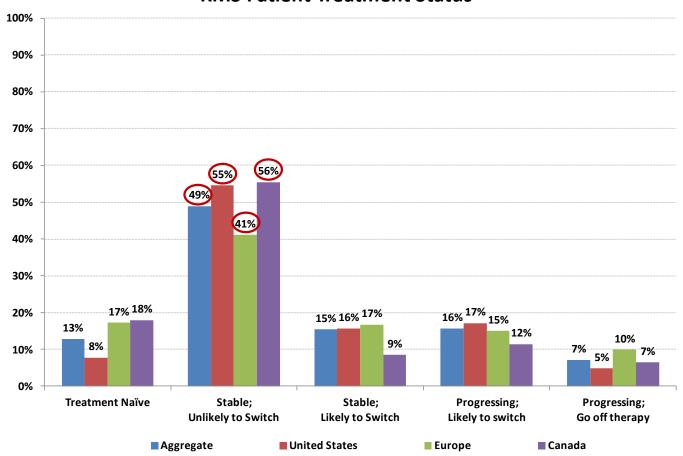




Globally, Physicians Currently Estimate ~50% of Their Patients Are Stable & Won't Switch



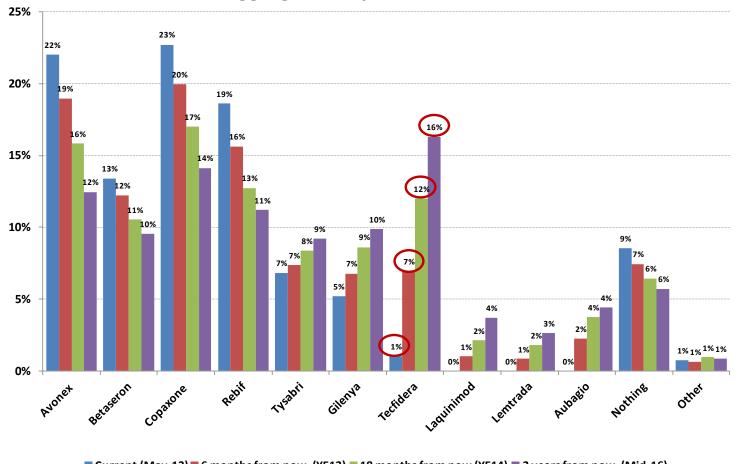
RMS Patient Treatment Status



Physicians Are Bullish on Tecfidera at the Expense of ABCRs Among All Patients



Time Series: Aggregate Respondents & RMS Treatments

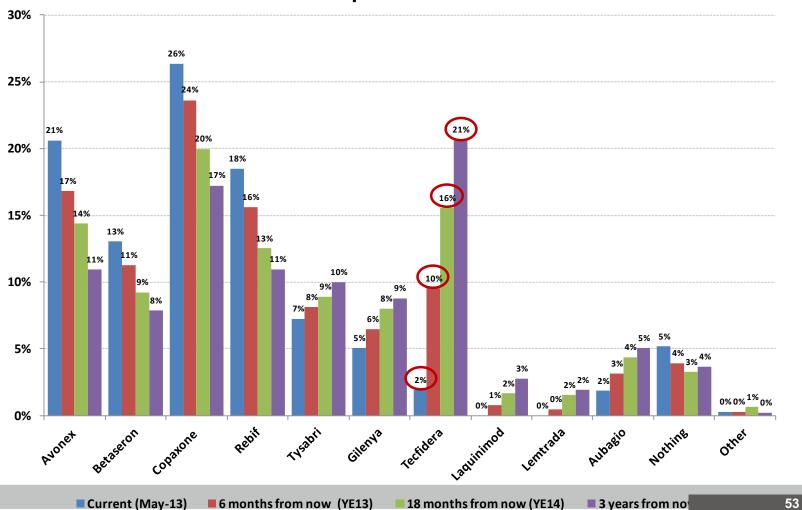


■ Current (May-13) ■ 6 months from now (YE13) ■ 18 months from now (YE14) ■ 3 years from now (Mid-16)

U.S. Physicians Predict Rapid RMS Market Share Gains for Tecfidera



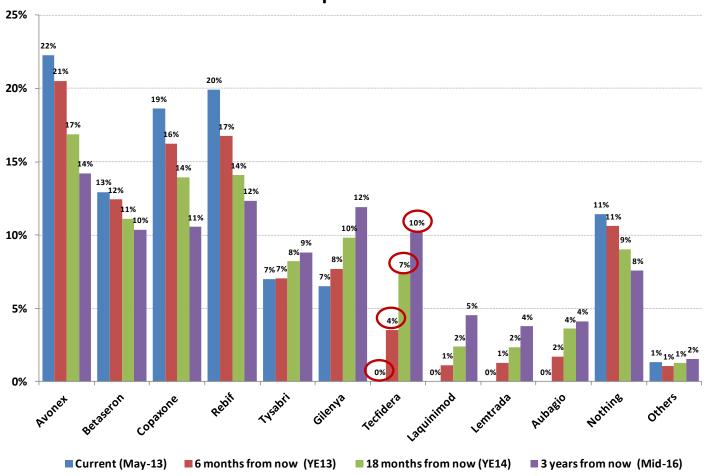
Time Series: U.S. Respondents & RMS Treatments



EU Physicians' Predict Rapid Tecfidera Growth & Continued Gains for Gilenya



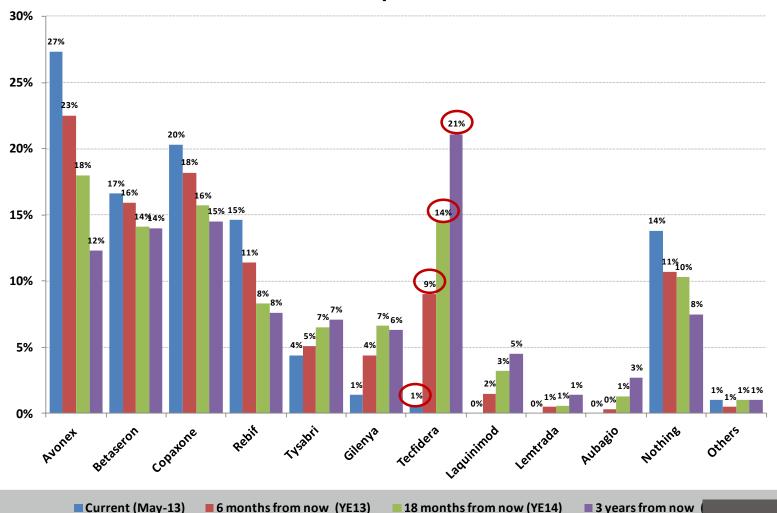
Time Series: E.U. Respondents & RMS Treatments



Canadian Physicians Similarly Predict Rapid RMS Market Share Expansion for Tecfidera



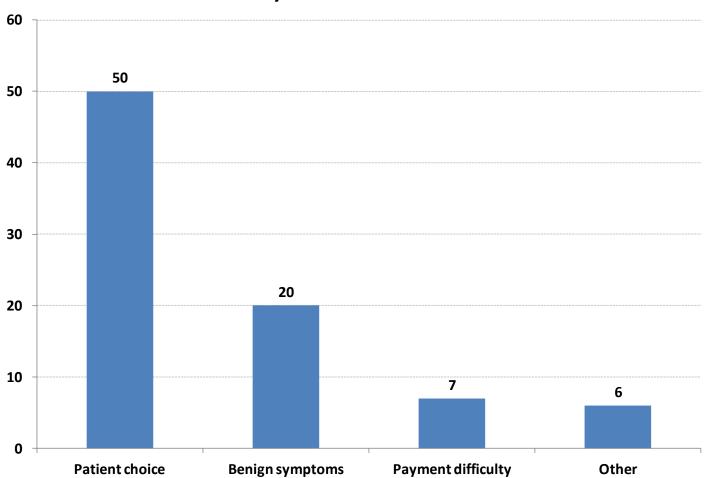
Time Series: Canadian Respondents & RMS Treatments



Patients Are the Primary Reason Physicians Elect "No Treatment"

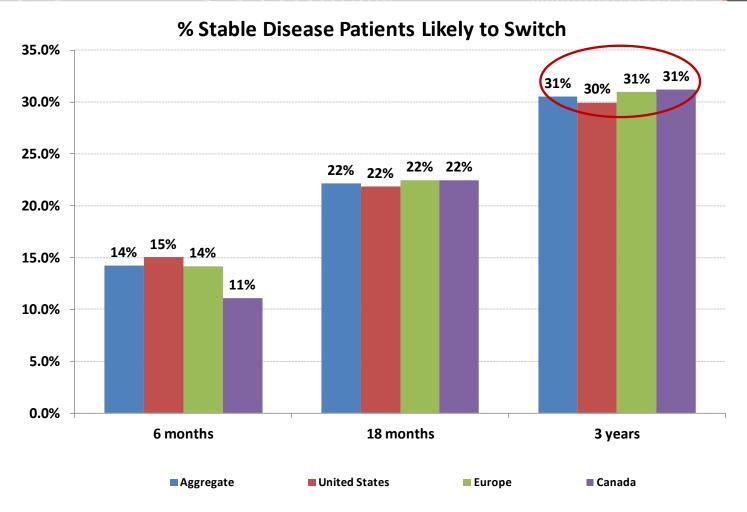


Reasons Why Patients Elect No Treatment



Globally, ~1/3 of Stable MS Patients Are Expected to Switch to New Therapy in 3 Years

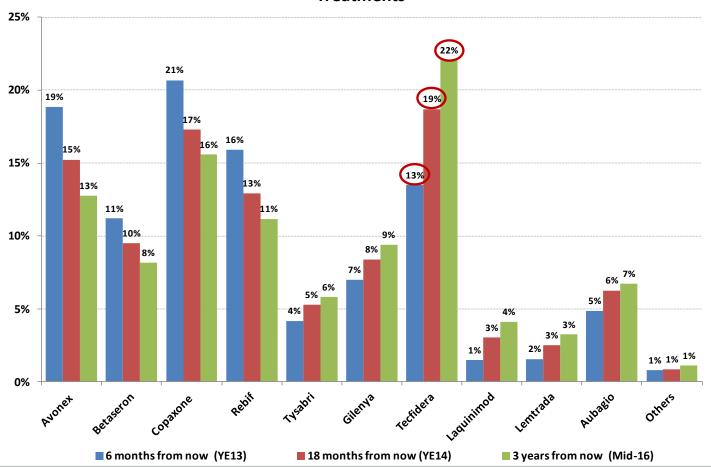




Globally, Physicians Predict Tecfidera Will Lead Among New Patients in 18 Months



Time Series: Aggregate Treatment Naïve Patients & Potential Treatments

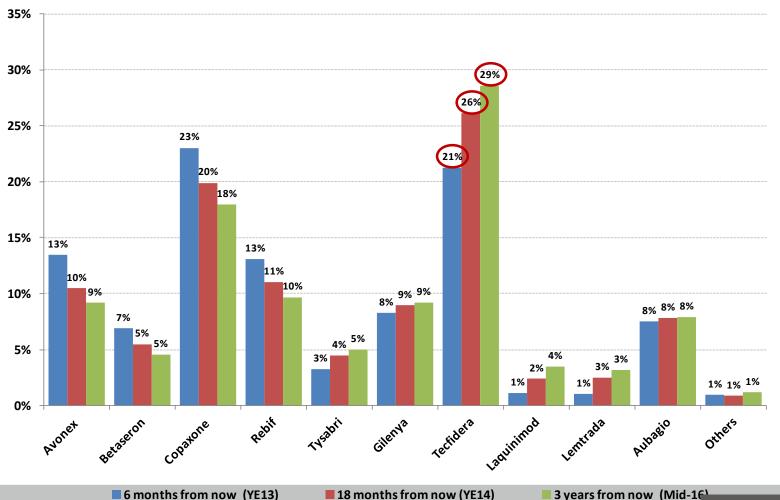


371

U.S. Physicians Predict Tecfidera Will Lead Among New Patients within 18 Months



Time Series: U.S. Treatment Naïve Patients & Potential Treatments



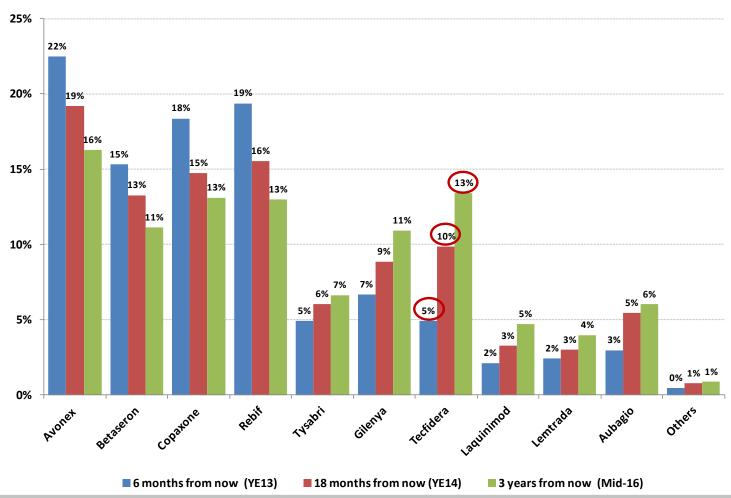
3 years from now

59

EU Physicians Expect Tecfidera to Be the Leading Oral MS Agent Among New Patients



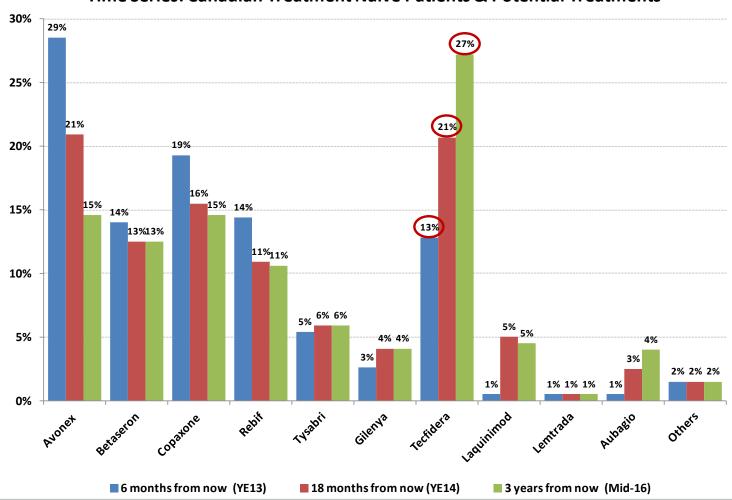
Time Series: E.U. Treatment Naïve Patients & Potential Treatments



Canadian Physicians Expect Tecfidera to Dominate Other Oral Drugs & Lead Overall



Time Series: Canadian Treatment Naïve Patients & Potential Treatments

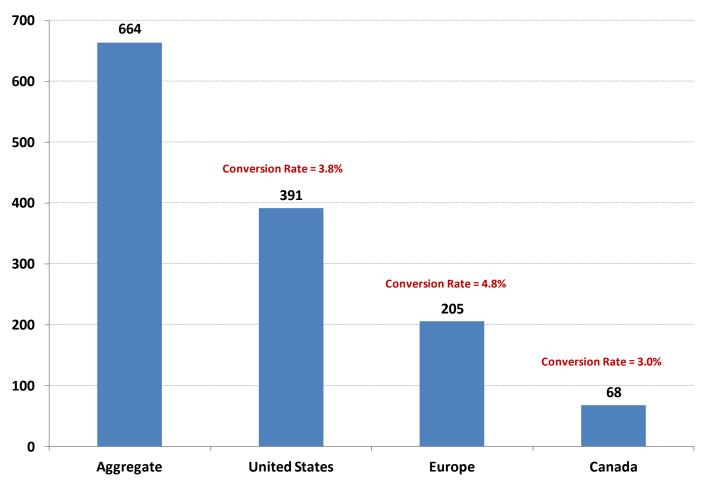


RECEPTOS, INC.

3-5% of RMS Patients Were "Warehoused" In Anticipation of Tecfidera



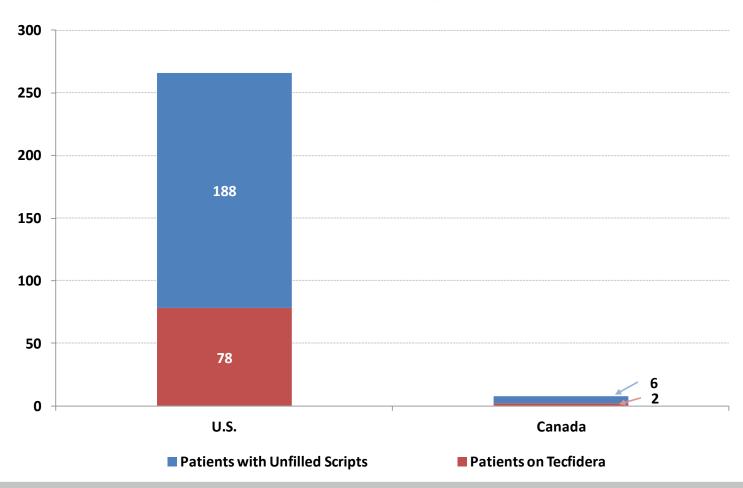
Total # of RMS Patients "Warehoused" for Tecfidera Prior to Launch



U.S. Physicians Estimate that ~30% of Written Tecfidera Scripts Have Been Filled



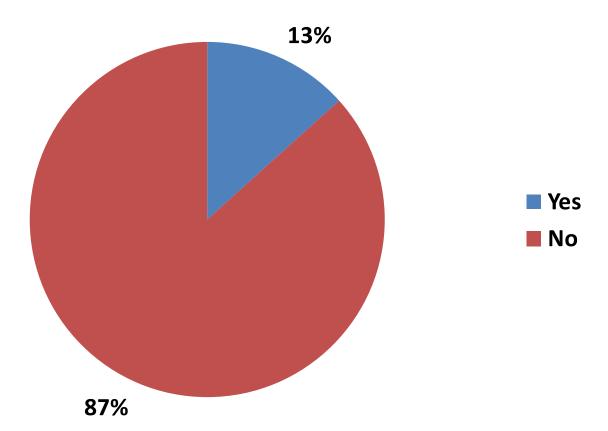
Total # of Patients Received Tecfidera Scripts vs. Those On Tecfidera



For 87% of U.S. Physicians, Reimbursement Has Not Limited Tecfidera Prescribing



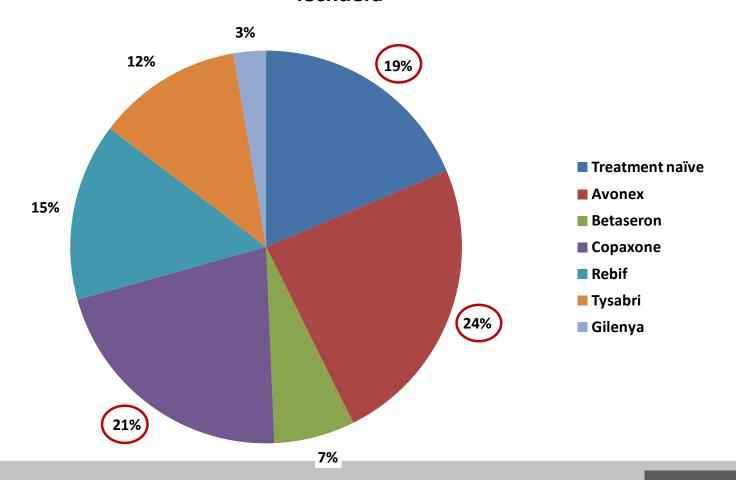
U.S. (n=45): Has reimbursement for Tecfidera limited your ability to prescribe the drug?



Most Early U.S. Tecfidera Patients Are From Avonex, Copaxone or Treatment Naive



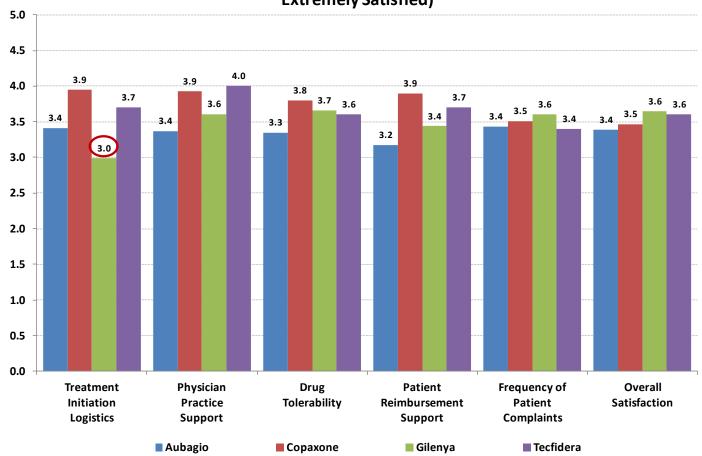
U.S. (n=21): Previous Status of Current RMS Patients on Tecfidera



Overall: Treatment Initiation Logistics Remain Gilenya's Biggest Headwind...



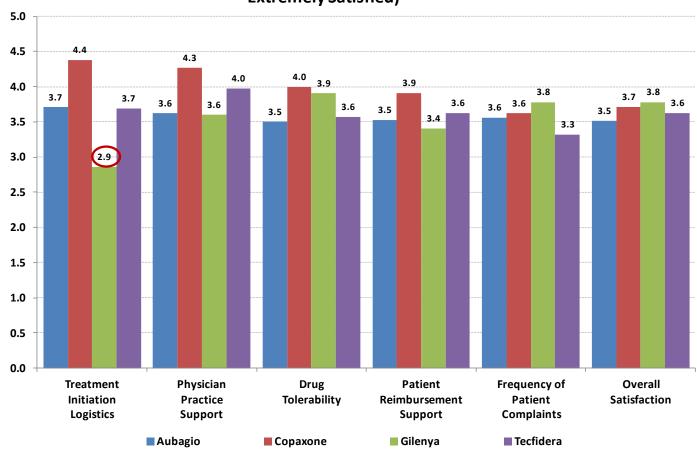
Aggregate: Average Ratings per Therapy (1=Extremely Dissatisfied, 5= Extremely Satisfied)



...Particularly Among U.S. Physicians Despite Good on Treatment Experience



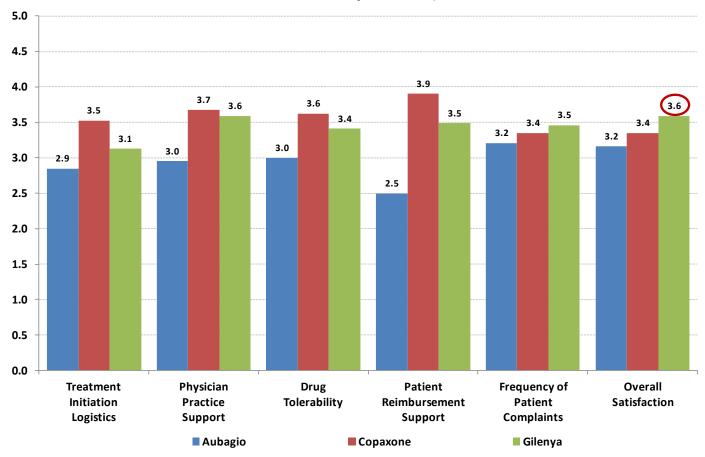
U.S.: Average Ratings per Therapy (1=Extremely Dissatisfied, 5= Extremely Satisfied)



Gilenya Receives High Marks among EU Physicians...



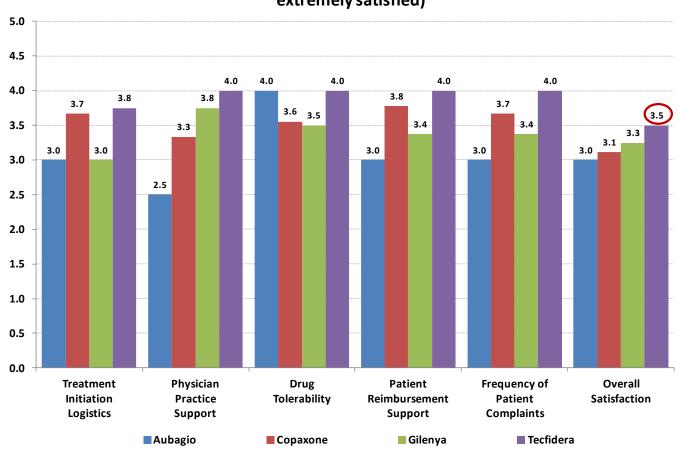
E.U.: Average Ratings per Therapy (1=extremely dissatisfied, 5= extremely satisfied)



...While Canadian Physicians See Tecfidera as a Leader on Key Characteristics



Canada: average ratings per therapy (1=extremely dissatisfied, 5= extremely satisfied)

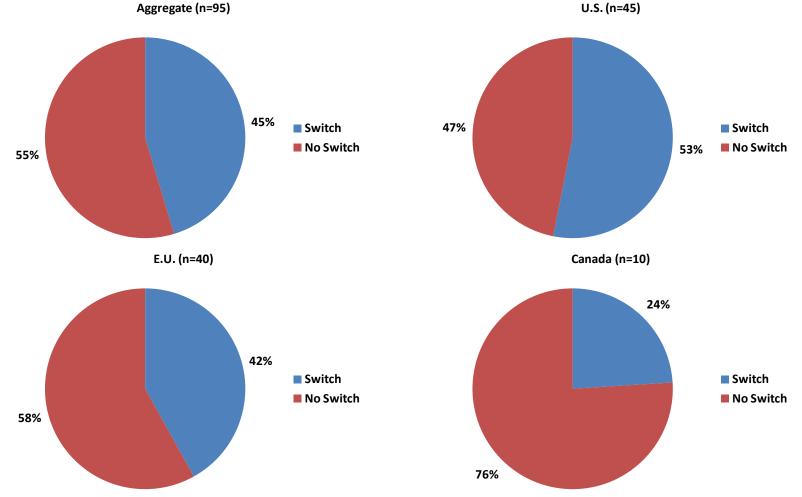


382

U.S. Physicians Predict >50% of Current Copaxone Patients Will Switch to Copaxone 3x/week if Approved; Less in other Countries



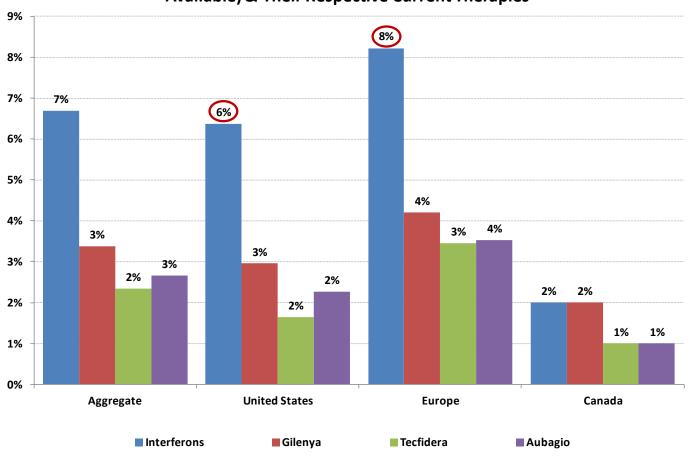
% Patient Switch from Existing Copaxone to 3x/Week Copaxone If Approved



Generic Copaxone could take 6-8% of Patients from Interferons in U.S. & E.U.



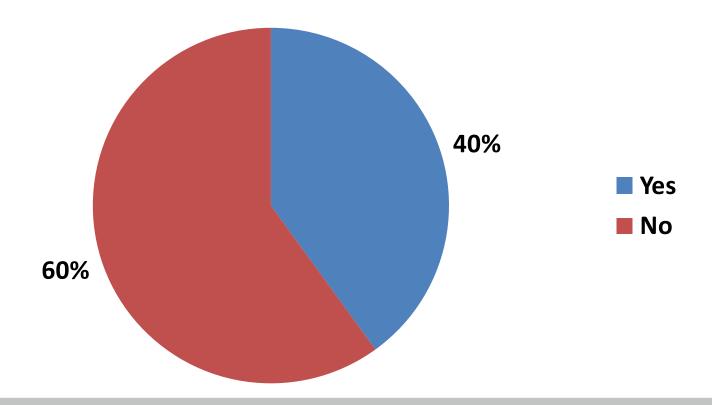
% Patients Potentially Switching to Generic Copaxone (If Becomes Available) & Their Respective Current Therapies



~60% of Physicians Believe It Would be Unethical to Require 1st-line Generic Copaxone in RMS Patients



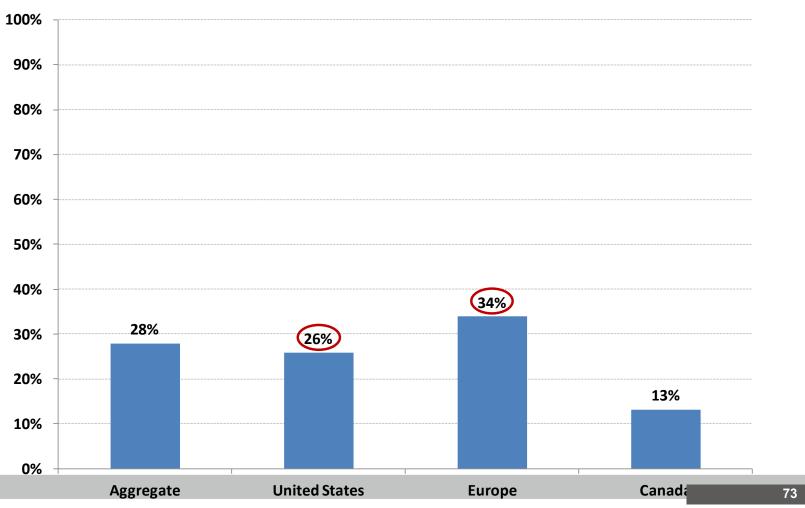
Aggregate (n=95): If generic Copaxone becomes available, would it be ethical to require its use as a recommended 1st line therapy for RMS?



In U.S. & EU ~1/4 to 1/3 of SPMS Patients Continue to Have Relapsing Disease



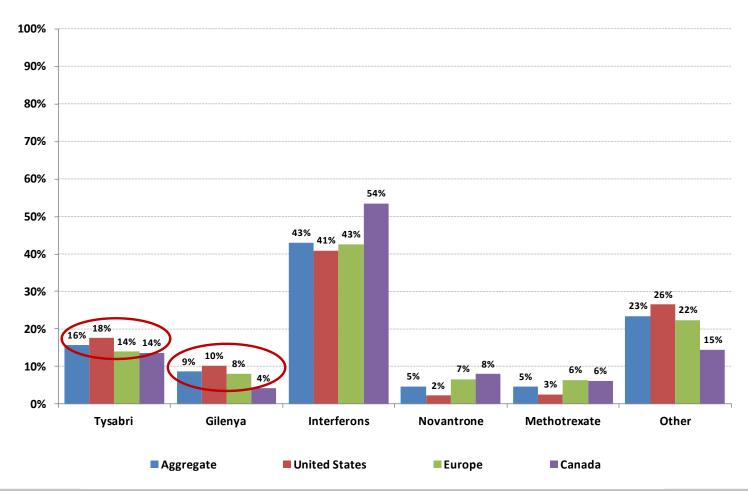
% SPMS Patients Continue to have Relapses



Tysabri & Gilenya Experience Substantial Use in Treatment of SPMS Patients



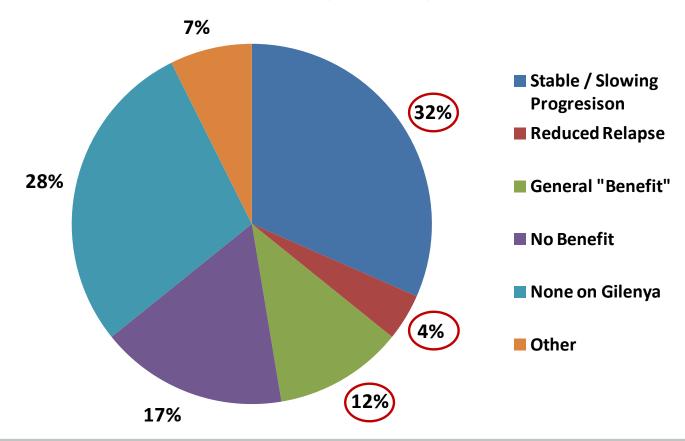
% SPMS Patients & Their Respective Therapies



~Half of Physicians Believe Gilenya Provides Some Benefit to SPMS Patients



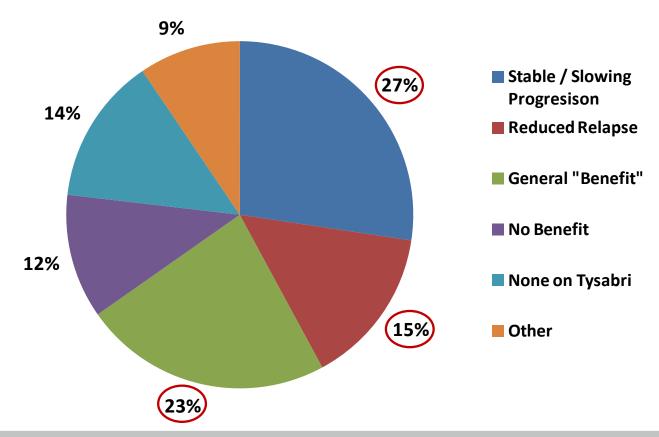
Aggregate (n=95): Benefit of Gilenya in SPMS Patients per Respondents



~65% Physicians Believe Tysabri Provides Some Benefit to SPMS Patients



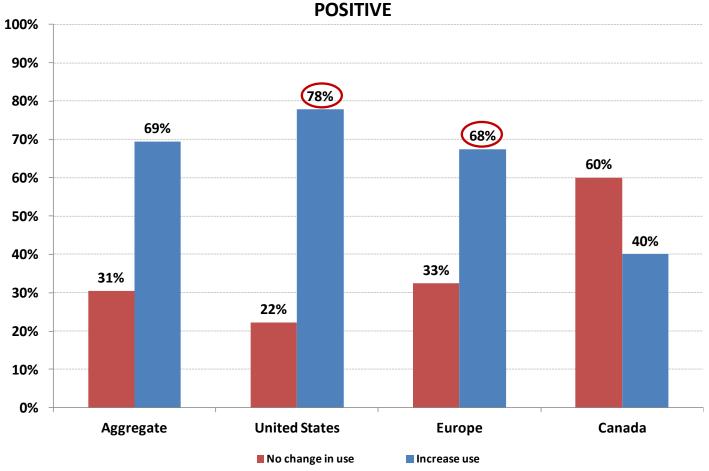
Aggregate (n=95): Benefit of Tysabri in SPMS Patients per Respondents



Positive Phase III ASCEND Results Would Lead 70-80% of Physicians to Increase Use of Tysabri in SPMS Patients



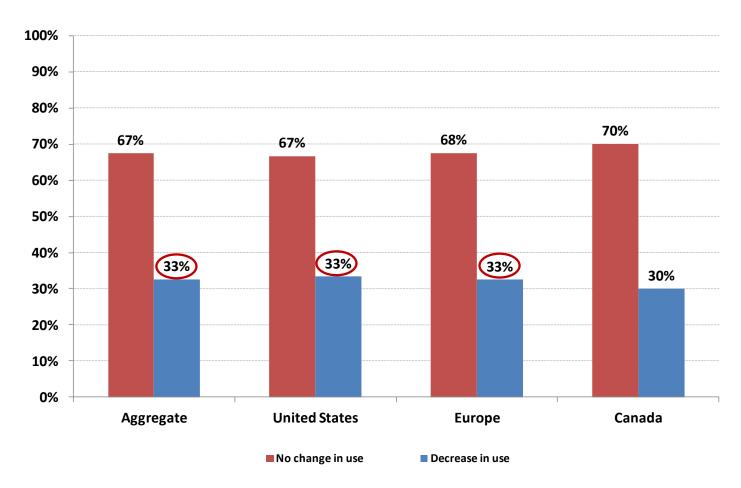
Change in Tysabri Use in SPMS Patients if ASCEND Trial is POSITIVE



Negative Phase III ASCEND Results Would Only Lead ~33% of Physicians to Decrease Use of Tysabri in SPMS Patients



Change in Tysabri use in SPMS Patients if ASCEND Trial is NEGATIVE



"Best In Class" S1P Receptor Modulator vs. Gilenya



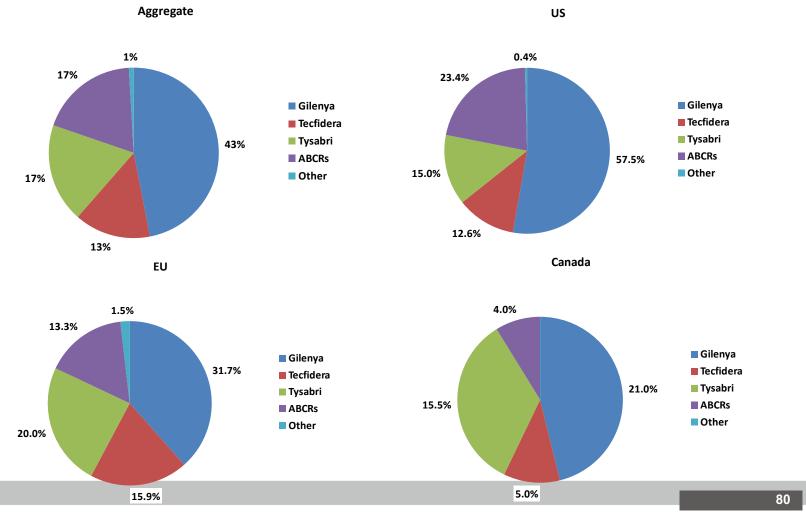
Drug	Novel S1P1 inhibitor	Gilenya (fingolimod)
Description	Novel S1P1 inhibitor is a selective S1P1 receptor modulator currently in clinical development for the treatment of relapsing multiple sclerosis.	Fingolimod (Gilenya) is metabolized by sphingosine kinase to the active metabolite, fingolimod-phosphate. Fingolimod-phosphate is a sphingosine 1-phosphate receptor modulator, and binds with high affinity to sphingosine 1-phosphate receptors 1, 3, 4, and 5. Fingolimod-phosphate blocks the capacity of lymphocytes to egress from lymph nodes, reducing the number of lymphocytes in peripheral blood. The mechanism by which fingolimod exerts therapeutic effects in multiple sclerosis is unknown, but may involve reduction of lymphocyte migration into the central nervous system.
Lymphocyte Reductions during treatment:	Efficacy comparable to fingolimod (Gilenya)	Between 70% (Study 1) and 83% (Study 2) of patients were relapse- free at 2 years. Risk of disability progression as measured by EDSS was reduced by 30% (Study 1) and 29% (Study 2)
Lymphocyte Reductions during treatment:	Lymphocytes counts decrease to 30- 50% of baseline (e.g. decrease 50- 70%)	In a study in which 12 subjects received GILENYA 0.5 mg daily, the lymphocyte count decreased to approximately 60% of baseline within 4-6 hours after the first dose. With continued daily dosing, the lymphocyte count continued to decrease over a 2-week period, reaching a nadir count of approximately 500 cells/µL or approximately 30% of baseline.
Lymphocyte Recovery:	T1/2=19hrs; 3 day recovery	Peripheral lymphocyte count increases are evident within days of stopping fingolimod treatment and typically normal counts are reached within 1 to 2 months.
Cardiovascular (CV) profile:	No impact on heart-rate or atrioventricular conduction and no requirement for first-dose monitoring Thorough QT Study (TQTc) Trial Result: No evidence of QTc prolongation	Decrease in heart rate and/or atrioventricular conduction after first dose of GILENYA: Monitor patients In a thorough QT interval study of doses of 1.25 or 2.5 mg fingolimod at steady-state, when a negative chronotropic effect of fingolimod was still present, fingolimod treatment resulted in a prolongation of QTc, with the upper bound of the 90% confidence interval (CI) of 14.0 ms. There is no consistent signal of increased incidence of QTc outliers, either absolute or change from baseline, associated with fingolimod treatment. In MS studies, there was no clinically relevant prolongation of QT interval, but patients at risk for QT prolongation were not included in clinical studies.
Hepatotoxicity: Liver Function Test (LFTs) >3x Upper Limit Normal (ULN)	No increases over 28 day dosing trial	ALT/AST increased in 14% of patients versus 5% of patients treated with placebo

392

A "Best In Class" S1P Receptor Modulator Would Take Most Share from Gilenya, Tysabri & ABCRs



Potential Switchers to the "Best In Class" S1P Receptor Modulator if Approved



TRENDS IN MULTIPLE SCLEROSIS TREATMENT

Respondent Distribution				
Specialty	Neurology			
Trends	Multiple Sclerosis			
Number of Respondents	95 Neurologists			
Respondent Distribution	United States, Europe, and Canada			
Survey Date	May 2013			



Geographic DistributionSource: Google Maps

Responses represent an average of U.S. neurologists (n=45), European neurologists (n=40), Canadian neurologists (n=10), and aggregate responses (n=95), unless otherwise noted.

Inclusion Criteria

S1. How many total MS patients do you currently follow?

	Aggregate	U.S.	EU	Canada
Current number of patients with MS - MEAN	267.8	334.1	173.1	348.5
Current number of patients with MS - MEDIAN	165	200	120	200
Current number of patients with MS - SUM	25,443	15,035	6,923	3,485

S2. Where are you currently located?

47.4%	United States
10.5%	Canada
42.1%	Europe: England (2x); UK (7x); France (8x); Germany (10x); Italy (8x); Spain (5x)

S3. Are you currently participating in any ongoing clinical trials where the data has not yet been presented regarding the following products?

Yes	No	
0.0%	100.0%	Tecfidera/BG12 (dimethyl fumarate)
0.0%	100.0%	Lemtrada (alemtuzumab)
0.0%	100.0%	Aubagio (teriflunomide)
0.0%	100.0%	Laquinimod
0.0%	100.0%	Daclizumab
0.0%	100.0%	Ocrelizumab
0.0%	100.0%	RPC-1063 (S1P receptor modulator)
0.0%	100.0%	Siponimod aka BAF-312 (S1P receptor modulator)
0.0%	100.0%	Ponesimod (S1P receptor modulator)
0.0%	100.0%	ONP-4641 (S1P receptor modulator)

Purpose of Survey

This survey asks neurologists about their current and projected treatment of multiple sclerosis (MS) patients.

Patient Population Background

1. Which best describes the setting in which you see the majority of your patients with MS?

	Aggregate	U.S.	EU	Canada
Academic clinic or facility	33.7%	20.0%	47.5%	40.0%
Community clinic or facility	20.0%	2.2%	45.0%	0.0%
Private office	45.3%	77.8%	5.0%	60.0%
Other: private clinic (1x)	1.1%	0.0%	2.5%	0.0%

2. How many patients with relapsing forms of MS (referred to as RMS in this survey) do you follow?

	Aggregate	U.S.	EU	Canada
Current number of patients with RMS - MEAN	175.7	226.3	106.5	225.0
Current number of patients with RMS - MEDIAN	100	120	80	140
Current number of patients with RMS - SUM	16,693	10,185	4,258	2,250

What is your annual growth rate for RMS patients?

	Aggregate	U.S.	EU	Canada
Estimated annual growth rate of RMS patients	13.1%	12.0%	15.4%	8.9%

How many patients with secondary progressive forms of MS do you follow?

	Aggregate	U.S.	EU	Canada
Current number of patients with secondary progressive MS - MEAN	55.9	65.2	39.2	81.0
Current number of patients with secondary progressive MS - MEDIAN	30	35	25	38
Current number of patients with secondary progressive MS - SUM	5,315	2,936	1,569	810

What is your estimated annual growth rate of secondary progressive MS patients?

	Aggregate	U.S.	EU	Canada
Estimated annual growth rate of secondary	9.5%	10.7%	9.0%	6.5%
progressive MS patients	3.570	10.7 70	3.070	0.570

3. What percent of your RMS patients have been on disease modifying therapy (any) for less than 1 year?

	Aggregate	U.S.	EU	Canada
Percent of RMS patients on therapy for less than 1 year	25.5%	17.1%	34.4%	28.0%

4. Describe your familiarity with each of the following products that have been approved recently or are in clinical development:

Tecfidera/BG12 (dimethyl fumarate)

	Aggregate	U.S.	EU	Canada		
Familiar only through CME or company information	31.6%	28.9%	37.5%	20.0%		
Familiar with clinical data	50.5%	66.7%	32.5%	50.0%		
Not familiar with data or product	17.9%	4.4%	30.0%	30.0%		

Lemtrada (alemtuzumab)

	Aggregate	U.S.	EU	Canada
Familiar only through CME or company information	35.8%	35.6%	40.0%	20.0%
Familiar with clinical data	31.6%	40.0%	25.0%	20.0%

Not familiar with data or product	32.6%	24.4%	35.0%	60.0%

Aubagio (teriflunomide)

	Aggregate	U.S.	EU	Canada
Familiar only through CME or company information	35.8%	35.6%	40.0%	20.0%
Familiar with clinical data	40.0%	64.4%	20.0%	10.0%
Not familiar with data or product	24.2%	0.0%	40.0%	70.0%

Laquinimod

	Aggregate	U.S.	EU	Canada
Familiar only through CME or company information	36.8%	44.4%	32.5%	20.0%
Familiar with clinical data	32.6%	31.1%	30.0%	50.0%
Not familiar with data or product	30.5%	24.4%	37.5%	30.0%

Daclizumab

	Aggregate	U.S.	EU	Canada
Familiar only through CME or company information	32.6%	37.8%	30.0%	20.0%
Familiar with clinical data	11.6%	11.1%	12.5%	10.0%
Not familiar with data or product	55.8%	51.1%	57.5%	70.0%

Ocrelizumab

	Aggregate	U.S.	EU	Canada
Familiar only through CME or company information	28.4%	35.6%	22.5%	20.0%
Familiar with clinical data	8.4%	8.9%	10.0%	0.0%
Not familiar with data or product	63.2%	55.6%	67.5%	80.0%

RPC-1063 (S1P receptor modulator)

	Aggregate	U.S.	EU	Canada
Familiar only through CME or company information	12.6%	13.3%	15.0%	0.0%
Familiar with clinical data	3.2%	4.4%	2.5%	0.0%
Not familiar with data or product	84.2%	82.2%	82.5%	100.0%

Siponimod aka BAF-312 (S1P receptor modulator)

	Aggregate	U.S.	EU	Canada
Familiar only through CME or company information	9.5%	8.9%	12.5%	0.0%
Familiar with clinical data	3.2%	4.4%	2.5%	0.0%
Not familiar with data or product	87.4%	86.7%	85.0%	100.0%

Ponesimod (S1P receptor modulator)

	Aggregate	U.S.	EU	Canada
Familiar only through CME or company information	9.5%	11.1%	10.0%	0.0%
Familiar with clinical data	4.2%	4.4%	5.0%	0.0%
Not familiar with data or product	86.3%	84.4%	85.0%	100.0%

ONP-4641 (S1P receptor modulator)

	Aggregate	U.S.	EU	Canada
Familiar only through CME or company information	9.5%	13.3%	7.5%	0.0%
Familiar with clinical data	4.2%	4.4%	5.0%	0.0%
Not familiar with data or product	86.3%	82.2%	87.5%	100.0%

5. Please estimate the proportion of your current RMS patients who:

	Aggregate	U.S.	EU	Canada
Are treatment naïve to a disease modifying therapy	12.9%	7.8%	17.2%	18.0%
Are stable on their current disease modifying therapy and are <u>unlikely</u> to switch	49.0%	54.5%	41.1%	55.5%
Are stable on their current disease modifying therapy but will <u>likely</u> switch	15.4%	15.7%	16.8%	8.5%
Are progressing on disease modifying therapy and will likely switch therapy	15.7%	17.2%	15.0%	11.5%
Are progressing on disease modifying therapy and will go off therapy	7.1%	4.8%	9.9%	6.5%

6. For your overall RMS patients, what percent are currently on the following therapies? What percent do you expect will be on the following therapies in 6 months, 18 months, and 3 years?

Aggregate

Aggregate	Currently (May 2013) (n=89)	6 months (November 2013)	18 months (November 2014)	3 years (May 2016)
Avonex	22.0%	19.0%	15.8%	12.5%
Betaseron	13.4%	12.2%	10.5%	9.6%
Copaxone	22.7%	19.9%	17.0%	14.1%
Rebif	18.6%	15.6%	12.7%	11.2%
Tysabri	6.8%	7.4%	8.4%	9.2%
Gilenya (FTY-720 or fingolimod)	5.2%	6.8%	8.6%	9.9%
Tecfidera (BG-12 or oral dimethyl fumarate)	1.0%	6.9%	12.0%	16.3%
Laquinimod		1.0%	2.1%	3.7%
Lemtrada (alemtuzumab)		0.8%	1.8%	2.6%
Aubagio (teriflunomide)	0.9%	2.2%	3.7%	4.4%
Nothing	8.5%	7.5%	6.4%	5.7%
Other: See Appendix	0.8%	0.6%	0.9%	0.9%

U.S.

	Currently (May 2013)	6 months (November 2013)	18 months (November 2014)	3 years (May 2016)
Avonex	20.6%	16.8%	14.4%	10.9%
Betaseron	13.0%	11.2%	9.2%	7.8%
Copaxone	26.3%	23.6%	20.0%	17.2%
Rebif	18.5%	15.6%	12.5%	11.0%
Tysabri	7.2%	8.1%	8.9%	10.0%
Gilenya (FTY-720 or fingolimod)	5.1%	6.5%	8.0%	8.8%
Tecfidera (BG-12 or oral dimethyl fumarate)	1.9%	9.5%	15.5%	20.6%
Laquinimod		0.8%	1.6%	2.8%
Lemtrada (alemtuzumab)		0.5%	1.6%	1.9%
Aubagio (teriflunomide)	1.8%	3.1%	4.4%	5.0%
Nothing	5.2%	3.9%	3.3%	3.7%
Other: See Appendix	0.3%	0.3%	0.6%	0.2%

ΕU

	Currently (May 2013) (n=34)	6 months (November 2013)	18 months (November 2014)	3 years (May 2016)
Avonex	22.3%	20.5%	16.9%	14.2%
Betaseron	12.9%	12.4%	11.1%	10.4%
Copaxone	18.6%	16.2%	14.0%	10.6%
Rebif	19.9%	16.8%	14.1%	12.4%
Tysabri	7.0%	7.1%	8.2%	8.8%
Gilenya (FTY-720 or fingolimod)	6.5%	7.7%	9.8%	11.9%
Tecfidera (BG-12 or oral dimethyl fumarate)	0.0%	3.5%	7.4%	10.2%
Laquinimod		1.1%	2.4%	4.5%
Lemtrada (alemtuzumab)		1.3%	2.4%	3.8%
Aubagio (teriflunomide)	0.0%	1.7%	3.6%	4.1%
Nothing	11.4%	10.6%	9.0%	7.6%
Other: See Appendix	1.3%	1.1%	1.3%	1.6%

Canada

	Currently (May 2013)	6 months (November 2013)	18 months (November 2014)	3 years (May 2016)
Avonex	27.3%	22.5%	18.0%	12.3%
Betaseron	16.6%	15.9%	14.1%	14.0%
Copaxone	20.3%	18.2%	15.7%	14.5%
Rebif	14.6%	11.4%	8.3%	7.6%
Tysabri	4.4%	5.1%	6.5%	7.1%
Gilenya (FTY-720 or fingolimod)	1.4%	4.4%	6.6%	6.3%
Tecfidera (BG-12 or oral dimethyl fumarate)	0.6%	9.0%	14.4%	21.1%
Laquinimod		1.5%	3.2%	4.5%
Lemtrada (alemtuzumab)		0.5%	0.6%	1.4%
Aubagio (teriflunomide)	0.0%	0.3%	1.3%	2.7%
Nothing	13.8%	10.7%	10.3%	7.5%
Other: See Appendix	1.0%	0.5%	1.0%	1.0%

If your patients are not on any therapy, please explain why?

See Appendix for a summary of responses

7. Of your stable disease patients who are likely to switch, what percentage are likely to seek a new therapy over the next 6 months? What percentage is likely to seek new therapy in the next 18 months and 3 years?

	Aggregate	U.S.	EU	Canada
Percent who are likely to seek a new therapy over the next 6 months	14.3%	15.0%	14.2%	11.1%
Percent who are likely to seek a new therapy over the next 18 months	22.1%	21.8%	22.5%	22.0%
Percent who are likely to seek a new therapy over the next 3 years	30.5%	30.0%	31.0%	31.2%

8. Among patients **new to disease modifying therapy (treatment naïve)**, indicate the percent you expect to start on each of the following therapies during the next year to 3 years:

Aggregate

	6 months from now (November 2013)	18 months from now (November 2014)	3 years from now (May 2016)
Avonex	18.8%	15.2%	12.7%
Betaseron	11.2%	9.5%	8.2%
Copaxone	20.7%	17.3%	15.6%
Rebif	15.9%	12.9%	11.2%
Tysabri	4.2%	5.3%	5.8%
Gilenya (FTY-720 or fingolimod)	7.0%	8.4%	9.4%
Tecfidera (BG-12 or oral dimethyl fumarate)	13.5%	18.7%	22.0%
Laquinimod	1.5%	3.0%	4.1%
Lemtrada (alemtuzumab)	1.6%	2.5%	3.2%
Aubagio (teriflunomide)	4.9%	6.3%	6.7%
Other: Nothing (1x); synacthen depot (1x);			
steroid (1x); Novantrone (1x); LFN pegyle (1x); daclizumab, ocrelizumab (1x); cytoxan (1x); campath 45 (1x); azatioprine (1x)	0.8%	0.9%	1.1%

U.S.

	6 months from now (November 2013)	18 months from now (November 2014)	3 years from now (May 2016)
Avonex	13.4%	10.5%	9.2%
Betaseron	6.9%	5.5%	4.6%
Copaxone	23.0%	19.9%	18.0%
Rebif	13.1%	11.0%	9.7%
Tysabri	3.3%	4.5%	5.0%
Gilenya (FTY-720 or fingolimod)	8.3%	9.0%	9.2%
Tecfidera (BG-12 or oral dimethyl fumarate)	21.3%	26.1%	28.6%
Laquinimod	1.1%	2.4%	3.5%
Lemtrada (alemtuzumab)	1.0%	2.5%	3.2%
Aubagio (teriflunomide)	7.5%	7.8%	7.9%
Other	1.0%	0.8%	1.2%

ΕU

	6 months from now (November 2013)	18 months from now (November 2014)	3 years from now (May 2016)
Avonex	22.5%	19.2%	16.3%
Betaseron	15.3%	13.3%	11.1%
Copaxone	18.4%	14.8%	13.1%
Rebif	19.4%	15.6%	13.0%
Tysabri	4.9%	6.1%	6.6%
Gilenya (FTY-720 or fingolimod)	6.7%	8.9%	10.9%
Tecfidera (BG-12 or oral dimethyl fumarate)	4.9%	9.9%	13.4%
Laquinimod	2.1%	3.3%	4.7%
Lemtrada (alemtuzumab)	2.4%	3.0%	4.0%
Aubagio (teriflunomide)	3.0%	5.5%	6.0%
Other	0.5%	0.8%	0.9%

Canada

	6 months from now (November 2013)	18 months from now (November 2014)	3 years from now (May 2016)
Avonex	28.5%	20.9%	14.6%
Betaseron	14.0%	12.5%	12.5%
Copaxone	19.3%	15.5%	14.6%
Rebif	14.4%	10.9%	10.6%
Tysabri	5.4%	5.9%	5.9%
Gilenya (FTY-720 or fingolimod)	2.6%	4.1%	4.1%
Tecfidera (BG-12 or oral dimethyl fumarate)	12.8%	20.7%	27.2%
Laquinimod	0.5%	5.0%	4.5%
Lemtrada (alemtuzumab)	0.5%	0.5%	0.5%
Aubagio (teriflunomide)	0.5%	2.5%	4.0%
Other	1.5%	1.5%	1.5%

9. Prior to the availability of Tecfidera, how many of your overall RMS patients did you have "warehoused" or awaiting therapy that you now plan to treat with Tecfidera?

	Aggregate	U.S.	EU	Canada
# of RMS patients warehoused or awaiting Tecfidera therapy - MEAN	7.0	8.7	5.1	6.8
# of RMS patients warehoused or awaiting Tecfidera therapy - MEDIAN	2	3	3	2
# of RMS patients warehoused or awaiting Tecfidera therapy - SUM	664	391	205	68

10. Please estimate the number of patients for whom you have written a Tecfidera prescription and of these, please estimate the number currently on Tecfidera (e.g. filled prescription)?

	Aggregate (n=85)	U.S.	EU (n=30)	Canada
# of RMS patients for whom I have written Tecfidera so far - MEAN	3.2	5.9	0.0	0.8
# of RMS patients for whom I have written Tecfidera so far - MEDIAN	0	2	0	0
# of RMS patients for whom I have written Tecfidera so far - SUM	274	266	0	8
# of RMS patients currently on Tecfidera (e.g. filled prescription)- MEAN	0.9	1.7	0.0	0.2
# of RMS patients currently on Tecfidera (e.g. filled prescription)- MEDIAN	0	0	0	0
# of RMS patients currently on Tecfidera (e.g. filled prescription)- SUM	79	78	0	2

11. To date, has reimbursement for Tecfidera limited your ability to successfully prescribe the drug?

	Aggregate (n=55)	U.S.	Canada
Yes, reimbursement has reduced my early usage of Tecfidera	20.0%	13.3%	50.0%
No, reimbursement has not reduced my early usage of Tecfidera	80.0%	86.7%	50.0%

Of your RMS patients that have filled their Tecfidera prescription, how many (in WHOLE NUMBERS) were receiving the following therapies?

	Aggregate (n=23)	U.S. (n=21)	Canada (n=2)
Treatment naïve patient – MEAN	0.7	0.7	0.5
Treatment naïve patient – MEDIAN	0	0	1
Treatment naïve patient – SUM	15	14	1
Avonex – MEAN	0.8	0.9	0.5
Avonex – MEDIAN	1	1	1
Avonex – SUM	19	18	1
Betaseron – MEAN	0.2	0.2	0.0
Betaseron – MEDIAN	0	0	0
Betaseron – SUM	5	5	0
Copaxone – MEAN	0.7	0.8	0.0
Copaxone – MEDIAN	1	1	0
Copaxone – SUM	16	16	0
Rebif – MEAN	0.5	0.5	0.0
Rebif – MEDIAN	0	0	0
Rebif – SUM	11	11	0
Tysabri – MEAN	0.4	0.4	0.0
Tysabri – MEDIAN	0	0	0
Tysabri – SUM	9	9	0
Gilenya – MEAN	0.1	0.1	0.0
Gilenya – MEDIAN	0	0	0
Gilenya – SUM	2	2	0
Other – MEAN	0.0	0.0	0.0
Other – MEDIAN	0	0	0
Other – SUM	0	0	0

12. Please rate on a scale of 1-5 (1 = extremely dissatisfied to 5 = extremely satisfied) for each of the following therapies with regard to your experience:

Aggregate

7.99.09				
	Aubagio	Copaxone	Gilenya	Tecfidera
Treatment initiation logistics	3.4 (n=63)	3.9 (n=94)	3.0 (n=91)	3.7 (n=46)
Drug company's overall physician practice support	3.4 (n=65)	3.9 (n=94)	3.6 (n=92)	4.0 (n=47)
Drug tolerability	3.3 (n=63)	3.8 (n=94)	3.7 (n=90)	3.6 (n=39)
Overall patient reimbursement support	3.2 (n=62)	3.9 (n=94)	3.4 (n=89)	3.7 (n=39)
Frequency of patient complaints on treatment	3.4 (n=60)	3.5 (n=94)	3.6 (n=88)	3.4 (n=35)
Patients' overall satisfaction with drug	3.4 (n=60)	3.5 (n=94)	3.6 (n=88)	3.6 (n=36)

U.S.

	Aubagio	Copaxone	Gilenya	Tecfidera
Treatment initiation logistics	3.7 (n=41)	4.4 (n=45)	2.9 (n=44)	3.7 (n=42)
Drug company's overall physician practice support	3.6 (n=42)	4.3 (n=45)	3.6 (n=45)	4.0 (n=43)
Drug tolerability	3.5 (n=40)	4.0 (n=45)	3.9 (n=43)	3.6 (n=35)
Overall patient reimbursement support	3.5 (n=40)	3.9 (n=45)	3.4 (n=42)	3.6 (n=35)
Frequency of patient complaints on treatment	3.6 (n=39)	3.6 (n=45)	3.8 (n=41)	3.3 (n=31)
Patients' overall satisfaction with drug	3.5 (n=39)	3.7 (n=45)	3.8 (n=41)	3.6 (n=32)

ΕU

	Aubagio	Copaxone	Gilenya
Treatment initiation logistics	2.9 (n=20)	3.5 (n=40)	3.1 (n=39)
Drug company's overall physician practice support	3.0 (n=21)	3.7 (n=40)	3.6 (n=39)
Drug tolerability	3.0 (n=21)	3.6 (n=40)	3.4 (n=39)
Overall patient reimbursement support	2.5 (n=20)	3.9 (n=40)	3.5 (n=39)
Frequency of patient complaints on treatment	3.2 (n=19)	3.4 (n=40)	3.5 (n=39)
Patients' overall satisfaction with drug	3.2 (n=19)	3.3 (n=40)	3.6 (n=39)

Canada

	Aubagio	Copaxone	Gilenya	Tecfidera
Treatment initiation logistics	3.0 (n=2)	3.7 (n=9)	3.0 (n=8)	3.8 (n=4)
Drug company's overall physician practice support	2.5 (n=2)	3.3 (n=9)	3.8 (n=8)	4.0 (n=4)
Drug tolerability	4.0 (n=2)	3.6 (n=9)	3.5 (n=8)	4.0 (n=4)
Overall patient reimbursement support	3.0 (n=2)	3.8 (n=9)	3.4 (n=8)	4.0 (n=4)
Frequency of patient complaints on treatment	3.0 (n=2)	3.7 (n=9)	3.4 (n=8)	4.0 (n=4)
Patients' overall satisfaction with drug	3.0 (n=2)	3.1 (n=9)	3.3 (n=8)	3.5 (n=4)

13. In June 2012, TEVA disclosed top-line data from the Phase III GALA study looking at Copaxone 40 mg/1 ml administered three times weekly.

Please click below for top-line data from Teva's press release:

http://ir.tevapharm.com/phoenix.zhtml?c=73925&p=irol-newsArticle&ID=1705260&highlight=

If Copaxone 40mg/1ml three times weekly formulation is approved please estimate the percentage of your existing Copaxone patients you plan to switch to the new 3x-weekly formulation.

	Aggregate	U.S.	EU	Canada
Percent of your existing Copaxone patients you plan to switch to the new 3x-weekly formulation	45.3%	53.1%	41.9%	24.0%

14. If a generic version of Copaxone becomes available, please estimate the percentage of patients on the following therapies that you'd switch to generic Copaxone.

	Aggregate	U.S.	EU	Canada
Interferon	6.7%	6.4%	8.2%	2.0%
Gilenya	3.4%	3.0%	4.2%	2.0%
Tecfidera	2.3%	1.6%	3.5%	1.0%
Aubagio	2.7%	2.3%	3.5%	1.0%

15. If an AB-rated (e.g., substitutable) generic version of Copaxone became available, in your opinion would it be ethical to require that Copaxone be used as recommended first-line therapy in your RMS patients?

	Aggregate	U.S.	EU	Canada
Yes	40.0%	26.7%	52.5%	50.0%
No	60.0%	73.3%	47.5%	50.0%

16. What percent of your SPMS patients continue to have relapses?

	Aggregate	U.S.	EU	Canada
Percent of your SPMS patients that continue to have relapses	27.9%	25.8%	33.9%	13.2%

17. What percent of your SPMS patients are treated with the following?

	Aggregate	U.S.	EU	Canada
Tysabri	15.6%	17.6%	14.0%	13.6%
Gilenya	8.7%	10.2%	8.0%	4.2%
Interferons	42.9%	40.9%	42.5%	53.5%
Novantrone	4.7%	2.3%	6.7%	8.0%
Methotrexate	4.6%	2.6%	6.5%	6.2%
Other: See Appendix	23.5%	26.5%	22.4%	14.5%

18. Please describe how your SPMS patients treated with Gilenya appear to benefit, if at all (i.e., disability progression, etc.):

See Appendix for a summary of responses

19. Please describe how your SPMS patients treated with <u>Tysabri</u> appear to benefit, if at all (i.e., EDSS improvement, etc.):

See Appendix for a summary of responses

20. Tysabri is being evaluated in the Phase III ASCEND Trial for SPMS patients. Topline results from this trial are expected in 2015.

Please click below to view the trial design.

http://clinicaltrials.gov/ct2/show/NCT01416181?term=tysabri+ASCEND&rank=1

How would POSITIVE ASCEND trial results (meets primary endpoint) change your Tysabri use in SPMS patients?

	Aggregate	U.S.	EU	Canada
Increase use	69.5%	77.8%	67.5%	40.0%
No change in use	30.5%	22.2%	32.5%	60.0%
Decrease use	0.0%	0.0%	0.0%	0.0%
Overall change	15.5%	17.9%	13.1%	14.0%

How would NEGATIVE ASCEND trial results (fails primary endpoint) change your Tysabri use in SPMS patients?

	Aggregate	U.S.	EU	Canada
Increase use	0.0%	0.0%	0.0%	0.0%
No change in use	67.4%	66.7%	67.5%	70.0%
Decrease use	32.6%	33.3%	32.5%	30.0%
Overall change	-9.8%	-7.4%	-12.8%	-9.0%

If you indicated decrease use of Tysabri, please list what therapy (ies) you would use in its place:

See Appendix for a summary of responses

21. Please read the following product profile. Comparative data and a description from the label for fingolimod (Gilenya) have been provided for your reference.

Drug	Novel S1P1 inhibitor	Gilenya (fingolimod)
Description	Novel S1P1 inhibitor is a selective S1P1 receptor modulator currently in clinical development for the treatment of relapsing multiple sclerosis.	Fingolimod (Gilenya) is metabolized by sphingosine kinase to the active metabolite, fingolimod-phosphate. Fingolimod-phosphate is a sphingosine 1-phosphate receptor modulator, and binds with high affinity to sphingosine 1-phosphate receptors 1, 3, 4, and 5. Fingolimod-phosphate blocks the capacity of lymphocytes to egress from lymph nodes, reducing the number of lymphocytes in peripheral blood. The mechanism by which fingolimod exerts therapeutic effects in multiple sclerosis is unknown, but may involve reduction of lymphocyte migration into the central nervous system.
Lymphocyte Reductions during treatment:	Efficacy comparable to fingolimod (Gilenya)	Between 70% (Study 1) and 83% (Study 2) of patients were relapse- free at 2 years. Risk of disability progression as measured by EDSS was reduced by 30% (Study 1) and 29% (Study 2)
Lymphocyte Reductions during treatment:	Lymphocytes counts decrease to 30-50% of baseline (e.g. decrease 50-70%)	In a study in which 12 subjects received GILENYA 0.5 mg daily, the lymphocyte count decreased to approximately 60% of baseline within 4-6 hours after the first dose. With continued daily dosing, the lymphocyte count continued to decrease over a 2-week period, reaching a nadir count of approximately 500 cells/ μ L or approximately 30% of baseline.
Lymphocyte Recovery:	T1/2=19hrs; 3 day recovery	Peripheral lymphocyte count increases are evident within days of stopping fingolimod treatment and typically normal counts are reached within 1 to 2 months.
Cardiovascular (CV) profile:	No impact on heart-rate or atrioventricular conduction and no requirement for first-dose monitoring Thorough QT Study (TQTc) Trial Result: No evidence of QTc prolongation	Decrease in heart rate and/or atrioventricular conduction after first dose of GILENYA: Monitor patients In a thorough QT interval study of doses of 1.25 or 2.5 mg fingolimod at steady-state, when a negative chronotropic effect of fingolimod was still present, fingolimod treatment resulted in a prolongation of QTc, with the upper bound of the 90% confidence interval (CI) of 14.0 ms. There is no consistent signal of increased incidence of QTc outliers, either absolute or change from baseline, associated with fingolimod treatment. In MS studies, there was no clinically relevant prolongation of QT interval, but patients at risk for QT prolongation were not included in clinical studies.
Hepatotoxicity: Liver Function Test (LFTs) >3x Upper Limit Normal (ULN)	No increases over 28 day dosing trial	ALT/AST increased in 14% of patients versus 5% of patients treated

Assuming the S1P1 inhibitor were FDA approved in 2018 with the product profile outlined in the table above, please indicate in what proportion of your patients you would use the novel S1P1 inhibitor instead of:

	Aggregate	U.S.	EU	Canada
Gilenya	42.8%	57.5%	31.7%	21.0%
Tecfidera	13.2%	12.6%	15.9%	5.0%
Tysabri	17.1%	15.0%	20.0%	15.5%
ABCRs	17.1%	23.4%	13.3%	4.0%
Other: Tecfidera (1x); methotrexate (1x); immuno- suppressors (1x); Copaxone (1x); Azatioprina (1x)	0.8%	0.4%	1.5%	0.0%

Appendix: Summary of responses.

Question 6: For your overall RMS patients, what percent are currently on the following therapies? What percent do you expect will be on the following in 6 months, 18 months and 3 years? Please specify other.

5	Cytoxan
14	Daclizumab, ocrelizumab
18	Steroids
28	Novantrone
39	Extavia
54	Mitoxantrone
55	Mitoxantrone
67	Azatioprina
68	Immunosuppressants
80	Pegylated interferon
81	Synacthen Depot
85	PEG IFN

Question 6: If your patients are not on any therapy, please explain why?

1	Insurance, patient decision
2	Some can't afford it no matter what, and some don't care. Some prefer holistic approach.
3	Don't want it
4	Patient choice
5	Benign disease
6	Benign MS and also due to high cost of medications
7	Some may refuse or this therapy is not needed.
8	Patient refusal
9	Their desire not to take anything
11	Some patients simply refuse to be on any agent
12	Do not like to be on medicine
13	A few patients refuse therapy and think they are doing well
14	Very mild disease, or uninsured
15	Difficulty paying
16	Most feel that their symptoms don't require rx even when told about the unpredictable nature of the disease in the future
17	There are always some who feel their disease will not be severe, do not want to take medication or have reactions to any
17	medication given to them.
18	They refuse - alternative therapies
19	No active lesions
20	Resistant or unable to afford
21	Intolerant of side effects, their choice
22	Side effects, mild sx
23	Refuse therapy, needle/compliance issues
24	Benign disease and patient preference not to treat
25	Patient choice - usually concern about long term side effects
26	Refusal
27	Don't want to try anything
28	Stable no progression
29	Some patients refuse, some are too severely impaired
31	Their informed choice.
33	Their refusal
35	They can't afford it, they primary or secondary progressive multiple sclerosis
36	Patients decline
37	Chronic stable benign MS or not yet started or newly diagnosed
38	Other immune diseases Antibodies

39	Patient is unwilling to take medication (unable to comprehend need, but makes own decisions)
40	Refused, don't feel they need treatment
41	They want alternative medications.
42	Disease mild - preferred not to take Rx
43	All patients are and are expected to be on therapy.
44	Their choice
45	They have considered the potential side effects and do not want to use the medications at this time
47	Insufficiently active MS
48	Side effect profile; and lack of efficacy
49	Personal decision
50	Very low MS-activity
51	Side effects and comorbidities
52	Do not meet criteria
54	They don't want
55	Choice of patient
56	Patients are in stable condition with no relapse for more than two years, suffered from side effects under treatment
57	Disease not in progress
58	Mild symptoms, low rate of relapses. Not wishing to have any therapy.
59	Not indicated
60	That do not need
61	Do not want any therapy
62	Patient decision. Adverse event
63	Because they do not want a medication. Or they do Not want a medication at all.
64	Sometimes they don't want it and sometimes the risk is too high.
65	Patient choice, sometimes side effects, costs or availabilities of the drug, guidelines such as NICE
66	Personal decision of patients
67	Refusal or higher EDSS disability
69	Stable
70	Contraindications or patient willingness
71	Choice of patient
72	Not compliant
73	Refusal
74	Don't want therapy
75	Benign form
76	Pregnant
77	Their wish
79	They don't want any therapy because of drugs' adverse events
80	They refuse the disease and the treatment
81	They refuse
82	No need
84	Patients wish
85	MS too mild of patient refuses to be treated
86	Do not want therapy, benign
87	Their choice
88	Patient preference, benign MS
90	Patient wishes, benign MS
91	Patient choice usually
92	Reimbursement
93	Clinically stable or do not desire to be on medication
94	Patients do not want therapy
95	Unable to afford or non-compliant

Question 17: What percent of your SPMS patients are treated with the following? Please specify other.

1	Copaxone
3	Nothing
5	Cytoxan
7	Copaxone
8	Cytoxan; rituxan
10	Copaxone
11	No therapy
12	On no treatment
14	Nothing
16	Copaxone
18	Achthar; nothing
20	Copaxone
21	Steroids, Copaxone
23	Copaxone
24	Copaxone
25	Copaxone
27	Nothing
29	Copaxone
31	No treatment
34	Copaxone
35	None
37	Copaxone
39	Copaxone
42	Copaxone
45	Steroids
47	No treatment
52	No treatment
54	Azatioprina
55	Azatioprina
56	No treatment
58	No therapy
61	Corticosteroids
62	No treatment
66	No specific therapy
69	No therapy
71	Azatioprine
74	Nothing
75	Aucun traitement
77	Cortisone
79	Azathioprin, cyclophosphamid
81	Synacthen depot
85	Imurel 3 % and nothing 83%
86	Copaxone
91	Copaxone
94	Immuran, doxciclilic

Question 18: Please describe how your SPMS patients treated with Gilenya appear to benefit, if at all (i.e., disability progression, etc.):

1	About 50% of patients benefit on Gilenya
2	Probably benefit. Seems to help slow down their gradual progression.
3	None
7	No more relapses

9 Stabilize 12 Some do well but most continue to progress 13 Tolerability is better than interferons, slows progression	
1.3 I Tolerability is better than interferons, slows progression	
14 Disability progression 15 Delays in progression	
17 A few seem to stabilize, none improve.	
18 Disability progression might be affected.	
20 Stabilized progression 22 No real benefit yet	
22 No real benefit yet 24 Hopeful that it delays progression of disability	
29 Less attacks, slowed progression 30 I believe it slows disability progression over time	
32 I can say I see any difference so far 33 So far they've been stable	
34 Not enough data	
35 If approved by FDA I will use it. 36 Less relapses and improved MRIs	
38 Slight improvement in day-to-day activities. 39 It seems to be working really well for slowing, or more often stopping disability progression	
40 Very well tolerated, good efficacy, oral	
41 My secondarily progressive are referred out, but they are stable	
43 Progression seems to stop on Gilenya.	
45 Progression seems to stop on Glienya. 45 Walking seems to be better.	
48 Halting disease progression seemingly with good side-effect profile	
50 Slow progression, less disability	
51 Improvement in EDSS, quality of life, percentage of handicap decreased, few side effects	
53 Safety	
54 A little	
55 They are stable	
56 Not treated patients, no benefit	
58 Appears to reduce progression of disability and rate of relapses a bit.	
59 Benefit	
60 Overall improvement	
61 Slower progression	
62 No benefit	
65 May help to improve or maintain mobility and independent living at home	
66 Reduction of disability	
68 Slow the disability progression and reduce the rate of relapses	
69 No benefit	
70 Better quality of life and less disability progression	
71 No more relapses	
72 Moderate	
73 Less progression	
74 Reduced relapses	
75 No patients on Gilenya	
76 Nothing	
77 Disability progression	
78 Only 10% of my patients are treated with Gilenya and they are satisfied	
In my opinion, there IS no benefit of Gilenya for these nations. My nations who are still treate	ed by this drug were
considered RMS a few months ago.	·
80 No benefit	
83 SPMS Patients with Gilenya have a good benefit, EDSS showed no progress	
84 Stable symptoms	
88 Less disease progression	

89	None are
90	Reduced disability progression
91	Only had 1 patient and they stopped due to progression issues
92	No patient treated
93	Not tried
94	Not approved in the province for SPMS
95	Don't use Gilenya

Question 19: Please describe how your SPMS patients treated with Tysabri appear to benefit, if at all (i.e., EDSS improvement, etc.):

1	About 70% have some subjective and/or objective improvement /change of progression on Tysabri
2	Same as above, only more pronounced.
3	Seem to be stable
4	Hard to tell
5	60% response
6	Some patients report improvement of gait and energy level
7	No more relapses or MRI activity
8	No patients treated
9	Stabilize and more hope
11	I have had some patients with SPMS who seem to have stabilized clinically while taking Tysabri with regard to EDSS.
12	Relapses stop but progression does not
13	Less disability and more mobility
14	Disability progression
15	Excellent maintenance of deficit
16	Most seem to benefit and worsen with discontinuation
17	They stop progressing.
20	Stabilized
21	Less progression
22	Fewer relapses
23	Feel better, less progression, stable MRI
24	Hopeful that it delays progression of disability
25	Also no experience unless they are having relapses - in which case, relapses seem to cease
26	Literature claims it is effective.
27	They don't
28	Stabilization of disease, improvement in well-being
29	Less attacks, less progression
30	I believe it slows EDSS worsening, and frequency of relapses
31	No patients
32	Most if not all seem to have stabilized over the past few years
33	So far most have been stable
34	High efficacy
35	They will definitely benefit
36	Less relapses and improved MRIs
37	Stabilization, often some improvement
38	Moderate improvement in EDSS scores.
39	Not yet started
40	Good efficacy, few relapses, less disability
41	EDSS seems to be holding as per MS Neurology group
42	NA - not approved for SPMS
43	Progression stops on Tysabri.
44	Not clear
45	Overall improved ADLS
48	Less side-effects and improved mobility
49	Slow progression, no relapses

50	Nearly no relapses
51	Severe patients, progress in EDSS, increased relapses
52	Slowing of progression
53	Mild collateral effects
54	Stability of progression
55	Stable
56	No patients, no benefit
57	Maybe progress is not so fast
58	Appears to reduce rate of relapses, development of new symptoms, and progression of disability and a bit.
59	EDSS
60	Overall improvement
61	Slower progression
62	No benefit
63	SPMS patients do not get it from me.
64	No patients
	Can be used if other agents have failed but often have modest effect. It can relieve some symptoms and help maintain
65	function
66	Reduction of disabling attacks
67	None
68	Slow the disability progression and improves the quality of life
69	No benefit
70	Better quality of life/ function
71	No more relapses, less fatigue
72	Moderate
73	Stable disease
74	Reduced relapses
76	Less relapsing
77	Disability progression
78	Patients treated with Tysabri appear a clinical benefit and a positive improvement in the EDSS score
79	No effect
80	No benefit
82	Very good
83	I can say SPMS patients with Tysabri have stable EDSS and have a good benefit
84	Certain improvement ,less tired
88	Fewer relapses
89	None are
90	Reduced relapses
91	Have not had any
92	No patient treated
93	Not indicated
94	Not approved in the province for SPMS
95	Improved QOL

Question 20: You indicated decreased use of Tysabri, please list what therapy (ies) you would use in its place:

6	Mitoxantrone
9	Gilenya or BG-12
11	I might consider Campath if it becomes available.
12	BG-12
13	More Gilenya and Aubagio
16	Probably Copaxone, Tecfidera, Gilenya
24	Possibly an interferon or Tecfidera, despite absence of data
26	Novantrone methotrexate
27	No therapy
29	Probably an oral drug

22	Lamburda
33	Lemtrada
35	Tecfidera
38	Tecfidera, alemtuzamab
40	Gilenya
44	Oral agents
49	Interferon
54	Immunosuppressant
58	Gilenya, interferons
61	Copaxone, betaferon
63	An interferon, Gilenya or One of the New medications, that will appear
64	Interferon, Copaxone, Gilenya
65	Interferons perhaps, or any of the other cheaper alternatives first
68	Gilenya or laquinimod
69	Only therapies with proven benefit
72	Gilenya
74	Gilenya
77	Gilenya
83	Same benefit without potential PML, when patient got therapy for more than 3 years!!!
91	Gilenya, tecfidera
93	Laquinimod



Disclosures Appendix Analyst Certification

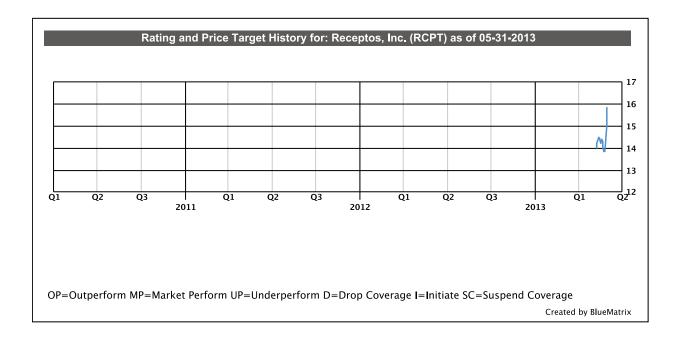
I, Marko Kozul, M.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

Valuation

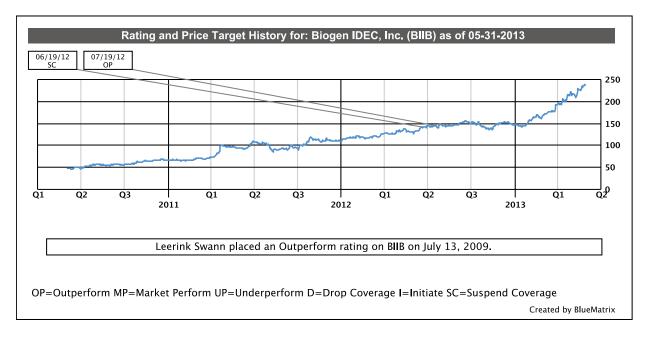
RCPT shares are poised to appreciate near and longer term driven by clinical progress and the commercialization of lead compound RPC-1063. We apply a 12-month valuation of RCPT shares of ~\$30 a share based on a discounted cash flow analysis. We apply a discount rate of 11% and a terminal growth rate of 1% which translates to a 10x terminal multiple which we believe is comparable to biotechnology companies in a similar development stage.

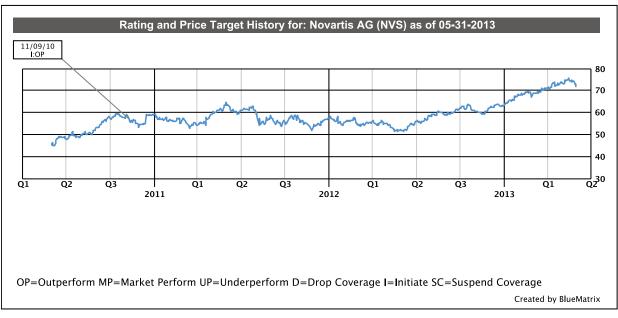
Risks to Valuation

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.

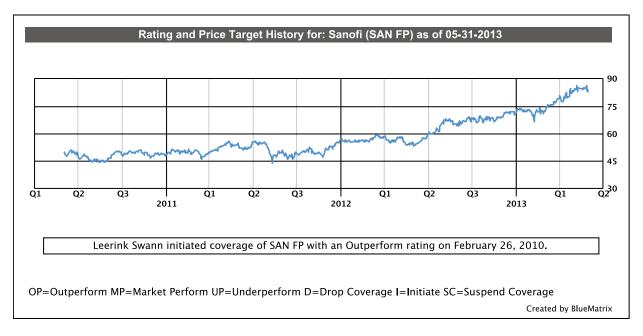


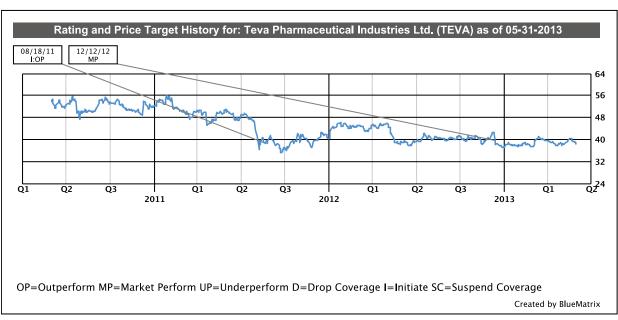














	Distribution of Ratings/Investment Banking Services (IB) as of 03/31/13 IB Serv./Past 12 Mos.					
Rating		Count	Percent	Count	Percent	
BUY [OP]		107	61.14	32	29.91	
HOLD [MP] SELL [UP]		68 0	38.86 0.00	0	0.00 0.00	

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.

From October 1, 2006 through January 8, 2009, the relevant benchmarks for the above definitions were the Russell 2000® 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.

Definitions of Leerink Swann Ratings prior to October 1, 2006 are shown below:

Outperform (Buy): We expect this stock to outperform its benchmark by more than 10 percentage points over the next 12 months.

<u>Market Perform (Hold/Neutral):</u> We expect this stock to perform within a range of plus or minus 10 percentage points of its benchmark over the next 12 months.

<u>Underperform (Sell):</u> We expect this stock to underperform its benchmark by more than 10 percentage points over the next 12 months.

For the purposes of these definitions, the relevant benchmark were the Russell 2000® Health Care Index for issuers with a market capitalization of less than \$2 billion and the S&P 500® 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 asset management group and 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 Publishing 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, the Private Client Division, Institutional Equities, and Investment Banking. Analysts, however, are not compensated for a specific investment banking services transaction.

Leerink Swann Consulting LLC, an affiliate of Leerink Swann LLC, 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 Swann LLC makes a market in Receptos, Inc., Biogen IDEC, Inc. and Teva Pharmaceutical Industries Ltd.

Leerink Swann LLC is willing to sell to, or buy from, clients the common stock of Novartis AG and Sanofi 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 and Teva Pharmaceutical Industries Ltd.

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

©2013 Leerink Swann LLC. All rights reserved. This document may not be reproduced or circulated without our written authority.

	Leerink Swann 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				
	Irene Lau	(415) 905-7256	irene.lau@leerink.com				
	Rene Shen	(212) 277-6074	rene.shen@leerink.com				
	Gena Wang, Ph.D.	(212) 277-6073	gena.wang@leerink.com				
	Paul Matteis	(617) 918-4585	paul.matteis@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				
	Swati Kumar	(617) 918-4576	swati.kumar@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				
	responential such such such such such such such such	(212) 277 0001					
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				
Supervisory Analysts	Robert Egan	•	bob.egan@leerink.com				
. , , , , , , , , , , , , , , , , , , ,	Amy N. Sonne		amy.sonne@leerink.com				
Research Assistant	Paul Matteis	(617) 918-4585	paul.matteis@leerink.com				
	George Villarina	(212) 277-6012	george.villarina@leerink.com				
	Coorgo vinarina	(212) 211 0012	goorgo.viiidiiiid@iooriiiid.oorii				

New York 1251 Avenue of Americas, 22nd Floor New York, NY 10020 (888) 347-2342 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