

# Acceleron Pharma Inc. (XLRN)

Raising Price Target to \$36 on Increased Confidence

MARKET DATA	
Price	\$28.07
52-Week Range:	\$16.78 - \$30.00
Shares Out. (M):	26.5
Market Cap (\$M):	\$743.9
Average Daily Vol. (000):	91.0
Cash (M):	\$116
Cash/Share:	\$4.13
Enterprise Value (M):	\$842
Float (M):	24.3
LT Debt (M):	\$14
Source: Thomson Reuters and JMP Securities LLC	

FY DEC		2012A	2013E	2014E
Revenue (\$M)	1Q		\$15.0A	\$4.9
	2Q		\$26.4A	\$12.2
	3Q		\$4.3A	\$5.4
	4Q		\$4.7	\$20.9
	FY	\$15.3	\$50.4	\$43.4
EPS	1Q		\$0.12A	(\$0.33)
	2Q		\$0.44A	(\$0.09)
	3Q		(\$5.62)A	(\$0.34)
	4Q		(\$0.32)	\$0.13
	FY	(\$1.43)	(\$0.82)	(\$0.57)
	P/E	NM	NM	NM
Source: Company r	eports an	d JMP Securities	LLC	



MARKET OUTPERFORM | Price: \$28.07 | Target Price: \$36.00

## **INVESTMENT HIGHLIGHTS**

We reiterate our Market Outperform rating on Acceleron Pharma and increase our price target from \$32 to \$36, based on elevated confidence in the sotatercept/ ACE-536 program following positive Phase II data in β-thalassemia presented at ASH 2013. Recall, updated sotatercept results show meaningful increases in hemoglobin (Hgb) levels in the latest dosing cohort of 0.5mg/kg. Specifically, a third of non-transfusion dependent patients saw ≥2g/dL gains in Hgb, exceeding our expectation of a 1.5g/dL increase as a measure of clinical benefit. We anticipate further Hgb gains as the trial escalates in dose and continued safety given the favorable tolerability profile shown to date. We believe these data further de-risk sotatercept/ ACE-536 development and, therefore, are lowering our discount factor for the program from 30% to 25%, incrementally lowering our blended discount rate from 32.9% to 30.7%. Revenue assumptions in our model remain unchanged. Our new \$36 price target is derived through a synthesis of DCF (\$31.20), comparable (\$44.61) and CAGR model valuation methodologies (\$35.17).

Acceleron represents a compelling opportunity in the biotech space over the course of the next several years. Our view is drawn from the company's focus and understanding of TGF beta biology, as well as developmental and commercialization advantages offered through its strategic partnership with Celgene (CELG, MO, \$175 PT). In our opinion, the company's first wave of product candidates, validated by Celgene's significant investment, is but the tip of the iceberg that, over time, should create significant value for shareholders.

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# FIGURE 1. Upcoming Milestones

Timing	Drug	Milestones
4Q13	Sotatercept	Update from Sotatercept Phase II trial in $\beta$ -thalassemia at ASH (Dec 9)
4Q13/1Q14	Sotatercept	Initiation of Phase II trial in patients with end stage renal disease (ESRD)
4Q13/1Q14	Dalantercept	Preliminary data from Phase II, GOG-sponsored trials in ovarian cancer
1Q14	Sotatercept & ACE-536	Initiation of RP2D expansion cohorts in ongoing β-thalassemia Phase II trials
1Q14	Dalantercept	Preliminary data from dose-escalation stage of Phase II RCC trial in combination with axitinib; start of randomized stage versus axitinib alone
2Q14	Sotatercept & ACE-536	Presentation of dose escalation Phase II results in $\beta\text{-thalassemia}$ and MDS
3Q14	Dalantercept	Initiation of Phase II trial(s) in additional indication(s)
4Q14	Sotatercept & ACE-536	Final results from Phase II trials in β-thalassemia and MDS
4Q14	Sotatercept & ACE-536	Initiation of Phase III trial in β-thalassemia and/or MDS
4Q14	ACE-083	Initiation of Phase I trial in muscular dystrophy

Source: JMP Securities LLC and Company Reports



FIGURE 2. Discounted Cash Flow Model

Discounted Cash Flow Model	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
Product Revenues	20132	2017	ZUISE	ZUTUL	ZUITE	ZUTUL	ZUIJL	ZUZUL	ZUZIL	LULLL	ZUZUL	ZUZTE	ZUZUL
Sotatercept / ACE-536 Royalty Revenue	_	-	_	3.3	34.7	123.9	252.5	429.8	583.0	743.4	856.9	953.2	1.010.7
Dalantercept	-	-	-	-	-	41.9	90.1	213.8	424.3	688.9	924.9	1,147.1	1,351.1
Collaboration Revenue	50.4	43.4	42.9	88.0	80.8	133.8	87.2	40.9	135.0	49.5	94.5	59.9	65.9
Total Revenues	50.4	43.4	42.9	91.2	115.4	299.6	429.8	684.5	1,142.3	1,481.8	1,876.3	2,160.1	2,427.7
Cost of product sales				0.0	0.0	4.2	8.1	19.2	37.4	59.7	78.0	94.2	107.3
COGS as a % of Dalantercept Revenue				0.0	0.0	10%	9%	9%	9%	9%	8%	8%	89
Gross Profit	50.4	43.4	42.9	91.2	115.4	295.4	421.7	665.2	1,104.9	1,422.1	1,798.3	2,065.9	2,320.5
R&D expense	35.1	39.7	57.5	69.0	79.3	87.3	95.1	103.7	113.0	123.2	131.8	138.4	145.3
R&D as a % of revenue	70%	91%	134%	76%	69%	29%	22%	15%	10%	8%	7%	6%	69
SG&A expense	13.4	17.9	19.7	21.7	30.4	34.9	38.8	41.9	44.8	47.7	50.4	51.9	53.4
SG&A as a % of revenue	27%	41%	46%	24%	26%	12%	9%	6%	4%	3%	3%	2%	29
Total operating expenses	48.5	57.6	77.2	90.7	109.7	122.2	133.9	145.6	157.9	170.9	182.2	190.3	198.8
% Margin	96%	133%	180%	99%	95%	41%	31%	21%	14%	12%	10%	9%	8%
Operating income (EBIT)	1.9	(14.2)	(34.3)	0.5	5.7	173.2	287.7	519.6	947.0	1,251.2	1,616.1	1,875.6	2,121.7
Taxes	0.3	(2.1)	(8.6)	0.2	2.0	60.6	86.3	181.9	331.5	437.9	565.6	656.5	742.6
Tax rate	15%	15%	25%	30%	35%	35%	30%	35%	35%	35%	35%	35%	35%
After tax operating income	1.6	(12.0)	(25.7)	0.4	3.7	112.6	201.4	337.8	615.6	813.3	1,050.5	1,219.1	1,379.1
Discount year		0.25	1.25	2.25	3.25	4.25	5.25	6.25	7.25	8.25	9.25	10.25	11.25
Discount factor	1.0	1.1	1.4	1.8	2.4	3.1	4.1	5.3	7.0	9.1	11.9	15.6	20.4
PV	1.6	(11.3)	(18.4)	0.2	1.5	36.1	49.3	63.3	88.3	89.2	88.1	78.3	67.7
Residual value of cash flow	\$ 797.29	1								1	Termina	I Value	263.3
+ Cash and Cash equivalents	84.70												
Company value	\$ 881.99												
- Long-term debt on 12/31/14	-												
Value of equity	\$ 881.99												
Value per share	\$ 31.20												
Fully diluted shares outstanding on 12/31/14	28.26												
Blended discount rate	30.7%												
Longer-term growth rate	5%												

Source: Company reports and JMP Securities LLC

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## **Company Description**

Acceleron Pharma (XLRN) is a Cambridge, MA biotechnology company focused on the discovery, development, and commercialization of its ligand trap fusion proteins directed against components of TGF $\beta$  signaling pathway. These fusion proteins have shown clinical potential in the treatment of anemia disorders related to  $\beta$ -thalassemia and myelodysplastic syndromes, as well as in the treatment of solid cancers, muscle wasting disorders, and other indications impacted by dysregulated TGF $\beta$ .

Since 2008, the company has benefited by robust strategic collaboration with Celgene related to its development lead programs, sotatercept and ACE-536, entitling the company to full reimbursement on both programs and eligibility for up to \$567MM in development, regulatory, and commercial milestones, and a  $\geq$ 20% royalty on worldwide sales, by our estimates. Sotatercept and ACE-536 are currently in Phase II trials for the treatment of  $\beta$ -thalassemia and low/intermediate-1 MDS with pivotal Phase III trials expected to initiate in the first half of 2014.

Dalantercept, the company's wholly owned, clinical-stage fusion protein, is directed against ALK1, a key mediator of tumor angiogenesis that functions independently from the VEGF axis. Dalantercept is currently in Phase II evaluation for the treatment of second-line RCC in combination with TKI therapy.

#### **Investment Risks**

**Clinical.** Drug development is an inherently risky business. Clinical trials always carry a risk of failure and Acceleron's assets (sotatercept, ACE-536, Dalantercept, or future drug candidates) may fail to demonstrate meaningful enough levels of efficacy in current or future clinical trials.

**Regulatory and commercial.** The ability of Acceleron or its partners to market its drugs depends on those drugs obtaining approval from the FDA and foreign regulatory agencies. Failure to achieve approval or delays in the timelines to approval could negatively impact the company's share price.

Competitive. Hereditary anemic disorders represent an increasingly competitive field and Acceleron faces competition from companies with development-stage drug candidates addressing similar biologic mechanisms, and from companies attempting to broaden the applicable indications for products already approved for use. Some of these companies may possess substantially greater R&D and commercial resources than Acceleron or its partners. As such, there is no assurance Acceleron will be competitive or differentiated from other drug products.

**Partners.** Acceleron has formed development and commercial partnerships with Celgene and is highly dependent on these partnerships for non-dilutive sources of capital, and for the potential commercialization of sotatercept and/or ACE-536. Changes to these partnership arrangements could have a substantially negative impact to the company's share price.

**Financial.** Following its IPO, we estimate that Acceleron will end 4Q13 with approximately \$87MM in cash and cash equivalents - adequate resources to fund operations into 2015, according to Acceleron financial guidance. We anticipate that Acceleron is likely to seek additional equity financing in the form of a secondary offering in order to complete the development of its drug candidates, creating dilution risk for existing shareholders.



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JMP Securities was manager or co-manager of a public offering, and received compensation for doing so, for Acceleron Pharma Inc. in the past 12 months.

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Market Outperform (MO): JMP Securities expects the stock price to outperform relevant market indices over the next 12 months.

Market Perform (MP): JMP Securities expects the stock price to perform in line with relevant market indices over the next 12 months.

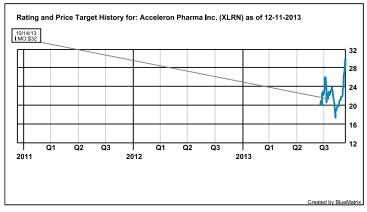
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JMP Securities Research Ratings and Investment Banking Services: (as of December 11, 2013)

							# Co's	
							Receiving	
							IB	
		# Co's	%		# Co's	%	Services in	% of Co's
	Regulatory	Under	of	Regulatory	Under	of	Past 12	With This
JMP Rating	Equivalent	Coverage	Total	Equivalent	Coverage	Total	Months	Rating
MARKET OUTPERFORM	Buy	228	54.94%	Buy	228	54.94%	89	39.04%
MARKET PERFORM	Hold	137	33.01%	Hold	137	33.01%	25	18.25%
MARKET UNDERPERFORM	Sell	5	1.20%	Sell	5	1.20%	0	0%
COVERAGE IN TRANSITION		45	10.84%		45	10.84%	0	0%
TOTAL:		415	100%		415	100%	114	27.47%

### **Stock Price Chart of Rating and Target Price Changes:**

Note: First annotation denotes initiation of coverage or 3 years, whichever is shorter. If no target price is listed, then the target price is N/A. In accordance with NASD Rule 2711, the chart(s) below reflect(s) price range and any changes to the rating or price target as of the end of the most recent calendar guarter. The action reflected in this note is not annotated in the stock price chart. Source: JMP Securities.





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## **Acceleron Pharma Inc. (XLRN)**



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