

Acceleron Pharma Inc. (XLRN)

Highlights from Our Boston Biotech Day

MARKET DATA

Price	\$25.17
52-Week Range:	\$16.78 - \$57.89
Shares Out. (M):	26.5
Market Cap (\$M):	\$667.0
Average Daily Vol. (000):	139.0
Cash (M):	\$204
Cash/Share:	\$6.49
Enterprise Value (M):	\$933
Float (M):	27.0
LT Debt (M):	\$0

Source: Thomson Reuters and JMP Securities LLC

MARKET OUTPERFORM | Price: \$25.17 | Target Price: \$53.00

INVESTMENT HIGHLIGHTS

Brief takeaways following our fireside chat with Acceleron Pharma management; reiterate Market Outperform rating and \$53 price target based on our DCF and SOTP valuations. Acceleron CEO John Knopf and Chief Business Officer Steve Ertel joined us for our Boston Biotech Conference on Friday. Focusing primarily on the beta thal program, key takeaways included an overview of anticipated registration endpoints, guidance regarding data updates at ASH, and brief thoughts regarding reimbursement in the global landscape.

Regarding clinical/registration endpoints:

Transfusion-dependent patients:

- Primary endpoint of reduction in transfusion burden by 25-40%.
- Secondary endpoints likely to include reductions in iron overload (I/O). I/O is a key unmet need, as patients only begin to see reductions with the highest doses of Exjade (iron chelator) that have accompanying tox.
- Reduced organ damage could also serve as a secondary endpoint; however, iron content regarded as a decent proxy. The company is not looking to report on organ damage specifically in ongoing trials, although it is performing liver MRIs in some patients.

Non-transfusion dependent (NTD) patients:

- Primary endpoint of Hgb increases $\geq 1.5\text{g/dL}$
- Potential secondary endpoints of reduced skin/leg ulcers, reductions in iron overload. I/O remains a problem, stemming from defective red blood cell maturation.
- Reductions in spleen enlargements - 80% of NTD patients are splenectomized, 20% with spleens retained, spleen size being monitored in these patients for reductions/slowed enlargement.

Consistency between U.S. and EU regulatory feedback on approvable endpoints regarding reimbursement landscape:

- EU transfusion cost upwards of \$30K per year
- Iron chelators = EU reimbursement at \$40-\$60K per year
- Total beta-thal care reimbursement currently around \$60-\$100K
- Exjade is approved and reimbursed for NTD patients
- Exjade sales were \$900MM WW, 25% in the U.S., implying reimbursement support ex-U.S.

FY DEC		2013A	2014E	2015E
Revenue (\$M)	1Q	\$15.0	\$3.3A	\$5.2
	2Q	\$26.4	\$4.1A	\$5.4
	3Q	\$4.3	\$5.4	\$20.9
	4Q	\$11.5	\$5.9	\$34.8
	FY	\$57.2	\$18.7	\$57.9
EPS	1Q	\$0.12	(\$0.30)A	--
	2Q	\$0.44	(\$0.52)A	--
	3Q	(\$5.62)	(\$0.42)	--
	4Q	(\$0.64)	(\$0.42)	--
	FY	(\$4.15)	(\$1.68)	(\$0.77)

Source: Company reports and JMP Securities LLC

STOCK PRICE PERFORMANCE



Reiterated confidence in luspatercept (ACE-536) being the candidate to be moved forward in beta-thal and MDS over sotatercept. Sotatercept being designated for CKD.

Next luspatercept data to be presented at ASH:

- Beta-thal – full data from 1.0mg/kg cohort, potential early data from 1.25mg/kg cohort.
- MDS data – higher dose levels, longer follow-up from existing dose levels
- MDS development path TBD in collaboration with CELG, in light of Revlimid success in non-del 5q. However, development opportunities seen throughout low/intermediate-2 MDS.
- No updates on sotatercept at ASH.

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 β signaling pathway. These fusion proteins have shown clinical potential in the treatment of anemia disorders related to β -thalassemia and myelodysplastic syndromes, as well as in the treatment of solid cancers, muscle wasting disorders, and other indications impacted by dysregulated TGF β .

Since 2008, the company has benefited from 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 β -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 upon 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 upon 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 on the company's share price.

Financial. Following its IPO, we estimated Acceleron would end 4Q13 with approximately \$87MM in cash and cash equivalents - adequate resources to fund operations into 2015, according to Acceleron's 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 of securities for Accelaron Pharma Inc. (XLRN) in the past 12 months, and received compensation for doing so.

JMP Securities Investment Opinion Definitions:

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.

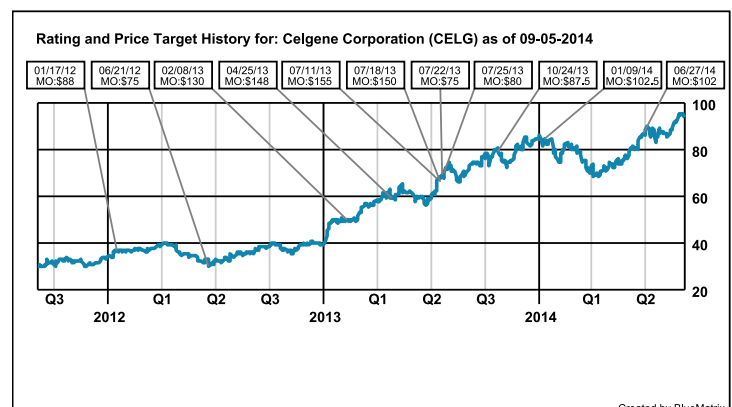
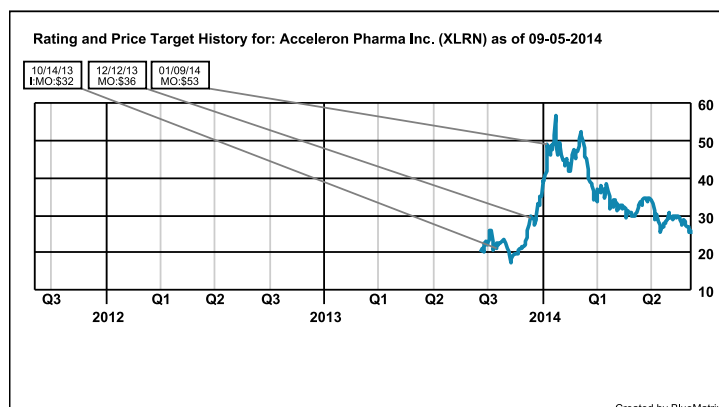
Market Underperform (MU): JMP Securities expects the stock price to underperform relevant market indices over the next 12 months.

JMP Securities Research Ratings and Investment Banking Services: (as of September 8, 2014)

JMP Rating	Regulatory Equivalent	# Co's Under Coverage	% of Total	Regulatory Equivalent	# Co's Under Coverage	% of Total	# Co's Receiving IB Services in Past 12 Months	% of Co's With This Rating
MARKET OUTPERFORM	Buy	273	60.53%	Buy	273	60.53%	105	38.46%
MARKET PERFORM	Hold	138	30.60%	Hold	138	30.60%	19	13.77%
MARKET UNDERPERFORM	Sell	4	0.89%	Sell	4	0.89%	0	0%
COVERAGE IN TRANSITION		36	7.98%		36	7.98%	0	0%
TOTAL:		451	100%		451	100%	124	27.49%

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 quarter. The action reflected in this note is not annotated in the stock price chart. Source: JMP Securities.



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