

# Acceleron Pharma Inc. (XLRN)

Our Takeaways Following the Company's Education Session on MDS

## MARKET DATA

Price	\$24.92
52-Week Range:	\$16.78 - \$57.89
Shares Out. (M):	26.5
Market Cap (\$M):	\$660.4
Average Daily Vol. (000):	248.0
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

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



**MARKET OUTPERFORM** | Price: \$24.92 | Target Price: \$53.00

## INVESTMENT HIGHLIGHTS

**Unmet clinical need and clear clinical activity underscore opportunity for luspatercept in lower risk MDS; reiterate our Market Outperform rating and \$53 price target on Acceleron Pharma based on our DCF and SOTP valuation methodologies.** Today, Acceleron hosted the first in its series of educational webcasts, discussing the treatment landscape in lower risk MDS with invited speaker Dr. Uwe Platzbecker of Universitätsklinikum Carl Gustav Carus in Dresden, Germany. Among the key takeaways: 1) while ESAs will likely continue as the treatment backbone for lower risk MDS, significant need remains among patients underserved by Revlimid and HMA therapy; and 2) the breadth of the luspatercept market opportunity in the EU is further underscored by the lack of formal EU registration with ESAs and reimbursement for red blood cell (RBC) transfusion. Finally, at ~33% response rate in transfusion-dependent disease, luspatercept is seen as confidently meeting the threshold for meaningful benefit.

**Treatment paradigm for MDS seen as likely to evolve using sequential therapy rather than combined regimens.** In Dr. Platzbecker's view, ESAs are likely to continue as the backbone of initial therapy for most patients with lower risk MDS, before proceeding with alternative agents (e.g., Revlimid, HMAs, clinical trials). However, the proportion of ESA non-responders is large, ~60-70% of patients, implying a significant near-frontline opportunity for novel regimens including luspatercept/sotatercept. Better biomarker development was described as a key requisite for upfront therapy selection, including the potential use of combination regimens.

**Appropriate therapy selection for lower risk MDS, including Revlimid, remains complicated by multi-factorial patient segmentation and an absence of clear biomarkers.** As noted during the presentation, while up to 25% of patients with non-del 5q MDS have shown benefit from Revlimid therapy, these responses have lacked any clear correlation with clinical presentation (e.g., transfusion load, cytopenia, blast count, serum EPO levels) or cytogenetics. In our view, this underscores a broad opportunity for luspatercept in lower risk MDS, irrespective of an expanded Revlimid label for non-del 5q, given its activity in both Revlimid naïve and refractory patients. We also note that Dr. Platzbecker's view that all lines of lower-risk MDS, including ESA naïve patients, were seen as open territory with luspatercept given the clinical activity seen to date.

**Clinical data to date with luspatercept seen as meeting the threshold of clear and meaningful activity.** Citing a 5-10% margin of error among historical trials in MDS, Dr. Platzbecker viewed improvement in at least 20% of patients as an appropriate threshold for concluding clinical meaningfulness. Given a ~33% (4 or 12) erythroid response rate among transfusion-dependent patients treated with active doses ( $\geq 0.5\text{mg/kg}$ ), data to date with luspatercept were described as very encouraging. We note that an ongoing

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Phase I MDS study continues to enroll patients in the highest dose cohort of 1.75mg/kg, with the potential to be presented at ASH. A twelve-month Phase I extension study for patients achieving responses is expected to be initiated near term.

**Acceleron continues to represent a compelling opportunity in the biotech space over the course of the next several years, in our opinion.** Our view is drawn from the company's focus and understanding of TGF beta biology, and developmental and commercialization advantages offered through its strategic partnership with Celgene (CELG, MO, \$102 PT).

## 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, and in the treatment of solid cancers, muscle wasting disorders, and other indications impacted by dysregulated TGF $\beta$ .

Since 2008, the company has benefited from robust strategic collaboration with Celgene related to its development lead programs, sotatercept and luspatercept (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 luspatercept are currently in Phase II trials for the treatment of  $\beta$ -thalassemia and low/intermediate-1 MDS.

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, luspatercept, 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 luspatercept. Changes to these partnership arrangements could have a substantially negative impact on the company's share price.

**Financial.** Acceleron finished 2Q14 with \$204MM in cash and cash equivalents, adequate resources to fund operations into 2015. 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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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.

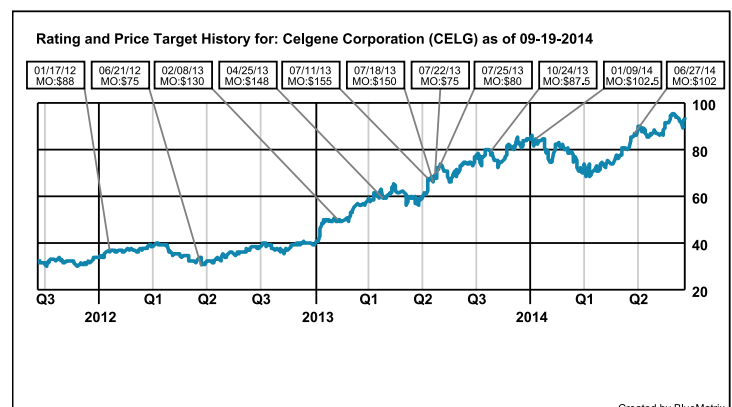
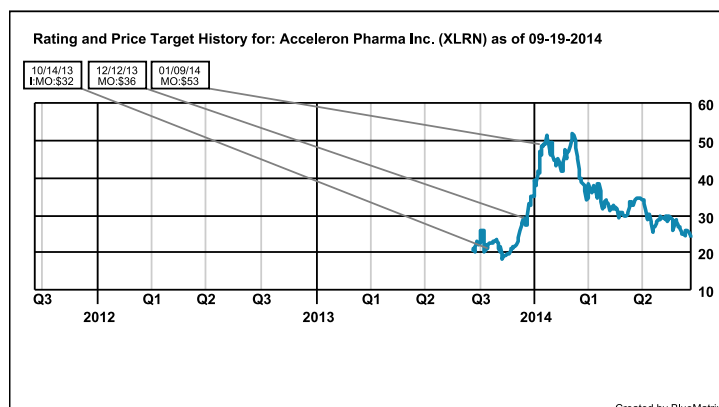
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JMP Rating	Regulatory Equivalent	# Co's Under Coverage	% of Total	Regulatory Equivalent	# Co's Under Coverage	% of Total	# Co's Receiving IB Services in Past 12 Months	% of Co's With This Rating
MARKET OUTPERFORM	Buy	274	60.62%	Buy	274	60.62%	101	36.86%
MARKET PERFORM	Hold	138	30.53%	Hold	138	30.53%	19	13.77%
MARKET UNDERPERFORM	Sell	4	0.88%	Sell	4	0.88%	0	0%
COVERAGE IN TRANSITION		36	7.96%		36	7.96%	0	0%
TOTAL:		452	100%		452	100%	120	26.55%

### 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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