Acceleron Pharma Inc. (XLRN)

Beta-Thal Franchise Updated at EHA

MARKET DATA

Price	$33.88
52-Week Range:	$16.78 - $57.89
Shares Out. (M):	26.5
Market Cap ($M):	$897.8
Average Daily Vol. (000):	383.0
Cash (M):	$214
Cash/Share:	$6.81
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	$5.2	$5.4
	3Q	$4.3	$5.4	$20.9
	4Q	$11.5	$20.9	$34.8
	FY	**$57.2**	**$34.8**	**$42.9**
EPS	1Q	$0.12	($0.30)A	--
	2Q	$0.44	($0.37)	--
	3Q	($5.62)	($0.41)	--
	4Q	($0.64)	$0.05	--
	FY	**($4.15)**	**($1.01)**	**($1.12)**

Source: Company reports and JMP Securities LLC

STOCK PRICE PERFORMANCE

MARKET OUTPERFORM | Price: $33.88 | Target Price: $53.00

INVESTMENT HIGHLIGHTS

We reiterate our Market Outperform rating and $53 price target on Acceleron Pharma following updated presentations of Sotatercept and ACE-536 activity in Beta-thalassemia at the European Hematology Association (EHA) conference in Milan. Newly presented data included treatment effects from an additional dose cohort in each study, longer follow-up from previously described patients, and debut results from transfusion-dependent patients, where substantial reductions in transfusions were seen in a small sample size. In our view, there are several key takeaways: 1) both sotatercept and ACE-536 continue to show dose-dependent increases in hemoglobin (Hb) in non-transfusion dependent patients; 2) maximum effective doses have yet to be achieved, implying the potential for additional benefit from further dose escalation; 3) both agents appear to benefit transfusion-dependent patients, despite small patient numbers to date as evidenced by reductions on transfusion burden and serum ferritin levels; and 4) exemplary safety profiles are maintained, particularly when baseline comorbidities are considered.

With all signs continuing to point to the positive, we remain encouraged by the sotatercept/ACE-536 prospects in beta-thal. We would view added clarity around sotatercept/ACE-536's impact on secondary endpoint in non-transfusion dependent patients (e.g., reduced serum ferritin levels, iron and bone metabolism, reduced rate of iron chelation therapy) as measures for increased confidence ahead of the next data from additional dose cohorts. Our $53 price target is derived through our DCF and SOTP valuation methodologies.

Key highlights from the Sotatercept beta-thalassemia update. Compared to the presentation at ASH, the major update was comprised of data from seven patients (four non-transfusion dependent (NTD)) patients treated with sotatercept at the latest dose cohort of 0.75mpk Q3W (see Figure 2 for patient baseline characteristics). Among NTD patients in this cohort, all achieved maximum Hgb increases ≥1g/dL while 50% achieved Hgb increases ≥2g/dL, compared to 83% and 33% of patients, respectively, at the next highest dose of 0.5mpk (Figure 3). Mean duration of effect at 0.75mpk remains immature given the brevity of follow-up; however, the consistency of mean Hgb increases at 0.5 and 0.3mpk (Figure 4) would suggest the ability to readily sustain 1.5-2.5g/dL Hgb increases over time. Furthermore, an analysis of total drug exposure and Hgb change suggests that further gains (up to 3.8g/dL) may be had from continued dose escalation (Figure 5) and time on therapy. Among transfusion-dependent patients, reductions in transfusion burden were proportional to dose with two-thirds of patients achieving ≥20% reductions in transfusion burden (one achieving ≥50% burden reduction) at 0.75mpk. Of note, no new safety signals were observed in either the new 0.75mpk dose cohort

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or in the previous cohorts with additional follow-up (one incident of Grade 2 bone pain, phlebitis, and Grade 3 ventricular extrasystoles having been previously reported and largely attributed to patient history.

ACE-536 data are similarly impressive despite the earlier stage of maturity compared to sotatercept. Data cut-off for the ACE-536 update was April 28. Among NTD patients, ACE-536 appears to largely maintain its dose-dependent relationship with increasing hemoglobin levels. However, while it may at this point be difficult to determine the incremental effect on Hgb via dose escalation from 0.6 to 0.8mpk given the small size of the 0.8mpk cohort (three patients, with one patient suspending therapy after cycle 3 for undisclosed reasons; Figure 9), we are particularly encouraged by the data among the transfusion-dependent patients, who achieved ≥65% reductions in transfusion burden for a four-cycle treatment period, along with significant reductions in serum ferritin levels (a surrogate marker for free blood iron levels and risk factor for iron overload). We believe that ultimately, ACE-536 will continue to show the same linear dose response as has been seen with sotatercept.

Next steps. As a result of today's dataset, we maintain our expectation that XLRN, along with partner Celgene (CELG, MO, $205 PT), will commence Phase III clinical trials of either or both of these sister molecules.

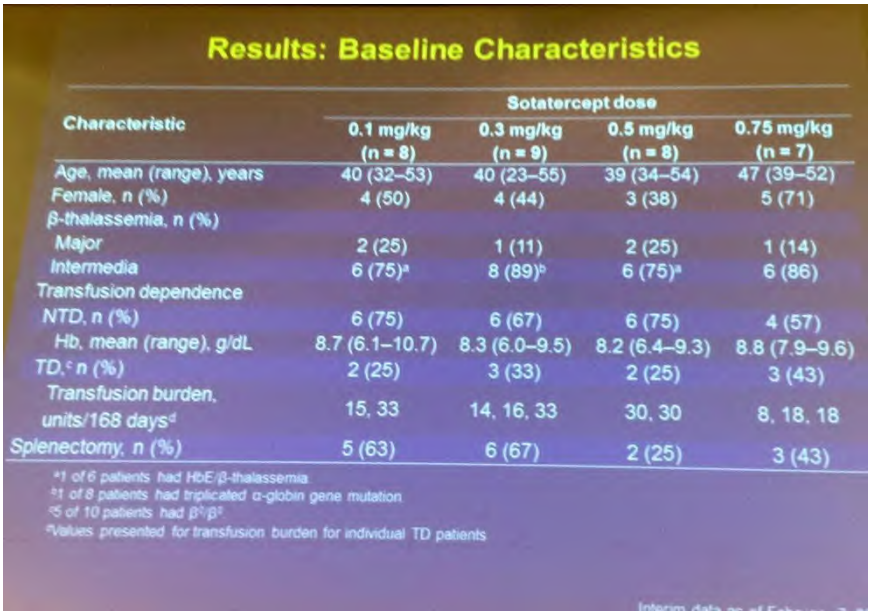
We believe Acceleron represents a compelling opportunity in the biotech space over the course of the next several years. Our view is drawn from the company's focus and understanding of TGF beta biology, as well as developmental and commercialization advantages offered through its strategic partnership with Celgene.

FIGURE 1. Upcoming Milestones

Timing	Drug	Milestones
2Q14	Dalantercept	Initiation of Phase IIa study in HCC in combination with Nexavar
3Q14	Dalantercept	Initiation of Phase II trial plus Avastin in GBM
4Q14	Sotatercept & ACE-536	Final results from Phase II trials in β-thalassemia and MDS
4Q14/1Q15	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

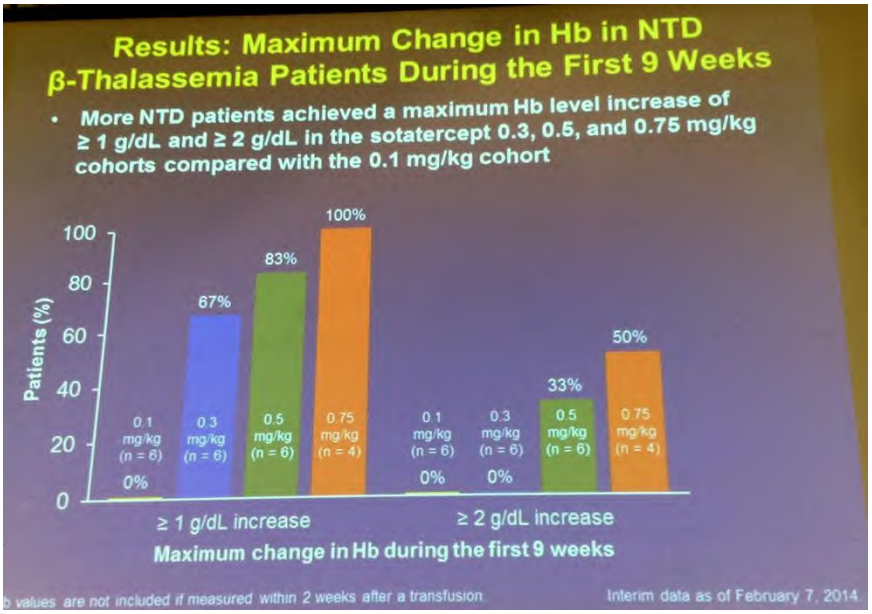
Source: Company Reports

FIGURE 2. Sotatercept Phase II Baseline Characteristics



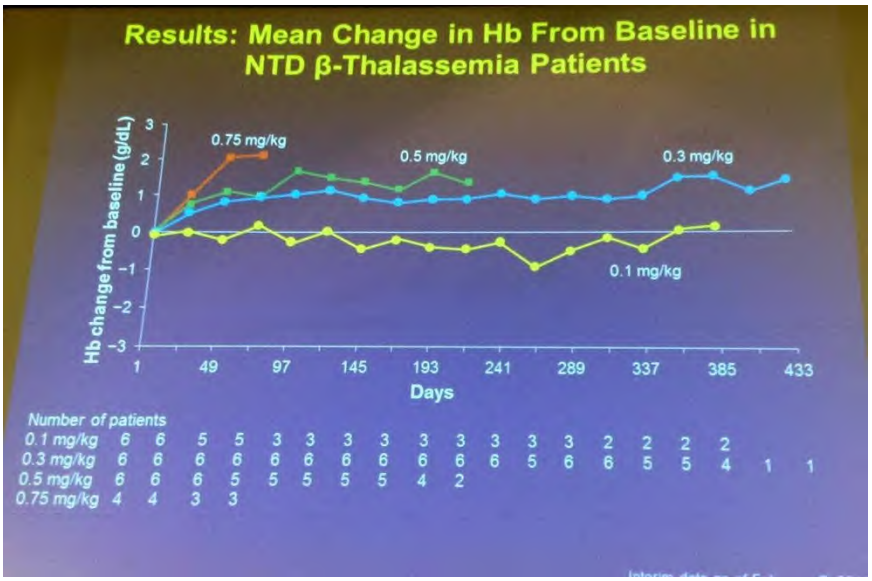
Source: EHA 2014

FIGURE 3. Max Change in Hb Levels in NTD Patients on Sotatercept



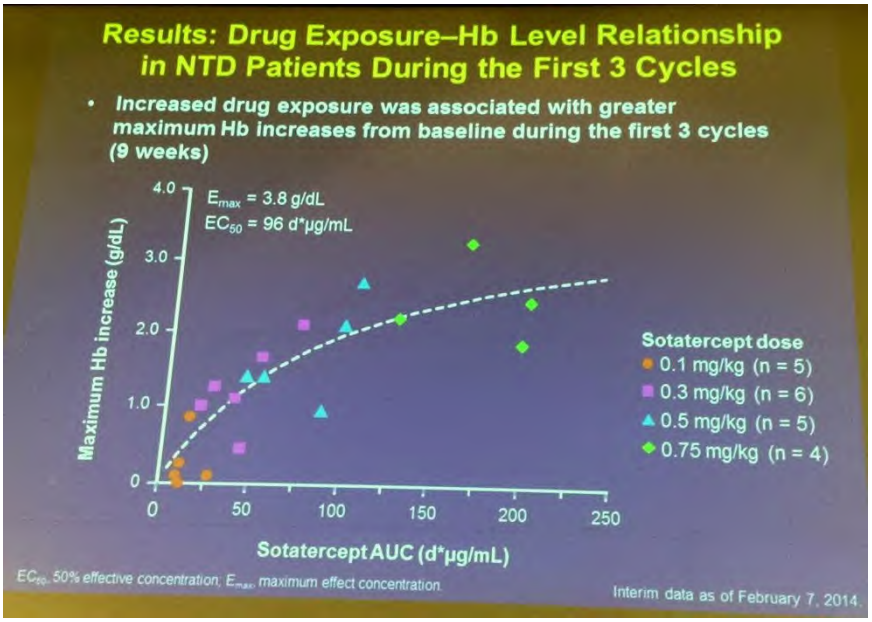
Source: EHA 2014

FIGURE 4. Mean Change in Hb versus Baseline in NTD Patients



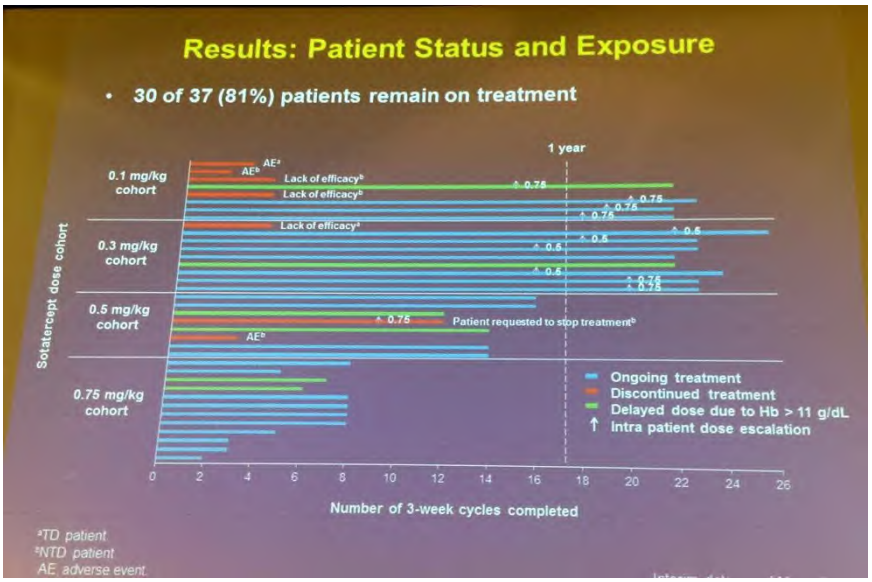
Source: EHA 2014

FIGURE 5. Relationship Between Drug Exposure and Hb Impact in Non-Transfusion Dependent Patients



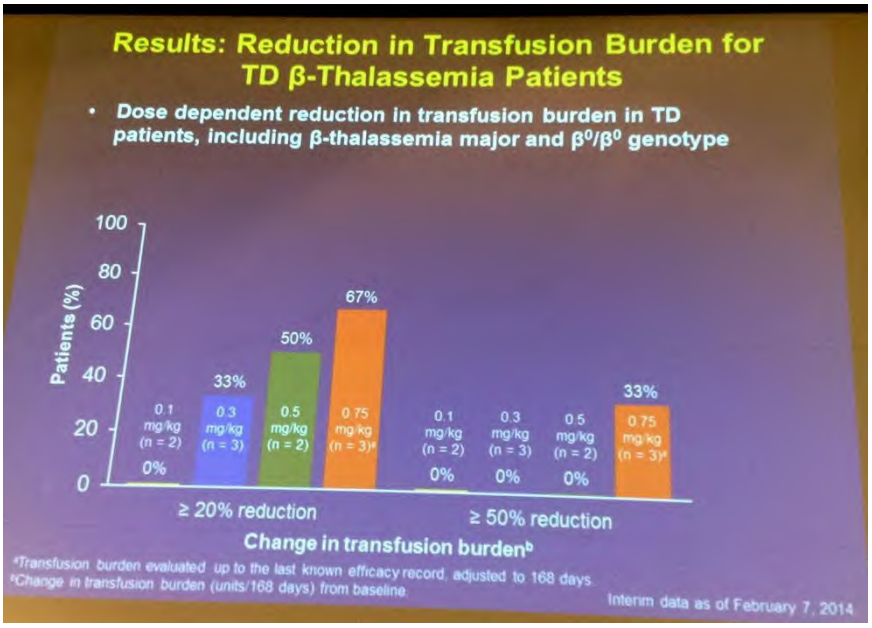
Source: EHA 2014

FIGURE 6. Patient Outcome and Duration on Therapy with Sotatercept



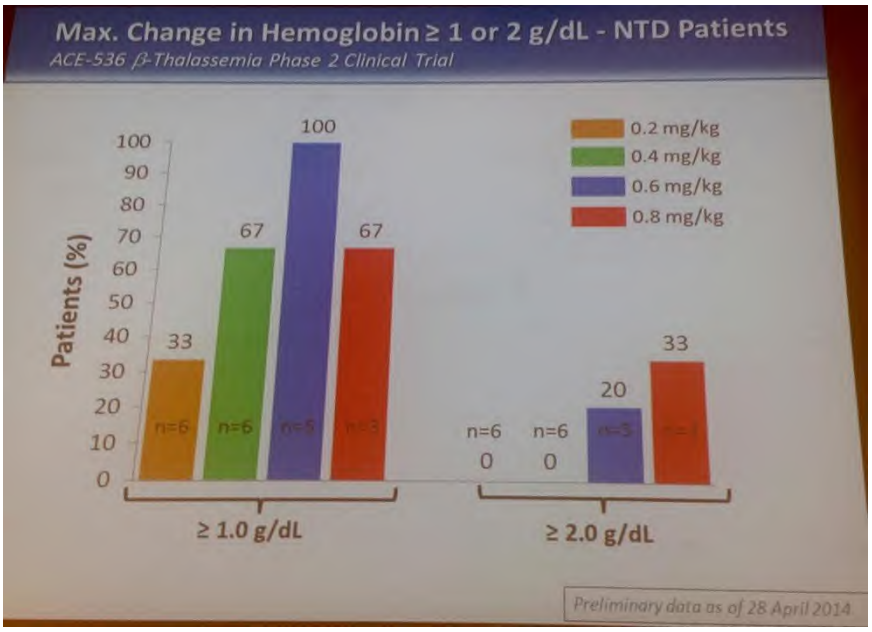
Source: EHA 2014

FIGURE 7. Reduction in Transfusion Burden in TD Patients on Sotatercept



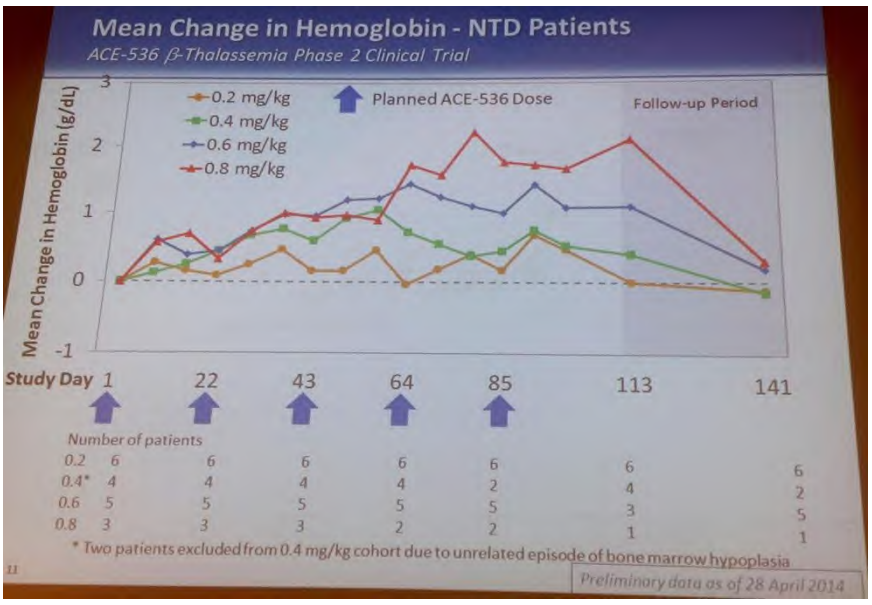
Source: EHA 2014

FIGURE 8. Hb Change with ACE-536



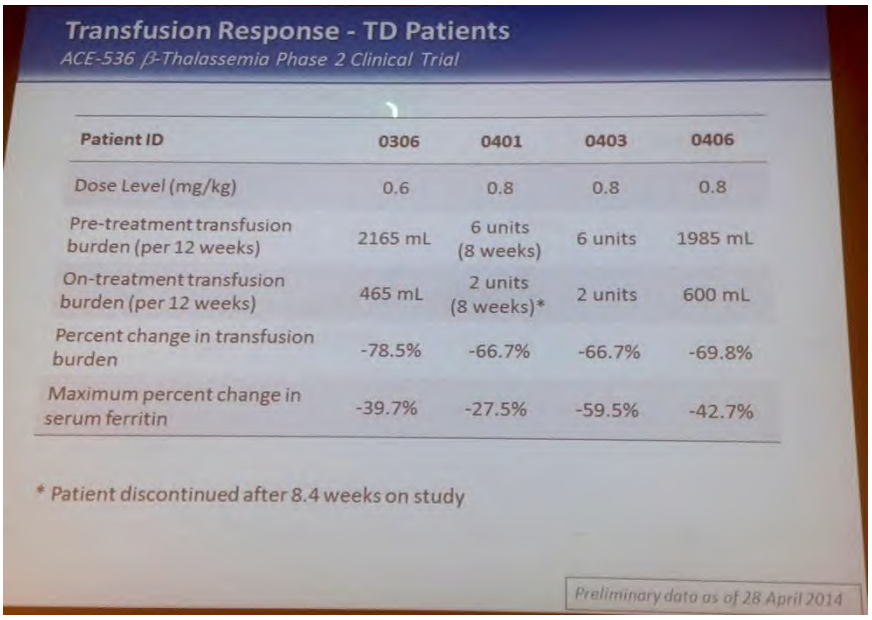
Source: EHA 2014

FIGURE 9. Mean Change in Hb Levels with ACE-536



Source: EHA 2014

FIGURE 10. ACE-536 Outcomes in Transfusion-Dependent Patients



Transfusion Response - TD Patients

ACE-536 β-Thalassemia Phase 2 Clinical Trial

Patient ID	0306	0401	0403	0406
Dose Level (mg/kg)	0.6	0.8	0.8	0.8
Pre-treatment transfusion burden (per 12 weeks)	2165 mL	6 units (8 weeks)	6 units	1985 mL
On-treatment transfusion burden (per 12 weeks)	465 mL	2 units (8 weeks)*	2 units	600 mL
Percent change in transfusion burden	-78.5%	-66.7%	-66.7%	-69.8%
Maximum percent change in serum ferritin	-39.7%	-27.5%	-59.5%	-42.7%

* Patient discontinued after 8.4 weeks on study

Preliminary data as of 28 April 2014

Source: EHA 2014

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 TGFβ 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 TGFβ.

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 ≥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 Acceleron Pharma Inc. (XLRN) in the past 12 months, and received compensation for doing so.

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

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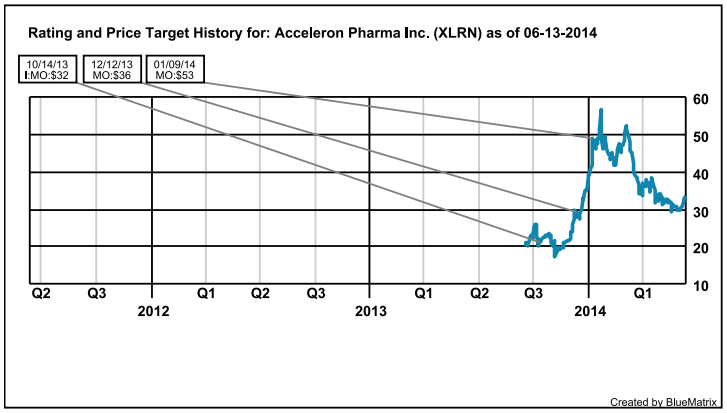
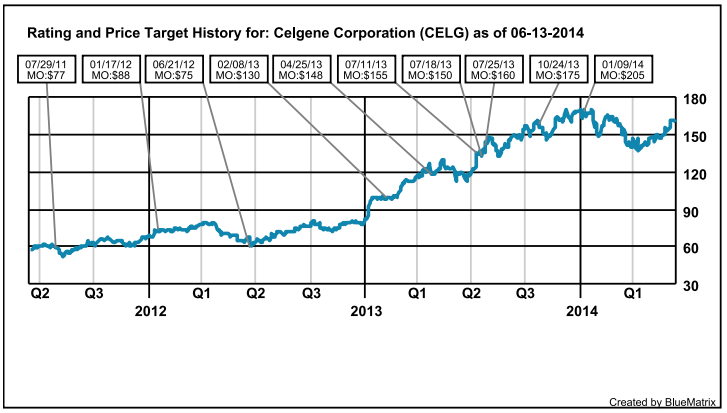
JMP Rating	Regulatory Equivalent	# Co's Under Coverage	*% of Total*	Regulatory Equivalent	# Co's Under Coverage	*% of Total*	# Co's Receiving IB Services in Past 12 Months	*% of Co's With This Rating*
MARKET OUTPERFORM	Buy	261	*58.92%*	Buy	261	*58.92%*	98	*37.55%*
MARKET PERFORM	Hold	135	*30.47%*	Hold	135	*30.47%*	19	*14.07%*
MARKET UNDERPERFORM	Sell	4	*0.90%*	Sell	4	*0.90%*	0	*0%*
COVERAGE IN TRANSITION		43	*9.71%*		43	*9.71%*	0	*0%*
TOTAL:		443	*100%*		443	*100%*	117	*26.41%*

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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