Acceleron Pharma Inc. (XLRN) — Overweight

Phase III Beta-Thal Trial Initiation in 2015, Reiterate Overweight

CONCLUSION

Acceleron ended 2Q:14 with cash of $204.3 million. Acceleron and partner Celgene are evaluating sotatercept and luspatercept (ACE-536) in both beta-thalassemia and myelodysplastic syndromes (MDS) with additional data expected at the American Society of Hematology (ASH) meeting in December. The partners will select one of these drugs for the Phase III program to begin in 2015. The partners are also conducting a Phase II study of sotatercept in ESRD with data at the American Society of Nephrology (ASN) meeting in November. After positive data for wholly owned dalantercept in renal cell carcinoma (RCC), we expect to see Phase II expansion data. Acceleron will file an IND for ACE-083 for muscle wasting disorders this year. We reiterate our Overweight rating and price target of $65.

- **Phase III Trial Initiations Expected In 2015.** Acceleron recently presented data from beta-thalassemia trials of sotatercept and luspatercept showing increased erythropoiesis and reduction in transfusion burden. Furthermore, the administration of both therapies seem to lead to improvements in many of the co-morbidities associated with the disease. Acceleron and partner Celgene will soon determine which dosing regimen will be used in the expansion cohorts for both drugs to begin in the fall. The partners will report additional data on luspatercept or sotatercept at ASH in December before determining which drug to advance into pivotal trials. We expect the Phase III program will comprise 2 studies; one for transfusion dependent and the other for transfusion independent patients.

- **First Luspatercept MDS Data Promising.** This Phase II trial in low-to-intermediate-1 risk MDS risk patients showed dose dependent increases in hemoglobin levels in patients with low transfusion burden. In the MDS population however, most patients in fact have high transfusion burden. In the trial, a third of patients with high transfusion burden who were treated with 0.5mg/kg doses or higher either became transfusion free or had a reduction in burden by at least 40%. In this population, the mortality of patients is directly correlated with transfusion burden and anemia. The company is also examining sotatercept in following treatment with either Revlimid or Vidaza in more severe MDS patients. We expect to see data from this study presented at ASH in December.

RISKS TO ACHIEVEMENT OF PRICE TARGET

Sotatercept, ACE-536 and/or dalantercept may fail in the clinic or to gain regulatory approval. The Celgene partnership may falter. Acceleron may require additional capital or could face future unforeseen litigation.

COMPANY DESCRIPTION

Acceleron is developing novel drugs for hematology and cancer.

PRICE: US$28.43
TARGET: US$65.00
Proj. EV of $1.9B + YE:14E net cash

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Changes	Previous	Current
Rating	—	Overweight
Price Tgt	—	US$65.00
FY14E Rev (mil)	US$22.5	US$23.6
FY15E Rev (mil)	—	US$20.4
FY14E EPS	US$(1.47)	US$(1.41)
FY15E EPS	US$(1.98)	US$(1.75)

52-Week High / Low	US$57.89 / US$15.00
Shares Out (mil)	31.6
Market Cap. (mil)	US$898.4
Avg Daily Vol (000)	275
Book Value/Share	US$5.38
Net Cash Per Share	US$6.47
Debt to Total Capital	0%
Div (ann)	NA
Fiscal Year End	Dec

Price Performance - 1 Year

Source: Bloomberg

YEAR	REVENUE (US$ m)						EARNINGS PER SHARE (US$)					
	Mar	Jun	Sep	Dec	FY	FY RM	Mar	Jun	Sep	Dec	FY	FY P/E
2013A	15.0	26.4	4.3	11.3	57.0	15.8x	0.08	0.62	(0.86)	(0.65)	(0.97)	NM
2014E	3.3A	4.1A	3.1	13.1	23.6	38.1x	(0.30)A	(0.52)A	(0.42)	(0.16)	(1.41)	NM
2015E	2.6	12.6	2.6	2.6	20.4	44.0x	(0.47)	(0.19)	(0.53)	(0.55)	(1.75)	NM

Additional Detail

- **Sotatercept Active in ESRD.** In a recent Phase IIa study, Sotatercept showed benefit in end stage renal disease (ESRD). In the study, patients single 0.3mg/kg and 0.5mg/kg doses of sotatercept showed mean peak Hb increases of 0.5g/dL and 0.8g/dL versus 0.1g/dL for placebo at 28 days. In addition, 3/8 (37%) of the 0.3mg/kg and 2/5 (40%) of the 0.5mg/kg patients achieved Hb increases of >1.0g/dL versus only 1/5 (20%) placebo patients. Sotatercept was well tolerated with only mild-to-moderate AEs unrelated to the therapy. The partners will report additional Phase IIa data at the American Society of Nephrology (ASN) meeting in November. Celgene has initiated a Phase IIb ESRD study with a 60-patient dose-escalation and 230-patient randomized control study versus ESA.

- **Dalantercept Progressing.** Acceleron recently reported positive interim data from the dose escalation portion of a Phase II trial examining dalantercept with axtinib (INLYTA) in renal cell carcinoma (RCC) at ASCO in June. Based on this data, the company has initiated the randomized, placebo-controlled portion of the study that will look at dalantercept + axtinib versus axtinib alone in ~130 patients. Progression free survival (PFS) is the primary endpoint of the trial. The company has just recently initiated the Phase Ib portion of a trial that looks at dalantercept + Nexavar (sorafenib) in hepatocellular carcinoma (HCC).

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Acceleron Pharma Inc.
Quarterly Earnings Estimates
($ in thousands, except per share data)

8/12/2014

	1QA	2QA	3QA	4QA	2013E	1QA	2QE	3QE	4QE	2014E	1QE	2QE	3QE	4QE	2015E
Collaboration Revenue:															
License and milestone	$12,515	$22,891	$638	$7,638	$43,682	$0	$0	$100	$10,100	$10,200	$100	$10,100	$100	$100	$10,400
Cost-sharing, net	2,497	3,537	3,632	3,632	13,298	3,307	4,078	3,000	3,000	13,385	2,500	2,500	2,500	2,500	10,000
Total Revenues	**$15,012**	**$26,428**	**$4,270**	**$11,270**	**$56,980**	**$3,307**	**$4,078**	**$3,100**	**$13,100**	**$23,585**	**$2,600**	**$12,600**	**$2,600**	**$2,600**	**$20,400**
Operating Expenses:															
Research and Development	8,780	8,911	8,143	10,216	36,050	11,765	12,677	13,000	14,000	51,442	13,500	14,500	15,500	16,500	60,000
General and Administrative	3,096	3,365	3,011	4,756	14,228	3,750	8,712	4,250	5,000	21,712	5,000	5,000	5,000	5,000	20,000
Total Operating Expenses	**$11,876**	**$12,276**	**$11,154**	**$14,972**	**$50,278**	**$15,515**	**$21,389**	**$17,250**	**$19,000**	**$73,154**	**$18,500**	**$19,500**	**$20,500**	**$21,500**	**$80,000**
Operating Income/(Loss)	**$3,136**	**$14,152**	**($6,884)**	**($3,702)**	**$6,702**	**($12,208)**	**($17,311)**	**($14,150)**	**($5,900)**	**($49,569)**	**($15,900)**	**($6,900)**	**($17,900)**	**($18,900)**	**($59,600)**
Operating Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Total Other Income/(Expense)[1]	(1,489)	(1,074)	(11,629)	(14,659)	(28,851)	3,088	761	725	700	5,274	675	650	625	600	2,550
Pretax Income/(Loss)	**$1,647**	**$13,078**	**($18,513)**	**($18,361)**	**($22,149)**	**($9,120)**	**($16,550)**	**($13,425)**	**($5,200)**	**($44,295)**	**($15,225)**	**($6,250)**	**($17,275)**	**($18,300)**	**($57,050)**
Pretax Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Income Tax	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0
Tax Rate	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%
Net Income/(Loss)	**$1,647**	**$13,078**	**($18,513)**	**($18,361)**	**($22,149)**	**($9,120)**	**($16,550)**	**($13,425)**	**($5,200)**	**($44,295)**	**($15,225)**	**($6,250)**	**($17,275)**	**($18,300)**	**($57,050)**
Net Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Net Income/(Loss) per Share	**$0.08**	**$0.62**	**($0.86)**	**($0.65)**	**($0.97)**	**($0.30)**	**($0.52)**	**($0.42)**	**($0.16)**	**($1.41)**	**($0.47)**	**($0.19)**	**($0.53)**	**($0.55)**	**($1.75)**
Basic Shares Outstanding	20,954	20,954	21,500	28,123	22,883	30,321	31,552	31,750	32,000	31,406	32,250	32,500	32,750	33,000	32,625

Source: Company reports and Piper Jaffray & Co. analysis.
Note: Acceleron competed its IPO on September 18, 2013 and has not yet provided fully quarterly results for 2012.
1. 2012, 1Q:13 and 2Q:13 Total Other Income/(Expense) line incl. extinguishment of convertible preferred stock and change in fair value of warrants.

Current disclosure information for this company can be found at http://www.piperjaffray.com/researchdisclosures.

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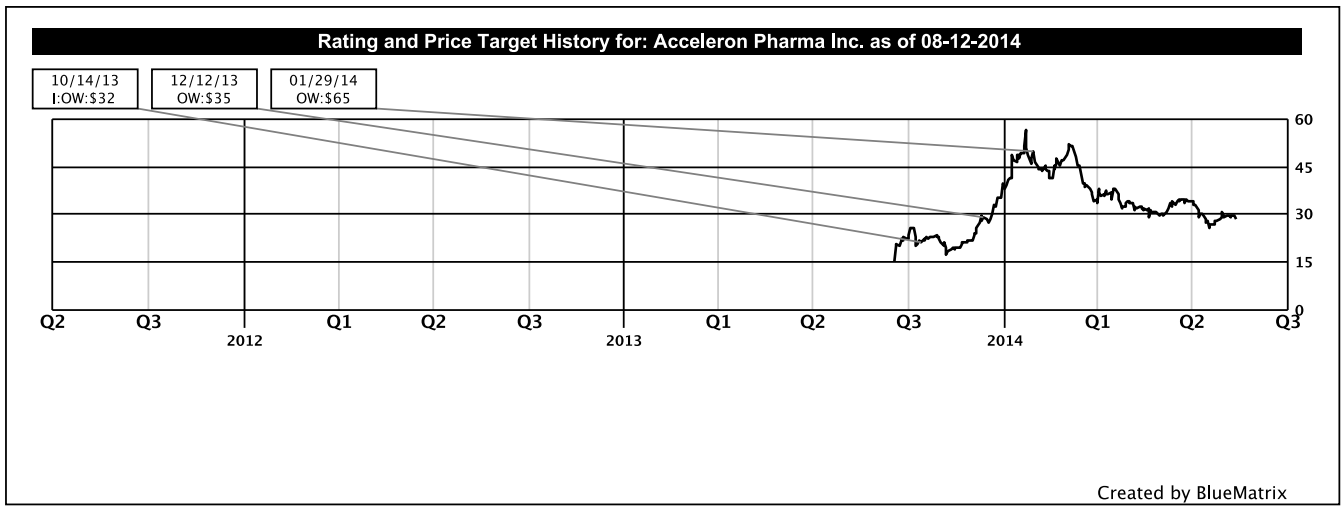

Notes: The boxes on the Rating and Price Target History chart above indicate the date of the Research Note, the rating, and the price target. Each box represents a date on which an analyst made a change to a rating or price target, except for the first box, which may only represent the first Note written during the past three years.

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R: Resuming Coverage
T: Transferring Coverage
D: Discontinuing Coverage
S: Suspending Coverage
OW: Overweight
N: Neutral
UW: Underweight
NA: Not Available
UR: Under Review

Distribution of Ratings/IB Services
Piper Jaffray

			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OW]	363	61.73	94	25.90
HOLD [N]	214	36.39	23	10.75
SELL [UW]	11	1.87	0	0.00

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