

Accelaron Pharma Inc. (XLRN)

Overweight

Sotatercept Active in ESRD Anemia, Reiterate Overweight

CONCLUSION

Accelaron and partner Celgene presented encouraging interim Phase IIa data on sotatercept for the treatment of anemia in end-stage renal disease (ESRD) patients at the National Kidney Foundation Spring Meeting in Las Vegas. Sotatercept demonstrated dose-dependant mean peak hemoglobin (Hb) increases and continues to enroll patients at higher doses. Celgene has initiated a larger two-part 290-patient Phase II ESRD study. XLRN shares are trading down today (in a red tape) over concerns about 3 patient discontinuations, although we believe this is unfounded considering 2 were protocol violations. We reiterate our Overweight rating and price target of \$65.

- Sotatercept Active in ESRD.** In the Phase IIa study, 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 patient. Further, only one 0.3mg/kg patient (13%) and no 0.5mg/kg (0%) required rescue EPO therapy versus 2/5 (40%) on placebo. Sotatercept was well tolerated with only mild-to-moderate AEs unrelated to the therapy. The trial is still enrolling patients at 0.7mg/kg every 28-day dose and will explore a 4th cohort with a 0.7mg/kg loading dose followed by 0.4mg/kg every 2 weeks. Celgene has already initiated a Phase II ESRD study with a 60-patient dose-escalation and 230-patient randomized control study versus ESA.
- So Why ARE XLRN Shares Down Today?** We believe concern over sotatercept patient discontinuations are unfounded considering 2 were due to protocol violations (1 prior EPO therapy and 1 high blood pressure) and one patient required EPO rescue therapy. In addition, the "apparent" high placebo response (20% >1.0g/dL) was really just one patient. Lastly, the differences in baseline Hemoglobin measures is raising some questions regarding the comparability between the placebo and the experimental arms. Despite these questions, we argue the dose-dependent activity for sotatercept supports higher and more frequent dosing in ESRD patients.
- Potential Blockbuster Anemia Drugs.** Beyond ESRD, Sotatercept is also currently in Phase II trials for Beta-thalassemia and MDS with updated data expected at the European Hematology Association (EHA) meeting in June. We anticipate Celgene will begin a Phase III trial in Beta-thalassemia in late 2014 or early 2015. Celgene is responsible for all costs of these drugs going forward with Accelaron retaining co-promote rights in North America and low-to-mid 20% royalties.

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. Accelaron may require additional capital or could face future unforeseen litigation.

COMPANY DESCRIPTION

Accelaron is developing novel drugs for hematology and cancer.

YEAR	REVENUE (m)						EARNINGS PER SHARE ()					
	Mar	Jun	Sep	Dec	FY	FY RM	Mar	Jun	Sep	Dec	FY	FY P/E
2013A	15.0	26.4	4.3	11.5	57.2	18.2x	0.08	0.62	(0.86)	(0.64)	(0.96)	NM
2014E	3.6	3.6	3.1	13.1	23.5	44.2x	(0.41)	(0.45)	(0.50)	(0.22)	(1.58)	NM
2015E	—	—	—	—	20.4	50.9x	—	—	—	—	(2.01)	NM

PRICE: US\$33.42

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)	—	23.5
FY15E Rev (mil)	—	20.4
FY14E EPS	—	(1.58)
FY15E EPS	—	(2.01)
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52-Week High / Low	US\$57.89 / US\$15.00	
Shares Out (mil)	31.1	
<i>Incl. impact of recent 2.76M share offering</i>		
Market Cap. (mil)	US\$1,039.4	
Avg Daily Vol (ooo)	307	
Book Value/Share	US\$6.01	
Net Cash Per Share	US\$7.24	
<i>Pro forma cash incl. recent offering less notes payable</i>		
Debt to Total Capital	7%	
<i>\$16.9M in notes payable</i>		
Div (ann)	NA	
Fiscal Year End	Dec	

Price Performance - 1 Year



Source: Bloomberg

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

2/26/2014

	<u>2012A</u>	<u>1QA</u>	<u>2QA</u>	<u>3QA</u>	<u>4QA</u>	<u>2013A</u>	<u>1QE</u>	<u>2QE</u>	<u>3QE</u>	<u>4QE</u>	<u>2014E</u>
Total Revenues	\$15,254	\$15,012	\$26,428	\$4,270	\$11,521	\$57,231	\$3,638	\$3,638	\$3,100	\$13,100	\$23,476
Operating Expenses:											
Research and Development	35,319	8,780	8,911	8,143	10,216	36,050	11,000	12,000	13,000	14,000	50,000
General and Administrative	8,824	3,096	3,365	3,011	4,756	14,228	4,000	4,500	4,500	5,000	18,000
Total Operating Expenses	\$44,143	\$11,876	\$12,276	\$11,154	\$14,972	\$50,278	\$15,000	\$16,500	\$17,500	\$19,000	\$68,000
Operating Income/(Loss)	(\$28,889)	\$3,136	\$14,152	(\$6,884)	(\$3,451)	\$6,953	(\$11,362)	(\$12,862)	(\$14,400)	(\$5,900)	(\$44,524)
Operating Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Total Other Income/(Expense)	(3,693)	(1,489)	(1,074)	(11,629)	(14,659)	(28,851)	(1,000)	(1,050)	(1,100)	(1,150)	(4,300)
Pretax Income/(Loss)	(\$32,582)	\$1,647	\$13,078	(\$18,513)	(\$18,110)	(\$21,898)	(\$12,362)	(\$13,912)	(\$15,500)	(\$7,050)	(\$48,824)
Pretax Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Income Tax	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%
Net Income/(Loss)	(\$32,582)	\$1,647	\$13,078	(\$18,513)	(\$18,110)	(\$21,898)	(\$12,362)	(\$13,912)	(\$15,500)	(\$7,050)	(\$48,824)
Net Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Other Items	0	0	0	0	0	0	0	0	0	0	0
Net income applicable to common shareholders¹	(\$32,582)	\$1,647	\$13,078	(\$18,513)	(\$18,110)	(\$21,898)	(\$12,362)	(\$13,912)	(\$15,500)	(\$7,050)	(\$48,824)
Net Income/(Loss) per Share	(\$1.55)	\$0.08	\$0.62	(\$0.86)	(\$0.64)	(\$0.96)	(\$0.41)	(\$0.45)	(\$0.50)	(\$0.22)	(\$1.58)
Basic Shares Outstanding ²	21,062	20,954	20,954	21,500	28,123	22,898	30,000	31,000	31,250	31,500	30,938

Source: Company reports and Piper Jaffray & Co. analysis.

Note: Acceleron completed its IPO on September 18, 2013 and a follow-on offering on January 22, 2014

1. Net income applicable to common shareholders for 2012A, 1Q:13A, 2Q:13A, 3Q:13A and 2013A assumes full conversion of convertible preferred shares.

2. Shares Outstanding for 2012A, 1Q:13A, 2Q:13A, 3Q:13A and 2013A are Piper Jaffray estimates that assume conversion of convertible preferred shares.

Current disclosure information for this company can be found at

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Legend:

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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				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OW]	357	61.03	86	24.09
HOLD [N]	208	35.56	20	9.62
SELL [UW]	20	3.42	0	0.00

Note: Distribution of Ratings/IB Services shows the number of companies currently in each rating category from which Piper Jaffray and its affiliates received compensation for investment banking services within the past 12 months. FINRA rules require disclosure of which ratings most closely correspond with "buy," "hold," and "sell" recommendations. Piper Jaffray ratings are not the equivalent of buy, hold or sell, but instead represent recommended relative weightings. Nevertheless, Overweight corresponds most closely with buy, Neutral with hold and Underweight with sell. See Stock Rating definitions below.

Analyst Certification — Edward A. Tenthoff, Sr Research Analyst — David N. Lebowitz, CFA, Research Analyst

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- **Neutral (N):** Anticipated to perform in line relative to the median of the group of stocks covered by the analyst.
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