

Accelaron Pharma Inc. (XLRN)

Overweight

Preclinical Studies Published Elucidating Mechanism of Anemia Pipeline

CONCLUSION

Two new articles in Nature Medicine were published on sotatercept and ACH-536 discussing preclinical studies demonstrating mechanism of erythropoiesis independent of EPO. The publications follow the recent announcement of positive data for sotatercept in anemia in end-stage renal disease (ESRD) patients who are on dialysis, which will be presented at the National Kidney Foundation Spring Clinical meeting being held April 22-26 in Las Vegas. We also await Phase II data on sotatercept in MDS and Beta-thalassemia at the EHA meeting in June. We reiterate our Overweight rating and price target of \$65.

- **Sotatercept and ACH-536 Stimulate RBC Growth.** Two new articles published in Nature Medicine on sotatercept and ACH-536 detail preclinical studies that identify the respective mechanisms for stimulation of red blood cell growth independent EPO. Both compounds function at later stage of the erythropoiesis process than EPO, allowing the drug to have efficacy in EPO failure patients as well as potentially being used in combination with or in lieu of EPO for a variety of disorders including MDS and Beta-thalassemia.
- **ESRD Sotatercept Data Shows Hemoglobin Benefit.** Celgene announced preliminary Phase II data on sotatercept in ESRD in dialysis patients in a poster presentation at the upcoming National Kidney Foundation Spring Clinical meeting on April 23rd. Single doses of 0.3 and 0.5mg/kg sotatercept showed mean hemoglobin change of 0.5 and 0.9g/dL versus 0.1g/dL for placebo at 28-days. Sotatercept was well tolerated, with only mild-to-moderate AEs that were unrelated to the therapy.
- **Potential Blockbuster Anemia Drugs.** Celgene has already initiated a larger two-part Phase II ESRD study that includes a 60-patient dose-escalation portion and 230-patient randomized control study versus ESA. Sotatercept is also currently in Phase II trials for Beta-thalassemia and myelodysplastic syndromes (MDS) with updated data expected at the European Hematology Association (EHA) meeting in June.
- **Dalantcept Data at ASCO.** Accelaron is conducting a Phase II trial of dalantcept in combination with axitinib in renal cell carcinoma (RCC). We expect to see preliminary data from this trial in addition to Phase II head and neck cancer data at the American Society of Oncology (ASCO) meeting in June.

RISKS TO ACHIEVEMENT OF PRICE TARGET

Sotatercept, ACE-536 and/or dalantcept 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.

PRICE: US\$39.01

TARGET: US\$65.00

Proj. EV of \$1.9B + YE14E net cash

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| Changes | Previous | Current |
|---|-----------------------|------------|
| Rating | — | Overweight |
| Price Tgt | — | US\$65.00 |
| FY13A Rev (mil) | — | 57.2 |
| FY14E Rev (mil) | — | 23.5 |
| FY13A EPS | — | (0.96) |
| FY14E EPS | — | (1.58) |
| <hr/> | | |
| 52-Week High / Low | US\$57.89 / US\$15.00 | |
| Shares Out (mil) | 31.0 | |
| <i>Incl. impact of recent 2.76M share offering</i> | | |
| Market Cap. (mil) | US\$1,209.3 | |
| Avg Daily Vol (ooo) | 269 | |
| Book Value/Share | US\$9.00 | |
| Net Cash Per Share | US\$8.55 | |
| <i>Proforma 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

| YEAR | REVENUE (m) | | | | | | EARNINGS PER SHARE () | | | | | |
|-------|--------------|------|-----|------|------|-------|------------------------|--------|--------|--------|--------|--------|
| | Mar | Jun | Sep | Dec | FY | FY RM | Mar | Jun | Sep | Dec | FY | FY P/E |
| 2012A | — | — | — | — | 15.3 | 79.0X | — | — | — | — | (1.55) | NM |
| 2013A | 15.0 | 26.4 | 4.3 | 11.5 | 57.2 | 21.1X | 0.08 | 0.62 | (0.86) | (0.64) | (0.96) | NM |
| 2014E | 3.6 | 3.6 | 3.1 | 13.1 | 23.5 | 51.5X | (0.41) | (0.45) | (0.50) | (0.22) | (1.58) | NM |

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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

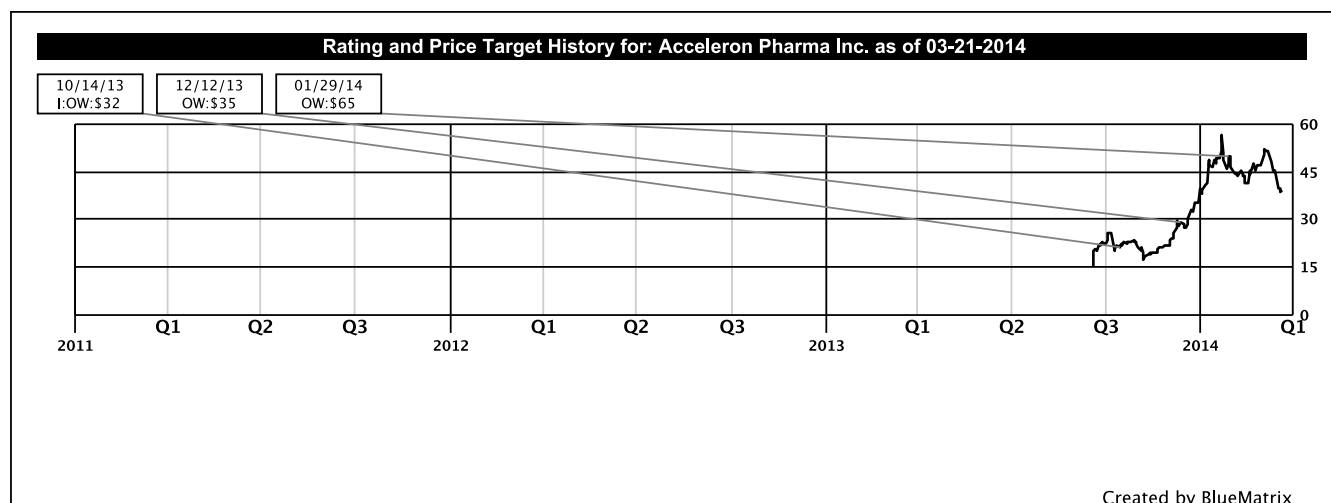
1. Net income applicable to common shareholders for 2012A, 1Q:13A, 2Q:13A, 3Q:13A and 2013A assumes full conversion of convertible preferred shares.

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

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Legend:

I: Initiating Coverage
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] | 351 | 59.19 | 83 | 23.65 |
| HOLD [N] | 222 | 37.44 | 22 | 9.91 |
| SELL [UW] | 20 | 3.37 | 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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- **Underweight (UW):** Anticipated to underperform relative to the median of the group of stocks covered by the analyst.

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