

Acceleron Pharma Inc. (XLRN)

Dalantercept Misses in Ovarian Cooperative Group Study; Lowering Price Target to \$52

MARKET DATA

| | |
|---------------------------|-------------------|
| Price | \$25.39 |
| 52-Week Range: | \$16.78 - \$57.89 |
| Shares Out. (M): | 26.5 |
| Market Cap (\$M): | \$672.8 |
| 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

MARKET OUTPERFORM | Price: \$25.39 | Target Price: \$52.00

INVESTMENT HIGHLIGHTS

No-go decision for the Gynecologic Oncology Group study in ovarian cancer seen as an incremental negative to the company sponsored program in RCC; reiterate Market Outperform rating on Acceleron, but lower price target to \$52 from \$53.

This morning, Acceleron announced top-line results from the second of two Phase II trials of dalantercept monotherapy led by Gynecologic Oncology Group (GOG) and the NCI in recurrent epithelial ovarian cancer. Of the thirty patients enrolled, ≥ 6 -month PFS was achieved in six (20%) of patients, narrowly missing the trial's prespecified threshold (7/30 (23%) with ≥ 6 -month PFS) for expanding recruitment to a larger Phase II analysis. Recall that a similar, no-go decision was reached by the GOG in 4Q13 over a monotherapy trial in recurrent endometrial cancer. While the GOG trial outcomes do not, in our view, invalidate the ongoing company sponsored programs in RCC and HCC, they do imply an incrementally more challenging road ahead versus prior expectations. As such, we have increased our discount rate for dalantercept (market assumptions remaining limited to in RCC) to 40% from 35%, depressing our per share NPV valuation of the program from \$5.50 to \$4.08. Success in HCC and GBM (as with ovarian cancer previously) remain sources of upside to our valuation. Our price target change to \$52 from \$53 is based on a synthesis of our DCF (\$48.19) and NPV sum-of-the-parts (\$54.95) methodologies.

| 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



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FOR DISCLOSURE AND FOOTNOTE INFORMATION, REFER TO JMP FACTS AND DISCLOSURES SECTION.

FIGURE 1. Upcoming Milestones

| Timing | Drug | Milestones |
|----------|-----------------------|--|
| 4Q14 | Sotatercept | Phase IIa CKD update at ASN Meeting |
| 4Q14 | Sotatercept & ACE-536 | Interim update from Phase II trials in β -thalassemia and MDS at ASH |
| 4Q14 | ACE-083 | Initiation of Phase I trial in muscular dystrophy |
| Mid-2015 | Sotatercept & ACE-536 | Initiation of Phase III trial in β -thalassemia and/or MDS |

Source: JMP Securities LLC and Company Reports

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 β signaling pathway. These fusion proteins have shown clinical potential in the treatment of anemia disorders related to β -thalassemia and myelodysplastic syndromes, and in the treatment of solid cancers, muscle wasting disorders, and other indications impacted by dysregulated TGF β .

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 β -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.

JMP FACTS AND DISCLOSURES

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JMP Securities Disclosures:

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JMP Securities was manager or co-manager of a public offering of securities for Accelaron 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.

Market Perform (MP): JMP Securities expects the stock price to perform in line with relevant market indices over the next 12 months.

Market Underperform (MU): JMP Securities expects the stock price to underperform 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% | 20 | 14.49% |
| 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% | 121 | 26.77% |

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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