Siena Market Line
2nd week of April 2015

# Acceleron Pharma, Inc.

NASDAQ:XLRN

**Analyst:** Pamela Juergens
**Sector:** Healthcare

## BUY

Price Target: $58.76

## Key Statistics as of 4/8/2015

| | |
|---|---|
| Market Price: | $35.16 |
| Industry: | Biotechnology |
| Market Cap: | $1.15 B |
| 52-Week Range: | $23.61-48.50 |
| Beta: | N/A |

## Thesis Points:

- robust pipeline, diversified across different clinical needs
- poised for significant value creation in 2015
- lucrative partnership with pharmaceutical giant

## Company Description:

Acceleron Pharma, Inc. is a clinical stage biopharmaceutical company that focuses on the discovery, development, and commercialization of protein therapeutics for cancer and rare diseases. The company is a leader in discovering and developing protein therapeutics that regulate cellular growth and repair. Acceleron looks to unlock the body's ability to regulate the growth and repair of various cells and tissues including red blood cells, muscle, bone and the vasculature. The company is developing luspatercept and sotatercept therapeutic candidates to treat anemia and associated complications in patients with ß-thalassemia and myelodysplastic syndromes. Acceleron is also developing dalantercept therapeutic candidate to treat renal cell carcinoma and hepatocellular carcinoma and ACE-083 used to promote muscle growth and function in specific treated muscle groups.

## Thesis

Acceleron has a highly productive discovery and development platform that is creating a deep and robust pipeline. Their pipeline is widely diversified across many different diseases, many of which have a large unmet need for treatment. They are poised for significant value creation in 2015, as they have many upcoming catalysts as their products move into different stages of development, which will drive their stock price. They also have a potentially lucrative partnership with pharmaceutical giant Celgene, that provides them with opportunities in the future.

## Robust Product Pipeline Poised for Value Creation in 2015

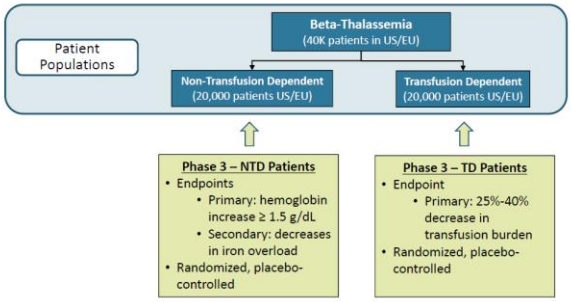
Acceleron has a pipeline of biologic therapies, and have four internally developed programs in clinical trials.

***Hematology Franchise:*** Acceleron's lead product candidates include Luspatercept and Sotatercept. Luspatercept (ACE-536) is an investigational protein therapeutic that increases red blood cell levels by targeting molecules in the TGF-β superfamily. Acceleron is developing Luspatercept and Sotatercept in partnership with Celgene to treat anemia in patients with rare blood disorders including β-Thalassemia and myelodysplastic syndromes. They are also developing sotatercept in indications where its effects could be beneficial in addition to treating anemia, including chronic kidney disease and multiple myeloma.

Myelodysplastic syndromes (MDS) are hematological medical conditions with ineffective production of all blood cells. Patients with MDS can develop severe anemia and require blood transfusions. In some cases the disease worsens and the patient develops low blood counts cause by progressive bone marrow failure. MDS are all disorders of the hematopoietic stem cells in the bone marrow. In MDS hematopoiesis (blood production) is disorderly and ineffective. There are approximately 125,000 MDS patients in the United States and Europe. 70,000 of these patients are considered low risk, and 55,000 are considered high risk. There are currently three treatments on the market, including Revlimid, Vidaza, and Dacogen which are used in addition to red blood cell transfusions. The therapeutic goal of lustracept is to increase hemoglobin levels in patients and allow them to achieve transfusion independence. In December 2014, Acceleron announced positive results from phase 2 trials of luspatercept. Preliminary results demonstrated increases in hemoglobin levels, decreases in the transfusion burden and established transfusion independence in low risk patients. They were also able to identify potential biomarkers to select patients that are most likely to respond to the treatment. This clinical trial was designed as a two-stage study. The first stage was a dose escalation design; and the second stage,the expansion stage, allows for each patient's dose of luspatercept to be titrated to maximize the potential clinical benefit for that patient. In 2015, there are several things expected to happen with the program that will serve as catalysts for the stock price. First they expect to complete the expansion phase of the clinical trial in order to determine the proper dosing for patients. Positive results from the final stage of the phase 2 trial will enable them to continue to rapidly advance their MDS program in partnership with Celgene, including initiated Phase 3 clinical trial in MDS. Under the terms of their agreement with Celgene, Acceleron is responsible for conducting the Phase 1 and initial Phase 2 trials and Celgene will conduct the subsequent Phase 2 and Phase 3 clinical studies. Additionally, Acceleron is responsible for manufacturing luspatercept for the Phase 1 and Phase 2 clinical trials and Celgene is responsible for the manufacture of Phase 3 and commercial supplies. Acceleron is eligible to receive up $217 million in development, regulatory and commercial milestones, as well as tiered double-digit royalties on worldwide net sales.

Beta- Thalassemias ( β-Thalassemia) are a group of inherited blood disorders caused by reduced or absent synthesis of the beta chains of hemoglobin that result in variable outcomes ranging from severe and asymptomatic individuals. β-Thalassemia is caused by mutations in the HBB gene on chromosome 11. The

severity of the disease depends on the nature of the mutation. Approximately 40,000 patients are affected by β-Thalassemia in the US and Europe. About half are non-transfusion dependent and half are transfusion dependent. For non-transfusion dependent patients there is currently no effective therapy, and iron chelation is used to manage iron overload. Transfusion dependent patients receive red blood cell transfusions and iron chelation is used to manage iron overload. The therapeutic goal of luspatercept and sotatercept in the treatment of β-Thalassemia is to increase hemoglobin levels, reduce the transfusion burden, reduce iron overload, and improve disease complications. In December, Acceleron and Celgene reported positive results from ongoing Phase 2 trials of luspatercept and sotatercept for the treatment of β-Thalassemia. Activity in both non-transfusion and transfusion dependent patients that met and exceeded endpoints expected to be used in Phase 3 trials. These results indicate that the program has the potential to be disease modifying across a broad spectrum of β-Thalassemia patients, from those that are very heavily transfused to those or receive few or no transfusion and have no other treatment options to manage their disease. In 2015, Acceleron expects to determine the proper dosing in Q1 and Q2, complete Phase 2 in Q2 and Q3, and initiate Phase 3 trials in Q4 which when initiated should drive their stock price.




***Sotatercept for Chronic Kidney Disease:*** Chronic Kidney disease is the third indication in Acceleron's clinical trials with Celgene. This indication is for End Stage Renal Disease (ESRD) patients on hemodialysis. There are approximately 700,000 ESRD patients in the US and Europe. During the course of 2014, Celgen generated data that indicated that sotatercept could positively affect several important clinical complications for ESRD patients including anemia, mineral abnormalities, bone loss, and vascular calcification. The treatment options for anemia and mineral abnormalities are limited; erythropoiesis-stimulating agents (ESAs) and iron, and phosphate binders and calcimimetics respectively. There are currently no effective therapies for bone loss and vascular calcification. Preliminary Phase 2 results demonstrated increases in in cortical bone mineral density, reductions in vascular calcification and increases in hemoglobin levels. This is a large opportunity for Acceleron because there is currently a large unmet need for this treatment. Upcoming catalysts in 2015 include completion of the ongoing Phase 2a, completing the dose finding stage of the ongoing Phase 2b study and initiating the randomized, controlled stage of Phase 2b study.

***Muscle Franchise:*** Acceleron is investigating ACE-083 for muscle loss. ACE-083 is an investigational protein therapeutic that has been designed for local administration to increase muscle mass and strength in specific muscle groups. Acceleron is developing ACE-083 for diseases in which improved muscle strength may provide a clinical benefit, such as inclusion body myositis and certain forms of muscular dystrophy. There are currently 11,000 Inclusion Body Myositis patients in the US and 16,000 Muscular Dystrophy patients in the US. ACE-083 is formulated to block select proteins in the TGF-β superfamily that function to modulate muscle growth, and in doing so can increase muscle mass and strength. The rare diseases Acceleron is looking to target with this treatment are unique because only a limited number of muscles are impacted and suffer a loss in size and function. In preclinical animal studies ACE-083 has shown selective and dose dependent increases in muscle mass in treated muscles with no effects in untreated muscles. The upcoming catalysts in 2015 include the completion of a Phase 1 study in Q2, and the initiation of a Phase 2 study in end of Q3 or Q4.

***Dalantercept:*** Dalantercept (ACE-041) is a program wholly-owned by Acceleron, for the treatment of Renal Cell Carcinoma (RCC) and Hepatocellular Carcinoma (HCC), which have 65,000 new patients per year in the US, and 31,000 new patients per year in the US respectively. Dalantercept is an investigational protein therapeutic that inhibits angiogenesis by

preventing proteins in the TGF-β from interacting with activin receptor like kinase 1 (ALK1), a cell surface receptor on proliferating endothelial cells. Acceleron recently reported new preliminary data from the DART study, an ongoing clinical Phase 2 trial. In the DART trial, dalantercept is being evaluated in combination with axitinib, an approved VEGFR tyrosine kinase inhibitor in patients with advanced RCC, who have progressed on one or more VEGFR tyrosine kinase inhibitor and no more than three prior treatments. This means all of the patients were second to fourth line patients who had failed on at least one prior VEGF therapy. In the DART study the combination of dalantercept and axitinib generated an objective response rate of 25%, and the preliminary mean progression-free survival is 8.3 months. Based on the positive data for this trial they are now enrolling patients in the randomized, placebo-controlled stage of Phase 2. This program is a large opportunity for Acceleron because there is a large need for the treatment worldwide. Worldwide sale sif anti-VEGFs in RCC were $2.6 billion in 2013, and worldwide sales of anti-VEGFs in HCC were $766 million in 2013. Upcoming catalysts for 2015 include dose escalation data and initiation of the randomized, placebo-controlled stage of Phase 2.

## Partnership with Celgene

In February 2008, Acceleron entered into a worldwide strategic collaboration with pharmaceutical giant Celgene for the joint development and commercialization of sotatercept, and in August 2011 they expanded the collaboration to include ACE-536. In total Acceleron receive $75 million in upfront payments and they are eligible to receive up to $367 million in milestone payments for the sotatercept program and up to $200 million in milestone payments for the ACE-536 program, as well as royalties. Further, in April 2014 Celgen paid $47 million to venture capitalists to increase their stake in Acceleron to almost 15% ownership. In the deal Celgene paid close to triple the $15 per share price. Celgene currently has seven approved drugs on the market, but still relies mainly on one cancer drug. They need to build up their pipeline beyond 2020, in order to continue to compete with pharmaceutical giants such as Merck, and Pfizer. Celgene currently has 37 active alliances, and in 2014 they ranked among the most generous among large biotechs with an average upfront payment of $222 million compared to the industry average of $70 million. This demonstrates their willingness to pay to expand their pipeline. Acceleron presents an interesting opportunity for Celgene. They have more pipeline assets in various stages of development that could help add to Celgene's pipeline, which would translate to more milestone and upfront payments for Acceleron. With $189 million cash and cash equivalents on their balance sheet, expected to provide the company funding into the second half of 2017, they make an attractive acquisition candidate especially for a company that could potentially owe them more than $500 million in milestone payments and already owns about 15% of them. Celgene is valued at $97 billion and they have cash and cash equivalents of $7.5 billion, and they could easily acquire the rest Acceleron well above their market price.

# CENTER FOR GLOBAL FINANCIAL STUDIES

| Acceleron Pharma, Inc. | XLRN | Analyst: Pamela Juergens | Current Price: $35.02 | Intrinsic Value: $49.60 | Target Value: $58.76 | Divident Yield: 0% | Target Return: 1-y Return: 67.78% | BULLISH |
|---|---|---|---|---|---|---|---|---|

## General Info

| | |
|---|---|
| Sector | Healthcare |
| Industry | Biotechnology |
| Last Guidance | (Invalid Identifier) |
| Next earnings date | NM |

## Market Data

| | |
|---|---|
| Enterprise value | $938.01 |
| Market Capitalization | $224,790.42 |
| Daily volume | 7.72 |
| Shares outstanding | 32.64 |
| Diluted shares outstanding | 31.52 |
| % shares held by institutions | 77.53% |
| % shares held by insiders | 1.02% |
| Short interest | 6.50% |
| Days to cover short interest | 8.97 |
| 52 week high | $48.50 |
| 52-week low | $23.61 |
| 5y Beta | 0.00 |
| 6-month volatility | 60.30% |

## Peers

| Peers | Market Cap. |
|---|---|
| PDL BioPharma, Inc | $1,126.24 |
| Achillion Pharmaceuticals, Inc | $1,207.72 |
| Array BioPharma, Inc | $1,060.30 |
| Sangamo Biosciences Inc | $1,067.98 |
| Merrimack Pharmaceuticals, Inc | $1,323.43 |
| Acorda Therapeutics, Inc | $1,447.98 |

## Current Capital Structure

| | |
|---|---|
| Total debt/market cap | 0.00% |
| Cost of Borrowing | #DIV/0! |
| Interest Coverage | 2051.06% |
| Altman Z | 20.51 |
| Debt Rating | D |
| Levered Beta | 1.06 |
| WACC (based on market value weights) | 8.95% |

## Past Earning Surprises

| | Revenue | EBITDA | Norm. EPS |
|---|---|---|---|
| Last Quarter | -65.2% | 0.0% | |
| Last Quarter-1 | -35.7% | 0.0% | |
| Last Quarter -2 | 49.5% | 0.0% | |
| Last Quarter -3 | 0.0% | 0.0% | |
| Last Quarter -4 | -15.7% | 0.0% | |

## Management

| Professional | Title | Comp. FY2012 | Comp. FY2013 | Comp. FY2014 |
|---|---|---|---|---|
| Knopf, John | Founder, Chief Executive Officer | $ 1,955,300.00 | $ 2,633,884.00 | $ - |
| Maniatis, Tomas | Co-Founder, Chairman of Scient | $ 20,000.00 | $ 333,441.00 | $ - |
| Quisel, John | Senior Vice President and Gener | $ 561,415.00 | $ 910,219.00 | $ - |
| Sherman, Matthew | Chief Medical Officer and Execut | $ 598,101.00 | $ 999,216.00 | $ - |
| Ptashne, Mark | Co-Founder and Member of Sci | $ - | $ - | $ - |
| McLaughlin, Kevin | Chief Financial Officer, Principal | $ - | $ - | $ - |

## 10y-Median Performance

| | XLRN | Peers | Industry | All U.S. firms |
|---|---|---|---|---|
| Growth | 0.0% | 17.5% | 13.8% | 7.4% |
| ROIC | -5.9% | 9.1% | 1.1% | 14.3% |
| NOPLAT Margin | 30.4% | 17.2% | 7.3% | 10.4% |
| REV./Invested Capital | -19.5% | 53.0% | 15.0% | 137.4% |
| Excess Cash/Rev. | N/A | 113.2% | 13.5% | 12.9% |
| Total Cash /Rev. | 1206.0% | 141.9% | 12.3% | 15.2% |
| Unlevered Beta | | 1.06 | 1.09 | 0.95 |
| TEV/REV | 45.3x | 5.4x | 6.2x | 2.5x |
| TEV/EBITA | 0.0x | 30.2x | 17.4x | 13.1x |
| PE | | 54.3x | 31.3x | 23.5x |
| P/BV | 1.5x | 5.2x | 2.3x | 2.2x |

## Non-GAAP Adjustments in estimates computations

| | | | |
|---|---|---|---|
| Operating Leases Capitalization | 100% | Straightline | 10 years |
| R&D Exp. Capitalization | 100% | Straightline | 10 years |
| Expl./Drilling Exp. Capitalization | 0% | N/A | N/A |
| SG&A Capitalization | 0% | N/A | N/A |

## Proforma Assumptions

| | $ - |
|---|---|
| Money market rate as of today | 0.59% |
| Annual increase (decrease) in interest rates | 0.1% |
| Yield Spread acceleration | 1.2 |
| Marginal Tax Rate | 37.5% |
| Risk-Free rate | 2.5% |
| Tobin's Q | 1.00 |
| Op. Cash/Rev. | 2% |
| Growth in PPE | NPPE Growth follows Revenue Growth |
| Long term Growth | 8.0% |
| Long term EBITDA Margin | 50.0% |
| Long term NOPLAT Margin | 20.0% |
| Long term ROIC | 20.0% |
| Most recent Unlevered Beta | |
| Long term Unlevered Beta | 1.02 |

## Forecast

| | Rev. Growth | Adj. Op. Cost/Rev | Invested Capital | NOPLAT Margin | ROIC | WACC |
|---|---|---|---|---|---|---|
| LTM | -74.4% | 404.6% | $261.00 | -84% | -4.7% | 8.9% |
| NTM | 130.2% | 400.0% | $430.67 | -146% | -18.8% | 9.0% |
| NTM+1 | 21.8% | 225.0% | $695.26 | 42% | 4.0% | 9.1% |
| NTM+2 | 35.0% | 137.5% | $1,154.19 | 119% | 9.5% | 9.2% |
| NTM+3 | 197.9% | 93.8% | $1,882.58 | 89% | 12.8% | 9.1% |
| NTM+4 | 210.6% | 71.9% | $2,942.80 | 69% | 18.8% | 9.1% |
| NTM+5 | 80.4% | 60.9% | $4,136.74 | 68% | 21.5% | 9.2% |
| NTM+6 | 72.4% | 55.5% | $5,584.79 | 66% | 25.5% | 9.3% |
| NTM+7 | 30.9% | 52.7% | $6,794.48 | 66% | 24.8% | 9.4% |
| NTM+8 | 27.4% | 51.4% | $7,983.80 | 66% | 25.7% | 9.5% |

## Valuation

| | Invested Capital x (ROIC-WACC) | Enterprise Value (UFCF Valuation only | Total Debt | Other claims | Equity Value | UDCF Valuation | Relative Valuation | Weighted Price Per Share |
|---|---|---|---|---|---|---|---|---|
| **LTM** | -$35.59 | $2,081.03 | $0.00 | -$72.34 | $2,153.37 | $68.90 | -$4.49 | $50.56 |
| **NTM** | -$119.92 | $2,463.38 | $0.00 | -$47.25 | $2,510.62 | $83.67 | -$12.97 | $59.51 |
| **NTM+1** | -$35.48 | $3,272.59 | $0.00 | -$33.96 | $3,306.55 | $111.60 | -$6.66 | $82.03 |
| **NTM+2** | $3.37 | $4,564.47 | $0.00 | -$7.93 | $4,572.40 | $156.29 | -$5.43 | $115.86 |
| **NTM+3** | $70.02 | $6,718.41 | $0.00 | $153.42 | $6,564.98 | $226.82 | -$20.78 | $164.92 |
| **NTM+4** | $286.09 | $10,312.28 | $0.00 | $588.44 | $9,723.84 | $342.67 | -$77.68 | $237.58 |
| **NTM+5** | $510.41 | $16,041.36 | $0.00 | $915.60 | $15,125.76 | $543.01 | -$137.48 | $372.89 |
| **NTM+6** | $903.15 | $26,491.58 | $0.00 | $1,479.09 | $25,012.49 | $929.92 | -$240.72 | $637.26 |
| **NTM+7** | $1,042.43 | $46,932.51 | $0.00 | $1,602.01 | $45,330.50 | $1,768.02 | -$305.60 | $1,249.62 |
| **NTM+8** | $81,895.58 | $94,064.19 | $0.00 | $1,645.39 | $92,418.81 | $2,831.11 | -$382.12 | $2,027.80 |

## Monte Carlo Simulation Assumptions

| | Base | Stdev | Min | Max | Distribution |
|---|---|---|---|---|---|
| **Revenue Variation** | **0** | **10%** | **N/A** | **N/A** | **Normal** |
| **Op. Costs Variation** | **0** | **10%** | **N/A** | **N/A** | **Normal** |
| **Country Risk Premium** | **6%** | **N/A** | **5%** | **7%** | **Triangular** |
| **Long term Growth** | **8%** | **N/A** | **3%** | **17%** | **Triangular** |

## Monte Carlo Simulation Results

| | Intrinsic Value | 1y-Target |
|---|---|---|
| Mean est. | $50.56 | $59.51 |
| σ(ε) | $0.32 | $0.25 |
| 3 σ(ε) adjusted price | $49.60 | $58.76 |
| Current Price | $35.02 | |
| Analysts' median est. | | $57.17 |