

Equity Research

March 6, 2014

Price: \$31.64 (03/5/2014)

Price Target: NA

OUTPERFORM (1)

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Key Data

| | |
|------------------------|-----------------|
| Symbol | NASDAQ: AGIO |
| 52-Week Range: | \$44.04 - 15.77 |
| Market Cap (MM): | \$986.3 |
| Net Debt (MM): | \$0.0 |
| Cash/Share: | \$35.39 |
| Dil. Shares Out (MM): | 31.2 |
| Enterprise Value (MM): | \$777.9 |
| ROIC: | NA |
| ROE (LTM): | NA |
| BV/Share: | \$4.59 |
| Dividend: | NA |

| FY (Dec) | 2013A | 2014E | 2015E |
|---------------------------|----------|----------|----------|
| Earnings Per Share | | | |
| Q1 | \$(0.37) | \$(0.40) | - |
| Prior Q1 | - | \$(0.37) | - |
| Q2 | \$(0.34) | \$(0.40) | - |
| Prior Q2 | - | \$(0.36) | - |
| Q3 | \$(0.49) | \$(0.20) | - |
| Prior Q3 | - | \$(0.16) | - |
| Q4 | \$(0.39) | \$(0.31) | - |
| Prior Q4 | \$(0.36) | \$(0.26) | - |
| Year | \$(1.52) | \$(1.30) | \$(0.96) |
| Prior Year | \$(1.48) | \$(1.15) | \$(0.85) |
| P/E | NM | NM | NM |
| Consensus EPS | \$(1.62) | \$(0.86) | \$(0.45) |
| Prior Year | - | \$(1.09) | \$(0.83) |

Consensus source: Thomson Reuters

Revenue (MM)

| | | | |
|------------|--------|--------|--------|
| Year | \$25.5 | \$38.4 | \$55.0 |
| Prior Year | \$25.1 | \$38.0 | - |
| EVS | 30.5x | 20.3x | 14.1x |

Earnings Update

First Clinical Data Coming Earlier Than Expected

The Cowen Insight

More important than Q4 numbers, Agios announced the first clinical data on AG-221 in IDH2-mutant liquid tumors will be presented at AACR next month. This is much earlier than prior expectations of data in Q4, and suggests things are going well, as does management's commitment to initiating Phase II expansion cohorts later in 2014. We expect AGIO to outperform as the clinical data mature.

Financials In Line...

Agios reported a net loss of \$12.4MM in Q4, slightly above our \$11.3MME due mainly to higher SG&A. The company finished the year with \$193MM cash, sufficient to fund operations through late 2016, exclusive of potential future milestone payments or partnerships. The company issued guidance for YE:14 cash of more than \$130MM.

...But AACR Presentation On AG-221 Is The Big News.

Agios announced that the first clinical data on its first clinical trial, a Phase I dose escalation study of AG-221 in IDH2-mutant hematologic tumors, will be featured in an [oral presentation at AACR](#) on April 6. While the company would disclose nothing specific about the content of the presentation, we have to believe this is a positive sign, for two main reasons:

(1) Agios had previously suggested that it would be reasonable to expect an initial data presentation at ASH in December; this much earlier timing, just six months after the first patient entered the trial, would suggest that things are going rather well. Indeed, management has consistently said that corporate policy is only to present cohorts of data from trials when enough information is available to tell a cohesive story. Given that management asserts that now is "the right time to present the data," we believe the data must be robust enough to give confidence in the story told.

(2) Management has told us that endpoints examined in the trial will include safety, PK, PD, and clinical assessment. Given that management has now committed to initiating Phase II expansion cohorts in 2014, it appears likely the early results are quite promising on at least some of these metrics. In particular, we suspect Agios would not move into Phase II if they were not, at a minimum, seeing a reduction in the serum 2-HG biomarker, which would be quite encouraging. We will be interested to see if there is evidence of cellular differentiation or morphological responses in the AACR data, but given the advanced stage of these patients and likely suboptimal dosing to date, we would consider such findings a positive surprise.

AG-120 And AG-348 Also Moving Along.

AG-120's Phase I trials for solid and liquid tumors with IDH1 mutations remain on track to begin imminently. Agios has also now completed IND-enabling activities for AG-348 in the orphan metabolic disease, PK deficiency, and is on track to begin single- and multiple-ascending dose studies in healthy volunteers by mid-2014.

At A Glance

Our Investment Thesis

Agios' lead candidates in cancer, AG-221 and AG-120, target several cancers mutant in the metabolic genes IDH2 and IDH1, respectively. AG-221 entered Phase I in September, and AG-120 will follow in early 2014. Each has shown encouraging preclinical efficacy. We believe each has the potential to demonstrate clinical proof of concept in 2014, and estimate that they could generate \$650MM+ and \$450MM+ in global revenue, respectively. Agios' leading IEM candidate is AG-348 for pyruvate kinase deficiency, a rare form of hemolytic anemia. The unmet need is great in this indication, and there may be 1,000 - 3,000 diagnosed patients in the U.S. alone. AG-348 will enter Phase I in mid-2014, and we would expect proof of concept data by 2015. We believe AG-348 could generate \$600MM+ in peak revenue, and is wholly owned by Agios. We expect multiple value-creating clinical readouts to drive stock outperformance over the next 12-18 months.

Forthcoming Catalysts

- Present first clinical data on AG-221 for IDH2m cancer at AACR (April)
- Move AG-221 into Phase II (2014)
- Possible clinical data on AG-120 for IDH1m cancer (H2:14/H1:15)

Base Case Assumptions

- Agios succeeds in developing at least two targeted cancer therapies

Upside Scenario

- Agios succeeds in developing more than two targeted cancer therapies

Downside Scenario

- Agios is not successful in developing any drugs

Price Performance



Source: Bloomberg

Company Description

Agios Pharmaceuticals is leveraging its leading expertise in cellular metabolism to develop therapeutics in two related areas: (1) cancer metabolism and (2) inborn errors of metabolism (IEMs). All of Agios' programs follow a "precision medicine" strategy, meaning that the targeted patients are well-defined and prospectively identifiable, that the drug candidates are tailored to meet the specific patient segments' needs, and that a biomarker is available to provide early proof of mechanism in humans. We applaud this approach, as we believe it drives to value inflection points quickly, while minimizing risk and potentially abbreviating time to market. A partnership with Celgene provides nondilutive financing and validation of Agios' leadership position in cancer metabolism, while Agios retains meaningful economics.

Investment Thesis

Agios Pharmaceuticals is leveraging its leading expertise in cellular metabolism to develop therapeutics in two related areas: (1) cancer metabolism and (2) inborn errors of metabolism (IEMs). All of Agios' programs follow a "precision medicine" strategy, meaning that the targeted patients are well-defined and prospectively identifiable, that the drug candidates are tailored to meet the specific patient segments' needs, and that a biomarker is available to provide early proof of mechanism in humans. We applaud this approach, as we believe it drives to value inflection points quickly, while minimizing risk and potentially abbreviating time to market. Agios' lead candidates in cancer, AG-221 and AG-120, target several cancers mutant in the metabolic genes IDH2 and IDH1, respectively. AG-221 entered Phase I in September, and AG-120 will follow in early 2014. Each has shown encouraging preclinical efficacy. We believe each has the potential to demonstrate clinical proof of concept in 2014, and estimate that they could generate \$650MM+ and \$450MM+ in global revenue, respectively. A partnership with Celgene provides nondilutive financing and validation of Agios' leadership position in cancer metabolism, while Agios retains meaningful economics. Agios' leading IEM candidate is AG-348 for pyruvate kinase deficiency, a rare form of hemolytic anemia. The unmet need is great in this indication, and there may be 1,000 - 3,000 diagnosed patients in the U.S. alone. AG-348 will enter Phase I in mid-2014, and we would expect proof of concept data by 2015. We believe AG-348 could generate \$600MM+ in peak revenue, and is wholly owned by Agios. We expect multiple value-creating clinical readouts to drive stock outperformance over the next 12-18 months.

Upcoming Agios Milestones

| Event | Timing |
|---|-------------|
| Begin enrollment in AG-120's Phase I trials in IDH1m cancers | Early 2014 |
| Begin enrollment in AG-348's Phase I trial in PK deficiency | Mid:2014 |
| Start of Phase II expansion cohorts for AGI-221 in IDH2m liquid tumors | 2014 |
| Possible initial proof of mechanism data for AGI-120 in IDH1m tumors, start of Phase II expansion cohorts | H2:14/H1:15 |
| Possible initial proof of mechanism data for AGI-348 in PK deficiency | H2:14/2015 |

Source: Cowen and Company

Agios Pharmaceuticals Quarterly P&L Model (\$MM)

| | Q1:13A | Q2:13A | Q3:13A | Q4:13A | 2013A | Q1:14E | Q2:14E | Q3:14E | Q4:14E | 2014E |
|--|-----------------|-----------------|-----------------|-----------------|-----------------|-----------------|-----------------|-----------------|-----------------|-----------------|
| Product Revenue | | | | | | | | | | |
| Collaboration Revenue | 6.3 | 6.3 | 6.3 | 6.7 | 25.5 | 6.7 | 7.0 | 14.0 | 10.7 | 38.4 |
| Total Revenue | 6.3 | 6.3 | 6.3 | 6.7 | 25.5 | 6.7 | 7.0 | 14.0 | 10.7 | 38.4 |
| COGS | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 |
| R&D | 11.5 | 13.0 | 14.8 | 15.3 | 54.5 | 15.8 | 16.0 | 16.5 | 16.7 | 65.0 |
| SG&A | 1.9 | 1.8 | 2.5 | 3.7 | 9.9 | 4.0 | 4.2 | 4.4 | 4.6 | 17.2 |
| Total Expenses | 13.3 | 14.8 | 17.3 | 19.0 | 64.4 | 19.8 | 20.2 | 20.9 | 21.3 | 82.2 |
| Operating Income/Loss | (7.0) | (8.5) | (11.1) | (12.2) | (38.9) | (13.1) | (13.2) | (6.9) | (10.6) | (43.8) |
| Non-Operating Income | 0.0 | 0.0 | 0.0 | 0.0 | 0.1 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 |
| Pre-tax Income/Loss | (7.0) | (8.5) | (11.1) | (12.2) | (38.8) | (13.1) | (13.2) | (6.9) | (10.6) | (43.8) |
| Tax rate (%) | NM | NM | NM | NM | NM | NM | NM | NM | NM | NM |
| Provision for income taxes | 0.2 | 0.1 | 0.1 | (0.2) | 0.2 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 |
| Net Income (Loss) From Operations | (7.2) | (8.6) | (11.2) | (12.0) | (39.1) | (13.1) | (13.2) | (6.9) | (10.6) | (43.8) |
| Cumulative Preferred Stock Dividends | (1.8) | (1.8) | (0.6) | | | | | | | |
| GAAP EPS | (\$0.37) | (\$0.34) | (\$0.49) | (\$0.39) | (\$1.52) | (\$0.40) | (\$0.40) | (\$0.20) | (\$0.31) | (\$1.30) |
| Diluted Shares | 24.1 | 25.0 | 22.7 | 31.2 | 25.8 | 32.8 | 33.4 | 33.7 | 34.7 | 33.7 |

Source: Cowen and Company

Agios Pharmaceuticals Annual P&L Model (\$MM)

| | 2013A | 2014E | 2015E | 2016E | 2017E | 2018E |
|--|-----------------|-----------------|-----------------|-----------------|-----------------|-----------------|
| Product Revenue | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | 8.0 |
| Collaboration Revenue | 25.5 | 38.4 | 55.0 | 67.0 | 25.0 | 27.0 |
| Total Revenue | 25.5 | 38.4 | 55.0 | 67.0 | 25.0 | 35.0 |
| COGS | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 |
| R&D | 54.5 | 65.0 | 70.0 | 74.0 | 76.0 | 78.0 |
| SG&A | 9.9 | 17.2 | 19.0 | 21.0 | 23.0 | 40.0 |
| Total Expenses | 64.4 | 82.2 | 89.0 | 95.0 | 99.0 | 118.0 |
| Operating Income/Loss | (38.9) | (43.8) | (34.0) | (28.0) | (74.0) | (83.0) |
| Non-Operating Income | 0.1 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 |
| Pre-tax Income/Loss | (38.8) | (43.8) | (34.0) | (28.0) | (74.0) | (83.0) |
| Tax rate (%) | NM | NM | NM | NM | NM | NM |
| Provision for income taxes | 0.2 | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 |
| Net Income (Loss) From Operations | (39.1) | (43.8) | (34.0) | (28.0) | (74.0) | (83.0) |
| GAAP EPS | (\$1.52) | (\$1.30) | (\$0.96) | (\$0.73) | (\$1.87) | (\$1.90) |
| Diluted Shares | 25.8 | 33.7 | 35.5 | 38.2 | 39.5 | 43.8 |

Source: Cowen and Company

Valuation Methodology And Risks

Valuation Methodology

Biotechnology:

In calculating our 12-month target price, we employ one or more valuation methodologies, which include a discounted earnings analysis, discounted cash flow analysis, net present value analysis and/or a comparable company analysis. These analyses may or may not require the use of objective measures such as price-to-earnings or price-to-sales multiples as well as subjective measures such as discount rates.

We make investment recommendations on early stage (pre-commercial) biotechnology companies based upon an assessment of their technology, the probability of pipeline success, and the potential market opportunity in the event of success. However, because these companies lack traditional financial metrics, we do not believe there are any good methodologies for assigning a specific target price to such stocks.

Investment Risks

Biotechnology:

There are multiple risks that are inherent with an investment in the biotechnology sector. Beyond systemic risk, there is also clinical, regulatory, and commercial risk. Additionally, biotechnology companies require significant amounts of capital in order to develop their clinical programs. The capital-raising environment is always changing and there is risk that necessary capital to complete development may not be readily available.

Risks To The Price Target

Agios Pharmaceuticals is developing several, currently preclinical, drug candidates in the areas of cancer metabolism and inborn errors of metabolism. All of Agios' drug candidates face clinical and regulatory risk. With the future development path depending on the evolution of clinical data, future revenue forecasts are uncertain. The commercial outlook for Agios' candidates could additionally be altered by safety/efficacy findings, emerging competition, alterations in the medical treatment paradigm, or changes in the pricing environment. Some of Agios' projected market exclusivity depends on patents, which are subject to challenge by generic drugmakers.

Addendum

Stocks Mentioned In Important Disclosures

| Ticker | Company Name |
|--------|---------------------------|
| AGIO | Agios Pharmaceuticals Inc |

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Cowen and Company Rating System effective May 25, 2013

Outperform (1): The stock is expected to achieve a total positive return of at least 15% over the next 12 months

Market Perform (2): The stock is expected to have a total return that falls between the parameters of an Outperform and Underperform over the next 12 months

Underperform (3): Stock is expected to achieve a total negative return of at least 10% over the next 12 months

Assumption: The expected total return calculation includes anticipated dividend yield

Cowen and Company Rating System until May 25, 2013

Outperform (1): Stock expected to outperform the S&P 500

Neutral (2): Stock expected to perform in line with the S&P 500

Underperform (3): Stock expected to underperform the S&P 500

Assumptions: Time horizon is 12 months; S&P 500 is flat over forecast period

Cowen Securities, formerly known as Dahlgren Rose & Company, Rating System until May 25, 2013

Buy – The fundamentals/valuations of the subject company are improving and the investment return is expected to be 5 to 15 percentage points higher than the general market return

Sell – The fundamentals/valuations of the subject company are deteriorating and the investment return is expected to be 5 to 15 percentage points lower than the general market return

Hold – The fundamentals/valuations of the subject company are neither improving nor deteriorating and the investment return is expected to be in line with the general market return

Cowen And Company Rating Definitions

Distribution of Ratings/Investment Banking Services (IB) as of 12/31/13

| Rating | Count | Ratings Distribution | Count | IB Services/Past 12 Months |
|----------|-------|----------------------|-------|----------------------------|
| Buy (a) | 415 | 59.20% | 68 | 16.39% |
| Hold (b) | 270 | 38.52% | 4 | 1.48% |
| Sell (c) | 16 | 2.28% | 1 | 6.25% |

(a) Corresponds to "Outperform" rated stocks as defined in Cowen and Company, LLC's rating definitions. (b) Corresponds to "Market Perform" as defined in Cowen and Company, LLC's ratings definitions. (c) Corresponds to "Underperform" as defined in Cowen and Company, LLC's ratings definitions.

Note: "Buy", "Hold" and "Sell" are not terms that Cowen and Company, LLC uses in its ratings system and should not be construed as investment options. Rather, these ratings terms are used illustratively to comply with FINRA and NYSE regulations.

Agios Pharmaceuticals Inc Rating History as of 03/05/2014

powered by: BlueMatrix



Legend for Price Chart:

I = Initiation | 1 = Outperform | 2 = Market Perform | 3 = Underperform | UR = Price Target Under Review | T = Terminated Coverage | \$xx = Price Target | NA = Not Available

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