Initiation
Neutral

AGIO, AGIO US
Price: $31.91

Price Target: $35.00

Agios Pharmaceuticals

Strong Science / Technology, However Value Creation Weighted to 2H14 / 1H15; Initiate at Neutral

We are initiating coverage of Agios Pharma (AGIO) with a Neutral rating and $35 Dec 2014 price target. The company's platform technology is rooted in very elegant and groundbreaking science with its focus on "flux biochemistry." This allows for the identification of targets that are likely to be critical components of biological pathways involved in cancer and orphan diseases – the two main therapeutic areas of focus at Agios. The company's three lead assets are early stage: AG-221 (IDH2m inhibitor) and AG-120 (IDH1m inhibitor), both for oncology indications, and AG-348 (PKR activator), for an orphan disease known as pyruvate kinase deficiency. While we are very impressed with the science, the management team, and the validating R&D collaboration with Celgene, the company is still early-stage. Indeed, AG-221 is only now entering a phase 1 study with AG-120 and AG-348 to follow in 2014, putting de-risking clinical data and meaningful value-creating events 12+ months away. We recognize the investor appetite for early-stage biotechs; our Neutral rating reflects our view that current valuation balances the strong science and potential for differentiated therapies with the development stage of the company.

- **Significant technology value supported by the scientific literature.** A large number of scientific publications on cell metabolism in oncology and the role of IDH in tumor proliferation provide validation of Agios's approach and the mechanism behind AG-221 and AG-120. Similarly, publications and internal data support the mechanism and opportunity for AG-348 in pyruvate kinase deficiency.

- **Collaboration with Celgene provides a good source of future non-dilutive R&D funding.** So far, Agios has received ~$141 million in cash and ~$50 million in an equity investment from the collaboration. Currently, Celgene has exclusive opt-in rights to AG-221 and AG-120, though Agios has US opt-in rights for the latter. Agios is also eligible for $120 million in milestones plus royalties on each program.

- **Data from lead programs weighted toward 2H14/2015.** Initial clinical data for AG-211, AG-120, and AG-348 are expected in the 2H14/1H15 time frame. In our view, the phase 1 AG-221 data will be important in de-risking the overall technology platform, as they will be the first human data on any development program (this, of course, assumes early clinical data mirror encouraging preclinical data thus far).

- **Neutral rating; $35 Dec 2014 PT.** Our YE'14 PT of $35 for AGIO is based on technology value from select clinical oncology/orphan biotech companies at a similar stage, plus net cash. Since Agios's programs are earlier stage, we apply a ~10% discount.

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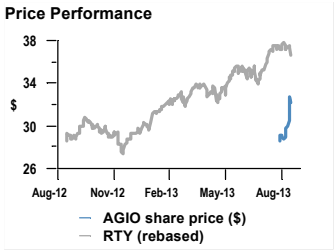
Price Performance



Agios Pharmaceuticals (AGIO;AGIO US)

FYE Dec	2012A	2013E	2014E	2015E
Analyst Adjusted Diluted EPS ($)				
Q1 (Mar)	-	(0.39)A	-	-
Q2 (Jun)	-	(0.39)	-	-
Q3 (Sep)	-	(0.31)	-	-
Q4 (Dec)	-	(0.32)	-	-
FY	(1.18)	(1.38)	(1.07)	(0.39)

Source: Company data, Bloomberg, J.P. Morgan estimates.

Company Data	
Price ($)	31.91
Date Of Price	16 Aug 13
52-week Range ($)	33.45-18.00
Market Cap ($ mn)	926.82
Fiscal Year End	Dec
Shares O/S (mn)	29
Price Target ($)	35.00
Price Target End Date	30-Dec-14

See page 20 for analyst certification and important disclosures.

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Agios Pharmaceuticals (AGIO)

Neutral

Investment Thesis

Strong science / technology supported by scientific literature…

Agios's core science focuses on identifying drug targets in deregulated metabolic pathways. In particular, with the use of high throughput mass spectrometry and the "flux biochemistry" approach, many drug targets have been identified that modulate critical biological pathways. Using this technology, the company's initial oncology assets target mutations in the enzymes isocitrate dehydrogenase 1 and 2, or IDH1 (AG-120) and IDH2 (AG-221), respectively. Additionally, Agios's IEM program targets deficiencies in the pyruvate kinase (PK) pathway (AG-348). Our literature review highlights supportive early-stage research related to cell metabolism, particularly mutations with IDH1 and IDH2, and the role in tumor proliferation, as well as mutations associated with PK-leading hemolysis. The scientific rationale appears very robust to us. Indeed, in an IDH2 AML animal model, AG-221 demonstrated a dose-dependent decrease in leukemia and a survival advantage compared with standard chemotherapy. We note that Agios's approach has advantages in that predictive metabolic biomarkers are identified and genetically defined patient populations can be targeted. This is a growing trend in drug development, particularly in oncology.

… Celgene collaboration provides validation; source of non-dilutive funding

In our view, the Celgene collaboration provides additional validation of Agios's approach. Of note, Agios has received $141.2 million in cash and ~$50 million in equity investment to date from this collaboration. Currently, Celgene has exclusive rights to AG-221 and AG-120, though Agios has US opt in rights for the latter. Agios is eligible for $120 million in milestones and royalties on both programs. Our understanding is that Celgene will make an opt-in decision once maximally tolerated dose (MTD) is identified for each program. We assume Agios will opt in for US rights to AG-120, post IND acceptance by the FDA.

That said, de-risking weighted toward the 2H14 / 1H15 time frame

Data from Agios's three lead programs are weighted toward 2H14 / 1H15, so there are fewer value creation catalysts in the near term based on human clinical data. AG-221 is currently in a phase 1 study in hematologic malignancies (first patient to be dosed in 3Q), while AG-120 will enter an initial phase 1 trial early-2014. Finally, for AG-348, a phase 1 trial is expected in 2014. In our view, the AG-221 phase 1 data will be important in de-risking the overall technology, as it will be the first human data. We believe the initial data from the program are likely to be presented at the 2014 American Society of Hematology meeting. In our view, Agios' lead programs all look very robust based on elegant science, which of course assumes that clinical data mirror the preclinical data thus far.

Valuation – appropriately reflects strong science foundation and the potential of differentiated therapeutics, with the early stage of the company

Our YE'14 price target of $35 for AGIO is based on a comparable company analysis. For comparables we use select biotech companies with a focus on oncology and/or orphan diseases. Our comparable group has a mean market cap of ~1.1B and a firm value of - ~890M. Since Agios is currently in preclinical development relative to our comparable companies in clinical development, we apply an appropriate ~10%

discount deriving a firm value of ~817M. Adding net cash of $225M we derive a value of $35/share (priced as of end of day 8/16/13).

Risks to Rating and Price Target

Clinical / Development Risk

Predicting the outcomes of clinical trials can be difficult, particularly when a program does not have de-risked early-stage clinical data. Indeed, currently, all supportive data for Agios programs, while having sound scientific rationale, are pre-clinical or just entering human clinic trials. As such, at this point, even establishing proof-of-concept for key programs is a source of clinical risk (AG-221, AG-120, and AG-348). Additionally, proof-of-concept can potentially be established in one or more programs, while later-stage clinical development is currently unclear and not yet established with the FDA (i.e., size and scope of trial).

Financial Risk

Following completion of its initial public offering, Agios has ~$225 million in cash. Of note, the company will not have commercial revenues until the 2017-18+ time frame, in our estimation. That said, Agios does have a research collaboration with Celgene that should provide non-dilutive cash in the near term. However, as the company continues to invest in its pipeline, cash levels will be important to monitor, particularly in the later stages of clinical development. Therefore, Agios may choose to raise additional capital, which could dilute current shareholders.

Regulatory Risk

There is no assurance in drug development that regulatory authorities will approve a novel therapeutic. Additionally, regulators may not agree with the later-stage development program of a particular compound, requiring a longer duration, larger size, or additional clinical trials.

Commercial Risk

Agios does not have experience commercializing therapeutics. Currently, Celgene can opt in for the global rights to AG-221 and AG-120, though Agios can opt into for US rights for the latter. AG-348 is an unencumbered asset. Thus, commercialization, given no prior experience, is a risk should the company decide to commercialize one or more assets on its own, in the US or globally.

Intellectual Property Risk

As of end-May 2013, Agios has ~30 pending US patent applications and ~120 pending foreign patent applications. However, no patents have been issued to date. Agios will need patents to protect key assets. An inability to receive issuance or defend patents is a risk to the business.

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Company Description

Agios, a biopharmaceutical company, focuses on cellular metabolism research, particularly in oncology/hematology and orphan diseases. The company uses its proprietary technology focused on cellular metabolism to identify novel drug targets. All of Agios's key programs are early stage. The lead oncology assets are AG-221 and AG-120 (various oncology/hematology indications), while the lead inborn errors of metabolism (IEM) asset is AG-348 for pyruvate kinase deficiency, an orphan disease. Of note, the company has a research collaboration with Celgene, from which Agios has received $141 million in cash and ~$50 million in equity investment to date. Post the IPO, in July, the company has ~$225 million in cash based on its 3/31/13 balance sheet.

Overview

Background and Pipeline / Catalysts

Agios was founded in 2007, with a focus on small molecule development based on cellular metabolism in oncology and inborn errors of metabolism (IEM). The company's technology revolves around "flux biochemistry" and identifying deregulated metabolic pathways. Via this technology, the initial oncology assets target mutations in the enzymes isocitrate dehydrogenase 1 and 2, or IDH1 (AG-120) and IDH2 (AG-221), respectively. Additionally, Agios's IEM program targets mutated pyruvate kinase enzyme (AG-348). Beyond the aforementioned assets, Agios is using the technology for early-stage research in both oncology and IEM.

Agios's initial public offering (IPO) took place in July 2013, and J.P. Morgan was a lead underwriter for the transaction. Post the IPO, the company has ~$225M in cash, based on its 3/31/13 balance sheet. The company estimates that its cash position to be sufficient through the late-2016 time frame, but we note this does not factor in future milestone payments from Celgene.

Key Pipeline Assets / Catalysts

The pipeline in both oncology and IEM is very early (see Table 1). The oncology pipeline is comprised of 2 key assets, AG-221 and AG-120. AG-221 is IDH2m inhibitor and is currently in a phase 1 study in IDH2 positive hematologic malignancies (patient dosing to begin in 3Q). The other oncology asset, AG-120 (an IDH1 inhibitor), will enter an initial phase 1 trial in early 2014. Finally, for AG-348, a phase 1 trial is also expected in 2014. Proof-of-concept data for all 3 compounds are expected in the 2H14 / 1H15 time frame, with data for AG-221 being first. In our view, the AG-221 data will be important in de-risking the overall technology as they will be the first patient data.

Table 1: Timelines for key valuation creation catalysts

Drug	Mechanism	Indication	Status	Proof-of-Concept Data
AG-221	IDH2m Inhibitor	Oncology	IND filed; Phase 1 initiated	2H14
AG-120	IDH1m Inhibitor	Oncology	Phase 1 early-2014	1H15
AG-348	PKR Activator	PKD	Phase 1 2014	2H14/1H15

Source: J.P. Morgan

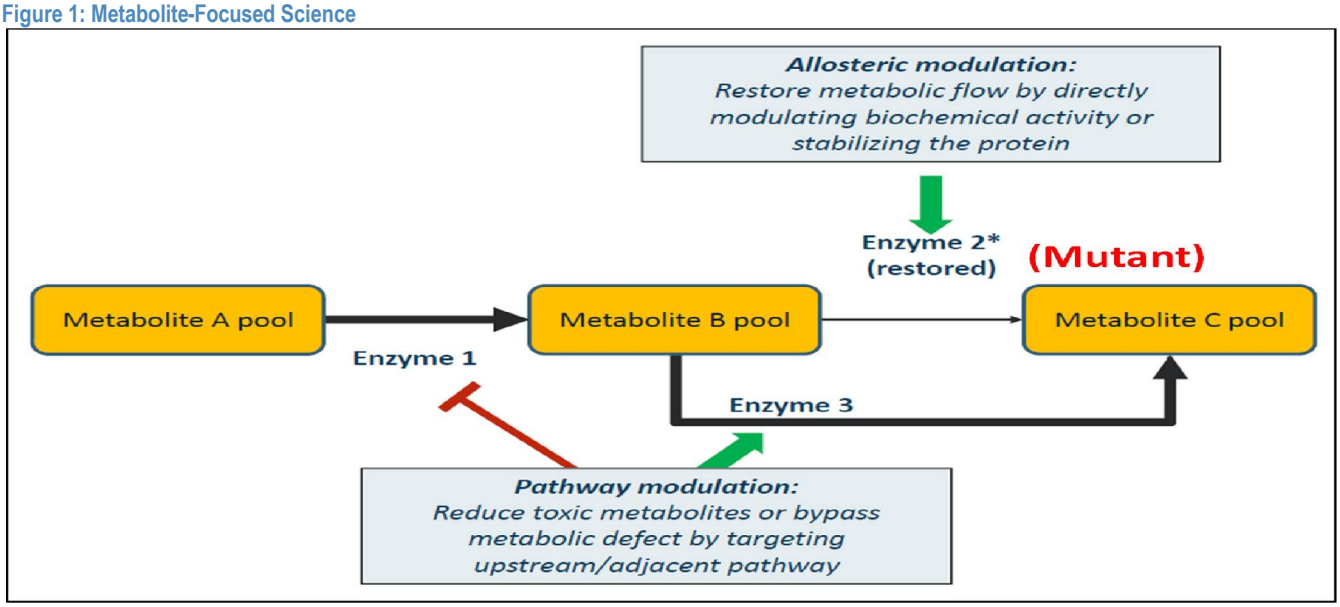
Scientific Rationale / Technology

Metabolite-Focused Science

Agios's core science revolves around metabolic enzyme drug discovery. In particular, high throughput mass spectrometry and the "flux biochemistry" technology (measures the speed with which metabolites accumulate or are removed in enzymatic pathways) allows for identification of deregulated enzymatic pathways that drive disease (see Figure 1). The approach has advantages in that predictive metabolic biomarkers are identified and genetically defined patient populations can be targeted. We note this is a growing trend in drug development, particularly in oncology.[1]

In oncology alone, our literature review highlights growing interest in tumor cell metabolism. Indeed, Cairns et al noted that mutations in oncogenes / tumor suppessor genes can impact cell metabolism and enhance proliferation (specifics on IDH1 and IDH2 mutations are discussed in more detail in subsequent pages).[2] Additionally, our literature review highlights that mutations associated with pyruvate kinase (PK) can lead to hemolysis (i.e., destruction of red blood cells).[3]

Figure 1: Metabolite-Focused Science



Source: Adapted from Agios company presentation

Intellectual Property

As of end-May 2013, Agios currently has ~30 pending US patent applications and ~120 pending foreign patent applications. However, no patents have been issued to date (though some key patents have been published). Based on our conversation with the company, key patents, when issued, would expire during the 2027-34 time frame.

[1] Hayashi et al. Journal of Clinical Pharmacy and Therapeutics. 38: 62-67 (2013)
[2] Cairns et al. Nature Reviews. 2: 85-95 (2011)
[3] Diez et al. Blood. 106: 1851-1856 (2005)

Celgene Collaboration

In April 2010, Agios entered into an R&D collaboration agreement with Celgene focused on targeting cancer metabolism. Agios is responsible for research and development through phase 1. The discovery collaboration ends in April 2014, though Celgene can extend the collaboration by one year in 2014 and 2015. For each year extended, Agios will receive a $20 million extension payment. Our model assumes Celgene will extend the collaboration in both 2014 and 2015. To date, the company has received $141.2 million in payments from Celgene and ~$50 million in equity investments.

In our view, the Celgene collaboration provides validation to Agios's propriety technology. Currently, Celgene has exclusive rights to AG-221 and AG-120, though Agios has US opt-in rights for the latter. Agios is also eligible for $120 million in milestones and low- to mid-teens royalties on each program. For AG-221, Celgene would be fully responsible for global development and commercialization costs if the company moves forward with the program. However, for AG-120, should Celgene opt to move forward with development and Agios opts to retain US rights for the compound, developments costs will be split equally.

In September 2012 and March 2013, Agios completed the development candidate requirements for the Celgene collaboration for AG-221 and AG-120, respectively. Our understanding is that Celgene will make its opt-in decision once maximally tolerated dose (MTD) is identified for each program (before expansion cohort phase). That said, Agios would have to make a decision on AG-120 after IND acceptance by the FDA. Should the company decide NOT to opt in to the rights for AG-120, Agios would have the option to retain US rights to the Glutaminase program (pre-clinical). Given the market opportunity for AG-120 (3X the size of AG-221), we believe it is likely Agios will opt in for US rights to the compound.

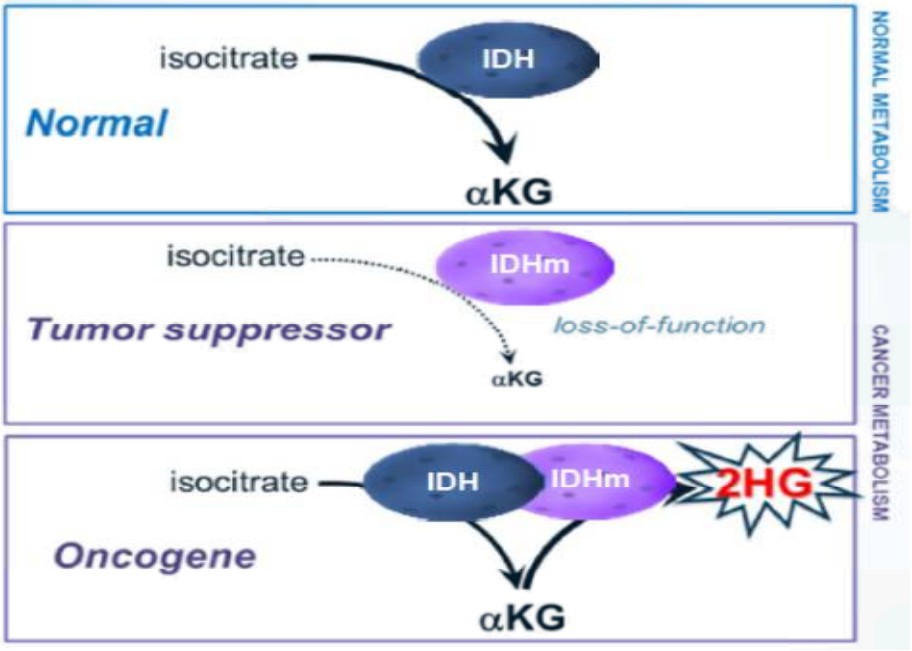
AG-221 (IDH2m Inhibitor)

AG-221 is an IDH2 mutant inhibitor and Agios's latest-stage development candidate. Recall, an IND for the program was submitted in late June 2013. Our sense from talking to Agios is that the company is in the process of clinical trial site activation (i.e., IRB approval process, etc.) and that the first patient is on track to dose in 3Q. We believe the initial data from the program are likely to be presented at the 2014 American Society of Hematology meeting.

- **Scientific rationale:** Isocitrate dehydrogenase (IDH) is a metabolic enzyme that converts isocitrate to α-ketoglutarate (α-KG), a key intermediary metabolite in the Krebs Cycle.[4] However, IDH enzyme mutations lead to an increase in another metabolite, 2-hydroxyglutarate (2-HG).[5] It is thought that elevated levels 2-HG contributes to malignant transformation.[6] We note both IDH1 and IDH2 mutations lead to increase 2-HG (see Figure 2).[3]

[4] Wang et al. Science. 340: 622-626 (2013)
[5] Yen et al. The Oncologist. 17: 5-8 (2012)
[6] Figueroa et al. Cancer Cell. 19: 553-567 (2010)

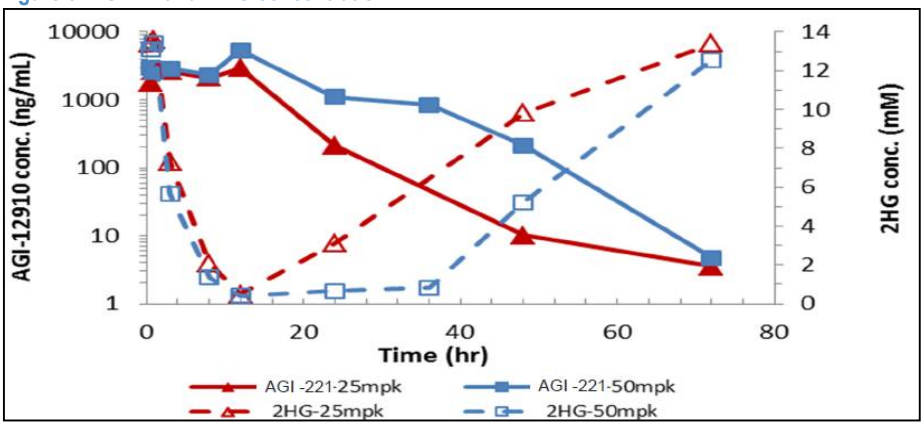
Figure 2: IDH1 and IDH2 mutations leading to 2-hydroxyglutarate increase



Source: Agios company presentation

- **Supportive preclinical data:** Both IDH1 and IDH2 mutations are thought to be associated increases in 2-HG in AML (a potential lead indication for the program).[7] Of note, our literature review shows plenty of recent publications to support the role of IDH1/IDH2 mutations in AML. Agios has shown in pre-clinical models that AG-221 (AG-12910) decreases 2-HG concentrations (see Figure 3). Additionally, in an IDH2 AML animal model, AG-221 demonstrated a dose-dependent decrease in leukemia and a survival advantage compared with standard chemotherapy, with highest drug dose cohort all living until study completion (see Figure 4).

Figure 3: AG-221 and 2-HG concentration



Source: Agios company presentation

[7] Gross et al. Journal of Experimental Medicine. 207: 339-344 (2010)

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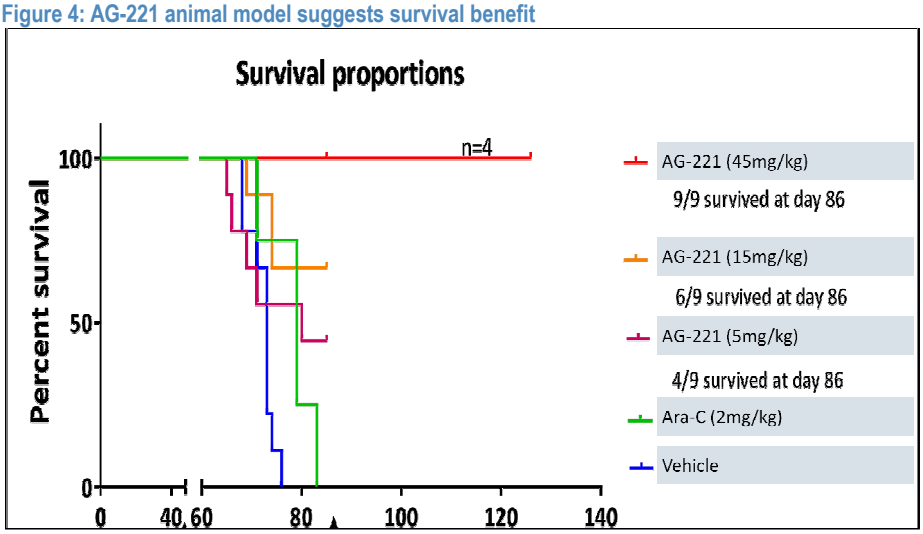
Figure 4: AG-221 animal model suggests survival benefit



Source: Agios company presentation

- **Next steps:** Agios has initiated a phase 1 study in hematological malignancies, and the first patient dosed will be in 3Q (see Table 2). Based on addressable patient population with IDH2 mutations, we suspect that the majority of subjects enrolled in the trial will be AML patients (see Table 3). The primary goal of the study will be to assess the safety and tolerability, as well determine a maximally tolerated dose (MTD) for subsequent studies / expansion cohorts. Secondary objectives include dose-limiting toxicities (DLTs), PK / PD, 2-HG concentration decreases, and clinical activity (i.e., overall response rate, etc.).

Table 2: Phase 1 AG-221 trial design

Title	A Phase 1 Study of AG-221, a Small Molecule Inhibitor of Mutant IDH2, in Patients with Advanced Hematologic Malignancies
Trial size	57
Dosing	AG-221 BID on days 1-28
Patient Population	R/R AML patients; R/R MDS patients; untreated AML ≥60 but not a candidate for standard therapy
Primary Objectives	Safety / Tolerability Determine MTD
Secondary Objectives	Assess dose-limiting toxicities PK / PD Clinical activity (i.e., ORR, CR, PR etc). 2-HG levels
Data Timelines	2H14 Initial data likely to be at ASH 2014

Source: Agios company presentations; clinicaltrials.gov

- **When are proof-of-concept data expected?** We anticipate proof-of-concept data in the 2H14 time frame. Outside of safety and establishing an MTD, we believe 2-HG levels and clinical activity will be important to monitor. In the phase 1 study of AG-221, we would be particularly focused on reduction of 2-HG levels, as this would further validate the scientific approach and confer observations in preclinical models. Of note, our sense is that clinical activity could be difficult to gauge in sub-therapeutic doses cohorts initially.
- **Market opportunity:** Given the early stage of the program, we do not currently have a market build for AG-221. That said, we believe the addressable population in AML and MDS, i.e., the studied population in phase 1, is about ~10K in the US, EU and Japan (see Table 3). For reference, assuming orphan pricing of $200K and 60-65% market penetration, we estimate peak market opportunity for AG-221 could approach $1.2-1.3 billion in AML and MDS/MPN alone.

Table 3: AG-221 addressable patient population

Disease	% IDH2 mutation	Addressable patient population
Acute Myeloid Leukemia (AML)	15%	7,200
MDS/MPN	5%	2,000
Angio-immunoblastic NHL	25%	400
Others* (melanoma, glioma, chondro)	3-5%	1,500
Type II D-2HG Aciduria	100%	50 reports

Source: Agios company presentation

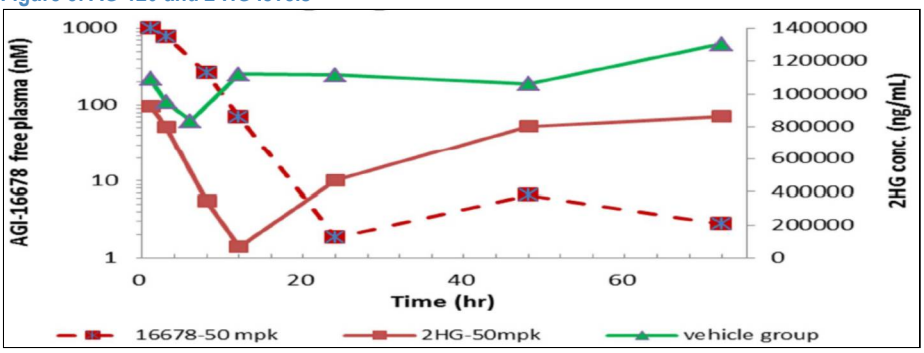
AG-120 (IHD1m Inhibitor)

AG-120 is an IDH1 inhibitor that is expected to enter the clinical in early 2014 in both oncology and hematology indications that are IHD1 mutant positive. At this point the phase 1 indication has not been selected, though given literature review and from speaking to the company, our sense is that the indications that are most likely to be included are low-grade glioma/secondary GBM, chondrosarcoma and AML / MDS.

- **Scientific rationale:** As we highlighted above, both IDH1 and IDH2 mutations lead to increase 2-HG (see Figure 2).[3]
- **Supportive preclinical data:** Our literature review shows plenty of supportive evidence for IDH1 mutations resulting in increased 2HG levels. In particular, there is strong support that IHD mutations may promote growth in glioma cells, and malignant glioma cells have evaluated 2-HG levels.[8,9] Agios has shown in pre-clinical models that AG-120 (AGI-16678) decreases 2-HG levels over time (see Figure 5).

[8] Rohle et al. Science. 340: 626-630
[9] Dang et al. Nature. 462 (7274): 739-744

Figure 5: AG-120 and 2-HG levels



Source: Agios company presentation

- **Next steps:** An IND is expected to be submitted in early 2014 and phase 1 trials expected to start in the same time frame.
- **When are proof-of-concept data expected?** We anticipate proof-of-concept data in the 1H15 time frame.
- **Market opportunity:** At this point the lead indication for the AG-120 program is unclear. That said, the addressable patient population for AG-120 size is 3X the AG-221 program (see Table 4). Thus, we estimate the agent has also blockbuster potential.

Table 4: AG-120 addressable patient population

Disease	% IDH1 mutation	Addressable patient population
Low grade glioma & 2ary GBM	70%	11,000
Chondrosarcoma	>50%	4,600
Acute Myeloid Leukemia (AML)	8%	3,600
MDS/MPN	5%	2,000
Intrahepatic Cholangiocarcinoma	20%	1,600
Others	1-2%	8,000+

Source: Agios company presentation

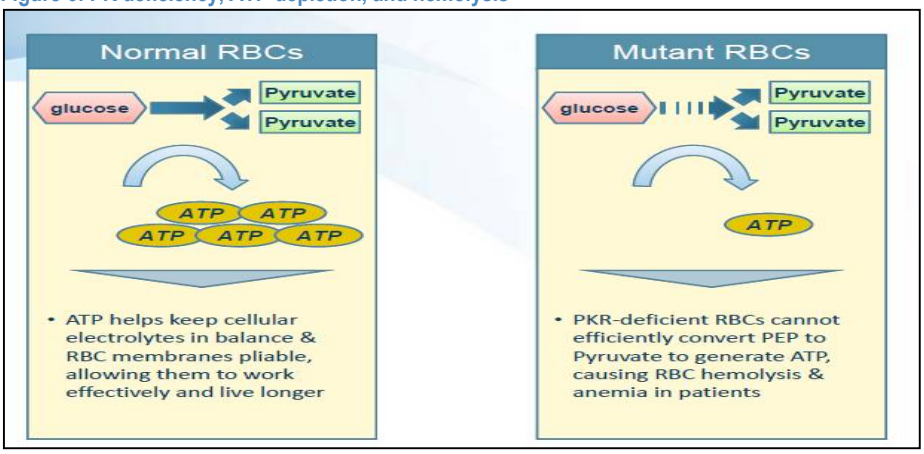
AG-348 (PKR activator)

A phase 1 trial of AG-348 in pyruvate kinase (PK) deficiency is expected to start in 2014. There are currently no approved therapies for PK deficiency. Agios has initiated IND enabling studies. Of note, the company owns the global rights to AG-348.

- **Scientific rationale:** PK deficiency is caused by mutations in the PK-LR gene.[10] In particular, PK deficiency leads to depletion of adenosine triphosphate (ATP), which results in hemolysis (see Figure 6). Indeed, a key clinical manifestation of PK deficiency is anemia.[2]

[10] Zanella et al. British Journal of Hematology. 130: 11-25 (2005)

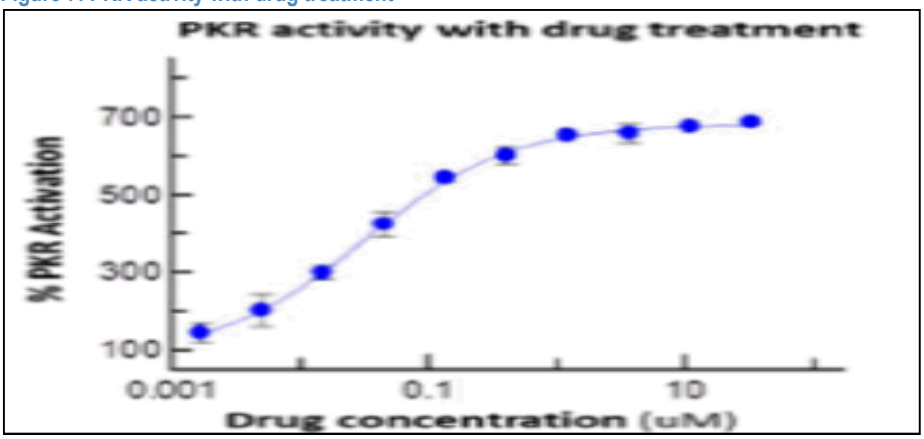
Figure 6: PK deficiency, ATP depletion, and hemolysis



Source: Agios company presentation

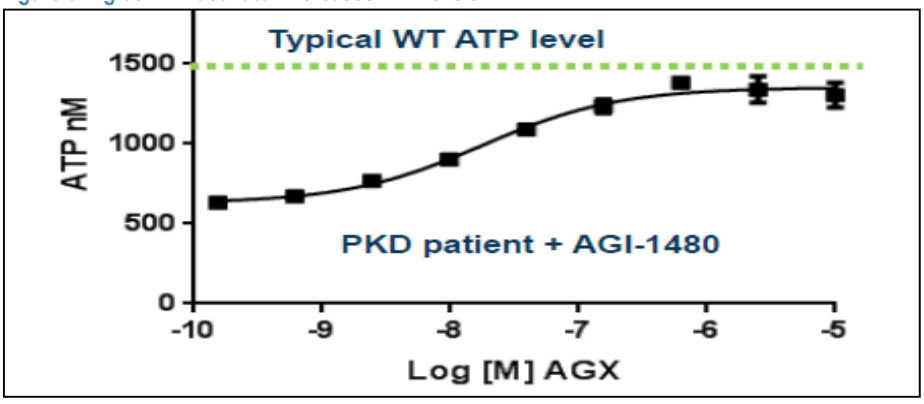
- **Supportive preclinical data:** Agios has shown the company's PKR activator both increases PK activity and restores ATP levels (see Figure 7 and Figure 8).

Figure 7: PKR activity with drug treatment



Source: Agios company presentation

Figure 8: Agios PKR activator increases ATP levels



Source: Agios company presentation

- **Next steps:** A phase 1 trial is expected to start in 2014. Based on our conversation with Agios, the trial could include healthy volunteers or patients. Our sense is that later-stage trials could assess rate of transfusion independence.
- **When are proof-of-concept data expected?** Our sense is that a the initial data from a phase 1 study could be available in the 2H14/1H15 time frame.
- **Market opportunity:** The epidemiology of PK deficiency is still a bit of black box. However, this does not come as a surprise, in our view, given the orphan nature of the disease. Agios currently estimates there are currently 1,000-3000 and 1,500-4,500 patients in the US and EU, respectively. AG-348 also has blockbuster potential in our estimation.

Financial Outlook

Income Statement

We currently do not include any product revenues in our model as the pipeline is in early stages of development. However, we do include revenues associated with the Celgene collaboration and funding from research grants. We project 2013-17 total revenues of $25M, $35M, $65M, $31M and $60M, respectively (see Table 6). We expect 2013-17 R&D expenses to increase only modestly, as we assume Celgene will be responsible for later-stage development for AG-221 and 50% of AG-120. Additionally, we assume only a modest increase in 2013-17 SG&A since we do not anticipate any product launches during this period. We project 2013-17 GAAP EPS of $(1.38), $(1.07), $(0.39), $(1.37) and $(0.69), respectively.

Balance Sheet and Cash Flows

At the end of March 2013, Agios had $116M in cash and equivalents. Following the IPO completed in July, we estimate Agios currently has ~$225M in cash and equivalents, based on its end-March 2013 balance sheet (see Table 7 and Table 8). We currently assume no additional equity raises near term.

Valuation

Our year-end 2014 price target of $35 for AGIO is based on a comparable company analysis. For comparables we have selected other biotech companies with a focus on oncology and/or orphan disease. Our comparable group has a mean market cap of ~1.1B and a firm value of ~890M. Since Agios is currently in preclinical development relative to our comparable in clinical development we apply an appropriate ~10% discount deriving a firm value of $817M. Adding net cash of $225M we derive a value of $35/share (see Table 5).

Table 5: Agios Valuation Metrics

Company	Ticker	Rating	Analyst	Stock Price	Shares Outstanding	Market cap ($M)	Cash ($M)	Debt ($M)	Firm Value ($M)	Phase of Development
Alnylam	ALNY	OW	Meacham	$48.26	63	$3,038	$236	$0	$2,802	Ph2
Bluebird Bio	BLUE	OW	Kasimov	$29.24	24	$694	$237	$0	$457	Ph2/3
ChemoCentryx	CCXI	OW	Meacham	$12.03	43	$515	$76	$0	$439	Ph2/3
Clovis Oncolgy	CLVS	OW	Kasimov	$67.05	30	$2,023	$390	$0	$1,633	Ph1
Epizyme	EPZM	NC	-	$31.34	28	$891	$165	$0	$726	Ph1
Infinity Pharmaceuticals	INFI	OW	Kasimov	$17.81	48	$855	$303	$0	$552	Ph2
Merrimack Pharmaceuticals	MACK	OW	Meacham	$3.34	102	$341	$195	$162	$309	Ph2/3
PTC Therapeutics	PTCT	OW	Meacham	$15.66	25	$390	$168	$4	$227	Ph3
Prosensa	RNA	OW	Gordon	$26.25	35	$919	$150	$9	$778	Ph3
Sarepta Therpeutics	SRPT	NC	-	$33.08	34	$1,109	$168	$3	$944	Ph2
Synageva	GEVA	N	Mecham	$46.16	27	$1,268	$309	$0	$959	Ph3
Min						$341	$76	$0	$227	
Max						$3,038	$390	$162	$2,802	
Mean						$1,095	$218	$16	$893	
Agios (Min)	AGIO			$14.98	30	$451	$225	$0	$227	
Agios (Max)	AGIO			$100.48	30	$3,027	$225	$0	$2,802	
Agios (Mean)	AGIO			$37.11	30	$1,118	$225	$0	$893	
Discount				$35	30	$1,042	$225	$0	$817	

Source: J.P. Morgan estimates.; Bloomberg. Note: Priced as of 8/16/13-.

Management

David Schenkein, MD – Chief Executive Officer

Dr. Schenkein joined Agios in August 2009, as CEO and member of the Board of Directors. He has been a hematologist/oncologist for 20+ years. Prior to Agios, Dr. Schenkein was SVP of clinical hematology/oncology at Genentech and SVP of clinical research at Millennium, where he oversaw the approval many therapeutics, including Velcade. Dr. Schenkein holds a BA in chemistry from Wesleyan University and MD from State University of New York Upstate Medical School.

Duncan Higgons – Chief Operating Officer

Mr. Higgons joined Agios in May 2009, as COO. Prior to Agios, he has held management positions as COO, Chief Commercial Officer and interim CEO at Archemix Corporation and TransForm Pharmaceuticals. Mr. Higgons holds a BSc in mathematics from King's College University of London and MSc in economics from London Business School.

Scott Biller, PhD – Chief Scientific Officer

Dr. Biller joined Agios in September 2010, as CSO. Prior to Agios, he was VP / head of global chemistry discovery at Novartis Institutes for Biomedical Research and VP of pharmaceutical candidate optimization, as well as executive director of drug discovery chemistry, at Bristol Myers Squibb. Dr. Biller holds a BS in chemistry from MIT and PhD in organic chemistry from Caltech.

Michael Su, PhD – Senior Vice President Research & Development

Dr. Su was a founder and now has served as SVP of R&D (since 2012). Prior to Agios, he was general director/VP of the Biomedical Engineering Research Laboratory at ITRI in Taiwan, as well program executive/VP for the Novartis kinase collaboration for Vertex. Dr. Su holds a PhD in biochemistry from Duke University.

Glenn Goddard, CPA – Vice President Finance

Mr. Goddard joined Agios in July 2010, as VP of finance. Prior to Agios, he worked as VP of finance at Archemix and corporate controller at ImmunoGen. Mr. Goddard holds a BS in accounting from Bentley College and is a certified public accountant in the state of Massachusetts.

John Evans - Vice President Development & Operations

Mr. Evans joined Agios in September 2009, as VP of Development and Operations. Prior to Agios, he severed a director of product development at Infinity Pharmaceuticals, as well as positions at MedImmune, Bayer, and the pharmaceuticals practice of Mckinsey and Company. Mr. Evans holds a BA in English from Yale, a Masters in biotechnology from University of Pennsylvania, and MBA from Wharton.

Camille Henderson. PhD – Director of Human Resources

Dr. Henderson joined Agios in 2010. Prior to Agios, she worked at Genentech conducting human resources strategy. Dr. Henderson holds a PhD in history with a focus on change management / race theory from the University of Chicago.

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Financial Statements

Table 6: Income Statement, 2011A-2017E

	2011A	2012A	2013E	2014E	2015E	2016E	2017E
Revenues							
Product Revenues			0.0	0.0	0.0	0.0	0.0
Collaboration / Grant Revenues / Milestones	21.8	25.1	25.2	35.2	65.2	31.3	60.0
Total Revenues	**21.8**	**25.1**	**25.2**	**35.2**	**65.2**	**31.3**	**60.0**
Operating Expenses							
Cost of sales	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Research and development	31.3	41.0	49.5	56.9	62.6	65.7	69.0
Sales, general and administrative	7.2	7.1	7.9	8.6	9.1	9.5	10.0
Total Operating expenses	**38.5**	**48.1**	**57.3**	**65.5**	**71.6**	**75.2**	**79.0**
Operating Income	**(16.6)**	**(23.0)**	**(32.1)**	**(30.4)**	**(6.5)**	**(43.9)**	**(19.0)**
Interest Income	0.1	0.1	0.0	0.0	0.0	0.0	0.0
Interest expense	(7.2)	2.8	(0.8)	(0.8)	(0.8)	(0.8)	(0.8)
Other expense	(3.1)	(7.2)	(7.2)	(7.2)	(7.2)	(7.2)	(7.2)
Total Other Income	(10.2)	(4.3)	(7.9)	(7.9)	(7.9)	(7.9)	(7.9)
Pretax Income	(26.8)	(27.3)	(40.1)	(38.3)	(14.4)	(51.8)	(26.9)
Income tax (benefit)	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Net Income (loss)	(26.8)	(27.3)	(40.1)	(38.3)	(14.4)	(51.8)	(26.9)
GAAP EPS	**(8.90)**	**(1.18)**	**(1.38)**	**(1.07)**	**(0.39)**	**(1.37)**	**(0.69)**
Diluted Shares outstanding	*3.0*	*23.1*	*29.0*	*35.7*	*36.7*	*37.7*	*38.7*

Source: J.P. Morgan estimates, Agios

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Table 7: Balance Sheet, 2011-2015E

	2011A	2012A	2013E	2014E	2015E
	FY	FY	FY	FY	FY
Assets					
Cash and cash equivalents	117.7	91.3	182.6	140.4	126.8
Marketable securities	61.5	36.7	36.7	36.7	36.7
Prepaid expenses and other current assets	0.8	0.9	1.1	1.3	1.6
Deferred tax assets	10.6	1.2	1.2	-	-
Total Current Assets	*190.6*	*130.1*	*221.7*	*178.4*	*165.1*
Property and equipment, net	3.2	3.6	3.9	4.2	4.6
Restricted cash	0.6	0.6	0.6	0.6	0.6
Deferred tax assets, net of current portion	0.0	2.7	2.7	-	-
Other assets	0.1	0.0	0.0	0.0	0.0
Total Long Term Assets	*3.9*	*6.9*	*7.19*	*4.84*	*5.24*
Total Assets	**194.47**	**137.0**	**228.87**	**183.21**	**170.35**
Current Liabilities					
Accounts payable	3.6	3.3	3.3	3.3	3.3
Accrued expenses	1.5	1.7	1.7	1.7	1.7
Income taxes payable	17.9	4.9	4.9	0.0	0.0
Deferred revenue	25.1	25.1	25.1	25.1	25.1
Deferred rent	0.0	0.1	0.1	0.1	0.1
Restricted stock liability	0.1	0.1	0.1	0.1	0.1
Total Current Liabilities	*48.2*	*35.1*	*35.1*	*30.2*	*30.2*
Deferred revenue, net of current portion	82.7	57.6	57.6	57.6	57.6
Deferred rent, net of current portion	0.4	0.3	0.3	0.3	0.3
Restricted stock liability, net of current portion	0.0	0.0	0.0	0.0	0.0
Total Long Term Liabilities	*83.2*	*58.0*	*58.00*	*58.00*	*58.00*
Total Liabilities	**131.3**	**93.1**	**93.11**	**88.24**	**88.24**
Series A convertible preferred stock, $0.001 par value; 33,188,889 shares authorized	32.9	32.9			
Series B convertible preferred stock, $0.001 par value; 5,190,551 shares authorized	5.7	5.7			
Series C convertible preferred stock, $0.001 par value; 15,882,389 shares authorized	77.3	77.3			
Common stock, $0.001 par value; 75,000,000, 78,300,000, and 78,300,000 shares authorized at December 31, 2011 and 2012, and March 31, 2013 (unaudited), respectively	0.0	0.0			
Additional paid-in capital	1.1	2.0			
Accumulated other comprehensive income (loss)	0.0	(0.0)			
Accumulated deficit	(53.9)	(74.0)			
Total Shareholders' Equity	**63.1**	**43.9**	**135.76**	**94.98**	**82.12**
Total Liabilities and Equity	**194.47**	**137.0**	**228.87**	**183.21**	**170.35**

Source: J.P. Morgan estimates, Agios

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Table 8: Cash Flow, 2011A-2015E

	2011A	2012E	2013E	2014E	2015E
	FY	FY	FY	FY	FY
Net loss	(23.71)	(20.10)	(40.06)	(38.26)	(14.38)
Depreciation	0.8	1.2	1.3	1.4	1.6
Net loss on disposal of fixed assets	0.0	0.0	0.0	0.0	0.0
Stock-based compensation expense	0.4	0.7	0.7	0.7	0.7
Deferred rent	0.2	(0.0)	(0.0)	(0.0)	(0.0)
Deferred taxes	(10.7)	6.7	6.7	0.0	0.0
Amortization (accretion) of premium (discount) on investments	0.4	0.3	0.3	0.3	0.3
Changes in operating assets and liabilities:					
Prepaid expenses and other assets	0.1	(0.1)	0.2	0.2	0.3
Accounts payable	0.8	(0.3)	0.0	0.0	0.0
Accrued expenses and other liabilities	0.5	0.2	0.0	0.0	0.0
Income taxes payable	17.9	(13.0)	0.0	(4.9)	0.0
Deferred revenue	(1.8)	(25.1)	0.0	0.0	0.0
Net change in Working Capital	17.5	(38.3)	0.2	(4.7)	0.3
Net Cash From Operations	**(15.09)**	**(49.55)**	**(30.88)**	**(40.50)**	**(11.56)**
Purchases of marketable securities	(105.9)	(88.52)			
Proceeds from maturities and sales of marketable securities	85.5	113.04	123.85		
Purchases of property and equipment	(1.9)	(1.48)	(1.62)	(1.78)	(1.96)
Purchase of long-term investment					
Net Cash from Investing	**(22.36)**	**23.04**	**122.23**	**(1.78)**	**(1.96)**
Net proceeds from issuance of Series C convertible preferred stock	**77.30**	-			
Net proceeds from stock option exercises and issuance of common and restricted common stock	0.06	0.14			
Repayment of capital lease and note obligations					
Net Cash from Financing	**77.36**	0.14	-	-	**-**
Net Increase (Decrease) in Cash	**39.91**	**(26.36)**	**91.35**	**(42.28)**	**(13.52)**
Cash and cash equivalents at beginning of period	**77.88**	**117.66**	**91.30**	**182.65**	**140.36**
Cash and cash equivalents at end of period	**117.66**	**91.30**	**182.65**	**140.36**	**126.84**

Source: J.P. Morgan estimates.

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Agios Pharmaceuticals: Summary of Financials

Income Statement - Annual	FY12A	FY13E	FY14E	FY15E
Revenues	25	25	35	65
Cost of products sold	0	0	0	0
Gross profit	-	-	-	-
SG&A	(7)	(8)	(9)	(9)
R&D	(41)	(49)	(57)	(63)
Operating income	(23)	(32)	(30)	(6)
EBITDA	(23)	(32)	(30)	(6)
Net interest (income) / expense	(4)	(8)	(8)	(8)
Other income / (expense)	-	-	-	-
Income taxes	0	0	0	0
Net income - GAAP	(27)	(40)	(38)	(14)
Net income - recurring	(27)	(40)	(38)	(14)
Diluted shares outstanding	23	29	36	37
EPS - excluding non-recurring	(1.18)	(1.38)	(1.07)	(0.39)
EPS - recurring	(1.18)	(1.38)	(1.07)	(0.39)

Balance Sheet and Cash Flow Data	FY12A	FY13E	FY14E	FY15E
Cash and cash equivalents	91	183	140	127
Accounts receivable	-	-	-	-
Inventories	-	-	-	-
Other current assets	2	2	1	2
Current assets	130	222	178	165
PP&E	4	4	4	5
Total assets	137	229	183	170
Total debt	-	-	-	-
Total liabilities	93	93	88	88
Shareholders' equity	44	136	95	82
Net income (including charges)	(20)	(40)	(38)	(14)
D&A	1	1	1	2
Change in working capital	(38)	0	(5)	0
Other	8	8	1	1
Cash flow from operations	(50)	(31)	(40)	(12)
Capex	(1)	(2)	(2)	(2)
Free cash flow	(47)	(25)	(34)	(6)
Cash flow from investing activities	23	122	(2)	(2)
Cash flow from financing activities	0	0	0	0
Dividends	-	-	-	-
Dividend yield	-	-	-	-

Income Statement - Quarterly	1Q13A	2Q13E	3Q13E	4Q13E
Revenues	6A	6	6	6
Cost of products sold	0A	0	0	0
Gross profit	-	-	-	-
SG&A	(2)A	(2)	(2)	(2)
R&D	(11)A	(12)	(13)	(14)
Operating income	(7)A	(7)	(9)	(9)
EBITDA	(7)A	(7)	(9)	(9)
Net interest (income) / expense	(2)A	(2)	(2)	(2)
Other income / (expense)	-	-	-	-
Income taxes	0A	0	0	0
Net income - GAAP	(9)A	(9)	(11)	(11)
Net income - recurring	(9)A	(9)	(11)	(11)
Diluted shares outstanding	23A	23	35	35
EPS - excluding non-recurring	(0.39)A	(0.39)	(0.31)	(0.32)
EPS - recurring	(0.39)A	(0.39)	(0.31)	(0.32)

Ratio Analysis	FY12A	FY13E	FY14E	FY15E
Sales growth	15.0%	0.2%	39.7%	85.3%
EBIT growth	38.3%	39.8%	(5.6%)	(78.7%)
EPS growth - recurring	(86.7%)	16.9%	(22.3%)	(63.4%)
Gross margin	-	-	-	-
EBIT margin	(91.6%)	(127.7%)	(86.3%)	(9.9%)
EBITDA margin	(91.6%)	(127.7%)	(86.3%)	(9.9%)
Tax rate	0.0%	0.0%	0.0%	0.0%
Net margin	(108.7%)	(159.2%)	(108.8%)	(22.1%)
Net Debt / EBITDA	-	-	-	-
Net Debt / Capital (book)	-	-	-	-
Return on assets (ROA)	(16.5%)	(21.9%)	(18.6%)	(8.1%)
Return on equity (ROE)	(51.0%)	(44.6%)	(33.2%)	(16.2%)
Enterprise value / sales	-	-	-	-
Enterprise value / EBITDA	-	-	-	-
Free cash flow yield	(6.3%)	(2.7%)	(3.0%)	(0.5%)

Source: Company reports and J.P. Morgan estimates.
Note: $ in millions (except per-share data).Fiscal year ends Dec

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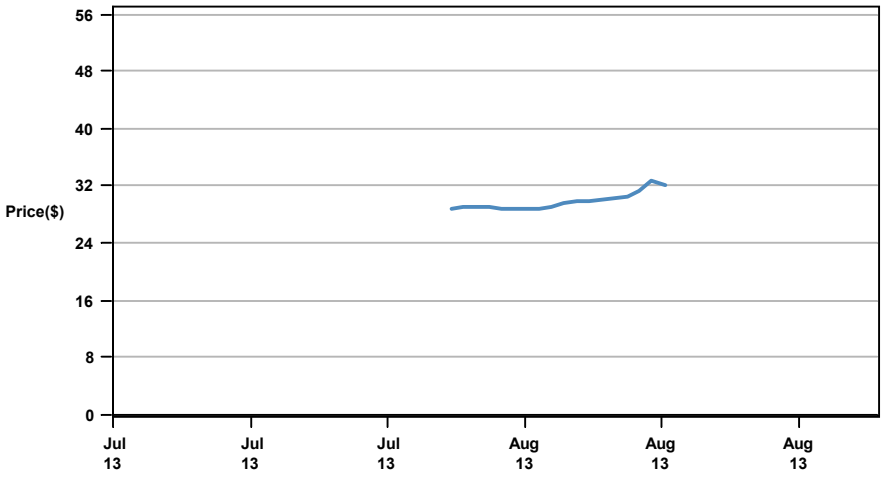
Agios Pharmaceuticals (AGIO, AGIO US) Price Chart



Source: Bloomberg and J.P. Morgan; price data adjusted for stock splits and dividends.

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IB clients*	76%	66%	55%

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