March 7, 2014

OUTPERFORM

Reason for report:

EARNINGS

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LEERINK

AGIOS PHARMACEUTICALS, INC.

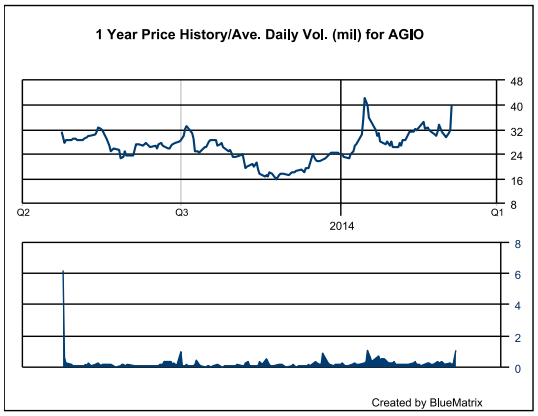
Earlier-Than-Expected Initial Clinical Data at AACR May Support Platform

• **Bottom Line:** AGIO announced initial Phase I data presentation for the lead candidate AG-221 in IDH2 mutant hematologic malignancies at the upcoming AACR (American Association for Cancer Research) conference. Although the abstract is still not available (to be released at 1pm, April 4), the data come substantially ahead of prior guidance (ASH) and in light of a prior management statement that it only intends to present meaningful clinical data, it appears to us that some activity has already been observed while the dose escalation continues and a maximal tolerated dose (MTD) has not been reached. We believe potential proof of principle data on the lead IDH2 inhibitor could have positive read-through on the IDH1 program (entering Phase I in early 2014) and could potentially provide valuable early clinical validation for AGIO's broader technology platform in cancer metabolism. With two more assets on track entering clinical development (AG-120, IDH1 inhibitor and AG-348, PKR activator), we believe AGIO is transforming into an interesting clinical story in 2014 with good amount of news flow in the next 1-2 years. We are increasing our valuation from $38 to $51 based on higher probability of success in clinical programs.

• **AG-221 initial data could be promising.** The abstract entitled, "Clinical safety and activity in a Phase I trial of AG-221, a first in class, potent inhibitor of the IDH2-mutant protein, in patients with IDH2 mutant positive advanced hematologic malignancies" will be presented at 3:45-4:05pm on Sunday, April 6 as one of 4 talks in the Clinical Trials Symposium, "Novel Immune and Targeted Therapies for Hematologic Malignancies and Solid Tumors" (LINK). AACR stated to us that the abstract will be released at 1pm on Friday, April 4. Management noted that majority of the patients enrolled so far were AML (acute myeloid leukemia) patients bearing IDH2 mutations. However, the extension cohorts for all three indications (AML, MDS – myelodysplastic syndromes and MPD – myeloid proliferative disorders) are expected to start later in 2014.

• **On track to initiate two trials for AG-120 (IDH1).** AGIO is on track to initiate two Phase I trials in solid tumor and hematologic malignancies in early 2014. We believe positive IDH2 data could have positive read through to the AG-120 given similar mechanism of action. Timing for the 2nd generation IDH1 inhibitor (better penetration to the blood-brain barrier [BBB]) is still unclear and management noted current focus for the IDH1 program remains AG-120.

Key Stats:	**(NASDAQ:AGIO)**
S&P 600 Health Care Index:	**1,311.61**
Price:	**$31.91**
Price Target:	$51.00 from $38.00
Methodology:	NPV and Sum of the parts
52 Week High:	$33.45
52 Week Low:	$18.00
Shares Outstanding (mil):	30.3
Market Capitalization (mil):	$966.9
Book Value/Share:	$0.16
Cash Per Share:	$7.42
Dividend (ann):	$0.00
Dividend Yield:	0.0%



Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2013A	**$6.3**	**$6.3**	**$6.3**	**$6.7**	**$25.4**	**($0.39)**	**($2.80)**	**($0.52)**	**($0.40)**	**($2.83)**	**NM**
2014E - New	**$6.7**	**$2.8**	**$5.4**	**$5.4**	**$20.5**	**($0.42)**	**($0.56)**	**($0.50)**	**($0.52)**	**($1.99)**	**NM**
2014E - Old	--	--	--	--	$7.3	--	--	--	--	($1.77)	NM
2015E	--	--	--	--	$7.3	--	--	--	--	($2.40)	NM

Source: Company Information and Leerink Partners LLC Research
Revenues in millions.
2013 Q3 10Q reported ($0.47) in EPS. However, we believe the correct EPS should be ($0.52) due to calculation error with cumulative preferred stock dividends.

INVESTMENT THESIS

AGIO's strong platform in cellular metabolism has resulted in seminal discoveries which the company has been able to capitalize and translate into a full array of early clinical or late-preclinical pipeline agents targeting cancer and ultra-orphan indications of inborn errors of metabolism (IEMs). AGIO is a clear leader in the discipline of cancer metabolism, a potentially fruitful area of exploration for new cancer therapeutics. AGIO's most advanced candidates AG-221 and AG-120 targeting mutations in the enzymes isocitrate dehydrogenase 1 and 2 (IDH1 and IDH2). Both targets are genetically validated and mutations have been identified in acute myeloid leukemia, brain cancer, sarcoma, and biliary tract cancers. Third candidate AG-348 targets the ultra-orphan blood disorder of pyruvate kinase deficiency which is an IEM manifested by severe hemolysis. Although we are clearly mindful that AGIO's pipeline is very early, there is very strong genetic validation for the lead candidates. The observations of single mutation in IDH1 and IDH2 (isocitrate dehydrogenase) on a single allele being associated with cancer point to gain of function alterations that are well suited for drug therapeutics. As AGIO pioneered the field, there does not appear to be visible competition. AGIO also formed a strong partnership with CELG (OP) not only funds the programs but also leaves good upside including full US rights for 1 in 3 compounds. These terms, based purely on the cancer metabolism platform with compounds still on the drawing board, are impressive and in our view provide clear validation for AGIO. Additionally, AGIO is leveraging its metabolism platform to target rare IEMs that we believe could provide a rapid path to market. Its lead IEM compound AG-348, appears to be able to accomplish the difficult task of activating multiple defective forms of pyruvate kinase-R and potentially provides a therapy for pyruvate kinase deficiency (PKD), a rare blood disorder.

AG-348 has completed IND-enabling studies, initial indication likely in adult PKD patients. AGIO has completed IND-enabling studies for AG-348 and plans to initiate Phase I trial in healthy volunteers in mid-2014. Management noted that next step clinical trial in pyruvate kinase deficiency (PKD) patients will be conducted in adult patients, although details of the trial have not been disclosed.

Cash runway into 4Q:16. AGIO reported $6.7M in revenues and ($0.40) in EPS for 4Q:13. The company ended the quarter with $194M in cash. The company is expected to receive $20M in extension milestone payment from CELG in mid-2014 and guided to $130M in cash by YE:14. We adjust our model to reflect these changes. As a result, our revenue project changes from $7.3M to $20.5M, and our EPS estimate changes from ($1.77) to ($1.99) for 2014.

AGIO Upcoming Catalysts

Compound	Timing	Event
AG-221 (IDH2)	Apr 6, '14, AACR	Initial data from Phase I dose escalation study in hematologic malignancies with IDH2 mutations
	2H:14	Phase I expansion cohorts in AML, MDS and MPD
AG-120 (IDH1)	Early '14	Initiate Phase I trials in solid tumor and hematologic malignancies with IDH1 mutations
AG-348 (PKR activator)	Mid '14	Initiate Phase I dose escalation study in healthy volunteers

Source: Company reports and Leerink Swann LLC

AGIO Product Pipeline

Drug	Status	Note
AG-221 (IDH2 inhibitor)	Phase I	Phase I dose escalation study in IDH2m hematologic malignancies initiated in 3Q:13.
AG-120 (IDH1 inhibitor)	Preclinical	Initiate two Phase I trials in solid tumor and hematologic malignancies in 1Q:14
AG-348 (PKR activator)	Preclinical	IND filing and initiate Phase I trial in healthy volunteer in mid '14

Source: Company reports and Leerink Swann LLC

VALUATION

We are increasing our valuation from $38 to $51 to reflect higher probability of success following recent clinical development. We are increasing our probability of success from 25% to 50% for AG-221 (IDH2), from 20% to 30% for AG-120 (IDH1), and maintain 15% for AG-348 (PKD). Our valuation for AGIO is based on NPV and sum of the parts methodology. We use 10% discount rate and believe it is appropriate given probability weighted sales projection. Our royalty assumption is 10-13% for IDH2 w/w sales and IDH1 Ex-US sales. We include $500M valuation for the platform and other pipeline, and estimated $130M cash at YE:14.

RISKS TO VALUATION

- All pipeline assets are still in early-stage clinical or preclinical development and many hurdles remain.
- AGIO's agents have been all first-in-class. Clinical toxicity and efficacy of Agios compounds as well as proof of principle remain to be established.
- Additional funding will be required before turning profitable.

AGIO Income Statement ($M)	2011A	2012A	Mar-13A	Jun-13A	Sep-13A	Dec-13A	2013A	Mar-14E	Jun-14E	Sep-14E	Dec-14E	2014E	2015E	2016E	2017E	2018E
Collaboration agreements																
Royalties																3,191
Sales																0
Total revenue	21,837	25,106	6,268	6,268	6,268	6,744	25,548	6,744	2,818	5,454	5,454	20,470	7,272	0	0	3,191
COGS																0
% of revenue																*5%*
R&D	31,253	41,037	11,462	12,958	14,803	15,279	54,502	16,043	16,685	17,352	18,046	68,126	70,170	72,275	74,443	76,676
G&A	7,215	7,064	1,852	1,836	2,534	3,707	9,929	3,744	3,782	3,819	3,858	15,202	16,723	18,395	20,234	30,000
% of revenue																
Total operating expenses	38,468	48,101	13,314	14,794	17,337	18,986	64,431	19,787	20,466	21,171	21,904	83,328	86,892	90,670	94,677	106,676
Net income (loss) from operations	(16,631)	(22,995)	(7,046)	(8,526)	(11,069)	(12,242)	(38,883)	(13,043)	(17,648)	(15,717)	(16,450)	(62,858)	(79,620)	(90,670)	(94,677)	(103,486)
Investment income	132	69	8	5	13	29	55	0	0	0	0	0	0	0	0	0
Net income (loss) before income taxes	(16,499)	(22,926)	(7,038)	(8,521)	(11,056)	(12,213)	(38,828)	(13,043)	(17,648)	(15,717)	(16,450)	(62,858)	(79,620)	(90,670)	(94,677)	(103,486)
Provision (benefit) for income taxes	7,207	(2,824)	190	99	121	169	579	0	0	0	0	0	0			
Tax rate																
Net income (loss)	(23,706)	(20,102)	(7,228)	(8,620)	(11,177)	(12,382)	(39,407)	(13,043)	(17,648)	(15,717)	(16,450)	(62,858)	(79,620)	(90,670)	(94,677)	(103,486)
Cumulative preferred stock dividends	(3,100)	(7,190)	(1,798)	(1,798)	(567)	0	(4,162)	0	0	0	0	0	0			
Net income (loss) to common stockholders	(26,806)	(27,292)	(9,026)	(10,418)	(11,744)	(12,382)	(43,569)	(13,043)	(17,648)	(15,717)	(16,450)	(62,858)	(79,620)	(90,670)	(94,677)	(103,486)
Net loss per share	**(8.90)**	**(1.18)**	**(0.39)**	**(2.80)**	**(0.52)**	**(0.40)**	**(2.83)**	**(0.42)**	**(0.56)**	**(0.50)**	**(0.52)**	**(1.99)**	**(2.40)**	**(2.61)**	**(2.59)**	**(2.70)**
Basic shares	3,013	23,133	23,390	3,723	22,744	31,153	15,415	31,309	31,465	31,623	31,781	31,544	33,122	34,778	36,517	38,342
Dilutive shares			23,390	27,176	26,687	33,645	27,724	33,813	33,983	34,152	34,323	34,068	35,771	42,560	44,688	46,922

Source: Company Reports and Leerink Partners

Disclosures Appendix

Analyst Certification

I, Howard Liang, Ph.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

Valuation

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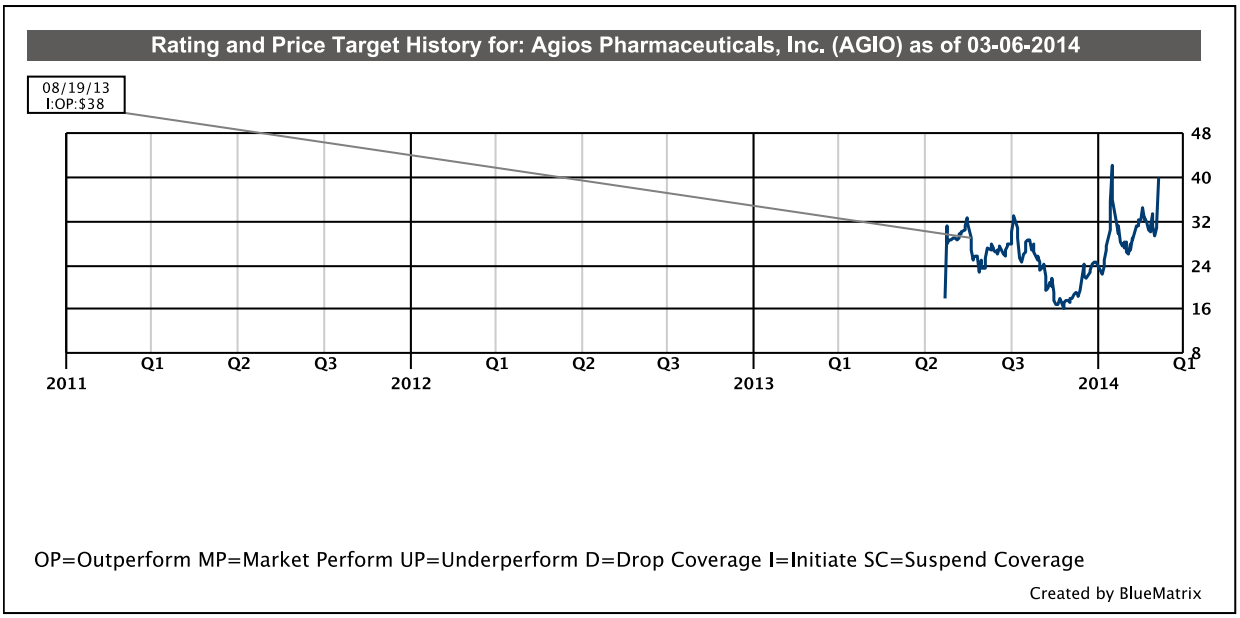

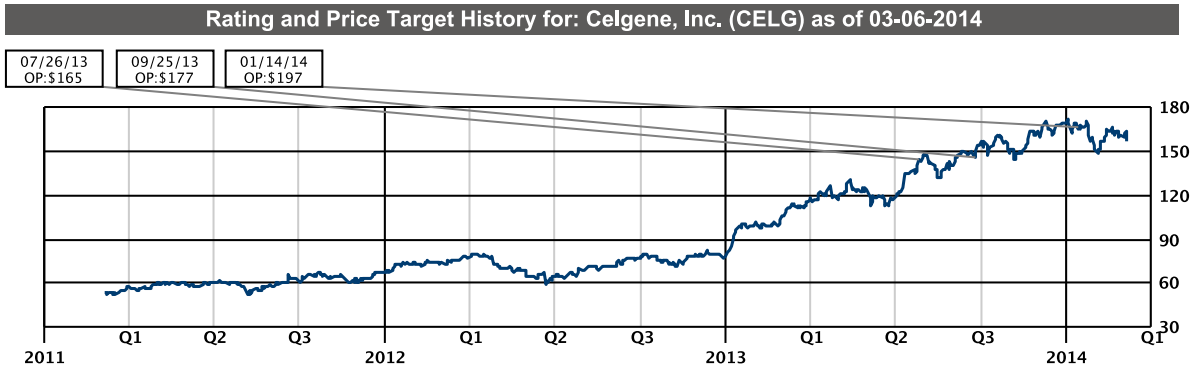

Leerink Swann initiated coverage of CELG with an Outperform rating on February 7, 2003. On June 11, 2013, Leerink Swann began a transition to specific price targets for the stocks under its coverage, replacing valuation ranges.

OP=Outperform MP=Market Perform UP=Underperform D=Drop Coverage I=Initiate SC=Suspend Coverage

Created by BlueMatrix

Distribution of Ratings/Investment Banking Services (IB) as of 12/31/13				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	118	64.50	30	25.00
HOLD [MP]	65	35.50	2	3.00
SELL [UP]	0	0.00	0	0.00

Explanation of Ratings

Outperform (Buy): We expect this stock to outperform its benchmark over the next 12 months.

Market Perform (Hold/Neutral): We expect this stock to perform in line with its benchmark over the next 12 months.

Underperform (Sell): We expect this stock to underperform its benchmark over the next 12 months.The degree of outperformance or underperformance required to warrant an Outperform or an Underperform rating should be commensurate with the risk profile of the company.

For the purposes of these definitions the relevant benchmark will be the S&P 600® Health Care Index for issuers with a market capitalization of less than $2 billion and the S&P 500® Health Care Index for issuers with a market capitalization over $2 billion.

Important Disclosures

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Leerink Consulting LLC, an affiliate of Leerink Partners, is a provider of evidence-based strategy and consulting to the healthcare industry.

In the past 12 months, the Firm has received compensation for providing investment banking services to Agios Pharmaceuticals, Inc. .

Leerink Partners LLC makes a market in Agios Pharmaceuticals, Inc. and Celgene, Inc.

In the past 12 months, an affiliate of the Firm, Leerink Swann Consulting LLC, has received compensation for providing non-securities services to: Celgene, Inc.

Leerink Partners LLC has acted as a co-manager for a public offering of Agios Pharmaceuticals, Inc. in the past 12 months.

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