April 7, 2014

OUTPERFORM

Reason for report:

COMPANY UPDATE

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LEERINK

AGIOS PHARMACEUTICALS, INC.

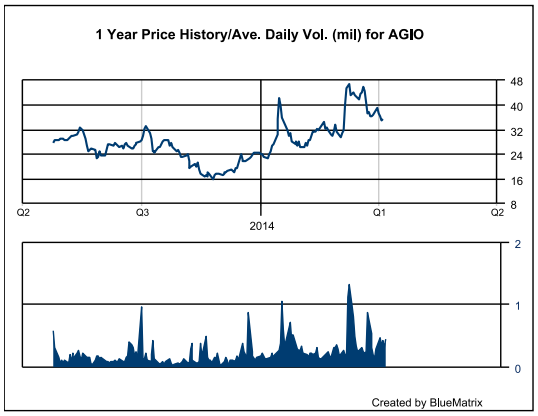
Remarkable Early Data Position AG-221 As a Potential Breakthrough Agent

• **Bottom Line:** The first clinical data from AGIO/CELG's (OP) AG-221 (IDH2 mutant inhibitor) Phase I showed remarkable activity even in the two lowest-dose cohorts with 6 of 7 evaluable patients with AML and IDH2 mutation achieving objective responses, including 3 complete remissions (CRs) and 2 complete remissions with incomplete platelet recovery (CRp); and another CR (complete response) reported from an early assessment of 2 later-added patients in the lowest dose cohort. The robust activity includes induction of differentiation reminiscent of ATRA in acute promyelocytic leukemia (APL) and knock-down of oncometabolite 2-HG by more than 90%. While data are clearly still early and duration of response is unknown, the data likely represent the best case scenario that can be hoped for an AML drug from the first readout and position AG-221 as a potential breakthrough therapeutic for AML. We believe this initial validation for the IDH2 program has a positive read through to the IDH1 program, at the minimum to its utility in hematological malignancies. We are increasing our valuation from $51 to $65 to reflect increased probability of success for both IDH2 and IDH1 programs.

• **Remarkable data may provide a game changing therapeutic in AML.** Initial 10 patients from the first two cohorts were heavily pretreated elderly (median age of 62.5 years old) AML patients, a patient population where 5-year survival (11-12%) has not changed since 1984. Excluding 3 disease-related deaths in the Cohort 1 (30mg BID), both remaining patients achieved CR or CRp at cycle 4. For the Cohort 2 (50mg BID), 4 out of 5 patients achieved objective responses with a shorter treatment duration, including 1 CR at cycle 3, 1 CR, 1 CRp and 1 PR at cycle 2. The efficacy data were further supported by a CR achieved at Day 15 in one additional evaluable patient from the Cohort 1. While patient numbers are small, there appears to be more than enough activity to support a potential accelerated approval strategy based on a single arm Phase II trial assuming even a 2-3 month duration of response. Dosing escalation has now reached 75mg BID and 100mg QD, each treated 5 patients. Data from these cohorts could be presented at either European Hematology Association (EHA) Annual Congress (June 11-15, 2014 in Milan) or American Society of Hematology (ASH) Annual Meeting (Dec 6-9, 2014 in San Francisco).

• **So far so good on safety in first 22 patients.** While safety data are still early, we believe both the severity of the condition (AML) and remarkable activity could lower the hurdle for safety and tolerability.

Key Stats:	**(NASDAQ:AGIO)**
S&P 600 Health Care Index:	**1,233.05**
Price:	**$35.48**
Price Target:	$65.00 from $51.00
Methodology:	NPV + $500M for Platform / Pipeline + $194M incash
52 Week High:	$49.79
52 Week Low:	$15.77
Shares Outstanding (mil):	30.3
Market Capitalization (mil):	$1,075.0
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	$6.7	$2.8	$5.4	$5.4	$20.5	($0.42)	($0.56)	($0.50)	($0.52)	($1.99)	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.

Please refer to pages 8 - 11 for Important Disclosures, Price Charts and Analyst Certification.

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.

Well-tolerated safety profile supports continued dosing escalation. We are impressed by the rapid enrollment and as of March 20, 2014, there were 22 AML and MDS patients with IDH2 mutation (including the first 4 cohorts) evaluable for safety and no dose limiting toxicities were reported. There were four deaths due to complications of disease-related sepsis, all of which occurred in cycle 1, with 3 from cohort 1 and 1 from cohort 4. There were 2 possibly drug-related serious adverse events including one Grade 2 hyper-leukocytosis and differentiation syndrome and one Grade 3 confusion and respiratory failure patient with sepsis.

Positive read through to the IDH1 program. Positive AG-221 data provide validation of targeted therapy against IDH2 mutations. Although AG-120 and AG-221 are two distinctive compounds, IDH2 and IDH1 share many similarities and the predictability of AG-221 preclinical data to clinical activity is noteworthy. While we do not know if IDH inhibitors will work as well in solid tumors, we believe there is a sizable opportunity ($1B+) even in liquid tumors.

AG-221 Phase I Patient Characteristics

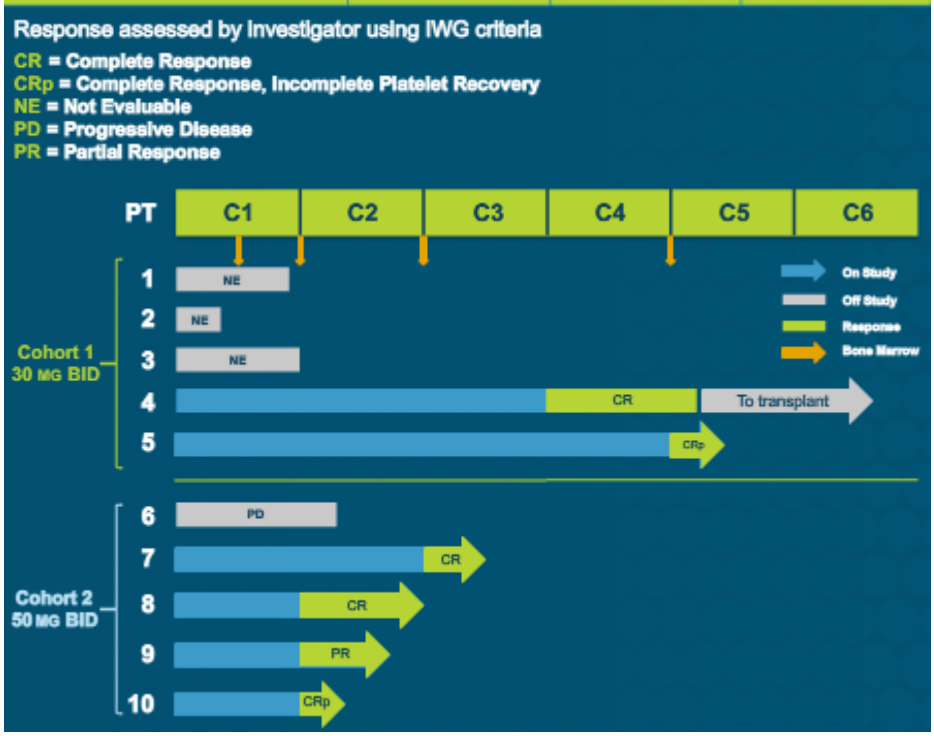
Patient Characteristics	
ITT Population N Median Age, years (range)	10 62.5 (53-74)
ECOG Performance Status, n 0 1 2	 8 2 0
Prior Regimens (range) Prior BMT Secondary AML	2 (1-4) 1 0
IDH2 Mutations, n R140 R172	10 8 2
Median Cycles of AG-221 Completed (range)	2 (<1-4+)

Cohort	Patient #	Tumor Genetics	Characteristics of Prior Therapy
1	1	R140Q, FLT3-ITD, CEPBA	Relapse 1 → Re-induction Failure
	2	R140Q	Primary Induction Failure
	3	R140Q	Relapse 1 → Re-induction Failure
	4	R140Q, NPM1	Primary Induction Failure
	5	R172K, DNMT3A, CEBPA, ASXL1	Relapse (Post allo-transplant) –> Re-induction Failure
2	6	R140Q	Relapse 1→ Re-induction Failure
	7	R140Q, NPM1	Relapse 1 → Re-induction Failure
	8	R140Q, NPM1	Relapse 1
	9	R172K	Primary Induction Failure
	10	R140Q, NPM1	Relapse 1

Source: Stein et al, 2013 AACR, #CT103

AG-221 Phase I Efficacy data in Evaluable Cohorts 1 and 2

	30 mg BID N=5	50 mg BID N=5	Total N=10
CR	1	2	3
CRp	1	1	2
PR		1	1
PD		1	1
NE	3	0	
Overall Response Rate	**2/2**	**4/5**	**6/7**



Source: Stein et al, 2013 AACR, #CT103

AGIO Upcoming Catalysts

Compound	Timing	Event
AG-221 (IDH2)	Medical conferences in 2014	Updated 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)	2014	Ongoing two 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 Partners 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	Initiated 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 Partners LLC

VALUATION

We are increasing our valuation from $51 to $65 to reflect higher probability of success following recent clinical development. We are increasing our probability of success from 50% to 70% for AG-221 (IDH2), from 30% to 40% 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 $194M cash at the beginning of 2014.

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	2011A	2012A	2013A	Mar-14E	Jun-14E	Sep-14E	Dec-14E	2014E	2015E	2016E	2017E	2018E
Collaboration agreements												
Royalties												4,467
Sales												0
Total revenue	21,837	25,106	25,548	6,744	2,818	5,454	5,454	20,470	7,272	0	0	4,467
COGS												0
% of revenue												*5%*
R&D	31,253	41,037	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	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	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)	(38,883)	(13,043)	(17,648)	(15,717)	(16,450)	(62,858)	(79,620)	(90,670)	(94,677)	(102,209)
Investment income	132	69	55	0	0	0	0	0	0	0	0	0
Net income (loss) before income taxes	(16,499)	(22,926)	(38,828)	(13,043)	(17,648)	(15,717)	(16,450)	(62,858)	(79,620)	(90,670)	(94,677)	(102,209)
Provision (benefit) for income taxes	7,207	(2,824)	579	0	0	0	0	0	0			
Tax rate												
Net income (loss)	(23,706)	(20,102)	(39,407)	(13,043)	(17,648)	(15,717)	(16,450)	(62,858)	(79,620)	(90,670)	(94,677)	(102,209)
Cumulative preferred stock dividends	(3,100)	(7,190)	(4,162)	0	0	0	0	0	0			
Net income (loss) to common stockholders	(26,806)	(27,292)	(43,569)	(13,043)	(17,648)	(15,717)	(16,450)	(62,858)	(79,620)	(90,670)	(94,677)	(102,209)
Net loss per share	**(8.90)**	**(1.18)**	**(2.83)**	**(0.42)**	**(0.56)**	**(0.50)**	**(0.52)**	**(1.99)**	**(2.40)**	**(2.61)**	**(2.59)**	**(2.67)**
Basic shares	3,013	23,133	15,415	31,309	31,465	31,623	31,781	31,544	33,122	34,778	36,517	38,342

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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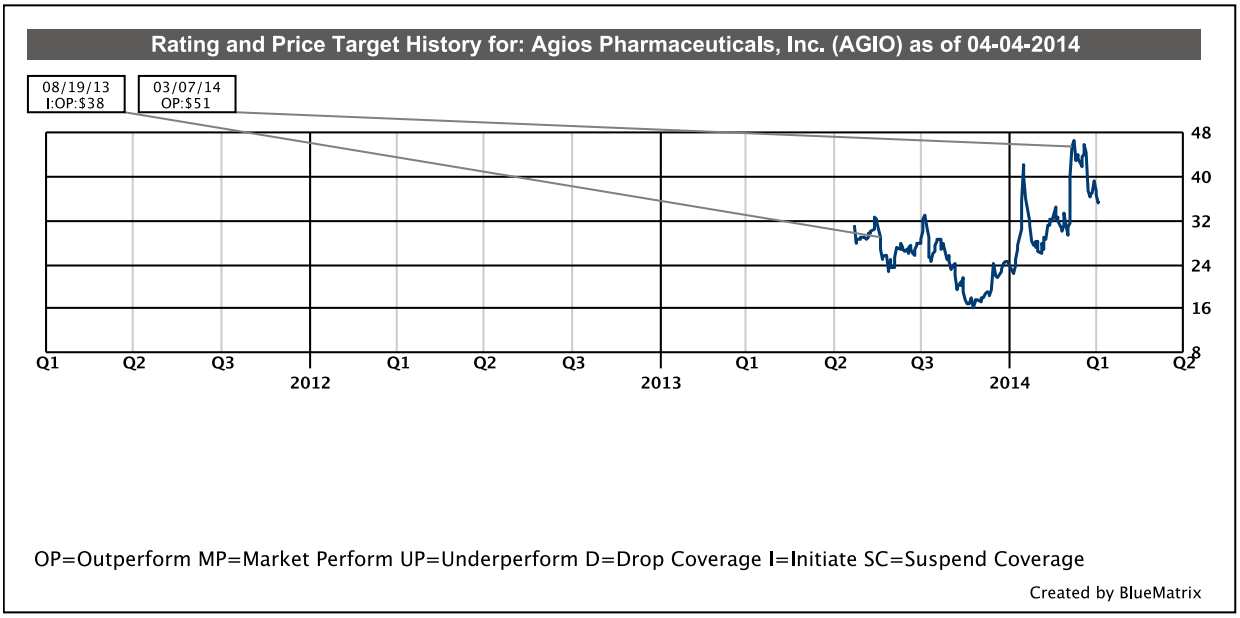

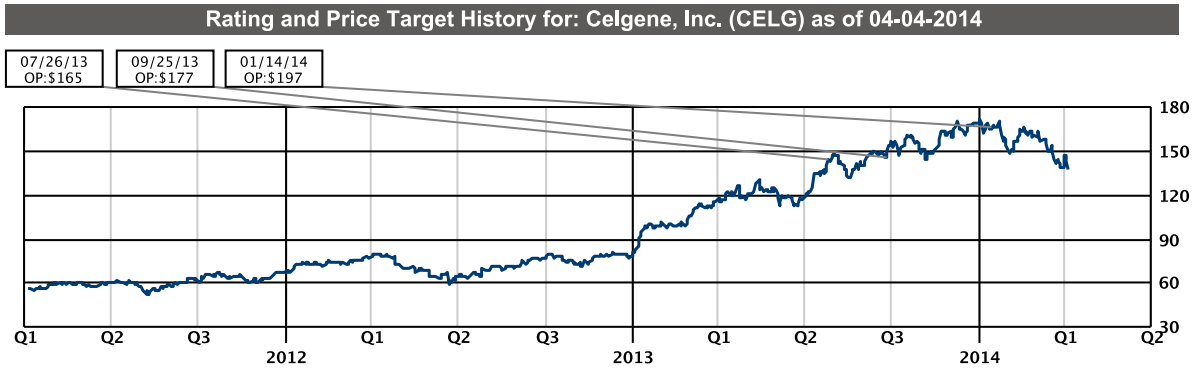

Leerink Swann initiated coverage of CELG with an Outperform rating on February 7, 2003. On June 11, 2013, Leerink Swann began a transition to specific price targets for the stocks under its coverage, replacing valuation ranges.

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 03/31/14				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	131	68.23	46	35.11
HOLD [MP]	61	31.77	3	4.92
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 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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