May 9, 2014

OUTPERFORM

Reason for report:

EARNINGS

Howard Liang, Ph.D.
(617) 918-4857
Howard.Liang@Leerink.com

Gena Wang, Ph.D., CFA
(212) 277-6073
Gena.Wang@Leerink.com

Richard Goss
(617) 918-4059
Richard.Goss@leerink.com

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AGIOS PHARMACEUTICALS, INC.

Near-term Newsflow at EHA as Clinical Portfolio Broadens to 3 Compounds

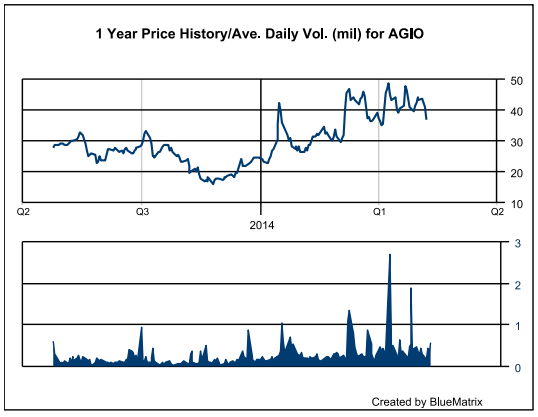
• **Bottom Line:** On its 1Q:14 earnings call, AGIO highlighted a late breaker presentation at the European Hematology Association (EHA) meeting for the AG-221 (IDH2 inhibitor) program as well as several Phase I initiations for both AG-120 (IDH1 inhibitor) and AG-348 (PKR activator). We believe the full presentation at EHA (and potentially to some extent the abstract to be released on May 21) could provide the first look on durability of responses associated with AG-221, which seems to be the main unknown in the agent's profile. While the clinical development of AG-221 remains a near-term focus, clinical progress and initial data from AG-120 and AG-348 (2015) could provide diversification to long-term valuation. Our price target on AGIO remains $65.

• **Updated Phase I data at EHA could provide insight on AG-221.** AGIO announced that an updated AG-221 Phase I data will be presented as a late breaker at EHA (June 12-15, 2014 in Milan, Italy). Given the deadline for LBA submission (April 14-20), we expect somewhat limited update from the abstracts (to be released on May 21) since the AACR presentation, which had a data cutoff date of March 20. However, full presentation at the meeting should provide some important updates, likely to include: 1) a longer follow-up to assess the duration of the response from the initial patients; 2) higher dose cohorts to assess if the response is dose dependent and if MTD has been reached. AGIO and partner CELG (OP) expect to initiate the expansion cohort by end-2014.

• **Two additional compounds in Phase I.** AGIO advanced both early stage compounds into clinical development and initiated 2 Phase I trials for AG-120 (IDH1 inhibitor) in solid tumor and hematologic malignancies, as well as a Phase I dose escalation study in healthy volunteers for AG-348 (PKR activator). Initial data from both programs will be presented at medical meetings in 2015. As we mentioned before, positive AG-221 data provide validation of targeted therapy against IDH2 mutations, which should have positive read-through to the AG-120 program targeting IDH1 mutations. While we do not know if IDH inhibitors will work as well in solid tumors, we believe there is a sizable opportunity even in liquid tumors.

• **Cash runway into mid-2017.** AGIO reported $8.4M in revenues and ($0.39) in EPS for 1Q:14. The company ended the quarter with $167M in cash. AGIO is expected to receive $20M in extension milestone payment from CELG in late 2014. Together with $95M net proceeds from a recent capital raise, AGIO guided to $200M in cash by YE:14 with runway into mid-2017. We adjust our model to reflect these changes. As a result, our revenue changes from $20.5M to $22.1M, and our EPS changes from ($1.99) to ($1.95) for 2014E.

Key Stats:	**(NASDAQ:AGIO)**
S&P 600 Health Care Index:	**1,199.05**
Price:	**$36.79**
Price Target:	$65.00
Methodology:	NPV + $500M for Platform / Pipeline + Cash
52 Week High:	$49.79
52 Week Low:	$15.77
Shares Outstanding (mil):	33.9
Market Capitalization (mil):	$1,247.2
Book Value/Share:	$0.14
Cash Per Share:	$7.54
Dividend (ann):	$0.00
Dividend Yield:	0.0%
Cash Per Share: Pro forma	



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**	**$25.5**	**($0.39)**	**($2.80)**	**($0.52)**	**($0.40)**	**($2.83)**	**NM**
2014E - New	**$8.4A**	**$2.8**	**$5.5**	**$5.5**	**$22.1**	**($0.39)A**	**($0.55)**	**($0.49)**	**($0.51)**	**($1.95)**	**NM**
2014E - Old	$6.7	$2.8	$5.4	$5.4	$20.5	($0.42)	($0.56)	($0.50)	($0.52)	($1.99)	NM
2015E - New	**--**	**--**	**--**	**--**	**$7.3**	**--**	**--**	**--**	**--**	**($2.38)**	**NM**
2015E - Old	--	--	--	--	$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 6 - 9 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's strong partnership with CELG 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.

AGIO Upcoming Catalysts

Compound	Timing	Event
AG-221 (IDH2)	May 21, '14	EHA abstract release
	EHA (June 12-15, '14)	LBA presentation from Phase I dose escalation study in hematologic malignancies with IDH2 mutations
	By YE:14	Phase I expansion cohorts in AML, MDS and MPD
AG-120 (IDH1)	Medical conferences in 2015	Initial data from two Phase I trials in solid tumor and hematologic malignancies with IDH1 mutations
AG-348 (PKR activator)	Medical conferences in 2015	Interim data from 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	Initiated Phase I trial in healthy volunteer in 2Q:14

Source: Company reports and Leerink Partners LLC

VALUATION

Our $65 valuation for AGIO is based on a sum-of-parts NPV analysis. Our $65 valuation for AGIO is based on NPV and sum-of-the-parts methodology. Our probability of success is 70% for AG-221 (IDH2), 40% for AG-120 (IDH1), and 15% for AG-348 (PKD). We use 10% discount rate and believe it is appropriate given our 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 an estimated $240M in cash.

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-14A	Jun-14E	Sep-14E	Dec-14E	2014E	2015E	2016E	2017E
Collaboration agreements											
Royalties											
Sales											
Total revenue	21,837	25,106	25,548	8,411	2,818	5,454	5,454	22,137	7,272	0	0
COGS											
% of revenue											
R&D	31,253	41,037	54,502	17,407	18,103	18,827	19,581	73,918	76,136	78,420	80,772
G&A	7,215	7,064	9,929	3,288	3,321	3,354	3,388	13,351	14,686	16,154	17,770
% of revenue											
Total operating expenses	38,468	48,101	64,431	20,695	21,424	22,182	22,968	87,269	90,821	94,574	98,542
Net income (loss) from operations	(16,631)	(22,995)	(38,883)	(12,284)	(18,606)	(16,728)	(17,514)	(65,132)	(83,549)	(94,574)	(98,542)
Investment income	132	69	55	36	0	0	0	36	0	0	0
Net income (loss) before income taxes	(16,499)	(22,926)	(38,828)	(12,248)	(18,606)	(16,728)	(17,514)	(65,096)	(83,549)	(94,574)	(98,542)
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)	(12,248)	(18,606)	(16,728)	(17,514)	(65,096)	(83,549)	(94,574)	(98,542)
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)	(12,248)	(18,606)	(16,728)	(17,514)	(65,096)	(83,549)	(94,574)	(98,542)
Net loss per share	**(8.90)**	**(1.18)**	**(2.83)**	**(0.39)**	**(0.55)**	**(0.49)**	**(0.51)**	**(1.95)**	**(2.38)**	**(2.57)**	**(2.55)**
Basic shares	3,013	23,133	15,415	31,395	33,852	34,021	34,191	33,365	35,033	36,785	38,624
Dilutive shares			27,724	33,813	36,283	36,464	36,646	35,802	37,592	39,471	46,445

Source: Company Reports and Leerink Partners

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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.

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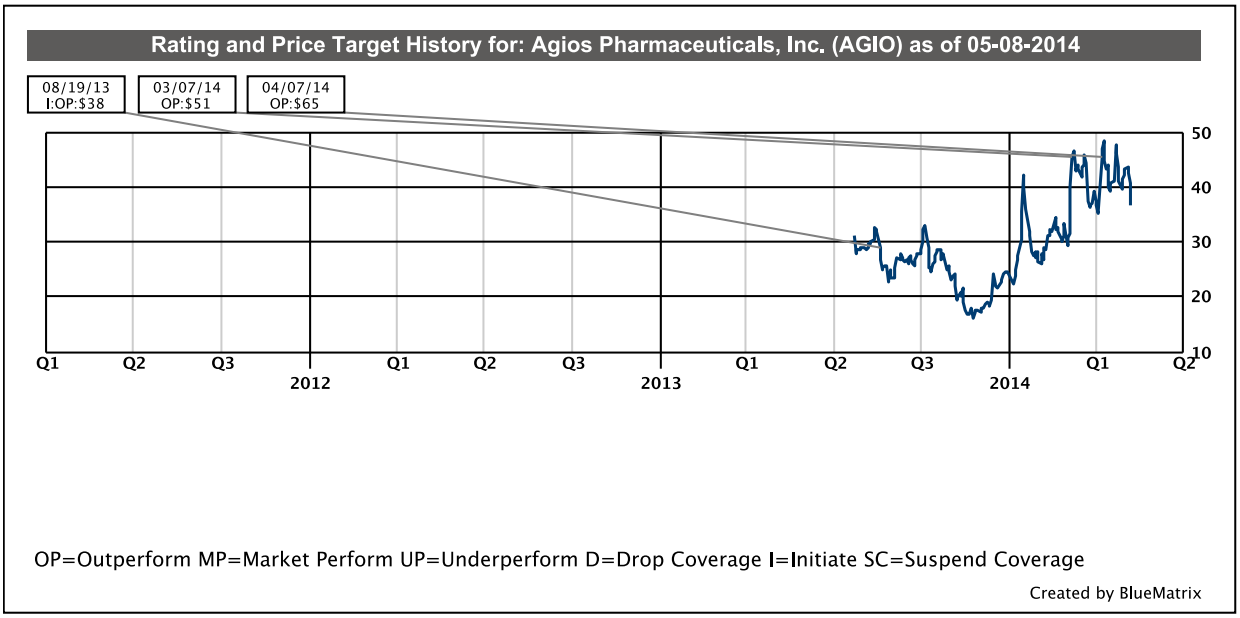

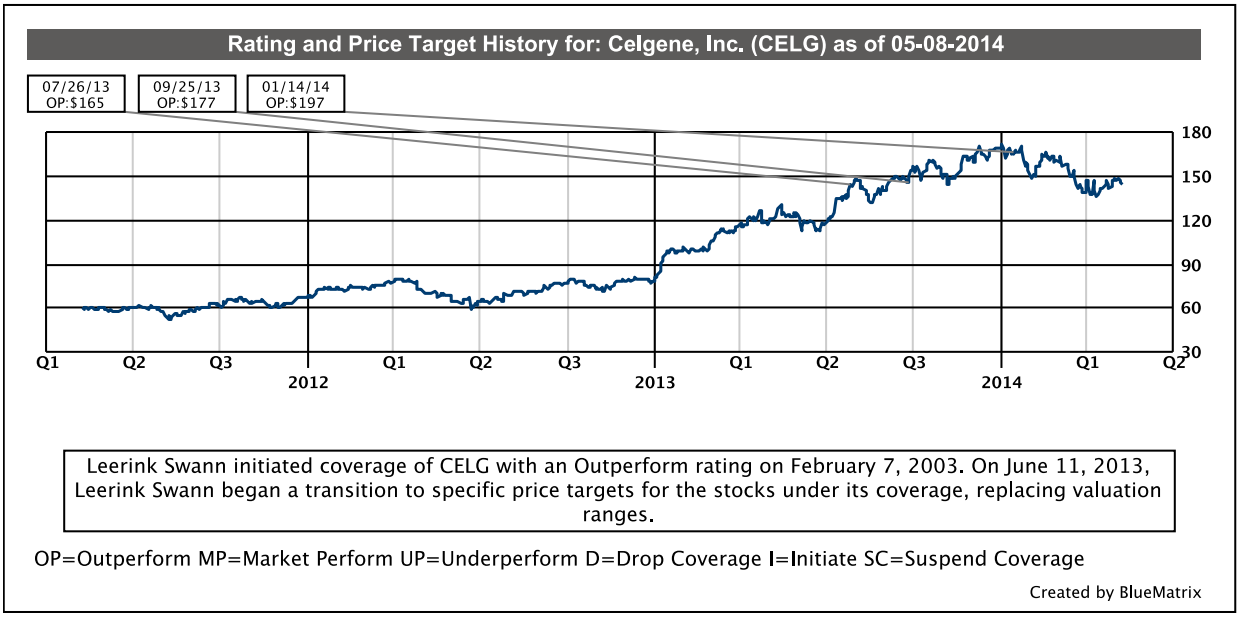
Rating and Price Target History for: Celgene, Inc. (CELG) as of 05-08-2014
07/26/13
OP:$165
09/25/13
OP:$177
01/14/14
OP:$197
180
150
120
90
60
30
Q1
Q2
Q3
2012
Q1
Q2
Q3
2013
Q1
Q2
Q3
2014
Q1
Q2
Leerink Swann initiated coverage of CELG with an Outperform rating on February 7, 2003. On June 11, 2013, 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

This information (including, but not limited to, prices, quotes and statistics) has been obtained from sources that we believe reliable, but we do not represent that it is accurate or complete and it should not be relied upon as such. All information is subject to change without notice. This is provided for information purposes only and should not be regarded as an offer to sell or as a solicitation of an offer to buy any product to which this information relates. The Firm, its officers, directors, employees, proprietary accounts and affiliates may have a position, long or short, in the securities referred to in this report, and/or other related securities, and from time to time may increase or decrease the position or express a view that is contrary to that contained in this report. The Firm's salespeople, traders and other professionals may provide oral or written market commentary or trading strategies that are contrary to opinions expressed in this report. The Firm's proprietary accounts may make investment decisions that are inconsistent with the opinions expressed in this report. The past performance of securities does not guarantee or predict future performance. Transaction strategies described herein may not be suitable for all investors. Additional information is available upon request by contacting the Editorial Department at One Federal Street, 37th Floor, Boston, MA 02110.

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MEDACorp is a network of healthcare professionals, attorneys, physicians, key opinion leaders and other specialists accessed by Leerink and it provides information used by its analysts in preparing research.

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.

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

Leerink Partners LLC Equity Research

Director of Equity Research	**John L. Sullivan, CFA**	(617) 918-4875	john.sullivan@leerink
Associate Director of Research	**Alice C. Avanian, CFA**	(617) 918-4544	alice.avanian@leerink
Healthcare Strategy	**John L. Sullivan, CFA**	(617) 918-4875	john.sullivan@leerink
	Alice C. Avanian, CFA	(617) 918-4544	alice.avanian@leerink
Biotechnology	**Howard Liang, Ph.D.**	(617) 918-4857	howard.liang@leerink
	Joseph P. Schwartz	(617) 918-4575	joseph.schwartz@leerink
	Marko Kozul, M.D.	(415) 905-7221	marko.kozul@leerink
	Michael Schmidt, Ph.D.	(617) 918-4588	michael.schmidt@leerink
	Gena Wang, Ph.D., CFA	(212) 277-6073	gena.wang@leerink
	Jonathan Chang, Ph.D.	(617) 918-4015	jonathan.chang@leerink
	Paul Matteis	(617) 918-4585	paul.matteis@leerink
	Richard Goss	(617) 918-4059	richard.goss@leerink
Life Science Tools and Diagnostics	**Dan Leonard**	(212) 277-6116	dan.leonard@leerink
	Justin Bowers, CFA	(212) 277-6066	justin.bowers@leerink
Pharmaceuticals/Major	**Seamus Fernandez**	(617) 918-4011	seamus.fernandez@leerink
	Ario Arabi	(617) 918-4568	ario.arabi@leerink
	Aneesh Kapur	(617) 918-4576	aneesh.kapur@leerink
Specialty Pharmaceuticals, Generics	**Jason M. Gerberry, JD**	(617) 918-4549	jason.gerberry@leerink
	Christopher W. Kuehnle, JD	(617) 918-4851	chris.kuehnle@leerink
Medical Devices, Cardiology & Orthopedics	**Danielle Antalffy**	(212) 277-6044	danielle.antalffy@leerink
	Richard Newitter	(212) 277-6088	richard.newitter@leerink
	Ravi Misra	(212) 277-6049	ravi.misra@leerink
Healthcare Services	**Ana Gupte, Ph.D.**	(212) 277-6040	ana.gupte@leerink
Healthcare Technology & Distribution	**David Larsen, CFA**	(617) 918-4502	david.larsen@leerink
	Christopher Abbott	(617) 918-4010	chris.abbott@leerink
Sr. Editor/Supervisory Analyst	**Mary Ellen Eagan, CFA**	(617) 918-4837	maryellen.eagan@leerink
Supervisory Analysts	Robert Egan		bob.egan@leerink
	Amy N. Sonne		amy.sonne@leerink
Editorial	Cristina Diaz-Dickson	(617) 918-4548	cristina.diaz-dickson@leerink

New York
299 Park Avenue, 21st floor
New York, NY 10171
(888) 778-1653

Boston
One Federal Street, 37th Floor
Boston, MA 02110
(800) 808-7525

San Francisco
201 Spear Street, 16th Floor
San Francisco, CA 94105
(800) 778-1164