

Reason for report:

## EARNINGS

## IGNYTA, INC.

## On Track Clinical Development, ASCO To Provide More Color on Clinical Activity

• **Bottom Line:** RXDX reported 1Q:14 earnings report and highlighted its on track clinical development program and its upcoming ASCO oral presentation from the Phase I Italian trial for the lead candidate RXDX-101 (oral pan-Trk, ROS1, ALK inhibitor). Although there will likely be no data on the Trk+ patients, the presentation should provide more color on RXDX-101 activity in patients who had prior ALK inhibitor treatment. Positive data should have read-through to potential clinical activity in Trk+ cancers where in vitro activity is ~10X more potent vs. ROS1 and ALK. Recent approval of NVS' (OP) Zykadia in NSCLC does not change our thesis since we believe RX-101 value largely lies in the market potential for pan-Trk cancers. We reiterate OP rating and \$14 valuation for RXDX.

• **Updated Phase I data may provide more color on RXDX-101 activity in crizotinib resistant patients.** An update of the Italian Phase I trial (ALKA-372-001) will be presented as an oral presentation at ASCO (#2502). Although there likely will be no data in patients with Trk

rearrangement and at dose higher than 1600mg/m<sup>2</sup>, we expect to see longer follow-up data from ALK+ or ROS+ patients, particularly these were in early cycles of treatment at last update (IASLC 2014). It will be important to see if there is any activity in these patients who had prior treatments of crizotinib or Zykadia. We believe positive data should have read through to potential clinical activity in Trk+ cancers.

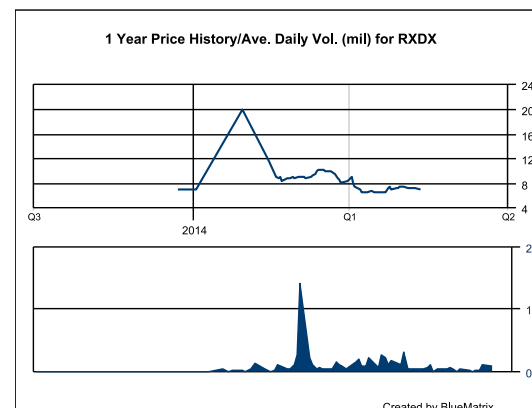
• **On track clinical development.** Following the recent submission of an IND for RXDX-101, RXDX is on track to initiate the STARTRK-1 Phase I/II global trial in 3Q:14 in advanced solid tumors with ALK, ROS1 or TrkA/B/ C genetic alteration.

• **Model update.** For 1Q:14, RXDX reported \$2.2M / \$1.8M in R&D and SG&A expenses vs. our estimates of \$4.5 / \$2.0M. EPS was (\$0.28) vs. our estimate of (\$0.33). The company ended the quarter with \$100.4M cash, sufficient to support operation into 2016. We adjust our estimates to reflect these updates. As a result, our 2014 EPS estimate changes from (\$1.61) to (\$1.35).

## Key Stats:

(NASDAQ:RXDX)

<b>S&amp;P 600 Health Care Index:</b>	<b>1,237.84</b>
<b>Price:</b>	<b>\$7.15</b>
Price Target:	\$14.00
Methodology:	Probability-weighted DCF analysis, 10% discount rate
52 Week High:	\$20.00
52 Week Low:	\$1.00
Shares Outstanding (mil):	19.6
Market Capitalization (mil):	\$140.1
Book Value/Share:	\$5.37
Cash Per Share:	\$5.13
Dividend (ann):	\$0.00
Dividend Yield:	0.0%
<i>Book Value: Pro Forma</i>	
<i>Cash Per Share: Pro Forma</i>	



Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2013A	--	--	--	--	0.0	--	--	--	--	(\$3.83)	NM
2014E - New	0.0A	0.0	0.0	0.0	0.0	(\$0.28)A	(\$0.26)	(\$0.37)	(\$0.41)	(\$1.35)	NM
2014E - Old	0.0A	0.0	0.0	0.0	0.0	(\$0.33)	(\$0.36)	(\$0.43)	(\$0.47)	(\$1.61)	NM
2015E - New	--	--	--	--	0.0	--	--	--	--	(\$1.81)	NM
2015E - Old	--	--	--	--	0.0	--	--	--	--	(\$1.92)	NM

Source: Company Information and Leerink Partners LLC Research

## INVESTMENT THESIS

RXDX is a biotech company with a focus on targeted therapies and molecular diagnosis in oncology. RXDX's approach of targeting molecular oncogenic drivers has historically resulted in highly effective agents. Despite low incidence of many genetic alterations including Trk and ROS1 rearrangement, molecular screening started to become a standard practice in NSCLC where oncogenic driver mutations have been identified in ~70% of adenocarcinoma patients. Clinical successes with EGFR, ALK, ROS1, HER2, BRAF inhibitors have shown high efficacy in several cancer types. Although RXDX's lead candidate RXDX-101 (oral pan-Trk, ROS1 and ALK inhibitor) is still in early stage clinical development, we believe historical precedence suggests a higher-than-average chance of success in identifying highly effective agents which we believe is increasingly the direction for the development of new cancer therapies. We believe the main value for RXDX-101 lies in the market potential for cancers with Trk genetic alteration, an emerging target for NSCLC. Although many compounds have activity against Trk, so far, only a handful of companies have focused clinical programs targeting Trk, whereas RXDX has dedicated effort to target Trk in multiple tumor type with the potential to develop a first-in-class Trk inhibitor. Although still early with limited clinical data, observed ALK activity bodes well for RXDX-101 as a Trk inhibitor, where in vitro activity is ~10X more potent vs. ROS1 and ALK. The new global Phase I study with modified continuous dosing schedule could provide insight on safety and clinical activity. Additionally, Technology platform and integrated Rx/Dx strategy with genomic and epigenomic mining of oncolome provide potentially six targeted therapies with companion diagnostics in 2015 and beyond.

**RXDX – Upcoming Events**

Timing	Event
<b>RXDX-101 (Pan-Trk, ROS1, ALK inhibitor)</b>	
ASCO 2014	Phase I update from the Italian trial
3Q:14	Initiation of STARTRK-1 Phase I/II dose escalation study with continuous dosing
	Basket Trial expansion cohorts in TrkA, TrkB, TrkC, ROS1, ALK
ASCO 2015	STARTRK data
<b>Spark-1 (Rx/Dx novel target)</b>	
2015	IND candidate

Source: Company Reports

**RXDX – Pipeline**

Stage of Development	Current Status/Upcoming Developments
<b>RXDX-101 (Pan-Trk, ROS1, ALK inhibitor)</b>	
Phase I	Phase I dose escalation study of intermittent dosing in Italy with 20-30 pts in each of ALK, ROS1 or TrkA alterations.
STARTRK-1	Global Phase I/II dose escalation study in US, EU and Asia with continuous dosing in 6-24 pts with Trk, ROS, or ALK alterations to be initiated in 3Q:14
	Basket trial expansion cohorts at RP2D with 15-20 pts in each cohort with TrkA, TrkB, TrkC, ROS1 or ALK alterations
STARTRK-2	Pivotal registration trials in most promising tumor types and targets based on ORR and other clinical observations
<b>RXDX-102 (Pan-Trk inhibitor)</b>	
Preclinical	Back-up to RXDX-101
<b>Spark-1 Rx/Dx program</b>	
Discovery	IND candidate in 2015
<b>Spark-2 Rx/Dx program</b>	
Discovery	
<b>Spark-3 Rx/Dx program</b>	
Discovery	

Source: Company Reports

## VALUATION

Our \$14 price target is derived from a probability-weighted DCF analysis. We project US launch for RXDX-101 in NSCLC (both ROS1+ and TrkA re-arrangement, 50% probability) in 2018, and EU launch in 2019. We assume full internal commercialization in both the US and EU with 8% sales royalty to Nerviano. We include \$100M cash in 1Q:14 following recent IPO and assign \$40M valuation to pipeline. We assign no terminal value to RXDX-101. We believe 10% discount rate is appropriate given probability-weighted sales estimates.

## RISK TO VALUATION

- RXDX-101 is at early stage with limited clinical efficacy data in patients with ROS1 and ALK genetic alteration.
- Although some preclinical data showed RXDX-101 activity against crizotinib resistant cell lines, activity in crizotinib resistant patients is still unknown.
- New toxicity may emerge under the modified continuous dosing regimen in STARTRK Phase I/IIa study.
- Underlying market opportunity for Trk remains to be clarified.
- Competitive landscape remains widely open and other competitors could emerge rapidly.
- Even with good efficacy as a monotherapy, RXDX-101 may need to be combined with other agents to be competitive.
- Financing risk – Current cash only support operation into 2016 and RXDX will require additional capital raise before turning to profitability.

RXDX - Income Statement (\$000, except per share value)	2012A	2013A	Mar-14A	Jun-14E	Sep-14E	Dec-14E	2014E	2015E	2016E	2017E	2018E	2019E
RXDX-101 sales - US							0	0	0	0	8,471	58,747
% to RXDX							92%	92%	92%	92%	92%	92%
RXDX-101 sales - Ex-US							0	0	0	0	0	21,173
% to RXDX							92%	92%	92%	92%	92%	92%
<b>Total revenue</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>7,793</b>	<b>73,526</b>
COGS	0	0	0	0	0	0	0	0	0	0	678	6,394
R&D	708	10,171	2,183	3,056	4,584	5,272	15,095	30,274	43,897	54,871	54,871	53,225
SG&A	548	3,731	1,756	2,107	2,739	3,013	9,616	15,383	16,921	33,843	50,764	55,841
<b>Total expenses</b>	<b>1,256</b>	<b>13,902</b>	<b>3,939</b>	<b>5,163</b>	<b>7,324</b>	<b>8,285</b>	<b>24,711</b>	<b>45,657</b>	<b>60,818</b>	<b>88,714</b>	<b>106,313</b>	<b>115,459</b>
<b>Operating Income</b>	<b>(1,256)</b>	<b>(13,902)</b>	<b>(3,939)</b>	<b>(5,163)</b>	<b>(7,324)</b>	<b>(8,285)</b>	<b>(24,711)</b>	<b>(45,657)</b>	<b>(60,818)</b>	<b>(88,714)</b>	<b>(98,520)</b>	<b>(41,933)</b>
Other income (expenses)	0	(106)	(163)	0	0	0	(163)	0	0	0	0	0
Interest income (expenses)	(23)	(204)										
Tax	1	2	5	0	0	0	5	0				
<b>Net income to common shares</b>	<b>(1,280)</b>	<b>(14,214)</b>	<b>(4,107)</b>	<b>(5,163)</b>	<b>(7,324)</b>	<b>(8,285)</b>	<b>(24,879)</b>	<b>(45,657)</b>	<b>(60,818)</b>	<b>(88,714)</b>	<b>(98,520)</b>	<b>(41,933)</b>
<b>EPS - basic</b>	<b>(2.00)</b>	<b>(3.83)</b>	<b>(0.28)</b>	<b>(0.26)</b>	<b>(0.37)</b>	<b>(0.41)</b>	<b>(1.35)</b>	<b>(1.81)</b>	<b>(2.00)</b>	<b>(2.19)</b>	<b>(2.03)</b>	<b>(0.86)</b>
<b>EPS - dilutive</b>	<b>(2.00)</b>	<b>(3.83)</b>	<b>(0.28)</b>	<b>(0.26)</b>	<b>(0.37)</b>	<b>(0.41)</b>	<b>(1.35)</b>	<b>(1.81)</b>	<b>(2.00)</b>	<b>(2.19)</b>	<b>(2.03)</b>	<b>(0.86)</b>
Basic shares	640	3,712	14,501	19,580	19,775	19,973	18,457	25,237	30,456	40,486	48,527	48,576
Dilutive shares	640	3,712	16,186	21,268	21,467	21,668	20,147	26,940	32,167	42,199	50,241	50,291

Sources: Company Reports, Leerink Partners

## Disclosures Appendix

### Analyst Certification

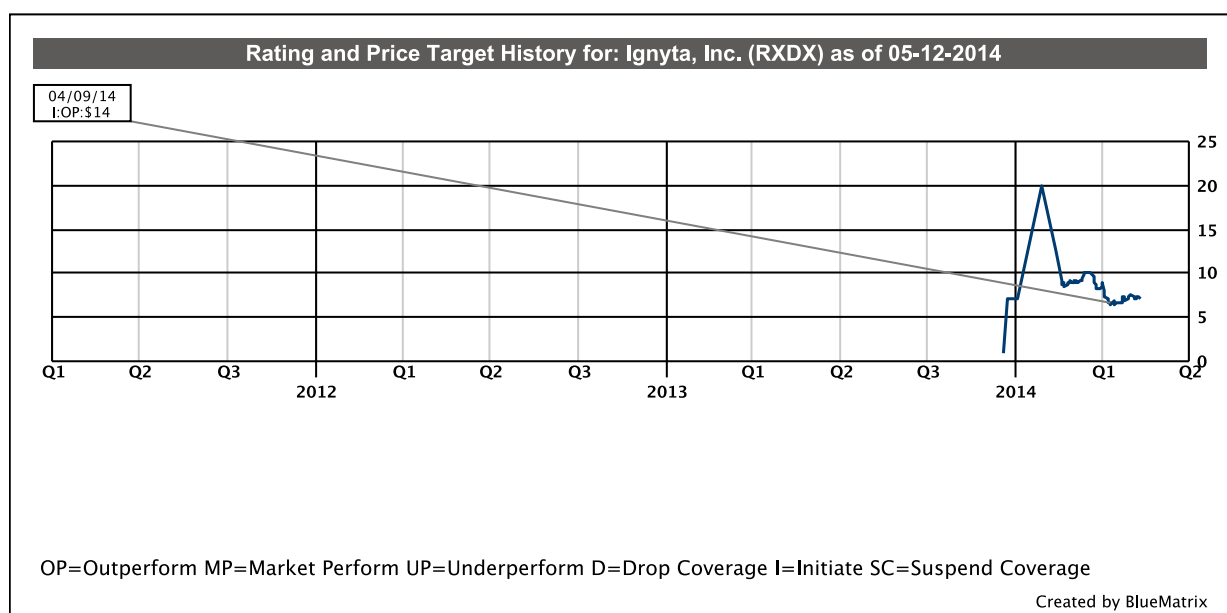
I, Howard Liang, Ph.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

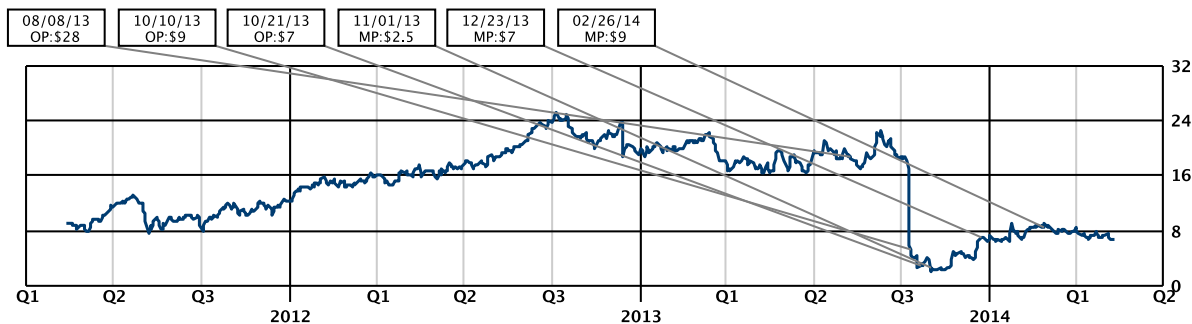
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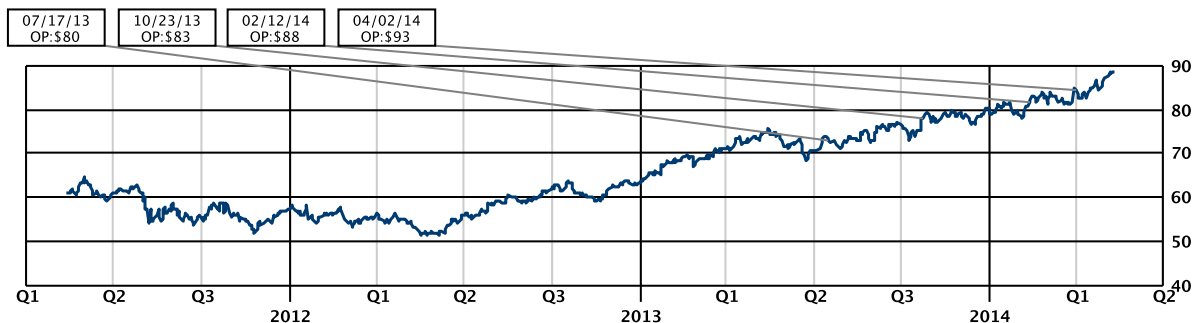


**Rating and Price Target History for: Ariad Pharmaceuticals, Inc. (ARIA) as of 05-12-2014**


Leerink Swann placed an Outperform rating on ARIA on November 5, 2005. 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

**Rating and Price Target History for: Novartis AG (NVS) as of 05-12-2014**


Leerink Swann initiated coverage of NVS with an Outperform rating on November 9, 2010. 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				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			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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**In the past 12 months, the Firm has received compensation for providing investment banking services to Ignyta, Inc. .**

**Leerink Partners LLC makes a market in Ignyta, Inc. and Ariad Pharmaceuticals, Inc.**

**Leerink Partners LLC is willing to sell to, or buy from, clients the common stock of Novartis AG on a principal basis.**

**Leerink Partners LLC has acted as the manager for a public offering of Ignyta, Inc. in the past 12 months.**

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