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Biotechnology
Company Update
April 24, 2014
**BUY**

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# IGNYTA, INC.

## RXDX-101 Accepted for Oral Presentation at ASCO; Reiterate Buy Rating, $20PT

RXDX (NASDAQ)

| Company & Market Data | |
|---|---|
| Closing Price (as of April 23, 2014): | $7.29 |
| Rating: | BUY |
| Price Target: | $20.00 |
| 52 Week Range: | $1.00 - $20.00 |
| Shares Outstanding (MM): | 20 |
| Market Capitalization (MM): | $146 |
| Cash (MM): | $51.8 |
| Debt (MM): | $9.0 |
| Fiscal Year End: | Dec |

| Estimates | | | |
|---|---|---|---|
| **EPS** | **2013A** | **2014E** | **2015E** |
| **1Q** | — | $(0.37) | $(0.33) |
| **2Q** | — | $(0.24) | $(0.34) |
| **3Q** | — | $(0.28) | $(0.35) |
| **4Q** | — | $(0.29) | $(0.36) |
| **Full Year** | $(3.83) | $(1.15) | $(1.38) |
| **Revenue (MM)** | $0.0 | $0.0 | $0.0 |

Ignyta is developing personalized oncology drugs using diagnostic tests to identify patients most likely to respond to therapy. The company's lead product RXDX-101, is a TrkA/B/C, ROS1, ALK inhibitor in Phase I development for the treatment of solid tumors. Ignyta hopes to move the program into Phase II development in 2015 for multiple indications including NSCLC. The San Diego-based company is also in pre-clinical development of other targeted cancer therapies based on proprietary Oncolome molecular expression database.

Phase I data for RXDX-101 in treatment of solid tumors has been accepted for an oral presentation at the ASCO meeting Saturday 5/31/14 at 1:39 in Hall D2. In our view, this is a material event for RXDX shares with two important takeaways: 1) pulls forward presentation of Phase I data by 4-6 months and 2) increased visibility may accelerate enrollment in a continuous dosing Phase I study scheduled to begin in early 3Q14. Currently, the conference program for oral presentations does not appear to include data from Trk-based targeted therapies in development at other companies. We believe the decision by the ASCO scientific committee to provide a high profile venue for RXDX-101 is a validation of RXDX's first mover advantage and believe the visibility may help accelerate enrollment in a Phase I continuous dosing study scheduled to begin in early 3Q14. Importantly, acceptance of the abstract for oral presentation moves the potential inflection point for RXDX shares from the date abstracts are released for poster presentations (May 14) to the date of the oral presentation (May 31) and should allow for a more complete review of the Phase I. The abstract that will be released May 14 was submitted in late January 2014 and, in our view, is unlikely to disclose any new data. However, the oral presentation will contain data through at least mid May and, in our view, may include results from substantially all patients enrolled in the Italian Phase I study. We had expected the full Phase I data to be presented at ESMO or another medical meeting in the fall. Acceptance for an oral presentation at ASCO pulls that milestone forward 4-6 months. Reiterate Buy rating and $20.00 PT.

- **What's New?** ASCO accepted RXDX's abstract of Phase I RXDX-101 data for an oral presentation Saturday 5/31/14 at 1:39 in Hall D2. Partner Nerviano began enrolling the Phase I 3+3 dose escalation study in patients with TrkA, ROS1 or ALK gene fusions at two sites in Italy late in 2012. The protocol called for patients to be dosed once daily for 4 days of a weekly cycle and for 3 weeks of a 4 week schedule for a total of 12 days of a 28 day cycle. RXDX expects to present data from about 20 patients at ASCO from all six dose cohorts enrolled in the study including the highest dose of 1600mg/kg. Data presented previously was from the 4 lowest dose cohorts.
- **Important Incremental Update from IASLC Presentation in February:** In February 2014 at the International Association for the Study of Lung Cancer (IASLC) meeting, investigators presented preliminary Phase I data suggesting a clean safety profile and signs of clinical activity. A total of 18 patients had been enrolled with 17 in six different dose cohorts receiving at least one dose. However, follow up for the 2 highest dose cohorts was limited. Investigators reported 2 ALK+ patients demonstrated sustained clinical response. There were no dose-limiting toxicities with only 1 case of Grade III toxicity (asthenia). RXDX-101 demonstrated a half life of 20 hours and pharmacokinetics consistent with QD dosing.
- **Our Take:** We encourage investors to focus primarily on the adverse event profile at ASCO as RXDX moves into a U.S.-based continuous dosing Phase I study in early 3Q14 and would view any PRs or CRs as unexpected upside. We expect the continuous dosing Phase I study to provide the first meaningful glimpse into potential efficacy in late 4Q14 or 1Q15.

**Table 1.**

| Ignyta Income Statement | | | | | | | | | | | | |
|---|---|---|---|---|---|---|---|---|---|---|---|---|
| *(in $ millions)* | 2013A | | 1Q14E | 2Q14E | 3Q14E | 4Q14E | 2014E | | 1Q15E | 2Q15E | 3Q15E | 4Q15E | 2015E |
| Total product revenue | 0.0 | | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 |
| Other Revenue | 0.0 | | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 |
| Total Revenue | $0.0 | | $0.0 | $0.0 | $0.0 | $0.0 | $0.0 | | $0.0 | $0.0 | $0.0 | $0.0 | $0.0 |
| COGS | 0.0 | | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 |
| Gross profit | $0.0 | | $0.0 | $0.0 | $0.0 | $0.0 | $0.0 | | $0.0 | $0.0 | $0.0 | $0.0 | $0.0 |
| G&A | 3.7 | | 2.0 | 1.5 | 1.5 | 1.6 | 6.6 | | 1.8 | 1.8 | 1.7 | 1.9 | 7.2 |
| Research & development | 3.2 | | 3.0 | 3.3 | 4.0 | 4.0 | 14.3 | | 4.7 | 4.9 | 5.2 | 5.2 | 20.0 |
| Operating profit (loss) | ($6.9) | | (5.0) | (4.8) | (5.5) | (5.6) | ($20.9) | | (6.5) | (6.7) | (6.9) | (7.1) | ($27.2) |
| Interest income | 0.0 | | 0.1 | 0.1 | 0.1 | 0.1 | 0.3 | | 0.1 | 0.1 | 0.1 | 0.1 | 0.3 |
| Interest expense | (0.2) | | (0.2) | (0.2) | (0.2) | (0.2) | (0.7) | | (0.2) | (0.2) | (0.2) | (0.2) | (0.7) |
| Other | (0.1) | | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 |
| Taxes | 0.0 | | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 |
| Net profit (loss) | (7.2) | | (5.1) | (4.9) | (5.5) | (5.7) | (21.3) | | (6.6) | (6.8) | (7.0) | (7.2) | (27.7) |
| Earnings (loss) per share from continuing ops | ($1.94) | | ($0.37) | ($0.24) | ($0.28) | ($0.29) | ($1.15) | | ($0.33) | ($0.34) | ($0.35) | ($0.36) | ($1.38) |
| One-time gains (expenses) | ($0.54) | | $0.00 | $0.00 | $0.00 | $0.00 | $0.00 | | $0.00 | $0.00 | $0.00 | $0.00 | $0.00 |
| Net income (loss) as reported | (14.2) | | (5.1) | (4.9) | (5.5) | (5.7) | (21.3) | | (6.6) | (6.8) | (7.0) | (7.2) | (27.7) |
| Earnings (loss) per share as reported | ($3.83) | | ($0.37) | ($0.24) | ($0.28) | ($0.29) | ($1.15) | | ($0.33) | ($0.34) | ($0.35) | ($0.36) | ($1.38) |
| Weighted average common shares | 3.7 | | 13.9 | 20.0 | 20.0 | 20.0 | 18.5 | | 20.0 | 20.0 | 20.0 | 20.0 | 20.0 |

*Source: Company reports and Ladenburg Thalmann estimates*

**Company and Industry-Specific Risks**

We think the primary risks of an investment in RXDX shares include, but are not limited to:

**Clinical:** While efficacy and safety of other ALK inhibitors for NSCLC has been well characterized in both clinical trials and commercial experience, there can be no assurance RXDX-101 will demonstrate clinically meaningful activity in NSCLC and other solid tumors. Additionally, RXDX-101 also inhibits ROS1 and TrkA/B/C. While there is a theoretical connection between inhibition of these tyrosine kinases and anti-tumor activity for a range of solid tumors including NSCLC, colon and glioblastoma, among others, there can be no assurances that future studies can be designed to evaluate the potential efficacy of co-inhibition of these tyrosine kinases or will confirm a positive impact on disease progression or survival, if a study is conducted. In the absence of clinical outcomes data, there can be no assurance that clinicians will accept or recognize the benefit of RXDX-101 over existing ALK inhibitors such as crizotinib. Additionally, the company is developing additional targeted cancer therapies based on its proprietary Oncolome database. There can be no assurance any future studies of pipeline programs will be adequate to support regulatory approval, reimbursement or commercial acceptance of pipeline programs. Lastly, RXDX relies on a virtual clinical development business model based on a small in-house management group and third party contractors. Loss of one or more executives could have an adverse impact of future clinical trials management.

**Regulatory:** RXDX is subject to oversight by multiple groups at the U.S. FDA including the Oncologic Drugs Advisory Committee for oncology drug development and Office of In Vitro Diagnostic Device Evaluation and Safety for companion diagnostics. There can be no assurance registration studies will be adequate to support regulatory filing with ODAC for RXDX-101 or any other pipeline product. Additionally, we expect the companion to diagnostic for RXDX-101 and other pipeline programs to be commercialized through diagnostic partners. There can be no assurance RXDX or its diagnostic partners will win timely PMA clearance for companion to RXDX-101 or any other pipeline product.

**Competition:** We are not aware of any other company developing a pan-inhibitor of ALK, ROS1 and TrkA/B/C. Additionally, there are currently no ROS1 or TrkA/B/C inhibitors approved for treatment of solid tumors in the U.S. or Europe. However, several companies have disclosed plans to develop therapies targeting TrkA/B/C. We believe RXDX-101 is currently the most advanced TrkA/B/C program in clinical development. There can be no assurance RXDX will be successful in maintaining its current leadership for timely commercialization of a TrkA/B/C inhibitor. Finally, several companies are developing second-generation ALK inhibitors with better blood-brain barrier than crizotinib. Some of these programs are more advanced than RGDX-101.

**Financing:** The company believes its financial resources will fund operations into at least 2017. However, depending on the pace of business development, RXDX may need additional capital to fund operations through Phase II proof-of-concept studies of RXDX-101. If Phase II studies are successful, RXDX may need access to additional capital through either internal sources or partnerships to fund registration studies and to fund commercialization. There can be no assurance RXDX will have access to capital in the future on adequate terms, or at all.

**Partnership:** RXDX will rely on partnerships with CROs, diagnostic product companies and other service providers to support clinical development and U.S. regulatory filings for RXDX-101 and its other pipeline programs. Additionally, we expect the company to seek commercial partners for RXDX-101 and its other pipeline programs in geographies outside the United States including Europe and Asia. There can be no assurance the partners will be successful in maintaining a steady supply of drug product, provide adequate support for clinical trials enrollment, optimize appropriate companion diagnostics or offer appropriate commercialization support in Europe, Asia and other regions outside the U.S. Lastly, the company licensed rights to RXDX-101 and RXDX-102 from Nerviano Medical Sciences. While Nerviano is not responsible for conducting any future clinical development, the two companies have signed a service agreement for additional

manufacturing and clinical support services through 2014. There can be no assurance Nerviano will provide adequate support for timely future development of RXDX-101.

**Product Liability:** Pharmaceutical companies may face potential product liability lawsuits associated with adverse events – both currently identified and identified through future clinical trials and commercial experience. Product liability claims may result in limiting future product promotion, removal of one or more products from the market and potential for financial penalties and fines that may adversely impact RXDX's cash flow and financial position, including cash balance and ability to meet various debt covenants.

**Limited Operating History:** While the company was formed in 2012, RXDX had limited operations as a drug development company prior to May 2013. This limited operating history may restrict the scope of information available for investors to form an investment opinion. RXDX is classified as an emerging growth company and is entitles to more limited disclosure requirements, which may make shares of RXDX less attractive to investors. The company went public in November 2013 through a reverse merger and trading volume in shares of RXDX has limited due in part to the small number of registered shares. There can be no assurance that there will be a liquid and orderly market for trading of RXDX shares in the near term, or ever. Additionally, if one or more holders of common stock covered by an effective registration statement seeks to sell stock, the share price may be adversely impacted.

**Debt Repayment:** The company has a $10M debt facility with Silicon Valley Bank Corp. that matures in December 2017. There can be no assurance RXDX will have adequate funds to repay the loan facility or that alternative debt financing will be available on acceptable terms, if at all.

# APPENDIX A: IMPORTANT RESEARCH DISCLOSURES

**ANALYST CERTIFICATION**

I, Kevin DeGeeter, attest that the views expressed in this research report accurately reflect my personal views about the subject security and issuer. Furthermore, no part of my compensation was, is, or will be directly or indirectly related to the specific recommendation or views expressed in this research report, provided, however, that:

The research analyst primarily responsible for the preparation of this research report has or will receive compensation based upon various factors, including the volume of trading at the firm in the subject security, as well as the firm's total revenues, a portion of which is generated by investment banking activities.

Additional information regarding the contents of this publication will be furnished upon request. Please contact Ladenburg Thalmann, Compliance Department, 570 Lexington Avenue, 11th floor, New York, New York 10022 (or call 212-409-2000) for any information regarding current disclosures, and where applicable, relevant price charts, in regard to companies that are the subject of this research report.

**COMPANY BACKGROUND**

Ignyta is developing personalized oncology drugs using diagnostic tests to identify patients most likely to respond to therapy. The company's lead product RXDX-101, is a TrkA/B/C, ROS1, ALK inhibitor in Phase I development for the treatment of solid tumors. Ignyta hopes to move the program into Phase II development in 2015 for multiple indications including NSCLC. The San Diego-based company is also in pre-clinical development of other targeted cancer therapies based on proprietary Oncolome molecular expression database.

**VALUATION METHODOLOGY**

Our $20.00 price target is based on a DCF analysis assuming 25% discount rate, 21.5 million shares on a fully diluted basis, terminal year (2022) FCF of $168M and 15% long-term growth rate.

**RISKS**

These risk factors (clinical, regulatory, competition, financing, partnership, product liability, limited operating history, and debt repayment) do not constitute all the potential risks of investing in the subject company's shares. Investors should refer to the company's SEC filings including the most recent forms 10-K and 10-Q for further details on the risks associated with an investment in the subject company's shares.

**STOCK RATING DEFINITIONS**

Buy: The stock's return is expected to exceed 12.5% over the next twelve months.

Neutral: The stock's return is expected to be plus or minus 12.5% over the next twelve months.

Sell: The stock's return is expected to be negative 12.5% or more over the next twelve months.

Investment Ratings are determined by the ranges described above at the time of initiation of coverage, a change in risk, or a change in target price. At other times, the expected returns may fall outside of these ranges because of price movement and/or volatility. Such interim deviations from specified ranges will be permitted but will become subject to review.

**RATINGS DISPERSION AND BANKING RELATIONSHIPS AS OF (April 24, 2014)**

| Rating | % | IB % |
|---|---|---|
| **BUY** | 75.1 | 55.0 |
| **NEUTRAL** | 24.9 | 40.0 |
| **SELL** | 0.0 | 0.0 |

**COMPANIES UNDER ANALYST'S COVERAGE**

ADMA Biologics, Inc. (ADMA)
BG Medicine, Inc. (BGMD)
Exact Sciences Corp. (EXAS)
Genomic Health Inc. (GHDX)
Myriad Genetics Inc. (MYGN)
Novavax, Inc. (NVAX)
Response Genetics, Inc. (RGDX)
Sequenom Inc. (SQNM)
Aeolus Pharmaceuticals Inc. (AOLS)
diaDexus, Inc. (DDXS)
Genetic Technologies, Ltd. (GENE)
Mesoblast Ltd. (MBLTY)
Navidea Biopharmaceuticals Inc. (NAVB)
Opko Health, Inc. (OPK)
Ignyta, Inc. (RXDX)

**COMPANY SPECIFIC DISCLOSURES**

Ladenburg Thalmann & Co. Inc had an investment banking relationship with the Ignyta, Inc. within the last 12 months.

Ladenburg Thalmann & Co. Inc received compensation for investment banking services from Ignyta, Inc. within the past 12 months.

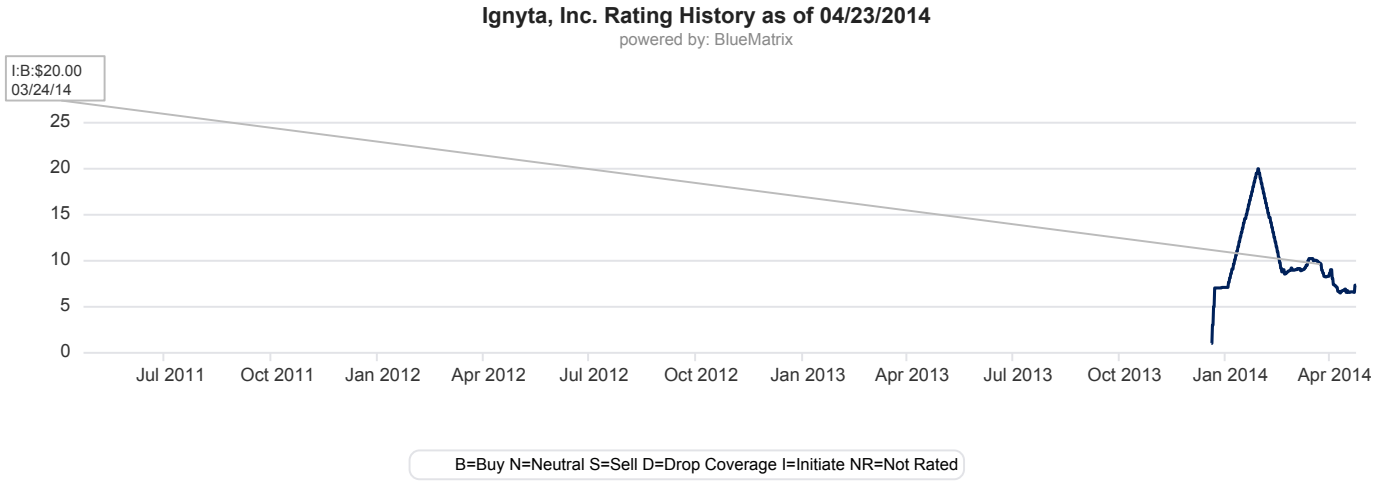
Ladenburg Thalmann & Co. Inc. has managed or co-managed a public offering for Ignyta, Inc. within the past 12 months.

Ladenburg Thalmann & Co. Inc. makes a market in Ignyta, Inc..

Ladenburg Thalmann & Co. Inc. expects to receive compensation for investment banking and/or advisory services from Ignyta, Inc. within the next 3 months.

Ladenburg Thalmann & Co. Inc. intends to seek compensation for investment banking and/or advisory services from Ignyta, Inc. within the next 3 months.

**INVESTMENT RATING AND PRICE TARGET HISTORY**

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