Zacks Small-Cap Research

August 14, 2012
Jason Napodano, CFA
312-265-9421
jnapodano@zacks.com

scr.zacks.com
111 North Canal Street, Chicago, IL 60606

Nile Therapeutics, Inc. (NLTX-OTC)

NLTX: Can A New CEO Secure Financing For Nile's Cenderitide?

Current Recommendation	**Outperform**
Prior Recommendation	N/A
Date of Last Change	01/26/2010
Current Price (08/14/12)	$0.11
Target Price	**$1.00**

UPDATE

We view Nile Therapeutics as a high risk / high reward speculative play where at $0.11 the stock is trading like a call option. We believe the current market capitalization vastly under-values the potential for cenderitide if management can secure financing to move the drug into phase 2.

We reiterate our 'Outperform' rating and $1 target. We caution investors that the name is high risk, and that future dilutive financings are a possibility if a deal does not get done by the middle of September 2012.

SUMMARY DATA

52-Week High	**$0.88**
52-Week Low	**$0.11**
One-Year Return (%)	**-86.34**
Beta	**2.15**
Average Daily Volume (sh)	**46,462**
Shares Outstanding (mil)	**43**
Market Capitalization ($mil)	**$5**
Short Interest Ratio (days)	**0.25**
Institutional Ownership (%)	**0**
Insider Ownership (%)	**23**
Annual Cash Dividend	**$0.00**
Dividend Yield (%)	**0.00**
5-Yr. Historical Growth Rates	
Sales (%)	**N/A**
Earnings Per Share (%)	**N/A**
Dividend (%)	**N/A**
P/E using TTM EPS	**N/A**
P/E using 2012 Estimate	**N/A**
P/E using 2013 Estimate	**N/A**

Risk Level	**Above Average**
Type of Stock	**Small-Growth**
Industry	**Med-Biomed/Gene**

ZACKS ESTIMATES

Revenue
(In millions of $)

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2011	0 A	0.3 A	0.8 A	0.2 A	1.4 A
2012	0.2 A	0 A	0 E	0 E	0.2 E
2013					0.0 E
2014					0.0 E

Earnings per Share
(EPS is operating earnings before non-recurring items)

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2011	-$0.03 A	-$0.03 A	-$0.03 A	-$0.04 A	-$0.13 A
2012	-$0.02 A	-$0.01 A	-$0.01 E	-$0.02 E	-$0.06 E
2013					-$0.16 E
2014					-$0.15 E

© Copyright 2012, Zacks Investment Research. All Rights Reserved.

WHAT'S NEW

Financial Review

On August 14, 2012, Nile Therapeutics reported financial results for the second quarter ended June 30, 2012. Nile did not report any revenue in the second quarter 2012. This was in-line with our model. The company has recorded all revenues and expense reimbursement associated with the phase 1 trial done in collaboration with Medtronic. In total, Nile booked $1.55 million in revenues from the Medtronic collaboration, most recently $195,500 in the first quarter 2012. We do not expect any future revenues in 2012 outside of a new collaboration for cenderitide.

Net loss for the second quarter 2012 totaled $0.35 million, or 1 cent per share. Included in the net loss was a gain of $0.42 million in reduction in outstanding warrant liability (non-cash). Loss was driven by $0.33 million in R&D and $0.44 million in SG&A. As of June 30, 2012, Nile reported cash and equivalents of $0.78 million. Management finds this sufficient to fund operations into the fourth quarter 2012. We note that Nile burned approximately $0.6 million in the first quarter and $0.8 million in the second quarter 2012. Therefore, we model the cash running out in early October 2012. We believe the company must secure financing before late September 2012 to avoid insolvency.

Can The New CEO Get It Done?

On August 3, 2012, Nile announced that Darlene Horton, MD, was appointed as the company's President and Chief Executive Officer (CEO). Dr. Horton formerly served as the company's Chief Medical Officer (CMO) from June 2012. Dr. Horton brings a nice background to Nile, having outstanding relevant experience with Natrecor:

- Before joining Nile, Dr. Horton was previously CMO of Itero Biopharmaceuticals, a venture-backed company that developed and out-licensed a biosimilar protein therapeutic. Previous to Itero, Dr. Horton served as Senior Vice President, Clinical Research at Scios, Inc., a Johnson & Johnson (J&J) company. During her 12 year tenure at Scios, she was the clinical lead for Natrecor, a commercial product indicated for the treatment of acute heart failure. Dr. Horton led the program from late-stage development through approval and commercialization. She presented the program to the FDA's Cardiovascular and Renal Advisory Committee prior to successful approval. Following the acquisition of Scios by J&J, Dr. Horton also served as the Head of the Cardiovascular Therapeutic Area Center of Excellence for all of J&J pharmaceutical companies.

Dr. Horton will be paid a monthly salary of $28,314. She is nicely incentivized to secure financing or a deal for the company. Dr. Horton's salary will increase to $33,333 and she will be eligible to receive an annual performance cash bonus in the amount up to 30% of the new base ($120,000 total), along with 10-year stock options, if Nile can secure funding via a partnership or financing to initiate and fund to completion a phase 2 clinical trial for cenderitide. We also note that Dr. Horton also has a change in control provision that would pay her a cash payment of 5% of the proceeds for such a transaction.

We expect that the phase 2 program with cenderitide in post-acute heart failure will cost an estimated $15 to $20 million and take 3 years to enroll and complete. Nile's current market capitalization is just shy of $5 million. We find it difficult to believe that the company will be able to secure $15 to $20 million in financing through the equity markets. A deal with a larger organization looking to fund the trial seems the only viable option at this point.

Call Option Thesis

We continue to view Nile Therapeutics as a call option on cenderitide. At today's price, investors need to be comfortable losing their entire investment. However, if successful in a phase 2 trial for post-acute heart failure, we think cenderitide could be worth as much as $80 to $100 million in value.

On March 26, 2012, Dr. Hsiao D. Lieu, MD, FACC, Nile Therapeutics' (NLTX) Vice President of Clinical Research, presented the results from the company's phase 1 safety and pharmacokinetic / pharmacodynamic (PK/PD) clinical trial with cenderitide at the 61th annual Scientific Session of the American College of Cardiology (ACC) meeting held in Chicago, IL. The poster presentation board #628 (http://www.nilethera.com/docs/ACC_HLIEU_2012.pdf) was titled, "Initial Experience with Subcutaneous Infusion of Cenderitide in Patients with Chronic Heart Failure."

The design of the study was a multicenter, dose escalating evaluation of the PK and PD responses to various subcutaneous doses of cenderitide boluses and infusion regimens in patients with chronic heart failure. The phase 1 study enrolled a total of 58 patients (45 on cenderitide + 13 placebo) and employed three parts:

	Description	Dose	Number
Part A	Subcutaneous bolus, 1 mL, dose escalation design	Cohort 1: Day 1 : 12 µg Day 2: 24 µg Cohort 2: Day 1: 100 µg, Day 2: 200 µg Cohort 3: Day 1 150 µg, Day 2: 200 µg	Cohort 1: N=2 Cohort 2: N=2 Cohort 3: N=8
Part B	Subcutaneous 24-hr infusion. In the dose confirmation cohorts, subjects were randomized 2:1 to cenderitide: placebo	Lead-in cohort A: 18 µg/hr Lead-in cohort B: 36 µg/hr Dose-confirmation cohort 1: 18 µg /hr Dose-confirmation cohort 2: 24 µg /hr	Cohort A: N=2 Cohort B: N=2 Cohort 1: N=10 Placebo 1: N=5 Cohort 2: N=10 Placebo 2: N=5
Part C	Subcutaneous 24-hr infusion with dosing adjusted by weight (µg/kg/hr): Planned dosing range 15 – 30 with 1.25 ug/kg/hr increments for each additional 5 kg, starting at 70 Kg. Subjects randomized 3:1 to cenderitide: placebo	Cenderitide: Actual dosed range 15 to 30 µg /kg/hr	Cenderitide: N=9 Placebo: N=3

Patients were infused using Medtronic's MiniMed Paradigm pump, currently indicated for continuous infusion of insulin. The pump utilizes MiniMed's minimally invasive, subcutaneous micro-needle technology. The goal of the phase 1 study was two-fold. From Medtronic's point of view, they wanted to test the feasibility of using the MiniMed Paradigm for indications outside of continuous insulin therapy. From Nile's point of view, the company wanted to achieve steady-state target plasma PK levels, reduce weight-based variability in dosing, and demonstrate a dose-dependent reduction in mean maximum systolic blood pressure (SBP).



...The Results...

The data show the trial was a success, from both Medtronic's and Nile's standpoint. We see nothing that prohibits movement into a phase 2 program.

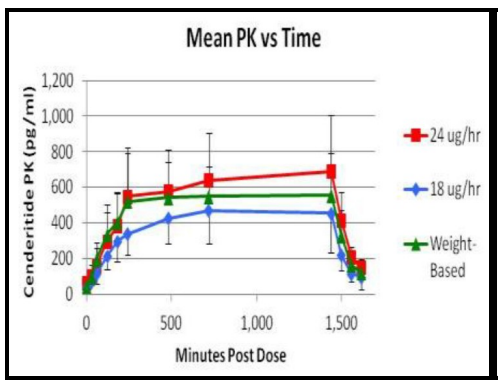


Figure 1

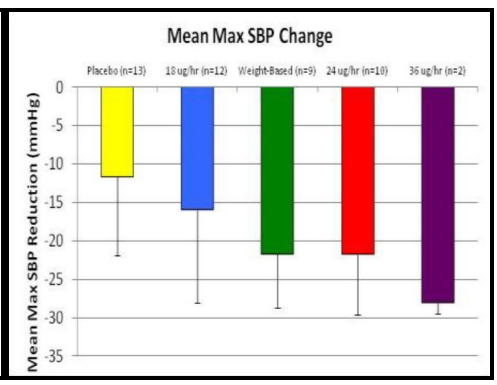


Figure 2

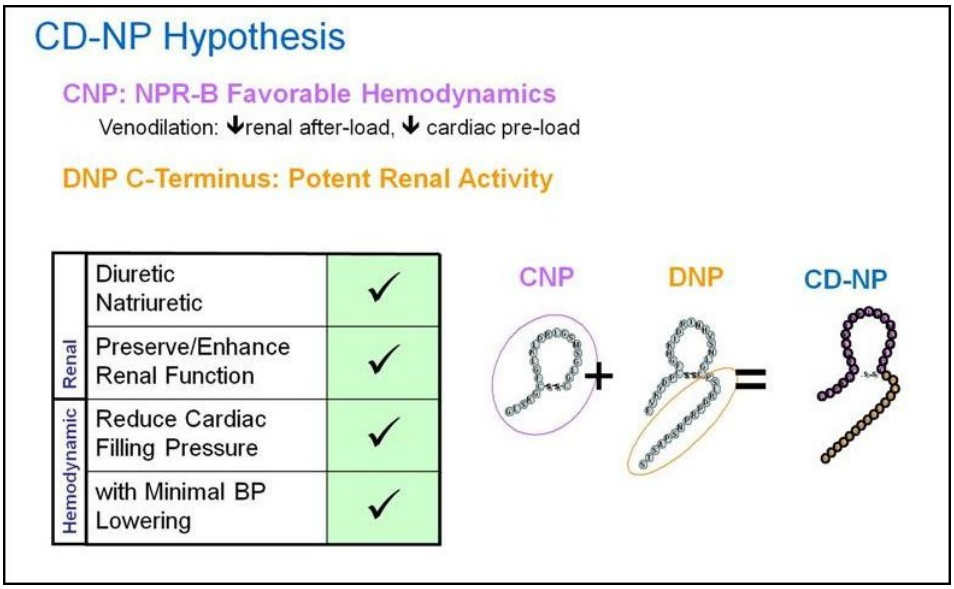
Figure-1 shows the achieved steady-state plasma levels with 18 ug/hr, 24 ug/hr, and weight-based doses. Based on preclinical and previous phase 2 data in acute heart failure patients, management at Nile targeted a cenderitide plasma concentration of 600 pg/ml. We believe the 24 ug/hr or the weight-based dose will be what Nile moves into the next proof-of-concept program.

Figure-2 shows the dose-dependent reduction in systolic blood pressure (SBP). Nile was targeting a reduction in blood pressure between 5 and 10 points beyond the placebo. Based on the results depicted in Figure-2, 18 ug/hr, 24 ug/hr, and weight-based dose all were within the target range. The dose of 36 ug/hr reduced SBP outside the target safety range.

The phase 1 trial was not adequately powered to test efficacy endpoints such as glomerular filtration rate (GFR), creatinine, cystatin-C, or urine output. We note that previous phase 2 data with cenderitide was presented at the 60th annual Scientific Session of the American College of Cardiology (ACC). The poster presentation (board #256) demonstrates encouraging proof-of-concept in 77 patients with acute decompensated heart failure (ADHF), including dose-dependent reductions in mean eGFR and serum creatinine. This trial showed trends in the all-important improvement / preservation of renal function and reductions in blood pressure designed by to make cenderitide a blockbuster drug.

...Little Background Info...

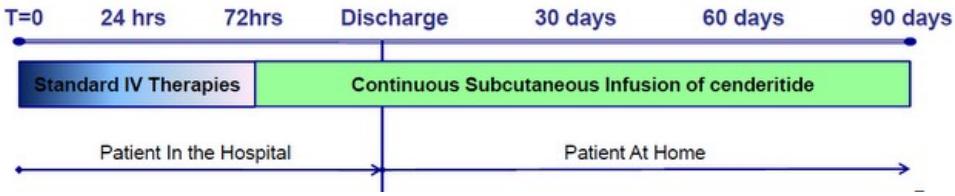
Cenderitide is Nile's novel chimeric natriuretic peptide. Natriuretic peptides are naturally occurring proteins produced in the heart and blood vessels that cause natriuresis (the removal of sodium and water from the blood). Cenderitide was rationally designed by scientists at the Mayo Clinic's cardio-renal research labs to confer both arterial and venodilation activity. Cenderitide is a combination of two natriuretic peptides, CNP and DNP. The drug combines the n-terminus of the CNP molecule, preserving the favorable venodilating and hemodynamic properties, with the c-terminus of the DNP molecule, preserving the potent direct renal activity.



Preclinical data demonstrates the drug has both natriuretic and diuretic properties. Animal studies yielded very encouraging data with respect to improvement in GFR (improvement in renal function) and less risk of hypotension as compared to BNP. Preclinical data on the mechanism of action was published in the Journal of the American College of Cardiology.

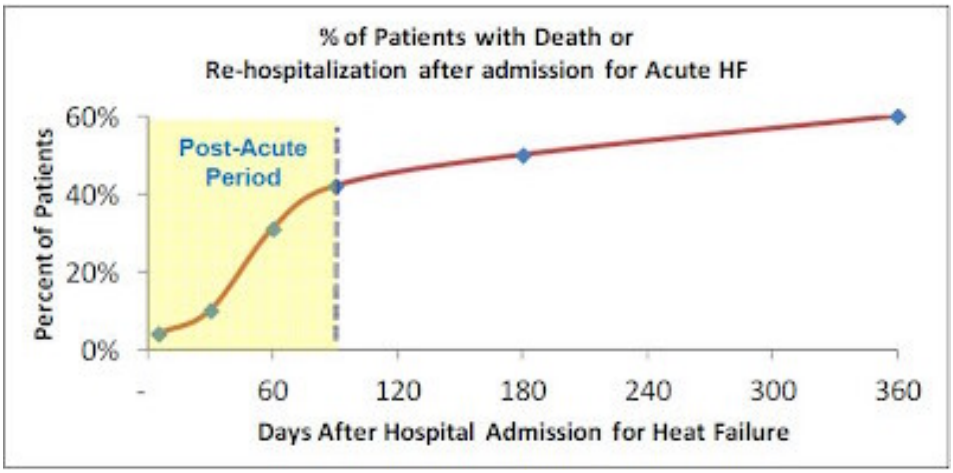
…A Potential Blockbuster Indication…

We believe the post-acute heart failure indication could be a blockbuster opportunity for cenderitide. In the U.S., approximately 5 million people (~2% of the population) suffer from heart failure (HF). The incidence is even higher for patients above the age of 65, where approximately 6% to 10% of the population is at risk. There are an estimated 650,000 new cases diagnosed annually. Treatment of heart failure generates annual costs of approximately $35 billion, of which approximately $3 billion is spent on drugs and $19 billion is spent in the acute hospital setting.



On March 31, 2011, the U.S. FDA granted Nile “Fast Track” designation for cenderitide for reducing cardiovascular mortality and cardiovascular re-hospitalization in the post-acute period in patients with acute decompensated heart failure (ADHF).

There is nothing on the market for this post-acute indication. During the acute (in-hospital) phase, patients receive standard-of-care, which includes various vasodilators (nitroglycerin), diuretics (furosemide), and inotropes (dobutamine or levosimedan). Chronic heart failure patients are typically on beta blockers, ACE inhibitors, ARB inhibitors, and may even have an implantable cardio-rhythm device. However, the 30-90 day window after hospital discharge is where the risk of re-hospitalization and death are highest. The graph below shows the post-acute risk period that Nile is focusing its development on with cenderitide.



PROJECTED FINANCIALS

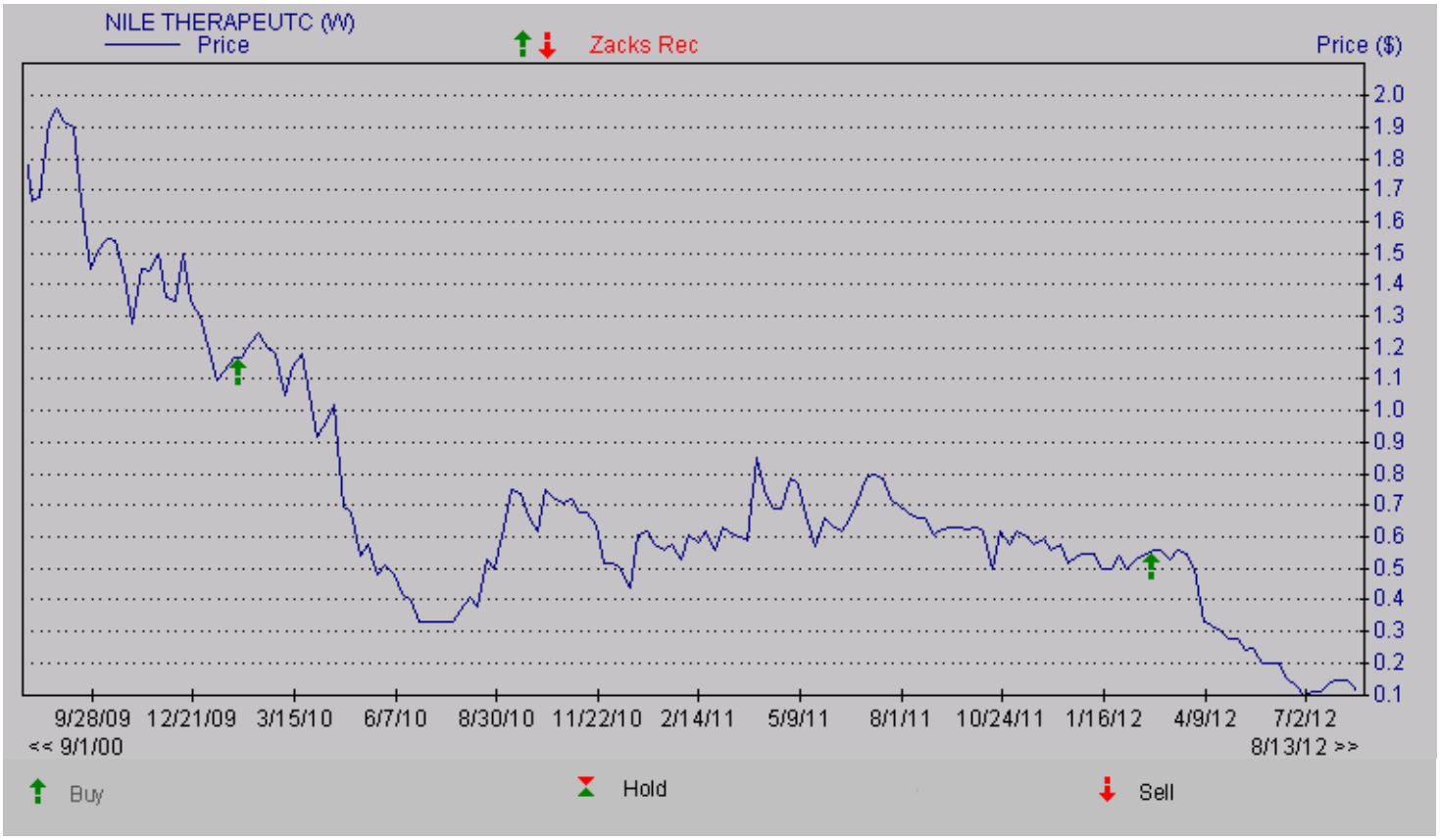
Nile Therapeutics, Inc.
Income Statement

	2010 A	2011 A	Q1 A	Q2 A	Q3 E	Q4 E	2012 E	2013 E	2014 E
cenderitide (U.S. Sales)	$0	$0	$0	$0	$0	$0	$0	$0	$0
YOY Growth	-	-	-	-	-	-	-	-	-
cenderitide (Row Sales)	$0	$0	$0	$0	$0	$0	$0	$0	$0
YOY Growth	-	-	-	-	-	-	-	-	-
cenderitide (Royalties)	**$0**	**$0**	**$0**	**$0**	**$0**	**$0**	**$0**	**$0**	**$0**
YOY Growth	-	-	-	-	-	-	-	-	-
Grants, Licenses & Milestones	**$0**	**$1.4**	**$0.2**	**$0**	**$0**	**$0**	**$0.2**	**$0.0**	**$0.0**
YOY Growth	-	-	-	-	-	-	-	-	-
Total Revenues	**$0**	**$1.4**	**$0.2**	**$0**	**$0**	**$0**	**$0.2**	**$0.0**	**$0.0**
YOY Growth	-	-	-	-	-	-	-	-	-
R&D	$4.1	$4.1	$0.5	$0.3	$0.4	$0.5	$1.7	$5.0	$5.0
% R&D	-	-	-	-	-	-	-	-	-
SG&A	$2.2	$2.1	$0.5	$0.4	$0.5	$0.5	$1.9	$2.2	$2.5
% SG&A	-	-	-	-	-	-	-	-	-
Operating Income	($6.3)	($4.9)	($0.8)	($0.8)	($0.9)	($1.0)	($3.4)	($7.2)	($7.5)
Operating Margin	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*
Interest & Other Income	$0.3	$0.0	($0.0)	$0.4	$0.3	$0.0	$0.7	$0.1	$0.1
Pre-Tax Income	($6.0)	($4.9)	($0.8)	($0.4)	($0.6)	($1.0)	($2.7)	($7.1)	($7.4)
Taxes	$0	$0	$0	$0	$0	$0	$0	$0	$0
Tax Rate	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*
Net Income	($6.0)	($4.9)	($0.8)	($0.4)	($0.6)	($1.0)	($2.7)	($7.1)	($7.4)
Net Margin	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*
Reported EPS	**($0.19)**	**($0.13)**	**($0.02)**	**($0.01)**	**($0.01)**	**($0.02)**	**($0.06)**	**($0.16)**	**($0.15)**
YOY Growth	*-39.8%*	*-30.3%*	*-43.6%*	*-67.2%*	*-51.7%*	*-44.4%*	*-50.7%*	*144.4%*	*-6.2%*
FAS-123R Expense	$1.1	$0.8	$0.2	$0.1	$0.1	$0.2	$0.6	$1.0	$1.5
EPS Impact of FAS-123R	*($0.03)*	*($0.02)*	*($0.00)*	*($0.00)*	*($0.00)*	*($0.00)*	*($0.01)*	*($0.02)*	*($0.03)*
Weighted Ave. Shares Out	32.1	37.3	39.7	43.0	43.1	43.1	42.2	45.0	50.0

Source: Zacks Investment Research, Inc. *Jason Napodano, CFA*

© Copyright 2012, Zacks Investment Research. All Rights Reserved.

HISTORICAL ZACKS RECOMMENDATIONS



DISCLOSURES

The following disclosures relate to relationships between Zacks Investment Research ("ZIR") and Zacks Small-Cap Research ("Zacks SCR") and the issuers covered by the Zacks SCR analysts in the Small-Cap Universe.

ZIR or Zacks SCR Analysts do not hold or trade securities in the issuers which they cover. Each analyst has full discretion on the rating and price target based on their own due diligence. Analysts are paid in part based on the overall profitability of Zacks SCR. Such profitability is derived from a variety of sources and includes payments received from issuers of securities covered by Zacks SCR for non-investment banking services. No part of analyst compensation was, is or will be, directly or indirectly, related to the specific recommendations or views expressed in any report or blog.

ZIR and Zacks SCR do not make a market in any security nor do they act as dealers in securities. Zacks SCR has never received compensation for investment banking services on the small-cap universe. Zacks SCR does not expect received compensation for investment banking services on the small-cap universe. Zacks SCR has received compensation for non-investment banking services on the small-cap universe, and expects to receive additional compensation for non-investment banking services on the small-cap universe, paid by issuers of securities covered by Zacks SCR. Non-investment banking services include investor relations services and software, financial database analysis, advertising services, brokerage services, advisory services, investment research, and investment management.

Additional information is available upon request. Zacks SCR reports are based on data obtained from sources we believe to be reliable, but is not guaranteed as to accuracy and does not purport to be complete. Because of individual objectives, the report should not be construed as advice designed to meet the particular investment needs of any investor. Any opinions expressed by Zacks SCR Analysts are subject to change. Reports are not to be construed as an offer or the solicitation of an offer to buy or sell the securities herein mentioned. Zacks SCR uses the following rating system for the securities it covers. Buy/Outperform: The analyst expects that the subject company will outperform the broader U.S. equity market over the next one to two quarters. Hold/Neutral: The analyst expects that the company will perform in line with the broader U.S. equity market over the next one to two quarters. Sell/Underperform: The analyst expects the company will underperform the broader U.S. Equity market over the next one to two quarters.

The current distribution of Zacks Ratings is as follows on the 1036 companies covered: Buy/Outperform- 15.5%, Hold/Neutral- 76.9%, Sell/Underperform – 6.9%. Data is as of midnight on the business day immediately prior to this publication.

© Copyright 2012, Zacks Investment Research. All Rights Reserved.