

Vital Therapies, Inc.

Update on ELAD Clinical Program; Highlights From Investor Meetings; Raising Price Target to \$36

On Wednesday, July 9, Vital Therapies provided an update on the status of the ELAD clinical program, and in subsequent days we hosted investor meetings with the management team. We highlight herein the major points of discussions in the investor meetings. We also revise a few assumptions in our model, the most important being the pricing increase for the ELAD system from \$125,000 to \$150,000 per treatment at launch. As a result, we increase our price target from \$28 to \$36 and maintain our Outperform rating (exhibit 1, on page 4).

In the July 9 update on the status of the ELAD clinical program, the most incremental information was regarding the guidance from European Medical Agency (EMA) related to the VTI-210 study. The ELAD clinical development program covers several major subgroups of ALF and acute-on-chronic liver failure (ACLF) with survival as the primary endpoint. We illustrate the ELAD system development timeline in exhibit 2, on page 5, and summarize below.

- As of July 9, 123 of the 200 expected patients have been enrolled into the VTI-208 study, the first Phase III study of the ELAD clinical and registrational program. Among the 48 clinical sites now open throughout the United States, the United Kingdom, Spain, and Australia, 37 have enrolled at least one patient. The company continues to expect top-line data during second quarter 2015. VTI-208 is a randomized, open-label, multicenter, controlled study investigating the effects of ELAD in combination with standard therapy of the study site versus standard therapy alone in patients with alcohol-induced liver decompensation (AILD). The primary endpoint is overall survival at 90 days; secondary endpoints of the study include overall survival at 28 days and Model for End-Stage Liver Disease (MELD)-based time to progression. Long-term effects of ELAD will also be reported, as subjects will be followed for an additional five years in an extension study. The VTI-208 study is designed with 95% power to demonstrate a 20% delta in overall survival between the study arms.
- For the second Phase III study, VTI-210, which is primarily conducted in Europe in acute alcoholic hepatitis (AAH) patients who failed steroids, the recent EMA guidance stipulates that patients be stratified based on AAH diagnosis by biopsy or clinical grounds without biopsy. Vital Therapies will amend study protocols to comply with the EMA guidance, and increase the total targeted patient enrollment from 120 to 150. Top-line data from the study is now expected during 2016 instead of early 2016. VTI-210 is a randomized, open-label, multicenter, controlled study investigating the effects of ELAD in combination with standard therapy of the study site versus standard therapy alone in AAH patients, a subset of AILD. VTI-210 will be primarily conducted in Europe, where steroid use in AAH patients is considered frontline therapy. Initiated in April 2014, the study will enroll acute alcoholic hepatitis (AAH) patients who have failed at least seven days but no more than nine days of steroid therapy, according to predefined criteria. The primary endpoint of the study is overall survival at 90 days; the secondary objective of the study is survival at 28 days. Similar to the VTI-208 study, the VTI-210 study is designed with 95% power to achieve statistical significance for the primary endpoint of overall survival.

Vital Therapies, Inc. is a hybrid biopharmaceutical-medical technology company based in San Diego, California, focused on the development of its ELAD technology system as a treatment for patients with acute liver failure.

Y. Katherine Xu, Ph.D.
+1 212 237 2758
kxu@williamblair.com

Filippo Petti
+1 212 237 2741
fpetti@williamblair.com

Please consult pages 6-7 of this report for all disclosures. Analyst certification is on page 6. William Blair & Company, L.L.C. does and seeks to do business with companies covered in its research reports. As a result, investors should be aware that the firm may have a conflict of interest that could affect the objectivity of this report. Investors should consider this report as a single factor in making an investment decision.

July 14, 2014

Stock Rating: **Outperform**
Company Profile: **Aggressive Growth**
Price Target: **\$36.00**

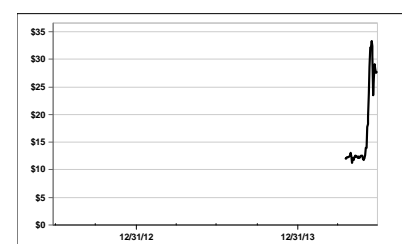
Symbol: VTL (NASDAQ)
Price: \$23.52 (52-Wk.: \$11-\$35)
Market Value (mil.): \$513
Fiscal Year End: December
Long-Term EPS Growth Rate:
Dividend/Yield: None

	2013A	2014E	2015E
Estimates			
EPS FY	\$-74.86	\$-3.08	\$-2.27
CY		\$-3.08	\$-2.27
Sales (mil.)	0	0	0
Valuation			
FY P/E	NM	NM	NM
CY P/E		NM	NM

Trading Data (FactSet)	
Shares Outstanding (mil.)	1
Float (mil.)	15
Average Daily Volume	98,733

Financial Data (FactSet)	
Long-Term Debt/Total Capital (MRQ)	0.0
Book Value Per Share (MRQ)	-95.7
Return on Equity (TTM)	-45.0

Two-Year Price Performance Chart



Sources: FactSet, William Blair & Company estimates

As part of the process for preparing a possible marketing authorization application (MAA) based primarily on the VTI-210 study, the company submitted a request for advice through the Scientific Advice Working Party (SAWP) process at the EMA on topics related to the production and testing section of a future MAA, as well as on aspects of the VTI-210 protocol design. On June 30, the company received a detailed response from SAWP that confirmed that the production and testing guidance was consistent with the company's prior interaction with the regulatory agency. In addition, the SAWP guidance also allowed for the stratification of study patients into groups based on AAH diagnosis either by biopsy or by clinical grounds without biopsy. As a result, the company will amend the study protocol to be consistent with the SAWP guidance, and will increase the size of the VTI-210 study from 125 patients to a minimum of 150 patients. The company now expects preliminary data from the study to be available during 2016, as compared with the previous guidance of late 2015 or early 2016. The company noted that four sites are currently open, but have not enrolled any patients.

- ***Enrollment in the Phase II/III VTI-212 study in patients with FHF or SILF is underway and slightly ahead of schedule.*** The Phase II single-arm portion of the study will enroll 40 patients with fulminant hepatic failure (FHF) or surgery-induced liver failure (SILF). The planned primary endpoint of the Phase II component of the study is 28-day survival, which will be compared with historical or matched controls. Enrollment in the study is in progress and we continue to anticipate top-line data from the Phase II portion of the study by late 2015 or early 2016. We note that results from the Phase II portion of the study might be sufficient for an expedited regulatory approval pathway; however, in the event a Phase III study is necessary for the indication, the study design would be finalized based on analysis of the Phase II component.

During our investor meetings, besides the update on the clinical programs, the following topics were the most discussed: 1) potential pricing of the ELAD system at launch and overall commercial prospect; 2) gross margin assumptions related to the ELAD system; 3) commercialization assumptions associated with the customized heart-lung machine component of the ELAD system; and 4) the company's cash runway, projected to sustain operations through second quarter 2016.

- ***Pricing consultants to Vital Therapies suggest that ELAD could be priced in the range of \$150,000 to \$275,000 per treatment, suggesting a potential market for ELAD of over \$4.5 billion in the United States alone. There is no known direct competition in development.*** We note that the cost associated with a liver transplant is estimated to be more than \$500,000. If ELAD can not only save but also prolong the lives of patients by decades who are either ineligible for transplant or waitlisted for transplant, the value proposition for ELAD therapy would be strong, in our opinion. We note that the addressable patient population in the United States for the ELAD system is approximately 30,000. Using the pricing assumptions offered by management, we calculate that the U.S. commercial market for ELAD could range between \$4.5 billion and \$8.3 billion. With an additional 20,000 addressable patients with AAH in the Europe, the European commercial opportunity for ELAD could range between \$3.0 billion and \$5.5 billion. With a well-defined market and no competition in sight, ELAD could become the standard-of-care for these indications and a commercial blockbuster should Phase III programs be successful.
- ***Cost of goods sold is expected to decrease from 20% at launch to around 10% at peak sales.*** We include three major components in our model assumption for cost of goods sold (COGS) for the ELAD system. First, there are cartridge costs of roughly \$10,000 per unit (four cartridges per unit, or one treatment). Second, we include costs associated with dedicated specialists assigned to monitoring the ELAD system during therapy of \$10,000 per treatment. We note that upon commercialization, the company plans to launch with 50 dedicated specialists tending to the ELAD device at the various treatment centers in the United States. The company is currently using 20 specialists in the ELAD clinical program, with six teams comprising three specialists along with an additional two floating specialists. Third, we include associated amortization of \$10,000 per treatment. Based on these assumptions, we estimate cost of goods sold of \$30,000, or a COGS of 20%, based on a course price of \$150,000. Management noted that COGS will gradually decrease as the cartridge production cost decreases with scale and as operation of the system transfers from company-hired specialists to center nursing staff. At peak sales, the company anticipates COGS at 10%.
- ***Full-scale commercialization of ELAD will require about 1,000 heart-lung machines, with roughly 2.5 ELAD systems on average per treatment center.*** The company plans to commercialize the ELAD system on its own in both the United States and Europe. The market is highly concentrated, with an estimated 200 centers, composed of about 100 liver transplant centers and roughly 100 specialist intensive care units (ICUs) in the United States that treat ALF patients; we note that the market composition in Europe across the "big five" countries (Germany, France, the United Kingdom, Spain, and Italy) is similar to that of the United States. The company estimates that given the addressable

patient population in the regions, each center will require two to three heart-lung machines. With approximately 400 centers, we calculate that the company will require 1,000 heart-lung machines for full-scale commercialization.

The company owns 28 heart-lung machines and has recently ordered an additional 5 machines. The company plans to own between 40 and 50 heart-lung machines by the U.S. commercial launch by the end of 2016. We note the cost of the heart-lung bedside unit is around \$100,000 to \$150,000 and requires between six and eight weeks for delivery by the manufacturer (Sorin). Then the company will add the ELAD component to the heart-lung machine, which takes about a week. We estimate that the company may spend over \$140 million to acquire the necessary number of heart-lung machines over time for the peak commercial launch; however, we note that 1) the company may outsource the production of the heart-lung machine to an original equipment manufacturer (OEM), which could significantly drive down the cost of the units; and 2) the scale up to peak market penetration of the ELAD system into all centers will likely take several years, leading to a steady impact on capital expenditures. The company noted that it intends to rent the heart-lung bedside units to the centers for a minimal cost, likely \$1.

- ***The current cash runway is expected to sustain operations through top-line data from VTI-208 and into mid-2016. We believe the company may elect to opportunistically raise additional capital to extend the runway through top-line data from VTI-210 and VTI-212, which are anticipated during 2016.*** Management noted that current cash on hand is roughly \$90 million. The company anticipates to burn on average \$3.5 million per month through first half 2016. We believe that based on the current timelines of the ELAD clinical program, the company may choose to raise additional funding before the readout of the VTI-208 study anticipated in second quarter 2015, to extend the cash runway through the data readouts from the VTI-210 and VTI-212 studies.

We revised several assumptions in our model, resulting in an increase in our price target from \$28 to \$36. We maintain our estimate of the success of the clinical program at 70%, and our Outperform rating. Revisions to our model include:

- ***We increased our launch price assumption for ELAD therapy from \$125,000 to \$150,000, which is the lower end of the range suggested by the company's pricing consultants.*** We continue to believe that our assumption is conservative and that additional upside exists to our model given the wholesale acquisition cost (WAC) range between \$150,000 and \$275,000 based on analyses provided by pricing experts. We continue to assume a gross-to-net discount rate for ELAD of 15% and 8% in the United States and Europe, respectively. In addition, we maintain our annual 1% price increase in the United States and flat pricing in Europe.
- ***As a result, we increased our peak worldwide sales estimates for the ELAD system from \$1.3 billion to \$1.6 billion in 2032.*** We now assume peak sales in the United States and Europe of \$965 million and \$625 million, respectively, compared with our previous estimates of \$800 million and \$520 million, respectively. We continue to believe our market penetration estimate for the ELAD system in the overall U.S. and European ALF market of roughly 10% to 15% is conservative. Should the adoption rates for ELAD be higher, and if pricing is higher, the ELAD commercial opportunity could reach multibillion-dollar levels.
- ***Gradually decreased COGS from 20% at launch in 2017 to 10% at peak in 2030.*** We had previously modeled COGS flat at 15% from launch to peak.
- ***Included \$140 million in capital expenditures related to the ELAD system's heart-lung bedside unit.*** We now estimate that Vital Therapies will acquire an additional 950 heart-lung bedside units over the next decade for the ELAD commercial launch, allowing the company to place on average 2.5 units per treatment center both in the United States and in Europe.
- ***We have updated our time-of-valuation for our risk-adjusted model from the end of 2014 to mid-2015.***
- ***Our probability adjusted NPV model continues to assume a 70% probability of success for the ELAD system development program.***
- ***Our model now suggests a fair value for the ELAD system of \$34 at mid-2015, with \$21 attributed to the United States and \$13 to Europe. Adding \$2 of cash at mid-2015, we now derive a 12-month price target of \$36.*** We note that according to our model, the upper end of management's guidance on ELAD's pricing of \$275,000 per treatment would lead to a 12-month price target of \$92.

We believe a number of catalysts will drive value in Vital Therapies stock over the next 12-24 months, including: 1) top-line data from the Phase III VTI-208 study in AILD patients expected in second quarter 2015; 2) potential submission of the ELAD system biological license application (BLA) to the Food and Drug Administration by the end of 2015; 3) top-line data from the Phase II component of the VTI-212 study in FHF and SILF in late 2015 or early 2016; 4) top-line data from the Phase III VTI-210 study in AAH in 2016; and 5) potential FDA approval and U.S. commercial launch of the ELAD system in second half 2016.

Background on ELAD. The ELAD System is the first human-cell-based bioartificial liver (BAL) therapy to be evaluated in Phase III clinical development for the treatment of ALF. ELAD is an allogeneic cellular therapy system in which human-liver-derived cells, known as C3A cells, contained in a single-use disposable set of four hollow fiber cartridges, are incorporated into a reusable, customized heart-lung machine—a device typically used in open-heart surgery to support the body during the surgical procedure. The heart-lung machine provides extracorporeal circulation of the patient’s blood plasma to the cartridges containing the C3A cells for a two-way exchange of toxins, metabolites, and nutrients, and then returns the plasma to the patient. The ELAD system is specifically designed to simulate liver function while the patient’s liver is given an opportunity to recover its regenerative properties.

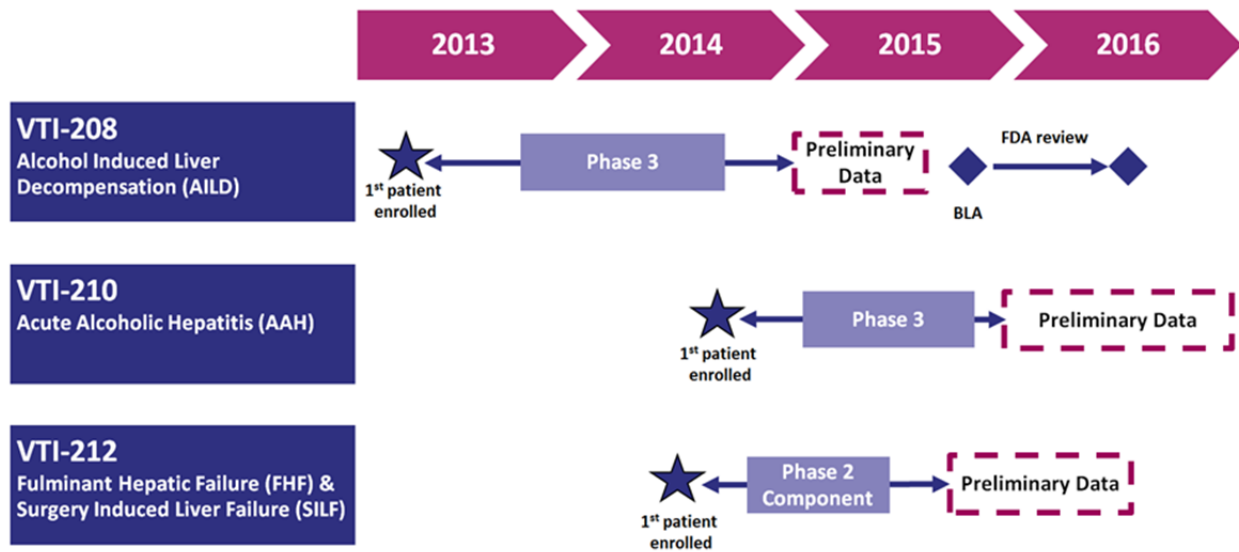
Key risks to our Outperform rating and price target include: 1) clinical risk of the Phase III program, which was based on trends observed in previous Phase II studies; 2) regulatory risk given the Food and Drug Administration’s (FDA) concern that the Phase III VTI-208 and VTI-210 studies are open label and not blinded; 3) regulatory risk associated with a drug/device combination requiring approval from FDA’s Center for Biologics Evaluation and Research and Center for Devices and Radiological Health; 4) reimbursement risk provided that the process might be long and arduous; 5) commercialization risk if the ALF market is smaller than expected and/or difficult to penetrate; 6) manufacturing risks associated with Vital Therapies’ proprietary C3A cells and cartridges; and 7) technical risk, considering that the ELAD system comprises a hybrid biologic and medical device and that a number of components of the medical device are outsourced by third parties.

Exhibit 1
Vital Therapies, Inc.
Sum-of-the-Parts Fair Value
(dollars in thousands, except shares)

Drug Candidate	Peak Sales	Stage of Development	Estimated Launch Date	Probability of Commercialization	Percentage of Sales to Company	Probability-Adjusted NPV	Value Per Share	Percentage of Fair Value
ELAD system—United States	\$964,177	Phase III	H2:2016	70%	100%	\$455,939	\$20.87	57.5%
ELAD system—European Union	\$627,036	Phase III	H1:2017	70%	100%	\$288,484	\$13.20	36.4%
Subtotal						\$744,423	\$34.07	93.9%
Net Cash at mid-Year 2015						\$66,555	\$3.05	8.4%
Net Present Value of additional Gain (Loss)*						(\$17,857)	(\$0.82)	(2.3%)
Sum-of-Parts Fair Value						\$793,121	\$36.30	100.0%

* Includes costs not directly related to programs above
Sources: Company reports and William Blair & Company, L.L.C. estimates

Exhibit 2
Vital Therapies, Inc.
Clinical and Regulatory Timelines for ELAD System



Source: Vital Therapies, Inc. and William Blair & Company, L.L.C.

William Blair & Company, L.L.C.

IMPORTANT DISCLOSURES

William Blair was a manager or co-manager of a public offering of equity securities for Vital Therapies, Inc. within the prior 12 months.

William Blair is a market maker in the security of Vital Therapies, Inc. and may have a long or short position.

William Blair intends to seek investment banking compensation in the next three months from Vital Therapies, Inc.

Within the past 12 months William Blair has provided or is providing investment banking services to or has an investment services relationship with Vital Therapies, Inc.

Additional information is available upon request.

This report is available in electronic form to registered users via R*Docs™ at www.rdocs.com or www.williamblair.com.

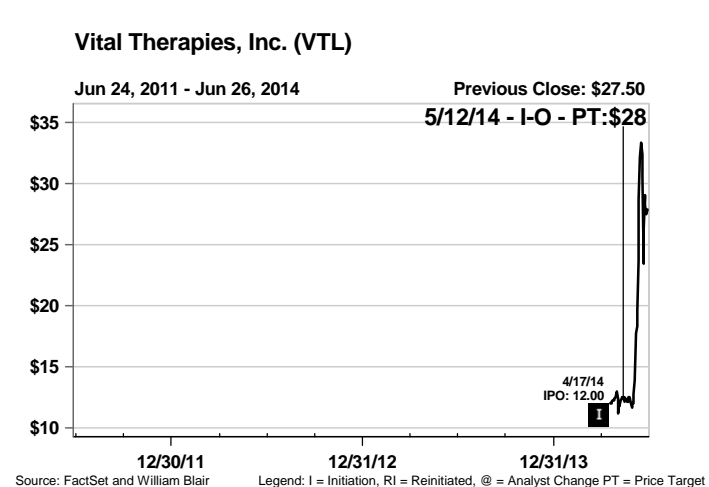
Please contact us at +1 800 621 0687 or consult williamblair.com/Research-and-Insights/Equity-Research/Coverage.aspx for all disclosures.

Y. Katherine Xu attests that 1) all of the views expressed in this research report accurately reflect his/her personal views about any and all of the securities and companies covered by this report, and 2) no part of his/her compensation was, is, or will be related, directly or indirectly, to the specific recommendations or views expressed by him/her in this report. We seek to update our research as appropriate, but various regulations may prohibit us from doing so. Other than certain periodical industry reports, the majority of reports are published at irregular intervals as deemed appropriate by the analyst.

DOW JONES: 16,943.81

S&P 500: 1,967.57

NASDAQ: 4,415.49



Current Rating Distribution (as of 06/30/14)

Coverage Universe	Percent	Inv. Banking Relationships*	Percent
Outperform (Buy)	67	Outperform (Buy)	16
Market Perform (Hold)	30	Market Perform (Hold)	2
Underperform (Sell)	1	Underperform (Sell)	0

*Percentage of companies in each rating category that are investment banking clients, defined as companies for which William Blair has received compensation for investment banking services within the past 12 months.

The compensation of the research analyst is based on a variety of factors, including performance of his or her stock recommendations; contributions to all of the firm's departments, including asset management, corporate finance, institutional sales, and retail brokerage; firm profitability; and competitive factors.

OTHER IMPORTANT DISCLOSURES

Stock ratings, price targets, and valuation methodologies: William Blair & Company, L.L.C. uses a three-point system to rate stocks. Individual ratings and price targets (where used) reflect the expected performance of the stock relative to the broader market (generally the S&P 500, unless otherwise indicated) over the next 12 months. The assessment of expected performance is a function of near-, intermediate-, and long-term company fundamentals, industry outlook, confidence in earnings estimates, valuation (and our valuation methodology), and other factors. Outperform (O) – stock expected to outperform the broader market over the next 12 months; Market Perform (M) – stock expected to perform approximately in line with the broader market over the next 12 months; Underperform (U) – stock expected to underperform the broader market over the next 12 months; not rated (NR) – the stock is not currently rated. The valuation methodologies used to determine price targets (where used) include (but are not limited to) price-to-earnings multiple (P/E), relative P/E (compared with the relevant market), P/E-to-growth-rate (PEG) ratio, market capitalization/revenue multiple, enterprise value/EBITDA ratio, discounted cash flow, and others.

Company Profile: The William Blair research philosophy is focused on quality growth companies. Growth companies by their nature tend to be more volatile than the overall stock market. Company profile is a fundamental assessment, over a longer-term horizon, of the business risk of the company relative to the broader William Blair universe. Factors assessed include: 1) durability and strength of franchise (management strength and track record, market leadership, distinctive capabilities); 2) financial profile (earnings growth rate/consistency, cash flow generation, return on investment, balance sheet, accounting); 3) other factors such as sector or industry conditions, economic environment, confidence in long-term growth prospects, etc. Established Growth (E) – Fundamental risk is lower relative to the broader William Blair universe; Core Growth (C) – Fundamental risk is approximately in line with the broader William Blair universe; Aggressive Growth (A) – Fundamental risk is higher relative to the broader William Blair universe.

The ratings, price targets (where used), valuation methodologies, and company profile assessments reflect the opinion of the individual analyst and are subject to change at any time.

Our salespeople, traders, and other professionals may provide oral or written market commentary or trading strategies—to our clients and our trading desks—that are contrary to opinions expressed in this research. Certain outstanding reports may contain discussions or investment opinions relating to securities, financial instruments and/or issuers that are no longer current. Always refer to the most recent report on a company or issuer before making an investment decision. Our asset management and trading desks may make investment decisions that are inconsistent with recommendations or views expressed in this report. We will from time to time have long or short positions in, act as principal in, and buy or sell the securities referred to in this report. Our research is disseminated primarily electronically, and in some instances in printed form. Electronic research is simultaneously available to all clients. This research is for our clients only. No part of this material may be copied or duplicated in any form by any means or redistributed without the prior written consent of William Blair & Company, L.L.C.

THIS IS NOT IN ANY SENSE A SOLICITATION OR OFFER OF THE PURCHASE OR SALE OF SECURITIES. THE FACTUAL STATEMENTS HEREIN HAVE BEEN TAKEN FROM SOURCES WE BELIEVE TO BE RELIABLE, BUT SUCH STATEMENTS ARE MADE WITHOUT ANY REPRESENTATION AS TO ACCURACY OR COMPLETENESS OR OTHERWISE. OPINIONS EXPRESSED ARE OUR OWN UNLESS OTHERWISE STATED. PRICES SHOWN ARE APPROXIMATE.

THIS MATERIAL HAS BEEN APPROVED FOR DISTRIBUTION IN THE UNITED KINGDOM BY WILLIAM BLAIR INTERNATIONAL, LIMITED, REGULATED BY THE FINANCIAL CONDUCT AUTHORITY (FCA), AND IS DIRECTED ONLY AT, AND IS ONLY MADE AVAILABLE TO, PERSONS FALLING WITHIN COB 3.5 AND 3.6 OF THE FCA HANDBOOK (BEING “ELIGIBLE COUNTERPARTIES” AND “PROFESSIONAL CLIENTS”). THIS DOCUMENT IS NOT TO BE DISTRIBUTED OR PASSED ON TO ANY “RETAIL CLIENTS.” NO PERSONS OTHER THAN PERSONS TO WHOM THIS DOCUMENT IS DIRECTED SHOULD RELY ON IT OR ITS CONTENTS OR USE IT AS THE BASIS TO MAKE AN INVESTMENT DECISION.

“William Blair” and “R*Docs” are registered trademarks of William Blair & Company, L.L.C. Copyright 2014, William Blair & Company, L.L.C. All rights reserved.