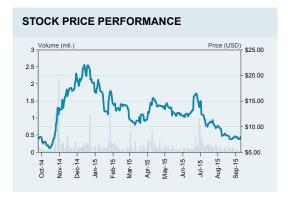


# Vitae Pharmaceuticals, Inc. (VTAE)

Phase I Results Demonstrate VTP-43742 is Off to a Good Start

MARKET DATA	
Price	\$7.93
52-Week Range:	\$5.41 - \$23.35
Shares Out. (M):	21.8
Market Cap (\$M):	\$172.9
Average Daily Vol. (000):	41.0
Cash (M):	\$80
Cash/Share:	\$3.68
Enterprise Value (M):	\$140
Float (M):	20.4
LT Debt (M):	\$0
Source: Thomson Reuters and JMP Securities LLC	

FY DEC		2014A	2015E	2016E
Revenue (\$M)	) 1Q		\$0.2A	
	2Q		\$0.2A	
	3Q	\$6.2	\$0.0	
	4Q	\$0.2	\$0.0	
	FY	\$8.7	\$0.0	\$0.0
EPS	1Q		(\$0.47)A	
	2Q		(\$0.45)A	
	3Q	(\$1.04)	(\$0.50)	
	4Q	(\$0.40)	(\$0.52)	
	FY	(\$3.61)	(\$1.93)	(\$2.07)
Source: Company reports and JMP Securities LLC				



MARKET PERFORM | Price: \$7.93 | Target Price: N/A

## **INVESTMENT HIGHLIGHTS**

Vitae Pharmaceuticals reports Phase I results from the single-ascending dose study of VTP-43742, and while data show the drug has favorable pharmacologic properties, we would like to see more data before we are confident in the probability of success; reiterate our Market Perform rating. Recall, VTP-43742 is the company's wholly-owned RORyt program that we believe is potentially a best-inclass inhibitor for autoimmune disease. The company stated that the single-ascending dose study in normal volunteers indicated that the compound was safe and well-tolerated, and exhibited a half-life of 30 hours, consistent with the requirement for around-the-clock suppression of the target. In ex vivo experiments, VTAE also showed that VTP-43742 was capable of suppressing the inflammatory cytokine, IL-17A, in blood obtained from these volunteers by up to 90% and sustained that effect for 24 hours.

Phase I multiple-ascending dose (MAD) began in August; we await data in psoriasis patients before the end of the year. In our view, the results from the MAD study are exponentially more important than the data from a single-dose trial in a population of normal individuals. Not only do we want to see a consistent effect in the Phase Ib patient population, but with other pharmacologic properties as well, such as longer-term safety, metabolism, and consistency of effect. We are less concerned about the psoriasis indication, per se, as we are about continued suppression of IL-17A, the key to success for a therapy targeted at autoimmune disease. Recall, the psoriasis patient cohort is being conducted as a randomized, double-blind, placebo-controlled trial over four weeks with the percentage change in PASI score as an expected endpoint.

Autoimmune disease and VTP-43742. As a reminder to investors, Vitae is developing the preclinical compound VTP-43742 for the treatment of various autoimmune related diseases, including multiple sclerosis, psoriasis, and rheumatoid arthritis. Disease can arise when a number of immune cells are incorrectly triggered or conversely suppressed, causing the immune system to begin recognizing "self" as "non-self". Th17 cells are a specific subset of white blood cells that stimulate the activity of effector cells, such as macrophages, through the secretion of various cytokines (Figure 1). Suppression of Th17 cells has become a potent and validated mechanism supported by several marketed products as well as others in development (Stelara/ustekinumab, JNJ (NC); secukinumab, Novartis (NC); brodalumab, Amgen (NC); and ixekizumab, Eli Lilly (NC)). The central signaling pathway that is activated in stimulated Th17 cells is the RAR-related orphan receptor γ-t (ROR-γt).

VTP-43742 was developed to inhibit signaling of ROR- $\gamma$ t (Figure 2) while maintaining an ideal balance of selectivity, efficacy, and bioavailability. Earlier studies with inhibitors of ROR family proteins found isoform selectivity to be critical to avoid unwanted side effects, likely related to the important role that the ROR- $\beta$ 

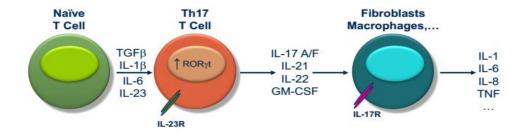
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isoform plays in eye and neuronal development. Preclinical studies of VTP-43742 have demonstrated activity in a mouse model of multiple sclerosis, EAE, as well as a high degree of oral bioavailability and long half-life. Figure 2 demonstrates that animals treated with VTP-43742 showed improvement in clinical score and a high degree of myelination compared to control mice, suggesting significant efficacy (Figures 3 and 4). When VTP-43742 was explored at higher dosages and in comparison to a mouse anti-IL17 inhibitor, exceptional preclinical activity on the model progression was observed, essentially bringing clinical score to baseline. Additionally, examination of transcriptional expression of IL-17A and IL-23R (Th17 specific markers) demonstrated decreased levels in line with a rebalancing of the immune system. We believe this activity is on par with fingolimod and corticosteroid treatment. Histological characterization of treated mice shows a remarkable lack of disease infiltrating immune cells (Figure 5).

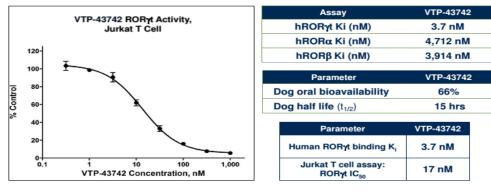
Vitae is an early-stage drug discovery and development company that uses its proprietary CONTOUR structure-based drug design platform for the development of therapeutic candidates directed against validated targets that are difficult-to-drug. The CONTOUR platform continues to develop small molecules with class defining biochemical efficacy, with readouts expected at year end and in 1H16 for several important pipeline assets, including VTP-43742 safety data in 2H15 and efficacy data in psoriasis by year end.

FIGURE 1. Th17 Immune Regulation



Source: Company Reports

FIGURE 2. VTP-43742 Biochemical Activity and Isoform Selectivity



Source: Company Reports



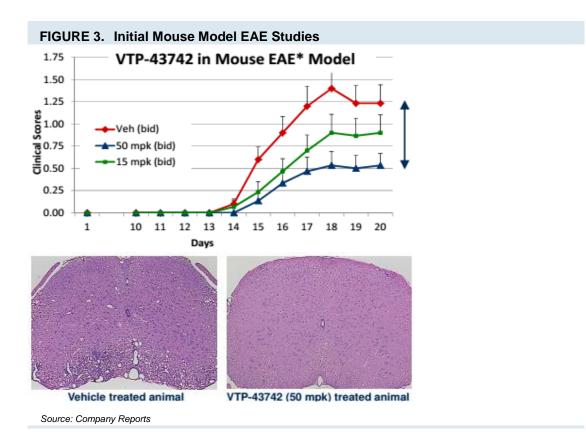
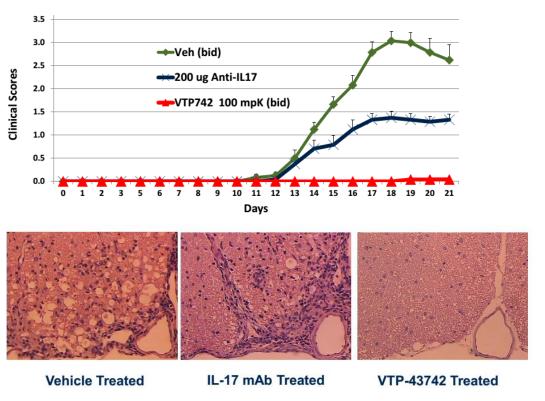


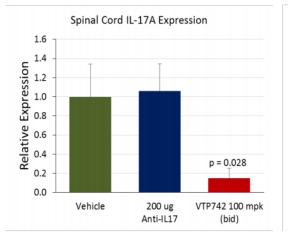
FIGURE 4. Mouse Model EAE Studies with Active Comparator

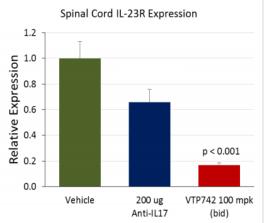


Source: Company Reports



FIGURE 5. Expression Levels of Immune Related Biomarkers





Source: Company Reports



# FIGURE 6. Upcoming Catalysts

Timing	Program	Catalyst
mid-15	VTP-43742	Complete Phase I proof-of-concept safety and PK trial in healthy volunteers
	(RORyt)	
2H15	VTP-34072	Phase II top-line results expected in type-2 diabetes monotherapy
2H15	VTP-43742	Initiation of a multiple ascending dose Phase I in psoriatic patients
	(RORyt)	
2H15	VTP-43742	Completion of multiple ascending dose Phase I in psoriatic patients
	(RORyt)	
2H15	VTP-38543	Initiate Phase I safety and PK trial clinical trial
	(LXRβ)	
2016	VTP-38543	Results from Phase I trial in atopic dermatitis
	(LXRβ)	
2016	VTP-38443	File IND/ Initiate Phase I clinical trial in acute coronary syndrome
	(LXRβ)	
2016	VTP-43742	Initiate Phase II trial (large indication)
	(RORyt)	
2016	VTP-43742	Initiate Phase II trial (rare/orphan indication)
	(RORyt)	

Source: JMP Securities LLC and Company Reports

September 9, 2015 5



# **Company Description**

Vitae is a biotechnology company focused on leveraging a discovery and development platform for the advancement of small molecule drugs to treat important unmet clinical diseases. Utilizing the company's proprietary Contour structure-based discovery platform, Vitae is able to rapidly discover novel lead molecules with desired target efficacy and biological stability that support significant derisking at very early stages of drug development. Vitae has initially focused its development on two targets that treat large patient markets: type-2 diabetes and Alzheimer's disease. The company's most advanced clinical asset is VTP-34072, an inhibitor of 11-β HSD1, a preclinically validated target in diabetes and metabolic disease that is currently in Phase II clinical trials. Data is expected from this trial in the second half of 2015.

The second asset, BI 1147560, an IND-ready inhibitor of BACE-1, a target of high interest in the treatment of Alzheimer's. The company has also used its platform to develop preclinical candidate inhibitors against difficult-to-target pathways in autoimmune disease, cardiovascular disease, and dermatological conditions. These wholly owned assets include VTP-43742, a ROR-γt inhibitor strongly implicated in autoimmune diseases such as multiple sclerosis, psoriasis, and rheumatoid arthritis. Additionally, the company has developed VTP-38443 for the treatment of acute coronary syndrome, and VTP-38543 for the treatment of atopic dermatitis, both of which stimulate the LXRβ receptor. Vitae is also developing an as-yet unnamed program to develop preclinical compounds for immune-oncology applications.

#### **Investment Risks**

Potential risks to our investment thesis and price target include, but are not limited to:

Clinical and regulatory. If either VTP-34072 in diabetes or BI 1147560 in Alzheimer's is not able to meet any of its primary outcomes or suffer from safety and tolerability issues, Vitae or Boehringer Ingelheim (BI) may choose to end development in any of its current indications. Additionally, if the FDA and EMEA do not approve VTP-34072 or BI 1147560, Vitae's stock price would likely suffer.

Partnering. Vitae has partnered with (BI) in the development of VTP-34072 in diabetes. BI is responsible for the continued clinical and commercial development of VTP-34072 and may decide to end development for one or more indications. If it were necessary for Vitae to develop and market any of its programs due to the loss or inability to retain a partner, it may be difficult to develop an internal commercial structure. Management has limited experience in commercial and marketing activities.

Competitive. The diabetes market is crowded and saturated with low-cost generic manufacturers of metformin and sulfonylureas. It may be difficult for BI and Vitae to garner significant market share. The high bar for safety and efficacy differentiation for the diabetes primary care market may limit adoption. BI 1147560 is not the only BACE-1 inhibitor in development and will not be a first-in-class therapy if Merck/Ligand are successful in bringing their drug to market. It may be difficult to compete in a market dominated by these therapies.

Financial. Vitae currently derives revenue from research and development funding and from license or collaboration agreements. The company sold ~6,875,000 shares in September 2014, raising net proceeds of ~\$51.15MM. We expect this funding to be able to carry it through to 2016. Like most non-profitable biotechnology companies, VTAE will likely need to seek additional financing, exposing current investors to dilutive risk.



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The research analyst(s) who prepared this report does/do hereby certify that the views presented in this report are in accordance with my/our personal views on the securities and issuers discussed in this report. As mandated by SEC Regulation AC no part of my/our compensation was, is or will be directly or indirectly related to the specific views or recommendations expressed herein. This certification is made under the obligations set forth in SEC Regulation AC. Any other person or entity may not use it for any other purpose. This certification is made based on my/our analysis on the date of this report's publication. I/We assume no obligation to update this certification to reflect any facts, circumstances or events that may subsequently come to my/our attention. Signed Michael G. King

#### **JMP Securities Disclosures:**

JMP Securities currently makes a market in the security of Vitae Pharmaceuticals, Inc.

JMP Securities was manager or co-manager of a public offering of securities for Vitae Pharmaceuticals, Inc. (VTAE) in the past 12 months, and received compensation for doing so.

JMP Securities expects to receive OR intends to seek compensation for investment banking services from Vitae Pharmaceuticals, Inc. in the next 3 months.

## **JMP Securities Investment Opinion Definitions:**

Market Outperform (MO): JMP Securities expects the stock price to outperform relevant market indices over the next 12 months.

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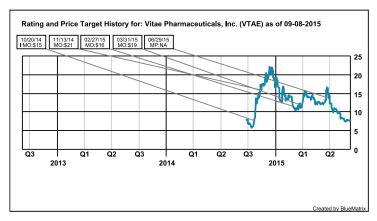
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							# Co's		
							Receiving		
							IB		
		# Co's	%		# Co's	%	Services in	% of Co's	
	Regulatory	Under	of	Regulatory	Under	of	Past 12	With This	
JMP Rating	Equivalent	Coverage	Total	Equivalent	Coverage	Total	Months	Rating	
MARKET OUTPERFORM	Buy	295	62.90%	Buy	295	62.90%	86	29.15%	
MARKET PERFORM	Hold	147	31.34%	Hold	147	31.34%	16	10.88%	
MARKET UNDERPERFORM	Sell	6	1.28%	Sell	6	1.28%	0	0%	
COVERAGE IN TRANSITION		21	4.48%		21	4.48%	4	19.05%	
TOTAL:		469	100%		469	100%	106	22.60%	

## **Stock Price Chart of Rating and Target Price Changes:**

Note: First annotation denotes initiation of coverage or 3 years, whichever is shorter. If no target price is listed, then the target price is N/A. In accordance with NASD Rule 2711, the chart(s) below reflect(s) price range and any changes to the rating or price target as of the end of the most recent calendar quarter. The action reflected in this note is not annotated in the stock price chart. Source: JMP Securities.





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