

June 11, 2015

## Tokai Pharmaceuticals, Inc.

### Highlights From William Blair's 35th Annual Growth Stock Conference

We hosted Tokai management at William Blair's 35th Annual Growth Stock Conference (June 9-11, Chicago). The presentation, given by CEO Jodie Morrison, provided an update on the imminent initiation of the pivotal Phase III ARMOR3-SV study, as well as on the development of the companion diagnostic for ARMOR3-SV.

**Management reiterated that the initiation of the ARMOR3-SV study is on track to begin in second quarter. Management remains confident in its potential to break into the prostate cancer treatment market by fulfilling a niche unmet need: M1 patients with the AR-V7 splice variant.** Over the past year, an efficient path to potential approval was identified for galeterone; the niche population to conduct ARMOR3-SV will be the chemo-naïve, metastatic castration-resistant prostate cancer (mCRPC) patients (or M1 patients) who express a splice variant of the androgen receptor (AR), AR-V7. M1 patients expressing AR-V7 are unresponsive to Xtandi (Astellas and Medivation [MDVN \$113.73; Outperform]) or Zytiga (Johnson & Johnson [JNJ \$99.13]). Although galeterone contains similar mechanisms to those of both drugs, it goes one step further with the additional ability to directly degrade the AR, including the AR-V7 variant.

- **As previously guided, the Phase III ARMOR3-SV study is still expected to begin in second quarter 2015, with top-line data expected by year-end 2016.** The multi-national Phase III ARMOR-SV study is to be conducted at 110 clinical sites throughout the United States, Canada, Australia, and western Europe. According to various literature available to date, the prevalence of AR-V7 variant ranges from low teens to around 25%. Tokai is prepared to screen over 1,000 patients to enroll the targeted 148 patients for ARMOR3-SV. We expect this endeavor to best characterize the prevalence of AR-V7 in the M1 setting.
- **The bottleneck to initiation of ARMOR3-SV has been the development and validation of the companion diagnostic, partnered with Qiagen.** Management reiterated that final validation of the assay remains on track for second quarter 2015, and the trial will immediately begin enrollment once the assay is in place.
- **Management announced that there will be a futility analysis in ARMOR3-SV when 50% of events have occurred, which management estimates will happen in first half 2016.** The analysis is not designed to test for potential early stop of the study due to efficacy; however, the timing of the analysis might provide some visibility into the enrollment speed and more refined estimate in the timing of the final data release.
- **Management believes that AR-V7 screening could become the standard of care, supported by the rising recognition of importance and popularity of precision medicine.** If ARMOR3-SV is successful, it is likely that AR-V7 screening becomes standard of care in M1 patients when selecting treatment options, in our opinion, and the next step would be to push galeterone earlier into the treatment paradigm. Moreover, payers may also push testing for AR-V7 into becoming a first step in the standard of care so as to streamline diagnosis and proper treatment and avoid paying for expensive therapies that may not work.

Tokai Pharmaceuticals, Inc. is a biopharmaceutical company based in Cambridge, Massachusetts, focused on the development of galeterone and an androgen receptor-degradation platform to address prostate cancer and potentially other hormone-driven cancers.

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Stock Rating: **Outperform**  
Company Profile: **Aggressive Growth**  
Price Target: \$44.00

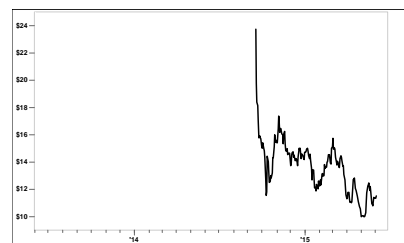
Symbol: TKAI (NASDAQ)  
Price: \$13.87 (52-Wk.: \$10-\$30)  
Market Value (mil.): \$288  
Fiscal Year End: December  
Long-Term EPS Growth Rate:  
Dividend/Yield: None

	2014A	2015E	2016E
<b>Estimates</b>			
EPS FY	\$-3.60	\$-2.16	\$-2.30
CY		\$-2.16	\$-2.30
Sales (mil.)	0	0	0
<b>Valuation</b>			
FY P/E	NM	NM	NM
CY P/E		NM	NM

<b>Trading Data (FactSet)</b>	
Shares Outstanding (mil.)	22
Float (mil.)	2
Average Daily Volume	75,045

<b>Financial Data (FactSet)</b>	
Book Value Per Share (MRQ)	4.1
Return on Equity (TTM)	-34.9

#### Two-Year Price Performance Chart



Sources: FactSet, William Blair & Company estimates

- **The company reconfirmed that the current cash position would sustain operations into 2017, which encompasses the top-line data release of the ARMOR3-SV study around year-end 2016. The company does not expect to partner out galeterone prior to Phase III data release.** We believe that should ARMOR3-SV be successful, the most likely outcome is for Tokai to be acquired by a larger player in the field, who has the development and commercialization muscles to push galeterone and follow-on candidates from Tokai's AR degradation platform onto the market and expand their indications.

**Key Catalysts Driving Value in the Next 12-24 Months** include: 1) finalization of the companion diagnostic assay for ARMOR3-SV; 2) initiation of the pivotal ARMOR3-SV study in chemo-naïve mCRPC patients with AR-V7 in June; 3) data presentation at ESMO in September; 4) the planned futility analysis in ARMOR3-SV in first half 2016; and 5) top-line data from ARMOR3-SV by year-end 2016.

**We maintain our Outperform rating and price target at \$44 (exhibit 1). Our Outperform rating is centered on our belief that Tokai's lead asset, galeterone, will become an essential component of the armamentarium against prostate cancer.** We estimate worldwide sales for galeterone will reach \$1.8 billion in the United States and Europe in 2027 in the AR-V7 variant population alone. Assuming an 85% probability of success, our probability-adjusted NPV model suggests a fair value for Tokai shares at \$44.

**Key risks to our Outperform rating and price target include:** 1) clinical risk of the Phase III program; 2) regulatory risk related to receiving approval for galeterone in the United States and Europe; 3) development and approval of the companion diagnostic; 4) reimbursement risk; and 5) financing risk.

**Exhibit 1**  
**Tokai Pharmaceuticals, Inc.**  
**Sum-of-the-Parts Fair Value**  
(dollars in thousands, except shares)

Drug	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
Galeterone	\$ 1,814,414	Pre-Phase III	H1:2018	85%	100% US; 25% ex-US	\$984,638	\$43.17	98.6%
<b>Subtotal</b>						<b>\$984,638</b>	<b>\$43.17</b>	<b>98.6%</b>
<b>Net Cash at year-end 2015</b>						\$21,659	\$0.95	2.2%
<b>Net Present Value of additional Gain (Loss)*</b>						(\$8,000)	(\$0.35)	(0.8%)
<b>Sum-of-Parts Fair Value</b>						<b>\$998,297</b>	<b>\$43.77</b>	<b>100.0%</b>

Sources: William Blair & Company, L.L.C. estimates

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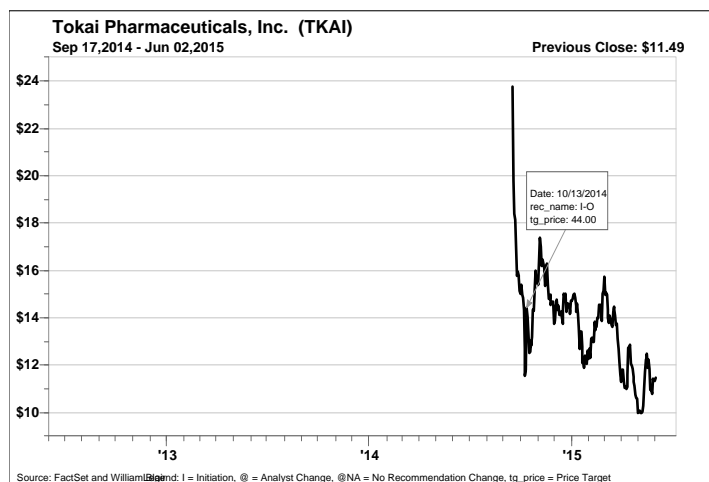
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DOW JONES: 18,000.40

S&P 500: 2,105.20

NASDAQ: 5,076.69



## **Current Rating Distribution (as of 05/31/15)**

Coverage Universe	Percent	Inv. Banking Relationships*	Percent
Outperform (Buy)	65	Outperform (Buy)	14
Market Perform (Hold)	33	Market Perform (Hold)	3
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.

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