Zacks Small-Cap Research

June 1, 2015
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CASI Pharma (CASI-NASDAQ)

CASI: A commercial stage biopharmaceutical company focusing on cancer and autoimmune diseases and competitively positioned with an integrated U.S./China drug development model--initiating with Buy rating

Current Recommendation	**Buy**
Prior Recommendation	N/A
Date of Last Change	02/11/2015
Current Price (05/29/15)	$1.30
Twelve-Month Target Price	**$4.50**

OUTLOOK

CASI is a **commercial stage** biopharmaceutical company with a therapeutic focus on cancer and autoimmune diseases. Its lead oncology drug candidate ENMD-2076 is in multiple Phase II clinical trials in both North America and China. CASI is on track to initiate the import drug registration process for Zevalin™ and Marqibo™ in China and to start commercial launch of Zevalin in Hong Kong. CASI is also developing 2ME2 for autoimmune disorders.

CASI's unique dual development approach leverages its expertise and resources in both North America and greater China, which differentiates itself from local competitors in each of the territory.

We are optimistic about CASI's prospect and rating its shares Buy.

SUMMARY DATA

52-Week High	**$2.03**
52-Week Low	**$1.16**
One-Year Return (%)	**-23.03**
Beta	**1.47**
Average Daily Volume (sh)	**15,556**
Shares Outstanding (mil)	**32**
Market Capitalization ($mil)	**$45**
Short Interest Ratio (days)	**1.92**
Institutional Ownership (%)	**N/A**
Insider Ownership (%)	**N/A**
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 2015 Estimate	**N/A**
P/E using 2016 Estimate	**N/A**
Zacks Rank	**N/A**

Risk Level	**High,**
Type of Stock	**Small-Growth**
Industry	**Med-Drugs**
Zacks Rank in Industry	**N/A**

ZACKS ESTIMATES

Revenue
(in millions of $)

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2014	0.00 A	0.00 A	0.00 A	0.00 A	0.00 A
2015	0.02 A	0.05 E	0.10 E	0.20 E	0.37 E
2016					0.50 E
2017					1.00 E

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

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2014	-$0.05 A	-$0.06 A	-$0.07 A	-$0.05 A	-$0.23 A
2015	-$0.06 A	-$0.05 E	-$0.06 E	-$0.06 E	-$0.23 E
2016					-$0.23 E
2017					-$0.33 E

Zacks Projected EPS Growth Rate - Next 5 Years % **N/A**

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KEY POINTS

- We are initiating coverage of CASI Pharmaceuticals, Inc. (CASI) with a **Buy** rating. Our 12-month price target is $4.50 per share.

- CASI is a **commercial stage** biopharmaceutical company with a therapeutic focus on cancer and autoimmune disease. The company's business can be divided into two segments: internal drug development and acquiring drug candidates from external partners.

- For internal drug development, CASI is advancing its lead candidate, ENMD-2076, using a unique **dual drug development approach**, which leverages the company's expertise and resources in both US and China. This dual development approach has the advantages of lower cost, high R&D productivity due to the large talent pool in China, and a large patient pool for clinical trials.

- The lead candidate from its pipeline is **ENMD-2076**, which is in multiple **Phase II** clinical trials for the treatment of breast cancer, soft tissue sarcoma and ovarian clear cell carcinoma. A new **Phase II** trial of the candidate will be initiated in 3Q2015 for the treatment of advanced fibrolamellar carcinoma **(FLC)**, and the company plans to pursue this indication for accelerated approval per FDA's guidance. ENMD-2076 has been granted orphan drug designation for ovarian cancer, multiple myeloma, acute myeloid leukemia and hepatocellular carcinoma (HCC).

- Another candidate is **2ME2,** which has demonstrated efficacy for cancer and autoimmune disease in preclinical studies with an excellent safety profile. 2ME2 is being reformulated under a new drug delivery method and CASI plans to initiate a human PK study in 2015.

- CASI plans to continue to acquire drug candidates from external sources, either for greater China rights, or global rights depending on the opportunity available and the stage of the asset. CASI has acquired greater China rights for three cancer drugs: Zevalin, Marqibo and Evomela, from Spectrum Pharmaceuticals, Inc. with Zevalin and Marqibo already approved in the U.S., and Evomela under the FDA review. The greater China rights include China, Taiwan, Hong Kong and Macau.

- The company is initiating commercial activity of **Zevalin** in Hong Kong in 3Q15, and is initiating the process for import drug registration for Zevalin and Marqibo. The company intends to initiate the import drug registration process for Evomela in China once its partner has received drug approval from the U.S. FDA.

- Sales of Zevalin in Hong Kong may not be meaningful since Hong Kong is a relatively small market for oncology. But we believe the launch of Zevalin in Hong Kong is strategically important to CASI in a few aspects. It will raise the visibility of Zevalin in China and when Zevalin gets final approval in China, it would be easier to penetrate the market. CASI will also gain marketing experience from Zevalin launch in Hong Kong, which is important for product launches in China in the future.

- The import drug registration process in China is shorter for FDA approved drugs than it is for development stage candidates. We expect Zevalin and Marqibo to be approved in 2019 and 2020 respectively in China based on the import drug registration process in the country. We expect Evomela to be approved in 2020 if it can receive the FDA approval this year. Therefore by 2020 CASI will have three branded cancer drugs in the Chinese market and the company will become a key player in the field of oncology in China. We also expect the company to execute its plan to acquire additional drug assets.

- We see great opportunity for CASI as China will become the second largest pharmaceutical market in the world in a few years. Both cancer and autoimmune disease markets are significant in China. We believe CASI has a great potential to grow and will become a key player in these two markets in

China in the near future. The current valuation of the company is low and we encourage investors to accumulate its shares at the current market price.

OVERVIEW

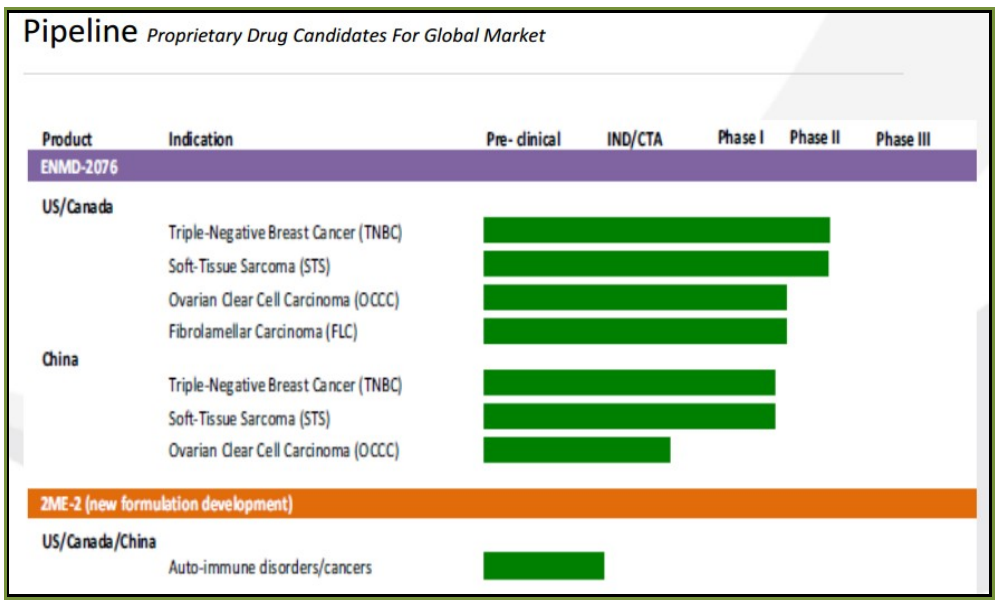
CASI Pharmaceuticals, Inc. (**CASI**-NASDAQ) is a commercial stage biopharmaceutical company focused on the acquisition, development and commercialization of innovative therapeutics for **cancer** and **autoimmune disease** for the global market with a commercial focus **on greater China area** including Taiwan, Hong Kong and Macau.

The company aims to become an integrated biopharmaceutical company with significant market share in China, while establishing partnerships for global development and commercialization. In order to achieve this goal, CASI has implemented a **unique business model**: pursue its internal proprietary pipeline development and at the same time acquiring additional drug candidates to build a rich product portfolio.

The **internal drug development** utilizes a **dual development strategy** to leverage its expertise and resources in North America and China to bring safer, more effective, and/or easier-to-use drugs to patients and to develop them more cost-effectively while its **import drug business** in China takes advantage of shorter timeframe of the drug registration process in the country.

CASI's internal drug pipeline includes **ENMD-2076**, a proprietary, novel, first-in-class oncology drug candidate that has demonstrated promising activity in several cancer indications. ENMD-2076 is currently in three multiple-site **Phase II** clinical trials with ongoing biomarker analysis. CASI expects to initiate a **Phase II** trial in fibrolamellar carcinoma (**FLC**) in 3Q2015.

Also included in its pipeline is **2ME2** (2-methoxyestradial) currently under **reformulation development** for autoimmune disorders. 2ME2 is an orally active compound that has antiproliferative, antiangiogenic and anti-inflammatory properties. The inhibition of angiogenesis is an important approach to the treatment of both cancer and autoimmune diseases such as rheumatoid arthritis. 2ME2 has potential as a single agent in rheumatoid arthritis based on its antiangiogenic, anti-inflammatory, and anti-osteoclastic properties. 2ME2 has also demonstrated positive preclinical results for multiple sclerosis. It can also potentially be used in combinational therapies with current standard of care products for those indications due to its excellent safety profile.



Source: company presentation

In September 2014, CASI acquired from **Spectrum Pharmaceuticals, Inc.** exclusive rights **for greater China** to three oncology products, including **Zevalin** ® (ibritumomab tiuxetan), approved in the U.S. for advanced non-Hodgkin's lymphoma, **Marqibo**® (vincristine sulfate liposome injection), approved in the U.S. for advanced adult Ph- acute lymphoblastic leukemia (ALL), as well as **Evomela**™ (captisol enabled™ melphalan), which is under the FDA review for marketing approval and is expected to be approved by the FDA in October 2015.

The company has initiated the drug registration process to obtain marketing approval for these products in China and will initiate commercial activity of Zevalin® in Hong Kong.

Products under Development for Import Drug Registraion in Greater China

Product	Description	Indication	Status	Partner's Status in SPECTRUM
ZEVALIN®	ibritumomab tiuxetan injection, a CD20-directed radiotherapeutic antibody	Advanced non-Hodgkin's lymphoma (NHL)	Preparing commercial launch in Hong Kong; Preparing Import Drug Registration applications for China and Taiwan	Approved and marketed
MARQIBO®	vinCRIStine sulfate LIPOSOME injection	Advanced adult Ph- acute lymphoblastic leukemia (ALL)	Preparing Import Drug Registration applications for China, Taiwan, and Hong Kong	Approved and marketed
Captisol Enabled™ Melphalan	Captisol Enabled™ (propylene glycol-free) melphalan	Multiple myeloma (MM) transplant setting	Preparing Import Drug Registration applications for China, Taiwan, and Hong Kong	A New Drug Application filed with FDA in 2014

Source: company presentation

CASI Pharmaceuticals, Inc. was founded in 1991 and was formerly known as EntreMed, Inc. The company changed its name to CASI Pharmaceuticals, Inc. in June 2014 as part of the restructuring and turnaround of the company that started in 2012 with the investment by IDG-Accel and later Kleiner Perkins Caufield China. In the short time since, the company has established an office and R&D center in Beijing, initiated three trials for its lead drug ENMD-2076 with one trial recently expanded to China, acquired greater China rights to two U.S. approved drugs and one NDA-stage drug candidate, and has made inroads into their second internal drug, 2ME2. We expect the momentum to continue for this company.

CASI is headquartered in Rockville, Maryland and has a wholly owned subsidiary and R&D center in Beijing, China.

INVESTMENT THESIS

ENMD-2076 for Multiple Cancers

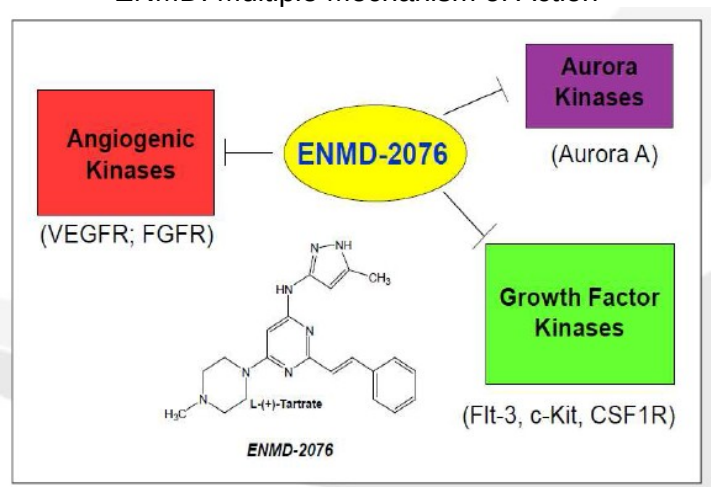
CASI is dedicated to the development of its **proprietary pipeline**, which, we believe, will provide substantial value to the company.

The company's lead drug candidate is **ENMD-2076**, a selective Aurora A and angiogenic kinase inhibitor for the treatment of cancer. CASI is developing ENMD-2076 in the U.S. and Canada for approval by the FDA, and as part of its development strategy, has expanded its U.S. trial in triple-negative breast cancer (TNBC) to China sites, and will follow a similar pathway to include China sites for its ongoing

U.S./Canada trials in advanced/metastatic soft tissue sarcoma (STS), and in advanced ovarian clear cell carcinomas (OCCC). The combined data can potentially be used to support global filings as well as import drug registrations in China.

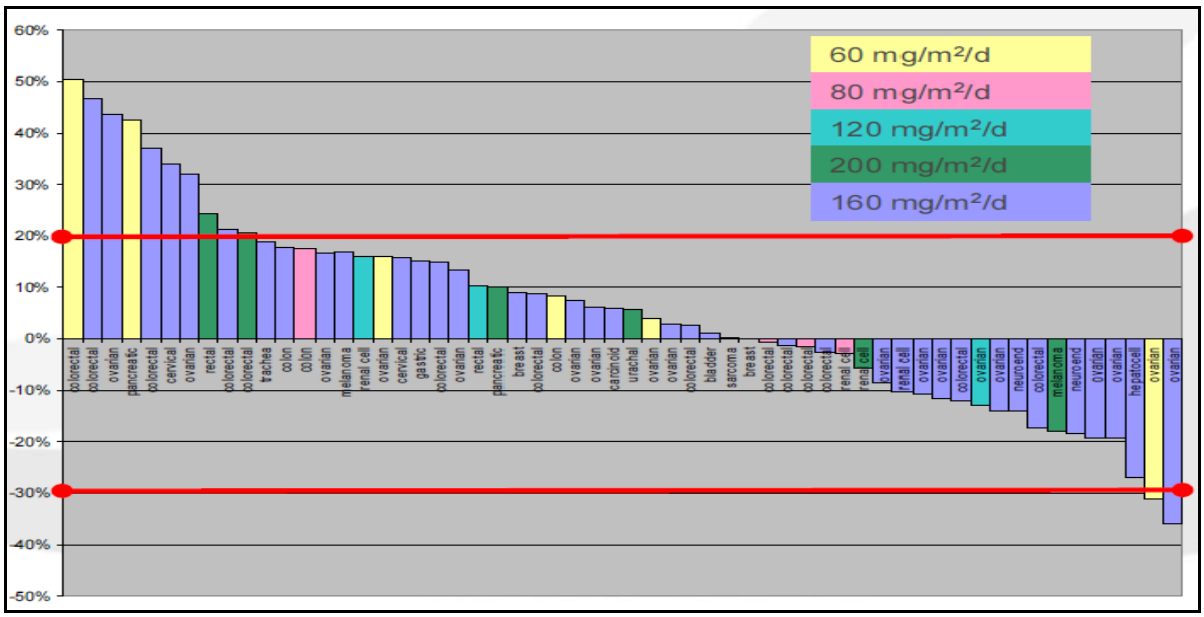
ENMD-2076 is an **orally-active**, Aurora A/angiogenic kinase inhibitor with a unique kinase selectivity profile. ENMD-2076 exerts its effects through multiple mechanisms of action, including anti-proliferative activity and the inhibition of angiogenesis. **Aurora kinases** are key regulators of cell division, and are often over-expressed in human cancers. In addition to targeting Aurora kinase, ENMD-2076 also targets the VEGFR, Flt-3, and FGFR3 kinases which have been shown to play important roles in the development of many cancers.

ENMD: Multiple Mechanism of Action



Source: Company presentation

ENMD-2076 has demonstrated significant, dose-dependent **preclinical anti-cancer efficacy** as a single agent in multiple xenograft models (e.g. breast, colon, leukemia and liver), as well as in ex vivo human leukemia patient cells. ENMD-2076 also has shown promising activity in **Phase I** clinical trials in solid tumors including ovarian, breast, liver, renal and sarcoma, as well as in leukemia and multiple myeloma. In these tumors, ENMD-2076 achieved partial response rate of 3% and stable disease of 85% with overall response rate of 88%, which we think is very promising. The recommended dose for ENMD-2076 is 160 mg/m^2/d.



Source: company presentation

CASI also has completed a **Phase II** trial of ENMD-2076 in ovarian cancer. The company is currently conducting multiple **Phase II** studies of ENMD-2076 in triple-negative breast cancer (TNBC), advanced/metastatic soft tissue sarcoma (STS), and in advanced ovarian clear cell carcinomas (OCCC) in North America.

In addition to the clinical trials in the U.S. and Canada, CASI has received approvals from the China Food and Drug Administration (CFDA) to initiate Phase II clinical trials for ENMD-2076 in TNBC, advanced/metastatic sarcoma, and advanced ovarian clear cell carcinoma in the country. The company has initiated the TNBC trial and currently is recruiting patients while is in the planning process for trials in China for the other two indications. The clinical trials in China will supplement the company's ongoing Phase II trial currently underway in North America.

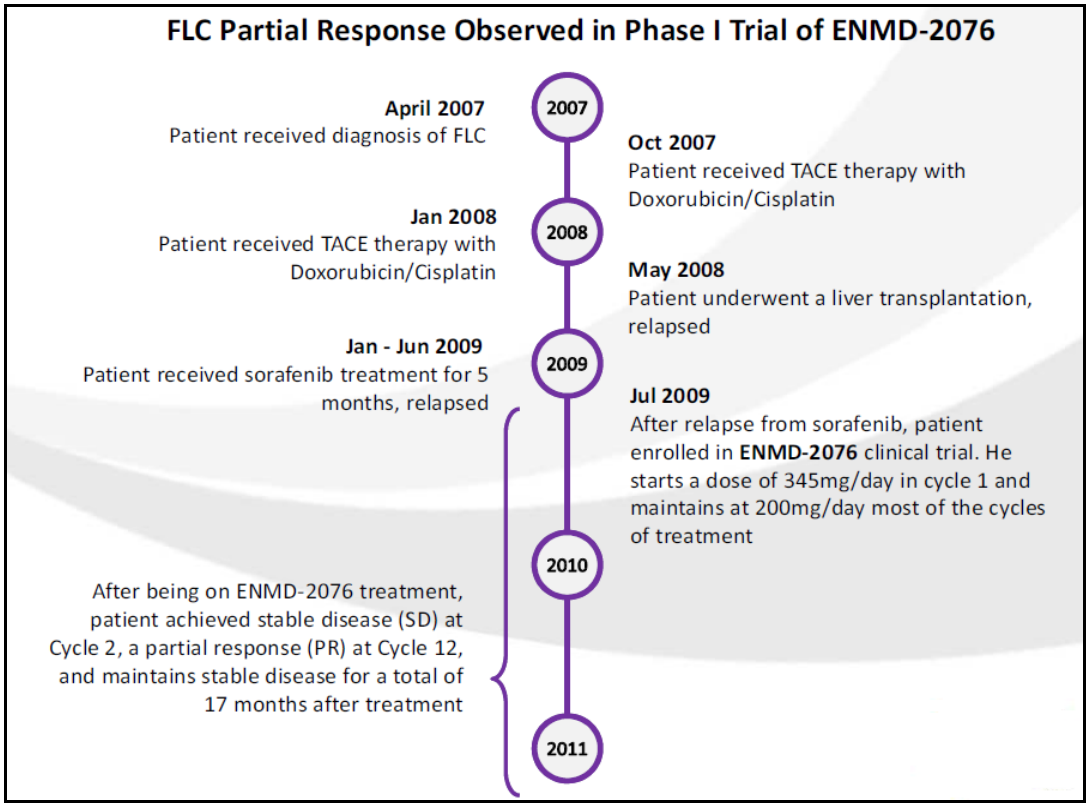
The status and development of ENMD-2076 is outlined below:

Disease Indication	*Status*
Advanced Solid tumors	Phase I trial completed
Leukemia	Phase I trial completed
Multiple Myeloma	Phase I trial completed
Ovarian Cancer	Phase II trial completed
Triple-Negative Breast Cancer	Phase II currently ongoing in both US and China
Advanced/Soft Tissue Sarcoma	Phase II currently ongoing in Canada, clinical trial application approved by CFDA, in the process of initiation in China
Healthy Volunteer	Phase I crossover bioequivalent trial being completed, Food effect study ongoing in the US
Advanced Ovarian Clear Cell Carcinoma	Phase II currently enrolling, clinical trial application approved by CFDA, in the process of initiation in China

ENMD-2076 has received orphan drug designation from the FDA for the treatment of ovarian cancer, multiple myeloma, acute myeloid leukemia and hepatocellular carcinoma (HCC).

New Indication of ENMD-2076 for FLC

In previous Phase I trials of ENMD-2076, partial response and 6-month progression free survival (PFS) patients not only included TNBC, STS and ovarian cancer, but also included advanced fibrolamellar carcinoma **(FLC).** The following chart showed very promising result of ENMD-2076 for the treatment of FLC patient in the Phase I trial.



Source: company presentation

Scientifically there is a strong rationale that ENMD-2076 may provide clinical benefits to FLC patients due to the fact that VEGFR, FGFR and aurora kinase are generally over expressed in liver cancer patients.

In September 2014, CASI filed an investigational new drug application (IND) with the FDA to conduct a **Phase II** trial in advanced FLC and received allowance from the Agency soon after. In February 2015, CASI held a meeting with the FDA and received the Agency's guidance on clinical and regulatory development plan.

Based on the guidance from the FDA,

- CASI plans to conduct a **single arm trial** to evaluate the objective response rate (ORR) of ENMD-2076 in FLC patients as the primary endpoint.

- The trial will be conducted as a Simon **2-stage study** with interim futility analysis released after 16 patients reach a clinical event. Futility would be declared if none of the patients experience an objective response and would result in the termination of the study.

- If, however, one or more patients experience an objective response, the study would continue with additional patients. At that time, the company would expect to meet with the FDA to discuss what clinical efficacy endpoint would be considered reasonably likely to provide clinical benefit and support an accelerated approval based on the surrogate endpoint of tumor response.

- Assuming that the results of the proposed study show that ENMD-2076 improves objective tumor response, CASI would expect to propose a confirmatory clinical trial and, in parallel, submit an **NDA for accelerated approval** based on objective tumor response rates.

- CASI will submit to the FDA a request for **breakthrough therapy** designation whenever clinical data meets the appropriate criteria.

We think the feedback from the FDA is very encouraging, which provides ENMD-2076 a clear clinical and regulatory path going forward. We are especially encouraged that ENMD-2076 could potentially gain accelerated approval if the primary endpoint is met in the Phase II study.

FLC is a **rare form** of liver cancer that usually occurs in young adults who have no history of liver disease. FLC patients typically present with a palpable abdominal mass but no symptoms, although pain, weight loss and jaundice may occur. The typical treatment is surgical removal of the tumor. When the tumor cannot be removed surgically or when there is distant spread, systemic treatment is used. Currently, there is **no standard systemic therapy regimen** for FLC**.**

Studies have shown that FLC appears to have a **better prognosis** than typical HCC. The population-based relative survival of patients with FLC in the US is 73% at 1 year and 32% at 5 years. In contrast, HCC relative survival is 26% at 1 year and 7% at 5 years.

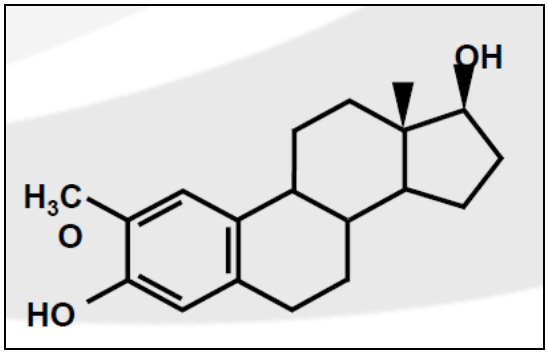
FLC comprises approximately 5% of all hepatocellular carcinomas (HCC). The total incidence of HCC is estimated to be 780,000 each year worldwide according to the World Health Organization. Therefore, each year, approximately 39,000 people are diagnosed with FLC worldwide.

Peak sales of ENMD-2076 for FLC alone could be in the neighborhood of $200 million worldwide if we assume a reasonable cost of $50,000 for each patient and about 10% market share of new incidence when ENMD-2076 is approved for FLC.

2ME2 for Autoimmune Disease/Cancer

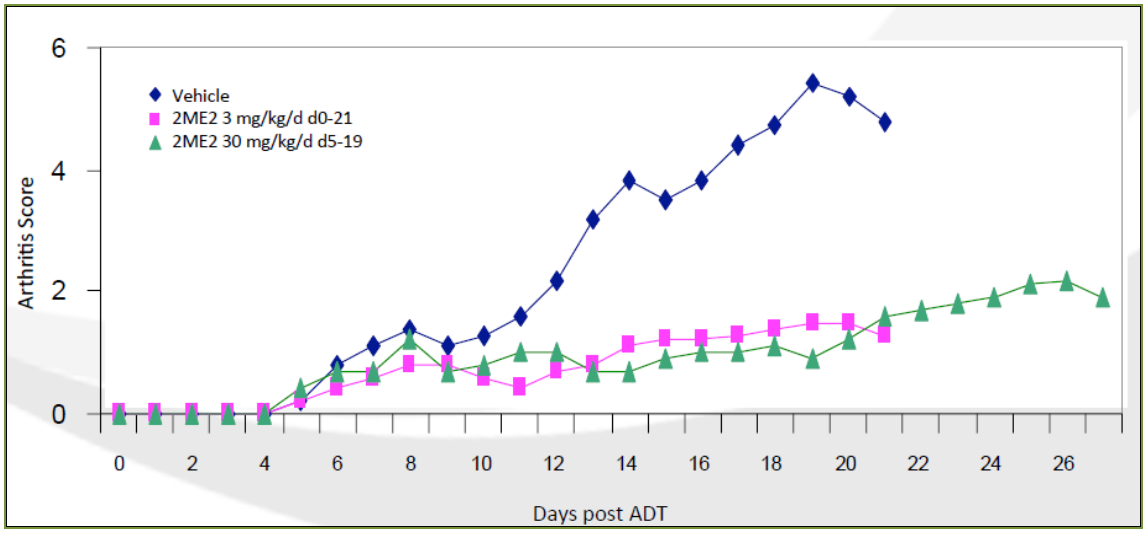
CASI's proprietary pipeline also includes 2ME2 for the treatment of **autoimmune disease** such as rheumatoid arthritis, multiple sclerosis, and **cancer.**

2ME2 (2-methoxyestradiol) is an orally active compound that has antiproliferative, antiangiogenic and anti-inflammatory properties. The inhibition of angiogenesis is an important approach to the treatment of both cancer and rheumatoid arthritis (RA). 2ME2 has potential as a single agent in RA based on its antiangiogenic, anti-inflammatory, and anti-osteoclastic (bone resorption) properties. It can also potentially be used in combinational therapies with current standard of care products for those indications due to its excellent safety profile.



Preclinical animal studies of 2ME2 have demonstrated significant efficacy for multiple cancers and autoimmune disease, including RA and multiple sclerosis (MS). Additional preclinical studies are ongoing in collaboration with research institutes in the US.

The following picture demonstrates strong efficacy of 2ME2 for arthritis in an animal model.



Source: company presentation

The company also completed multiple **Phase I and II** clinical studies in cancer patients with solid tumors and multiple myeloma. 2ME2 has demonstrated excellent safety profile and promising activities in these studies. The previous clinical challenge for the product is attributed to its very poor bioavailability, which prohibited the reach of optimal therapeutic level in patients. The current development focus is to improve product solubility and bioavailability through its internal novel drug delivery technology platform.

CASI has filed an IND of 2ME2 with the FDA for rheumatoid arthritis and is exploring multiple strategies for the development of 2ME2 including potential internal development and partnership opportunities.

Product Sales from Zevalin Will be Generated in 2015

CASI is also dedicated to the expansion of its product pipeline through the acquisition of drug candidates, which, we think, will provide sustainable growth for the company.

Zevalin IV injection is a CD20-directed radiotherapeutic antibody. It is approved in the US for the treatment of relapsed or refractory, low-grade or follicular B-cell non-Hodgkin's lymphoma **(FNHL).** Zevalin is also indicated for the treatment of patients with previously untreated follicular non-Hodgkin's Lymphoma who achieve a partial or complete response to first-line chemotherapy. A Phase III trial in diffused large B cell lymphoma **(DLBCL)** is ongoing

Zevalin therapeutic regimen consists of two components: **rituximab**, and Yttrium-90 **(Y-90)** radiolabeled Zevalin for therapy. Zevalin builds on the combined effect of a targeted biologic monoclonal antibody augmented with the therapeutic effects of a beta-emitting radioisotope.

Zevalin has been cleared for marketing in Hong Kong and CASI plans to officially commence commercial activities with the drug in **3Q2015**. Since Hong Kong is a relatively small market for oncology, we don't expect meaningful sales in Hong Kong. However, we think the launch of Zevalin in Hong Kong is strategically important to CASI in a few aspects. ***First*****,** since Hong Kong is close to mainland China, which has a much larger cancer patient population, launching Zevalin in Hong Kong will raise the drug's visibility in both Hong Kong and mainland China, which could help penetrate the China market when Zevalin gets final approval in China. ***Second***, CASI will also gain marketing experience from Zevalin launch in Hong Kong, which is important for future products launch in China. ***Third****,* we believe it is the first step of the company's building up its commercialization capabilities, a record breaking in the company's history.

CASI is currently **registering Zevalin in China**. Since Zevalin is already approved in the US, we believe that gaining approval from local regulatory authorities for commercialization in greater China will require a

shorter timeframe compared to clinical-stage drugs. It usually takes about 3 to 4 years to get approval of an imported drug in China. We expect Zevalin to be approved by CFDA in 2019.

According to International Agency for Research on Cancer (IARC), annual incidence of NHL is estimated at 46,563 cases with a mortality of 29,201cases in China in 2015. The five year prevalence is estimated at 69,200 cases, including 13% of cases in FNHL and 30% of cases in DLBCL.

The retail price of Zevalin per dose in the US is approximately $50,000 and the treatment cycle is one dose. The price tag in China for imported drugs is similar to its overseas price, but usually will be 100% out of pocket cost to patients. We assume 10% penetration of the NHL market by Zevalin, which means peak sales will be approximately **$150 million or more** in China.

More Imported Drugs Will be Launched in China

In addition to Zevalin, CASI is also in the process of registering Marqibo in China and we estimate it will be approved in 2020.

Marqibo is a novel, **liposome-encapsulated**, formulation of **vincristine sulfate**, a microtubule inhibitor. Marqibo is approved in the US for the treatment of adult patients with Philadelphia chromosome-negative (Ph-) acute lymphoblastic leukemia **(ALL)** in second or greater relapse or whose disease has progressed following two or more anti-leukemia therapies.

Spectrum is also conducting a **Phase III** trial of Marqibo for front line treatment of aggressive NHL and **Phase III** trial for front line treatment of adult Ph(-) ALL in the US. Once these new indications are approved by the FDA, CASI plans to expand the same indications in China.

Since Marqibo is already approved in the US, it falls into the imported drug registration process category, which takes a shorter timeframe for gaining marketing approval in China than clinical stage candidates.

According to IARC, annual incidence of leukemia is estimated at 70,240 cases with a mortality of 58,746 cases in China in 2015. The five-year prevalence of leukemia is estimated at 73,694 cases including approximately 10,000 Ph(-) adult ALL. The retail cost of Marqibo in the US is approximately $11,700 per dose, and treatment cycle is 28 days, 4 doses per cycle, multiple cycle regimen.

We assume 10% market penetration, the peak sales of Marqibo will be about **$40 million for one cycle** treatment in China. When treatment cycles increase and new indications are added, the sales will increase accordingly.

Evomela for Multiple Myeloma, A Great Commercial Opportunity in China

The third imported drug is **Evomela™** (captisol-enabled PG-free melphalan).

Melphalan inhibits DNA replication and transcription causing cytotoxicity in dividing and non-dividing cells including multiple myeloma. Evomela is a new propylene glycol (PG)-free IV formulation of melphalan developed for a high-dose conditioning treatment prior to hematopoietic stem cell transplantation (HSCT) in patients suffering from multiple myeloma (MM) and for the palliative treatment of multiple myeloma patients for whom oral therapy is not appropriate. Evomela completely avoids the use of PG, which is used as a co-solvent in the current formulation of melphalan and has been reported to cause renal and cardiac side-effects that limit the ability to deliver higher quantities of intended therapeutic compounds. The use of captisol technology to reformulate melphalan is anticipated to allow for longer administration durations and slower infusion rates, potentially enabling clinicians to avoid reductions and safely achieve a higher dose intensity of pre-transplant chemotherapy.

Spectrum Pharmaceuticals, Inc. filed a NDA with the FDA in December 2014. We expect Evomela to be approved in October 2015. CASI will submit import drug registration application for Evomela in greater

China after its approval by the FDA in the US. We estimate Evomela will be approved in China in 2020 if it can get approval in 2015 by the FDA.

IARC estimates that approximately 12,197 new MM patients will be diagnosed with a mortality of 9,038 cases in China in 2015. The five-year prevalence of MM in China is estimated at 14,100 cases. **Branded or generic melphalan is currently not available in China**. This gives CASI a great opportunity to promote and expand Evomela in China once it is approved by the CFDA.

Market Opportunity in China

CASI's mission is to become an integrated biopharmaceutical company with a commercial focus on greater China area. The company's wholly-owned Chinese subsidiary is aggressively executing the China portion of its drug development strategy, including seeking regulatory approvals and conducting local clinical trials, pursuing local funding opportunities and strategic collaborations, and implementing the company's plan for development and commercialization in the Chinese market.

The company's **dual drug development strategy** allows CASI to continue to tap into the front-line innovations in the U.S. and to incorporate China as a significant clinical location to advance drug development with reduced costs, large patient population in multiple indications, and accumulation of data that supports a global registration strategy.

In addition to China as a strategic clinical location, CASI is also focused on the China commercial opportunity. China is the fastest growing pharmaceutical market in the world. With the rapid growth of the country's economy and increased spending in healthcare by both the government and private sector, China is projected to be the 2nd largest pharmaceutical market by 2016 and the largest oncology market by 2019.



Cancer has been a big challenge for the China healthcare industry. Both incidence and death rate for cancer have been rising dramatically in China and around the world. For example, cancer incidence is estimated to reach 1.7 million in the US and 3.4 million in China in 2015, which is expected to increase to 2.1 million in the US and 4.4 million in China by 2025. Five year prevalence is approximately 4.8 million in the US and 5 million in China.

Currently, there is no leading brand oncology company in China and most leading local Chinese pharmaceutical companies achieved success through branded generics. A company acquiring innovative oncology products from the West and commercializing in China with scale and efficiency will have a significant lead to become a major market participant. We believe CASI with time can achieve this.

We believe CASI will become a key player in China's oncology market in about five years when both imported and locally approved cancer drugs become the choice of oncologists in China.

There are also unmet medical needs in China for autoimmune disease and CASI's 2ME2 has the potential to tap this multi-billion dollar market.



Source: prevention strategy of cancer in Eastern Asia

Strong Balance Sheet

As of March 31, 2015, CASI had cash of $9.5 million. The current cash balance should be able to fund the company's operations into 3Q2016 according to our financial model.

Also CASI received investments from IDG ACCEL and Kleiner Perkins Caufield & Byers China, two major venture capital firms in China. These firms tend to have long-term investment strategies, have representatives on the company's Board of Directors and are in line with the company's mission.

Experienced Management Team

Ken K. Ren, Ph.D., CEO

Dr. Ren joined CASI Pharmaceuticals in April 2012 as interim Chief Executive Officer, and after successful completion of the one-year interim period, was appointed Chief Executive Officer in April 2013, and in December 2014 was elected to the Board of Directors. Prior to joining CASI Pharmaceuticals from 2005-2012, Dr. Ren was the president of Accelovance (China), a subsidiary of Accelovance, Inc., which is a Maryland-based contract research organization. Prior to Accelovance, Dr. Ren was the founder of New Jersey-based AHT Inc., which merged with Novemed, a portfolio investment of a blue-chip public company in Hong Kong. He was a co-founder of the China Innovation Center for Life Science (U.S.A.) Corp., a New York-based consulting firm in partnership with the Chinese Ministry of Science and Technology which provided consulting services to health-care and pharmaceutical companies in both the U.S. and China. Over the past 15 years Dr. Ren has been involved in several U.S./China based start-up companies in pharmaceutical development or services. Dr. Ren was a research scientist at Pfizer from 1993–1995 and a Research Fellow at Rockefeller University from 1990–1993. He received his medical degree at the Shandong University School of Medicine in China in 1986 and a Ph.D. from State University of New York at Buffalo in 1990.

Cynthia W. Hu, JD., COO and GC

Ms. Hu joined CASI Pharmaceuticals in June 2006 as Vice President, General Counsel & Secretary, and in December 2008 was appointed Chief Operating Officer. Prior to joining CASI Pharmaceuticals, from January 2000 to May 2006, Ms. Hu served as senior attorney for the corporate and finance practice group at Powell Goldstein LLP in Washington, DC, where she advised clients on all corporate and financing matters, including complex public and private financings, mergers and acquisitions, SEC and regulatory compliance, and corporate governance and compliance. Before that, Ms. Hu served as corporate and securities counsel for a NYSE-listed financial institution and prior to that was in private practice with increasing levels of responsibilities, including at Klehr, Harrison, Harvey & Branzburg, LLP and Littman & Krooks, LLP focusing on corporate transactions and compliance with corporate and securities laws.

Rong Chen, MD, Ph.D. CMO

Dr. Chen joined CASI Pharmaceuticals in March 2015 as Chief Medical Officer. Dr. Chen has more than 25 years experience in the pharmaceutical industry, with knowledge and skills from drug development, drug registration, medical governance and medical affairs after product launch. Prior to joining CASI from 2008-2015, Dr. Chen was VP, Area Medical Director and Chief Medical Governance Officer at GSK China/Hong Kong area and in 2010 was promoted to VP, Head of Regulatory Center of Excellence, Company Representative having overall responsibility for the registration of drug and vaccine products. Prior to GSK, Dr. Chen was Medical Director, Global Medical Lead at Bayer-Schering Pharma and from 2001-2005 was Medical Director at Sanofi. Dr. Chen was co-author of various published articles and has presented at many international conferences. Dr. Chen is a medical physician and received his Ph.D. in Clinical Pharmacology from the Royal Adelaide Hospital, University of Adelaide, Australia in 1989.

Sara B. Capitelli, CPA, VP Finance

Ms. Capitelli joined CASI Pharmaceuticals in January 2011 as Vice President, Finance and Principal Accounting Officer. Prior to joining CASI Pharmaceuticals, from May 2010 to January 2011, Ms. Capitelli served as Controller for the Association for Financial Professionals in Bethesda, Maryland. From 1999-2008, Ms. Capitelli served as Senior Manager with Ernst & Young, where she provided audit and consulting services for small and large public and private companies. Prior to that, she served as Director, Financial Planning and Reporting of Cable & Wireless USA, a wholly-owned subsidiary of Cable & Wireless plc. Ms. Capitelli holds a CPA license in both Maryland and Virginia. Ms. Capitelli received her Bachelor of Science, Business Administration – Accounting, from Bucknell University.

VALUATION AND RECOMMENDATION

We are initiating coverage of CASI Pharmaceuticals (CASI) with a **Buy** rating. Our 12-month price target is **$4.50** per share. Our call is based on the company's fundamentals and attractive valuation.

CASI is a commercial stage biopharmaceutical company focusing on the development and commercialization of therapeutics for cancer and autoimmune disease and is competitively positioned with an integrated U.S./China drug development model. In addition to China as a strategic clinical location, CASI is also focused on the China commercial opportunity, China being projected to be the 2nd largest pharmaceutical market by 2016 and the largest oncology market by 2019.

CASI currently has three products in various stages of the import drug registration process with CFDA in China, with one product (Zevalin) initiating commercial activities in Hong Kong by 3Q15. Two of these three products are approved in the U.S. and marketed by its collaborator Spectrum Pharmaceuticals, Inc. and a NDA is filed for the third drug with the U.S. FDA in December 2014. Product revenue will be generated in 2015 for the first time in the company's history. When all three products are approved in China (we estimate the approval in 2019 or 2020), CASI will become a key player in China's oncology market and revenue could increase dramatically due to rapid market penetration.

In addition to imported drugs from the US, CASI is also developing its proprietary pipeline with two lead candidates ENMD-2076 for cancers and 2ME2 for autoimmune disease using its **unique dual drug development strategy.** This dual drug development approach leverages the company's expertise and resources in both the U.S. and China**,** which has many advantages over traditional drug development approaches. These advantages include low cost, high productivity due to large talent pool in China, and large patient pool for clinical trials.

ENMD-2076 is already in multiple **Phase II** clinical trials targeting breast cancer, soft tissue sarcoma, and ovarian clear cell carcinoma. A new Phase II trial will be initiated soon to target fibrolamellar carcinoma (FLC). The company also plans to initiate human PK clinical trial of 2ME2 prior to the end of 2015.

CASI also has a strong balance sheet, which will last through the end of 2016, and an experienced management team, who, we believe, is able to lead the company to the next level.

When it comes to the valuation, we believe CASI's shares are undervalued at the current market price based on the company's strong fundamentals. Currently, shares of the company are trading at around $1.40 per share which values the company at $45 million in market cap based on approximately 32.5 million outstanding shares. We believe this undervalues the company based on what we have discussed above.

Based on our long term financial model, we expect CASI to become profitable in 2020 with an EPS of $0.01 based on total revenue of $25 million. EPS will grow to $0.58 while revenue will reach $65 million in fiscal 2021. If we use the average biotech P/E ratio of 30x, using 25% discount rate for six years, we come up with our price target of $4.50 per share. This price target values the company at $146 million in market cap, which is still conservative in our view.

However, there are some **risks** associated with our price target.

Our price target assumes the final approval of the three imported drugs in 2019 or 2020 in China and the final approval of ENMD-2076 and 2ME2. Although risks associated with imported drug registration are relatively low in China, we can't completely rule out the possibility of disapproval, or delay of the approval.

Further, ENMD-2076 and 2ME2 are still in the development stage. The risks associated with drug development are high, especially for early stage candidates. Both ENMD-2076 and 2ME2 have a high

hurdle to overcome both clinically and regulatorily. Any delay or failure in clinical development or regulatory approval will cause the share price to decline dramatically.

Cash burn is also a concern. While current cash can last into 3Q2016, CASI may need to raise new funds to finance its clinical programs and operations until it can generate positive cash flow in 2020. We remind investors that any equity financing will dilute the existing shareholder base and may cause the share price to fall.

PROJECTED INCOME STATEMENT

	2013 (Dec)	2014 (Dec)	2015 (Dec)					2016 (Dec)	2017 (Dec)	2018 (Dec)	2019 (Dec)	2020 (Dec)	2021 (Dec)
$ in million except per share data	FY	FY	Q1	Q2	Q3	Q4	FYE	FYE	FYE	FYE	FYE	FYE	FYE
Grant revenue	$0.00	$0.02	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00
Royalties	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00
Product revenue	$0.00	$0.00	$0.02	$0.05	$0.10	$0.20	$0.37	$0.50	$1.00	$2.00	$10.00	$25.00	$65.00
Total Revenues	**$0.00**	**$0.02**	**$0.02**	**$0.05**	**$0.10**	**$0.20**	**$0.37**	**$0.50**	**$1.00**	**$2.00**	**$10.00**	**$25.00**	**$65.00**
YOY Growth	-	-	-	-	-	-	-	-	-	-	-	-	-
CoGS	0.00	0.00	0.01	0.01	0.03	0.05	0.09	0.13	0.25	0.50	2.00	5.00	13.00
Gross Income	**$0.00**	**$0.02**	**$0.02**	**$0.04**	**$0.08**	**$0.15**	**$0.28**	**$0.38**	**$0.75**	**$1.50**	**$8.00**	**$20.00**	**$52.00**
Gross Margin	-	-	73.6%	75.0%	75.0%	75.0%	75.0%	75.0%	75.0%	75.0%	80.0%	80.0%	80.0%
R&D	$2.75	$2.77	$0.87	$0.82	$0.90	$1.00	$3.59	$4.00	$4.50	$6.00	$7.50	$9.00	$10.50
% R&D	-	-	-	-	-	-	-	-	-	-	-	-	-
SG&A	$2.99	$3.76	$0.92	$1.00	$1.20	$1.40	$4.52	$5.50	$6.50	$8.00	$9.50	$10.50	$12.50
%SG&A	-	-	-	-	-	-	-	-	-	-	-	-	-
Other Expenses	$0.00	$19.68	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00
Operating Income	**($5.7)**	**($26.2)**	**($1.8)**	**($1.8)**	**($2.0)**	**($2.3)**	**($7.8)**	**($9.1)**	**($10.3)**	**($12.5)**	**($9.0)**	**$0.5**	**$29.0**
Operating Margin	-	-	-	-	-	-	-	-	-	-	-	-	44.62%
Other Net	$0.0	$0.0	($0.0)	$0.0	$0.0	$0.0	($0.0)	$0.0	$0.0	$0.0	($0.1)	($0.1)	($0.1)
Pre-Tax Income	**($5.7)**	**($26.2)**	**($1.8)**	**($1.8)**	**($2.0)**	**($2.3)**	**($7.9)**	**($9.1)**	**($10.3)**	**($12.5)**	**($9.1)**	**$0.4**	**$28.9**
Income taxes(benefit)	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Tax Rate	-	-	-	-	-	-	-	-	-	-	-	-	-
Reported Net Income	**($5.7)**	**($26.2)**	**($1.8)**	**($1.8)**	**($2.0)**	**($2.3)**	**($7.9)**	**($9.1)**	**($10.3)**	**($12.5)**	**($9.1)**	**$0.4**	**$28.9**
YOY Growth	-	-	-	-	-	-	-	-	-	-	-	-	
Net Margin	-	-	-	-	-	-	-						
Diluted Shares Out	26.1	28.6	32.4	34.0	35.5	37.0	34.7	40.0	45.0	38.0	45.0	48.0	50.0
Reported EPS	**($0.22)**	**($0.92)**	**($0.06)**	**($0.05)**	**($0.06)**	**($0.06)**	**($0.23)**	**($0.23)**	**($0.23)**	**($0.33)**	**($0.20)**	**$0.01**	**$0.58**
One time charge	*$0.00*	*$19.68*	*$0.00*	*$0.00*	*$0.00*	*$0.00*	*$0.00*	*$0.00*	*$0.00*	*$0.00*	*$0.00*	*$0.00*	*$0.00*
Non GAAP Net Income	**($5.7)**	**($6.5)**	**($1.8)**	**($1.8)**	**($2.0)**	**($2.3)**	**($7.9)**	**($9.1)**	**($10.3)**	**($12.5)**	**($9.1)**	**$0.4**	**$28.9**
Non GAAP EPS	**($0.22)**	**($0.23)**	**($0.06)**	**($0.05)**	**($0.06)**	**($0.06)**	**($0.23)**	**($0.23)**	**($0.23)**	**($0.33)**	**($0.20)**	**$0.01**	**$0.58**

Source: Company filings and Zacks estimates

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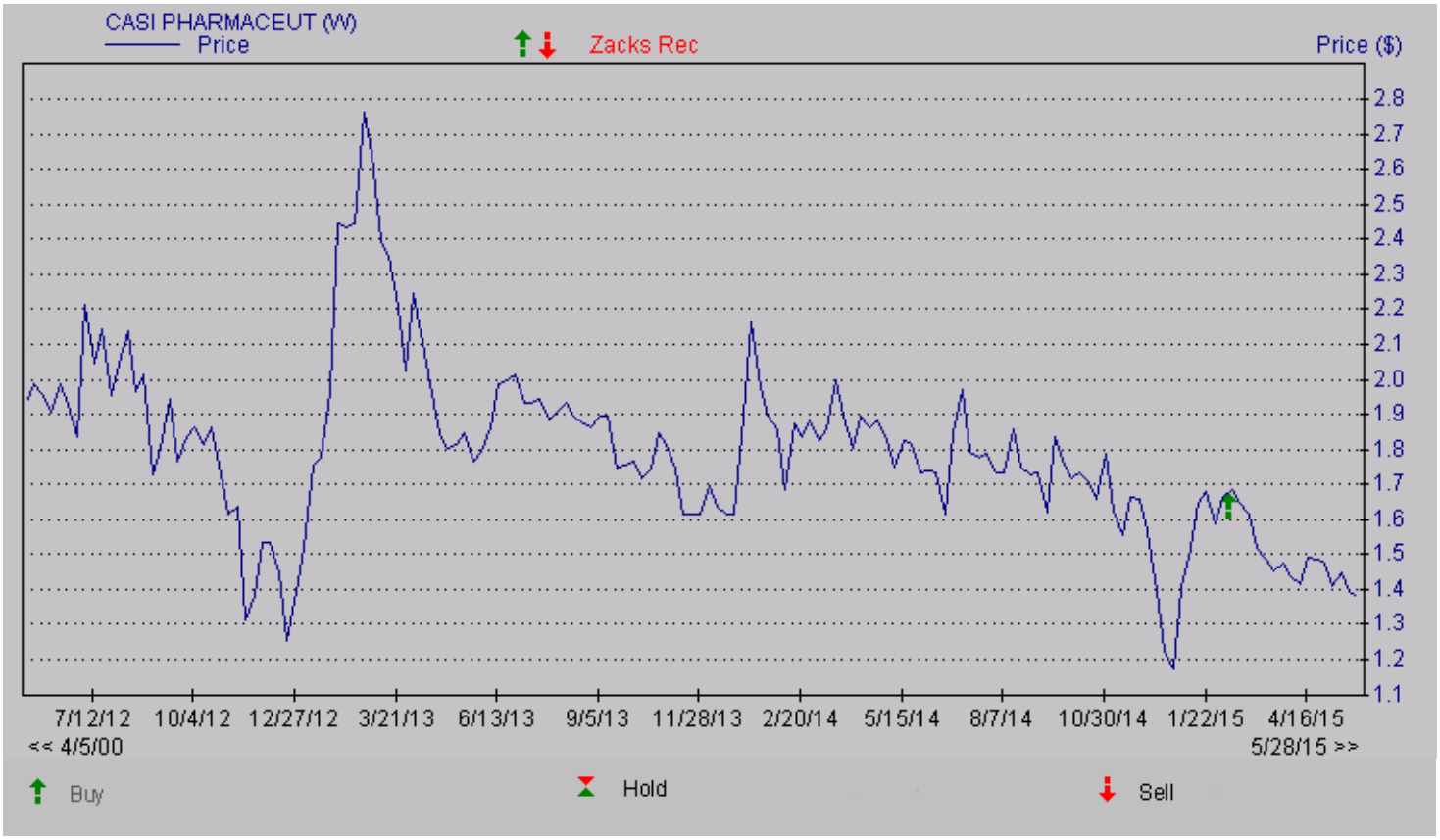

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