Zacks Small-Cap Research

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Celator Pharma (CPXX-NASDAQ)

CPXX: Unique proprietary CombiPlex platform technology coupled with novel liposomal/nanoparticle delivery systems – initiating with Outperform

Current Recommendation	**Outperform**
Prior Recommendation	N/A
Date of Last Change	04/15/2014
Current Price (05/19/14)	$2.56
12-Month Target Price	**$7.00**

OUTLOOK

Celator is a late stage drug development company with a focus on cancer. The Company has a pipeline based on its unique proprietary CombiPlex platform technology and liposomal/nanoparticle delivery system.

Its lead candidate CPX-351 is a combination of cytarabine and daunorubicin co-encapsulated in a synergistic ratio. CPX-351is in an ongoing Phase III trial and top line data will be available in 2Q15.

Fundamentals of Celator are strong and we are optimistic about the prospect of the company. We rate the shares of the company Outperform.

SUMMARY DATA

52-Week High	**$5.80**
52-Week Low	**$2.43**
One-Year Return (%)	**N/A**
Beta	**N/A**
Average Daily Volume (sh)	**15,150**
Shares Outstanding (mil)	**26**
Market Capitalization ($mil)	**$64**
Short Interest Ratio (days)	**0.57**
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 2014 Estimate	**N/A**
P/E using 2015 Estimate	**N/A**
Zacks Rank	**N/A**

Risk Level	**N/A,**
Type of Stock	**N/A**
Industry	**Pharmaceutical**
Zacks Rank in Industry	**N/A**

ZACKS ESTIMATES

Revenue
(in millions of $)

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2013	0.00 A	0.00 A	0.00 A	0.00 A	0.00 A
2014	0.00 A	0.00 E	0.00 E	0.00 E	0.00 E
2015					0.00 E
2016					0.00 E

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

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2013	-$0.71 A	-$0.18 A	-$0.14 A	-$0.13 A	-$0.95 A
2014	-$0.16 A	-$0.18 E	-$0.20 E	-$0.18 E	-$0.73 E
2015					-$0.39 E
2016					-$0.49 E

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

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KEY POINTS

- We are initiating coverage of Celator Pharmaceuticals (CPXX) with an Outperform rating. Our 12-month price target is $7.00.

- Celator is an emerging specialty pharmaceutical company focused on developing new and more effective treatments for cancers. The company has developed a unique, proprietary **CombiPlex** platform technology which identifies synergistic molar ratios for two or more agents in a combination therapy. This molar ratio is maintained in blood using **liposomal/nanoparticle** delivery system.

- Based on this platform technology, Celator has established a diversified pipeline. Its lead program **CPX-351** is a liposomal injection of a synergistic 5:1 molar ratio of cytarabine and daunorubicin for the treatment of acute myeloid leukemia (AML). CPX-351 is currently in a Phase III clinical study and enrollment is expected to be completed in 4Q14. Top line data will be available in 2Q15 and overall survival data is projected in 1Q16. Previous clinical studies of CPX-351 demonstrated significant efficacy improvements versus control and the drug was safe and well tolerated. We expect CPX-351 to be approved in 2017 by the FDA and in 2018 by the EMA.

- Celator's second clinical program is **CPX-1**, a liposomal formulation of irinotecan: floxuridine, for the treatment of colorectal cancer. Celator has completed a Phase I and a Phase II study of CPX-1, which demonstrated improved efficacy against colorectal cancer. Celator is evaluating the clinical development program for CPX-1 and additional clinical studies could start **in 2015**.

- Other than CPX-351 and CPX-1, Celator has two **preclinical programs** aimed at treating solid tumors. CPX-571 is a liposomal injection of irinotecan: cisplatin and CPX-8 is a taxane nanoparticle based on the Company's proprietary nanoparticle platform technology.

- Balance sheet remains strong. As of March 31, 2014, Celator held cash and cash equivalents of $20.8 million. The Company has $10 million outstanding debt. Current cash is expected to last until 4Q15 and is primarily supporting the Company's currently enrolling Phase III clinical study of CPX-351.

- We are optimistic about Celator's technology and drug candidates. The Company's fundamentals remain strong. We think Celator is undervalued at current market price and encourage investors to accumulate its shares. Risk is still high at this time though. However, we believe Celator is a name for investors with a long term investment horizon and high risk tolerance.

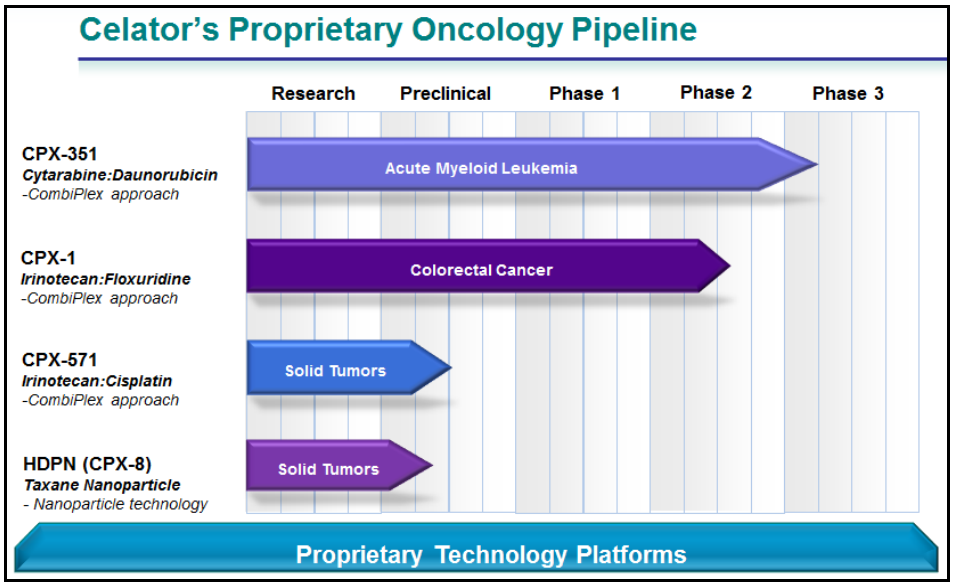
OVERVIEW

Celator Pharmaceuticals (CPXX) is a late stage development pharmaceutical company focused on developing new and more effective therapies to treat **cancer.**

CPXX's key advantage relies on its proprietary **drug ratio** technology platform **CombiPlex®**, which represents a novel approach that identifies molar ratios of drugs that will provide a synergistic benefit, and locks the desired ratio in a **nano-scale drug delivery vehicle** that maintains the ratio in patients with the goal of improving clinical outcomes.

Based on the CombiPlex platform, the Company has developed a pipeline targeting various cancers. The Company's pipeline includes two clinical stage products, **CPX-351**, a liposomal formulation of cytarabine:daunorubicin for the treatment of acute myeloid leukemia (AML), and **CPX-1**, a liposomal formulation of irinotecan:floxuridine, for the treatment of colorectal cancer. The pipeline also includes two

preclinical stage product candidates, **CPX-571**, a liposomal formulation of irinotecan:cisplatin, and **CPX-8**, a hydrophobic docetaxel prodrug nanoparticle (HDPN).



Celator was founded in 1999 and is headquartered in Ewing, New Jersey. The Company also has an office in Vancouver, Canada.

INVESTMENT THESIS

The Unique, Proprietary CombiPlex Platform Technology

Over the years, Celator has developed a unique, proprietary drug combination platform technology: **CombiPlex**.

Combination therapy is a powerful and effective therapeutic regimen in the treatment of cancers, which can generate increased efficacy and reduced side effects, leading to optimal therapeutic outcomes. For most patients with cancer, **standard of care** usually involves the use of **combinations** of individual drugs. This strategy is based on the proven premise that cancer cells can be destroyed more effectively by combining drugs with different and complementary mechanisms of action. Because many cancer drugs are toxic at certain dosing levels, clinicians typically select individual drugs that do not have overlapping toxicities. Drugs are typically combined at their "maximum tolerated dose" (MTD), the dose at which a drug has been shown to deliver benefit balanced by an acceptable level of toxicity.

This approach assumes that drugs combined at their individual MTD will result in therapies with maximum clinical benefit in patients. However, in many cases this is likely incorrect for two important reasons:

- *Preclinical research has shown that dosing individual drugs at MTD does not always produce combination drug regimens that deliver maximum efficacy since different ratio of the drugs may be synergistic, additive or antagonistic.*
- *Combining drugs at their synergistic ratio is not enough. Without a technology to maintain that ratio in patients, the individual drugs will be metabolized independently and at different rates. As a result, the ratio of drugs will change over time and may negatively impact their effectiveness if antagonistic ratios result.*

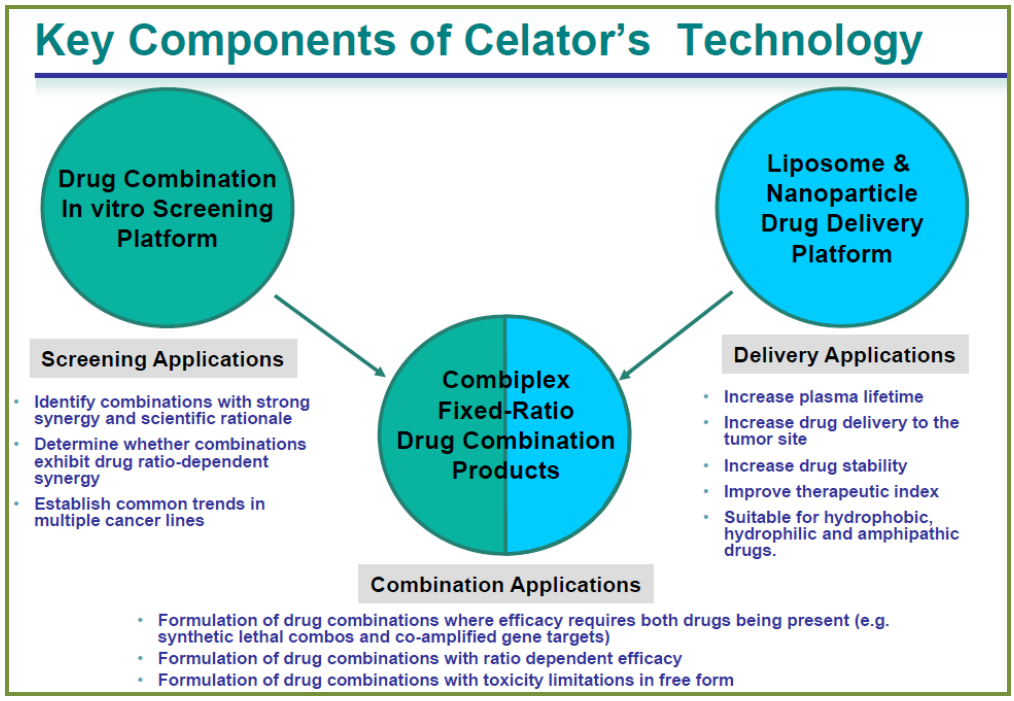
Through deep research, scientists at Celator have recognized that drug ratios can play a critical role in combination therapy. The results of multiple in vitro and preclinical studies have demonstrated that the molar ratios of drugs used can have a significant impact on the overall efficacy and safety of combination chemotherapy. It's already recognized that the same drugs combined at different ratios can result in distinctly varied efficacy and safety profiles. Depending on the ratio of the combined drugs, the outcome can be:

- **Additive –** the anti-cancer effect of the drugs is equal to the sum of the individual drugs.
- **Synergistic –** the anti-cancer effect of the drugs is greater than the sum of the individual drugs.
- **Antagonistic –** the anti-cancer effect of the drugs is less than the sum of the individual drugs.

Although synergy relationship can be evaluated readily in vitro where drug ratios can be controlled, the translation of such information in vivo is complicated by the fact that the individual drugs administered as a conventional combination or drug "cocktail" may be distributed, metabolized and eliminated differently. This prevents control of the drug ratio following administration and may result in exposure of cancer cells to antagonistic drug ratios with a corresponding loss of therapeutic activity.

However, the standard of care rarely takes advantage of the critical role that drug ratios may play in combination chemotherapy treatment. The ability to identify the ratio of drugs that will produce a synergistic benefit, and a technology that makes it possible to maintain and deliver that ratio in the body, could have a profound impact on combination chemotherapy efficacy. That's why Celator developed its CombiPlex technology platform.

A distinguishing feature of Celator's CombiPlex technology is the **proprietary delivery vehicles** developed to encapsulate and maintain drug combinations at the desired ratio after in vivo administration. Drug combinations used to treat cancer are often comprised of agents with very different chemical compositions and physical properties. Consequently, formulating drug combinations with such disparate features into a single pharmaceutical product that can release both drugs at the same rate in the body represents a significant technical challenge. Celator has developed two distinct nano-scale drug delivery technology platforms**: liposomes** and **nanoparticles,** which together provide great versatility in controlling the encapsulation and retention properties for therapeutic agents from a wide range of drug classes.



Source: company presentation

Both liposomal and nanoparticle delivery systems have the following advantages:

- control the ratio of the drugs in the carrier when prepared;
- maintain the ratio of the drugs in the carrier while circulating in the blood; and
- ensure that the drugs are released at the correct synergistic ratio when delivered to the tumor by the carrier;

CPX-351: Lead Candidate for AML

Rationale for CPX-351

Celator's lead drug candidate is **CPX-351**, which is a liposomal formulation of a synergistic **5:1 molar ratio** of **cytarabine** and **daunorubicin**, two agents commonly used to treat hematologic malignancies, particularly acute myeloid leukemia (**AML**).

In vitro screening of the drug ratio effects for cytarabine and daunorubicin were performed with a variety of different tumor cell lines. The results of this screening show the ratios of cytarabine and daunorubicin that are synergistic (green), additive (yellow) or antagonistic (red).

CPX-351 (Cytarabine:Daunorubicin) Heat Map

Cell Lines	Tissue	CI @ ED90				
		1:10	1:5	1:1	5:1	10:1
HCT-116	Colon	0.8	0.7	0.8	0.8	0.8
SW620	Colon	1.0	0.6	0.8	0.7	0.6
Nalm-6	Leukemia	1.8	1.6	1.1	1.1	1.1
P388	Leukemia	1.2	1.5	0.6	0.7	0.8
HL60	Leukemia	1.3	1.2	0.8	0.8	1.1
L1210	Leukemia	1.4	1.5	1.3	0.8	1.1
A253	Oral	1.1	1.0	1.1	0.8	0.9
BXPC-3	Pancreatic	1.1	1.1	1.2	1.0	0.9
IGROV-1	Ovarian	2.0	1.3	1.5	1.1	0.9
Capan-1	Pancreatic	1.0	0.9	1.0	0.9	1.6

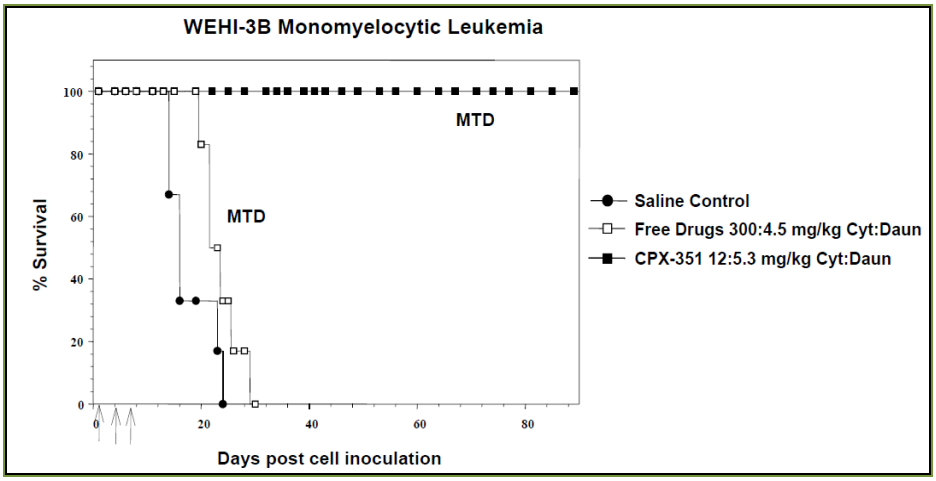
Synergistic
Additive
Antagonistic

As shown in the above chart, when tested in a panel of tumor **cell lines** including a number of leukemias**,** the combination of cytarabine plus daunorubicin consistently displayed synergy and avoided antagonism at a 5:1 molar ratio. Decreasing the drug ratio resulted in a loss of synergy and an increased occurrence of antagonism.

Preclinical Studies

After the synergistic ratio was determined, nano-scale **liposomes** were engineered that co-encapsulated the two agents and maintained the drug ratio for extended times after intravenous injection. The drug ratio dependency and superiority of the 5:1 molar ratio was also confirmed with these delivery systems in **preclinical** leukemia efficacy studies. CPX-351 exhibited superior therapeutic activity compared to the conventional administration of this drug cocktail in all leukemia models tested, with high proportions of cures, and evidence of in vivo synergy under conditions where the unencapsulated drug cocktail provided minimal anti-leukemic activity. Importantly, this increased activity was selective for leukemia cells.

In a preclinical leukemia model below, dramatic efficacy improvements were achieved by delivering a synergistic drug ratio.



Increased efficacy with CPX-351 was also observed **in mice** bearing bone marrow-engrafted human leukemia. In the absence of treatment the bone marrow is completely overrun with human leukemia cells within 21 days of intravenous tumor inoculation. The drug cocktail of cytarabine:daunorubicin administered at its maximum tolerated dose (MTD) caused a modest reduction of leukemia cells in the bone marrow as evidenced by bone marrow cross sections. These mice died from leukemia shortly after treatment terminated. **In contrast**, the bone marrows of mice treated with CPX-351 were completely cleared of tumor cells and survival of the mice was extended by months. Studies have also demonstrated that CPX-351 persists in the bone marrow for days and that the liposome encapsulated drugs are taken up more by leukemia cells than by normal bone marrow cells.

Clinical Experience

Based on these preclinical studies, three clinical studies have been completed with CPX-351 to date:

- a **Phase I** (study 101) dose escalation study in 48 advanced leukemia patients;
- a randomized, controlled **Phase II** (study 204) study in 126 newly diagnosed AML patients age 60 – 75;
- and a randomized, controlled **Phase II** (study 205) study in 125 AML patients in first relapse age 18 – 65;

CPX-351 has been granted **orphan drug status** by the FDA and EMA for the treatment of AML.

Phase I Study — Study 101

Study 101 was a Phase I dose escalation study in patients with relapsed/refractory leukemia or high-risk myelodysplastic syndrome (MDS). CPX-351 was administered every other day for 3 doses by 90-minute infusion. The MTD was determined to be 101 U/m^2 (1 Unit = 1.0 mg cytarabine + 0.44 mg daunorubicin which corresponds to a 5:1 molar ratio). Pharmacokinetic (PK) analysis confirmed maintenance of the 5:1 molar ratio for over 24 hours and prolonged circulation half-life. Complete response (CR) was first observed at 43 U/m^2, roughly one-half of the MTD. At study completion, 48 patients had been entered. Among 43 AML patients, CR was achieved in nine patients and complete response with incomplete normal blood cell recovery (CRi) was observed in one patient at 32 U/m^2. Dose-limiting toxicities were observed in three of six patients at 134U/m^2; 1 patient had persistent (>56 days) cytopenias, 1 patient had hypertensive crisis and 1 patient had congestive heart failure in the setting of sepsis.

The positive outcomes of this Phase I study supported evaluating the efficacy of CPX-351 in a Phase II setting.

Randomized Phase II Study--Study 204

Study 204 was a randomized, controlled Phase II study in **newly diagnosed AML** patients age 60 – 75, comparing CPX-351 (100 U/m^2) against first-line therapy (7+3 regimen of cytarabine plus daunorubicin using 100 mg/m^2 cytarabine per day for 7 days as a continuous infusion, combined with 60 mg/m^2 daunorubicin per day for the first 3 days). This study was intended to be a direct test of whether delivery of a fixed, synergistic ratio of the two drugs (**CPX-351**) provided increased clinical efficacy over conventional administration of the same agents (**the 7+3 regimen**).

Patients were randomized 2:1 to receive CPX-351 or 7+3, respectively, and patients were stratified by risk group where patients classified as high risk had either older age (70 – 75) or secondary AML (**sAML**) or complex cytogenetics (≥3 abnormalities). The primary endpoint of this study was response rate (CR + CRi) and a successful Phase II study was prospectively defined as a difference in response rate favoring CPX-351 with a p-value <0.1, which would support moving forward to a Phase III study.

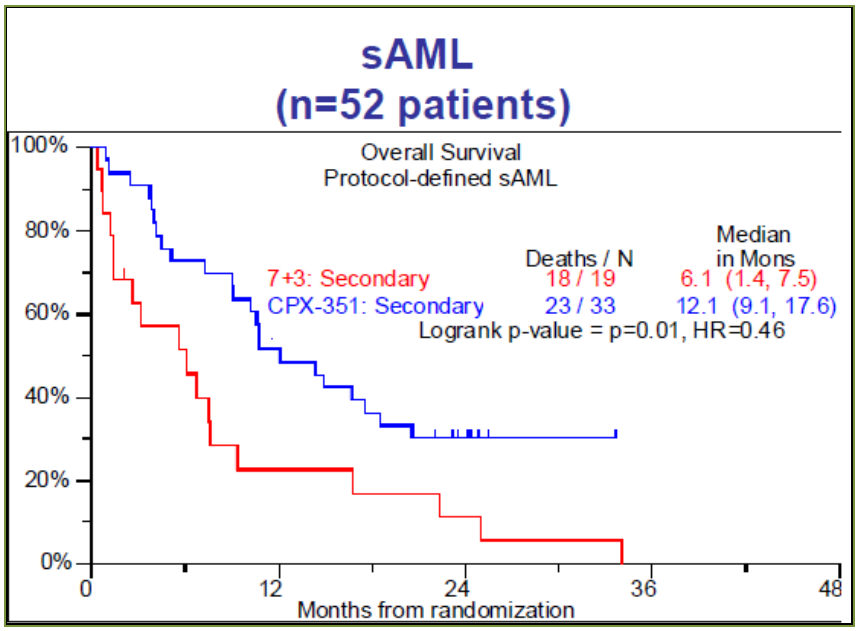
The table below presents the efficacy and early mortality results from Study 204 for the overall patient population as well as for sAML patients, a group that was prospectively identified for subset analysis in the protocol. sAML is AML that arises from a pre-existing myelodysplastic syndrome or myeloproliferative disease, or treatment-related AML, which results after chemotherapy or radiation therapy for a previous cancer. sAML is typically associated with a worse prognosis. In the overall population, patients treated with CPX-351 achieved relative improvements over the control arm; a 30% higher response rate (66.7% vs. 51.2%) and a 68% decrease in 60-day mortality rate (4.7% vs. 14.6%).

Study 204	All Patients		All Patients	sAML		sAML
	CPX-351 n=85	7+3 n=41	Relative Improvement	CPX-351 n=33	7+3 n=19	Relative Improvement
Response (CR+CRi) Rate (%)	66.7	51.2	30.3%	57.5	31.6	82.0%
60-Day Mortality (%)	4.7	14.6	67.8%	6.1	31.6	80.7%
Median Event-Free Survival (months)	6.5	2.0	225.0%	4.5	1.3	246.2%
Median Overall Survival (months)	14.7	12.9 *	14.0%	12.1	6.1 *	98.4%

*Includes patients deemed non-responders to 7+3 who crossed over and received CPX-351

One of the primary objectives of this randomized, controlled, Phase II clinical study was to identify a well-defined patient population exhibiting benefits from CPX-351 treatment that would be predictive of success in a Phase III setting. While improvements in leukemia clearance and response were observed for CPX-351 in all AML subgroups, the benefits were largest in high risk patients and sAML patients, in particular, which constitute approximately 41% of the older study population. In sAML patients, the response rate increased by 82% (57.5% vs. 31.6%), the 60-day mortality was decreased 81% (6.1% vs. 31.6%), and the median overall survival time increased approximately 98% (12.1 months vs. 6.1 months). Overall survival (OS) results in sAML demonstrated improvements with CPX-351 that were statistically significant (p=0.01) with an approximate 54% decrease in the risk of death (hazard ratio=0.46) after 24 months of follow-up. It is important to note that patient demographics and risk factors were well balanced between the two study arms indicating that the improvements were not likely due to imbalances between the treatment arms favoring CPX-351.

Significant **overall survival** improvement was achieved in newly diagnosed **sAML** patients as shown below.



An exploratory feature of this study design was that patients randomized to the control arm were allowed to **cross over** to receive CPX-351 if there was evidence of persistent AML after 7+3 treatment with little likelihood of achieving response. This design element provided important evidence that CPX-351 is active in patients who do not respond to the 7+3 regimen. Specifically, of the 10 non-responders to 7+3 who chose to cross over to CPX-351 treatment, four achieved complete response and all four were alive beyond one year. The efficacy of CPX-351 observed in these patients suggests that CPX-351 may be active in treating AML patients who fail front line treatment with the 7+3 regimen.

In terms of safety, a qualitatively similar profile of adverse events was observed for both CPX-351 and the 7+3 regimen. The key difference in adverse events between CPX-351 and 7+3 was greater myelosuppression with more prolonged median time to recovery from neutropenia (4 additional days) and thrombocytopenia (9 additional days) observed in the CPX-351 arm. This difference can be considered the flip side of the same effect that potent activity in the bone marrow had on leukemia clearance.

Associated with this greater myelosuppression were increased adverse events such as febrile neutropenia and infection. In spite of these increased AEs, there was a decrease in the 60-day mortality rate associated with CPX-351 compared to 7+3 (4.7% vs. 14.6%), indicating that the AEs associated with CPX-351 treatment were reversible and well managed.

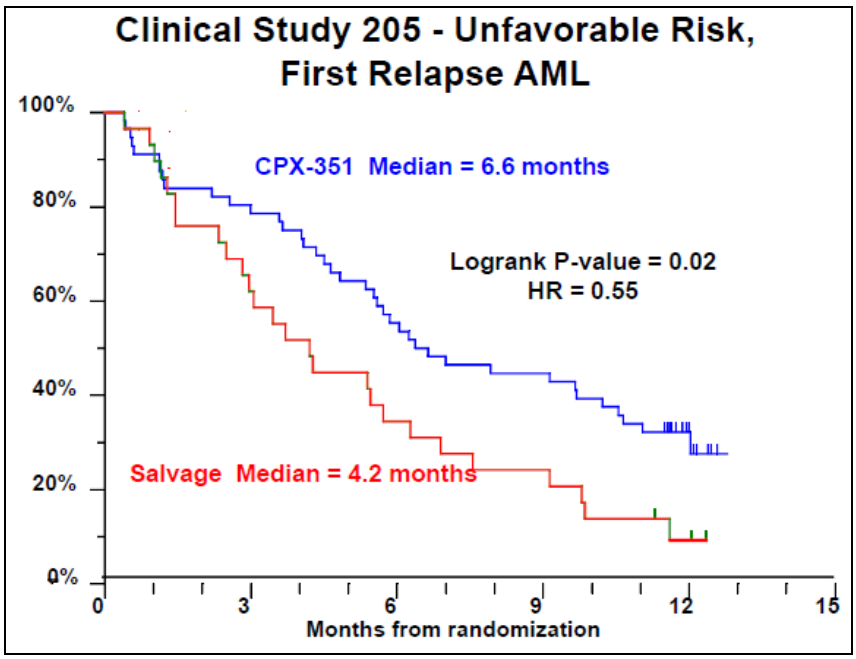
Randomized Phase II Study— Study 205

Study 205 was a second randomized, controlled **Phase II** study for patients with **first relapse AML**, age 18 – 65. Study 205 (supported through partnership with the Leukemia and Lymphoma Society®, LLS) was structured similarly to the newly diagnosed AML clinical study. Patients were randomized 2:1 to receive either CPX-351 (81 patients) administered at the same dose and schedule as used in Study 204 or control arm therapy consisting of investigator selected salvage treatment (44 patients). Patients were stratified by a methodology known as the European Prognostic Index (EPI) into three strata: favorable risk, intermediate risk, and poor risk based on a scoring system that evaluated duration of first remission, cytogenetics, age, and presence or absence of prior stem cell transplant. The risk factors used to calculate the EPI were well balanced between the CPX-351 and salvage treatment arms. The distributions across risk groups were similar to historical data with approximately 12%, 20% and 68% in favorable, intermediate and poor risk groups, respectively.

The primary endpoint of this study was survival at one year with secondary endpoints including response rate and 60-day mortality. Due to the lack of any established standard of care for first relapse AML, the choice of salvage treatment for the control arm was determined by the investigator. Historical data indicate that approximately 70% of patients with first relapse AML of this age will die of their leukemia within the first year, regardless of the salvage treatment used.

The results from the first relapse AML Phase II study closely mirrored those in newly diagnosed AML patients. CPX-351 exhibited higher response rate as well as improvements in OS and 1-year survival rate. Similar to the newly diagnosed AML study, the degree of benefit provided by CPX-351 was greater in patients with poor risk characteristics (by EPI criteria). For these patients who constituted 68% of the patients in this study, CPX-351 outperformed control arm salvage treatment with a 42% relative increase in response rate (39.3% vs. 27.6%), a 33% decrease in 60-day mortality (16.1% vs. 24.1%), a 57% relative increase in median OS (6.6 months vs. 4.2 months) and a 172% increase in 1-year survival rate (28.0% vs. 10.3%). The overall survival curves for the unfavorable risk (poor risk) first relapse AML patients revealed a statistically significant (p=0.02) improvement for CPX-351 with an approximate 45% decrease in the risk of death (hazard ratio=0.55).

Study 205	All Patients		All Patients	Poor-risk EPI		Poor-risk EPI
	CPX-351 (n=81)	Salvage (n=44)	Relative Improvement	CPX-351 (n=56)	Salvage (n=29)	Relative Improvement
Response (CR+CRi) Rate (%)	49.4	40.9	20.8%	39.3	27.6	42.4%
60-Day Mortality (%)	14.8	15.9	6.9%	16.1	24.1	33.2%
Median Event-Free Survival (months)	4.0	1.4	185.7%	1.9	1.2	58.3%
1-Year Survival (%)	35.8	27.3	31.1%	28.0	10.3	171.8%
Median Overall Survival (months)	8.5	6.3	34.9%	6.6	4.2	57.1%



The general safety profile was comparable for both study arms except for more prolonged neutropenia and thrombocytopenia following CPX-351 with median absolute neutrophil count recovery (>1000/µL) requiring 42 days vs. 34 days and median platelet recovery (>100,000/µL) requiring 45 days vs. 35 days. More prolonged myelosuppression led to higher rates of severe infections (66.7% vs. 61.4%), fatal infections (12.3% vs. 6.8%) and hemorrhage events.

The encouraging results obtained with CPX-351 in the first relapse AML study not only identify an additional AML population that is likely to benefit from CPX-351 treatment, but corroborate the efficacy and safety outcomes observed with CPX-351 in newly diagnosed AML patients.

To date, clinical studies using CPX-351 have demonstrated a toxicity profile that was deemed acceptable by the investigators. In the prechemotherapy era, AML invariably led to death from infections or major bleeding events within weeks to months of diagnosis. Active AML leads to death by producing a general shut down of blood cell function with loss of white blood cells and marked reductions in platelets and red blood cells. Loss of white blood cell function inevitably leads to risk of severe infections and loss of platelets leads to risk of major bleeding events. In this setting, physicians have traditionally accepted the risk of high rates of adverse events, including severe or life threatening but reversible adverse events, provided there is evidence of greater benefit in the form of objective response and prolonged survival.

The balancing of risk and benefit occurs at the start of every treatment course, and every investigator understands that the risk of withholding potentially toxic but useful treatment is death from progressive AML within a short period of time.

In summary, the adverse event profile is acceptable to investigators because no unacceptable adverse events have been encountered and the benefits of treatment appear to outweigh the risks.

The Ongoing Phase III Study of CPX-351---Study 301

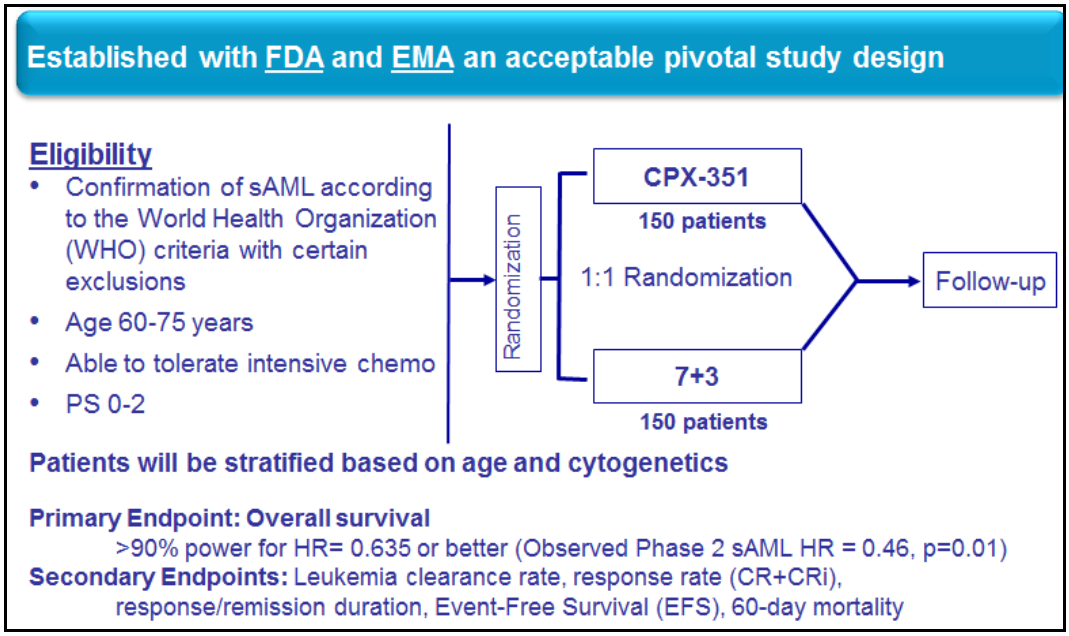
Based on the Phase I and two randomized Phase II studies, as well as discussions with the FDA and EMA, Celator initiated a pivotal **Phase III study** of CPX-351 in December 2012.

This Phase III trial is a multicenter, randomized, open-label clinical trial of CPX-351 versus conventional cytarabine and daunorubicin therapy ("7+3") as first-line therapy in patients 60-75 years old with high-risk (secondary) acute myeloid leukemia (AML). The Phase III study is being conducted in partnership with The Leukemia & Lymphoma Society® (LLS) through its Therapy Acceleration Program (TAP).

The study will enroll patients between the ages of 60 and 75 who have pathological diagnosis of AML according to WHO criteria (with at least 20 percent blasts in the peripheral blood or bone marrow) with confirmation of:

- Therapy-related AML;
- AML with a history of myelodysplasia (MDS);
- AML with a history of chronic myelomonocytic leukemia (CMMoL);
- De novo AML with chromosome abnormalities characteristic of MDS;

Patients will be randomized 1:1 to receive either CPX-351 (100u/m^2; Days 1, 3, and 5 by 90 minute infusion) or 7+3 (cytarabine 100mg/m^2/day by continuous infusion for 7 days and daunorubicin 60mg/m^2 on days 1, 2, and 3). Patients will be monitored for all clinical adverse events as well as laboratory evaluations. The **primary efficacy endpoint** of the study is overall survival. The study is being conducted in the United States and Canada with more than 40 leading cancer centers participating. The study is designed to randomize and treat **300 patients**, which provides greater than 90% power to detect an approximate 36.5% decrease in the risk of death (hazard ratio=0.635) with statistical significance ($p \leq 0.05$).



In December 2013, the independent Data and Safety Monitoring Board (DSMB) reviewed the first 75 patients and recommended the study continue as planned without any modifications.

In January 2013, the Phase III study reached 50% of the planned enrollment of 300 patients, slightly ahead of schedule. Celator anticipates completing the enrollment in the **fourth quarter of 2014**. Initial data from the study, on secondary efficacy endpoint of remission rate, could be available in the **second quarter of 2015**. The primary endpoint, overall survival, is projected to be available in the **first quarter 2016** with the NDA anticipated to be filed in the second half of 2016. We estimate CPX-351 to be approved in late 2017 by the FDA and in 2018 by the EMA.

If approved, CPX-351 may ultimately replace 7+3 on the basis of improved efficacy and acceptable safety.

In addition to the Phase III study of CPX-351 for sAML, Celator is also studying CPX-351 in **other patient populations** through investigator-initiated studies. Additional investigator-initiated studies that are underway and supported by the Company including:

- reduced intensity conditioning for HSCT (hematopoietic stem cell transplantation) utilizing donor bone marrow;
- reduced intensity conditioning for HSCT utilizing cord blood;
- AML patients with high-risk of early mortality (determined by the treating physician to be unsuitable or “unfit” for intensive chemotherapy);
- relapsed or refractory leukemia in children and young adults; and
- patients with higher risk myelodysplastic syndrome (MDS) or acute myeloid leukemia (AML), who are refractory to or in relapse after hypomethylating agent (HMA) therapy.

The Company plans to support additional investigator-initiated studies and possibly oncology cooperative group studies. The development of CPX-351 in other AML patient populations will help establish the utility of the drug. The differentiation strategy being pursued is for CPX-351 to replace the standard of care, the 7+3 regimen, in AML. There has been interest among hematologists in extending the use of CPX-351 to other hematologic malignancies such as myelodysplastic syndrome and acute lymphoblastic leukemia based on data obtained in Celator’s clinical studies as well as in preclinical studies.

Market Potential for CPX-351

Background of AML

Acute Myeloid Leukemia (AML) represents a group of clonal hematopoietic stem cell disorders in which both failure to differentiate and excessive proliferation in the stem cell compartment result in accumulation of non-functional cells termed myeloblasts. Untreated AML in all ages is rapidly fatal, with patients dying on average within a few months of diagnosis. Even with treatment, particular groups of AML patients continue to have a poor prognosis.

According to The American Cancer Society, in 2014, about 18,860 new cases of AML will be diagnosed, most will be in adults. About 10,460 AML patients will die from AML, almost all will be in adults. AML is generally a disease of older people and is uncommon before the age of 45. The average age of a patient with AML is about 66 years. Given that the median age at diagnosis of AML is 66 years, it is not surprising that the majority of patients diagnosed with AML are at high risk and in need of improved treatment options.

AML in older patients (age ≥60) is associated with increased risk of not responding to therapy and increased risk of dying from the treatment. The poor results of treatment in older patients with AML have led to reluctance to treat some elderly patients with intensive regimens designed to induce aplasia and complete remission.

Secondary AML (sAML) refers to the development of AML following the history of a previous disease, such as a myelodysplastic syndrome or a chronic myeloproliferative disorder. Secondary AML can also be a consequence of treatment with chemotherapy, including alkylating agents and topoisomerase II inhibitors, and/or radiotherapy, or due to exposure to environmental carcinogens. At the chromosomal level, specific karyotypic abnormalities have been identified and linked to sAML subtypes.

Secondary AML is reported as 20-30% of the AML incidence and is estimated as 40% of AML cases in patients >60 years of age. Prognosis for patients with AML is poor with median OS of 9-12 months, and sAML is even worse with median OS of 6-7 months with current treatments.

Current Treatment Regimens for AML

Treatments for AML patients usually include **induction therapy** followed by post-remission chemotherapy (**consolidation**) for most patients. For some patients, this will be followed by hematopoietic **stem cell transplantation**. Treatment options for AML patients vary depending on the patient's age, cytogenetics, and prognostic factors. Currently, the treatment options are often divided into two groups: one for patients younger than 60 and one for patients 60 years and older.

The goal of **induction chemotherapy** is to reduce the number of leukemic cells as well as return proper function to the bone marrow. The 7+3 regimen of cytarabine (100 mg/m^2 for 7 days) plus daunorubicin (45 to 60 mg/m^2 for 3 days) is the most common induction regimen for both age groups. Induction is successful in about 65% of all AML patients who get standard induction chemotherapy with daunorubicin and cytarabine. The actual chance of remission depends to a large part on a person's specific prognostic factors, such as age or the presence of certain gene or chromosome changes.

Post-remission **consolidation therapy** then aims to eradicate any residual disease in an attempt at cure. Post-remission chemotherapy includes high-dose cytarabine (ara-c; HiDAC) for patients younger than 60 years, whereas a 5 or 5 + 2 regimen of cytarabine plus an anthracycline or anthracenedione is preferred for patients older than 60 years. HiDAC has proven to be efficacious for young patients with good or intermediate prognosis. In patients younger than 60 years, HiDAC yields a 4-year disease-free survival rate of 44%, with relatively few relapses, but carries with it a 5% treatment-related mortality. In contrast, HiDAC failed to improve the outcome of patients older than 60 years. HiDAC has shown particular efficacy for patients with CBF DNA subunit abnormalities.

Older patients generally don't do as well as those younger than 60. They have trouble tolerating intensive treatment and often have chromosome changes in their leukemia cells that are linked to a poorer outlook.

	Age (yr)	
Parameter	**<60**	**≥60**
Induction chemotherapy	7 + 3	7 + 3
Postremission chemotherapy	HiDAC	5 or 5 + 2
Complete response rates (%)	65-85	40-55
Treatment-related mortality (%)	5-10	20-30
5-year disease-free survival (%)	30	5-10

HiDAC, high-dose cytarabine (1000-3000 mg/m^2 IV over 1-3 hr every 12 hr for 6 to 12 doses); 7 + 3, 7 days of cytarabine at 100 mg/m^2 + 3 days of an anthracycline or anthracenedione (most commonly idarubicin, 12 mg/m^2, mitoxantrone, 12 mg/m^2, or daunorubicin, 45 mg/m^2); 5 or 5 + 2, 5 days of cytarabine at 100 mg/m^2 alone or combined with 2 days of an anthracycline or anthracenedione.

Source: Cleveland Clinic

Allogeneic or autologous **bone marrow transplantation** is an additional option for post-remission therapy in adults with AML. For some patients younger than 60 years and for whom an HLA-matched sibling or matched unrelated donor is available, allogeneic stem cell transplantation should follow induction chemotherapy. However, this procedure has an associated 20% to 25% treatment-related mortality rate. For patients without a compatible donor or for whom age precludes such treatment, additional chemotherapy or autologous stem cell transplantation are options.

CPX-351 Advantage and Opportunity

The 7+3 regimen has been the **standard first-line treatment** for AML for over 40 years. Better therapies are urgently needed in view of the relatively poor prognosis for most patients with AML. Efforts to improve these outcomes, including intensification of treatment via modification of dose levels and schedules and/or addition of new cytotoxic and targeted therapies to existing regimens, have had limited success.

Despite the widespread use of the 7+3 regimen for AML, there has been little research focused on understanding how these two drugs interact on a cellular level. The different treatment schedules used for cytarabine and daunorubicin in the 7+3 regimen result in leukemia cells being exposed to constantly changing ratios, which may limit the effectiveness of this chemotherapy combination when some of those ratios are antagonistic. In fact, drug ratio dependency was observed and superiority of the 5:1 molar ratio was identified by Celator in pre-clinical studies, both in vitro and in vivo. Consequently, the CombiPlex technology platform was applied to this combination and nano-scale liposomes were engineered that co-encapsulated cytarabine and daunorubicin, to maintain the optimal synergistic drug ratio for extended periods of time after intravenous injection, with the aim of improving anti-leukemia activity.

From the existing clinical data, we believe **CPX-351** has the potential to replace 7+3 as the current standard of care for AML. CPX-351 contains the same 2 active ingredients, cytarabine and daunorubicin, but at a pre-determined synergistic 5:1 molar ratio. This synergistic ratio is preserved and maintained in human serum via co-encapsulation within a nanoparticle-size liposomal formulation. CPX-351 is administered as a 90-minute infusion on days 1, 3, and 5. Data from the pivotal study, along with data from the completed Phase II studies and the ongoing and proposed clinical studies (investigator-initiated or possibly cooperative group studies) are critical to establish the benefit of CPX-351 in patients with AML and other hematologic malignancies.

Celator selected sAML as the “get-to-market” strategy because this represents the fastest path to market for a Phase III study and a higher probability for success because a statistically significant survival advantage has already been demonstrated in the completed Phase II study. CPX-351 may provide potential benefit in other AML populations as well as other hematologic malignancies.

We estimate CPX-351 could be available to patients as early as 2017. Following the completion of the Phase III study, it is expected that pharmaceutical companies focused in oncology with interests in hematologic malignancies may be interested in gaining access to CPX-351 and the technology. This may result in licensing, merger or acquisition offers to Celator.

We estimate peak sales of CPX-351 in the range of $300 to $500 million globally.

CPX-1 for Colorectal Cancer

The rationale for CPX-1

The combination of **irinotecan** plus a fluorinated pyrimidine, typically **5-FU**, is a standard chemotherapy combination for the treatment of metastatic colorectal cancer **(CRC).** In the **FOLFIRI** (folinic acid (leucovorin), 5-FU and irinotecan) regimen used today, irinotecan is administered bi-weekly as a 90 minute infusion of 180 mg/m^2 on day 1 and 5-FU is administered starting with a bolus loading dose of 400 mg/m^2 followed by a continuous infusion of 2.4-3 gm/m^2 over 96 hours starting on day 1. Consequently, irinotecan: fluoropyrimidine ratios are continuously changing during the course of therapy, with the possibility of tumor cells being exposed to antagonistic drug ratios.

Celator chose floxuridine rather than 5-FU for evaluation of drug ratio dependent synergy because floxuridine displayed enhanced retention in liposomes as well as the fact that previous studies have demonstrated the clinical equivalency of floxuridine with 5-FU in comparative clinical studies. Celator conducted the screening of the combination of **irinotecan** and **floxuridine** for drug ratio dependent synergy in vitro in a panel of tumor cell lines. The 1:1 molar ratio of irinotecan:floxuridine was frequently synergistic and avoided antagonism.

CPX-1 (Irinotecan HCl:Floxuridine) Heat Map

Cell Lines	Tissue	CI @ ED75				
		1:10	1:5	1:1	5:1	10:1
Capan-1	Pancreatic	1.3	0.5	0.5	0.5	0.7
HT-29	Colorectal	0.8	0.3	0.5	3.4	10.0
SF-268	CNS/Glioma	0.8	1.1	1.1	1.3	1.2
HCT-116	Colorectal	0.2	0.2	0.2	1.0	4.4
LS180	Colorectal	0.9	0.8	0.3	1.1	0.5
A549	Lung	0.9	0.6	0.7	1.0	0.9
L1210	Leukemia	1.0	0.9	0.8	0.8	0.6
A375	Melanoma	0.9	0.6	0.6	0.8	1.2

Synergistic
Additive
Antagonistic

A liposome formulation was developed that co-encapsulated irinotecan and floxuridine (CPX-1) and maintained the synergistic 1:1 molar ratio in the blood for approximately 24 hours after intravenous injection, selectively delivering the drugs to solid tumors.

When tested in a range of **preclinical** solid tumor models, CPX-1 consistently provided improvements in therapeutic efficacy over the unencapsulated drug cocktail of irinotecan:floxuridine. For example, in a preclinical pancreatic tumor model, CPX-1 produced 100% tumor regression and long-term cures, whereas the drug cocktail provided only modest inhibition of tumor growth.

Phase I Clinical Study of CPX-1

Based on the promising preclinical results, Celator completed a **Phase I** dose escalation clinical study of CPX-1 in patients with advanced solid tumors. The primary goal for this study was to establish the safety profile for CPX-1, recommend a safe dose for further study in a Phase II setting, determine the dose dependent PK of CPX-1 and gather evidence of antitumor activity.

The MTD of CPX-1 was identified as 210 U/m^2 (1 Unit = 1.0 mg irinotecan + 0.36 mg floxuridine) and once established, seven patients with CRC were enrolled at this dose (extension cohort) to obtain additional safety and efficacy data. Severe AEs that were dose related included grade 3 or 4 diarrhea (24.2%) and neutropenia (12.1%). The target 1:1 molar ratio of irinotecan to floxuridine was maintained in the blood of all patients for 8-12 hours and in many cases up to 24 hours.

Complete response (CR), partial response (PR), stable disease (SD), and progressive disease (PD) were evaluable in 30 of 33 patients. Three patients achieved a PR, 21 patients achieved SD and six patients had PD. Disease control (CR, PR or SD) was observed in 11 of 15 (73.3%) patients with **CRC**. Among the 18 subjects with other tumor types, one PR in non-small cell lung cancer (NSCLC), and 11 SD were observed. Progression-free survival lasting greater than 6 months was observed in six patients with colorectal cancer and one patient each with pancreatic, ovarian and NSCLC. Colorectal cancer patients exhibited a favorable median progression free survival (PFS) of 5.4 months.

Phase II Clinical Study of CPX-1

Based on the favorable data for CRC patients from the Phase I study, Celator completed a **Phase II** study in **CRC** patients. A dose of 210 U/m^2 was administered bi-weekly to patients that were either irinotecan naïve (Arm A, 26 patients) or irinotecan refractory (Arm B, 33 patients). The median PFS, response rate and overall disease control rate for CPX-1 were greater than documented in the literature for FOLFIRI in **irinotecan-naïve** CRC patients. Similar results of PFS, response rate and overall disease control rate for CPX-1 were obtained in the **irinotecan refractory** arm. Although no confirmed PRs were achieved in this patient population, the proportion of patients benefiting from CPX-1 treatment and the PFS were similar to the documented in the literature benefit of **Vectibix**® (panitumomab), an approved drug for the treatment of irinotecan refractory patients.

The adverse event profile of CPX-1 was generally consistent with the combination of irinotecan and floxuridine. In this study, dose reductions and/or delays were frequently caused by: diarrhea, nausea and vomiting, or all three. Also observed were neutropenia, infections and fatigue. There was no severe stomatitis or rash. Based on the frequency of dose reductions and delays, the dose used in this study appears to be too high. The frequency of dose reductions and delays prevented an adequate assessment of efficacy for CPX-1.

What's Next for CPX-1

The approvals of Avastin® and Epidermal Growth Factor Receptor (EGFR) inhibitors (i.e., Erbitux® and Vectibix®) for treatment of metastatic CRC have transformed the standard of care for the management of CRC patients. The majority of these patients now receive chemotherapy (e.g., FOLFIRI) in combination with a biological agent. Even as the utilization of biological agents evolves, **FOLFIRI** will continue to be a treatment regimen with which biologicals will be combined.

The clinical results obtained to date with CPX-1 suggest that it is **more efficacious** than conventional **FOLFIRI** in treating metastatic **CRC**, both in terms of response/disease control rate as well as duration of disease control. Consequently, if clinical studies confirm a benefit for CPX-1 over FOLFIRI, **CPX-1** could substitute for FOLFIRI, initially in second line treatment of FOLFOX failures.

In addition to the opportunities for combining CPX-1 with approved biological agents, the demonstration that EGFR inhibitors are ineffective against CRC exhibiting K-Ras mutations has opened an additional window of opportunity for CPX-1 since clinical studies have shown that the response to FOLFIRI is not

diminished in CRC patients exhibiting K-Ras mutations. Given the results from the Phase I and Phase II studies, we believe that CPX-1 may provide a significant benefit over FOLFIRI in CRC patients.

Taken together, the scientific evidence in clinical and preclinical studies supports the investigation of CPX-1 in multiple CRC settings. In addition, other indications in which FOLFIRI has demonstrated favorable response rates (e.g., gastric cancer), as well as those in which CPX-1 exhibited activity in the Phase I study represent additional opportunities for clinical development.

Therefore, Celator is evaluating the possibility of conducting clinical trials to study the efficacy of CPX-1 versus FOLFIRI.

Preclinical Stage Pipeline

In addition to two clinical-stage products, CPX-351 and CPX-1, Celator also has identified two **preclinical-stage products** that have exhibited promising anticancer activity in animal models. These are CPX-571, a liposomal formulation containing the combination of irinotecan plus cisplatin, and CPX-8, a hydrophobic docetaxel prodrug nanoparticle, in this case a single drug formulation that utilizes the Company's proprietary prodrug nanoparticle technology.

CPX-571 for Lung Cancer

Small cell lung cancer (**SCLC)** is characterized by rapid disease progression, low cure rates and a high incidence of mortality. According to American Cancer Society, about 224,210 new cases will be diagnosed with lung cancer (SCLC and NSCLC) in the United States in 2014. About 159,260 patients will die from lung cancer in 2014. SCLC represents about 15% of all lung cancers. Therefore, about 34,000 new cases of SCLC will be diagnosed in the U.S. in 2014.

SCLC is more responsive to chemotherapy and radiation therapy than other cell types of lung cancer; however, a cure is difficult to achieve because SCLC has a greater tendency to be widely disseminated by the time of diagnosis.

Current standard of care for SCLC is the combination of **etoposide + cisplatin**. Although the current standard of care represents the most effective drug therapy for patients with SCLC, novel treatments are warranted to improve the survival rates, particularly for recurrent disease.

The combination of **irinotecan** and **cisplatin** is another combination therapy used for the treatment of SCLC. Five trials and two meta-analyses have evaluated the combination of etoposide and cisplatin versus irinotecan and cisplatin. One of the trials showed the superiority of the irinotecan and cisplatin combination. Subsequent trials and the meta-analyses support that the regimens provide equivalent clinical benefit with differing toxicity profiles. Irinotecan and cisplatin regimens led to less grade 3 to 4 anemia, neutropenia, and thrombocytopenia but more grade 3 to 4 vomiting and diarrhea than etoposide and cisplatin regimens. Treatment-related deaths were comparable between the two groups.

Irinotecan and **cisplatin** are two approved anticancer drugs which together constitute an active combination for treating SCLC. Celator has investigated whether the efficacy of this combination could be improved by controlling drug ratios following in vivo administration. Irinotecan and cisplatin combinations were evaluated systematically for drug ratio-dependent synergy in vitro using a panel of 20 tumor cell lines. In vitro screening informatics on drug ratio-dependent cytotoxicity identified a consistently antagonistic region between irinotecan:cisplatin molar ratios of 1:2 and 4:1, which was bordered by two synergistic regions. Liposomal co-formulations of these two agents were developed that maintained a fixed drug ratio for greater than 24 hours. Drug ratio-dependent antitumor activity was demonstrated in vivo for these liposome formulations and improved antitumor activity was observed for the liposome-encapsulated **7:1 molar ratio** of irinotecan:cisplatin (designated CPX-571) compared to the drug cocktail in all models tested, including an irinotecan-resistant model.

Celator is evaluating the possibility of conducting clinical trials with CPX-571.

CPX-8

CPX-8 is a hydrophobic **docetaxel prodrug nanoparticle**, a single drug formulation that utilizes the Company's proprietary prodrug nanoparticle technology.

The taxanes comprise a class of water-insoluble anticancer drugs that are used extensively to treat a range of cancers, including breast, ovarian and non-small cell lung. Whereas liposomes are well suited as nano-scale delivery systems for water soluble drugs, they typically are unable to retain hydrophobic agents such as the taxanes after administration in the body.

Celator has developed nanoparticle formulations of both paclitaxel and docetaxel based on its proprietary **hydrophobic prodrug technology platform** that are significantly more efficacious than the conventional commercially used formulations in preclinical tumor models.

Celator has developed a library of **taxane prodrugs** that have demonstrated a wide range of plasma elimination rates and tested them for efficacy compared to their conventional drug counterparts in preclinical solid tumor models. Although conventional paclitaxel in the form of Taxol and docetaxel in the form of Taxotere were able to induce regression of established HT-29 solid tumors, only paclitaxel and docetaxel formulated as prodrug nanoparticles were able to achieve a significant proportion of complete tumor regression and prolonged delay of tumor re-growth.

Interestingly, when **CPX-8** was tested for efficacy, not only was the degree of therapeutic activity greater than Taxotere when the two agents were compared at their respective MTDs, but CPX-8 also exhibited increased antitumor potency compared to Taxotere when administered at equivalent drug doses. Consequently, CPX-8 has been identified as Celator's lead nanoparticle-based formulation for development consideration and the National Cancer Institute's Nanotechnology Characterization Laboratory has selected this product for extensive in vitro and in vivo evaluation with an interest in seeing it advance towards clinical studies.

Strong Balance Sheet

As of March 31, 2014, Celator held cash and cash equivalents of $20.8 million.

In April 2013, Celator raised $32.5 million in a private placement. A total of 10,430,034 shares of common stock were sold at a price of $3.116 per share. Warrants to purchase 0.28 shares of common stock were issued for each share of common stock purchased. An additional 1,923,599 shares of common stock and corresponding warrants were issued at the closing to certain existing stockholders of the Company.

In May, 2014, Celator entered into a loan agreement with **Hercules Technology Growth Capital** (HTGC), a specialty finance company that provides customized debt financing to companies in life sciences and technology-related markets. Hercules will provide Celator with access to a term loan of **up to $15 million.**

The first $10 million of the term loan was funded at closing, and is repayable in installments over forty-two months, including an initial interest-only period of twelve months after closing. The interest-only period is extendable to October 2015, contingent upon completion of certain related development milestones. Pursuant to the loan and security agreement, Celator issued Hercules a warrant to purchase 158,006 shares of the Company's common stock at an exercise price of $2.67 per share. The remaining $5 million of the term loan can be drawn down at Celator's option at any time between December 15, 2014 and March 31, 2015. If Celator draws down the remaining $5 million of the term loan, the warrant will become exercisable for an additional 52,669 shares of the Company's common stock.

If Celator chooses to draw down the additional $5 million, it will have sufficient working capital to meet its financial needs into the fourth quarter of 2015. Current cash is primarily supporting the currently enrolling Phase III clinical study of CPX-351 as first-line therapy in patients, 60-75 years of age, with secondary acute myeloid leukemia (AML).

Experienced Management

The management team at Celator includes industry leaders who have played key roles in the development and commercialization of many pharmaceutical products.

Scott Jackson, R.Ph., M.B.A., Chief Executive Officer

Mr. Jackson was appointed to his current position in April 2008. He joined Celator Pharmaceuticals in October 2007 as Head of Commercial Development. Mr. Jackson has more than 20 years of experience in the pharmaceutical and biotechnology industry and has held positions of increasing responsibility in sales, marketing, and commercial development at Eli Lilly & Co., SmithKline Beecham, ImClone Systems Inc., Centocor (Johnson & Johnson), Eximias Pharmaceuticals and YM BioSciences (acquired Eximias). Mr. Jackson holds a B.S. in Pharmacy from the Philadelphia College of Pharmacy and Science and an M.B.A. from University of Notre Dame.

Lawrence Mayer, Ph.D., Founder, President & Chief Scientific Officer

Dr. Mayer has played a lead role in the discovery and development of a number of anti-cancer drugs. Dr. Mayer held senior management positions at the Canadian Liposome Company and QLT Inc. before joining the BC Cancer Agency where he directed the Investigational Drug Program. Dr. Mayer has authored over 200 publications and has more than 35 patents either awarded or pending. Dr. Mayer received his B.Sc. degree summa cum laude from Wartburg and Ph.D. degree from the University of Minnesota.

Fred Powell, Chief Financial Officer

Mr. Powell joins Celator from OraPharma, Inc., where as chief financial officer he helped establish the specialty healthcare company as a stand-alone business after its sale by Johnson & Johnson in late 2010 and remained through its acquisition by Valeant Pharmaceuticals in June, 2012. Prior to joining OraPharma, he was the chief financial officer of BMP Sunstone Corporation, a publicly-traded U.S. specialty pharmaceutical company with primary operations in China, overseeing rapid, double-digit growth and its eventual sale to Sanofi-Aventis. Powell also held top finance and administration positions at Eximias Pharmaceutical Corporation, Innaphase Corporation, and ERT (formerly EResearch Technology). Powell began his career with KPMG. He is a graduate of Penn State University.

Arthur C. Louie, M.D., Chief Medical Officer

Dr. Louie is a board certified oncologist with more than 25 years of experience in pharmaceutical research and development. He was a vice president of medical affairs at Human Genome Sciences Inc. and a vice president of clinical research at Xoma Corporation. He also held leadership roles in clinical development at Pro-Neuron, Inc., Pro-Virus Inc., and Chiron Therapeutics. Dr. Louie was a director of cancer research at Bristol-Myers and consulted for Johnson & Johnson and Genta Incorporated. He was responsible for the successful effort to secure FDA approval for Proleukin IL-2, which is used for the treatment of metastatic kidney cancer and for metastatic melanoma. Dr. Louie received a B.A. in Biology with honors from Haverford College, and an M.D. from New York University School of Medicine. He completed oncology fellowships at the National Cancer Institute and at Stanford University.

Derek Miller, Chief Business Officer

Mr. Miller has more than 20 years of product development and commercialization experience. He joins Celator from Genentech, a member of the Roche Group, where he held positions of increasing responsibility. In the past two years, Miller has been a key contributor in five new oncology product launches at Genentech. He recently served as National Director Field Reimbursement, BioOncology, and led a national team of reimbursement experts responsible for ensuring that health care providers and patients had access to a portfolio of ten oncology products. Before that, he was Marketing Director, Rituxan® Hematology, where he was responsible for all aspects of marketing for one of the most successful hematology products to date. Prior to joining Genentech, he held senior positions at Centocor, which was a wholly owned subsidiary of Johnson & Johnson, and GlaxoSmithKline. Miller holds an undergraduate degree in biology from the University of Delaware, as well as a master's of business administration from Villanova University.

Donna Cabral-Lilly, Ph.D., Head of Pharmaceutical Development

Dr. Cabral-Lilly was formerly Senior Director of Pharmaceutical Development at Control Delivery Systems (CDS), where she was responsible for chemistry, manufacturing and controls programs for several drug delivery products in clinical development. Prior to joining CDS, Dr. Cabral-Lilly was at Elan Pharmaceuticals where she directed the Analytical Research and Development Group. She has authored numerous scientific publications and is credited as inventor on several patents. Dr. Cabral-Lilly was an instructor at Boston University School of Medicine. She earned her Ph.D. from Boston University and was a post-doctoral fellow at Brandeis University.

Lisa DeLuca, Ph.D., Vice President, Regulatory Affairs

Dr. DeLuca joins Celator having recently worked at Sanofi-Topaz, Inc. (formerly Topaz Pharmaceuticals). As vice president, regulatory affairs and quality assurance, she was a key member of the management team responsible for the development of the company's lead drug product (0.5% ivermectin lotion) through clinical development, manufacturing optimization, NDA submission, launch, commercialization, and acquisition of the company by Sanofi Pasteur Inc. She led the successful NDA submission process achieving FDA approval within a single review cycle. Prior to that, she served as vice president, global regulatory affairs, for YM BioSciences (formerly Eximias Pharmaceuticals), leading registration activities for oncology and pain therapeutics, and as director, regulatory affairs, for AstraZeneca, where she led the IRESSA™ regulatory team and developed the regulatory strategy for the prostate cancer and pain portfolios. Dr. DeLuca was part of a company formed by Centocor called Apollon Inc., where she was responsible for the regulatory strategy for gene therapies for several types of cancer, infectious diseases, and allergies. She began her career in regulatory affairs at Centocor contributing to the company's first successful NDA filing for ReoPro® and the initial IND filing for Remicade®. She also held research positions at the Upjohn Company and SmithKline Beecham. Dr. DeLuca holds undergraduate and master's degrees in biology from Eastern Michigan University and earned her Ph.D. in biology from the University of Toledo.

Les Mintzmyer, Vice President, Manufacturing and Technical Operations

Les joins Celator from Laureate Biopharmaceutical Services, where he was vice-president of manufacturing operations. He and his team were responsible for delivering a broad spectrum of service offerings for contract clients, ranging from early development through commercial products. Prior to joining Laureate, he held senior positions at Avid Bioservices (Peregrine Pharmaceuticals), ImClone Corporation, Genentech, Inc., and Bristol-Myers Squibb Corporation. Les holds an undergraduate degree in biology/chemistry from the University of Nebraska at Kearney, as well as undergraduate and master's degrees in chemical engineering from Colorado State University.

VALUATION AND RECOMMENDATION

We are initiating coverage of Celator with an **Outperform** rating. Our 12-month price target is $7.00.

Celator is a late stage specialty pharmaceutical company with a focus on cancer. The company has built a decent pipeline using its unique, proprietary **CombiPlex** platform technology and **liposome/nanoparticle** delivery system.

Celator's lead clinical program **CPX-351** is in a **Phase III** clinical trial and top line data will be available in 2Q15. CPX-351 targets **AML** patients. Completed Phase II studies demonstrated that CPX-351 was safe and well tolerated in AML patients and that the drug candidate achieved significant efficacy improvements over control in high-risk AML patient populations such as secondary AML and poor-risk first relapse patients. Based on the positive Phase II data, we believe CPX-351 has a high success rate in the ongoing Phase III study. We expect CPX-351 to be approved by the FDA in late 2017 and by the EMA in 2018.

Celator's second program is **CPX-1** for **CRC.** CPX-1 has completed a **Phase II** clinical trial for CRC. Other than CPX-351 and CPX-1, Celator has two preclinical programs: CPX-571 and CPX-8 for solid tumors.

In terms of valuation, we think Celator's shares are undervalued at current market price. Currently Celator shares are trading at about $2.5 per share, which represents a market cap of $64 million based on 26.1 million outstanding shares. This undervalues Celator based on its relatively strong fundamentals. According to our model, we expect CPX-351 to be approved in 2017 by the FDA and in 2018 by the EMA. We model Celator will become profitable (EPS of $0.03) in 2018 based on CPX-351 sales of $35 million. Sales of CPX-351 will accelerate in 2019 after the company gains marketing experience and further market penetration. If we use a P/E multiple of 35x, coupled with EPS of $0.62 in 2019, and discounted at 25% for 5 years, we come up with our price target of $7.00, which represents a market cap of $182 million.

But keep in mind the **risks**. Since Celator is still a clinical stage company, there are still **clinical** and **regulatory** hurdles for the company to overcome. Even when CPX-351 is approved, there are still **commercial risks** since CPX-351 will be the first commercial product for the company. In addition, general market condition will also have significant impact on the company's share price down the road. However, overall, we believe Celator is a name for investors with a long term investment horizon and high risk tolerance.

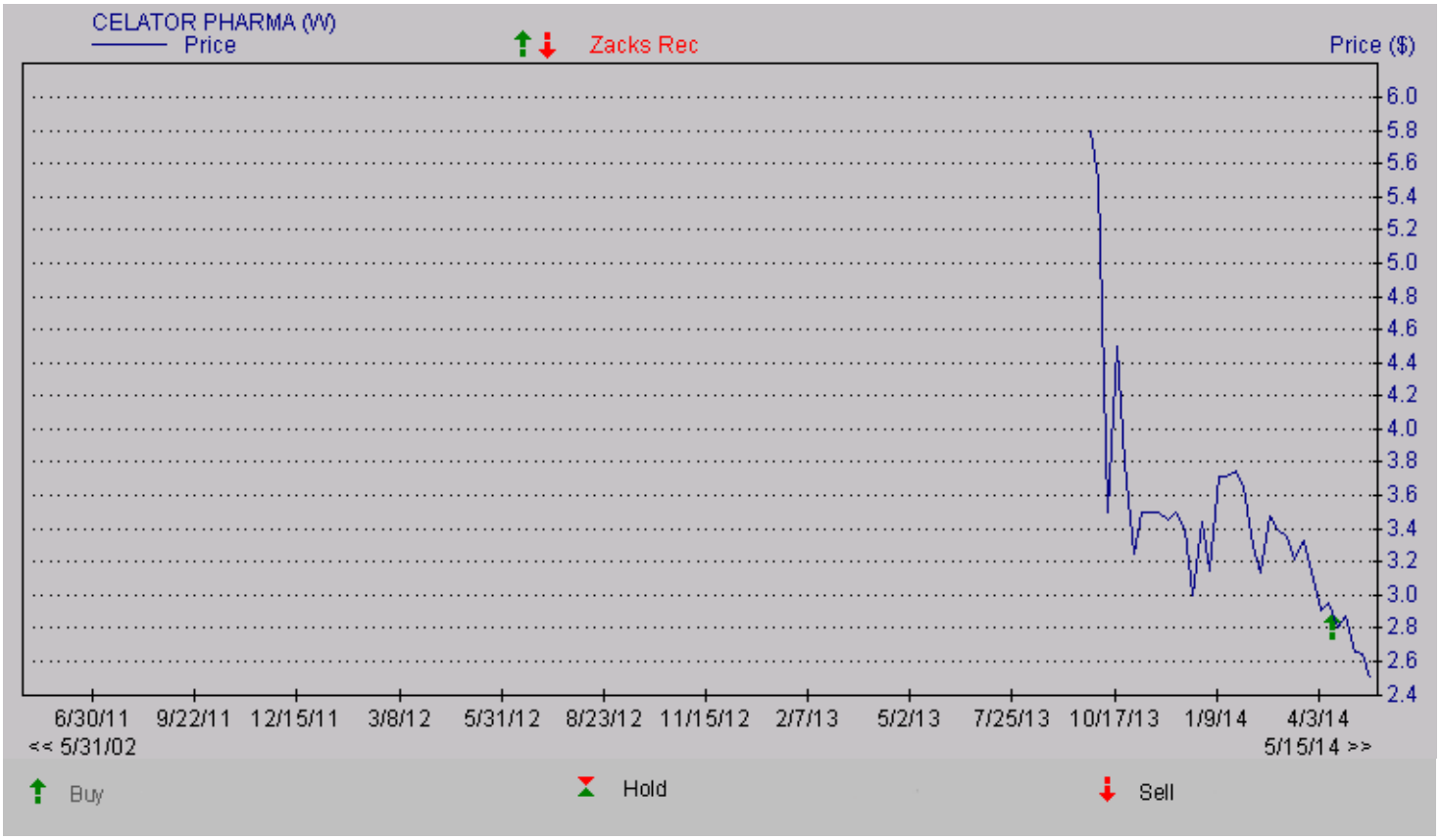
PROJECTED INCOME STATEMENT

	2012 (Dec)	2013A (Dec)	2014E (Dec)					2015E (Dec)	2016E (Dec)	2017E (Dec)	2018E (Dec)	2019E (Dec)
$ in million except per share data	FYA	FYA	Q1A	Q2E	Q3E	Q4E	FYE	FYE	FYE	FYE	FYE	FYE
Product revenue	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$3.50	$35.00	$75.00
Other revenue	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00	$0.00
Total Revenues	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$3.50**	**$35.00**	**$75.00**
YOY Growth	-	-	-	-	-	-	-	-	-	-	900.0%	114.3%
CoGS	0.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00	0.53	5.25	11.25
Gross Income	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$2.98**	**$29.75**	**$63.75**
Gross Margin	-	-	-	-	-	-	-	-	-	85.0%	85.0%	85.0%
R&D	$3.41	$8.86	$2.33	$3.20	$3.50	$4.25	$13.28	$7.50	$10.00	$13.50	$15.00	$17.50
% R&D	-	-	-	-	-	-	-	-	-	385.7%	42.9%	23.3%
SG&A	$4.10	$5.43	$1.89	$1.50	$1.55	$1.60	$6.54	$7.00	$8.50	$10.50	$12.50	$15.00
%SG&A	-	-	-	-	-	-	-	-	-	-	-	-
Other	$0.49	$0.32	$0.05	$0.05	$0.05	$0.05	$0.20	$0.20	$0.50	$0.50	$0.50	$0.00
Operating Income	**($8.0)**	**($14.6)**	**($4.3)**	**($4.8)**	**($5.1)**	**($5.9)**	**($20.0)**	**($14.7)**	**($19.0)**	**($21.5)**	**$1.8**	**$31.3**
Operating Margin	-	-	-	-	-	-	-	-	-	-	-	41.67%
Other Net	($4.3)	($7.6)	($0.0)	($0.2)	($0.2)	($0.2)	($0.6)	($0.8)	($0.8)	($0.5)	($0.1)	($0.1)
Pre-Tax Income	**($12.3)**	**($22.2)**	**($4.3)**	**($5.0)**	**($5.3)**	**($6.1)**	**($20.6)**	**($15.5)**	**($19.8)**	**($22.0)**	**$1.7**	**$31.2**
Income taxes(benefit)	($1.4)	($1.4)	$0.0	$0.0	$0.0	($1.1)	($1.1)	($1.0)	($1.0)	$0.0	$0.0	$0.0
Tax Rate	-	-	-	-	-	-	-	-	-	-	-	-
Reported Net Income	**($10.9)**	**($20.8)**	**($4.3)**	**($5.0)**	**($5.3)**	**($5.0)**	**($19.5)**	**($14.5)**	**($18.8)**	**($22.0)**	**$1.7**	**$31.2**
YOY Growth	-	-	-	-	-	-	-	-	-	-	-	-
Net Margin	-	-	-	-	-	-	-	-	-			
Diluted Shares Out	12.4	22.0	26.0	26.8	27.0	27.2	26.8	37.0	38.5	45.0	48.5	50.0
Reported EPS	**($0.88)**	**($0.95)**	**($0.16)**	**($0.18)**	**($0.20)**	**($0.18)**	**($0.73)**	**($0.39)**	**($0.49)**	**($0.49)**	**$0.03**	**$0.62**
One time charge	*$0.00*	*$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	**($10.9)**	**($20.8)**	**($4.3)**	**($5.0)**	**($5.3)**	**($5.0)**	**($19.5)**	**($14.5)**	**($18.8)**	**($22.0)**	**$1.7**	**$31.2**
Non GAAP EPS	**($0.88)**	**($0.95)**	**($0.16)**	**($0.18)**	**($0.20)**	**($0.18)**	**($0.73)**	**($0.39)**	**($0.49)**	**($0.49)**	**$0.03**	**$0.62**

Source: company filings and Zacks estimates

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