Zacks Small-Cap Research

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Celator Pharma (CPXX-NASDAQ)

CPXX: Zacks Company Report

Positive OS data reported for CPX-351, preparing to be a commercial stage organization

Current Price (05/31/16)	$30.08
Valuation	**$30.25**

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. The company just announced positive OS data from the Phase III trial and we expect its approval in the US in mid-2017.

Jazz just agreed to acquire CPXX at $30.25/share, which we think is fair and at a decent premium.

SUMMARY DATA

52-Week High	**$30.08**
52-Week Low	**$1.64**
One-Year Return (%)	**-19.52**
Beta	**-0.93**
Average Daily Volume (sh)	**215,652**
Shares Outstanding (mil)	**42.5**
Market Capitalization ($mil)	**$1280**
Short Interest Ratio (days)	**2.40**
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)
2014	0.00 A	0.00 A	0.00 A	0.00 A	0.00 A
2015	0.00 A	0.00 A	0.00 A	0.00 A	0.00 A
2016	0.00 A	0.00 E	0.00 E	0.00 E	0.00 E
2017					10.00 E

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

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2014	-$0.16 A	-$0.18 A	-$0.21 A	-$0.07 A	-$0.62 A
2015	-$0.14 A	-$0.15 A	-$0.15 A	-$0.13 A	-$0.57 A
2016	-$0.16 A	-$0.14 E	-$0.14 E	-$0.13 E	-$0.56 E
2017					-$0.41 E

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

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WHAT'S NEW

Jazz Acquires Celator for $30.25 per Share

On May 31, 2016, Jazz Pharmaceuticals plc (**JAZZ**) and Celator Pharmaceuticals (CPXX) announced that they have entered into a definitive agreement for Jazz Pharmaceuticals to acquire Celator for $30.25 per share in cash, or approximately **$1.5 billion**.

We think this transaction is well suited to advance the commercialization of VYXEOS and fit into both JAZZ AND CPXX's growth strategy.

- VYXEOS is the first product candidate to demonstrate a statistically significant improvement in Overall Survival in patients with high-risk (secondary) AML.
- U.S. FDA Breakthrough Therapy designation granted for VYXEOS.
- U.S. FDA and European Commission Orphan Drug designation for VYXEOS for the treatment of AML
- VYXEOS is an innovative product candidate based on the Celator CombiPlex® platform
- Anticipated long-lived exclusivity for VYXEOS
- Broadens Jazz Pharmaceuticals' hematology/oncology portfolio
- Worldwide development and commercialization rights to VYXEOS
- Synergies with Jazz Pharmaceuticals' commercial expertise and infrastructure

The transaction is structured as a tender offer and second step merger. The transaction is expected to close in the third quarter of 2016.

Jazz Pharmaceuticals expects to finance the transaction with a combination of cash on hand and borrowings under its senior secured credit facility.

We encourage shareholders of CPXX to tender your shares as we see the offer provides fair premium for CPXX shares.

Celator anticipates achieving the following key objectives:

- Continue to execute on the VYXEOS commercial readiness in the United States;
- Submit the New Drug Application (NDA) for VYXEOS by the end of Q3 2016;
- Continue to expand the clinical development of VYXEOS via investigator-initiated studies, oncology cooperative groups and company-sponsored studies;
- Prescription Drug User Fee Act (PDUFA) date (assuming FDA grants priority review) by mid-2017; and
- United States commercial launch (assuming FDA approval in mid-2017) in Q3 2017.

In addition to VYXEOS, Celator has built a decent pipeline using its unique, proprietary **CombiPlex** platform technology and **liposome/nanoparticle** delivery system. With the availability of top line data from its lead candidate CPX-531, the company is one step closer to the commercialization stage.

We think the positive overall survival data from the company's lead Phase III clinical program **CPX-351** serves as a **significant de-risk event** for Celator. We expect CPX-351 to be approved by the FDA in mid-2017 and by the EMA in 2018.

In terms of valuation, we think Jazz Pharma's offer provide a fair valuation plus a decent premium.

According to our model, we expect CPX-351 to be approved in mid-2017 by the FDA and in 2018 by the EMA. We model Celator will become profitable (EPS of $0.05) in 2018 based on CPX-351 sales of $40 million. Sales of CPX-351 will accelerate in 2019 after the company gains marketing experience and further market penetration. We think a P/E multiple of 38x is appropriate for CPXX at this point of time.

Coupled with EPS of $1.05 in 2020, and discounted at 20% for 4 years, we come up with fair value of VYXEOS of $19.00. Adding the platform technology, the $30.25/share offer from Jazz is fair in our view.

Celator is ready to Commercialize VYXEOS™

On March 14, 2016, Celator (CPXX) announced **positive results** from the **Phase III** trial of VYXEOS™ (cytarabine:daunorubicin) Liposome for Injection (CPX-351) in patients with high-risk (secondary) acute myeloid leukemia (AML) compared to the standard of care regimen of cytarabine and daunorubicin.

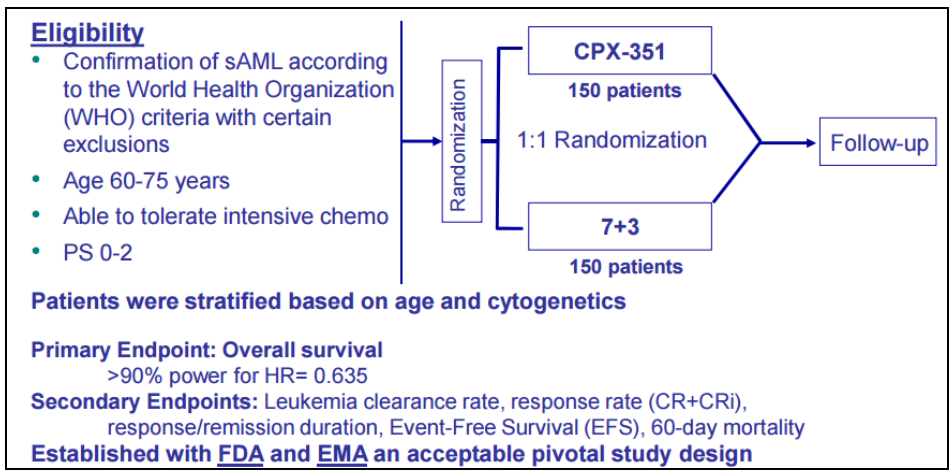
Background of the Phase III Trial

The randomized, controlled, Phase III study (NCT01696084), conducted at 39 centers in the United States and Canada, compared VYXEOS to the conventional cytarabine and daunorubicin treatment regimen (commonly referred to as 7+3) as first-line therapy.

The study enrolled 309 patients between the ages of 60 and 75 who had pathologically confirmed diagnosis of high-risk AML by WHO criteria including: therapy-related AML, AML with a history of myelodysplasia (MDS), AML with a history of chronic myelomonocytic leukemia (CMMoL), and de novo AML with karyotypic abnormalities characteristic of MDS.

Patients were randomized 1:1 to receive either VYXEOS (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/m2 on days 1, 2, and 3).

The study was conducted in partnership with The Leukemia & Lymphoma Society® (LLS) through its Therapy Acceleration Program (TAP), which has supported the clinical development of VYXEOS beginning in Phase II.



The Positive Data

The trial met its primary endpoint demonstrating a statistically significant improvement in overall survival.

- The median overall survival for patients treated with VYXEOS was 9.56 months compared to 5.95 months for patients receiving 7+3, representing a 3.61 month improvement in favor of VYXEOS.
- The hazard ratio (HR) was 0.69 (p=0.005) which represents a 31 percent reduction in the risk of death versus 7+3.
- The percentage of patients alive 12 months after randomization was 41.5% on the VYXEOS arm compared to 27.6% on the 7+3 arm.

- The percentage of patients alive 24 months after randomization was 31.1% on the VYXEOS arm compared to 12.3% on the 7+3 arm.
- VYXEOS also demonstrated a statistically significant improvement in **induction response rate** (CR+CRi of 47.7% versus 33.3%; p=0.016) and this significance was maintained for the analysis of CR alone (CR of 37.3% versus 25.6%, p=0.040).
- Sixty-day all-cause mortality was 13.7% versus 21.2%, in favor of patients treated with VYXEOS.

No substantial difference in Grade 3 or higher adverse events was observed between VYXEOS and 7+3. In the intent-to-treat population, Grade 3 or higher, hematologic adverse events were similar for overall infections, febrile neutropenia, and bleeding events. In the intent-to-treat population, Grade 3 or higher, non-hematologic adverse events were similar across all organ systems, including cardiac, gastrointestinal, general systems, metabolic disorders, musculoskeletal, nervous system, respiratory, skin and renal.

Data will be submitted for presentation at the American Society of Clinical Oncology (ASCO) 2016 Annual Meeting.

Based on these results the company expects to submit a New Drug Application (NDA) for VYXEOS with the US FDA at the end of **3Q16** and submit a Marketing Authorization Application (MAA) with the European Medicines Agency (EMA) in **1Q17**.

VYXEOS™ Shows No QT/QTc Interval Prolongation in an Intensive QT/QTc Study

On Dec. 8, 2015, Celator announced positive data from a **Phase II PK/PD** study of VYXEOS™ (CPX-351) at the ASH meeting.

This open-label, single arm, **Phase II PK/PD** study measured the effects of VYXEOS on cardiac repolarization during the first induction cycle and correlated electrophysiologic changes, as measured by the QT/QTc interval, with a detailed pharmacokinetic assessment of cytarabine and daunorubicin and their metabolites using exposure response modeling methodology.

This study, in adult patients with acute hematologic malignancies, was conducted to support the NDA filing in the US.

The study completed enrollment in June 2015. Each patient received a first induction of VYXEOS on days 1, 3 and 5 and, if necessary, a second induction for patients with reduced leukemia burden not yet achieving a disease-free state. Responding patients were eligible for up to four consolidation courses.

Twenty-six patients with acute myeloid leukemia (AML) or acute lymphoblastic leukemia (ALL) were enrolled and 21 were evaluable for QT interval change from baseline. VYXEOS **did not prolong the QT/QTc interval**, a positive **safety outcome**. The largest absolute and mean change from time-matched baseline was 12.56 and 8.03 ms, respectively (Day 1, hour 4). Mean QTcF change on Day 5 was unchanged from baseline. QTcF intervals >480 ms were never observed and no consistent QTcF intervals >450 ms were noted. Absolute QTcF increases between 30 and 60 ms were present in 4 of 25 patients and no changes >60 ms were observed. CPX-351 plasma concentration vs. time curves for day 1 and 5 exhibited a volume of distribution approximately equal to the plasma volume with prolonged single-exponential elimination half-lives for cytarabine and daunorubicin of ≥24 hours.

The study included patients with newly diagnosed AML, relapsed/refractory AML and relapsed/refractory ALL. Thirteen of the 26 patients responded. Responses were seen in each of the three acute leukemia patient populations. The high response rates corroborate the high level of efficacy observed in earlier studies with CPX-351.

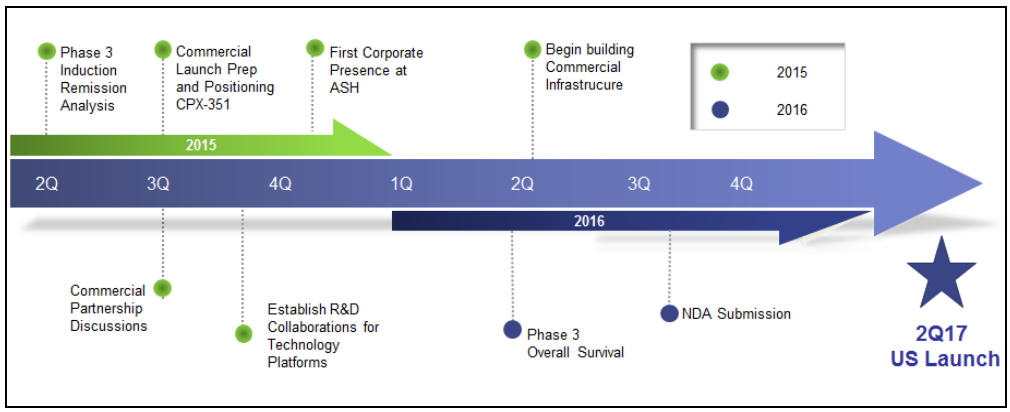
	Responders / # of Patients	Response Rate
Newly diagnosed AML	8 / 13	61.5%
Relapsed/Refractory AML	4 / 11	36.4%
Relapsed/Refractory ALL	1 / 2	50.0%

In addition, CPX-351 exposure was independent of renal function, indicating no need for dose adjustment when renal function is impaired.

Adverse events were similar in frequency and severity to those described in earlier studies.

Paving the Way to VYXEOS Commercialization

Based on the positive Phase III data and the PKPD data, we estimate CPXX will submit a NDA in the 3Q16 and that CPX-351 will be approved in mid-2017 by the FDA. Therefore, VYXEOS could be launched in the beginning of 3Q17.



The FDA granted **Fast Track designation** for CPX-351for the treatment of elderly patients with secondary AML. If approved, CPX-351 may ultimately replace 7+3 on the basis of improved efficacy and acceptable safety.

CPXX also plans a MAA submission in 1Q17 and we expect the approval of CPX-351 by the EMA in 1H18.

Celator Announces Positive Results from Phase II Study with VYXEOSTM

On April 25, 2016, CPXX announced that patients treated in a **Phase II** clinical study with VYXEOS™ Liposome for Injection with **newly diagnosed AML** demonstrated reduced healthcare resource use compared to conventional chemotherapy known as 7+3. These results were presented at the AMCP Managed Care & Specialty Pharmacy Annual Meeting in San Francisco, CA, April 19-22, 2016.

Key findings from the Phase II study include:

VYXEOS treated patients had;

- a longer EFS (event free survival), with a median of 197 days compared to 60 days with 7+3,

- a slightly longer time in the hospital, median of 38 days compared to 35 days. The majority of patients on the VYEXOS arm with hospital stays > 60 days also experienced a response (75%) with many having one or more risk factors,
- a higher ratio of median EFS days to median hospital days, with a ratio of 5.2 compared to 1.7, which means the time spent out of the hospital was longer for the patients treated with VYXEOS,
- fewer hospital admissions, median of 1 admission with VYXEOS compared to 2 admissions with 7+3,
- a higher rate of remission after one induction cycle (42/56 vs. 15/21; p=0.03), and
- received consolidation treatment more frequently as outpatients (15/37 vs. 2/15; p=0.02).

High risk, including secondary, AML patients treated with VYXEOS also had longer EFS and a favorable ratio of EFS days to hospital days.

This is the first time the company evaluated the impact of VYXEOS on hospital resource use in relation to its established clinical benefit. Although, the study saw longer hospital stays in VYXEOS treated patients, some of those costs are clearly offset by fewer hospital admissions and outpatient consolidation treatments in this group. This study suggests significant value that VYXEOS could provide for patients, providers and payers over the course of the disease.

Update on CombiPlex® Platform R&D

In late September 2015, Celator announced their successful R&D efforts to apply the **CombiPlex® technology** platform to optimize the efficacy of anticancer drug combinations incorporating molecularly targeted agents (MTAs).

CombiPlex is Celator's proprietary technology that aims to address several of the fundamental shortcomings of conventional combination regimens, as well as the inherent challenges in combination drug development. Celator is applying this technology to create new drug combinations that target pathways associated with tumor cell growth and/or resistance to treatment. Such MTAs are being widely pursued with an increasing focus on combining agents that target multiple cellular pathways in order to improve therapeutic responses.

Celator's efforts have focused on two combinations: the heat shock protein 90 inhibitor **AUY922** combined with **docetaxel** and the MEK inhibitor selumetinib combined with the Akt inhibitor ipatasertib.

In both cases, the drug combinations were stably co-formulated in Celator's proprietary hydrophobic prodrug nanoparticle delivery systems which provided well-coordinated plasma concentrations over 24 hours that were orders of magnitude higher than observed for the free drug combinations. For both combinations, the CombiPlex formulations provided significant improvements in efficacy over the free drugs in **human xenograft tumor models** including breast, colorectal and ovarian. Furthermore, there was evidence of strong drug ratio-dependent efficacy and in vivo synergy.

The largest improvements were observed in tumor models known to be more resistant to conventional dosing forms of the two combinations. Optimal efficacy was achieved at drug ratios of 1:2 and 2:1, respectively. In the case of docetaxel:AUY922, the conventional formulation provided negligible tumor growth delay at the maximum tolerated dose (MTD) in the OVCAR-8 human ovarian xenograft model with tumors reaching 1 gram in size in approximately 25 days. In contrast, dosing with Celator's proprietary formulation at MTD, tumors grew to only half that size after 50 days demonstrating potent tumor growth inhibition.

Another benefit observed during this research was the versatility and modular nature of the nanoparticle technology. Once formulation conditions were optimized for the two initial combinations, the drug components could be "mixed and matched," rapidly generating additional CombiPlex products with coordinated PK. The approach was readily extended to the B-Raf inhibitor GDC0879 and the FGFR

inhibitor LY2874455 and both were successfully co-formulated with the MEK inhibitor selumetinib resulting in coordinated drug exposure in the plasma.

This versatility was then taken one step further by generating a 3-drug combination, co-formulating selumetinib, AUY922 and docetaxel into a single hydrophobic prodrug nanoparticle (HPN) that exhibited a plasma half-life in the range of 10 hours in mice with no early distribution phase.

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 were diagnosed, most were in adults. About 10,460 AML patients died from AML, almost all were 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.

Key advantages of CPX-531 include:

- Prolonged overall survival
- Significant reduction in 60-day early mortality
- Higher remission rates could result in more transplant candidates
- More convenient and less resource-intensive dosing schedule
    - 3 x 90 minute infusions vs 7-day continuous infusion coupled with an IV push on days 1-3 during induction
    - Fixed, co-formulation of 2 drugs eliminates need to mix or identify optimal ratio
- Alopecia rarely observed
- Potential for outpatient treatment in consolidation phase

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 peak sales of CPX-351 will be in the range of $300 to $500 million for the first indication globally. The overall global market opportunity for all indications will well be over $1 billion.

CPXX Expands Clinical Development in AML and other Hematologic Malignancies

The company's clinical development strategy is designed to expand CPX-351 into important areas of unmet need in AML and other hematologic malignancies.

In June 2015, CPXX announced the expansion of an investigator-initiated clinical study of **CPX-351** in patients with untreated high-risk myelodysplastic syndrome **(MDS)** or acute myeloid leukemia **(AML),** at high risk of treatment-related mortality.

The expansion of the 32units/m^2 dose cohort is based on the encouraging efficacy and safety results observed so far. In order to expand the 32units/m^2 cohort, the protocol requires 5 or more complete responses and fewer than five deaths by day 28 in the first 20 patients. In this trial, CPX-351 resulted in 6 responses in the first 20 patients with 2 early treatment-related deaths by day 28.

CPX-351 surpassed both criteria and the number of patients in the cohort was increased from 20 to 30.

This investigator initiated trial and expansion represents the company's ongoing effort to expand the CPX-351's utility into additional cancer indications in addition to sAML. Data so far have demonstrated the advantages of CPX-351 over standard chemotherapy regimens in terms of safety and efficacy.

And in July, Celator announced the enrollment of an investigator-initiated **Phase II** clinical study evaluating CPX-351 as a treatment for patients with **newly diagnosed Acute Myeloid Leukemia (AML)** at high risk for induction treatment mortality.

This Phase II study will enroll up to 55 patients. The primary objective is to assess the preliminary efficacy and safety of multiple dose levels of CPX-351 in patients with newly diagnosed AML at high risk for induction treatment mortality. High risk for induction treatment mortality is defined as 30%-50% predicted risk of expiring by day 60.


RESEARCH
PRECLINICAL
PHASE 1
PHASE 2
PHASE 3
APPROVAL
Registration Study
Randomized, Controlled Study
High Risk (e.g. secondary) Acute Myeloid Leukemia age 60 and ≤75 years (enrolled)
Planned Sponsored Studies
High-risk AML patients age <60 years (Planned)
Induction failure AML patients age ≥18 years (Planned)
Estimated Data
Investigator-initiated Studies
Pre-conditioning prior to Cord Blood HSCT (Ongoing)
2Q16
Unfit AML due to co-morbidities (Ongoing - Expanded)
2Q16
HR-MDS and AML after HMA therapy (Ongoing)
3Q16
Unfit AML due to poor risk (Ongoing)
1Q17
Pediatric Pilot (Ongoing)
Lead into COG
Elderly AML (≥65 years) (Planned)
2Q17
Cooperative Group Studies
Newly Diagnosed AML or high-risk MDS Aged 18-60 (Planned)
2Q21
Children with Relapsed AML (Planned)
4Q17

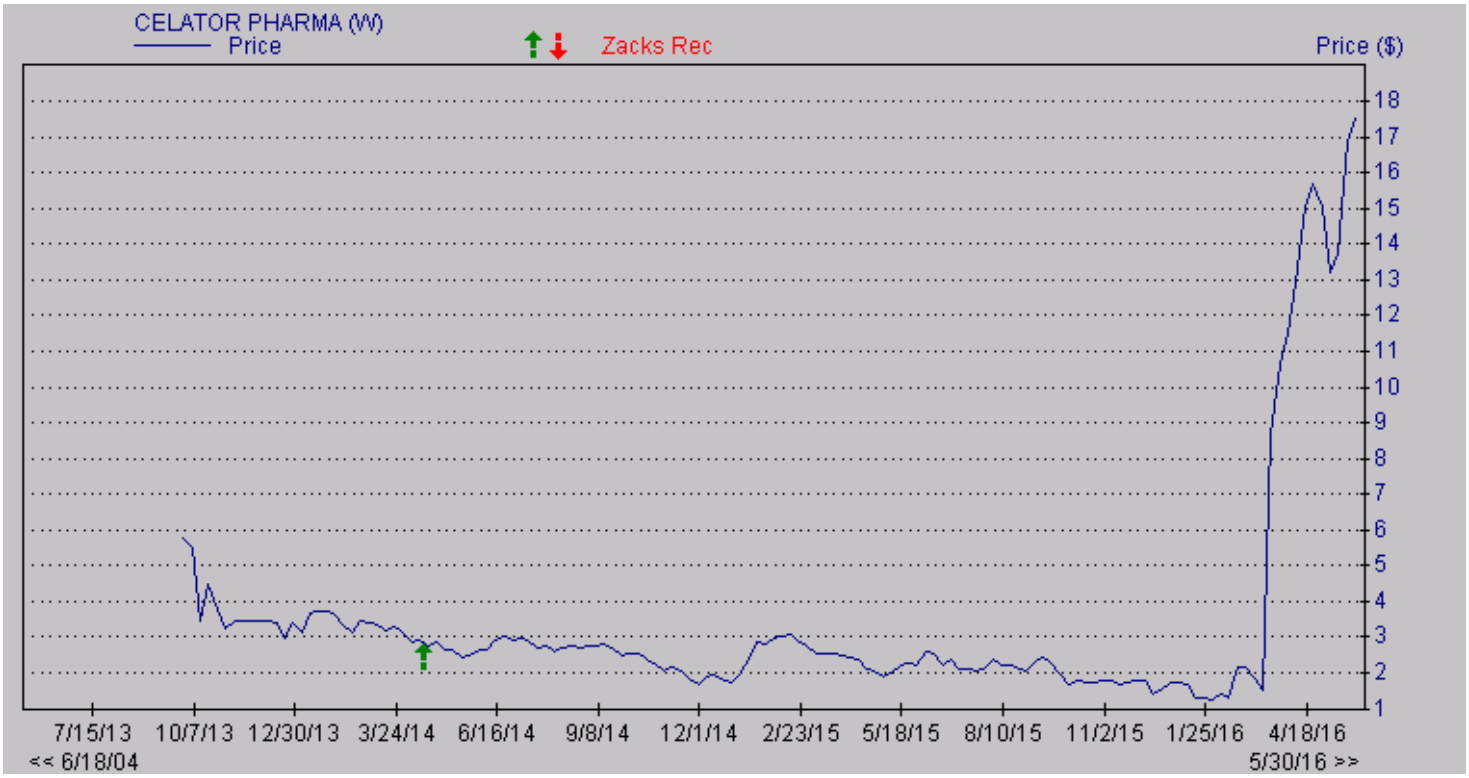
PROJECTED INCOME STATEMENT

	2015 (Dec)					2016E (Dec)					2017E (Dec)	2018E (Dec)	2019E (Dec)	2020E (Dec)
$ in million except per share data	Q1A	Q2A	Q3A	Q4A	FYA	Q1	Q2	Q3	Q4	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	$0.00	$10.00	$40.00	$75.00	$125.00
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	$0.00	$0.00
Total Revenues	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$10.00**	**$40.00**	**$75.00**	**$125.00**
YOY Growth	-	-	-	-	-	-	-	-	-	-	-	300.0%	87.5%	66.7%
CoGS	0.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00	1.50	6.00	11.25	18.75
Gross Income	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$0.00**	**$8.50**	**$34.00**	**$63.75**	**$106.25**
Gross Margin	-	-	-	-	-	-	-	-	-	-	85.0%	85.0%	85.0%	85.0%
R&D	$2.57	$2.42	$2.72	$2.62	$10.33	$2.54	$2.65	$2.70	$3.00	$10.89	$12.50	$15.00	$17.50	$19.50
% R&D	-	-	-	-	-	-	-	-	-	-	125.0%	37.5%	23.3%	15.6%
SG&A	$1.76	$2.01	$1.94	$1.95	$7.67	$2.47	$2.60	$2.80	$3.00	$10.87	$14.50	$16.00	$17.50	$18.50
%SG&A	-	-	-	-	-	-	-	-	-	-	145.0%	40.0%	23.3%	14.8%
Other	$0.05	$0.05	$0.05	$0.05	$0.20	$0.05	$0.05	$0.05	$0.05	$0.20	$0.50	$0.50	$0.00	$0.00
Operating Income	**($4.4)**	**($4.5)**	**($4.7)**	**($4.6)**	**($18.2)**	**($5.1)**	**($5.3)**	**($5.6)**	**($6.1)**	**($22.0)**	**($19.0)**	**$2.5**	**$28.8**	**$68.3**
Operating Margin	-	-	-	-	-	-	-	-	-	-	-	-	38.33%	54.60%
Other Net	($0.3)	($0.5)	($0.5)	($0.5)	($1.8)	($0.5)	($0.5)	($0.5)	($0.5)	($1.9)	($0.5)	($0.1)	($0.1)	($0.1)
Pre-Tax Income	**($4.7)**	**($5.0)**	**($5.2)**	**($5.1)**	**($20.0)**	**($5.5)**	**($5.8)**	**($6.0)**	**($6.5)**	**($23.9)**	**($19.5)**	**$2.4**	**$28.7**	**$68.2**
Income taxes(benefit)	$0.0	$0.0	$0.0	($0.7)	($0.7)	$0.0	$0.0	$0.0	($1.0)	($1.0)	$0.0	$0.0	$0.0	$5.0
Tax Rate	-	-	-	-	-	-	-	-	-	-	-	-	-	7.3%
Reported Net Income	**($4.7)**	**($5.0)**	**($5.2)**	**($4.4)**	**($19.3)**	**($5.5)**	**($5.8)**	**($6.0)**	**($5.5)**	**($22.9)**	**($19.5)**	**$2.4**	**$28.7**	**$63.2**
YOY Growth	-	-	-	-	-	-	-	-	-	-	-	-	-	120.3%
Net Margin	-	-	-	-	-	-	-	-	-	-	-	-	38.3%	50.6%
Diluted Shares Out	33.7	33.8	33.8	34.5	33.9	35.4	41.2	42.0	44.0	40.7	48.0	52.0	55.0	60.0
Reported EPS	**($0.14)**	**($0.15)**	**($0.15)**	**($0.13)**	**($0.57)**	**($0.16)**	**($0.14)**	**($0.14)**	**($0.13)**	**($0.56)**	**($0.41)**	**$0.05**	**$0.52**	**$1.05**
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*	*$0.00*	*$0.00*
Non GAAP Net Income	**($4.7)**	**($5.0)**	**($5.2)**	**($4.4)**	**($19.3)**	**($5.5)**	**($5.8)**	**($6.0)**	**($5.5)**	**($22.9)**	**($19.5)**	**$2.4**	**$28.7**	**$63.2**
Non GAAP EPS	**($0.14)**	**($0.15)**	**($0.15)**	**($0.13)**	**($0.57)**	**($0.16)**	**($0.14)**	**($0.14)**	**($0.13)**	**($0.56)**	**($0.41)**	**$0.05**	**$0.52**	**$1.05**

Source: company filings and Zacks estimates

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