Celladon Corp. (CLDN)

Revising our Risk Assessment Ahead of CUPID 2, Reiterate OP Rating and Raising PT to $29 (from $17)

- **With the final data monitoring committee (DMC) meeting prior to CUPID2 unblinding out of the way (and the trial proceeding), and with MYDICAR manufacturing scaled up and ready to go, the risk/reward equation for CLDN investors focuses almost purely on CUPID2's outcome.**
- **The unblinding of the Ph 2b CUPID2 trial remains set for April, and we take a closer look at the possible outcomes from the study in order to fine-tune our risk/reward assessment.** We see an asymmetric risk/reward equation, but note that it is a binary event. CUPID2 is a double-blind study evaluating a single intracoronary infusion of high-dose Mydicar (1x1013 DNase resistant particles) vs placebo in 250 patients with advanced systolic heart failure (HF)who are receiving concurrent maximal optimized therapy. The study database will close after all subjects have completed 12 months on study and at least 186 adjudicated HF-related hospitalizations have occurred. Our updated valuation is based on an assessment of the various outcomes from CUPID2 and the likelihood of its occurrence, and the impact each scenario has on 2020 sales (our valuation year).
- **Our base case: Mydicar demonstrates a statistically significant benefit vs placebo in the primary endpoint (60% likelihood, given prior positive data).**
- **The bear case: Mydicar fails in CUPID 2 (30% likelihood).**
- **The bull case: the clinical benefit with Mydicar is so dramatic that it could support accelerated approval in the US (10% likelihood).**
- **Reiterate OUTPERFORM and raising our PT to $29 (from $17).** Our price target is derived from a probability-weighted analysis of the outcomes of the CUPID2 trial (see next page).

January 22, 2015

Price
$17.26

Rating
OUTPERFORM

12-Month Price Target
$29 **(from $17)**

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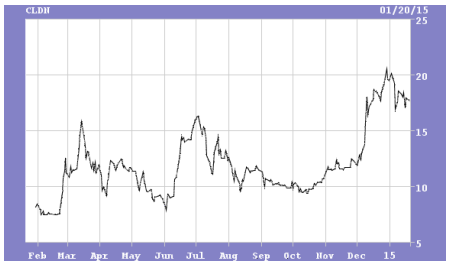
Company Information	
Shares Outst (M)	23.3
Market Cap (M)	$402.5
52-Wk Range	$7.45 - $20.85
Book Value/sh	$3.52
Cash/sh	$3.70
Enterprise Value (M)	$326.3
LT Debt/Cap %	11%
Cash Burn (M)	$34.2

Company Description

Celladon Corp. is based in San Diego, Ca. and is focused on the development of MYDICAR, a gene therapy product for increasing SERCA2a expression, currently in the Phase IIb CUPID 2 trial in advanced systolic heart failure.

FYE Dec	2013A	2014E			2015E		
REV	ACTUAL	CURR.	PREV.	CONS.	CURR.	PREV.	CONS.
Q1 Mar	--	0.0A		$0.0A	0.0E		$0.0E
Q2 Jun	0.0A	0.0A		0.0A	0.0E		0.0E
Q3 Sep	0.0A	0.0A		0.0A	0.0E		0.0E
Q4 Dec	0.0A	0.0E		0.0E	0.0E		0.0E
Year*	**0.0A**	**0.0E**		**$0.0E**	**0.0E**		**$0.0E**
Change	--	--			--		
	2013A	**2014E**			**2015E**		
EPS	**ACTUAL**	**CURR.**	**PREV.**	**CONS.**	**CURR.**	**PREV.**	**CONS.**
Q1 Mar	--	($0.60)A		($0.60)A	($0.34)E	($0.38)E	($0.38)E
Q2 Jun	--	($0.38)A		($0.38)A	($0.35)E	($0.32)E	($0.32)E
Q3 Sep	($0.45)A	($0.40)A		($0.40)A	($0.30)E		($0.30)E
Q4 Dec	($0.51)A	($0.33)E	($0.37)E	($0.40)E	($0.27)E	($0.28)E	($0.30)E
Year*	**($1.67)A**	**($1.71)E**	**($1.75)E**	**($1.73)E**	**($1.26)E**	**($1.27)E**	**($1.34)E**
P/E	--	--			--		
Change	--	-2%			26%		

Consensus estimates are from Thomson First Call.
** Numbers may not add up due to rounding.*



Source: Thomson Reuters

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Risks to the achievement of our price target include clinical failure, failure to achieve regulatory approval in Western markets and failure to achieve sales and earnings estimates.

Valuation

Scenario	Approval yr (EU/US)	2020 Sales in Incident Pop.	Multiple	Discount Rate (post-CUPID2)	PV of Sales in Prevalent Pop.	Price Target	Probability
Base	2018/2019	$240M	6	25%	$716M	$35	60%
Bear						$3	30%
Bull	2018/2018	$298M	6	15%	$1,420M	$68	10%
					Adjusted Price Target	**$29**	

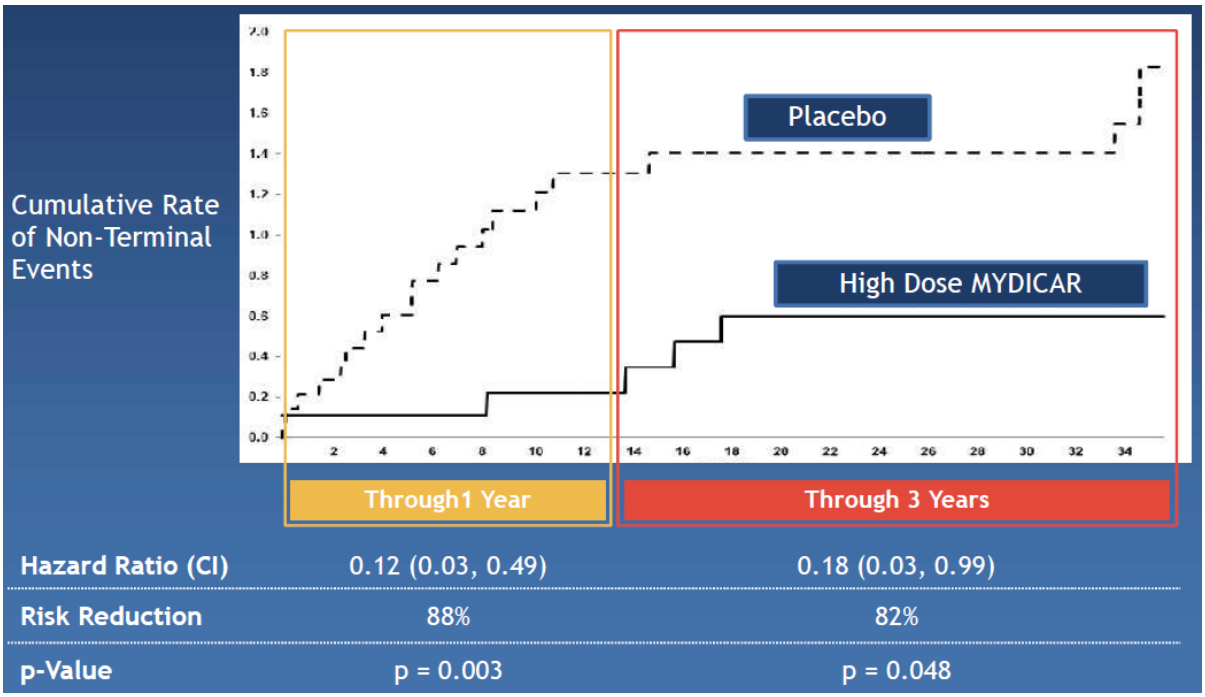
Note: our base and bull price targets are based on a 6x multiple of 2020 sales in the systolic HF incident population, plus the present value of future sales in the prevalent population, discounted back annually by 25% (base case) or 15% (bull case). Our bear price target is based on the estimated net cash per share in Q2:15.

Our base case: Mydicar demonstrates a statistically significant benefit vs placebo in the primary endpoint (60% likelihood, given prior positive data). The primary endpoint of CUPID2 is time-to-multiple HF-related hospitalizations in the presence of terminal events (defined as all-cause death, heart transplant or an LVAD implantation). We expect Mydicar to meet the primary endpoint, based on the significant and durable reduction in recurrent hospitalization (p=0.003, HR=0.12 at 1yr) observed with high-dose Mydicar (n=9) in CUPID1 (Fig. 1). Recall also that only 2/9 (23%) patients in the high-dose Mydicar group experienced a clinical event in the 12 months post-administration, compared to 7/14 (50%) in the placebo group (Fig. 2). We note that one of the poor responders from each group was found to have neutralizing AAV1 antibodies at the time of dosing (Mydicar uses a recombinant AAV1 vector to deliver the Serca2a gene). Although the magnitude of clinical benefit with Mydicar vs placebo is likely to decrease given the much larger enrollment of the CUPID2 study (n=250), we expect Mydicar to achieve at least a 45% risk reduction (HR=0.55) in recurrent events. The endpoint in CUPID2 was identified by the FDA as acceptable for a registration-directed Ph 3 trial, and CLDN has received an SPA for a 572 patient Ph 3 study. The EMA has also indicated that if a substantial treatment effect is observed in CUPID2 (with a continued acceptable safety profile), a safety database of about 205-230 patients could be sufficient to support an MAA submission, which would negate the need for a Ph 3 study for EU approval. In our base case we assume a positive readout from CUPID2 will lead CLDN to initiate a Ph 3 in 2016, which could support a 2018 BLA filing (leading to 2019 approval in the US). Based on the possible acceptance of the CUPID2 study by the EMA (along with supplemental safety data from other studies of Mydicar), we forecast EU approval a year earlier.

The bear case: Mydicar fails in CUPID 2 (30% likelihood). The major investor concern with CLDN has been whether the benefit observed with Mydicar in CUPID1 would be reproducible in a larger patient population. Recall that there were just nine patients enrolled in the high-dose Mydicar group in CUPID1, and 125 patients enrolled in the Mydicar arm in CUPID2. There is a possibility, of course, that the significant clinical benefit observed in CUPID1 was due to chance, and that a larger study would not duplicate the results seen in the earlier study. We acknowledge that a large number of pharmacological HF therapies considered promising in early stages have failed in late-stage development, due largely to patient variability in terms of coincident diseases, concurrent therapies or predisposing factors. We believe this variability can be mitigated with proper study design, and note that the extensive inclusion/exclusion criteria in the CUPID studies have resulted in patient populations with similar baseline characteristics (Fig. 3). If a statistically significant benefit is not observed with Mydicar in CUPID2, we would expect CLDN to conduct a study analysis that would point to an optimal development plan forward. This could entail another large Ph 2 study (perhaps in a more restricted study group if a subset analysis of CUPID2 supports it), or the possible abandonment of the systolic HF program altogether. With Mydicar being CLDN's sole product in the clinic, in the event of study failure we expect shares to fall to approximately cash levels (~$3/share anticipated in April).

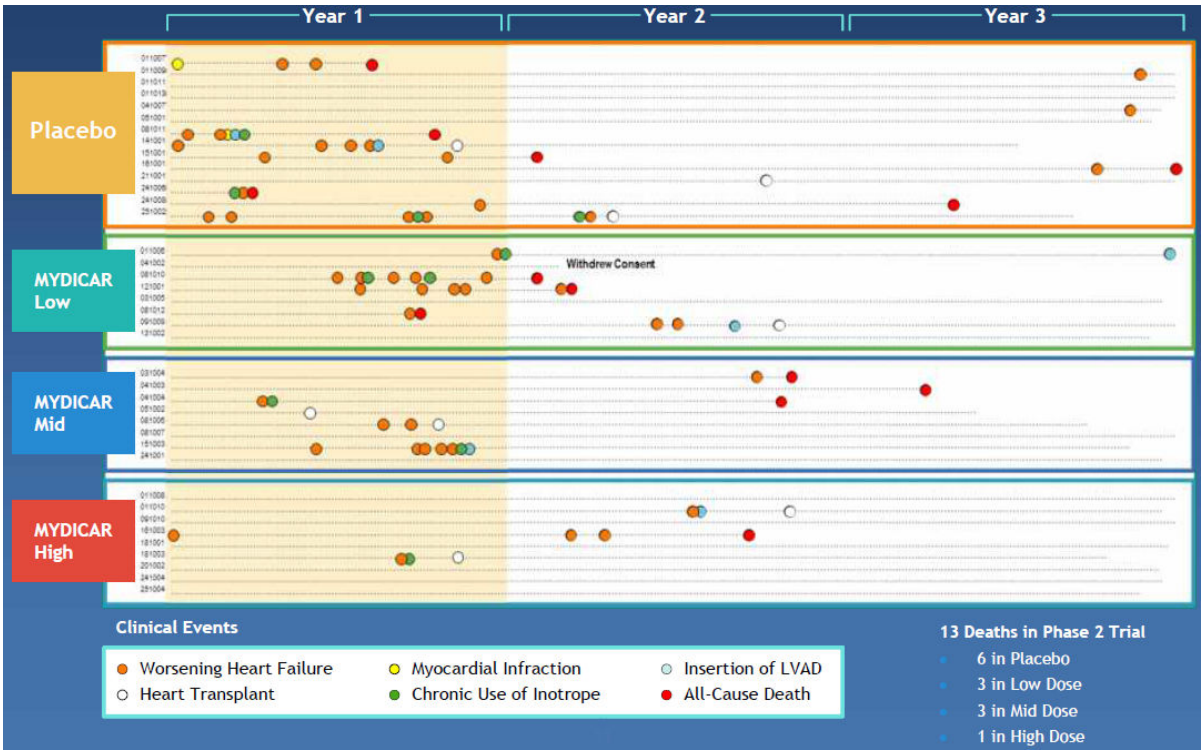
The bull case: the clinical benefit with Mydicar is so dramatic that it could support accelerated approval in the US (10% likelihood). CLDN has said that if Mydicar demonstrates a substantial clinical benefit in CUPID2 the company will meet with regulatory agencies to discuss whether Mydicar could qualify for expedited approval. We note that Mydicar has already been assigned Fast Track status by FDA and is also the first and only gene therapy to be granted Breakthrough Therapy designation. The latter designation could support the bypassing of a Ph 3 study, with perhaps just a confirmatory study required post-approval. Given the serious unmet need in HF, where the five year survival rate is below that of most cancers, we believe a significant reduction in terminal events and improved survival with Mydicar in CUPID2 could potentially suffice to support a BLA submission. Although it is not clear what the magnitude of such a benefit would need to be, we believe a risk reduction similar to that observed in CUPID1 (HR=0.12) would qualify. Under this bullish scenario a market launch could potentially occur in 2018 in both the US and EU.

Figure 1: Recurrent Hospitalization Rate In CUPID1



Source: Company data

Figure 2: Adjudicated Cardiovascular Clinical Events in CUPID1



Source: Company data

Figure 3: Baseline Patient Characteristics in CUPID1 and CUPID2

Characteristic	CUPID 1 (N = 39)	CUPID 2 (N = 250)
Ischemic HF	49%	48%
NT-proBNP (pg/mL)	2268 ± 2209	2834 ± 4137
LV-Ejection Fraction	25% ± 7	23% ± 6
6-Minute Walk Test (m)	345 ± 119	326 ± 81
Age (yrs)	61 ± 11	59 ± 11
Male	87%	82%
NYHA III or IV	100%	84%
White	90%	82%
Beta blocker	92%	93%
ACE/ARB	92%	88%
Aldosterone antagonist	46%	59%
Diuretic	92%	92%

Source: Company data

Milestones

April 2015	Data from Phase 2b CUPID2 trial
2015	Start Phase 1/2 study of MYDICAR in nAb-positive systolic heart failure patients
2015	Start Phase 2a trial for AV fistula maturation failure in end-stage renal disease (ESRD) patients
2015	Servier to potentially exercise option to license ex-US rights to small molecule SERCA2b program in diabetes

Financial Model

1/21/2015
Ticker: (CLDN:Nasdaq)
Celladon Corporation

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	2012A	2013A	Q1	Q2	Q3	Q4	2014E	2015E	2016E	2017E	2018E	2019E	2020E
Revenues:													
US Product Sales	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$111,108	$412,367
ex-US Product Sales	$0	0	$0	$0	$0	$0	0	0	0	0	56,463	231,272	388,676
Grant Revenue	$0	0	$0	$0	$0	$0	0	0	0	0	0	0	0
Total Revenues	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**56,463**	**342,380**	**801,043**
Cost and Expenses:													
Cost of Sales	0	0	0	0	0	0	0	0	0	0	0	11,111	41,237
R&D	13,314	16,927	5,218	4,981	5,316	5,422	20,937	22,796	26,562	29,992	32,465	35,141	38,037
SG&A	2,631	3,037	1,706	2,024	2,815	2,871	9,416	11,981	12,497	13,331	22,607	43,241	118,691
Total Operating Expenses	**15,945**	**19,964**	**6,924**	**7,005**	**8,131**	**8,294**	**30,354**	**34,776**	**39,059**	**43,324**	**55,072**	**89,492**	**197,965**
Operating Income (Loss)	(15,945)	(19,964)	(6,924)	(7,005)	(8,131)	(8,294)	(30,354)	(34,776)	(39,059)	(43,324)	1,391	252,888	603,078
Net Interest Income (Expense)	(73)	58	(51)	21	(235)	584	319	2,416	6,624	7,656	6,060	8,516	19,034
Other non-operating Income (Expense)	147	0	(187)	(8)	8	0	0	0	0	0	0	0	0
Income Before Income Taxes	(15,871)	(19,906)	(7,162)	(6,992)	(8,358)	(7,709)	(30,034)	(32,360)	(32,436)	(35,668)	7,451	261,404	622,112
Provision for Income Taxes	0	0	0	0	0	0	0	0	0	0	711	9,490	195,985
Net Income (Loss)	(15,871)	(19,906)	(7,162)	(6,992)	(8,358)	(7,709)	(30,034)	(32,360)	(32,436)	(35,668)	6,740	251,914	426,127
GAAP EPS	**(1.58)**	**(1.67)**	**(0.60)**	**(0.38)**	**(0.40)**	**(0.33)**	**(1.71)**	**(1.26)**	**(1.01)**	**(1.07)**	**0.20**	**7.50**	**12.68**
Total Shares Outstanding	10,262	12,035	18,500	18,534	23,298	23,323	23,323	28,348	33,423	33,523	33,598	33,598	33,598
Cash Burn	-	(15,074)	(9,927)	(7,304)	(7,188)	(9,758)	(34,177)	(34,413)	(38,707)	(42,973)	2,356	237,326	562,127
Cash Balance	32,649	18,370	57,629	51,172	95,122	86,198	86,198	205,252	309,717	262,767	257,472	493,471	877,705

Source: Wedbush Securities, Inc.

Analyst Biography

David Nierengarten, Ph.D.

David is an Analyst covering stocks in the Biotechnology/Biopharmaceuticals/BioDefense sector. His prior sell-side research experience at Robert W. Baird & Co. covered biotechnology companies of all market capitalizations, with a focus on oncology and rare diseases.

David received his B.S. (Biochemistry) from the University of Wisconsin-Madison and Ph.D. (Molecular and Cell Biology) from the University of California-Berkeley.

David's Edge: David's early stage venture capital investing experience gives him a balanced perspective on developmental-stage biotechnology companies and their ultimate risk/reward potential. His experience on the other side of that equation in a clinical-stage, venture backed biotechnology company provides him with insights into corporate operations. The combination of experiences creates a focus on value creation in this event-driven space.

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I, David M. Nierengarten, Ph.D., Dilip Joseph, certify that the views expressed in this report accurately reflect my personal opinion and that I have not and will not, directly or indirectly, receive compensation or other payments in connection with my specific recommendations or views contained in this report.

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Celladon Corp.	1,3,4,5

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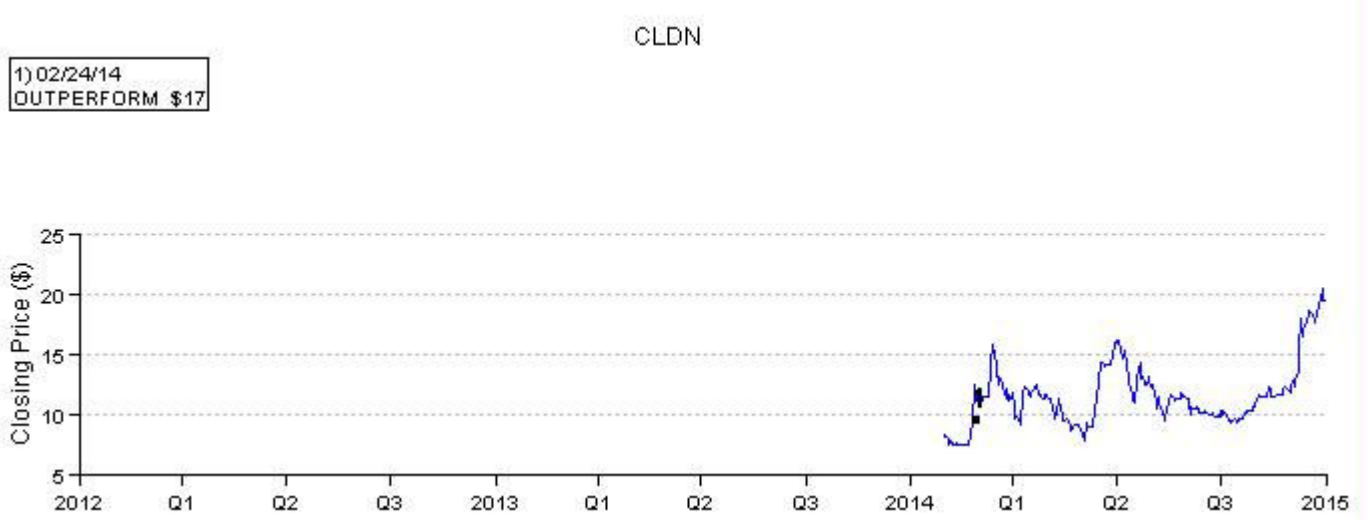

* WS changed its rating system from (Strong Buy/Buy/Hold/Sell) to (Outperform/ Neutral/Underperform) on July 14, 2009.
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