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Cerecor Inc. (CERC-NASDAQ)

CERC: CDG FIRST Trial Underway…

Based on our probability adjusted DCF model that takes into account potential future revenues from CERC-301, CERC-611, commercial operations, and sales of PRVs, using a 12% discount rate CERC is valued at $8.50/share.

Current Price (07/23/19)	$4.53
Valuation	**$8.50**

OUTLOOK

On July 15, 2019, Cerecor Inc. (CERC) announced that the first patient has been enrolled in the CDG FIRST (Congenital Disorders of Glycosylation Formative Retrospective Study) trial. This is a multicenter, non-interventional, retrospective study of patients diagnosed with PGM1-CDG, MPI-CDG, or SLC35C1-CDG and treated with or without D-galactose, D-mannose, or L-fucose, respectively. The company is investigating the natural history of CDGs, and following data collection will meet with the FDA to determine if any prospective studies are necessary. We anticipate an NDA filing for CERC-801 in the first half of 2021, with NDA filings for CERC-802 and CERC-803 approximately six and 12 months later, respectively. Since those compounds each have rare pediatric disease designation (RPDD), approval for each would be accompanied by a rare pediatric disease priority review voucher (PRV), which are fully transferrable and have been sold by other companies recently for $100 million.

SUMMARY DATA

52-Week High	**$7.22**
52-Week Low	**$3.02**
One-Year Return (%)	**-2.58**
Beta	**2.05**
Average Daily Volume (sh)	**277,182**
Shares Outstanding (mil)	**43**
Market Capitalization ($mil)	**$194**
Short Interest Ratio (days)	**N/A**
Institutional Ownership (%)	**54**
Insider Ownership (%)	**64**
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 2018 Estimate	**-7.3**
P/E using 2019 Estimate	**-7.2**

Risk Level	**High**
Type of Stock	**Small-Growth**
Industry	**Med-Biomed/Gene**

ZACKS ESTIMATES

Revenue
(in millions of $)

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2018	4.5 A	4.8 A	4.1 A	5.0 A	18.3 A
2019	5.4 A	5.1 E	5.3 E	5.6 E	21.0 E
2020					23.0 E
2021					25.0 E

Earnings per Share

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2018	-$0.12 A	-$0.19 A	-$0.71 A	-$0.18 A	-$1.20 A
2019	-$0.13 A	-$0.18 E	-$0.18 E	-$0.17 E	-$0.70 E
2020					-$0.64 E
2021					-$0.65 E

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WHAT'S NEW

Business Update

CDG FIRST Trial Underway

On July 15, 2019, Cerecor Inc. (CERC) announced the first patient has been enrolled in the CDG FIRST (Congenital Disorders of Glycosylation Formative Retrospective Study) trial. This is a multicenter, non-interventional, retrospective study of patients diagnosed with phosphoglucomutase 1 (PGM1) deficiency, mannose-phosphate isomerase (MPI) deficiency, or leukocyte adhesion deficiency type II (LADII) or SLC35C1. Each of those diseases is a congenital disorder of glycosylation (CDG), which are ultra-rare orphan metabolic diseases that affect fewer than 1,000 individuals worldwide.

Cerecor is developing CERC-801 (D-galactose), CERC-802 (D-mannose), and CERC-803 (L-fucose) for the treatment of PGM1 deficiency, MPI deficiency, and SLC35C1-CDG, respectively. The purpose of the CDG FIRST trial is to collect natural history data from patients diagnosed with CDGs along with any treatment-related data whether or not they had been treated with D-galactose, D-mannose, or L-fucose. Earlier in 2019, the FDA issued draft guidance on the use of natural history studies for drug development in rare diseases. Since the natural history, or the course a disease would take with no intervention, is typically limited for rare diseases, these types of studies are important to help understand the most useful types of outcomes to monitor.

We believe the company is hoping to collect data from approximately 10 patients each for CERC-801 and CERC-802 while for CERC-803 only a few patients will likely be needed given that it is incredibly rare. Following data collection, the company will meet with the FDA to determine if a prospective study will be necessary. We anticipate Cerecor will be in a position to meet with the FDA in the fourth quarter of 2019 regarding CERC-801. At that meeting, Cerecor will present all the data accumulated through the CDG FIRST trial for PGM1 deficiency patients along with the results from the Phase 1 PK study that was completed earlier in 2019. If a prospective study is required, we do not believe the FDA would require data from more than a few patients.

Precedent for a very small prospective trial is provided by Xuriden® (uridine triacetate), which was approved by the FDA in 2015 following an open-label study in four patients with hereditary orotic aciduria. Three of the four patients had previously been treated with uridine before being switched to Xuriden®. We believe this is a very good example of what the FDA may require for CERC-801, however we will not know whether a prospective study will be required until after the company meets with the agency.

Potential for Priority Review Voucher

Given that the number of patients for each of the CERC-800 products is very small, the most important aspect for those products is likely the fact that they are eligible for a priority review voucher (PRV) upon approval. A PRV allows the holder of the voucher to receive an expedited six-month review from the FDA for an NDA or biologics license application (BLA) instead of the usual ten-month review. The Food and Drug Administration Safety and Innovation Act (FDASIA) created the rare pediatric voucher in 2012 to specifically target the need for additional therapies for rare pediatric subsets of diseases (affect fewer than 200,000 individuals in the U.S.). Priority review vouchers are also awarded for the development of treatments for certain tropical diseases and medical countermeasures.

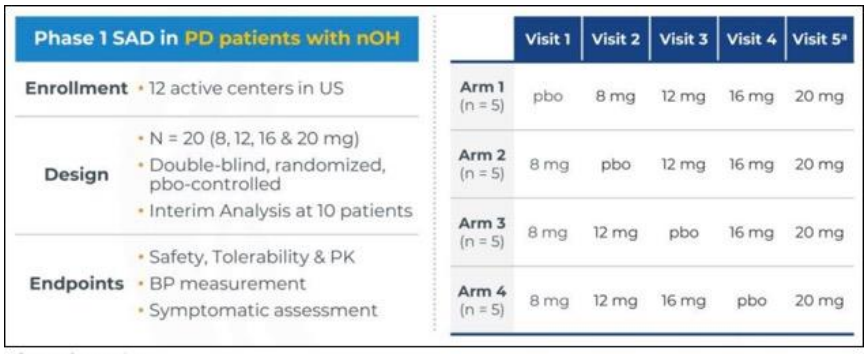
Priority review vouchers are fully transferrable, and a number of companies that have been issued the vouchers in the past have sold them, including one that was sold to AbbVie (ABBV) in Aug. 2015 for $350 million. The four most recent purchases are by Novartis (NVS) for $130 million in Dec. 2017, Jazz Pharmaceuticals for $125 million in Apr. 2017, an undisclosed buyer for $80.6 million in Aug. 2018, and Biohaven Pharmaceutical Holding Company for $105 million in Mar. 2019. While prices for PRVs have come down since AbbVie purchased one for $350 million in 2015, the price for them appears to have settled in the $80-$100 million range. The following table shows how many PRVs have been issued along with the current status of the voucher, if known.

Priority Review Vouchers			
Voucher Award Date	Voucher Type	Voucher Awardee	Voucher Status
2009	Tropical Disease	Novartis	Used for BLA for canakinumab
2012	Tropical Disease	Janssen	Used to accelerate approval of Tremfya (guselkumab) for plaque psoriasis
2014	Rare Pediatric Disease	BioMarin	Sold to Sanofi for $67.5M in Jul 2014; used for approval of Praluent
2014	Tropical Disease	Knight Therapeutics	Sold to Gilead for $125M in Nov 2014; used for approval of Odefsey
2015	Rare Pediatric Disease	United Therapeutics	Sold to AbbVie for $350M in Aug 2015
2015	Rare Pediatric Disease	Asklepion Pharmaceuticals	Transferred to Retrophin and sold to Sanofi for $245M in May 2015
2015	Rare Pediatric Disease	Wellstat Therapeutics	Transferred to AstraZeneca
2015	Rare Pediatric Disease	Alexion Pharmaceuticals	Used for approval of ALXN1210
2015	Rare Pediatric Disease	Alexion Pharmaceuticals	Not used
2016	Tropical Disease	PaxVax Bermuda	Not used (possibly sold to Gilead for ~$200M in 2Q16)
2016	Rare Pediatric Disease	Sarepta Therapeutics	Sold to Gilead for $125M in Feb 2017; used for approval of HIV treatment
2016	Rare Pediatric Disease	Ionis Pharmaceuticals	Not used
2017	Rare Pediatric Disease	Marathon Pharmaceuticals	Not used
2017	Rare Pediatric Disease	BioMarin	Sold for $125 million in Nov 2017
2017	Tropical Disease	Chemo Research, S.L.	Not used
2017	Rare Pediatric Disease	Novartis	Not used
2017	Rare Pediatric Disease	Ultragenyx Pharmaceutical	Sold to Novartis for $130 million in Dec. 2017; used for approval of siponimod
2017	Rare Pediatric Disease	Spark Therapeutics	Sold to Jazz Pharmaceuticals for $110 million in Apr 2018
2018	Rare Pediatric Disease	Ultragenyx Pharmaceutical	Sold to undisclosed buyer for $80.6 million in Aug. 2018
2018	Rare Pediatric Disease	Medicines Development	Not used
2018	Rare Pediatric Disease	GW Pharma	Sold to Biohaven for $105 million on Mar. 18, 2019
2018	Material Threat Medical Countermeasure	SIGA Technologies	Sold to Eli Lilly for $80 million on Nov. 1, 2018
2018	Tropical Disease	GlaxoSmithKline	Used by ViiV Healthcare for NDA for HIV-1 infection
2018	Rare Pediatric Disease	Leadiant Bioscience Inc	Not used
2018	Rare Pediatric Disease	Sobi and Novimmune	Not used
2019	Tropical Disease	Novartis	Not used
2019	Rare Pediatric Disease	Vertex	Not used
2019	Rare Pediatric Disease	Alexion Pharmaceuticals	Not used
2019	Tropical Disease	Sanofi	Not used
2019	Rare Pediatric Disease	Novartis	Not used

Source: raps.org / Zacks SCR

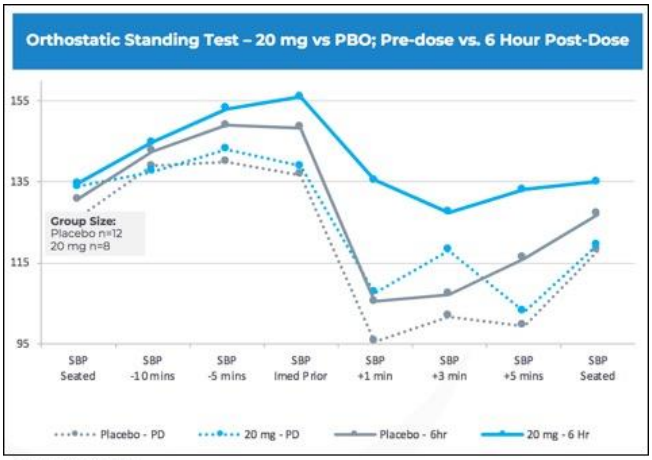
Positive Final Results for CERC-301 in nOH

On July 1, 2019, Cerecor announced positive final results for the Phase 1 clinical trial of CERC-301 in Parkinson's disease (PD) patients suffering from neurogenic orthostatic hypotension (nOH). The Phase 1 trial was a double blind, randomized, placebo controlled trial with 20 Parkinson's patients with nOH. Each patient had five visits in which they received four single escalating doses of drug (8, 12, 16, or 20 mg) or placebo. Patients then completed six orthostatic standing tests over a six hour period: blood pressure was recorded while in supine position with the head elevated, immediately before standing, and then one minute, three minutes, and five minutes after standing. This was repeated every hour for six hours. While the primary endpoints of the study were safety, tolerability, and pharmacokinetics, a key secondary endpoint was the effect of CERC-301 on blood pressure. An overview of the trial is given below.



Source: Cerecor, Inc.

The company reported that the final data from the study continues to show a rapid, robust, and sustained increase in systolic blood pressure (SBP) upon standing, with the 20 mg dose achieving clinically meaningful (>7 mmHg SBP) improvements with a maximum improvement of 29.1 mmHg. The following graph shows the increase in SBP was continuing up to six hours post dose. Given that the patient's blood pressure appeared to still be increasing six hours post dose, we anticipate the next trial will likely investigate time points beyond six hours, which could help to clearly differentiate CERC-301 from other nOH/OH treatments.



Source: Cerecor Inc.

Based on the results seen in the Phase 1 study, the company will likely be looking to expand the use of CERC-301 in other conditions where OH is a problem, including those with diabetes, where up to one-third may suffer from OH (Wu *et al*., 1999), and in dialysis patients, where a symptomatic decrease in blood pressure occurs in 15-50% of dialysis sessions (Ghaffar *et al*., 2015).

Conclusion and Valuation

We're glad to see that Cerecor has initiated the CDG FIRST trial and that the company remains on track to file for regulatory approval for CERC-801 in the first half of 2021. We continue to anticipate NDA filings for CERC-802 and CERC-803 to follow approximately six and 12 months later, respectively. The data for CERC-301 is very encouraging and the potential to move into other indications where OH is an issue could expand the market opportunity for that drug. Our valuation currently stands at $8.50.

PROJECTED FINANCIALS

Cerecor, Inc.	2018 A	Q1 A	Q2 E	Q3 E	Q4 E	2019 E	2020 E	2021 E
Commercial Group	$18.3	$5.4	$5.1	$5.3	$5.6	$21.4	$23.0	$25.0
CERC-301	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
CERC-611	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
CERC-406	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
LIcense Revenue	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Grant Revenue	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Total Revenues	**$18.3**	**$5.4**	**$5.1**	**$5.3**	**$5.6**	**$21.4**	**$23.0**	**$25.0**
Cost of Sales	$7.5	$1.9	$2.1	$2.1	$2.2	$8.3	$8.9	$9.8
Product Gross Margin	*59%*	*64%*	*59%*	*60%*	*61%*	*61%*	*61%*	*61%*
Research & Development	$5.8	$3.4	$3.8	$4.0	$4.2	$15.4	$17.0	$19.0
Acquired in-process R&D	$18.7	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
General & Administrative	$10.7	$2.7	$2.3	$2.4	$2.5	$9.9	$10.0	$10.5
Sales and Marketing	$8.5	$3.1	$3.0	$3.0	$3.0	$12.1	$13.0	$14.0
Amortization Expense	$4.5	$1.1	$1.2	$1.2	$1.2	$4.7	$4.8	$4.8
Impariment of Intangible Assets	$1.9	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Change in fair value of contingent consideration	$0.1	$0.2	$0.0	$0.0	$0.0	$0.2	$0.0	$0.0
Operating Income	($39.3)	($7.0)	($7.3)	($7.4)	($7.5)	($29.2)	($30.7)	($33.1)
Operating Margin	*-215%*	-	-	-	-	*-136%*	*-133%*	*-132%*
Other (expense) income	($0.8)	($0.3)	($0.3)	($0.3)	($0.3)	($1.2)	($1.2)	($1.2)
Pre-Tax Income	($40.1)	($7.3)	($7.6)	($7.7)	($7.8)	($30.4)	($31.9)	($34.3)
Income Taxes Paid	($0.0)	$0.2	$0.0	$0.0	$0.0	$0.3	$0.1	$0.1
Net Income	**($40.1)**	**($7.5)**	**($7.6)**	**($7.8)**	**($7.8)**	**($30.7)**	**($32.0)**	**($34.4)**
Net Margin		-	-	-	-			
Reported EPS	**($1.20)**	**($0.13)**	**($0.18)**	**($0.18)**	**($0.17)**	**($0.70)**	**($0.64)**	**($0.65)**
YOY Growth		-	-	-	-			
Basic Shares Outstanding	34.8	42.0	43.0	43.5	46.0	43.6	50.0	53.0

Source: Zacks Investment Research, Inc. *David Bautz, PhD*

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