Zacks Small-Cap Research
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# Cerecor Inc

**(CERC-NASDAQ)**

## *CERC: Unveils Rare Pediatric Disease Programs…*

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 $7.00/share.

| | |
|---|---|
| Current Price (11/26/18) | $3.52 |
| **Valuation** | **$7.00** |

## OUTLOOK

In October 2018, Cerecor, Inc. (CERC) announced the submission of three Orphan Drug Designation (ODD) requests for three substrate replacement therapies targeting congenital disorders of glycosylation (CDGs). The three submissions were for CERC-801, D-galactose to treat phosphoglucomutase 1 (PGM1) deficiency, CERC-802, D-mannose to treat Mannose-Phosphate Isomerase (MPI) deficiency, and CERC-803, L-fucose to treat Leukocyte Adhesion Deficiency Type II (LADII). Each program has been designated a Rare Pediatric Disease, which means that upon approval a Rare Pediatric Disease Priority Review Voucher (PRV) could be issued. PRVs are fully transferable and a number of them have recently sold for $80-$100 million.

## SUMMARY DATA

| | |
|---|---|
| **52-Week High** | **$5.66** |
| **52-Week Low** | **$1.18** |
| **One-Year Return (%)** | **198.30** |
| **Beta** | **1.48** |
| **Average Daily Volume (sh)** | **50,986** |
| **Shares Outstanding (mil)** | **41** |
| **Market Capitalization ($mil)** | **$144** |
| **Short Interest Ratio (days)** | **N/A** |
| **Institutional Ownership (%)** | **42** |
| **Insider Ownership (%)** | **80** |
| **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** | **18.6** |
| **P/E using 2018 Estimate** | **-6.3** |
| **P/E using 2019 Estimate** | **-6.1** |

| | |
|---|---|
| **Risk Level** | **Above Avg.** |
| **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) |
|---|---|---|---|---|---|
| 2017 | 0 A | 0 A | 25 A | 2 A | 28 A |
| 2018 | 4.5 A | 4.8 A | 4.1 A | 4.9 E | 18.2 E |
| 2019 | | | | | 22.0 E |
| 2020 | | | | | 25.0 E |

**Earnings per Share**

| | Q1 (Mar) | Q2 (Jun) | Q3 (Sep) | Q4 (Dec) | Year (Dec) |
|---|---|---|---|---|---|
| 2017 | -$0.19 A | -$0.14 A | -$0.52 A | -$0.11 A | -$0.42 A |
| 2018 | -$0.12 A | -$0.19 A | $0.71 A | -$0.10 E | -$1.11 E |
| 2019 | | | | | -$0.28 E |
| 2020 | | | | | -$0.22 E |

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## WHAT'S NEW

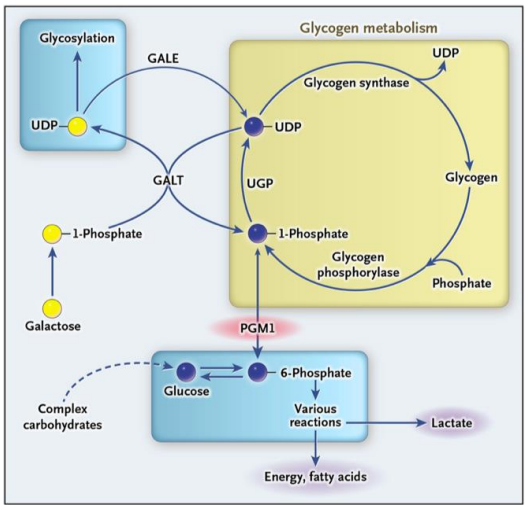
**Business Update**

*Rare Pediatric Disease Programs*

On October 31, 2018, Cerecor, Inc. (CERC) announced the submission of three Orphan Drug Designation (ODD) requests for three of the development products acquired from the purchase of Ichorion Therapeutics, Inc. Each of them is being developed for the treatment of ultrarare metabolic disorders known as Inborn Errors of Metabolism (IEM).

**CERC-801 (D-galactose)**: This is being developed for the treatment of Phosphoglucomutase 1 (PGM1) deficiency, a recently characterized congenital disorder of glycosylation (CDG). Patients with PGM1 deficiency have multiple clinical phenotypes, including dilated cardiomyopathy, exercise intolerance, and hepatopathy (Tegtmeyer *et al*., 2014). The multisystem deficiencies are a result of a lack of protein glycosylation, which is dependent upon the important metabolic intermediate glucose-1-phosphate.

PGM1 is responsible for the interconversion of glucose-1-phosphate and glucose-6-phosphate. In between meals, PGM1 drives the production of glucose-6-phosphate from glucose-1-phosphate, which is the product of glycogen breakdown. Following a meal, when glucose concentration is high, PGM1 drives the production of glucose-1-phosphate such that excess glucose can be stored as glycogen. The loss of PGM1 leads to a decreased amount of glucose-1-phosphate, which ultimately results in decreased protein glycosylation. The following figure shows the role of PGM1 in metabolism.




*Source*: Tegtmeyer *et al*., 2014

Galactose supplementation (1.5 g/kg/day) was recently tested in nine patients with PGM1 deficiency (Wong *et al*., 2017). Results of the study showed that supplementation was well tolerated, abnormal baseline results in liver enzymes normalized, and increases in protein glycosylation were noted. Longer duration trials are currently ongoing to better characterize the effect of galactose supplementation in this patient population.

**CERC-802 (D-mannose)**: This is being developed for the treatment of Mannose-Phosphate Isomerase (MPI) deficiency (Westphal *et al*., 2001). MPI is responsible for the interconversion of fructose-6-phosphate and mannose-6-phosphate along with maintaining the supply of mannose derivatives required for protein glycosylation. MPI deficiency is readily treated with mannose supplementation as there are mannose-specific cellular transporters that bring mannose into the cell, where it is converted into mannose-6-phosphate by hexokinase.

**CERC-803 (L-fucose)**: This is being developed for the treatment of Leukocyte Adhesion Deficiency Type II (LADII). LADII is characterized by leukocytosis, persistent infections, and severe mental and physical retardation. The cause of LADII is improper fucose metabolism that leads to the absence of selectin ligands on the surface of neutrophils. Fucose supplementation of patients with LADII can result in re-expression of selectin ligands, a decrease in neutrophil count, and improvement in mental and physical development (Marquardt *et al*., 1999).

In addition to seeking ODD, the FDA has designated each of those programs as Rare Pediatric Diseases, which makes them eligible for a Rare Pediatric Disease 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.

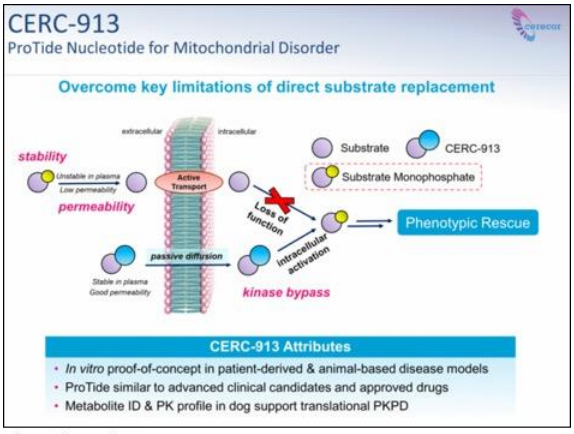
Priority review vouchers are fully transferrable, and a number of companies that have been issued the vouchers in the past have sold them, including one that was sold to AbbVie (ABBV) in Aug. 2015 for $350 million. The four most recent purchases are by an undisclosed buyer for $125 million in Nov. 2017, Novartis (NVS) for $130 million in Dec. 2017, Jazz Pharmaceuticals for $125 million in Apr. 2017, and an undisclosed buyer for $80.6 million in Aug. 2018. While prices for PRVs have come down since AbbVie purchased one for $350 million in 2015, the price for them has appeared to settle in the $100 million range. The following table shows how many priority review vouchers 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 | Not used |
| 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 |

Source: raps.org / Zacks SCR

In addition to the substrate replacement therapy products described above, Cerecor also has a platform technology for targeting diseases for which substrate replacement therapy is not feasible. The lead development candidate based on that technology is CERC-913, which uses a pro-drug technology ("ProTide") to treat mitochondrial DNA depletion sydromes (MDS). The technology is based on the ability to get a phosphorylated substrate into the cell where it is needed. Typically, phosphorylated substrates will not passively diffuse through the cell membrane, thus a disease characterized by the lack of a phosphorylated substrate inside the cell is not able to be treated through substrate replacement therapy. The ProTide technology allows a phosphorylated substrate to diffuse into the cell, at which time it is activated such that the substrate can be used for phenotypic rescue. This is shown in the following slide.




Source: Cerecor, Inc.

CERC-913 has not been granted rare pediatric disease designation, however Cerecor will be applying for that status and is confident that it will be granted.

*Enrollment Continuing for CERC-301 in Neurogenic Orthostatic Hypotension*

In August 2018, Cerecor announced the first patient has been enrolled in a Phase 1 study of its lead compound, CERC-301, for the treatment of neurogenic orthostatic hypotension (nOH) associated with Parkinson's disease. We anticipate topline data from the Phase 1 study of CERC-301 in nOH in the first half of 2019.

Orthostatic hypotension (OH) occurs due to an inability to maintain blood pressure upon standing. The technical definition of OH is a sustained reduction in systolic blood pressure of at least 20 mm Hg or diastolic blood pressure of at least 10 mm Hg within three minutes of standing. OH can result from either non-neurogenic or neurogenic causes and be either acute or chronic. Non-neurogenic causes include dehydration, cardiac abnormalities (bradycardia/tachyarrhythmia/myocardial infarction), and prolonged standing.

nOH is caused by decreased release of norepinephrine from sympathetic vasomotor neurons that can result in both inadequate vasoconstriction and heart rate upon standing (Freeman *et al*., 2011). The disorder occurs mostly in those with specific diseases caused by accumulation of alpha-synuclein, including Parkinson's disease (PD), multiple system atrophy (MSA), Lewy body dementia, and pure autonomic failure (PAF). It can also occur in patients with peripheral neuropathies, including diabetic neuropathy, amyloidosis, and Guillan-Barre syndrome.

In contrast to OH, which is relatively common in elderly patients (prevalence of 15% in those aged 65 to 69 to 26% in those aged 85 years or older, Rutan *et al*. 1992), nOH is a rare disorder and is considered as an orphan disease, affecting approximately 80,000 individuals with PD, MSA, and PAF in the U.S. (FDA briefing documents for Northera®).

Few studies have examined the prognosis for patients with nOH, however a study of 104 nOH patients over a 14-year period in Italy showed that the condition increased the risk of death three-fold compared to the general population in that area (Maule *et al*., 2012). On a day to day basis, the condition can result in severe morbidity for patients that includes significant drops in blood pressure during the day. This can lead to an interference in normal daily activities, with the increased debilitation potentially leading to a poor quality of life (QOL).

Non-pharmacological therapies for nOH include drinking more water (to help increase blood volume), increase salt in the diet, avoid carbohydrate-heavy meals, elevating the head of the bed, slowly rising when standing, and getting regular exercise. There are currently two therapies approved in the U.S. for the treatment of nOH: midodrine, which was approved in 1996, and droxidopa (Northera®), which was approved in 2014.

- Midodrine (ProAmatine®): This is a peripherally acting $\alpha$-andrenergic agonist. The drug was originally approved based on clinical data showing an effect on the surrogate endpoint of increase in standing blood pressure, which is thought to confer clinical beneft. It is currently available as a generic.

- Droxidopa (Northera®): This is a synthetic amino acid precursor for norepinephrine that is capable of crossing the blood-brain barrier. Droxidopa was originally approved in Japan for the treatment of hypotension and nOH in 1989. Chelsea Therapeutics acquired the rights to droxidopa and following approval of the compound by the FDA the company was acquired by Lundbeck for $658 million. Lundbeck reported 2017 revenues for Northera® of DKK 1,644 (approximately $270 million).

**Financial Update**

On November 13, 2018, Cerecor announced financial results for the third quarter of 2018. The company recorded net revenues of $4.1 million in the third quarter of 2018 compared to no product revenue in the third quarter of 2017 as a result of the acquisition of TRx in Nov. 2017 and Avadel in Feb. 2018. In the third quarter of 2018, cost of product sales were $3.1 million, sales and marketing expenses were $2.3 million, and amortization expense was $1.1 million. R&D expenses in the third quarter of 2018 were increased by $0.1 million compared to the third quarter of 2017 to $1.0 million. The increase was due to increased clinical costs and employee salaries partially offset by a decrease in stock-based compensation. G&A expenses were $1.9 million for the third quarter of 2018 compared to $2.2 million in the third quarter of 2017. The decrease was primarily due to lower legal, consulting, and professional expenses partially offset by an increase in stock-based compensation. The company reported an $18.5 million non-cash expense for acquired in-process research and development, which was related to the three preclinical assets

acquired from Ichorion. Net loss for the third quarter of 2018 was $24.6 million, compared to net income of $18.7 million in the third quarter of 2017. The large net loss was mainly driven by the acquired in-process research and development expense while the net income in 2017 was driven by net revenues of $25 million related to the sale of CERC-501 to Janssen.

As of September 30, 2018, Cerecor had $6.8 million in cash and cash equivalents, which was due in part to a private placement during the quarter with Armistice Capital, the majority shareholder of the company, that resulted in net proceeds of $3.9 million. The company also collected $3.8 million from escrow related to the sale of CERC-501 to Janssen in 2017. We believe this will be sufficient to fund operations through the first quarter of 2019.

As of Nov. 6, 2018, Cerecor had approximately 40.8 million shares of common stock outstanding and when factoring in stock options and reasonably priced warrants a fully diluted share count of 59.7 million.

## Conclusion and Valuation

Cerecor continues to execute on its business transformation strategy as it transitions to a fully-integrated pharmaceutical company. We continue to anticipate at least a couple of IND filings for the rare pediatric disease programs in 2019 along with Phase 1 data from CERC-301 in nOH in the first half of 2019. We have made one small change to our model based on the potential selling price for PRVs issued upon approval of the rare pediatric disease assets. Based on the fact that the last two PRV's to sell have sold for $80 million, we have dropped the potential sales price for PRVs in our model from $100 million to $80 million. This has resulted in a slight adjustment to our valuation to $7.00 per share.

## PROJECTED FINANCIALS

| Cerecor, Inc. | 2017 A | Q1 A | Q2 A | Q3 A | Q4 E | 2018 E | 2019 E | 2020 E |
|---|---|---|---|---|---|---|---|---|
| **Commercial Group** | $2.2 | $4.5 | $4.8 | $4.1 | $4.9 | $18.2 | $22.0 | $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** | $25.0 | $0.0 | $0.0 | $0.0 | $0.0 | $0.0 | $0.0 | $0.0 |
| **Grant Revenue** | $0.6 | $0.0 | $0.0 | $0.0 | $0.0 | $0.0 | $0.0 | $0.0 |
| **Total Revenues** | **$27.8** | **$4.5** | **$4.8** | **$4.1** | **$4.9** | **$18.2** | **$22.0** | **$25.0** |
| Cost of Sales | $1 | $0.9 | $1.4 | $3.1 | $1.4 | $6.8 | $5.3 | $6.0 |
| *Product Gross Margin* | - | *81%* | *70%* | *24%* | *71%* | *63%* | *76%* | *76%* |
| Research & Development | $4.4 | $1.6 | $1.1 | $1.0 | $1.2 | $5.0 | $5.2 | $5.5 |
| Acquired in-process R&D | $0.0 | $0.0 | $0.0 | $18.7 | $0.0 | $18.7 | $0.0 | $0.0 |
| General & Administrative | $7.9 | $2.9 | $3.0 | $1.9 | $3.0 | $10.8 | $10.2 | $10.5 |
| Sales and Marketing | $1 | $1.5 | $2.0 | $2.3 | $2.0 | $7.9 | $8.0 | $8.0 |
| Amortization Expense | $0 | $1.0 | $1.2 | $1.1 | $1.0 | $4.3 | $4.0 | $4.0 |
| Impariment of Intangible Assets | $0 | $0.0 | $1.7 | $0.2 | $0.0 | $1.9 | $0.0 | $0.0 |
| Operating Income | $13.9 | ($3.5) | ($5.7) | ($24.2) | ($3.7) | ($37.2) | ($10.7) | ($9.0) |
| *Operating Margin* | *50%* | - | - | - | - | *-204%* | *-49%* | *-36%* |
| Other (expense) income | ($0.1) | ($0.4) | ($0.3) | ($0.3) | ($0.3) | ($1.2) | ($1.2) | ($1.2) |
| Pre-Tax Income | $13.8 | ($3.9) | ($6.0) | ($24.6) | ($4.0) | ($38.4) | ($11.9) | ($10.2) |
| Income Taxes Paid | $2 | $0 | $0 | $0 | $0 | $0 | $0 | $0 |
| **Net Income** | **$11.9** | **($3.9)** | **($6.0)** | **($24.6)** | **($4.0)** | **($38.5)** | **($11.9)** | **($10.2)** |
| **EBITDA** | **-** | **($2.7)** | **($4.4)** | **($23.2)** | **($2.8)** | **($33.1)** | **($7.0)** | **($5.3)** |
| **Adjusted EBITDA** | **-** | **($1.4)** | **($1.3)** | **($4.0)** | **$0.1** | **($6.6)** | **$2.0** | **$3.5** |
| *Net Margin* | - | - | - | - | - | | | |
| **Reported EPS** | **$0.64** | **($0.12)** | **($0.19)** | **($0.71)** | **($0.10)** | **($1.11)** | **($0.28)** | **($0.22)** |
| *YOY Growth* | - | - | - | - | - | | | |
| Basic Shares Outstanding | 18.4 | 31.3 | 32.2 | 34.6 | 40.0 | 34.6 | 43.0 | 46.0 |

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

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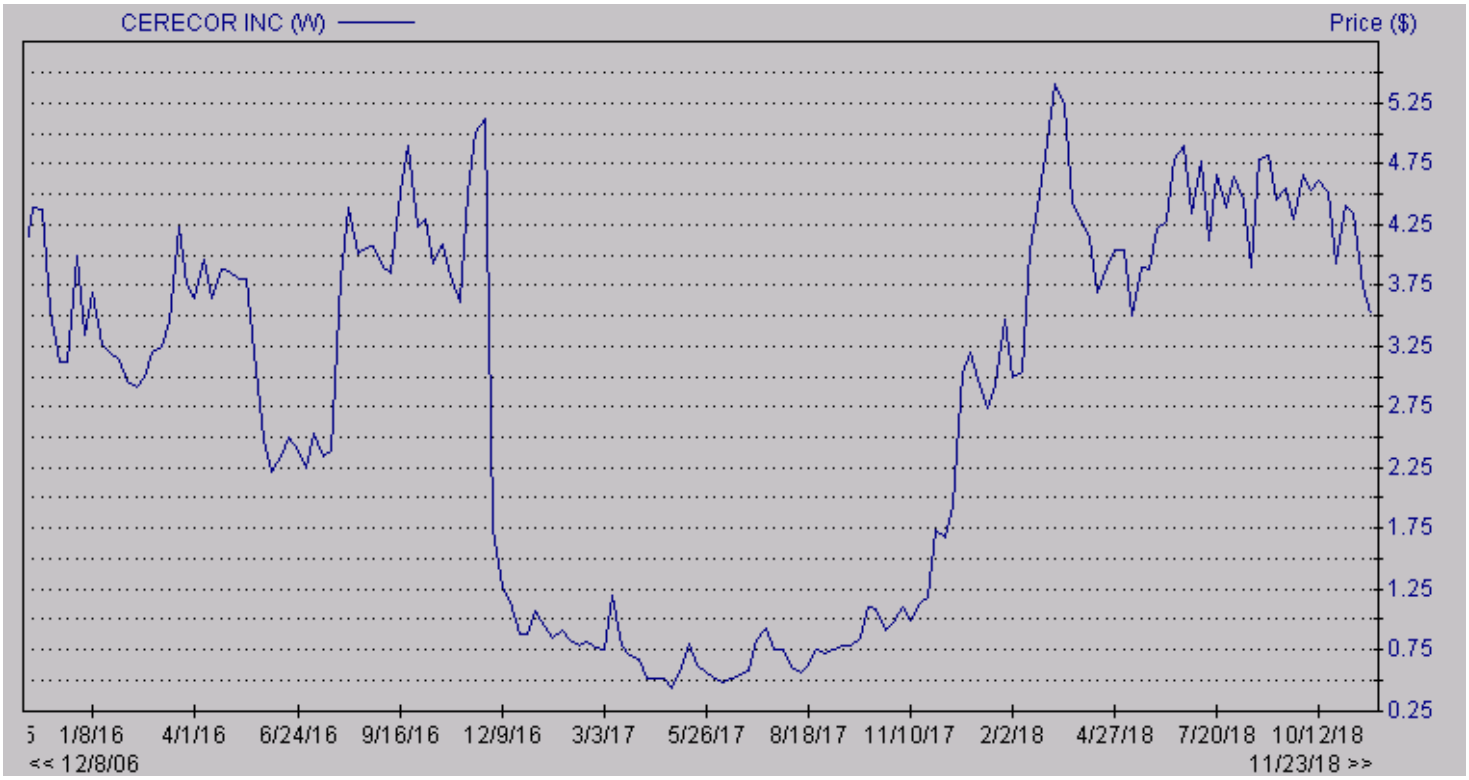
## HISTORICAL STOCK PRICE

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