# PTC Therapeutics (PTCT)

## *Q1:14 Update - New Ataluren Indications Coming, Roche SMA Partnership to Yield Derisking by YE:14*

- **PTCT reported Q1:14 results, ending the quarter with $247M in cash and equivalents which are expected to fund PTCT into 2016, beyond significant Phase III data readouts from ataluren's Phase III trial.** Q1 EPS was ($0.58) and revenues of $9.2M vs. our ($0.49) and $8.5M. We est. 2014 cash burn of $78M.
- **New indications for ataluren's clinical development are to be selected in 2014 and may offer rapid routes into the clinic with derisking proof-of-concept trials read-outs potentially ahead of its Phase III trial for ataluren in DMD expected in H1:15. (page 4-7).** PTCT highlighted work in choroideremia and Hurler's syndrome (MPS I) and published research provides promising evidence for ataluren's efficacy in aniridia, a rare, non-sense mutation-based condition that severely limits the vision of ~5,000 pts in the U.S (page 6).
- **The next event for PTCT is an update on a conditional approval opinion from the EMA in Q2:14 and the start of enrollment in a Phase III trial for ataluren in cystic fibrosis (nmCF) anticipated in the first half of 2014.** We note that regardless of a conditional approval decision by the EMA (potentially May 19), it is possible that PTCT will market ataluren on a named-patient basis in the EU and other territories starting in early 2015.
- **We anticipate that data from a Phase I trial of RG7800 potentially by YE:14 may, in addition to safety and PK data, confirm mechanistic action through the measurement SMN transcript levels in the blood plasma of healthy patients**. An ongoing natural history study could potentially validate surrogate endpoints in SMA, offering a rapid clinical path for RG7800 in this area of unmet medical need.
- **Recall, Isis (ISIS – Not Covered) recently presented data from their antisense SMA program (replay, slide deck) demonstrating promising improvements on HFMSE in children and CHOP-INTEND in infants (pg 3).** We highlight that Isis's compound is administered intrathecally, vs. the oral RG7800, complicating IRB approval, clinical trial design, enrollment and commercial strategy. RG7800 potentially has a more rapid path through the clinic and could be used earlier in disease progression, a time during which animal models suggest maximum benefit.
- **We reiterate our OUTPERFORM rating and 12-month price target of $55/share.** Our $55 price target is derived by applying an 8X multiple to estimated 2017 revenues for ataluren in nmDMD and nmCF, discounted 25% and 35% annually, respectively.

| FYE Dec | 2013E | 2014E | | | 2015E | | |
|---|---|---|---|---|---|---|---|
| REV | ACTUAL | CURR. | PREV. | CONS. | CURR. | PREV. | CONS. |
| Q1 Mar | $7.1A | $9.2A | $8.5E | $10.9E | $1.0E | | $7.0E |
| Q2 Jun | 6.9A | 1.0E | | $3.3E | 1.0E | | $2.0E |
| Q3 Sep | 16.3A | 1.0E | | $3.3E | 1.0E | | $2.0E |
| Q4 Dec | 4.4E | $1.0E | | $3.2E | $1.0E | | $2.0E |
| **Year*** | **$34.7E** | **$12.2E** | **$11.5E** | **$20.7E** | **$4.0E** | | **$15.8E** |
| Change | -- | -- | | | -- | | |
| | 2013E | 2014E | | | 2015E | | |
| EPS | ACTUAL | CURR. | PREV. | CONS. | CURR. | PREV. | CONS. |
| Q1 Mar | (3,244.59)A | ($0.58)EA | ($0.49)E | ($0.46)E | ($0.73)E | | ($0.62)E |
| Q2 Jun | (5.51)A | ($0.70)E | | ($0.70)E | ($0.66)E | | ($0.76)E |
| Q3 Sep | (0.19)A | ($0.71)E | | ($0.73)E | ($0.65)E | | ($0.74)E |
| Q4 Dec | (0.75)E | ($0.73)E | | ($0.76)E | ($0.68)E | | ($0.76)E |
| **Year*** | **($5.18)E** | **($2.73)E** | **($2.64)E** | **($2.67)E** | **($2.72)E** | | **($2.87)E** |
| P/E | -- | -- | | | -- | | |
| Change | -- | -- | | | -- | | |

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

**May 7, 2014**

**Price**
**$17.44**

**Rating**
**OUTPERFORM**

**12-Month Price Target**
**$55**

**Christopher N. Marai, Ph.D.**
(415) 274-6861
christopher.marai@wedbush.com

**Gregory R. Wade, Ph.D.**
(415) 274-6863
greg.wade@wedbush.com

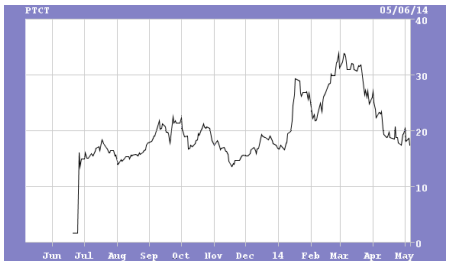
**David M. Nierengarten, Ph.D.**
(415) 274-6862
david.nierengarten@wedbush.com

### Company Information

| | |
|---|---|
| Shares Outst (M) | 30.1 |
| Market Cap (M) | $525.4 |
| 52-Wk Range | $13.04 - $34.65 |
| Book Value/sh | $5.74 |
| Cash/sh | $8.18 |
| Enterprise Value (M) | $278.8 |
| LT Debt/Cap % | 0.0 |
| Cash Burn (M) | $78.4 |
| Current Cash (M) | $246.6 |

### Company Description

PTC Therapeutics is a biopharmaceutical company focused on the development of orally administered, proprietary, small molecule drugs that target post-transcriptional control processes for orphan disorders including DMD and CF.




Source: Thomson Reuters

Risks to the attainment of our price target include 1) failure of ataluren in the clinic in DMD or CF; 2) regulatory failure of ataluren; and 3) inability to fund the development or execute on the commercializing of ataluren globally

## *Investment Thesis*

PTC is a biotechnology company focused on the development of ataluren, a molecularly targeted, orally delivered treatment for rare and ultra-rare diseases, including Duchenne muscular dystrophy and cystic fibrosis caused by nonsense mutations. Ataluren may also be effective in treating 2500 other rare diseases and certain cancers caused by nonsense mutations since its mechanism of action is broadly applicable to these molecular lesions. PTC is also developing a candidate for spinal muscle atrophy a fatal and rare disease that most severely impacts infants. We believe that ataluren will be shown to be safe and efficacious in ongoing Phase III trials in nmDMD as well as those set to begin (H1:14) in nmCF. In our opinion, the Street is overly discounting their lead Phase III program for ataluren in nmDMD, which showed mixed results overall in a Phase II trial, but positive trends and nominal statistically significant benefit in the current subgroups being evaluated in the Phase III trial. We believe that, given lessons learned from prior pioneering trials of ataluren in DMD, PTC has enriched their Phase III trial for success and that it is highly likely to show positive results in mid:2015. Final read-outs from a soon to be initiated (H1:14) trial for ataluren CF are anticipated by mid-2016. PTCT's SMA program, partnered with Roche, remains additional upside to our estimates. We believe that this program likely be accelerated through the clinic due to significant unmet medical need in this devastating disease and that breakthrough results in Phase I/II trials could form the basis for a registration filing as early as 2015.

## *Valuation Methodology*

Our $55 price target is derived by applying an 8X multiple to estimated 2017 revenues for ataluren in nmDMD and nmCF, discounted 25% and 35% annually, respectively. Conditional approval of ataluren in the EU by Q1:14 remains upside to our price target and we estimate would yield a 12-month price target of $75/share. We project that approval and commercialization of ataluren could generate ~$550 million in annual worldwide revenues in 2017 (our valuation year) in nmDMD and nmCF and potential peak global sales of >$1.5 billion. Success of PTC/Roche's SMA candidate RG7800 remains upside to our estimates. Similarly we arrive at our $55 price target by applying a 15x multiple to PTCT's fully taxed EPS in 2017 discounted back 20% annually.

## *Upcoming Milestones*

| | |
|---|---|
| May 19-22 | CHMP meeting: potential feedback from CHMP SAG meeting regarding request for reconsideration of conditional approval for ataluren for nmDMD in the EU |
| June 23-26 | CHMP meeting: potential feedback from CHMP SAG meeting regarding request for reconsideration of conditional approval for ataluren for nmDMD in the EU |
| Q2:14 | Potential MAA filing for conditional approval of ataluren for nmCF in the EU |
| Q2:14 | Potential new opinion following a re-examination of the negative opinion regarding conditional approval of ataluren for nmDMD in the EU |
| Q2:14 | Full enrollment in the confirmatory Phase III trial of ataluren in nmDMD |
| June 12-14 | Update on SMA program (likely preclinical results) potentially at the International SMA Research Group Meeting, National Harbor, MD |
| H1:14 | Initiation of a Phase III trial of ataluren in nmCF |
| H2:14 | Potential data from the Phase IIb open-label extension study of ataluren in the EU |
| 2014 | Nomination of one or two new indications for ataluren (likely Aniridia and/or MPS I) |
| 2014 | Open label trial updates for ataluren in nmDMD at a scientific conference (US study safety only, EU efficacy at 0, 6,12,18 months) |
| YE:14 | Potential conditional approval of ataluren for nmCF in the EU |
| YE:14/Q1:15 | Potential start of proof-of-concept Phase II trials (potentially pivotal) for ataluren in one or two indications (ahead of ataluren in nmDMD) |
| YE:14/Q1:15 | Potential top-line data and biomarker data from Roche/PTCT's Phase I healthy volunteers study of SMA candidate RG7800 |
| H1:15 | Top-line data from the confirmatory Phase III trial of ataluren in nmDMD |
| H2:15 | FDA and MAA filing for full approval of ataluren for nmDMD |
| H2:15 | Top-line data from the confirmatory Phase III trial of ataluren in nmCF |
| 2015 | Potential first commercial (named patient/compassionate use) sales of ataluren nmDMD in EU (or ROW) markets |
| 2015 | Potential initiation of pivotal Phase II/III trials RG7800 in patients with SMA |
| H1:16 | FDA and MAA filing for full approval of ataluren for nmCF |
| 2016 | First commercial sales of ataluren following potential approval on Phase III data |

## *Isis's SMNrx – April Data Update*

The data show that SMNrx treatment resulted in a mean increase of 8.3 points on the CHOP-INTEND motor function test in infants at 3 months compared to a natural history study indicating an average decline of 1.27 points / year. 6/7 subjects had a ≥5 point increase, 1 pt had a small (~1 pt) decrease.

In 7 infants evaluable for efficacy in the 12 mg cohort, just 2 (29%) have had a death/ventilation event at a median age at death/ventilation or April 7 (cut-off date) of 9.6 months. This qualitatively compares to 40-50% death/ventilation rate by 9.5 months in a previous natural history study in infants with 2 copies of SMN2. We highlight several differences between the natural history study and the SMNrx trial including differences in copy number: 2 in a subset of the natural history study, a mix in the SMNrx trial; care received: the natural history study included patients who only received palliative care, the SMNrx trial used more aggressive interventions; and the very small number of patients in the SMNrx trial. The death/ventilation endpoint is either death or ≥ 16 hours/day of ventilation continuously for > 2 weeks without an acute reversible illness.

In 18 children with SMA, SMNrx treatment resulted in a mean increase of 2.5 points on the HFMSE scale at 9 months. This compares to natural history data indicating a 0.34 point decline in the first 2 years and a 1.26 point decline in 3 years after screening. Increases on SMNrx were both dose and time dependent.

We believe that ISIS's results validate SMN2 splicing as a target in SMA, while highlighting the advantages of PTC/Roche's oral compound, RG7800. Both compounds increase the inclusion of exon 7 in the SMN2 transcript, which is normally excised in ~90% of SMN2 transcripts. The inclusion of exon 7 produces normal SMN protein. ISIS will run a sham-procedure controlled pivotal trial, because ISIS cannot ethically perform a spinal tap procedure on an infant to deliver placebo.

We note that PTCT/Roche's SMA Candidate RG7800 demonstrated a nearly 3-fold increase in SMN protein levels in the brain in early animal models, enough to nearly achieve healthy heterozygote protein levels. Although slightly lower than the levels caused by ISIS-SMNRX in preclinical models, this is encouraging data. We highlight that the ease and safety of systemic delivery of RG7800 may be preferential the intrathecally delivered SMNrx and may better facilitate delivery very early in life, a time during which animal models of SMA suggest maximum therapeutic benefit may be conferred.

## *Additional Indications Could Expand the Market for Ataluren*

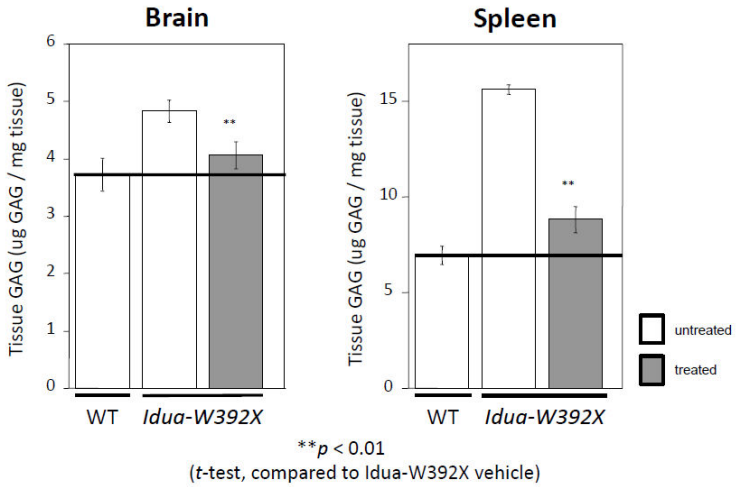
**Figure 1: Additional Indications Under Study**

| Author | Indication |
|---|---|
| Wang (2010) | Miyoshi myopathy |
| Yu (2014) | Long QT syndrome |
| Sarker (2011) | Infantile neuronal ceroid lipofuscinosis (INCL) |
| Miller (2013) | (Late) infantile neuronal ceroid lipofuscinosis (INCL) |
| Du (2013) | Ataxia telangiectasia |
| Goldman (2011, 2012) | Usher syndrome (USCH1C) |
| Zhou (2013) | Pseudoxanthoma elasticum |
| Kuschal (2013) | Xeroderma pigmentosum |
| Moosajee (unpublished) | Choroideremia |
| Gregory-Evans (2014) | Aniridia |
| Drake (2013) | Heritable pulmonary arterial hypertension |
| Tan (2011) | Carnitine palmitoyltransferase 1A deficiency |
| Buck (2012) | Methylmalonic aciduria (MMA) |
| Sanchez-Alcudia (2012) | Propionic acidemia (PA) |
| Bartolomeo (2013) | Maroteaux-Lamy syndrome (MPS VI) |
| Keeling (unpublished) | Hurler's syndrome (MPS I) |

*Source: Company data, Wedbush Securities, Inc.*

Hurler's syndrome (MPS I) is a severe disease caused by a lack of lysosomal α-L-iduronidase which helps break down glycosaminoglycans (GAG's). GAG's accumulate and cause organ damage, particularly in the heart and brain. In mouse models, ataluren inhibits the accumulation of GAG's in both the brain and the spleen.

**Figure 2: Ataluren has a Promising Effect in Mouse Models of Hurler's Syndrome**




*Source: Company data, Wedbush Securities, Inc.*

Hurler's syndrome is treated with bone marrow transplants (BMT) and Aldurazyme (Genzyme/BioMarin), an IV-delivered enzyme replacement therapy approved on a single 26-week pivotal trial enrolling 45 patients with endpoints of FVC and 6MWT. Naglazyme (BioMarin) is a similar IV delivered enzyme replacement therapy also approved on a small pivotal trial in MPS VI.

**Figure 3: Clinical Trial's Submitted with BLA Applications for Aldurazyme (MPS I) and Naglazyme (MPS VI)**

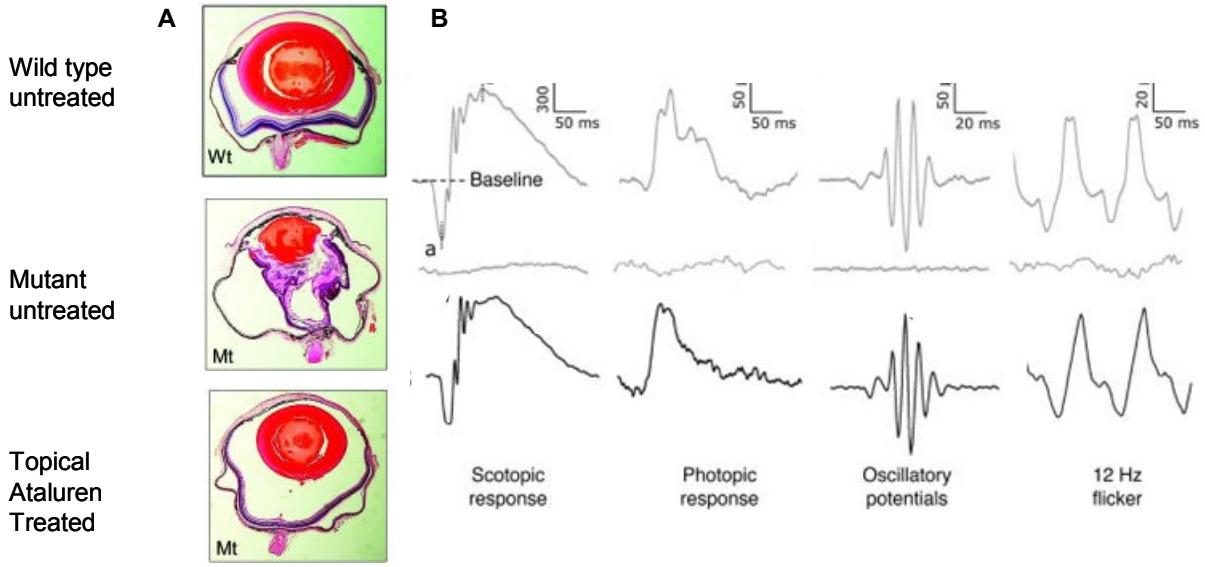
| | N | Length | Primary Endpoints | Design | NCT |
|---|---|---|---|---|---|
| Aldurazyme Phase I/II | 10 | 2 years (extended) | Urinary GAG excretion, liver and spleen size | Open-label | NCT01173016 |
| Aldurazyme pivotal | 45 (1:1) | 26 weeks | FVC and 6MWT | RCT | NCT00912925 |
| Aldurazyme long-term extension | 45 | 24 weeks | FVC and 6MWT | Open-label | NCT00146770 |
| Naglazyme Phase I/II | 7 | 24 weeks | Urinary GAG secretion, 6MWT | Open-label | NCT00048620 |
| Naglazyme Phase II | 10 | 48 weeks | Urinary GAG secretion, 12MWT | open-label | NCT00048711 |
| Naglazyme pivotal | 39 (1:1) | 24 weeks | 12MWT | RCT | NCT00067470 |

*Source: Company data, Wedbush Securities, Inc.*

Aniridia is an eye disorder characterized by complete or partial absence of the iris caused by mutations in the *PAX6* gene. PAX6 protein is involved in the early development of the eyes, brain, spinal cord and pancreas. Without PAX6, patients develop involuntary eye movements and underdevelopment of the fovea, responsible for sharp central vision.

Gregory-Evans et al. (2014) investigated the use of ataluren in a mouse model with a nonsense mutation in the *PAX6* gene. Mutant mouse eyes demonstrated significant deformation and ERG abnormalities. A topical formulation of ataluren dubbed, START, was developed to increase viscosity and thus surface contact time and reduce the irritation associated with water based eye drops. START application was initiated 14 days after birth (when the eyes open), and continued until sacrifice and analysis at day 60. Ataluren treatment normalizes eye histology (Figure 4A) and nerve response as assessed by ERG after light pulse (Figure 4B) and brings the amplitude of the low-light (scotopic) and high light (photopic) responses up to levels not significantly different from wild type mice.

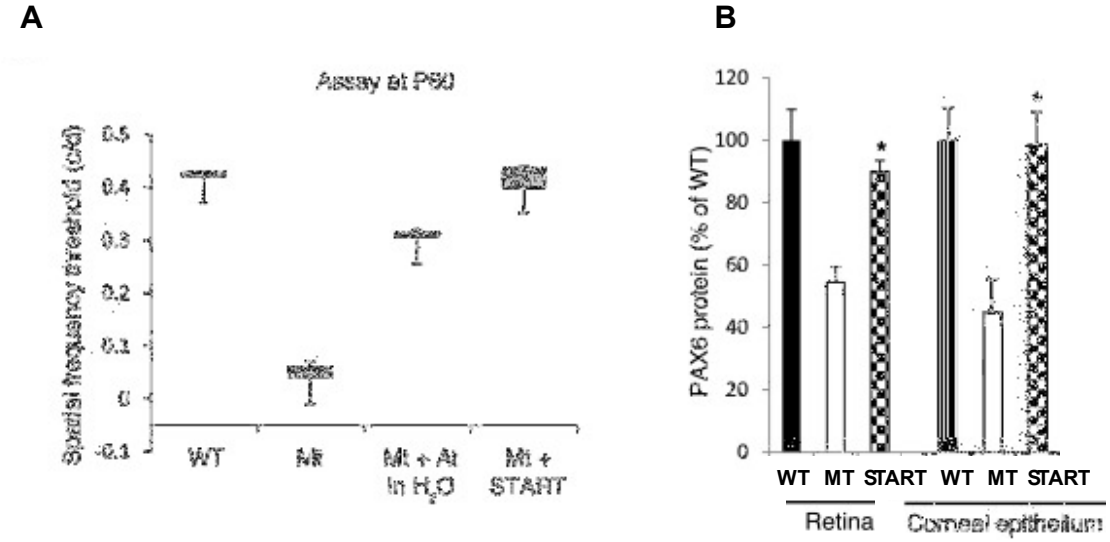
**Figure 4: Ataluren Normalizes Nerve Firing in a Mouse Model of Nonsense Mutation Aniridia**




*Source: Gregory-Evans et al. J Clin Invest. Jan 2, 2014; 124(1): 111–116.*

The researchers measured optokinetic tracking response in a virtual reality system to approximate visual acuity. Ataluren treated mice had much better tracking response than mutant (Mt) or ataluren in water treated mice (Figure 5A) and were not significantly different form responses seen in wild type animals (p = 0.42). Mice treated with Ataluren had increased levels of PAX6 protein in the retina and corneal epithelium (Figure 5B).

**Figure 5: Ataluren Increased Nerve Amplitude and PAX6 Protein Expression**




*Source: Gregory-Evans et al. J Clin Invest. Jan 2, 2014; 124(1): 111–116.*

Aniridia is a rare disease with no current treatment options. It could potentially be approved on a single pivotal trial, supported by PTC's large safety database. Although an optical formulation is technically not comparable to systemic formulation, it is reasonable to propose that an optical formulation with low systemic exposure would have a lower safety risk than a systemic administration. We highlight previous trials in rare eye disorders, though we note that Ligneous Conjunctivitis is an extremely rare disease with roughly 200 cases ever reported.

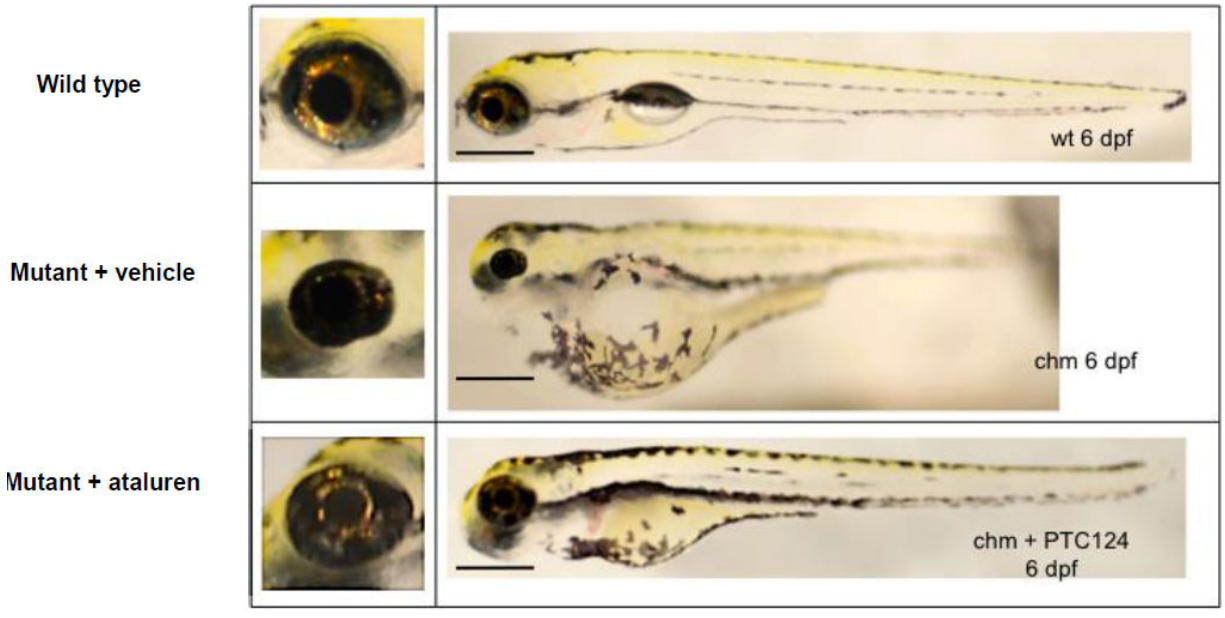
**Figure 6: Current Trials in Rare Eye Disorders Highlight a Path Forward for Ataluren in Aniridia or Choroideremia**

| | Gene Therapy Trial in Choroideremia | Human Plasminogen in Ligneous Conjunctivitis |
|---|---|---|
| **Phase** | Phase I | Phase III |
| **N** | 12 | 10 |
| **Efficacy Length** | 6 months | 12 weeks |
| **Safety Length** | 24 months | 26 weeks |
| **Primary Endpoint** | Visual acuity | # of eyes with recurrent ligneous membranes, # of eyes with reduction of overall membrane surface area |
| **Secondary endpoints** | Microperimetry, OCT and fundus autofluorescence | Safety |
| **Inclusion criteria** | Vision 6/60 or better in the study eye, male aged ≥18 years, diagnosed with choroideraemia | Diagnosed with ligneous conjunctivitis associated with Type I plasminogen deficiency |
| **Status** | Ongoing | Ongoing |
| **NCT** | NCT02077361 | NCT01554956 |

*Source: Company data, Wedbush Securities, Inc.*

Choroideremia is an X-linked disease caused by mutations in the *CHM* gene leading to progressive vision loss. Vision problems are due to an ongoing loss of cells in the retina and nearby network of blood vessels leading to complete blindness in late adulthood. Ataluren normalized eye morphology in a zebrafish model.

**Figure 7: Ataluren Normalized Eye Morphology in Nonsense Mutation Choroideremia**




*Source: Company data, Wedbush Securities, Inc.*

## *Financial Model*

WEDBUSH
PACGROW LIFE SCIENCES

**Christopher N. Marai Ph.D.**

5/7/2014

### PTC Therapeutics, Inc.

Annual Financial Results & Projections
($ in thousands except per share data)
Ticker: PTCT (Nasdaq)

| | FY:13A | Q1:14E | Q2:14E | Q3:14E | Q4:14E | FY:14E | FY:15E | FY:16E | FY:17E | FY:18E | FY:19E |
|---|---|---|---|---|---|---|---|---|---|---|---|
| Revenue: | | | | | | | | | | | |
| Ataluren Sales US - DMD | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 49,618 | 216,553 | 313,599 | 335,577 |
| Ataluren Sales EU - DMD | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 35,543 | 185,637 | 310,494 | 345,451 |
| Ataluren Sales ROW - DMD | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 715 | 10,692 | 47,266 | 93,713 |
| Ataluren Sales US - CF | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 94,984 | 267,911 | 344,683 |
| Ataluren Sales EU - CF | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 37,354 | 185,142 | 367,238 |
| Ataluren Sales ROW - CF | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 1,010 | 12,187 |
| Grant and other revenues | 3,370 | 70 | 1,000 | 1,000 | 1,000 | 3,070 | 4,000 | 0 | 0 | 0 | 0 |
| Collaboration revenue | 31,326 | 9,147 | 0 | 0 | 0 | 9,147 | 0 | 1,000 | 1,000 | 1,000 | 1,000 |
| Total Revenues | **$34,696** | **$9,217** | **$1,000** | **$1,000** | **$1,000** | **$12,217** | **$4,000** | **$86,876** | **$546,221** | **$1,126,423** | **$1,499,849** |
| Cost and Expenses: | | | | | | | | | | | |
| Costs of goods sold | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 8,588 | 54,522 | 112,542 | 149,885 |
| Research and Development | 54,875 | 15,889 | 15,250 | 15,500 | 16,000 | 62,639 | 59,500 | 62,305 | 66,497 | 67,837 | 69,204 |
| Sales, General and Administrative | 25,219 | 7,540 | 7,540 | 7,540 | 7,540 | 30,160 | 31,500 | 43,000 | 52,000 | 53,313 | 55,478 |
| Other | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 |
| Total Costs and Expenses | **$80,094** | **$23,429** | **$22,790** | **$23,040** | **$23,540** | **$92,799** | **$91,000** | **$113,893** | **$173,019** | **$233,692** | **$274,566** |
| Operating Income (loss) | (45,398) | (14,212) | (21,790) | (22,040) | (22,540) | (80,582) | (87,000) | (27,017) | 373,202 | 892,731 | 1,225,283 |
| Net Interest Income (Expense) | (6,083) | 171 | 740 | 676 | 612 | 2,199 | 2,049 | 2,577 | 3,834 | 9,769 | 19,188 |
| Other income / (Expense) | (92) | (57) | 0 | 0 | 0 | (57) | 0 | 0 | 0 | 0 | 0 |
| Income Before Income Taxes | (51,574) | (14,098) | (21,050) | (21,364) | (21,928) | (78,440) | (84,951) | (24,439) | 377,036 | 902,500 | 1,244,470 |
| Net Income | **($51,574)** | **($14,098)** | **($21,050)** | **($21,364)** | **($21,928)** | **($78,440)** | **($84,951)** | **($24,439)** | **$351,377** | **$708,087** | **$933,353** |
| GAAP Net Income | **($66,432)** | **($14,098)** | **($21,050)** | **($21,364)** | **($21,928)** | **($78,440)** | **($84,951)** | **($24,439)** | **$341,377** | **$698,087** | **$923,353** |
| GAAP Basic EPS with sFAS123 | **(5.18)** | **(0.58)** | **(0.70)** | **(0.71)** | **(0.73)** | **(2.73)** | **(2.72)** | **(0.76)** | **10.83** | **21.76** | **28.60** |
| GAAP Diluted EPS with sFAS123 | **(5.29)** | **(0.58)** | **(0.70)** | **(0.71)** | **(0.73)** | **(2.73)** | **(2.72)** | **(0.76)** | **10.83** | **21.76** | **28.60** |
| Weighted shares outstanding | 12,829 | 24,492 | 30,127 | 30,152 | 30,177 | 24,492 | 31,240 | 32,340 | 32,440 | 32,540 | 32,640 |
| Fully diluted shares outstanding | 12,565 | 24,492 | 30,127 | 30,152 | 30,177 | 28,737 | 31,240 | 32,340 | 32,440 | 32,540 | 32,640 |
| Cash Burn | (51,574) | (14,098) | (21,050) | (21,364) | (21,928) | (78,440) | (84,951) | (24,439) | - | - | - |
| Cash Balance | 142,468 | 246,585 | 225,425 | 204,061 | 182,133 | 182,133 | 178,623 | 238,622 | 574,123 | 1,267,424 | 2,193,489 |

*Source:Wedbush Securities and PacGrow Life Sciences*

***Analyst Biography***

*Chris Marai is an Analyst covering the Biotechnology/Biopharmaceuticals/BioDefense sector. Prior to Wedbush PacGrow Life Sciences, Dr. Marai was at Morgan Stanley where he specialized in quantitative modeling; he has also consulted for structure-based drug design companies and biotechnology startups.*
*Dr. Marai holds a B.S. in Chemistry from Trinity College, University of Toronto and a Ph.D. in Biochemistry and Structural Biology from Stony Brook University, New York.*
*Christopher's Edge: Dr. Marai has a strong quantitative background and has covered a wide range of disease areas including gastrointestinal, CNS, oncology and rare diseases. His quantitative background has translated into an exceptional track record predicting binary events and assessing risk as a sell-side analyst.*

**Analyst Certification**
I, Christopher N. Marai, Ph.D., Gregory R. Wade, Ph.D., David M. Nierengarten, Ph.D., 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.

Disclosure information regarding historical ratings and price targets is available at http://www.wedbush.com/ResearchDisclosure/DisclosureQ114.pdf

**Investment Rating System:**
Outperform: Expect the total return of the stock to outperform relative to the median total return of the analyst's (or the analyst's team) coverage universe over the next 6-12 months.
Neutral: Expect the total return of the stock to perform in-line with the median total return of the analyst's (or the analyst's team) coverage universe over the next 6-12 months.
Underperform: Expect the total return of the stock to underperform relative to the median total return of the analyst's (or the analyst's team) coverage universe over the next 6-12 months.

The Investment Ratings are based on the expected performance of a stock (based on anticipated total return to price target) relative to the other stocks in the analyst's coverage universe (or the analyst's team coverage).*

| **Rating Distribution (as of March 31, 2014)** | **Investment Banking Relationships (as of March 31, 2014)** |
|---|---|
| Outperform:54%<br>Neutral: 43%<br>Underperform: 3% | Outperform:22%<br>Neutral: 2%<br>Underperform: 0% |

The Distribution of Ratings is required by FINRA rules; however, WS' stock ratings of Outperform, Neutral, and Underperform most closely conform to Buy, Hold, and Sell, respectively. Please note, however, the definitions are not the same as WS' stock ratings are on a relative basis.

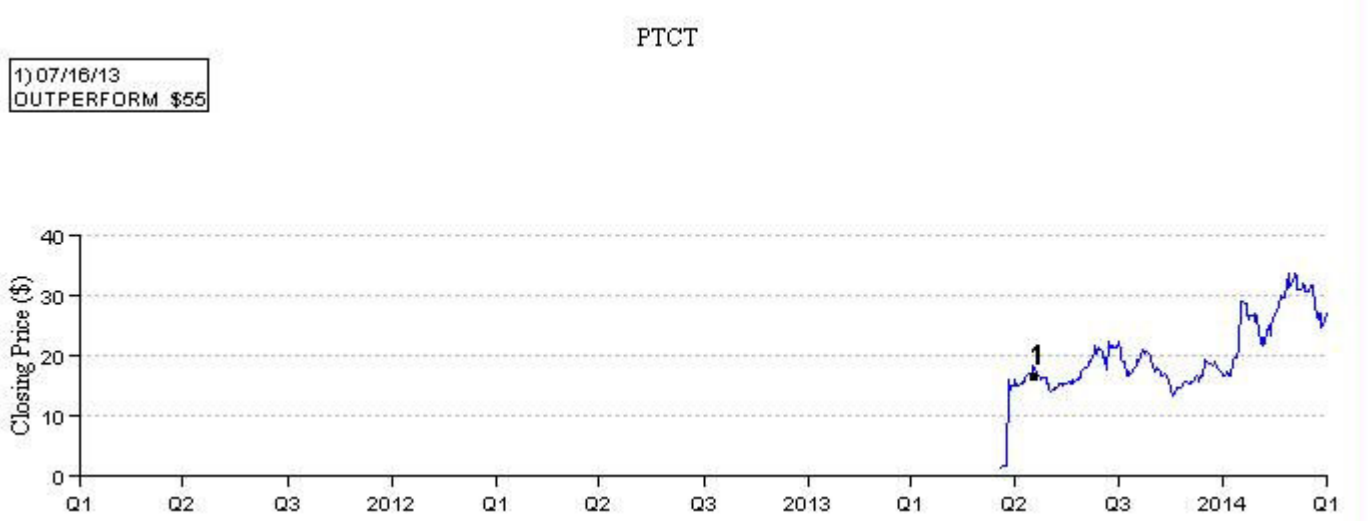
The analysts responsible for preparing research reports do not receive compensation based on specific investment banking activity. The analysts receive compensation that is based upon various factors including WS' total revenues, a portion of which are generated by WS' investment banking activities.

**Wedbush Equity Research Disclosures as of May 7, 2014**

| Company | Disclosure |
|---|---|
| PTC Therapeutics | 1,3,4,5,7 |

**Research Disclosure Legend**
1. WS makes a market in the securities of the subject company.
2. WS managed a public offering of securities within the last 12 months.
3. WS co-managed a public offering of securities within the last 12 months.
4. WS has received compensation for investment banking services within the last 12 months.
5. WS provided investment banking services within the last 12 months.
6. WS is acting as financial advisor.
7. WS expects to receive compensation for investment banking services within the next 3 months.
8. WS provided non-investment banking securities-related services within the past 12 months.
9. WS has received compensation for products and services other than investment banking services within the past 12 months.
10. The research analyst, a member of the research analyst's household, any associate of the research analyst, or any individual directly involved in the preparation of this report has a long position in the common stocks.
11. WS or one of its affiliates beneficially own 1% or more of the common equity securities.
12. The analyst maintains Contingent Value Rights that enables him/her to receive payments of cash upon the company's meeting certain clinical and regulatory milestones.

**Price Charts**
Wedbush disclosure price charts are updated within the first fifteen days of each new calendar quarter per FINRA regulations. Price charts for companies initiated upon in the current quarter, and rating and target price changes occurring in the current quarter, will not be displayed until the following quarter. Additional information on recommended securities is available on request.




* WS changed its rating system from (Strong Buy/Buy/Hold/Sell) to (Outperform/ Neutral/Underperform) on July 14, 2009.
Please access the attached hyperlink for WS' Coverage Universe: http://www.wedbush.com/services/cmg/equities-division/research/equity-research Applicable disclosure information is also available upon request by contacting Ellen Kang in the Research Department at (213) 688-4529, by email to ellen.kang@wedbush.com, or the Business Conduct Department at (213) 688-8090. You may also submit a written request to the following: Business Conduct Department, 1000 Wilshire Blvd., Los Angeles, CA 90017.

---

## OTHER DISCLOSURES

**RESEARCH DEPT. * (213) 688-4505 * www.wedbush.com**
EQUITY TRADING Los Angeles (213) 688-4470 / (800) 421-0178 * EQUITY SALES Los Angeles (800) 444-8076
CORPORATE HEADQUARTERS (213) 688-8000

The information herein is based on sources that we consider reliable, but its accuracy is not guaranteed. The information contained herein is not a representation by this corporation, nor is any recommendation made herein based on any privileged information. This information is not intended to be nor should it be relied upon as a complete record or analysis; neither is it an offer nor a solicitation of an offer to sell or buy any security mentioned herein. This firm, Wedbush Securities, its officers, employees, and members of their families, or any one or more of them, and its discretionary and advisory accounts, may have a position in any security discussed herein or in related securities and may make, from time to time, purchases or sales thereof in the open market or otherwise. The information and expressions of opinion contained herein are subject to change without further notice. The herein mentioned securities may be sold to or bought from customers on a principal basis by this firm. Additional information with respect to the information contained herein may be obtained upon request.

# WEDBUSH

## EQUITY RESEARCH DEPARTMENT

**(213) 688-4529**

**DIRECTOR OF RESEARCH**
Mark D. Benson (213) 688-4435

**MANAGER, RESEARCH OPERATIONS**
Ellen Kang (213) 688-4529

### RETAIL AND CONSUMER

**Consumer Products**
Rommel T. Dionisio (212) 938-9934
Alicia Reese (212) 938-9927

**Footwear, Apparel and Accessories**
Corinna Freedman (212) 668-9876

**Healthy Lifestyles**
Kurt M. Frederick, CFA CPA (415) 274-6822
Alicia Reese (212) 938-9927

**Restaurants**
Nick Setyan (213) 688-4519
Colin Radke (213) 688-6624

**Specialty Retail: Hardlines**
Joan L. Storms, CFA (213) 688-4537
John Garrett, CFA (213) 688-4523

Seth Basham, CFA (212) 938-9954

**Specialty Retail: Softlines**
Morry Brown (213) 688-4311
Taryn Kuida (213) 688-4505

### RETAIL/CONSUMER MARKET RESEARCH

Gabriella Santaniello (213) 688-4557

### INDUSTRIAL GROWTH TECHNOLOGY

**Clean Technology**
Craig Irwin (212) 938-9926

**Environmental Services / Building Products**
Al Kaschalk (213) 688-4539

**Water and Renewable Energy Solutions**
David Rose, CFA (213) 688-4319
James Kim (213) 688-4380

### TECHNOLOGY, INTERNET, MEDIA & SOCIAL MEDIA

**Communications and Application Software**
Shyam Patil, CFA (213) 688-8062
Andy Cheng (213) 688-4548

**Enterprise Security**
Sanjit Singh (415) 273-7323

**Computer Services: Financial Technology**
Gil B. Luria (213) 688-4501
Aaron Turner (213) 688-4429

**Enterprise Software**
Steve Koenig (415) 274-6801
Kevin Ikeda (213) 688-4423

**Entertainment: Retail**
Michael Pachter (213) 688-4474
Nick McKay (213) 688-4343
Nick Citrin (213) 688-4495

**Entertainment: Software**
Michael Pachter (213) 688-4474
Nick McKay (213) 688-4343
Nick Citrin (213) 688-4495

**Internet: Media and Gaming**
Michael Pachter (213) 688-4474
Nick McKay (213) 688-4343
Nick Citrin (213) 688-4495

**Internet: Social Media, Advertising & Technology**
Shyam Patil, CFA (213) 688-8062
Andy Cheng (213) 688-4548

**Media**
James Dix, CFA (213) 688-4315

**Movies and Entertainment**
Michael Pachter (213) 688-4474
Nick McKay (213) 688-4343
Nick Citrin (213) 688-4495

**Semiconductors**
Betsy Van Hees (415) 274-6869
Ryan Jue, CFA (415) 263-6669

### LIFE SCIENCES AND HEALTH CARE

**Biotechnology/Biopharmaceuticals/BioDefense**
Gregory R. Wade, Ph.D. (415) 274-6863
David M. Nierengarten, Ph.D. (415) 274-6862
Christopher N. Marai, Ph.D. (415) 274-6861
Dilip Joseph (415) 273-7308

**Emerging Pharmaceuticals**
Liana Moussatos, Ph.D. (415) 263-6626

**Healthcare Services - Managed Care**
Sarah James (213) 688-4503

**Medical Devices**
Tao Levy (212) 938-9948

**Medical Diagnostics and Life Sciences Tools**
Zarak Khurshid (415) 274-6823

| EQUITY SALES | | EQUITY TRADING | |
|---|---|---|---|
| Los Angeles | (213) 688-4470 / (800) 444-8076 | Los Angeles | (213) 688-4470 / (800) 421-0178 |
| San Francisco | (415) 274-6800 | San Francisco | (415) 274-6811 |
| New York | (212) 938-9931 | New York | (212) 344-2382 |
| Boston | (617) 832-3700 | Boston | (617) 832-3700 |

**CORPORATE HEADQUARTERS**
1000 Wilshire Blvd., Los Angeles, CA 90017-2465
Tel: (213) 688-8000 www.wedbush.com