


**UNITED STATES DISTRICT COURT
DISTRICT OF NEW JERSEY**

 Individually and on Behalf
of All Others Similarly Situated,

Plaintiff,

v.

PTC THERAPEUTICS, INC., STUART
PELTZ, and SHANE KOVACS,

Defendants.

Civil Action No.:

**CLASS ACTION COMPLAINT FOR
VIOLATIONS OF THE FEDERAL
SECURITIES LAWS**

JURY TRIAL DEMANDED

Plaintiff [REDACTED] (“Plaintiff”), by and through his attorneys, alleges the following upon information and belief, except as to those allegations concerning Plaintiff, which are alleged upon personal knowledge. Plaintiff’s information and belief is based upon, among other things, his counsel’s investigation, which includes without limitation: (a) review and analysis of regulatory filings made by PTC THERAPEUTICS, INC. (“PTC Therapeutics” or the “Company”), with the United States (“U.S.”) Securities and Exchange Commission (“SEC”); (b) review and analysis of press releases and media reports issued by and disseminated by PTC Therapeutics; and (c) review of other publicly available information concerning PTC Therapeutics.

NATURE OF THE ACTION AND OVERVIEW

1. This is a class action on behalf of purchasers of PTC Therapeutics securities between May 6, 2014 and February 23, 2016, inclusive (the “Class Period”), seeking to pursue remedies under the Securities Exchange Act of 1934 (the “Exchange Act”).

2. PTC Therapeutics is a biopharmaceutical company focused on the discovery, development and commercialization of orally administered, small molecule therapeutics targeting an area of RNA biology the Company refers to as post-transcriptional control. The Company’s lead product is “Translarna,” which is an oral, protein restoration therapy for the treatment of nonsense mutation Duchenne muscular dystrophy (“nmDMD”).

3. On February 23, 2016, the Company issued a press release entitled “PTC Receives Refuse to File Letter from FDA for Translarna™ (ataluren).” Therein, the Company stated that it received a Refuse to File letter from the United States Food and Drug Administration (“FDA”) regarding the Company’s New Drug Application (“NDA”) for Translarna because “the application was not sufficiently complete to permit a substantive

review.”

4. On this news, shares of PTC Therapeutics fell \$17.42 per share, or more than 61%, to close at \$10.84 per share on February 23, 2016, on unusually heavy trading volume.

5. Throughout the Class Period, Defendants made false and/or misleading statements, as well as failed to disclose material adverse facts about the Company’s business, operations, and prospects. Specifically, Defendants made false and/or misleading statements and/or failed to disclose: (1) that the Company’s NDA for Translarna that it submitted to the FDA was not sufficiently complete to permit a substantive review of the application; (2) that, as such, the application would not be reviewed nor approved by the FDA; (3) that the impending non-approval of the NDA would have a negative material impact on the Company’s operations and prospects; and (4) that, as a result of the foregoing Defendants’ statements about PTC Therapeutics’ business, operations, and prospects, were false and misleading and/or lacked a reasonable basis.

6. As a result of Defendants’ wrongful acts and omissions, and the precipitous decline in the market value of the Company’s securities, Plaintiff and other Class members have suffered significant losses and damages.

JURISDICTION AND VENUE

7. The claims asserted herein arise under Sections 10(b) and 20(a) of the Exchange Act (15 U.S.C. §§78j(b) and 78t(a)) and Rule 10b-5 promulgated thereunder by the SEC (17 C.F.R. § 240.10b-5).

8. This Court has jurisdiction over the subject matter of this action pursuant to 28 U.S.C. §1331 and Section 27 of the Exchange Act (15 U.S.C. §78aa).

9. Venue is proper in this Judicial District pursuant to 28 U.S.C. §1391(b) and

Section 27 of the Exchange Act (15 U.S.C. §78aa(c)). Substantial acts in furtherance of the alleged fraud or the effects of the fraud have occurred in this Judicial District. Many of the acts charged herein, including the dissemination of materially false and/or misleading information, occurred in substantial part in this Judicial District. In addition, the Company's principal executive offices are located within this Judicial District.

10. In connection with the acts, transactions, and conduct alleged herein, Defendants directly and indirectly used the means and instrumentalities of interstate commerce, including the United States mail, interstate telephone communications, and the facilities of a national securities exchange.

PARTIES

11. Plaintiff [REDACTED] as set forth in the accompanying certification, incorporated by reference herein, purchased PTC Therapeutics common stock during the Class Period, and suffered damages as a result of the federal securities law violations and false and/or misleading statements and/or material omissions alleged herein.

12. Defendant PTC Therapeutics is a Delaware corporation with its principal executive offices located at 100 Corporate Court, South Plainfield, New Jersey 07080.

13. Defendant Stuart Peltz ("Peltz") was, at all relevant times, Chief Executive Officer ("CEO") and a Director of PTC Therapeutics.

14. Defendant Shane Kovacs ("Kovacs") was, at all relevant times, Chief Financial Officer ("CFO") of PTC Therapeutics.

15. Defendants Peltz and Kovacs are collectively referred to hereinafter as the "Individual Defendants." The Individual Defendants, because of their positions with the Company, possessed the power and authority to control the contents of PTC Therapeutics'

reports to the SEC, press releases and presentations to securities analysts, money and portfolio managers and institutional investors, *i.e.*, the market. Each defendant was provided with copies of the Company's reports and press releases alleged herein to be misleading prior to, or shortly after, their issuance and had the ability and opportunity to prevent their issuance or cause them to be corrected. Because of their positions and access to material non-public information available to them, each of these defendants knew that the adverse facts specified herein had not been disclosed to, and were being concealed from, the public, and that the positive representations which were being made were then materially false and/or misleading. The Individual Defendants are liable for the false statements pleaded herein, as those statements were each "group-published" information, the result of the collective actions of the Individual Defendants.

SUBSTANTIVE ALLEGATIONS

Background

16. PTC Therapeutics is a biopharmaceutical company focused on the discovery, development and commercialization of orally administered, small molecule therapeutics targeting an area of RNA biology the Company refers to as post-transcriptional control. The Company's lead product is "Translarna," which is an oral, protein restoration therapy for the treatment of nmDMD.

Materially False and Misleading Statements Issued During the Class Period

17. The Class Period Begins on May 6, 2014. On that day, PTC Therapeutics issued a press release entitled, "PTC Therapeutics Reports First Quarter Financial Results and Provides Corporate Update." Therein, the Company, in relevant part, stated:

SOUTH PLAINFIELD, NJ — May 6, 2014 — PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported its financial results for the first quarter ended March 31, 2014.

"We are happy to report on the progress we have made in the first quarter of 2014

on each of the clinical, regulatory and corporate fronts. Our successful follow-on financing provides a strong foundation to advance our clinical programs and further PTC's pipeline of product candidates," stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "As we look ahead, we are excited to complete enrollment in our confirmatory Phase 3 ACT DMD clinical trial in nonsense mutation Duchenne muscular dystrophy, initiate our confirmatory Phase 3 clinical trial in nonsense mutation cystic fibrosis, and advance our SMA program, partnered with Roche and the SMA Foundation. We also plan to pursue additional indications for ataluren beyond nmDMD and nmCF and expect to initiate a Phase 2 proof-of-concept study for an additional indication in the second half of this year."

Corporate Highlights:

- **ACT DMD:** In April 2013, PTC initiated the confirmatory Phase 3 ACT DMD (Ataluren Confirmatory Trial in DMD) evaluating ataluren as a potential treatment for patients with nonsense mutation Duchenne muscular dystrophy (nmDMD). This study, which is the largest clinical study ever conducted in DMD, is on-track to complete enrollment by mid-2014.
- **ACT CF:** We are in the process of initiating the confirmatory Phase 3 ACT CF clinical trial. The design of our confirmatory Phase 3 trial is similar to the previous Phase 3 trial, with FEV1 as the primary endpoint and pulmonary exacerbations as a key secondary endpoint. The main change from the prior trial will be to exclude patients from enrolling who are using chronic inhaled tobramycin. Tobramycin is a ribosome binding drug that has been shown to confound ataluren's activity.
- **Additional Indications:** There have now been 20 publications demonstrating ataluren's activity in a number of different disease models. We plan to initiate a Phase 2 proof-of-concept clinical trial for ataluren in an additional indication in the second half of 2014.
- **EMA regulatory process:** In January 2014, the CHMP adopted a negative opinion regarding our application for the conditional marketing authorization of ataluren for the treatment of nmDMD. PTC has requested a re-examination of the CHMP opinion. A final decision from the CHMP is expected in the second quarter of 2014.
- **SMA Program Entered Clinical Development:** In January 2014, a Phase 1 clinical program was initiated which triggered a \$7.5 million milestone payment from Roche to PTC. The SMA Phase 1 clinical program is complemented by ongoing natural history and biomarker observational studies in SMA patients.

- **Follow-on Financing:** In February, we closed a registered public offering of common stock, in which we raised gross proceeds of \$126.5 million, before deducting underwriting discounts and commissions and other offering expenses.

First Quarter 2014 Financial Highlights:

- Cash, cash equivalents, and marketable securities totaled \$246.6 million at March 31, 2014 compared to \$142.5 million at December 31, 2013. The increase was primarily driven by net proceeds of \$118.2 million from PTC's follow-on equity financing as well as a \$7.5 million milestone achieved in our Roche collaboration.
- Revenue from grants and collaborations was \$9.2 million for the first quarter of 2014, compared to \$7.1 million for the same period in 2013.
- Total operating expenses were \$23.4 million for the first quarter of 2014, including \$3.7 million in non-cash, stock based compensation expense, compared to \$15.7 million for the same period in 2013, including \$0.6 million in non-cash, stock based compensation expense. The increase primarily results from additional costs associated with clinical trials, pre-commercial activities and public company expenses.
- Net loss for the first quarter of 2014 was \$14.1 million compared to a net loss of \$14.7 million for the same period in 2013.

18. On the same day, PTC Therapeutics filed its Quarterly Report with the SEC on Form 10-Q for the fiscal quarter ended March 31, 2014. The Company's Form 10-Q was signed by Defendant Kovacs, and reaffirmed the Company's financial results previously announced earlier that day.

19. On August 7, 2014, PTC Therapeutics issued a press release entitled, "PTC Therapeutics Reports Second Quarter Financial Results and Provides Corporate Update." Therein, the Company, in relevant part, stated:

SOUTH PLAINFIELD, NJ — August 7, 2014 — PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the second quarter ended June 30, 2014.

"This has been an exciting quarter for PTC. In May we received a positive opinion from the CHMP for marketing approval for Translarna™ (ataluren) an

investigational new drug in the US, for nonsense mutation Duchenne muscular dystrophy and we recently received the approval from the European Commission granting the conditional marketing authorization. It is an honor to bring the world's first therapy for Duchenne muscular dystrophy to patients who have been waiting too long for a treatment. We are actively focused on providing Translarna to patients as quickly as possible and are working aggressively to prepare for launch across the EU," stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "In addition to our efforts in nmDMD, we recently initiated our confirmatory Phase 3 clinical trial in nonsense mutation cystic fibrosis and it is our goal to initiate a Phase 2 proof-of-concept study for Translarna in a new indication, MPS I caused by a nonsense mutation, later this year. We expect the second half of the year will be a transformative time at PTC, as we expand our global commercial organization focused on our mission of bringing new therapies to patients with rare and neglected disorders."

Corporate Highlights:

- **ACT DMD:** The confirmatory Phase 3 ACT DMD (Ataluren Confirmatory Trial in DMD) trial of Translarna™ (ataluren) in patients with nonsense mutation Duchenne muscular dystrophy (nmDMD) is well underway. Enrollment is expected to be completed near term with initial, top-line data available in the second half of 2015.
- **ACT CF:** In June, the confirmatory Phase 3 ACT CF clinical trial was initiated. This trial is a 48-week, double-blind, placebo-controlled global study with FEV1 as the primary endpoint and pulmonary exacerbations as a key secondary endpoint. The trial will enroll patients who have a confirmed nonsense mutation, who are six years of age or older and whose FEV1 is greater than 40% and less than 90% predicted. Patients who are using chronic inhaled aminoglycosides will not be eligible for the trial. Aminoglycosides are ribosome-binding drugs and have been shown to interfere with Translarna's activity. Enrollment is expected to be completed in the second half of 2015, with data expected a year later.
- **Additional indication:** Based on an evaluation process and in discussion with outside experts, PTC has selected mucopolysaccharidosis type I (MPS I) as the next indication to pursue for Translarna. It is PTC's goal to initiate a Phase 2 proof-of-concept study for MPS I in the second half of 2014. MPS I is an inherited genetic disorder caused by a deficiency in an essential enzyme that is responsible for the breakdown of by-products of chemical reactions in the body's cells. Globally, MPS I occurs in about 1 in every 100,000 births. It is estimated that 60-80% of MPS I patients have their disease as a result of a nonsense mutation. There is no cure for MPS I and enzyme replacement therapies do not sufficiently address the central nervous system, skeletal or cardiac symptoms associated with the disorder. Prognosis of patients with MPS I is poor and there is an urgent need for

the development of new treatments targeting the underlying cause of MPS I.

- **Regulatory update:** In May 2014, the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) adopted a positive opinion regarding the application for the conditional marketing authorization of Translarna for the treatment of nmDMD in ambulatory patients aged 5 years and older. On August 4th, PTC was notified that the European Commission ratified the CHMP's positive opinion and Translarna was granted conditional marketing approval in the EU. In addition to our efforts in the EU, PTC is engaging in further dialogue with the FDA to discuss potential pathways to accelerate bringing Translarna to US patients.
- **Commercialization Plans:** Commercial launch activities have been initiated to support the anticipated launch of Translarna in selected countries in the first half of 2015, subject to the completion of each country's market access process and timeline. Market access timelines vary from country to country and can take over eighteen months in certain countries.
- **EAP Program:** In June 2014, PTC initiated a reimbursed expanded access program for Translarna for nmDMD patients in selected territories. The EAP program is intended to make Translarna available to patients before commercial product becomes available in specific countries in accordance with local regulations. Funded Named Patient Programs have already been authorized in Turkey and Spain, and the French National Agency for Medicines and Health Products Safety (ANSM) has recently granted a Temporary Authorization for Use (Autorisation Temporaire d'Utilisation de cohort — ATU) of Translarna in a cohort of nmDMD patients. PTC has recently initiated the supply of Translarna to the first patients authorized under the EAP program.
- **SMA Program:** In January 2014, a Phase 1a single ascending dose, placebo-controlled clinical trial in healthy volunteers was initiated. The primary objectives of this trial were to explore safety and pharmacokinetics of the drug candidate, RG7800. This trial has now completed and a multiple dose clinical trial in SMA patients is currently in preparation. Preliminary findings in the Phase 1a study indicate that RG7800 was well-tolerated at all dose levels studied. There were no deaths, serious adverse events (SAEs) or withdrawals due to adverse events (AEs) and no dose-related trends were identified. Additionally, RG7800 demonstrated a dose-dependent effect on SMN2 splicing, as shown by a change in the ratio of full-length SMN2 mRNA to SMN2 mRNA without exon 7 (SMND7), which may be interpreted as proof of mechanism in terms of the expected pharmacodynamic effect.

Second Quarter 2014 Financial Highlights:

- Cash, cash equivalents, and marketable securities totaled \$226.9 million at June 30, 2014 compared to \$142.5 million at December 31, 2013.
- Revenue from grants and collaborations was \$1.7 million for the second quarter of 2014, compared to \$6.9 million for the same period in 2013. The decrease was due to a decrease in the recognition of non-cash deferred revenue compared to the same period in 2013.
- Research and development expenses were \$18.3 million for the second quarter of 2014, including \$2.2 million in non-cash, stock based compensation expense, compared to \$14.7 million for the same period in 2013, including \$1.1 million in non-cash, stock-based compensation expense. The increase primarily results from additional costs associated with clinical trials including the manufacturing of drug product for our clinical trials and regulatory costs associated with the efforts to obtain conditional approval for Translarna in Europe.
- General and administrative expenses were \$8.7 million for the second quarter of 2014, including \$2.1 million in non-cash stock based compensation expense, compared to \$6.6 million for the same period in 2013, including \$0.8 million in non-cash stock based compensation expense. The increase primarily results from additional costs associated with efforts to obtain conditional approval for Translarna in Europe, pre-commercial activities and public company costs.
- Net loss for the second quarter of 2014 was \$25.1 million compared to a net loss of \$14.6 million for the same period in 2013.
- Shares issued and outstanding as of June 30, 2014 were 30.1 million, which includes 0.7 million shares of unvested restricted stock.
- In conjunction with the European approval and ongoing commercial launch activities, PTC now expects total 2014 operating expenses to be between \$103 million and \$113 million, excluding approximately \$17 million in non-cash stock-based compensation. PTC expects to end 2014 with approximately \$160 million to \$170 million in cash, cash equivalents and marketable securities.

20. On the same day, PTC Therapeutics filed its Quarterly Report with the SEC on Form 10-Q for the fiscal quarter ended June 30, 2014. The Company's Form 10-Q was signed by Defendant Kovacs, and reaffirmed the Company's financial results previously announced

earlier that day.

21. On November 6, 2014, PTC Therapeutics issued a press release entitled, “PTC Therapeutics Reports Third Quarter Financial Results and Provides Corporate Update.” Therein, the Company, in relevant part, stated:

SOUTH PLAINFIELD, NJ — November 6, 2014 — PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the third quarter ended September 30, 2014.

“During the quarter we continued to make significant progress during this transformative time for PTC. For the first time in the company’s history, we recognized revenue from the sale of Translarna™ (ataluren) for the treatment of Duchenne muscular dystrophy through our European reimbursed early access programs. Furthermore, before the end of the year, we plan to begin submitting a New Drug Application with the FDA on a rolling basis for Translarna as a treatment for nonsense mutation Duchenne muscular dystrophy (nmDMD). We are committed to making Translarna available to all patients who may benefit and this is another step forward in achieving this goal,” stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. “We continued to expand our clinical efforts with the completion of enrollment for our confirmatory Phase 3 ACT DMD clinical trial, the initiation of our confirmatory Phase 3 ACT CF clinical trial, the preparation of a Phase 2 proof-of-concept study of Translarna in MPS I, and the anticipated initiation of a Phase 1b/2a clinical trial of RG7800 in spinal muscular atrophy patients in conjunction with our partners, Roche and the SMA Foundation. Our discovery efforts are also progressing with plans to file an investigational new drug application, or IND, with the FDA for PTC596, our cancer stem cell program, and the declaration of a development candidate in our antibacterial program. After recently completing a successful public equity offering where we raised net proceeds of approximately \$117.5 million, PTC is well positioned to realize our goal of discovering, developing and commercializing new treatments that have the potential to transform the lives of patients.”

Corporate Highlights

- **ACT DMD:** The confirmatory Phase 3 ACT DMD (Ataluren Confirmatory Trial in DMD) trial evaluating Translarna™ in patients with nonsense mutation Duchenne muscular dystrophy (nmDMD) completed enrollment. Top-line data from the trial is expected in the fourth quarter of 2015.
- **ACT CF:** The confirmatory Phase 3 ACT CF (Ataluren Confirmatory Trial in CF) trial evaluating Translarna™ in patients with nonsense

mutation cystic fibrosis is underway. Enrollment is expected to be completed in the second half of 2015, with data approximately one year later.

- **Regulatory update:** PTC plans to submit a New Drug Application to the FDA on a rolling basis for Translarna™ as a treatment for nonsense mutation Duchenne muscular dystrophy (nmDMD) in the U.S. before the end of 2014.
- **Commercial launch:** Commercial activities are ongoing in support of the anticipated launch of Translarna across Europe, subject to the completion of each country's market access process and timeline. PTC expects to initially launch in Germany within the next few months, with other key countries expected to follow throughout 2015 and beyond.
- **Reimbursed Early Access Programs:** Reimbursed early access programs (EAP) are well underway and the company has already begun to receive payments and recognize revenue in connection with the initiation of supply of Translarna to patients under these programs.
- **SMA program:** Initiation of a Phase 1b/2a, multi-center, randomized, double-blind, placebo-controlled, multiple-dose clinical trial in SMA patients is anticipated in the coming weeks in conjunction with our partners, Roche and the SMA Foundation. PTC expects to receive a \$10 million milestone payment from Roche upon initiation of the study.
- **Oncology program:** Successfully completed IND-enabling studies for PTC596, an orally active small molecule that targets drug-resistant tumor stem cell populations. An investigational new drug (IND) application for PTC596 is expected to be submitted before the end of 2014 with plans to initiate a Phase 1 clinical trial in the first half of 2015.
- **Antibacterial program:** Selected a development candidate for the treatment of life-threatening infections caused by multi-drug resistant (MDR) Gram-negative gonorrhoea.
- **Financing:** Recently completed a successful public offering of common stock which raised net proceeds of approximately \$117.5 million. This additional capital is expected to help fund the business through late 2017.
- **Key appointments:** PTC continued to expand its team in preparation for the launch of Translarna in the European Union and the expansion of our clinical programs. Senior executives with strong rare disease and commercial launch experience have been appointed in European regional roles as well as across key countries including Germany, the UK, France, Italy and the Nordic Region. Leadership is also being added across

the organization including clinical development, regulatory affairs, medical affairs, supply chain and quality.

- **International headquarters:** During fiscal 2014, PTC established its international headquarters in Dublin, Ireland. This office will serve as the central hub for the commercial launch of Translarna to take advantage of its location with respect to PTC's third party manufacturing and supply chain. In addition, PTC has begun establishing subsidiaries in key European countries where Translarna is expected to initially become commercially available.
- **Upcoming Events:** PTC's Annual Science Day will be held on November 24th in New York City. PTC will also be participating in the following conferences in the fourth quarter:
 - Credit Suisse Healthcare Conference on November 11th in Arizona.
 - Panel at the Boston Biotech's CEO Conference on November 12th in New York.
 - Deutsche Bank BioFEST on December 2nd in Boston.
 - Panel at the Genetic Rx Conference on December 3rd in Boston.
 - Oppenheimer Annual Healthcare Conference on December 10th-11th in New York.

Third Quarter 2014 Financial Highlights:

- Cash, cash equivalents, and marketable securities totaled \$209.4 million at September 30, 2014 compared to \$142.5 million at December 31, 2013. Pro forma for PTC's recent public offering of common stock, cash, cash equivalents and marketable securities totaled \$326.9 million as of September 30, 2014.
- Revenue of \$0.1 million from net product sales of Translarna was recognized for the first time in the history of the company during the third quarter of 2014. Revenue from grants and collaborations was \$1.6 million for the third quarter of 2014, compared to \$16.3 million for the same period in 2013. The decrease was due to a milestone payment of \$10 million that was recognized in the third quarter of 2013 related to the SMA collaboration as well as a decrease in the recognition of non-cash deferred revenue.
- Research and development expenses were \$18.8 million for the third

quarter of 2014, including \$2.4 million in non-cash, stock-based compensation expense, compared to \$13.9 million for the same period in 2013, including \$0.7 million in non-cash, stock-based compensation expense. The increase primarily resulted from the additional stock-based compensation expense and an increase in clinical trial expenses.

- General and administrative expenses were \$10.5 million for the third quarter of 2014, including \$2.3 million in non-cash stock-based compensation expense, compared to \$6.7 million for the same period in 2013, including \$2.0 million in non-cash stock-based compensation expense. The increase primarily results from additional costs associated with commercial activities in support of the anticipated launch of Translarna across Europe and legal costs associated with establishing our international infrastructure.
- Net loss for the third quarter of 2014 was \$27.3 million compared to a net loss of \$4.4 million for the same period in 2013.
- Shares issued and outstanding as of September 30, 2014 were 30.1 million, which includes 0.7 million shares of unvested restricted stock. Pro forma for our recently completed public offering of common stock, shares issued and outstanding were 33.6 million.
- Based on our current run-rate of expenses, we now expect total cash operating expenses to be between \$93 million and \$103 million, excluding expected non-cash stock-based compensation of approximately \$17 million, for total operating expenses of approximately \$110 million to \$120 million. As a result of our recent financing, we now expect to end 2014 with approximately \$295 million to \$305 million in cash, cash equivalents and marketable securities.

22. On the same day, PTC Therapeutics filed its Quarterly Report with the SEC on Form 10-Q for the fiscal quarter ended September 30, 2014. The Company's Form 10-Q was signed by Defendant Kovacs, and reaffirmed the Company's financial results previously announced earlier that day.

23. On February 27, 2015, PTC Therapeutics issued a press release entitled, "PTC Therapeutics Reports Fourth Quarter and 2014 Full Year Financial Results and Provides Corporate Update." Therein, the Company, in relevant part, stated:

SOUTH PLAINFIELD, NJ — February 27, 2014 [sic] — PTC

Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the fourth quarter and full year ended December 31, 2014.

“2014 was a transformative year for PTC. We are now a growing commercial-stage biopharma company, focused on delivering and developing RNA-targeted therapies in the rare disease space,” stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. “We are proud to bring the first treatment for Duchenne muscular dystrophy to patients who suffer from this devastating disorder. Concurrent with our ongoing launch activities in Europe and around the world, we are beginning to build out our commercial infrastructure in the US in preparation for a potential launch in 2016. We are equally focused on bringing additional innovative therapies to patients by moving our earlier stage pipeline forward. Importantly, we completed two successful public equity financings in 2014, providing us with a strong cash position to advance our ongoing commercial and clinical efforts.”

Key 2014 Achievements

- **Translarna approved in the European Union:** PTC’s lead product, Translarna™ (ataluren), received marketing authorization in the EU in August 2014 for the treatment of nonsense mutation Duchene muscular dystrophy, representing the first-ever treatment approved for the underlying cause of the disease.
- **Launched in EU and select countries:** In the second half of 2014, reimbursed early access programs were authorized in a number of countries both within Europe and outside of Europe where the EMA approval is referenced. Translarna was recently launched on a commercial basis in Germany in December of 2014. Additional country launches are expected to continue across the EU throughout 2015, subject to successful completion of pricing and reimbursement negotiations. As of February 25, 2015, PTC had 42 DMD patients on commercial Translarna therapy through either reimbursed early access programs or commercial sales.
- **Rolling NDA submitted for Translarna in the US:** In December 2014, PTC began submitting a rolling New Drug Application to the FDA for the approval of Translarna in nonsense mutation DMD. Top-line data from the company’s ongoing Phase 3 ACT DMD trial is expected in the fourth quarter of this year which should form the basis for finalizing the NDA submission. Concurrently, PTC has begun building out its US commercial team and infrastructure in preparation for a potential US launch in the first half of 2016.
- **Confirmatory Phase 3 ACT CF trial initiated:** PTC initiated its global confirmatory Phase 3 ACT CF trial in nonsense mutation cystic fibrosis

patients. The trial is being conducted at approximately 90 clinical sites globally and is expected to enroll approximately 208 patients by the end of 2015. PTC intends to file for approval of Translarna for the treatment of nonsense mutation cystic fibrosis in the EU in the second half of 2015.

- **SMA program completed Phase 1a study, demonstrating proof of mechanism in healthy volunteers; Phase 2 study initiated in SMA patients:** In the spring of 2014, a Phase 1 clinical study in healthy volunteers was successfully completed. The single-ascending dose study was well tolerated at all dose levels studied. Importantly, a dose-dependent effect on SMN2 splicing was observed which may be interpreted as proof of mechanism based on the expected pharmacodynamic effect. A multiple-dose, Phase 2 clinical study called MOONFISH was initiated in November 2014 with results expected in 2016. PTC received \$17.5 million in milestone payments from Roche over the course of the year.
- **Advanced internally developed pipeline programs:** In the fourth quarter of 2014, PTC completed IND-enabling studies for PTC596, a cancer stem cell targeting program, and filed an investigational new drug application with the FDA. An open-label Phase 1 clinical study for this program is expected to begin in the first half of 2015. PTC also declared a development candidate and is initiating IND-enabling studies for its anti-bacterial program focused on the treatment of multi-drug resistant gonorrhea.
- **Established international headquarters in Dublin, Ireland:** This office will serve as the central hub for the commercialization of Translarna on a global basis and will enable PTC to take advantage of its proximity to our third-party manufacturing and supply chain.
- **Maintained strong balance sheet with \$315 million in cash and cash equivalents:** PTC completed two successful public equity offerings during the year raising net proceeds of approximately \$236 million. PTC finished 2014 with over \$315 million in cash and cash equivalents.

Upcoming Events:

PTC will participate in the following conferences in the first quarter:

- Cowen & Co 35th Annual Health Care conference on March 2nd in Boston, MA
- 27th Annual Roth Conference on March 9th in Orange County, CA
- Barclays Capital Healthcare Conference on March 10th in Miami, FL

Fourth Quarter and Full year 2014 Financial Highlights:

- Total revenues for the fourth quarter of 2014 were approximately \$12.7 million, including \$0.6 million in Translarna product sales revenue, which was recognized on a cash-basis, and \$12.0 million in grants and collaborations. This compared to total revenue in the fourth quarter of 2013 of approximately \$4.4 million. The increase in grants and collaborations revenue was due to receipt of a milestone payment of \$10 million from Roche recognized in the fourth quarter of 2014 related to our SMA collaboration. Total revenue in 2014 was \$25.2 million, including \$0.7 million in Translarna product sales revenue, which was recognized on a cash-basis and \$24.5 million in grants and collaborations revenue. Total invoiced Translarna product sales in 2014 was approximately \$2.5 million, of which approximately \$1.7 million has been booked as deferred revenue until cash payment has been received. This compared to total revenue in 2013 of \$34.7 million which was entirely from grants and collaborations. The decrease in grants and collaborations revenue primarily resulted from a reduction in non-cash deferred revenue of \$16.8 million in 2014 vs. 2013.
- Research and development expenses were \$26.9 million for the fourth quarter of 2014, including \$3.2 million in non-cash, stock-based compensation expense, compared to \$15.0 million for the same period in 2013, including \$1.6 million in non-cash, stock-based compensation expense. Research and development expenses for the full year 2014 were \$79.8 million, including \$9.7 million in non-cash, stock-based compensation expense compared to \$54.9 million for the same period in 2013, including \$4.3 million in non-cash, stock-based compensation expense. The increase in R&D expense for the quarter and year ended December 31, 2014 as compared to the prior year periods was primarily due to additional costs associated with our ongoing clinical trials, including the initiation of the phase 3 ACT CF trial, and increased costs associated with regulatory, quality and supply chain functions including the manufacturing of Translarna drug product.
- Selling, general and administrative expenses were \$18.0 million for the fourth quarter of 2014, including \$3.5 million in non-cash stock-based compensation expense, compared to \$7.5 million for the same period in 2013, including \$1.7 million in non-cash stock-based compensation expense. SG&A expenses for the full year 2014 were \$44.8 million, including \$9.6 million in non-cash stock-based compensation expense, compared to \$25.2 million in 2013, including \$4.1 million in non-cash stock-based compensation expense. The increase in SG&A expense for the quarter and year ended December 31, 2014 as compared to the prior year periods was primarily resulted from additional costs associated with commercial activities in support of the launch of Translarna across Europe

and select other regions as well as finance and legal costs associated with establishing our international infrastructure.

- Net loss for the fourth quarter of 2014 was \$27.3 million compared to a net loss of \$17.9 million for the same period in 2013. Net loss for the full year 2014 was \$93.8 million compared to \$51.6 million for the same period in 2013.
- Cash, cash equivalents, and marketable securities totaled \$315.2 million at December 31, 2014 compared to \$142.5 million at December 31, 2013. This increase was primarily as a result of two public equity offerings that were completed in 2014 which raised net proceeds of approximately \$236 million. We also received \$4.9 million in the fourth quarter of 2014 from the sale of net operating losses and research and development credits as part of the New Jersey Technology Business Tax Certificate Transfer Program.
- Shares issued and outstanding as of December 31, 2014 were 33.6 million, which includes 0.7 million shares of unvested restricted stock.

2015 Guidance

- Operating expense for the full year 2015 is anticipated to be between \$160- \$170 million, excluding expected non-cash stock-based compensation expense of approximately \$30 million, for total operating expenses of approximately \$190 million to \$200 million. These expenses will be primarily in support of our ongoing and planned confirmatory Phase 3 clinical trials for Translarna in nmDMD and nmCF, commercial launch activities for Translarna in the EU as well as pre-commercial activities in the US, and the continued research and clinical development of other product pipeline candidates.
- For 2015, PTC anticipates providing the number of nmDMD patients on Translarna therapy at the end of each quarter.

24. On March 2, 2015, PTC Therapeutics filed its Annual Report with the SEC on Form 10-K for the fiscal year ended December 31, 2014. The Company's Form 10-K was signed by Defendants Peltz and Kovacs, and reaffirmed the Company's financial results previously announced on February 27, 2015.

25. On May 4, 2015, PTC Therapeutics issued a press release entitled, "PTC Therapeutics Reports First Quarter 2015 Financial Results and Provides Corporate Update."

Therein, the Company, in relevant part, stated:

SOUTH PLAINFIELD, NJ – May 4, 2015 – PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the first quarter ending March 31, 2015.

“We are off to a strong start this year. In our first full quarter since the commercial launch, we have seen strong support for access to Translarna, the first treatment for nonsense mutation Duchene Muscular Dystrophy. We now have 82 patients on commercial therapy, nearly double the number on treatment since our last earnings call,” stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. “Our goal is to bring Translarna to patients across the globe as quickly as possible and we are pleased to have grown our commercial footprint to approximately 25 countries to date. We are also actively establishing our US infrastructure, in preparation for our anticipated US launch.”

Key First Quarter 2015 Corporate Highlights

- **Translarna commercial launch continues to accelerate.** PTC’s lead product, Translarna™ (ataluren), received marketing authorization in the EU in August 2014 for the treatment of nonsense mutation Duchene muscular dystrophy, representing the first-ever treatment approved for the underlying cause of the disease. As of April 30th, PTC has 82 DMD patients on commercial therapy through either reimbursed early access programs or commercial sales.
- **US commercial launch preparations are underway.** In December 2014, PTC began submitting a rolling New Drug Application to the FDA for the approval of Translarna in nonsense mutation DMD. Top-line data from the company’s ongoing Phase 3 ACT DMD trial is expected in the fourth quarter of this year which should form the basis for finalizing the NDA submission. Concurrently, PTC has begun building out its US commercial team and infrastructure in preparation for a potential US launch in the first half of 2016. Most recently, PTC announced the hiring of Eric Pauwels as head of commercial operations in the Americas.
- **Confirmatory Phase 3 ACT CF trial enrollment is on track.** PTC initiated its global confirmatory Phase 3 ACT CF trial in nonsense mutation cystic fibrosis patients in June 2014. The trial is being conducted at approximately 90 clinical sites globally and is expected to enroll approximately 208 patients by the end of 2015. PTC intends to file for approval of Translarna for the treatment of nonsense mutation cystic fibrosis in the EU in the second half of 2015.
- **Translarna pipeline will expand with a proof of concept study in**

aniridia. PTC plans to initiate a Phase 2 proof of concept study in nonsense mutation aniridia by year end 2015. Aniridia is a rare genetic disorder that results in disruption in the development of the eye. Pre-clinical data were presented at the Association for Research in Vision and Ophthalmology (ARVO) on May 3rd. PTC is also initiating a proof-of-concept Phase 2 study of Translarna in nonsense mutation MPS I in addition to its ongoing Phase 3 trials in nonsense mutation DMD and nonsense mutation CF. Because of its mechanism of action, Translarna has the potential to address numerous disorders caused by a nonsense mutation. PTC continues to evaluate additional indications to fully capture Translarna's potential.

- **SMA Phase 1 results highlighted at the annual meeting of the American Academy of Neurology.** On April 22nd at the AAN emerging science session, clinical data from the Phase 1 SMA study demonstrated a significant and dose-dependent increase in the production of full length SMN2 mRNA. After a single dose, an increase of approximately 80% was observed in the levels of full length SMN2 mRNA expression. Since the SMN2 gene in healthy volunteers is the same as in SMA patients, these results demonstrate proof of mechanism based on the expected pharmacodynamic effect. The Phase 1 clinical study was completed in the spring of 2014 and was well tolerated at all dose levels studied.
- **New preclinical and clinical developments in the SMA collaboration.** Dosing of the first cohort in the Phase 2 MOONFISH study successfully completed. RG7800 was well tolerated and preliminary review of the blinded data indicates substantial increases in full length SMN2 mRNA. Long term preclinical animal data have recently shown an eye finding at concentrations above those explored in patients. This finding was not observed in humans. As a precautionary measure, the collaboration partners decided to temporarily suspend dosing of additional patients to evaluate this finding and confirm next steps for MOONFISH.
- **Initiated cancer stem cell program Phase 1 study.** An open-label Phase 1 clinical study for this program targeting BMI1, a protein linked to drug resistant cancers, began in April. In the fourth quarter of 2014, PTC completed IND-enabling studies for PTC596 and filed an investigational new drug application with the FDA.

Upcoming Events:

PTC will participate in the following conferences in the second quarter:

- Deutsche Bank 40th Annual Healthcare conference May 6th in Boston, MA

- Bank of America Merrill Lynch Healthcare conference May 12th in Las Vegas, NV
- Barclays Select West Coast Biotech conference May 28th in Napa, CA

First Quarter 2015 Financial Highlights:

- Total revenues for the first quarter of 2015 were approximately \$7.5 million, including \$5.1 million in Translarna product sales revenue and \$2.4 million in grants and collaborations revenue. Total Translarna product sales in first quarter 2015 includes \$1.4 million of revenue which was deferred during 2014 when we recognized revenue on a cash basis. As of January 1, 2015, we began recognizing revenue for Translarna as product is shipped, given we have established a pattern of collectability. This compared to total revenue in the first quarter of 2014 of approximately \$9.2 million. The decrease in total revenue was due to receipt of a milestone payment of \$7.5 million from Roche recognized in the first quarter of 2014 related to our SMA collaboration, partially offset by Translarna product sales.
- Research and development expenses were \$27.9 million for the first quarter of 2015, including \$4.7 million in non-cash, stock-based compensation expense, compared to \$15.9 million for the same period in 2014, including \$1.9 million in non-cash, stock-based compensation expense. The increase in R&D expense for the first quarter 2015 as compared to the prior year quarter was primarily due to additional costs associated with our ongoing clinical trials and supply chain activities in support of the launch of Translarna as well as in conjunction with our expanding clinical-stage pipeline.
- Selling, general and administrative expenses were \$17.6 million for the first quarter of 2015, including \$5.1 million in non-cash, stock-based compensation expense, compared to \$7.5 million for the same period in 2014, including \$1.8 million in non-cash, stock-based compensation expense. The increase in SG&A expense for the first quarter 2015 as compared to the prior year period primarily resulted from additional costs associated with commercial activities in support of the launch of Translarna across Europe and other regions.
- Net loss for the first quarter of 2015 was \$37.9 million compared to a net loss of \$14.1 million for the same period in 2014.
- Cash, cash equivalents, and marketable securities totaled \$280.5 million at March 31, 2015 compared to \$315.2 million at December 31, 2014.

- Shares issued and outstanding as of March 31, 2015 were 33.9 million, which includes 0.4 million shares of unvested restricted stock.

26. On May 5, 2015, PTC Therapeutics filed its Quarterly Report with the SEC on Form 10-Q for the fiscal quarter ended March 31, 2015. The Company's Form 10-Q was signed by Defendant Kovacs, and reaffirmed the Company's financial results previously announced on May 4, 2015.

27. On July 30, 2015, PTC Therapeutics issued a press release entitled, "PTC Therapeutics Reports Second Quarter 2015 Financial Results and Provides Corporate Update."

Therein, the Company, in relevant part, stated:

SOUTH PLAINFIELD, NJ — July 30, 2015 — PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the second quarter ending June 30, 2015.

"PTC is progressing on many fronts across the organization. We are excited to be near the completion of the largest Duchenne muscular dystrophy clinical trial ever conducted with topline results expected in the fourth quarter," stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "Translarna is now commercially available in 12 countries and we have a presence in over 30 countries. We are also planning to submit an application for Translarna to treat nonsense mutation cystic fibrosis to the European Medicines Agency by the end of this year. This submission will include new and important analyses of our CF data."

Dr. Peltz continued, "This is just the beginning. There are many nonsense mutation-based disorders with high unmet medical need where no existing treatment is available. To deliver on our commitment of bringing hope to patients with rare and neglected genetic disorders and to maximize the potential of Translarna, our goal is to commence clinical investigations of at least ten new indications by the year 2020."

Key Second Quarter 2015 Corporate Highlights:

- **Translarna now available on a commercial basis in 12 countries.** As of July 28th, there are 106 DMD patients on commercial therapy, including patients from both direct commercial sales and reimbursed early access programs. New countries recently added include Denmark, Norway, and Brazil. Translarna received marketing authorization from the European Medicines Agency in August 2014 for the treatment of

nonsense mutation Duchene muscular dystrophy in ambulatory patients aged 5 and over, representing the first-ever treatment approved for the underlying cause of the disease.

- **Top-line data from Phase 3 ACT DMD expected in the fourth quarter of 2015.** In December 2014, PTC began submitting a rolling new drug application (NDA) to the FDA for the approval of Translarna in nonsense mutation DMD. Top-line data from the company's ongoing Phase 3 ACT DMD trial is expected in the fourth quarter of this year. PTC anticipates a potential US approval and commercial launch of Translarna for nonsense mutation DMD in the first half of 2016.
- **New analyses of previous Phase 3 CF data demonstrates greater response in younger patients not receiving chronic inhaled tobramycin.** Natural history data indicate that patients under 18 years of age experience more rapid rates of decline in pulmonary function. In preparation for the European application for Translarna for the treatment of nonsense mutation cystic fibrosis and in consultation with European thought leaders, PTC has performed additional analyses of the data from its previous Phase 3 trial completed in 2011. The subgroup of non-TOBI patients under 18 years of age experienced a robust treatment response with an increase in FEV1 and a 5.4% absolute benefit in FEV1 as well as a 60% reduction in pulmonary exacerbations rates versus placebo.
- **Enrollment of Phase 3 ACT CF trial on track to be completed by the end of 2015.** ACT CF is expected to enroll approximately 208 patients with nonsense mutation cystic fibrosis. Top-line data are expected to be available by the end of 2016.
- **PTC outlines Ten by '20 strategy for Translarna pipeline expansion.** Given its mechanism of action, Translarna has the potential to address numerous genetic disorders caused by a nonsense mutation. In addition to its advanced DMD and CF programs, PTC is now pursuing two additional indications, MPS I and aniridia. PTC's goal is to investigate Translarna's activity in a minimum of ten indications beyond DMD and CF by 2020 in order to deliver on its commitment to patients and maximize the potential of Translarna as both a product and a pipeline.
- **SMA program and Phase 2 MOONFISH study update.** Dosing of the first cohort in the Phase 2 MOONFISH study was successfully completed earlier in the year. RG7800 was well tolerated and preliminary review of the blinded data indicated substantial increases in full length SMN2 mRNA. As previously announced, an eye finding was detected in a longer term preclinical animal study at concentrations above those explored in patients. This finding was not observed in humans. As a precautionary measure, dosing of additional patients was suspended while the finding is

investigated. Analyses completed to date, combined with additional preclinical data expected later this fall, could potentially allow dosing to resume in patients in the first quarter of 2016.

Upcoming Events:

PTC will participate in the following conferences in the third quarter:

- Wedbush 2015 PacGrow Healthcare Conference August 12th in New York, NY
- Citi 10th Annual Biotech Conference September 10th in New York, NY
- J.P. Morgan 6th Annual “All Stars” Conference September 16th in London, UK
- Bank of America Global Healthcare Conference September 17th in London, UK

Second Quarter 2015 Financial Highlights:

- Translarna net product sales were \$6.2 million for the second quarter of 2015, representing 69% sequential growth versus \$3.7 million in adjusted net product sales in the first quarter of 2015. Translarna net product sales in first quarter of 2015 included \$1.4 million of revenue which was deferred during 2014 when we recognized revenue on a cash basis.
- Total revenues for the second quarter of 2015 were \$6.8 million, including \$6.2 million in Translarna net product sales revenue and \$0.6 million in grants and collaborations revenue. This compared to total revenue in the second quarter of 2014 of approximately \$1.7 million. The increase in total revenue was a result of the commercial launch of Translarna which received marketing authorization from the European Medicines Agency in August 2014.
- Research and development expenses were \$28.2 million for the second quarter of 2015, including \$4.0 million in non-cash, stock-based compensation expense, compared to \$18.3 million for the same period in 2014, including \$2.2 million in non-cash, stock-based compensation expense. The increase in R&D expense for the second quarter 2015 as compared to the prior year period was primarily due to additional costs associated with our ongoing clinical trials as well as our expanding clinical-stage pipeline.
- Selling, general and administrative expenses were \$17.2 million for the second quarter of 2015, including \$4.4 million in non-cash stock-based

compensation expense, compared to \$8.7 million for the same period in 2014, including \$2.1 million in non-cash stock-based compensation expense. The increase in SG&A expense for the second quarter 2015 as compared to the prior year period primarily resulted from additional costs associated with commercial activities in support of the launch of Translarna across Europe and other regions.

- Net loss for the second quarter of 2015 was \$38.4 million compared to a net loss of \$25.1 million for the same period in 2014.
- Cash, cash equivalents, and marketable securities totaled \$255.2 million as of June 30, 2015 compared to \$315.2 million as of December 31, 2014.
- Shares issued and outstanding as of June 30, 2015 were 34.2 million, which includes 0.4 million shares of unvested restricted stock.

28. On July 31, 2015, PTC Therapeutics filed its Quarterly Report with the SEC on Form 10-Q for the fiscal quarter ended June 30, 2015. The Company's Form 10-Q was signed by Defendant Kovacs, and reaffirmed the Company's financial results previously announced on July 30, 2015.

29. On November 9, 2015, PTC Therapeutics issued a press release entitled, "PTC Therapeutics Reports Third Quarter 2015 Financial Results, Provides Corporate Update and Reviews Key Findings From Act DMD." Therein, the Company, in relevant part, stated:

SOUTH PLAINFIELD, NJ — November 9, 2015 — PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the third quarter ending September 30, 2015.

"We recently reported results from our Phase 3 ACT DMD clinical trial and are now actively finalizing our regulatory submissions to both the FDA and EMA, which we plan to complete by the end of this year," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "Our ongoing launch of Translarna for Duchenne muscular dystrophy across Europe and other parts of the world continues to perform well, and based on the consistently positive feedback we are receiving from key opinion leaders, physicians, and patient advocacy groups regarding our ACT DMD results, we expect this momentum to build."

Key Third Quarter 2015 Corporate Highlights:

- **Translarna now available on a commercial basis in 13 countries.** As

of November 5th, there were 152 DMD patients on commercial therapy, including patients from both direct commercial sales and reimbursed early access programs. Translarna received marketing authorization from the European Medicines Agency (EMA) in August 2014 for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) in ambulatory patients aged 5 and over, representing the first-ever treatment approved for the underlying cause of the disease. More than 550 DMD patients are currently receiving Translarna therapy either through open-label extension studies or commercial access.

- **ACT DMD results confirm clinical benefit of Translarna in nonsense mutation Duchenne muscular dystrophy.** On October 15th, PTC announced results from the Phase 3 ACT DMD clinical trial of Translarna in patients with nmDMD. The totality of the clinical data from two large, placebo-controlled clinical trials across over 400 patients demonstrates Translarna's ability to slow disease progression. Today, on PTC's quarterly investor call the Company will review key findings from the ACT DMD clinical trial. In the overall intent-to-treat population, the primary endpoint of change from baseline in the 6-minute walk test (6MWT) demonstrated a 15 meter benefit ($p=0.213$), which was not statistically significant. A benefit of 47 meters (nominal $p=0.007$) was demonstrated in the pre-specified patient population of 300-400 meters at baseline as measured by the 6MWT, which is in line with the Company's prior experience in its Phase 2b trial and consistent with the evolving understanding of the 6MWT in DMD. A meta-analysis of the combined data from ACT DMD and the ambulatory decline phase patients from the Phase 2b trial demonstrate a statistically significant benefit for Translarna across the primary (6MWT) and key secondary endpoints (timed function tests). PTC plans to complete its rolling new drug application (NDA) submission to the U.S. Food and Drug Administration (FDA) and to submit the data to the EMA by the end of 2015.
- **ACT CF Phase 3 clinical trial on track with enrollment to be completed by the end of 2015.** Enrollment of the ACT CF Phase 3 clinical trial has been extremely robust due to high patient demand. Screening for the study has now closed, and enrollment will be completed before the end of 2015, with topline data expected about a year later. During the third quarter, PTC submitted a variation to its marketing authorization of Translarna to the EMA to request approval of Translarna for the treatment of cystic fibrosis, a potential second indication in the European Union.
- **Update on additional indications for Translarna.** Given its mechanism of action, Translarna has the potential to address numerous genetic disorders caused by a nonsense mutation. In addition to its advanced DMD and CF programs, PTC is pursuing two additional indications, MPS I and

aniridia, for which Translarna has received orphan drug designations from both the FDA and EMA. In May of this year, PTC amended the MPS I clinical trial protocol to allow patients currently using enzyme replacement therapy to be included in the trial. This protocol revision resulted in delays to opening clinical trial sites and accruing patients. As a result, PTC now expects data for Translarna in MPS I in 2016. PTC's goal is to investigate Translarna's activity in a minimum of ten indications beyond DMD and CF by 2020 in order to deliver on its commitment to patients and maximize the potential of Translarna as both a product and a pipeline.

- **SMA Program update.** Clinical data from the first cohort of the Phase 2a Moonfish study was recently presented at the 20th International Annual Congress of the World Muscle Society. This data demonstrated that treatment with RG7800 shifts SMN2 splicing toward the production of full length SMN mRNA and generated up to two-fold increases in SMN protein in patients with SMA. Pre-clinical investigations regarding our lead compound, RG7800, are ongoing after the observation of an unexpected finding in a chronic animal safety study in April. Concurrent with the advancement of our lead compound, a robust research effort regarding SMN2 splicing has continued to advance through IND-enabling studies. Additional data are expected in the coming months, which will be utilized to determine the best clinical development path forward for the SMA program. PTC and the program collaborators remain highly committed to this program and expect that clinical development will resume in early 2016.

Upcoming Events:

PTC will participate in the following conferences in the fourth quarter:

- Credit Suisse 24th Annual Healthcare Conference, November 11th in Scottsdale, AZ
- Stifel 2015 Healthcare Conference, November 18th in New York, NY
- Oppenheimer 26th Annual Healthcare Conference, December 9th in New York, NY

Third Quarter 2015 Financial Highlights:

- Translarna net product sales were \$9.8 million for the third quarter of 2015, representing 59% sequential growth versus \$6.2 million in net product sales in the second quarter of 2015. Translarna has generated \$21.0 million in net product sales through the first three quarters of 2015.

- Total revenues for the third quarter of 2015 were \$9.8 million. This compared to total revenue in the third quarter of 2014 of approximately \$1.7 million. The increase in total revenue was a result of the commercial launch of Translarna, which received marketing authorization from the EMA in August 2014, partially offset by lower grant revenue.
- Research and development expenses were \$30.6 million for the third quarter of 2015, including \$3.8 million in non-cash, stock-based compensation expense, compared to \$18.8 million for the same period in 2014, including \$2.4 million in non-cash, stock-based compensation expense. The increase in R&D expense for the third quarter 2015 as compared to the prior year period was primarily due to expansion of our clinical development activities including late stage studies in both Duchenne muscular dystrophy and cystic fibrosis.
- Selling, general and administrative expenses were \$21.4 million for the third quarter of 2015, including \$4.2 million in non-cash, stock-based compensation expense, compared to \$10.5 million for the same period in 2014, including \$2.3 million in non-cash, stock-based compensation expense. The increase in SG&A expense for the third quarter 2015 as compared to the prior year period primarily resulted from additional costs associated with commercial activities in support of the launch of Translarna across Europe and other regions.
- Net interest expense for the third quarter of 2015 was \$0.9 million compared to net interest income of \$0.4 million in the same period in 2014. The increase in interest expense is a result of the \$150 million convertible debt offering completed during the third quarter 2015. The debt was recorded on PTC's balance sheet at a discount, which will be amortized over the life of the bond.
- Net loss for the third quarter of 2015 was \$43.2 million compared to a net loss of \$27.3 million for the same period in 2014.
- Cash, cash equivalents, and marketable securities totaled \$371.5 million at September 30, 2015 compared to \$315.2 million at December 31, 2014. This includes net proceeds of approximately \$145.4 million from a \$150 million convertible debt offering completed in the third quarter.
- Shares issued and outstanding as of September 30, 2015 were 34.3 million, which includes 0.4 million shares of unvested restricted stock.

30. On the same day, PTC Therapeutics filed its Quarterly Report with the SEC on Form 10-Q for the fiscal quarter ended September 30, 2015. The Company's Form 10-Q was

signed by Defendant Kovacs, and reaffirmed the Company's financial results previously announced earlier that day.

31. The above statements contained in ¶¶ 17–30 were false and/or misleading, as well as failed to disclose material adverse facts about the Company's business, operations, and prospects. Specifically, these statements were false and/or misleading statements and/or failed to disclose: (1) that the Company's NDA for Translarna that it submitted to the FDA was not sufficiently complete to permit a substantive review of the application; (2) that, as such, the application would not be reviewed nor approved by the FDA; (3) that the impending non-approval of the NDA would have a negative material impact on the Company's operations and prospects; and (4) that, as a result of the foregoing Defendants' statements about PTC Therapeutics' business, operations, and prospects, were false and misleading and/or lacked a reasonable basis.

Disclosures at the End of the Class Period

32. On February 23, 2016, the Company issued a press release entitled "PTC Receives Refuse to File Letter from FDA for Translarna™ (ataluren)." Therein, the Company stated that it received a Refuse to File letter from the United States Food and Drug Administration ("FDA") regarding PTC's New Drug Application ("NDA") for Translarna because "the application was not sufficiently complete to permit a substantive review."

33. Subsequently, on the same day, February 23, 2016, JP Morgan Securities downgraded the Company's stock, and issued a report that sharply critiqued the Company's transparency regarding Translarna's NDA and its potential for FDA approval. The JP Morgan Securities report stated in relevant part:

This morning, PTC announced it has received a refuse to file (RTF) letter for Translarna in non-sense mutation Duchenne Muscular Dystrophy. The press

release does not provide much clarity on core issues, and, based on our conversation with the company, PTCT is still working through next steps. PTCT shares are down 40%+ and justifiably so. Currently, there are more questions than answers, including 1) nature / details of RTF, 2) the possibility of additional pivotal trial work in DMD for US approval, 3) status of EU conditional approval for Translarna, 4) pricing in the EU and 5) credibility of management. Indeed, the fact that limited details have been disclosed on the RTF leads us to believe the setback is likely not to be a minor delay (i.e., addressable in a short timeframe / a paper work issue). While we had been constructive on PTCT shares for some time, at this point with limited clarity on key value drivers, we cannot justify an Overweight rating. We are downgrading PTCT shares to Neutral, decreasing our Dec-16 price target to \$22 from \$81, and removing the stock from the J.P. Moran Analyst Focus List. * Select catalysts to monitor: Recall, Translarna is being evaluated as a potential treatment for CF as well as MPS1. Data from phase 2 POC from MPS 1 in 2016 and a potential EU conditional approval in CF in mid-year (not included in our model), as well as ACT CF phase 3 data readout by YE16 /early 2017. Importantly, in January, PTC completed its submission of the ACT DMD results to EMA in order to satisfy full approval and an expected in 2016.

* Highlighted changes to our model: We are adopting a more conservative outlook on Translarna approval across indications and commercial regions. DMD - In the US, we have lowered our POS assumption from 80 to 15%, at the same time decreasing our peak penetration estimates from 65% to 45% in the ambulatory segment.

34. On this news, shares of PTC Therapeutics fell \$17.42 per share, or more than 60%, to close at \$10.84 per share on February 23, 2016, on unusually heavy trading volume.

CLASS ACTION ALLEGATIONS

35. Plaintiff brings this action as a class action pursuant to Federal Rule of Civil Procedure 23(a) and (b)(3) on behalf of a class, consisting of all those who purchased PTC Therapeutics' securities between May 6, 2014 and February 23, 2016, inclusive (the "Class Period") and who were damaged thereby (the "Class"). Excluded from the Class are Defendants, the officers and directors of the Company, at all relevant times, members of their immediate families and their legal representatives, heirs, successors or assigns and any entity in which Defendants have or had a controlling interest.

36. The members of the Class are so numerous that joinder of all members is impracticable. Throughout the Class Period, PTC Therapeutics' securities were actively traded on the NASDAQ Stock Market (the "NASDAQ"). While the exact number of Class members is unknown to Plaintiff at this time and can only be ascertained through appropriate discovery, Plaintiff believes that there are hundreds or thousands of members in the proposed Class. Millions of PTC Therapeutics shares were traded publicly during the Class Period on the NASDAQ. As of August 4, 2014, PTC Therapeutics had 30,069,897 shares of common stock outstanding. Record owners and other members of the Class may be identified from records maintained by PTC Therapeutics or its transfer agent and may be notified of the pendency of this action by mail, using the form of notice similar to that customarily used in securities class actions.

37. Plaintiff's claims are typical of the claims of the members of the Class as all members of the Class are similarly affected by Defendants' wrongful conduct in violation of federal law that is complained of herein.

38. Plaintiff will fairly and adequately protect the interests of the members of the Class and has retained counsel competent and experienced in class and securities litigation.

39. Common questions of law and fact exist as to all members of the Class and predominate over any questions solely affecting individual members of the Class. Among the questions of law and fact common to the Class are:

(a) whether the federal securities laws were violated by Defendants' acts as alleged herein;

(b) whether statements made by Defendants to the investing public during the Class Period omitted and/or misrepresented material facts about the business, operations, and

prospects of PTC Therapeutics; and

(c) to what extent the members of the Class have sustained damages and the proper measure of damages.

40. A class action is superior to all other available methods for the fair and efficient adjudication of this controversy since joinder of all members is impracticable. Furthermore, as the damages suffered by individual Class members may be relatively small, the expense and burden of individual litigation makes it impossible for members of the Class to individually redress the wrongs done to them. There will be no difficulty in the management of this action as a class action.

UNDISCLOSED ADVERSE FACTS

41. The market for PTC Therapeutics' securities was open, well-developed and efficient at all relevant times. As a result of these materially false and/or misleading statements, and/or failures to disclose, PTC Therapeutics' securities traded at artificially inflated prices during the Class Period. Plaintiff and other members of the Class purchased or otherwise acquired PTC Therapeutics' securities relying upon the integrity of the market price of the Company's securities and market information relating to PTC Therapeutics, and have been damaged thereby.

42. During the Class Period, Defendants materially misled the investing public, thereby inflating the price of PTC Therapeutics' securities, by publicly issuing false and/or misleading statements and/or omitting to disclose material facts necessary to make Defendants' statements, as set forth herein, not false and/or misleading. Said statements and omissions were materially false and/or misleading in that they failed to disclose material adverse information and/or misrepresented the truth about PTC Therapeutics' business, operations, and prospects as

alleged herein.

43. At all relevant times, the material misrepresentations and omissions particularized in this Complaint directly or proximately caused or were a substantial contributing cause of the damages sustained by Plaintiff and other members of the Class. As described herein, during the Class Period, Defendants made or caused to be made a series of materially false and/or misleading statements about PTC Therapeutics' financial well-being and prospects. These material misstatements and/or omissions had the cause and effect of creating in the market an unrealistically positive assessment of the Company and its financial well-being and prospects, thus causing the Company's securities to be overvalued and artificially inflated at all relevant times. Defendants' materially false and/or misleading statements during the Class Period resulted in Plaintiff and other members of the Class purchasing the Company's securities at artificially inflated prices, thus causing the damages complained of herein.

LOSS CAUSATION

44. Defendants' wrongful conduct, as alleged herein, directly and proximately caused the economic loss suffered by Plaintiff and the Class.

45. During the Class Period, Plaintiff and the Class purchased PTC Therapeutics' securities at artificially inflated prices and were damaged thereby. The price of the Company's securities significantly declined when the misrepresentations made to the market, and/or the information alleged herein to have been concealed from the market, and/or the effects thereof, were revealed, causing investors' losses.

SCIENTER ALLEGATIONS

46. As alleged herein, Defendants acted with scienter in that Defendants knew that the public documents and statements issued or disseminated in the name of the Company were

materially false and/or misleading; knew that such statements or documents would be issued or disseminated to the investing public; and knowingly and substantially participated or acquiesced in the issuance or dissemination of such statements or documents as primary violations of the federal securities laws. As set forth elsewhere herein in detail, Defendants, by virtue of their receipt of information reflecting the true facts regarding PTC Therapeutics, his/her control over, and/or receipt and/or modification of PTC Therapeutics' allegedly materially misleading misstatements and/or their associations with the Company which made them privy to confidential proprietary information concerning PTC Therapeutics, participated in the fraudulent scheme alleged herein.

**APPLICABILITY OF PRESUMPTION OF RELIANCE
(FRAUD-ON-THE-MARKET DOCTRINE)**

47. The market for PTC Therapeutics' securities was open, well-developed and efficient at all relevant times. As a result of the materially false and/or misleading statements and/or failures to disclose, PTC Therapeutics' securities traded at artificially inflated prices during the Class Period. On March 17, 2015, the Company's stock closed at a Class Period high of \$77.53 per share. Plaintiff and other members of the Class purchased or otherwise acquired the Company's securities relying upon the integrity of the market price of PTC Therapeutics' securities and market information relating to PTC Therapeutics, and have been damaged thereby.

48. During the Class Period, the artificial inflation of PTC Therapeutics' stock was caused by the material misrepresentations and/or omissions particularized in this Complaint causing the damages sustained by Plaintiff and other members of the Class. As described herein, during the Class Period, Defendants made or caused to be made a series of materially false and/or misleading statements about PTC Therapeutics' business, prospects, and operations. These material misstatements and/or omissions created an unrealistically positive assessment of

PTC Therapeutics and its business, operations, and prospects, thus causing the price of the Company's securities to be artificially inflated at all relevant times, and when disclosed, negatively affected the value of the Company stock. Defendants' materially false and/or misleading statements during the Class Period resulted in Plaintiff and other members of the Class purchasing the Company's securities at such artificially inflated prices, and each of them has been damaged as a result.

49. At all relevant times, the market for PTC Therapeutics' securities was an efficient market for the following reasons, among others:

(a) PTC Therapeutics stock met the requirements for listing, and was listed and actively traded on the NASDAQ, a highly efficient and automated market;

(b) As a regulated issuer, PTC Therapeutics filed periodic public reports with the SEC and/or the NASDAQ;

(c) PTC Therapeutics regularly communicated with public investors *via* established market communication mechanisms, including through regular dissemination of press releases on the national circuits of major newswire services and through other wide-ranging public disclosures, such as communications with the financial press and other similar reporting services; and/or

(d) PTC Therapeutics was followed by securities analysts employed by brokerage firms who wrote reports about the Company, and these reports were distributed to the sales force and certain customers of their respective brokerage firms. Each of these reports was publicly available and entered the public marketplace.

50. As a result of the foregoing, the market for PTC Therapeutics' securities promptly digested current information regarding PTC Therapeutics from all publicly available sources and

reflected such information in PTC Therapeutics' stock price. Under these circumstances, all purchasers of PTC Therapeutics' securities during the Class Period suffered similar injury through their purchase of PTC Therapeutics' securities at artificially inflated prices and a presumption of reliance applies.

NO SAFE HARBOR

51. The statutory safe harbor provided for forward-looking statements under certain circumstances does not apply to any of the allegedly false statements pleaded in this Complaint. The statements alleged to be false and misleading herein all relate to then-existing facts and conditions. In addition, to the extent certain of the statements alleged to be false may be characterized as forward looking, they were not identified as "forward-looking statements" when made and there were no meaningful cautionary statements identifying important factors that could cause actual results to differ materially from those in the purportedly forward-looking statements. In the alternative, to the extent that the statutory safe harbor is determined to apply to any forward-looking statements pleaded herein, Defendants are liable for those false forward-looking statements because at the time each of those forward-looking statements was made, the speaker had actual knowledge that the forward-looking statement was materially false or misleading, and/or the forward-looking statement was authorized or approved by an executive officer of PTC Therapeutics who knew that the statement was false when made.

COUNT I

Violation of Section 10(b) of The Exchange Act and Rule 10b-5 (Against All Defendants)

52. Plaintiff repeats and realleges each and every allegation contained above as if fully set forth herein.

53. During the Class Period, Defendants carried out a plan, scheme and course of conduct which was intended to and, throughout the Class Period, did: (i) deceive the investing public, including Plaintiff and other Class members, as alleged herein; and (ii) cause Plaintiff and other members of the Class to purchase PTC Therapeutics' securities at artificially inflated prices. In furtherance of this unlawful scheme, plan and course of conduct, defendants, and each of them, took the actions set forth herein.

54. Defendants (i) employed devices, schemes, and artifices to defraud; (ii) made untrue statements of material fact and/or omitted to state material facts necessary to make the statements not misleading; and (iii) engaged in acts, practices, and a course of business which operated as a fraud and deceit upon the purchasers of the Company's securities in an effort to maintain artificially high market prices for PTC Therapeutics' securities in violation of Section 10(b) of the Exchange Act and Rule 10b-5. All Defendants are sued either as primary participants in the wrongful and illegal conduct charged herein or as controlling persons as alleged below.

55. Defendants, individually and in concert, directly and indirectly, by the use, means or instrumentalities of interstate commerce and/or of the mails, engaged and participated in a continuous course of conduct to conceal adverse material information about PTC Therapeutics' financial well-being and prospects, as specified herein.

56. These defendants employed devices, schemes and artifices to defraud, while in possession of material adverse non-public information and engaged in acts, practices, and a course of conduct as alleged herein in an effort to assure investors of PTC Therapeutics' value and performance and continued substantial growth, which included the making of, or the participation in the making of, untrue statements of material facts and/or omitting to state

material facts necessary in order to make the statements made about PTC Therapeutics and its business operations and future prospects in light of the circumstances under which they were made, not misleading, as set forth more particularly herein, and engaged in transactions, practices and a course of business which operated as a fraud and deceit upon the purchasers of the Company's securities during the Class Period.

57. Each of the Individual Defendants' primary liability, and controlling person liability, arises from the following facts: (i) the Individual Defendants were high-level executives and/or directors at the Company during the Class Period and members of the Company's management team or had control thereof; (ii) each of these defendants, by virtue of their responsibilities and activities as a senior officer and/or director of the Company, was privy to and participated in the creation, development and reporting of the Company's internal budgets, plans, projections and/or reports; (iii) each of these defendants enjoyed significant personal contact and familiarity with the other defendants and was advised of, and had access to, other members of the Company's management team, internal reports and other data and information about the Company's finances, operations, and sales at all relevant times; and (iv) each of these defendants was aware of the Company's dissemination of information to the investing public which they knew and/or recklessly disregarded was materially false and misleading.

58. The defendants had actual knowledge of the misrepresentations and/or omissions of material facts set forth herein, or acted with reckless disregard for the truth in that they failed to ascertain and to disclose such facts, even though such facts were available to them. Such defendants' material misrepresentations and/or omissions were done knowingly or recklessly and for the purpose and effect of concealing PTC Therapeutics' financial well-being and prospects from the investing public and supporting the artificially inflated price of its securities. As

demonstrated by Defendants' overstatements and/or misstatements of the Company's business, operations, financial well-being, and prospects throughout the Class Period, Defendants, if they did not have actual knowledge of the misrepresentations and/or omissions alleged, were reckless in failing to obtain such knowledge by deliberately refraining from taking those steps necessary to discover whether those statements were false or misleading.

59. As a result of the dissemination of the materially false and/or misleading information and/or failure to disclose material facts, as set forth above, the market price of PTC Therapeutics' securities was artificially inflated during the Class Period. In ignorance of the fact that market prices of the Company's securities were artificially inflated, and relying directly or indirectly on the false and misleading statements made by Defendants, or upon the integrity of the market in which the securities trades, and/or in the absence of material adverse information that was known to or recklessly disregarded by Defendants, but not disclosed in public statements by Defendants during the Class Period, Plaintiff and the other members of the Class acquired PTC Therapeutics' securities during the Class Period at artificially high prices and were damaged thereby.

60. At the time of said misrepresentations and/or omissions, Plaintiff and other members of the Class were ignorant of their falsity, and believed them to be true. Had Plaintiff and the other members of the Class and the marketplace known the truth regarding the problems that PTC Therapeutics was experiencing, which were not disclosed by Defendants, Plaintiff and other members of the Class would not have purchased or otherwise acquired their PTC Therapeutics securities, or, if they had acquired such securities during the Class Period, they would not have done so at the artificially inflated prices which they paid.

61. By virtue of the foregoing, Defendants have violated Section 10(b) of the

Exchange Act and Rule 10b-5 promulgated thereunder.

62. As a direct and proximate result of Defendants' wrongful conduct, Plaintiff and the other members of the Class suffered damages in connection with their respective purchases and sales of the Company's securities during the Class Period.

COUNT II

Violation of Section 20(a) of The Exchange Act (Against the Individual Defendants)

63. Plaintiff repeats and realleges each and every allegation contained above as if fully set forth herein.

64. The Individual Defendants acted as controlling persons of PTC Therapeutics within the meaning of Section 20(a) of the Exchange Act as alleged herein. By virtue of their high-level positions, and their ownership and contractual rights, participation in and/or awareness of the Company's operations and/or intimate knowledge of the false financial statements filed by the Company with the SEC and disseminated to the investing public, the Individual Defendants had the power to influence and control and did influence and control, directly or indirectly, the decision-making of the Company, including the content and dissemination of the various statements which Plaintiff contends are false and misleading. The Individual Defendants were provided with or had unlimited access to copies of the Company's reports, press releases, public filings and other statements alleged by Plaintiff to be misleading prior to and/or shortly after these statements were issued and had the ability to prevent the issuance of the statements or cause the statements to be corrected.

65. In particular, each of these Defendants had direct and supervisory involvement in the day-to-day operations of the Company and, therefore, is presumed to have had the power to

control or influence the particular transactions giving rise to the securities violations as alleged herein, and exercised the same.

66. As set forth above, PTC Therapeutics and the Individual Defendants each violated Section 10(b) and Rule 10b-5 by their acts and/or omissions as alleged in this Complaint. By virtue of their positions as controlling persons, the Individual Defendants are liable pursuant to Section 20(a) of the Exchange Act. As a direct and proximate result of Defendants' wrongful conduct, Plaintiff and other members of the Class suffered damages in connection with their purchases of the Company's securities during the Class Period.

WHEREFORE, Plaintiff prays for relief and judgment, as follows:

(a) Determining that this action is a proper class action under Rule 23 of the Federal Rules of Civil Procedure;

(b) Awarding compensatory damages in favor of Plaintiff and the other Class members against all defendants, jointly and severally, for all damages sustained as a result of Defendants' wrongdoing, in an amount to be proven at trial, including interest thereon;

(c) Awarding Plaintiff and the Class their reasonable costs and expenses incurred in this action, including counsel fees and expert fees; and

(d) Such other and further relief as the Court may deem just and proper.

Dated: March 3, 2016