PTC Therapeutics Reports Third Quarter 2018 Financial Results and Provides a Corporate Update

November 5, 2018

-Completed acquisition of Agilis Biotherapeutics gene therapy platform-
-In-licensed two rare disease therapies from Akcea Therapeutics-

SOUTH PLAINFIELD, N.J., Nov. 5, 2018 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the third quarter ending September 30, 2018.

"We have been aggressively pursuing our vision to build a leading, fully integrated, multiplatform biotech company," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "The addition of gene therapy aligns with our goal of developing treatments for more patients with rare disorders and the in-licensing of Tegsedi™ and Waylivra™ leverages our commercial expertise. Over the last 20 years, our desire to bring new therapeutics to patients has been based on scientific innovation and we are continuing that mission."

Third Quarter Financial Highlights:

- Total revenues for the third quarter of 2018 were $53.6 million, compared to $41.9 million in the same period in 2017. The change in total revenue was primarily a result of revenue from Emflaza®, which launched in May 2017.
- Translarna™ net product revenues were $30.4 million for the third quarter of 2018, compared to $32.0 million reported in the third quarter of 2017.
- Emflaza net product revenues were $22.6 million for the third quarter of 2018, compared to $9.8 million reported in the third quarter of 2017.
- GAAP R&D expenses were $54.4 million for the third quarter of 2018, compared to $30.0 million for the same period in 2017. Non-GAAP R&D expenses were $49.9 million for the third quarter of 2018, excluding $4.4 million in non-cash, stock-based compensation expense, compared to $26.4 million for the same period in 2017, excluding $3.6 million in non-cash, stock-based compensation expense. The increase in GAAP and non-GAAP R&D expense was primarily due to increased investment in research programs and advancement of the clinical pipeline, as well as the Akcea Therapeutics, Inc. ("Akcea") upfront licensing fee of $12 million paid during the third quarter of 2018.
- GAAP SG&A expenses were $38.4 million for the third quarter of 2018, compared to $31.4 million for the same period in 2017. Non-GAAP SG&A expenses were $33.9 million for the third quarter of 2018, excluding $4.5 million in non-cash, stock-based compensation expense, compared to $27.9 million for the same period in 2017, excluding $3.5 million in non-cash, stock-based compensation expense. The increase in GAAP and non-GAAP SG&A expense was primarily due to continued investment in commercial activities for Emflaza and Translarna, as well as $1.5 million in expenses related to PTC's acquisition of Agilis Biotherapeutics, Inc.
- Net loss for the third quarter of 2018 was $51.0 million, compared to a net loss of $33.7 million for the same period in 2017.
- Cash, cash equivalents, and marketable securities totaled approximately $249.4 million at September 30, 2018, compared to approximately $191.2 million at December 31, 2017.
- Shares issued and outstanding as of September 30, 2018 were 50.4 million.

2018 Guidance:

- PTC now anticipates full year 2018 net product revenues to be between $260 and $280 million, a decrease in the high-end range of its prior guidance of between $260 and $295 million. PTC reiterates Translarna net product revenue for the full year 2018 to be between $170 and $185 million. PTC projects a 5-year (December 31, 2022) compound annual growth rate of 15% for net product revenues, representing continued strong growth year-over-year by increasing penetration in current countries and pursuing opportunities for label expansion. PTC now anticipates full year 2018 Emflaza net product revenue to be between $90 and $95 million, a decrease in the high-end range of its prior guidance of between $90 and $110 million.
- GAAP R&D and SG&A expense for the full year 2018 are now anticipated to be between $315 and $325 million, an increase from PTC's prior guidance of between $280 and $290 million. The increase in anticipated full year 2018 GAAP R&D and SG&A expense is primarily due to increased spend related to the Agilis acquisition and the Akcea upfront licensing fee of $12 million paid during the third quarter.
- Non-GAAP R&D and SG&A expense for the full year 2018 is now anticipated to be between $280 and $290 million, excluding estimated non-cash, stock-based compensation expense of approximately $35 million, an increase from PTC's prior guidance of between $250 and $260 million, excluding estimated non-cash, stock-based compensation expense of approximately $30 million.

Key Third Quarter and Other Corporate Highlights:

- **Completed acquisition of Agilis Biotherapeutics adding a Central Nervous System (CNS) gene therapy platform.** Acquisition included three programs in rare CNS disorders including Aromatic L-Amino Acid Decarboxylase (AADC), Friedreich Ataxia and Angelman Syndrome**.** PTC plans to file a biologics license application (BLA) in AADC in 2019. Pre-commercial efforts, such as patient identification efforts are ongoing. PTC estimates that there are 5,000 AADC deficiency patients worldwide with 1,200 patients in the United States. In addition, PTC plans to file an investigational new drug application (IND) in Friedreich Ataxia in 2019.
- **PTC in-licensed Latin America commercial rights to Tegsedi™ and Waylivra™ leverages strong commercia expertise.** Tegsedi has been approved in the United States, European Union, and Canada for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis (hATTR). The polyneuropathic form of hATTR, occurs more frequently in individuals of Portuguese ancestry, where PTC estimates approximately 6,000 patients in Latin America are affected. PTC has started patient identification efforts and plan to submit an application for Tegsedi with ANVISA, the Brazilian regulatory authority in the first half of 2019.
- **Initial STRIDE registry data demonstrates that Translarna delays loss of ambulation.** Preliminary data from the first international drug registry for Duchenne patients receiving Translarna demonstrated participants continuing to walk years longer and are remaining more physically able than untreated children. The data confirms Translarna's long-term clinical benefit in delaying irreversible muscle loss in patients and was presented to experts at the 23rd International Annual Congress of the World Muscle Society.
- **Pursing label expansion with European Medicines Agency (EMA) for Translarna for non-ambulatory patients.** In the third quarter, PTC filed for an extension of its existing label for Translarna to include non-ambulatory patients. The EMA has validated the application and the regulatory process is ongoing.
- **Continued advancement of the spinal muscular atrophy (SMA) program.** Data demonstrating the clinical benefits of risdiplam in all types of SMA were presented at the 23rd International Annual Congress of the World Muscle Society. Babies from FIREFISH Part 1 study showed increased functional developmental milestones including sitting. The pivotal portion of FIREFISH is enrolling. Clinical data was presented for the first time for the Type 2 & 3 patients from the open label portion of SUNFISH demonstrating a median 3-point increase in motor function score which was supported by the increase of SMN protein measured in the blood. The pivotal portion of SUNFISH Part 2 study in Type 2 & 3 patients has completed enrollment.
- **Development in oncology program with two clinical advancements.** PTC initiated a Phase 1 study evaluating the safety of PTC596 in patients with diffuse intrinsic pontine glioma (DIPG). Additionally, the PTC299 study is now actively enrolling patients in acute myeloid leukemia (AML).

Non-GAAP Financial Measures:
In this press release, the financial results and financial guidance of PTC are provided in accordance with accounting principles generally accepted in the United States (GAAP) and using certain non-GAAP financial measures. In particular, the non-GAAP financial measures exclude stock-based compensation expense. This non-GAAP financial measure is provided as a complement to financial measures reported in GAAP because management uses this non-GAAP financial measure when assessing and identifying operational trends. In management's opinion, this non-GAAP financial measure is useful to investors and other users of PTC's financial statements by providing greater transparency into the historical and projected operating performance of PTC and the company's future outlook. Quantitative reconciliations of non-GAAP financial measures to their closest equivalent GAAP financial measures are included in the tables below.

Today's Conference Call and Webcast Reminder:
Today's conference call will take place at 4:30 pm ET and can be access by dialing (877) 303-9216 (domestic) or (973) 935-8152 (international) five minutes prior to the start of the call and providing the passcode 2477754. A live, listen-only webcast of the conference call can be accessed on the investor relations section of the PTC website at www.ptcbio.com. The accompanying slide presentation will be posted on the investor relations section of the PTC website. A webcast replay of the call will be available approximately two hours after completion of the call and will be archived on the company's website for two weeks.

About PTC Therapeutics, Inc.
PTC is a science-led, global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. Founded 20 years ago, PTC Therapeutics has successfully launched two rare disorder products and has a global commercial footprint. This success is the foundation that drives investment in a robust pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need.

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Forward Looking Statements:

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this release, other than statements of historic fact, are forward-looking statements, including the information provided under the heading "2018 Guidance", including with respect to (i) 2018 net product revenue and net sales guidance for Translarna and Emflaza and (ii) 2018 GAAP and non-GAAP R&D and SG&A expense guidance, and statements regarding: the future expectations, plans and prospects for PTC; expectations with respect to PTC's recently acquired gene therapy platform, including any potential regulatory submissions; PTC's expectations with respect to the licensing and potential commercialization of Tegsedi and Waylivra; expansion of commercialization of Translarna and Emflaza; advancement of PTC's joint collaboration program in SMA; PTC's strategy, future operations, future financial position, future revenues, projected costs; or intended use of proceeds from its public offering of common stock; and the objectives of management. Other forward-looking statements may be identified by the words "guidance", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: the outcome of pricing, coverage and reimbursement negotiations with third party payors for Emflaza and Translarna and any other product candidates for which PTC may commercialize in the future; whether, and to what extent, third party payors impose additional requirements before approving Emflaza prescription reimbursement; PTC's ability to complete any dystrophin study necessary in order to resolve the matters set forth in the denial to the Complete Response letter it received from the FDA in connection with its new drug application for Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD), and PTC's ability to perform additional clinical trials, non-clinical studies, and CMC assessments or analyses at significant cost; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area (EEA), including whether the European Medicines Agency (EMA) determines in future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; PTC's ability to enroll, fund, complete and timely submit to the EMA the results of Study 041, a randomized, 18-month, placebo-controlled clinical trial of Translarna for the treatment of nmDMD followed by an 18-month open-label extension, which is a specific obligation to continued marketing authorization in the EEA; expectations with respect to the potential financial impact or PTC's ability to realize the anticipated benefits of the acquisition of Agilis and its gene therapy platform, including with respect to the business of Agilis and expectations with respect to the potential achievement of development, regulatory and sales milestones and contingent payments to the former Agilis equityholders with respect thereto and PTC's ability to obtain marketing approval of PTC-AADC and other product candidates acquired from Agilis, will not be realized or will not be realized within the expected time period; expectations with respect to the potential financial impact and benefits of the collaboration and licensing agreement with Akcea Therapeutics, Inc., including with respect to the timing of regulatory approval of Tegsedi and Waylivra in countries in LATAM and the Caribbean, the commercialization of Tegsedi and Waylivra, and PTC's expectations with respect to contingent payments to Akcea based on net sales and the potential achievement of regulatory milestones; PTC's ability to realize the anticipated benefits of the acquisition of Emflaza, including the possibility that the expected benefits from the acquisition will not be realized or will not be realized within the expected time period; significant transaction costs, unknown liabilities, the risk of litigation and/or regulatory actions related to the acquisition of Emflaza or the acquisition of its gene therapy pipeline, as well as other business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of Translarna, Emflaza, PTC-AADC, Tegsedi, Waylivra or any of PTC's other product candidates; the enrollment, conduct, and results of studies under the SMA collaboration and events during, or as a result of, the studies that could delay or prevent further development under the program; PTC's scientific approach and general development progress; PTC's ability to satisfy its obligations under the terms of the senior secured term loan facility with MidCap Financial; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; and the factors discussed in the "Risk Factors" section of PTC's Annual Report on Form 10-K for the year ended December 31, 2017, Quarterly Reports on Form 10-Q for the periods ended March 31, 2018, June 30, 2018 and September 30, 2018 and Exhibit 99.2 to PTC's Current Report on Form 8-K filed on August 24, 2018, as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC. You are urged to carefully consider all such factors.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Translarna, Emflaza, PTC-AADC, Tegsedi or Waylivra.

The forward-looking statements contained herein represent PTC's views only as of the date of this press release and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this press release except as required by law.

PTC Therapeutics, Inc
Consolidated Statements of Operations
(In thousands, except per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	**2017**	**2018**	**2017**
Revenues:				
Net product revenue	$ 53,021	$ 41,780	$ 177,172	$ 116,113
Collaboration and grant revenue	570	73	1,224	249
Total revenues	53,591	41,853	178,396	116,362
Operating expenses:				
Cost of product sales	3,292	1,582	8,909	2,142
Amortization of Acquired intangible asset	5,793	9,716	16,815	9,952
Research and development (1)	54,368	30,024	118,337	88,222
Selling, general and administrative (2)	38,368	31,423	104,882	85,788
Total operating expenses	101,821	72,745	248,943	186,104
Loss from operations	(48,230)	(30,892)	(70,547)	(69,742)
Interest expense, net	(3,118)	(3,421)	(9,306)	(8,648)
Other income (expense), net	734	766	1,066	(1,373)

Loss before income tax expense	(50,614)	(33,547)	(78,787)	(79,763)
Income tax expense	(355)	(191)	(964)	(507)
Net loss attributable to common stockholders	$ (50,969)	$ (33,738)	$ (79,751)	$ (80,270)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	48,096,521	41,296,740	45,310,690	38,433,749
Net loss per share—basic and diluted (in dollars per share)	$ (1.06)	$ (0.82)	$ (1.76)	$ (2.09)
(1) Research and development reconciliation				
GAAP research and development	$ 54,368	$ 30,024	$ 118,337	$ 88,222
Less: share-based compensation expense	4,431	3,624	12,109	11,986
Non-GAAP research and development	$ 49,937	$ 26,400	$ 106,228	$ 76,236
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 38,368	$ 31,423	$ 104,882	$ 85,788
Less: share-based compensation expense	4,511	3,544	12,664	12,096
Non-GAAP selling, general and administrative	$ 33,857	$ 27,879	$ 92,218	$ 73,692

PTC Therapeutics, Inc
Summary Consolidated Balance Sheets
(In thousands, except per share data)

	September 30, 2018	December 31, 2017
Cash, cash equivalents and marketable securities	$ 249,404	$ 191,246
Total assets	**$ 1,028,627**	**$ 391,653**
Total debt	$ 150,925	$ 144,971
Total deferred revenue	13,160	11,891
Total liabilities	**$ 642,537**	**$ 235,216**
Total stockholders' equity (50,432,655 and 41,612,395 common shares issued and outstanding at September 30, 2018 and December 31, 2017, respectively)	386,090	156,437
Total liabilities and stockholders' equity	**$ 1,028,627**	**$ 391,653**

PTC Therapeutics, Inc
Reconciliation of GAAP to Non-GAAP Projected Full Year R&D and SG&A (In thousands)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A expense	$ 315,000	$ 325,000
Less: projected shared-based compensation expense	35,000	35,000
Total projected non-GAAP R&D and SG&A expense	**$ 280,000**	**$ 290,000**

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