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

August 7, 2018

Entered into agreement to acquire Agilis Biotherapeutics
In-licensed LATAM commercial rights to Tegsedi™ (inotersen) and Waylivra™ (volanesorsen)
EC approval of Translarna™ label expansion to children as young as 2 years of age
Compelling data from FIREFISH study

SOUTH PLAINFIELD, N.J., Aug. 7, 2018 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the second quarter ending June 30, 2018.

"We have made significant progress towards delivering on our strategic plan this year," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "This includes furthering our underlying business as well as executing on two external transactions. We look forward to bringing value to both patients and shareholders with the acquisition of Agilis, the LATAM commercialization rights for Tegsedi™ and Waylivra™ and realizing additional growth in the second half of the year."

Second Quarter Financial Highlights:

- Total revenues for the second quarter of 2018 were $68.7 million, compared to $48.0 million in the same period in 2017. The change in total revenue was a result of Emflaza®, which launched in May 2017, and the expanded commercialization of Translarna™.
- Translarna net product revenues were $47.8 million for the second quarter of 2018, representing 4% growth over $45.8 million reported in the second quarter of 2017.
- Emflaza net product revenues were $20.3 million for the second quarter of 2018, from $2.1 million reported in the second quarter of 2017.
- GAAP R&D expenses were $32.6 million for the second quarter of 2018, compared to $30.8 million for the same period in 2017. Non-GAAP R&D expenses were $28.7 million for the second quarter of 2018, excluding $3.9 million in non-cash, stock-based compensation expense, compared to $26.9 million for the same period in 2017, excluding $3.9 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.
- GAAP SG&A expenses were $33.5 million for the second quarter of 2018, compared to $28.9 million for the same period in 2017. Non-GAAP SG&A expenses were $29.4 million for the second quarter of 2018, excluding $4.1 million in non-cash, stock-based compensation expense, compared to $24.9 million for the same period in 2017, excluding $4.0 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 to support the Duchenne muscular dystrophy franchise.
- Net loss for the second quarter of 2018 was $9.5 million, compared to a net loss of $17.5 million for the same period in 2017.
- In April 2018, PTC completed a public offering of 4,600,000 shares of common stock, resulting in net offering proceeds of $117.9 million.
- Cash, cash equivalents, and marketable securities totaled approximately $296.1 million at June 30, 2018, compared to approximately $191.2 million at December 31, 2017.
- Shares issued and outstanding as of June 30, 2018 were 46.7 million.

2018 Guidance

- Full year 2018 net product revenues are anticipated to be between $260 and $295 million. PTC anticipates 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 anticipates Emflaza net product revenue for the full year 2018 to be between $90 and $110 million.
- GAAP R&D and SG&A expense for the full year 2018 is anticipated to be between $280 and $290 million.
- Non-GAAP R&D and SG&A expense for the full year 2018 is anticipated to be between $250 and $260 million, excluding estimated non-cash, stock-based compensation expense of approximately $30 million.

Key Second Quarter and Other Corporate Highlights:

- **Agreement to acquire Agilis Biotherapeutics gene therapy platform and four CNS clinical assets diversify and strengthen PTC's existing pipeline**. Following successful completion of the transaction, PTC plans a smooth transition of

operations and to rapidly accelerate the programs. PTC plans to submit a Biologics Licensing Application with FDA in 2019 for the lead program in AADC deficiency, based on compelling long-term clinical data. An IND submission to the U.S. FDA is expected in 2019 for the second lead program in Friedreich ataxia. The transaction is expected to close in the third quarter of 2018.

- **In-licensed LATAM commercial rights to Tegsedi™ (inotersen) and Waylivra ™ (volanesorsen) from Akcea Therapeutics.** The collaboration leverages PTC's global commercial infrastructure and adds to growing commercial pipeline. Tegsedi has received marketing authorization approval from the European Commission for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis (hATTR amyloidosis). PTC estimates that there are approximately 6,000 patients in LATAM and that it is well positioned to file for registration in key countries in LATAM for Tegsedi. Tegsedi has a PDUFA date of October 6, 2018. Waylivra is under regulatory review in the U.S., Europe and Canada for the treatment of people with familial chylomicronemia syndrome (FCS). Waylivra recently received a positive vote from the FDA'sDivision of Metabolism and Endocrinology Products Advisory Committee and has a PDUFA date of August 30, 2018. Waylivra is also in clinical development for Familial Partial Lipodystrophy, or FPL.
- **Compelling data from FIREFISH study and advancement of SMA clinical programs.** Data presented at the CureSMA conference demonstrated that at Day 182, over 90% of the babies in the FIREFISH study achieved a greater than 4-point increase in CHOP-INTEND score compared to baseline. The CHOP-INTEND data were further supported by video presented by the investigator in which babies demonstrated improved motor function including head control, rolling from supine, and sitting. Three babies were depicted sitting independently. Updated SMA data including additional sitting babies from part 1 of the FIREFISH study will be presented at the upcoming World Muscle Congress. Part 2 of the pivotal FIREFISH study is ongoing. SUNFISH, a pivotal trial in Type 2/3 SMA patients is also ongoing.
- **European Commission ratifies CHMP's positive opinion for the label expansion of Translarna for children as young as 2 years of age**. The authorization allows PTC to market Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy in ambulatory patients aged two years and older in the 28 countries that are Member States of the European Union, as well as European Economic Area members Iceland, Liechtenstein and Norway.
- **Focus on intensifying the conversion of Emflaza bridge patients.** There are currently hundreds of patients who have been prescribed Emflaza for whom commercial shipments were not yet made. PTC plans to intensify the efforts and programs to expedite the conversion of those prescriptions to commercial shipment in the second half of the year.
- **Publication of additional data demonstrating the clinically differentiated benefit of deflazacort.** The results published in Muscle and Nerve demonstrated Duchenne muscular dystrophy patients treated with deflazacort had notably less decline from baseline in 6-minute walk distance at Week 48 than those treated with prednisone/prednisolone. The extrapolated time to loss of ambulation was 8.58 years for deflazacort and 4.74 years with prednisone/prednisolone. In addition to the time to delay of loss of ambulation, patients on deflazacort demonstrated a slowing of disease progression as measured by physical function endpoints.

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 8284468. A live, listen-only webcast of the conference call and corresponding slides can be accessed on the investor relations section of the PTC website at www.ptcbio.com. 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. PTC's current Investor Presentation and R&D Day slides are available at the same website location.

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. For more information, please visit our website at www.ptcbio.com.

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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; our expectations with respect to the closing of our planned acquisition of Agilis Biotherapeutics, Inc. and potential subsequent regulatory submissions of any product candidates acquired upon closing; our 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; 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 NDA 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; PTC's expectations with respect to the closing of its planned acquisition of Agilis, and the other transactions contemplated in conjunction with the acquisition, including with respect to matters of timing, including the satisfaction of closing conditions, the anticipated financial impact and potential benefits to PTC, integration of Agilis into PTC's business and any product candidates PTC may acquire from Agilis into its business strategy assuming completion of the acquisition and other matters related to the acquisition; 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 planned acquisition of Agilis, 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, Tegsedi, Waylivra, PTC's other product candidates and any product candidates PTC may acquire upon completion of our planned acquisition of Agilis; 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 most recent Quarterly Report on Form 10-Q and Annual Report on Form 10-K 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, 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 June 30,		Six Months Ended June 30,	
	2018	**2017**	**2018**	**2017**
Revenues:				
Net product revenue	$ 68,170	$ 47,891	$ 124,151	$ 74,334
Collaboration and grant revenue	573	71	654	176
Total revenues	68,743	47,962	124,805	74,510
Operating expenses:				

Cost of product sales	2,572	758	5,616	797
Amortization of Acquired intangible asset	5,593	-	11,022	-
Research and development (1)	32,607	30,835	63,970	58,198
Selling, general and administrative (2)	33,545	28,866	66,514	54,365
Total operating expenses	74,317	60,459	147,122	113,360
Loss from operations	(5,574)	(12,497)	(22,317)	(38,850)
Interest expense, net	(2,884)	(3,008)	(6,187)	(5,227)
Other (expense) income, net	(673)	(1,820)	332	(2,139)
Loss before income tax expense	(9,131)	(17,325)	(28,172)	(46,216)
Income tax expense	(389)	(150)	(610)	(316)
Net loss attributable to common stockholders	$ (9,520)	$ (17,475)	$ (28,782)	$ (46,532)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	46,137,833	39,621,738	46,257,397	36,978,528
Net loss per share—basic and diluted (in dollars per share)	$ (0.21)	$ (0.44)	$ (0.62)	$ (1.26)
(1) Research and development reconciliation				
GAAP research and development	$ 32,607	$ 30,835	$ 63,970	$ 58,198
Less: share-based compensation expense	3,932	3,895	7,678	8,362
Non-GAAP research and development	$ 28,675	$ 26,940	$ 56,292	$ 49,836
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 33,545	$ 28,866	$ 66,514	$ 54,365
Less: share-based compensation expense	4,152	3,990	8,153	8,552
Non-GAAP selling, general and administrative	$ 29,393	$ 24,876	$ 58,361	$ 45,813

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

	June 30, 2018	December 31, 2017
Cash, cash equivalents and marketable securities	$ 296,106	$ 191,246
Total assets	**$ 511,773**	**$ 391,653**
Total debt	$ 148,870	$ 144,971
Total deferred revenue	10,540	11,891
Total liabilities	**$ 244,371**	**$ 235,216**
Total stockholders' equity (46,680,482 and 41,612,395 common shares issued and outstanding at June 30, 2018 and December 31, 2017, respectively)	267,402	156,437
Total liabilities and stockholders' equity	**$ 511,773**	**$ 391,653**

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

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A expense	$ 280,000	$ 290,000
Less: projected shared-based compensation expense	30,000	30,000
Total projected non-GAAP R&D and SG&A expense	**$ 250,000**	**$ 260,000**

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