

**UNITED STATES**  
**SECURITIES AND EXCHANGE COMMISSION**

WASHINGTON, D.C. 20549

**FORM 8-K**

**CURRENT REPORT**

**Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934**

Date of Report (Date of earliest event reported): **July 17, 2020**

**PTC THERAPEUTICS, INC.**

(Exact Name of Company as Specified in Charter)

|  |   |   |
|--|---|---|
| <b>Delaware</b><br>(State or Other Jurisdiction<br>of Incorporation) | <b>001-35969</b><br>(Commission<br>File Number) | <b>04-3416587</b><br>(IRS Employer<br>Identification No.) |
|--|---|---|

|   |                            |
|---|----------------------------|
| <b>100 Corporate Court</b><br><b>South Plainfield, NJ</b><br>(Address of Principal Executive Offices) | <b>07080</b><br>(Zip Code) |
|---|----------------------------|

Registrant's telephone number, including area code: (908) **222-7000**

**Not applicable**

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

**Securities registered pursuant to Section 12(b) of the Act:**

| <u>Title of each class</u>                | <u>Trading Symbol(s)</u> | <u>Name of each exchange on which registered</u> |
|---|--------------------------|--|
| Common Stock, \$0.001 par value per share | PTCT                     | Nasdaq Global Select Market                      |

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

**Item 1.01. Entry into a Material Definitive Agreement.**

On July 17, 2020, PTC Therapeutics, Inc. (the “Company”), RPI 2019 Intermediate Finance Trust (“RPI”), and, for the limited purposes set forth in the agreement, Royalty Pharma PLC, entered into a Royalty Purchase Agreement (the “Royalty Purchase Agreement”). Pursuant to the Royalty Purchase Agreement, the Company sold to RPI 42.933% (the “Assigned Royalty Payment”) of the Company’s right to receive sales-based royalty payments (the “Royalty”) on worldwide net sales of Roche’s risdiplam product and any other product (the “Products”) developed pursuant to the License and Collaboration Agreement (the “License Agreement”), dated as of November 23, 2011, by and among the Company, F. Hoffman-La Roche Ltd., Hoffman-La Roche Inc. (together with F. Hoffman-La Roche Ltd, “Roche”), and, for the limited purposes set forth therein, the Spinal Muscular Atrophy Foundation (the “SMA Foundation”) under the SMA program, which is a collaboration among the Company, Roche and the SMA Foundation. In consideration for the sale of the Assigned Royalty Payments, RPI paid the Company \$650.0 million in cash consideration. The Company will retain a 57.067% interest in the Royalty and all economic rights to receive the remaining potential regulatory and sales milestone payments under the License Agreement, which milestone payments equal approximately \$400 million in the aggregate.

Under the Royalty Purchase Agreement, and in connection with its sale of the Assigned Royalty Payments, the Company has agreed to specified negative and affirmative covenants with respect to the exercise of its rights under the License Agreement, including the Company’s right to amend, modify, assign or terminate the License Agreement. The Company has agreed not to exercise its rights under the License Agreement in any manner that would be expected to adversely impact the Royalty or the Products. The Company must also enforce the terms of the License Agreement as RPI reasonably requests in the event of a breach of the License Agreement by Roche or the SMA Foundation. Subject to the satisfaction of certain conditions, the Company has also agreed to consult with RPI or act at RPI’s direction with respect to the Company’s exercise of its rights to enforce, defend, prosecute and maintain intellectual property rights under the License Agreement. Subject to certain customary exceptions, the Company has agreed not to grant a security interest in its interest in the License Agreement, the Products or the patent rights that cover the Products. The Royalty Purchase Agreement also contains representations and warranties, covenants and other negotiated provisions, including information rights and confidentiality obligations, customary for transactions of this nature. The Royalty Purchase Agreement will terminate 60 days following the earlier of the date on which Roche is no longer obligated to make any payments of the Royalty pursuant to the License Agreement and the date on which RPI has received one billion three hundred million U.S. dollars in respect of the Assigned Royalty Payments.

If, in the event of a termination of the License Agreement by Roche for convenience or by the Company for Roche’s material breach or patent challenge and the Company is then the owner of a majority of the Royalty based on consensus projections of the value of the Royalty and the Company has not otherwise undergone a change of control, the Company will have sole discretion with respect to the continued commercialization of the Products pursuant to the rights provided to the Company upon such termination of the License Agreement. In the event that the Company is not the owner of a majority of the Royalty based on consensus projections of the value of the Royalty, or if the Company has undergone a change of control, then, upon termination of the License Agreement by Roche for convenience or by the Company for Roche’s material breach or patent challenge, (1) the Company will have the first right to commercialize the Products pursuant to the rights provided to the Company upon such termination of the License Agreement and (2) if the Company does not elect to commercialize the Products, RPI will have the right to direct the Company to commercialize the Products pursuant to the rights provided to the Company upon such termination of the License Agreement, including by negotiating a license or sublicense with one or more third parties to commercialize a Product that has obtained regulatory approval.

The foregoing description of the Royalty Purchase Agreement does not purport to be complete and is qualified in its entirety by reference to the complete text of the Royalty Purchase Agreement, which will be filed as an exhibit to the Company’s quarterly report on Form 10-Q for the fiscal quarter ending June 30, 2020.

**Item 7.01. Regulation FD Disclosure.**

On July 20, 2020, the Company issued a press release in which it announced the closing of the Royalty Purchase Agreement. A copy of the press release is attached to this Current Report on Form 8-K (this “Report”) as Exhibit 99.1 and is incorporated by reference into this Item 7.01.

The information set forth in or incorporated by reference into this Item 7.01, including Exhibit 99.1, shall not be deemed to be “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), or otherwise subject to the liability of that section, and shall not be incorporated by reference into any registration statement or other document filed under the Securities Act of 1933 or the Exchange Act, except as shall be expressly set forth by specific reference in such filing.

**Item 9.01. Financial Statements and Exhibits.**

(d) Exhibits

| <b>Exhibit No.</b> | <b>Description</b>   |
|--------------------|--|
| 99.1               | <a href="#">Press Release, dated July 20, 2020, issued by PTC Therapeutics, Inc.</a> |
| 104                | The cover page from this Current Report on Form 8-K, formatted in Inline XBRL        |

**Cautionary Statement Concerning Forward Looking Statements**

This Report contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this Report, other than those of historical fact, are forward-looking statements, including statements regarding: the future expectations, plans and prospects for the Company; advancement of the Company's joint collaboration program in SMA, including any potential regulatory submissions, regulatory approvals or commercial prospects; the Company's strategy, future operations, future financial position, future revenues and projected costs; the Company's expected use of proceeds from the agreement with RPI; potential royalties and potential regulatory and sales milestone payments; 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. The Company'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 enrollment, conduct, and results of studies under the SMA program and events during, or as a result of, the studies that could delay or prevent further development under the SMA program, including any potential regulatory submissions and potential commercialization with regard to risdiplam; the eligible patient base and commercial potential of risdiplam or any of the Company's other product candidates; and the factors discussed in the "Risk Factors" section of the Company'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 the Company's other filings with the Securities and Exchange Commission. 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 or product candidate will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including risdiplam. The forward-looking statements contained herein represent the Company's views only as of the date of this Report and the Company 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 Report except as required by law. All website addresses given in this Report or incorporated herein by reference are for information only and are not intended to be an active link or to incorporate any website information into this Report.

**Signature**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this Report to be signed on its behalf by the undersigned hereunto duly authorized.

**PTC Therapeutics, Inc.**

Date: July 20, 2020

By: /s/ Mark Boulding  
Name: Mark Boulding  
Title: Executive Vice President and Chief Legal Officer



## PTC Therapeutics Announces Agreement to Monetize a Portion of the Risdiplam Royalty Stream for \$650 Million

*- Strategic partnership with Royalty Pharma plc. enables PTC to strengthen and advance its diversified rare disorders portfolio –*

*- Conference call scheduled for 8:30 am ET –*

**SOUTH PLAINFIELD, N.J., July 20, 2020** – PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced an agreement to monetize a portion of the risdiplam royalty stream for \$650 million from Royalty Pharma plc. The capital from the collaboration will enable PTC to further develop and expand its innovative rare disorder portfolio, particularly its validated splicing, Bio-e and gene therapy platforms.

“The discovery, development and expected commercialization of risdiplam exemplifies PTC’s strengths in novel scientific approaches to diseases with high unmet needs can generate value for the benefit of all of our stakeholders,” said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. “Today’s announcement of our strategic partnership with Royalty Pharma brings forward significant, non-dilutive capital to drive further innovation and growth across our robust and diverse rare disorder portfolio.”

Under the terms of the royalty purchase agreement, PTC Therapeutics will receive \$650 million in upfront cash from Royalty Pharma in return for approximately 43% of the risdiplam royalties, up to a specified amount. PTC Therapeutics maintains the majority of the risdiplam royalty stream and retains all economics associated with up to \$400 million in remaining regulatory and sales milestones.

Pablo Legorreta, Royalty Pharma’s Founder and Chief Executive Officer, stated, “Risdiplam is consistent with our focus on high value, differentiated therapeutics addressing diseases with high unmet medical need. We recognize the value and importance of an oral therapy for the treatment of all types of SMA. We are delighted to partner with PTC and to help fund their innovative pipeline of treatments for rare diseases.”

Wilmer Cutler Pickering Hale and Dorr LLP acted as legal advisor to PTC Therapeutics on the transaction. Goodwin Procter, Dechert and Maiwald acted as legal advisors to Royalty Pharma.

The risdiplam New Drug Application (NDA) for the treatment of Types 1, 2 and 3 spinal muscular atrophy (SMA) is under Priority Review by the U.S Food and Drug Administration, with a PDUFA date of August 24, 2020. A Marketing Authorization Application (MAA) is planned to be submitted to the European Medicines Agency (EMA), as well as filings in other international markets. The risdiplam SMA program is a collaboration between PTC, the SMA Foundation, and Roche.

### **Today's Conference Call:**

Today's conference call will take place at 8:30 am ET and can be accessed by dialing (877) 303-9216 (domestic) or (973) 935-8152 (international) five minutes prior to the start of the call and providing the passcode 3492277. 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](http://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 PTC’s website for 30 days following the call.

**About Risdiplam**

Risdiplam is an investigational survival motor neuron 2 (SMN2) splicing modifier for SMA and is an orally administered liquid. It is designed to increase and sustain SMN protein levels both throughout the central nervous system and in peripheral tissues of the body. Risdiplam is being studied in the broadest clinical trial program in SMA, with patients ranging from birth to 60 years old, and includes patients previously treated with other SMA-targeting therapies. The clinical trial population represents the diverse, real-world spectrum of people living with this disease. The risdiplam clinical development program was designed with the aim of enabling access for all appropriate patients.

**About PTC Therapeutics, Inc.**

PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. To learn more about PTC, please visit us at [www.ptcbio.com](http://www.ptcbio.com) and follow us on Facebook, on Twitter at @PTCBio, and on LinkedIn.

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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 historical fact, are forward-looking statements, including statements regarding: the future expectations, plans and prospects for PTC; advancement of PTC's joint collaboration program in SMA, including any potential regulatory submissions, regulatory approvals or commercial prospects; PTC's strategy, future operations, future financial position, future revenues and, projected costs; PTC's expected use of proceeds from the agreement with Royalty Pharma; potential royalties and potential regulatory and sales milestone payments; 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 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, including any potential regulatory submissions and potential commercialization with regards to risdiplam; the eligible patient base and commercial potential of risdiplam or any of PTC's other product candidates; 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 or product candidate will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including risdiplam.

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.

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