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**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): **August 8, 2024**

**PTC THERAPEUTICS, INC.**

(Exact Name of Company as Specified in Charter)

**Delaware**  
(State or Other Jurisdiction  
of Incorporation)

**001-35969**  
(Commission  
File Number)

**04-3416587**  
(IRS Employer  
Identification No.)

**500 Warren Corporate Center Drive**  
**Warren, NJ**  
(Address of Principal Executive Offices)

**07059**  
(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.

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**Item 2.02. Results of Operations and Financial Condition.**

On August 8, 2024, PTC Therapeutics, Inc. (the “Company”) announced its financial results for the quarter ended June 30, 2024. The full text of the press release issued in connection with the announcement is furnished as Exhibit 99.1 to this Current Report on Form 8-K (this “Report”) and is incorporated by reference into this Item 2.02.

**Item 7.01. Regulation FD Disclosure.**

The Company will host a conference call on August 8, 2024 at 4:30 PM eastern time, as previously announced. During this call the Company expects to review financial results for the quarter ended June 30, 2024, as well as other corporate highlights and updates.

Directions on how to access the conference call are included in the press release furnished as Exhibit 99.1 hereto.

The information in this Report (including Items 2.02 and 7.01 and Exhibit 99.1) shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing. 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.

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

(d) Exhibits

| <b>Exhibit No.</b> | <b>Description</b>   |
|--------------------|--|
| 99.1               | <a href="#">Press Release, dated August 8, 2024 issued by PTC Therapeutics, Inc.</a> |
| 104                | The cover page from this Current Report on Form 8-K, formatted in Inline XBRL        |

**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: August 8, 2024

By: /s/ Pierre Gravier  
Name: Pierre Gravier  
Title: Chief Financial Officer

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**PTC Therapeutics Provides Corporate Update and  
Reports Second Quarter 2024 Financial Results**

*– Continued strong revenue performance –*

*– NDAs for sepiapterin and Translarna™ submitted to FDA –*

*– Positive interim data readout from PTC518 PIVOT-HD study –*

*– On track to achieve remaining 2024 clinical and regulatory milestones –*

**WARREN, N.J., August 8, 2024** – PTC Therapeutics, Inc., (NASDAQ: PTCT) today announced a corporate update and financial results for the second quarter ending June 30, 2024.

"I am proud of our team's continued outstanding execution as we have accomplished all our objectives so far this year," said Matthew Klein, M.D., Chief Executive Officer. "We are in a strong cash position, submitted three drug approval applications to the FDA and remain on schedule to achieve the many milestones we have set for the remainder of 2024."

**Key Corporate Updates:**

- Second quarter 2024 total revenue of \$187 million
- Second quarter 2024 revenue for the DMD franchise was \$118 million
  - Translarna™ (ataluren) net product revenue was \$70 million, driven by continued access for patients in existing geographies and continued geographic expansion.
  - Emflaza® (deflazacort) net product revenue was \$47 million, driven by new patient starts and continued brand loyalty.

**Key Clinical and Regulatory Milestones:**

- PTC submitted an NDA to the FDA for sepiapterin for the treatment of PKU in July 2024. Further regulatory submissions are planned for Japan and Brazil later in 2024.
- PTC resubmitted the NDA for Translarna for the treatment of nmDMD in July 2024.
- PTC achieved all objectives in the interim readout of the Phase 2 PIVOT-HD study of PTC518 in Huntington's disease patients, which were disclosed in the second quarter.
- PTC plans to submit an NDA for vatiquinone for the treatment of Friedreich ataxia in late 2024.
- PTC expects to share topline data for the CardinALS trial of utreloxastat for the treatment of ALS in the fourth quarter of 2024.
- The BLA for PTC's gene therapy for AADC deficiency was accepted by FDA with priority review; the target regulatory action date is November 13, 2024.

**Second-Quarter 2024 Financial Highlights:**

- Total revenues were \$186.7 million for the second quarter of 2024, compared to \$213.8 million for the second quarter of 2023.
  - Total revenues include net product revenue across the commercial portfolio of \$133.2 million for the second quarter of 2024, compared to \$174.6 million for the second quarter of 2023. Total revenues
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also include royalty and manufacturing revenue of \$53.5 million for the second quarter of 2024, compared to \$39.2 million for the second quarter of 2023.

- Translarna net product revenues were \$70.4 million for the second quarter of 2024, compared to \$96.5 million for the second quarter of 2023. These results were due to the timing of bulk government orders.
- Emflaza net product revenues were \$47.3 million for the second quarter of 2024, compared to \$65.7 million for the second quarter of 2023. These results were driven by the expiration of Emflaza's orphan drug exclusivity in February 2024.
- Roche reported Evrysdi 2024 year-to-date sales of approximately CHF 838 million, resulting in royalty revenue of \$53.2 million to PTC for the second quarter of 2024, as compared to \$36.9 million for the second quarter of 2023.
- Based on U.S. GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were \$132.2 million for the second quarter of 2024, compared to \$185.9 million for the second quarter of 2023. The decrease in quarterly research and development expenses reflects strategic portfolio prioritization as PTC continues to focus its resources on its differentiated, high-potential programs. Included in second quarter 2024 R&D expense is a \$15.0 million regulatory success-based milestone related to the Censa acquisition.
- Non-GAAP R&D expenses were \$122.7 million for the second quarter of 2024, excluding \$9.4 million in non-cash, stock-based compensation expense, compared to \$170.3 million for the second quarter of 2023, excluding \$15.5 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were \$69.5 million for the second quarter of 2024, compared to \$88.4 million for the second quarter of 2023. The decrease in quarterly selling, general and administrative expenses reflects lower employee costs due to the reduction in workforce in 2023.
- Non-GAAP SG&A expenses were \$59.7 million for the second quarter of 2024, excluding \$9.8 million in non-cash, stock-based compensation expense, compared to \$74.6 million for the second quarter of 2023, excluding \$13.8 million in non-cash, stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was a loss of \$5.1 million for the second quarter of 2024, compared to a gain of \$128.9 million for the second quarter of 2023. The change in fair value of contingent consideration is primarily related to the strategic portfolio prioritization and the decision to discontinue the Friedreich ataxia and Angelman syndrome gene therapy programs in the second quarter of 2023. As a result, PTC determined that the fair value for all of the contingent consideration payable related to Friedreich ataxia and Angelman syndrome was \$0 and recorded a gain of \$129.8 million.
- Tangible asset impairment and losses (gains) on transactions, net, was a loss of \$1.8 million for the second quarter of 2024, compared to \$0 million for the second quarter of 2023. These results were due to the sale of certain assets related to gene therapy manufacturing and fixed asset impairments, partially offset by a gain on lease terminations and a gain on sales of certain fixed assets.
- Net loss was \$99.2 million for the second quarter of 2024, compared to net loss of \$198.9 million for the second quarter of 2023.
- Cash, cash equivalents, and marketable securities was \$1,093.3 million on June 30, 2024, compared to \$876.7 million on December 31, 2023. During the second quarter of 2024, PTC amended its agreement with Royalty Pharma and exercised one of its put options in exchange for \$250 million in cash, less royalties received.
- Shares issued and outstanding as of June 30, 2024, were 76,900,123.

**PTC Updates Full Year 2024 Financial Guidance:**

- PTC anticipates total revenues for full year 2024 to be between \$700 million and \$750 million.
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- PTC anticipates GAAP R&D and SG&A expenses for full year 2024 to be between \$740 million and \$835 million, including expected R&D expense milestone payments of up to \$65 million.
- PTC anticipates Non-GAAP R&D and SG&A expenses for full year 2024 to be between \$660 million and \$755 million, including expected R&D expense milestone payments of up to \$65 million and excluding estimated non-cash, stock-based compensation expense of \$80 million.
- PTC anticipates up to \$90 million of payments for full year 2024 upon achievement of potential regulatory success-based milestones from previous acquisitions, of which up to \$65 million will be recorded as R&D operating expenses. As of June 30, 2024, \$35 million in milestones have been achieved, of which \$15 million has been recorded as R&D operating expenses and \$20 million as contingent consideration payable.

**Non-GAAP Financial Measures:**

In this press release, the financial results of PTC are provided in accordance with GAAP and using certain non-GAAP financial measures. In particular, the non-GAAP R&D and SG&A expense financial measures exclude non-cash, stock-based compensation expense. These non-GAAP financial measures are provided as a complement to financial measures reported in GAAP because management uses these non-GAAP financial measures when assessing and identifying operational trends. In management's opinion, these non-GAAP financial measures are 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. Non-GAAP financial measures are not an alternative for financial measures prepared in accordance with GAAP. Quantitative reconciliations of the non-GAAP financial measures to their respective closest equivalent GAAP financial measures are included in the table below.

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

|   | <b>Three Months Ended June 30,</b> |                     | <b>Six Months Ended June 30,</b> |                     |
|---|------------------------------------|---------------------|----------------------------------|---------------------|
|   | <b>2024</b>                        | <b>2023</b>         | <b>2024</b>                      | <b>2023</b>         |
| <b>Revenues:</b>  |                                    |                     |                                  |                     |
| Net product revenue   | \$ 133,220                         | \$ 174,592          | \$ 310,824                       | \$ 362,149          |
| Collaboration revenue   | -                                  | -                   | -                                | 6                   |
| Royalty revenue   | 53,183                             | 36,853              | 84,337                           | 67,684              |
| Manufacturing revenue   | 301                                | 2,363               | 1,661                            | 4,351               |
| <b>Total revenues</b>   | <b>186,704</b>                     | <b>213,808</b>      | <b>396,822</b>                   | <b>434,190</b>      |
| <b>Operating expenses:</b>  |                                    |                     |                                  |                     |
| Cost of product sales, excluding amortization of acquired intangible assets | 15,527                             | 12,731              | 30,267                           | 26,875              |
| Amortization of acquired intangible asset                                   | 2,865                              | 47,397              | 54,395                           | 86,812              |
| Research and development (1)  | 132,169                            | 185,874             | 248,298                          | 380,998             |
| Selling, general and administrative (2)                                     | 69,500                             | 88,449              | 142,772                          | 175,363             |
| Change in the fair value of deferred and contingent consideration           | 5,100                              | (128,900)           | 5,000                            | (126,500)           |
| Intangible asset impairment   | -                                  | 217,800             | -                                | 217,800             |
| Tangible asset impairment and losses (gains) on transactions, net           | 1,761                              | -                   | 1,761                            | -                   |
| <b>Total operating expenses</b>   | <b>226,922</b>                     | <b>423,351</b>      | <b>482,493</b>                   | <b>761,348</b>      |
| Loss from operations  | (40,218)                           | (209,543)           | (85,671)                         | (327,158)           |
| Interest expense, net   | (43,490)                           | (29,415)            | (84,324)                         | (56,745)            |
| Other (expense) income, net   | (2,025)                            | 1,479               | (434)                            | 11,434              |
| Loss before income tax (expense) benefit                                    | (85,733)                           | (237,479)           | (170,429)                        | (372,469)           |
| Income tax (expense) benefit  | (13,446)                           | 38,596              | (20,326)                         | 34,627              |
| <b>Net loss attributable to common stockholders</b>                         | <b>\$ (99,179)</b>                 | <b>\$ (198,883)</b> | <b>\$ (190,755)</b>              | <b>\$ (337,842)</b> |
| <b>Weighted-average shares outstanding:</b>                                 |                                    |                     |                                  |                     |
| Basic and diluted (in shares)   | 76,725,070                         | 74,730,433          | 76,610,598                       | 74,232,624          |
| <b>Net loss per share—basic and diluted (in dollars per share)</b>          | <b>\$ (1.29)</b>                   | <b>\$ (2.66)</b>    | <b>\$ (2.49)</b>                 | <b>\$ (4.55)</b>    |
| <b>(1) Research and development reconciliation</b>                          |                                    |                     |                                  |                     |
| GAAP research and development   | \$ 132,169                         | \$ 185,874          | \$ 248,298                       | \$ 380,998          |
| Less: share-based compensation expense                                      | 9,428                              | 15,529              | 18,395                           | 30,842              |
| <b>Non-GAAP research and development</b>                                    | <b>\$ 122,741</b>                  | <b>\$ 170,345</b>   | <b>\$ 229,903</b>                | <b>\$ 350,156</b>   |
| <b>(2) Selling, general and administrative reconciliation</b>               |                                    |                     |                                  |                     |
| GAAP selling, general and administrative                                    | \$ 69,500                          | \$ 88,449           | \$ 142,772                       | \$ 175,363          |
| Less: share-based compensation expense                                      | 9,815                              | 13,842              | 19,226                           | 27,344              |
| <b>Non-GAAP selling, general and administrative</b>                         | <b>\$ 59,685</b>                   | <b>\$ 74,607</b>    | <b>\$ 123,546</b>                | <b>\$ 148,019</b>   |

**PTC Therapeutics, Inc.**  
**Summary Consolidated Balance Sheets**  
(in thousands, except share data)

|   | <b>June 30, 2024</b> | <b>December 31, 2023</b> |
|---|----------------------|--------------------------|
| Cash, cash equivalents and marketable securities  | \$ 1,093,293         | \$ 876,739               |
| <b>Total Assets</b>   | <b>\$ 1,916,355</b>  | <b>\$ 1,895,698</b>      |
| Total debt  | \$ 284,806           | \$ 284,213               |
| Total deferred revenue  | -                    | 801                      |
| Total liability for sale of future royalties  | 2,084,880            | 1,814,097                |
| <b>Total liabilities</b>  | <b>\$ 2,896,536</b>  | <b>\$ 2,714,253</b>      |
| Total stockholders' deficit (76,900,123 and 75,708,889 common shares issued and outstanding at June 30, 2024 and December 31, 2023, respectively) | \$ (980,181)         | \$ (818,555)             |
| <b>Total liabilities and stockholders' deficit</b>  | <b>\$ 1,916,355</b>  | <b>\$ 1,895,698</b>      |

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**PTC Therapeutics, Inc.**  
**Reconciliation of GAAP Milestone Payments Full Year 2024**  
(in millions)

|  | <b>Milestone Payments</b> |
|--|---------------------------|
|  | <b>Full Year 2024</b>     |
|  | (in millions)             |
| Projected GAAP R&D Expense Related Milestone Payments                      | \$ 65                     |
| Projected GAAP Contingent Consideration Payable Related Milestone Payments | 25                        |
| <b>Total Projected GAAP Milestone Payments</b>                             | <b>\$ 90</b>              |

**PTC Therapeutics, Inc.**  
**Reconciliation of GAAP to Non-GAAP Projected Full Year 2024 R&D and SG&A Expense**  
(In millions)

|  | <b>Low End of Range</b> |           | <b>High End of Range</b> |
|--|-------------------------|-----------|--------------------------|
| Projected GAAP R&D and SG&A Expense                        | \$ 740                  | \$        | 835                      |
| Less: projected non-cash, stock-based compensation expense | 80                      |           | 80                       |
| <b>Projected non-GAAP R&amp;D and SG&amp;A expense</b>     | <b>\$ 660</b>           | <b>\$</b> | <b>755</b>               |

**Acronyms:**

AADC: Aromatic L-Amino Acid Decarboxylase  
ALS: Amyotrophic Lateral Sclerosis  
BLA: Biologics License Application  
CHF: Confoederatio Helvetica Francs (Swiss francs)  
DMD: Duchenne Muscular Dystrophy  
EMA: European Medicines Agency  
FA: Friedreich Ataxia  
FDA: U.S. Food and Drug Administration  
GAAP: Generally Accepted Accounting Principles  
HD: Huntington's Disease  
MAA: Marketing Authorization Application  
NDA: New Drug Application  
nmDMD: Nonsense Mutation Duchenne Muscular Dystrophy  
PKU: Phenylketonuria  
R&D: Research and Development  
SG&A: Selling, General, and Administrative

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**Today's Conference Call and Webcast Reminder:**

To access the call by phone, please click [here](#) to register and you will be provided with dial-in details. To avoid delays, we recommend participants dial in to the conference call 15 minutes prior to the start of the call. The webcast conference call can be accessed on the Investor section of the PTC website at <https://ir.ptcbio.com/events-presentations>. A 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 30 days following the call.

**About PTC Therapeutics, Inc.**

PTC is a 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. The company's strategy is to leverage its strong scientific expertise and global commercial infrastructure to maximize value for its patients and other stakeholders. 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.

**For More Information:****Investors:**

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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 "PTC Updates Full Year 2024 Revenue Guidance", including with respect to (i) 2024 total revenue guidance, (ii) 2024 GAAP and non-GAAP R&D and SG&A expense guidance and (iii) 2024 acquisition related milestone payment guidance, and statements regarding: the future expectations, plans and prospects for PTC, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses, commercialization and other matters with respect to its products and product candidates; PTC's strategy, future operations, future financial position, future revenues, projected costs; the extent, timing and financial aspects of our strategic pipeline prioritization and reductions in workforce; 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.

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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 PTC's products or product candidates that PTC commercializes or may commercialize in the future; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in Brazil, Russia, the European Economic Area (EEA) and other regions, including whether the European Medicines Agency (EMA) determines in the re-examination process that the benefit-risk balance for the conditional marketing authorization for Translarna supports renewal of such authorization, or PTC's ability to identify other potential mechanisms by which it may provide Translarna to nmDMD patients in the EEA; PTC's ability to use the clinical data from its international drug registry study and real-world evidence concerning Translarna's benefits to support a continued marketing authorization for Translarna for the treatment of nmDMD in the EEA; PTC's ability to use 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, and from its international drug registry study to support a marketing approval for Translarna for the treatment of nmDMD in the United States; whether investigators agree with PTC's interpretation of the results of clinical trials and the totality of clinical data from its trials in Translarna; expectations with respect to Upstaza, including any regulatory submissions and potential approvals, commercialization, manufacturing capabilities, the potential achievement of development, regulatory and sales milestones and contingent payments that PTC may be obligated to make; expectations with respect to sepiapterin, including any regulatory submissions and potential approvals, commercialization, the potential achievement of development, regulatory and sales milestones and contingent payments that PTC may be obligated to make; expectations with respect to the commercialization of Evrysdi under PTC's SMA collaboration; expectations with respect to the commercialization of Tegsedi and Waylivra; the timing of and actual expenses incurred in connection with the discontinuation of PTC's preclinical and early research programs in gene therapy and reductions in workforce, which may be in different periods and may be materially higher than estimated; the savings that may result from the discontinuation of PTC's strategic pipeline prioritization and reductions in workforce, which may be materially less than expected; significant 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 PTC's products and product candidates; PTC's scientific approach and general development progress; PTC's ability to satisfy its obligations under the terms of its lease agreements; 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 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, Upstaza, Evrysdi, Tegsedi, Waylivra or sepiapterin.

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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