UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

(Mark One)

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended September 30, 2025

or

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from _____ to _____

Commission File Number: 0-22705

NEUROCRINE BIOSCIENCES, INC.

(Exact name of registrant as specified in its charter)

Delaware
(State or other jurisdiction of
incorporation or organization)

6027 Edgewood Bend Court
San Diego, CA
(Address of principal executive office)

33-0525145
(IRS Employer
Identification No.)

92130
(Zip Code)

(858) 617-7600
(Registrant's telephone number, including area code)

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

Title of each class	Trading Symbol	Name of each exchange on which registered
Common Stock, $0.001 par value	NBIX	Nasdaq Global Select Market

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days: Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act:

Large accelerated filer ☒ Accelerated filer ☐ Non-accelerated filer ☐ Smaller reporting company ☐ 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. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

The number of outstanding shares of the registrant's common stock, par value $0.001 per share, was 99,705,698 as of October 21, 2025.

NEUROCRINE BIOSCIENCES, INC.

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Part I. Financial Information

Item 1. Financial Statements

NEUROCRINE BIOSCIENCES, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS
(unaudited)

(in millions, except per share data)	September 30, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 340.2	$ 233.0
Available-for-sale debt securities	774.6	843.1
Accounts receivable	728.0	479.1
Inventory	69.3	57.4
Prepaid expenses	178.1	48.5
Other current assets	68.2	63.6
Total current assets	2,158.4	1,724.7
Deferred tax assets	368.5	485.7
Available-for-sale debt securities	998.5	739.5
Right-of-use assets	468.9	509.4
Equity investments	118.1	124.8
Property and equipment, net	97.3	82.6
Intangible assets, net	35.7	36.5
Other noncurrent assets	20.3	15.5
Total assets	$ 4,265.7	$ 3,718.7
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and accrued liabilities	$ 584.3	$ 461.6
Other current liabilities	53.7	46.1
Total current liabilities	638.0	507.7
Noncurrent operating lease liabilities	428.4	455.1
Other noncurrent liabilities	195.7	166.2
Total liabilities	1,262.1	1,129.0
Stockholders’ equity:		
Preferred stock, $0.001 par value; 5.0 shares authorized; no shares issued and outstanding	—	—
Common stock, $0.001 par value; 220.0 shares authorized; 99.7 and 99.4 shares issued and outstanding, respectively	0.1	0.1
Additional paid-in capital	2,696.3	2,554.6
Accumulated other comprehensive income	13.2	5.8
Retained earnings	294.0	29.2
Total stockholders’ equity	3,003.6	2,589.7
Total liabilities and stockholders’ equity	$ 4,265.7	$ 3,718.7

See accompanying notes to the condensed consolidated financial statements.

NEUROCRINE BIOSCIENCES, INC.

CONDENSED CONSOLIDATED STATEMENTS OF INCOME AND COMPREHENSIVE INCOME
(unaudited)

(in millions, except per share data)	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	**2024**	**2025**	**2024**
Revenues:				
Net product sales	$ 789.9	$ 616.6	$ 2,035.6	$ 1,709.4
Collaboration revenues	5.0	5.5	19.4	18.2
Total revenues	794.9	622.1	2,055.0	1,727.6
Operating expenses:				
Cost of revenues	14.0	8.0	34.5	24.7
Research and development	250.0	195.0	757.5	545.5
Acquired in-process research and development	0.3	1.0	0.4	9.5
Selling, general, and administrative	291.6	234.3	854.4	719.4
Total operating expenses	555.9	438.3	1,646.8	1,299.1
Operating income	239.0	183.8	408.2	428.5
Other income (expense):				
Unrealized gain (loss) on equity investments	30.6	(16.9)	(6.7)	(35.2)
Charges associated with convertible senior notes	—	—	—	(138.4)
Investment income and other, net	22.2	23.4	64.5	68.5
Total other income (expense), net	52.8	6.5	57.8	(105.1)
Income before provision for income taxes	291.8	190.3	466.0	323.4
Provision for income taxes	82.3	60.5	141.1	85.2
Net income	$ 209.5	$ 129.8	$ 324.9	$ 238.2
Foreign currency translation adjustments, net of tax	1.6	2.9	3.9	2.5
Unrealized (loss) gain on available-for-sale debt securities, net of tax	(0.9)	9.1	3.5	5.0
Comprehensive income	$ 210.2	$ 141.8	$ 332.3	$ 245.7
Earnings per share:				
Basic	$ 2.11	$ 1.28	$ 3.27	$ 2.37
Diluted	$ 2.04	$ 1.24	$ 3.19	$ 2.29
Weighted-average shares outstanding:				
Basic	99.4	101.1	99.3	100.6
Diluted	102.5	104.3	102.0	104.0

See accompanying notes to the condensed consolidated financial statements.

NEUROCRINE BIOSCIENCES, INC.

CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(unaudited)

(in millions)	Common Stock Shares	Common Stock $	Additional Paid-In Capital	Accumulated Other Comprehensive Income	Retained Earnings (Accumulated Deficit)	Total
Balance at June 30, 2025	99.0	$ 0.1	$ 2,597.2	$ 12.5	$ 84.5	$ 2,694.3
Net income	—	—	—	—	209.5	209.5
Other comprehensive income, net of tax	—	—	—	0.7	—	0.7
Stock-based compensation expense	—	—	53.7	—	—	53.7
Issuances of common stock under stock plans	0.7	—	45.4	—	—	45.4
Balance at September 30, 2025	99.7	$ 0.1	$ 2,696.3	$ 13.2	$ 294.0	$ 3,003.6
Balance at June 30, 2024	100.9	$ 0.1	$ 2,555.3	$ 2.5	$ (48.7)	$ 2,509.2
Net income	—	—	—	—	129.8	129.8
Other comprehensive income, net of tax	—	—	—	12.0	—	12.0
Stock-based compensation expense	—	—	41.5	—	—	41.5
Issuances of common stock under stock plans	0.3	—	26.4	—	—	26.4
Balance at September 30, 2024	101.2	$ 0.1	$ 2,623.2	$ 14.5	$ 81.1	$ 2,718.9
Balance at December 31, 2024	99.4	$ 0.1	$ 2,554.6	$ 5.8	$ 29.2	$ 2,589.7
Net income	—	—	—	—	324.9	324.9
Other comprehensive income, net of tax	—	—	—	7.4	—	7.4
Stock-based compensation expense	—	—	159.3	—	—	159.3
Issuances of common stock under stock plans	2.1	—	90.0	—	—	90.0
Repurchases of common stock	(1.8)	—	(107.6)	—	(60.1)	(167.7)
Balance at September 30, 2025	99.7	$ 0.1	$ 2,696.3	$ 13.2	$ 294.0	$ 3,003.6
Balance at December 31, 2023	98.7	$ 0.1	$ 2,382.0	$ 7.0	$ (157.1)	$ 2,232.0
Net income	—	—	—	—	238.2	238.2
Other comprehensive income, net of tax	—	—	—	7.5	—	7.5
Stock-based compensation expense	—	—	129.1	—	—	129.1
Issuances of common stock under stock plans	2.5	—	112.1	—	—	112.1
Balance at September 30, 2024	101.2	$ 0.1	$ 2,623.2	$ 14.5	$ 81.1	$ 2,718.9

See accompanying notes to the condensed consolidated financial statements.

NEUROCRINE BIOSCIENCES, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(unaudited)

(in millions)	Nine Months Ended September 30, 2025	2024
Cash flows from operating activities:		
Net income	$ 324.9	$ 238.2
Adjustments to reconcile net income to net cash from operating activities:		
Stock-based compensation expense	159.3	129.1
Charges associated with convertible senior notes	—	138.4
Impairment charges associated with leased properties	0.7	14.0
Depreciation	19.6	17.3
Accretion of discount on available-for-sale debt securities, net	(10.7)	(20.6)
Amortization of intangible assets	3.0	2.7
Changes in fair values of equity investments	6.7	35.2
Deferred income taxes	117.2	(91.8)
Non-cash lease expense	20.9	3.9
Other	0.7	—
Change in operating assets and liabilities:		
Accounts receivable	(248.9)	(41.8)
Inventory	(11.9)	(7.5)
Accounts payable and accrued liabilities	83.7	(33.9)
Income tax assets and liabilities	(54.3)	(4.8)
Other assets and liabilities, net	(16.6)	(25.5)
Cash flows from operating activities	394.3	352.9
Cash flows from investing activities:		
Purchases of available-for-sale debt securities	(996.8)	(744.3)
Sales and maturities of available-for-sale debt securities	821.7	716.7
Capital expenditures	(36.4)	(30.9)
Cash flows from investing activities	(211.5)	(58.5)
Cash flows from financing activities:		
Issuances of common stock under benefit plans	92.1	112.1
Repurchases of common stock	(167.7)	—
Payments to settle convertible senior notes	—	(308.8)
Cash flows from financing activities	(75.6)	(196.7)
Effect of exchange rate changes on cash and cash equivalents	—	0.3
Change in cash, cash equivalents and restricted cash	107.2	98.0
Cash, cash equivalents and restricted cash at beginning of period	241.0	259.1
Cash, cash equivalents and restricted cash at end of period	$ 348.2	$ 357.1
Supplemental disclosures:		
Accrued capital expenditures	$ 0.6	$ 0.8
Right-of-use assets acquired through operating leases	$ —	$ 9.0
Cash paid for interest	$ —	$ 1.6
Cash paid for income taxes	$ 73.6	$ 144.5

See accompanying notes to the condensed consolidated financial statements.

1. Organization and Significant Accounting Policies

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States (GAAP) for interim financial information and with the instructions of the Securities and Exchange Commission (SEC) on Form 10-Q and Rule 10-01 of Regulation S-X. Accordingly, they do not include all of the information and disclosures required by GAAP for complete financial statements. In the opinion of management, the condensed consolidated financial statements include all adjustments necessary, which are of a normal and recurring nature, for the fair presentation of our financial position and of the results of operations and cash flows for the periods presented. The accompanying unaudited condensed consolidated financial statements include the accounts of Neurocrine Biosciences and our wholly owned subsidiaries. All significant intercompany balances and transactions have been eliminated in consolidation. Certain reclassifications have been made to previously reported amounts to conform to the current period presentation.

These financial statements should be read in conjunction with the audited consolidated financial statements and notes thereto for the year ended December 31, 2024, included in our Annual Report on Form 10-K (the 2024 Form 10-K) filed with the SEC. The results of operations for the interim period shown in this report are not necessarily indicative of the results that may be expected for any other interim period or the full year. The condensed consolidated balance sheet as of December 31, 2024, has been derived from the audited financial statements as of that date, but does not include all of the information and footnotes required by GAAP for complete financial statements.

There were no significant changes to our significant accounting policies as disclosed in the 2024 Form 10-K.

Recently Adopted Accounting Pronouncements

In November 2023, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) 2023-07, Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures, which requires public entities to disclose information about their reportable segments' significant expenses and other segment items on an interim and annual basis. Public entities with a single reportable segment are required to apply the disclosure requirements in ASU 2023-07, as well as all existing segment disclosures and reconciliation requirements in ASC 280 on an interim and annual basis. We adopted ASU 2023-07 for annual reporting periods beginning January 1, 2024 and interim reporting periods beginning January 1, 2025. The adoption of ASU 2023-07 had no significant impact on our financial statement disclosures.

Recently Issued Accounting Pronouncements Not Yet Adopted

In December 2023, the FASB issued ASU 2023-09, Income Taxes (Topic 740): Improvements to Income Tax Disclosures, which requires public entities, on an annual basis, to provide disclosure of specific categories in the rate reconciliation, as well as disclosure of income taxes paid disaggregated by jurisdiction. ASU 2023-09 is effective for annual reporting periods beginning after December 15, 2024, with early adoption permitted. We are currently evaluating the impact that adoption of ASU 2023-09 will have on our financial statement disclosures.

In November 2024, the FASB issued ASU 2024-03, Income Statement–Reporting Comprehensive Income–Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses, which requires public entities to disclose specified information about certain costs and expenses on an interim and annual basis. ASU 2024-03 is effective for annual reporting periods beginning after December 15, 2026, and interim reporting periods beginning after December 15, 2027, with early adoption permitted. We are currently evaluating the impact that adoption of ASU 2024-03 will have on our financial statement disclosures.

2. Collaboration and License Agreements

Nxera Pharma UK Limited (Nxera)

In 2021, we entered into a collaboration and license agreement with Nxera (formerly Sosei Heptares) to develop and commercialize certain compounds containing sub-type selective muscarinic M1, M4, or dual M1/M4 receptor agonists, which we have the exclusive rights to develop, manufacture and commercialize worldwide, excluding in Japan, where Nxera retains the rights to develop, manufacture, and commercialize all compounds comprised of M1 receptor agonists, subject to certain exceptions. With respect to such rights retained by Nxera, we retain the rights to opt in to profit sharing arrangements, pursuant to which we and Nxera will equally share in the operating profits and losses for such compounds in Japan. Subject to specified conditions, we may elect to exercise such opt-in rights with respect to each such compound either before initiation of the first proof of concept Phase 2 clinical trial for such compound or following our receipt from Nxera of the top-line data from such clinical trial for such compound. We are responsible for all development, manufacturing, and commercialization costs of any collaboration product.

Direclidine (NBI-1117568) is a first-in-class, orally active, highly selective investigational M4 agonist in development as a potential treatment for schizophrenia. In connection with the initiation of a Phase 3 clinical study for direclidine in schizophrenia in May 2025, we paid Nxera a milestone of $15.0 million, which was expensed as research and development (R&D) in the second quarter of 2025. In connection with the successful completion of a long-term toxicity program for direclidine in April 2024, we paid Nxera a milestone of $15.0 million, which was expensed as R&D in the second quarter of 2024. In connection with the successful completion of the Phase 2 clinical study for direclidine in August 2024, we paid Nxera a milestone of $35.0 million, which was expensed as R&D in the third quarter of 2024.

Under the terms of the agreement, Nxera may be entitled to receive potential future payments of up to $2.5 billion upon the achievement of certain event-based milestones and is entitled to receive royalties on the future net sales of any collaboration product.

Unless earlier terminated, the agreement will continue on a licensed product-by-licensed product and country-by-country basis until the date on which the royalty term for such licensed product has expired in such country. On a licensed product-by-licensed product and country-by-country basis, royalty payments would commence on the first commercial sale of a licensed product and terminate on the later of (i) the expiration of the last patent covering such licensed product in such country, (ii) a number of years from the first commercial sale of such licensed product in such country and (iii) the expiration of regulatory exclusivity for such licensed product in such country.

We may terminate the agreement in its entirety or with respect to one or more targets upon 180 days' written notice to Nxera during the research collaboration term and upon 90 days' written notice to Nxera following the expiration of the research collaboration term. Following the expiration of the research collaboration term, Nxera may terminate the agreement on a target-by-target basis in the event that we do not conduct any material development activities outside of Japan with respect to a certain compound or licensed product within the applicable target class for a continuous period of not less than 365 days and do not commence any such activities within 120 days of receiving written notice. Either party may terminate the agreement, subject to specified conditions, (i) in the event of material breach by the other party, subject to a cure period, (ii) if the other party challenges the validity or enforceability of certain intellectual property rights, subject to a cure period, or (iii) if the other party becomes insolvent or takes certain actions related to insolvency.

Takeda Pharmaceutical Company Limited (Takeda)

In 2020, we entered into an exclusive license agreement with Takeda (the 2020 Takeda Agreement), pursuant to which we acquired the exclusive rights to develop and commercialize certain early to mid-stage psychiatry compounds, including luvadaxistat, NBI-1070770, osavampator (NBI-1065845), NBI-1065846, and three non-clinical stage compounds. Pursuant to the 2020 Takeda Agreement, osavampator was designated as a profit-share product, meaning we and Takeda would equally share in the operating profits and losses. Takeda also retained the right to opt-out of the profit-sharing arrangement, pursuant to which Takeda would be entitled to receive potential future payments upon the achievement of certain event-based milestones with respect to osavampator and receive royalties on the future net sales of osavampator (in lieu of equally sharing in the operating profits and losses).

In October 2024, we provided Takeda with written notice of termination of the license under the 2020 Takeda Agreement to develop and commercialize luvadaxistat and NBI-1065846, which became effective in April 2025. In January 2025, we and Takeda amended and restated the exclusive license agreement (the Restated Takeda Agreement) to, among other things, reflect the conversion from sharing operating profits and losses with respect to the development and commercialization of osavampator to a royalty-bearing license, the return of rights to osavampator in Japan to Takeda, and our previous termination of DAAO inhibitors under the 2020 Takeda Agreement, including luvadaxistat, and GPR139 agonists, including NBI-1065846.

Under the Restated Takeda Agreement, we will retain exclusive rights to develop and commercialize osavampator for all indications in all territories worldwide except Japan, where Takeda will reacquire exclusive development and commercialization rights. In addition, each party is responsible for development costs for osavampator in its respective territory, and each party is eligible to receive royalty payments based on the other party's net sales of osavampator in the other party's territory. Pursuant to the Restated Takeda Agreement and upon the successful development and commercialization of osavampator, we will incur tiered based royalties payable to Takeda in the mid-to-upper teens in the U.S. and low double-digits outside of the U.S. on a blended basis as a percentage of net sales. Additionally, we are entitled to receive royalties from Takeda on the future net sales of osavampator in Japan.

Osavampator is a potential first-in-class alpha-amino-3-hydroxy-5-methyl-4-isoxazole propionic acid (AMPA) positive allosteric modulator (PAM) in development for patients with inadequate response to treatment of major depressive disorder (MDD). In connection with the initiation of a Phase 3 clinical study for osavampator in MDD in January 2025, we paid Takeda a milestone of $37.5 million, which was expensed as R&D in the first quarter of 2025.

NBI-1070770 is a novel, selective, and orally active, negative allosteric modulator (NAM) of the NR2B subunit-containing N-methyl-D-aspartate (NMDA NR2B) receptor in development as a potential treatment for MDD. In connection with the initiation of a Phase 2 clinical study for NBI-1070770 in MDD in April 2024, we paid Takeda a milestone of $7.5 million, which was expensed as R&D in the second quarter of 2024.

Takeda may be entitled to receive potential future payments of up to $0.7 billion upon the achievement of certain event-based milestones and is entitled to receive royalties on the future net sales of any royalty-bearing product.

Unless earlier terminated, the Restated Takeda Agreement will continue on a licensed product-by-licensed product and country-by-country basis until the date on which, (i) for any royalty-bearing product, the royalty term has expired in such country; and (ii) for any profit-share product, for so long as we continue to develop, manufacture, or commercialize such licensed product. On a licensed product-by-licensed product and country-by-country basis, royalty payments would commence on the first commercial sale of a royalty-bearing product and terminate on the later of (i) the expiration of the last patent covering such royalty-bearing product in such country, (ii) a number of years from the first commercial sale of such royalty-bearing product in such country and (iii) the expiration of regulatory exclusivity for such royalty-bearing product in such country.

We may terminate the Restated Takeda Agreement in its entirety or in one or more (but not all) of the U.S., Japan, the European Union and the United Kingdom, or, collectively, the major markets, upon six months' written notice to Takeda (i) with respect to all licensed products prior to the first commercial sale of the first licensed product for which first commercial sale occurs, or (ii) with respect to all licensed products in one or more given target classes, as defined in the Restated Takeda Agreement, prior to the first commercial sale of the first licensed product in such target class for which first commercial sale occurs. We may terminate the Restated Takeda Agreement in its entirety or in one or more (but not all) of the major markets upon 12 months' written notice to Takeda (i) with respect to all licensed products following the first commercial sale of the first licensed product for which first commercial sale occurs, or (ii) with respect to all licensed products in one or more given target classes following the first commercial sale of the first licensed product in such target class for which first commercial sale occurs. Takeda may terminate the Restated Takeda Agreement, subject to specified conditions, (i) if we challenge the validity or enforceability of certain Takeda intellectual property rights or (ii) on a target class-by-target class basis, in the event that we do not conduct any material development or commercialization activities with respect to any licensed product within such target class for a specified continuous period. Subject to a cure period, either party may terminate the Restated Takeda Agreement in the event of any material breach, solely with respect to the target class of a licensed product to which such material breach relates, or in its entirety in the event of any material breach that relates to all licensed products, or if either party challenges the validity or enforceability of certain intellectual property rights.

Xenon Pharmaceuticals Inc. (Xenon)

In 2019, we entered into a collaboration and license agreement with Xenon to identify, research and develop sodium channel inhibitors, including NBI-921352 and three preclinical candidates, which compounds we have the exclusive rights to develop and commercialize. In connection with the agreement, we purchased 1.4 million shares (at $14.196 per share) of Xenon common stock in 2019, 0.3 million shares (at $19.9755 per share) of Xenon common stock in 2021, and 0.3 million shares (at $31.855 per share) of Xenon common stock in 2022. We are responsible for all development and manufacturing costs of any collaboration product, subject to certain exceptions.

NBI-921355 is an investigational, selective inhibitor of voltage-gated sodium channels $Na_v1.2$ and $Na_v1.6$ in development as a potential treatment of certain types of epilepsy. In connection with the initiation of a Phase 1 clinical study to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of NBI-921355 in healthy adult participants in February 2025, we paid Xenon a milestone of $7.5 million, which was expensed as R&D in the first quarter of 2025.

Under the terms of the agreement, Xenon may be entitled to receive potential future payments of up to $1.7 billion upon the achievement of certain event-based milestones and is entitled to receive royalties on the future net sales of any collaboration product. Xenon retains the right to elect to co-develop one product in a major indication, pursuant to which Xenon would receive a mid-single digit percentage increase in royalties earned on the future net sales of such product in the United States and we and Xenon would equally share in the development costs of such product in the applicable indication, except where such development costs relate solely to the regulatory approval of such product outside the United States.

Unless earlier terminated, the agreement will continue on a licensed product-by-licensed product and country-by-country basis until the expiration of the royalty term for such product in such country. Upon the expiration of the royalty term for a particular licensed product and country, the license obtained by us with respect to such product and country will become fully paid, royalty free, perpetual and irrevocable. We may terminate the agreement upon 90 days' written notice to Xenon, provided that such unilateral termination will not be effective for certain products until we have used commercially reasonable efforts to complete certain specified clinical studies. Either party may terminate the agreement in the event of a material breach in whole or in part, subject to specified conditions.

Voyager Therapeutics, Inc. (Voyager)

2019 Voyager Agreement

In 2019, we entered into a collaboration and license agreement with Voyager (the 2019 Voyager Agreement), pursuant to which we obtained certain rights to develop and commercialize product candidates, including the rights to gene therapy product candidates for the treatment of Friedreich's ataxia (FA) and two undisclosed programs. In April 2025, we mutually agreed with Voyager to discontinue the two undisclosed programs and the rights to the targets selected under these programs returned to Voyager. We are responsible for all development and commercialization costs of any collaboration product under the 2019 Voyager Agreement, subject to certain co-development and co-commercialization rights retained by Voyager.

In connection with the 2019 Voyager Agreement, we purchased 4.2 million shares (at $11.9625 per share) of Voyager common stock (the 2019 Voyager Shares), which are subject to certain transfer, beneficial ownership, and voting restrictions for a period of up to three years from the effective date of the 2023 Voyager Agreement (defined below).

In connection with the selection of a development candidate under the FA program pursuant to our collaboration with Voyager, we paid Voyager a milestone of $5.0 million, which was expensed as R&D in the first quarter of 2024.

Under the terms of the 2019 Voyager Agreement, Voyager may be entitled to receive potential future payments of up to $0.5 billion upon the achievement of certain event-based milestones and is entitled to receive royalties on the future net sales of any collaboration product, subject to certain co-development and co-commercialization rights retained by Voyager.

Unless terminated earlier, the 2019 Voyager Agreement will continue in effect until the expiration of the last to expire royalty term with respect to any collaboration product under the agreement or the last expiration or termination of any exercised co-development and co-commercialization rights by Voyager as provided for in the 2019 Voyager Agreement. We may terminate the 2019 Voyager Agreement upon 180 days' written notice to Voyager prior to the first commercial sale of any collaboration product under the 2019 Voyager Agreement or upon one year after the date of notice if such notice is provided after the first commercial sale of any collaboration product under the 2019 Voyager Agreement.

2023 Voyager Agreement

In 2023, we entered into a collaboration and license agreement with Voyager, which we amended in April 2024 (as amended, the 2023 Voyager Agreement), pursuant to which we acquired the global rights to the gene therapy products directed to the gene that encodes glucosylceramidase beta 1 (GBA1) for the treatment of Parkinson's disease and other diseases associated with GBA1 (the GBA1 Program), and three gene therapy programs directed to rare central nervous system (CNS) targets, each enabled by Voyager's next-generation TRACER™ capsids.

With respect to collaboration products subject to the GBA1 Program, we are responsible for all development and commercialization costs of any such products, including in the U.S., where Voyager retains certain co-development and co-commercialization rights. Voyager may elect to exercise such rights, pursuant to which we and Voyager would equally share in the operating profits and losses of such products in the U.S. (in lieu of Voyager being entitled to receive potential future payments of certain event-based milestones upon their achievement in the U.S. and receive royalties on the future net sales of such products in the U.S.), following Voyager's receipt of the top-line data from a first clinical trial Parkinson's disease. However, if we and Voyager elect to focus on an indication other than Parkinson's disease prior to Voyager's receipt of top-line data from a first clinical trial for Parkinson's disease, then Voyager may elect to exercise such co-development and co-commercialization rights after the later of: (i) Voyager's receipt of top-line data from the first clinical trial of a product that is the subject of the GBA1 Program or (ii) the date we and Voyager decide not to pursue Parkinson's disease as an indication for development under the GBA1 Program. Irrespective of Voyager's election to exercise such rights, Voyager may be entitled to receive potential future payments upon the achievement of certain event-based milestones outside the U.S. and would be entitled to receive royalties on the future net sales of any such product outside the U.S.

With respect to collaboration products subject to the three gene therapy programs directed to rare CNS targets, we are responsible for all development and commercialization costs for any such products.

In connection with the 2023 Voyager Agreement, we purchased 4.4 million shares (at $8.88 per share) of Voyager common stock (the 2023 Voyager Shares), which are subject to certain transfer, beneficial ownership, and voting restrictions for a period of up to three years from the effective date of the 2023 Voyager Agreement. In addition, as part of the collaboration, Jude Onyia, Ph.D., Chief Scientific Officer of Neurocrine Biosciences, was appointed to Voyager's board of directors. Dr. Onyia (or another individual designated by us) will be nominated for election to Voyager's board of directors annually for a maximum duration of 10 years from the effective date of the 2023 Voyager Agreement. As a result, our equity investment in Voyager became subject to the equity method of accounting, and Voyager became a related party, following our purchase of the 2023 Voyager Shares, after which, together with the 2019 Voyager Shares, we owned approximately 19.9% of the voting stock of Voyager. We elected the fair value option to account for our equity investment in Voyager as we believe it creates greater transparency regarding the investment's fair value at future reporting dates.

In connection with the selection of development candidates under the GBA1 program pursuant to our collaboration with Voyager, we expensed milestones of $3.0 million as R&D in both the second and third quarters of 2024.

Under the terms of the 2023 Voyager Agreement, Voyager may be entitled to receive potential future payments of up to $6.1 billion upon the achievement of certain event-based milestones and is entitled to receive royalties on the future net sales of any collaboration product, subject to certain co-development and co-commercialization rights retained by Voyager.

Unless terminated earlier, the 2023 Voyager Agreement will continue in effect until the expiration of the last to expire royalty term with respect to any collaboration product under the 2023 Voyager Agreement or the last expiration or termination of any exercised co-development and co-commercialization rights by Voyager as provided for in the 2023 Voyager Agreement. We may terminate the 2023 Voyager Agreement upon 180 days' written notice to Voyager prior to the first commercial sale of any collaboration product under the 2023 Voyager Agreement or upon one year after the date of notice if such notice is provided after the first commercial sale of any collaboration product under the 2023 Voyager Agreement.

Sanofi S.A. (Sanofi)

In 2014, we entered into a license agreement with Sanofi, pursuant to which we acquired the global rights to develop and commercialize certain corticotropin-releasing factor type 1 (CRF-1) receptor antagonists, including crinecerfont. We launched CRENESSITY® (crinecerfont) in the U.S. as a first-in-class U.S. Food and Drug Administration (FDA)-approved treatment of classic congenital adrenal hyperplasia (CAH) in December 2024. We are responsible for all manufacturing, development, and commercialization costs of any licensed product.

Under the terms of our license agreement with Sanofi, Sanofi may be entitled to receive potential future payments of up to $10.0 million upon the achievement of certain event-based milestones and is entitled to receive royalties at tiered percentage rates ranging from 3.0% to 5.0% on our future net sales of CRENESSITY in the U.S. for the longer of 16 years or the life of the related patent rights.

Mitsubishi Tanabe Pharma Corporation (MTPC)

In 2015, we out-licensed the rights to valbenazine in Japan and other select Asian markets to MTPC. In 2020, we entered into a commercial supply agreement with MTPC, pursuant to which we supply MTPC with valbenazine drug product for commercial use in such markets. MTPC launched DYSVAL® (valbenazine) in Japan for the treatment of tardive dyskinesia in June 2022 and subsequently in other select Asian markets, where it is marketed as REMLEAS® (valbenazine). MTPC is responsible for all development, manufacturing, and commercialization costs of valbenazine in such markets.

Under the terms of our license agreement with MTPC, we may be entitled to receive potential future payments of up to $30.0 million upon the achievement of certain sales-based milestones and are entitled to receive royalties at tiered percentage rates on future MTPC net sales of valbenazine for the longer of 10 years or the life of the related patent rights. MTPC may terminate the agreement upon 180 days' written notice to us. In such event, all out-licensed product rights would revert to us.

AbbVie Inc. (AbbVie)

In 2010, we out-licensed the global rights to elagolix to AbbVie. AbbVie launched ORILISSA® (elagolix tablets) in the U.S. for the treatment of moderate to severe pain associated with endometriosis in August 2018 and ORIAHNN® (elagolix, estradiol and norethindrone acetate capsules and elagolix capsules) in the U.S. for the treatment of heavy menstrual bleeding due to uterine fibroids in June 2020. AbbVie is responsible for all development and commercialization costs of elagolix.

Under the terms of our license agreement with AbbVie, we may be entitled to receive potential future payments of up to $366.0 million upon the achievement of certain event-based milestones and are entitled to receive royalties at tiered percentage rates on future AbbVie net sales of elagolix for the longer of 10 years or the life of the related patent rights. AbbVie may terminate the agreement upon 180 days' written notice to us. In such event, all out-licensed product rights would revert to us.

3. Fair Value Measurements

The fair value hierarchy consists of the following three levels:

Level 1 – Quoted prices (unadjusted) in active markets for identical assets or liabilities.

Level 2 – Quoted prices for similar assets or liabilities in active markets, quoted prices for identical or similar assets or liabilities in markets that are not active or inputs that are observable, either directly or indirectly, for substantially the full term of the asset or liability.

Level 3 – Unobservable inputs that reflect our own assumptions about the assumptions that market participants would use in pricing the asset or liability when there is little, if any, market activity for the asset or liability at the measurement date.

The following table presents a summary of certain financial assets, which were measured at fair value on a recurring basis.

	September 30, 2025			December 31, 2024		
	Fair	Leveling		Fair	Leveling	
(in millions)	Value	Level 1	Level 2	Value	Level 1	Level 2
Cash and cash equivalents	$ 340.2	$ 340.2	$ —	$ 233.0	$ 233.0	$ —
Available-for-sale debt securities	1,773.1	—	1,773.1	1,582.6	—	1,582.6
Equity investments	118.1	118.1	—	124.8	124.8	—
	$ 2,231.4	$ 458.3	$ 1,773.1	$ 1,940.4	$ 357.8	$ 1,582.6

Concentration of Credit Risk

Financial instruments that potentially subject us to concentrations of credit risk include cash and cash equivalents, investments in available-for-sale debt securities, and accounts receivable.

To minimize the risks related to cash and cash equivalents and investments in available-for-sale debt securities, we have established guidelines related to credit ratings and maturities intended to safeguard principal balances and maintain liquidity. Our investment portfolio is maintained in accordance with our investment policy, which defines allowable investments, specifies credit quality standards, and limits the credit exposure of any single issuer.

As of September 30, 2025 and December 31, 2024, we held available-for-sale debt securities with a total fair value of $349.8 million and $458.7 million, respectively, that were in unrealized loss positions totaling $0.5 million and $1.7 million, respectively. Available-for-sale debt securities that had been in unrealized loss positions for longer than twelve months were not significant as of September 30, 2025 or December 31, 2024. Unrealized losses on available-for-sale debt securities are primarily caused by changes in interest rates. Our investments in available-for-sale debt securities are of high credit quality, and we do not intend to sell these investments and it is not more likely than not that we will be required to sell these investments before their maturity. Accrued interest receivables on available-for-sale debt securities totaled $18.4 million and $14.4 million, respectively, as of September 30, 2025 and December 31, 2024 and are included in other current assets on the condensed consolidated balance sheets. We do not measure an allowance for credit losses for accrued interest receivables. For the purposes of identifying and measuring an impairment, accrued interest is excluded from both the fair value and amortized cost basis of the debt security. Uncollectible accrued interest receivables associated with an impaired debt security are reversed against interest income upon identification of the impairment.

To minimize the risks related to accounts receivable, which are typically unsecured, we monitor the financial performance and creditworthiness of our customers so that we can properly assess and respond to changes in their credit profiles.

The following table presents the percent of total gross product sales for each of our customers who individually accounted for 10% or more of total gross product sales.

	Nine Months Ended September 30,	
	2025	2024
Customer A	41 %	44 %
Customer B	28 %	28 %
Customer C	17 %	13 %

The following table presents the percent of total accounts receivable for each of our customers who individually accounted for 10% or more of total accounts receivable.

	September 30, 2025	December 31, 2024
Customer A	45 %	41 %
Customer B	29 %	37 %
Customer C	15 %	< 10 %
Customer D	< 10 %	11 %

4. Earnings per Share

Earnings per share were calculated as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in millions, except per share data)	2025	2024	2025	2024
Net income - basic and diluted	$ 209.5	$ 129.8	$ 324.9	$ 238.2
Weighted-average common shares outstanding:				
Basic	99.4	101.1	99.3	100.6
Effect of dilutive securities	3.1	3.2	2.7	3.4
Diluted	102.5	104.3	102.0	104.0
Earnings per share:				
Basic	$ 2.11	$ 1.28	$ 3.27	$ 2.37
Diluted	$ 2.04	$ 1.24	$ 3.19	$ 2.29
Shares excluded from diluted per share amounts because their effect would have been anti-dilutive	3.3	1.7	4.3	1.9

5. Leases

Our operating leases that have commenced have terms that expire beginning 2026 through 2036 and consist of office space and research and development laboratories, including our corporate headquarters. Certain of these lease agreements contain clauses for renewal at our option. As we were not reasonably certain to exercise any of these renewal options at commencement of the associated leases, no such options were recognized as part of our right-of-use (ROU) assets or operating lease liabilities.

The following table presents supplemental operating lease information for operating leases that have commenced.

	Nine Months Ended September 30,	
(in millions, except weighted average data)	**2025**	**2024**
Operating lease cost	$ 49.0	$ 27.9
Sublease income	(2.6)	(1.3)
Net operating lease cost	$ 46.4	$ 26.6
Cash paid for amounts included in the measurement of operating lease liabilities	$ 28.2	$ 24.0

	September 30,	
	2025	**2024**
Weighted average remaining lease term	10.1 years	10.3 years
Weighted average discount rate	4.9 %	5.0 %
Restricted cash related to letters of credit issued in lieu of cash security deposits	$ 7.8	$ 7.8

The following table presents approximate future non-cancelable minimum lease payments under operating leases and sublease income as of September 30, 2025.

(in millions)	**Operating Leases**	**Sublease Income**
2025 (3 months remaining)	$ 9.7	$ (0.9)
2026	58.0	(3.5)
2027	59.6	(3.5)
2028	60.2	(3.5)
2029	60.4	(3.5)
Thereafter	371.6	(5.3)
Total operating lease payments (sublease income)	619.5	$ (20.2)
Less imputed interest	139.8	
Total operating lease liabilities	479.7	
Less current operating lease liabilities included in other current liabilities	51.3	
Noncurrent operating lease liabilities	$ 428.4	

New Campus Facility

On February 8, 2022, we entered into a lease agreement for a four-building campus facility to be constructed in San Diego, California, including a six-year option for the construction of a fifth building. This campus facility, comprised of office space and research and development laboratories, now serves as our new corporate headquarters.

The construction of the new campus facility was phased. In connection with the completion of the first phase of construction relating to office space, we recognized ROU assets of $199.0 million and operating lease liabilities of $189.8 million in December 2023. In connection with the completion of the second phase of construction relating to laboratory space, we recognized ROU assets of $258.9 million and operating lease liabilities of $211.7 million in October 2024.

Impairment of ROU Assets

From time to time, we reassess the asset groupings for corporate ROU assets that are actively being marketed for sublease in connection with leased office space that has been vacated as we continued to occupy our new campus facility. For asset groups where impairment is triggered, we use discounted cash flow models (an income approach) with Level 3 inputs to estimate the fair values of the asset groups. The significant assumptions used in the discounted cash flows models include projected sublease income over the remaining lease term, expected downtime prior to the commencement of executed or future subleases, and discount rates that reflect a market participant's assumptions in valuing the asset groups.

We recognized impairment charges totaling $14.0 million in the second quarter of 2024, of which $11.3 million and $2.7 million, respectively, was related to the ROU assets and tenant improvements associated with the underlying leased properties. Impairment charges were not significant for the first nine months of 2025.

6. Stockholders' Equity

Share Repurchases

In February 2025, our Board of Directors authorized a new share repurchase program (the 2025 Repurchase Program) under which we may repurchase up to $500.0 million of our common stock, subject to market conditions. Under the 2025 Repurchase Program, we repurchased 1.5 million shares on the open market for a cost of $167.7 million during the first nine months of 2025. As of September 30, 2025, we had $332.3 million remaining under the 2025 Repurchase Program.

Under the 2025 Repurchase Program, share repurchases may be made from time to time at management's discretion through a variety of methods, such as open-market transactions including pre-set trading plans, privately negotiated transactions, accelerated share repurchases, and other transactions in accordance with applicable securities laws. Shares repurchased under the 2025 Repurchase Program are retired immediately, resulting in an immediate reduction of the outstanding shares used to calculate the weighted-average common shares for both basic and diluted earnings per share, and included in the category of authorized but unissued shares. The excess of the purchase price over the par value of the common shares was recorded as reductions to retained earnings and additional paid-in capital.

In November 2024, we entered into an accelerated share repurchase transaction (the 2024 Repurchase Program) with a third-party financial institution to repurchase an aggregate of $300.0 million of our common stock. Shares repurchased under the 2024 Repurchase Program were retired immediately upon receipt, resulting in an immediate reduction of the outstanding shares used to calculate the weighted-average common shares for both basic and diluted earnings per share in the period received, and included in the category of authorized but unissued shares. At inception, we paid the financial institution $300.0 million using cash on hand and took initial delivery of 2.0 million shares in November 2024. The fair market value of the 2.0 million initial shares received was $240.5 million, with the excess of the fair market value over the par value of the initial shares received recorded as reductions to retained earnings and additional paid-in capital. The remaining $59.5 million of the repurchase price was recorded as a reduction to additional paid-in capital. The 2024 Repurchase Program was completed in February 2025, at which time we received an additional 0.3 million shares upon settlement, with the excess of the fair market value over the par value of the settlement shares received recorded as an increase to additional paid-in capital and a reduction to retained earnings. In total, we repurchased 2.3 million shares under the 2024 Repurchase Program at an average price of $131.83 per share, which represents the daily volume-weighted average price of our common stock over the term of the transaction, less a negotiated discount.

The following table presents information relating to purchases of our common stock.

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in millions, except per share data)	2025	2024	2025	2024
Total number of shares repurchased	—	—	1.8	—
Amount repurchased	$ —	$ —	$ 227.2	$ —
Average price per share	$ —	$ —	$ 127.31	$ —

7. Intangible Assets

The following table presents information relating to our recognized intangible assets.

(in millions)	September 30, 2025			December 31, 2024		
	Gross Carrying Amount	Accumulated Amortization	Net Carrying Amount	Gross Carrying Amount	Accumulated Amortization	Net Carrying Amount
Developed product rights [1]	$ 43.2	$ 11.3	$ 31.9	$ 40.5	$ 7.7	$ 32.8
Acquired IPR&D [2]	$ 3.8	$ —	3.8	$ 3.7	$ —	3.7
Total intangible assets, net			$ 35.7			$ 36.5

(1) Developed product rights have a useful life of 10 to 16 years.

(2) Acquired IPR&D is considered indefinite lived until the completion or abandonment of the associated research and development efforts.

The following table presents approximate future annual amortization expense for our finite-lived intangible assets as of September 30, 2025.

(in millions)	Amount
Year ending December 31, 2025 (3 months remaining)	$ 1.0
Year ending December 31, 2026	$ 4.1
Year ending December 31, 2027	$ 4.1
Year ending December 31, 2028	$ 4.1
Year ending December 31, 2029	$ 4.1
Thereafter	$ 14.5

8. Convertible Senior Notes

On May 2, 2017, we completed a private placement of $517.5 million in aggregate principal amount of 2.25% fixed-rate convertible senior notes due May 15, 2024 (the 2024 Notes) and entered into the 2017 Indenture with respect to the 2024 Notes. Interest on the 2024 Notes was due semi-annually on May 15 and November 15 of each year.

In 2020, we repurchased $136.2 million in aggregate principal amount of the 2024 Notes for an aggregate repurchase price of $186.9 million in cash. In 2022, we repurchased $210.8 million in aggregate principal amount of the 2024 Notes for an aggregate repurchase price of $279.0 million in cash.

In January 2024, we provided notice to the holders of the 2024 Notes electing to settle all conversions of the 2024 Notes which occur on or after January 15, 2024, in cash. Consequently, the embedded conversion option of the 2024 Notes (the conversion feature) required bifurcation and separate accounting from the 2024 Notes as it no longer qualified for the equity scope exception under ASC 815, Derivatives and Hedging. Upon bifurcation of the conversion feature in the first quarter of 2024, we recorded a derivative liability at a fair value of $126.6 million (Level 3) and a corresponding debt discount that was accreted over the remaining term of the 2024 Notes using the straight-line method. Subsequent changes in the fair value of the derivative liability and accretion of the associated debt discount were recorded in other expense, net on the condensed consolidated statements of income and comprehensive income.

During the second quarter of 2024, holders of the 2024 Notes converted $169.8 million in aggregate principal amount of the 2024 Notes for $308.2 million in cash, reflecting a conversion premium of $138.4 million calculated based on the per share volume-weighted average price (VWAP) for each of the 30 consecutive trading days during the observation period (as more fully described in the 2017 Indenture). The 2024 Notes were settled in full upon maturity on May 15, 2024.

The following table presents a summary of charges recognized in connection with the bifurcation of the conversion feature of the 2024 Notes and conversions of the 2024 Notes by holders during the first nine months of 2024.

(in millions)	Amount
Accretion of debt discount associated with derivative liability	$ 126.6
Change in fair value of derivative liability	9.6
Loss on extinguishment of convertible senior notes	2.2
Charges associated with convertible senior notes	$ 138.4

9. Supplemental Financial Information

Inventory consisted of the following:

(in millions)	September 30, 2025	December 31, 2024
Raw materials	$ 40.1	$ 33.7
Work in process	13.2	10.9
Finished goods	16.2	12.8
	69.5	57.4
Less inventory reserves	(0.2)	—
Total inventory	$ 69.3	$ 57.4

Prior to FDA approval of CRENESSITY in December 2024, all costs related to its manufacturing were expensed as R&D in the period incurred. As a result, our physical inventories as of September 30, 2025 and December 31, 2024 included active pharmaceutical product with no cost basis. Costs related to the manufacturing of bulk drug product, finished bottling, and other labeling activities that occurred post-FDA approval are included in the inventory values as of September 30, 2025 and December 31, 2024.

Accounts payable and accrued liabilities consisted of the following:

(in millions)	September 30, 2025	December 31, 2024
Sales rebates and reserves	$ 237.9	$ 144.2
Accrued employee related costs	105.1	107.5
Accrued development costs	82.4	50.8
Current branded prescription drug fee	39.0	49.2
Accounts payable and other accrued liabilities	119.9	109.9
Total accounts payable and accrued liabilities	$ 584.3	$ 461.6

Other noncurrent liabilities consisted of the following:

(in millions)	September 30, 2025	December 31, 2024
Noncurrent income taxes payable	$ 195.5	$ 160.7
Other noncurrent liabilities	0.2	5.5
Total other noncurrent liabilities	$ 195.7	$ 166.2

The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the condensed consolidated balance sheets that sum to the total of the same such amounts shown in the condensed consolidated statements of cash flows.

(in millions)	September 30, 2025	September 30, 2024
Cash and cash equivalents	$ 340.2	$ 349.1
Restricted cash included in other noncurrent assets	8.0	8.0
Total cash, cash equivalents, and restricted cash	$ 348.2	$ 357.1

10. Segment Reporting and Disaggregation of Relevant Expense Captions

Neurocrine Biosciences operates as a single global business segment dedicated to the research and development, commercialization, and sale of pharmaceuticals primarily in the U.S. for the treatment of neurological, neuroendocrine, and neuropsychiatric disorders. There were no changes to the accounting policies of the segment as disclosed in the 2024 Form 10-K.

The determination of a single business segment is consistent with the consolidated financial information regularly reviewed by the Chief Executive Officer as chief operating decision maker (CODM) in assessing segment performance and deciding how to allocate resources on a consolidated basis.

The CODM assesses performance for the segment and decides how to allocate resources based on net income that also is reported on the consolidated statements of income and comprehensive income as consolidated net income. The CODM uses net income to monitor budget and forecast versus actual results in assessing segment performance and to evaluate income generated from segment assets in deciding how to allocate resources. The measure of segment assets is reported on the consolidated balance sheets as total consolidated assets.

The following table presents information about reported segment revenues, segment profit, and significant segment expenses.

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in millions)	**2025**	**2024**	**2025**	**2024**
Revenues:				
INGREZZA net product sales	$ 686.6	$ 612.9	$ 1,856.2	$ 1,698.4
CRENESSITY net product sales	98.1	—	165.8	—
Other revenues [1]	10.2	9.2	33.0	29.2
Total revenues	794.9	622.1	2,055.0	1,727.6
Less:				
Cost of revenues	14.0	8.0	34.5	24.7
Research and development:				
External research and development	141.8	82.9	370.3	250.4
Payroll and benefits	74.6	55.4	221.8	167.9
Milestones	1.0	38.8	61.5	71.4
Other research and development [2]	32.6	17.9	103.9	55.8
Total research and development	250.0	195.0	757.5	545.5
Acquired in-process research and development	0.3	1.0	0.4	9.5
Selling, general, and administrative	291.6	234.3	854.4	719.4
Unrealized (gain) loss on equity investments	(30.6)	16.9	6.7	35.2
Charges associated with convertible senior notes	—	—	—	138.4
Interest income and other, net	(22.2)	(23.4)	(64.5)	(68.5)
Provision for income taxes	82.3	60.5	141.1	85.2
Net income	$ 209.5	$ 129.8	$ 324.9	$ 238.2

(1) Other revenues primarily consist of net product sales for ALKINDI and EFMODY and royalties earned on AbbVie net sales of elagolix and MTPC net sales of valbenazine.

(2) Other research and development consists of indirect costs incurred for the benefit of multiple research and development programs, including facility-based expenses (such as rent expense) and other overhead allocations.

11. Legal Proceedings

In March 2025, we received a notice from Zydus Lifesciences Global FZE (Zydus FZE) that it had filed an abbreviated new drug application, or ANDA, with the FDA seeking approval of a generic version of INGREZZA SPRINKLE (valbenazine). The ANDA contained a Paragraph IV Patent Certification alleging that certain of our patents covering INGREZZA SPRINKLE are invalid and/or will not be infringed by Zydus FZE's importation, manufacture, use or sale of the medicine for which the ANDA was submitted. We filed suit in the U.S. District Court for the District of Delaware in April 2025 against Zydus Pharmaceuticals (USA) Inc. and its affiliates Zydus FZE, Zydus Worldwide DMCC (entity subsequently dismissed), Zydus Lifesciences Limited, and Zydus Healthcare (USA) LLC (entity subsequently dismissed) (collectively, Zydus). We also filed suit in the U.S. District Court for the District of New Jersey in April 2025 against Zydus seeking to prevent Zydus from selling a generic version of INGREZZA SPRINKLE.

In January 2025, we filed suit in the U.S. District Court for the District of Delaware against Spruce Biosciences, Inc. (Spruce), seeking a declaratory judgement of invalidity of one of Spruce's patents. In June 2025, the Court dismissed the case without prejudice for lack of case or controversy. In January 2025, we also initiated post-grant review against another Spruce patent in the U.S. Patent and Trademark Office. Spruce disclaimed all challenged claims of that patent. In addition, we have initiated judicial and administrative proceedings against Spruce patents in other jurisdictions.

From time to time, we may also become subject to other legal proceedings or claims arising in the ordinary course of our business. We currently believe that none of the claims or actions pending against us is likely to have, individually or in the aggregate, a material adverse effect on our business, financial condition or results of operations. Given the unpredictability inherent in litigation, however, we cannot predict the outcome of these matters.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following Management's Discussion and Analysis of Financial Condition and Results of Operations section contains forward-looking statements, which involve risks and uncertainties. Our actual results could differ materially from those anticipated in these forward-looking statements as a result of various factors, including those set forth in Part II, Item 1A under the caption "Risk Factors." The interim financial statements and this Management's Discussion and Analysis of Financial Condition and Results of Operations should be read in conjunction with the financial statements and notes thereto for the year ended December 31, 2024 and the related Management's Discussion and Analysis of Financial Condition and Results of Operations, which are contained in our Annual Report on Form 10-K for the year ended December 31, 2024 and our Quarterly Report on Form 10-Q for the six months ended June 30, 2025.

Overview

Neurocrine Biosciences is a neuroscience-focused, biopharmaceutical company with a simple purpose: to relieve suffering for people with great needs, but few options. We are dedicated to discovering and developing life-changing treatments for patients with under-addressed neuropsychiatric, neurological, and neuroendocrine disorders.

Our portfolio of products includes U.S. Food and Drug Administration (FDA) approved treatments for tardive dyskinesia (TD), chorea associated with Huntington's disease, classic congenital adrenal hyperplasia (CAH), and endometriosis and uterine fibroids in collaboration with AbbVie Inc. (AbbVie). In addition, we have a diversified portfolio of multiple compounds in mid- to late-phase development across our core therapeutic areas and an expanding early-phase pipeline that includes a range of modalities including small molecules, peptides, proteins, antibodies, conjugates, and gene therapy.

We launched INGREZZA® (valbenazine) in the U.S. as the first FDA-approved drug for the treatment of TD in May 2017 and for the treatment of chorea associated with Huntington's disease in August 2023 and launched CRENESSITY® (crinecerfont) in the U.S. as a first-in-class FDA-approved treatment of CAH in December 2024.

We estimate that TD affects approximately 800,000 people in the U.S., that approximately 90% of the 40,000 people in the U.S. affected by Huntington's disease will develop chorea, and that CAH affects at least 20,000 people in the U.S. Key elements of our commercial strategy include maximizing the opportunities in INGREZZA and CRENESSITY through consistent and effective commercial execution, continued development of valbenazine as the best-in-class treatment for new patient populations, and to lead the evolving understanding of vesicular monoamine transporter 2 (VMAT2) biology and its role in disease. INGREZZA net product sales totaled $1.9 billion and $1.7 billion, respectively, for the first nine months of 2025 and 2024 and accounted for substantially all of our total net product sales during each of these reporting periods. CRENESSITY net product sales totaled $165.8 million for the first nine months of 2025.

Our partner Mitsubishi Tanabe Pharma Corporation (MTPC) launched DYSVAL® (valbenazine) in Japan for the treatment of TD in June 2022 and subsequently in other select Asian markets, where it is marketed as REMLEAS® (valbenazine). We receive royalties at tiered percentage rates on MTPC net sales of valbenazine.

Our partner AbbVie launched ORILISSA® (elagolix tablets) in the U.S. for the treatment of endometriosis in August 2018 and ORIAHNN® (elagolix, estradiol and norethindrone acetate capsules and elagolix capsules) in the U.S. for the treatment of heavy menstrual bleeding due to uterine fibroids in June 2020. We receive royalties at tiered percentage rates on AbbVie net sales of elagolix.

2025 Business Highlights

- In February 2025, our Board of Directors authorized a new share repurchase program (the 2025 Repurchase Program) under which we may repurchase up to $500.0 million of our common stock, subject to market conditions. The 2025 Repurchase Program is in addition to the $300.0 million accelerated repurchase program (the 2024 Repurchase Program) that was announced in October 2024 and completed in February 2025. During the first nine months of 2025, we repurchased 1.5 million shares on the open market under the 2025 Repurchase Program and received an additional 0.3 million shares upon settlement of the 2024 Repurchase Program in February 2025.
- In January 2025, we received Centers for Medicare and Medicaid Services (CMS) notification that INGREZZA qualifies for the small biotech exception under the Medicare Drug Price Negotiation Program, which provides exemption from selection until 2027 for initial price applicability in 2029. In addition, we expanded formulary access for INGREZZA, significantly improving coverage to now include approximately 70% of TD and Huntington's disease Medicare beneficiaries to support long-term growth.
- Appointed Sanjay Keswani, M.D., as Chief Medical Officer (CMO) and member of the Company's executive management team effective June 2, 2025.

- Announced planned expansion of the INGREZZA and CRENESSITY sales teams to maximize commercial momentum. Expansion to be completed by the end of the first quarter of 2026.

2025 Pipeline Highlights

- Initiated two Phase 3 clinical studies for direclidine (NBI-1117568), a potential first-in-class, orally active, highly selective investigational M4 agonist, in schizophrenia.
- Initiated a Phase 3 clinical study for osavampator (NBI-1065845), a potential first-in-class alpha-amino-3-hydroxy-5-methyl-4-isoxazole propionic acid (AMPA) positive allosteric modulator (PAM), in major depressive disorder (MDD).
- Initiated a Phase 1 clinical study for NBIP-1435, an investigational, long-acting corticotropin-releasing factor type 1 (CRF-1) receptor antagonist administered as a subcutaneous injection for the potential treatment of CAH.
- Initiated a Phase 1 clinical study for NBI-921355, an investigational, selective inhibitor of voltage-gated sodium channels $Na_v1.2$ and $Na_v1.6$ in development for the potential treatment of certain types of epilepsy.
- Initiated a Phase 1 clinical study for NBI-1140675, an investigational, oral, selective second-generation small molecule VMAT2 inhibitor in development for the potential treatment of certain neurological and neuropsychiatric conditions.
- Announced top-line data from a Phase 4 study, KINECT-PRO™, demonstrating clinically meaningful and sustained effects of INGREZZA capsules on the physical, social, and emotional impacts experienced by patients living with TD, irrespective of TD severity or underlying psychiatric condition.
- Presented new data from a post-hoc analysis of the Phase 4 KINECT-PRO open-label study confirming that robust rates of symptomatic remission of tardive dyskinesia were achieved with once-daily INGREZZA capsules. The analysis also showed sustained improvements in patient-reported outcomes among participants who achieved symptomatic remission.
- Presented new data from the Phase 2 SAVITRI™ study, which showed statistically significant and clinically meaningful improvement in depression severity at Day 28 and Day 56 with once-daily oral administration of 1 mg osavampator.
- Announced the Phase 3 study of valbenazine for the adjunctive treatment of schizophrenia did not meet the primary endpoint.

Results of Operations for the Three and Nine Months Ended September 30, 2025 and 2024

Revenues

Net Product Sales

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in millions)	**2025**	**2024**	**2025**	**2024**
INGREZZA	$ 686.6	$ 612.9	$ 1,856.2	$ 1,698.4
CRENESSITY	98.1	—	165.8	—
Other	5.2	3.7	13.6	11.0
Total net product sales	$ 789.9	$ 616.6	$ 2,035.6	$ 1,709.4

Compared with the comparable periods last year, the increase for the third quarter and first nine months of 2025 primarily reflected increased net product sales of INGREZZA, driven by total prescriptions on strong patient demand partially offset by lower net price due to new market access investments to support long-term growth, and CRENESSITY, which was launched in the U.S. as a first-in-class FDA-approved treatment of CAH in December 2024.

Collaboration Revenues

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in millions)	**2025**	**2024**	**2025**	**2024**
Royalties	$ 4.8	$ 4.6	$ 18.0	$ 13.5
Other	0.2	0.9	1.4	4.7
Total collaboration revenues	$ 5.0	$ 5.5	$ 19.4	$ 18.2

Total collaboration revenues for all periods presented primarily reflected royalties earned on AbbVie net sales of elagolix and MTPC net sales of valbenazine.

Operating Expenses

Cost of Revenues

(*in millions*)	Three Months Ended September 30, 2025	Three Months Ended September 30, 2024	Nine Months Ended September 30, 2025	Nine Months Ended September 30, 2024
Cost of revenues	$ 14.0	$ 8.0	$ 34.5	$ 24.7

Compared with the comparable periods last year, the increase for the third quarter and first nine months of 2025 primarily reflected increased net product sales of INGREZZA and increased royalties payable on net product sales of CRENESSITY.

Research and Development

We support our drug discovery and development efforts through the commitment of significant resources to discovery, research and development programs, and business development opportunities. Costs are reflected in the applicable development stage based upon the program status when incurred. Therefore, the same program could be reflected in different development stages in the same reporting period. For several of our programs, the research and development activities are part of our collaborative arrangements.

(*in millions*)	Three Months Ended September 30, 2025	Three Months Ended September 30, 2024	Nine Months Ended September 30, 2025	Nine Months Ended September 30, 2024
Late stage	$ 52.2	$ 21.6	$ 126.3	$ 68.9
Early stage	16.1	19.2	54.5	77.6
Research and discovery	73.5	42.1	189.5	103.9
Milestones	1.0	38.8	61.5	71.4
Payroll and benefits	74.6	55.4	221.8	167.9
Facilities and other	32.6	17.9	103.9	55.8
Total research and development	$ 250.0	$ 195.0	$ 757.5	$ 545.5

Late Stage. Late stage consists of costs incurred for product candidates in Phase 2 registrational studies and all subsequent activities.

Compared with the comparable periods last year, the increase for the third quarter and first nine months of 2025 primarily reflected increased investment in the Phase 3 programs for osavampator in MDD and direclidine in schizophrenia, partially offset by lower spend related to crinecerfont in CAH.

Early Stage. Early stage consists of costs incurred for product candidates after the approval of an investigational new drug application by the applicable regulatory agency through Phase 2 non-registrational studies.

Compared with the comparable periods last year, the decrease for the third quarter and first nine months of 2025 primarily reflected the successful progression of the Phase 2 program for direclidine in schizophrenia to late-stage in the fourth quarter of 2024 and lower spend on certain early-stage neuropsychiatry programs, partially offset by increased investment in our early-stage muscarinic portfolio.

Research and Discovery. Research and discovery consists of costs incurred prior to the approval of an investigational new drug application by the applicable regulatory agency.

Compared with the comparable periods last year, the increase for the third quarter and first nine months of 2025 primarily reflected increased investment in gene therapy and other preclinical development programs.

Milestones. Milestones consists of costs incurred in connection with the achievement of development milestones under collaborative arrangements. The following table presents milestones expense by collaboration partner.

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in millions)	**2025**	**2024**	**2025**	**2024**
Nxera Pharma UK Limited	$ —	$ 35.0	$ 15.0	$ 50.0
Takeda Pharmaceutical Company Limited	—	—	37.5	7.5
Xenon Pharmaceuticals Inc.	—	—	7.5	—
Voyager Therapeutics, Inc.	—	3.0	—	11.0
Other	1.0	0.8	1.5	2.9
Total milestones	$ 1.0	$ 38.8	$ 61.5	$ 71.4

Refer to Note 2 to the condensed consolidated financial statements for more information on our significant collaboration and license agreements.

Payroll and Benefits. Payroll and benefits consists of costs incurred for salaries and wages, payroll taxes, benefits, and stock-based compensation associated with employees involved in research and development activities. Stock-based compensation may fluctuate from period to period based on factors that are not within our control, such as our stock price on the dates stock-based grants are issued.

Compared with the comparable periods last year, the increase for the third quarter and first nine months of 2025 primarily reflected higher headcount and increases of $7.2 million and $19.0 million, respectively, in non-cash stock-based compensation expense.

Facilities and Other. Facilities and other consists of indirect costs incurred for the benefit of multiple programs, including facility-based expenses (such as rent expense) and other overhead allocations.

Compared with the comparable periods last year, the increase for the third quarter and first nine months of 2025 primarily reflected increased facility-based expenses related to our new campus facility.

Selling, General, and Administrative

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in millions)	**2025**	**2024**	**2025**	**2024**
Selling, general, and administrative	$ 291.6	$ 234.3	$ 854.4	$ 719.4

Compared with the comparable periods last year, the increase for the third quarter and first nine months of 2025 primarily reflected continued investment in our commercial organization (including the expansion of our psychiatry and long-term care sales team completed in September 2024 and CRENESSITY-related headcount and commercial launch activities) and increases of $5.0 million and $11.2 million, respectively, in non-cash stock-based compensation expense. In addition, the increase for the first nine months of 2025 was partially offset by decreased impairment charges associated with our vacated legacy campus facilities.

Other Income (Expense)

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in millions)	**2025**	**2024**	**2025**	**2024**
Unrealized gain (loss) on equity investments	$ 30.6	$ (16.9)	$ (6.7)	$ (35.2)
Charges associated with convertible senior notes	—	—	—	(138.4)
Investment income and other, net	22.2	23.4	64.5	68.5
Total other income (expense), net	$ 52.8	$ 6.5	$ 57.8	$ (105.1)

Compared with the comparable periods last year, the change for the third quarter and first nine months of 2025 primarily reflected prior year charges associated with the convertible senior notes that matured in May 2024 and periodic fluctuations in the fair values of our equity investments.

Provision for Income Taxes

(in millions)	Three Months Ended September 30, 2025	Three Months Ended September 30, 2024	Nine Months Ended September 30, 2025	Nine Months Ended September 30, 2024
Provision for income taxes	$ 82.3	$ 60.5	$ 141.1	$ 85.2

The effective tax rate for the third quarter and first nine months of 2025 and 2024 varied from the federal and state statutory rates primarily due to credits generated for research activities, certain nondeductible expenses, excess tax benefits related to stock-based compensation, fluctuations in state effective tax rates, and valuation allowances recorded in certain foreign and domestic state jurisdictions for tax benefits that management cannot conclude that it is more likely than not will be realized in the future. In addition, the effective tax rate for the third quarter of 2025 reflects increased tax expense related to foreign operations and the enactment of the One Big Beautiful Bill Act (OBBBA).

Net Income

(in millions)	Three Months Ended September 30, 2025	Three Months Ended September 30, 2024	Nine Months Ended September 30, 2025	Nine Months Ended September 30, 2024
Net income	$ 209.5	$ 129.8	$ 324.9	$ 238.2

Compared with the comparable periods last year, the change for the third quarter and first nine months of 2025 primarily reflected increased INGREZZA and CRENESSITY net product sales, continued investments in our commercial organization (including the expansion of our psychiatry and long-term care sales team completed in September 2024 and CRENESSITY-related headcount and commercial launch activities) and expanded pre-clinical and clinical portfolio, periodic fluctuations in milestones expense associated with the achievement of development milestones under collaborative arrangements, periodic fluctuations in the fair values of our equity investments, and prior year charges associated with the convertible senior notes that matured in May 2024.

Liquidity and Capital Resources

Sources of Liquidity

We believe that our existing capital resources, funds generated by anticipated INGREZZA and CRENESSITY net product sales, and investment income will be sufficient to satisfy our current and projected funding requirements for at least the next 12 months. However, we cannot guarantee that our existing capital resources and anticipated revenues will be sufficient to conduct and complete all of our research and development programs or commercialization activities as planned. We may seek to access the public or private equity markets whenever conditions are favorable or pursue opportunities to obtain debt financing in the future. We may also seek additional funding through strategic alliances or other financing mechanisms. However, we cannot provide assurance that adequate funding will be available on terms acceptable to us, if at all.

Information Regarding Our Financial Condition

(in millions)	September 30, 2025	December 31, 2024
Total cash, cash equivalents, and marketable securities	$ 2,113.3	$ 1,815.6
Working Capital:		
Total current assets	$ 2,158.4	$ 1,724.7
Less total current liabilities	638.0	507.7
Total working capital	$ 1,520.4	$ 1,217.0

Information Regarding Our Cash Flows

(in millions)	Nine Months Ended September 30, 2025	2024
Cash flows from operating activities	$ 394.3	$ 352.9
Cash flows from investing activities	(211.5)	(58.5)
Cash flows from financing activities	(75.6)	(196.7)
Effect of exchange rate changes on cash and cash equivalents	—	0.3
Change in cash, cash equivalents, and restricted cash	$ 107.2	$ 98.0

Cash Flows from Operating Activities

Compared with the comparable period last year, the increase primarily reflected increased net product sales of INGREZZA and CRENESSITY, partially offset by continued investments in our commercial organization (including the expansion of our psychiatry and long-term care sales team completed in September 2024 and CRENESSITY-related headcount and commercial launch activities) and expanded pre-clinical and clinical portfolio. The increase in accounts receivable was driven by higher total gross product sales. The increase in accounts payable and accrued liabilities was driven by higher revenue-related reserves for discounts and allowances attributed to higher gross product sales combined with expanded formulary access for INGREZZA. In addition, the increase in income tax assets and liabilities primarily related to timing of foreign tax expense recognition, partially offset by the federal tax benefit on current income taxes payable from the enactment of the OBBBA.

Cash Flows from Investing Activities

Periodic fluctuations in cash flows from investing activities primarily reflected timing differences related to our purchases, sales, and maturities of debt security investments and changes in our portfolio-mix.

Cash Flows from Financing Activities

Compared with the comparable period last year, the change reflected $167.7 million in repurchases of our common stock during the first nine months of 2025 under the $500.0 million 2025 Repurchase Program that was authorized by our Board of Directors in February 2025, decreased proceeds from issuances of our common stock, and $308.8 million in cash payments to settle the convertible senior notes in the second quarter of 2024.

Material Cash Requirements

In the pharmaceutical industry, it can take a significant amount of time and capital resources to successfully complete all stages of research and development and commercialize a product candidate, which ultimate length of time and spend required cannot be accurately estimated as it varies substantially according to the type, complexity, novelty and intended use of a product candidate.

The funding necessary to execute our business strategies is subject to numerous uncertainties and we may be required to make substantial expenditures if unforeseen difficulties arise in certain areas of our business. In particular, our future capital requirements will depend on many factors, including:

- the commercial success of INGREZZA and CRENESSITY;
- continued scientific progress in our research and clinical development programs;
- the magnitude and complexity of our research and development programs;
- progress with preclinical testing and clinical trials;
- the time and costs involved in obtaining regulatory approvals;
- the costs involved in filing and pursuing patent applications, enforcing patent claims, or engaging in interference proceedings or other patent litigation;
- costs associated with securing adequate coverage and reimbursement for our products;
- competing technological and market developments;
- developments related to any future litigation;
- the cost of commercialization activities and arrangements, including our advertising campaigns; and
- the cost of manufacturing our product candidates.

In addition to the foregoing factors, we have significant future capital requirements, including:

External Business Developments

In addition to our independent efforts to develop and market products, we may enter into collaboration and license agreements or acquire businesses from time-to-time to enhance our drug development and commercial capabilities. With respect to our existing collaboration and license agreements, we may be required to make potential future payments of up to $14.0 billion upon the achievement of certain milestones.

Refer to Note 2 to the condensed consolidated financial statements for more information on our significant collaboration and license agreements.

Share Repurchase Program

In addition to the foregoing future capital requirements, in February 2025, our Board of Directors authorized the 2025 Repurchase Program under which we may repurchase up to $500.0 million of our common stock, subject to market conditions. The 2025 Repurchase Program is in addition to the $300.0 million 2024 Repurchase Program that was announced in October 2024 and completed in February 2025. Under the 2025 Repurchase Program, we repurchased 1.5 million shares on the open market for a cost of $167.7 million during the first nine months of 2025. As of September 30, 2025, we had $332.3 million remaining under the 2025 Repurchase Program.

Critical Accounting Policies and Estimates

There were no changes to our critical accounting policies as disclosed in our Annual Report on Form 10-K for the year ended December 31, 2024.

Interest Rate Risk

We maintain a diversified investment portfolio consisting of low-risk, investment-grade debt securities with maturities of up to three years, including investments in commercial paper, securities of government-sponsored entities and corporate bonds that are subject to interest rate risk. The primary objective of our investment activities is to preserve principal and maintain liquidity. If a 1% unfavorable change in interest rates were to have occurred on September 30, 2025, it would not have had a material effect on the fair value of our investment portfolio as of that date.

Forward-Looking Statements

This Quarterly Report on Form 10-Q contains forward-looking statements that involve a number of risks and uncertainties. Although our forward-looking statements reflect the good faith judgment of our management, these statements can only be based on facts and factors currently known by us. Consequently, these forward-looking statements are inherently subject to risks and uncertainties, and actual results and outcomes may differ materially from results and outcomes discussed in the forward-looking statements.

Forward-looking statements can be identified by the use of forward-looking words such as “believes,” “expects,” “hopes,” “may,” “will,” “plan,” “intends,” “estimates,” “could,” “should,” “would,” “continue,” “seeks,” “proforma,” or “anticipates,” or other similar words (including their use in the negative), or by discussions of future matters such as the development of new products, technology enhancements, possible changes in legislation and other statements that are not historical. These statements include but are not limited to statements under the captions “Risk Factors,” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” as well as other sections in this report. You should be aware that the occurrence of any of the events discussed under the heading in Part II titled “Item 1A. Risk Factors” and elsewhere in this report could substantially harm our business, results of operations and financial condition and that if any of these events occurs, the trading price of our common stock could decline and you could lose all or a part of the value of your shares of our common stock.

The cautionary statements made in this report are intended to be applicable to all related forward-looking statements wherever they may appear in this report. We urge you not to place undue reliance on these forward-looking statements, which speak only as of the date of this report. Except as required by law, we assume no obligation to update our forward-looking statements, even if new information becomes available in the future.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

A discussion of our exposure to, and management of, market risk appears in Part I, Item 2 of this Quarterly Report on Form 10-Q under the heading “Interest Rate Risk” and is incorporated into this Part I, Item 3 by reference.

Item 4. Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in our reports required by the Securities Exchange Act of 1934, as amended (the Exchange Act), is recorded, processed, summarized and reported within the timelines specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, management recognized that any controls and procedures, no matter how well designed and operated, can only provide reasonable assurance of achieving the desired control objectives, and in reaching a reasonable level of assurance, management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

As required by SEC Rule 13a-15(b), we carried out an evaluation, under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, of the effectiveness of the design and operation of our disclosure controls and procedures as of the end of the quarter covered by this report. Based on the foregoing, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level for the period covered by this report.

Changes in Internal Control over Financial Reporting

An evaluation was also performed under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, of any changes to our internal control over financial reporting that occurred during the quarter ended September 30, 2025, and that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

There were no significant changes in our internal controls over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the quarter ended September 30, 2025, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Part II. Other Information

Item 1. Legal Proceedings

In March 2025, we received a notice from Zydus Lifesciences Global FZE (Zydus FZE) that it had filed an abbreviated new drug application, or ANDA, with the FDA seeking approval of a generic version of INGREZZA SPRINKLE (valbenazine). The ANDA contained a Paragraph IV Patent Certification alleging that certain of our patents covering INGREZZA SPRINKLE are invalid and/or will not be infringed by Zydus FZE's importation, manufacture, use or sale of the medicine for which the ANDA was submitted. We filed suit in the U.S. District Court for the District of Delaware in April 2025 against Zydus Pharmaceuticals (USA) Inc. and its affiliates Zydus FZE, Zydus Worldwide DMCC (entity subsequently dismissed), Zydus Lifesciences Limited, and Zydus Healthcare (USA) LLC (entity subsequently dismissed) (collectively, Zydus). We also filed suit in the U.S. District Court for the District of New Jersey in April 2025 against Zydus seeking to prevent Zydus from selling a generic version of INGREZZA SPRINKLE.

In January 2025, we filed suit in the U.S. District Court for the District of Delaware against Spruce Biosciences, Inc. (Spruce), seeking a declaratory judgement of invalidity of one of Spruce's patents. In June 2025, the Court dismissed the case without prejudice for lack of case or controversy. In January 2025, we also initiated post-grant review against another Spruce patent in the U.S. Patent and Trademark Office. Spruce disclaimed all challenged claims of that patent. In addition, we have initiated judicial and administrative proceedings against Spruce patents in other jurisdictions.

From time to time, we may also become subject to other legal proceedings or claims arising in the ordinary course of our business. We currently believe that none of the claims or actions pending against us is likely to have, individually or in the aggregate, a material adverse effect on our business, financial condition or results of operations. Given the unpredictability inherent in litigation, however, we cannot predict the outcome of these matters.

Item 1A. Risk Factors

The following information sets forth risk factors that could cause our actual results to differ materially from those contained in forward-looking statements we have made in this Quarterly Report on Form 10-Q and those we may make from time to time. If any of the following risks actually occur, our business, operating results, prospects or financial condition could be harmed. Additional risks not presently known to us, or that we currently deem immaterial, may also affect our business operations. The risk factors set forth below with an asterisk (*) contain changes to the risk factors set forth in our Annual Report on Form 10-K for the fiscal year ended December 31, 2024.

Summary Risk Factors

We face risks and uncertainties related to our business, many of which are beyond our control. In particular, risks associated with our business include:

- We may not be able to continue to successfully commercialize INGREZZA or any of our product candidates if they are approved in the future.
- We may not be able to successfully launch CRENESSITY.
- If physicians and patients do not continue to accept INGREZZA or do not accept CRENESSITY, or our sales and marketing efforts are not effective, we may not generate sufficient revenue.
- We face intense competition, and if we are unable to compete effectively, the demand for our products may be reduced.
- Government and third-party payors may impose sales and pharmaceutical pricing controls on our products or limit coverage and/or reimbursement for our products or impose policies and/or make decisions regarding the status of our products that could limit our product revenues and delay sustained profitability.
- Because the development of our product candidates is subject to a substantial degree of technological uncertainty, we may not succeed in developing any of our product candidates.
- Our clinical trials may be delayed for safety or other reasons, or fail to demonstrate the safety and efficacy of our product candidates, which could prevent or significantly delay their regulatory approval.
- Enacted healthcare reform, drug pricing measures and other recent legislative initiatives, including the Inflation Reduction Act of 2022 (IRA), could adversely affect our business.
- We have increased the size of our organization and will need to continue to increase the size of our organization. Such increases may not be sufficient and we may encounter difficulties with managing our growth, which could adversely affect our results of operations.

- We are transforming our research and development strategies to include the development of biologics, which requires substantial investment, including in personnel and facilities. We may encounter difficulties as we expand and may fail to successfully develop or commercialize our biologic product candidates, which could adversely affect our results of operations.
- If we are unable to retain and recruit qualified scientists and other employees or if any of our key senior executives discontinues his or her employment with us, it may delay our development efforts or impact our commercialization of INGREZZA, CRENESSITY, or any product candidate approved by the FDA in the future.
- Use of our approved products or those of our collaborators could be associated with side effects or adverse events.
- We currently depend on a limited number of third-party suppliers. The loss of these suppliers, or delays or problems in the supply of INGREZZA, CRENESSITY, or our product candidates, could materially and adversely affect our ability to successfully develop or commercialize INGREZZA, CRENESSITY, or any of our product candidates.
- We depend on our current collaborators for the development and commercialization of several of our products and product candidates and may need to enter into future collaborations to develop and commercialize certain of our product candidates.
- We currently have no manufacturing capabilities. If third-party manufacturers of INGREZZA, CRENESSITY, or any of our product candidates fail to devote sufficient time and resources to our concerns, or if their performance is substandard, our ability to commercialize existing products, conduct clinical trials and develop new products could be impaired and our costs may rise.
- We license some of our core technologies and drug leads and candidates from third parties. If we default on any of our obligations under those licenses, or violate the terms of these licenses, we could lose our rights to those technologies and drug candidates or be forced to pay damages.
- If we are unable to protect our intellectual property, our competitors could develop and market products based on our discoveries, which may reduce demand for our products.
- Our customers are concentrated and therefore the loss of a significant customer may harm our business.
- We may need additional capital in the future. If we cannot raise additional funding, we may be unable to fund our business plan and our future research, development, commercial and manufacturing efforts.
- We expect to increase our expenses for the foreseeable future, and we may not be able to sustain growth and profitability.

Risks Related to Our Company

We may not be able to continue to successfully commercialize INGREZZA or any of our product candidates if they are approved in the future.

We launched INGREZZA in the U.S. as the first FDA-approved drug for the treatment of tardive dyskinesia in May 2017 and for the treatment of chorea associated with Huntington's disease in August 2023. Our ability to produce INGREZZA revenues consistent with expectations ultimately depends on our ability to continue to successfully commercialize INGREZZA and secure and maintain adequate third-party reimbursement. Our experience in marketing and selling pharmaceutical products began with INGREZZA's approval in 2017, when we hired our sales force and established our distribution and reimbursement capabilities, all of which are necessary to successfully commercialize our current and future products. We have continued to invest in our commercial infrastructure and distribution capabilities, including the recent expansion of our psychiatry and long-term care sales teams for INGREZZA in September 2024. While our team members and consultants have experience marketing and selling pharmaceutical products, we may face difficulties related to managing the rapid growth of our personnel and infrastructure, and there can be no guarantee that we will be able to maintain the personnel, systems, arrangements and capabilities necessary to continue to successfully commercialize INGREZZA or any product candidate approved by the FDA, or equivalent foreign authorities, in the future.

We may not be able to successfully launch CRENESSITY.

In December 2024, we announced FDA approval and launched CRENESSITY capsules and oral solution as an adjunctive treatment to glucocorticoid replacement to control androgens in adult and pediatric patients four years of age and older with classic CAH. We have also established our commercial team and hired our U.S. sales force for CRENESSITY. The successful commercial launch of CRENESSITY depends on the extent to which patients and physicians accept and adopt CRENESSITY as a treatment for CAH, and we do not know whether our expectations or estimates in this regard, or those of investors or securities analysts, will be accurate. Physicians may not prescribe CRENESSITY and patients may be unwilling to use CRENESSITY. In addition, patients may be unwilling to use CRENESSITY if reimbursement is not provided or reimbursement is inadequate to cover a significant portion of the cost to the patient. CRENESSITY is a first-in-class therapy for children and adults with classic CAH and will therefore require us to expend substantial time and resources to educate physicians and other healthcare providers about the benefits of CRENESSITY. If we are unable to provide our sales force with effective materials, including medical and sales literature to help them inform and educate potential customers about the benefits of CRENESSITY, our efforts to commercialize CRENESSITY may not be successful. Further, any negative publicity related to CRENESSITY, or negative development for CRENESSITY in our post-marketing commitments or in regulatory processes in other jurisdictions, may adversely impact the potential of CRENESSITY and our commercial results. If the commercialization of CRENESSITY and future sales are less successful than anticipated by us or our investors or securities analysts, our stock price could decline and our business may be harmed.

If physicians and patients do not continue to accept INGREZZA or do not accept CRENESSITY, or our sales and marketing efforts are not effective, we may not generate sufficient revenue.

The commercial success of INGREZZA and CRENESSITY will depend upon the acceptance of these products as safe and effective by the medical community and patients.

The market acceptance of INGREZZA and CRENESSITY could be affected by a number of factors, including:

- the timing of receipt of marketing approvals for additional indications;
- the safety and efficacy of the products;
- the pricing of these products;
- the availability of healthcare payor coverage and adequate reimbursement for the products;
- public perception regarding these products;
- the success of existing competitor products addressing our target markets or the emergence of equivalent or superior products; and
- the cost-effectiveness of the products.

If the medical community, patients and payors do not continue to accept our products as being safe, effective, superior and/or cost effective, we may not generate sufficient revenue.

**** We face intense competition, and if we are unable to compete effectively, the demand for our products may be reduced.***

The biotechnology and pharmaceutical industries are subject to rapid and intense technological change. We face, and will continue to face, competition in the development and marketing of our products and product candidates from academic institutions, government agencies, research institutions and biotechnology and pharmaceutical companies.

Competition may also arise from, among other things:

- other drug development technologies;
- methods of preventing or reducing the incidence of disease, including vaccines; and
- new small molecule or other classes of therapeutic agents.

Developments by others (including the development of generic equivalents) may render our product candidates or technologies obsolete or noncompetitive.

We are commercializing and performing research on or developing products for the treatment of several disorders, including tardive dyskinesia, chorea associated with Huntington's disease, classic congenital adrenal hyperplasia, uterine fibroids, endometriosis, pain, Parkinson's disease, schizophrenia, epilepsy, and other neurology, neuropsychiatry, neuroendocrinology, and neuroimmunology-related diseases and disorders, and there are a number of competitors to our products and product candidates. If one or more of our competitors' products or programs are successful (including the development of generic equivalents), the market for our products may be reduced or eliminated.

- INGREZZA competes with AUSTEDO® (deutetrabenazine), marketed by Teva Pharmaceuticals Industries, for the treatment of tardive dyskinesia in adults and chorea associated with Huntington's disease. A once-daily dosing of AUSTEDO (AUSTEDO XR) was introduced in February 2023. Additionally, there are a number of commercially available medicines used to treat tardive dyskinesia off-label, such as XENAZINE® (tetrabenazine) and generic equivalents, and various antipsychotic medications (e.g., clozapine), anticholinergics, benzodiazepines (off-label), and botulinum toxin. In addition, there are several programs in clinical development by other companies targeting Huntington's disease.
- CRENESSITY competes with high dose corticosteroid monotherapy which is the current standard of care to both correct the endogenous cortisol deficiency as well as reduce the excessive adrenocorticotropic hormone levels for patients with CAH. In the U.S. alone, there are more than two dozen companies manufacturing steroid-based products. In addition, there are several programs in clinical development by other companies targeting CAH.
- Our investigational treatments for potential use in schizophrenia and depression may in the future compete with several development-stage programs being pursued by other companies. In addition, there are a number of different anti-psychotic and anti-depressant medications currently used in these patient populations.
- Our investigational treatments for potential use in neurology, neuropsychiatry, neuroendocrinology and neuroimmunology may in the future compete with numerous approved products and development-stage programs being pursued by several other companies.

Compared to us, many of our competitors and potential competitors have substantially greater:

- capital resources;
- sales and marketing experience;
- research and development capabilities and capacity, including personnel and technology;
- regulatory experience;
- preclinical study and clinical testing experience;
- manufacturing, marketing and distribution experience; and
- production facilities.

Moreover, increased competition in certain disorders or therapies may make it more difficult for us to recruit or enroll patients in our clinical trials for similar disorders or therapies.

**** Government and third-party payors may impose sales and pharmaceutical pricing controls on our products or limit coverage and/or reimbursement for our products or impose policies and/or make decisions regarding the status of our products that could limit our product revenues and delay sustained profitability.***

Our ability to continue to commercialize INGREZZA and successfully launch and commercialize CRENESSITY will depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available. The continuing efforts of government and third-party payors to contain or reduce the costs of healthcare and the price of prescription drugs through various means may impact our revenues. These payors' efforts could decrease the price that we receive for any products we may develop and sell in the future.

Assuming we obtain coverage for a given product by a third-party payor, the resulting reimbursement rates may not be adequate or may require co-payments that patients find unacceptably high. Patients who are prescribed medications for the treatment of their conditions, and their prescribing physicians, generally rely on third-party payors to reimburse all or part of the costs associated with their prescription drugs. Patients are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover all or a significant portion of the out-of-pocket cost of our products. Coverage decisions may depend upon clinical and economic standards that disfavor new drug products when more established or lower cost therapeutic alternatives are already available or subsequently become available regardless of whether they are approved by the FDA for that particular use. Coverage decisions by payors for our competitors' products may also impact coverage for our products.

Government authorities and other third-party payors are developing increasingly sophisticated methods of controlling healthcare costs, such as by limiting coverage and the amount of reimbursement for particular medications. Further, no uniform policy requirement for coverage and reimbursement for drug products exists among third-party payors in the U.S. Therefore, coverage and reimbursement for drug products can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. In addition, communications from government officials, media outlets, and others regarding healthcare costs and pharmaceutical pricing could have a negative impact on our stock price, even if such communications do not ultimately impact coverage or reimbursement decisions for our products.

There may also be significant delays in obtaining coverage and reimbursement for newly approved drugs or indications, and coverage may be more limited than the purposes for which the drug is approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for coverage and reimbursement does not imply that a drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. In addition, we could also be subject to amendments in our rebate agreements with pharmaceutical benefit managers that require us to pay larger rebate amounts or modify our formulary position, which could have a material adverse effect on our business. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future. For example, government authorities could make a decision that adversely impacts the status of one of our products, which could impact the eligibility and/or the amount of government reimbursement for that product.

As a pharmaceutical manufacturer, we are subject to various federal statutes and regulations requiring the reporting of price data and the subsequent provision of concessions to certain purchasers/payors, including state Medicaid programs. Federal agencies issue guidance to manufacturers related to the interpretation of laws and regulations, and this guidance has changed and may change or be updated over time. In interpreting these laws, regulations and guidance, manufacturers may make reasonable assumptions to fill gaps, and these reasonable assumptions may need to be updated upon issuance of additional agency guidance.

If coverage and reimbursement are not available or reimbursement is available only to limited levels, we may be unable to successfully commercialize INGREZZA, CRENESSITY, or any of our product candidates for which we obtain marketing approval in the future. Our inability to promptly obtain coverage and profitable reimbursement rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition. Further, a majority of our current revenue is derived from federal healthcare program payors, including Medicare and Medicaid. Thus, changes in government reimbursement policies, government negotiation of the price of any of products, reductions in payments and/or our suspension or exclusion from participation in federal healthcare programs could have a material adverse effect on our business.

Further, the use of physician telehealth services remains elevated compared to prior years, fueled by expansion of coverage and reimbursement for telehealth services across public and private insurers. The limitations that telehealth places on the ability to conduct a thorough physical examination may impact the ability of providers to screen for tardive dyskinesia or chorea associated with Huntington’s disease, leading to fewer patients being diagnosed and/or treated.

Outside the U.S., reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies. The EU provides options for EU Member States to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. An EU Member State may approve a specific price for the medicinal product, it may refuse to reimburse a product at the price set by the manufacturer or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market.

To obtain reimbursement for our products in some European countries, including some EU Member States, we may be required to compile additional data comparing the cost-effectiveness of our products to other available therapies. The Health Technology Assessment (HTA) of medicinal products is becoming an increasingly common part of the pricing and reimbursement procedures in some EU Member States, including those representing the larger markets. The extent to which pricing and reimbursement decisions are influenced by the HTA of the specific medicinal product currently varies between EU Member States. If we are unable to obtain favorable pricing and reimbursement status in EU Member States for product candidates that we may successfully develop and for which we may obtain regulatory approval, any anticipated revenue from and growth prospects for those products in the EU could be negatively affected.

Legislators, policymakers, and payors may continue to propose and implement cost-containing measures to keep healthcare costs down. For example, in April 2025, the President issued an executive order that, among other things, directs specified agency heads to develop a Center for Medicare and Medicaid Innovation model that enables the Medicare program to obtain better value for high-cost prescription drugs and biological products. In May 2025, the President also issued an executive order directing the administration to take immediate steps to end global freeloading and take additional aggressive action should drug manufacturers fail to offer American consumers the Most-Favored Nation lowest price. The specifics of these proposals and policies are unclear and, as a result, there is uncertainty as to how these and other potential legal and regulatory changes may impact our business. These policies could reduce or limit the prices we are able to charge for our products and product candidates that we may successfully develop and for which we may obtain regulatory approval or the level of reimbursement available for our products from governmental authorities or third-party payors. In addition, the OBBBA is expected to reduce Medicaid spending and enrollment by implementing work requirements for some beneficiaries, capping state-directed payments, reducing federal funding and limiting provider taxes used to fund the program. The OBBBA also narrows access to the Patient Protection and Affordable Care Act (ACA) marketplace exchange enrollment and declines to extend the ACA enhanced advanced premium tax credits, set to expire in 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. Further, an increasing number of countries use prices for medicinal products established in other countries as "reference prices" to help determine the price of the product in their own territory. Consequently, a downward trend in prices of medicinal products in some countries could contribute to similar downward trends elsewhere, including in the U.S.

Because the development of our product candidates is subject to a substantial degree of technological uncertainty, we may not succeed in developing any of our product candidates.

Only a small number of research and development programs ultimately result in commercially successful drugs.

Potential products that appear to be promising at early stages of development may not reach the market for a number of reasons. These reasons include the possibilities that the potential products may:

- be found ineffective or cause harmful side effects during preclinical studies or clinical trials;
- fail to receive necessary regulatory approvals on a timely basis or at all;
- be precluded from commercialization by proprietary rights of third parties;
- be difficult to manufacture on a large scale; or
- be uneconomical to commercialize or fail to achieve market acceptance.

If any of our product candidates encounters any of these potential problems, we may never successfully market that product candidate.

**** Our clinical trials may be delayed for safety or other reasons, or fail to demonstrate the safety and efficacy of our product candidates, which could prevent or significantly delay their regulatory approval.***

Before obtaining regulatory approval for the sale of any of our potential products, we must subject these product candidates to extensive preclinical and clinical testing to demonstrate their safety and efficacy for humans. Clinical trials are expensive, time consuming and may take years to complete and the outcomes are uncertain.

In connection with the clinical trials of our product candidates, we face the risks that:

- the FDA or similar foreign regulatory authority may not allow an IND or foreign equivalent filings required to initiate human clinical studies for our drug candidates or the FDA or similar foreign regulatory authorities may require additional preclinical studies as a condition of the initiation of Phase 1 clinical studies, or additional clinical studies for progression from Phase 1 to Phase 2, or Phase 2 to Phase 3, or for NDA approval;
- the product candidate may not prove to be effective or as effective as other competing product candidates;
- we may discover that a product candidate may cause harmful side effects or results of required toxicology or other studies may not be acceptable to the FDA or similar foreign regulatory authorities;
- clinical trial results may not replicate or improve upon the results of previous trials;
- we or the FDA or similar foreign regulatory authorities may suspend or vary the trials;
- the results may not be statistically significant;
- clinical site initiation or patient recruitment and enrollment may be slower or more difficult than expected;
- the FDA or similar foreign regulatory authorities may not accept the data from any trial or trial site outside of the U.S.;

- a study is compromised due to patients dropping out and not completing the trials;
- unforeseen disruptions or delays may occur, caused by geopolitical and macroeconomic developments, man-made or natural disasters, public health pandemics or epidemics, armed conflicts, trade restrictions, tariffs, the ongoing shutdown of the U.S. federal government and the resulting effects on its regulatory agencies, or other business interruptions; and
- regulatory requirements may change.

These risks and uncertainties impact all of our clinical programs and any of the clinical, regulatory or operational events described above could change our planned clinical and regulatory activities. Geopolitical tensions could also affect our ability to obtain supplies of our investigational products, which could cause delays or otherwise disrupt our clinical trials and research and development efforts. Some of our suppliers and research and development collaborators are located in China, exposing us to the possibility of supply disruption in the event of changes to the laws, rules, regulations, and policies of the governments of the U.S. or China. Any such changes to laws or the adoption of tariffs or other restrictions could impact our ability to contract with certain Chinese biotechnology companies, cause delays, or have other adverse effects on the development of certain of our research programs.

In addition, late-stage clinical trials are often conducted with patients having the most advanced stages of disease. During the course of treatment, these patients can die or suffer other adverse medical effects for reasons that may not be related to the pharmaceutical agent being tested but which can nevertheless adversely affect clinical trial conduct, completion and results. Any failure or substantial delay in completing clinical trials for our product candidates may severely harm our business.

Even if the clinical trials are successfully completed, we cannot guarantee that the FDA or similar foreign regulatory authorities will interpret the results as we do, and more trials could be required before we submit our product candidates for approval. The FDA and similar foreign regulatory authorities have substantial discretion in the approval process and may either refuse to accept an application for substantive review or may form the opinion after review of an application that the application is insufficient to allow approval of a product candidate. To the extent that the FDA or similar foreign regulatory authorities do not accept our application for review or approve our application, we may be required to expend significant additional resources, which may not be available to us, to conduct additional trials in support of potential approval of our product candidates. Depending on the extent of these additional trials or any other studies that might be required, approval of any applications that we submit may be significantly delayed. It is also possible that any such additional studies, if performed and completed, may not be considered sufficient by the FDA or similar foreign regulatory authorities and we may be forced to delay or abandon our applications for approval.

**** We have increased the size of our organization and will need to continue to increase the size of our organization. Such increases may not be sufficient and we may encounter difficulties with managing our growth, which could adversely affect our results of operations.***

Since 2017, our number of full-time employees has grown from approximately 200 to over 1,800. Although we have substantially increased the size of our organization, we may need to add additional qualified personnel and resources, especially with the recent increase in the size of our sales force. Our current infrastructure may be inadequate to support our development and commercialization efforts and expected growth. Future growth will impose significant added responsibilities on our organization, including the need to identify, recruit, maintain and integrate additional employees and implement and expand managerial, operational and financial systems and may be costly and take time away from running other aspects of our business, including development and commercialization of our product candidates. For example, we implemented a new company-wide enterprise resource planning (ERP) system in 2024 to streamline certain existing business, operational, and financial processes. This project has required and may continue to require investment of capital and human resources, the re-engineering of processes of our business, and the attention of many employees who would otherwise be focused on other aspects of our business. Any deficiencies in the design of the ERP system could adversely affect the effectiveness of our internal control over financial reporting or our ability to accurately maintain our books and records, provide accurate, timely and reliable reports on our financial and operating results, or otherwise operate our business. Any of these consequences could have an adverse effect on our results of operations and financial condition.

Our future financial performance and our ability to commercialize INGREZZA, CRENESSITY, or any of our product candidates that receive regulatory approval in the future, will partially depend on our ability to manage any future growth effectively. In particular, as we commercialize INGREZZA and CRENESSITY, we will need to support the training and ongoing activities of our sales force and will likely need to continue to expand the size of our employee base for managerial, operational, financial and other resources. To that end, we must be able to successfully:

- manage our development efforts effectively;
- integrate additional management, administrative and manufacturing personnel;
- further develop our marketing and sales organization;
- compensate our employees on adequate terms in an increasingly competitive, inflationary market;
- attract and retain personnel; and
- maintain sufficient administrative, accounting and management information systems and controls.

We may not be able to accomplish these tasks or successfully manage our operations and, accordingly, may not achieve our research, development and commercialization goals. Our failure to accomplish any of these goals could harm our financial results and prospects.

**** We are transforming our research and development strategies to include the development of biologics, which requires substantial investment, including in personnel and facilities. We may encounter difficulties as we expand and may fail to successfully develop or commercialize our biologic product candidates, which could adversely affect our results of operations.***

We are transforming our research and development strategies to include the development of biologics, including peptides, proteins, antibodies, conjugates, and gene therapies. As a company, we do not have experience successfully developing and commercializing biologics and our current infrastructure may be inadequate to support the expected growth and transformation of processes, personnel, and technologies required for these new programs. We have hired employees with expertise in these modalities, but we will need to hire additional qualified personnel and expand our management, administrative, and technical staff to support the research and development organization. If we are unable to identify, recruit and integrate additional employees with the requisite skills, or effectively manage our transformation activities, the development of our biologic product candidates may not be successful, or be delayed or paused indefinitely. Additionally, the manufacture of biologics and cognate devices are more complex than the manufacture of small molecule therapies. We currently have no manufacturing capabilities for biologic product candidates and devices and rely on third-party manufacturers. We may encounter delays in production and delivery of our biologic product candidates and devices by our third-party manufacturers or other vendors, which would result in corresponding delays to our development and commercialization of such biologic candidates. In addition, the regulatory requirements in the United States and in other countries governing biologics are evolving and the FDA or comparable foreign regulatory authorities may change the requirements, or identify different regulatory pathways, for approval for any of our biologic candidates. As a result, we may be required to change our regulatory strategy or to modify our applications for regulatory approval, which could delay and impair our ability to complete the preclinical and clinical development and manufacture of, and obtain regulatory approval for, our biologic candidates. We have made, and expect to continue making, substantial investments in our research and development personnel and facilities, as well in external innovation to support our expansion into the development of our biologics. If any of these risks occur and we fail to successfully develop or commercialize our biologic product candidates, we may not realize a return on our investments which could have an adverse effect on our results of operations and financial condition.

**** If we are unable to retain and recruit qualified scientists and other employees or if any of our key senior executives discontinues his or her employment with us, it may delay our development efforts or impact our commercialization of INGREZZA, CRENESSITY, or any product candidate approved by the FDA in the future.***

We are highly dependent on the principal members of our management, commercial and scientific staff. The loss of any of these people could impede the achievement of our objectives, including the successful commercialization of INGREZZA, the launch of CRENESSITY, or the commercialization of any product candidate approved by the FDA in the future. Furthermore, recruiting and retaining qualified scientific personnel to perform research and development work in the future, along with personnel with experience marketing and selling pharmaceutical products, is critical to our success. We may be unable to attract and retain personnel on acceptable terms given the competition among biotechnology, pharmaceutical and healthcare companies, universities and non-profit research institutions for experienced scientists and individuals with experience marketing and selling pharmaceutical products. We may face particular retention challenges in light of the recent rapid growth in our personnel and infrastructure and the perceived impact of those changes upon our corporate culture. In addition, we rely on a significant number of consultants to assist us in formulating our research and development strategy and our commercialization strategy. Our consultants may have commitments to, or advisory or consulting agreements with, other entities that may limit their availability to us.

Use of our approved products or those of our collaborators could be associated with side effects or adverse events.

As with most pharmaceutical products, use of our approved products or those of our collaborators could be associated with side effects or adverse events which can vary in severity (from minor adverse reactions to death) and frequency (infrequent or prevalent). Side effects or adverse events associated with the use of our products or those of our collaborators may be observed at any time, including after a product is commercialized, and reports of any such side effects or adverse events may negatively impact demand for our or our collaborators' products or affect our or our collaborators' ability to maintain regulatory approval for such products. Side effects or other safety issues associated with the use of our approved products or those of our collaborators could require us or our collaborators to modify or halt commercialization of these products or expose us to product liability lawsuits which will harm our business. We or our collaborators may be required by regulatory agencies to conduct additional studies regarding the safety and efficacy of our products which we have not planned or anticipated. Furthermore, there can be no assurance that we or our collaborators will resolve any issues related to any product related adverse events to the satisfaction of the FDA or any regulatory agency in a timely manner or ever, which could harm our business, prospects and financial condition.

**** We currently depend on a limited number of third-party suppliers. The loss of these suppliers, or delays or problems in the supply of INGREZZA, CRENESSITY, or our product candidates, could materially and adversely affect our ability to successfully develop or commercialize INGREZZA, CRENESSITY, or any of our product candidates.***

The manufacture of pharmaceutical products requires significant expertise and capital investment, including the development of process controls required to consistently produce the active pharmaceutical ingredients (API), the finished drug product and packaging in sufficient quantities while meeting detailed product specifications on a repeated basis. Manufacturers of pharmaceutical products may encounter difficulties in production, such as difficulties with production costs and yields, process controls and validation, quality control and quality assurance, including testing of stability, impurities and impurity levels and other product specifications by validated test methods, compliance with strictly enforced U.S., state and non-U.S. regulations, and disruptions or delays caused by geopolitical and macroeconomic developments, man-made or natural disasters, public health pandemics or epidemics, armed conflicts, trade restrictions, tariffs, the ongoing shutdown of the U.S. federal government and the resulting effects on its regulatory agencies, or other business interruptions. We depend on a limited number of suppliers for the production (including API) of INGREZZA, CRENESSITY and our product candidates and for the packaging of INGREZZA and CRENESSITY. If our third-party suppliers for INGREZZA, CRENESSITY, or any of our product candidates encounter these or any other manufacturing, quality or compliance difficulties, our ability to successfully develop or commercialize INGREZZA, CRENESSITY, or any of our product candidates could be materially and adversely affected.

In addition, if our suppliers fail or refuse to supply us with INGREZZA, CRENESSITY, or any of our product candidates, or their APIs for any reason, or terminate our supply agreements or do not perform as agreed, it would take a significant amount of time and expense to qualify a new supplier. The FDA and similar foreign regulatory authorities must approve manufacturers of the active and inactive pharmaceutical ingredients and certain packaging materials used in pharmaceutical products. The loss of a supplier could require us to obtain regulatory clearance and to incur validation and other costs associated with the transfer of the API or product manufacturing processes. If there are delays in qualifying new suppliers or facilities or if a new supplier is unable to meet FDA or a similar foreign regulatory authority's requirements for approval, there could be a shortage of INGREZZA, CRENESSITY, or any of our product candidates, which could materially and adversely affect our ability to successfully develop or commercialize INGREZZA, CRENESSITY, or any of our product candidates.

**** We depend on our current collaborators for the development and commercialization of several of our products and product candidates and may need to enter into future collaborations to develop and commercialize certain of our product candidates.***

We depend on our current collaborators for the development and commercialization of several of our products and product candidates and may need to enter into future collaborations to develop and commercialize certain of our product candidates. For example, we depend on AbbVie for the manufacture and commercialization of ORILISSA and ORIAHNN and for the continued development of elagolix. We collaborate with MTPC for the commercialization of DYSVAL in Japan and for the continued development and commercialization of valbenazine for movement disorders in other select Asian markets. Some of our other collaborators include Nxera Pharma UK Limited (formerly Sosei Heptares), Takeda Pharmaceutical Company Limited, Voyager Therapeutics, Inc., and Xenon Pharmaceuticals Inc. Additionally, we depend on collaborators for the development of some of our biologics leads and candidates.

Our current and future collaborations and licenses could subject us to a number of risks, including:

- strategic collaborators may sell, transfer or divest assets or programs related to our partnered product or product candidates;
- we may be required to undertake the expenditure of substantial operational, financial and management resources;
- we may be required to assume substantial actual or contingent liabilities;
- we may not be able to control the amount and timing of resources that our strategic collaborators devote to the development or commercialization of our products or product candidates;
- we may not be able to influence our strategic collaborator's decisions regarding the development and collaboration of our partnered product and product candidates, and as a result, our collaboration partners may not pursue or prioritize the development and commercialization of those partnered products and product candidates in a manner that is in our best interest;
- strategic collaborators may select indications or design clinical trials in a way that may be less successful than if we were doing so;
- strategic collaborators may not conduct collaborative activities in a timely manner, provide insufficient funding, terminate a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new version of a product candidate for clinical testing;
- strategic collaborators may not pursue further development and commercialization of products resulting from the strategic collaboration arrangement or may elect to discontinue research and development programs;
- disagreements or disputes may arise between us and our strategic collaborators that result in delays or in costly litigation or arbitration that diverts management's attention and consumes resources;
- strategic collaborators may experience financial difficulties;
- strategic collaborators may not properly maintain, enforce or defend our intellectual property rights or may use our proprietary information in a manner that could jeopardize or invalidate our proprietary information or expose us to potential litigation;
- we or strategic collaborators could terminate the arrangement (in whole or in part) or allow it to expire, which would delay the development and commercialization, result in disagreements or disputes or may increase the cost of developing and commercializing our products or product candidates;

- strategic collaborators could develop, either alone or with others, products or product candidates that may compete with ours; and
- our strategic collaborator's decisions regarding the development and commercialization of a partnered product or product candidate within their territory(ies) could negatively impact us in the territories where we have development and commercialization rights for such product or product candidate.

If any of these issues arise, it may delay and/or negatively impact the development and commercialization of drug candidates and, ultimately, our generation of product revenues.

**** We currently have no manufacturing capabilities. If third-party manufacturers of INGREZZA, CRENESSITY, or any of our product candidates fail to devote sufficient time and resources to our concerns, or if their performance is substandard, our ability to commercialize existing products, conduct clinical trials and develop new products could be impaired and our costs may rise.***

We have in the past utilized, and intend to continue to utilize, third-party manufacturers to produce the drug compounds we use in our clinical trials and for the commercialization of our products. We have limited experience in manufacturing products for commercial purposes and do not currently have any manufacturing facilities. Establishing internal commercial manufacturing capabilities would require significant time and resources, and we may not be able to timely or successfully establish such capabilities. Consequently, we depend on, and will continue to depend on, several contract manufacturers for all production of products for development and commercial purposes, including INGREZZA and CRENESSITY. If we are unable to obtain or retain third-party manufacturers, we will not be able to develop or commercialize our products, including INGREZZA and CRENESSITY.

The manufacture of our products for clinical trials and commercial purposes is subject to specific FDA and equivalent foreign regulations, including current good manufacturing practice (cGMP) regulations. Our third-party manufacturers might not comply with FDA or equivalent foreign regulations relating to manufacturing our products for clinical trials and commercial purposes or other regulatory requirements now or in the future. Our reliance on contract manufacturers also exposes us to the following risks:

- contract manufacturers may encounter difficulties in achieving volume production, quality control or quality assurance, and also may experience shortages in qualified personnel or materials and ingredients necessary to conduct their operations. As a result, our contract manufacturers might not be able to meet our clinical schedules or adequately manufacture our products in commercial quantities when required;
- switching manufacturers may be difficult because the number of potential manufacturers is limited. It may be difficult or impossible for us to find a replacement manufacturer quickly on acceptable terms, or at all;
- our contract manufacturers may not perform as agreed or may not remain in the contract manufacturing business for the time required to successfully produce, store or distribute our products or product candidates; and
- drug manufacturers are subject to ongoing periodic unannounced inspection by the FDA, the U.S. Drug Enforcement Administration, equivalent foreign regulatory authorities, and other agencies to ensure strict compliance with cGMP and other government regulations and corresponding foreign standards. Any delay, interruption, or other issue that arises in the manufacture of our products or product candidates as a result of a failure of a third-party manufacturer to pass regulatory inspections or maintain cGMP compliance could significantly impair our ability to develop our product candidates or to obtain approval for or successfully commercialize our products.

Further, changes in federal policy could affect the geopolitical landscape and could give rise to circumstances that negatively affect our business. The third parties that manufacture our products have manufacturing facilities located in Europe. The U.S. has implemented, and has proposed to further implement, tariffs that may increase the costs of our third-party manufacturers and the expense to us to produce the drug compounds we use in our clinical trials and for the commercialization of our products. If such actions were to materially affect us or our third-party manufacturers, we may not be able to successfully develop our product candidates or commercialize our products.

Our current dependence upon third parties for the manufacture of our products may reduce our profit margin, if any, on the sale of INGREZZA, CRENESSITY, or our future products and our ability to develop and deliver products on a timely and competitive basis.

**** We license some of our core technologies and drug leads and candidates from third parties. If we default on any of our obligations under those licenses, or violate the terms of these licenses, we could lose our rights to those technologies and drug candidates or be forced to pay damages.***

We are dependent on licenses from third parties for some of our key technologies. These licenses typically subject us to various commercialization, reporting and other obligations. If we fail to comply with these obligations, we could lose important rights. If we were to default on our obligations under any of our licenses, we could lose some or all of our rights to develop, market and sell products covered by these licenses. In addition, several of our collaboration and license agreements allow our licensors to terminate such agreements if we challenge the validity or enforceability of certain intellectual property rights or if we commit a material breach in whole or in part of the agreement and do not cure such breach within the agreed upon cure period. In addition, if we were to violate any of the terms of our licenses, we could become subject to damages. Likewise, if we were to lose our rights under a license to use proprietary research tools, it could adversely affect our existing collaborations or adversely affect our ability to form new collaborations. We also face the risk that our licensors could, for a number of reasons, lose patent protection or lose their rights to the technologies we have licensed, thereby impairing or extinguishing our rights under our licenses with them.

**** Our customers are concentrated and therefore the loss of a significant customer may harm our business.***

We have entered into agreements for the distribution of INGREZZA with a limited number of specialty pharmacy providers and distributors. In addition, CRENESSITY is distributed by one specialty pharmacy provider. Four of these customers represent over 90% of our total gross product sales. If any of our significant customers becomes subject to bankruptcy, is unable to pay us for our products or wants to terminate their relationship with us, or if we otherwise lose any of these significant customers, our revenue, results of operations and cash flows would be adversely affected. Also, we may need to enter into agreements with additional distributors or specialty pharmacy providers, and there is no guarantee that we will be able to do so on commercially reasonable terms or at all. Even if we replace the loss of a significant customer, we cannot predict with certainty that such transition would not result in a decline in our revenue, results of operations and cash flows.

We may need additional capital in the future. If we cannot raise additional funding, we may be unable to fund our business plan and our future research, development, commercial and manufacturing efforts.

Our future funding requirements will depend on many factors and we may need to raise additional capital to fund our business plan and our future research, development, commercial and manufacturing efforts.

Our future capital requirements will depend on many factors, including:

- the commercial success of INGREZZA and CRENESSITY;
- the cost of commercialization activities and arrangements, including advertising campaigns;
- continued scientific progress in our R&D and clinical development programs;
- the magnitude and complexity of our research and development programs;
- progress with preclinical testing and clinical trials;
- the time and costs involved in obtaining regulatory approvals;
- the cost involved in filing and pursuing patent applications, enforcing patent claims, or engaging in interference proceedings or other patent litigation;
- costs associated with securing adequate coverage and reimbursement for our products;
- competing technological and market developments;
- developments related to any future litigation;
- the cost of manufacturing our product candidates;
- the impact of pandemics or epidemics on our business; and
- the cost of any strategic alliances, collaborations, product in-licensing, or acquisitions.

We may seek additional funding through public or private sales of our securities, including equity securities. In addition, we have previously financed capital purchases and may continue to pursue opportunities to obtain debt financing in the future. Additional equity or debt financing might not be available on reasonable terms, if at all. Any additional equity financings will be dilutive to our stockholders and any debt financings may involve operating covenants that restrict our business.

We expect to increase our expenses for the foreseeable future, and we may not be able to sustain growth and profitability.

We received FDA approval for INGREZZA for tardive dyskinesia in April 2017 and for chorea associated with Huntington's disease in August 2023. We received FDA approval for CRENESSITY capsules and oral solution as an adjunctive treatment to glucocorticoid replacement to control androgens in adult and pediatric patients four years of age and older with classic CAH in December 2024. Our partner AbbVie received FDA approval for ORILISSA for endometriosis in July 2018 and for ORIAHNN for uterine fibroids in May 2020. Additionally, our partner MTPC received Japanese Ministry of Health, Labour, and Welfare approval for DYSVAL for the treatment of tardive dyskinesia in March 2022. However, we have not yet obtained regulatory approvals for any other product candidates. Even if we continue to succeed in commercializing INGREZZA, or are successful in commercializing CRENESSITY or any of our product candidates, we may not be able to sustain profitability. We also expect to continue to incur significant operating and capital expenditures as we:

- commercialize INGREZZA for tardive dyskinesia and chorea associated with Huntington's disease;
- commercially launch CRENESSITY as an adjunctive treatment to glucocorticoid replacement to control androgens in adult and pediatric patients four years of age and older with classic CAH;
- seek regulatory approvals for our product candidates or for additional indications for our current products;
- develop, formulate, manufacture and commercialize our product candidates;
- in-license or acquire new product development opportunities;
- implement additional internal systems and infrastructure; and
- hire additional clinical, scientific, sales, marketing and administrative personnel.

We expect to increase our expenses and other investments in the coming years as we fund our operations and capital expenditures. Thus, our future operating results and profitability may fluctuate from period to period due to the factors described above, and we will need to generate significant revenues to achieve and maintain profitability and positive cash flow on a sustained basis. We may not be able to generate these revenues, and we may never achieve profitability on a sustained basis in the future. In addition, there is no guarantee that our prioritization determinations regarding our R&D and clinical development programs, including the acceleration or discontinuation of certain programs and product candidates, will generate their expected benefits and/or meet investor expectations. Our prioritization decisions may also adversely affect other internal programs and initiatives as well as our ability to recruit and retain skilled and motivated personnel. Our failure to maintain or increase profitability on a sustained basis could negatively impact the market price of our common stock.

**** The independent clinical investigators and contract research organizations that we rely upon to conduct our clinical trials may not be diligent, careful or timely, or may make mistakes in the conduct of our trials.***

We depend on independent clinical investigators and CROs to conduct our clinical trials under their agreements with us. The investigators are not our employees, and we cannot control the amount or timing of resources that they devote to our programs. If our independent investigators fail to devote sufficient time and resources to our drug development programs, or if their performance is substandard, or not in compliance with good clinical practices (GCPs), it may delay or prevent the approval of our regulatory applications and our introduction of new treatments. The CROs we contract with for execution of our clinical trials play a significant role in the conduct of the trials and the subsequent collection and analysis of data. Failure of the CROs to meet their obligations could adversely affect clinical development of our product candidates. Moreover, these independent investigators and CROs may also have relationships with other commercial entities, some of which may compete with us. If independent investigators and CROs assist our competitors at our expense, it could harm our competitive position.

**** We are subject to ongoing obligations and continued regulatory review for INGREZZA and CRENESSITY. Additionally, our product candidates, if approved, could be subject to labeling and other post-marketing requirements and restrictions.***

Regulatory approvals for any of our product candidates may be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate. For INGREZZA, CRENESSITY, and any product candidate that the FDA or a comparable foreign regulatory authority approves, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion and recordkeeping for the product is subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with GCPs for any clinical trials that we conduct post-approval. In addition, advertising and promotional materials for approved products must comply with FDA regulations and those of foreign regulatory authorities and may be subject to other potentially applicable federal and state laws. As part of the Make America Healthy Again (MAHA) Commission's recent Strategy Report, the current administration has prioritized stricter oversight of direct-to-consumer advertising, including increasing the amount of information manufacturers provide regarding risks associated with the use of prescription drugs and ensuring that advertisements are not false, misleading or lacking in fair balance through coordination across government agencies. In September 2025, the FDA Office of Prescription Drug Promotion issued numerous untitled letters and warning letters to drug manufacturers regarding advertising and promotion, including one untitled letter addressed to us which alleges that certain claims made in promotional material for INGREZZA are misleading. Although we believe that our advertisement complies with applicable laws and regulations, resolving the concerns stated in the letter or future letters we may receive could negatively impact the effectiveness of our advertising campaigns, increase compliance and media costs, and reduce demand for our products.

Failure to comply with these ongoing regulatory requirements, or later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, may result in, among other things:

- restrictions on the marketing or manufacturing of the product, changes in the product's label, misbranding allegations, withdrawal of the product from the market, or voluntary or mandatory product recalls;
- fines, warning or untitled letters or holds on clinical trials;
- refusal by the FDA or similar foreign regulatory authorities to approve pending applications or supplements to approved applications filed by us, or suspension or revocation of product license approvals;
- adverse inspection findings, enforcement actions, or other activities that temporarily delay manufacture and distribution of our products;
- product seizure or detention, or refusal to permit the import or export of products; and
- product injunctions or the imposition of civil or criminal penalties.

The occurrence of any of these events may adversely affect our business, prospects and ability to achieve or sustain profitability on a sustained basis.

The U.S. Supreme Court's June 2024 decision in *Loper Bright Enterprises v. Raimondo* overturned the longstanding *Chevron* doctrine, under which courts were required to give deference to regulatory agencies' reasonable interpretations of ambiguous federal statutes. The *Loper* decision could result in additional legal challenges to regulations and guidance issued by federal agencies, including the FDA, on which we rely. Any such legal challenges, if successful, could have a material impact on our business. Additionally, the *Loper* decision may result in increased regulatory uncertainty, inconsistent judicial interpretations, and other impacts to the agency rulemaking process, any of which could adversely impact our business and operations. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action or as a result of legal challenges, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, our business could be materially harmed.

If the market opportunities for our products and product candidates are smaller than we believe they are, our expected revenues may be adversely affected, and our business may suffer.

Certain of the diseases that INGREZZA, CRENESSITY, and our product candidates are being developed to address are in underserved and underdiagnosed populations. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who will seek treatment utilizing our products or product candidates, may not be accurate. If our estimates of the prevalence or number of patients potentially on therapy prove to be inaccurate, the market opportunities for INGREZZA, CRENESSITY, and our product candidates may be smaller than we believe they are, our prospects for generating expected revenue may be adversely affected and our business may suffer.

**** Because our operating results may vary significantly in future periods, our stock price may decline.***

Our quarterly revenues, expenses and operating results have fluctuated in the past and are likely to fluctuate significantly in the future. Our financial results are unpredictable and may fluctuate, for among other reasons, due to seasonality and timing of customer purchases and commercial sales of INGREZZA and CRENESSITY, royalties from out-licensed products, the impact of Medicare Part D coverage, including redesign of the Part D benefit enacted as part of the IRA, our achievement of product development objectives and milestones, clinical trial enrollment and expenses, research and development expenses and the timing and nature of contract manufacturing, contract research payments, fluctuations in our effective tax rate, disruptions caused by geopolitical and macroeconomic developments, man-made or natural disasters, public health pandemics or epidemics, armed conflicts, trade restrictions, tariffs, the ongoing shutdown of the U.S. federal government and the resulting effects on its regulatory agencies, or other business interruptions. Because a majority of our costs are predetermined on an annual basis, due in part to our significant research and development costs, small declines in revenue could disproportionately affect financial results in a quarter. Thus, our future operating results and profitability may fluctuate from period to period, and even if we become profitable on a quarterly or annual basis, we may not be able to sustain or increase our profitability. Moreover, as our company and our market capitalization have grown, our financial performance has become increasingly subject to quarterly and annual comparisons with the expectations of securities analysts or investors. The failure of our financial results to meet these expectations, either in a single quarterly or annual period over a sustained period of time, could cause our stock price to decline.

**** Changes in tax laws or regulations that are applied adversely to us or our customers may have a material adverse effect on our business, cash flows, financial condition or results of operations.***

New tax laws or regulations could be enacted at any time, and existing tax laws or regulations could be interpreted, modified or applied in a manner that is adverse to us or our customers, which could adversely affect our business and financial condition. Legislation commonly referred to as the One Big Beautiful Bill Act (the OBBBA), enacted in 2025, the IRA enacted in 2022, the Coronavirus Aid, Relief, and Economic Security Act enacted in 2020, and legislation informally titled the Tax Cuts and Jobs Act enacted in 2017, made significant changes to the U.S. tax laws. For example, the Tax Cuts and Jobs Act required taxpayers to capitalize and amortize U.S.-based and non-U.S. based research and experimental (R&E) expenditures over five and fifteen years, respectively. The OBBBA restored the deductibility of domestic R&E expenditures in the year incurred for tax years beginning after December 31, 2024, but retained the capitalization and amortization requirement for foreign R&E expenditures. Future guidance from the Internal Revenue Service and other tax authorities with respect to any legislation may affect us, and certain aspects of such legislation could be repealed or modified or sunset in future years. In addition, it is uncertain if and to what extent various states will conform to federal tax laws.

Furthermore, our tax obligations and effective tax rate in the jurisdictions in which we conduct business could increase as a result of international tax developments, including the implementation of the Organization for Economic Co-operation and Development's (OECD) Base Erosion and Profit Shifting "Two-Pillar" framework, which involves the reallocation of taxing rights in respect of certain multinational enterprises above a fixed profit margin to the jurisdictions in which they carry on business (referred to as Pillar One) and the imposition of a minimum effective corporate tax rate (referred to as Pillar Two). Certain countries in which we conduct business have enacted, or are in the process of enacting, core provisions of the Pillar Two rules. We continue to evaluate and assess the potential impact of these new rules, including on our effective tax rate, and our eligibility to qualify for any transition relief or safe harbor. Any changes in tax laws, including any new tax legislation or initiatives, could not only significantly increase our tax provision, cash tax liabilities, and effective tax rate, but could also have a material impact on the value of our deferred tax assets, result in significant one-time charges and ongoing compliance costs, and increase our future tax expense.

Our effective tax rate may fluctuate, and we may incur obligations in tax jurisdictions in excess of accrued amounts.

We have a multinational tax structure and are subject to income tax in the U.S. and various foreign jurisdictions, including the United Kingdom and Switzerland. Our effective tax rate is influenced by many factors including changes in our operating structure, changes in the mix of our earnings among countries, our allocation of profits and losses among our subsidiaries, our intercompany transfer pricing agreements and rules relating to transfer pricing, our inability to secure or sustain acceptable agreements with tax authorities, the impact of stock-based compensation, the availability of U.S. research and development tax credits, the results of examinations and audits of our tax filings, changes in accounting for income taxes, and future changes in tax laws and regulations in the U.S. and foreign countries. Significant judgment is required in determining our tax liabilities including management's judgment for uncertain tax positions. The Internal Revenue Service, other domestic taxing authorities, or foreign taxing authorities may disagree with our interpretation of tax laws as applied to our operations. Our reported effective tax rate and after-tax cash flows may be materially and adversely affected by tax assessments in excess of amounts accrued for our financial statements. This could cause us to experience an effective tax rate significantly different from previous periods or our current expectations.

**** The price of our common stock is volatile.***

The market prices for securities of biotechnology and pharmaceutical companies historically have been highly volatile, and the market for these securities has from time to time experienced significant price and volume fluctuations that are unrelated to the operating performance of particular companies. For example, the applicability of the Medicare drug price negotiation provisions in the IRA negatively affected investor sentiment and resulted in significant volatility. Furthermore, especially as we and our market capitalization have grown, the price of our common stock has been increasingly affected by quarterly and annual comparisons with the valuations and recommendations of the analysts who cover our business. If our results do not meet these analysts' forecasts, the expectations of our investors or the financial guidance we provide to investors in any period, which is based on assumptions that may be incorrect or that may change from quarter to quarter, the market price of our common stock could decline. Over the course of the last 12 months, the price of our common stock has ranged from approximately $84 per share to approximately $155 per share.

The market price of our common stock may fluctuate in response to many factors, including:

- sales of INGREZZA and CRENESSITY;
- failure of CRENESSITY to achieve commercial success;
- the results of our clinical trials;
- reports of safety issues related to INGREZZA or CRENESSITY;
- any delay in filing an IND, NDA, marketing authorization application (MAA), or other regulatory submission for any of our product candidates and any adverse development or perceived adverse development with respect to the applicable regulatory agency's review of that IND, NDA, MAA, or other regulatory submission, including but not limited to the imposition of a temporary or permanent clinical hold by a regulatory agency;
- developments concerning new and existing collaboration agreements;
- announcements of technological innovations or new therapeutic products by us or others, including our competitors;
- general economic and market conditions, including economic and market conditions affecting the biotechnology industry;
- developments in patent or other proprietary rights;
- developments related to the FDA, the Centers for Medicare & Medicaid Services (CMS) and foreign regulatory agencies;
- government regulation, including the IRA;
- future sales of our common stock by us or our stockholders;
- any trading activity pursuant to a share repurchase program;
- comments by securities analysts;
- additions or departures of key personnel;
- fluctuations in our operating results;
- potential litigation matters;
- government and third-party payor coverage and reimbursement;

- failure of any of our product candidates to achieve commercial success even if approved;
- disruptions caused by geopolitical and macroeconomic developments, man-made or natural disasters, public health pandemics or epidemics, armed conflicts, trade restrictions, tariffs, including protectionist or retaliatory measures taken by the United States or other countries, the ongoing shutdown of the U.S. federal government and the resulting effects on its regulatory agencies, or other business interruptions; and
- public concern as to the safety of our drugs.

In addition, we are a member of the S&P MidCap 400 index. If we cease to be represented in the S&P MidCap 400 index, or other indexes or indexed products, as a result of our market capitalization falling below the threshold for inclusion in the index, certain institutional shareholders may, due to their internal policies and investment guidelines, be required to sell their shareholdings. Such sales may result in further negative pressure on our stock price and, when combined with reduced trading volume and liquidity, could adversely affect the value of your investment and your ability to sell your shares.

**** There can be no assurance that any share repurchases will enhance long-term stockholder value.***

In October 2024, our Board of Directors authorized a share repurchase program to repurchase up to $300 million of our common stock and we subsequently entered into an accelerated share repurchase (ASR) transaction to repurchase the entirety of this authorized amount. The purchase period for this ASR transaction ended in February 2025 and an aggregate of 2.3 million shares were delivered to us at an average repurchase price of $131.83 per share. Additionally, in February 2025, our Board of Directors authorized a share repurchase program under which we may repurchase up to $500 million of our common stock (of which $332.3 million remained available as of September 30, 2025). This subsequent share repurchase authorization was in addition to the $300 million accelerated share repurchase program that was announced in October 2024 and completed in early February 2025. Our share repurchases may change from time to time, and we can provide no assurance that we will repurchase shares of our common stock at favorable prices, in particular amounts, or at all, and any repurchases may not enhance long-term stockholder value or prove to be the best use of our cash. If our Board of Directors authorizes any additional share repurchase programs, it could affect the trading price of our stock and increase volatility.

**** Compliance with changing laws, regulations and standards relating to various aspects of our business, including corporate governance, workforce initiatives and public disclosure, may result in additional expenses and failure to comply with such laws, regulations and standards could adversely affect our business.***

Changing laws, regulations and standards relating to various aspects of our business, including corporate governance, workforce initiatives and public disclosure, including as a result of the Dodd-Frank Wall Street Reform and Consumer Protection Act, new SEC regulations and Nasdaq rules and executive orders, are creating uncertainty for companies such as ours. These laws, regulations and standards are subject to varying interpretations in some cases due to their lack of specificity, and as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies, which could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure, policies and governance practices. We are committed to maintaining high standards of corporate governance, workforce initiatives and public disclosure. As a result, our efforts to comply with evolving laws, regulations and standards have resulted in, and are likely to continue to result in, increased selling, general and administrative expenses and management time related to compliance activities. If we fail, or are perceived to fail, to comply with these laws, regulations and standards, our reputation may be harmed and we might be subject to litigation, sanctions, investigations or other regulatory proceedings by regulatory authorities, such as the SEC. Any such action could adversely affect our financial results and the market price of our common stock.

Increasing use of social media could give rise to liability and result in harm to our business.

Our employees are increasingly utilizing social media tools and our website as a means of communication. Despite our efforts to monitor social media communications, there is risk that the unauthorized use of social media by our employees to communicate about our products or business, or any inadvertent disclosure of material, nonpublic information through these means, may result in violations of applicable laws and regulations, which may give rise to liability and result in harm to our business. In addition, there is also risk of inappropriate disclosure of sensitive information, which could result in significant legal and financial exposure and reputational damages that could potentially have a material adverse impact on our business, financial condition and results of operations. Furthermore, negative posts or comments about us or our products on social media could seriously damage our reputation, brand image and goodwill.

We may be subject to claims that we or our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

As is commonplace in the biotechnology industry, we employ individuals who were previously employed at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although no claims against us are currently pending, we may be subject to claims that these employees or we have inadvertently or otherwise used or disclosed trade secrets or other proprietary information of their former employers. Litigation may be necessary to defend against these claims. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to management.

Risks Related to Our Industry

**** Enacted healthcare reform, drug pricing measures and other recent legislative initiatives, including the IRA, could adversely affect our business.***

The business and financial condition of pharmaceutical and biotechnology companies are affected by the efforts of government and third-party payors to contain or reduce the costs of healthcare and to lower drug prices. In the U.S., comprehensive drug pricing legislation enacted by the Federal government implements, for the first time, government control over the pricing of certain prescription pharmaceuticals. Moreover, in some foreign jurisdictions, pricing of prescription pharmaceuticals is also subject to government control. Additionally, other federal and state laws impose obligations on manufacturers of pharmaceutical products, among others, related to disclosure of new drug products introduced to the market and increases in drug prices above a specified threshold.

For example, the IRA provides for, among other things: (1) the Secretary of the HHS to negotiate the price of certain high-expenditure, single-source drugs and biologics covered under Medicare; (2) the redesign of the Medicare Part D prescription drug benefit to lower patient out-of-pocket costs and increase manufacturer liability; and (3) drug manufacturers to pay rebates on drugs whose prices increase greater than the rate of inflation. The IRA also extends enhanced subsidies for individuals purchasing health insurance coverage in the ACA marketplaces through plan year 2025 and in 2025, eliminated the "donut hole" under the Medicare Part D program and created a new, permanent cap on beneficiary out-of-pocket spending for Part D drugs, in addition to a newly established manufacturer discount program. The IRA permits HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years. HHS has issued and updated and will continue to issue and update guidance as these programs are implemented. These provisions took effect progressively beginning in 2023. On August 15, 2024, HHS announced the negotiated prices of the first 10 drugs that were subject to price negotiations (for initial price applicability year 2026), although the Medicare drug price negotiation program is currently subject to legal challenges. On January 17, 2025, HHS announced its selection of fifteen additional drugs covered under Part D for negotiation in 2025 (for initial price applicability year 2027). Certain high-expenditure Part B and Part D drugs/biologics will be selected for negotiation in 2026 (for initial price applicability year 2028) and annually thereafter. AUSTEDO and AUSTEDO XR, marketed by Teva Pharmaceuticals Industries, have been selected for the Medicare drug negotiation program in 2025 (for initial price applicability year 2027). If the negotiation program results in a decrease in the price of AUSTEDO or AUSTEDO XR, it may result in increased competitive pressure on INGREZZA. We were notified in January 2025 that INGREZZA qualifies for the small biotech exception, which provides an exemption from selection until 2027 (for initial price applicability year 2029, pursuant to which negotiated pricing would go into effect, if selected). If negotiated for initial price applicability year 2029, we expect that the negotiated price for INGREZZA would be constrained by the "short monopoly" price ceiling and temporary price floor for small biotech drugs.

Additionally, on January 1, 2025, CMS implemented those provisions of the IRA establishing a new Medicare Part D manufacturer discount program. Under this discount program and subject to certain exceptions, manufacturers must give a 10 percent discount on Part D program drugs in the initial coverage phase, and a 20 percent discount on Part D drugs when the beneficiary enters the catastrophic coverage phase (the phase after the patient incurs costs above the initial phase out-of-pocket threshold, which is $2,000 in 2025, indexed to inflation thereafter annually). However, the IRA allows the 10 and 20 percent discounts to be phased in over a multi-year period for "specified manufacturers" and "specified small manufacturers". During this phase-in period, such manufacturers would pay a lower percentage discount on Medicare Part D program drugs. In April 2024, the Company was notified by CMS that it qualified as a "specified small manufacturer" and will receive the discount phase-in discussed above for INGREZZA. INGREZZA is reimbursed under Medicare Part D, and increased discounts could impact INGREZZA revenues, while also having an industry-wide impact on the cost of other Part D program drugs such as AUSTEDO and AUSTEDO XR. The overall impact on INGREZZA revenues is inherently uncertain and difficult to predict, and we are still evaluating the potential impact of this discount program and our designation as a "specified small manufacturer."

Our designation as a "specified small manufacturer" under the new Medicare Part D manufacturer discount program and INGREZZA's qualification for the small biotech exception for purposes of the Medicare drug price negotiation program are subject to various requirements and there is no assurance that we will continue to qualify for these exemptions in the future. The loss or potential loss of these exemptions, including as a result of a third party acquiring us, could have an adverse impact on our business.

Prior to the IRA's enactment, the most significant recent federal legislation impacting the pharmaceutical industry occurred in March 2010, when the ACA was signed into law. The ACA was intended to broaden access to health insurance and reduce the number of uninsured individuals, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add transparency requirements for the healthcare and health insurance industries, impose taxes and fees on the health industry and impose additional health policy reforms.

Other legislative changes have been adopted since the ACA was enacted. These changes include aggregate reductions to Medicare payments to providers of up to 2% per fiscal year pursuant to the Budget Control Act of 2011, which began in 2013 and, due to subsequent legislative amendments to the statute, including the Infrastructure Investment and Jobs Act and Consolidated Appropriations Act of 2023, will remain in effect until 2032. The American Taxpayer Relief Act of 2012, among other things, further reduced Medicare payments to several providers, including hospitals and cancer treatment centers, increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

We participate in the Public Health Service's 340B Drug Pricing Program (which is administered by the Health Resources and Services Administration), the Medicaid Drug Rebate program, and other federal and state government pricing programs. Participation in some of these programs is required in order to obtain reimbursement of our drug products under Medicaid or Medicare Part B. These programs generally require that we provide discounts or pay rebates to certain payers when our products are dispensed to beneficiaries of these programs. Pricing and rebate calculations are complex and are often subject to interpretation by us, governmental or regulatory agencies, and the courts, which can change and evolve over time. Furthermore, regulatory and legislative changes, and judicial rulings relating to these programs and policies could introduce additional uncertainty for our business and impact our product prices and rebate liability. These could include expansion of the 340B Drug Pricing Program and growth of entities claiming entitlement under this program, changes to the calculation of rebates under the programs, or other regulatory changes impacting reimbursement. Continued expansion of the 340B Drug Pricing Program and growth of entities claiming entitlement to 340B pricing, including in ways that may be inconsistent with the statutory scheme, could impact our revenue.

The current administration is pursuing policies to reduce regulations and expenditures across the government including at HHS, the FDA, CMS and related agencies. These actions, presently directed by executive orders or memoranda from the Office of Management and Budget, may propose policy changes that create additional uncertainty for our business. These actions include, for example, (a) directives to reduce agency workforces, (b) rescinding a Biden administration executive order tasking the Center for Medicare and Medicaid Innovation to consider new payment and healthcare models to limit drug spending, (c) eliminating the Biden administration's executive order that directed HHS to establish an AI task force and developing a strategic plan, (d) directing HHS and other agencies to lower prescription drug costs through a variety of initiatives, including by improving upon the Medicare Drug Price Negotiation Program and establishing Most-Favored Nation pricing for pharmaceutical products, (e) as part of the MAHA Commission's recent Strategy Report, working across government agencies to increase enforcement of direct-to-consumer pharmaceutical advertising, and (f) directing certain federal agencies to enforce existing law regarding hospital and plan price transparency and by standardizing prices across hospitals and health plans, each of which creates uncertainty for us and could negatively impact our business.

At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. For example, on January 5, 2024, the FDA approved Florida's SIP proposal to import certain drugs from Canada for specific state healthcare programs. It is unclear how this program will be implemented, including which drugs will be chosen, and whether it will be subject to legal challenges in the U.S. or Canada. Other states have also submitted SIP proposals that are pending review by the FDA. Any such approved importation plans, when implemented, may result in lower drug prices for products covered by those programs. Further, certain states through legislation have created a state PDAB to help control costs of drugs for that state. The functions of the PDABs vary by state, and may include among other things, recommending or setting upper limits on the price the state pays for certain drugs, performing drug affordability reviews, and advising state lawmakers on additional ways to reduce the state's drug spending. It is possible that the actions taken by the PDABs may result in lower prices for certain drug products sold in their states.

The implementation of these cost containment measures may prevent us from being able to generate revenue, attain sustained profitability or commercialize our drugs, particularly since the majority of our current revenue is derived from federal healthcare programs, including Medicare and Medicaid.

If we are unable to protect our intellectual property, our competitors could develop and market products based on our discoveries, which may reduce demand for our products.

Our success will depend on our ability to, among other things:

- obtain patent protection for our products;
- preserve our trade secrets;
- prevent third parties from infringing upon our proprietary rights; and
- operate without infringing upon the proprietary rights of others, both in the U.S. and internationally.

Because of the substantial length of time and expense associated with bringing new products through the development and regulatory approval processes in order to reach the marketplace, the pharmaceutical industry places considerable importance on obtaining patent and trade secret protection for new technologies, products and processes. Accordingly, we intend to seek patent protection for our proprietary technology and compounds. However, we face the risk that we may not obtain any of these patents and that the breadth of claims we obtain, if any, may not provide adequate protection of our proprietary technology or compounds. Additionally, if our employees, commercial collaborators or consultants use generative artificial intelligence (AI) technologies to develop our proprietary technology and compounds, it may impact our ability to obtain or successfully defend certain intellectual property rights.

We also rely upon unpatented trade secrets and improvements, unpatented know-how and continuing technological innovation to develop and maintain our competitive position, which we seek to protect, in part, through confidentiality agreements with our commercial collaborators, employees and consultants. We also have invention or patent assignment agreements with our employees and some, but not all, of our commercial collaborators and consultants. However, if our employees, commercial collaborators or consultants breach these agreements, we may not have adequate remedies for any such breach, and our trade secrets may otherwise become known or independently discovered by our competitors.

In addition, although we own a number of patents, the issuance of a patent is not conclusive as to its validity or enforceability, and third parties may challenge the validity or enforceability of our patents. We cannot assure you how much protection, if any, will be given to our patents if we attempt to enforce them and they are challenged in court or in other proceedings. It is possible that a competitor may successfully challenge our patents or that challenges will result in limitations of their coverage. Moreover, competitors may infringe our patents or successfully avoid them through design innovation. In addition, potential competitors have in the past and may in the future file an abbreviated new drug application (ANDA) with the FDA seeking approval to market a generic version of our products, or our competitors' products, before the expiration of the patents covering our products or our competitors' products, as applicable.

To prevent infringement or unauthorized use, we have in the past and may in the future need to file infringement claims, which are expensive and time-consuming. In addition, in an infringement proceeding a court may decide that a patent of ours or a patent of a competitor is not valid or is unenforceable or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover its technology. Derivation proceedings declared by the U.S. Patent and Trademark Office may be necessary to determine the priority of inventions with respect to our patent applications (or those of our licensors) or a patent of a competitor. Litigation or derivation proceedings may fail and, even if successful, may result in substantial costs and be a distraction to management. Litigation or derivation proceedings, including proceedings of a competitor, may also result in a competitor entering the marketplace faster than expected. We cannot assure you that we will be able to prevent misappropriation of our proprietary rights, particularly in countries where the laws may not protect such rights as fully as in the U.S.

**** Changes in the FDA, the U.S. Patent and Trademark Office, other government agencies or comparable foreign regulatory authorities could hinder their ability to hire and retain key leadership and other personnel, delay the development and commercialization of new products or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.***

The ability of the FDA or comparable foreign regulatory authorities to review and approve new products, and the ability of the U.S. Patent and Trademark Office and other government agencies to perform their normal business functions, can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and manage user fee programs, and statutory, regulatory, and policy changes (such as reductions in force). FDA review performance has fluctuated in recent years as a result. In addition, government funding of other government agencies or comparable foreign regulatory authorities on which our operations may rely, including the U.S. Patent and Trademark Office, the Patent Trial and Appeal Board and those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA, other government agencies or comparable foreign regulatory authorities may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, including beginning on October 1, 2025 and continuing to the present, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical employees and stop critical activities to the extent they are not funded by existing available user fees. A prolonged government shutdown could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, government shutdowns could impact our ability to access the public markets and obtain additional capital in the future.

Proposed healthcare reform, drug pricing measures and other prospective legislative initiatives could adversely affect our business.

We expect that there will continue to be a number of federal and state proposals to implement additional government controls over the pricing of prescription pharmaceuticals. Increasing emphasis on reducing the cost of healthcare in the U.S. will continue to put pressure on the pricing and reimbursement of prescription pharmaceuticals.

In addition, certain jurisdictions outside of the U.S., including the EU, have instituted price ceilings on specific products and therapies, as described further in the risk factor titled “Government and third-party payors may impose sales and pharmaceutical pricing controls on our products or limit coverage and/or reimbursement for our products or impose policies and/or make decisions regarding the status of our products that could limit our product revenues and delay sustained profitability.”

We are currently unable to predict what other additional legislation or regulation, if any, relating to the healthcare industry may be enacted in the future or what effect recently enacted federal or equivalent foreign legislation or any such additional legislation or regulation would have on our business, particularly in light of the recent U.S. Presidential and Congressional elections. The pendency or approval of such proposals or reforms could result in a decrease in our stock price or limit our ability to raise capital or to enter into collaboration agreements for the further development and commercialization of our programs and products.

**** Any relationships with healthcare professionals, principal investigators, consultants, customers (actual and potential) and third-party payors in connection with our current and future business activities are and will continue to be subject, directly or indirectly, to federal and state healthcare laws. If we are unable to comply, or have not fully complied, with such laws, we could face penalties, contractual damages, reputational harm, diminished profits and future earnings and curtailment or restructuring of our operations.***

Our business operations and activities may be directly, or indirectly, subject to various federal and state healthcare laws, including without limitation, fraud and abuse laws, false claims laws, data privacy and security laws, as well as transparency laws regarding payments or other items of value provided to healthcare providers. These laws may restrict or prohibit a wide range of business activities, including, but not limited to, research, manufacturing, distribution, pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. These laws may impact, among other things, our current activities with principal investigators and research subjects, as well as current and future sales, marketing, patient co-payment assistance and education programs.

Such laws include:

- the federal Anti-Kickback Statute which prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid;
- the federal civil and criminal false claims laws, including the federal civil False Claims Act, and Civil Monetary Penalties Laws, which impose criminal and civil penalties against individuals or entities for, among other things, knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;
- HIPAA, which imposes criminal and civil liability for, among other things, executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- HIPAA, as amended by HITECH and its implementing regulations, which also imposes obligations, including mandatory contractual terms, on covered entities, including certain healthcare providers, health plans and healthcare clearinghouses, as well as their business associates and their covered subcontractors, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to CMS information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), other healthcare professionals (such as physician assistants and nurse practitioners) and teaching hospitals, and applicable manufacturers and applicable group purchasing organizations to report annually to CMS ownership and investment interests held by physicians and their immediate family members; and
- analogous state, local and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third party payors, including private insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures or drug pricing; state laws that require disclosure of price increases above certain identified thresholds as well as of new commercial launches in the state; state laws that create Prescription Drug Price Affordability Boards to review or attempt to cap drug spending; state and local laws that require the registration of pharmaceutical sales representatives; state and local "drug take back" laws and regulations; and state and foreign laws governing the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our business arrangements will comply with applicable healthcare laws may involve substantial costs. While our interactions with healthcare professionals, including our speaker programs and other arrangements have been structured to comply with these laws and related guidance, it is possible that governmental and enforcement authorities will conclude that our business practices, business practices of our vendors or consultants, or a rogue employee's activities, may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws. For example, we maintain a patient assistance program to help eligible patients afford our products. These and other types of programs have become the subject of governmental scrutiny, and numerous organizations, including pharmaceutical manufacturers, have been subject to litigation, enforcement actions and settlements related to their patient assistance programs. In August 2025, we received a civil investigative demand from the U.S. Department of Justice (DOJ) requesting certain documents and information related to our sales and marketing of INGREZZA. We are cooperating with the DOJ's request. No assurance can be given as to the timing or outcome of the DOJ's investigation. If our operations or activities or those of our vendors are found to be in violation of any of the laws described above or any other applicable governmental regulations, we may be subject to, without limitation, significant civil, criminal and administrative penalties, damages, monetary fines, disgorgement, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, reputational harm, diminished profits and future earnings and curtailment or restructuring of our operations, any of which could adversely affect our ability to operate.

Any sales of our product once commercialized outside the U.S. will also likely subject us to foreign equivalents of the healthcare laws mentioned above, among other foreign laws. Additionally, because of our U.S. and international operations, we are also subject to anti-corruption laws and regulations, in the United States and internationally, including but not limited the U.S. Foreign Corrupt Practices (FCPA), the U.K. Bribery Act 2010, and other applicable anti-bribery and corruption laws. Anti-corruption laws are interpreted broadly and prohibit corporations and individuals from engaging in certain activities to obtain or retain business or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any foreign government official, government staff member, political party, or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity. The FCPA also imposes accounting standards and requirements on publicly traded U.S. corporations and their foreign affiliates, which are intended to prevent the diversion of corporate funds to the payment of bribes and other improper payments. Recent years have seen substantial increase in the global enforcement of anti-corruption laws. Our operations outside the United States could increase the risk of such violations. Our business is also heavily regulated and involves significant interaction with foreign officials. In many countries outside the U.S., independent clinical investigators conducting our clinical trials and prescribers of our products are employed by government entities, and purchasers themselves can be government entities. As such, our interactions with such investigators, prescribers and purchasers may be subject to regulation under the FCPA, as well as other similar under anti-corruption laws and/or regulations enacted by other countries. Failure to comply with these laws, where applicable, can result in significant penalties, including the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgement, imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal and equivalent foreign healthcare programs, and additional reporting requirements and regulatory oversight, any of which could adversely affect our ability to operate our business and our results of operations.

**** International trade policies, including tariffs, sanctions and trade barriers may adversely affect our business, financial condition, results of operations and prospects.***

We operate in a global economy, and our business depends on a global supply chain for the development, manufacturing, and distribution of our products, and for the advancement of our development programs. There is inherent risk, based on the complex relationships among the U.S. and the countries in which we conduct our business, that political, diplomatic, and national security factors can lead to global trade restrictions and changes in trade policies and export regulations that may adversely affect our business and operations.

We source and procure APIs, precursor chemicals, and specialized equipment from international suppliers, with substantial reliance on foreign contract manufacturers in Europe. Tariff policies, particularly those affecting pharmaceutical products, could increase our costs and reduce our profitability. Additionally, recent policy discussions have included potential targeted tariffs or other trade measures specifically aimed at pharmaceutical products and ingredients as part of broader healthcare cost control or national security initiatives. In April 2025, the U.S. Department of Commerce initiated an investigation on imports of pharmaceuticals and pharmaceutical ingredients, which may result in the current U.S. Presidential administration taking actions to impose tariffs on the pharmaceutical industry. The U.S. Presidential administration also recently indicated that it may impose a 100% tariff on any branded or patented pharmaceutical product, unless a company is building their pharmaceutical manufacturing plant in the U.S. The specific impact of the investigation and announcements to enact substantial tariffs on patented pharmaceutical products remain uncertain at this time but could negatively impact our business and operations. Unlike consumer goods, pharmaceuticals face unique regulatory constraints that make rapid supply chain adjustments particularly difficult and costly. Should tariffs be imposed specifically targeting pharmaceutical imports, our production costs could rise, and it would be difficult and costly to qualify alternative sources within another country with a lower tariff rate or within the United States, as developing and qualifying alternative sources typically requires substantial time, investment, and regulatory approvals.

Unlike many industries, our ability to pass increased costs to customers is limited by the structure of pharmaceutical pricing and reimbursement systems. As a result, cost increases due to tariffs may be difficult or impossible to pass through to customers.

Current or future tariffs will also result in increased research and development expenses, including with respect to increased costs associated with APIs, raw materials, laboratory equipment and research materials and components. Trade restrictions affecting the import of materials necessary for clinical trials could result in delays to our development timelines. Increased development costs and extended development timelines could place us at a competitive disadvantage compared to companies operating in regions with more favorable trade relationships and could reduce investor confidence and negatively impact our business, results of operations, financial condition and growth prospects.

Trade disputes, tariffs, restrictions and other political tensions between the United States and other countries may also exacerbate unfavorable macroeconomic conditions including inflationary pressures, foreign exchange volatility, financial market instability, and economic recessions or downturns. The ultimate impact of current or future tariffs and trade restrictions remains uncertain and could materially and adversely affect our business, financial condition, and prospects. While we actively monitor these risks, any prolonged economic downturn, escalation in trade tensions, or deterioration in international perception of U.S.-based companies could materially and adversely affect our business, ability to access the capital markets or other financing sources, results of operations, financial condition and prospects. In addition, tariffs and other trade developments have and may continue to heighten the risks related to the other risk factors described elsewhere in this report.

We could face liability if a regulatory authority determines that we are promoting INGREZZA, CRENESSITY, or any of our product candidates that receives regulatory approval, for "off-label" uses.

A company may not promote "off-label" uses for its drug products. An off-label use is the use of a product for an indication that is not described in the product's FDA-approved label in the U.S. or for uses in other jurisdictions that differ from those approved by the applicable regulatory agencies. Physicians, on the other hand, may prescribe products for off-label uses. Although the FDA and other regulatory agencies do not regulate a physician's choice of drug treatment made in the physician's independent medical judgment, they do restrict promotional communications from companies or their sales force with respect to off-label uses of products for which marketing clearance has not been issued. However, companies may share truthful and not misleading information that is otherwise consistent with a product's FDA approved labeling. A company that is found to have promoted off-label use of its product may be subject to significant liability, including civil and criminal sanctions.

If the FDA or any other governmental agency, including equivalent foreign authorities, initiates an enforcement action against us, or if we are the subject of a *qui tam* suit brought by a private plaintiff on behalf of the government, and it is determined that we violated prohibitions relating to the promotion of products for unapproved uses, we could be subject to substantial civil or criminal fines or damage awards and other sanctions such as consent decrees and corporate integrity agreements pursuant to which our activities would be subject to ongoing scrutiny and monitoring to ensure compliance with applicable laws and regulations. Any such fines, awards or other sanctions would have an adverse effect on our revenue, business, financial prospects and reputation.

**** If our information technology systems, those third parties upon which we rely, or our data is or were compromised, we could experience adverse impacts resulting from such compromise, including, but not limited to, interruptions to our operations such as our clinical trials, claims that we breached our data protection obligations, harm to our reputation, regulatory investigations or actions, litigation, fines and penalties, and a loss of customers or sales.***

We are increasingly dependent on information technology systems and infrastructure, including mobile technologies and technology systems and infrastructure of third parties upon whom we rely, including CROs and other vendors, to operate our business. In the ordinary course of our business, we and the third parties upon which we rely, collect, receive, store, process, generate, disclose, make accessible, protect, dispose of, transmit, use, safeguard, share and transfer, or collectively, process, confidential and sensitive electronic information on our networks and in our data centers. This information includes, among other things, de-identified or pseudonymous sensitive personal data (including health data), our intellectual property and proprietary information, the confidential information of our collaborators and licensees, and the personal data of our employees. It is important to our operations and business strategy that this electronic information remains secure and is perceived to be secure.

The size and complexity of our information technology systems, and those of third-party vendors with whom we contract, and the volume of data we retain, make such systems potentially vulnerable to a variety of evolving threats, including but not limited to social-engineering attacks (including through deep fakes, which may be increasingly more difficult to identify as fake, and phishing attacks), malicious code, malware (such as malicious code, adware, and command and control (C2)), denial-of-service attacks, credential harvesting, personnel misconduct or error, ransomware attacks, supply-chain attacks, software bugs, server malfunctions, software or hardware failures, loss of data or other information technology assets, attacks enhanced or facilitated by AI, telecommunications failures, and other similar threats. In addition, AI has and will continue to make existing threats more sophisticated and difficult to detect, increase the volume of threats, and generate new threats.

Cyber-attacks, malicious internet-based activity, online and offline fraud, and other similar activities threaten the confidentiality, integrity, and availability of our sensitive information and information technology systems, and those of the third parties upon which we rely. Such threats continue to rise, are increasingly difficult to detect, and come from a variety of sources, including traditional computer "hackers," threat actors, "hacktivists," organized criminal threat actors, personnel (such as through theft or misuse), sophisticated nation states, and nation-state-supported actors (also referred to as APTs). Some actors now engage and are expected to continue to engage in cyber-attacks, including without limitation nation-state actors for geopolitical reasons and in conjunction with military conflicts and defense activities. During times of war and other major conflicts, we and the third parties upon which we rely may be vulnerable to a heightened risk of these attacks, including retaliatory cyber-attacks, which could materially disrupt our systems and operations, as well as our ability to conduct clinical trials.

Ransomware attacks are also becoming increasingly prevalent and severe, and can lead to significant interruptions in our operations (including our ability to conduct clinical trials), loss of sensitive data (including related to our clinical trials) and income, reputational harm, and diversion of funds. To alleviate the financial, operational and reputational impact of a ransomware attack, it may be preferable to make extortion payments, but we may be unwilling or unable to do so (including, for example, if applicable laws or regulations prohibit such payments). Similarly, supply chain attacks have increased in frequency and severity, and we cannot guarantee that third parties in our supply chain have not been compromised or that they do not contain exploitable defects, vulnerabilities, or bugs that could result in a breach of or disruption to our information technology systems and infrastructure or the information technology systems and infrastructure of third parties that support our operations.

Remote work has increased risks to our information technology systems and data, as certain of our employees work from home, utilizing network connections, computers and devices outside our premises, including at home, while in transit or in public locations.

Additionally, natural disasters, public health pandemics or epidemics, terrorism, war and geopolitical conflicts, and telecommunication and electrical failures may result in damage to or the interruption or impairment of key business processes, or the loss or corruption of confidential information, including intellectual property, proprietary business information and personal data.

Future or past business transactions (such as acquisitions or integrations) could expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. Furthermore, we may discover security issues that were not found during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

As cyber threats continue to evolve, we may be required to expend significant additional resources to continue to modify or enhance our protective measures or to investigate and remediate any information security vulnerabilities or modify our business activities (including our clinical trial activities) to try to protect against security incidents.

We take steps designed to detect, mitigate, and remediate vulnerabilities in our information security systems (such as our hardware and/or software, including that of third parties upon which we rely). We may not, however, detect and remediate all such vulnerabilities including on a timely basis. Further, we may experience delays in developing and deploying remedial measures and patches designed to address identified vulnerabilities. Vulnerabilities could be exploited and result in a security incident.

We rely on third-party service providers and technologies to operate critical business systems to process sensitive information in a variety of contexts, including, without limitation, cloud-based infrastructure, data center facilities, encryption and authentication technology, employee email and other functions. We also rely on third-party service providers to provide other products, services, parts, or otherwise to operate our business, including clinical trial sites and investigators, contractors, manufacturers, suppliers and consultants. Our ability to monitor these third parties' information security practices is limited, and these third parties may not have adequate information security measures in place. If our third-party service providers or CROs experience a security incident or other interruption, we could experience adverse consequences. In addition, supply-chain attacks have increased in frequency and severity, and we cannot guarantee that third parties' infrastructure in our supply chain or our third-party partners' supply chains have not been compromised or otherwise subject to a security incident. While we may be entitled to damages if our third-party service providers fail to satisfy their privacy or security-related obligations to us, any award may be insufficient to cover our damages, or we may be unable to recover such award.

Although to our knowledge we, or the third parties upon who we rely, have not experienced a security incident or disruption to date that is material to us, we and our vendors have been, either directly or indirectly, the target of cybersecurity incidents and expect them to continue. While we have implemented security measures designed to protect our data security and information technology systems, such measures may not prevent such events. Furthermore, while we have implemented certain redundancies designed to avoid interruptions to our operations, not all potential events can be anticipated and interruptions to our operations could lead to decreased productivity.

If we (or a third party upon whom we rely) experience a security incident, ransomware attack or are perceived to have experienced a security incident, we may experience material adverse consequences. Such material consequences may include: government enforcement actions (for example, investigations, fines, penalties, audits and inspections); additional reporting requirements and/or oversight; restrictions on processing sensitive information (including personal data); litigation (including class claims); indemnification obligations; negative publicity; reputational harm (including but not limited to damage to our patient, partner, or employee relationships); monetary fund diversions; diversion of management's attention; interruptions in our operations (including availability of data, loss of connectivity to our network or internet); financial loss (including decreased productivity resulting from interruptions in our operations); and other similar harms. Similarly, the loss of clinical trial data from completed or ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. In addition, theft of our intellectual property or proprietary business information could require substantial expenditures to remedy. Applicable data privacy and security obligations may also require us to notify relevant stakeholders, including affected individuals, customers, regulators, and investors, of security incidents. Such disclosures are costly, and the disclosure or the failure to comply with such requirements could lead to adverse consequences.

Our contracts, with for example third parties or CROs, may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our data privacy and security obligations. We also cannot be sure that our insurance coverage will be adequate or sufficient to protect us from or to mitigate liabilities arising out of our privacy and security practices, that such coverage will continue to be available on commercially reasonable terms or at all, or that such coverage will pay future claims.

In addition to experiencing a security incident, third parties may gather, collect, or infer sensitive information about us from public sources, data brokers, or other means that reveals competitively sensitive details about our organization and could be used to undermine our competitive advantage or market position. Additionally, our sensitive information could be leaked, disclosed, or revealed as a result of or in connection with our employees', personnel's, or vendors' potential use of generative AI technologies.

**** Unfavorable geopolitical and macroeconomic developments could adversely affect our business, financial condition or results of operations.***

Our business could be adversely affected by conditions in the U.S. and global economies, the U.S. and global financial markets and adverse geopolitical and macroeconomic developments, including potential future disruptions in access to bank deposits due to bank failures, tariffs and trade barriers, the ongoing shutdown of the U.S. federal government and the resulting effects on its regulatory agencies, geopolitical tensions, and military conflicts. General business and economic conditions that could affect our business, financial condition or results of operations include fluctuations in economic growth, inflation and interest rates, debt and equity capital markets, liquidity of the global financial markets, the availability and cost of credit, investor and consumer confidence, and the strength of the economies in which we, our manufacturers and suppliers, and our collaborators operate. A weak or declining global economy due to geopolitical tensions or tariffs and trade barriers could also strain our suppliers and manufacturers, possibly resulting in supply disruption. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

If we fail to obtain or maintain orphan drug designation or other regulatory exclusivity for some of our product candidates, our competitive position would be harmed.

In addition to any patent protection, we rely on forms of regulatory exclusivity to protect our products such as orphan drug designation. A product candidate that receives orphan drug designation can benefit from a streamlined regulatory process as well as potential commercial benefits following approval. Currently, this designation provides market exclusivity in the U.S. for seven years and EU for 10 years if a product is the first such product approved for such orphan indication. This market exclusivity does not, however, pertain to indications other than those for which the drug was specifically designated in the approval, nor does it prevent other types of drugs from receiving orphan designations or approvals in these same indications. Further, even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the product is clinically superior to the orphan product or a market shortage occurs.

In the EU, orphan exclusivity may be reduced to six years if the drug no longer satisfies the original designation criteria or can be lost altogether if the marketing authorization holder consents to a second orphan drug application or cannot supply enough drug, or when a second applicant demonstrates its drug is “clinically superior” to the original orphan drug.

If we do not have adequate patent protection for our products, then the relative importance of obtaining regulatory exclusivity is even greater. We may not be successful obtaining orphan drug designations for any indications and, even if we succeed, such product candidates with such orphan drug designations may fail to achieve FDA approval. Even if a product candidate with orphan drug designation may receive marketing approval from the FDA, it may fail to result in or maintain orphan drug exclusivity upon approval, which would harm our competitive position.

The technologies we use in our research as well as the drug targets we select may infringe the patents or violate the proprietary rights of third parties.

We cannot assure you that third parties will not assert patent or other intellectual property infringement claims against us or our collaborators with respect to technologies used in potential products. If a patent infringement suit were brought against us or our collaborators, we or our collaborators could be forced to stop or delay developing, manufacturing or selling potential products that are claimed to infringe a third party’s intellectual property unless that party grants us or our collaborators rights to use its intellectual property. In such cases, we could be required to obtain licenses to patents or proprietary rights of others in order to continue to commercialize our products. However, we may not be able to obtain any licenses required under any patents or proprietary rights of third parties on acceptable terms, or at all. Even if our collaborators or we were able to obtain rights to the third party’s intellectual property, these rights may be non-exclusive, thereby giving our competitors access to the same intellectual property. Ultimately, we may be unable to commercialize some of our potential products or may have to cease some of our business operations as a result of patent infringement claims, which could severely harm our business.

Our business operations may subject us to disputes, claims and lawsuits, which may be costly and time-consuming and could materially and adversely impact our financial position and results of operations.

From time to time, we may become involved in disputes, claims and lawsuits relating to our business operations. In particular, we may face claims related to the safety of our products, intellectual property matters, employment matters, tax matters, commercial disputes, competition, sales and marketing practices, environmental matters, personal injury, insurance coverage and acquisition or divestiture-related matters. Any dispute, claim or lawsuit may divert management’s attention away from our business, we may incur significant expenses in addressing or defending any dispute, claim or lawsuit, and we may be required to pay damage awards or settlements or become subject to equitable remedies that could adversely affect our operations and financial results.

Litigation related to these disputes may be costly and time-consuming and could materially and adversely impact our financial position and results of operations if resolved against us. In addition, the uncertainty associated with litigation could lead to increased volatility in our stock price.

Our employees, independent contractors, principal investigators, consultants, commercial partners and vendors may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements.

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees and independent contractors, such as principal investigators, consultants, commercial partners and vendors, or by employees of our commercial partners could include failures to comply with FDA regulations, to provide accurate information to the FDA, to comply with manufacturing standards we have established, to comply with federal and state healthcare fraud and abuse laws, to report financial information or data accurately, to maintain the confidentiality of our trade secrets or the trade secrets of our commercial partners, or to disclose unauthorized activities to us. In particular, sales, marketing and other business arrangements in the healthcare industry are subject to extensive laws intended to prevent fraud, kickbacks, self-dealing and other abusive practices. Employee and independent contractor misconduct could also involve the improper use of individually identifiable information, including, without limitation, information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. Any action against our employees, independent contractors, principal investigators, consultants, commercial partners or vendors for violations of these laws could result in significant civil, criminal and administrative penalties, fines and imprisonment.

We face potential product liability exposure far in excess of our insurance coverage.

The use of any of our potential products in clinical trials, and the sale of any approved products, including INGREZZA and CRENESSITY, may expose us to liability claims. These claims might be made directly by consumers, healthcare providers, pharmaceutical companies or others selling our products. We have product liability insurance coverage for both our clinical trials as well as related to the sale of INGREZZA and CRENESSITY in amounts consistent with customary industry practices. However, our insurance may not reimburse us or may not be sufficient to reimburse us for any expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive, and we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability from any current or future clinical trials or approved products. A successful product liability claim, or series of claims, brought against us would decrease our cash reserves and could cause our stock price to fall. Furthermore, regardless of the eventual outcome of a product liability claim, any product liability claim against us may decrease demand for our approved products, including INGREZZA and CRENESSITY, damage our reputation, result in regulatory investigations that could require costly recalls or product modifications, cause clinical trial participants to withdrawal, result in costs to defend the related litigation, decrease our revenue, and divert management's attention from managing our business.

Our activities involve hazardous materials, and we may be liable for any resulting contamination or injuries.

Our research activities involve the controlled use of hazardous materials. We cannot eliminate the risk of accidental contamination or injury from these materials. If an accident occurs, a court may hold us liable for any resulting damages, which may harm our results of operations and cause us to use a substantial portion of our cash reserves, which would force us to seek additional financing.

**** We are subject to stringent and changing obligations related to data privacy and information security. Our actual or perceived failure to comply with such obligations could have a material adverse effect on our reputation, business, financial condition or results of operations.***

In the ordinary course of our business, we process confidential and sensitive information, including personal data, proprietary and confidential business data, trade secrets, intellectual property, data we collect about clinical trial participants in connection with clinical trials, and sensitive third-party data, on our networks and in our data centers. We are subject to numerous federal, state, local and foreign laws, orders, codes, regulations and regulatory guidance regarding privacy, data protection, information security and the processing of personal information (including clinical trial data), the number and scope of which are expanding, changing, subject to differing applications and interpretations, and may be inconsistent among jurisdictions. Our data processing activities may also subject us to other data privacy and security obligations, such as industry standards, external and internal privacy and security policies, contracts and other obligations that govern the processing of data by us and by third parties on our behalf.

Laws regarding privacy, data protection, information security and the processing of personal data are becoming increasingly common in the U.S. at both the federal and state level. Additionally, in the past few years, numerous U.S. states—including California, Virginia, Colorado, Connecticut, and Utah—have enacted comprehensive privacy laws that impose certain obligations on covered businesses, including providing specific disclosures in privacy notices and affording residents with certain rights concerning their personal data. As applicable, such rights may include the right to access, correct, or delete certain personal data, and to opt-out of certain data processing activities, such as targeted advertising, profiling, and automated decision-making. The exercise of these rights may impact our business and ability to provide our products and services. Certain states also impose stricter requirements for processing certain personal data, including sensitive information, such as conducting data privacy impact assessments. These state laws allow for statutory fines for noncompliance. For example, the California Consumer Privacy Act, as amended by the California Privacy Rights Act of 2020 (collectively, CCPA), requires businesses to provide specific disclosures in privacy notices, and honor requests of California residents to exercise certain privacy rights. The CCPA allows for fines for noncompliance (up to $7,500 per intentional violation). Although some U.S. comprehensive privacy laws and the CCPA exempt some data processed in the context of clinical trials, these laws may increase compliance costs and potential liability with respect to other personal data we may maintain about California residents. Other states have also enacted data privacy laws and we expect more jurisdictions to pass similar laws in the future. These developments may further complicate compliance efforts, and may increase legal risk and compliance costs for us and the third parties upon whom we rely.

Additionally, HIPAA, as amended by HITECH, imposes specific requirements relating to the privacy, security, and transmission of individually identifiable health information.

Laws in Europe regarding privacy, data protection, information security and the processing of personal data have also been significantly reformed and continue to undergo reform. For example, the EU's General Data Protection Regulation (EU GDPR) and the UK's GDPR (UK GDPR) (collectively, GDPR) impose strict requirements for processing the personal data of individuals located, respectively, within the European Economic Area (EEA) and the UK, and the Swiss Federal Act on Data Protection similarly applies to the collection and processing of personal data, including health-related information, in Switzerland. The GDPR provides for enhanced data protection obligations for processors and controllers of personal data, including, for example, obligations relating to: processing health and other sensitive data; obtaining consent of individuals; providing notice to individuals regarding data processing activities; responding to data subject requests; taking certain measures when engaging third-party processors; notifying data subjects and regulators of data breaches; and implementing safeguards to protect the security and confidentiality of personal data. The GDPR impose substantial fines for breaches of data protection requirements. For example, under the GDPR, such fines can be up to four percent of global revenue or 20 million euros under the EU GDPR / 17.5 million pounds sterling under the UK GDPR, whichever is greater in either case, and also allow for private litigation related to processing of personal data brought by classes of data subjects or consumer protection organizations authorized at law to represent their interests. The GDPR and other changes in laws or regulations associated with the enhanced protection of certain types of sensitive data, such as EU regulations governing clinical trial data and other healthcare data, could require us to change our business practices or lead to government enforcement actions, private litigation or significant penalties against us and could have a material adverse effect on our business, financial condition or results of operations.

We may be subject to additional foreign data laws. For example, in Canada, the Personal Information Protection and Electronic Documents Act (PIPEDA) and various related provincial laws, as well as Canada's Anti-Spam Legislation (CASL), may apply to our operations. As another example, the General Data Protection Law, Lei Geral de Proteção de Dados Pessoais (LGPD) (Law No. 13,709/2018), may apply to our operations. The LGPD broadly regulates processing personal data of individuals in Brazil and imposes compliance obligations and penalties comparable to those of the EU GDPR. We also target customers in Asia and may be subject to new and emerging data privacy regimes in Asia, including Japan's Act on the Protection of Personal Information and Singapore's Personal Data Protection Act.

In the ordinary course of business, we may transfer personal data from Europe and other jurisdictions to the U.S. or other countries. Certain jurisdictions have enacted data localization laws and cross-border personal data transfers laws. For example, countries in the EEA and the UK have significantly restricted the transfer of personal data to the U.S. and other countries, whose privacy laws it generally believes are inadequate. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and UK to the U.S. in compliance with law, such as the EEA standard contractual clauses, the UK's International Data Transfer Agreement / Addendum, and the EU-U.S. Data Privacy Framework and the UK extension thereto (which allows for transfers for to relevant U.S.-based organizations who self-certify compliance and participate in the Framework), these mechanisms are subject to legal challenges, and there is no assurance that we can satisfy or rely on these measures to lawfully transfer personal data to the U.S. If we cannot implement a valid compliance mechanism for cross-border personal data transfers or if the requirements for a legally-compliant transfer are too onerous, we

may face increased exposure to regulatory actions, substantial fines and injunctions against processing or transferring personal data from Europe or elsewhere. The inability to import personal data to the U.S. may significantly and negatively impact our business operations, including by limiting our ability to conduct clinical trial activities in Europe and elsewhere; limiting our ability to collaborate with parties subject to European and other data protection laws or requiring us to increase our personal data processing capabilities in Europe and/or elsewhere at significant expense. Other jurisdictions may adopt or have already adopted similarly stringent interpretations of their data localization and cross-border data transfer laws. Additionally, companies that transfer personal data out of the EEA and UK to other jurisdictions, particularly to the U.S., are subject to increased scrutiny from regulators, individual litigants, and activist groups. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers out of Europe for allegedly violating the GDPR's cross-border data transfer limitations. Regulators in the U.S., such as the Department of Justice, are also increasingly scrutinizing certain personal data transfers and have proposed and may enact certain data export restrictions and localization requirements. For example, the Preventing Access to U.S. Sensitive Personal Data and Government-Related Data by Countries of Concern or Covered Persons rule finalized by the Department of Justice in late 2024, enacting the Biden administration's executive order Preventing Access to Americans' Bulk Sensitive Personal Data and United States Government-Related Data by Countries of Concern went into effect in April 2025.

Our employees and personnel are permitted to use generative AI technologies to perform some of their work, and the disclosure and use of personal information data in generative AI technologies is subject to various privacy laws and other privacy obligations. Governments have passed and are likely to pass additional laws regulating generative AI. Our use of this technology could result in additional compliance costs, regulatory investigations and actions, and consumer lawsuits. Furthermore, any use of generative AI to develop our proprietary technology and compounds may also impact our ability to obtain or successfully defend certain intellectual property rights. If we are unable to use generative AI, it could make our business less efficient and result in competitive disadvantages.

In addition to data privacy and security laws, we may contractually be subject to industry standards adopted by industry groups and, we are, or may become subject to such obligations in the future. We are also bound by contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. We publish privacy policies, marketing materials and other statements regarding data privacy and security. Regulators in the U.S. are increasingly scrutinizing these statements, and if these policies, materials or statements are found to be deficient, lacking in transparency, deceptive, unfair, misleading, or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators or other adverse consequences.

Our obligations related to data privacy and security (and consumers' data privacy expectations) are quickly changing in an increasingly stringent fashion and creating uncertainty. These obligations may be subject to differing applications and interpretations, which may be inconsistent among jurisdictions or in conflict. Preparing for and complying with these obligations requires us to devote significant resources (including, without limitation, financial and time-related resources). These obligations may necessitate changes to our information technologies, systems and practices and those of any third parties that process personal data on our behalf. In addition, these obligations may even require us to change our business model.

Although we endeavor to comply with all applicable data privacy and security obligations, we may at times fail (or be perceived to have failed) to do so. Moreover, despite our efforts, our personnel or third-parties upon whom we rely may fail to comply such obligations that impacts our compliance posture. If we fail, or are perceived to have failed, to address or comply with data privacy and security obligations, we could face significant consequences. These consequences may include, but are not limited to, government enforcement actions, litigation (including class claims), additional reporting requirements and/or oversight, bans on processing personal data, imprisonment of company officials, and orders to destroy or not use personal data. In particular, plaintiffs have become increasingly more active in bringing privacy-related claims against companies, including class claims and mass arbitration demands. Some of these claims allow for the recovery of statutory damages on a per violation basis, and, if viable, carry the potential for monumental statutory damages, depending on the volume of data and the number of violations. Any of these events could have a material adverse effect on our reputation, business, financial condition or results of operations.

Item 5. Other Information

During the period from July 1, 2025 to September 30, 2025, our officers (as defined in Rule 16a-1(f) under the Exchange Act) and/or directors adopted or terminated contracts, instructions or written plans for the purchase or sale of our securities as noted below:

Name and Title	Action	Date	Trading Arrangement		Total Shares Authorized to be Sold***	Expiration Date
			Rule 10b5-1*	Non-Rule 10b5-1**		
William Rastetter Director	Adoption	8/6/2025	X		18,000	5/19/2026
George Morrow Director	Adoption	8/26/2025	X		15,000	5/19/2026
Eric Benevich Chief Commercial Officer	Adoption	8/28/2025	X		12,830	2/4/2026
Gary Lyons Director	Adoption	9/3/2025	X		15,000	5/19/2026
Leslie Norwalk Director	Adoption	9/12/2025	X		15,000	9/12/2026

* Intended to satisfy the affirmative defense of Rule 10b5-1(c)

** Not intended to satisfy the affirmative defense of Rule 10b5-1(c)

*** Represents the maximum number of shares that may be sold pursuant to the 10b5-1 arrangement. The number of shares sold is dependent on the satisfaction of certain conditions as set forth in the written plan and the satisfaction of applicable vesting conditions of equity awards.

In addition, our executive officers have entered into sell-to-cover arrangements adopted pursuant to Rule 10b5-1 authorizing the pre-arranged sale of shares to satisfy tax withholding obligations of the Company arising exclusively from the vesting of time-vesting or performance-vesting restricted stock units and the related issuance of shares. The amount of shares to be sold to satisfy the Company's tax withholding obligations under these arrangements is dependent on future events which cannot be known at this time, including the future trading price of Company shares. The expiration date relating to these arrangements is dependent on future events which cannot be known at this time, including the final vest date of the applicable time-vesting or performance-vesting restricted stock units and the officer's termination of service.

Item 6. Exhibits

The following exhibits are filed as part of, or incorporated by reference into, this report:

Exhibit		
3.1	Description:	Certificate of Incorporation, as amended
	Reference:	Incorporated by reference to Exhibit 3.1 of the Company's Quarterly Report on Form 10-Q filed on November 5, 2018
3.2	Description:	Bylaws, as amended
	Reference:	Incorporated by reference to Exhibit 3.2 of the Company's Quarterly Report on Form 10-Q filed on October 30, 2024
4.1	Description:	Form of Common Stock Certificate
	Reference:	Incorporated by reference to the Company's Registration Statement on Form S-1 (Registration No. 333-03172)
31.1	Description:	Certification of Chief Executive Officer pursuant to Rules 13a-14 and 15d-14 promulgated under the Securities Exchange Act of 1934
31.2	Description:	Certification of Chief Financial Officer pursuant to Rules 13a-14 and 15d-14 promulgated under the Securities Exchange Act of 1934
32*	Description:	Certifications of Chief Executive Officer and Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	Description:	Inline XBRL Instance Document. – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.
101.SCH	Description:	Inline XBRL Taxonomy Extension Schema Document.
101.CAL	Description:	Inline XBRL Taxonomy Extension Calculation Linkbase Document.
101.DEF	Description:	Inline XBRL Taxonomy Extension Definition Linkbase Document.
101.LAB	Description:	Inline XBRL Taxonomy Extension Label Linkbase Document.
101.PRE	Description:	Inline XBRL Taxonomy Extension Presentation Linkbase Document.
104	Description:	Cover Page Interactive Data File (formatted as Inline XBRL with applicable taxonomy extension information contained in Exhibit 101)

* These certifications are being furnished solely to accompany this quarterly report pursuant to 18 U.S.C. Section 1350, and are not being filed for purposes of Section 18 of the Securities Exchange Act of 1934 and are not to be incorporated by reference into any filing of Neurocrine Biosciences, Inc., whether made before or after the date hereof, regardless of any general incorporation language in such filing.

Except as specifically noted above, the Company's Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, and Current Reports on Form 8-K have a Commission File Number of 000-22705.

SIGNATURES

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 thereunto duly authorized.

NEUROCRINE BIOSCIENCES, INC.

Dated: October 28, 2025

/s/ Matthew C. Abernethy

Matthew C. Abernethy
Chief Financial Officer
(Duly authorized officer and Principal Financial Officer)

Exhibit 31.1

CERTIFICATION OF CHIEF EXECUTIVE OFFICER PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Kyle W. Gano, Chief Executive Officer of Neurocrine Biosciences, Inc., certify that:

1. I have reviewed this quarterly report on Form 10-Q of Neurocrine Biosciences, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)), for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Dated: October 28, 2025

/s/ Kyle W. Gano

Kyle W. Gano

Chief Executive Officer

Exhibit 31.2

CERTIFICATION OF CHIEF FINANCIAL OFFICER PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Matthew C. Abernethy, Chief Financial Officer of Neurocrine Biosciences, Inc., certify that:

1. I have reviewed this quarterly report on Form 10-Q of Neurocrine Biosciences, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)), for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Dated: October 28, 2025

/s/ Matthew C. Abernethy

Matthew C. Abernethy
Chief Financial Officer

Exhibit 32

CERTIFICATIONS OF
CHIEF EXECUTIVE OFFICER AND CHIEF FINANCIAL OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report of Neurocrine Biosciences, Inc. (Company) on Form 10-Q for the period ended September 30, 2025 as filed with the Securities and Exchange Commission on the date hereof (Report), I, Kyle W. Gano, Chief Executive Officer of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to my knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d), of the Securities Exchange Act of 1934; and

(2) That information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

October 28, 2025

By: /s/ Kyle W. Gano

Name: Kyle W. Gano
Title: Chief Executive Officer

In connection with the Quarterly Report of Neurocrine Biosciences, Inc. (Company) on Form 10-Q for the period ended September 30, 2025 as filed with the Securities and Exchange Commission on the date hereof (Report), I, Matthew C. Abernethy, Chief Financial Officer of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to my knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d), of the Securities Exchange Act of 1934; and

(2) That information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

October 28, 2025

By: /s/ Matthew C. Abernethy

Name: Matthew C. Abernethy
Title: Chief Financial Officer