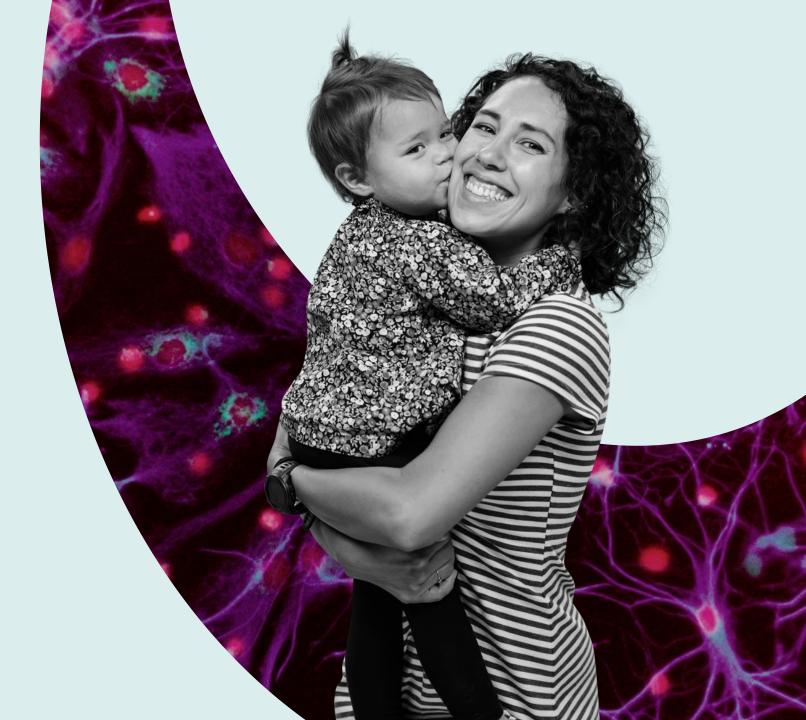
## Advancing Life-Changing Discoveries in Neuroscience

Q2 2024 Corporate Presentation August 1, 2024

Nasdaq: NBIX





### Safe Harbor Statement and Non-GAAP Financial Measures

In addition to historical facts, this presentation contains forward-looking statements that involve a number of risks and uncertainties. These statements include, but are not limited to, statements related to: the benefits to be derived from our products and product candidates; the value our products and/or our product candidates may bring to patients; the continued success of INGREZZA; our financial and operating performance, including our future revenues, expenses, or profits; our collaborative partnerships; expected future clinical and regulatory milestones; and the timing of the initiation and/or completion of our clinical, regulatory, and other development activities and those of our collaboration partners. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements are: our future financial and operating performance; risks and uncertainties associated with the commercialization of INGREZZA; risks that the crinecerfont New Drug Applications (NDAs) may not obtain regulatory approval, such approval may be delayed, or may not receive the benefits associated with priority review; risks related to the development of our product candidates; risks associated with our dependence on third parties for development, manufacturing, and commercialization activities for our products and product candidates, and our ability to manage these third parties; risks that the FDA or other regulatory authorities may make adverse decisions regarding our products or product candidates; risks that clinical development activities may not be initiated or completed on time or at all, or may be delayed for regulatory, manufacturing, or other reasons, may not be successful or replicate previous clinical trial results, may fail to demonstrate that our product candidates are safe and effective, or may not be predictive of real-world results or of results in subsequent clinical trials; risks that the potential benefits of the agreements with our collaboration partners may never be realized; risks that our products, and/or our product candidates may be precluded from commercialization by the proprietary or regulatory rights of third parties, or have unintended side effects, adverse reactions or incidents of misuse; risks associated with government and third-party regulatory and/or policy efforts which may, among other things, impose sales and pharmaceutical pricing controls on our products or limit coverage and/or reimbursement for our products; risks associated with competition from other therapies or products, including potential generic entrants for our products; and other risks described in our periodic reports filed with the SEC, including our Quarterly Report on Form 10-Q for the quarter ended June 30, 2024. Neurocrine Biosciences disclaims any obligation to update the statements contained in this presentation after the date hereof other than as required by law.

In addition to the financial results and financial guidance that are provided in accordance with accounting principles generally accepted in the United States (GAAP), this presentation also contains the following Non-GAAP financial measures: Non-GAAP R&D expense, Non-GAAP SG&A expense, and Non-GAAP net income and net income per share. When preparing the Non-GAAP financial results and guidance, the Company excludes certain GAAP items that management does not consider to be normal, including recurring cash operating expenses that might not meet the definition of unusual or non-recurring items. In particular, these Non-GAAP financial measures exclude: non-cash stock-based compensation expense, charges associated with convertible senior notes, impairment charges associated with leased properties, non-cash amortization expense related to acquired intangible assets, acquisition and integration costs, changes in fair value of equity security investments, changes in foreign currency exchange rates and certain adjustments to income tax expense. These Non-GAAP financial measures are provided as a complement to results provided in accordance with GAAP as management believes these Non-GAAP financial measures help indicate underlying trends in the Company's business, are important in comparing current results with prior period results and provide additional information regarding the Company's financial position.

Management also uses these Non-GAAP financial measures to establish budgets and operational goals that are communicated internally and externally and to manage the Company's business and evaluate its performance. The Company provides guidance regarding combined R&D and SG&A expenses on both a GAAP and a Non-GAAP financial results is included in the attached financial information.



## Well-Positioned for Sustained & Long-term Growth

Commercial		R&D Focus	Strong Financial Position				
TARDIVE	AGREZZA® *  albenazine) capsules  E DYSKINESIA AND CHOREA  O WITH HUNTINGTON'S DISEASE  2024 Annual  Net Sales Guidance Raised	Neurology Neuroendocrinology Neuropsychiatry	~\$1.7B  Cash and Investments as of 6/30/2024				
Billion	and Narrowed from \$2.1 - \$2.2 Billion	Robust Pipeline	Strong Balance Sheet				
~600,000	Affected by Tardive Dyskinesia in the U.S.; ~65% are undiagnosed	Multiple Compounds in Mid- to Late-Stage Studies	Durable Cash Flows				
~90%	of the ~40,000 People in the U.S. Diagnosed with Huntington's Disease Who Will Develop Chorea	Rapidly Growing Early- Stage Portfolio	Attractive P&L Profile				



### Where Are We Today?

- Discovered and Developed Three Novel FDA-Approved Programs
- **Deep Expertise** in Neuroscience Drug Development
- Fully-Integrated Organization with Both R&D and Commercial Capabilities
- Growing Blockbuster Commercial Product in INGREZZA with Strong IP Protection
- Future Blockbuster Opportunity with Crinecerfont
- Largest Portfolio of Muscarinic Compounds in Clinical Development
- Strong Financial Profile That Can Support Significant R&D Investment

**Building a Leading Neuroscience-Focused Company** 





### Q2 2024 Highlights and 2024 Key Milestones and Activities

#### Q2 2024 / Recent Highlights

- INGREZZA® (valbenazine) Net Product Sales of \$580M
  - Represents YoY Sales Growth of 32% vs. Q2 2023
  - Growth Driven By Strong Underlying Demand and Improvement in Q2 Gross To Net Dynamics
- Announced Expansion of INGREZZA Psychiatry and LTC Sales
   Teams to Help Accelerate Appropriate Diagnosis and Treatment
- Launched INGREZZA® SPRINKLE (valbenazine) Oral Granules Formulation for Patients Who Have Difficulty Swallowing
- Positive Phase 2 Top-Line Data For NBI-'845 (AMPA Potentiator) in Adults with Major Depressive Disorder (MDD)
- Crinecerfont Granted Priority Review for the Treatment of Pediatric and Adult Patients with Classical CAH
- Presented Adult and Pediatric Phase 3 CAHtalyst<sup>™</sup> Study Results at ENDO With Parallel Publication of Results in the New England Journal of Medicine (NEJM)
- Pipeline Progress Includes Several Recently Initiated Studies:
  - Phase 2 Study of NBI-'770 (Oral NMDA NR2B NAM) for MDD
  - Phase 1 Study of NBI-'567 (M1 Agonist)
  - Phase 1 Study of NBI-'986 (M4 Antagonist)
- Settled Senior Convertible Notes Due in May in Cash

#### **2024 Key Milestones and Activities**

- NBI-'845 End of Phase 2 Meeting with FDA in 2H 2024;
   Engaging with Agency on Registration Path Forward
- On Track to Report Phase 2 Top-Line Data for NBI-'568 (M4 Agonist)
   For the Treatment of Schizophrenia in Q3 2024
  - Anticipate Disclosing Absolute PANNS Scores, Placebo-Adjusted PANNS Scores, Effect Size, Safety and Tolerability Profile
  - Will Disclose via Press Release and Webcast Conference Call
- On Track to Report Phase 2 Top-Line Data for Luvadaxistat (DAAO Inhibitor) for CIAS in Q3 2024
- Advancing Broadest and Most Diverse Muscarinic Portfolio in Industry with NBI-'568 (M4 Agonist) in Phase 2 and Four Ongoing Phase 1 Studies
  - NBI-'567 (M1 Agonist)
  - NBI-'569 (M4 Agonist)
  - NBI-'570 (Dual M1 / M4 Agonist)
- In Collaboration with Nxera (Formerly Sosei Heptares)

- NBI-'986 (M4 Antagonist)
- On October 11, Kevin Gorman Will Retire as Chief Executive Officer and Kyle Gano, Currently Chief Business Development and Strategy Officer, Will Succeed Him in the CEO Role
- Crinecerfont PDUFA Dates Set for December 29, 2024 (Capsule Formulation) and December 30, 2024 (Oral Solution Formulation)



### **Building and Maximizing the Pipeline**

# of Programs by Stage

Phase 1	Phase 2	Phase 3	NDA
5	8	2	2

			DI 4	Dhara	Db 0	NID A	Milastana
			Phase 1	Phase 2	Phase 3	NDA	Milestone
Neurology							
valbenazine*	Dyskinetic Cerebral Palsy	VMAT2 Inhibitor			•		Phase 3 Ongoing
NBI-827104 <sup>2</sup>	EE-CSWS	Ca <sub>v</sub> 3.1, 3.2, 3.3		•			Phase 2 Ongoing
NBI-921352 <sup>3</sup>	SCN8A-DEE	Na <sub>v</sub> 1.6		•			Phase 2 Ongoing
NBI-1076986	Movement Disorders	M4 Antagonist					Phase 1 Ongoing
Neuroendocrinol	ogy						
crinecerfont <sup>4</sup>	CAH: Adults	CRF-R1					PDUFA Dates on
crinecerfont4	CAH: Pediatrics	CRF-R1					12/29 and 12/30
Efmody	Adrenal Insufficiency	GC Receptor			Announced Positive	Phase 2 Results ✓	Next Steps: TBD
Efmody	CAH	GC Receptor			Announced Positive	Phase 2 Results ✓	Next Steps: TBD
Neuropsychiatry							
valbenazine*	ATS	VMAT2 Inhibitor					Phase 3 Ongoing
NBI-1065845 <sup>5</sup>	Inadequate Response-MDD	AMPA Potentiator			Announced Positive	Phase 2 Results V	Engaging with FDA
luvadaxistat <sup>5</sup>	CIAS	DAAO			Amiounced i ositive	Thase 2 Nesults V	Phase 2 Data: Q3"
		_					Phase 2 Data: Q3'
NIDI_11175601	Schizonhrenia	IVIA AGODIST					
NBI-1117568 <sup>1</sup>	Schizophrenia MDD	M4 Agonist					
NBI-1070770 <sup>5</sup>	MDD	NMDA NR2B NAM					Phase 2 Ongoing
NBI-1070770 <sup>5</sup> NBI-1117570 <sup>1</sup>	MDD CNS Indications	NMDA NR2B NAM M1/M4-Dual					Phase 2 Ongoing Phase 1 Ongoing
NBI-1070770 <sup>5</sup>	MDD	NMDA NR2B NAM					Phase 2 Ongoing

<sup>\*</sup> Mitsubishi Tanabe Pharma Corporation (MTPC) has commercialization rights in Japan and other select Asian markets

<sup>†</sup> Nxera Pharma UK Limited (formerly Sosei Heptares) has retained rights in Japan; Neurocrine Biosciences may opt-in to a 50:50 cost and revenue share upon certain development events



In-licensed program =

<sup>(1)</sup> Nxera Pharma (2) Idorsia Ltd (3) Xenon Pharmaceuticals Inc (4) Sanofi (5) Takeda Pharmaceutical Company Ltd Neurocrine Biosciences has global rights unless otherwise noted.

### **Q2 2024 Financial Summary**

#### \$ Millions, Except Non-GAAP Earnings Per Share

Item	Q2 2024	Q2 2023	Highlights / Comments
Revenue - Product Sales, Net - Collaboration Revenue	\$590 \$584 \$6	\$453 \$446 \$6	INGREZZA Sales of \$580M Represents YoY Growth of 32% Driven by Strong Underlying Patient Demand and Improvement in Gross-to-Net Dynamics
Non-GAAP R&D Expense	\$175	\$122	Increase Driven by Expanded / Advancing Portfolio and Includes \$27M for Development Milestones Achieved Under Our Collaborations
Non-GAAP Acquired IPR&D Expense	\$3	\$0	
Non-GAAP SG&A Expense	\$201	\$177	Increase Driven by Incremental Investment in Crinecerfont- Related Headcount and Pre-Launch Activities, and Continued Investment in INGREZZA
Non-GAAP Net Income	\$169	\$126	Increase Driven by Higher INGREZZA Sales Partially Offset by Incremental Operating Expenses
Non-GAAP Earnings per Share, Diluted	\$1.63	\$1.25	Represents YoY Growth of 30%
Cash and Investments (Period End)	\$1,677	\$1,319	Settled Outstanding 2024 Notes Upon Maturity in May 2024 for \$309M in Cash



### YTD 2024 Financial Summary

#### \$ Millions, Except Non-GAAP Earnings Per Share

Item	1H 2024	1H 2023	Highlights / Comments		
Revenue	\$1,106	\$873			
- Product Sales, Net	\$1,093	\$862	INGREZZA Sales of ~\$1.1B Represents YoY Growth of 28% vs. 1H 2023		
- Collaboration Revenue	\$13	\$12			
Non-GAAP R&D Expense	\$318	\$248	Increase Driven by Expanded / Advancing Portfolio and Includes \$33M for Development Milestones Achieved Under Our Collaborations		
Non-GAAP Acquired IPR&D Expense	\$9	\$144	2023 Expense Associated with Voyager Collaboration		
Non-GAAP SG&A Expense	\$416	\$394	Increase Driven by Incremental Investment in Crinecerfont- Related Headcount and Pre-Launch Activities, And Continued Investment in INGREZZA		
Non-GAAP Net Income	\$294	\$76	Increase Driven by Higher INGREZZA Sales Partially Offset by Incremental Operating Expenses and Lower Total Payments for Upfront Fees / Development Milestones Associated in Connection With Our Collaborations		
Non-GAAP Earnings per Share, Diluted	\$2.83	\$0.76 Represents YoY Growth of 272% vs. 1H 2023			
Cash and Investments (Period End)	\$1,677	\$1,319 Settled Outstanding 2024 Notes Upon Maturi for \$309M in Cash			



# Raised 2024 INGREZZA Net Sales Guidance and Updated Expense Guidance

Item (\$ Millions)	2023 Actuals	2024 Previous Guidance Range	2024 Current Guidance Range	Comments
INGREZZA Net Product Sales <sup>1</sup>	<b>ZZA Net Product Sales</b> <sup>1</sup> \$1,836 \$2,100 -		\$2,100 - \$2,200 \$2,250 - \$2,300	
GAAP R&D Expense <sup>2</sup> \$56		\$665 - \$695	\$665 - \$695	
Non-GAAP R&D Expense <sup>3</sup>	\$497	\$600 - \$630	\$600 - \$630	
GAAP and Non-GAAP IPR&D <sup>4</sup>	\$144	\$6	\$9	Updated GAAP and Non- GAAP Guidance Ranges
GAAP SG&A Expense <sup>5</sup>	\$888	\$920 - \$940	\$955 - \$975	
Non-GAAP SG&A Expense <sup>3, 5</sup>	\$757	\$810 - \$830	\$830 - \$850	

- 1. INGREZZA sales guidance reflects expected net product sales of INGREZZA in tardive dyskinesia and chorea associated with Huntington's disease.
- 2. GAAP R&D guidance includes \$33 million of expense for development milestones in connection with our collaborations (Nxera, Voyager and Takeda) achieved or deemed probable to achieve. These milestone expenses are associated with our advancing pre-clinical and clinical pipeline.
- 3. Non-GAAP guidance adjusted to exclude estimated non-cash stock-based compensation expense of approximately \$65 million in R&D and \$110 million in SG&A and \$14 million leased office space impairment charge in SG&A.
- 4. Acquired in-process R&D (IPR&D) is included in guidance once significant collaboration and licensing arrangements have been completed.
- 5. SG&A guidance range reflects expense for ongoing commercial initiatives supporting INGREZZA growth including the announced planned expansion of the psychiatry and long-term care sales teams and pre-launch commercial activities for crinecerfont.



# Corporate Sustainability: "A" Rated at MSCI and Rank in 11<sup>th</sup> Percentile for Biotech at Sustainalytics

Our Purpose: Relieve Suffering for People with Great Needs, but Few Options





Adhere to the highest product quality and safety standards

Comprehensive Quality System that aligns with:

- Good Manufacturing Practices (GMP)
- Good Laboratory Practices (GLP)
- Good Clinical Practices (GCP)



Invest in our people and communities

Industry-leading employee engagement and diversity

- Top decile employee engagement among biopharmaceutical peers
- Gender and racial/ethnic diversity above biotech industry benchmark\*



Minimize our impact on the environment

Improving profitability and yields through green chemistry

- ~30% improvement in yields
- ~65% reduction in waste
- ~65% reduction in water use

\*According to a <u>study</u> by the Biotechnology Innovation Organization Click <u>here</u> to see Neurocrine's 2024 ESG Report







## Our Medicines, Our Patients

### **Multiple Commercial Products**

In the U.S.



TARDIVE DYSKINESIA

CHOREA ASSOCIATED WITH HD



**ENDOMETRIOSIS** 



**UTERINE FIBROIDS** 

#### In the U.S. and Europe



hydrocortisone granules in capsules for opening

ADRENAL INSUFFICIENCY

#### In Europe



Hydrocortisone modifiedrelease hard capsules

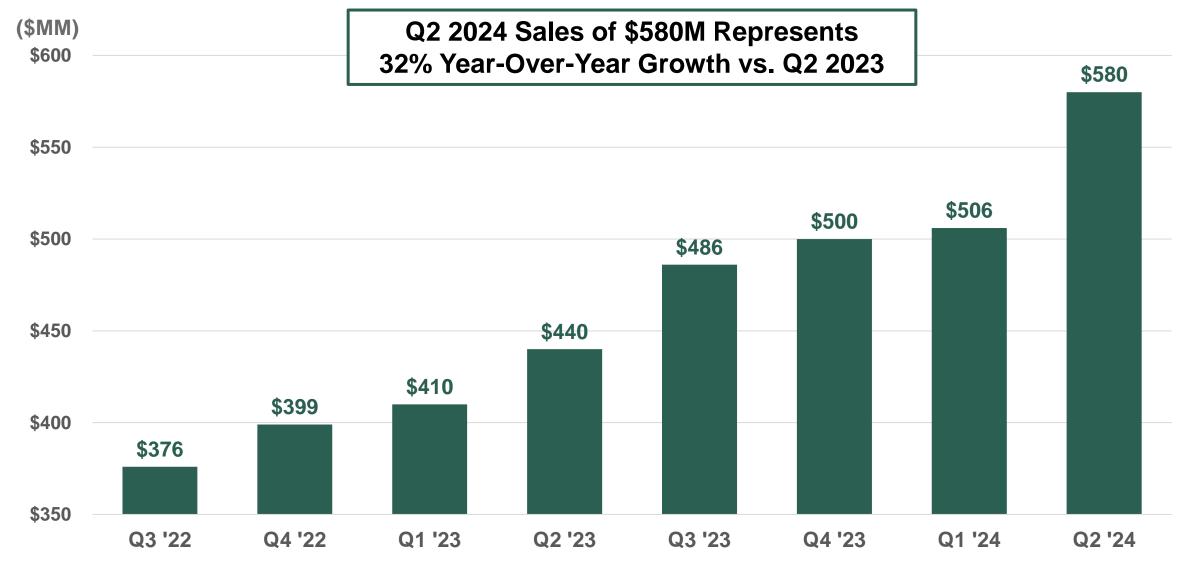
CONGENITAL ADRENAL HYPERPLASIA







### **INGREZZA Quarterly Net Sales Performance**

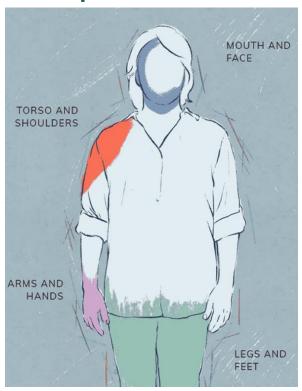




### **Substantial Impact on TD Patients and Care Partners**

Movement disorder caused by prolonged use of antipsychotics and anti-nausea medications

## Uncontrollable, abnormal and repetitive movements





>50%

of patients experience meaningful emotional, social and psychological impact\*

#### **Job Performance**

Patients believe TD affects their ability to perform their job

#### Low Self-Worth

Psychiatric patients may already have difficulty gaining stability and social acceptance

#### **Isolation**

Loss of physical control may make patients more likely to withdraw from social situations

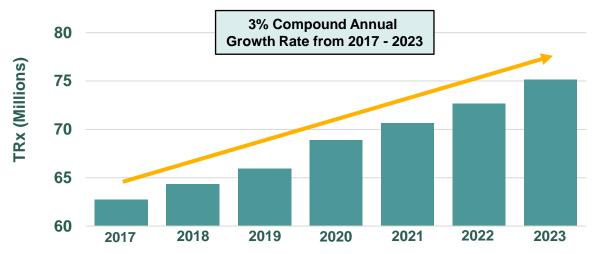


<sup>\* &</sup>lt;a href="https://www.takeontd.com/">https://www.takeontd.com/</a> Source: IQVIA's SMART Audit, Quarterly Data for Antipsychotic Class

## Nascent Tardive Dyskinesia Market Presents Significant Opportunity



#### **Increasing Antipsychotic Prescriptions (U.S.)**

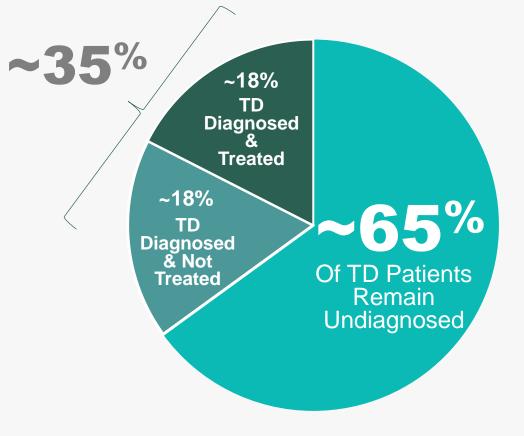


#### NEUROCRINE® BIOSCIENCES

Sources: Neurocrine Biosciences Quarterly Data, IQVIA SMART VMAT2 = Vesicular Monoamine Transporter 2

## Approximately 65% of TD Patients Remain Undiagnosed

✓ Only half of diagnosed patients receive treatment with a VMAT2 inhibitor like INGREZZA



# INGREZZA® Approved by the FDA for the Treatment of Chorea Associated with Huntington's Disease

#### **INGREZZA**

Simple once-a-day treatment targeted for symptom control of chorea movements

Safety profile consistent with and supported by extensive safety data in tardive dyskinesia

In randomized, double-blind, placebo-controlled KINECT-HD study, treatment with valbenazine resulted in a placebo-adjusted mean reduction in the TMC\* score of 3.2 units (p < 0.0001)

Chorea affects
~90% of the 40,000
patients with HD in the U.S.

Rare neurodegenerative disorder in which neurons within the brain break down



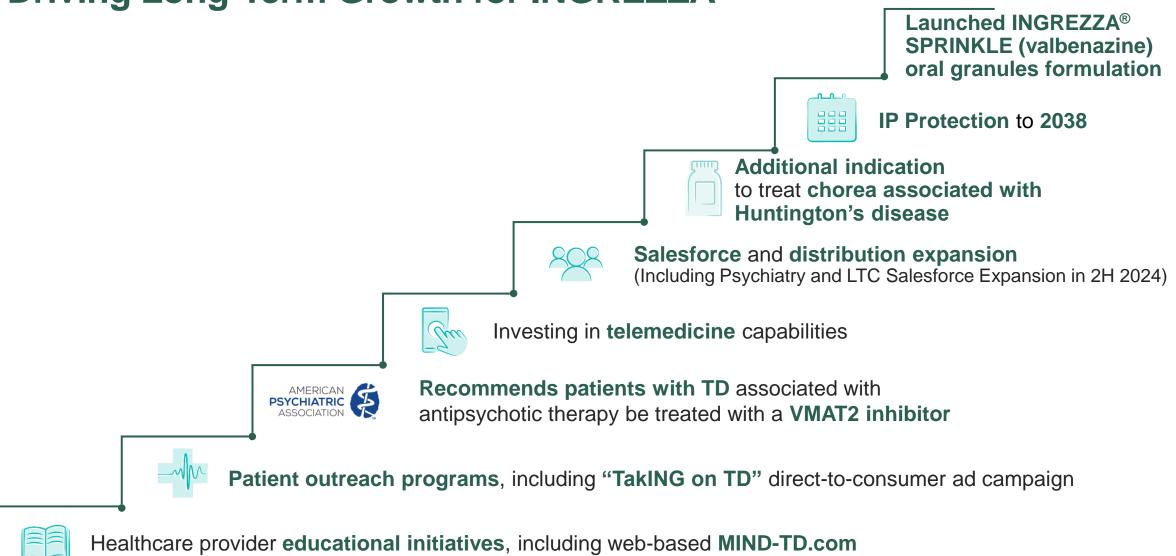
Patients develop involuntary abnormal, abrupt or irregular movements







### **Driving Long-Term Growth for INGREZZA**









## Neuropsychiatry Pipeline

## NBI-1065845\* (AMPA Potentiator): Reported Positive Phase 2 Top-Line Study Results in Adults with Major Depressive Disorder

## **Inadequate Response to Treatment** in Major Depressive Disorder (MDD)



~1/3 of the 16 million+ people in the U.S. who live with MDD do not respond to available antidepressants.



MDD symptoms are characterized by a persistently depressed mood or loss of interest in daily activities that can impact normal daily functioning, relationships, and overall quality of life.



**Current treatments range** from selective serotonin reuptake inhibitors (SSRIs), serotonin norepinephrine reuptake inhibitors (SNRIs), and antidepressants along with behavioral therapy.

#### NBI-1065845 (or NBI-'845)

#### Potent first-in-class AMPA potentiator

- Oral
- Once daily

Antidepressant effects may be mediated by activation of AMPA and resultant downstream pathways

#### **Phase 2 SAVITRI Study:**

- Met primary endpoint with statistically significant reduction in MADRS total score at day 28
- Met key secondary endpoints, including statistically significant reduction in MADRS total score at day 56
- NBI-'845 was generally well-tolerated

#### **Next Steps:**

Conducting end of Phase 2 meeting with FDA;
 Initiating Phase 3 studies in 2025



# NBI-1065845\* (AMPA Potentiator): SAVITRI<sup>™</sup> Phase 2 Study Summary Results

#### **EFFICACY**

- The study met its primary and key secondary endpoints
- Once-daily, oral administration of NBI-'845 produced a statistically significant change from baseline in Montgomery Åsberg Depression Rating Scale (MADRS) total score at both Day 28 (primary) and Day 56 (secondary).
- In the full analysis data set, the least squares (LS) mean differences from baseline in MADRS total score were:

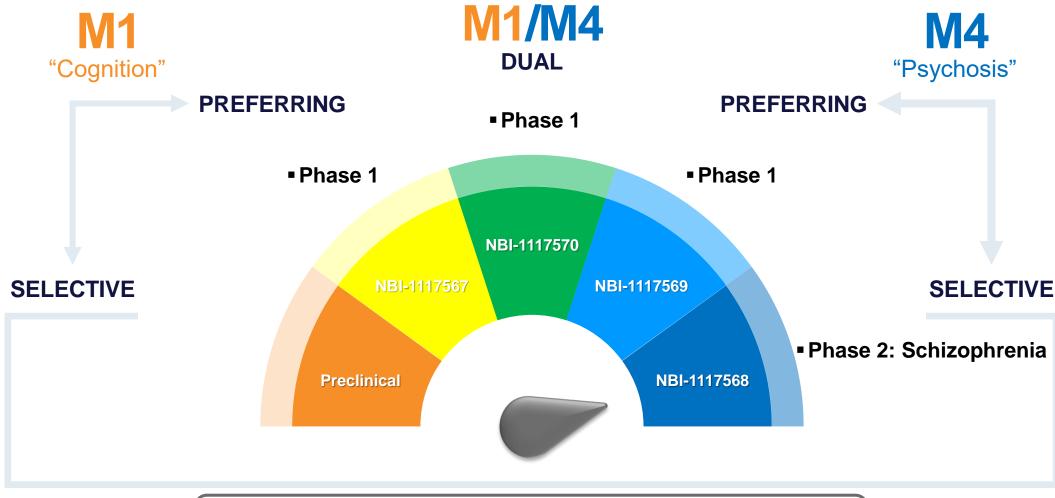
Statistically Significant Dose	Day 28	Day 56
Improvement over Placebo	-4.3	-7.5
p-value	0.0159	0.0016
Effect size	0.53	0.73
Other Dose	Day 28	Day 56
Improvement over Placebo	-3.0	-3.6
p-value	0.0873	0.1082
Effect size	0.39	0.33

#### **SAFETY AND TOLERABILITY**

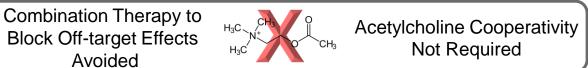
- NBI-1065845 was generally well-tolerated
- Most common adverse event was headache, of which, a majority were transient and mild in severity
- Adverse event profile for both doses of NBI-1065845 were comparable to placebo
- No seizures, deaths, or serious adverse events
- No psychotomimetic or dissociative events throughout the study
- Discontinuation rates were low throughout the study

### Muscarinic Platform Includes Multiple Clinical Programs

From M1 to M4 Selective Orthosteric Agonists







### **Developing Novel Muscarinic System Portfolio**

#### **Neurocrine Biosciences Advancing Muscarinic Portfolio (Largest in Industry)**

- > Phase 2 placebo-controlled study of NBI-1117568\*, a selective M4 agonist, as a potential treatment for schizophrenia with top-line data on track in Q3 2024
  - ✓ NBI-1117568 offers the potential for an improved safety profile:
    - ☐ Without the need of combination therapy to minimize side effects
    - ☐ Avoids the need of cooperativity with acetylcholine when compared to non-selective muscarinic agonists and positive allosteric modulators in development
- Phase 1 studies ongoing for:
  - ✓ NBI-1117567\* (M1 preferring agonist)
  - ✓ NBI-1117569\* (M4 preferring agonist) ⊢ for central nervous system disorders
  - ✓ NBI-1117570\* (dual M1 / M4 agonist)
- NBI-1076986 (M4 antagonist) for movement disorders



# Luvadaxistat\*: D-Amino Acid Oxidase (DAAO) Inhibitor in Phase 2 Study with Top-Line Data On Track in Q3 2024

## Cognitive Impairment Associated with Schizophrenia (CIAS)



Affects approximately **80% of the 3.5 million** people in the U.S. diagnosed with schizophrenia



CIAS symptoms are characterized by poor mental function and include difficulty paying attention, processing information and making decisions



No U.S. FDA-approved treatments specifically indicated for CIAS

#### Luvadaxistat

#### Potent first-in-class DAAO inhibitor

- Once daily
- No titration requirement

Hypofunction of glutamatergic signaling has been implicated in the pathophysiology of schizophrenia

Phase 2 INTERACT study data showed luvadaxistat met secondary endpoints of cognitive assessment

#### **Ongoing Phase 2 study in CIAS**

- Evaluate safety and efficacy of luvadaxistat compared to placebo on improving cognitive performance in participants with schizophrenia
- Top-line data read-out on track in Q3 2024

# Valbenazine\*: ATS Study Will Inform Development of Our Next-Generation VMAT2 Inhibitors Including NBI-1065890 (Currently in Phase 1)

#### **Adjunctive Treatment of Schizophrenia (ATS)**



Schizophrenia is one of the **leading causes of disability** worldwide, affecting **up to 3.5M people** in the U.S. alone.



A serious, chronic mental illness that causes **abnormal thoughts**, **feelings** and actions.



Estimated that ~30% of patients with schizophrenia in the U.S. do not adequately respond to antipsychotic therapy, underscoring a clear unmet need for improved pharmacological approaches.







Neuroendocrinology Pipeline

## Classic Congenital Adrenal Hyperplasia (CAH)



#### **Rare Genetic Disorder**

Enzyme deficiency & reduced cortisol levels and excess androgen levels

U.S. ~30,000\*





## **Treatment Options Stagnant for 70 Years**

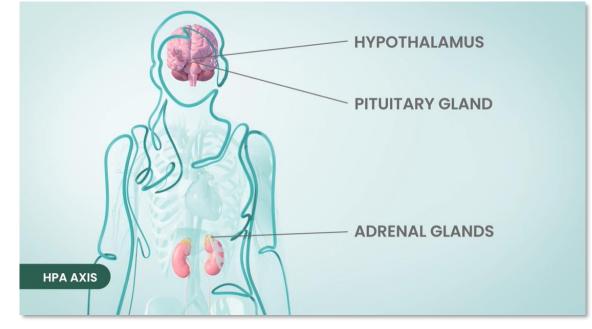


- Hormone replacement
- Do not address underlying issue











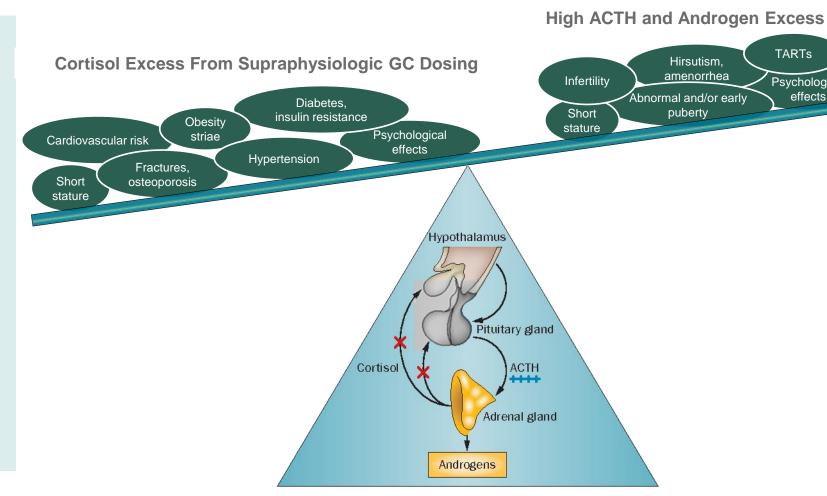
## Classic Congenital Adrenal Hyperplasia Due to 21-Hydroxylase **Deficiency (210HD CAH)**

#### 210HD CAH Results in:

- Impaired Synthesis of Cortisol and (Often) Aldosterone
- Excess Adrenal Androgen Production

#### **Treatment Must Balance Consequences of:**

- Supraphysiologic Glucocorticoid (GC) Doses
- High ACTH and Androgen **Excess**





Adapted from: Han TS et al. Nat Rev Endocrinol. 2014;10(2):115-24.

TARTs

Psychological

effects

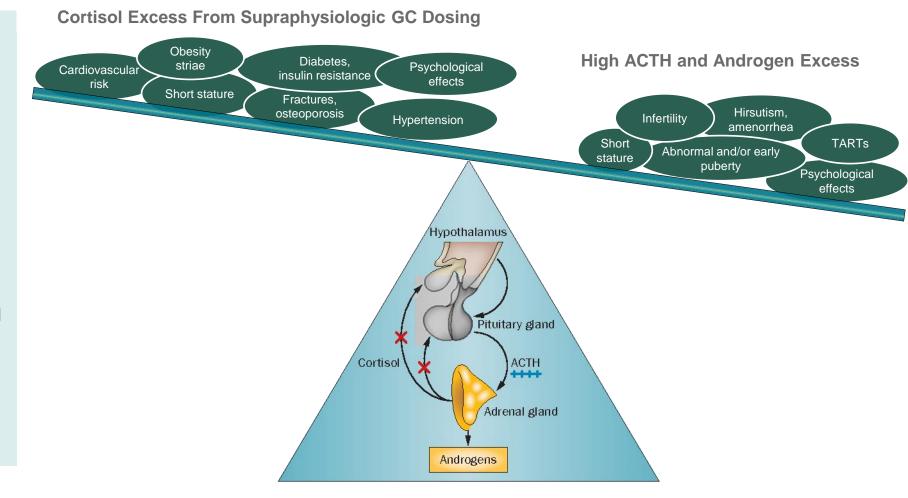
# Classic Congenital Adrenal Hyperplasia Due to 21-Hydroxylase Deficiency (210HD CAH)

#### 210HD CAH Results in:

- Impaired Synthesis of Cortisol and (Often) Aldosterone
- Excess Adrenal Androgen Production

## **Treatment Must Balance Consequences of:**

- Supraphysiologic Glucocorticoid (GC) Doses
- High ACTH and Androgen Excess





# Classic Congenital Adrenal Hyperplasia Due to 21-Hydroxylase Deficiency (210HD CAH)

#### 210HD CAH Results in:

- Impaired Synthesis of Cortisol and (Often) Aldosterone
- Excess Adrenal Androgen Production

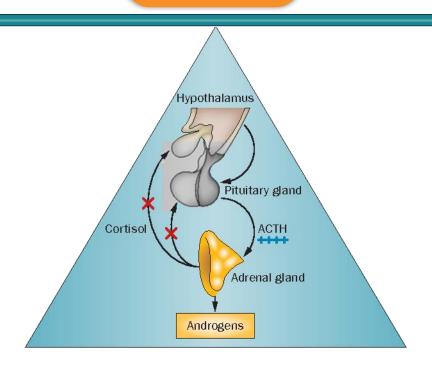
## **Treatment Must Balance Consequences of:**

- Supraphysiologic Glucocorticoid (GC) Doses
- High ACTH and Androgen Excess

**Reduced GC Dosing** 

crinecerfont

**Androgen Control** 







## CAHtalyst<sup>™</sup> Adult and Pediatric Study Androgen Reduction

Percent Change\* in
Androstenedione at Week 4
(Following Glucocorticoid Stable Period)

Study Characteristic	Adult Study (N = 182)	Pediatric Study (N = 103)	Key Takeaways
Patients Receiving Crinecerfont	-45%	-54%	Substantial and Meaningful Reduction in Androgens with Crinecerfont by 4 Weeks
Patients Receiving Placebo	+21%	+33%	Increase in Androgens on Placebo Reflecting Poor Disease Control Despite High Dose Steroids
Placebo-Adjusted Difference (Patients Receiving Crinecerfont – Patients Receiving Placebo)	-66%	-86%	Similar to Androgen Reduction Observed in Phase 2 Open-Label Studies (14 days)
P-value	<0.0001	<0.0001	

Phase 3 Study Date Presented at ENDO in June and Published in the New England Journal of Medicine (Link Here)



# CAHtalyst<sup>TM</sup> Adult and Pediatric Study Glucocorticoid Dose Reduction While Maintaining Androgen Control

Percent of Subjects Achieving a Glucocorticoid Daily Dose ≤ 11 mg/m²/day While Maintaining Androgen Control

CAHtalyst <sup>™</sup> Trial Participants	Adult Study @ Week 24	Pediatric Study @ Week 28	Key Takeaways
Patients Receiving Crinecerfont	63%	30%	Substantial Percentage of Patients on Crinecerfont Achieved Physiologic GC Dose with Androgen Control
Patients Receiving Placebo	18%	0%	No Pediatric Patients on Placebo Achieved Physiologic GC Dose Reflecting Inadequacy of GC to Treat High Androgen
Placebo-Adjusted Difference (Patients Receiving Crinecerfont – Patients Receiving Placebo)	45%	30%	Similar Results in Adult and Pediatric Patients Considering Differences at Baseline and in Trials
P-value	<0.0001	0.0009*	

In Addition, Treatment with Crinecerfont in Adult and Pediatric Patients Resulted in Significant Percent Reduction in Glucocorticoid Dose while Maintaining Androgen Control (p<0.0001 both studies)



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## CAHtalyst<sup>™</sup> Adult and Pediatric Study Safety and Tolerability

- Crinecerfont Treatment was Overall Well-Tolerated with Few Serious Adverse Events (SAEs),
   None Were Assessed as Related to Crinecerfont
- Most Common Adverse Events During the Double-Blind, Placebo-Controlled Period of the Adult Study were Fatigue, Headache, and Coronavirus Infection
- Most Common Adverse Events During the Double-Blind, Placebo-Controlled Period of the Pediatric Study were Headache, Fever, Vomiting, Upper Respiratory Tract Infection, and Nasopharyngitis
- No Safety Concerns Related to Adrenal Crisis



### **Neurocrine Next Steps Regarding Crinecerfont**

- New Drug Applications (NDAs) Have Been Accepted by the FDA
- FDA Granted Priority Review For Crinecerfont for Adult and Pediatric Patients with CAH
- Prescription Drug User Fee (PDUFA) Target Action Dates Set for December 29 for the Capsule Formulation and December 30 for the Oral Solution Formulation
  - ➤ If Approved, Neurocrine Can Activate a Rare Pediatric Disease Designation Priority Review Voucher Which Could Be Utilized to Accelerate the Review Process for a Future Registrational Program
- Neurocrine's Rare Endocrinology Commercial Team Fully Hired and Focused on Market Development Initiatives to Better Understand the CAH Community
- Disease State Education Includes "What the C@H!", An Educational Initiative That Aims To:
  - ➤ Close the Gap in the Need for Helpful Information About CAH
  - > Acknowledges Frustrations and Challenges Experienced by the Community Managing the Condition
- The Open-Label Treatment Periods for the CAHtalyst<sup>TM</sup> Pediatric and Adult Studies are Ongoing







## Neurology Pipeline

## Valbenazine\*: Registrational Program in Dyskinetic Cerebral Palsy

#### **Dyskinetic Cerebral Palsy (DCP)**



A form of cerebral palsy (CP) that affects ~15% of the approximately 500,000 to 1M people in the U.S. diagnosed with the disease.



Can result in a range of developmental delays, physical difficulties and involuntary muscle movements.



No approved treatments. Many patients take off-label drugs with low efficacy and unwanted side effects.



## Well-Positioned for Sustained & Long-term Growth

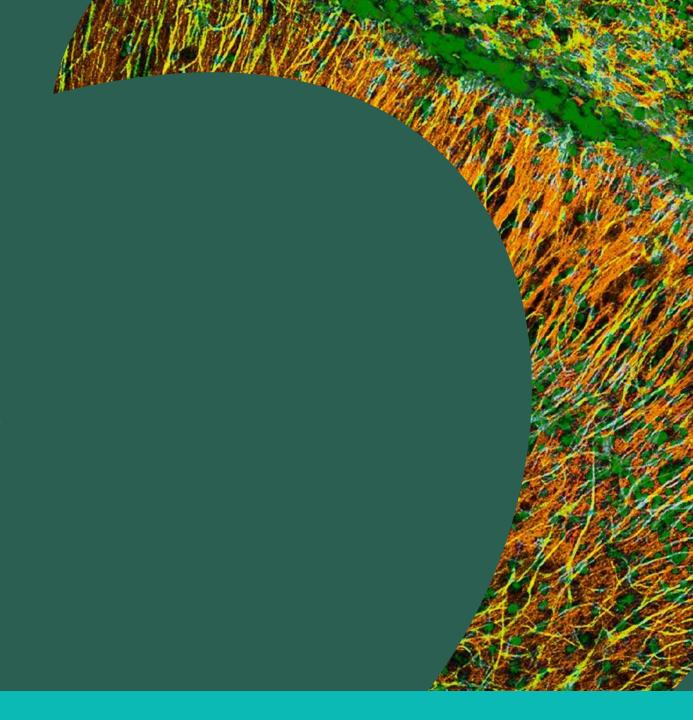
Commerc	ial	R&D Focus	Strong Financial Position
TARDIVE	AGREZZA® *  Albenazine) capsules  A DYSKINESIA AND CHOREA  WITH HUNTINGTON'S DISEASE  2024 Annual  Net Sales Guidance Raised	Neurology Neuroendocrinology Neuropsychiatry	~\$1.7B  Cash and Investments as of 6/30/2024
Billion	and Narrowed from \$2.1 - \$2.2 Billion	Robust Pipeline	Strong Balance Sheet
~600,000	Affected by Tardive Dyskinesia in the U.S.; ~65% are undiagnosed	Multiple Compounds in Mid- to Late-Stage Studies	Durable Cash Flows
~90%	of the ~40,000 People in the U.S. Diagnosed with Huntington's Disease Who Will Develop Chorea	Rapidly Growing Early- Stage Portfolio	Attractive P&L Profile





# GAAP to Non-GAAP Reconciliations

neurocrine.com



#### NEUROCRINE BIOSCIENCES, INC.

## CONDENSED CONSOLIDATED STATEMENTS OF INCOME (unaudited)

	Three Months Ended June 30,			ths Ei e 30,	hs Ended e 30,	
(in millions, except per share data)		2024	2023	2024		2023
Revenues:						
Net product sales	\$	583.8	\$ 446.3	\$ 1,092.8	\$	861.6
Collaboration revenue		6.4	 6.4	 12.7		11.5
Total revenues		590.2	452.7	1,105.5		873.1
Operating expenses:						
Cost of revenues		9.2	11.5	16.7		20.0
Research and development		191.1	145.8	350.5		285.3
Acquired in-process research and development		2.5		8.5		143.9
Selling, general and administrative		242.0	221.8	485.1		464.5
Total operating expenses		444.8	379.1	 860.8		913.7
Operating income (loss)		145.4	73.6	244.7		(40.6)
Other (expense) income:						
Unrealized (loss) gain on equity securities		(19.9)	37.3	(18.3)		39.5
Charges associated with convertible senior notes		(49.7)		(138.4)		
Investment income and other, net		22.8	 10.7	 45.1		19.4
Total other (expense) income, net		(46.8)	 48.0	 (111.6)		58.9
Income before provision for income taxes		98.6	121.6	133.1		18.3
Provision for (benefit from) income taxes		33.6	 26.1	 24.7		(0.6)
Net income	\$	65.0	\$ 95.5	\$ 108.4	\$	18.9
Earnings per share, basic	\$	0.64	\$ 0.98	\$ 1.08	\$	0.19
Earnings per share, diluted	\$	0.63	\$ 0.95	\$ 1.04	\$	0.19
Weighted average common shares outstanding, basic		100.8	97.6	100.3		97.4
Weighted average common shares outstanding, diluted		103.9	100.2	103.8		100.3



## NEUROCRINE BIOSCIENCES, INC. CONDENSED CONSOLIDATED BALANCE SHEETS (unaudited)

(in millions)	 June 30, 2024	De	cember 31, 2023
Cash, cash equivalents and marketable securities	\$ 1,038.9	\$	1,031.6
Other current assets	630.9		575.4
Total current assets	1,669.8		1,607.0
Deferred tax assets	419.5		362.6
Debt securities available-for-sale	637.8		687.5
Right-of-use assets	262.9		276.5
Equity security investments	143.6		161.9
Property and equipment, net	80.1		70.8
Intangible assets, net	33.5		35.5
Other noncurrent assets	57.8		49.6
Total assets	\$ 3,305.0	\$	3,251.4
Convertible senior notes	\$ _	\$	170.1
Other current liabilities	398.5		484.7
Total current liabilities	398.5		654.8
Noncurrent operating lease liabilities	256.2		258.3
Other noncurrent long-term liabilities	141.1		106.3
Stockholders' equity	2,509.2		2,232.0
Total liabilities and stockholders' equity	\$ 3,305.0	\$	3,251.4



#### NEUROCRINE BIOSCIENCES, INC.

### RECONCILIATION OF GAAP TO NON-GAAP FINANCIAL RESULTS (unaudited)

	Three Months Ended June 30,				Six Months Ended June 30,				
(in millions, except per share data)	2024			2023		2024		2023	
GAAP net income 1	\$	65.0	\$	95.5	\$	108.4	\$	18.9	
Adjustments:									
Stock-based compensation expense - R&D		15.8		23.8		32.8		37.6	
Stock-based compensation expense - SG&A		27.3		44.7		54.8		70.8	
Charges associated with convertible senior notes 2		49.7		_		138.4		_	
Impairment charges associated with leased properties <sup>3</sup>		14.0		_		14.0		_	
Non-cash amortization related to acquired intangible assets		0.9		0.9		1.8		1.8	
Changes in fair value of equity security investments <sup>4</sup>		19.9		(37.3)		18.3		(39.5)	
Other		0.1		0.2		0.3		0.4	
Income tax effect related to reconciling items <sup>5</sup>		(23.8)		(2.1)		(75.1)		(13.8)	
Non-GAAP net income	\$	168.9	\$	125.7	\$	293.7	\$	76.2	
Diluted earnings per share:									
GAAP	\$	0.63	\$	0.95	\$	1.04	\$	0.19	
Non-GAAP	\$	1.63	\$	1.25	\$	2.83	\$	0.76	

- 1. Three and six months ended June 30, 2024 reflect \$26.5 million and \$32.6 million, respectively, of development milestone expense achieved under collaboration agreements. Six months ended June 30, 2023 reflects IPR&D expense of \$143.9 million related to expansion of strategic partnership with Voyager Therapeutics, Inc.
- 2. Reflects charges associated with the settlement of convertible senior notes conversions.
- 3. Reflects impairment charges associated with leased office space that has been vacated as the Company continues to occupy its new campus facility.
- 4. Reflects periodic fluctuations in the fair values of the Company's equity security investments.
- 5. Estimated income tax effect of Non-GAAP reconciling items are calculated using applicable statutory tax rates, taking into consideration any valuation allowance and adjustments to exclude tax benefits or expenses associated with charges associated with convertible senior notes and non-cash stock-based compensation.



## NEUROCRINE BIOSCIENCES, INC. RECONCILIATION OF GAAP TO NON-GAAP EXPENSES (unaudited)

	Three Months Ended June 30,					Six Months Ended June 30,				
(in millions)		2024		2023		2024		2023		
GAAP cost of revenues	\$	9.2	\$	11.5	\$	16.7	\$	20.0		
Adjustments:										
Non-cash amortization related to acquired intangible assets		0.9		0.9		1.8		1.8		
Non-GAAP cost of revenues	\$	8.3	\$	10.6	\$	14.9	\$	18.2		
	Thre			Three Months Ended June 30,			Six Months Ended June 30,			
(in millions)		2024		2023		2024		2023		
GAAP R&D	\$	191.1	\$	145.8	\$	350.5	\$	285.3		
Adjustments:										
Stock-based compensation expense		15.8		23.8		32.8		37.6		
Non-GAAP R&D	\$	175.3	\$	122.0	\$	317.7	\$	247.7		
		Three Months Ended June 30,			Six Months Ended June 30,					
(in millions)		2024		2023		2024		2023		
GAAP SG&A	\$	242.0	\$	221.8	\$	485.1	\$	464.5		
Adjustments:										
Stock-based compensation expense		27.3		44.7		54.8		70.8		
Impairment charges associated with leased properties		14.0		_		14.0		_		
Non-GAAP SG&A	\$	200.7	\$	177.1	\$	416.3	\$	393.7		
		Three Months Ended June 30,				Six Months Ended June 30,				
(in millions)		2024		2023		2024		2023		
GAAP other (expense) income, net	\$	(46.8)	\$	48.0	\$	(111.6)	\$	58.9		
Adjustments:										
Charges associated with convertible senior notes		49.7		_		138.4		_		
Changes in fair value of equity security investments		19.9		(37.3)		18.3		(39.5)		
Other		0.1		0.2		0.3		0.4		
Non-GAAP other income, net	\$	22.9	\$	10.9	\$	45.4	\$	19.8		



## Advancing Life-Changing Discoveries in Neuroscience

Q2 2024 Corporate Presentation August 1, 2024

Nasdaq: NBIX



