

# Advancing Life-Changing Discoveries in Neuroscience

Q3 2024

Corporate Presentation

October 30, 2024

Nasdaq: NBIX



You Deserve *Brave* Science™



# 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; the timing of the initiation and/or completion of our clinical, regulatory, and other development activities and those of our collaboration partners; and our intention to enter into an accelerated share repurchase transaction, including the expected dollar amounts and the timing of the transaction. 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; constraints, volatility, or disruptions in the capital markets or other factors affecting our ability to enter into or complete an accelerated share repurchase transaction; and other risks described in our periodic reports filed with the SEC, including our Quarterly Report on Form 10-Q for the quarter ended September 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, vacated legacy campus facility costs, net of sublease income, 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 basis. A reconciliation of these GAAP financial results to Non-GAAP financial results is included in the attached financial information.

# Well-Positioned for Sustained & Long-term Growth

## Commercial



TARDIVE DYSKINESIA AND CHOREA  
ASSOCIATED WITH HUNTINGTON'S DISEASE  
2024 Annual

**\$2.30 - \$2.32  
Billion**

Net Sales Guidance Raised  
and Narrowed from  
\$2.25 - \$2.30 Billion

**~800,000**

Affected by Tardive  
Dyskinesia in the U.S.;  
~75% are undiagnosed

**~90%**

of the ~40,000 People in  
the U.S. Diagnosed with  
Huntington's Disease Who  
Will Develop Chorea

## R&D Focus

Neurology  
Neuroendocrinology  
Neuropsychiatry

Robust Pipeline

Multiple Compounds in  
Mid- to Late-Stage Studies

Rapidly Growing Early-  
Stage Portfolio

## Strong Financial Position

**~\$1.9B**

Cash and Investments  
as of 9/30/2024

Strong Balance Sheet

Durable Cash Flows

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



# Recent Highlights and Future Key Milestones and Activities

## Q3 2024 / Recent Highlights

- Kyle W. Gano, Ph.D. succeeds Kevin Gorman as CEO
- **INGREZZA® (valbenazine) Net Product Sales of \$613M**
  - Represents YoY Sales Growth of 26% vs. Q3 2023
  - Growth Driven By Strong Underlying Demand and Improvement in Gross To Net Dynamics
- **Completed Expansion of INGREZZA Psychiatry and LTC Sales Teams to Help Accelerate Appropriate Diagnosis and Treatment of Tardive Dyskinesia and Huntington’s Disease Chorea**
- **Presented KINECT®-HD2 Interim Data at the 2024 MDS International Congress of Parkinson’s Disease and Movement Disorders**
  - Demonstrated Robust and Sustained Improvements in Chorea Associated with HD Through Week 104 Irrespective of Antipsychotic Use
- **Specialty Endocrinology Salesforce Conducting CAH Disease State Education and Identifying Where Patients Seek Treatment**
- **Reported Positive Phase 2 Results for NBI-’568 (M4 Agonist) for the Treatment of Schizophrenia**
- **Board of Directors Authorized \$300M Share Repurchase Plan**

## Future Key Milestones and Activities

- **Company Intends to Enter Into a \$300M Accelerated Share Repurchase Transaction, Subject to Market Conditions**
- **Crinecerfont PDUFA Dates Set for December 29, 2024 (Capsule Formulation) and December 30, 2024 (Oral Solution Formulation)**
- **NBI-’845 and NBI-568 End of Phase 2 Meetings with FDA**
- **Initiating Registrational Studies for NBI-’845 (AMPA Potentiator) in MDD and NBI-’568 (M4 Agonist) in Schizophrenia in First Half of 2025**
- **On Track to Report Phase 2 Top-Line Data for NBI-’770 (NMDA NR2B NAM) in 2025**
- **Advancing Broadest and Most Diverse Muscarinic Portfolio in Industry including NBI-’568 and Four Ongoing Phase 1 Studies;**
  - NBI-’567 (M1 Agonist)
  - NBI-’569 (M4 Agonist)
  - NBI-’570 (Dual M1 / M4 Agonist)
  - NBI-’986 (M4 Antagonist)

In Collaboration with Nxera  
Pharma UK Limited

LTC = Long-Term Care; CAH = Congenital Adrenal Hyperplasia; M4 = M4 Muscarinic Receptor; PDUFA = Prescription Drug User Fee Act; FDA = U.S. Food and Drug Administration; AMPA = alpha-amino-3-hydroxy-5-methyl-4-isoxazole propionic acid; MDD = Major Depressive Disorder; NMDA NR2B NAM = n-methyl-d-aspartate Receptor Subtype 2B Negative Allosteric Modulator; M1 = M1 Muscarinic Receptor

# Building and Maximizing the Pipeline

# of Programs by Stage



			Phase 1	Phase 2	Phase 3	NDA
<b>Neurology</b>						
<b>valbenazine*</b>	<i>Dyskinetic Cerebral Palsy</i>	<i>VMAT2 Inhibitor</i>	[Progress bar: Phase 1 to Phase 2]			
<b>NBI-921352<sup>2</sup></b>	<i>SCN8A-DEE</i>	<i>Na<sub>v</sub>1.6</i>	[Progress bar: Phase 1 to Phase 2]			
<b>NBI-1076986</b>	<i>Movement Disorders</i>	<i>M4 Antagonist</i>	[Progress bar: Phase 1 to Phase 2]			
<b>Neuroendocrinology</b>						
<b>crinecerfont<sup>3</sup></b>	<i>CAH: Adults</i>	<i>CRF-R1</i>	[Progress bar: Phase 1 to Phase 3]			
<b>crinecerfont<sup>3</sup></b>	<i>CAH: Pediatrics</i>	<i>CRF-R1</i>	[Progress bar: Phase 1 to Phase 3]			
<b>Efmody</b>	<i>Adrenal Insufficiency</i>	<i>GC Receptor</i>	[Progress bar: Phase 1 to Phase 3]			
<b>Efmody</b>	<i>CAH</i>	<i>GC Receptor</i>	[Progress bar: Phase 1 to Phase 3]			
<b>Neuropsychiatry</b>						
<b>valbenazine*</b>	<i>ATS</i>	<i>VMAT2 Inhibitor</i>	[Progress bar: Phase 1 to Phase 3]			
<b>NBI-1065845<sup>4</sup></b>	<i>Inadequate Response-MDD</i>	<i>AMPA Potentiator</i>	[Progress bar: Phase 1 to Phase 3]			
<b>NBI-1117568<sup>1</sup></b>	<i>Schizophrenia</i>	<i>M4 Agonist</i>	[Progress bar: Phase 1 to Phase 3]			
<b>NBI-1070770<sup>5</sup></b>	<i>MDD</i>	<i>NMDA NR2B NAM</i>	[Progress bar: Phase 1 to Phase 2]			
<b>NBI-1117570<sup>1</sup></b>	<i>CNS Indications</i>	<i>M1/M4-Dual</i>	[Progress bar: Phase 1 to Phase 1]			
<b>NBI-1117569<sup>1</sup></b>	<i>CNS Indications</i>	<i>M4-Preferring</i>	[Progress bar: Phase 1 to Phase 1]			
<b>NBI-1117567<sup>1†</sup></b>	<i>CNS Indications</i>	<i>M1-Preferring</i>	[Progress bar: Phase 1 to Phase 1]			
<b>NBI-1065890</b>	<i>CNS Indications</i>	<i>VMAT2 Inhibitor</i>	[Progress bar: Phase 1 to Phase 1]			

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

† Nxera Pharma UK Limited 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 =

(1) Nxera Pharma UK Limited (2) Xenon Pharmaceuticals Inc (3) Sanofi S.A. (4) Takeda Pharmaceutical Company Limited

Neurocrine Biosciences has global rights unless otherwise noted.

# Q3 2024 Financial Summary

\$ Millions, Except Non-GAAP Earnings Per Share

Item	Q3 2024	Q3 2023	Highlights / Comments
<b>Revenue</b>	\$622	\$499	INGREZZA Sales of \$613M Represents YoY Growth of 26% Driven by Strong Underlying Patient Demand and Improvement in Gross-to-Net Dynamics
- Product Sales, Net	\$617	\$492	
- Collaboration Revenue	\$5	\$7	
<b>Non-GAAP R&amp;D Expense</b>	\$180	\$125	Increase Driven by Expanded / Advancing Portfolio and Includes \$39M for Development Milestones Achieved Under Our Collaborations
<b>Non-GAAP Acquired IPR&amp;D Expense</b>	\$1	\$0	
<b>Non-GAAP SG&amp;A Expense</b>	\$205	\$170	Increase Driven by Incremental Investment in Crinecerfont-Related Headcount and Pre-Launch Activities, and Continued Investment in INGREZZA
<b>Non-GAAP Net Income</b>	\$189	\$156	Increase Driven by Higher INGREZZA Sales Partially Offset by Incremental Operating Expenses
<b>Non-GAAP Earnings per Share, Diluted</b>	\$1.81	\$1.54	Represents YoY Growth of 18%
<b>Cash and Investments (Period End)</b>	\$1,872	\$1,550	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	2024 YTD*	2023 YTD*	Highlights / Comments
<b>Revenue</b>	\$1,728	\$1,372	YTD INGREZZA Sales of ~\$1.7B Represents YoY Growth of 27% vs. YTD 2023
- Product Sales, Net	\$1,709	\$1,353	
- Collaboration Revenue	\$18	\$19	
<b>Non-GAAP R&amp;D Expense</b>	\$498	\$373	Increase Driven by Expanded / Advancing Portfolio and Includes \$71M for Development Milestones Achieved Under Our Collaborations
<b>Non-GAAP Acquired IPR&amp;D Expense</b>	\$10	\$144	2023 IPR&D Related to Voyager Collaboration
<b>Non-GAAP SG&amp;A Expense</b>	\$621	\$563	Increase Driven by Incremental Investment in Crinecerfont-Related Headcount and Pre-Launch Activities, And Continued Investment in INGREZZA
<b>Non-GAAP Net Income</b>	\$483	\$232	Increase Driven by Higher INGREZZA Sales Partially Offset by Incremental Operating Expenses and Lower Total Payments for Upfront Fees / Development Milestones in Connection With Our Collaborations
<b>Non-GAAP Earnings per Share, Diluted</b>	\$4.64	\$2.31	Represents YoY Growth of 100% vs. YTD 2023
<b>Cash and Investments (Period End)</b>	\$1,872	\$1,550	Settled Outstanding 2024 Notes Upon Maturity in May 2024 for \$309M in Cash



# Raised 2024 INGREZZA Net Sales Guidance and Revised Expense Guidance

Item (\$ Millions)	2023 Actuals	2024 Previous Guidance Range	2024 Current Guidance Range	Comments
INGREZZA Net Product Sales <sup>1</sup>	\$1,836	\$2,250 - \$2,300	\$2,300 - \$2,320	Raised Sales Guidance
GAAP R&D Expense <sup>2</sup>	\$565	\$665 - \$695	\$700 - \$720	Revised GAAP and Non-GAAP Expense Guidance Ranges
Non-GAAP R&D Expense <sup>3</sup>	\$497	\$600 - \$630	\$635 - \$655	
GAAP and Non-GAAP IPR&D <sup>4</sup>	\$144	\$9	\$10	
GAAP SG&A Expense <sup>5</sup>	\$888	\$955 - \$975	\$970 - \$990	
Non-GAAP SG&A Expense <sup>3, 5</sup>	\$757	\$830 - \$850	\$825 - \$845	

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 \$71 million of expense for development milestones achieved or deemed probable to achieve under collaborations (Nxera Pharma UK Limited, Takeda Pharmaceutical Company Limited, Voyager Therapeutics, Inc.) of which \$39 million was recognized in the third quarter 2024 . 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 \$65 million in R&D and \$125 million in SG&A and vacated legacy campus facility costs, including office space impairment charges of approximately \$20 million in SG&A. SG&A stock-based compensation includes an approximate \$15 million charge to be recognized in the fourth quarter associated with the retirement of our CEO in October 2024.
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 expansion of the psychiatry and long-term care sales teams in September and pre-launch commercial activities for crinecerfont.



# Corporate Sustainability: “A” Rating Reaffirmed 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 [study](#) by the Biotechnology Innovation Organization

Click [here](#) to see Neurocrine’s 2024 ESG Report



# Our Medicines, Our Patients

# Multiple Commercial Products

In the U.S.


 **INGREZZA**<sup>®</sup> \*  
(valbenazine) capsules

TARDIVE DYSKINESIA

CHOREA ASSOCIATED WITH HD

 **Orilissa**<sup>®</sup> †  
elagolix tablets 150 mg  
200 mg

ENDOMETRIOSIS

 **OriaHnn**<sup>®</sup> †  
elagolix, estradiol and  
norethindrone acetate capsules  
and elagolix capsules 300 mg/1 mg/0.5 mg  
and 300 mg

UTERINE FIBROIDS

In the U.S. and Europe

 **Alkindi**<sup>®</sup>

hydrocortisone granules  
in capsules for opening

ADRENAL INSUFFICIENCY

In Europe

 **Efmody**<sup>®</sup>

Hydrocortisone modified-  
release hard capsules

CONGENITAL ADRENAL  
HYPERPLASIA



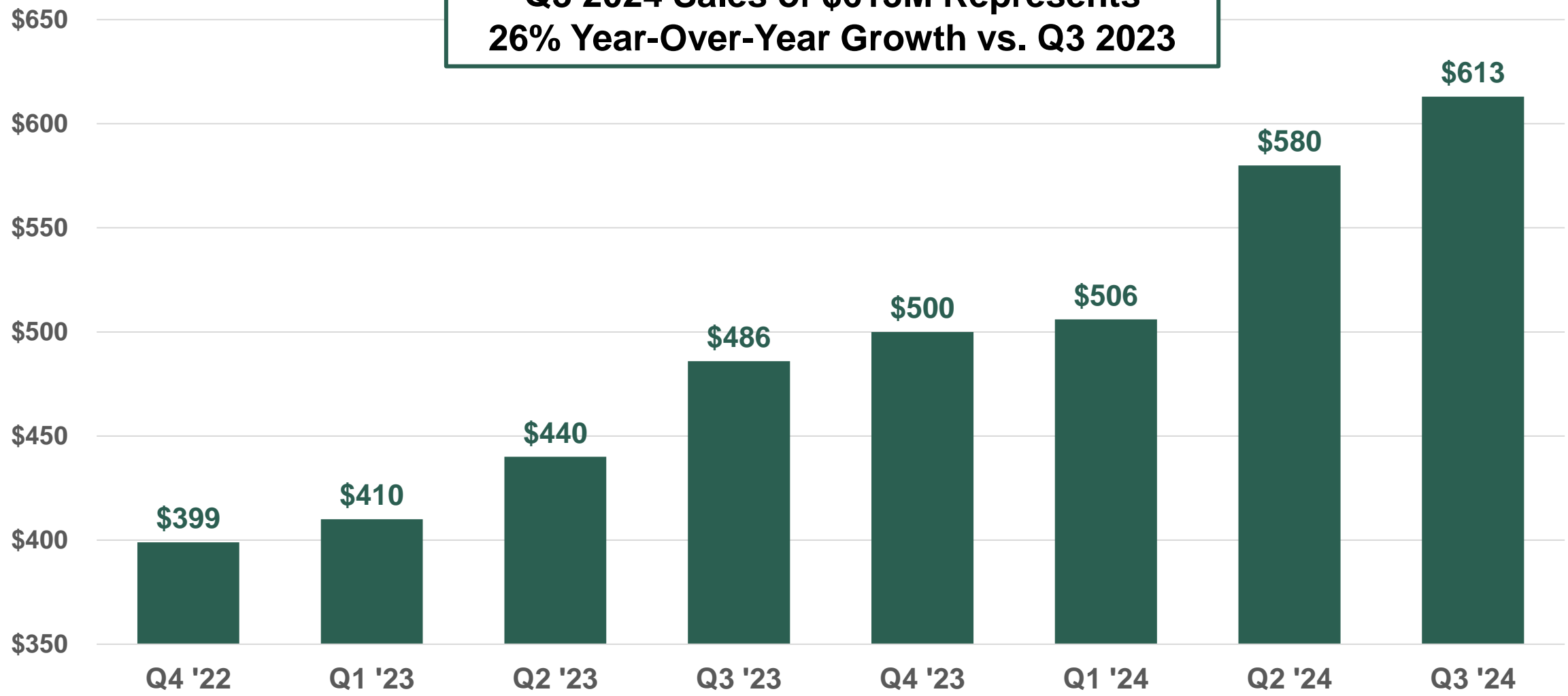


**INGREZZA<sup>®</sup>**  
(valbenazine) capsules

# INGREZZA Quarterly Net Sales Performance

(\$Million)

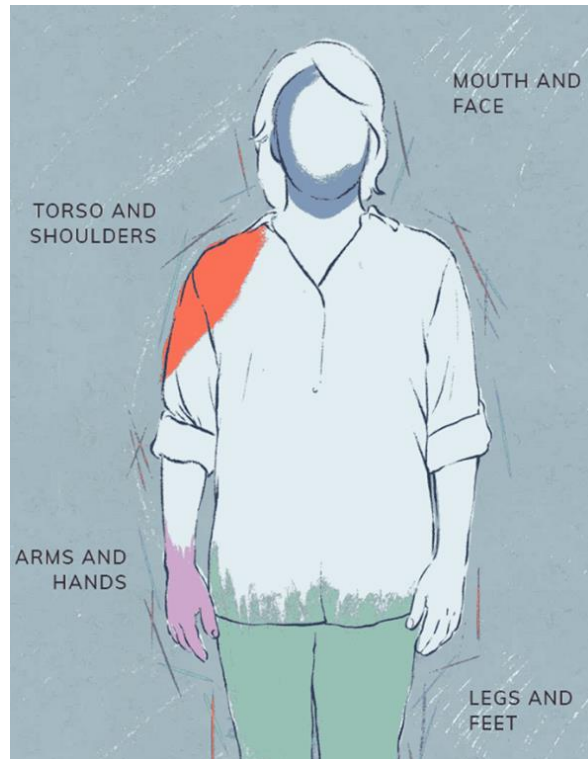
**Q3 2024 Sales of \$613M Represents  
26% Year-Over-Year Growth vs. Q3 2023**



# 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

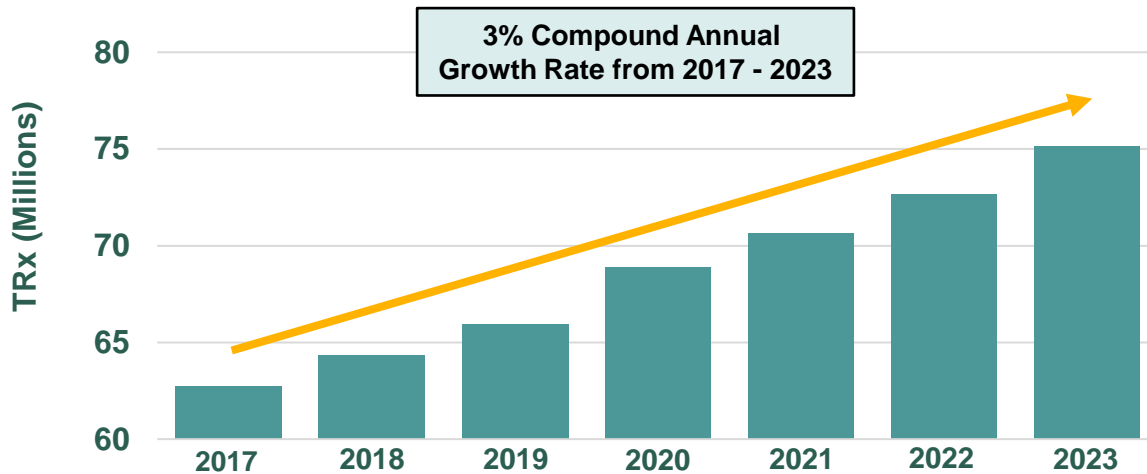
\* <https://www.takeontd.com/> Source: IQVIA's SMART Audit, Quarterly Data for Antipsychotic Class

# Nascent Tardive Dyskinesia Market Presents Significant Opportunity



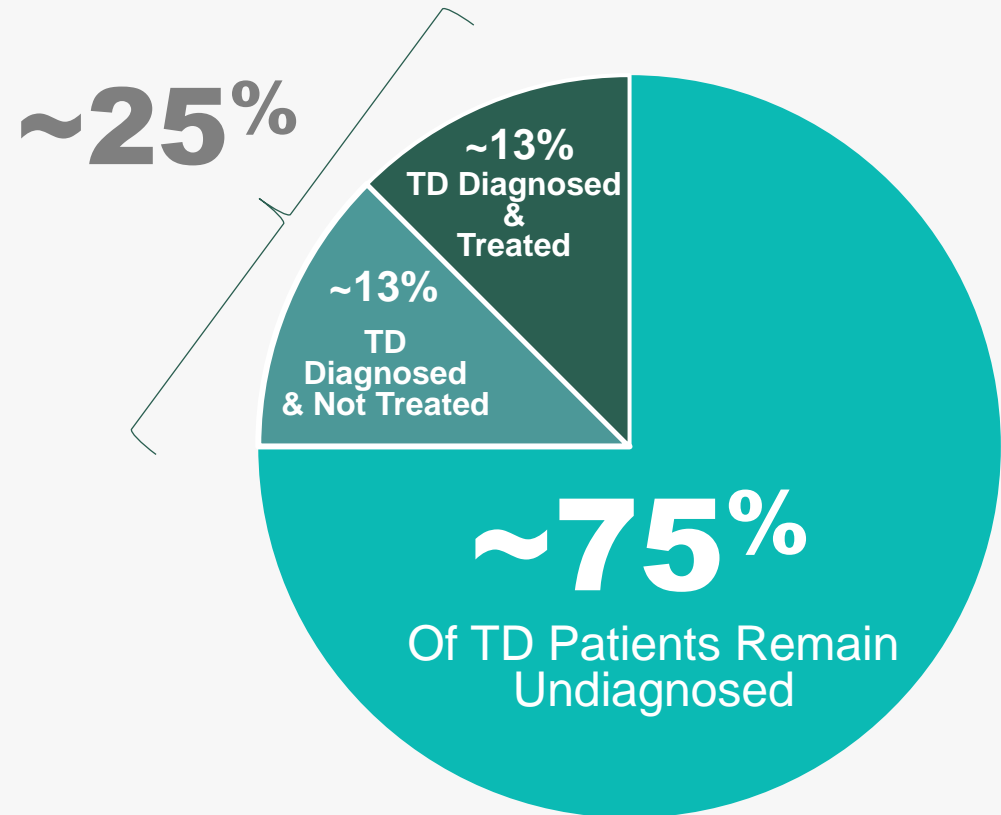
Source: U.S. Claims Data and 31 Global Scientific Publications;  
U.S. Tardive Dyskinesia Prevalence Estimates Updated Biannually; Last Update in October 2024

## Increasing Antipsychotic Prescriptions (U.S.)



## Approximately 75% 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

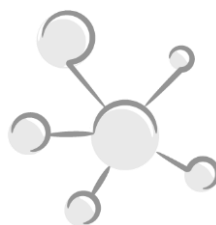
In randomized, double-blind, placebo-controlled KINECT-HD study, **treatment with valbenazine resulted in a placebo-adjusted mean reduction in the TMC\* score of 3.2 units ( $p < 0.0001$ )**

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

### INGREZZA MAKES DOSING SIMPLE FROM THE START:



No complex dose adjustments



Always one capsule, once daily



Taken any time

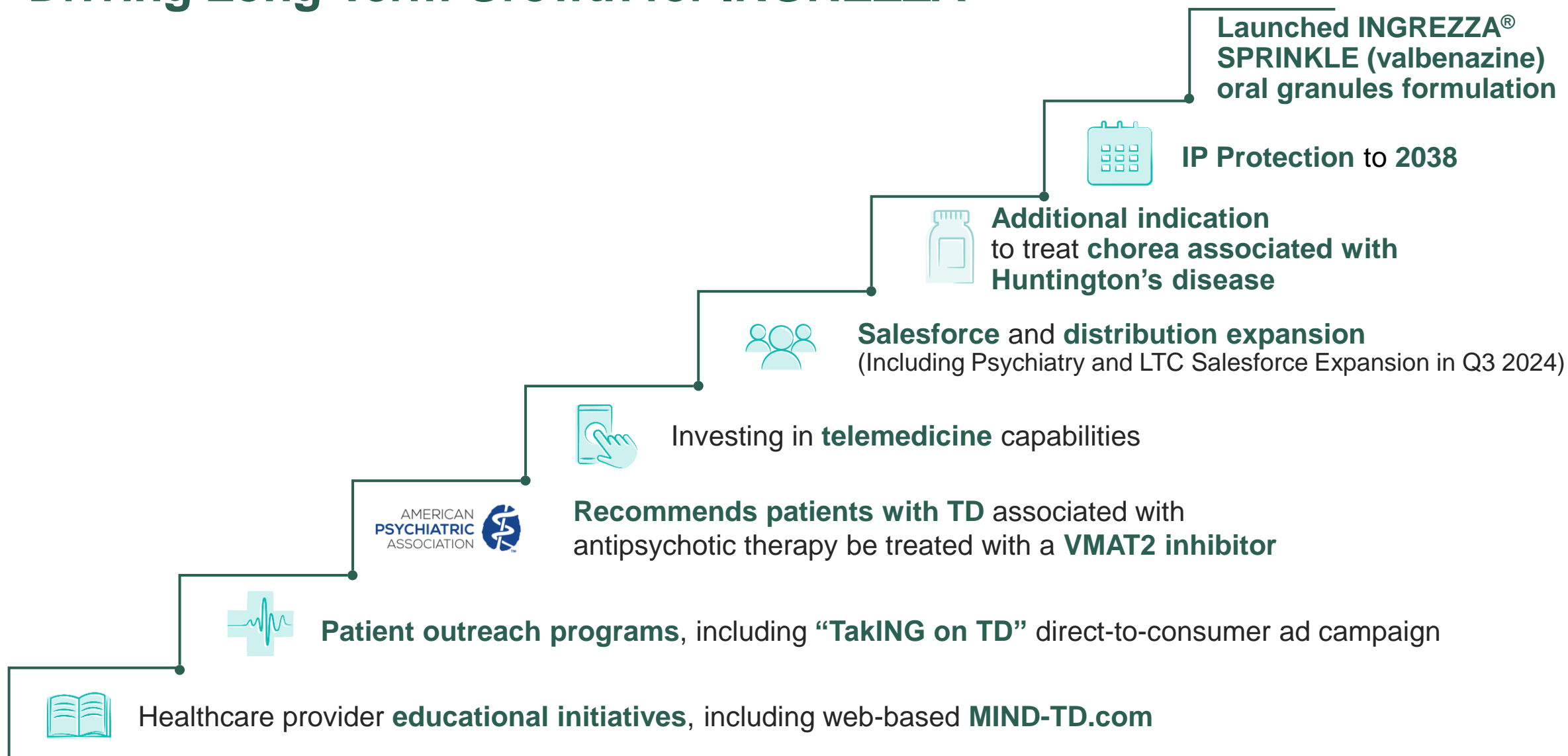


With or without food



Can be added to most stable mental health regimens

# 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 first half of 2025

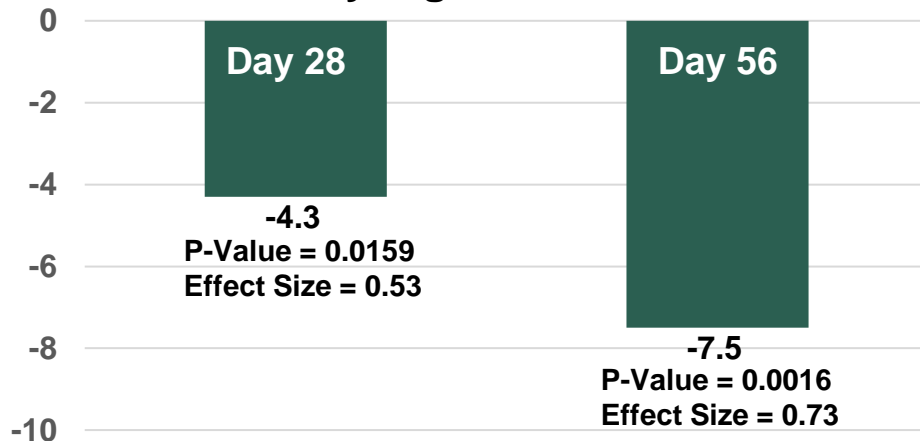


# NBI-1065845\* (AMPA Potentiator): SAVITRI™ Phase 2 Study Summary Results

## 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).

### Least Squares Mean Change From Baseline in MADRS for Statistically Significant NBI-'845 Dose

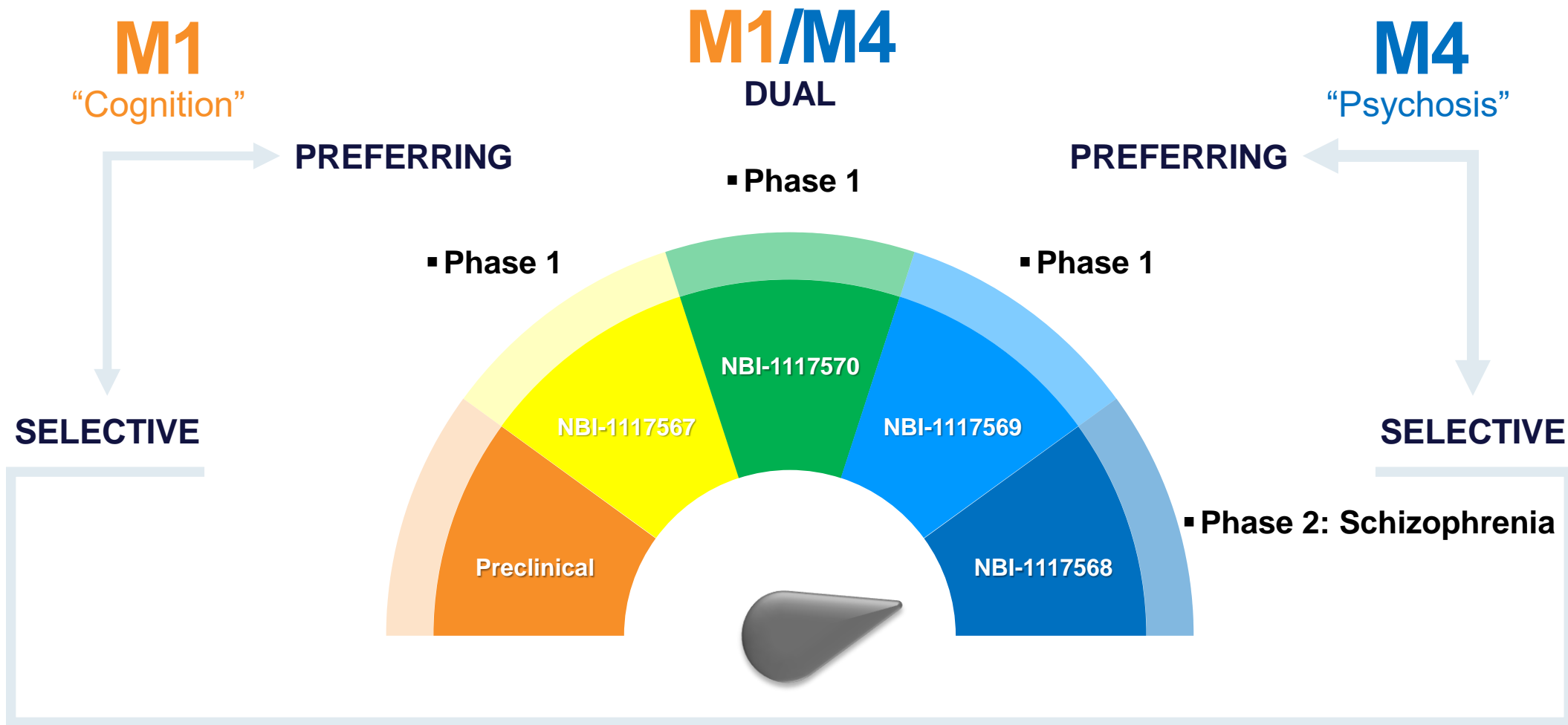


## SAFETY AND TOLERABILITY

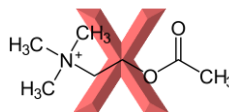
- NBI-1065845 was generally well-tolerated
- Most common adverse event was headache, of which, a majority were transient and mild in severity
- Adverse event profile for both doses of NBI-1065845 were comparable to placebo
- No seizures, deaths, or serious adverse events
- No psychotomimetic or dissociative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

# Muscarinic Platform Includes Multiple Clinical Programs

*From M1 to M4 Selective Orthosteric Agonists*



Combination Therapy to  
Block Off-target Effects  
Avoided



Acetylcholine Cooperativity  
Not Required

# 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 positive Phase 2 proof-of-concept results**

✓ 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)

✓ NBI-1117570\* (dual M1 / M4 agonist)

} for central nervous system disorders

✓ NBI-1076986 (M4 antagonist) for movement disorders

# Summary of Phase 2 Topline Results

Once-Daily 20mg Dose: Efficacy, Safety, and Tolerability Results Support Advancement to Phase 3

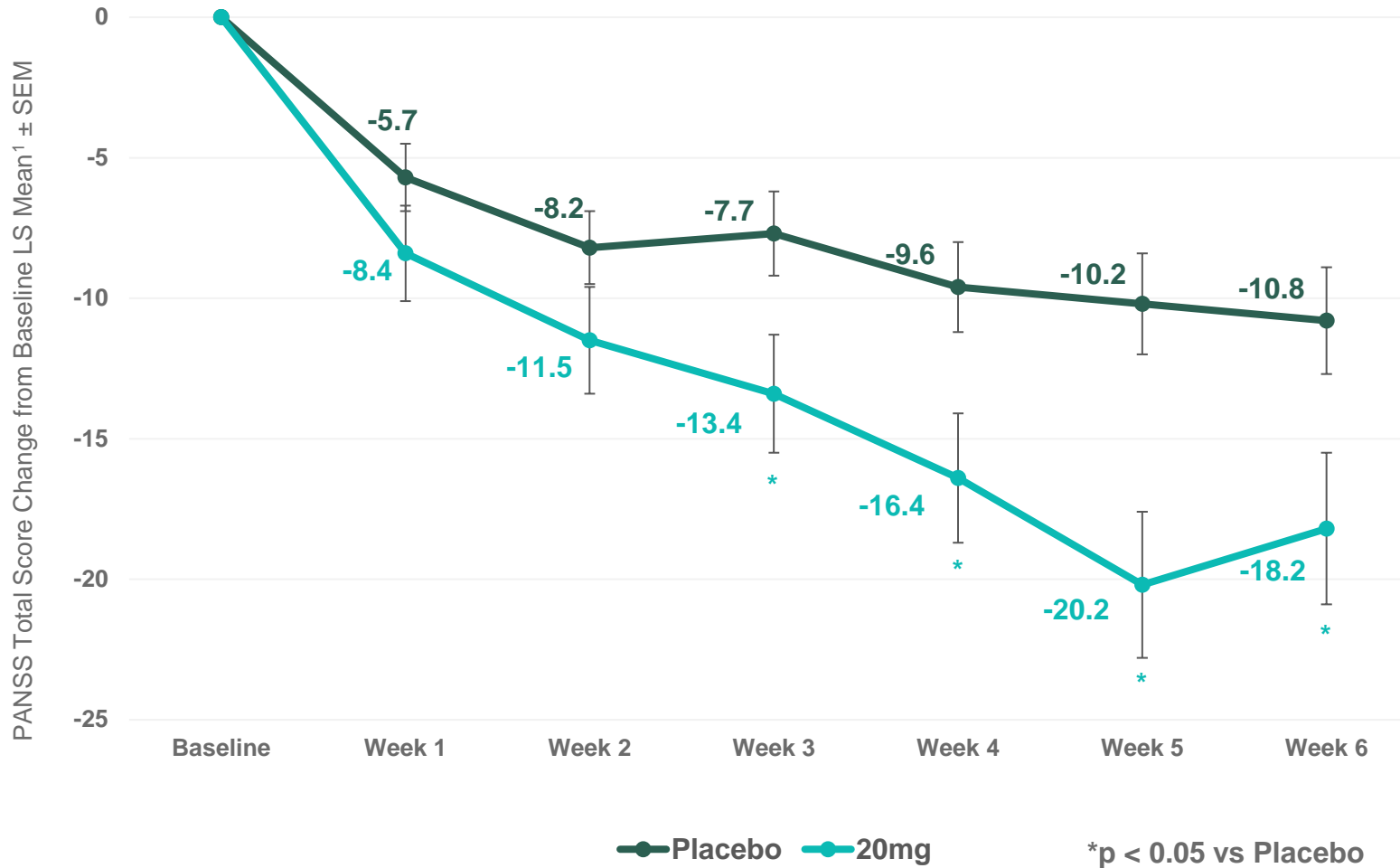
20mg Once-daily Demonstrated Statistically Significant and Clinically Meaningful Improvements Across Primary and Additional Endpoints

Generally Safe and Well-tolerated Across All Doses Tested

Efficacy, Safety and Tolerability Profile Combined With Once-daily Dosing Supports Advancement to Phase 3 Development

- **PANSS Total Score Change: -18.2**
- **PANSS Total Score Change vs. Placebo: -7.5 (p=0.011)**
- **Effect Size: 0.61**
- **CGI-S Change vs. Placebo: -0.7 (p<0.001)**
- **Marder Factor Score Change vs. Placebo:**
  - **Positive: -3.0 (p=0.004)**
  - **Negative: -1.9 (p=0.028)**
- Treatment discontinuation rates due to adverse events were similar between NBI-'568 and placebo
- Adverse events with the highest incidence were somnolence, dizziness, and headache
- Nausea, constipation and other gastrointestinal adverse events were low in frequency and similar to placebo
- NBI-'568 was not associated with a greater increase in weight than placebo
- NBI-'568 Phase 3 program in Schizophrenia expected to begin in early 2025
- Evaluating additional indications for NBI-'568
- Advancing follow-on compounds in muscarinic agonist portfolio

# Once-Daily 20mg Dose Demonstrated Clinically Meaningful and Statistically Significant Efficacy at Week 3, 4, 5, and 6



20mg QD Efficacy Data Week 4 – Week 6			
Week	4	5	6**
<b>PANSS Total Score</b>			
<b>LS Mean<sup>1</sup></b>	-16.4	-20.2	-18.2
<b>LS Mean Difference vs. Placebo<sup>1</sup></b>	-6.8 p = 0.008	-10.0 p < 0.001	-7.5 p = 0.011
<b>Effect Size<sup>2</sup></b>	0.53	0.72	0.61



# Once-Daily 20mg Dose Demonstrated Statistically Significant Improvement in Additional Endpoints

Week 6	CGI-S		Marder Factor — Positive		Marder Factor — Negative	
	Placebo N=68	20mg QD N=35	Placebo N=68	20mg QD N=35	Placebo N=68	20mg QD N=35
LS Mean Change from Baseline*	-0.5	-1.2	-2.8	-5.8	-1.2	-3.1
LS Mean Difference vs. Placebo*		-0.7 p < 0.001		-3.0 p = 0.004		-1.9 p = 0.028

# NBI-'568 Was Generally Safe and Well Tolerated at All Doses Studied

Treatment-Emergent Adverse Events Occurring in  $\geq 5\%$  of NBI-'568 All Treated Group

	Placebo N=70	20mg QD N=40	40mg QD N=39	60mg QD N=34	30mg BID N=27	All Treated N=140
<b>Somnolence</b>	2 (2.9)	5 (12.5)	2 (5.1)	7 (20.6)	1 (3.7)	15 (10.7)
<b>Dizziness</b>	1 (1.4)	5 (12.5)	3 (7.7)	4 (11.8)	1 (3.7)	13 (9.3)
<b>Headache</b>	14 (20.0)	1 (2.5)	5 (12.8)	1 (2.9)	5 (18.5)	12 (8.6)
<b>Nausea</b>	2 (2.9)	2 (5.0)	3 (7.7)	3 (8.8)	0	8 (5.7)
<b>Constipation</b>	2 (2.9)	2 (5.0)	3 (7.7)	1 (2.9)	1 (3.7)	7 (5.0)

**5.0% Treatment Discontinuation Rate Due to Adverse Events  
Across All NBI-'568 Arms vs. 4.3% For Placebo**

# NBI-'568 is the First and Only Muscarinic M4 Selective Orthosteric Agonist in Clinical Development

Type of Muscarinic Activation	Subtype Selectivity	Requires Endogenous Ligand (Acetylcholine)
Pan Agonism	<b>Low</b> Targets M1-M5	<b>No</b>
Positive Allosteric Modulation	<b>High</b> Targets only M4	<b>Yes</b>
<b>Selective Agonism (NBI-'568)</b>	<b>High</b> Targets only M4 >500-fold agonist selectivity for the M4 receptor over other muscarinic receptors	<b>No</b>

## Large Opportunity For NBI-'568, A Novel And Differentiated Asset



With no reliance on innate acetylcholine levels, NBI-'568 is the **first and only highly selective orthosteric M4 agonist**, potentially introducing a **new modality for treatment**.



NBI-'568 potentially offers a compelling and competitive benefit-risk profile



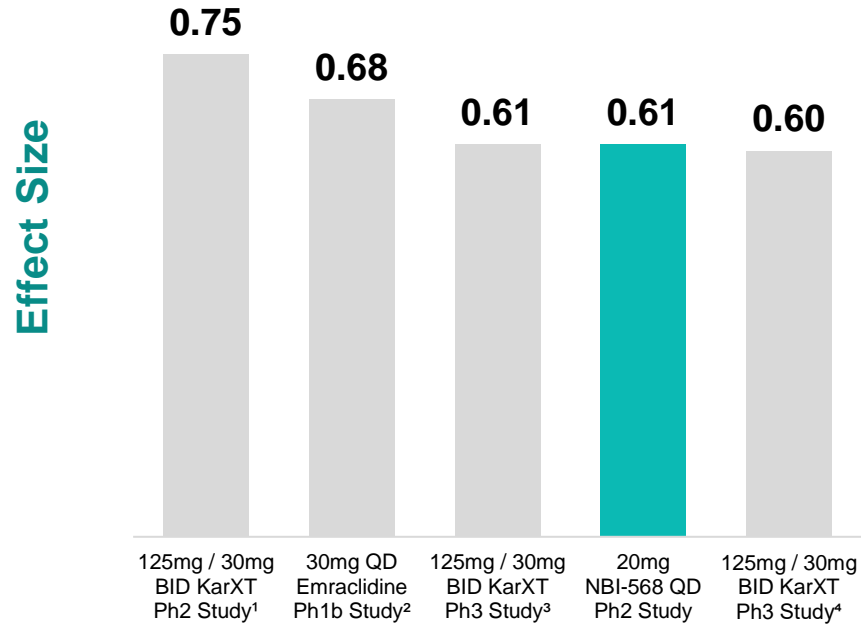
Convenience of **once-daily dosing with or without food**



Increased conviction in **indication expansion opportunities** for NBI-'568 and Neurocrine's muscarinic portfolio

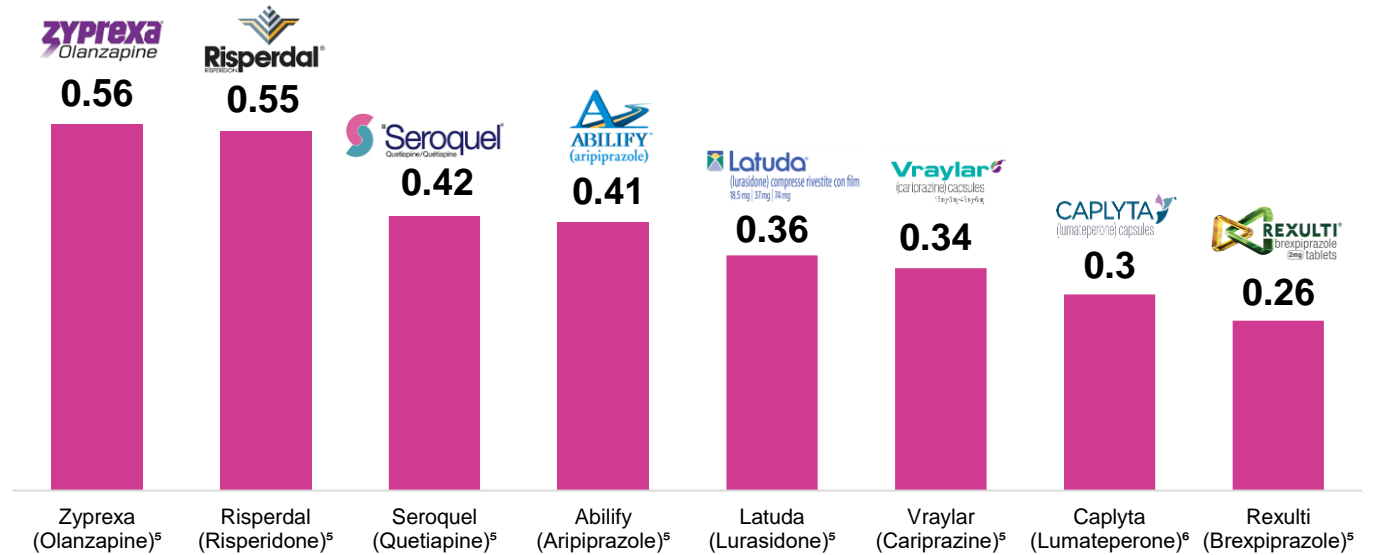
# NBI-'568 Effect Size Comparable to Known Muscarinic Programs and Leading Antipsychotics

## Clinical-Stage Muscarinic Programs



Sites	12	5	22	15	30
Randomization Ratio (active:placebo)	1:1	2:1	1:1	2:1	1:1
Weeks of Treatment	5	6	5	6	5
Date	Nov '19	Jun '21	Aug '22	Aug '24	Mar '23

## Leading Approved Treatments



Source: 1. Brannan S, et al. N Engl J Med. 2021;384(8):717-726. 2. Krystal J, et al. Lancet. 2022;400(10369):2210-2220. 3. Kaul I, et al. Lancet. 2024;403(10422):160-170. 4. Kaul I, et al. JAMA Psychiatry. 2024;81(8):749-756. 5. Huhn M, et al. Lancet. 2019;394(10202):939-951. 6. Correll CU, et al. JAMA Psychiatry. 2020;77(4):349-358.

# Validation of Selective Orthosteric Agonist Mechanism Strengthens Conviction In Opportunities For Industry Leading Muscarinic Portfolio

	Primary Mechanism (M1-M4)	Phase	Therapeutic Areas	Potential Areas For Development
NBI-1117568	M4 agonist	2	Psychosis Cognition	Alzheimer's Disease Bipolar Disorder Lewy Body Dementia Parkinson's Disease Schizophrenia
NBI-1117567	M1 agonist	1		
NBI-1117569	M4 agonist	1		
NBI-1117570	M1 / M4 dual agonist	1		
NBI-1076986	M4 antagonist	1	Movement Disorders	Dystonia Parkinson's Disease Tremor



# 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



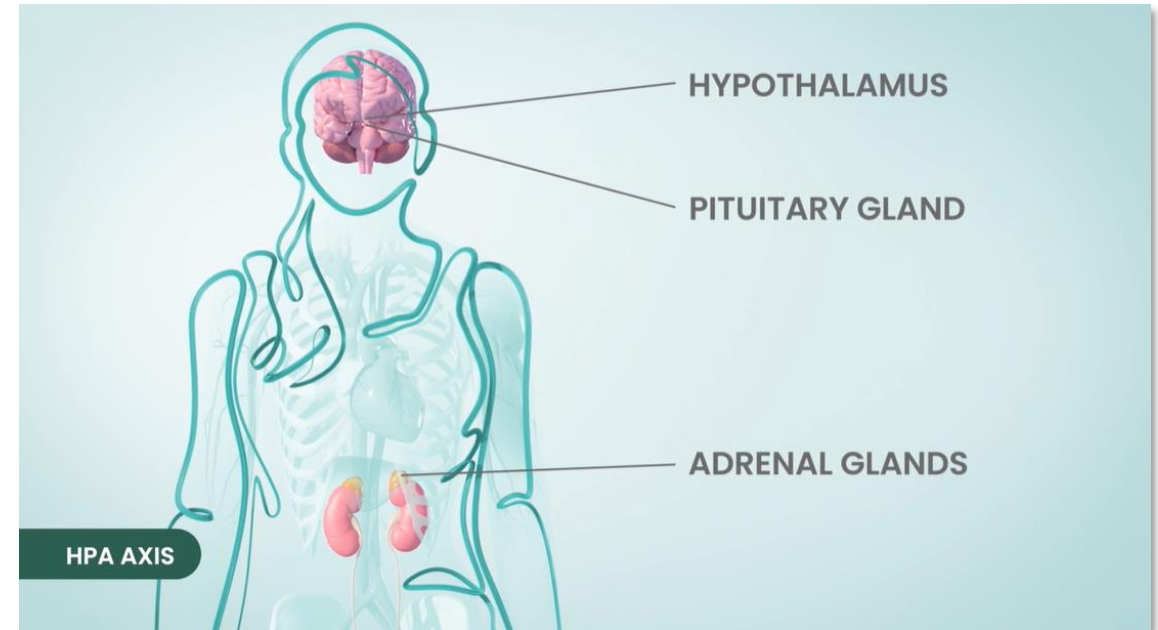
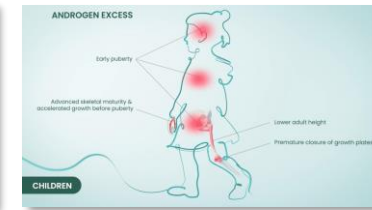
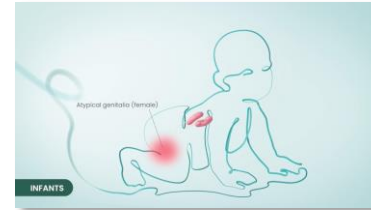
**Complex and Highly Variable Symptoms**



**Treatment Options Stagnant for 70 Years**



- Hormone replacement
- Do not address underlying issue



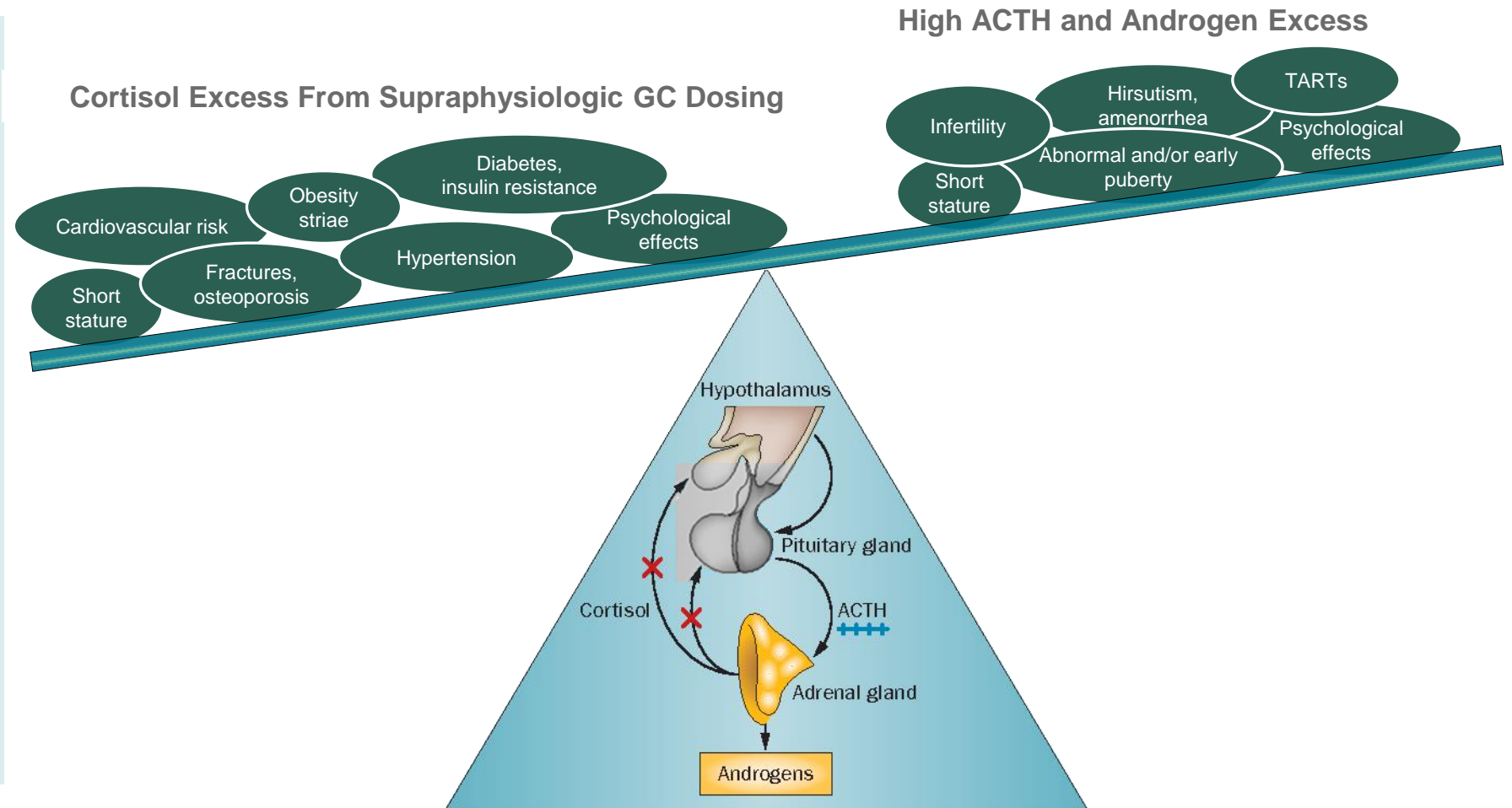
# Classic Congenital Adrenal Hyperplasia Due to 21-Hydroxylase Deficiency (21OHD CAH)

## 21OHD 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.

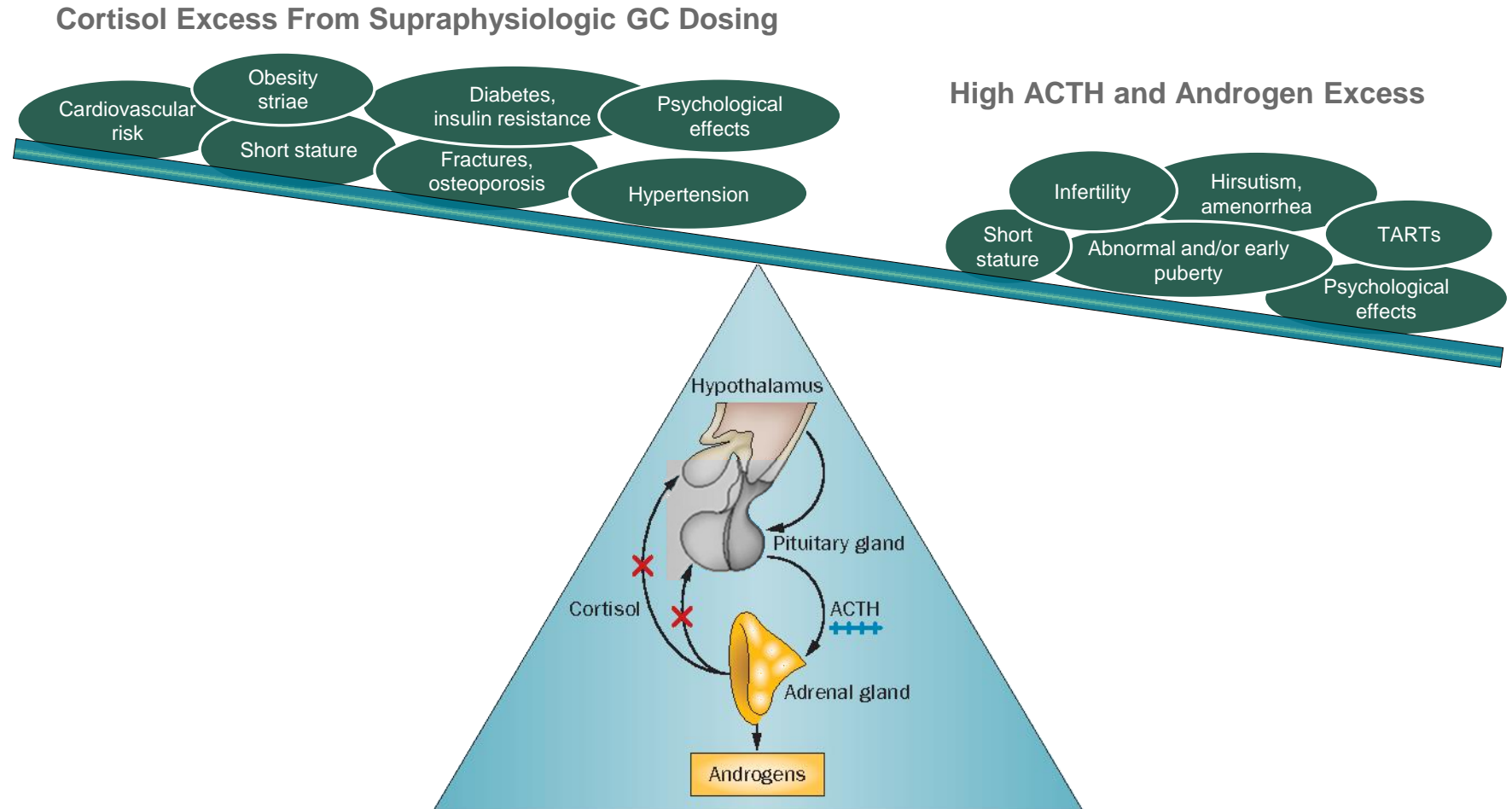
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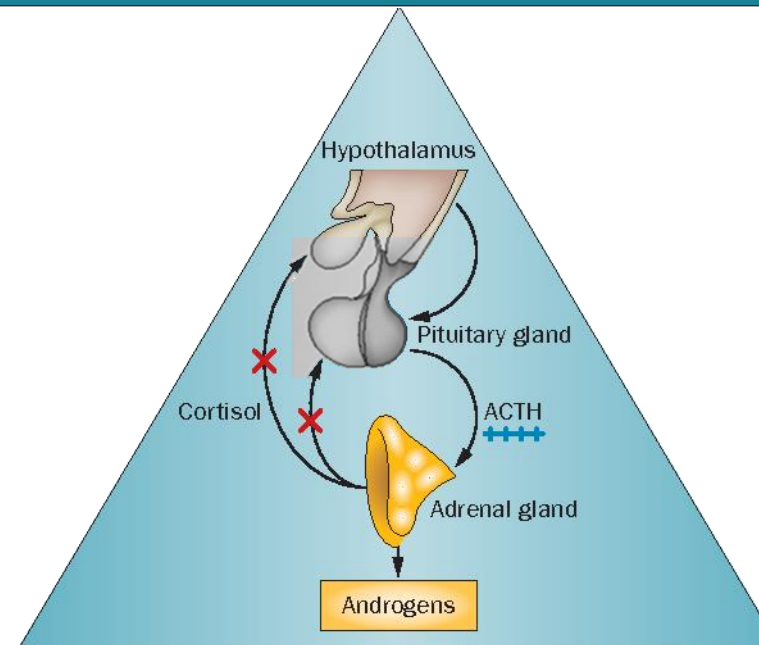
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Reduced GC Dosing

**crinecerfont**

Androgen Control



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

# CAHtalyst™ Adult and Pediatric Study Androgen Reduction

Study Characteristic	Percent Change* in Androstenedione at Week 4 (Following Glucocorticoid Stable Period)		Key Takeaways
	Adult Study (N = 182)	Pediatric Study (N = 103)	
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 Data Presented at ENDO in June and Published in the New England Journal of Medicine ([Link Here](#))

# CAHtalyst™ Adult and Pediatric Study Glucocorticoid Dose Reduction While Maintaining Androgen Control

CAHtalyst™ Trial Participants	Percent of Subjects Achieving a Glucocorticoid Daily Dose $\leq 11$ mg/m <sup>2</sup> /day While Maintaining Androgen Control		
	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 <u>with</u> 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)**

# CAHtalyst™ 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

- FDA Granted Breakthrough Therapy Designation and 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
- Open-Label Treatment Periods for the CAHtalyst™ 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 500K 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

## Commercial



**INGREZZA<sup>®</sup> \***  
(valbenazine) capsules

TARDIVE DYSKINESIA AND CHOREA  
ASSOCIATED WITH HUNTINGTON'S DISEASE  
2024 Annual

**\$2.30 - \$2.32**  
**Billion**

Net Sales Guidance Raised  
and Narrowed from  
\$2.25 - \$2.30 Billion

**~800,000**

Affected by Tardive  
Dyskinesia in the U.S.;  
~75% are undiagnosed

**~90%**

of the ~40,000 People in  
the U.S. Diagnosed with  
Huntington's Disease Who  
Will Develop Chorea

## R&D Focus

Neurology  
Neuroendocrinology  
Neuropsychiatry

Robust Pipeline

Multiple Compounds in  
Mid- to Late-Stage Studies

Rapidly Growing Early-  
Stage Portfolio

## Strong Financial Position

**~\$1.9B**

Cash and Investments  
as of 9/30/2024

Strong Balance Sheet

Durable Cash Flows

Attractive P&L Profile





# GAAP to Non-GAAP Reconciliations

[neurocrine.com](https://www.neurocrine.com)

**NEUROCRINE BIOSCIENCES, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF INCOME**  
**(unaudited)**

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
<i>(in millions, except per share data)</i>				
Revenues:				
Net product sales	\$ 616.6	\$ 491.8	\$ 1,709.4	\$ 1,353.4
Collaboration revenue	5.5	7.0	18.2	18.5
Total revenues	<u>622.1</u>	<u>498.8</u>	<u>1,727.6</u>	<u>1,371.9</u>
Operating expenses:				
Cost of revenues	8.0	11.2	24.7	31.2
Research and development	195.0	142.2	545.5	427.5
Acquired in-process research and development	1.0	—	9.5	143.9
Selling, general, and administrative	234.3	204.2	719.4	668.7
Total operating expenses	<u>438.3</u>	<u>357.6</u>	<u>1,299.1</u>	<u>1,271.3</u>
Operating income	<u>183.8</u>	<u>141.2</u>	<u>428.5</u>	<u>100.6</u>
Other income (expense):				
Unrealized loss on equity investments	(16.9)	(40.1)	(35.2)	(0.6)
Charges associated with convertible senior notes	—	—	(138.4)	—
Investment income and other, net	23.4	14.5	68.5	33.9
Total other income (expense), net	<u>6.5</u>	<u>(25.6)</u>	<u>(105.1)</u>	<u>33.3</u>
Income before provision for income taxes	<u>190.3</u>	<u>115.6</u>	<u>323.4</u>	<u>133.9</u>
Provision for income taxes	<u>60.5</u>	<u>32.5</u>	<u>85.2</u>	<u>31.9</u>
Net income	<u>\$ 129.8</u>	<u>\$ 83.1</u>	<u>\$ 238.2</u>	<u>\$ 102.0</u>
Earnings per share, basic	\$ 1.28	\$ 0.85	\$ 2.37	\$ 1.05
Earnings per share, diluted	\$ 1.24	\$ 0.82	\$ 2.29	\$ 1.01
Weighted average common shares outstanding, basic	101.1	97.9	100.6	97.5
Weighted average common shares outstanding, diluted	104.3	101.1	104.0	100.6

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

<i>(in millions)</i>	September 30, 2024	December 31, 2023
Cash, cash equivalents, and marketable securities	\$ 1,228.0	\$ 1,031.6
Other current assets	648.6	575.4
Total current assets	<u>1,876.6</u>	<u>1,607.0</u>
Deferred tax assets	454.4	362.6
Marketable securities	643.9	687.5
Right-of-use assets	257.3	276.5
Equity investments	126.7	161.9
Property and equipment, net	80.0	70.8
Intangible assets, net	34.5	35.5
Other noncurrent assets	61.6	49.6
Total assets	<u>\$ 3,535.0</u>	<u>\$ 3,251.4</u>
Convertible senior notes	\$ —	\$ 170.1
Other current liabilities	429.7	484.7
Total current liabilities	<u>429.7</u>	<u>654.8</u>
Noncurrent operating lease liabilities	251.4	258.3
Other noncurrent liabilities	135.0	106.3
Stockholders' equity	2,718.9	2,232.0
Total liabilities and stockholders' equity	<u>\$ 3,535.0</u>	<u>\$ 3,251.4</u>



**NEUROCRINE BIOSCIENCES, INC.**  
**RECONCILIATION OF GAAP TO NON-GAAP FINANCIAL RESULTS**  
**(unaudited)**

<i>(in millions, except per share data)</i>	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
GAAP net income <sup>1</sup>	\$ 129.8	\$ 83.1	\$ 238.2	\$ 102.0
Adjustments:				
Stock-based compensation expense - R&D	14.8	17.2	47.6	54.8
Stock-based compensation expense - SG&A	26.7	30.6	81.5	101.4
Charges associated with convertible senior notes <sup>2</sup>	—	—	138.4	—
Vacated legacy campus facility costs, net of sublease income <sup>3</sup>	3.0	—	17.0	—
Non-cash amortization related to acquired intangible assets	0.9	0.9	2.7	2.7
Changes in fair values of equity investments <sup>4</sup>	16.9	40.1	35.2	0.6
Other	—	4.1	0.3	4.5
Income tax effect related to reconciling items <sup>5</sup>	(2.9)	(19.9)	(78.0)	(33.7)
Non-GAAP net income <sup>1</sup>	\$ 189.2	\$ 156.1	\$ 482.9	\$ 232.3
Diluted earnings per share:				
GAAP	\$ 1.24	\$ 0.82	\$ 2.29	\$ 1.01
Non-GAAP	\$ 1.81	\$ 1.54	\$ 4.64	\$ 2.31

1. Three and nine months ended September 30, 2024 reflect \$38.8 million and \$71.4 million, respectively, of expense for development milestones achieved under collaborations. Nine months ended September 30, 2024 reflects IPR&D expense of \$9.5 million. Nine months ended September 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 and other costs associated with our vacated legacy campus facilities, net of sublease income, as we transition to occupy our new campus facility.
4. Reflects periodic fluctuations in the fair values of equity 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 September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
<i>(in millions)</i>				
GAAP cost of revenues	\$ 8.0	\$ 11.2	\$ 24.7	\$ 31.2
Adjustments:				
Non-cash amortization related to acquired intangible assets	0.9	0.9	2.7	2.7
Non-GAAP cost of revenues	<u>\$ 7.1</u>	<u>\$ 10.3</u>	<u>\$ 22.0</u>	<u>\$ 28.5</u>

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
<i>(in millions)</i>				
GAAP R&D	\$ 195.0	\$ 142.2	\$ 545.5	\$ 427.5
Adjustments:				
Stock-based compensation expense	14.8	17.2	47.6	54.8
Non-GAAP R&D	<u>\$ 180.2</u>	<u>\$ 125.0</u>	<u>\$ 497.9</u>	<u>\$ 372.7</u>

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
<i>(in millions)</i>				
GAAP SG&A	\$ 234.3	\$ 204.2	\$ 719.4	\$ 668.7
Adjustments:				
Stock-based compensation expense	26.7	30.6	81.5	101.4
Vacated legacy campus facility costs, net of sublease income	3.0	—	17.0	—
Other	—	3.9	—	3.9
Non-GAAP SG&A	<u>\$ 204.6</u>	<u>\$ 169.7</u>	<u>\$ 620.9</u>	<u>\$ 563.4</u>

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
<i>(in millions)</i>				
GAAP other income (expense), net	\$ 6.5	\$ (25.6)	\$ (105.1)	\$ 33.3
Adjustments:				
Charges associated with convertible senior notes	—	—	138.4	—
Changes in fair values of equity investments	16.9	40.1	35.2	0.6
Other	—	0.2	0.3	0.6
Non-GAAP other income, net	<u>\$ 23.4</u>	<u>\$ 14.7</u>	<u>\$ 68.8</u>	<u>\$ 34.5</u>

# Advancing Life-Changing Discoveries in Neuroscience

Q3 2024

Corporate Presentation

October 30, 2024

Nasdaq: NBIX



You Deserve *Brave* Science™

