# J.P. Morgan Healthcare Conference

January 9, 2024





#### Safe Harbor Statement

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 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 U.S. federal or state legislative or regulatory and/or policy efforts which may result in, among other things, an adverse impact on our revenues or potential revenue; risks associated with 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 guarter ended September 30, 2023. Neurocrine Biosciences disclaims any obligation to update the statements contained in this presentation after the date hereof.





You Deserve Brave Science®



UNDERSTANDING THE BRAIN TO TREAT THE BODY



RELIEVING PATIENT SUFFERING

3

TAKING RESPONSIBILITY BEYOND MEDICINE







Relieve suffering for people with great needs, but few options





## **Multiple Commercial Products**

In the U.S.



TARDIVE DYSKINESIA

CHOREA-HUNTINGTON'S DISEASE



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



\*Mitsubishi Tanabe Pharma Corporation (MTPC) has commercialization rights in Japan and other select Asian markets †Under license from Bial ‡AbbVie has global commercialization rights

## Well-positioned for sustained and long-term growth

Commerc	ial	R&D Focus	Strong Financial Position			
TD AND T \$1.82B - \$1.84B	<b>GREZZA®</b> nazine) capsules HD CHOREA 2023 Annual Net Sales Guidance	<ul> <li>Neurology</li> <li>Neuroendocrinology</li> <li>Neuropsychiatry</li> </ul>	~\$1.5B Cash and Investments as of 9/30/2023			
~600,000	Affected by Tardive Dyskinesia (TD) in the U.S.; <b>65%</b> are undiagnosed	Multiple Compounds in Mid- to Late-Stage Studies	Durable Cash Flows			
~90% of the 41,000 People in the U.S. Diagnosed with Huntington Disease Will Develop Chorea		Rapidly Growing Early- Stage Portfolio	Auacuver de l'ionie			



### 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** 





# of Programs by Stage

Phase 3 Phase 1 Phase 2 8 4

			Phase 1	Phase 2	Phase 3	NDA	Milestone	
Neurology								
valbenazine*	Sprinkle Formulation	VMAT2 Inhibitor				•	PDUFA: 4/30/2024	
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					Submitted CTA	
Neuroendocrinology								
crinecerfont <sup>4</sup>	CAH: Adults	CRF-R1					NDA: 2024	
crinecerfont <sup>4</sup>	CAH: Pediatrics	CRF-R1					NDA: 2024	
Efmody	Adrenal Insufficiency	GC Receptor					Phase 2 Data: 1H '24	
Efmody	CAH	GC Receptor					Phase 2 Data: 1H '24	

#### Neuropsychiatry

valbenazine*	ATS	VMAT2 Inhibitor	Phase 3 Ongoing
NBI-1065845 <sup>5</sup>	Inadequate Response-MDD	AMPA	Phase 2 Data: 1H '24
luvadaxistat <sup>5</sup>	CIAS	DAAO	Phase 2 Data: 2H '24
NBI-1117568 <sup>1</sup>	Schizophrenia	M4 Agonist	Phase 2 Data: 2H '24
NBI-1070770 <sup>5</sup>	MDD	NMDA NR2B NAM	Phase 2 Initiating
NBI-1117570 <sup>1</sup>	CNS Indications	M1/M4-Dual	Phase 1 Ongoing
NBI-1117569 <sup>1</sup>	CNS Indications	M4-Preferring	Phase 1 Ongoing
NBI-1117567 <sup>1†</sup>	CNS Indications	M1-Preferring	Phase 1 Initiating
NBI-1065890	CNS Indications	VMAT2 Inhibitor	Submitted CTA



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In-licensed program = (1) Sosei Heptares (2) Idorsia Ltd (3) Xenon Pharmaceuticals Inc (4) Sanofi (5) Takeda Pharmaceutical Company Ltd Neurocrine Biosciences has global rights unless otherwise noted.

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# of Programs by Stage

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 5
 8
 4
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NBI-1076986							
Neuroendocrinol	ogy						
crinecerfont <sup>4</sup>							
crinecerfont <sup>4</sup>	CAH: Pediatrics	CRF-R1					NDA: 2024
Efmody	Adrenal Insufficiency	GC Receptor					Phase 2 Data: 1H '24
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# Congenital Adrenal Hyperplasia

#### 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.



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High ACTH and Androgen Excess

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Mallappa A and Merke DP. *Nat Rev Endocrinol.* 2022;43(1):91-159. ACTH, adrenocorticotropic hormone; GC, glucocorticoids; TARTs, testicular adrenal rest tumors.



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## **Key Milestones in 2024 and Longer Term**

#### 2024

- April PDUFA for Valbenazine Oral Granules Sprinkle Formulation
- Submitting New Drug Application for Crinecerfont in Adult and Pediatric CAH Indications
- Advancing Five Phase 1 Programs including Four Muscarinic Compounds and next generation VMAT2 Inhibitor
- Anticipating Top-Line Data for Five Phase 2 Programs
  - Efmody for Adrenal Insufficiency and CAH in 1H
  - NBI-'845 (AMPA Potentiator) for Inadequate Response to Treatment in Major Depressive Disorder in 1H
  - NBI-'568 (M4 Agonist) for Schizophrenia in 2H
  - Luvadaxistat (DAAO Inhibitor) for Cognitive Impairment Associated with Schizophrenia in 2H
- Developing Sustainable R&D Engine

#### Longer Term

- Anticipated Crinecerfont Approved in U.S. (2025) and in Europe (2026)
- Advancement of 2 Gene Therapy Programs Into Clinical Development (2025)
- Advancement of 20 Development Candidates (Through 2027)
- Sustainable R&D Engine Generating 1-2 Mid-to-Late-Stage Clinical Read-Outs Every Year
- Clinical Pipeline
  - Weighted Towards Neurology vs.2023
  - Balanced Between Biologics and Small Molecules
  - Focused on Clinically and / or Genetically Validated Targets
  - Majority of Candidates Considered "Next" or "Best-In-Class"
- INGREZZA® (valbenazine) Market Exclusivity Into 2038



# What Brave science can mean to patients







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