Advancing Life-Changing Discoveries in Neuroscience

Kevin Gorman, CEO

38th Annual J.P. Morgan Healthcare Conference
January 13, 2020

NASDAQ: NBIX
Safe Harbor Statement

In addition to historical facts, this press release contains forward-looking statements that involve a number of risks and uncertainties. These statements include, but are not limited to, statements related to: our preliminary unaudited financial information; the benefits to be derived from our products and product candidates, including INGREZZA and our partnered product, ORILISSA; the value INGREZZA, ORILISSA, and/or our product candidates may bring to patients; the continued success of the launch of INGREZZA; AbbVie’s launch of ORILISSA; the opicapone NDA; our financial and operating performance, including our future expenses; our collaborative partnerships; and the timing of 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: risks and uncertainties associated with items that may be identified during the financial statement closing process that cause adjustments to the estimates included in this press release; our future financial and operating performance; risks associated with the commercialization of INGREZZA and ORILISSA; risks that the opicapone NDA may not obtain regulatory approval from the FDA or such approval may be delayed or conditioned; risks related to the development of our product candidates; risks associated with our dependence on third parties for development and manufacturing activities related to INGREZZA and our 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 associated with our dependence on AbbVie for the commercialization of ORILISSA and the continued development of elagolix; risks associated with our dependence on BIAL for development and manufacturing activities related to opicapone, and our ability to manage BIAL; risks that clinical development activities may not be 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; and other risks described in our periodic reports filed with the SEC, including without limitation our quarterly report on Form 10-Q for the quarter ended September 30, 2019. Neurocrine disclaims any obligation to update the statements contained in this press release after the date hereof.
25-Year Legacy of Delivering on Hope for Patients

Neuroscience-based biopharmaceutical company

Focus on discovering novel treatments for diseases with significant clinical need

Unique expertise in neurology, neuroendocrinology and neuropsychiatry

Passion for delivering on hope for patients who may feel invisible, unheard and marginalized
Proven Approach to Delivering on Hope for Patients

Expanding potential new indications for patients

Valbenazine:
- Chorea in Huntington Disease

Elagolix:
- Uterine Fibroids
- Polycystic Ovary Syndrome

Highly effective commercial organization to deliver treatments to patients

Valbenazine:
- Most prescribed & most-preferred therapy for tardive dyskinesia
- ~132,700 prescriptions filled in 2019

Elagolix:
- Treatment of pain associated with endometriosis

Discovering life-changing medicines for diseases with significant clinical need

Dynamic R&D Engine Since 1997
- 25+ Investigational New Drugs (INDs) Filed
- 125+ clinical studies in 10,000+ participants

Key Development Programs
- Congenital Adrenal Hyperplasia (CAH)
- Parkinson’s Disease
- Epilepsy

New Modalities
- Gene Therapy
- Ion Channel Inhibitors

* AbbVie has global commercialization rights
## Diversified Portfolio with Multi-Stage Programs

<table>
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<tr>
<th>PROGRAM</th>
<th>THERAPEUTIC AREA</th>
<th>PHASE 1</th>
<th>PHASE 2</th>
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</table>

Neurocrine Biosciences has global rights unless otherwise noted.

* Mitsubishi Tanabe Pharma has commercialization rights in East Asia
† AbbVie has global commercialization rights
‡ BIAL retains commercialization rights outside U.S. and Canada

Voyager Therapeutics has co-commercialization option for U.S. market following the ongoing Phase II RESTORE-1 study
§ Neurocrine Biosciences has the exclusive option to license from Idorsia
2020: Expand Potential Indications and Advance Clinical Programs

3 Approved Medicines in 4 Indications

INGREZZA® (valbenazine) for Tardive Dyskinesia
ORILISSA® (elagolix) for Endometriosis*
Opicapone for Parkinson's Disease
Elagolix for Uterine Fibroids*

3 Pivotal Clinical Trial Programs

Global Registrational-Enabling Study for CAH‡ (adults)
Registrational-Enabling Studies for NB Ib-1817 in Parkinson’s Disease
Phase III Study of Valbenazine for Chorea in Huntington Disease

5 Early-to-Mid Stage R&D Programs

Advancement of Phase IIa Study of Crinecerfont in CAH (pediatric)
Initiation of Phase II Study of NBI-921352 in SCN8A-DEE¶
Initiation of Phase II Study of ACT-709478 in Epilepsy§
Phase II Study of Elagolix in PCOS*‖
New VMAT2 Inhibitor

* AbbVie has global commercial rights
† PDUFA (Prescription Drug User Fee Act) Target Action Date
‡ CAH (Congenital Adrenal Hyperplasia)
§ Neurocrine Biosciences has the exclusive option to license from Idorsia
¶ SCN8A – DEE (SCN8A Developmental and Epileptic Encephalopathy)
‖ PCOS (Polycystic Ovary Syndrome)
Our Medicines
Our Patients
1st FDA-approved Treatment for Adults with Tardive Dyskinesia (TD) – Launched in 2017

Most-Prescribed and Most-Preferred TD Therapy

- Rapid Improvement in Involuntary Movements
- Generally Well Tolerated
- Ease-of-Use: One Capsule, Once daily
Tardive Dyskinesia (TD): An Overview

TD is a movement disorder characterized by uncontrollable, abnormal and repetitive movements of the face, torso and/or other body parts, which may be disruptive & negatively impact patients.

1 in 5
U.S. adults live with a mental illness.

TD, one of the challenges associated with mental illness, is estimated to affect at least
500,000 people in the U.S.

TD can look different day-to-day. Symptoms can be severe and are often persistent and irreversible.

TD is caused by prolonged use of antipsychotics, commonly prescribed to treat schizophrenia, bipolar disorder & depression, & certain anti-nausea medications.

According to a survey of patients with TD, the condition affects their:

- Ability to sleep 61%
- Self-esteem 68%
- Ability to exercise 39%
INGREZZA Continues to Exceed Expectations

INGREZZA Net Sales and ~TRx

**INGREZZA Net Product Sales ($ in Millions)**

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* Q4 2019 are preliminary, unaudited Net Product Sales
Valbenazine: Chorea in Huntington Disease
Phase III Study to Treat Chorea in Adult Patients with Huntington Disease Ongoing

Chorea in Huntington Disease (HD)
An involuntary movement disorder estimated to affect approximately 90% of the 30,000 HD patients in the U.S. HD is a rare neurodegenerative disorder in which neurons within the brain break down. Patients with chorea in HD develop abnormal, abrupt or irregular movements.

Common symptoms of chorea can affect various body parts and interfere with speech, swallowing, posture and gait.

Need for chorea treatment options with better safety profile as current treatments are associated with increased risk of depression and suicidality.

Valbenazine*

- Targeted symptom control of chorea movements as measured by the Unified Huntington Disease Rating Scale (UHDRS) and Total Maximal Chorea (TMC)
- Promising safety profile without troublesome side effects
- Phase III study initiated in November 2019 with expected completion in 2021

* Valbenazine in Huntington disease is investigational and not approved in the U.S.
1st FDA-approved oral treatment for women with moderate-to-severe endometriosis pain in over a decade - Launched in 2018

- Less Estrogen = Less Painful Endometriosis Legions
  Addresses three most common types of endometriosis pain: painful periods; pelvic pain between periods; pain with sex*

- Oral Administration
  2 dosage options based on severity of symptoms and treatment objectives

- Safety & Tolerability Profile
  Proven efficacy & safety in largest endometriosis clinical program

* There are two different types of ORILISSA: 150 mg (taken once a day) or 200 mg (taken twice a day). Only the 200 mg dose was proven to work for pain with sex.
Elagolix*: Potential Expanded Indications in Women’s Health

### Uterine Fibroids
NDA Submitted with PDUFA in Q2 2020

- **7 million** women with symptomatic uterine fibroids
- **2.8 million** women currently diagnosed
- **400,000** new diagnoses annually
- Most common pelvic growth affecting 70% - 80% of women by the age of 50
- **7 million** women with symptomatic uterine fibroids
- **2.8 million** women currently diagnosed
- **400,000** new diagnoses annually
- **1** drug approved by FDA in the past **20 years**
- Approximately **220,000** hysterectomies performed annually

### Polycystic Ovary Syndrome
Phase II Study Ongoing

- **3.5 million** women affected in the United States
- No FDA-approved treatment options
- **$1.6B** in annual healthcare costs

*AbbVie has global commercial rights*
Opicapone* for Parkinson’s Disease
Target PDUFA Date of April 26, 2020

Parkinson’s Disease (PD)

1 million people impacted in the U.S.

2 out of 3 patients are on carbidopa/levodopa (standard-of-care)

Standard-of-care loses effectiveness over time requiring dose and frequency escalation to control symptoms

Current adjunctive treatments have limited efficacy and tolerability

Opicapone†

- Novel COMT inhibitor as adjunctive therapy to levodopa/carbidopa in patients with Parkinson’s disease experiencing OFF episodes
- Significant and sustained reduction of daily OFF time and increase of ON time without troublesome dyskinesia
- Once-a-day dosing with no titration needed
- Generally well tolerated – no signal of liver toxicity or diarrhea
- Approved in the EU since 2016‡

* In-licensed from BIAL in 2017
† Opicapone is investigational and not approved in the U.S.
‡ BIAL retains commercialization rights outside U.S. and Canada
Discovering life-changing medicines for diseases with significant clinical need

Key Development Programs
- Congenital Adrenal Hyperplasia (CAH)
- Parkinson’s Disease
- Epilepsy

New Modalities
- Gene Therapy
- Ion Channel Inhibitors
Crinecerfont*: Classic Congenital Adrenal Hyperplasia

Global Registrational-Enabling Study Initiation in Adults Planned for Mid-2020
Advancement of Phase IIa Pediatric Study Ongoing

**Crinecerfont**

- Potent, selective, orally-active, non-peptide corticotropin releasing factor type 1 (CRF1) receptor antagonist

*Crinecerfont is investigational and not approved in the U.S. or Europe*

### Congenital Adrenal Hyperplasia

- **Rare genetic disorder** caused by enzyme deficiency which leads to reduced adrenal steroids and excess androgen levels with up to **30,000** people impacted in the U.S. and a similar number in Europe

- **Complex and highly variable symptoms** including adrenal crisis, virilization, hirsutism, precocious puberty, fertility problems and abnormal growth

- **Excess corticosteroid treatment** leads to additional clinical problems including bone loss, Cushing’s disease and metabolic syndrome

### Overtreatment Hypercortisolism

- Cushingoid features
  - Central obesity
  - Osteoporosis
  - Insulin Resistance
  - Impaired Glucose Tolerance

### Undertreatment Hyperandrogenism

- Adrenal Crisis
  - Increased Adrenal Androgen
  - Hirsutism
  - Amenorrhea
  - Infertility

- Low Blood Pressure
  - Salt Loss
  - Fatigue, Lack of Energy
  - Increased Requirements for Glucocorticoid Replacement

### Glucocorticoid

- Hypertension

### Mineralocorticoid

- Cushing’s disease and metabolic syndrome

* J.P. Morgan Healthcare Conference 2020
Expanding Reach: Innovative Partners with Novel Science to Address Unmet Medical Need

**Gene Therapy**

Opportunity to Expand Footprint into Key Areas of Neuroscience with Novel Modalities

- **NBib-1817**\(^*\)
  for Parkinson’s disease

- Friedreich’s ataxia

- Two undisclosed CNS programs

**Precision Medicine**

Establish Strong Presence to Address Unmet Medical Needs in Epilepsy and Other Neurological Disorders

- **NBI-921352**\(^*\)
  for SCN8A-DEE (epilepsy)

- **ACT-709478**\(^{†}\)
  for rare pediatric epilepsy

* Investigational and not approved in the U.S.

† Neurocrine Biosciences has the exclusive option to license from Idorsia

Research collaboration

Research collaboration
**NBib-1817*: Gene Therapy for Parkinson’s Disease**
Planning to Start 2nd Registrational Study in 2020

**Moderate to Advanced PD**

- **One million** patients with PD in the U.S., with moderate to advanced stages of PD typically occurring four years after diagnosis

- **Loss of neurons and critical AADC enzyme** in the midbrain that produce dopamine leads to progressive loss of motor function and less responsiveness to levodopa

- **Severe, debilitating loss of motor function** including rigidity, postural instability, gait freezing and difficulty with speech and swallowing

**NBib-1817†**

- **One-time treatment** restores AADC enzyme activity and enhance the conversion of levodopa and restore motor function

- **Improvement in ON time and reduction in OFF time** at 1-year timepoint

- **>7-year shift in disease progression** seen at 1 year as measured by modified Hoehn and Yahr scale

- **Durable expression of the AADC enzyme** observed at 15-years post-administration in non-human primates

- **RESTORE-1 and RESTORE-2 studies**: Registrational-enabling study ongoing and 2nd planned for 2020

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* In-licensed from Voyager Therapeutics
† NBib-1817 is investigational and not approved in the U.S.
NBI-921352*: Selective Na\textsubscript{v}1.6 Inhibitor for Rare Pediatric Epilepsy

Initiation of Pediatric SCN8A-DEE\textsuperscript{†} Clinical Program in 2H 2020

**SCN8A-DEE**

- Rare form of early-onset epilepsy with occurrence of **seizures** beginning in the first 18 months of life and a **high incidence of sudden unexpected death in epilepsy**
- **Physical and psychological symptoms** include recurrent seizures of all types, developmental delays, learning difficulties, muscle spasms, poor coordination, sleep problems, and autistic-like features
- **No approved treatments** with off-label options associated with **poor outcomes, safety and tolerability**

**NBI-921352‡**

- **First potent and selective inhibitor** to precisely target the sodium channel affected by the genetic mutation of SCN8A - Na\textsubscript{v}1.6
- Impact the lives of **SCN8A-DEE patients** and additional **1 million patients with focal seizures**, 50% of whom are refractory to existing treatments
- **Initiation of Phase II study** in SCN8A-DEE in 2H 2020
- **Potential fast track to approval in SCN8A-DEE** given significant clinical need and lack of treatment options

\* In-licensed from Xenon Pharmaceuticals
\textsuperscript{†} SCN8A-DEE (SCN8A developmental and epileptic encephalopathy)
\textsuperscript{‡} NBI-921352 is investigational and not approved in the U.S.
Our Vision for the Future
Transformation into Fully Integrated Neuroscience-Focused Company: Well-Positioned for Sustained Growth

<table>
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3 Pipeline Programs
- Opicapone†
- Parkinson’s Disease
- Elagolix†

4 Indications
- Parkinson’s Disease
- Endometriosis
- Uterine Fibroids

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¶ Planned or projected

* Approved or submitted
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