

Neurocrine® BIOSCIENCES

Advancing Life-Changing Discoveries in Neuroscience

> Q4 2019 / Year-End February 4, 2020

> > **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: 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 guarter ended September 30, 2019. Neurocrine disclaims any obligation to update the statements contained in this presentation after the date hereof.

This presentation refers to certain non-GAAP financial measures. These non-GAAP financial measures should not be considered replacements for, and should be read together with, the most comparable GAAP financial measures. Reconciliations of non-GAAP financial results to the most directly comparable GAAP financial results are included at the end of this presentation and in our earnings release. In addition, Neurocrine provides guidance regarding combined research and development and sales, general and administrative expenses on both a GAAP and non-GAAP basis.

Neurocrine 2019 Q4 Highlights & 2020 Key Activities



Q4 2019 Highlights

- INGREZZA® (valbenazine) Net Product Sales:
 - \$238MM with 42,100 TRx in Q4 2019
 - \$753MM with 132,700 TRx in FY 2019
- Advanced CAH Development Programs
- Initiated Phase III Study of Valbenazine for Chorea in Huntington Disease
- Announced Licensing Agreement with Xenon for NBI-921352 and related Sodium Channel Inhibitors for the Treatment for Epilepsy
- Announced Option Agreement* with Idorsia for Rights to License ACT-709478 for the Treatment of Epilepsy

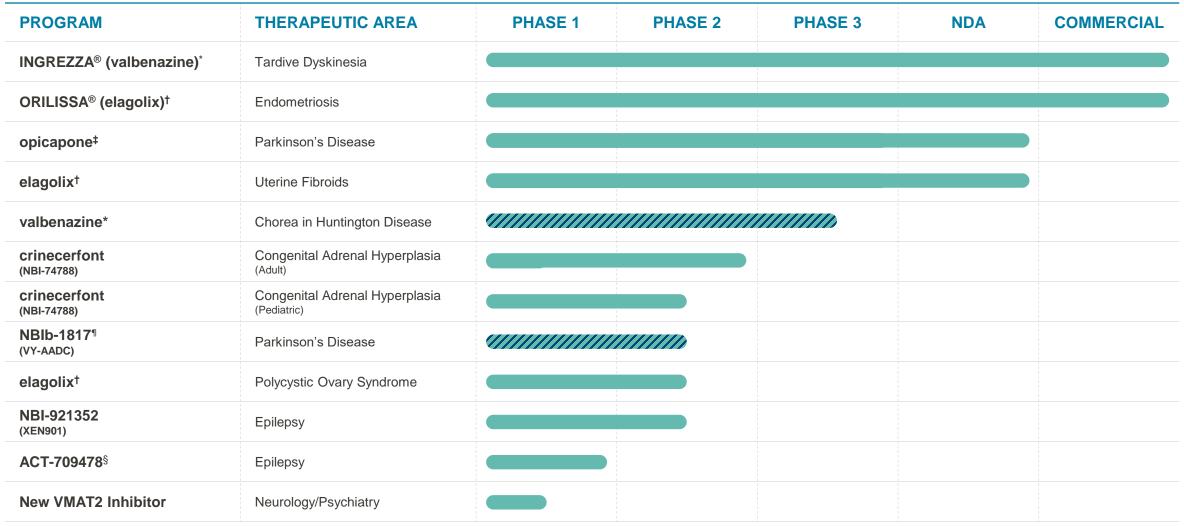
2020 Key Activities

- Continued Focus on INGREZZA Commercial Execution and "Talk About TD" Disease State Awareness Campaign
- Prepare for Opicapone Commercial Launch;
 PDUFA April 26th
- Initiate Global Registrational Study for Crinecerfont in Adult CAH Patients in Mid-2020
- Implement Amended Protocol for RESTORE-1 Registration Study for NBIb-1817 by Mid-Year and Initiate RESTORE-2 Registration Study in 2H 2020
- Initiate Phase II Studies for NBI-921352 and ACT-709478 in Rare Pediatric Epilepsies in 2H 2020

TRx = Total Prescriptions; CAH = Congenital Adrenal Hyperplasia; FDA = U.S. Food and Drug Administration; PDUFA = Prescription Drug User Fee Act * Announced Agreement in January 2020

Diversified Portfolio with Multi-Stage Programs





Neurocrine Biosciences has global rights unless otherwise noted.

Legend Registrational

^{*} Mitsubishi Tanabe Pharma has commercialization rights in East Asia

[†] AbbVie has global commercialization rights

[‡] BIAL retains commercialization rights outside U.S. and Canada

 $^{^{\}rm 1}$ 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







3 Approved Medicines in 4 Indications

INGREZZA® (valbenazine) for Tardive Dyskinesia

ORILISSA® (elagolix) for Endometriosis*

Opicapone for Parkinson's Disease

Elagolix for Uterine Fibroids*

APPROVED

APPROVED

April 26, 2020 PDUFA[†]

Q2 2020 PDUFA†



3 Pivotal Clinical Trial Programs

Initiate Global Registrational-Enabling Study for CAH[‡] (adults)

Registrational-Enabling Studies for NBIb-1817 in Parkinson's Disease

Phase III Study of Valbenazine for Chorea in Huntington Disease

Mid-2020

2020

Ongoing

5 Early-to-Mid Stage R&D Programs



Phase IIa Study of Crinecerfont in CAH (pediatric)

Initiate Phase II Study of NBI-921352 in SCN8A-DEE®

Initiate Phase II Study of ACT-709478 in Epilepsy§

Phase II Study of Elagolix in PCOS*

New VMAT2 Inhibitor

Ongoing

2H 2020

2H 2020

Ongoing

Ongoing

^{*} AbbVie has global commercial rights

[¶] SCN8A – DEE (SCN8A Developmental and Epileptic Encephalopathy)

[†] PDUFA (Prescription Drug User Fee Act) Target Action Date § Neurocrine Biosciences has the exclusive option to license from Idorsia

[‡] CAH (Congenital Adrenal Hyperplasia) PCOS (Polycystic Ovary Syndrome)



Financial Summary

\$ Millions, Except Non-GAAP EPS

Item	Q4 '19	Q4 '18	2019	2018
Revenue - INGREZZA Product Sales, Net - Collaboration Revenue	\$244.1 237.9 6.2	\$131.4 130.3 1.1	\$788.1 752.9 35.2	\$451.2 409.6 41.6
Non-GAAP R&D Expense	47.9	29.9	164.2	119.6
Non-GAAP SG&A Expense	87.4	60.5	304.6	217.1
Non-GAAP Net Income	102.2	38.4	283.8	70.5
Non-GAAP Earnings per Share, Diluted	\$1.05	\$0.40	\$2.96	\$0.74
Cash and Investments (Period End)	\$970.2	\$866.9		

All income statement items, except revenue, are non-GAAP financial measures—if this slide is in hard copy, see reconciliations accompanying the presentation



2020 GAAP and Non-GAAP Expense Guidance

\$ Millions	2020 Guidance Range			
Item	2019 Actuals	Low	High	
Combined GAAP R&D and SG&A Expenses	\$554	\$740	\$770	
Combined Non-GAAP R&D and SG&A Expenses	\$469	\$620	\$650	

Increase in 2020 operating expenses reflect:

- Increased investment in R&D programs including three registrational programs
- Meaningful investments across early stage programs including Voyager and Xenon collaborations
- Continued investment in INGREZZA
- Marketing costs associated with the anticipated launch of opicapone

The below items impact GAAP-Only Guidance:

- Approximately \$100 million of share-based compensation and \$20 million expected milestone payment to BIAL connected with the expected approval of opicapone by the FDA during the second quarter
- Does not include other future potential milestones or IPR&D associated with current collaborations or future business development activities

IPR&D = In-Process Research and Development











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:

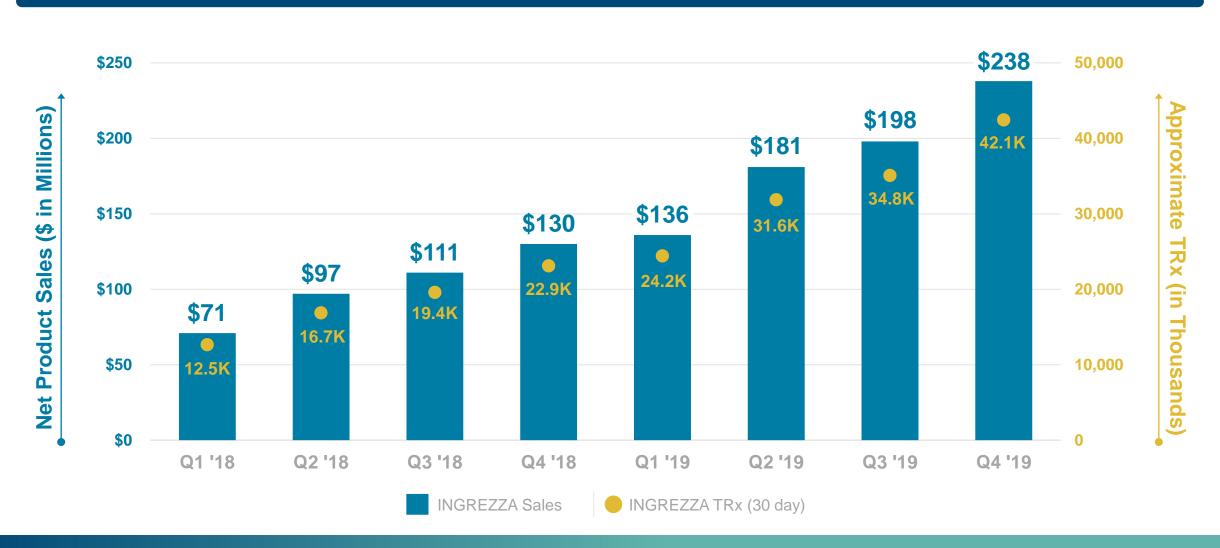


39%

INGREZZA Continues to Exceed Expectations



INGREZZA Net Sales and ~TRx



Valbenazine: Chorea in Huntington Disease



Phase III Study to Treat Chorea in Adult Patients with Huntington Disease

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.





elagolix tablets 150 mg 200 mg Neurocrine Biosciences discovered and developed through Phase II; AbbVie received FDA approval and responsible for commercialization

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

Q4 2019 Earnings Presentation

^{*} 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

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

new diagnoses annually

400,000

1 drug approved by FDA in the past 20 years



Approximately 220,000 hysterectomies performed annually

Polycystic Ovary Syndrome

Phase II Study Ongoing

Most

common hormonal disorder

in women of reproductive age



3.5 million

women affected in the United States



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

Crinecerfont*: Classic Congenital Adrenal Hyperplasia



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

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

Crinecerfont

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

Overtreatment Hypercortisolism

Undertreatment Hyperandrogenism

Cushingoid features
Central obesity
Osteoporosis
Insulin Resistance
Impaired Glucose
Tolerance

Glucocorticoid

Adrenal Crisis
Increased Adrenal
Androgen
Hirsutism
Amenorrhea
Infertility

Hypertension

Mineralocorticoid

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

Q4 2019 Earnings Presentation

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



Expanding Reach: Innovative Partners with Novel Science to Address Unmet Medical Need

Gene Therapy

Precision Medicine

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

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

Research collaboration

Research collaboration

^{*} Investigational and not approved in the U.S.

[†] Neurocrine Biosciences has the exclusive option to license from Idorsia

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

^{*} In-licensed from Voyager Therapeutics

[†] NBIb-1817 is investigational and not approved in the U.S.

NBI-921352*: Selective Na_V1.6 Inhibitor for Rare Pediatric Epilepsy



Initiation of Pediatric SCN8A-DEE[†] 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[‡]

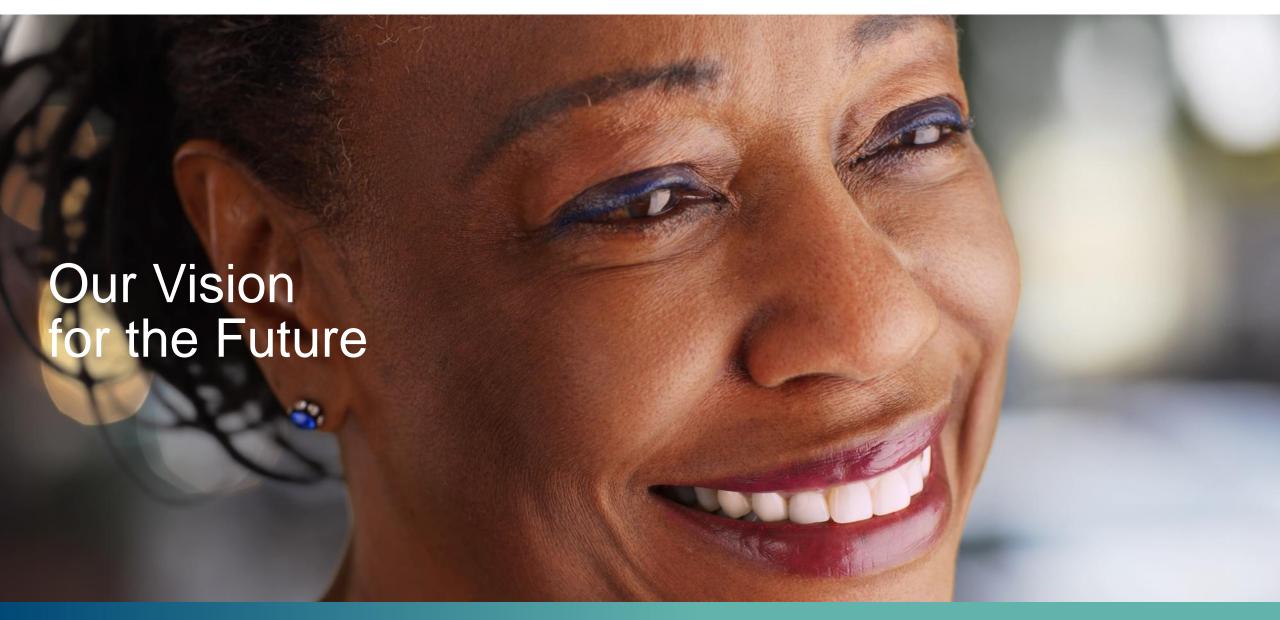
- First potent and selective inhibitor to precisely target the sodium channel affected by the genetic mutation of SCN8A - Na_V1.6
- Impact the lives of SCN8A-DEE patients and additional 1 million patients with focal seizures, 50% of whom are refractory to existing treatments
- Initiation of Phase II 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

[†] SCN8A-DEE (SCN8A developmental and epileptic encephalopathy)

[‡]NBI-921352 is investigational and not approved in the U.S.





Transformation into Fully Integrated Neuroscience-Focused Company: Well-Positioned for Sustained Growth





Strong Commercial Capabilities

INGREZZA
Blockbuster
Potential;
Experienced,
Neuro/Psych Field
Sales Team



Proven R&D with Strong Multi-stage Pipeline

Three Approved
Medicines in
Four Indications and
Three Additional
Programs in Pivotal
Studies in 2020





Solid Financial Position to Invest

~\$970MM Cash and Investments (as of Q4 2019)



Neurocrine® BIOSCIENCES

GAAP to Non-GAAP Reconciliations

NEUROCRINE BIOSCIENCES, INC.

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



	7	Three Mon Deceml			Twelve Months Ended December 31,		
(in millions, except per share data)		2019		2018	2019	2018	
GAAP net income	\$	34.0	\$	18.0 \$	37.0 \$	21.1	
Adjustments:							
Milestones received from licenses and collaborations A		_		_	(20.0)	(40.0)	
Non-cash collaboration revenue B		(0.9))	_	(0.9)	_	
Acquired in-process research and development (IPR&D) C		36.2		4.8	154.3	4.8	
Milestones paid related to licenses and collaborations - R&D D		_		_	10.0	10.0	
Share-based compensation expense – R&D		7.4		4.6	25.8	26.2	
Share-based compensation expense - SG&A		13.9		8.5	49.5	31.8	
Non-cash interest related to convertible debt		5.2		4.8	20.3	18.9	
Changes in fair value of equity security investments E		7.2		_	13.0	_	
Income tax effect related to reconciling items F		(0.8)		(2.3)	(5.2)	(2.3)	
Non-GAAP net income	\$	102.2	\$	38.4 \$	283.8	70.5	
Net income per diluted common share:							
GAAP	\$	0.35	\$	0.19 \$	0.39 \$	0.22	
Non-GAAP	\$	1.05	\$	0.40 \$	2.96 \$	0.74	

A During the third quarter of 2019 and third quarter of 2018, the Company recognized event-based milestones from AbbVie of \$20.0 million and \$40.0 million, respectively, for regulatory milestones associated with elagolix.

^B During the fourth quarter of 2019, the Company recognized non-cash collaboration revenue from Mitsubishi Tanabe Pharma Corporation (MTPC) under the collaboration and license agreement entered into in 2015.

^c During 2019, the Company incurred IPR&D expenses of \$118.1 million and \$36.2 million, respectively, in association with collaboration and license agreements entered into with Voyager and Xenon and \$4.8 million in association with a research collaboration agreement entered into with Jnana Therapeutics Inc during fourth quarter of 2018.

^D During each of the second quarter of 2019 and first quarter of 2018, the Company incurred milestone expenses of \$10.0 million related to event-based milestones for opicapone related to the Company's collaboration with BIAL.

^E The Company's investments include equity security investments in Voyager and Xenon. The Company recognized unrealized losses of \$7.2 million for the fourth quarter of 2019 and \$13.0 million for full-year 2019 to adjust its equity security investments to fair value.

F Estimated income tax effect of non-GAAP reconciling items are calculated using applicable statutory tax rates, taking into consideration any valuation allowance.



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

	 Three Months Ended				
(in millions, except per share data)	arch 31, 2019	June 30, 2019	September 30, 2019	December 31, 2019	December 31, 2019
GAAP net income	\$ (102.1) \$	51.3	\$ 53.8	\$ 34.0	\$ 37.0
Adjustments:					
Milestones received from licenses and collaborations A	_	_	(20.0)	_	(20.0)
Non-cash collaboration revenue ^B	_	_	_	(0.9)	(0.9)
Acquired in-process research and development (IPR&D) C	113.1	5.0	_	36.2	154.3
Milestones paid related to licenses and collaborations - R&D D	_	10.0	_	_	10.0
Share-based compensation expense – R&D	5.4	6.0	7.0	7.4	25.8
Share-based compensation expense - SG&A	10.4	11.9	13.3	13.9	49.5
Non-cash interest related to convertible debt	4.9	5.1	5.1	5.2	20.3
Changes in fair value of equity security investments E	(1.7)	(21.0)	28.5	7.2	13.0
Income tax effect related to reconciling items F	(2.3)	(1.1)	(1.0)	(0.8)	(5.2)
Non-GAAP net income	\$ 27.7 \$	67.2	\$ 86.7	\$ 102.2	\$ 283.8
Net income per diluted common share:					
GAAP	\$ (1.12) \$	0.54	\$ 0.56	\$ 0.35	\$ 0.39
Non-GAAP	\$ 0.29 \$	0.71	\$ 0.90	\$ 1.05	\$ 2.96

A During the third quarter of 2019, the Company recognized a \$20.0 million event-based milestone as revenue upon FDA acceptance of AbbVie's NDA submission of elagolix for the treatment of uterine fibroids.

B During the fourth quarter of 2019, the Company recognized non-cash collaboration revenue from MTPC under the collaboration and license agreement entered into in 2015.

^c The Company incurred IPR&D expenses of \$118.1 million and \$36.2 million, respectively, in association with collaboration and license agreements entered into with Voyager and Xenon during 2019.

During the second quarter of 2019, the Company incurred milestone expenses of \$10.0 million related to FDA acceptance of the opicapone NDA for Parkinson's disease.

E The Company's investments include equity security investments in Voyager and Xenon. The Company recognized unrealized (gains) losses to adjust its equity security investments to fair value.

F Estimated income tax effect of non-GAAP reconciling items are calculated using applicable statutory tax rates, taking into consideration any valuation allowance.

NEUROCRINE BIOSCIENCES, INC. RECONCILIATION OF GAAP TO NON-GAAP FINANCIAL RESULTS

(unaudited)



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