Neurocrine® BIOSCIENCES

Advancing Life-Changing Discoveries in Neuroscience

Q1 2019
Corporate Presentation
April 29, 2019

neurocrine.com

NASDAQ: NBIX

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 Neurocrine's 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 planned submission of the NDA for opicapone; the collaboration with Voyager Therapeutics; 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: the Company's future financial and operating performance; risks and uncertainties associated with the commercialization of INGREZZA and ORILISSA, including the likelihood of continued revenue and prescription growth of INGREZZA and ORILISSA; risks or uncertainties related to the development of the Company's product candidates; risks and uncertainties relating to competitive products and technological changes that may limit demand for INGREZZA, ORILISSA, or a product candidate; risks associated with the Company's dependence on third parties for development and manufacturing activities related to INGREZZA and the Company's product candidates, and the ability of the Company to manage these third parties; risks that the FDA or other regulatory authorities may make adverse decisions regarding INGREZZA, ORILISSA, opicapone, or the Company's other product candidates; risks associated with the Company's dependence on AbbVie for the commercialization of ORILISSA and the development of elagolix; risks that clinical development activities may not be completed on time or at all; risks that clinical development activities 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 benefits of the agreements with our collaboration partners may never be realized, including Voyager, BIAL, and Mitsubishi Tanabe; risks that INGREZZA, ORILISSA, 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 the Company's periodic reports filed with the Securities and Exchange Commission, including without limitation the Company's quarterly report on Form 10-Q for the quarter ended March 31, 2019. Neurocrine disclaims any obligation to update the statements contained in this presentation after the date hereof.

Neurocrine Q1 2019 Highlights and 2019 Key Activities

First Quarter 2019 Highlights

- Q1 2019 INGREZZA® Net Product Sales of $136MM with 24,200 TRx
- Positive Interim Results from Ongoing Phase II Study in Congenital Adrenal Hyperplasia
- “Talk About TD” National Disease State Awareness Program Launched
- Closing of Strategic Gene Therapy Collaboration with Voyager Therapeutics

2019 Key Activities

- “Talk About TD”- National Disease State Awareness Program
- Field Sales Team Fully Deployed in New Territories
- Advancement of CAH into Pediatrics and Initiation of Registration Study in Adults
- New Drug Application (NDA) Filing with FDA for Opicapone
- Progress Gene Therapy Collaboration with Voyager Therapeutics Across Parkinson’s Disease, Friedreich’s Ataxia, and Two Other Programs

2019: A Defining Year of Significant Milestones

2 NDA Submissions: Parkinson's Disease & Uterine Fibroids

Opicapone (Parkinson's disease) — **Q2 2019**

Elagolix (Uterine fibroids) *submitted by AbbVie* — **Mid-2019**

Advancing Congenital Adrenal Hyperplasia Program

Positive interim results from Phase II Study (adults) — **COMPLETED**

Phase IIa initiation (pediatric) — **Q3 2019**

Pivotal study initiation (adults), pending FDA discussion in Q2 — **2H 2019**

Progressing Early Stage R&D

Progress **Voyager Therapeutics** gene therapy collaboration - advance Phase II Parkinson's disease program and select **lead candidate** for Friedreich's ataxia

Continued **advancement of compounds in clinical development pipeline**

2020: Potential for 3 Approved Treatments in 4 Indications

2018

2 APPROVED TREATMENTS IN 2 INDICATIONS

INGREZZA® (valbenazine) capsules
Tardive Dyskinesia

Orilissa® elagolix tablets 150mg 200mg
Endometriosis

2019

2 NEW DRUG APPLICATIONS

Opicapone
Parkinson's Disease

Elagolix
Uterine Fibroids

2020

3 Approved treatments in 4 indications

MULTI-STAGE, DIVERSIFIED PIPELINE

- **NBI-74788** (Congenital Adrenal Hyperplasia)
- **Next Gen VMAT2 Inhibitor**
- **Novel CNS Compound**
- **Voyager Collaboration** (Parkinson's Disease, Friedreich's Ataxia, 2 Others)

Neurocrine BIOSCIENCES

Our Medicines
Our Patients

INGREZZA®
(valbenazine) capsules

1st FDA-approved Treatment for Tardive Dyskinesia; Launched in 2017

Most-Prescribed and Most-Preferred TD Therapy

- **Rapid Improvement** in Involuntary Movements
- **Safe & Well Tolerated**
- **Ease of Use**: One capsule, Once-daily

INGREZZA®

Most Prescribed Tardive Dyskinesia (TD) Therapy

Tardive dyskinesia
affects approximately 500,000 patients in the U.S.

- **Involuntary Movement Disorder Caused by Prolonged Antipsychotic Use for Bipolar, Schizophrenia, and Depression**
- **Can Negatively Impact Patients' Daily Function, Productivity and Quality of Life**
- **More than a 400% Increase in Antipsychotic Prescriptions from 1990-2018 (~68MM TRx in 2018)**

Efficacy:
Rapid and Robust

- **~30% Reduction in TD Severity at 6 Weeks**
- **Significant efficacy as early as 2 weeks**
- **Sustained Reductions in TD Severity Through 48 Weeks**

Label:
No Boxed Warning

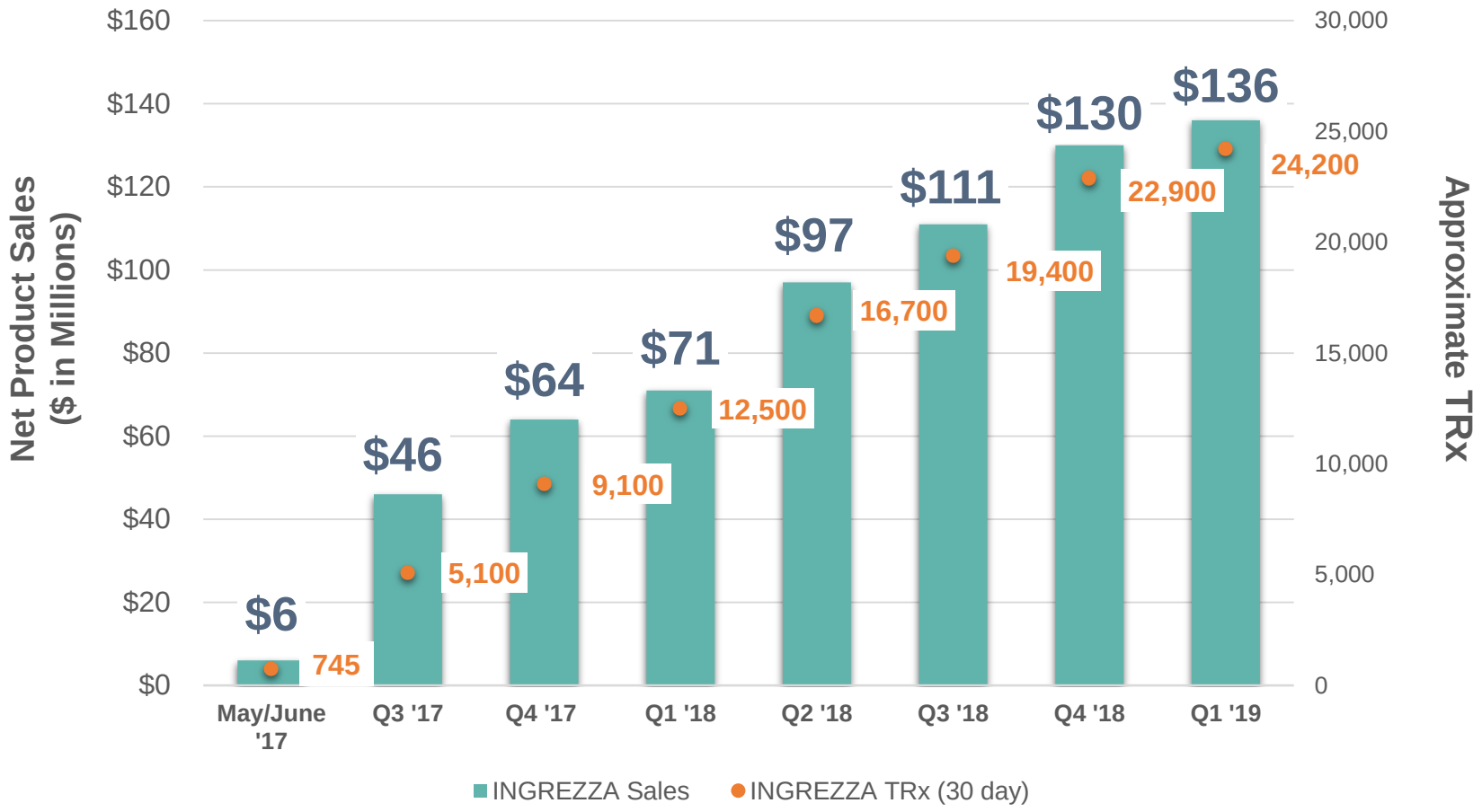
- **No Boxed Warning**
- **Generally Well-tolerated Across a Broad Range of Adult TD Patients**
- **Concomitant Use with Psychiatric Medications**

Use:
Convenient Dosing

- **Convenient One Capsule, Once-daily Dosing Without Complex Titration**
- **Two Dosing Options that Work**

A Successful Launch, Expectations Exceeded

INGREZZA Net Sales and ~TRx



★ INGREZZA Launch Update

- **Strong Demand:** $400+ million net sales in 2018
- **Favorable Coverage:** ~ 90% lives covered by insurance
- **High Fulfillment Rate:** 70%+ INGREZZA prescriptions are dispensed
- **Affordable for Patients:** ~80% of patients pay less than $10 out-of-pocket

2019 Areas of Investment

- **Field Sales Team Deployment in New Territories:** Increased team 50% in 2H 2018 to further educational efforts
- ***Talk About TD:*** Disease state educational program (TV/digital)
- **RE-KINECT Real World Study:** Ongoing

Tardive Dyskinesia Overview: Symptoms

Oral and Facial Dyskinesia

- Abnormal tongue and lip movements
- Retractions of the corners of the mouth
- Abnormal eyelid closure or eyebrow movements
- Bulging of the cheeks
- Chewing movement

Limb Dyskinesia

- “Piano-playing” finger movements
- Tapping foot movements
- Dystonic extensor postures of the toes

Torso Dyskinesia

- Shoulder shrugging

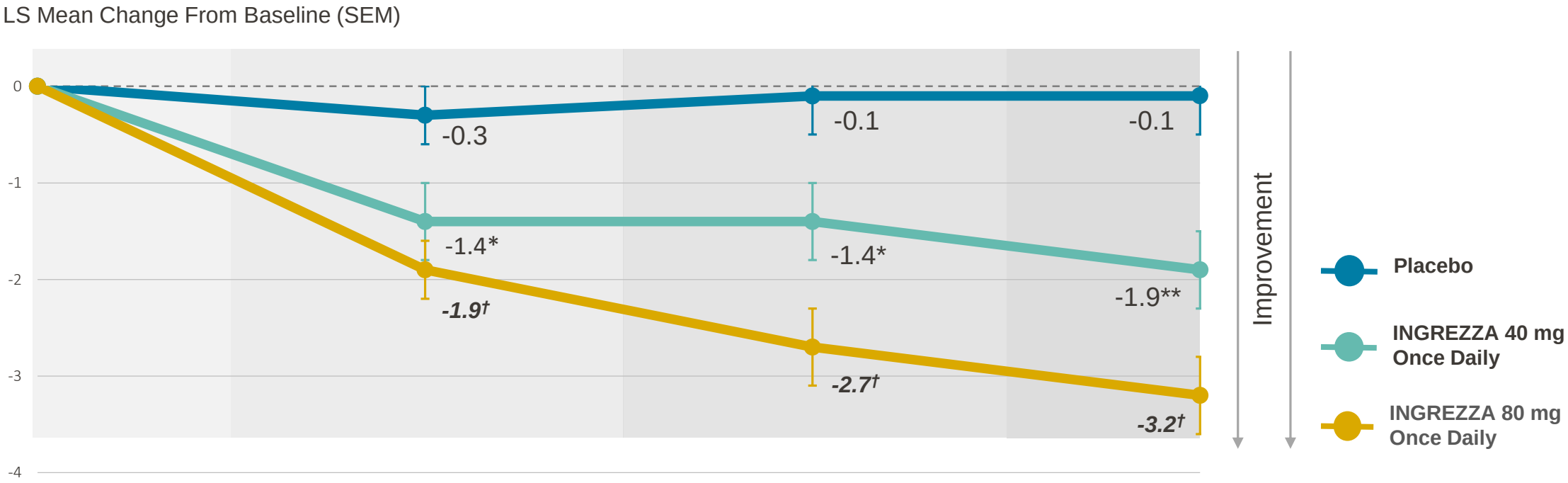
Axial Dystonia

- Twisting of the torso
- Rocking and swaying movements
- Rotatory or thrusting hip movements

KINECT 3: INGREZZA® Reduction in Abnormal Involuntary Movement Scores at Each Study Visit Through Week Six

AIMS Change from Baseline by Study Visit (ITT Population)

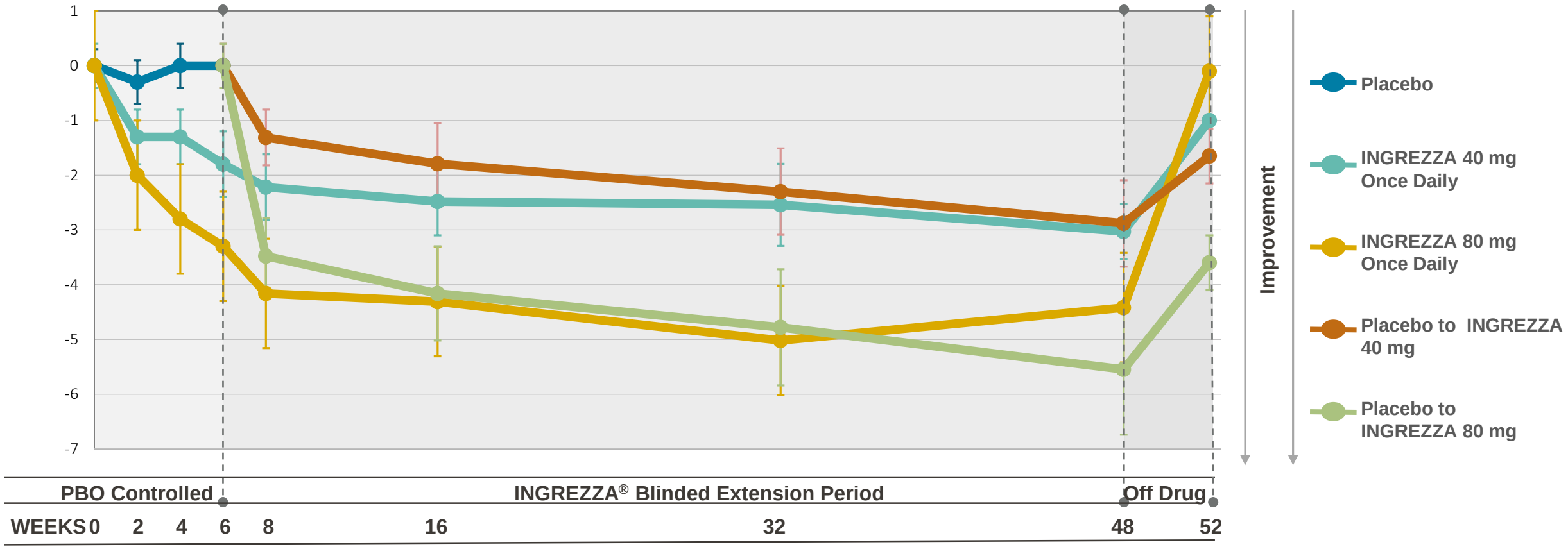
WEEK 0: Baseline	WEEK 2	WEEK 4	WEEK 6
n=76 n=70 n=79	n=76 n=70 n=77	n=73 n=64 n=73	n=69 n=63 n=70



P values vs placebo: * <0.05 (nominal), ** <0.01 (nominal), † ≤0.001. AIMS change from baseline at weeks 2 and 4 not controlled for multiplicity. Data presented for ITT analysis set. Change in AIMS score analyzed by MMRM model. Treatment differences determined by comparison of LS means.
Hauser RA, et al. Am J Psychiatry. 2017. Mar 21: doi: 10.1176/appi.ajp.2017.16091037. [Epub ahead of print]. Data on file. Neurocrine Biosciences.

KINECT 3: AIMS Change from Baseline for INGREZZA® Groups (Long-Term Extension Period)

AIMS Mean Change (SEM) from Baseline (ITT Population)



DB, double-blind. Data presented for ITT analysis set.
INGREZZA [package insert]. San Diego, CA: Neurocrine Biosciences; 2017.

Orilissa®
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

- **Rapid, Sustained Pain Relief**
  Addresses three most common types of endometriosis pain
- **Oral Administration**
  2 dosage options based on severity of symptoms and treatment objectives
- **Safety & Tolerability Profile**
  Proven efficacy & safety in largest endometriosis clinical program

ORILISSA® (elagolix) Overview

Largest Ever Endometriosis Program Conducted to Date

3,000,000 patients
with moderate to severe endometriosis in U.S.

- **Chronic and Painful Disease Affecting ~10% of Women in Reproductive Age**
- **Three Common Symptoms: 1) Painful Periods (Dysmenorrhea), 2) Non-Menstrual Pelvic Pain (NMPP), 3) Pain with Sex**
- **A Leading Cause of Hysterectomy and Infertility**

Efficacy:
Rapid and Robust

- **Efficacy as Early as 1 Month**
- **Approximately 45-75% Responder Rates Based on Combined Pain and Functional Impact Scales for Dysmenorrhea and NMPP**
- **~ 80-90% Responder Rates Based on Patient Global Impression of Change (Minimally to Very Much Improved)**
- **In Two, 6-month Replicate Phase III studies, All Women Had A BMD Z-score Above -2.0, Within The Normal Age-Adjusted Range**

Label:
No boxed warnings or required monitoring

- **150 mg QD and 200 mg BID Dosing Options**
- **Taken With or Without Food**
- **24 Months of Therapy for 150 mg QD with Physician Judgement Thereafter Based upon Treatment Goals**

AbbVie
Collaboration

- **Discovered and Developed by NBIX Through Completion of Phase II studies**
- **In June 2010, AbbVie and Neurocrine Entered into Worldwide Development and Commercialization Collaboration**
- **Significant Development and Commercial Milestones Plus a Tiered, Double-Digit Royalty on Net Sales**
- **ORILISSA Commercialized by AbbVie in August 2018**

Elagolix For Women's Health (Partnered with AbbVie)

ORILISSA® Approved for Endometriosis; Uterine Fibroids NDA Submission Expected in Mid-2019

ENDOMETRIOSIS

Impacts **10%** of childbearing women

7.5 million women in the United States

3.0 million diagnosed with moderate to severe

300,000 new diagnoses annually

105,123 days women were hospitalized in 2010 because of their disease

Approximately **125,000** hysterectomies performed/year

>$69B in societal burden / year

UTERINE FIBROIDS

Most common pelvic growth affecting **≥20%** of all women by age of 59 of childbearing women

9 million women with symptomatic uterine fibroids

3 million women currently diagnosed

450,000 new diagnoses / year

1 drug approved by FDA in past **20 years**

Approximately **250,000** hysterectomies performed annually

Leading cause of **infertility**

Our Investigational Therapies

Diversified Portfolio with Multi-Stage Pipeline

Program/Therapy	Disorder	Stage of Development					Partner
		1	2	3	NDA	Commercial	
INGREZZA® (valbenazine) capsules	Tardive Dyskinesia						Mitsubishi Tanabe Pharma (Asia)
Orilissa® elagolix tablets	Endometriosis						abbvie (Worldwide)
elagolix	Uterine Fibroids						
opicapone	Parkinson's Disease						Bial (NBI Rights: U.S. & Canada)
NBI-74788	Congenital Adrenal Hyperplasia						
VY-AADC	Parkinson's Disease						Voyager Therapeutics (NBI Rights: Worldwide*)
New VMAT2 Inhibitor	Neurology/Psychiatry Disorders						
Novel CNS	Neurology/Psychiatry Disorders						

Neurology

Endocrinology

*Voyager has profit share option for U.S. market following the ongoing Phase II RESTORE-1 study

Opicapone: Pending Q2 NDA Submission for Parkinson's Disease

Discovered and Launched in Europe by BIAL; In-licensed in 2017

The Need: **Parkinson's Disease**

1 million
people impacted in the U.S.

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

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

Current augmentative treatments have **limited efficacy and tolerability**

The Opportunity: **Opicapone***

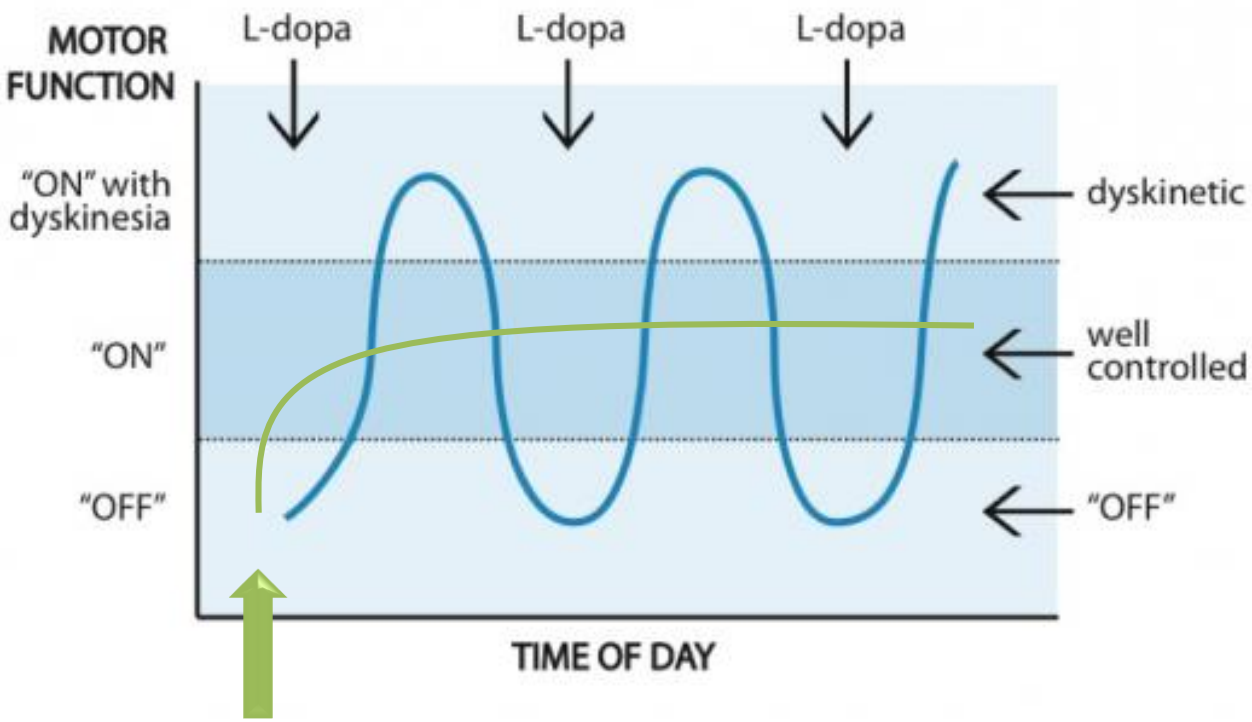
- **Novel COMT inhibitor as adjunctive therapy** to levodopa/DOPA decarboxylase inhibitors in Parkinson's patients with OFF-episodes
- **Significant and sustained reduction of daily OFF-time** and **increase of ON-time** without troublesome dyskinesia
- **Once-a-day dosing** with no titration needed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

*Opicapone is investigational and not approved in the U.S.

Opicapone: Reducing "OFF-Time" For Patients with Parkinson's Disease

- **Parkinson's Disease (PD): Lifelong, Incurable, Progressive**
- **2nd Most Common Neurodegenerative Disease Following Alzheimer's Disease**
- **Approximately One Million Patient Cases in the United States**
- **While Incidence Rates Expected To Remain Constant, Prevalence Will Increase As A Result Of The Aging Population**
  - Increasing Life Expectancy
  - >10M Elderly People By 2020
- **Approximately Two-Thirds of Patients on L-dopa/C-dopa therapy**

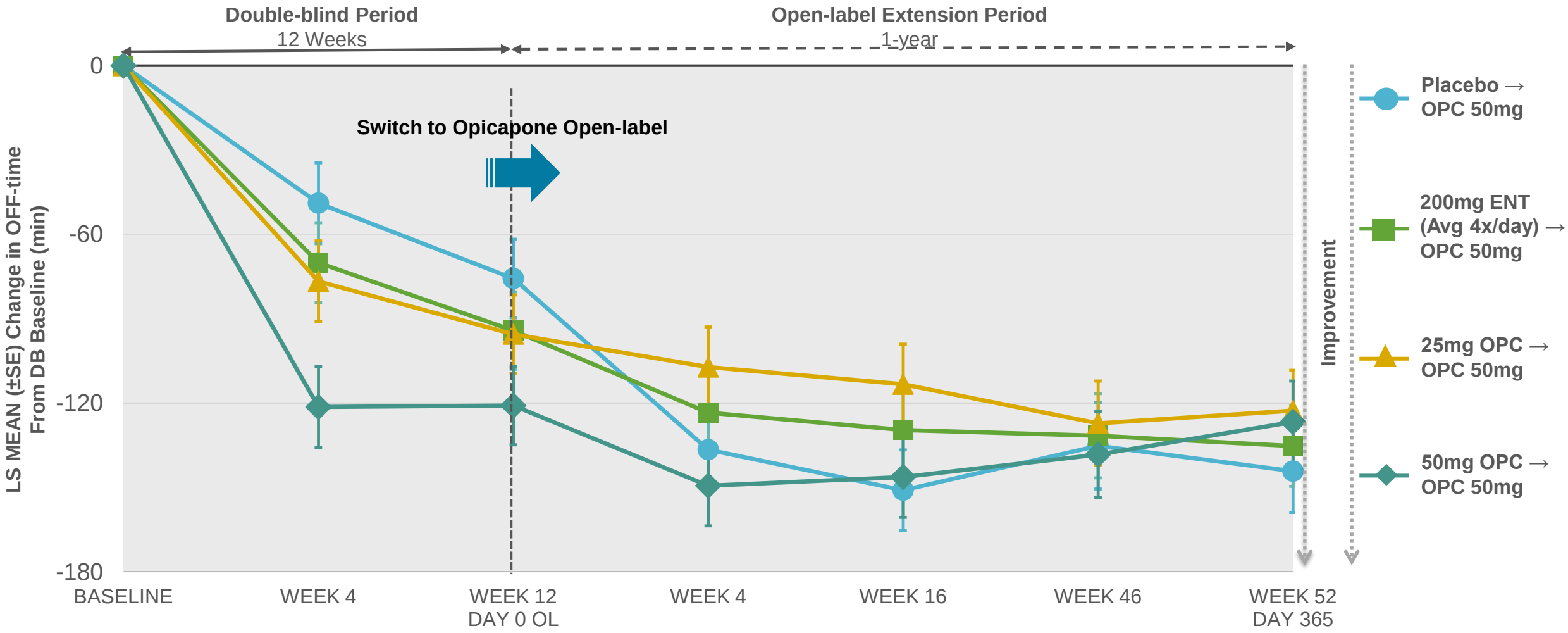
COMT Inhibition Reduces "OFF-time" and Increases "ON-time" Without Troublesome Dyskinesia



Sources: Datamonitor and Medical Literature review

Phase III, BIPARK-1: Once Daily Opicapone Shows Maintenance of Effect at One Year*

Mean Change in Absolute OFF-time



* During Open-label portion, all Subjects were rolled-over to 50mg OPC. Movement Disorder Society 2016.
LS mean = lease squares mean as estimated from the mixed model for repeated measurements; SE = standard error

NBI-74788: Classic Congenital Adrenal Hyperplasia

Positive Interim Phase II Results in Adults; Phase II Pediatric Trial Planned to Commence in Q3

The Need: **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.

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

Excess corticosteroid treatment lead to additional clinical problems including bone loss, Cushing's disease, metabolic syndrome

The Opportunity: **NBI-74788***

- **Potent, selective, orally-active**, non-peptide corticotropin releasing factor type 1 (CRF1) receptor antagonist
- **Prevents excessive production of androgens** without need for supraphysiologic doses of corticosteroids
- **May optimize glucocorticoid dosing**
- **May reduce clinical consequences** of current treatments and underlying disease

*NBI-74788 is investigational and not approved in the U.S.

NBI-74788: Positive Interim Phase II Data (Adults)

INTERIM RESULTS

- Reduction of at least 50 percent from baseline in 17-hydroxyprogesterone and adrenocorticotropic hormone levels in more than 50 percent of CAH patients treated for 14 days
- Meaningful reductions in other biomarkers, including androstenedione
- Well tolerated with no serious adverse events reported to date

NEXT STEPS

- Meet with FDA to discuss the registration program for NBI-74788 in adults
- Initiate Phase IIa trial in pediatric patients

Elagolix: Pending NDA Submission for Uterine Fibroids in Mid-2019

Discovered & Developed by Neurocrine Biosciences to Phase II; Further Development by AbbVie

The Need: **Uterine Fibroids**

3 million
women diagnosed with symptomatic uterine fibroids (UF)

450,000
new diagnoses annually

One drug approved by FDA in past **20 years**

250,000 hysterectomies
performed annually to manage UF

Current non-surgical treatments have **limited efficacy and safety risks**

The Opportunity: **Elagolix***

- **Significant reduction in heavy menstrual bleeding** in up to 68.5% of women for up to 12 months (Phase III)
- **Orally administered**

*Elagolix in uterine fibroids is investigational and has not been proven safe and effective.

Voyager Therapeutics Collaboration

Voyager Gene Therapy Collaboration to Address Key Neuroscience Programs

Collaboration leverages strengths and efforts of both companies towards developing and commercializing life-changing treatments for severe neurological diseases

Programs:

Neurocrine Biosciences gains development and commercialization rights to four gene therapy programs:

- **VY-AADC for Parkinson's disease**
- **VY-FXN01 for Friedreich's ataxia**
- **Two additional programs to be determined**

Voyager received $165 million upfront ($115MM cash/$50MM equity), along with funding for ongoing development of each program, and up to $1.7 billion in potential development, regulatory and commercial milestone payments

VY-AADC: Gene Therapy for Parkinson's Disease

Developed by Voyager Therapeutics

The Need: **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

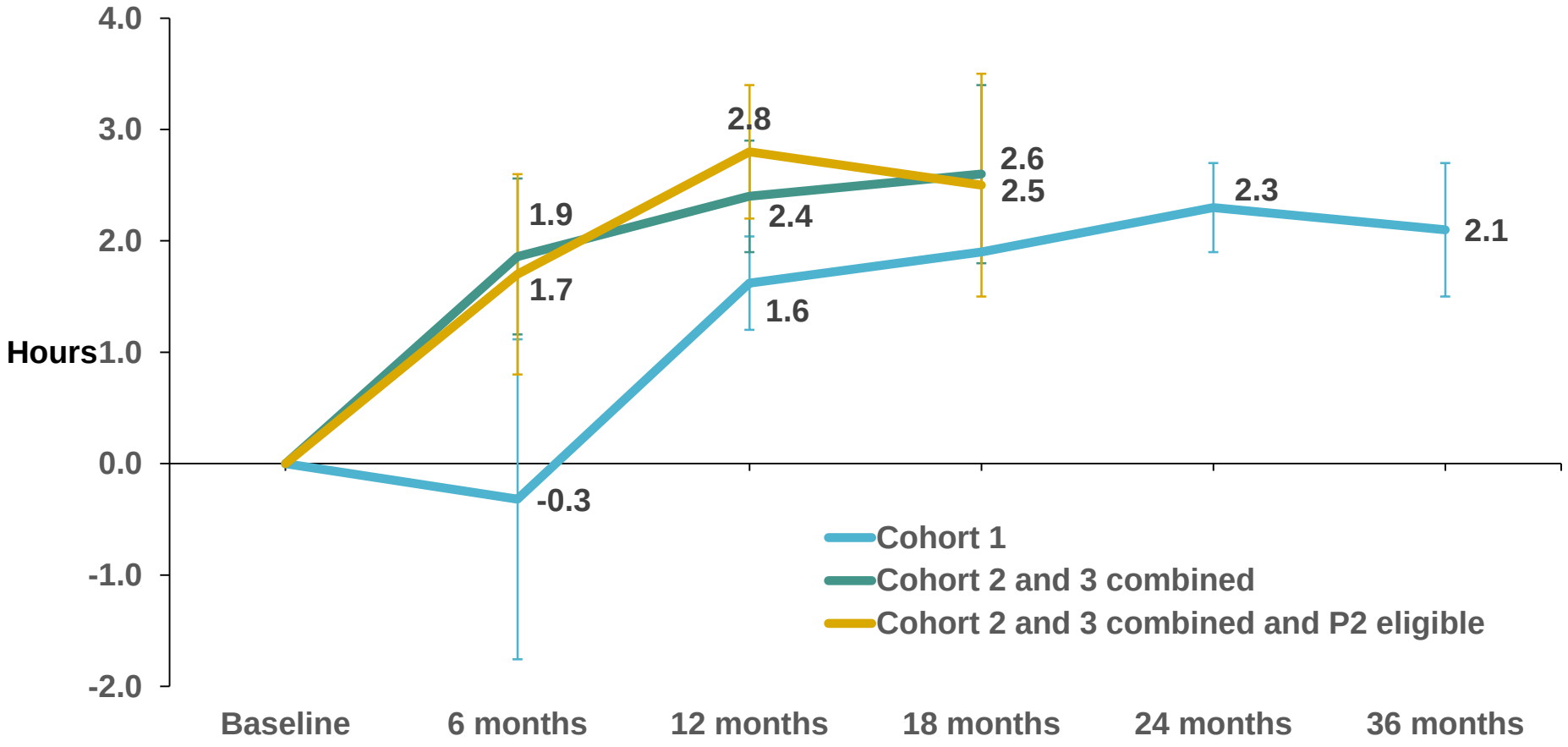
The Opportunity: **VY-AADC**

- **One-time treatment** restores AADC enzyme activity and improves levodopa sensitivity with potential to improve clinical motor function
- **Improvement in good 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 Phase II trial** - first patient dosed in December 2018

*VY-AADC is investigational and not approved in the U.S.

VY-AADC: Durable, Clinically Meaningful Improvements in Good "ON-Time"

2.8 hour improvement at 1 year in Cohort 2/3 combined and Phase II eligible group (n=7)



Source: Voyager Therapeutics press release 11/7/18
Note: Estimated placebo data from Marks et al, Lancet Neurol, 2010; Olanow et al, Ann Neurol, 2015

VY-FXN01: Gene Therapy for Friedreich's Ataxia

Discovered by Voyager Therapeutics

The Need: **Friedreich's Ataxia**

~6,400
patients impacted in the U.S.

Autosomal Recessive Disorder
Mutations in the frataxin (FXN) gene reduce production of frataxin protein

Typical age of onset is 10-12 years
and life expectancy is reduced to 35-45 years due to cardiac and neurological complications

Current treatments manage symptoms but
do not modify disease progression

The Opportunity: **VY-FXN01**

- **One-time treatment** to deliver the functional FXN gene and restore frataxin levels to at least 50% of normal in relevant neurons and cardiac myocytes
- **Novel BBB penetrant capsids** to optimize delivery and CNS transduction
- **Systemic delivery** of VY-FXN01 allows for administration to the affected tissues in the heart and CNS
- **Promising preclinical data** in knockout mouse model of Friedreich's ataxia

*VY-FXN01 is investigational and not approved in the U.S.

Our Vision
for the Future

2019: A Defining Year of Significant Milestones

2 NDA Submissions: Parkinson's Disease & Uterine Fibroids

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Progressing Early Stage R&D

Progress **Voyager Therapeutics** gene therapy collaboration - advance Phase II Parkinson's disease program and select **lead candidate** for Friedreich's ataxia

Continued **advancement of compounds in clinical development pipeline**

Neurocrine Biosciences: 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

3 Approved Medicines in 4 Indications in 2020

Strategic Partnerships

abbvie

Mitsubishi Tanabe Pharma America

jnana Therapeutics

Voyager THERAPEUTICS

Bial

Solid Financial Position to Invest

~$700MM Cash and Investments Following $165MM Upfront Payment for Voyager Collaboration

Neurocrine® BIOSCIENCES

Advancing Life-Changing Discoveries in Neuroscience

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April 29, 2019

neurocrine.com

NASDAQ: NBIX