Neurocrine Biosciences, Inc.

ADDRESSING CRITICAL UNMET NEEDS IN NEUROLOGY AND ENDOCRINOLOGY
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# Clinical Pipeline – Today

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<th>Target</th>
<th>Partner</th>
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<th>2</th>
<th>3</th>
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2017 Anticipated Clinical / Regulatory Events

• NDA Actions
  » FDA decision on INGREZZA NDA for Tardive Dyskinesia
  » Elagolix NDA Filing for Endometriosis
  » End of Phase II meeting for INGREZZA in Tourette Syndrome

• Clinical Trial Readouts
  » Phase 3
    • Elagolix for Uterine Fibroids (initial top-line data)
  » Phase 2
    • Adult and Pediatric Tourette Studies ⇒ Calendar Turn end of ‘16
  » Phase 1
    • CRF Antagonist in Congenital Adrenal Hyperplasia

• Clinical Trial Initiations
  » Phase 3
    • INGREZZA in Tourette Syndrome
  » Phase 2
    • Essential Tremor
Preparing for Commercialization in 2017

• Marketing
  • Market Research & Planning
  • Digital PR
  • Healthcare Professional Promotion
  • Patient Advocacy
  • Market Access
    • Field account executives
  • Commercial Analytics & Operations

• Sales
  • National & Regional Management
  • Field Operations & Training
  • Field Representatives - 150 to be hired

• Medical Affairs
  • Medical Communications
  • Training & Operations
  • Metrics
  • Health Economic Outcomes Research
  • Medical Science Liaisons – 12
INGREZZA
Tardive Dyskinesia

Selective, once-daily VMAT-2 Inhibitor for Movement Disorders
Tardive Dyskinesia (TD) Epidemiology
Current Picture: US

17MM
...with Schizophrenia, Bipolar Disorder, or MDD

6MM
...on Antipsychotics

2MM
...with a Movement Disorder and Using an Antipsychotic

500k
...with TD

280k
...with Moderate to Severe TD

In a Condition with No Standard of Care, TD is a Large Underserved Market With the Opportunity to Increase in Size By...

1. Reducing undercoding of TD
2. Improving accuracy of coding

Values rounded to nearest 500k-million.
Kinect 3 Topline Results – Efficacy

AIMS assessments at screening and every two weeks thereafter

- Placebo (n=80)
- 40mg (n=80)
- 80mg (n=80)

Blinded 1-Year Safety Extension
Placebo randomized to 40mg or 80mg of INGREZZA

Week 6
Primary Endpoint

Kinect 4 - Separate One-Year Safety Study (n=150)
Kinect 3: AIMS Primary Endpoint Met

AIMS Score Change from Baseline (LS Mean ±SEM)

Placebo N=76
Valbenazine 40mg N=70
Valbenazine 80mg N=79

-0.1
-1.9
p<0.000
p=0.002
p=0.002

Week 6, ITT
AIMS Score Change by Study Visit

Week 0  | Week 2  | Week 4  | Week 6
---|---|---|---
Placebo (n=76)  | Valbenazine 40 mg (n=70)  | Valbenazine 80 mg (n=79)

*P<0.05 vs. placebo
AIMS responders (%)

- AIMS dyskinesia total score reduced by ≥ 50% from baseline to Week 6

Week 6, ITT

Placebo: N=76, 9% responders
Valbenazine 40 mg: N=70, 24% responders, p=0.02
Valbenazine 80 mg: N=79, 40% responders, p<0.0001

Kinect 3: AIMS Responder Rate Higher with Valbenazine
Kinect 3 Topline Results – Efficacy

» **Week six reduction in AIMS (ITT population)**
  - 80mg dosing group 3.1 points (Least-Squares Mean) greater than PBO
    - *P*-value <0.0001
  - 40mg dosing group 1.8 points (Least-Squares Mean) greater than PBO
    - *P*-value 0.0021

» **Week Six reduction in AIMS (Per-Protocol population)**
  - 80mg dosing group 3.6 points (Least-Squares Mean) greater than PBO
    - *P*-value <0.0001
  - 40mg dosing group 2.1 points (Least-Squares Mean) greater than PBO
    - *P*-value 0.0009

» **Week Six reduction in CGI (Per-Protocol population)**
  - 80mg dosing group 0.4 points (Least-Squares Mean) greater than PBO
    - *P*-value 0.0122
  - 40mg dosing group 0.4 points (Least-Squares Mean) greater than PBO
    - *P*-value 0.0097
Efficacy by Concomitant Antipsychotic Use

**A. AIMS Score Change from Baseline to Week 6**

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<tr>
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<th>All Subjects</th>
<th>Atypical Antipsychotics</th>
<th>Typical Antipsychotics</th>
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<td>n</td>
<td>44 45</td>
<td>24 25</td>
<td>7 5</td>
<td>11 13</td>
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<tr>
<td>Mean Change from Baseline</td>
<td>-1.1 -3.6</td>
<td>-1.5 -2.6</td>
<td>-0.4 -2.4</td>
<td>-5.9</td>
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- Placebo
- Valbenazine

**B. CGI-TD Score at Week 6**

<table>
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<tr>
<th></th>
<th>All Subjects</th>
<th>Atypical Antipsychotics</th>
<th>Typical Antipsychotics</th>
<th>None</th>
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<tbody>
<tr>
<td>n</td>
<td>44 45</td>
<td>24 25</td>
<td>7 5</td>
<td>11 13</td>
</tr>
<tr>
<td>Mean Score</td>
<td>3.1 2.3</td>
<td>3.2 2.5</td>
<td>2.9 1.8</td>
<td>3.2 2.2</td>
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</table>

- Placebo
- Valbenazine
Kinect 3: No Clinically Important Dose-Dependent Differences by AE

| Study 1304: AEs Reported in ≥ 2%, and Higher than Placebo (Placebo-controlled Period) | Valbenazine |
|---|---|---|---|
| | 40 mg N=72 | 80 mg N=79 | Placebo N=76 |
| Any AE | 40% | 51% | 43% |
| Somnolence | 6% | 5% | 4% |
| Akathisia | 4% | 3% | 1% |
| Dry mouth | 7% | - | 1% |
| Arthralgia | 1% | 4% | 1% |
| Dyskinesia | - | 4% | - |
| Vomiting | - | 4% | - |
| Anxiety | 1% | 3% | - |
| Fatigue | 3% | 1% | 1% |
| Weight increased | 1% | 3% | - |
| Insomnia | 1% | 3% | 1% |
INGREZZA
Tourette Syndrome

Selective, once-daily VMAT-2 Inhibitor for Movement Disorders
Prevalence Rate:
  » 0.6-1.1%

Estimated 400k patients in the U.S.
  » 250-300k with moderate-severe symptoms

4:1 in boys:girls

Only 1 new FDA approved therapy in past 30 years
  » Approved therapies include haloperidol, pimozide [risk for Tardive Dyskinesia], and aripiprazole
T-Forward - Phase 2 Adult Tourette Study

Data expected calendar turn 2016

Assessments at screening and every two weeks thereafter

- Primary = Yale Global Tic Severity Scale
- Secondary
  - Rush Video Scale
  - Premonitory Urge for Tics Scale

Screening

Placebo (n=30)

Dose 1 (n=30)

Dose 2 (n=30)

Week 8 Primary Endpoint
T-Force GREEN - Phase 2 Child/Adolescent Tourette Study

Data expected calendar turn 2016

Assessments at screening and every two weeks thereafter

- Primary = Yale Global Tic Severity Scale
- Secondary
  - Rush Video Scale
  - Premonitory Urge for Tics Scale

*Two-week off-drug follow-up
U.S. Patents – Composition of matter expiry Oct 2029
  » Potential Hatch/Waxman provision could extend to 2034
  » Claims cover composition of matter, pharmaceutical composition, methods of use for movement disorders and VMAT2 inhibition

Corresponding patents in Europe and Japan

Protection extends to most major markets globally
Elagolix
Endometriosis

First-in-class, orally-administered GnRH Antagonist
Claims Data Define a Large And Underserved Endometriosis Patient Population That Will Increase in Size with Awareness

75MM
Women of Reproductive Age (15-49)

7.5MM
With Endometriosis

5MM
Seeking Treatment With Endometriosis-related Symptoms

2.5MM
Diagnosed / Treated

A Significant Opportunity Today and For Growth in the Future

Values rounded to nearest 500k-million.

Truven Health Analytics
The Effect Of Elagolix On The Endometrium: Safety Results From Two Randomized, Placebo-controlled Studies In Women With Endometriosis-associated Pain

Participants were:
- premenopausal women (18-49 years)
- surgically diagnosed with endometriosis
- moderate/severe dysmenorrhea and non-menstrual pelvic pain
Endometriosis-associated Pain Assessments
• DYS and NMPP were each assessed using a 4-point scale (none, mild, moderate, severe pain), recorded in a daily, electronic, endometriosis pain impact diary.

Quality of Life Assessment
• 30-item Endometriosis Health Profile (EHP-30) is a self-administered questionnaire:
  • 5 core dimensions
    – 11 questions assessing pain
    – 6 questions assessing control and powerlessness
    – 6 questions assessing emotional well-being
    – 4 questions assessing social support
    – 3 questions assessing self-image
  • 1 modular questionnaire composed of 5 questions about sexual intercourse was included
• Each EHP-30 question was scored from 0 [never] to 4 [always]), measured at baseline and months 1, 3 and 6, and normalized to a scale of 0-100 for each dimension. Lower scores indicate better quality of life.

Safety
• Adverse events and changes in bone mineral density were recorded.
Elagolix Met Co-primary Endpoint in Phase 3 Program

**Dysmenorrhea**

Compared to placebo, there were significant, dose-dependent increases in the proportion of dysmenorrhea responders and decreases (improvement) from baseline in mean dysmenorrhea scores with elagolix treatment.

*Results were similar in Study 2.*
Elagolix Met Co-primary Endpoint in Phase 3 Program
Non-Menstrual Pelvic Pain

Non-Menstrual Pelvic Pain Responders

Co-primary Endpoint

Compared to placebo, there were significant, dose-dependent increases in the proportion of non-menstrual pelvic pain responders and decreases (improvement) from baseline in mean non-menstrual pelvic pain scores with elagolix treatment.

Results were similar in Study 2.
Mean Percentage Change from Baseline to Month 6 in Bone Mineral Density of the Lumbar Spine, Total Hip, and Femoral Neck

**Pivotal 1**

- **Lumbar Spine**: 0.47
- **Total Hip**: 0.22
- **Femoral Neck**: -0.32

**Pivotal 2**

- **Lumbar Spine**: 0.56
- **Total Hip**: 0.59
- **Femoral Neck**: 0.32

**Placebo**

**Elagolix 150mg QD**

**Elagolix 200mg BID**
Effects of Elagolix on the EHP-30 Pain Dimension

Compared to placebo, there were significant, dose-dependent decreases (improvements) from baseline in mean EHP-30 pain scores with elagolix treatment.

The statistical significance vs. placebo is indicated for $P<0.05$ (*), $P<0.01$ (**), and $P<0.001$ (***)

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Effects of Elagolix on the EHP-30 Control and Powerlessness Dimension

Compared to placebo, there were significant, dose-dependent decreases (improvements) from baseline in mean EHP-30 control and powerlessness scores with elagolix treatment.

The statistical significance vs. placebo is indicated for $P<0.05$ (*), $P<0.01$ (**), and $P<0.001$ (***).
Compared to placebo, 6 months of elagolix treatment led to:

- A dose-dependent reduction in endometrial thickness
- An increase in the percentage of normal quiescent/minimally stimulated patterns, with no adverse endometrial findings, such as hyperplasia
- A dose-dependent increase in the percentage of women with amenorrhea
Summary of Violet Petal and Solsticce

• **Efficacy**
  - Both studies displayed consistent efficacy at all time points for both doses
  - 12 month time point for Violet Petal was consistent with 6 month time point

• **Safety**
  - Elagolix was well tolerated in both studies
  - Bone mineral density (BMD) mean change from baseline was numerically similar in each study, however, Violet Petal showed less BMD change than Solstice for the 150mg dose
Elagolix
Uterine Fibroids

First-in-class, orally-administered GnRH Antagonist
Uterine Fibroids is a Large Underserved Market In Need of an Effective Pharmaceutical Agent

75MM
US Women of Reproductive Age (15-49)

9MM
Seeking Treatment with UFRS

4MM
Seeking Treatment with UFRS-B

3MM
UF Diagnosed / Treated

A Significant Opportunity Today and For Growth in the Future

>250K Hys

UFRS = Uterine Fibroid related symptom
UFRS-B = UFRS bleeding only
Elagolix Phase 2b UF Study Design

Primary Endpoint: Reduction in Uterine Blood Flow
Additional Details on Phase 2b Study

- **Primary Efficacy Endpoint** –
  - Reduction in uterine blood flow, treatment month six compared to baseline period (alkaline hematin assay)

- **Secondary Endpoints (baseline to treatment month 6)**
  - Number of bleeding days
  - Menstrual bleeding scores (days of bleeding, severity)
  - Uterine volume
  - Hemoglobin concentration
  - Fibroid volume
  - BMD (safety)

- **Study subject details**
  - ~520 pre-menopausal women
  - Confirmed diagnosis of uterine fibroids via ultrasound
  - Heavy uterine bleeding associated with fibroids
Elagolix UF Efficacy From AbbVie R&D Day 6/3/16

**Heavy Menstrual Bleeding**

- PBO (n=64): 26.6%
- E300 BID (n=62): 91.9%
- E300 BID + add-back (n=620): 79%

**Bone Mineral Density**

- Placebo: 0.78%
- E Alone: -3.59%
- E + add-back: -0.12%

---

E = Elagolix 300 mg BID

LDA = low-dose Activella = Estradiol/Norethindrone Acetate (E2 0.5 mg/ NETA 0.1 mg) QD

SDA = standard dose Activella (add back) = (E2 1.0 mg/ NETA 0.5 mg) QD

*P vs. PBO: <0.001;  
**P vs. PBO: 0.148
Two Initiated Phase 3 Studies

• Both Phase 3 pivotal studies of elagolix in uterine fibroids began in the first quarter of 2016

• Studies include two replicate, pivotal, six-month efficacy and safety clinical trials followed by a six-month safety and efficacy extension study evaluating 400 women per trial

• The primary endpoint in Phase 3 studies will be the same as that employed in the Phase 2b study; percent of subjects with reduction in uterine blood flow as measured by the alkaline hematin method

• Top-line efficacy data expected in 2017
• 6 Issued U.S. Patents covering:
  » Composition of matter
  » Pharmaceutical composition
  » Methods of use for endometriosis and uterine fibroids
• Patent term expire 2024 (not including potential patent term extension of approximately 5 years)
• Corresponding Patents issued in Japan and Europe (expiring in 2024)
• Protection extends to most major markets globally
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<td><strong>Revenues and other Income</strong></td>
<td>$ 25.1 million</td>
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<td><strong>Operating Expenses</strong></td>
<td>$ 114.0 million</td>
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<td><strong>Net Loss</strong></td>
<td>$ (88.9 million)</td>
<td>$ (96.4 million)</td>
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<td><strong>Cash, Investments, Receivables</strong></td>
<td>$ 464.3 million</td>
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2017 Milestones

• January – Topline Data Adult Tourette Study
• February - Anticipated Advisory Committee Meeting
• March – Topline Data Juvenile Tourette Study
• April – PDUFA Date for Ingrezza
• June – Ingrezza Commercial Sales Team Launch
• 1\textsuperscript{st} Half – Begin PoC Study in Essential Tremor
• 1\textsuperscript{st} Half – Begin Phase 1b in Congenital Adrenal Hyperplasia
• 2\textsuperscript{nd} Half – New Compound Enters Clinical Development
• 3\textsuperscript{rd} Quarter – NDA Filing Elagolix for Endometriosis
• 4\textsuperscript{th} Quarter – Topline Phase 3 Data Elagolix for Uterine Fibroids
Neurocrine Biosciences, Inc.

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