33rd Annual JPMorgan Healthcare Conference

Monday, January 13, 2014
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Acorda’s Corporate Objective

To be the leading biopharmaceutical company delivering therapies that restore neurological function and improve lives
2013 Achievements

- AMPYRA® unaudited net revenue of ~$302 million; up 13.5% from 2012
- Announced positive Phase 2 data for dalfampridine in post-stroke deficits
- Obtained two new U.S. AMPYRA patents and successfully defended a European patent against opposition
- Filed NDA for Diazepam Nasal Spray
- Acquired neuropathic pain assets: Qutenza® and NP-1998
- Initiated second Phase 1 trial of GGF2 in heart failure
- Initiated Phase 1 trial of rHIgM22 in multiple sclerosis
- Initiated Phase 2 trial of AC105 acute spinal cord injury
# Acorda’s 2011 Pipeline

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<thead>
<tr>
<th>THERAPY</th>
<th>R&amp;D</th>
<th>PRE CLINICAL</th>
<th>PHASE 1</th>
<th>PHASE 2</th>
<th>PHASE 3/4</th>
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<tbody>
<tr>
<td>AMPYRA®</td>
<td>Walking in MS</td>
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## Acorda’s Pipeline in 2013

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<tr>
<td>QUTENZA®</td>
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AMPYRA Commercial Update

• 2013 net revenue of ~$302 million
• 4Q13 net revenue of ~$84 million
• Over 85,000 patients have tried AMPYRA to date
• Continued modest organic growth expected, coupled with price increase
AMPYRA Net Sales Launch to 2013

* Ten months, Mar – Dec ‘10
**Unaudited; audited 2013 net sales figures not yet available
AMPYRA U.S. Exclusivity

- 4 Orange Book patents providing protection to 2027
- New patent allowed in 2013, awaiting issuance
- Several additional applications pending
- Orphan exclusivity until 2017
Dalfampridine in Post-Stroke Deficits
Stroke Overview

• ~7 million people in U.S. have had a stroke\(^1\)
  – ~Half have mobility issues
  – ~800,000 new cases/yr
• Annual U.S. stroke costs ~$38.6 billion
• No drug therapy indicated for people with post-stroke walking deficits
• Successful dalfampridine proof-of-concept (POC) study completed 2013

\(^1\)American Heart Association 2012
POC Study: Overall Timed Walk Result

N=78, p=0.027

Note: p value from a mixed statistical model with sequence, period, visit and treatment as fixed effects and subject as a random effect.
Percentages of Patients Reaching Threshold Change from Baseline in Walking Speed by Period

Period 1

Period 2

Increase in Walking Speed from Baseline

Patients (%)
Post-Stroke Deficits Phase 3 Next Steps

• FDA meeting in December 2013
  – Integrating design recommendations into Phase 3 protocol
• Phase 3 study planned to begin Q2 2014
  – Pending QD study results and FDA agreement on final protocol
• Key design elements
  – Parallel group study comparing two doses to placebo
  – Walking as primary endpoint
  – Interim analysis: potentially accelerates second Phase 3 study
Diazepam Nasal Spray
Cluster Seizures: U.S. Epidemiology

~2,300,000 people with active epilepsy

~1/3
Treatment-resistant

~760,000 people with treatment-resistant epilepsy

~175,000 people with cluster seizures

Source: Martinez C et al; "Prevalence of Acute Repetitive Seizures (ARS) in the United Kingdom." Epilepsy Research; V.87; 2009
Diazepam Nasal Spray Opportunity

• Diastat® (diazepam rectal gel) is only approved outpatient therapy for cluster seizures
  – Primarily used in pediatric patients
  – Diastat peak sales of $100 million
• Significant underserved market
  – Limited Diastat uptake in adult market
• High enthusiasm among physicians, patients and care-givers for an intranasal option
Diazepam Nasal Spray Overview

- NDA filed in 2013
- 505(b)(2) based on Diastat (diazepam rectal gel)
  - Diastat indicated for increased bouts of seizure activity ("cluster seizures")
- Exclusivity
  - Orphan drug: 7 years
  - Issued patent to 2029
- Potential launch in 2014
  - Commercial launch readiness ongoing
- Leverages existing sales infrastructure
Comparable PK for Intranasal Spray and Rectal Gel Diazepam

Clinical Stage Programs
GGF2 (USAN: cimaglermin alfa)

- Natural growth factor related to EGF
- Therapeutic targets for treatment of cardiac and neurological repair
- Phase 1 study in Chronic Heart Failure completed
  - Tolerability of a single infusion over a range of doses
  - Cardiac function measured by ejection fraction
- Fast Track designation from FDA
Phase 1 Study Results

- Favorable safety and tolerability through 0.75 mg/kg
- Dose-limiting toxicity at 1.50 mg/kg
  - Hepatotoxicity in one patient (met Hy’s Law criteria)
  - Transient and reversible
  - Most common AE: headache
- Dose-response trend for improved cardiac function following single doses
- Dose-response and dose-limiting toxicity consistent with in vivo models
Change in EF Following Single Dose of GGF2 or Placebo

ΔEF (versus screening)

Baseline EF for Placebo/Treatment cohorts: ~ 29%

Days post Treatment

GGF2 (mg/kg)

- Placebo
- 0.007
- 0.021
- 0.063
- 0.189
- 0.378
- 0.756
- 1.515
Second Phase 1 Clinical Trial in Heart Failure

• Three dose levels for tolerability and several exploratory measures of efficacy
  – Ejection fraction
  – Measures of endurance
  – PK interaction

• Trial initiated September 2013
  – Estimated enrollment: 28

• Paused enrollment in December 2013 pending review of additional preclinical data with FDA
• Current immunomodulatory therapies for MS reduce frequency of relapses
• Remyelinating therapies are the next potential major advance in MS
  – More than 400,000 people in U.S. with MS
• Recombinant human IgM effective in promoting remyelination and improving function in preclinical models

rHlGm22: Remyelinating Therapy for MS
rHlgM22: Remyelinating Antibody

- Directly stimulates remyelinating cells
- Phase 1 trial in MS patients
  - Single ascending dose
  - With or without current MS medications
  - Any disease type
  - Estimated enrollment: 60
  - Estimated study completion: 1Q15

- Outcomes:
  - Safety and tolerability
  - Functional measures
  - Imaging and biomarkers
Neuropathic Pain Assets

• **QUTENZA®** (capsaicin) 8% patch
  – FDA-approved for post-herpetic neuralgia
  – Commercial relaunch in January 2014 using existing infrastructure

• **NP-1998**
  – Phase 3 ready, topical solution containing 20% prescription strength capsaicin
  – Potential to address significantly larger neuropathic pain markets
    • Astellas currently studying Qutenza in painful diabetic neuropathy (PDN)
  – Evaluating quickest route to market
## U.S. Prevalence of Neuropathic Pain

<table>
<thead>
<tr>
<th>Condition</th>
<th>2011</th>
<th>2016</th>
<th>2021</th>
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<tbody>
<tr>
<td>Painful diabetic neuropathy</td>
<td>3.2M</td>
<td>3.8M</td>
<td>4.3M</td>
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<tr>
<td>HIV/AIDS-related neuropathy</td>
<td>566,530</td>
<td>615,500</td>
<td>657,490</td>
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<tr>
<td>Post-herpetic neuralgia</td>
<td>172,940</td>
<td>153,360</td>
<td>149,900</td>
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NP-1998 Overview

• Liquid formulation containing 20% capsaicin
  - Applied to the patient using an applicator
• 15-minute total procedure time
• Phase 2 PHN study: single 5-minute application provided up to 3 months of pain relief
• U.S. patent extends to 2027
AC105 Overview

- Proprietary neuroprotective magnesium formulation
- Preclinical studies showed improved recovery of motor function
- Phase 2 trial initiated
  - U.S. Department of Defense $2.67M contract
  - Fast track designation for SCI
- No FDA-approved therapies to treat acute SCI
Business Development & 2014 Highlights
Business Development Strategy

• Leverage neurology and specialty commercial expertise and infrastructure
• Focus on late-stage/commercial products that can be accretive in the near-term
• Open to earlier-stage products with innovative science and significant unmet medical need
2014 Highlights

- Continued AMPYRA growth
- Phase 3 trial initiation for QD dalfampridine in post-stroke deficits
- Potential Diazepam Nasal Spray approval
- Re-launch of QUTENZA patch
- Development plan for NP-1998
- Continued clinical development of GGF2, rHIgM22, AC105
2014 Financial Guidance

- AMPYRA U.S. net sales: $328-$335 million
- FAMPYRA® & Zanaflex® revenue: ~$25 million
- SG&A: $180-$190 million
- R&D: $60-$70 million
### Financial Summary

<table>
<thead>
<tr>
<th>($ in millions)</th>
<th>Quarter Ended</th>
<th>YTD</th>
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<tbody>
<tr>
<td></td>
<td>9/30/2013</td>
<td>9/30/2012</td>
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<tr>
<td>Cash, cash equivalents, short and long-term investments</td>
<td>$349.4</td>
<td>$318.7</td>
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<tr>
<td>Net Ampyra revenue</td>
<td>$77.8</td>
<td>$69.8</td>
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<td>Zanaflex branded/authorized generic revenue</td>
<td>$1.8</td>
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<td>Royalty revenue</td>
<td>$2.9</td>
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<tr>
<td>Total revenues</td>
<td>$84.9</td>
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<tr>
<td>Total operating expenses</td>
<td>$73.5</td>
<td>$67.1</td>
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<tr>
<td>Non-GAAP net income</td>
<td>$15.0</td>
<td>$15.2</td>
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This slide contains GAAP and non-GAAP financial measures. Non-GAAP net income excludes certain items. Information regarding our use of non-GAAP measures, a description of excluded items, and a reconciliation of those measures to GAAP is available in our third quarter 2013 financial results press release, which is now available in the investor relations section of website at www.acorda.com.