Developing clinical-stage products in neurology, psychiatry and orphan indications

BUSINESS UPDATE CONFERENCE CALL

April 9, 2015
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Agenda

• INTRODUCTION
• FINANCIAL REVIEW AND OUTLOOK
• THERAPEUTICS DIVISION REVIEW
• DIAGNOSTICS DIVISION REVIEW
• 2015 EXPECTED MILESTONES
• Q&A
# Focused Execution Delivers Rich Pipeline

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<th>Asset</th>
<th>Pre-Clinical</th>
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<th>Value Driving Milestone</th>
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<td><strong>Eltoprazine: PD-LID</strong></td>
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<td>Potential strategic transaction in 2015</td>
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<td><em><em>ESS</em>: 50+% TBSA Severe Burns</em>*</td>
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<td>Phase 2b program clinical data in 2016</td>
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<td><strong>MANF: Retinitis Pigmentosa (Orphan)</strong></td>
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<td>Potential PoC in orphan ocular in 2018</td>
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* = upon exercise of exclusive option to acquire ESS from Lonza
A Year of Building Solid Fundamentals

Executed our strategy to assemble undervalued clinical-stage assets and incubate to significant value inflections

**Therapeutics Division**
- Eltoprazine platform establishes neurology/psychiatry pipeline
- Opportunistic potential acquisition of ESS diversifies pipeline with clinical-stage, ultra-orphan program
- MANF orphan ocular strategy establishes fastest path to market

**Diagnostics Division**
- LymPro Test® IUO commercialization expands Alzheimer’s diagnosis market
- MSPrecise® poised for staged near-term commercial opportunity
- Georgetown assays position company to control significant market share in the emerging AD IUO blood diagnostics market
- Premier suite of diagnostics creates optionality for exit strategy
Financial Review and Outlook
Path to Eventual Up-listing

Access to Lincoln Park Capital Facility

Series E primarily for ESS and MSP precise transactions

Exploring funding options to execute plan

Eventual Listing on National Exchange

Strategic Transaction to fuel pipeline
Therapeutics Division Review
Therapeutics Division: Milestone Achievements

Eltoprazine
- Published Phase 2a clinical study results in BRAIN for the treatment of PD-LID
- Opened an IND application with the neurology division of the FDA to advance Eltoprazine into Phase 2b clinical studies

MANF
- Received Orphan Drug Designation (ODD) from the FDA for the treatment of RP
- Submitted an application to the FDA for ODD for the treatment of retinal artery occlusion (RAO)
- Announced positive preclinical data on the effects of MANF for the protection from vision loss in animal models of RP and RAO

ESS
- Entered into exclusive option agreement with Lonza Walkersville to acquire subsidiary Cutanogen Corporation, holder of licensing rights to intellectual property related to ESS for the potential treatment of severe burns
- Dismissed with prejudice the litigation that had previously encumbered ESS
- Amended the Lonza exclusive option agreement allowing for the extension of the option period through August 31, 2015
Eltoprazine Ready to Commence Phase 2b Program in PD-LID

**Exceptional safety profile:**
- Administered to 682 humans (volunteers and patients)
- Up to two years dosing studied by Solvay (now Abbvie)

Clinical Indications
- Parkinson’s disease (PD) L-Dopa Induced Dyskinesia (LID):
  - **Open IND with Phase 2b ready to commence 2Q 2015**
  - Retained Chiltern as CRO for US/EU clinical study
  - Clinical data published in Brain (2/15); no L-Dopa interference
  - Strong secondary endpoints achieved in psychiatric aspects of PD
- Alzheimer’s aggression: Phase 2 being evaluated
  - Data package in aggression produced by Solvay (now Abbvie)
- Adult ADHD: Phase 2 complete
  - Positive Phase 2 data on attention & hyperactivity/impulsivity in adults
Eltoprazine: PD Market Opportunity

- 1M+ Americans patients have PD
- 60-80% diagnosed with PD-LID
- 60,000 new diagnoses annually
- 3M million by 2032
- Total cost to U.S.: $25B
- Key unmet medical need: LID
  - Other PD symptoms addressed: Cognition, other psychiatric measures
- Market opportunity: $750M in US*
- Patent pending: protection through 2031
  - New Chemical Entity (NCE)
  - Regulatory Exclusivity Pathway

*Source: MJFF Foundation
MANF has “Blue-Sky” Potential

**Preclinical Programs**

- Potential paradigm shift in cell protection and restoration
- $Multi-billion opportunity
- Lead programs in orphan ocular indications
  - Retinitis Pigmentosa (orphan granted)
  - Retinal artery occlusion (potential orphan)
  - Wolfram’s (potential orphan)
- Potential in other indications
  1. Parkinson’s
  2. Diabetes
  3. Myocardial infarction
  4. Hearing loss (potential orphan)
  5. Wound healing
  6. Other apoptosis-related disorders
ESS Acquisition Could Add Significant Value

- Autologous, skin graft replacement for 50+% TBSA severe burns
- Biologics/drug regulatory pathway in office of combination products
- Orphan Drug Designation received in 2012
- Active IND as of May 2014
- Partially funded by US Gov’t grant: AFIRM ($725k remaining on current grant)
  - Project has been funded by DoD for last 10+ years
- Patient Population: ~2000 average patients per year
  - Cost of treatment per patient: $1.6M, w/ complications: $10M+
- Secondary applications: pediatric burn 30%+ burns, diabetic foot ulcers, cosmetics
- Initiating 10 patient Phase 2 clinical trial
## Preparing for “Exit Strategy” to Unlock Value

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<th>Diagnostic Candidates</th>
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<th>Analytical Performance</th>
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*Upon exercise of exclusive option

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Exploring Strategic Options for Monetization
Diagnostics Division: Pipeline Achievements

**LymPro Test®**

- Presented positive LymPro data at the 12th International Conference on Alzheimer’s and Parkinson’s Diseases and Related Neurological Disorders
- Established the Company’s first Investigational Use Only (IUO) Alzheimer’s biomarker services collaboration with Anavex Life Sciences Corp.
  - Entered into a Letter of Intent with Anavex to plan additional scope of further biomarker services for its next Alzheimer’s clinical study (Phase 2/3)
- Announced the availability of LymPro Test biomarker services for use by the pharmaceutical industry for IUO

**MSPrecise®**

- Acquired MS diagnostics company Diogenix, Inc. to bolster near-term revenue
- Completed integration of Diogenix into Amarantus Diagnostics corporate infrastructure

**Georgetown Assays**

- Entered into a one-year, exclusive option agreement with Georgetown University to license patent rights for blood based biomarkers for AD and memory loss
LymPro Status and BD Strategy

- Currently available as IUO for clinical trials
- Completing multivariate analysis to support CLIA pathway
- Business development initiative commenced 4Q 2014
- **Moving forward with robust BD strategy**
  - Recent hiring of Ravi Kiron bolsters capabilities

6-12 month sales cycle initiated at CTAD (November)
Compelling Commercial Opportunity

- Highly differentiated lead diagnostic that will significantly improve the diagnostic paradigm in MS as an adjunct to standard of care (oligoclonal banding)
- Peak sales potential in North America of ~$300M
- ~200 US MS clinics allowing for economical selling and marketing support
- Potential to transition into blood test
- Strong pharmacoeconomic reimbursement rationale for payers
  — Due to strong MS drug pricing and high misdiagnosis rate
- Initial regulatory pathway: LDT under CLIA
- Expected commercial launch 4Q 2015
Positioning Division as Potential Market Leader in Neurodiagnostics

- MSPrecise product ready to initiate commercialization process
  - Strong pharmacoeconomic rationale for reimbursement
  - CLIA commercialization pathway being prosecuted to deliver in 2015
  - $300 million peak sales potential in US
- Positioned as leading Alzheimer’s blood-based biomarker service provider
  - LymPro Test, Exosome (amyloid & tau), Lipids
    - Screening of subjects to enrich clinical trial populations
    - Longitudinal comparison of pharmacodynamic activity
  - Distribution channel in place to support US/EU clinical trials
  - ~$150M IUO market for pharma trials / ~$3B commercial market
Significant Steps Taken to Prepare for “Exit”

- Retained an executive search firm to identify a Chief Executive Officer for the Diagnostics division
- Retained Ravi Kiron, Ph.D. as Senior Vice President of Business Development
- Promoted Colin Bier, Ph.D., to Chief Development Officer to oversee the commercialization of the Company’s assays under CLIA
- Retained a consulting firm specialized in the sale of tax credits, to market the $7.5 million of New Jersey tax credits obtained in the Diogenix acquisition
- Established an Alzheimer’s disease Diagnostics Scientific Advisory Board with three internationally-renowned AD and neurological disorder specialists, Paula T. Trzepacz, M.D., Jeffrey L. Cummings, M.D., Sc.D., and Robert A. Stern, Ph.D.
“Exit” Strategy Accelerated as a Priority

- Evaluating strategic options for the diagnostics business unit
- Potential options under consideration
  - Potential sale of the division for cash (and maintain a royalty)
  - IPO or RTO
  - License the technologies to a third party
  - Evaluate a combination of the above
- Intend to focus on maintaining a significant financial interest in the diagnostics business
- Will allow us to focus internal resources on Therapeutics division

Transaction expected to fuel advancement of Therapeutics pipeline
2015 Expected Milestones

• Initiate Phase 2b clinical study of Eltoprazine in PD-LID in 2Q 2015
• Complete enrollment of Phase 2b clinical study of Eltoprazine in PD-LID
• Complete acquisition of Cutanogen
  – Initiate Phase 2 study of ESS mid-year 2015
• MANF progression towards first-in-man:
  – RP ODD application in EU
  – Initiate GMP Manufacturing
  – RAO ODD applications from the FDA and EU
• Execute strategic transaction for Diagnostics division
• Pursuing National stock exchange up-listing
Developing clinical-stage products in neurology, psychiatry and orphan indications