ADVANCED PROTEOMICS AND FLOW CYTOMETRY APPROACHES FOR DEVELOPMENT OF BIOMARKERS AND PERSONALIZED MEDICINE

CAPRION PROTEOMICS

TVM Conference
October 7, 2013
CAPRION PROTEOMICS

• Founded in 2000

• Located in:
  – Montreal, Canada
  – Menlo Park, California

• 85 employees (65 scientists)

• Leading service provider to pharma, biotech and gov’t research institutions focused on personalized medicine and biologics drug development
  - Mass spec-based Proteomics technology for protein biomarker discovery and validation
  - Multiparametric flow cytometry for GLP immune monitoring services

• Developing diversified pipeline of In-vitro diagnostic product candidates in 3 major disease areas

• Venture backed from 2000-07 under Caprion Pharmaceuticals, Inc.

• Spun-out as independent service business in 2007

• Private equity ownership since 2007
  – Great Point Partners, LLC (2007-12)
  – Chicago Growth Partners (since July 2012)
DUAL-TRACK BIOMARKER BUSINESS MODEL

Proteomics and Biomarker Services

• Fee-for-service business model in partnership with pharma, biotechs and not-for-profit organizations

• Biomarkers/Personalized Medicine
  – Biomarker discovery - CellCarta
  – Multiplexed MRM-MS assays for biomarker verification & validation

• Drug Target discovery & validation

• GLP multi-parametric flow cytometry services for immune monitoring

• Over 50 leading pharma and biotech industry clients

• EBITDA positive since 2006

Diagnostic Pipeline

• Leverage non-dilutive funding for development of novel in-vitro diagnostic products:
  – Infectious disease
  – Diabetes
  – Oncology

• Discover and validate biomarker candidates & retain IP rights until clinical validation stage

• Pursue commercial development of LDT tests from CLIA lab operation in Montreal

• Out-license hospital-based tests to major Dx companies

• Over $30 million of non-dilutive funding secured since 2009
PARTNERSHIP-DRIVEN BUSINESS MODEL

Proteomics & Immunology Services

- Merck
- Janssen
- Pfizer
- Genentech
- Mitsubishi Tanabe Pharma
- Amgen
- Boehringer Ingelheim
- Seattle Genetics
- Astellas
- GSK
- Debiopharm
- Merck Serono
- Novartis
- Medicago
- Gilead
- Abbott Laboratories
- Takeda
- Amplimmune
- AstraZeneca
- Vertex
- Sanofi

In vitro Diagnostics

- Infectious diseases (2008-14)
  - National Institutes of Health
  - NIAID

- Oncology (2009-16)
  - Indi Integrated Diagnostics, Inc
  - PMPC
  - National Cancer Institute

- Diabetes (2009-13)
  - CQDM
STRONG REVENUE AND PROFITABILITY GROWTH TRACK RECORD

2009 - 2012E
Project CAGR = 54.7%
Revenue CAGR = 40.0%
EBITDA CAGR = 67.9% $18.2
TECHNICAL APPROACHES IN MASS SPEC-BASED PROTEOMICS

Biomarker Discovery (LC-MS/MS)
• Non-hypothesis based discovery approach
• Label-free, gel-free quantitative MS
• Shotgun sequencing
• Profile 1000’s of proteins in 100’s samples
• Identify differentially expressed proteins as candidate biomarkers

Multiplexed Assays (MRM)
• Targeted quantification of up to 350 specific proteins
• Target list from MS, literature, transcriptomics
• Rapid assay development
• Ab-free or Ab enrichment step
• Synthetic labeled standards for quantification
• Can be set up as diagnostic platform for LDTs
FLOW CYTOMETRY AND IMMUNE MONITORING SERVICES

- Established in 2004 in Montreal as National Immune Monitoring Laboratory (NIML)
- Acquired by Caprion from Genome Quebec, CHUM and UdeM in 2011
- Provides immune response monitoring assays to address discovery, pre-clinical, and Phase I-III clinical development objectives
- Develops and routinely performs 17-color flow cytometry assays to gather highly detailed and clinically critical information on immune responses to a therapeutic, or to vaccines
- Demonstrated ability to design, perform, and interpret both standardized and customized complex immunological assays in a Good Laboratory Practice (“GLP”) mode
- Performed over 50 immune monitoring studies for pharma, biotech and government institution clients
IMMUNECARTA- INTEGRATED FLOW CYTOMETRY
AND IMMUNE MONITORING SERVICES

Cell Enumeration:
of specific immune cells from blood or lymphoid tissues

Immune Phenotyping:
cellular differentiation, maturation, activation, senescence, inhibitory molecules, apoptosis

Functional Profiling:
cell signaling, cytokine secretion profile, proliferation, degranulation

Antigenic response, epitope mapping:
inflammatory or suppressive response to immune-modulating agents, cancer cells, auto-antigens

Serology & Soluble Markers:
soluble inflammatory mediators and antibody titers in response to immune modulating agents

- On-demand assay development, customization, and qualification/validation.
- High-throughput, GLP/GCLP flow cytometry for multi-parametric single-cell analysis.
- High-throughput analysis of complex immunological data sets and interpretation.
- Sample management, tracking and storage in a real-time monitored and controlled environment.
- Blood processing, cryopreservation and storage.
- On-site training for blood processing and lab manual preparation (for harmonization of multi-centric studies).
- Consulting Services (Immune monitoring study design, review of protocols, etc.).
CAPRION PARTNERING WITH Dx COMPANIES TO BRIDGE BIOMARKER INNOVATION GAP

Discovery
- Generation of a biomarker hypothesis
- Identification of candidate markers

Validation
- Develop prototype assay
- Clinical validation of candidate markers using assay
- Assay refinement & development IVD test
- Regulatory test approval

Commercialisation
- Distribution to laboratories
- Auditing for result consistency
- Clinician/Lab education

Traditional focus and Expertise of large diagnostic product companies

Innovation Gap
Caprion Expertise
**CAPRION IN VITRO DIAGNOSTIC PRODUCT DEVELOPMENT PIPELINE**

### Active Programs

<table>
<thead>
<tr>
<th>Disease Area</th>
<th>Programs</th>
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<tbody>
<tr>
<td><strong>Oncology</strong></td>
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<td>Lung nodules</td>
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<tr>
<td>Neuroendocrine tumors</td>
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<td><strong>Infectious diseases</strong></td>
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<td>Tuberculosis</td>
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<td>Brucellosis</td>
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<td>Lyme disease</td>
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<td>Pediatric fever</td>
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<td><strong>Diabetes</strong></td>
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<td>Disease progression</td>
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<td>Drug response prediction</td>
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Over $30 million of non-dilutive funding secured from gov’t contracts and industry partners for Dx candidate discovery and validation in 3 major disease areas since 2009

**Partners**

- Indi
- PMPC
- cqm
- NIAID

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CAPRION: INDUSTRY-LEADING SUPPLIER OF PROTEOMICS BIOMARKER SERVICES

• Over 45 Pharma and Biotech clients
  − Strong base of repeat customers
• Over 130 large-scale biomarker projects completed in last 4 years
  − Covering all major disease areas
  − Demonstrated experience and capabilities in analysis of broad range of human and animal model biological samples, including plasma, serum, CSF, urine, sputum, exudate, cells and tissues
  − Roughly equal split between pre-clinical and clinical studies
  − High customer conversion rate from biomarker discovery projects to multiplexed assay services for biomarker validation
• Significant revenue growth prospects in biomarker and personalized medicine market
• Scaleable and transferable platform, process, and infrastructure
• Pipeline of fully funded Dx product development programs with IP rights retained
Strong unmet need and Rx/Dx industry demand for innovative and validated companion Dx candidates

“Biomarker innovation gap” unlikely to be resolved by private sector alone due to prevailing Rx, Dx and VC industry dynamics

Significant business model challenges for biomarker discovery technology platform companies
   - Sustainability and risk-reward challenges of pure service model
   - Venture capital availability challenges for pure biomarker product development models

Caprion is one example of economically viable strategies from hybrid business models that leverage strategic funding from both public and private sector sources

Looking ahead: need to balance differing needs of service and diagnostics units to properly capitalize on growth potential from both divisions