Revolutionizing the Treatment of Cancer

June 2014
Safe Harbor Statement

The statements that follow (including projections and business trends) are forward-looking statements. Rexahn's actual results may differ materially from anticipated results and expectations expressed in these forward-looking statements, including as a result of certain risks and uncertainties, such as Rexahn's lack of profitability, the need for additional capital to operate its business to develop its product candidates; the risk that Rexahn's development efforts relating to its product candidates may not be successful; the possibility of being unable to obtain regulatory approval of Rexahn's product candidates; the risk that the results of clinical trials may not be completed on time or support Rexahn's claims; demand for and market acceptance of Rexahn's drug candidates; Rexahn's reliance on third party researchers and manufacturers to develop its product candidates; Rexahn's ability to develop and obtain protection of its intellectual property; and other risk factors set forth from time to time in our filings with the Securities and Exchange Commission. Rexahn assumes no obligation to update these forward-looking statements.
Identify novel drug targets which are specific to cancer cells:

- Increased efficacy, reduced toxicity
- Efficacy against multiple drug resistant cancer cells
- Synergism with existing cytotoxic compounds

Develop in-licensed targeted drug delivery platforms:

- Nano-Polymer-Drug Conjugate System (NPDCS) combines existing anticancer agents with a polymer/signaling moiety which directs the drug directly to a tumor
- Lipid-Coated Albumin Nanoparticle (LCAN) to enhance delivery of oligonucleotides
Rapidly advancing pipeline: Initiated three clinical trials in 2013 with data in 2014

**Pipeline**

- **Supinoxin (RX-5902):** Phosphorylated p68 RNA Helicase inhibitor. **Phase I** clinical trial in cancer patients with solid tumors initiated in August 2013.

- **RX-3117:** Next generation cancer cell specific nucleoside analog. Completed European exploratory **Phase I** trial in cancer patients with solid tumors. **Phase Ib** clinical trial in cancer patients initiated in January 2014.

- **Archexin:** Akt-1 inhibitor completed an exploratory **Phase IIa** clinical trial in pancreatic cancer. **Phase IIa** clinical trial in cancer patients with metastatic renal cell carcinoma initiated in January 2014.

- **Nano-Polymer-Drug Conjugate System (NPDCS)**
  - **RX-21101:** Polymer conjugated form of docetaxel containing a signaling moiety which directs the drug into the tumor increasing efficacy and minimizing toxicity

**Extensive intellectual property position**
## Robust Oncology Pipeline

<table>
<thead>
<tr>
<th>Drug Candidate</th>
<th>Mechanism of Action</th>
<th>Preclinical</th>
<th>Phase I</th>
<th>Phase II</th>
<th>Phase III</th>
</tr>
</thead>
<tbody>
<tr>
<td>Supinoxin™ (RX-5902)</td>
<td>p68 RNA Helicase inhibitor</td>
<td></td>
<td>2014</td>
<td></td>
<td></td>
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<tr>
<td>RX-3117</td>
<td>Cancer cell specific nucleoside analog</td>
<td></td>
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<td>2014</td>
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<tr>
<td>Archexin®</td>
<td>Akt-1 inhibitor</td>
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**Targeted Drug Delivery Platform**

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<td>RX-21101</td>
<td>Docetaxel conjugate</td>
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<td></td>
<td>2014</td>
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<tr>
<td>RX-0201-nano</td>
<td>Akt-1 inhibitor</td>
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- Anticipated progress during 2014
Milestones Being Pursued for 2014

- ✔ Initial data from Supinoxin Phase I Clinical trial
- Complete RX-3117 corporate partnership (mid year)
- Complete Supinoxin Phase I clinical trial (4Q14)
- Complete safety component of Archexin Phase IIa clinical trial (4Q14)
- Complete patient enrollment in RX-3117 Phase I clinical trial (4Q14/1Q15)
## Financial Highlights

<table>
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<tr>
<th><strong>Rexahn Financial Highlights</strong></th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Ticker</strong></td>
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<td><strong>Exchange</strong></td>
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<tr>
<td><strong>Market Price (6/11/14)</strong></td>
</tr>
<tr>
<td><strong>Market Capitalization (6/11/14)</strong></td>
</tr>
<tr>
<td><strong>Shares Outstanding (6/11/14)</strong></td>
</tr>
<tr>
<td><strong>Insider Ownership</strong></td>
</tr>
<tr>
<td><strong>Cash Balance (3/31/14)</strong></td>
</tr>
<tr>
<td><strong>Monthly Estimated Cash Burn for 2014</strong></td>
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Supinoxin™(RX-5902)
Supinoxin: Potential Best-in-Class p68 Helicase Inhibitor

**Mechanism**
- Inhibition of phosphorylated p68 RNA helicase
- Blocks upregulation of cancer related genes

**Potential Indications**
- Solid tumors: pancreas, NSCLC, colon, renal and other solid tumors

**Potential Advantages**
- Anti-proliferative effects
- Synergistic with cytotoxic agents
- Efficacy against drug resistant cancer cells
- Orally bioavailable

**Patent**
- New chemical entity

**Clinical Development**
- Phase I clinical trial in cancer patients initiated August 2013
Supinoxin: Mechanism of action

- Phosphorylated p68 is highly expressed in cancer cells but not in normal cells and upregulates cancer-related genes
- Supinoxin selectively inhibits phosphorylated p68 RNA Helicase
  - Decreased proliferation/growth of cancer cells
  - Synergism with cytotoxic agents
  - Activity against drug resistant cancer cells

Upregulation of Cyclin D1, C-jun and C-myc

Cancer cell Proliferation/Tumor growth
Supinoxin: Increased Survival in Human Renal Cell Carcinoma and Pancreatic Cancer Xenograft Models

Treatment with Supinoxin on days 1 to 20 produced a survival benefit beyond 65 days.
Supinoxin™ Phase I Dose-Escalation Clinical Trial Design

- Initiated August 2013; conducted at three clinical oncology centers in the US
- Cancer patients with solid tumors (various types) that have previously failed treatment with approved therapies and shown progression of disease

Accelerated dose-escalation design:
- 1 subject per dose; decision to escalate dose made by DMSB after completion of one cycle (3 weeks on drug and 1 week off)
- Dose levels to increase until grade 2 related adverse event occurs; after which 3 patients per dose will be followed

- Patients have the possibility to receive up to 6 cycles of treatment if disease does not progress
- Patients are scanned (CT or MRI) for assessment of solid tumors
  - Scan is repeated at the end of every 2 cycles of treatment to determine if disease has progressed
- phospho-P68 biomarker samples are collected at the time of tumor assessments
Supinoxin™ Phase I Dose-Escalation Trial: Status

- Study on-going; maximum tolerated dose (MTD) not yet achieved
- 150 mg oral dose cycle ongoing; 25, 50 and 100 mg dose cycles complete
- No drug related adverse events
- 2 patients have received 2 cycles of treatment; 1 patient has received 6 cycles of treatment

Pharmacokinetics
- Dose-proportional exposure
- Estimated oral bioavailability of 51%
- Similar pharmacokinetics to what was seen in preclinical models
**RX-3117: Next Generation Nucleoside Compound**

**Mechanism of Action**
- Cancer cell specific nucleoside compound that inhibits DNA synthesis
- Activated by UCK1 & UCK2

**Potential Indications**
- Solid tumors: pancreas, NSCLC, colon, renal and other solid tumors

**Potential Advantages**
- Effective against gemcitabine-resistant human cancer cell lines
- Orally administered
- Specifically targeted against cancer cells; reduced adverse events

**Patent**
- New chemical entity

**Clinical Development**
- Completed exploratory **Phase I** clinical trial in cancer patients that confirmed oral bioavailability and initial safety
- **Phase Ib** clinical trial in cancer patients initiated January 2014
RX-3117 has shown anti-tumor effects across a broad variety of tumor types in animal models (Colon, Non-Small Cell Lung, Small Cell Lung, Pancreatic, Renal, Ovarian and Cervical)
RX-3117: Efficacy in Animal Models

RX-3117 offers benefits based on overall-survival via oral administration in nude mice
The efficacy of RX-3117 was examined in 12 different human tumor (Colon, Non-Small Cell Lung, Small Cell Lung, Pancreatic, Renal, Ovarian and Cervical)
**RX-3117: Exploratory Phase I clinical Trial (Completed)**

- Exploratory Phase I clinical trial in cancer patients was conducted in Europe in 2012

- Objectives:
  - Evaluate oral bioavailability and pharmacokinetics
  - Assess safety and tolerability

- Drug administration cohorts:
  - 20 mg IV (n=3)
  - 50 mg oral (n=3)
  - 100 mg oral (n=3)

- Results:
  - Nine subjects, ages 47 to 67 years, were enrolled
  - RX-3117 was orally bioavailable with $T_{\text{max}}$ of 2-3 hours, $T_1/2$ of 14-21 hours, and oral bioavailability of 33 to 56%
  - RX-3117 was well tolerated with no post-dose adverse events, laboratory abnormalities, or ECG changes emerging through 7 days of follow-up
RX-3117: Phase Ib Study Design

- Initiated January 2014
- Cancer patients with solid tumors
- Up to 30 patients and three clinical sites
- Treatment cycle is 28 days
  - Dosing 3 times a week for 3 weeks followed by 1 week off
- Dose Finding Study Design
  - Escalation decisions based on safety, dosing, PK, laboratory, etc
  - Patients may receive up to 8 cycles of treatment
  - Anti-tumor activity secondary endpoint
  - Patients will be scanned (CT or MRI) prior to initiating treatment and after every 2 cycles
RX-3117 Phase Ib Dose-Escalation Trial: Status

- Study on-going; maximum tolerated dose (MTD) not yet achieved
- 30 and 60 mg dose cycles complete
- 100 mg dosing cycle is ongoing
- No drug related adverse events
Archexin®
Archexin: Potential Best-in-Class Akt-1 Inhibitor

**Mechanism of Action**
- Novel inhibitor of the protein kinase Akt-1 – Major cancer cell signaling protein
- Activated Akt-1 only present in cancer cells
- Increases apoptosis by inhibiting Akt-1 expression and activation
- Inhibition of Akt-1-mediated drug resistance

**Potential Indications**
- Metastatic renal cell carcinoma

**Potential Advantages**
- Only compound in clinical development to selectively inhibit Akt-1
- Promising safety profile in humans

**Patent**
- Composition of matter and use patent

**Clinical Development**
- **Phase I** trial in cancer patients completed
- Pancreatic cancer- **Phase IIa** completed
- **Phase IIa** trial in metastatic renal cell carcinoma (RCC) - ongoing
Archexin: Phase I Clinical trial (completed)

- **Phase I objective**
  - To determine maximum tolerated dose, safety and pharmacokinetic profiles

- **Phase I results:**
  - MTD was 250 mg/m²/d in Patients with an advanced cancer after up to two cycles of treatment
  - The dose limiting toxicity was Grade 3 fatigue; no significant hematological abnormalities

- Phospho-Akt-1 being developed as a clinical biomarker

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Archexin: Phase IIa Study in Metastatic Pancreatic Cancer (completed)

- Open label 2-stage study to assess the safety and efficacy of Archexin in combination with gemcitabine
- 31 subjects enrolled (10 for safety, 21 for efficacy) with ages ranging 18-65 years with metastatic pancreatic cancer
- Archexin in combination with gemcitabine provided a median survival of 9.1 months compared to the historical survival data of 5.65 months (Burris et al., 1997, J. Clin Oncol 15:2403) for standard single agent gemcitabine therapy
Archexin® Phase IIa Renal Carcinoma Clinical Trial Design

- Initiated January 2014
- Administered in combination with everolimus (Afinitor®) as a second line therapy to treat subjects with metastatic renal cell carcinoma
  - Resistance to mTOR inhibitors (everolimus) mediated by upregulation of Akt-1

- Part A – Identify maximum tolerated dose up to a target dose of 250 mg/m²/day in combination with everolimus
  - Dosing escalation decisions will be made following 1 cycle of therapy in 3 subjects
  - Assessment will include safety, dosing, PK, laboratory and physical exam

- Part B – Determine safety and efficacy of Archexin in 30 additional subjects with metastatic renal cell carcinoma
  - Randomized, 2:1 ratio of everolimus plus Archexin vs everolimus alone
  - Primary endpoint progression free survival following up to 8 cycles of therapy
  - Subjects are scanned (CT or MRI) for assessment of solid tumors at the end of every 2 treatment cycles
Nano-Polymer-Drug Conjugate System (NPDCS)
Nano-Polymer-Drug Conjugate System (NPDCS)

- NPDCS co-developed with the University of Maryland at Baltimore
- Targeted delivery of chemotherapeutic agent to tumor
  - Reduced systemic exposure to chemotherapeutic agent
    - Decreased adverse events
    - Increased efficacy
- Elimination of toxic solvent to solubilize insoluble chemotherapeutic agents
  - Decreased adverse events
- RX-21101 (Docetaxel-polymer conjugate) is the first candidate from the NPDCS platform
RX-21101

- Maximal Tolerable Dose (MTD) in Mice
  - RX-21101 showed a 6 fold increase in MTD compared to free docetaxel
RX-21101

- Xenograft Study using KB Sarcoma cells

RX-21101:
- Complete inhibition of tumor growth
- No change in body weight
- Increased survival
Corporate Overview
Milestones | Highlights
Milestones Being Pursued for 2014

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