This presentation contains forward-looking statements, as that term is defined in the Private Securities Litigation Reform Act of 1995. These statements are indicated by words or phrases such as "believes," "expects," "anticipates," "estimates," "plans," "continuing," "ongoing," "projected" and similar words or phrases and relate to future events or our future results of operations or future financial performance, including, but not limited to, statements regarding Raptor’s expectations regarding the markets for its products, its expected net product sales for PROCYSBI in 2015, projected non-GAAP operating expenses for 2015; its expectations for market and label expansion, the timing of clinical trial activities and results, regulatory decisions and updates and product launches. These statements are only estimates, projections and forecasts and involve known and unknown risks, uncertainties and other factors, which may cause the company’s actual results to be materially different from these forward-looking statements. Raptor cautions readers not to place undue reliance on any such forward-looking statements, which speak only as of the date they were made. Factors which may contribute to differences in actual results include, among others: market acceptance and sales of PROCYSBI in the U.S. and the EU; Raptor's ability to expand the use of RP103 and to receive regulatory approval for other indications; Raptor's reliance on a single active pharmaceutical ingredient supplier, a single third-party manufacturer and other third parties in connection with drug product development; compliance with healthcare regulations, ongoing regulatory requirements and potential penalties; any serious adverse side effects associated with PROCYSBI or any other future products and product liability claims; third-party payor coverage, reimbursement and pricing; enacted and future healthcare legislation; Raptor’s ability to obtain and maintain orphan drug or other regulatory exclusivity for PROCYSBI or any other future products; the integration of European operations with U.S. operations; relationships with key scientific and medical collaborators; intellectual property protection and claims and continued license rights; and Raptor’s ability to fund its operations and make required payments on its debt. Certain of these risks, uncertainties and other factors are described in greater detail in the company’s periodic filings from time to time with the Securities and Exchange Commission (the "SEC"), which Raptor strongly urges you to read and consider, including Raptor’s Annual Report on Form 10-K for the year ended December 31, 2014 filed with the SEC on March 2, 2015 and other periodic reports filed with SEC, all of which are available free of charge on the SEC's web site at http://www.sec.gov. Subsequent written and oral forward-looking statements attributable to Raptor or to persons acting on its behalf are expressly qualified in their entirety by the cautionary statements set forth in Raptor’s reports filed with the SEC. Raptor expressly disclaims any intent or obligation to update any forward-looking statements except as may be required by law.
Company Overview

• Leading next-generation rare disease biopharma company

• High impact, high touch, high margin products

• Worldwide operational capabilities

• Multiple late-stage pipeline programs with significant potential, 2015 milestones

• Proven and experienced biotechnology management team

• Track record of rapidly developing and commercializing breakthrough therapies

• Pipeline diversification from internal and external development
Where We’ve Been, Where We’re Going

2015
- Key personnel hired
- Completion of CyNCH treatment period
- $80-90M 2015 revenue guidance
- RP 103 Huntington’s program clarity
- RP 103 CyNCh data
- RP 103 Phase 2 interim mitochondrial disease data
- Portfolio expansion

2014
- PROCYSBI EU launch
- Upward revision of PROCYSBI guidance
- Presentation of 18-month RP103 Phase 2/3 CYST-HD results
- DaVita screening project
- Appointment of Julie Anne Smith as CEO-designate

2013
- PROCYSBI EU launch
- Full enrollment of Phase 2b pediatric NASH CyNCH study

2009
- NASDAQ listing

2015
- PROCYSBI US launch
- PROCYSBI EU approval
- Full enrollment of Phase 2b pediatric NASH CyNCH study

RP103 – Exploiting the Potential of Cysteamine

- **Anti-Oxidant and Cellular Protection:**
  - ↑ Intracellular Glutathione

- **Anti-Fibrotic:**
  - TGFβ Dependent and Independent Mechanisms

- **Proteostatic:**
  - Transglutaminase Inhibitor
  - HSP Upregulator
Portfolio Addresses Unmet Needs Across a Spectrum of Serious Diseases

- **Nephropathic Cystinosis, >6 yo**
- **Nephropathic Cystinosis, 2-6 yo**
- **RP 103**
  - Huntington’s disease
- **RP 103**
  - Pediatric NASH
- **RP 103**
  - Mitochondrial diseases
- Novel agents
  - Cancer metabolism
- Novel agents
  - Metabolic diseases

**Future Acquisitions**

**Next Milestone**

- EU country, ROW launches (2015)
- sNDA PDUFA (August 14, 2015)
- Regulatory path defined (2015); 36-month data (2H 2015)
- CyNCh data (2H 2015)
- Interim Phase 2 data (2015)
- IND candidate nomination
- IND candidate nomination
PROCYSBI – Nephropathic Cystinosis

- Rare, inherited, fatal metabolic lysosomal storage disease
- Inadequate treatment leads to widespread cellular damage, progressive multi-organ failure and death
- 500 US patients, 800 EU, 700 International; expansion opportunities

Cystine Crystals Seen on Renal Biopsy Scanning Electron Microscopy

- US approval (2013), EU approval (2014)
- Orphan exclusivity; patents to 2027
- Good efficacy profile
- 12-hour dosing
- Good tolerability profile
- Flexible administration
PROCYSBI – Successful Global Launch

- 2014 global net product sales $69.5M (top end of the revised guidance range $65-$70M)
- 2015 revenue guidance in the range of $80-$90M
- PROCYSBI currently sold in US, Germany, Austria, Switzerland, Norway, Denmark and Brazil
- Additional EU, Latin America and Middle East launches anticipated in 2015

*U.S. product launch in mid-2013
†EU product launch in Apr 2014
Positioned for Sustainable Growth Beyond 2020 – PROCYSBI Genetic Screening Program

2014

- DaVita Partnership

2015

- Prospective Screening Analysis
  - Identification of potentially undiagnosed nephropathic cystinosis patients
    - 16 patients – homozygous CTNS mutations
    - 52 patients – heterozygous CTNS mutations
    - 500 additional patients – single mutation
  - Confirmation of clinical diagnosis
    - Open clinical protocol
    - Slit lamp exam, DNA cheek swab
    - Genetic counseling
    - Results provided to treating nephrologist

2020 and beyond

- Market Expansion Opportunities
  - Additional opportunities
    - Late-onset cystinosis patients
    - End-stage renal disease (ESRD) dialysis population
    - Transplant patients
RP103 – Huntington’s Disease

- Rare genetic neurodegenerative progressive disease that causes striatal and cortex neuron degradation
- Motor, cognitive and behavioral deterioration leading to severe physical and mental disability and premature death 15-20 years from onset

Unmet Medical Need
- Limited symptomatic treatments
- No approved disease-modifying therapies
- Premature / unpredictable mortality

Significant Market Opportunity
- 30,000 U.S. patients
- ~100,000 worldwide patients

Strong Scientific Rationale
- MOA suggests multifold attack
- Antioxidant effects
- Protein folding, neurotranscriptional benefit, cysteine mobilization
- Supportive preclinical data

Promising Clinical Data
- Significance not met on primary endpoint but positive signals in interim Phase 2/3 data
- Statistical significance in slowing disease progression in subset population
Key Inclusion Criteria

- Adults (Age 18 to 65) with HD for at least one year, CAG repeat length > 38
- Unified Huntington’s disease rating scale (UHDRS) TMS ≥ 5, Total functioning capacity (TFC) > 10

Primary Endpoint:

- Change from baseline to 18 months in the Total Motor Score (TMS) component of the Unified Huntington Disease Rating Scale (UHDRS) between placebo and RP103 treated groups

Secondary Endpoints:

- Functional, Motor
- Neuropsychological
- Cognitive
• Strong trend in RP103-treated patients with 34% slower disease progression in TMS versus placebo
  − 2.17 point decline; p=0.19
• Statistically significant slowing of disease progression (58%) in RP103-treated patients not on tetrabenazine
  − 3.94 point decline; p=0.03
  − Slower decline across a variety of TMS subscales
• No unexpected adverse events
  − Most common adverse events (AEs) – nausea, vomiting, abdominal pain, constipation, breath odor
    ▪ RP103 arm: 48/52 patients
    ▪ Placebo arm: 38/44 patients
  − Serious adverse events (SAEs) – 5 in RP103 arm (3 resulted in discontinuations), 4 in placebo arm (1 resulted in discontinuation)
Mean Change in UHDRS TMS in ITT Population

Mean Change in UHDRS TMS in Per-Protocol Population Not on Tetrabenazine

Components of UHDRS TMS

<table>
<thead>
<tr>
<th>Endpoint</th>
<th>Placebo</th>
<th>RP103</th>
</tr>
</thead>
<tbody>
<tr>
<td>UHDRS TMS</td>
<td>44</td>
<td>47</td>
</tr>
<tr>
<td>Maximal dystonia</td>
<td>44</td>
<td>47</td>
</tr>
<tr>
<td>Maximal chorea</td>
<td>44</td>
<td>47</td>
</tr>
<tr>
<td>Dystanshetra</td>
<td>44</td>
<td>47</td>
</tr>
<tr>
<td>Tongue Protrusion</td>
<td>44</td>
<td>47</td>
</tr>
<tr>
<td>Ocular pursuit</td>
<td>44</td>
<td>47</td>
</tr>
<tr>
<td>Saccade initiation</td>
<td>44</td>
<td>47</td>
</tr>
<tr>
<td>Saccade velocity</td>
<td>44</td>
<td>47</td>
</tr>
<tr>
<td>Finger taps</td>
<td>44</td>
<td>47</td>
</tr>
<tr>
<td>Pronate/Supinate hands</td>
<td>44</td>
<td>47</td>
</tr>
<tr>
<td>Luria</td>
<td>44</td>
<td>47</td>
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<tr>
<td>Rigidity-arms</td>
<td>44</td>
<td>47</td>
</tr>
<tr>
<td>Bradykinesia-body</td>
<td>44</td>
<td>47</td>
</tr>
<tr>
<td>Gait</td>
<td>44</td>
<td>47</td>
</tr>
<tr>
<td>Tandem walking</td>
<td>44</td>
<td>47</td>
</tr>
<tr>
<td>Retropulsion pull test</td>
<td>44</td>
<td>47</td>
</tr>
</tbody>
</table>
RP103 – Pediatric Non Alcoholic Fatty Liver Disease (NAFLD), Nonalcoholic Steatohepatitis (NASH)

- NAFLD ranges from excess fatty deposition in the liver to NASH, characterized by inflammation, fibrosis and hepatic ballooning
- Children with NASH will likely require liver transplants in their 3rd – 4th decade
- Untreated, 15%-25% progress to cirrhosis with increased risk of liver failure and cancer

Unmet Medical Need
- No approved therapeutic options, only lifestyle changes

Significant Market Opportunity
- 9-13% of children in western population have NAFLD
- 1.5% of children have NASH

Strong Scientific Rationale
- MOA suggests multifold attack
- Anti-fibrotic effects
- Matrix remodeling
- Anti-inflammatory / reduction in reactive oxidative species

Promising Clinical Data
- Positive proof-of-concept data
Pediatric NASH – A Real Disease

MRI of 12 yo obese child with NASH

MRI of normal liver

Disease Spectrum of NAFLD

Normal

Steatosis

NASH

Cirrhosis


Source: Reuters and Perspectum Diagnostics
Phase 2a – Improvement in Liver Transaminases with RP103 Prototype

Primary endpoint: 7 of 11 subjects (64%) achieved the primary endpoint
• All 11 subjects had a statistically significant reduction in ALT at 24 weeks from baseline (p = 0.002)

Statistically significant improvements in ALT, AST, CK-18 and adiponectin were observed at week 24.

Dohil, R et al. Aliment Pharmacol Ther, 2011
• CyNCh study
  - Phase 2b double-blind, placebo-controlled, 18-month, 169-patient study with observation period
  - Conducted by National Institutes of Diabetes and Digestive and Kidney Disease (NIDDK) under a Cooperative Research and Development Agreement (CRADA)

• Primary endpoint (▲)
  - Two-point improvement in NAFLD Activity Score (NAS) and no worsening in fibrosis on biopsy in 8 to 17 yo with biopsy-confirmed severe NAFLD/NASH (NAS 4-5)

• Secondary endpoints (△)
  - Liver enzymes - alanine transaminase (ALT), aspartate transaminase (AST), gamma-glutamyl transpeptidase (GGT); other markers of oxidation and anti-oxidant status
  - Reduction in MRI-determined hepatic fat fraction
  - Safety and tolerability
### RP103 – Sole Pediatric-Focused Therapy in NASH Landscape

<table>
<thead>
<tr>
<th>Product (Company)</th>
<th>Phase</th>
<th>Indication</th>
<th>Mechanism of Action</th>
<th>Milestones</th>
</tr>
</thead>
<tbody>
<tr>
<td>RP103 (Raptor)</td>
<td>Phase 2b</td>
<td>Pediatric NASH</td>
<td>Increases glutathione Inhibit TGFβ signaling</td>
<td>2H 2015</td>
</tr>
<tr>
<td>Aramchol (Galmed)</td>
<td>Phase 2/3</td>
<td>Adult NASH</td>
<td>Synthetic bile/fatty acid conjugate</td>
<td>Interim analysis 2H 2015</td>
</tr>
<tr>
<td>Obeticholic acid (Intercept)</td>
<td>Phase 2b</td>
<td>Adult NASH</td>
<td>Semi-synthetic bile acid analogue; FXR agonist</td>
<td>Initiation of Phase 3 studies in 2H 2015</td>
</tr>
<tr>
<td>PX-104 (Phenex)</td>
<td>Phase 2</td>
<td>Adult NAFLD</td>
<td>Synthetic FXR agonist</td>
<td>NA</td>
</tr>
<tr>
<td>Cenicriviroc (Tobira)</td>
<td>Phase 2b</td>
<td>Adult NASH</td>
<td>CCR2/CCR5 antagonist</td>
<td>Mid-2016</td>
</tr>
<tr>
<td>Actos (Takeda)</td>
<td>Phase 2</td>
<td>Adult NASH with type 2 diabetes</td>
<td>PDE-4 inhibitor/PPAR-γ agonist</td>
<td>NA</td>
</tr>
<tr>
<td>GFT505 (Genfit)</td>
<td>Phase 2b</td>
<td>Adult NASH</td>
<td>PPAR-α/δ agonist</td>
<td>✓ March 2015</td>
</tr>
<tr>
<td>Simtuzumab (Gilead)</td>
<td>Phase 2b</td>
<td>Adult NASH cirrhosis and advanced fibrosis</td>
<td>LOXL2 monoclonal antibody</td>
<td>48-week data in 2H 2015</td>
</tr>
<tr>
<td>Emricasan (Conatus)</td>
<td>Phase 2</td>
<td>Adult NASH/NAFLD</td>
<td>Caspase protease inhibitor</td>
<td>✓ March 2015</td>
</tr>
<tr>
<td>Victoza/Saxenda (Novo Nordisk)</td>
<td>Phase 2</td>
<td>Adult NASH</td>
<td>GLP-1 receptor agonant</td>
<td>✓ May 2014</td>
</tr>
</tbody>
</table>

Source: Clinicaltrials.gov
**RP103 – Mitochondrial Diseases Including Leigh Syndrome**

- Mitochondrial diseases – genetic, clinically heterogeneous group of disorders caused by dysfunction of the mitochondrial respiratory chain as a result of mutation of nuclear DNA or mitochondrial DNA.

- Nearly all organ systems affected, leading to neurological, cardiac, renal, endocrine, hepatic and muscular impairments; death within the first decade of life.

<table>
<thead>
<tr>
<th>Unmet Medical Need</th>
<th>Market Opportunity</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Supportive care only</td>
<td>• 1 in 40,000 newborns affected by Leigh syndrome</td>
</tr>
<tr>
<td>• No approved therapies</td>
<td></td>
</tr>
<tr>
<td>• Poor prognosis for Leigh syndrome</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Strong Scientific Rationale</th>
<th>Clinical Data</th>
</tr>
</thead>
<tbody>
<tr>
<td>• MOA suggests multifold attack</td>
<td>• Proof-of-concept study ongoing</td>
</tr>
<tr>
<td>• Mitochondrial protectant</td>
<td></td>
</tr>
<tr>
<td>• Antioxidant effects</td>
<td></td>
</tr>
<tr>
<td>• Supportive preclinical data</td>
<td></td>
</tr>
</tbody>
</table>
Near-Term Business Strategy

Next stage

Market Expansion
- Increased US market penetration
- EU and ROW country launches
- Pediatric label expansion filing
- Late-onset patient potential

Advance Pipeline
- High-potential late-stage development programs
- Pediatric NASH (Phase 2b)
- HD (Phase 2/3, regulatory clarity)
- Mitochondrial disease (Phase 2)

Business Development
- Capitalize on rare disease market and clinical development expertise
- Product vs. technology focus
- Diversify pipeline beyond cysteamine
Going Beyond the First Product

RPTP 5-year Stock Performance 2010-Today

30% CAGR

Licensed (2008)
# Financial Highlights

## Selected Income Sheet Data:

<table>
<thead>
<tr>
<th></th>
<th>First Quarter Ended March 31, 2015</th>
<th>2015 Financial Guidance</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Revenue</strong></td>
<td>$20.5 million</td>
<td>$80-$90 million</td>
</tr>
<tr>
<td><strong>Operating Expenses</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Research and Development</td>
<td>$16.6 million</td>
<td></td>
</tr>
<tr>
<td>Selling, General and Administrative</td>
<td>$14.8 million</td>
<td></td>
</tr>
<tr>
<td>Total Operating Expenses</td>
<td>$31.4 million</td>
<td>$115-$125 million</td>
</tr>
<tr>
<td><strong>Net Loss (GAAP)</strong></td>
<td>$(20.2) million</td>
<td></td>
</tr>
<tr>
<td><strong>Net Loss (Non-GAAP)</strong></td>
<td>$(16.8) million</td>
<td></td>
</tr>
</tbody>
</table>

## Selected Balance Sheet Data:

<table>
<thead>
<tr>
<th></th>
<th>As of March 31, 2015</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Cash &amp; Equivalents†</strong></td>
<td>$134.5 million</td>
</tr>
<tr>
<td><strong>Total Assets</strong></td>
<td>$173.0 million</td>
</tr>
<tr>
<td><strong>Total Debt</strong></td>
<td>$120.0 million</td>
</tr>
<tr>
<td><strong>Common Shares Outstanding:</strong></td>
<td>69.1 million</td>
</tr>
</tbody>
</table>

*Excludes stock compensation expense of $12.0 million
† Excludes $92.0 million in net proceeds from April 8th secondary offering
## Recent & Upcoming Key Events

<table>
<thead>
<tr>
<th>PROCYSBI</th>
<th>Anticipated Timing</th>
</tr>
</thead>
<tbody>
<tr>
<td>Launched in Norway, Denmark</td>
<td>✓ 1Q 2015</td>
</tr>
<tr>
<td>Initial sales in Latin America</td>
<td>✓ 1Q 2015</td>
</tr>
<tr>
<td>FDA decision for label expansion in cystinosis patients aged 2-6 years</td>
<td>August 14, 2015</td>
</tr>
<tr>
<td>Launch in additional EU, ROW countries</td>
<td>2015</td>
</tr>
<tr>
<td>Update on genetic screening program</td>
<td>2015</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>RP103 – Huntington’s Disease</th>
<th>Timing</th>
</tr>
</thead>
<tbody>
<tr>
<td>Regulatory update from FDA and EMA</td>
<td>2015</td>
</tr>
<tr>
<td>36-month Phase 2/3 CYST-HD data</td>
<td>2H 2015</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>RP103 – NASH</th>
<th>Timing</th>
</tr>
</thead>
<tbody>
<tr>
<td>Completion of 12-month treatment period in Phase 2b CyNCH study</td>
<td>✓ Jan 2015</td>
</tr>
<tr>
<td>Phase 2b CyNCh data</td>
<td>2H 2015</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>RP103 - Leigh Syndrome and Other Mitochondrial Disorders</th>
<th>Timing</th>
</tr>
</thead>
<tbody>
<tr>
<td>Interim Phase 2 data</td>
<td>2H 2015</td>
</tr>
</tbody>
</table>