Forward-Looking Statements

This presentation contains forward-looking statements. Such statements are valid only as of today and we disclaim any obligation to update this information. These statements are only estimates and predictions and are subject to known and unknown risks and uncertainties that may cause actual future experiences and results to differ materially from the statements made. These statements are based on our current beliefs and expectations as to such future outcomes. Drug discovery and development involve a high degree of risk. Factors that might cause such a material difference include, among others, uncertainties related to the ability to raise additional capital required to complete the development programs described herein, uncertainties related to the disruptions at our sole supplier of CLR 131, the ability to attract and retain partners for our technologies, the identification of lead compounds, the successful preclinical development thereof, the completion of clinical trials, the FDA review process and other government regulation, the ability of our pharmaceutical collaborators to successfully develop and commercialize drug candidates, competition from other pharmaceutical companies, product pricing and third-party reimbursement. This presentation includes industry and market data that we obtained from industry publications and journals, third-party studies and surveys, internal company studies and surveys, and other publicly available information. Industry publications and surveys generally state that the information contained therein has been obtained from sources believed to be reliable. Although we believe the industry and market data to be reliable as of the date of this presentation, this information could prove to be inaccurate. Industry and market data could be wrong because of the method by which sources obtained their data and because information cannot always be verified with complete certainty due to the limits on the availability and reliability of raw data, the voluntary nature of the data gathering process and other limitations and uncertainties. In addition, we do not know all of the assumptions that were used in preparing the forecasts from the sources relied upon or cited herein. A complete description of risks and uncertainties related to our business is contained in our periodic reports filed with the Securities and Exchange Commission including our Form 10-K for the year ended December 31, 2018.
## Company Highlights

- Developing orphan and rare oncology pipeline
- Validated cancer-targeting platform
- Demonstrated activity in 3 hematologic malignancies
- 4 Phase 2 clinical data readouts planned for 2019
- Efficient capital allocation and low fixed-cost structure

*Multiple, Value-Creative, Near Term Milestone Potential*
## Projected Clinical Development Milestones

<table>
<thead>
<tr>
<th>PDC Program</th>
<th>2019</th>
<th>2020</th>
<th>2021</th>
</tr>
</thead>
<tbody>
<tr>
<td>CLR 131 Phase 2 Hematologic Malignancies</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>1Q</td>
<td>2Q</td>
<td>3Q</td>
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<tr>
<td>CLR 131 Phase 1 Multiple Myeloma</td>
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<tr>
<td>CLR 131 Phase 1 Pediatric</td>
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<tr>
<td>Proprietary PDC</td>
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</tbody>
</table>

### CLR 131 Granted Five Orphan Drug Designations, Four Rare Pediatric Designations and 2 Fast Track Designations Including Multiple Myeloma

1. Multiple Myeloma
2. Top Line Data
3. Assumes decision to initiate
4. Lymphoplasmacytic Lymphoma
5. Marginal Zone Lymphoma
6. Median Overall Survival Cohorts 1-4

- **Interim Data**
- **Initiations**
- **Data**
Overview

Phase 2 R/R Hematologic Malignancies

Phase 1 R/R Multiple Myeloma

Phase 1 R/R Pediatric Malignancies
Validated Market, Therapeutic Isotope & Targeted Delivery

• 2020 Radiotherapeutic Market Forecast
  – ~$9.31 billion revenue
  – CAGR of 10.2% through 2025

• Recent Transactions
  – Advanced Accelerator Applications - $3.9B
  – Endocyte - $2.1B
  – Fusion - $100M Financing

• Validated therapeutic isotope I-131
  – Azedra™ (iobenguane I-131)
  – Bexxar™ (CD-20 antibody I-131)
  – MIBG-131 (MIBG I-131)

• CLR 131 validated cancer targeting
  – Small molecule phospholipid ether
  – Multiple payloads tested

**CLR 131: Combination of a Validated Delivery Platform and Payload**

## CLR 131 Hematologic Malignancies Clinical Studies

### R/R Hematologic Malignancies Phase 2 Study
- **Multiple Myeloma Interim Data**
  - Average 7th line systemic treatment
  - 30% Overall Response Rate
  - 100% Disease Control Rate
- **Diffuse Large B-cell Lymphoma Interim Data**
  - Average 4th line systemic treatment
  - 33% Overall Response Rate
  - 50% Disease Control rate
- **Waldenstrom’s (LPL) Patient Case Study**
  - >98% reduction in total tumor volume
  - Duration of response at 200+ days

### R/R Multiple Myeloma Phase 1 Study
- All 6 cohorts safe and tolerable
- Average 6th line systemic treatment
- **Cohort 6 Activity**
  - 50% Overall Response Rate
  - 50% Minimal Response
  - 100% Disease Control Rate
- No patients experiencing:
  - Peripheral neuropathy
  - Deep vein thrombosis
  - Cardiotoxicities
  - Embolisms
  - Gastrointestinal toxicities
- No change in liver enzymes or renal function
- Cytopenias most common adverse events, all viewed as predictable and manageable

#### A Single 25 mCi/m² Dose Achieved 30%+ Response Rates in Heavily Pretreated Patients Across 3 Hematologic Cancers

#### Fractionated 37.5mCi/m² Dose Achieved 50% Response Rate in Heavily Pretreated Patients with Multiple Myeloma

1. All evaluable patients achieved ≥ Stable Disease
1. Overview

2. Phase 2 R/R Hematologic Malignancies

3. Phase 1 R/R Multiple Myeloma

4. Phase 1 R/R Pediatric Malignancies
Patients Screened

N=10 MM
N=10 DLBCL
N=10 CLL/SLL, MZL, LPL
N=10 MCL

Interim efficacy assessments; expand cohorts based on performance

20-30 MM
10-30 DLBCL
10-30 CLL/SLL, MZL, LPL
10-30 MCL

Final Efficacy Assessments
Follow-up (≥ 1 yr After Last Dose)

• Primary endpoint is efficacy as determined by response rate
• Patients received a single 25mCi/m² dose; potential for a 2nd cycle
• Patients now receive a fractionated 37.5mCi/m² dose; potential for a 2nd cycle

Day 1
Cycle 1
(18.75mCi/m² x 2)
Day 1 & Day 8

Day 75-180
Cycle 2
(18.75mCi/m² x 2)
Day 1 & Day 8

25mCi/m² Bolus Dose with Demonstrated Activity in at Least 3 Hematologic Cancer Types Tested to Date
Response Rates for On-Market Fourth and Fifth Line TRX are 15% & 8%

Response Rate for CLR 131 Seventh Line Average TRX Achieves 30%

Phase 2 R/R Multiple Myeloma Patient Case Study

- Male, 78 years old with 90% bone marrow involvement
- 2 prior lines of systemic treatment; patient’s best prior response is a PR
- Single 25mCi/m^2 dose of CLR 131
- Patient achieves PR, continues to respond 120+ days post treatment

**Strong and Durable Response; Expected to Receive Second Cycle**
New Drugs Needed in R/R DLBCL

Response Rates for On-Market
Third Line TRX is 20%

Response Rate for CLR 131
Fourth Line Average TRX Achieves 33%

1. SEER Cancer Statistics Fact Sheet: Non-Hodgkin Lymphoma (DLBCL represents between 25% - 30% of NHL); Accessed April 22, 2019
2. Data reported is not from a head to head clinical study
3. Lugano Classification
4. Duration of Response
Phase 2 R/R DLBCL Patient Case Study

DLBCL is an Aggressive Form of Lymphoma

**Patient Case Study**

Scan Day 1

Scan Day 90

- Male, 52 years old with subpectoral lymph node mass
  - MYC positive (>40%); BCL-2 negative
- 3 prior lines of treatment (R-CHOP, RICE and chemo-soup)
- Relapse within 10 months of 1\textsuperscript{st} line, 1 month post 2\textsuperscript{nd} line, and determined to be refractory to therapy in 3\textsuperscript{rd} line
- Patient continues to be responsive; 220+ days post treatment

![Graph showing tumor volume reduction over time](image)
Phase 2 LPL (Waldenstrom’s) Patient Case Study

- Baseline pleural effusion & multiple large tumor nodules; third line treatment
- Following 1st infusion
  - Dramatic improvements in multiple disease related pathologies with limited cytopenias
- CT day 187 showed 98% reduction in overall tumor burden and complete resolution of 4/5 tumors

![Graph showing lesion size over time after CLR 131 infusion]
Proposed R/R Multiple Myeloma Pivotal Study

Proposed Pivotal Study Design
(Later Line MM Trial)

Screening

Optimal Dosing from Phase 2
\( n = 15-20 \)

Phase 2b

Interim Assessment

\( n = 60-80 \)

Phase 3

Proposed Pivotal Study Design

- Relapsed/refractory \( \geq 4^{th} \) line Multiple Myeloma
- \(~20\) patients to be enrolled prior to interim assessment
- Pivotal, single-arm
  - Primary endpoint: Overall Response Rate (ORR)
  - Secondary endpoints: Overall Survival (OS), Progression Free Survival (PFS)

Program Timing\(^1\)

- Phase 2 to complete 2H19
- Pivotal study initiation 2H20 to 1H21
- NDA submission 2023

Clinical Costs\(^1\)

- Pivotal study = $20 - $25 million
- Eligible for pivotal study SBIR Grant up to $4M\(^2\)

---

1. Overview

2. Phase 2 R/R Hematologic Malignancies

3. Phase 1 R/R Multiple Myeloma

4. Phase 1 R/R Pediatric Malignancies
CLR 131 Phase 1 & 2 Safety Profile

### Drug Related Adverse Events Phase 1

<table>
<thead>
<tr>
<th>System Organ Class Preferred Term</th>
<th>All Treated Subjects N=26 n (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Anemia</td>
<td>9 (35)</td>
</tr>
<tr>
<td>Lymphocyte count decreased</td>
<td>18 (69)</td>
</tr>
<tr>
<td>Neutropenia</td>
<td>13 (50)</td>
</tr>
<tr>
<td>Thrombocytopenia</td>
<td>16 (62)</td>
</tr>
<tr>
<td>White blood cell count decreased</td>
<td>15 (58)</td>
</tr>
</tbody>
</table>

### Drug Related Adverse Events Phase 1 & 2

<table>
<thead>
<tr>
<th>System Organ Class Preferred Term</th>
<th>All Treated Subjects N=50 n (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Anemia</td>
<td>18 (36)</td>
</tr>
<tr>
<td>Lymphocyte count decreased</td>
<td>24 (48)</td>
</tr>
<tr>
<td>Neutropenia</td>
<td>25 (50)</td>
</tr>
<tr>
<td>Thrombocytopenia</td>
<td>30 (60)</td>
</tr>
<tr>
<td>White blood cell count decreased</td>
<td>26 (52)</td>
</tr>
</tbody>
</table>

Abbreviations: n=Number of Subjects with AEs; N=Number of Subjects Exposed

- Consistent adverse event profile
  - Modest reduction in occurrence of adverse events (AE’s) observed in Phase 2
  - Cytopenias are predictable and manageable
  - No unexpected drug related adverse events
- Fractionated dosing reduces AE’s and increases administered drug
- No changes in liver function, no peripheral neuropathy or other debilitating AEs

CLR 131 Demonstrates a Safe & Well Tolerated Adverse Event Profile

1. Grade 3 and 4 related adverse events as of 3/01/19
**New Treatments are Needed**

### On Market Product Data

<table>
<thead>
<tr>
<th>Drug</th>
<th>Median Overall Survival (mOS) in 3&lt;sup&gt;rd&lt;/sup&gt; Line</th>
<th>2016.01.02 00:00:00</th>
</tr>
</thead>
<tbody>
<tr>
<td>All Drugs&lt;sup&gt;2&lt;/sup&gt;</td>
<td>9.5 - 14.5 Months</td>
<td>11.9 Months</td>
</tr>
<tr>
<td>Carfilzomib&lt;sup&gt;3&lt;/sup&gt;</td>
<td>11.9 Months</td>
<td>18.6 Months</td>
</tr>
<tr>
<td>Daratumumab&lt;sup&gt;4&lt;/sup&gt;</td>
<td></td>
<td>11.9 Months</td>
</tr>
<tr>
<td>Pomal. (+dex)&lt;sup&gt;5&lt;/sup&gt;</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**CLR 131 Phase 1 Single Dose Cohort TRX<sup>1</sup>**

<table>
<thead>
<tr>
<th>Median Overall Survival (mOS) in 6&lt;sup&gt;th&lt;/sup&gt; Line</th>
<th>2016.01.02 00:00:00</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Cohort 6 18.75mCi/m&lt;sup&gt;2&lt;/sup&gt; Fractionated Dose</strong></td>
<td></td>
</tr>
<tr>
<td>• Deemed safe &amp; tolerable</td>
<td></td>
</tr>
<tr>
<td>• 50% Overall Response Rate</td>
<td></td>
</tr>
<tr>
<td>• 50% Minimal Response Rate</td>
<td></td>
</tr>
<tr>
<td>- Patient still on study;</td>
<td></td>
</tr>
<tr>
<td>48% M-protein reduction</td>
<td></td>
</tr>
<tr>
<td>• 100% Disease Control Rate</td>
<td></td>
</tr>
<tr>
<td>• Clear dose response observed</td>
<td></td>
</tr>
</tbody>
</table>

**Third Line Average mOS is ~12 Months & ~9 Months for Dual<sup>6</sup>/Penta<sup>7</sup> Refractory**

**Single 30 Minute Infusion of CLR 131 Achieves mOS of 22 Months**

---

1. Data reported is not from a head to head clinical study
2. Traditional monotherapy chemotherapy, protease inhibitor, and immunomodulating agents
6. Defined as refractory to at least one proteasome inhibitor and one immunomodulatory. Defined as refractory to Revlimid, Pomalyst, Velcade, Kyprolis, and Darzalex.
1. Overview

2. Phase 2 R/R Hematologic Malignancies

3. Phase 1 R/R Multiple Myeloma

4. Phase 1 R/R Pediatric Malignancies
Pediatric Clinical Development Strategy

*FDA Agreement on Phase 1 Accelerated Study Design*

**Proposed Phase 2/3 Pivotal Study Design**
- Granted ODD & RPDD for NB, RMS, Osteo & Ewing's Sarcoma
- Eligible for Fast Track, Breakthrough and SPA submissions
- Initial enrollment of 10-15 patients to confirm dose; upon appropriate efficacy expand into Phase 3
- Phase 3 pivotal study single arm ~65 patients
  - Primary endpoint: Overall Response Rate
  - Secondary endpoints: EFS\(^3\), CBR\(^4\), PFS

**Program Timing**
- Phase 1 to complete 3Q20
- Phase 2/3 pivotal initiation 2Q21
- NDA submission 2023

**Clinical Costs**
- Phase 1 = ~$4 million
- Phase 2/3 pivotal study = ~$11 - $12 million

*Approval in Any Indication May Provide Priority Review Voucher and Potential for NCCN Compendium Listing for Other Tumor Types*

# CLR 131 & MIBG Product Profile Comparison

**MIBG I-131 Currently Second Line Standard of Care for Neuroblastoma**

<table>
<thead>
<tr>
<th>Profile</th>
<th>CLR 131</th>
<th>Naxitamab &amp; Omburtamab</th>
<th>MIBG I-131</th>
</tr>
</thead>
<tbody>
<tr>
<td>Delivery Vehicle/Payload</td>
<td>Phospholipid Ether (PLE)/Iodine-131</td>
<td>Bispecific Antibody &amp; Antibody Drug Conjugate/Iodine-131</td>
<td>Meta-iodobenzylguanidine/Iodine-131</td>
</tr>
<tr>
<td>Therapeutic Regimen</td>
<td>Single 30 minute infusion Total dose ~\textbf{45-80mCi}</td>
<td>Naxi: 3mg/kg 3x wk 1 35 min IV Ombur: depot directly into CNS Total dose ~\textbf{75mCi}</td>
<td>3-5 cycles, ~300 mCi per cycle, 90-120 minute infusion Total dose ~\textbf{1000-1500mCi}</td>
</tr>
<tr>
<td>Hospitalization</td>
<td>TBD\textsuperscript{1}</td>
<td>Naxi: Outpatient Ombur: TBD (depot requires surgery)</td>
<td>4-8 days</td>
</tr>
<tr>
<td>Capable to Cross the Blood Brain Barrier</td>
<td><a href="#">Green</a></td>
<td><a href="#">Red</a></td>
<td><a href="#">Red</a></td>
</tr>
<tr>
<td>Ability to Target Metastasis</td>
<td><a href="#">Green</a></td>
<td><a href="#">Red</a></td>
<td><a href="#">Red</a></td>
</tr>
<tr>
<td>Stem Cell Transplant Support</td>
<td><a href="#">Yellow</a></td>
<td><a href="#">Green</a></td>
<td><a href="#">Red</a></td>
</tr>
<tr>
<td>NB Response Rate</td>
<td>TBD</td>
<td>TBD</td>
<td>20-60% (~30%)</td>
</tr>
<tr>
<td>Liver Function Changes</td>
<td>0\textsuperscript{2}</td>
<td>NR</td>
<td>79.6%</td>
</tr>
</tbody>
</table>

1. To Be Determined  2. In adults

<table>
<thead>
<tr>
<th>Favorable/Posesses</th>
<th>Not Yet Known</th>
<th>Deficient/Lacks</th>
</tr>
</thead>
<tbody>
<tr>
<td><a href="#">Green</a></td>
<td><a href="#">Yellow</a></td>
<td><a href="#">Red</a></td>
</tr>
</tbody>
</table>
Corporate Information
Capitalization as of June 15, 2019

**Common Stock Outstanding** 9,396,036

Reserved for issuance:

- Convertible Preferred Stock 537,500
- Warrants 9,318,747
- Employee Stock Options 484,964

**Fully Diluted** 19,737,247

**Cash/Equivalents as of March 31 plus May 20 Financing** ~$19.5 million

*Cash Believed to Be Adequate to Fund Operations Through 2020*
Company Summary

- CLR 131 exhibits activity in at least 3 hematologic malignancies
- At maximum dose tested to date, 50% Overall Response Rate in R/R Multiple Myeloma
- Pediatric study initiated, potential for accelerated regulatory pathway and pediatric voucher
- 4 Phase 2 clinical data readouts planned for 2019

Proof of Concept in Lead Clinical Program with Multiple Value-Creative, Near Term Milestone Potential
Thank You