CIK-Chimeric Antigen Receptor (CAR) platform

Life Science Summit 2014

Roland Turck, President & COO

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Formula Pharma – Investment summary

- Privately held, onco-immunotherapy company
- Virtual operating structure, with highly experienced management
- Developing CAR platform using CIK cells and non-viral transfection
- Raising bridge financing of $500,000 + in 2014
- Raising Series B round of $10 MM by 1Q2015
- Key value inflection point within 2 years
- Strong interest already from large pharma
“CAR” is a breakthrough technology

Emma Whitehead: the first B-ALL child cured with CAR-T (ref: Dr. Carl June at UPenn)


CAR T cells are working...
Complete tumor eradication within a matter of weeks
“CAR”: a genetically engineered adoptive cell therapy

- T-cells from the patient or donor obtain binding properties of a mAb
- Targeting tumor antigen
- No MHC restriction for antigen recognition
- Strong intracellular signal leading to potent cytokine secretion
- Penetration of tumor infiltrated organs
- Immunological memory and persistence of the therapeutic effect
# Keen industry and investor interest in CAR-T

<table>
<thead>
<tr>
<th>Biotech originator</th>
<th>Partner</th>
<th>Deals and raises</th>
<th>Latest stage R&amp;D candidate</th>
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</table>
| Juno Therapeutics | MSKCC, Fred Hutchinson, Seattle Children | - Series A round closed in 12/2013  
- $300 M in capital raises within past 12 months | Phase-1/2 pipeline |
| Kite Pharma | | - IPO in 8/2014, raised $130 M  
- Pre-IPO: $85 M since 2011  
- $1.2 B market cap | Ph-1/2a & phase-2; two lead targets |
| Cellectis | Pfizer | - $2.8 B deal 6/14, with $80 M upfront, plus $185 M in milestone payments per target  
- Market cap: $250 M | Pre-clinical pipeline |
| BlueBird Bio | Celgene, Baylor | - Partnering deal in 3/2103  
$225 M per therapeutic target  
- Market cap: $1.3 B | Pre-clinical stage pipeline |
| Novartis | UPenn, Carl June | - R&D support in 2012  
$20 M contribution  
- Major investment in CMC capabilities in 2014 | Phase-2, FDA breakthrough status |
First clinical results are stunning, yet...

• A large percentage of patients is not eligible or fails
  – e.g. not enough CAR cells can be generated from autologous source
  – 10 - 30% failure rate
  – Significant toxicity (“Cytokine Storm”)
• Most development work focused on CD19 target
• Most development work focused on autologous setting
  – Graft versus Host Disease associated with T-cells used in allo-setting
• CAR-T approaches typically use viral transfection methods
  – Overhang of manufacturing and regulatory complexities

CAR technology is not yet optimized.

⇒ There is a strong case for different approaches
Formula’s approach is distinct
Formula’s CAR technology is based on CIK cells

Today virtually all CARs are **T-cell** based

Formula’s technology focuses on **Cytokine Induced Killer (CIK) CAR cells**

- CIK have both T-cell and **NK (Natural Killer) -like properties**
  - Different cell types, particularly NK cells may provide a broader effect
  - CIKs are not MHC restricted
  - Non-CAR CIK cells were successfully used in cancer therapy

- Potential for **less GvHD**
  - CIK cells were shown to have less GvHD compared to T-cells
  - Relevant in an allogeneic setting

**New targets** CD23 and CD 123 in addition to CD19
Formula’s manufacturing process is proprietary

Formula’s technology is based on NON-viral transfection, with stable expression

- May offer regulatory and manufacturing advantages
- Proprietary, simplified and potentially less expensive manufacturing process

=> Formula’s distinct CAR technology could have a variety of clinical, regulatory, CMC, commercial advantages

✓ Pre-clinical POC established; planned start of ph-1 trial in 4Q2015
✓ GMP manufacturing capabilities established at academic institution
  • Achieve human PoC for 2 targets within 2-3 years
  • Near-term value inflection point opening partnering and exit opportunities
## CAR-CIK pipeline

**Targeting minimal residual disease settings in areas of high unmet need**

<table>
<thead>
<tr>
<th>Indication</th>
<th>Sponsor</th>
<th>Discovery</th>
<th>Pre-clin. PoC</th>
<th>Pre-IND</th>
<th>Phase 1 2015</th>
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<tbody>
<tr>
<td>CD 19 - ALL post allo SCT – establish clinical PoC for platform</td>
<td>Academic / Formula</td>
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<tr>
<td>CD 123 - AML</td>
<td>Academic / Formula</td>
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<tr>
<td>CD 23 - B-cell malignancies</td>
<td>Academic / Formula</td>
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<tr>
<td>Other targets</td>
<td>Formula</td>
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**Funding plan**

- **Bridge financing**
  - $500,000 +
  - Potential interest already for initial $150,000
  - Structure => convertible note with 25% discount over B-round valuation
  - Capital raise targeted to start in early November; closing targeted for January

- **VC financing**
  - Minimum funding target: $10 million
    - PoC study (per target): $3.5 million
    - GMP manufacturing $2 million
    - Pre-Clinical work: $1.5 million
  - Capital raise starting in November; closing targeted for March 2015

- **Strategic partnering activities (incl. corporate VCs) in parallel**
## Management

<table>
<thead>
<tr>
<th>Name</th>
<th>Title</th>
<th>Past affiliations</th>
</tr>
</thead>
<tbody>
<tr>
<td>Maurits Geerlings, MD, MBA</td>
<td>Chief Executive Officer</td>
<td>Infinity Pharma; Cephalon; Prism Pharma; Alexion Pharma; Actinium Pharma; MSKCC</td>
</tr>
<tr>
<td>Roland Turck, MD</td>
<td>President &amp; Chief Operating Officer</td>
<td>Bayer Healthcare; Schering AG; Berlex, Charité</td>
</tr>
<tr>
<td>Robert De Jager, MD</td>
<td>Chief Medical Officer</td>
<td>ICON Clinical Research; Daiichi Pharma; Kosan Bioscience; Conforma Therapeutics; AKZO-Nobel; Poniard Pharma; MSKCC</td>
</tr>
<tr>
<td>Claude Nicaise, MD</td>
<td>Regulatory Affairs</td>
<td>Alexion Pharmaceuticals; Bristol Myers Squibb</td>
</tr>
<tr>
<td>Steve Sweeney, MS</td>
<td>VP, Clinical Operations</td>
<td>Infinity Pharmaceuticals; Pfizer Integrity Clinical LLC</td>
</tr>
<tr>
<td>Jeffrey Mattis, PhD</td>
<td>VP, Pharmaceutical Dev &amp; Manufacturing</td>
<td>Centocor; Molecular Targeting Technology</td>
</tr>
<tr>
<td>Steve Feder, Esq</td>
<td>General Counsel &amp; Secretary</td>
<td>Safeguard Scientifics, Pepper Hamilton LLP, Ballard Spahr LLP, White and Williams LLP</td>
</tr>
<tr>
<td>Eric Steager, MBA</td>
<td>VP, Finance</td>
<td>GPX Enterprises, Safeguard Scientifics, Ankaa Capital Partners, Banc One, Ernst &amp; Young</td>
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## Corporate governance & advisors

<table>
<thead>
<tr>
<th>Board of Directors</th>
<th>Role</th>
<th>Affiliations (Past/Present)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Maurits Geerlings, MD, MBA</td>
<td>Chief Executive Officer; co-founder</td>
<td>Infinity Pharma; Cephalon; Prism Pharma; Alexion Pharma; Actinium Pharma; MSKCC</td>
</tr>
<tr>
<td>Giorgio Mosconi, MD, PhD</td>
<td>Co-founder &amp; Director</td>
<td>Pierrel USA, Acureon, Vicuron; Biosearch Italy; Bristol-Myers Squibb; Marion Merrell Dow</td>
</tr>
<tr>
<td>Martyn Greenacre, MBA</td>
<td>Director</td>
<td>Cephalon, Delsys Pharma, Zynaxis, SmithKline Beecham; various life science companies boards</td>
</tr>
<tr>
<td>Mort Collins, PhD</td>
<td>Director</td>
<td>Battelle Ventures; Data Science Ventures; Cardinal Ventures; The Liposome Company; Alkermes; numerous Life science companies boards</td>
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### Advisors

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<tr>
<td>Daniel von Hoff</td>
<td>Chairman, Scientific Advisory Board – Formula Pharmaceuticals Formerly, President AACR; Board member ASCO; FDA Advisory Committee</td>
</tr>
<tr>
<td>David Scheinberg</td>
<td>Scientific Founder – Formula Pharmaceuticals Chairman, Molecular Pharmacology and Chemistry Program, Sloan-Kettering Institute; Chairman, Experimental Therapeutics Center, Memorial Sloan-Kettering Cancer Center</td>
</tr>
<tr>
<td>Bruce Burlington</td>
<td>Formerly, Deputy Director, FDA; Formerly, EVP Regulatory Affairs at Wyeth Pharma</td>
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<tr>
<td>Dave Williams</td>
<td>Formerly, Chairman &amp; CEO of Sanofi Pasteur</td>
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<tr>
<td>Tommy Thompson</td>
<td>Formerly, US Secretary of Health &amp; Human Services</td>
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<tr>
<td>Frederick (“Fred”) Frank</td>
<td>Vice Chairman at Burrill &amp; Company; Chairman at Burrill Securities</td>
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Contact us:

- **CEO & Founder Maurits Geerlings**: MGeerlings@formulapharma.com
- **COO & President: Roland Turck**: RTurck@formulapharma.com