
CIK-Chimeric Antigen Receptor (CAR) platform

Life Science Summit 2014

Roland Turck, President & COO

Formula Pharmaceuticals, Inc.

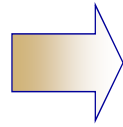
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Formula Pharma – Investment summary

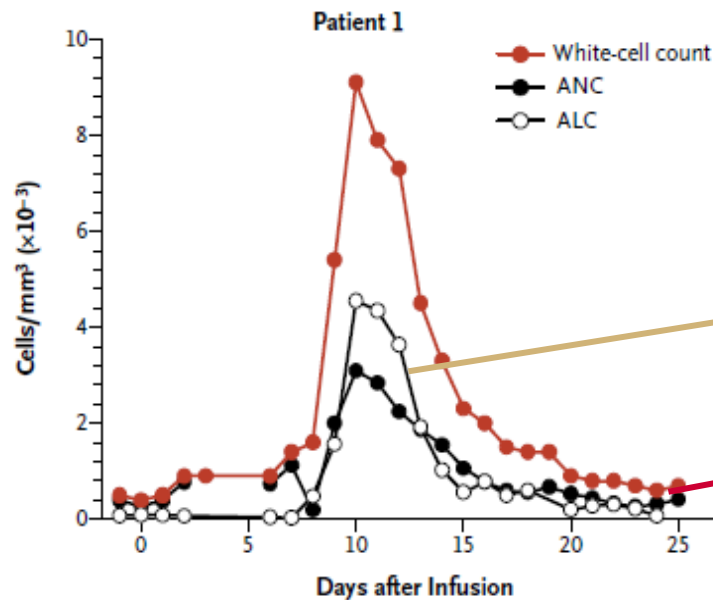
- **Privately held**, onco-immunotherapy company
- **Virtual** operating structure, with highly experienced management
- Developing **CAR platform** using CIK cells and non-viral transfection
- Raising **bridge financing of \$500,000 +** in 2014
- Raising **Series B round of \$10 MM** by 1Q2015
- **Key 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)



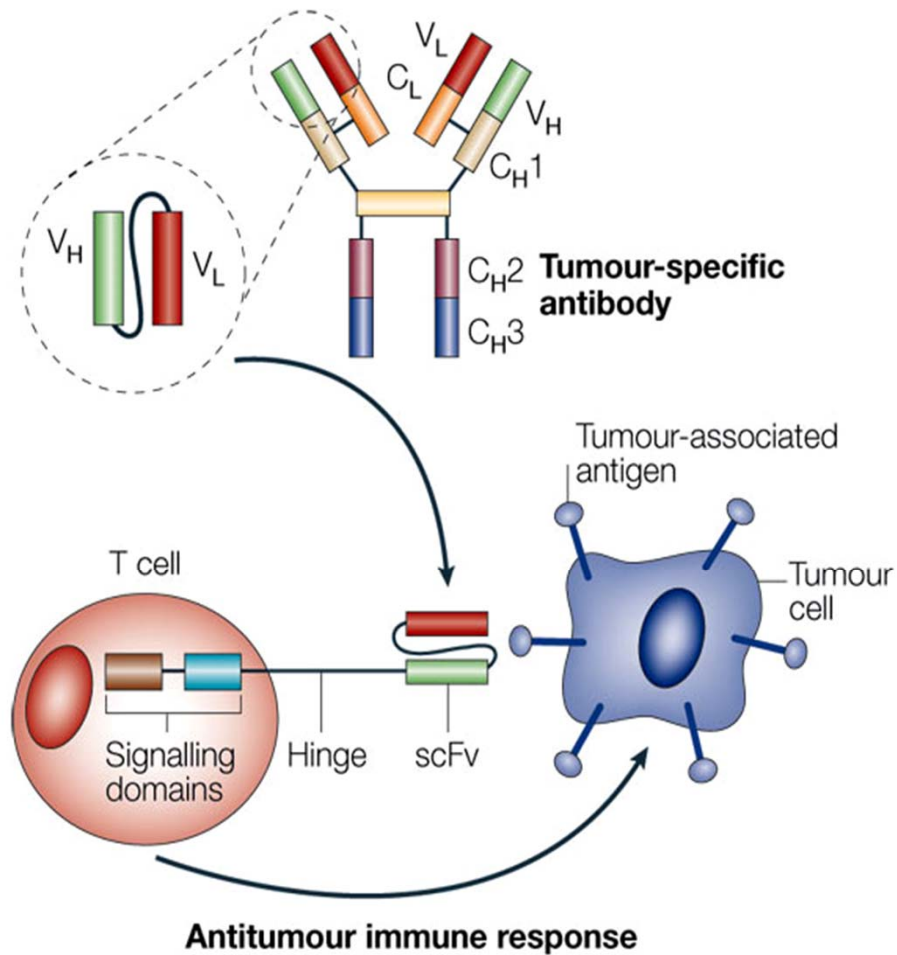
Grupp S. (2013), *N Engl J Med*, 3, 106



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

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Keen industry and investor interest in CAR-T

Biotech originator	Partner	Deals and raises	Latest stage R&D candidate
Juno Therapeutics	MSKCC, Fred Hutchinson, Seattle Children	<ul style="list-style-type: none"> - Series A round closed in 12/2013 - > \$300 M in capital raises within past 12 months 	Phase-1/2 pipeline
Kite Pharma		<ul style="list-style-type: none"> - 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
Collectis	Pfizer	<ul style="list-style-type: none"> - \$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	<ul style="list-style-type: none"> - Partnering deal in 3/2103 \$225 M per therapeutic target - Market cap: \$1.3 B 	Pre-clinical stage pipeline
Novartis	UPenn, Carl June	<ul style="list-style-type: none"> - 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

Indication	Sponsor	Discovery	Pre-clin. PoC	Pre-IND	Phase 1 2015
CD 19 - ALL post allo SCT – establish clinical PoC for platform	Academic / Formula				
CD 123 - AML	Academic / Formula				
CD 23 - B-cell malignancies	Academic / Formula				
Other targets	Formula				

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

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

Corporate governance & advisors

Board of Directors	Role	Affiliations (Past/Present)
Maurits Geerlings, MD, MBA	Chief Executive Officer; co-founder	Infinity Pharma; Cephalon; Prism Pharma; Alexion Pharma; Actinium Pharma; MSKCC
Giorgio Mosconi, MD, PhD	Co-founder & Director	Pierrel USA, Acureon, Vicuron; Biosearch Italy; Bristol-Myers Squibb; Marion Merrell Dow
Martyn Greenacre, MBA	Director	Cephalon, Delsys Pharma, Zynaxis, SmithKline Beecham; various life science companies boards
Mort Collins, PhD	Director	Battelle Ventures; Data Science Ventures; Cardinal Ventures; The Liposome Company; Alkermes; numerous Life science companies boards
Advisors		
Daniel von Hoff	Chairman, Scientific Advisory Board – Formula Pharmaceuticals Formerly, President AACR; Board member ASCO; FDA Advisory Committee	
David Scheinberg	Scientific Founder – Formula Pharmaceuticals Chairman, Molecular Pharmacology and Chemistry Program, Sloan-Kettering Institute; Chairman, Experimental Therapeutics Center, Memorial Sloan-Kettering Cancer Center	
Bruce Burlington	Formerly, Deputy Director, FDA; Formerly, EVP Regulatory Affairs at Wyeth Pharma	
Dave Williams	Formerly, Chairman & CEO of Sanofi Pasteur	
Tommy Thompson	Formerly, US Secretary of Health & Human Services	
Frederick (“Fred”) Frank	Vice Chairman at Burrill & Company; Chairman at Burrill Securities	

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Contact us:

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- COO & President: Roland Turck: RTurck@formulapharma.com