Moleculin

NASDAQ: MBRX
Non-Confidential Information
January 14, 2019

Disclaimer

All statements contained herein other than statements of historical fact, including statements regarding our future results of operations and financial position, our business strategy and plans, and our objectives for future operations, are forward-looking statements. The words “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “intend,” “expect,” and similar expressions are intended to identify forward looking statements. We have based these forward-looking statements largely on our current expectations and projections about future events and trends that we believe may affect our financial condition, results of operations, business strategy, short-term and long-term business operations and objectives, and financial needs. These statements are only predictions and involve known and unknown risks, uncertainties, and other factors, including those discussed under Item 1A. "Risk Factors" in our most recently filed Form 10-K filed with the Securities and Exchange Commission ("SEC") and updated from time to time in our Form 10-Q filings and in our other public filings with the SEC. Any forward-looking statements contained in this release speak only as of its date. Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the future events and trends discussed in this presentation may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward looking statements. More detailed information about Moleculin is set forth in our filings with the Securities and Exchange Commission. Investors and security holders are urged to read these documents free of charge on the SEC’s web site at http://www.sec.gov.

Robust pipeline

3 distinctly different technologies, all with blockbuster potential

World-leading collaboration

MD Anderson Cancer Center/Mayo Clinic/Emory

Breakthrough disruptive technologies

Annamycin for AML: designed to be non-cardiotoxic, avoids MDR1

WP1066: STAT3 inhibitor that also stimulates immune response

WP1122: metabolic inhibitor with improved BBB transmission and animal model activity against pancreatic cancer

Highly experienced leadership

Veteran pharma/biotech, life science micro-cap managers

Proprietary positioning

Orphan drug and/or patents, exclusive licenses (applied and received)

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Experienced Management Team

Walter Klemp,
Chairman, President & CEO

Don Picker,
PhD
Chief Science Officer

Jonathan P. Foster,
CPA, CGMA
EVP & CFO

Robert Shepard,
MD, FACP
Chief Medical Officer

Sandra Silberman,
MD & PhD
Chief Medical Officer – New Products

Selected Prior Experience

soliton · ZENO · Inc. 500 · Drypers · Coopers & Lybrand

SYNERGY PHARMACEUTICALS · TAPESTRY Life Science

Rx CARBOPLATIN
Carboplatin Injection BP 150 mg/15ml
For Intravenous use only

InfuSystem INFUSION MADE EASY · LSG Sky Chefs · Drypers · Schlumberger · Deloitte.

DANA-FARBER CANCER INSTITUTE · UNIVERSITY OF VIRGINIA 1819 · HARVARD UNIVERSITY · Takeda · FDA · Tufts UNIVERSITY

AstraZeneca · Roche · Bristol-Myers Squibb · NOVARTIS · Pfizer

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The NEW ENGLAND JOURNAL of MEDICINE

THE LANCET Oncology Haematology

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DANA-FARBER CANCER INSTITUTE

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Board Of Directors

Robert E. George - Chair of Audit and Nominating & Governance Committees

Mr. George joined our board of directors upon our IPO. He was a partner with the international accounting firm of PricewaterhouseCoopers (PWC) for 27 years until 2010. Mr. George currently serves as Chairman of the Audit Committee for The University of Texas Health Science Center at Houston and, since June 2011, has been a member of The University of Texas at Austin, McCombs Graduate School of Business accounting faculty.

Michael D. Cannon - Chair of Compensation Committee

Between 1997 and 2004, Mr. Cannon was the Chief Science Officer, EVP and a Director of SICOR, Inc. until its acquisition by Teva Pharmaceutical Industries, Inc. SICOR focused on generic finished dosage injectable pharmaceuticals, active pharmaceutical ingredients and generic biopharmaceuticals. From July 2005 to December 2009, Mr. Cannon was a member of the scientific advisory board of Trevi Health Ventures LP. Mr. Cannon currently serves on the board of directors of other privately held biotech companies.

John M. Climaco, JD – Lead Independent Director

Most recently the Executive Vice President of Perma-Fix Medical S.A, a Polish subsidiary of the Perma-Fix Environmental Services, Inc. (NASDAQ: PESI) where he has served as a director since 2013. From 2003 to 2012, Mr. Climaco served as President and Chief Executive Officer, as well as a member of the Board of Directors of Axial Biotech, Inc., which he cofounded in 2003. Mr. Climaco has served as a member of the Board of Directors for Digirad Corporation (NASDAQ: DRAD), PDI, Inc. (NASDAQ: PDII) and InfuSystem Holdings, Inc. (NASDAQ: INFU). From 2001 to 2007, he practiced law for the firm of Fabian and Clendenin. Mr. Climaco holds a J.D. from the University of California Hasting College of the Law.

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Focus on Highly Resistant Tumors

Example Indications

- R/R AML
- Glioblastoma
- Pancreatic
- Met melanoma
- Other

Common Characteristics

- Multidrug resistance
- Immune evasion
- Upregulation of oncogenic transcription factors
- Increase in dependence on glycolysis

Regulatory Advantage

- Significant unmet need
- Modest gains = new drug approval
- Accelerated approval pathway

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3 Core Technologies

Next Generation Anthracycline

- Avoids multidrug resistance
- Little to no cardiotoxicity

Immune/Transcription Modulator

- Enables immune response
- Inhibits p-STAT3, c-Myc and HIF-1α

Metabolism/ Glycosylation Inhibitor

- Prodrug enables drug-like properties in glucose decoy
- Altering glycosylation enables immune checkpoint inhibitors

Potential for Combination

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Development Pipeline

	Preclinical	Clinical Preparation	Phase 1/2	Collaboration
AML	Annamycin			MD Anderson Cancer Center
	Annamycin + WP1066			
Brain Tumors	WP1066 - Glioblastoma, melanoma metastasized to the brain			MD Anderson Cancer Center
	WP1066 - Pediatric brain tumors			MAYO CLINIC, EMORY UNIVERSITY
	WP1122 - Glioblastoma			
	WP1122 + Avastin - Glioblastoma			UNIVERSITY OF BERGEN
Pancreatic Cancer	WP1066/WP1732			
	WP1122/WP1234			
Other Cancers	WP1220 - Cutaneous T-Cell Lymphoma			UNIVERSITY OF GDANSK
	WP1066 - Ocular tumors			

Next Gen Anthracycline | Immune/Transcription | Metabolism/Glycosylation | Combination

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Technology Review

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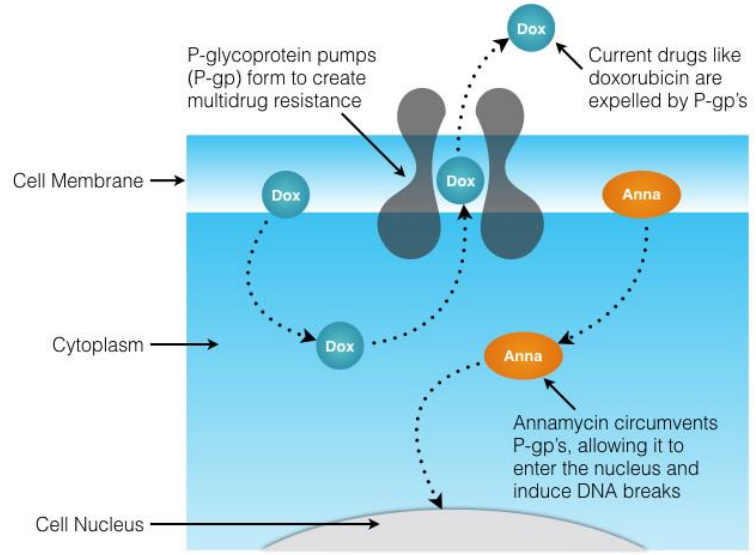
Annamycin (Next Generation Anthracycline)

Critical Advantages over Leading Drug	Leading AML induction therapy drugs are cardiotoxic and lose efficacy due to multidrug resistance Annamycin has little to no cardiotoxicity, avoids multidrug resistance, has been shown to be more potent in AML cell lines and has shown activity in patients who failed standard of care
Potential to Significantly Improve Health	Annamycin has shown the potential to significantly improve health in a Phase I/II acute myeloid leukemia (AML) trial Orphan Drug status as single agent for relapsed or refractory AML
Positioned for Accelerated Approval	Annamycin appears to be well suited for an accelerated approval pathway in the US, and in Europe Absence of any approved second-line drug for most AML patients represents a significant unmet need Potentially shorter time scale for saving lives than with typical cancer drugs

Annamycin Process



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Annamycin Delivers

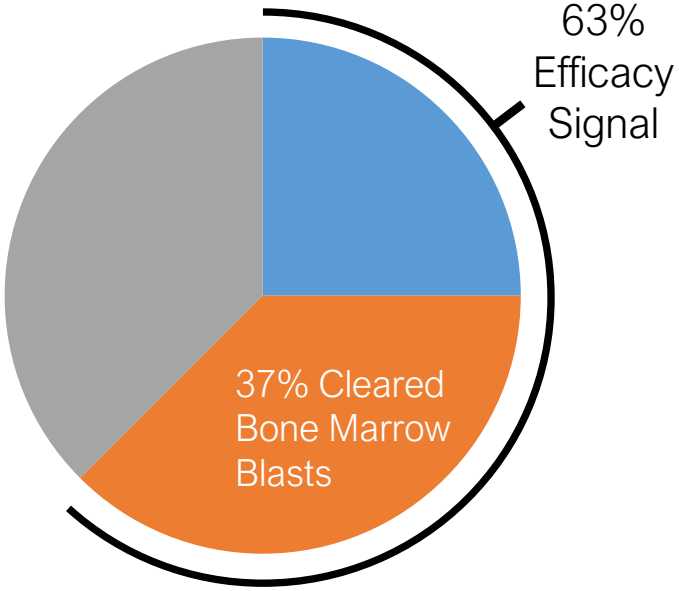
Remarkable Performance

In a proof-of-concept Phase I/II clinical trial, Annamycin was given to patients who had failed an average of five previous induction therapy attempts

37% of those patients cleared enough of their leukemic cells to qualify for a bone marrow transplant

We believe repeating this performance in a larger clinical trial, could warrant new drug approval

Relapsed/Refractory Acute Leukemia Patients



Annamycin gives new hope to patients who have run out of options

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Annamycin Recap and Status

Annamycin is a “Next Generation” anthracycline designed to be non-cardiotoxic and avoid multidrug resistance

Prior developer failed financially and lost rights to license

Although impressive activity was shown in prior acute leukemia trials, developer did not properly close out or establish an appropriate RP2D

Moleculin is repeating Phase 1/2 both in US and EU

US Trial (MB-104) has begun treating patients; EU trial is about to begin

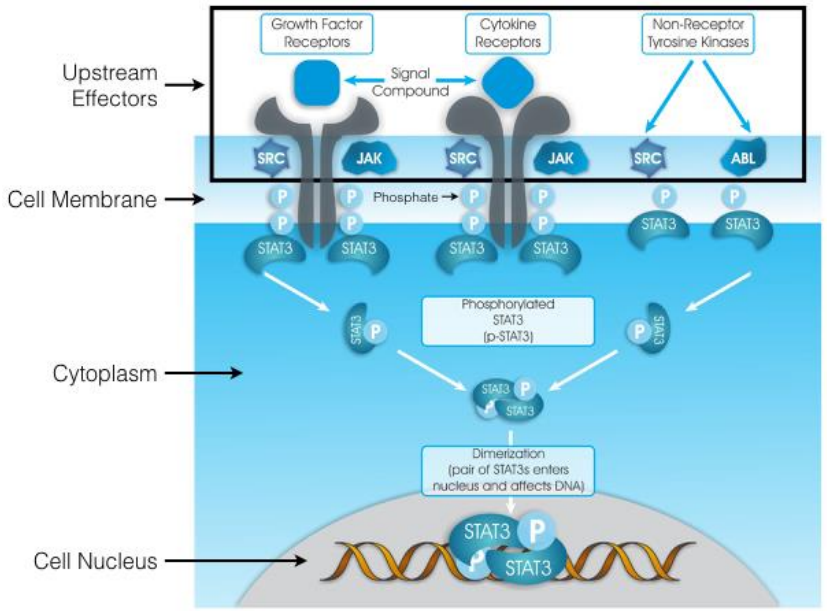
Repeat of prior results should afford Annamycin an accelerated approval pathway as a 2nd line induction therapy for R/R AML

Other indications include sarcomas, lewis lung carcinoma and squamous cell carcinoma, among others

WP1066 Immune/Transcription Modulators

Based On Natural Compound	Built from chemical backbone of propolis (caffeic acid benzyl ester)
Unique Dual Action	First-in-class drug to both directly inhibit tumor signaling (p-STAT3, HIF-1α, c-Myc) while also stimulating patient immune response (Tregs)
Activity Against Hardest-to-Treat Cancers	Pre-clinical testing shows high level of activity against pancreatic cancer, metastatic melanoma, glioblastoma and others; yet very low potential for toxicity
Independently Validated	Subject of numerous peer reviewed journals validating findings across multiple institutions around the world

STAT3 is Activated by Multiple Upstream Effectors



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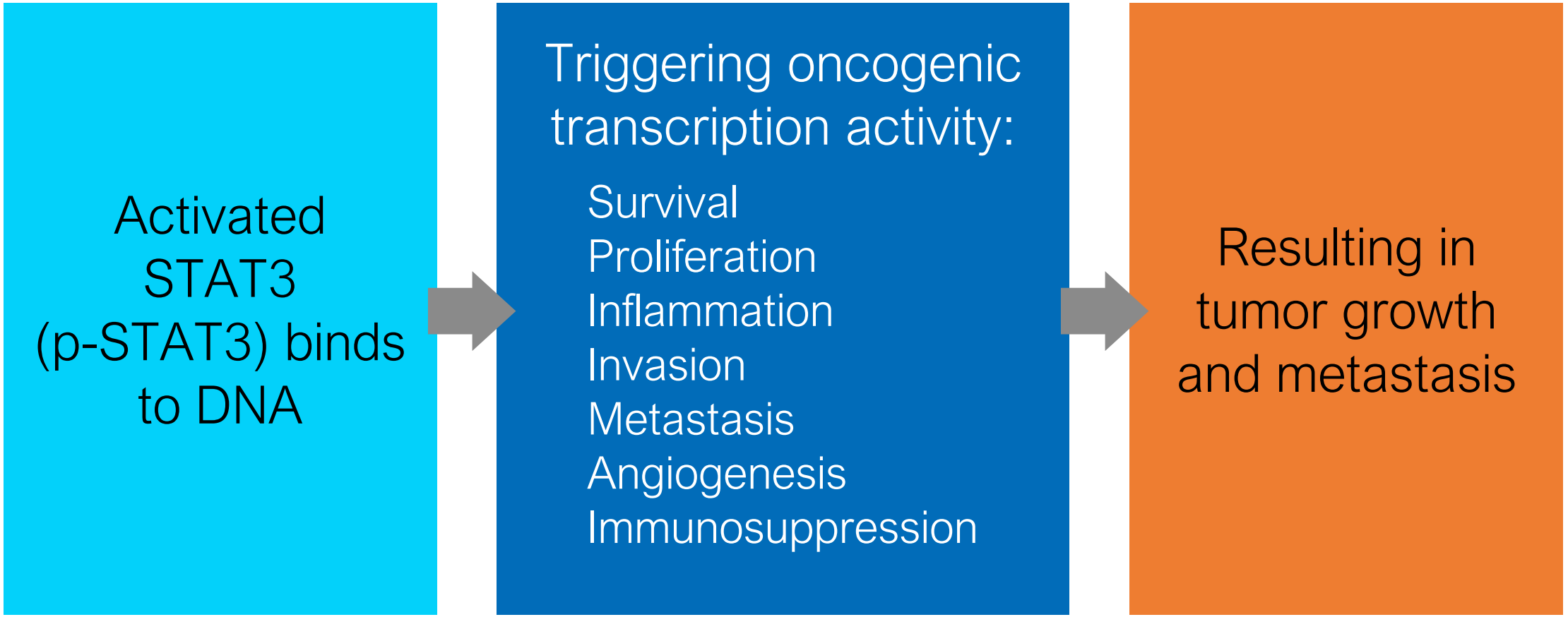
STAT3 is a Master Regulator of Tumor Progression



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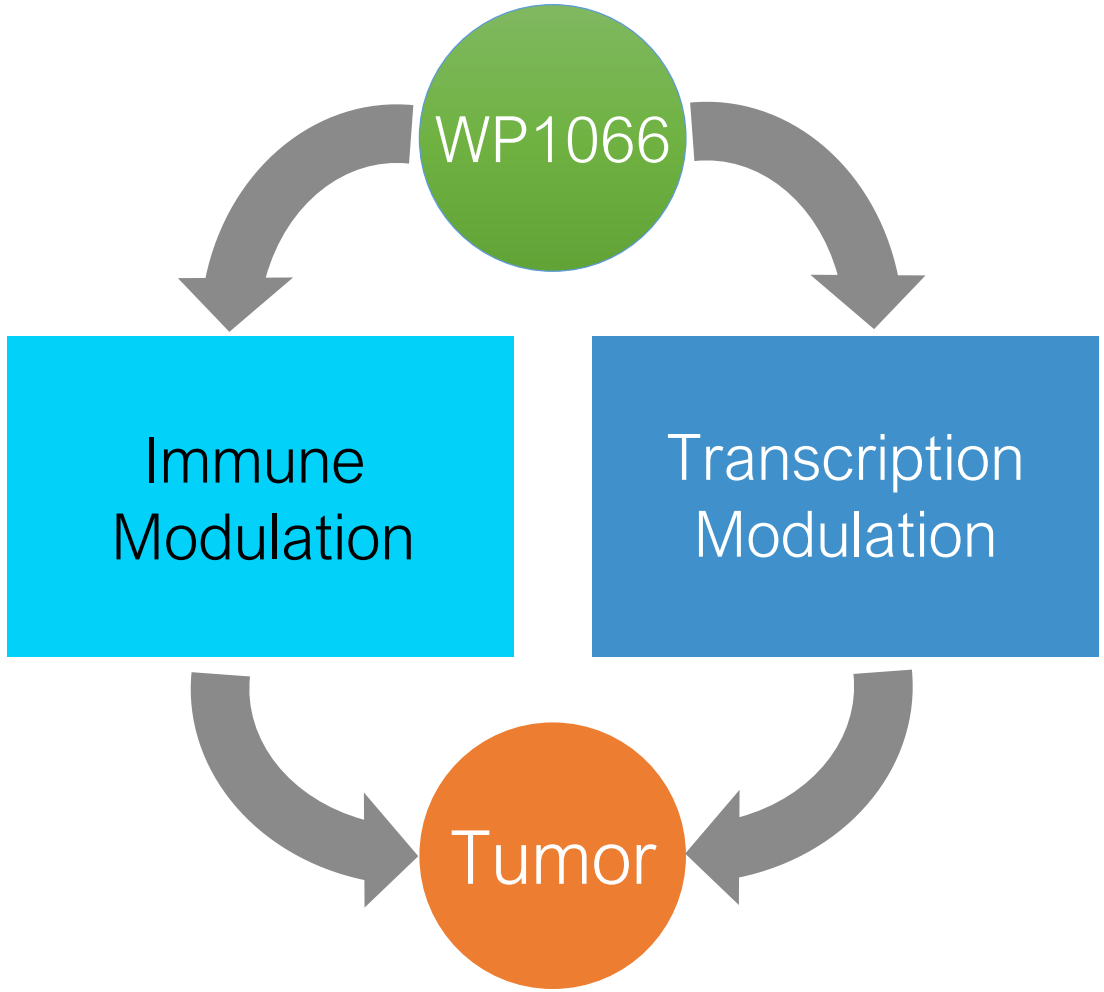
WP1066 Affects Tumors Directly and Indirectly

WP1066 modulates transcription factors resulting in direct tumor cytotoxicity while also stimulating a natural immune response by reducing Regulatory T-cells (Tregs)



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Transcriptional Control of Cancer

Immunosuppressive Oncogenic Axis

HIF-1α — STAT3 — c-Myc

Implications for:

- GBM
- Pancreatic cancer
- Other cancers

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WP1066 Recap and Status

WP1066 is a small molecule that inhibits p-STAT3 by accelerating proteasomal degradation (not by blocking phosphorylation) without respect to upstream signaling

WP1066 also inhibits HIF1-α, c-Myc and Tregs

Currently in Phase 1 trial at MD Anderson for GBM and melanoma metastasized to the brain (WP1066 crosses BBB); in 3rd cohort of dose ranging; planned surgical expansion

Oral administration (due to lack of solubility) is demonstrating bioavailability in patients

Recently began IND-enabling work on WP1732, a fully water soluble analog of WP1066 that does not cross BBB, and shows impressive accumulation in pancreas

WP1122 Metabolism/Glycosylation Inhibitor

Addicted to Sugar	A brain tumor requires as much as 18 to 37 times as much glucose to survive as a healthy brain cell
Starving a Tumor to Death	This eventually led to the theory that, if we feed tumor cells a glucose decoy (one that can't convert into energy), we can kill the tumor This works well in a laboratory setting, but the problem is making these decoys drugable
Breakthrough design	WP1122 is a prodrug of 2-deoxyglucose (2-DG) that increases half-life, enables transmission across the blood brain barrier and improves other drug-like properties
Potential to Change Standard of Care	WP1122 (at suboptimal doses) performs as well or better than temozolomide in live human brain tumors; even better performance by combining the two drugs was shown in trials

Tumors are hyper-consumers of glucose and starve to death without it

Tumor cell

Normal cell

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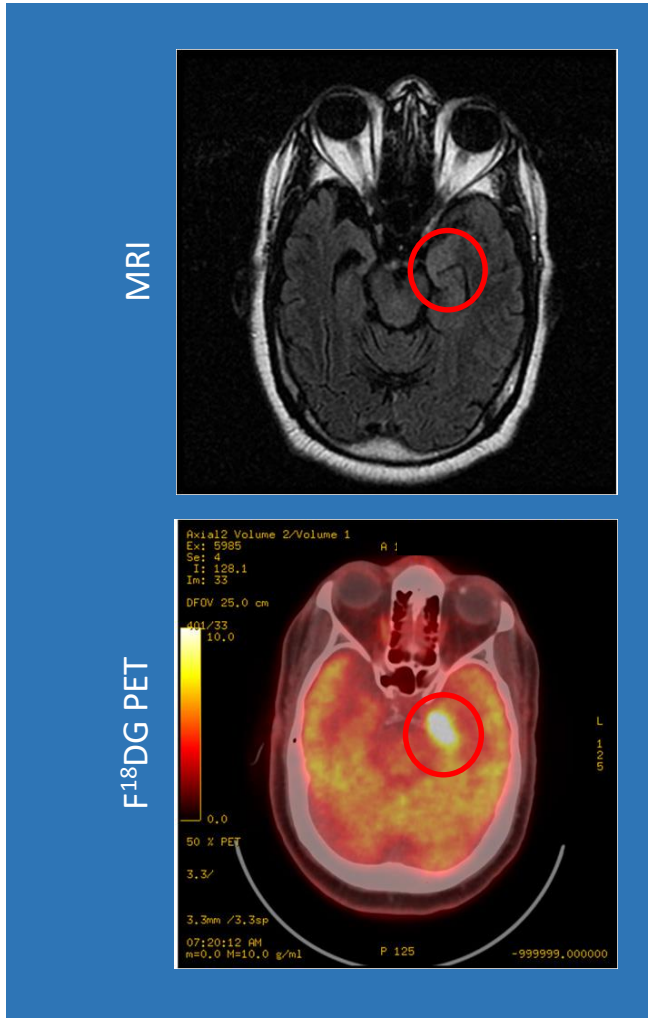
Many Tumors are Highly Glycolytic



Tumors rely preferentially on glycolysis even in the presence of abundant oxygen

For example: PET diagnostic imaging relies on a modified glucose with a radio-tracer ($F^{18}DG$)

Tumors over-consume $F^{18}DG$ because of their dependence on glycolysis

Cutting off this "fuel supply" (inhibiting glycolysis) results in targeted tumor cell death

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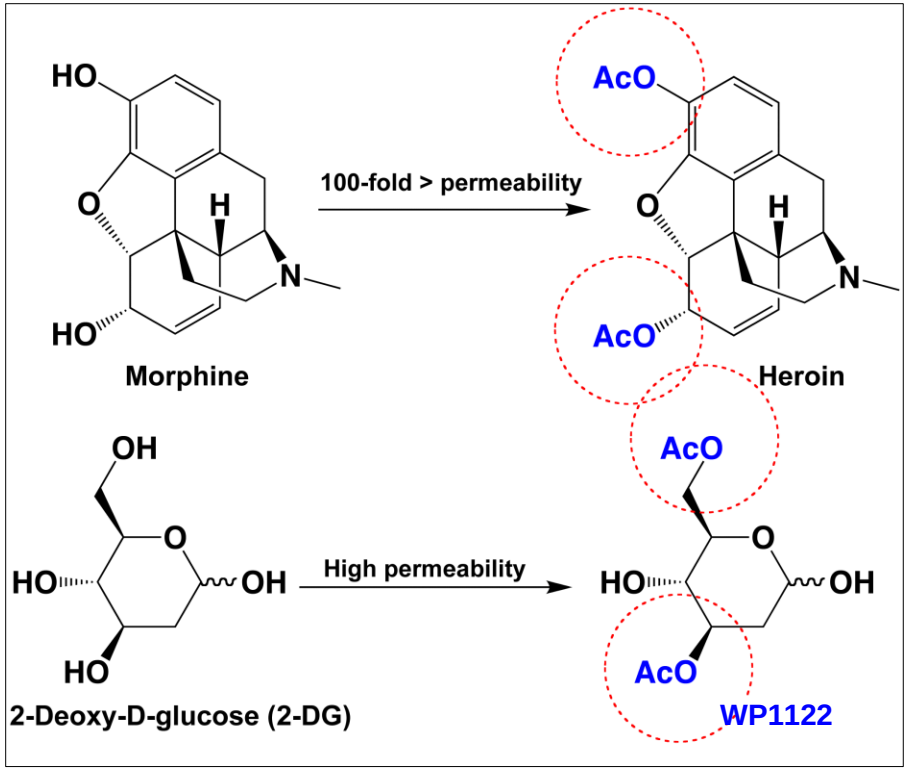
Chemistry – Improving the Drug-Like Properties of 2-DG and Targeting Brain Cancers

Example of heroin

Heroin is the diacetyl ester of morphine that increases by 100-fold levels of morphine in the brain

WP1122 is the diester of 2-DG of our design, which greatly enhances CNS uptake and levels 2-DG in the brain



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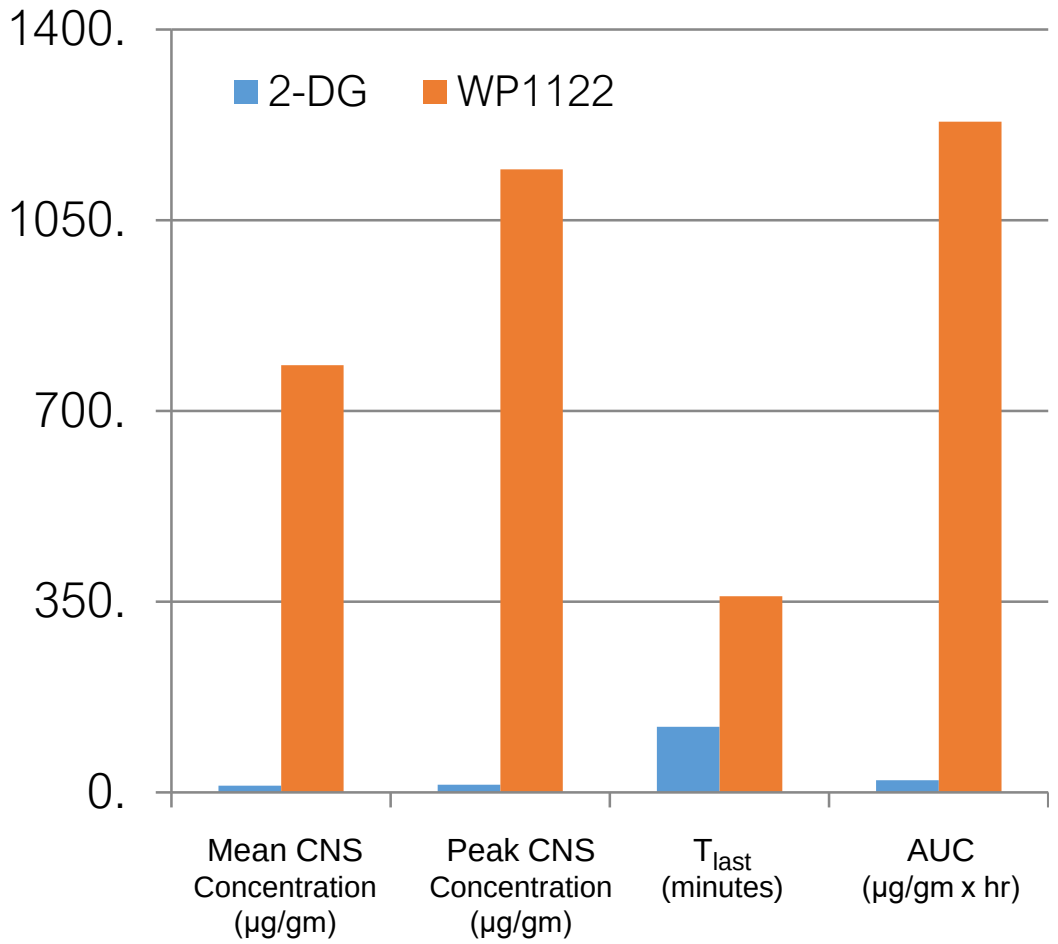
Pharmacology (PK/PD)



2-DG CNS distribution and retention was measured after oral administration of equimolar amounts of 2-DG and its diacetate WP1122

CNS distribution and retention of 2-DG is dramatically higher when generated from WP1122

No observed systemic toxicity

Data presented at annual meeting of:

National Brain Tumor Society

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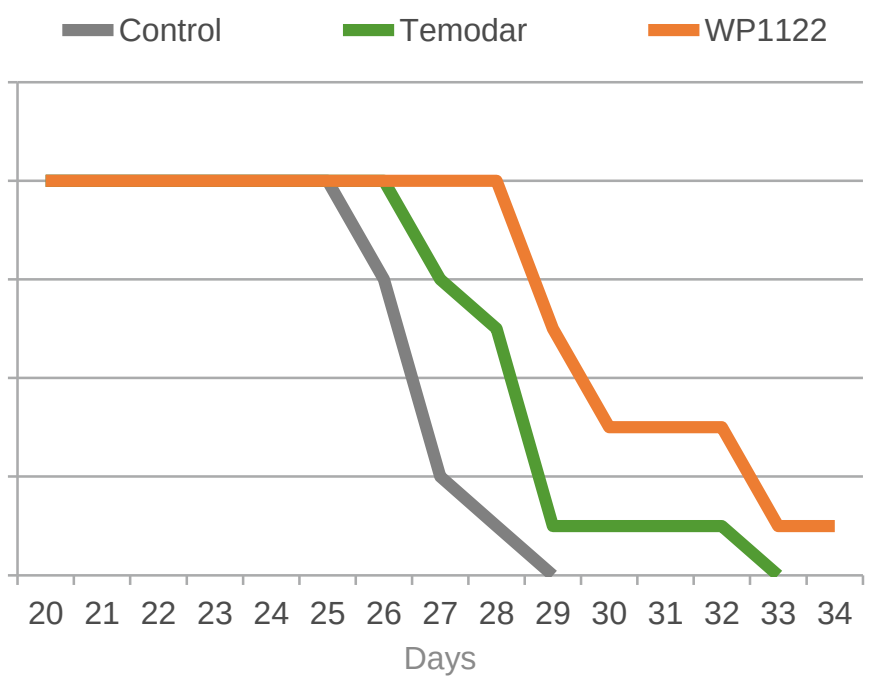
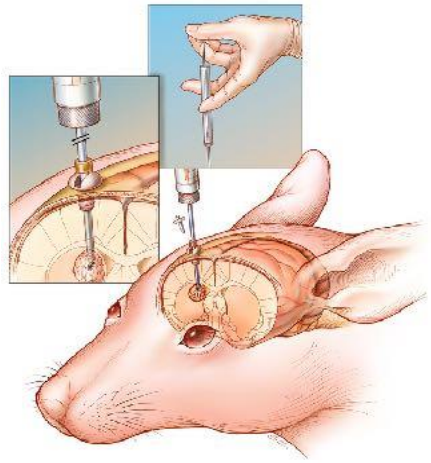
WP1122 is Effective *In Vivo* against Gliomas - July 2017





Orthotopic Glioblastoma Model in Mice

Human brain tumors injected into mice

WP1122 performed as well or better than temazolomide

Not shown is that a combination of both performed even better

WP1122 used alone has at least the same or greater activity than temozolomide (Temodar®), a current standard of care in patients diagnosed with glioblastoma

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WP1122 Shows In Vivo Activity Against Pancreatic Cancer - January 2018

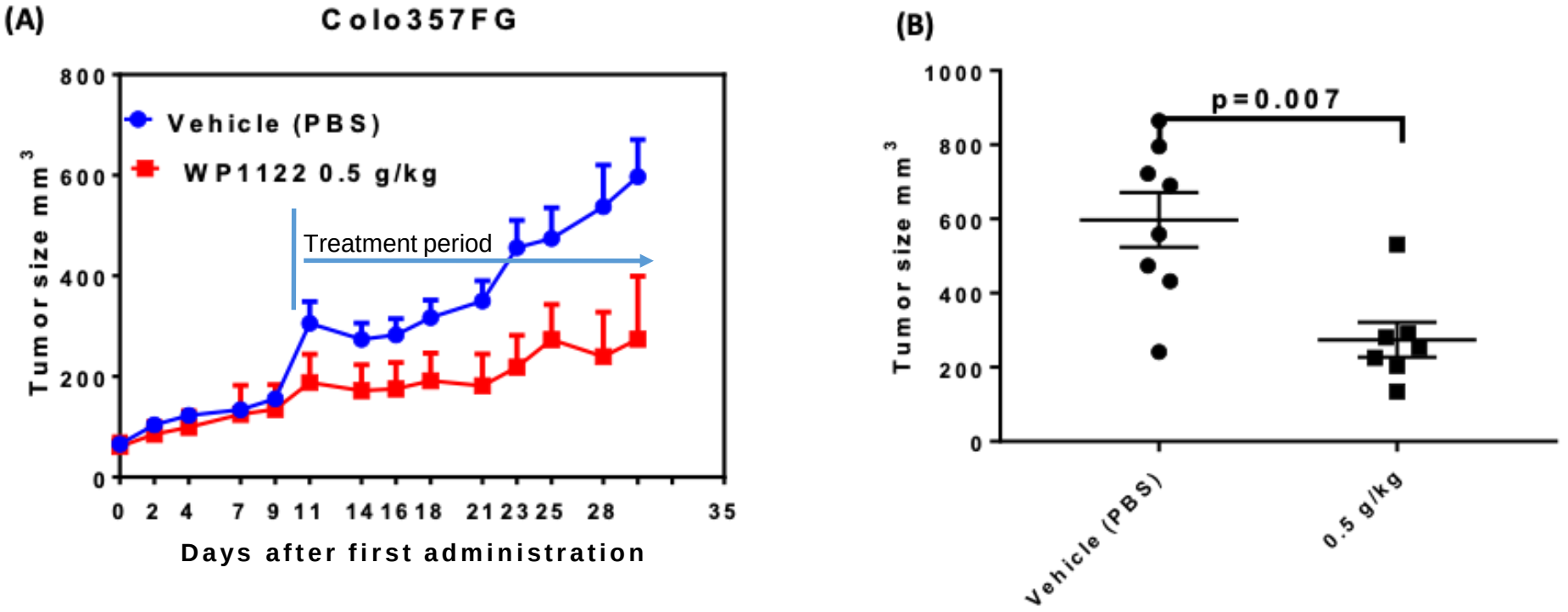

Figure 1. WP1122 inhibits tumor growth in pancreatic Colo 357FG tumor model

Nude athymic mice, were implanted subcutaneously with 1 mln of Colo357 FG cells. At day ten animals were treated with 0.5 g/kg of WP1122 administered orally, once a day for 5 days (M-F) every week. Tumor volume was measured using electronic caliper. **(A)** change in tumor volume in time; **(B)** tumor volume distribution at day 30.

Inhibitors of Sugar Metabolism in Cancer and Immunotherapeutic Implications

Glycolysis
Glycosylation

2-DG
2-Deoxyglucose
or
2-Deoxymannose?

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WP1122 Recap and Status

WP1122 is a prodrug of 2-DG that increases circulation time and ability to cross BBB and provides other drug-like properties

Utilizing Warburg principle, WP1122 converts to 2-DG in highly glycolytic tumor cells causing autophagy due to energy starvation

Evaluating oral versus IV administration and about to begin IND-enabling work

Recent published literature suggests that 2-DG may also enable deglycosylation of PD-L1 (see abstract in following slide)

WP1234 is an analog of WP1122 with improved organ distribution to the pancreas

Am J Cancer Res 2018;8(9):1837-1846
www.ajcr.us /ISSN:2156-6976/ajcr0083303

American Journal of Cancer Research

ISSN: 2156-6976

Original Article

Deglycosylation of PD-L1 by 2-deoxyglucose reverses PARP inhibitor-induced immunosuppression in triple-negative breast cancer

Bin Shao[1,2], Chia-Wei Li[1], Seung-Oe Lim[1,3], Linlin Sun[1,4], Yun-Ju Lai[5], Junwei Hou[1], Chunxiao Liu[1], Chiung-Wen Chang[1], Yufan Qiu[1], Jung-Mao Hsu[1], Li-Chuan Chan[1,6], Zhengyu Zha[1], Huiping Li[2], Mien-Chie Hung[1,6,7]

Abstract: Triple-negative breast cancer (TNBC), the most difficult-to-treat breast cancer subtype, lacks well-defined molecular targets. TNBC has increased programmed death-ligand 1 (PD-L1) expression, and its immunosuppressive nature makes it suitable for immune checkpoint blockade therapy. However, the response rate of TNBC to anti-PDL1 or anti-programmed cell death protein 1 (PD-1) therapy remains unsatisfactory, as only 10-20% of TNBC patients have a partial response. Glycosylated PD-L1, the functional form of PD-L1, is required for PD-L1-PD-1 interaction. TNBC cells have significantly higher levels of glycosylated PD-L1 than non-TNBC cells do. In a screening of glucose analogs to block PD-L1 glycosylation, we found that 2-deoxyglucose (2-DG) can act as a glucose analog to decrease PD-L1 glycosylation. Because PARP inhibition upregulates PD-L1, 2-DG reduced PARP inhibition-mediated expression of glycosylated PD-L1. The combination of PARP inhibition and 2-DG had potent anti-tumor activity. Together, our results provide a strong rationale for investigating the targeting of PD-L1 glycosylation in TNBC further.

Milestones and Financials Review

Anticipated Milestones	Potential Timeframe
NextGen – Anthracycline - Annamycin	
Initial IRB (Institutional Review Board) approvals and site initiations of various clinical sites participating in our Phase I/II clinical trial of Annamycin	**Accomplished** and ongoing 2019
Complete cohort of 150 mg/m2 - prior trial recommended phase Ii dose (RP2D)	2019
Start treating patients in Annamycin Phase I/II clinical trial in Poland	Q1-2019 (Screening has begun with drug in country)
Announcement of initial clinical data for Annamycin trial	2019
Poland clinical trial (MB-105) begins Phase II	2020
Approach FDA on US trial (MB-104) on dose expansion using Poland trial data	2020
Immune/Transcription Modulator – WP1066	
Announcement of initial clinical data from WP1066 clinician sponsored trial	2019
Phase I surgical cohort begins	Second Half of 2019
Transfer clinician sponsored trial WP1066 IND to Moleculin	Second Half of 2019
Emory Physician Led Pediatric Medulloblastoma Trial begins	Second Half of 2019
Announcement of further benefits of our sponsored research agreement with MD Anderson	**Accomplished** and Ongoing into 2019
Announce filing and approval of CTA for WP1220 for the treatment of cutaneous T-cell lymphoma (CTCL)	2019 **(CTA Filed)**
Assess WP1220 initial patient data	Q4-2019
IND for WP1732 submitted	First Half of 2019
Dose first patient in Phase I trial for WP1732	2020
Announce further research preclinical results on WP1066 family	First Half of 2019
Metabolism/Glycosylation Inhibitor	
Begin preclinical work on WP1122	**Accomplished**
File IND 1122	2020
General Clinical	
Announce a fourth and fifth approved clinical trial	2019

Financial Statement Summary

In thousands $ - except for loss per share and shares outstanding	For the Nine Months Ended 9/30/18	For the Year Ended 12/31/17
	(Unaudited)	(Audited)
Statement of Operations Data		
Revenue	-	-
Research and development	6,801	4,545
General and administrative	3,886	4,108
Total operating loss	(10,687)	(8,653)
Net loss	(9,091)	(9,805)
Net loss per common share – basic and diluted	$ (0.36)	(0.53)
Balance Sheet Data		
Cash and cash equivalents	$ 8,600	7,714
Prepaid expenses and other	760	588
Total current assets	9,360	8,302
Total assets	20,899	19,483
Total current liabilities	3,129	2,215
Total liabilities	5,931	2,365
Accumulated deficit	(23,571)	(14,480)
Total stockholders' equity	14,968	17,118
Shares outstanding	26,861,497	21,469,109
Shares outstanding as of December 31, 2018 – 28,528,663		

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The above financial information is summarized from the Company's MOST CURRENT FORMS 10-Q & 10-K filed with the SEC (www.sec.gov)

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3 distinctly different technologies, all with blockbuster potential

World-leading collaboration

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Breakthrough disruptive technologies

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WP1066: STAT3 inhibitor that also stimulates immune response

WP1122: metabolic inhibitor with improved BBB transmission and animal model activity against pancreatic cancer

Highly experienced leadership

Veteran pharma/biotech, life science micro-cap managers

Proprietary positioning

Orphan drug and/or patents, exclusive licenses (applied and received)

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