



Optimizing Immunotherapies

CORPORATE OVERVIEW

(Please contact us to request confidential version of presentation with supporting data)



Forward Looking Statements:

Certain statements contained in this presentation or in other documents of Vycellix, Inc. (the “Company”), along with certain statements that may be made by management of the Company orally in presenting this material, may contain “forward-looking statements” as defined in the Private Securities Litigation Reform Act of 1995. These statements can be identified by the fact that they do not relate strictly to historic or current facts. They use words such as "estimate," "expect," "intend," "believe," "plan," "anticipate," “projected” and other words and terms of similar meaning in connection with any discussion of future operating or financial performance or condition. These statements are based upon the current beliefs and expectations of the Company's management and are subject to significant risks and uncertainties.

Corporate Overview

- Closely-held biotech advancing innovative small molecules that optimize and enhance human immune response against disease with a focus on cancers
- Founded in 2015 on IP developed by medical researchers at Sweden's Karolinska Institutet (KI)
- Pipeline of novel, targeted small molecules for *ex vivo* and *in vivo* modulation of cells
- Lead products have demonstrated unrivaled enhancement of cytotoxic lymphocytes
- Scientific founders include KI's current Head of Gene & Cell Therapy Group and KI's former Dean of Research

Vycellix Founding Board of Directors:



Evren Alici, M.D., Ph.D.: CEO & Chairman

- Head, Gene and Cell Therapy Group, Division of Hematology, Department of Medicine, Karolinska Institutet, Karolinska University Hospital, Stockholm



Hans-Gustaf Ljunggren, M.D., Ph.D.: Chief Medical Officer

- Former Dean of Research, Karolinska Institutet and Founder of the Center for Infectious Medicine, Department of Medicine, Karolinska Institutet, Karolinska University Hospital, Stockholm



Douglas W. Calder: President

- Strategic Advisor to the NSU Cell Therapy Institute; former Executive VP & Officer roles at the Vaccine & Gene Therapy Institute (VGTI), Accentia Biopharmaceuticals, Viragen and Biovest International



Samuel Duffey, Esq.: Exec VP, General Counsel

- CEO of ViraCell, a hollow-fiber manufacturing VLP vaccine company based at Texas A&M; former roles include President & CEO of Accentia Biopharmaceuticals and Biovest International, and attorney at SEC



Michael J. Keller, J.D.: Exec VP, Intellectual Property Officer

- CEO, Keller Life Science Law; former roles include Chief Patent Counsel for IVAX, partner at leading IP law firms, and technology transfer roles at the National Cancer Institute (NCI)

Unrivaled Enhancement of NK cells & T cells

Lead product candidates: **VY-OZ** & **VY-X**

VY-OZ

- Optimizing gene modification

VY-X

- Rapidly increasing *ex vivo* and *in vivo* immune response

Pre-IND Candidates: A Pipeline in Each Molecule

VY-OZ

Small Molecule Immunomodulator

Ex Vivo Applications

OPTIMIZING ADOPTIVE CELL THERAPIES

Genetic Modification Enhancement Platform

Unrivaled Transduction Rates for Lymphocytes and Stem Cells

IMPROVING GENE EDITING EFFICACY

Optimizes CRISPR-Cas9 Gene Editing Rates

Decreases gRNA Recognition & Increases Editing Efficacy

VY-OZ

In Vivo Applications

SUPPRESSING CERTAIN RNA PATHWAYS

Adjuvant Therapy for Dampening Viral Inflammation

Myositis, Myocarditis, Encephalitis, Meningitis

Adjuvant Therapy to Oncolytic Viral Vector Therapies

Enhances Distribution of Oncolytic Virus Within Tumor

VY-X

Small Molecule Immunomodulator

Ex Vivo Applications

OPTIMIZING ADOPTIVE CELL THERAPIES

Platform to Boost Serial Killing for Cytotoxic Lymphocytes

Unrivaled Increased Levels of Perforin & Granzyme B

VY-X

In Vivo Applications

HIGHLY SPECIFIC ANTI-TUMOR AGENT

Significantly Inhibits Tumor Development in Myeloma Models

Targeted Expression of Perforin and Granzyme B

Product Development Strategy:

- ✓ Platform technology for the efficient, cost-effective modulation of cell and gene therapies
- ✓ Monotherapy and/or adjuvant therapeutics targeting cancers and infectious disease
- ✓ Enhancers of gene editing for CRISPR-Cas9 systems



Pipeline Review

VY-OZ

VY-OZ: *Ex Vivo* Genetic Modification

Despite NK cells being inherently resistant to retroviral infections, **VY-OZ**:

- Enables retroviral and lentiviral gene delivery to NK cells, targeting intracellular antiviral defense mechanisms
- Provides dose dependent effect with no observed toxicity

VY-OZ *Ex Vivo* Platform Summary

- Optimizes genetic modification of T cells & NK cells
- Supports design of:
 - NK cells expressing cytokines
 - Silenced inhibitory receptors
 - Activating receptors
 - CARs
- Increases gene editing rates for CRISPR systems



Pipeline Review

VY-X

VY-X Ex Vivo Platform: Enhances Serial Killing

Standard practice for adoptive cell transfer is limited by processes, including *in vitro* cytokine exposure, that fail to obtain sufficient serial killing by each cytotoxic lymphocyte.

In contrast, **VY-X**:

- Is a novel RNA construct that is highly specific and leads to a significantly increased load of perforin and granzyme B in NK cells and T cells
- Overcomes the obstacles of limited efficacy of adoptive transfer of NK cells and T cells by increasing cytotoxic activity

VY-X Ex Vivo Platform: Increases the Killing Power of Cytotoxic Cells

- **1600-2000x** increase in Perforin
- **800-1100x** increase in Granzyme B
- First known report of a RNA construct leading to such a dramatic difference in the proteomic profile of cytotoxic lymphocytes

VY-X In Vivo: Direct Cancer Therapeutic

Because **VY-X** is a novel RNA construct that is highly specific and leads to a significantly increased load of perforin and granzyme B in NK cells and cytotoxic T cells, it is being advanced as a direct therapeutic.

- Shows significant delay in tumor development compared to active controls in experimental tumor systems
- Safe with no off-target effects or severe adverse events observed
- Believed to improve clinical outcomes when combined with other immunotherapies

IP Protected Technology Platform

- Assets are not encumbered by any external ownership rights, royalty obligations or other restrictions
- Market exclusivity strategy includes patents, trade secrets and planned orphan drug protections
- IP filing: “Enhanced Gene Delivery to Natural Killer Cells, Hematopoietic Stem Cells and Macrophages”
- IP filing: “Novel RNA Construct”

KEY RESEARCH PARTNERSHIPS:



Study Results Expected by Summer 2017

Vycellix and Moffitt Cancer Center Announce a Collaborative Research Study to Evaluate Novel Product Candidates for Improving Adoptive Cell Immunotherapies for Cancer

Studies designed to enhance T cell and NK cell potencies through improved genetic modification and increased cytolytic granules

SARASOTA and TAMPA, Fla. – Feb. 27, 2017 – Vycellix, Inc., a biopharmaceutical company advancing innovative small molecules to enhance cellular therapies, facilitate manufacturing processes and optimize human immune response against disease, today announced that it has entered into a collaborative research arrangement with Moffitt Cancer Center, an NCI Comprehensive Cancer Center, to further validate Vycellix’s novel product candidates, VY-OZ and VY-X. The planned studies aim to evaluate the molecules’ potential as a platform technology capable of enhancing the potency of adoptive cell immunotherapies for cancer.

Pre-clinical studies conducted at Sweden’s Karolinska Institute have demonstrated that Vycellix’s molecules significantly enhance cancer targeting cytotoxic lymphocytes such as cytotoxic T cells and natural killer cells (NK cells). The Moffitt team, led by James J Mulé, Ph.D., Associate Center Director for Translational Science, will conduct additional studies evaluating each molecule’s respective ability to improve genetic modification of cancer immune lymphocytes and increase their killing capacity.

According to Dr. Mulé, “We are intrigued by the potential to make genetic modification processes more efficient and to ‘super-charge’ targeted T cells and NK cells by inducing significantly increased loads of cancer-killing granules. We look forward to evaluating how these molecules can improve a broad array of important cell-based cancer immunotherapies we are advancing at Moffitt.”

Investment Highlights:

- **VY-OZ & VY-X** (*ex vivo*) represent immediate market opportunities as platforms to optimize adoptive immunotherapy by:
 - *enhancing genetic modification*
 - *improving serial killing capacity*
- **VY-X** (*in vivo*) represents a highly-specific anti-cancer agent enhancing T cell/NK cell mediated immune response
- **VY-OZ** (*ex vivo*) represents a genome editing optimization agent to increase gene editing rates for CRISPR-Cas9 systems using viral vectors
- Other market opportunities for pipeline: oncolytic viral vector uses, targeting viral inflammation and veterinary applications
- IP provides long-term market exclusivity protections
- Significant corporate partnering/licensing interest
- World-class scientific leadership



Optimizing Immunotherapies

**To request Confidential slide deck
with supporting data, please contact:**

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