Developing a pipeline of innovative therapeutics and vaccines that deliver on the promise of gene-based medicine

Corporate Overview
January 2017
Statements herein relating to future financial or business performance, conditions or strategies and other financial and business matters, including expectations regarding future revenues and operating expenses, are forward-looking statements within the meaning of the Private Securities Litigation Reform Act. GenVec cautions that these forward-looking statements are subject to numerous assumptions, risks and uncertainties, which change over time. Factors that may cause actual results to differ materially from the results discussed in the forward-looking statements or historical experience include risks and uncertainties, including the failure by GenVec to secure and maintain relationships with collaborators; risks relating to clinical trials; risks relating to the commercialization, if any, of GenVec’s proposed product candidates (such as marketing, regulatory, patent, product liability, supply, competition and other risks); dependence on the efforts of third parties; dependence on intellectual property; and risks that we may lack the financial resources and access to capital to fund our operations. Further information on the factors and risks that could affect GenVec’s business, financial conditions and results of operations are contained in GenVec’s filings with the U.S. Securities and Exchange Commission (SEC), which are available at www.sec.gov. The forward-looking statements speak only as of the date of this presentation, and GenVec assumes no duty to update forward-looking statements.
Biotechnology company leveraging its proprietary AdenoVerse™ platform to develop cutting-edge gene-based medicines

Developing a novel treatment for hemophilia A using PEC (pulmonary endothelial cell) delivery technology

Exploring opportunities in regenerative medicine, oncology, hematology, infectious diseases, and other cell and gene therapies

Validating partnerships demonstrating the power and value of GenVec technology including:
  • Novartis: regenerative medicine gene therapy for hearing loss
  • TheraBiologics: neural stem cell therapy for oncology indications
# GenVec Pipeline

## Program Area and Indication
- **Gene and Cell Therapies**
- **Infectious Disease Vaccines**

## Partner / Collaborator
- **Hearing Loss and Balance Disorders**: Novartis
- **Oncology**: Therabiologics
- **Hemophilia A**: Washington University in St Louis
- **RSV**: Available for partnering
- **HSV-2**: Available for partnering
- **Malaria**: NMRC / NIH-LMIV
- **FMD**: Merial

## Technology Highlights
- **Hearing Loss and Balance Disorders**: Regenerative medicine gene therapy
- **Oncology**: Ex vivo engineered neural stem cells
- **Hemophilia A**: PEC delivery + gene editing
- **RSV**: Prophylactic vaccine
- **HSV-2**: Prophylactic and therapeutic vaccine
- **Malaria**: Discovery of new Malaria vaccines
- **FMD**: DIVA compatible vaccine

## Development Status
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<td>Hearing Loss and Balance Disorders</td>
<td>Novartis</td>
<td>Regenerative medicine gene therapy</td>
<td>First gene therapy for hearing; Phase 1/2 clinical trial ongoing</td>
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<td>2nd generation product to enter the Phase 1 trial in 1H2017</td>
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<td>First U.S. conditionally approved FMD molecular vaccine</td>
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Opportunity in Hemophilia A

- Genetic disorder causing deficient production of blood clotting factor VIII (FVIII)
  - Approximately 18,000 Americans are living with hemophilia A (approximately 1 in 5,000 births)
  - About 60% of patients have severe disease (producing less than 1% of normal FVIII levels)

- Current therapy relies on prophylactic treatment with blood factors and emergency treatment for bleeding events

- Peak annual costs for treatment per patient per year of approximately $364,000, with average costs exceeding $160,000

- High need for curative approaches that can allow patients to live normally at lower lifetime costs
Stages in the development of products for bleeding disorders

**Plasma-derived clotting factors**
Developed in the late 1960s into the 1970s;
Significant safety concerns due to prevalence of contamination from viral pathogens

**Recombinant clotting factors**
Developed in the late 1980s into the 1990s;
Eliminated the safety concerns of plasma-derived products

**Extended half-life clotting factors**
Recent and ongoing development;
Decreased treatment frequency

**Novel therapies and modalities**

_The promise for the future: gene therapy and other new agents_

Notable companies with approved products or products in development in the categories shown above

- **Shire**
  - CSL Behring
  - octapharma®
- **Biogen**
  - **Biomarin**
- **uniQure**
Monogenic disease, with a clear correlation between circulating blood clotting factor levels and severity of disease

Even small increases in blood clotting factor levels from baseline can result in significant clinical improvements

Gene therapy can potentially provide sustained levels of circulating blood clotting factors to restore normal function

Potential to eliminate patient dependence on a lifetime of blood clotting factor injections

While promising, choice of vector and target organ has presented challenges, particularly for hemophilia A
Portfolio of Technologies
Combination Provides Correction of Genetic Disorders

Key technologies

- PEC delivery
- Gene editing
- AdenoVerse™ vectors

Proprietary product design incorporating all the key technologies

Advantages

- Harnessing lung for protein expression
- Therapeutic gene durable expression
- Systemic administration

January 2017
Corporate Overview
PEC Delivery Platform
New Paradigm for Molecular Medicines

- Allows for specific gene therapeutic delivery into the lung endothelium via AdenoVerse™ vectors
- Lung endothelium is turned into a surrogate long-term production factory for therapeutic proteins
- Can be used to address multiple unmet medical needs (hemophilia, passive immunotherapies, etc.)
- PEC delivery combined with AdenoVerse™ vectors can:
  - Limit liver toxicities observed with systemic administration of more traditional gene therapeutics
  - Provide long-term production of native proteins
Step 1: Loading of granulocytes with adenovectors

Step 2: Hand-off of adenovectors to lung endothelial cells

Step 3: Endothelial cell transduction and gene integration

Step 4: Therapeutic protein production and secretion into blood stream
Upon systemic administration in mice, a preferred distribution to the lung was observed.

Technology developed by David Curiel at Washington University in St. Louis.

GenVec has an exclusive option to PEC technology.
Gene Editing Technologies
Tools to Perform Genome surgery

Delivery of the complete gene editing payload to targeted cells

Target cell genome DNA

Precise double-stranded break created

Integration of the therapeutic gene

Durable expression via permanent addition of the therapeutic gene sequence
GenVec’s AdenoVerse platform provides a unique set of solutions for the key Gene Editing field requirements

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<th>Gene Editing Requirements</th>
<th>AdenoVerse Platform Solutions</th>
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| “Hit and run” (limited off-target effects) | Non-integrating vector:  
• Transient nuclease expression minimizing potential off-target cuts  
• Reduced chromosomal positional effects and risk of insertional mutagenesis |
| Large Capacity                  | Delivery of large payload (up to 12Kb):  
• All the components in a single vector  
• Multiple expression cassettes |
| Low Immunogenicity              | • AdenoVerse vectors can be designed to have low immunogenicity properties |
Systemic administration has been a long-standing challenge for gene therapeutics.

GenVec’s AdenoVerse platform addresses this hurdle:

- No-to-low sensitivity to pre-existing neutralizing antibodies
- Reduction of liver sequestration, minimizing hepatotoxicities

![Graph showing titer (IC-90) for GC46, SAV7, and Ad5 serotypes. Novel non-human serotypes vs. Commonly used human serotype.]
Blood consists of cellular components (platelets, red and white blood cells) and plasma

Plasma is a liquid, which contains numerous proteins

Many important proteins are synthetized by the liver

Hepatic proteins are involved in many functions:

- Coagulation (blood factor, fibrinogen, complement, etc.)
- Metabolism (apolipoproteins)
- Carrier (albumin, transferrin, etc.)
- Hormonal (hepcidin, thrombopoietin, etc.)
PEC Technology Applications
Examples of Potential Indications

- **Bleeding disorders**
  - Hemophilia A (FVIII)
  - Hemophilia B (FIX)

- **Metabolic disorders**
  - Type 1 diabetes

- **Respiratory diseases**
  - Alpha 1-antitrypsin deficiency
  - Chronic obstructive pulmonary disease

- **Anemia**
  - Erythropoietin deficiency

- **Passive Immunizations**
  - Anti-pathogen or anti-toxin agents
Select preferred AdenoVerse™ platform vectors for systemic administration

Construct program vectors incorporating PEC delivery technology

Show proof of principle pulmonary endothelial cell delivery in *in vitro* and *in vivo* models

Identify leads for pre-clinical development
AdenoVerse™ Platform
Diverse Biological Properties and Broad Applications

A library of adenoviral vectors with diverse and unique biological properties

- Vectors for eliciting long-lasting adaptive immune responses
- Vectors for immunologically stealth gene delivery

Multiple vectors each suited to a broad range of applications

A large set of therapeutic areas covered by the platform

Proprietary cell lines for efficient manufacturing
New Gene-Based Technologies and Approaches

- Product-focused vector design to deliver payloads to the desired cellular targets

Translational expertise across a spectrum of diseases:
- Manufacturing and clinical development

The AdenoVerse Platform Solutions

AdenoVerse™ Platform
Technology Platform Enabling Therapeutics

Delivery Barriers

INNOVATION

Novel Therapeutic Product Opportunities

VALUE CREATION
AdenoVerse™ Platform

Advantages

Adenovector Advantages

- Efficient transduction in dividing cells and non-dividing cells
- Non-integrating transgene limits the probability of disturbance of vital cellular genes

AdenoVerse Strengths

- Vectors with no or very low seroprevalence in the human population
- Large packaging capability (up to 12 kb) with multiple expression cassettes
- Improved safety with multiple deletions in vector genomes
- Administered to over 3,000 clinical study subjects
- Scalable platform with efficient manufacturing process and attractive cost of goods
- Strong and expanding IP position
AdenoVerse™ Platform
Overcoming the Known Adenovector Limitations

Standard Adenovector Known Limitations

- Pre-existing immunity
- Innate immunity associated toxicity
- Vector liver sequestration

GenVec’s AdenoVerse Adenovector Superiority

- Proprietary adenovector backbones from rare human and non-human primate serotypes with low to no seroprevalence in the human population
- Adenovector deleted for E1, E3, and E4 regions, limiting the expression of viral proteins mainly responsible for innate immune responses
- Some of these adenovectors appear not to be sequestered in the liver and could be suitable for systemic delivery

The AdenoVerse platform is the next generation of adenovector technology and has unprecedented potential for new medical treatments
AdenoVerse™ Platform
Identifying Vectors for New Therapeutic Applications

Data from screenings to identify vectors that efficiently transduce cells of interest

AdenoVerse Vectors

GenVec’s Screening Methodology

Vectors with preferred performance characteristics for any given application and unique need

Vector A

Vector B

Vector C

Vector D

Data shown from human primary T cell screening
AdenoVerse™ Platform
Diverse Biological Properties and Broad Applications

A library of adenoviral vectors with diverse and unique biological properties

- **Vectors for eliciting long-lasting adaptive immune responses**
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**Immuno-therapies**
- Vaccines
  - Prophylactic & Therapeutic

**Oncolytics**
- Gene Editing
- Cell Therapies
- Nucleic Acid Therapeutics

**Key Applications**
- Infectious Disease
- Oncology
- Otology
- Ophthalmology
- Cardiology
- Neurology
- Rheumatology
- Rare Diseases
Validated technology for vaccine applications

Industry leading vaccine platform for generating T cell responses

Proprietary novel adenoviral vectors with outstanding properties

Vectors with distinct advantages for molecular vaccines
  - High-level, durable antibody responses
  - High-level T cell responses
  - Repeat administration boosts responses
CGF166 is currently in a Phase 1/2 clinical trial in patients with severe to profound hearing loss.

Currently recruiting patients for the fourth cohort in ongoing Phase 1/2 clinical study.

Safety and efficacy analysis from first three cohorts supports further dose escalation.

Agreement provides GenVec with up to $206.6 million in milestone payments, in addition to royalties on sales.
Hearing loss is a multi-billion dollar market opportunity

Disabling condition with high and increasing prevalence worldwide

An estimated 1 in 6 adult Americans suffer from hearing loss

90% of hearing loss is sensorineural

No current pharmaceutical treatment options
### Causes of Sensory Cell Loss

- Age Related
- Infection
- Drug Induced
- Sound Trauma
The Solution
Generate New Sensory Cells

Deliver the Atonal gene to the supporting cells using an adenovector

Produce Atonal protein in supporting cells

Trigger conversion of supporting cells into sensory cells
Regeneration of Sensory Cells

Damaged and Untreated

Major loss of sensory cells

Damaged and Treated

New sensory cells


Restoration of Hearing

Multicenter trial of 26 to 45 patients with severe to profound hearing loss

**Part A: Safety**
- Single, 3-patient cohort
- Dose: 20 µl

**Part B: Dose Volume Escalation**
- 2-5 cohorts of 3 patients each
- Dose volume between 30 µl and 90 µl

**Part C: Efficacy**
- Single cohort of 20 patients at dose determined by Part B
- Option to resize
Second generation adenovector-engineered neural stem cell (NSC) product for the treatment of primary and metastatic tumors

NSCs selectively target tumor cells and vasculature

TheraBiologics emerged out of the pioneering neural stem cell research and development of Dr. Karen Aboody at City of Hope

NSCs penetrate main tumor mass

NSCs selectively target invasive tumor cells

NSCs target new blood vessels
Two unique and synergistic biological properties make neural stems cells an ideal vehicle for targeted cancer treatment

**Homing: “GPS-like” tumor site locator**
- NSCs navigate towards invasive tumor sites in various tissues, including the brain, post systemic administration
- NSCs exploit tumor angiogenesis as a homing mechanism to locate tumor and metastatic sites

**Trojan Horse: Stealth payload delivery**
- NSCs can be genetically modified
- NSCs can carry therapeutic payloads to tumor sites, shielded within the NSC carrier
- NSCs retain the tumor homing function upon genetic modification and payload hauling
NSCs are transduced by a proprietary GenVec adenovector to express and secrete carboxylesterase (CE) = NSC.CE

When administered, these NSCs migrate to tumor sites where they express the CE therapeutic payload within the tumor

The secreted CE enzyme then provides a radius of action throughout the tumor site to catalyze the conversion of irinotecan to SN-38

- SN-38 is 1,000 times more toxic to tumor cells than irinotecan
NSC.CE Product
Pre-clinical Efficacy in a Neuroblastoma Model

TheraBiologics Data

Days After Neuroblastoma Injection

Percent Survival

0 25 50 75 100

0 100 200 300 400

Untreated
Irinotecan only
NSC.CE + Irinotecan
First indication: Recurrent high grade glioma
- Phase 1 clinical trial ongoing currently using a first generation product
- Plan to switch to the second generation collaboration product employing GenVec’s adenovector in 1H2017
- The Phase 1 clinical development is fully funded by grants

Second indication: Metastatic neuroblastoma
- Systemic administration of the product
- Preclinical work ongoing using the second generation product
- Preclinical development is fully grant-funded

GenVec and TheraBiologics are working closely together on:
- Process development for the production of this novel cell therapy
- Exploring additional grant funding opportunities to support the program
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<sup>1</sup>As of December 1, 2016; <sup>2</sup>Stock price as of January 17, 2017; <sup>3</sup>As of September 30, 2016
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