GENVEC

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

Corporate Overview

November 2015

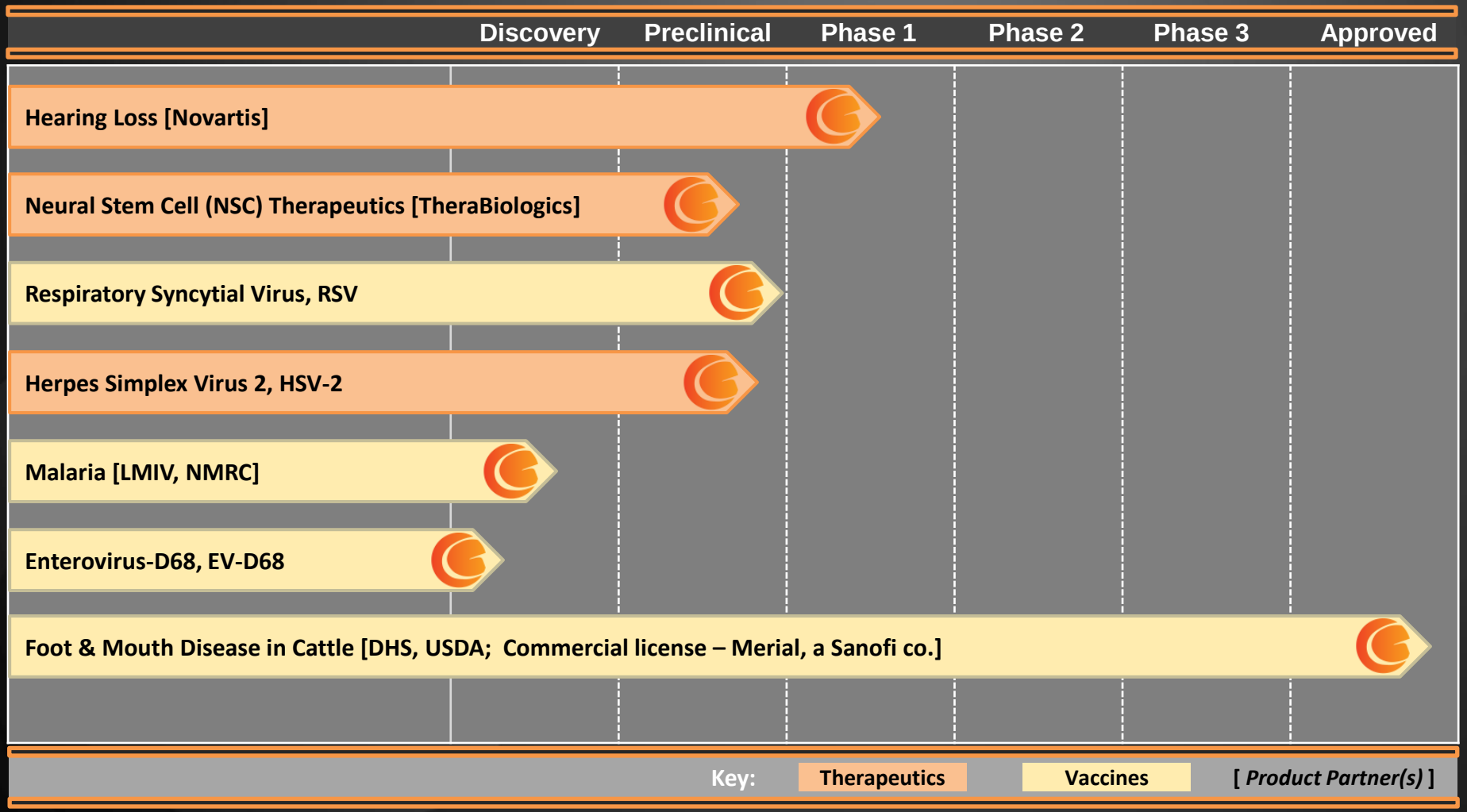
Safe Harbor Statement

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.

Investment Highlights

- Pioneering biotechnology company focused on leveraging its proprietary AdenoVerse™ gene delivery platform to develop a pipeline of cutting-edge therapeutics and vaccines
- Clinical stage hearing loss program partnered with Novartis
- GenVec therapeutics and vaccines safely administered to over 3,000 clinical study subjects
- AdenoVerse™ platform provides multiple opportunities for product development and licensing, and is supported by strong IP

GenVec Pipeline

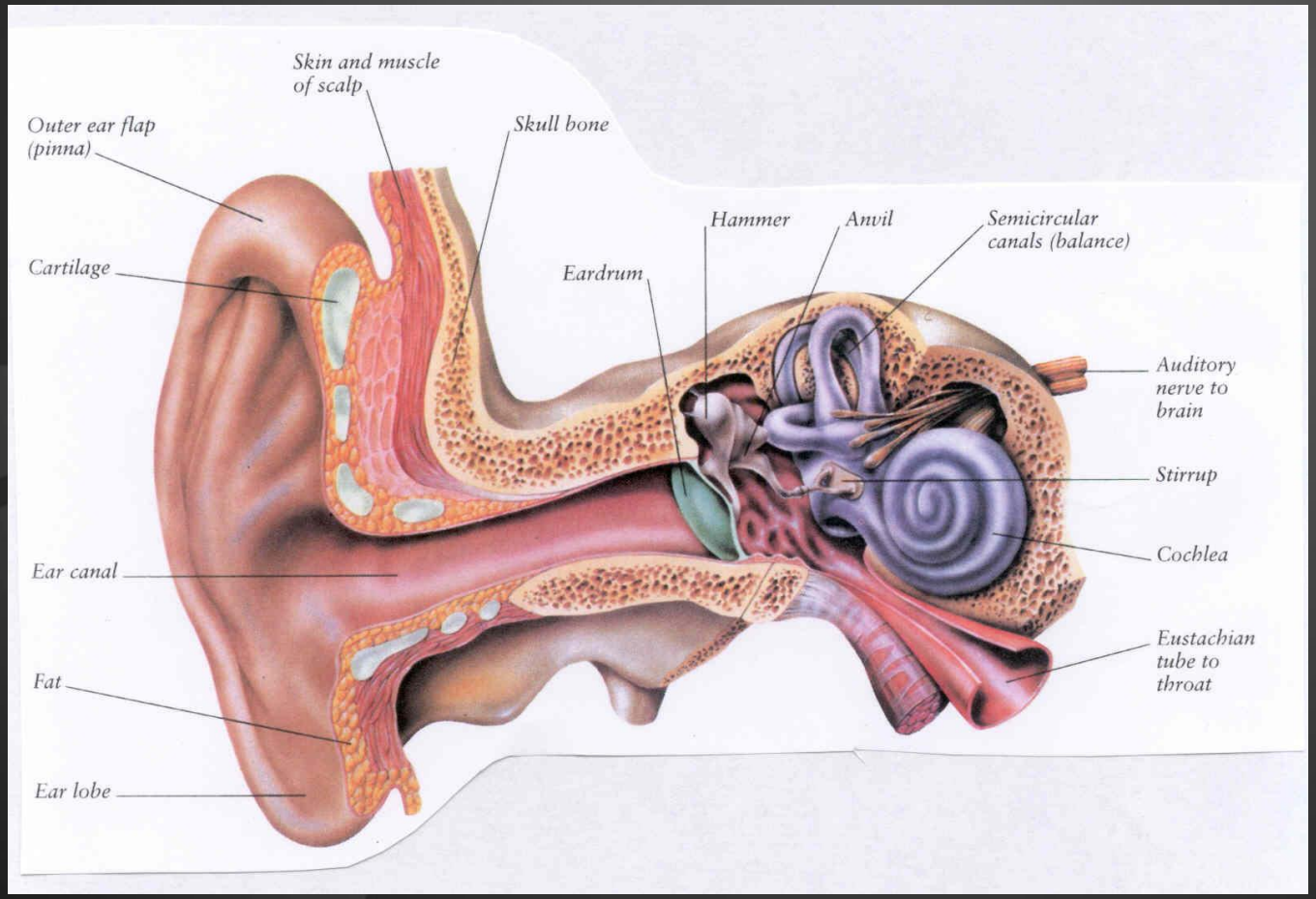


Hearing and Balance Program

The Opportunity

- Hearing loss is a multi-billion dollar market opportunity
- Disabling conditions 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

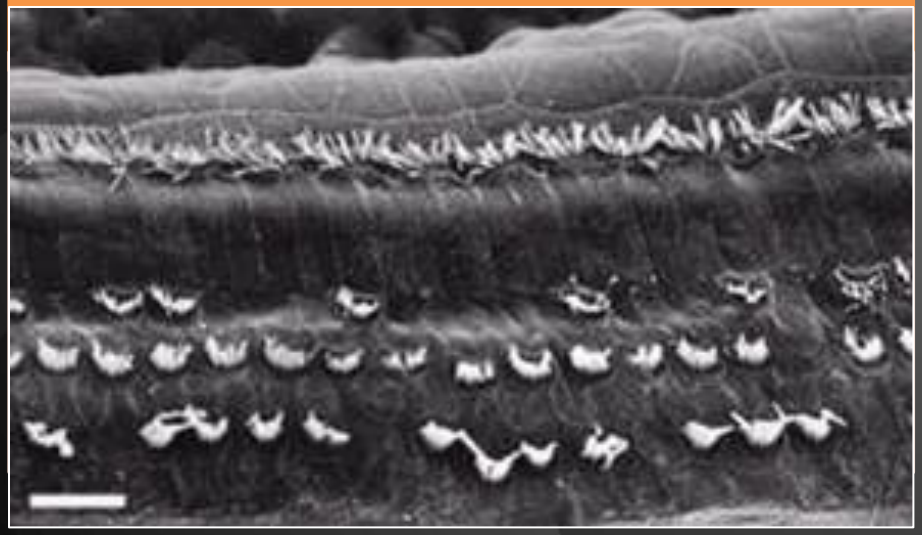
How We Hear


Skin and muscle of scalp
Outer ear flap (pinna)
Skull bone
Cartilage
Hammer
Anvil
Semicircular canals (balance)
Eardrum
Auditory nerve to brain
Stirrup
Cochlea
Ear canal
Eustachian tube to throat
Fat
Ear lobe

The Problem

Loss of Sensory Cells

Sensory Cells



Causes of Sensory Cell Loss

- Age Related
- Infection
- Drug Induced
- Sound Trauma

The Solution

Generate New Sensory Cells

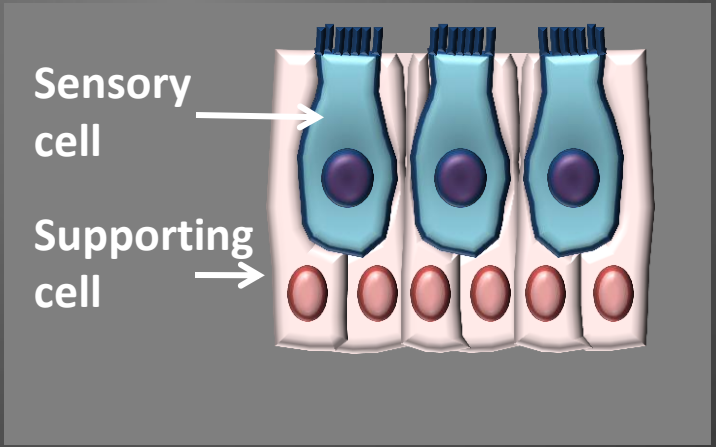
Deliver the Atonal gene to the supporting cells using an adenovector

↓

Produce Atonal protein in supporting cells

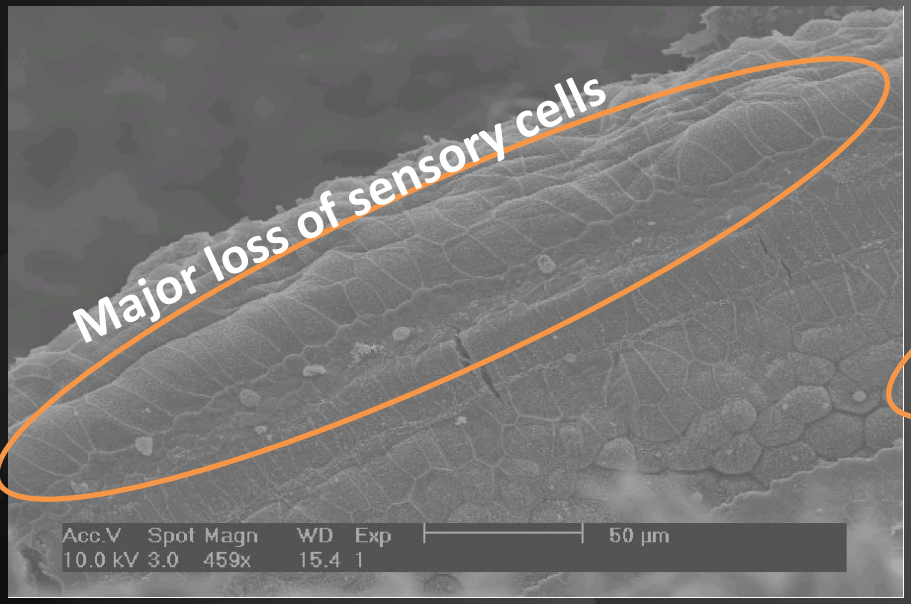
↓

Trigger conversion of supporting cells into sensory cells

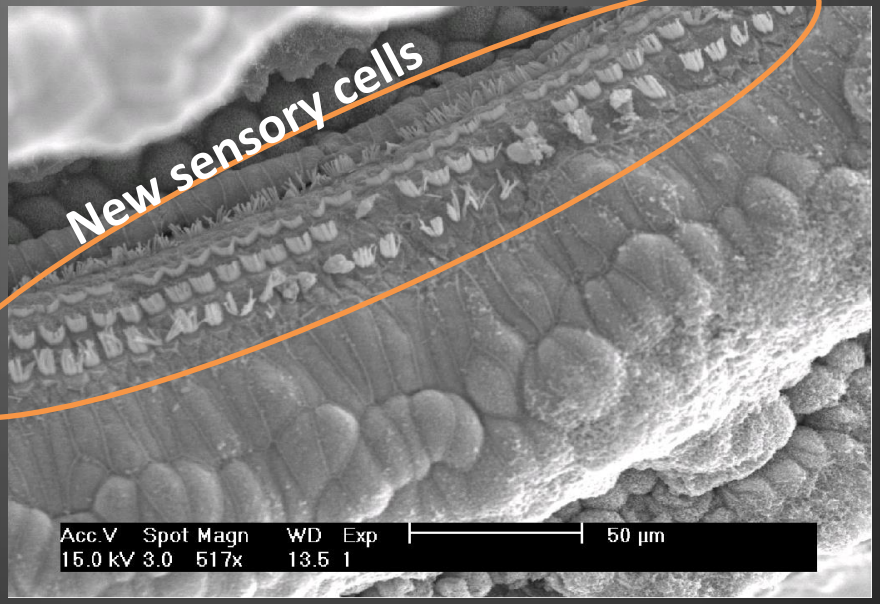


Regeneration of Sensory Cells

Damaged and Untreated

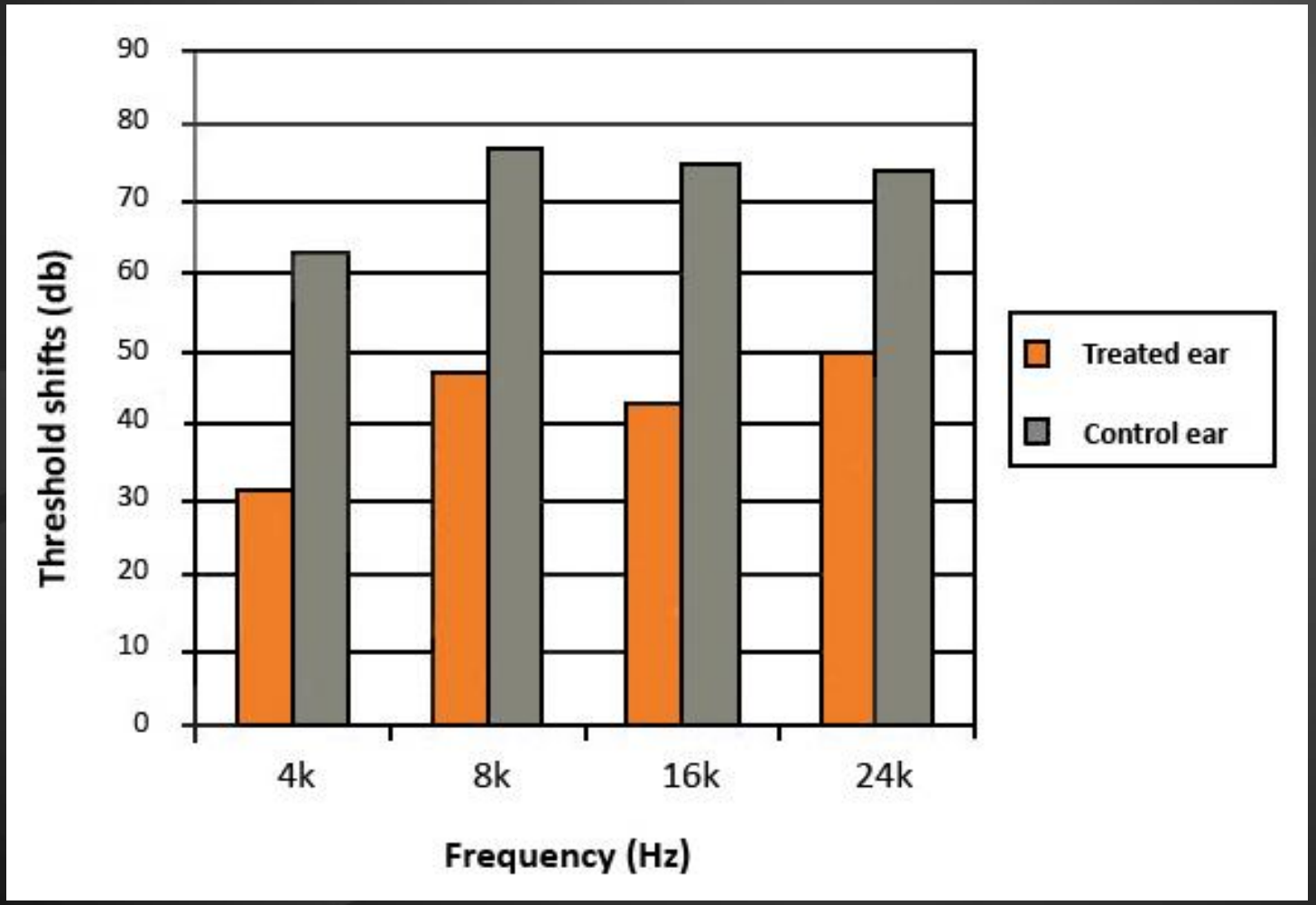


Damaged and Treated



Data taken from Izumikawa *et al*. study, *Nature Medicine*, 2005, 11(3): 271-276

Restoration of Hearing



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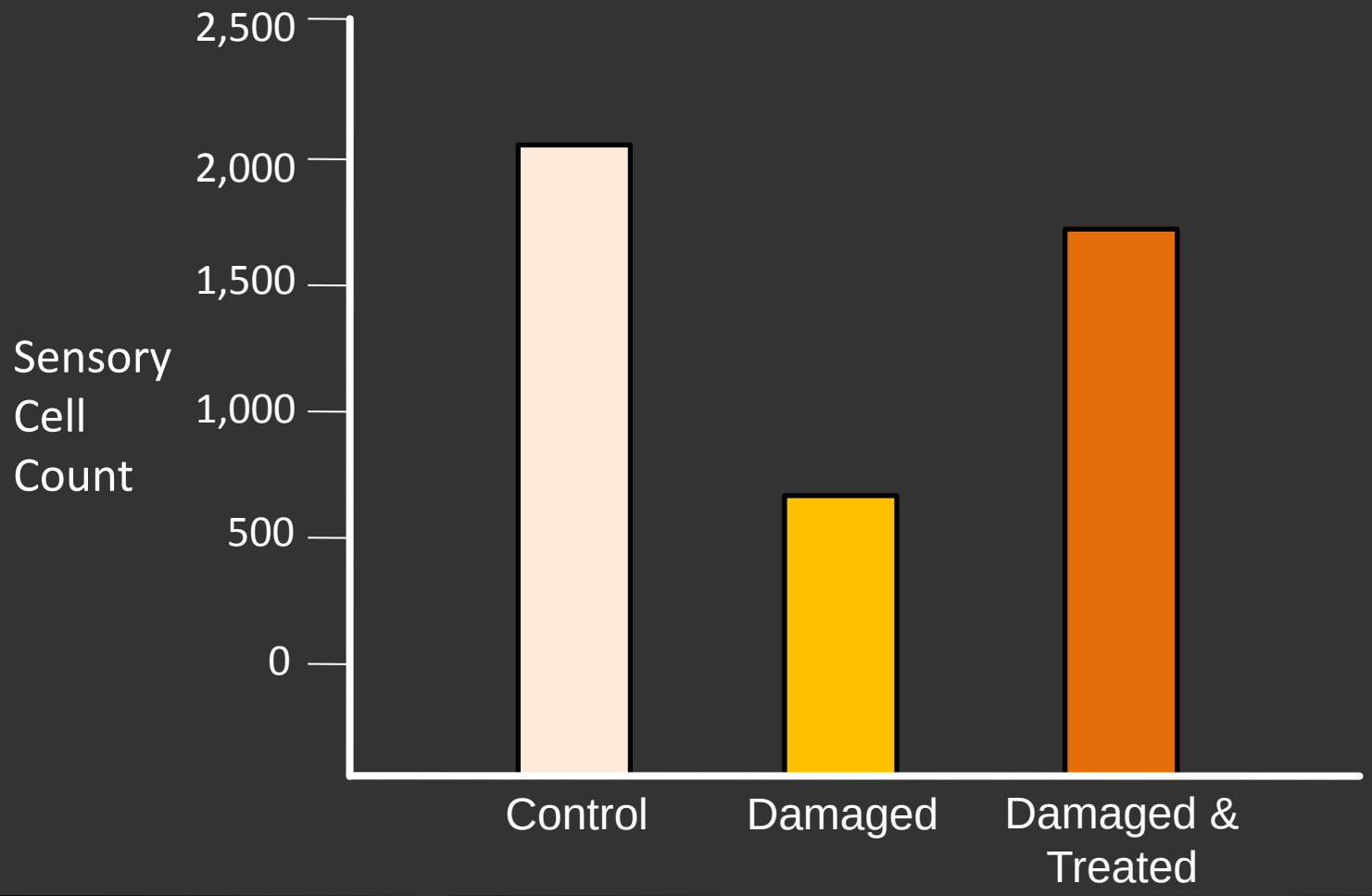
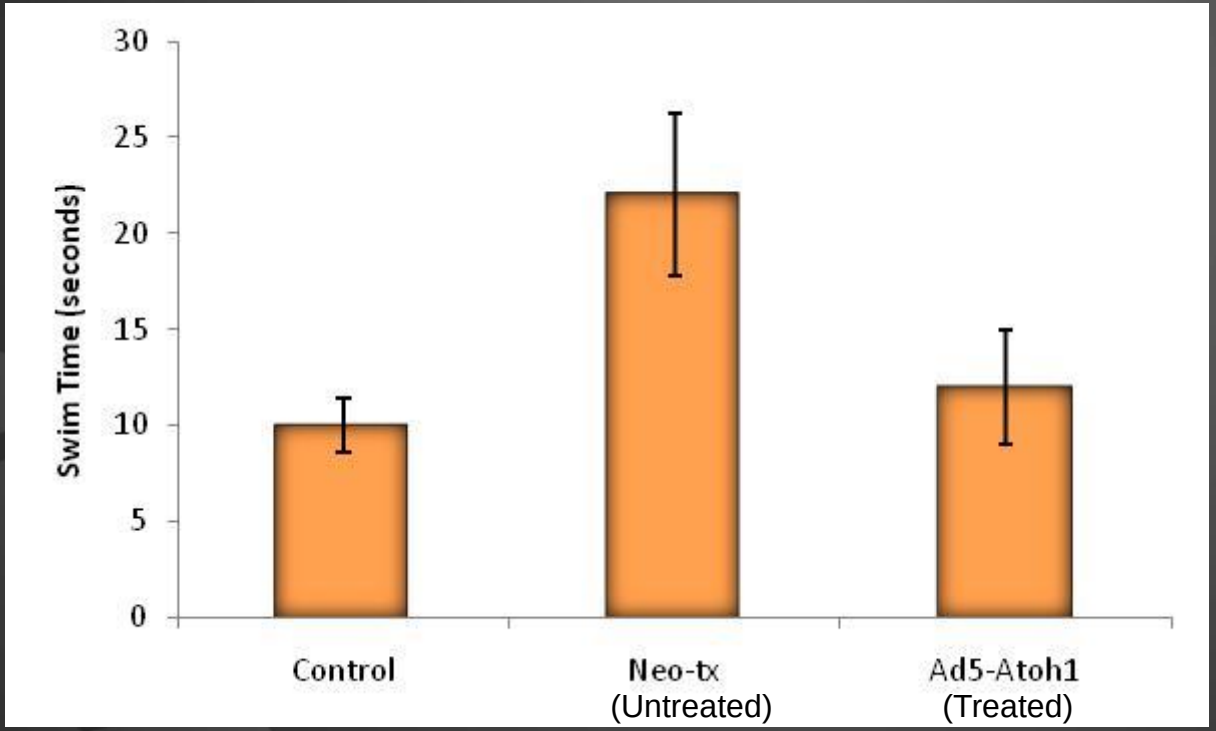
Regeneration of Sensory Cells



Schlecker et al. Gene Therapy, 2011, 18: 884-890

Restoration of Balance

Mice improved swim test scores 8 weeks after Atonal gene delivered

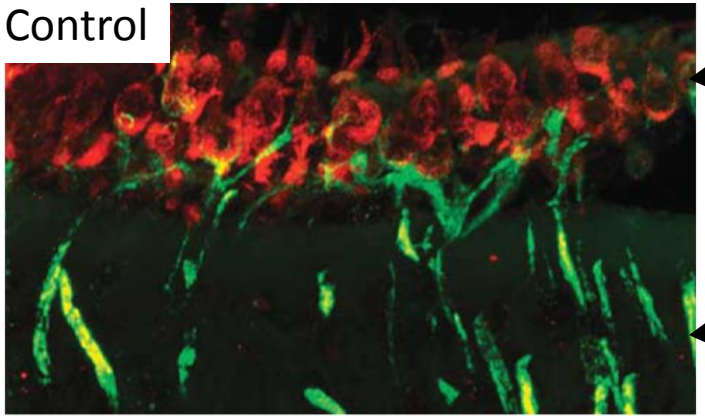


Baker *et al.* 2009. *Adv. Otorhinolaryngol.* 66: 52-63

Sensory Cell Innervation

Regenerated Sensory Cells Attract Neuronal Outgrowths

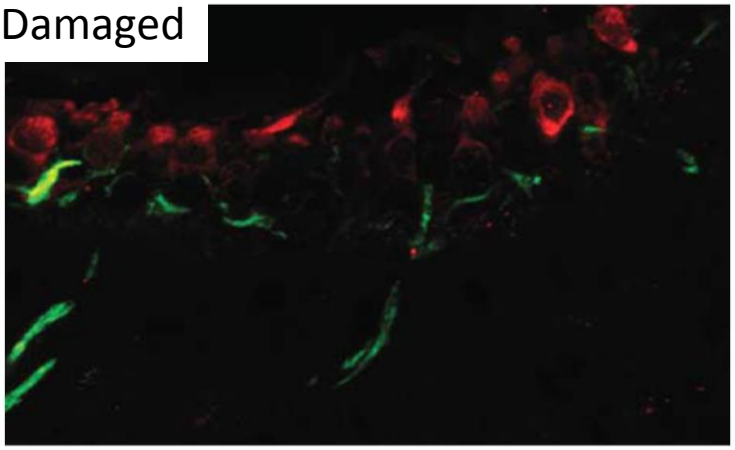
Control



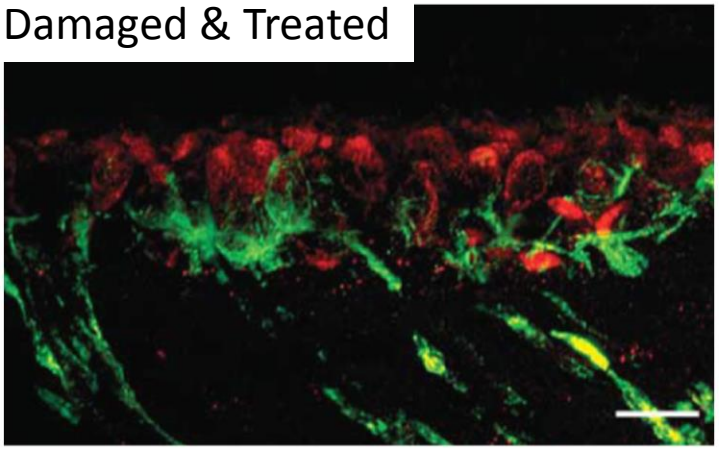
Sensory Cells (red)

Neurons (green)

Damaged



Damaged & Treated



Schlecker et al. Gene Therapy, 2011, 18: 884-890

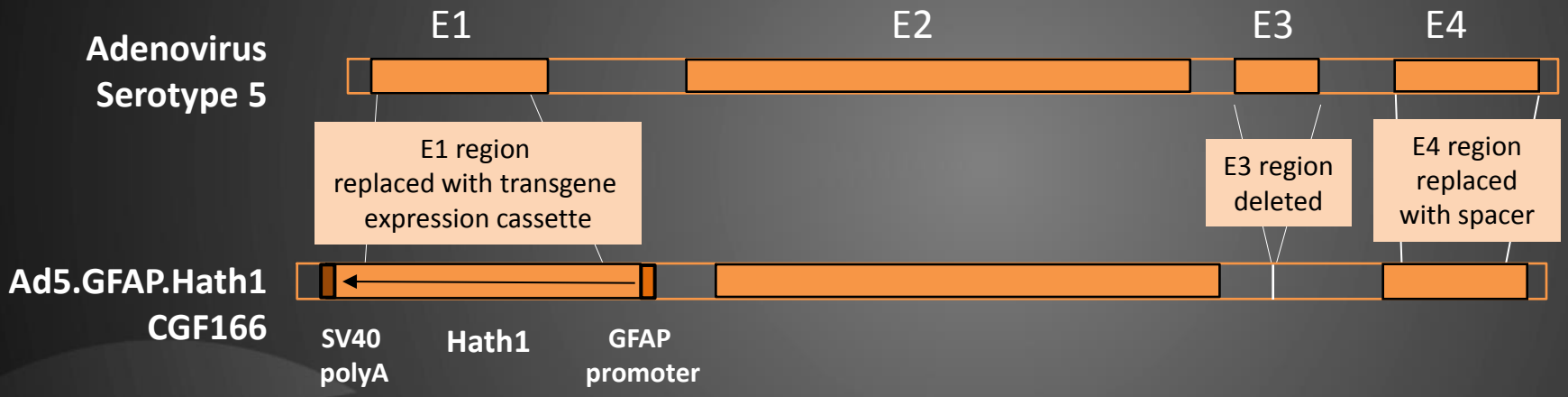
Novartis Collaboration

Overview

- Novartis responsible for worldwide development and commercialization
- Up to $206.6 million in milestone payments ($5.6 million realized to date)
- Royalties on sales
- GenVec qualified by Novartis to support program

Clinical Candidate (CGF166)

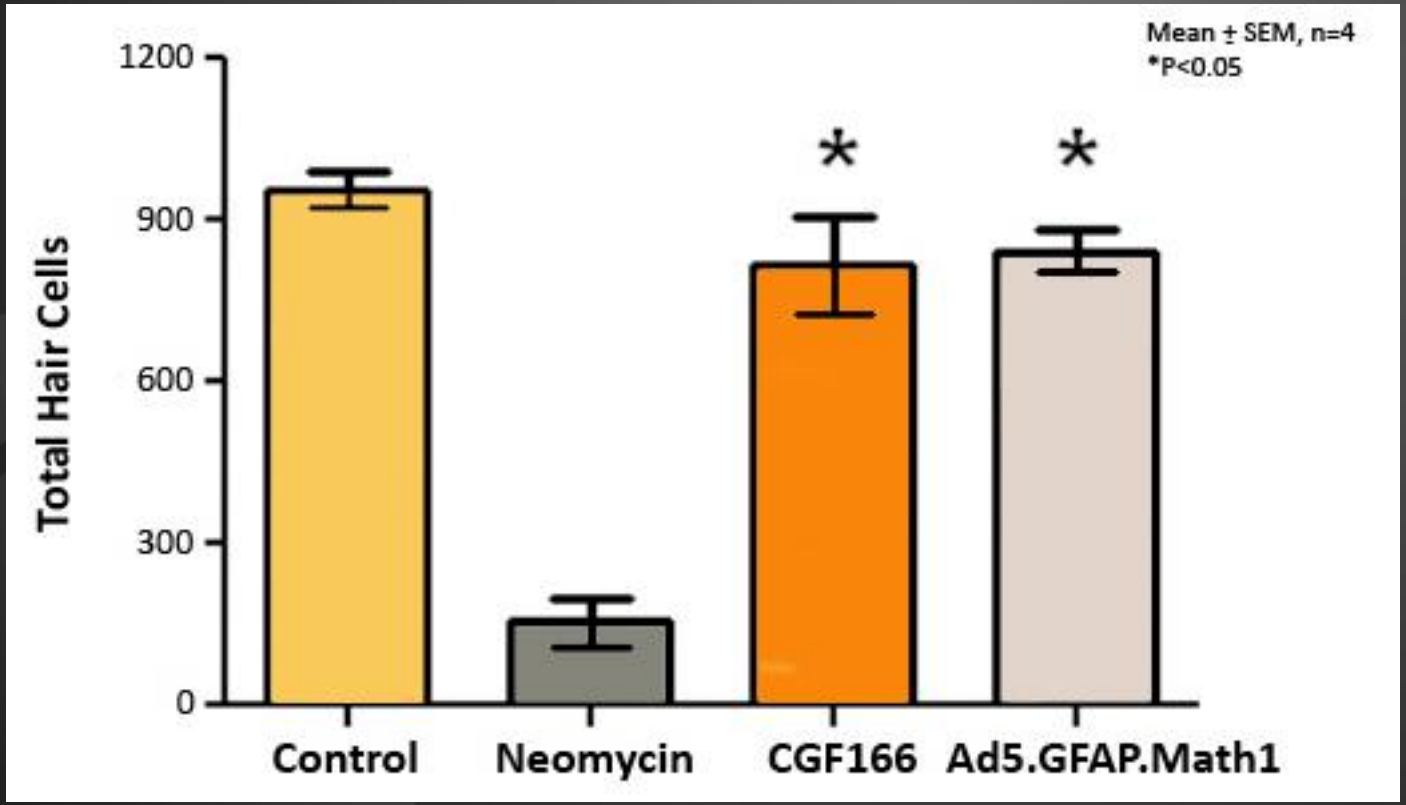
Vector Schematic



- CGF166 leverages GenVec's AdenoVerse™ Platform:
  - Vector backbone safely administered to over 3,000 subjects
  - E1, E3, E4-deleted adenovirus serotype 5 (Ad5) vector
  - Replication-incompetent, non-integrating vector
  - Engineered to deliver the human atonal transgene expressed under the control of a cell specific promoter to restrict expression to inner ear supporting cells

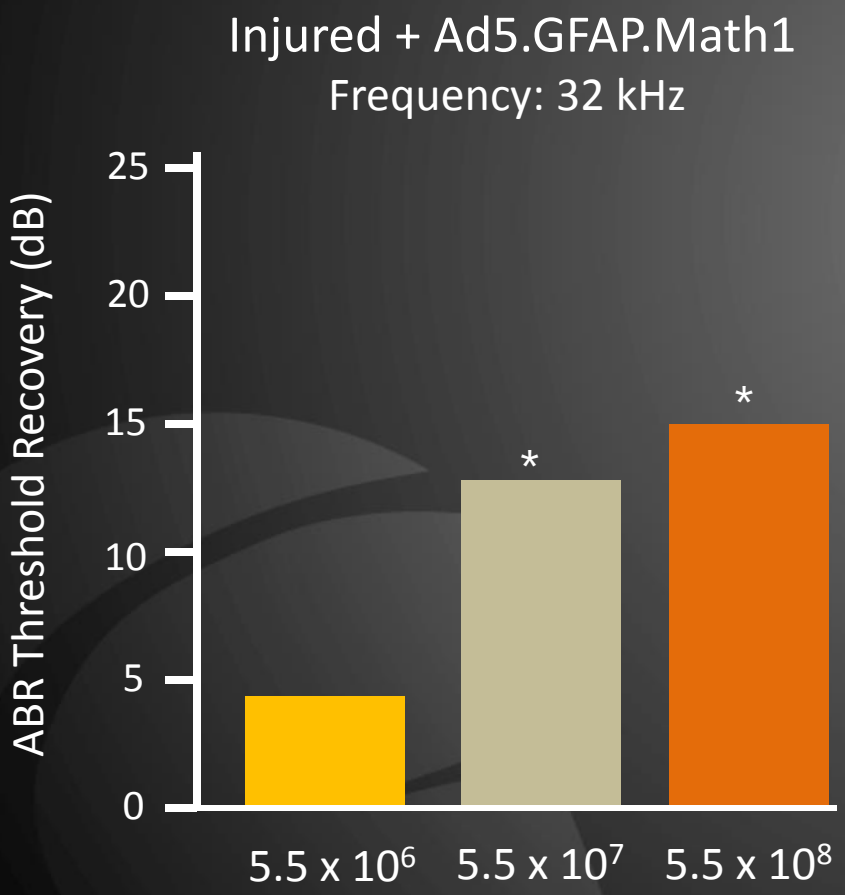
CGF166 Preclinical Testing

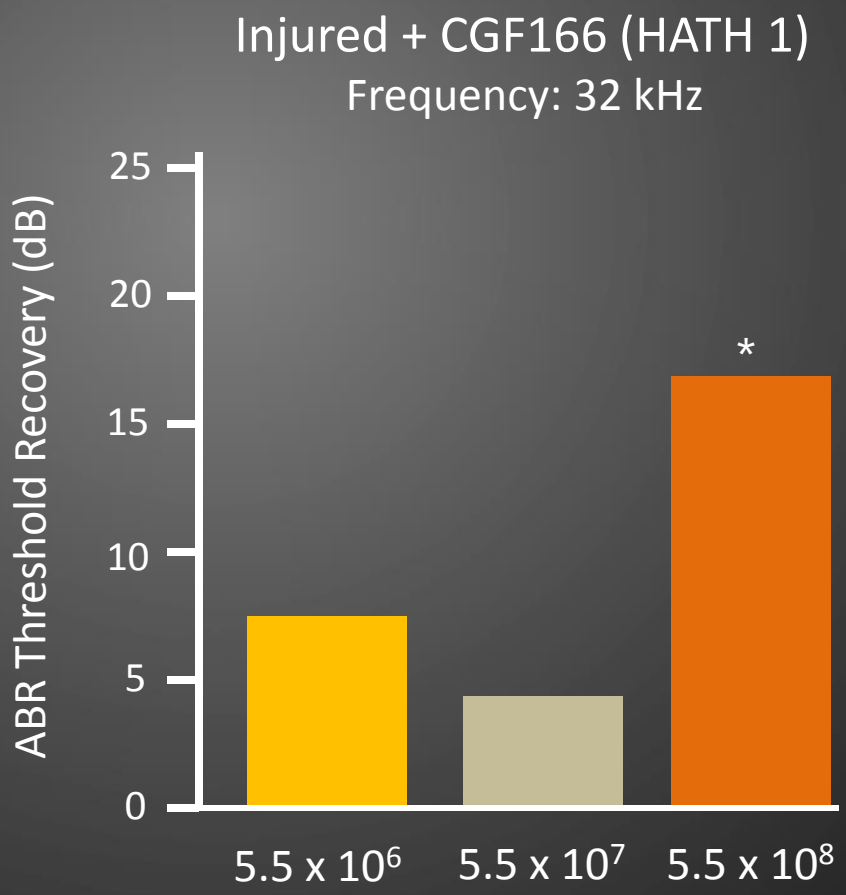
Sensory Cell Regeneration Data



CGF166 Preclinical Testing

Hearing Functional Restoration





Mean ± SEM; *P<0.05, n = ~15

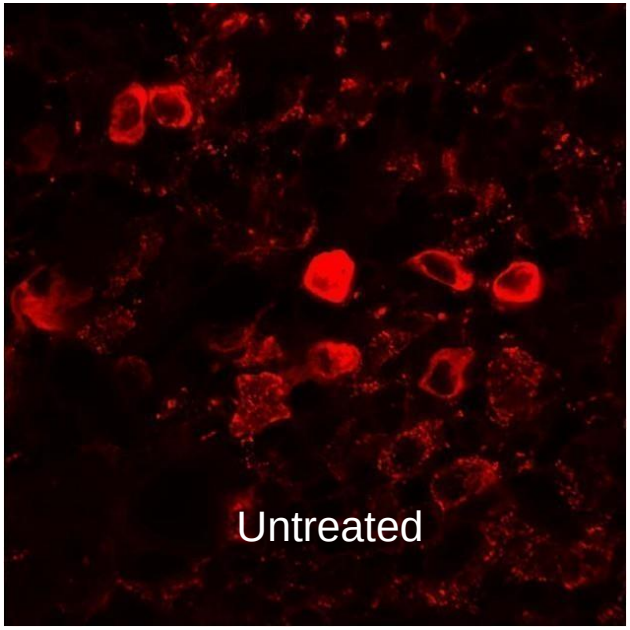
CGF166 Preclinical Testing

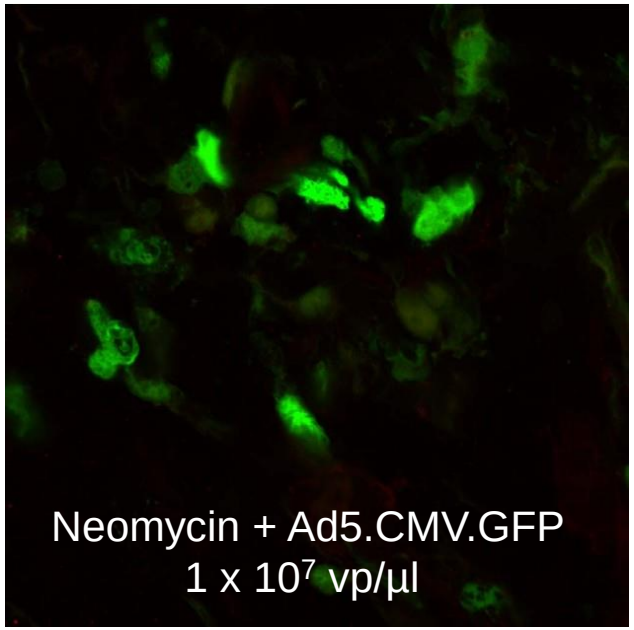
Sensory Cell Regeneration in Human Tissue

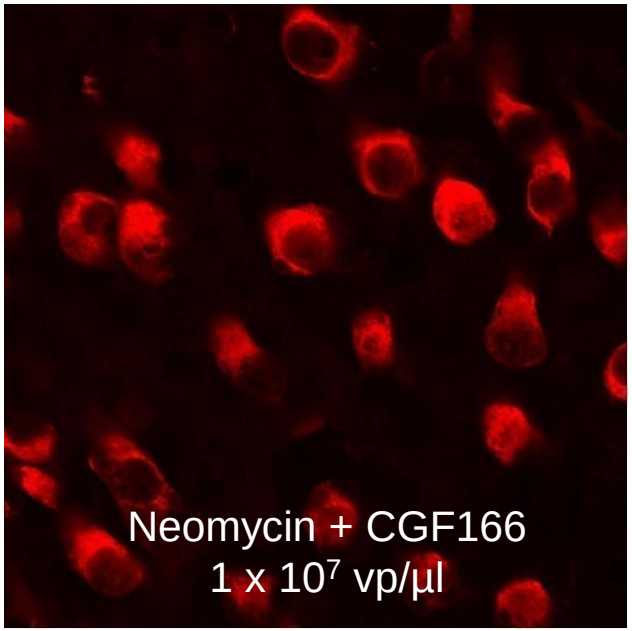
Untreated tissue - human inner ear, schwannoma patient

Post Ad5.CMV.GFP *– green marker shows transduced supporting cells, no sensory hair cells evident*

Post CGF166 *- red Anti-myosin VIIa hair cell marker shows* ***regenerated sensory hair cells in human tissue***







Clinical Plan Overview

Trial Design

- 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

Clinical Endpoints

Primary Endpoints	Secondary Endpoints	Exploratory Endpoints
• Change from baseline in pure tone audiometry measured at frequencies between 0.5 and 16 kHz	• Speech recognition • Balance function measurements	• Jacobson Dizziness Handicap Inventory • Hearing Handicap Inventory for Adults • Tinnitus Reaction Questionnaire

Efficacy Criteria

dB Level	Noise Source
10	Normal breathing
20	Rustling leaves
30	Quiet conversation
50	Normal conversation
60	Loud television
80	Noisy office
100	Loud car horn
120	Jet plane take-off (100 feet)
130	Threshold of pain

Change in dB Level	Impact
1 dB	Generally not perceptible
3 dB	Just barely perceptible
5 dB	Clearly noticeable
10 dB	Twice or ½ as loud
20 dB	Four times or ¼ as loud

- The minimum criteria for efficacy are an "inversion" of established criteria for ototoxicity
- A 10 dB change is considered meaningful

Key Inclusion/Exclusion Criteria

Key Inclusion Criteria

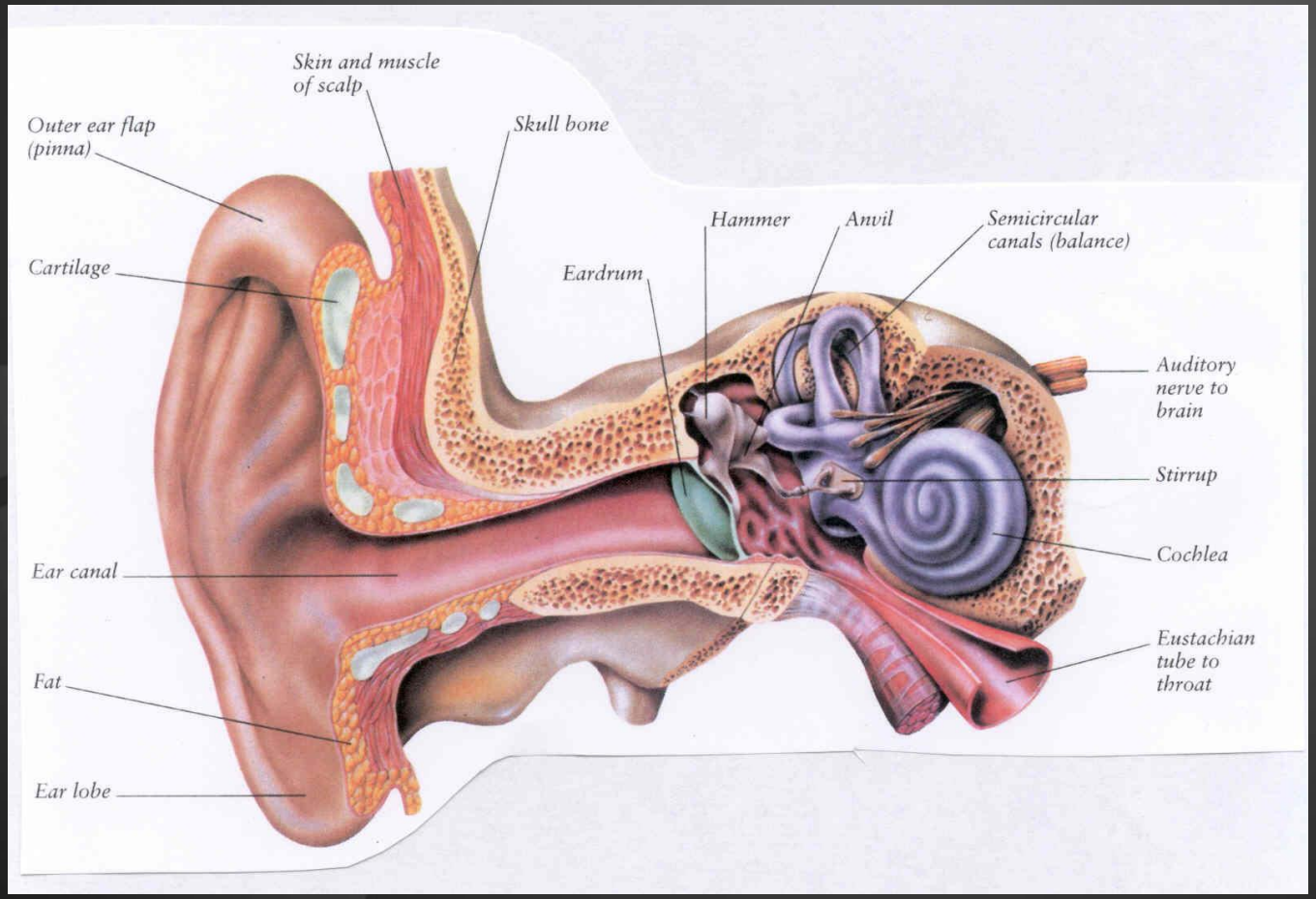
- Patients 18-75 years old with acquired bilateral hearing loss
- Minimal residual hearing (< 110 dB)
- Stable pure tone thresholds for at least 6 months
- Pure tone threshold > 50 dB for lower frequencies and > 70 dB for higher frequencies
- Patients eligible for general anesthesia and surgery

Key Exclusion Criteria

- Known genetic disease associated with hearing loss
- Known cause of hearing loss that is associated with structural damage
- Unilateral vestibular dysfunction in the non-treatment ear

Drug Delivery

Intra-Labyrinthine (IL)



CGF166 Delivery System

Drug Product

Infusion Device

Infusion Pump

CGF166 Overview

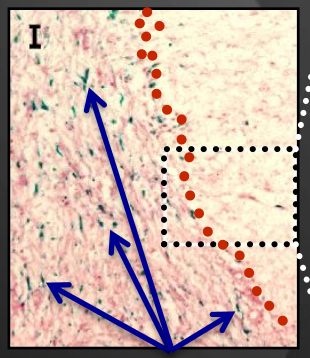
A Novel Therapy for Hearing Loss

- Demonstrated ability to deliver material to cochlea and vestibular system
- Demonstrated efficacy in various models (explants and *in vivo*)
- Ongoing Phase 1/2 clinical study designed to evaluate safety and efficacy

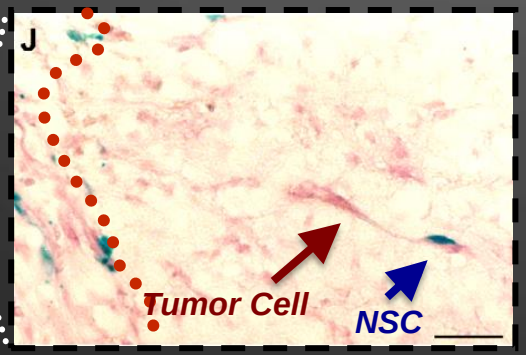
TheraBiologics Collaboration

Neural Stem Cell Mediated Cancer Therapy

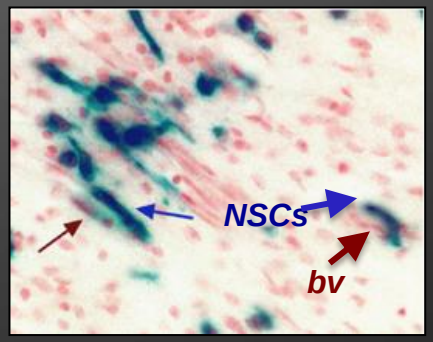
- Benefits of Neural Stem Cells (NSCs):
  - Inherent ability to track down cancer cells
  - Able to cross blood brain barrier
  - Able to penetrate tumor mass
  - Provide an efficient vehicle to deliver a therapeutic payload within the tumor and metastatic sites



NSCs penetrate main tumor mass



NSCs selectively target invasive tumor cells

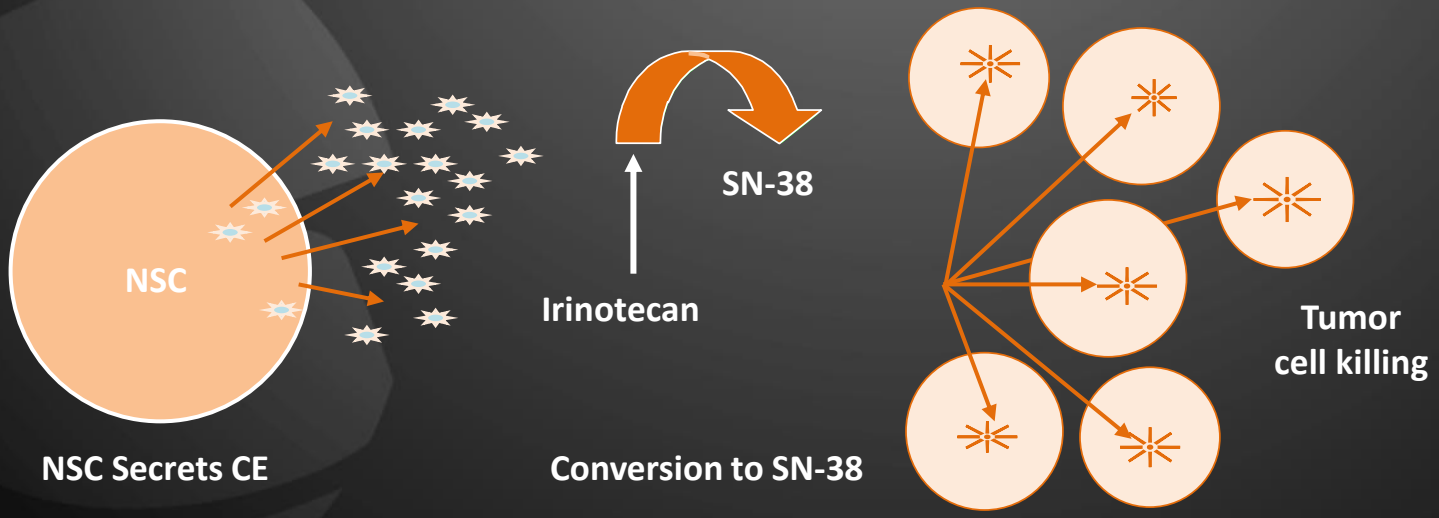


NSCs target new blood vessels

TheraBiologics Collaboration

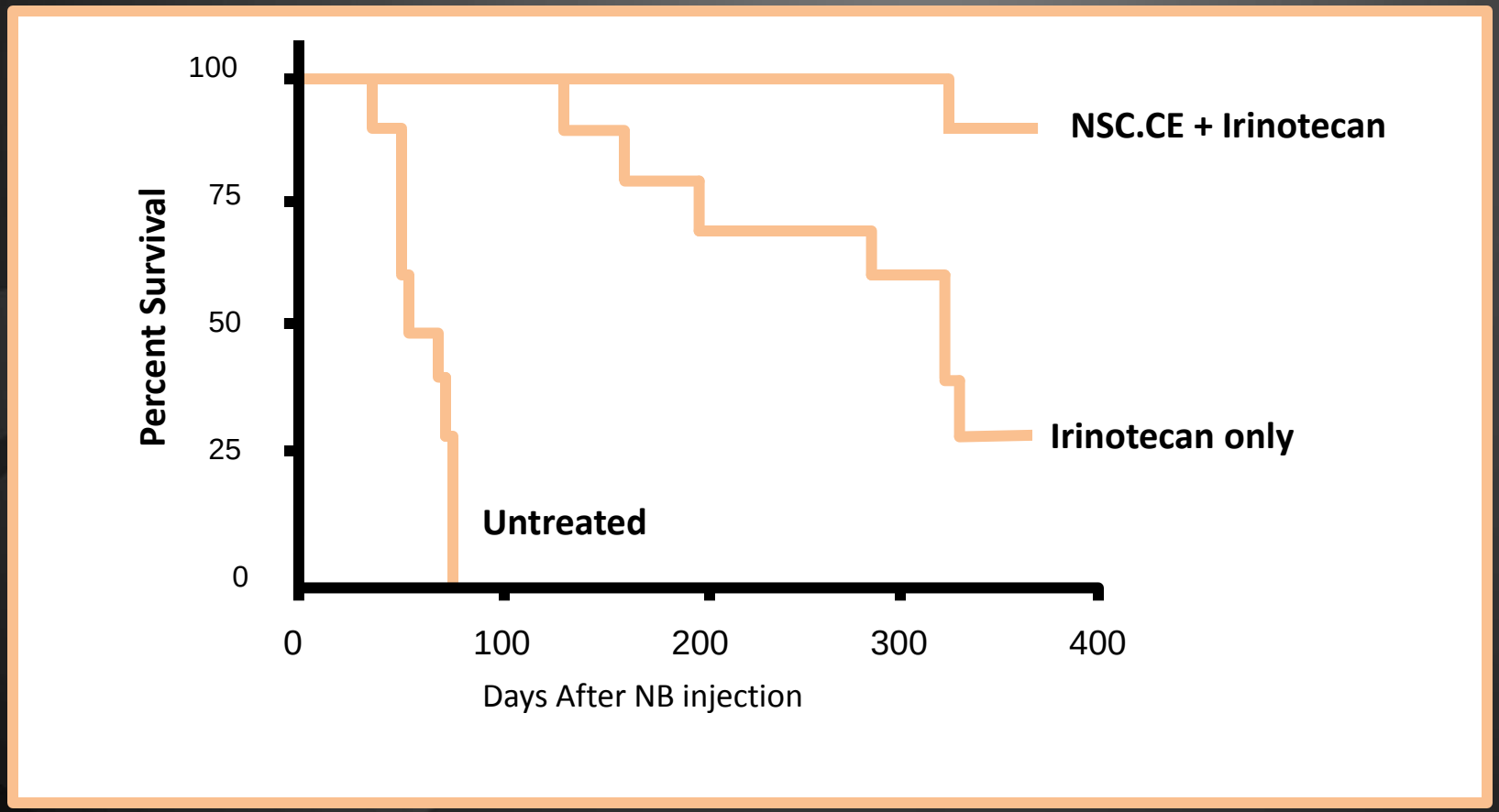
Neural Stem Cell CE Cancer Therapy

- NSCs transduced by proprietary GenVec adenovector express and secrete carboxylesterase (CE) within the tumor
- Secreted CE enzyme provides radius of action throughout the tumor
- CE converts irinotecan to SN-38, which is 1,000x more toxic to tumor cells than irinotecan alone



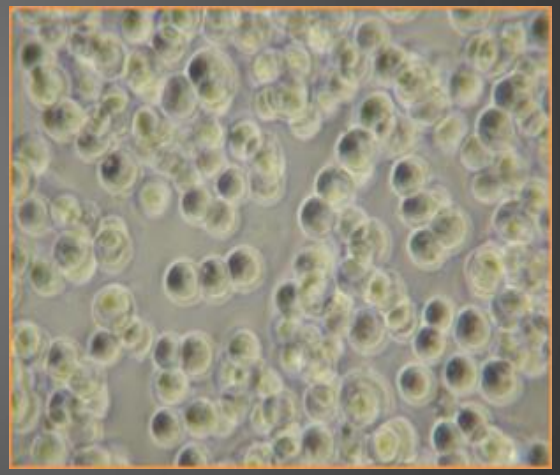
TheraBiologics Collaboration

Pre-clinical Efficacy in Neuroblastoma Model



Adenovector Advantages

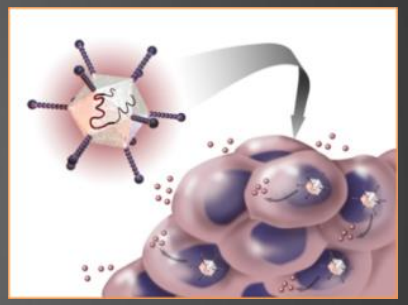
- Excellent safety profile facilitated by additional viral genome deletions
- Efficient transduction in dividing and non-dividing cells
- Low probability of disturbance of vital cellular genes due to the non-integrating transgene
- Grow in high titers to support product manufacturing and commercialization



AdenoVerse™

GenVec's Proprietary Technology Platform

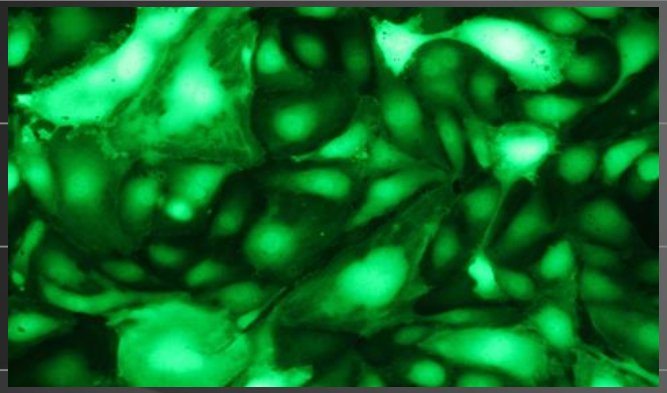
- GenVec's adenovectors have superior performance characteristics for therapeutics and vaccines:
  - Vectors with no or very low seroprevalence in the human population
  - Featuring Tunable Tropism technology to allow cell-type specific gene delivery
  - Large packaging capacity (>12 kb) with multiple expression cassettes



- GenVec's capabilities to support vector development and manufacturing:
  - Proprietary vector construction methodology
  - Extensive cGMP manufacturing expertise
  - Proprietary packaging cell lines supported by FDA Master File

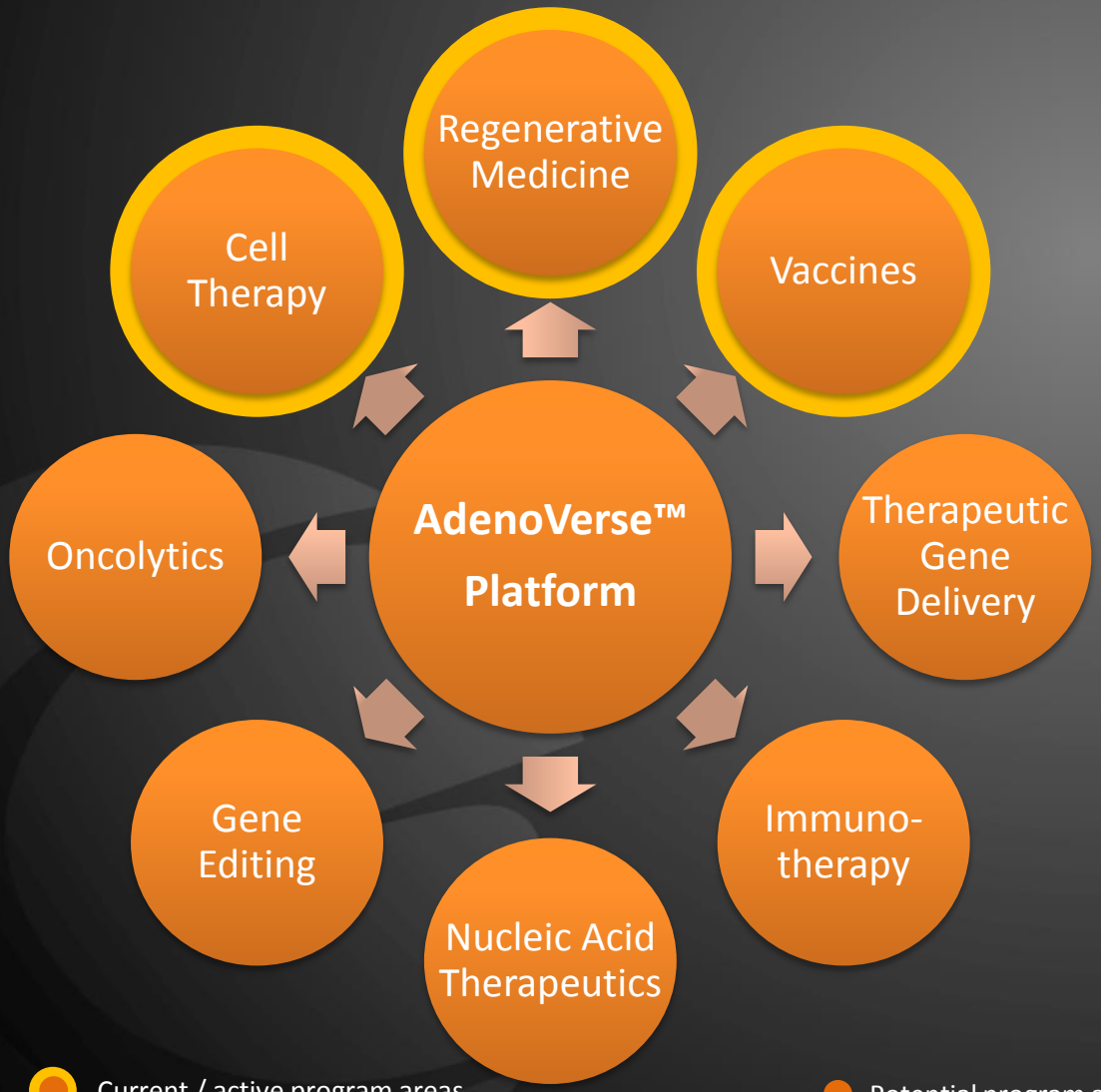
Enhanced Vector Performance

Transduction of Cells in Culture



GenVec Technology

Applications of the AdenoVerse™ Platform



Pioneering the value and versatility of adenovector gene delivery solutions for

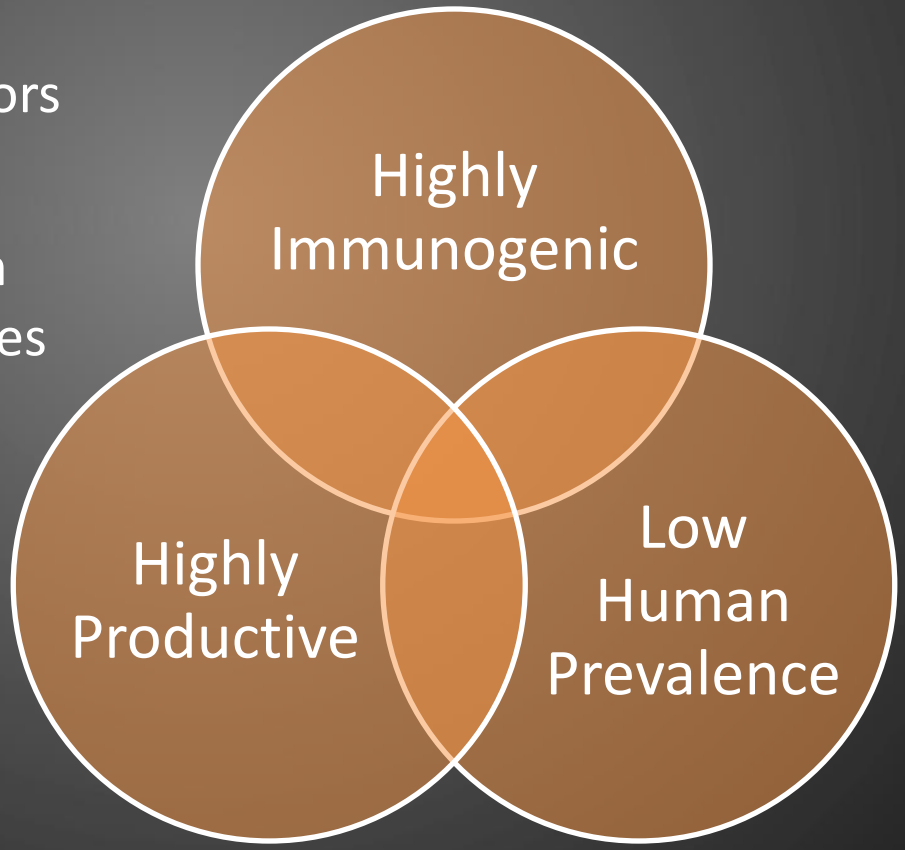
A broad spectrum of applications across indications and therapeutic areas

GENVEC

Non-Human GC Vectors

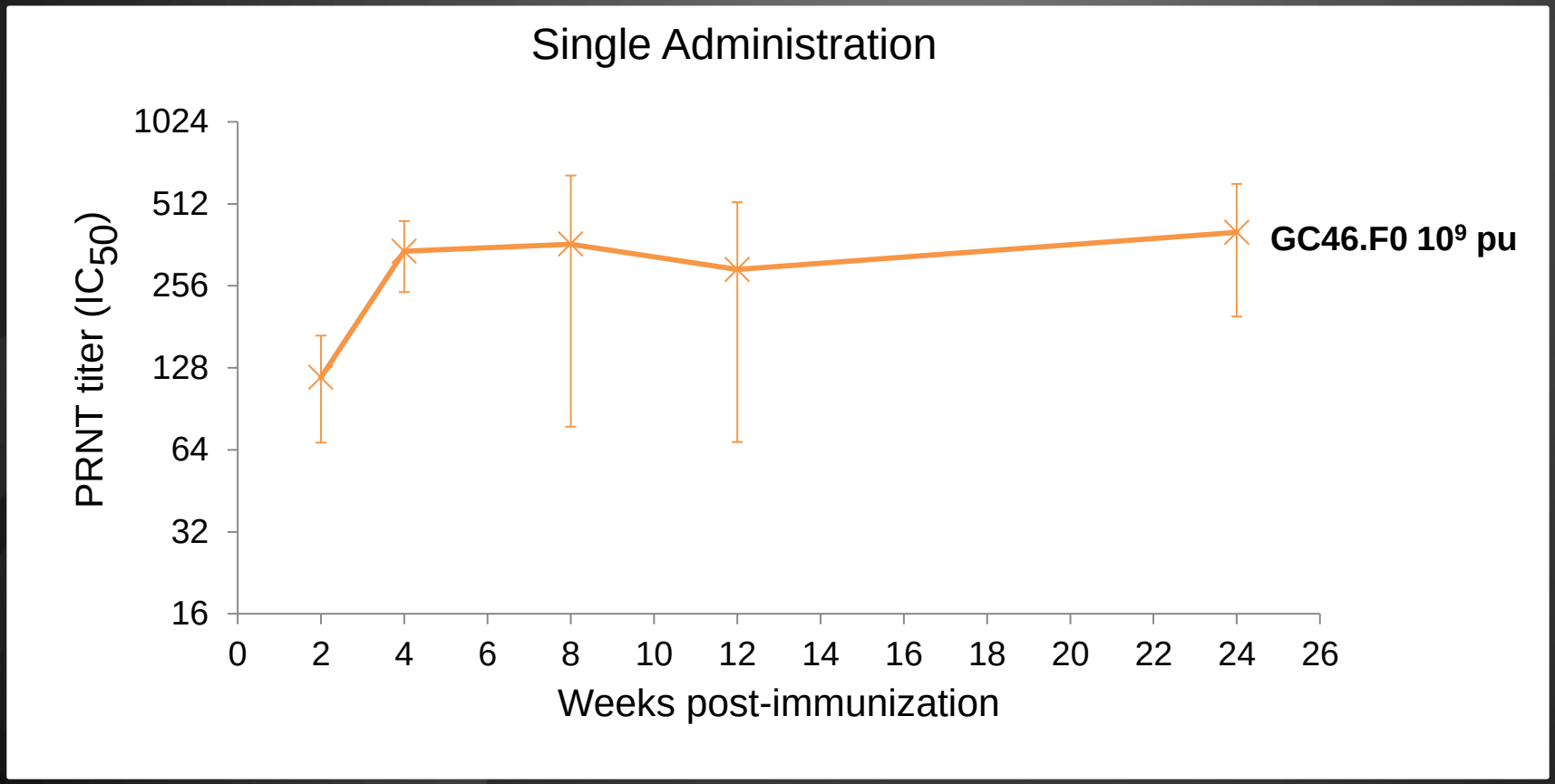
Proprietary Vectors, Excellent Performance

- Discovered and developed proprietary novel adenoviral vectors with outstanding properties
- Productive pre-IND meetings with the FDA for two product candidates
- GC vectors include gorilla vectors with distinct advantages for molecular vaccines
  - High-level, durable antibody responses
  - High-level T cell responses
  - Repeat administration boosts responses



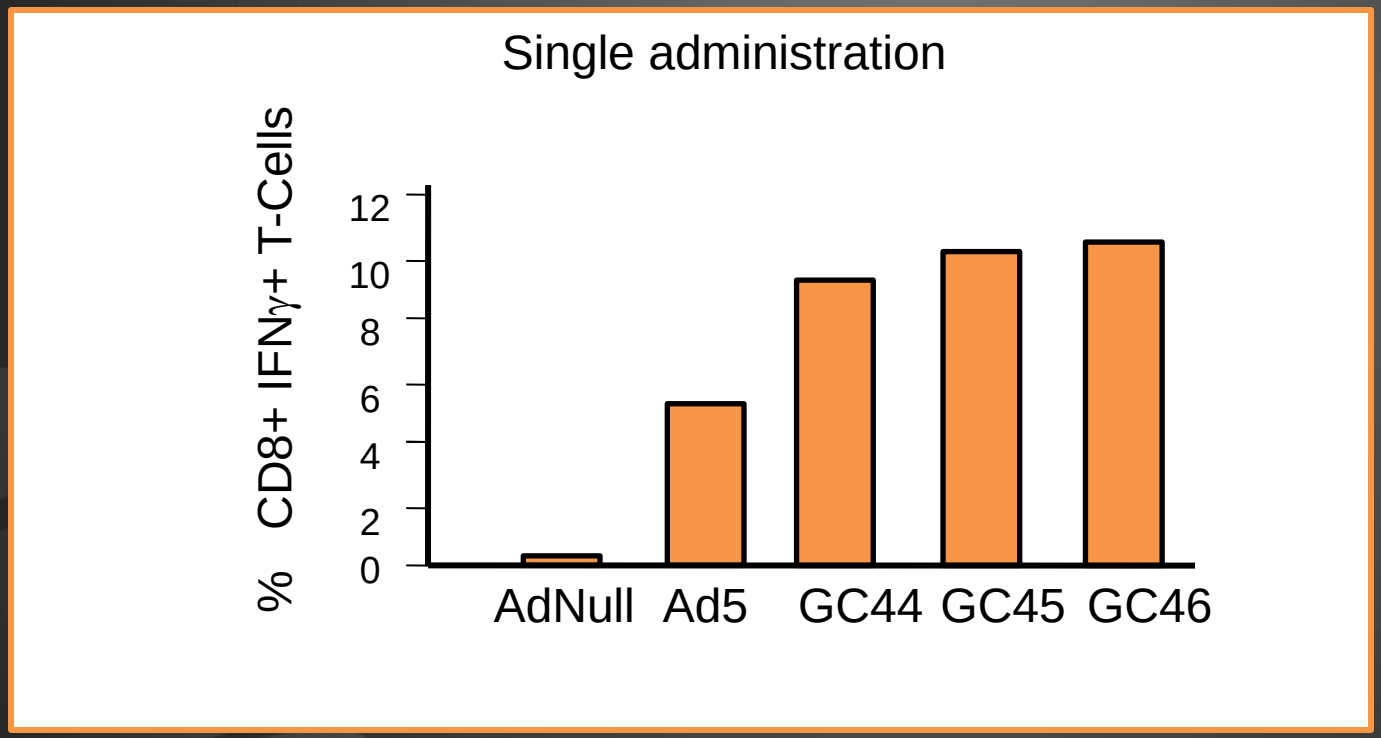
Superior Antigen Responses

High-level, Durable Antibody Response



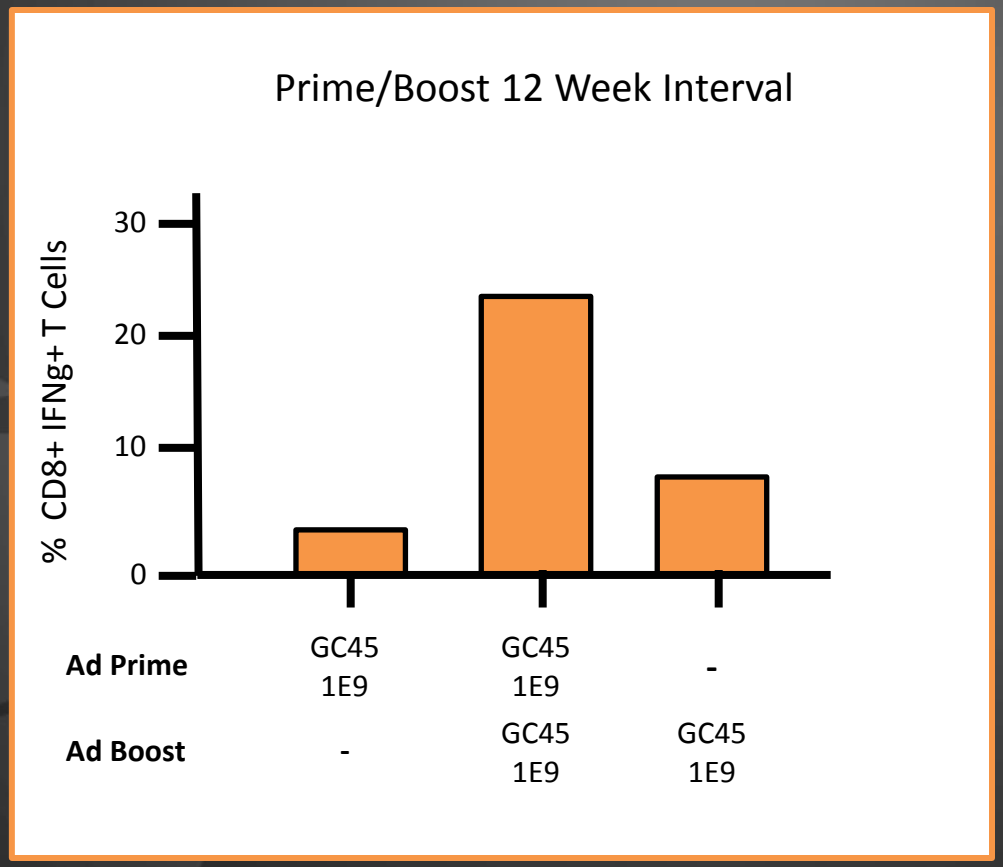
Superior Antigen Responses

High-Level T Cell Response



Superior Antigen Responses

Repeat Administration Boosts Response



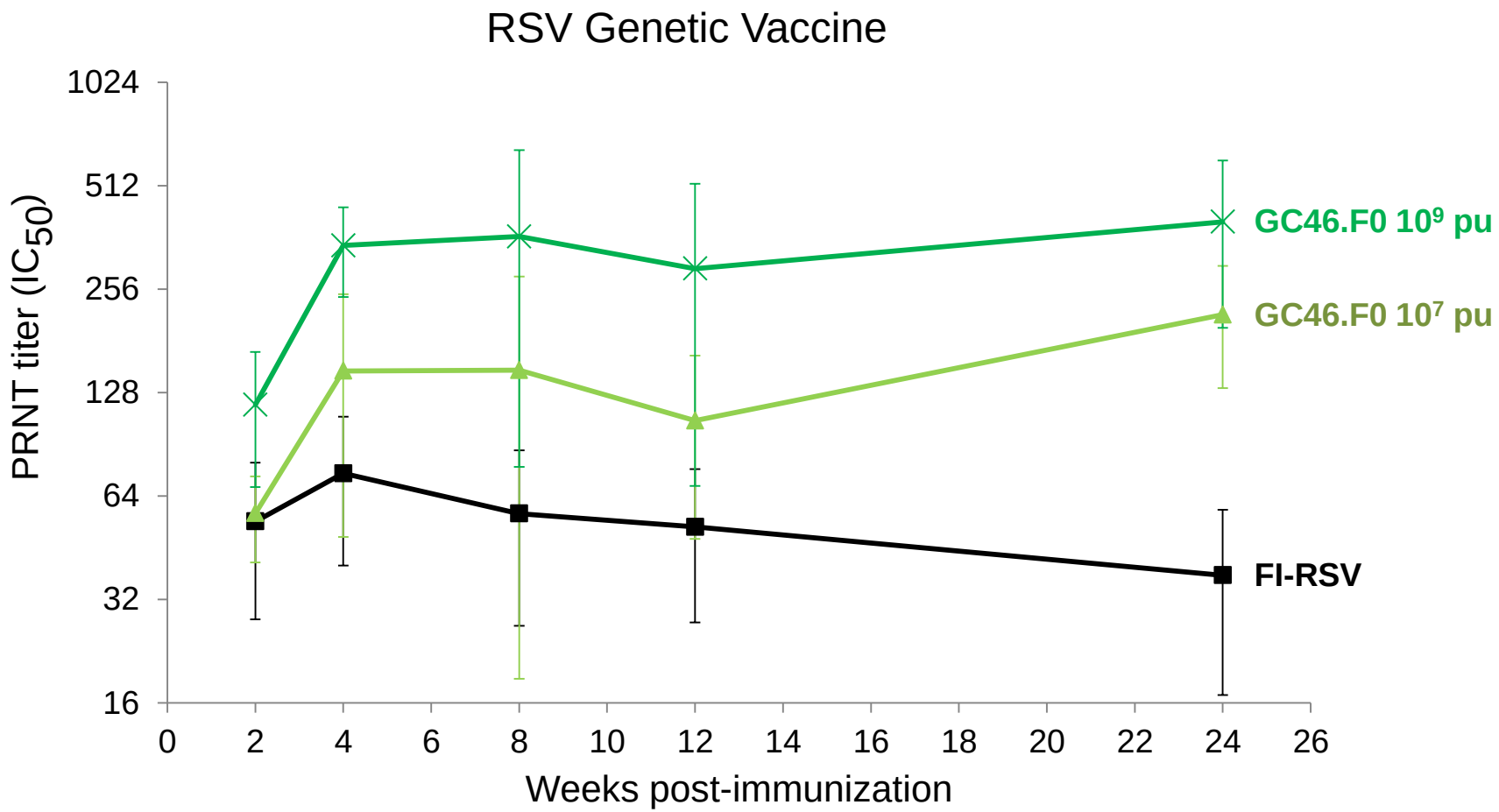
RSV Vaccine

A Critical, Unmet Medical Need

- RSV infection is a significant cause of serious respiratory illness globally
- Most affected are children, elderly, and high-risk adults
- No approved vaccine available
- Market is only partially addressed by Synagis®, which provides passive immunity and is indicated only for high-risk infants

GC Vector for RSV Vaccine

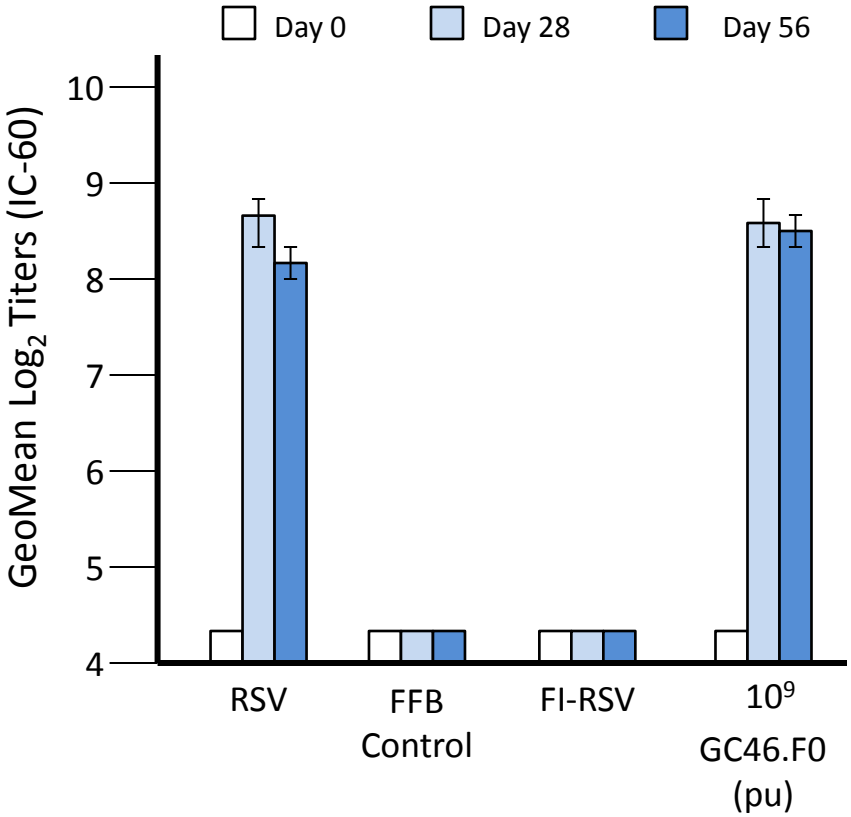
Durable Neutralizing Antibody from a Single Dose



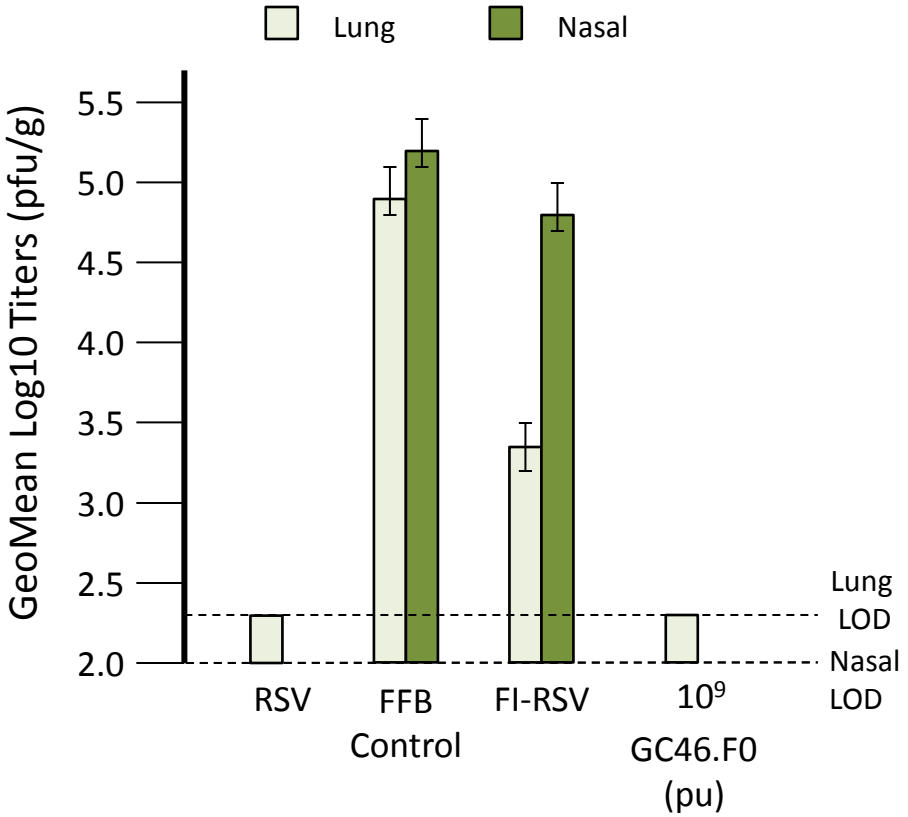
Single Administration

Protects Upper and Lower Airways Against RSV Challenge

Serum neutralizing antibody



Protection against RSV



- Lung and nasal titers of RSV were reduced to undetectable levels

RSV Genetic Vaccine

Summary

- Encouraging preclinical data
  - Upper and lower respiratory tracts protected
  - Good breadth of RSV neutralization
  - No evidence of RSV disease potentiation
  - Intra-muscular route of administration effective
- Lead vaccine (GV2311) selected
- Productive pre-IND meeting with FDA

Financial Summary

Common Shares Outstanding[1]	• 17.3 million shares
Exchange: Symbol	• NASDAQ: GNVC
Market Capitalization[2]	• $35.8 million
Cash and Investments[3]	• $9.9 million
Employees	• 15

[1]As of October 31, 2015; [2]Stock price as of November 6, 2015; [3]As of September 30, 2015

Investment Highlights

- Pioneering biotechnology company focused on leveraging its AdenoVerse™ gene delivery platform to develop a pipeline of cutting-edge therapeutics and vaccines
- Clinical stage hearing loss program partnered with Novartis
- GenVec therapeutics and vaccines safely administered to over 3,000 clinical study subjects
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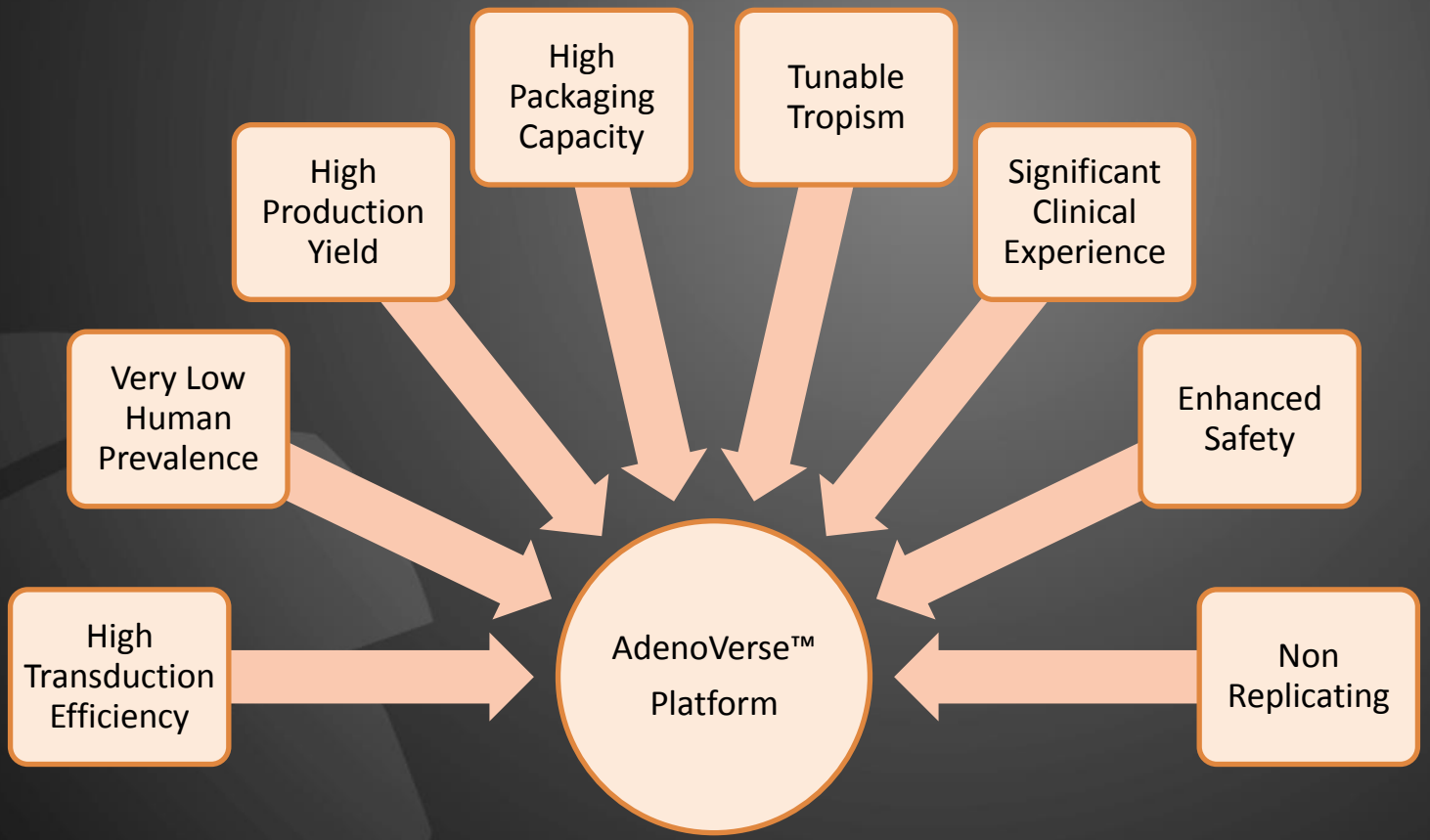
AdenoVerse™

GenVec's Proprietary Technology Platform



www.genvec.com