



American Gene Technologies

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Preliminary results



Untreated Control



AG1103 Treated



The *Disruptive* Potential of Gene Technologies



Single treatment cures that can eliminate a life time of chronic care symptom management and treatment.



Targeted, locally administered therapies that do not cause systemic toxicities.



Consistent, cost-efficient and -effective vector delivery streamlines the development and the regulatory process (80% reusable platform components speed development)



Genetic therapies will replace a half-trillion dollars worth of today's drugs in the coming decade. Genetic drug investors will be on the winning side of a game-changing event.

Better, Safer, Cheaper & Faster

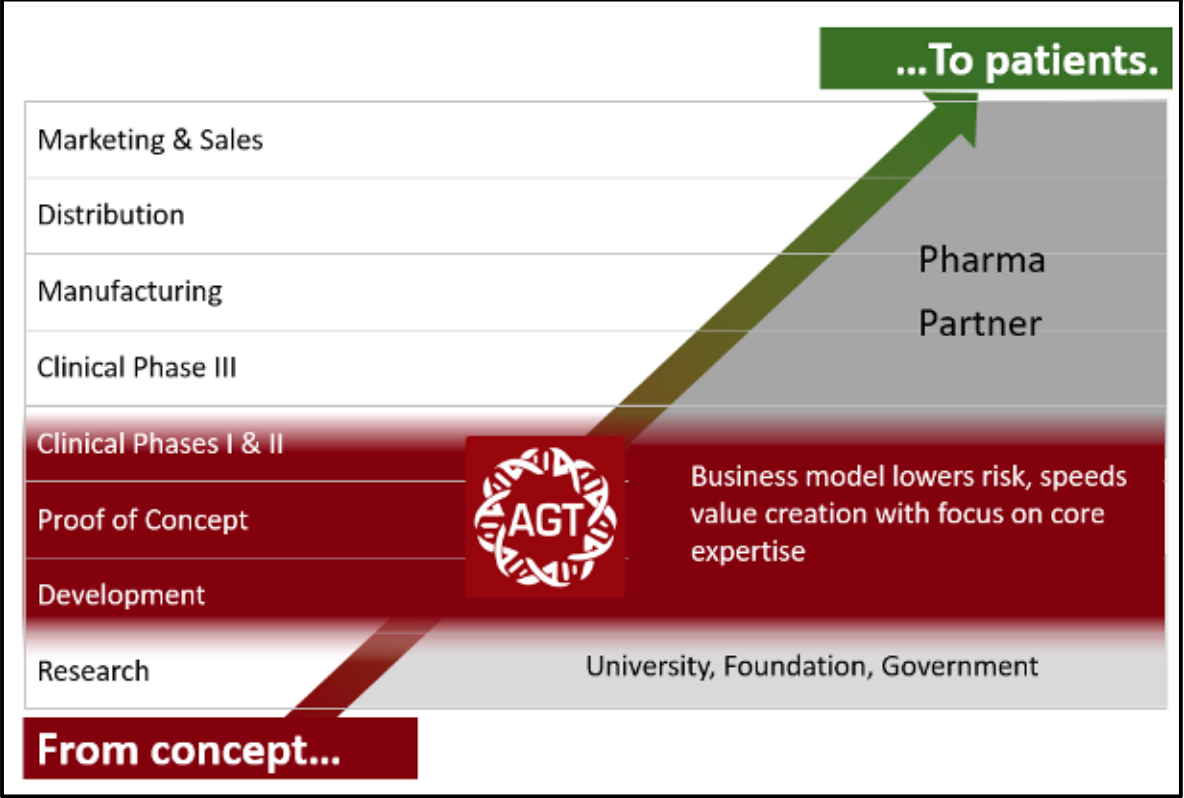




- AGT is pioneering a proprietary therapeutic platform that delivers genetically-engineered **cures** to a host of disease indications.
- Our unique lentiviral vector technology platform offers improved outcomes:
 - Lenti advantages over AAV vectors:
 - Non-immunogenic (allows multiple administrations)
 - Non-inflammatory (greater therapeutic index – higher expression)
 - Transient Vector™:
 - Allows shaping PK curve (matching therapeutic needs)
 - Temporary expression (greater safety – broader disease applicability)
 - Proprietary immune stimulating vectors
 - Broad immuno-oncology applications
 - Improved safety over CAR-T approaches



Platform Yields Multiple Monetization Options



Clinical PoC positions *each* therapeutic candidate for:

- Spinout
- Pharma out-licensing
- Acquisition
- IPO

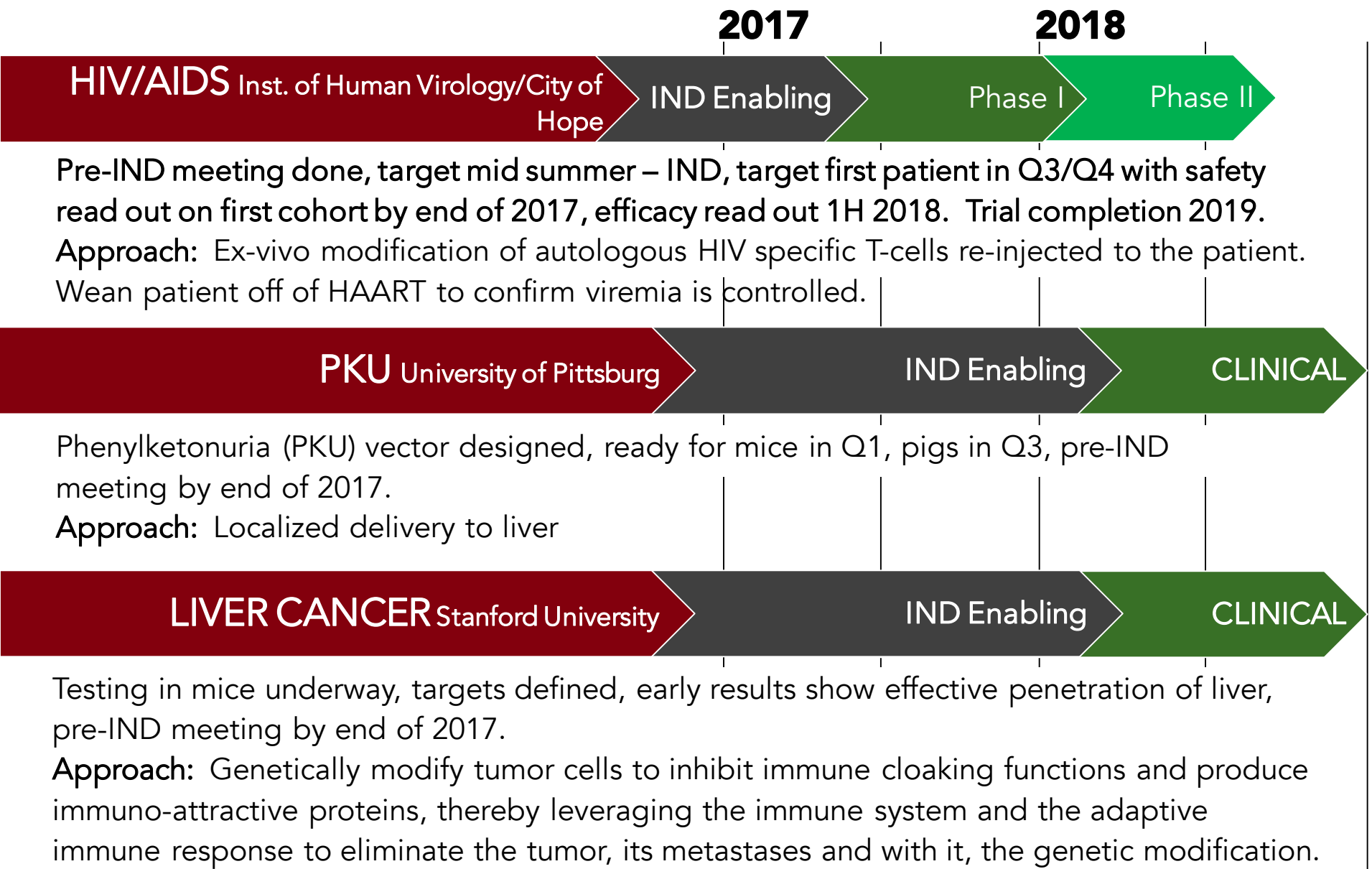
Collaborators, partners and competitors can out-license our technology.

Multiple Monetization Options After Reaching Clinical Status.





Pipeline Positions for Multiple Market Validation





Market Opportunity

Disease	Market	Patients	Current Treatment	Price	Total Available Market
HIV	US and EU HIV cases eligible for AGT Functional Cure (20% of infected population).*	400,000	\$500,000 lifetime cost of treatment (not cure). Treatment has side effects (sometimes serious).	\$200,000	\$80 Billion
PKU	43,110 cases in North American (~1 in 12,500 live births), 81,861 in Europe and Russia.	125,000	\$45,000 per year (dietary supplements). Biomarin Kuvan (has some effect) \$330,000,000 per year in revenue.	\$100,000	\$12.5 Billion
Liver Cancer (HCC)	89,000 annual cases in US and Europe.	89,000	No efficacious treatment available.	\$200,000	\$17.8 Billion per year

* Total HIV infections worldwide: 44 million. In vivo version will allow wider commercialization.





- 9 patent “families” providing multiple angles of protection over key developments and technological **innovations**
- 18 Patents in process:
 - 7 PCT Status
 - 11 Provisional Status
- Estimated IP “liquidation valuation” of \$100M
- Broad, robust protection of “foundation” innovations to viral vector technologies such as “Transient Vector™” and “ImmunoTox™” (immune-regulatory vector for oncology applications).





Clinic Entry Can Yield Inflection in Valuation – ROI Opportunity

- AveXis (NASDAQ:AVXS), clinical stage – Phase 1 for spinal muscular atrophy (orphan), or SMA, type 1, market cap: **\$1.4 billion**
- BluebirdBio (NASDAQ:BLUE), clinical stage – Phase 3 for β – thalassemia and Cerebral ALD (orphan), along with other earlier stage products, market cap: **\$2.7 billion**
- AGT, soon to be clinical stage – Phase 1/2 for HIV (mass market), along with other earlier stage products, market cap: **\$100 million**





Market Comparisons and Strategic Deals

Type	Company	Market Cap (1/23/17)	Market Cap IPO	Raised	IPO Date	IPO Price	52-Wk High/Low
CAR-T	Kite Pharma (KITE)	\$2,290 M	\$627 M	\$134 M	6 / 2014	\$17.00	\$64.30 / \$38.41
	Bellicum (BLCM)	\$335 M	\$471 M	\$140 M	12 / 2014	\$19.00	\$23.11 / \$7.24
	Juno Therapeutics (JUNO)	\$2,070 M	\$2,850 M	\$265 M	12 / 2014	\$24.00	\$49.72 / \$17.52
	Cellectis (ZVA.F)	€581 M	\$1,500 M	\$228 M	3 / 2015	\$41.50	\$29.48/ \$14.43
AAV	uniQure (QURE)	\$133 M	\$285 M	\$82 M	2 / 2014	\$17.00	\$19.03 / \$5.30
	Spark (ONCE)	\$1,770 M	\$1,200 M	\$161 M	1 / 2015	\$23.00	\$65.99/ \$21.20
	RENGENXBIO (RGNX)	\$527 M	\$443 M	\$122 M	9 / 2015	\$22.00	\$24.55 / \$7.07
	Dimension (DMTX)	\$108 M	\$355 M	\$72 M	10 / 2015	\$13.00	\$10.47 / \$4.00
	Voyager (VYGR)	\$307M	\$360 M	\$70 M	11 / 2015	\$14.00	\$18.25/ \$8.12
	AveXis (AVXS)	\$1,410 M	\$413 M	\$95 M	2/2016	\$20.00	\$72.72 / \$16.11
Lentivirus	BluebirdBio (BLUE)	\$2,650 M	\$2,662 M(est)	\$101 M	6 / 2013	\$17.00	\$79.70 / \$35.37
	Intrexon (XON)	\$2.620 M	\$1,523 M	\$160 M	8 / 2013	\$16.00	\$40.24 / \$21.98

BMS + uniQure:
 \$100M up-front
 up to \$2.5Bn
 for 10 drugs

Celgene + Juno:
 \$1Bn for 10% stake,
 licensing rights

Bayer + Dimension:
 \$20M up-front
 up to \$232M for milestones
 on Hemophilia A





AGT Advantages

Experience

- Ten years of Lentiviral Vector experience and platform development.

IP

- Unique IP and technology that applies to broader set of diseases with greater efficiency and efficacy.

Collaborators

- Top research collaborators from outstanding institutions with resources to leverage AGT IP.

Trials

- Multiple near-term opportunities for clinical success: HIV, Phenylketonuria, Liver Cancer.

Partnerships

- Proprietary gene-delivery technologies with opportunities for out-licensing.

Scalability

- Scalable drug development process and business model allows greater growth, rapid product pipeline development after initial clinical successes.





Collaborators

Bringing decades of research to accelerate our development



Roscoe Brady MD, PhD

National Academy of Sciences, Chief of the Developmental and Metabolic Neurology Branch
National Institutes of Health, Lasker Foundation Clinical Medical Research Award
2007 National Medal of Technology and Innovation



Robert R. Redfield MD

Division Head and Professor of Medicine, Microbiology and Immunology
Chief of Infectious Diseases and Director of the HIV program
Presidential Advisory Council on HIV/AIDS



Gerard Vockley MD, PhD

Chief, Division of Medical Genetics
Professor of Pediatrics and Professor of Human Genetics
University of Pittsburgh



Dean W. Felsher MD, PhD

Associate Professor of Medicine (Oncology) and Pathology
Stanford University School of Medicine



Robert Clarke PhD

Dean For Research, Georgetown University Medical Center, Professor Of Oncology
Professor, Department Of Oncology
Co-director, Breast Cancer Program



Ted Dawson MD, PhD

Leonard and Madlyn Abramson Professor in Neurodegenerative Diseases
Professor of Neurology and Neuroscience
Director, Institute for Cell Engineering, Johns Hopkins University



Frances Lefcort PhD

Professor and Department Head
Department of Cell Biology & Neuroscience, Montana State University



Experienced Executives, Advisors, Focused on Value Creation



Jeff Galvin

Chairman, CEO

B.A. Econ Harvard

Successful Tech
Entrepreneur

Computer Scientist

Angel Investor



David Pauza, PhD

Chief Science Officer

Ph.D. Berkeley

Faculty University of
Wisconsin, Madison

Associate Director IHV
University of Maryland



Neil Lyons, CPA

Senior Finance Advisor

Florida Southern
College

Public Biotech CFO (10
years, with international
clinical trials experience
Deloitte (10 years)
Honeywell, Verizon,
Alcatel



Dean Felsher, MD, PhD

Science Advisory Board

Stanford Medical School

Dean of Translational Medicine
Stanford Medical School

Professor of Oncology
Professor of Pathology
Stanford University



Conclusion

Focused business model speeds investor return

Multiple commercialization and liquidity opportunities

Strong team supports company potential

