UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

FORM 8-K

CURRENT REPORT PURSUANT
TO SECTION 13 OR 15(D) OF THE
SECURITIES EXCHANGE ACT OF 1934

Date of report (Date of earliest event reported): **May 7, 2015**

PLASMATECH BIOPHARMACEUTICALS, INC.
(Exact name of registrant as specified in its charter)

Delaware	**0-9314**	**83-0221517**
(State or other jurisdiction of incorporation)	(Commission File Number)	(I.R.S. Employer Identification No.)

4848 Lemmon Avenue, Suite 517, Dallas, TX 75219
(Address of principal executive offices) (Zip Code)

(214) 905-5100
(Registrant's telephone number, including area code)

PLASMATECH BIOPHARMACEUTICALS, INC.
(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Item 7.01. Regulation FD Disclosure
Item 8.01. Other Items

Presentations relating to our technology, business and corporate financial structure will be made to investors during May 2015. The presentation is attached as Exhibit 99.1 and is incorporated herein.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits.

99.1 Presentation entitled “PlasmaTech Biopharmaceuticals”

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

PlasmaTech Biopharmaceuticals, Inc.
(Registrant)

By: /s/ Stephen B. Thompson
Stephen B. Thompson
Vice President Finance Chief Accounting Officer

Date: May 7, 2015

EXHIBIT INDEX

Exhibit Number	
99.1	Presentation entitled “PlasmaTech Biopharmaceuticals”

Exhibit 99.1


plasmaTECH™
BIOPHARMACEUTICALS
NASDAQ: PTBI
Investor Presentation

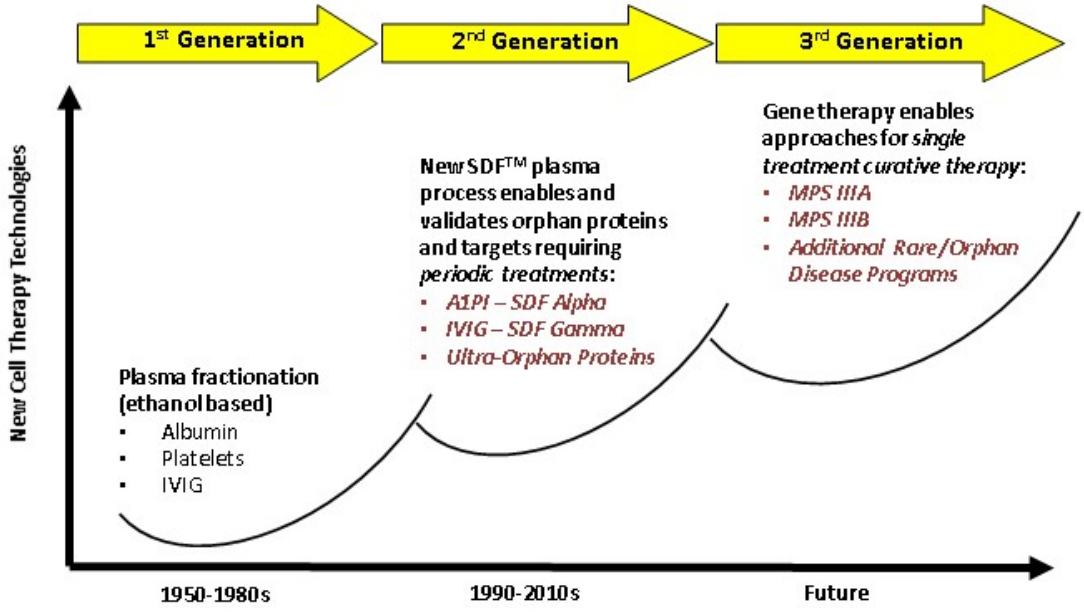
Safe Harbor Statement

This presentation contains certain statements that may be forward-looking within the meaning of Section 27a of the Securities Act of 1933, as amended, including statements relating to the product portfolio and pipeline and clinical programs of the company, the market opportunities for the Plasma Technologies fractionation technology, MuGard, ProctiGard, and the other mucoadhesive hydrogel products, and the company´s goals and objectives. These statements are subject to numerous risks and uncertainties, including but not limited to the risks detailed in the Company's Annual Report on Form 10-K for the year ended December 31, 2014, and other reports filed by the company with the Securities and Exchange Commission.

This presentation does not constitute an offer or invitation for the sale or purchase of securities or to engage in any other transaction with PlasmaTech or its affiliates. The information in this presentation is not targeted at the residents of any particular country or jurisdiction and is not intended for distribution to, or use by, any person in any jurisdiction or country where such distribution or use would be contrary to local laws or regulations.

PTBI Strategic Imperative

Progression of Cell Therapy to Gene Therapy



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Overview

PlasmaTech & Abeona Merger

Building a world class cell & gene therapy company focused on rare diseases

MPS IIIA & MPS IIIB Programs (Abeona)

- AAV-based gene therapies for MPS III & MPS IIIB
- Orphan Drug and Pediatric Rare Disease Designations from FDA
- Plan to file IND in MPS IIIB in June 2015, first patient in - 4Q15

Alpha-1 Deficiency & Ultra-Orphan Proteins (PlasmaTech)

- Proprietary SDF process expands yields significantly relative to Cohn process
- Scaling up Alpha-1 protease inhibitor (A1PI), plan to file IND in 4Q15
- Evaluating process for IVIG and ultra-orphan proteins

Overview

Abeona Therapeutics

- ✓ Founded in March 2013
- ✓ Lead products are licensed gene therapy technology from **Nationwide Children's Hospital** (Columbus, OH)
- ✓ ***Lead products***: AAV9 gene therapy for MPS IIIA & MPS IIIB
- ✓ Received the ***FDA Orphan Drug Designation*** for both MPS IIIA and MPS IIIB clinical programs
- ✓ Received ***Pediatric Rare Disease Designations*** for both programs
- ✓ Conducted RAC and Pre-IND meetings
- ✓ Raised $4.8M for MPS IIIA and IIIB programs with leading Sanfilippo Foundations worldwide

NATIONWIDE CHILDREN'S
When your child needs a hospital, everything matters.

Abeona – Roman goddess, protector of children leaving home

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MPS III

Sanfilippo Syndromes

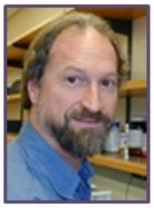
- ✓ Rare lysosomal storage disease affecting children
- ✓ Deficiency in one of four cellular enzymes (***MPS IIIA, IIIB, IIIC and IIID***)
- ✓ Onset between ages 2 and 6; inability to walk by 10. Progressive, severe neurological and muscular disorder
- ✓ Aggressive behavior, seizures, loss of speech/vision, inability to sleep
- ✓ Treatments for comparable diseases (MPS I) are $250,000 to $450,000 per year
- ✓ ***No FDA approved treatments***
- ✓ **Active and supportive Foundations as partners**

Courtesy: Chester Hembree

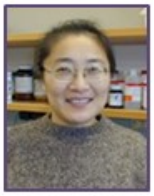
Scientific Team

Nationwide Children's

Kevin Flanigan, MD
Clinical Investigator



Doug McCarty, Ph.D.
Scientific Founder



Haiyan Fu, Ph.D.
Scientific Founder

Scientific Advisors

Joseph Muenzer, Ph.D.
Univ of North Carolina

Brian Kaspar, Ph.D.
Nationwide Children's
and Ohio State

Barry Byrne, MD, Ph.D.
Univ of Florida
Powell Gene Therapy Center

Maria Escolar, MD
University of Pittsburgh
Center for Neurodegenerative
Disorders

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Global Foundation Support

2013 Global Genes "Champions of Hope" Award – Co-Recipients

STOPSANFILIPPO FUNDACIÓN — Spain

fundación SANFILIPPO B — Spain

Team Sanfilippo Foundation — USA

FONDATION SANFILIPPO Suisse — Switzerland

ABBY GRACE FOUNDATION Cure Sanfilippo — USA

The Children's Medical Research Foundation, Inc. A Cure for Kirby — USA

Ben's Dream The Sanfilippo Research Foundation — USA

The Sanfilippo Children's Research Foundation — Canada

National MPS Society Support for Families. Research for a Cure. — USA

CURE — USA

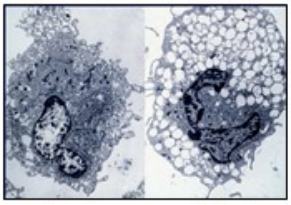
REDSANFILIPPO FUNDACIÓN — Mexico

Sanfilippo Children's Foundation Funding research, finding gems — Australia

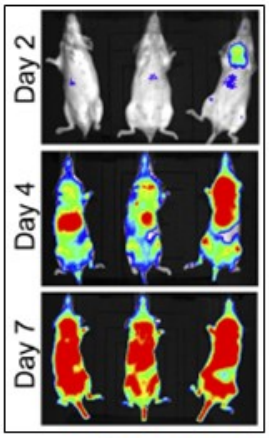
MPS III

Abeona's Gene Therapy Approach

- ✓ **AAV9 single injection gene therapies, intravenous delivery for MPS IIIA and MPS IIIB**
- ✓ ***Lysosomal Storage Diseases***
  - Ideal candidates for gene therapy – Bystander cell uptake
  - Requires lower levels of delivery to cells for therapeutic benefit
  - Fast track therapy status decreases time to market
- ✓ ***Established delivery system***
  - High safety profile, strong preclinical efficacy observed
  - Gene therapy drug product crosses blood/brain barrier
  - Long-term gene expression in the CNS and peripheral tissues
  - Patients and families very supportive of approach
- ✓ ***Market readiness***
  - First approved gene therapy product - (Glybera)
  - Patient driven need – time is critical for kids
  - No FDA approved therapies available



Normal MPS III



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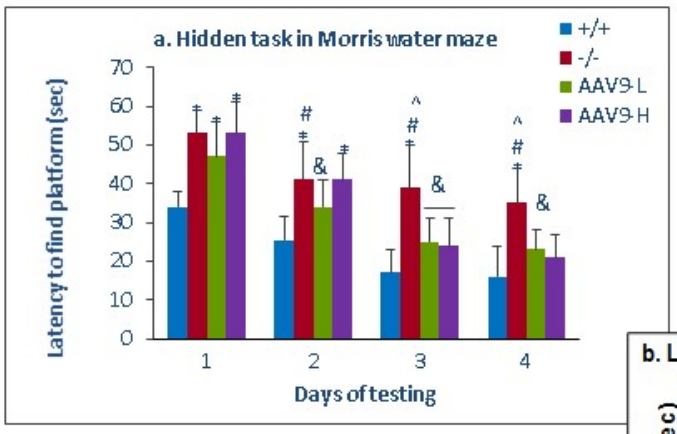
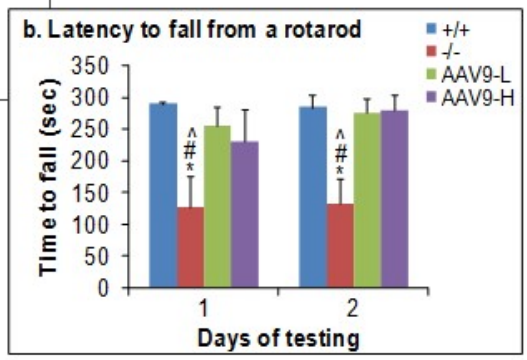
Ref: Asokan 2012

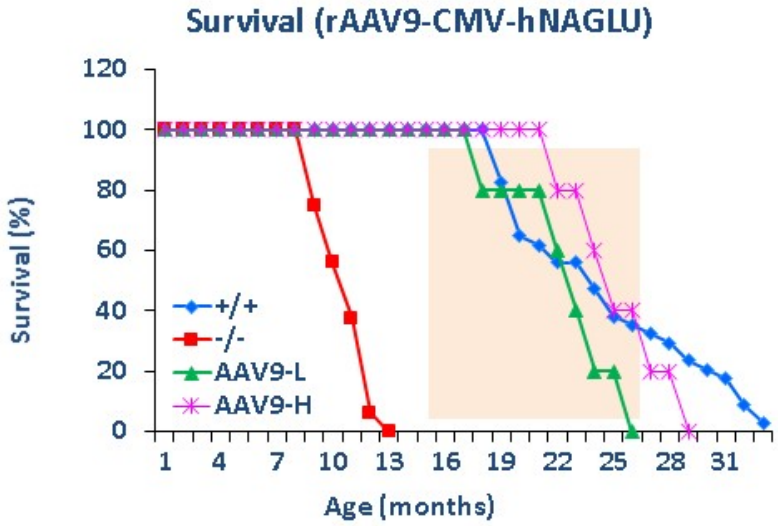
Normalization of Behavior and Muscle Function in 4-6 Week Old MPS IIIB Mice after a Single Injection of rAAV9-CMV-hNAGLU



5.0 - 5.5 months old
(4 months post-injection)



IV Infusion of rAAV9-hNAGLU Vector at 4 – 6 Weeks of Age
Normalized The Survival in MPS IIIB Mice



Survival Increased 100% after single IV treatment

MPS III

Critical Abeona Competitive Advantages

- ✓ ***Pre-clinical Effectiveness***: Only MPS therapy to demonstrate in ***cognitive***, ***muscular*** and ***survival*** benefits!
- ✓ ***Neurotropism:*** Only therapy in development that cross blood brain barrier (AAV9)
  - Single IV injection, compared to multiple injections yearly
  - No ports needed; no drilling into child's skull for access
  - Use of self-complementary technology for MPS IIIA – improved efficacy
- ✓ ***Lasting Treatment Effects:*** Pre-clinical data shows lasting treatment from months to years after delivery
- ✓ ***Safety Profile:*** No significant adverse events to date
- ✓ Abeona granted ***Orphan Drug Designation*** for both AAV9 MPS IIIA and IIIB drugs
- ✓ Abeona received ***Rare Pediatric Disease Designations*** for both MPS IIIA and IIIB drugs
- ✓ Backing of multiple international Sanfilippo Foundations; science featured on ***CBS's 60 Minutes*** in June 2014

Comparables

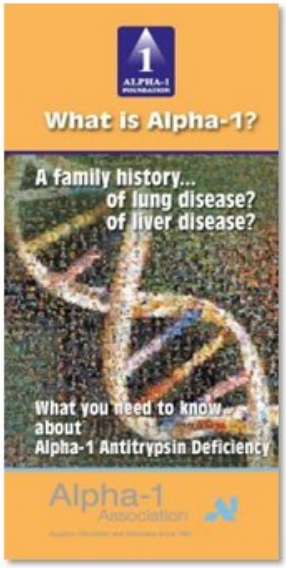
Cell & Gene Therapy Companies

Company (Ticker)	Market Cap	Indication – Stage of Development
Avalanche Bio (AAVL)	$820.1 M	AAVs for opthalmology – plan to file IND 2H 2015
Bluebird Bio (BLUE)	$4.56 B	Multiple programs (lenti, CAR T) – multiple clinical stage programs
Bellicum Pharma (BLCM)	$744.12 M	Adjunct T-cell therapy/dedritic cell vaccine/CAR T – in Phase 1/2 trials
BioMarin Pharma (BMRN)	$19.39 B	MPS I, MPS VI, multiple approved products and programs - Commercial
Intrexon Corp (XON)	$4.74 B	Toolkit synthetic biology company – multiple partnerships
Juno Therapeutics (JUNO)	$5.24B	Cell-based cancer immunotherapies – multiple Phase 1 and 1/2 trials
Kite Pharma (KITE)	$2.65 B	CAR T immunotherapies in oncology – multiple Phase 1 and one 2/3 trial
Spark Therapeutics (ONCE)	$1.51 B	Rare disease gene therapy – one Phase 3, one ½, other IND-enabling
uniQure N.V. (QURE)	$564.3 M	Approved Glybera for LLD, other Phase 1 in hemophilia - Commercial
Ziopharm Oncology (ZIOP)	$1.38 B	Gene & cell therapies (CAR T), partnered XON – one Phase 1/2, other pre
China Biologics (CBPO)	$2.45 B	Plasma fractionator in China – Commercial
Prometic Life Sciences (PLI.to)	$1.40 B	Affinity chromatography for plasma proteins
PlasmaTech/Abeona (PTBI)	***$85 M***	***Cell & gene therapies (AAV9, MPS IIIA&B, Alpha-1 Def) –file IND 6/2015***

Alpha-1

Alpha-1 Deficiency – Inherited COPD

- ✓ ***AATD is an under-diagnosed hereditary condition***
  - Genetic condition whereby insufficient AAT protein produced by liver; leads to COPD and liver disease
  - Roughly 1 in every 2,500 Americans have AATD; 5 times more prevalent than Cystic Fibrosis
  - Up to 3% of all people diagnosed with COPD may have undetected AATD
- ✓ ***Alpha-1 Market Drivers***
  - Increased clinician awareness;
  - Early detection; newborn screening in NY and MA
  - Grifols' SPARTA study (doubling dose); new indications
- ✓ ***New Sources of Alpha-1 Required***
  - Current supply based upon 75-year old Cohn process
  - Cohn fractionation does not selectively target Alpha-1; low yields
  - Proprietary SDF process targets high-value proteins, increasing yields



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SDF Process

Benefits of SDF Process over Cohn

Kinder, Gentler	✓ Simple 2-Stage Sodium Citrate Precipitation + Diafiltration ✓ No ethanol or PH changes like Cohn method ✓ Less denaturing of select proteins ✓ Roughly 2-3 day process, versus 6-day for Cohn
Yield Improvements	✓ ***Alpha-1 yield increase ~10X*** ✓ ***IVIG yield increase ~ 20%*** ✓ *Potential for multiple ultra-orphan proteins*
Margin Improvements	✓ ***Yield improvements could drive 80% product margins versus ~30% Cohn process margin***
Proprietary Intellectual Property	✓ Three issued US and WW patents, foreign counterparts pending; additional patent filings

Alpha-1 Opportunity

- ✓ ***Orphan Drug Characteristics***
  - Up to 300,000 potential patients in US and Europe; only roughly 10,000 on replacement therapy today
  - A1PI replacement therapy is reimbursed at $100,000 per patient annually
  - Patients on therapy for 22 years (average)
- ✓ ***High value, high growth market***
  - A1PI global market in 2014 was ~$900 million; growing at greater than 20% annually
  - Current Cohn yields may be insufficient to address future demand
- ✓ ***Abbreviated Regulatory Pathway***
  - BLA approval pathway 351(a) – estimated $4 to 6 million per protein to regulatory approval
  - A1PIs have been approved on short, 50-patient Phase 1/Pivotal safety and bioequivalency trials
  - Anticipate SDF Alpha approval in 24 month timeframe
- ✓ ***Collaborative Development & Commercialization Strategy***
  - Initiated contract manufacturing relationships; optimizing downstream chromatography, viral inactivation and lyophilization stages of process
  - Evaluating SDF process for additional ultra-orphan proteins, such as C-1 esterase inhibitor, fibrinogen and plasminogen
  - Multiple product opportunities enhance partnering opportunities globally

SDF Gamma

Intravenous Immunoglobulin (IVIG)

- ✓ SDF Process increases IVIG yield by ~20%
- ✓ ~50% of the $15 billion plasma protein market
- ✓ IVIG is the high value protein with utility in patients with decreased or abolished antibody production capabilities

Neurology	Hematology	Dermatology	Other
✓ Guillain Barre	✓ Immune thrombo-cytopenia	✓ Kawasaki Syndrome	✓ Primary antibody deficiencies
✓ Lambert Eaton Syndrome	✓ Post BMT	✓ Dermatomyositis	✓ Vasculitis
✓ Multifocal motor neuropathy	✓ Myeloma and CLL	✓ Toxic epidermal necrolysis	✓ Autoimmune uveitis
✓ Myasthenia gravis	✓ Immune neutropenia	✓ Atopic dermatitis	✓ Birdshot retinochoro-idopathy
✓ Stiff person syndrome	✓ Parovirus B19 associated aplasia	✓ Blistering diseases	✓ Mucous membrane pemphigoid

SAB

Leaders in Alpha-1 and Mucositis

Eugene Zurlo, BS, MS Pharmacy, *Chairman*

- 56 years experience
- Founder/Chairman/ Inventor Plasma Technologies, LLC (Licensor)
- Baxter Hyland, Millipore, NY Blood Center, Alpine Biologics, Ayerst Laboratories

Robert Sandhaus, MD, PhD

- Professor of Medicine: National Jewish Health, Denver CO
- Clinical Director: Alpha-1 Foundation
- Medical Director, Founder: AlphaNet
- Extensive plasma therapeutics industry experience

Charles Heldebrant, PhD

- Alpha Therapeutic Corporation
- Grifols: development, regulatory clearance, production of AAT product (sold to Baxter)
- Extensive experience in biological & pharmaceutical product development, regulatory, quality, validation

Charlie Strange, MD

- Professor Pulmonary, Critical Care, Allergy, and Sleep Medicine, University S Carolina, Charleston SC
- Director: Alpha-1 Foundation Research Registry
- Clinical trial design & rare diseases expert

Allan Louderback, PhD

- Head of Biomechanical Research: Baxter Hyland
- Founder/President CRO served: Baxter, Dade, Amgen, Biogen, Nichols, Technion, NY Blood Center
- Co-inventor Plasma Technologies SDF Process

Stephen T. Sonis, DMD, DMSc

- Clinical Professor of Oral Medicine, Harvard, Senior Surgeon, Brigham and Women's Hospital and Dana-Farber Cancer Institute, Founder, CSO Biomodels
- Expert in epithelial injury due to cancer therapy
- Author >200 original publications, 9 books, 5 patents

Hydrogel Polymer Products

Two FDA-cleared Commercial Products

- ✓ **Patented mucoadhesive hydrogel delivery system enables extended delivery of drugs to mucosal tissue**

- ✓ **MuGard® for Oral Mucositis ("OM")**
  - $1 billion market, underserved, few competing products with demonstrated clinical benefit
  - >400K OM patients annually in the US
  - Four commercial partners in 5 geographic regions
  - Commercial launches in Europe, Korea, China to drive royalties in 2015/2016

- ✓ **ProctiGard™ for Rectal Mucositis/Radiation Proctitis**
  - Filed with FDA and received marketing clearance in just 90 days (July 2014)
  - Large market opportunity with no good treatment options
  - Partnering discussions ongoing

MuGard™ Oral Mucoadhesive Hydrogel

ProctiGard™

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Management & Board of Directors

Management

Tim Miller, Ph.D. – *President & CEO*
- Extensive scientific, product development and clinical operations experience
- Multiple IND submissions
- Ph.D. in Pharmacology (Gene Therapy/Cystic Fibrosis), Case Western University

Harrison Wehner - *CFO*
- 21 years healthcare & biotech IB, financial advisory, M&A
- Senior positions: Canaccord Genuity, CitiGroup, UBS

Stephen Thompson – *VP Finance, Treasurer, Sec*
- 26 years financing and accounting
- Prior CFO and Controller experience

David Nowotnik, Ph.D. - *SVP R&D*
- 41 years experience pharmaceutical R&D, quality systems, regulatory affairs
- Bristol-Myers Squibb, Amersham International, Guilford Pharmaceuticals

Board of Directors

Steven Rouhandeh, *Executive Chairman*
- SCO Capital Partners
- Founder SCO Financial Group
- Deutsche Bank, Cravath

Mark Alvino
- Bradley Woods, Griffin Securities

Stephen Howell, M.D.
- UCSD, UCSD Cancer Center
- Miliken Foundation prize: cancer chemo

Jeffrey Davis, *COO & Director*
- 20+ years, CEO & CFO roles
- Bell Laboratories, Philips Medical Systems
- Deutsche Bank

Mark Ahn, Ph.D.
- Genentech, Galena Biopharma, Bristol-Myers Squibb, Amgen

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Capitalization Table

Capitalization	Shares Outstanding	WAEP
Outstanding common shares (PTBI)	22,332,135	-
Warrants (PTBIW, fully traded)	3,500,000	$5.00
Options	233,834	$23.60
Primary Total	**22,332,135**	
Fully Diluted Total	**26,065,969**	

Recent events:

- Does not include 3,979,761 common shares to be issued to Abeona upon closing
- Does not include 577,756 "old" warrants outstanding with WAEP of $46.50
- No long term debt, no convertible preferred stock

Milestones

Milestones	Timing
Finalize Abeona acquisition	May 2015
File IND for MPS IIIB	June/July 2015
First Patient In, MPS IIIB in USA	Sept./Oct 2015
Complete and file IND for MPS IIIA	2H 2015
First Patient in, MPS IIIB in Europe, Australia	1H 2016
In-license Complementary Gene Therapy Programs	2015-16
SDF Alpha™ validation, characterization	2015
SDF Alpha™ *clinical study and BLA filing*	2016
SDF Alpha™ regulatory approval and revenue	2016-17
Follow-on plasma product targets: ultra-orphan proteins, and additional hydrogel platform products	2016-17+

Investment Highlights

PlasmaTech & Abeona Merger

Building a world class cell therapy company focused on rare diseases

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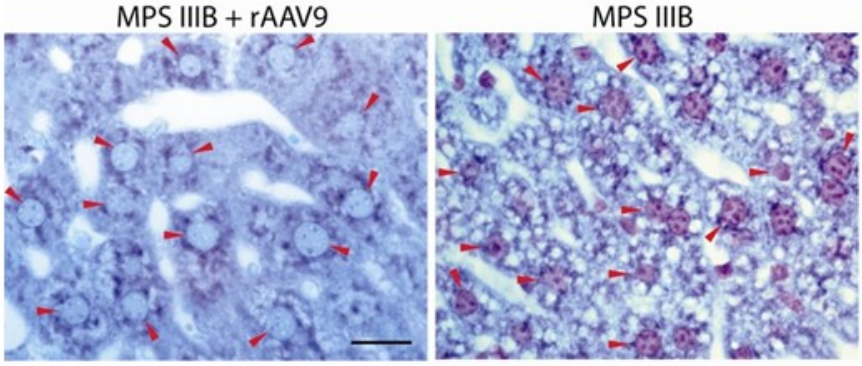
Addendum 1

Abeona Pre-Clinical Data

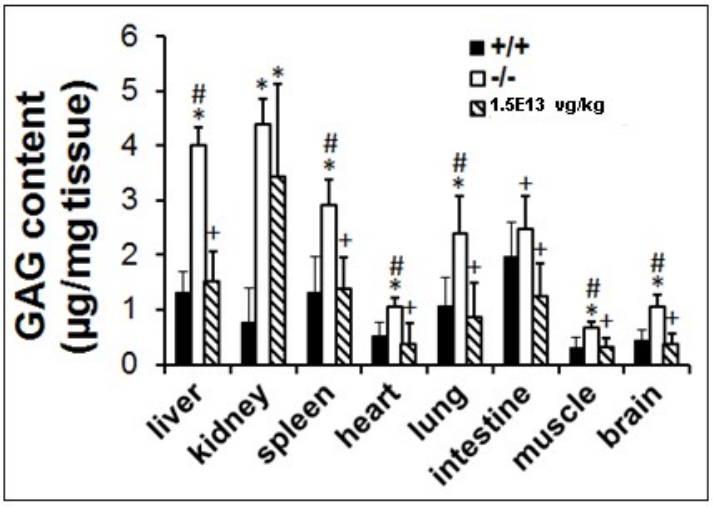
Clearance of Lysosomal Storage Pathology in the Liver of MPS IIIB Mice after an IV rAAV9.NAGLU Infusion



6 months post-injection. The red arrows indicate the nuclei of liver cells. Note the decrease in "storage lesions" or holes in the cells from the liver of a treated animal on the left. This data demonstrates that an IV injection of AAV9 can get into the liver, introduce a functioning copy of the gene that is altered in Sanfilippo syndrome and allow lysosomes to function appropriately.

IV Delivery of rAAV9.CMV.NAGLU Induces Clearance of Lysosomal GAG Storage in the CNS and Somatic Tissues

6 months post-injection



Lower bars are better.

Demonstrates that treated IIIB mice have reduced GAG content, similar to unaffected animals, in multiple tissues compared to untreated IIIB mice (with exception of kidney).

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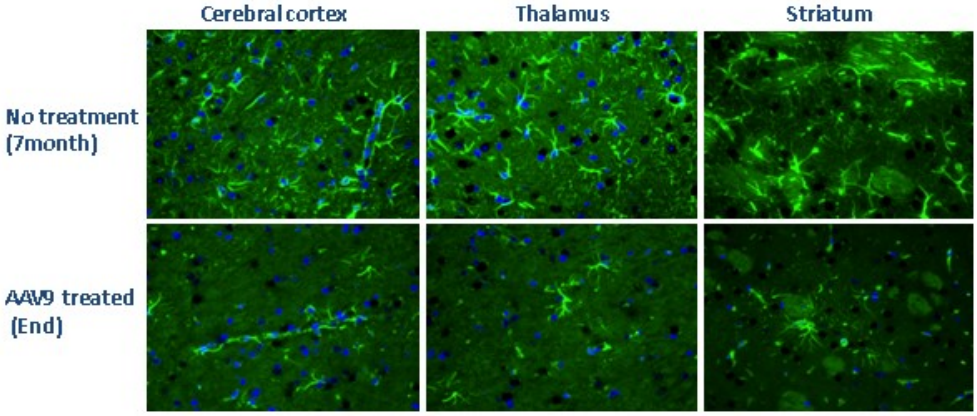
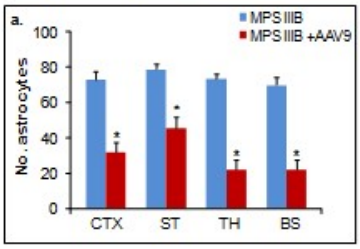
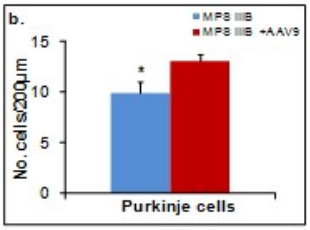
IV Delivered AAV9-CMV-NAGLU Corrects Secondary Neuropathology in MPS IIIB Mice: Astrocytosis and Neurodegeneration



Shorter bars are better. Indicates less trauma and destruction of neurons.





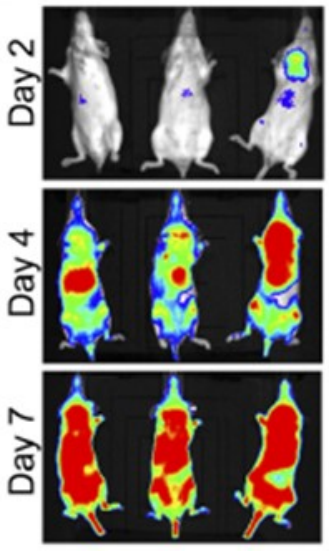
Longer bars are better. Indicates more neurons and less degeneration

scAAV9 Delivered by IV Demonstrates Systemic Transduction in Mice

Self-complementary AAV9 virus delivered intravenously to mice.

The AAV9 is delivering a gene to make the mouse glow where the AAV9 got into cells, referred to as "expression".

This demonstrates that by 7 days post-injection, the AAV9 vector administered by IV injection has been incorporated into cells all over the body and is "expressing" the gene of interest.



Ref: Asokan 2012

IV Delivery of rAAV9.CMV.NAGLU Induces Clearance of Lysosomal GAG Storage in the CNS and Somatic Tissues

rAAV9-mediated rNAGLU expression in MPS IIIB mouse brain

Vector dose (vg/kg)	AOI	NAGLU activity (% of wt levels)				
		1mo pi	3mo pi	6mo pi	12mo pi	End
$1x10^{13}$	4-6wk	60-560%	80-440%	60-312%	44-159%	268-721%
$1x10^{13}$	4-6mo	n/a	n/a	n/a	0-40%*	-
$2x10^{13}$	4-6wk	40-440%	50-674%	40-440%	55-419%	377-621%
$2x10^{13}$	4-6mo	n/a	n/a	n/a	n/a	560-1,549%

- ✓ NAGLU expression at or above heterozygote (and often above wild-type)
- ✓ Comparison of lifetime exposure at lower levels (as in heterozygotes) versus treated patients may not be relevant – because CNS and somatic tissues have abundant storage of GAGs that have to be cleared

MPS IIIB – 6 Month Toxicology Study in Non-Human Primates

IV delivery of rAAV9-CMV-hNAGLU in NHP

NHP	Age (y)	*AAV9 Abs (titer)	**IS	Vector dose (vg/kg)	Termination (pi)
Group 1					
NT1	10.3	1:64	-	Saline	6wk
S1	10.5	1:32	-	1E13	6wk
S2	11.0	Neg	-	1E13	3mo
Group 2					
NT-2	2.3	Neg	-	Saline	3mo
M1	2.3	Neg	-	2E13	3mo
M2	2.1	Neg	-	2E13	3mo
M3	2.0	1:16	+	2E13	3mo
M4	1.9	1:4	-	2E13	6mo
M5	1.8	1:32	-	2E13	6mo
M6	2.3	1:1000	+	2E13	6mo

*: Pre-existing Abs; **IS: immunosuppression with prednisolone.

- ✓ **Study Complete**
- ✓ **Single Intravenous Injection of rAAV9.CMV.Naglu**
- ✓ **Standard clinical and toxicology assessments**
- ✓ **No Adverse events observed**
- ✓ **No specific abnormalities in blood chemistry, hematology or histology**

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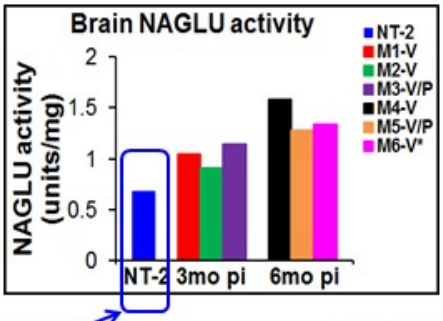
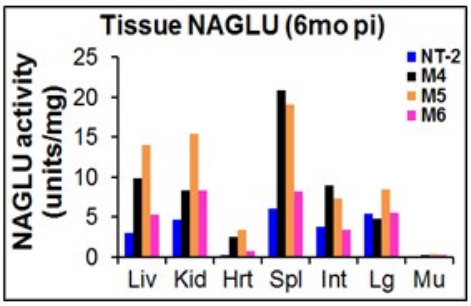
Persistence of NAGLU Expression in CNS at 6 Month in Non-Human Primate





Untreated, wild type animal

(2 yr old; 2 x 10^{13} vg/kg)

Brain and somatic NAGLU activity remains supraphysiologic at 6 months despite modest decreases in peripheral serum NAGLU levels

No Evidence for Autoimmune Responses in Non-Human Primates

✓ Antibody responses to human NAGLU was observed in NHP

However:

- No evidence for autoimmune pathology in any tissues at 6 months post-injection
- MPSIIIB mice – null for NAGLU – did not show evidence for clearance of transduced cells over two years despite Ab response
- No CTL responses by ELISpot in NHPs

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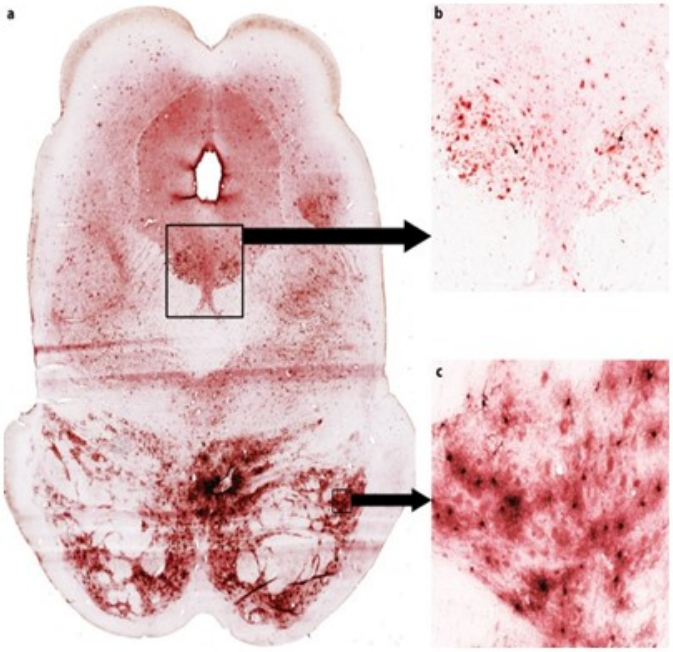
Systemic Delivery of scAAV9 is Broadly Distributed in the CNS of Non-Human Primates



A dose of scAAV9-CBA-GFP was injected into 3 year old cynomolgous macaque via saphenous vein.

GFP expression in the brain was assayed at 3 weeks post injection by immunohistochemistry in 40 um coronal sections.

Image from Foust and Kaspar, submitted

Red/brown staining = expression of scAAV9 transgene

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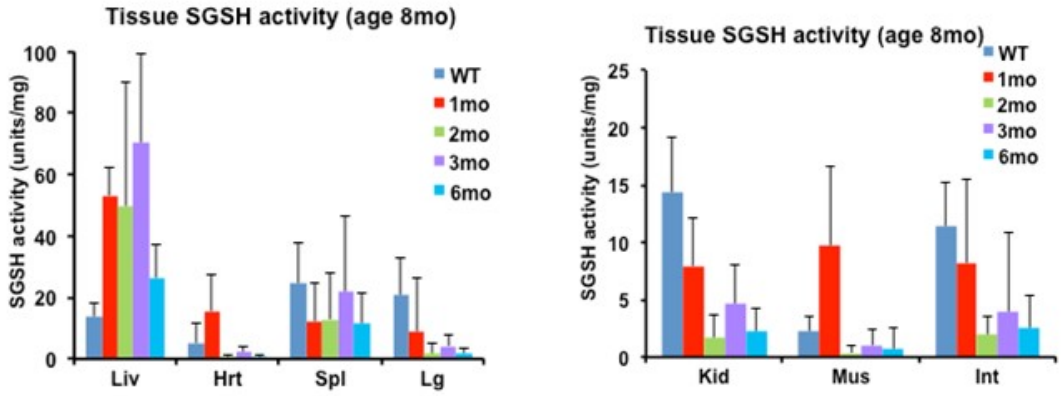
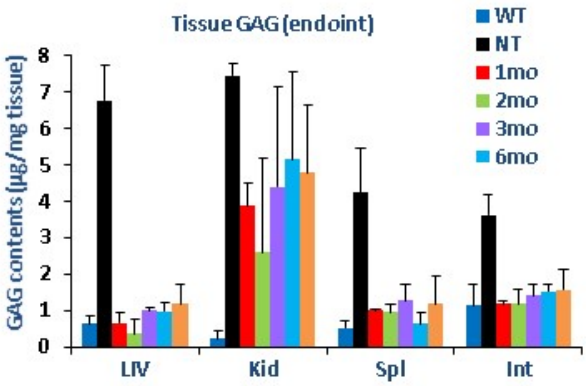
Intravenous Delivery of scAAV9-U1a-SGSH Increases SGSH Activity In MPS IIIA Treated Mice 8 Months Post-Treatment

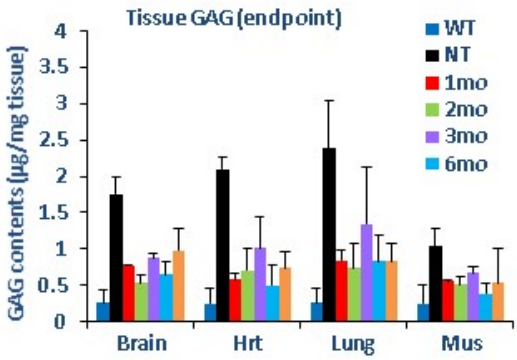


MPS IIIA mice were treated at the indicated ages (ie- "3 mo" indicates a 3 month old IIIA mouse treated with an IV dose of scAAV-U1a-SGSH) and the indicated tissues were tested for SGSH enzyme activity at 8 months age. WT = unaffected mice.

Lower bars are indicative of normalizing SGSH activity similar to unaffected mice. Data demonstrated that AAV9-SGSH delivered by IV are able to normalize SGSH activity in most of these tissues.

IV scAAV9-U1s-SGSH Reduces GAG Content in CNS and Peripheral Organs > 9 months

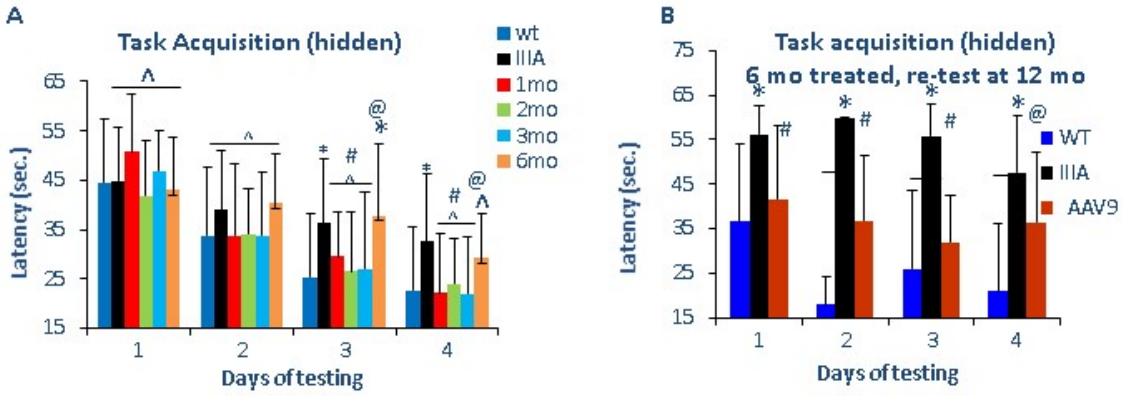




Mice were treated at the indicated ages (1-6 mo) with scAAV9-U1a-SGSH vector and the indicated tissues were tested for GAG content at >9 mo age. (n=3-6/group).

Data demonstrate that IV delivery of AAV9 vector is able to functionally normalize the GAG content in these tissues months after injection. WT = unaffected mice; NT = MPS IIIA mice that did not receive treatment.

IV Delivery of scAAV9-U1a-SGSH Corrects Behavioral Deficiency in MPS IIIA Mice with Beneficial Effects lasting 1 Year Post-Treatment

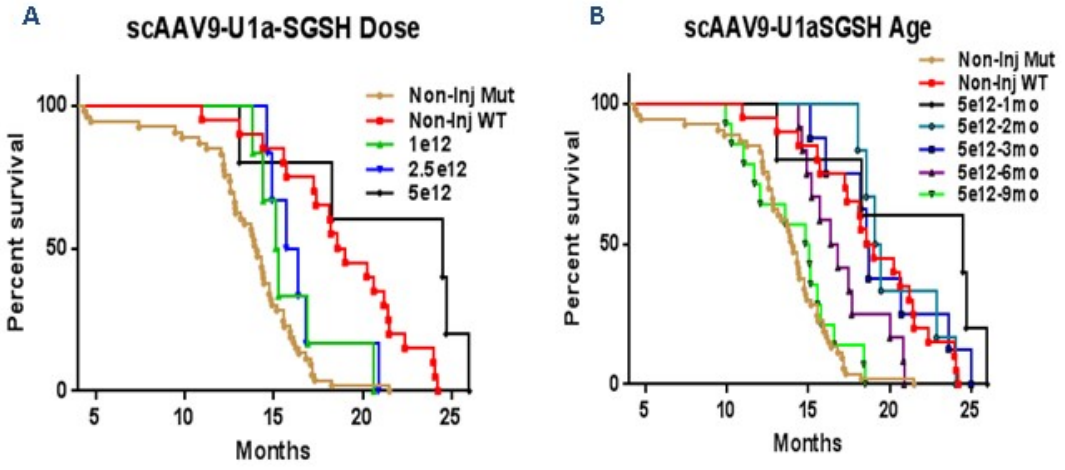


MPS IIIA mice and wt littermates were tested in the Morris water maze at 7.5 mo age for ability to find a hidden platform.

Mice were treated with vector at 1-6 mo age (A).

Mice treated at 6 mo age were re-tested at 12 mo age (B).

IV Delivery of scAAV9-UA1-SGSH Increases Survival of MPS IIIA Mice



Groups of MPS IIIA mice were treated at 1 mo age with different doses (A), or at different ages with vector (B) of scAAV9-U1a-SGSH vector by IV injection. Life spans were compared to wt or untreated MPS IIIA mice.

Preclinical Data Summary – IV Delivery of AAV9 Vectors For Treatment of MPS IIIA and MPS IIIB

- MPS III mice were treated at ages 1mo, 2mo, 3mo, 6mo and 9mo were treated with an intravenous injection of AAV9 vector carrying correct version of gene.
- The treatment led to the restoration of MPS III enzyme activity and reduction of GAG content to normal levels throughout the CNS and in somatic tissues in all treated groups.
- Treatment of MPS III Mice 1-6 months of age resulted in significant improvement in:
  - ✓ Cognitive performance in finding a hidden task in Morris water maze
  - ✓ Neuromuscular function (swimming ability)
  - ✓ Survival
- Results were diminished in animals treated at 9 mo of age.
- All preclinical studies (MPS IIIB mice, non-human primates, wt mice) for MPS IIIB are complete
- Final MPS IIIA GLP toxicology study in unaffected mice initiated
  - ✓ FDA will allow IND submission after 6 week data point

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MPS IIIA and MPS IIIB Natural History Study

- **Study Site – Nationwide Children's Hospital, Ohio, USA**
- **Enrollment complete: 25 subjects, 15 MPS IIIA and 10 MPS IIIB**
- FDA requested Natural History study to support clinical endpoints
- Study visits – assessments at Months 0, 6, and 12:
  - ✓ Neurocognitive (Leiter) and parental rating assessments (ABAS II)
  - ✓ Timed functional motor tests
  - ✓ Standard laboratory assessments
  - ✓ Serum/leukocyte NAGLU or SGSH activity
  - ✓ Quality of life (PedsQL)
  - ✓ Urine GAG levels
  - ✓ Overnight sleep actigraphy

All 25 patients will be tested for AAV9 neutralizing antibodies

- Study visits – assessments at Months 0 and 12:
  - ✓ Brain MRI (including DTI and ^{1}H spectroscopy)
  - ✓ CSF for standard chemistries/cell counts and NAGLU or SGSH activity
- 8 subjects through 6 month follow up appointments

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