

---

---

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**  
Washington, D.C. 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): **April 26, 2019**

**BioTime, Inc.**

(Exact name of registrant as specified in its charter)

**California**  
(State or other jurisdiction  
of incorporation)

**1-12830**  
(Commission  
File Number)

**94-3127919**  
(IRS Employer  
Identification No.)

**1010 Atlantic Avenue  
Suite 102  
Alameda, California 94501**  
(Address of principal executive offices)

**(510) 521-3390**  
(Registrant's telephone number, including area code)

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))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

---

---

**Item 8.01 - Other Events**

On April 26, 2019, BioTime announced that Dr. Edward D. Wirth, BioTime's Chief Medical Officer, was presenting at the 26<sup>th</sup> Annual American Society for Neural Therapy and Repair (ASNTR) Annual Conference on April 26, 2019 at 10:30am. Dr. Wirth's presentation, entitled "*Top-line 12-month Results from the SCiStar Study - A Phase 1/2a Trial of Human Embryonic Stem Cell-Derived Oligodendrocyte Progenitor Cells (OPC1) in Patients with Subacute Cervical Spinal Cord Injury*", is included as Exhibit 99.2 to this report, which is incorporated by reference.

**Item 9.01 – Financial Statements and Exhibits.**

(d) Exhibits.

<b>Exhibit Number</b>	<b>Description</b>
99.1	<a href="#">Press release dated April 26, 2019</a>
99.2	<a href="#">Presentation entitled "Top-line 12-month Results from the SCiStar Study - A Phase 1/2a Trial of Human Embryonic Stem Cell-Derived Oligodendrocyte Progenitor Cells (OPC1) in Patients with Subacute Cervical Spinal Cord Injury"</a>

---

**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.

BIOTIME, INC.

Date: April 29, 2019

By: /s/ Brian M. Culley

Brian M. Culley  
Chief Executive Officer

---





**BIOTIME TO PRESENT SCiSTAR CLINICAL STUDY TOP-LINE DATA AT THE 26<sup>TH</sup> ANNUAL AMERICAN SOCIETY FOR NEURAL THERAPY AND REPAIR CONFERENCE**

**ALAMEDA, CA – April 26, 2019 – BioTime, Inc.** (NYSE American and TASE: BTX), a clinical-stage biotechnology company developing new cellular therapies, announced today that Edward D. Wirth, III, M.D., Ph.D., Chief Medical Officer of BioTime, will present at the 26<sup>th</sup> Annual American Society for Neural Therapy and Repair (ASNTR) Annual Conference on April 26<sup>th</sup>, 2019 at 10:30am EDT part of Session 6: “*Spinal Cord Injury*”. Dr. Wirth’s presentation is entitled “*Top-line 12-month Results from the SCiStar Study - A Phase 1/2a Trial of Human Embryonic Stem Cell-Derived Oligodendrocyte Progenitor Cells (OPC1) in Patients with Subacute Cervical Spinal Cord Injury*”. ASNTR will be held April 25 – 27, 2019 at the Sheraton Sand Key Resort in Clearwater Beach, Florida.

“We believe the primary goals of the SCiStar Study, which were to observe the safety of OPC1 in cervical spinal cord injury patients as well as other important metrics including related to the optimal timing of OPC1 injection, tolerability of the immunosuppression regimen, engraftment of OPC1 cells, and rates of motor recovery observed among different study subpopulations, have all been successfully achieved,” stated Dr. Wirth. “We now are in the process of analyzing the full data set from the SCiStar Study to inform how best to proceed with this promising program. We expect to propose a clinical plan to the U.S. Food and Drug Administration later this year and expect to share the outcome of those discussions when they are available.”

“We appreciate the support of the California Institute for Regenerative Medicine, the world’s largest institution dedicated to bringing the future of cellular medicine closer to reality, whose generous grant funding to date of \$14.3 million has helped advance the clinical development of our OPC1 program and generate these encouraging clinical results in patients with traumatic spinal cord injuries,” stated Brian M. Culley, Chief Executive Officer of BioTime. “We look forward to continuing our partnership with CIRM and will support their mission to accelerate stem cell treatments to patients with unmet medical needs and fast-track the development of the most promising stem cell technologies.”

The SCiStar Study is an open-label, single-arm trial testing three sequential escalating doses of OPC1 administered 21 to 42 days post-injury, at up to 20 million OPC1 cells in 25 subjects with subacute motor complete (AIS-A or AIS-B) cervical (C-4 to C-7) acute spinal cord injuries (SCI). These individuals have essentially lost all movement below their injury site and experience severe paralysis of the upper and lower limbs. AIS-A subjects have lost all motor and sensory function below their injury site, while AIS-B subjects have lost all motor function but may have retained some minimal sensory function below their injury site. The primary endpoint in the SCiStar study was safety as assessed by the frequency and severity of adverse events related to OPC1, the injection procedure, and immunosuppression with short-term, low-dose tacrolimus. Secondary outcome measures included neurological functions as measured by upper extremity motor scores and motor level on International Standards for Neurological Classification of Spinal Cord Injury (ISNCSCI) examinations at 30, 60, 90, 180, 270, and 365 days after injection of OPC1.

Below are a summary of key findings from the SCiStar Study. A copy of Dr. Wirth’s presentation will be available on the Events section of BioTime’s website concurrent with his presentation at ASNTR.

---

- **Overall safety profile of OPC1 to date is excellent**
  - Magnetic resonance imaging (MRI) scans at 12 months post-injection of OPC1 has shown no evidence of adverse changes in any of the 25 SCiStar study subjects treated with OPC1.
  - To date, there have been no unexpected serious adverse events (SAEs) related to the OPC1 cells.
  - No concerning safety issues and no intraoperative complications have been noted
  - No SCiStar study subjects had worsening of neurological function post-injection
  - No adverse findings observed on follow-up MRI scans
  - Immunosuppression with tacrolimus (an immunosuppressive drug utilized mainly after allogeneic organ transplant to lower the risk of organ rejection) was well-tolerated
  
- **Majority of SCiStar subjects who received 10M or 20M OPC1 cells exhibited robust motor recovery in upper extremities**
  - Three subjects (Cohort 1) received a sub-therapeutic dose of 2M cells to evaluate the initial safety of injecting OPC1 into lesions in the cervical spinal cord. All other subjects (Cohorts 2-5) received 10M or 20M cells.
  - At 12 months, 95% (21/22) of SCiStar study subjects in Cohorts 2-5 recovered at least one motor level on at least one side and 32% (7/22) of these subjects recovered two or more motor levels on at least one side. The average improvement in upper extremity motor score as measured by the ISNCSCI scale for these subjects was 8.9 points.
  - Notably, no SCiStar study subjects saw decreased motor function following administration of OPC1 and subjects either retained for 12 months the motor function recovery seen through 6 months or experienced further motor function recovery from 6 to 12 months.
  
- **MRI scans consistent with durable engraftment through 1 year post-injection**
  - All three SCiStar study subjects in Cohort 1 and 95% (21/22) of SCiStar study subjects in Cohorts 2 to 5 have MRI scans at 12 months consistent with the formation of a tissue matrix at the injury site, which is encouraging evidence that OPC1 cells have engrafted at the injury site and helped to prevent cavitation, a destructive process that occurs within the spinal cord following spinal cord injuries, and typically results in permanent loss of motor and sensory function.

## About OPC1

OPC1 is an oligodendrocyte progenitor cell (OPC) therapy currently being tested in a Phase I/IIa clinical trial known as SCiStar for the treatment of acute spinal cord injuries. OPCs are naturally-occurring precursors to the cells which provide electrical insulation for nerve axons in the form of a myelin sheath. SCI occurs when the spinal cord is subjected to a severe crush or contusion injury and typically results in severe functional impairment, including limb paralysis, aberrant pain signaling, and loss of bladder control and other body functions. The clinical development of the OPC1 program has been partially funded by a \$14.3 million grant from the [California Institute for Regenerative Medicine](#). OPC1 has received Regenerative Medicine Advanced Therapy (RMAT) designation for the treatment of acute SCI and has been granted Orphan Drug designation from the U.S. Food and Drug Administration (FDA).

## About BioTime, Inc.

BioTime is a clinical-stage biotechnology company developing new cellular therapies for degenerative retinal diseases, neurological conditions associated with demyelination, and aiding the body in detecting and combating cancer. BioTime's programs are based on its proprietary cell-based therapy platform and associated development and manufacturing capabilities. With this platform BioTime develops and manufactures specialized, terminally-differentiated human cells from its pluripotent and progenitor cell starting materials. These differentiated cells are developed either to replace or support cells that are dysfunctional or absent due to degenerative disease or traumatic injury, or administered as a means of helping the body mount an effective immune response to cancer. BioTime's clinical assets include (i) OpRegen<sup>®</sup>, a retinal pigment epithelium transplant therapy in Phase I/IIa development for the treatment of dry age-related macular degeneration, the leading cause of blindness in the developed world; (ii) OPC1, an oligodendrocyte progenitor cell therapy in Phase I/IIa development for the treatment of acute spinal cord injuries; and (iii) VAC2, an allogeneic cancer immunotherapy of antigen-presenting dendritic cells currently in Phase I development for the treatment of non-small cell lung cancer. For more information, please visit [www.biotimeinc.com](http://www.biotimeinc.com).

---

## Forward-Looking Statements

BioTime cautions you that all statements, other than statements of historical facts, contained in this press release, are forward-looking statements. Forward-looking statements, in some cases, can be identified by terms such as “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “design,” “intend,” “expect,” “could,” “plan,” “potential,” “predict,” “seek,” “should,” “would,” “contemplate,” “project,” “target,” “tend to,” or the negative version of these words and similar expressions. Such statements include, but are not limited to, statements relating to the timing of when we propose a clinical plan to the U.S. Food and Drug Administration and the sharing of the outcome of those discussions when they are available, and that MRI results are supportive evidence showing that OPC1 cells have durably engrafted to help prevent cavitation at the injury site. Forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause BioTime’s actual results, performance or achievements to be materially different from future results, performance or achievements expressed or implied by the forward-looking statements in this press release, including, without limitation, risk and uncertainties related to: BioTime’s ability to raise additional capital when and as needed, to advance its product candidates; BioTime’s ability to develop and commercialize product candidates; the failure or delay in starting, conducting and completing clinical trials or obtaining FDA or foreign regulatory approval for BioTime’s product candidates in a timely manner; the therapeutic potential of BioTime’s product candidates, and the disease indications for which BioTime intends to develop its product candidates; BioTime’s ability to conduct and design successful clinical trials, to enroll a sufficient number of patients, to meet established clinical endpoints, to avoid undesirable side effects and other safety concerns, and to demonstrate sufficient efficacy of its product candidates; developments by BioTime competitors that make BioTime’s product candidates less competitive or obsolete; BioTime’s ability to manufacture its product candidates for clinical development and, if approved, for commercialization, and the timing and costs of such manufacture; the performance of third parties in connection with the development and manufacture of BioTime’s product candidates, including third parties conducting clinical trials as well as third-party suppliers and manufacturers; the potential of BioTime’s cell therapy platform, and BioTime’s plans to apply its platform to research, develop and commercialize our product candidates; BioTime’s ability, and the ability of its licensors, to obtain, maintain, defend and enforce intellectual property rights protecting BioTime’s product candidates, and BioTime’s ability to develop and commercialize its product candidates without infringing the proprietary rights of third parties; BioTime’s ability to recruit and retain key personnel; and BioTime’s ability to successfully integrate the operations of Asterias into BioTime. BioTime’s forward-looking statements are based upon its current expectations and involve assumptions that may never materialize or may prove to be incorrect. All forward-looking statements are expressly qualified in their entirety by these cautionary statements. For a detailed description of BioTime’s risks and uncertainties, you are encouraged to review its documents filed with the SEC including its recent filings on Form 8-K, Form 10-K and Form 10-Q. You are cautioned not to place undue reliance on forward-looking statements, which speak only as of the date on which they were made. BioTime undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made, except as required by law.

### **BioTime Inc. IR**

Ioana C. Hone

([ir@biotimeinc.com](mailto:ir@biotimeinc.com))

(510) 871-4188

### **Solebury Trout IR**

Gitanjali Jain Ogawa

([Gogawa@troutgroup.com](mailto:Gogawa@troutgroup.com))

(646) 378-2949

###

---







**NYSE American: BTX**

**Top-line 12-month Results from the SCiStar Study - A Phase 1/2a  
Trial of Human Embryonic Stem Cell-Derived Oligodendrocyte  
Progenitor Cells (OPC1) in Patients with Subacute Cervical Spinal  
Cord Injury**

*Edward D. Wirth, III, MD, PhD  
ASNTR 26<sup>th</sup> Annual Conference*

**April 26, 2019**

# Forward Looking Statements

This presentation is for informational purposes only and is not an offer to sell or a solicitation of an offer to buy any securities of BioTime, Inc. (“BioTime” or the “Company”). This presentation includes certain information obtained from trade and statistical services, third party publications, and other sources. BioTime has not independently verified such information and there can be no assurance as to its accuracy.

All statements in this presentation, other than statements of historical fact, are forward-looking statements within the meaning of federal securities laws. In some cases, you can identify forward-looking statements by terms such as “may,” “will,” “expect,” “plan,” “anticipate,” “strategy,” “designed,” “could,” “intend,” “believe,” “estimate,” “target,” or “potential” and other similar expressions, or the negative of these terms. Forward-looking statements include, without limitation, statements regarding the potential line extensions in other neurodegenerative diseases, that MRI results are supportive evidence showing that OPC1 cells have durably engrafted to help prevent cavitation at the injury site, and the ability to successfully address in future studies issues that may negatively impact motor recovery. Forward-looking statements involve risks, uncertainties and assumptions that may cause BioTime’s actual results, performance or achievements to be materially different from those expressed or implied by the forward-looking statements, including, without limitation risks and uncertainties relating to: BioTime’s ability to develop new cell product candidates on the timelines discussed; the outcome or success of clinical trials; BioTime’s ability to obtain FDA and/or foreign regulatory approval for product candidates; competition from products manufactured and sold or being developed by other companies; the price of and demand for BioTime’s products; ability to obtain and maintain intellectual property protection for BioTime’s product candidates; BioTime’s ability to access adequate capital to fund current and planned business and operations; expected synergies and benefits of the Asterias acquisition; and other risk factors described in BioTime’s most recent annual report on Form 10-K and subsequent quarterly reports on Form 10-Q and current reports on Form 8-K filed with the Securities and Exchange Commission.

BioTime cautions you not to place undue reliance on any forward-looking statement. All forward-looking statements in this presentation are current only as of the date hereof and BioTime does not undertake any obligation to update any forward-looking statement to reflect new information, future developments or otherwise, except as required by law.

# OPC1: hESC-Derived Oligodendrocyte Progenitor Cells (OPCs)

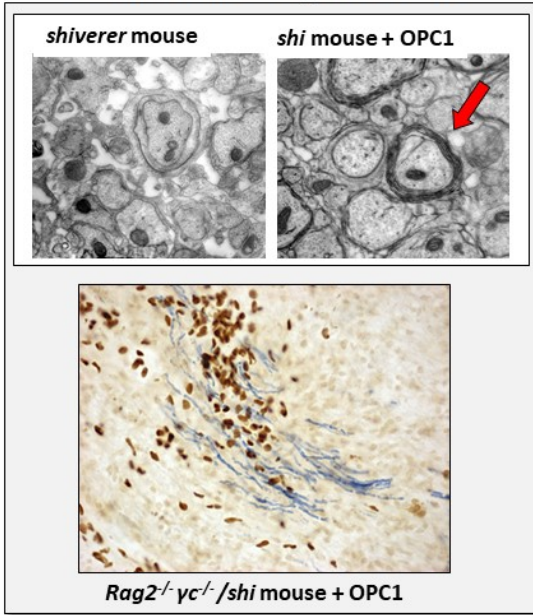


## OPC1

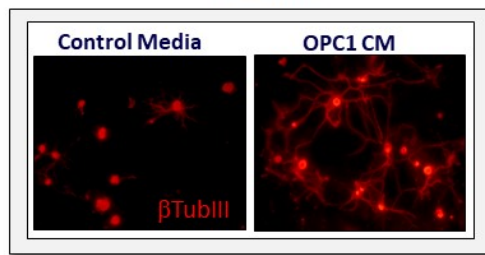
- Cryopreserved Allogeneic Cell Population
- Derived from an NIH-Registered Human Embryonic Stem Cell line (hESC)
- Characterized Composition of Cells:
  - Oligodendrocyte progenitors
  - Neural progenitors
  - Infrequent mature neural cells and
  - Rare other characterized cell types
- Three identified functions
  - Produces neurotrophic factors
  - Induces remyelination
  - Induces vascularization
- “Off the shelf” administration
- First indication: spinal cord injury
- Potential line extensions in other neurodegenerative diseases

# OPC1: Three Major Physiologically Relevant Functional Activities

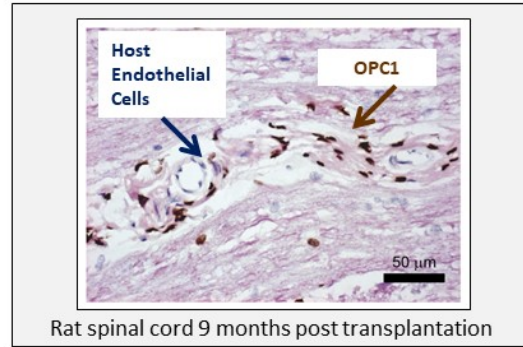
1. Wraps host neurons and forms compact myelin sheaths\*



2. Produces neurotrophic factors and stimulates neurite outgrowth\*\*



3. Stimulates neovascularization\*

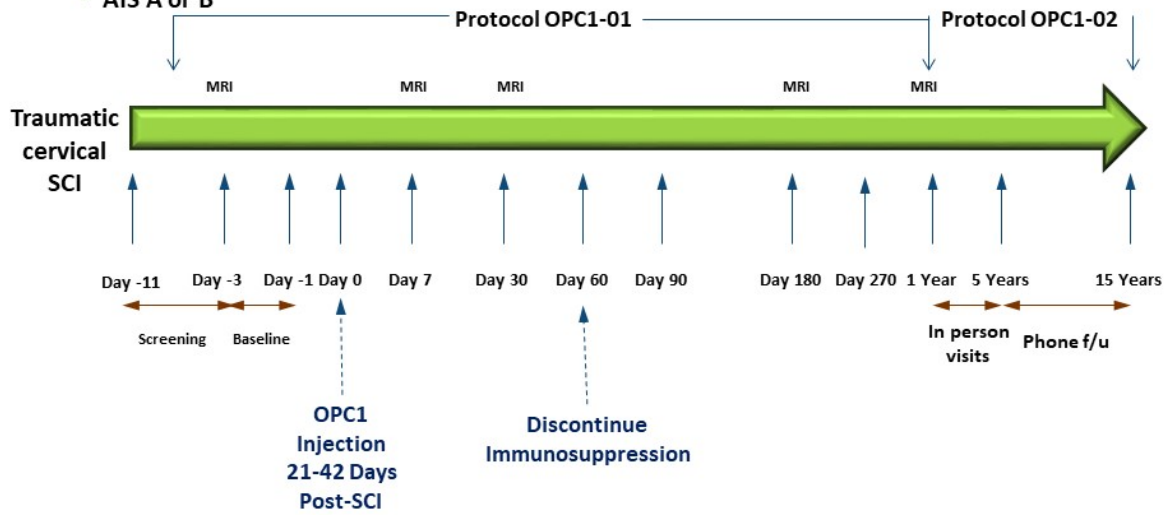


# SCiStar Study Schema

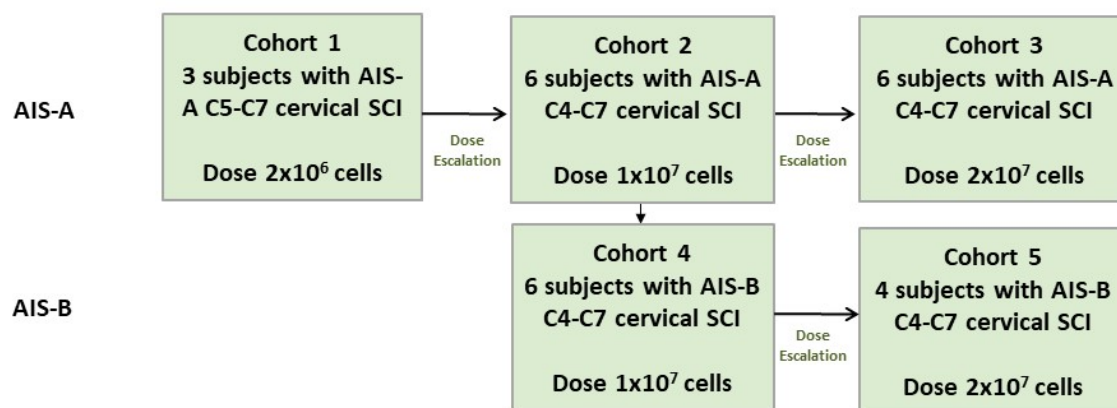
- Open Label Trial
- 21-42 days post SCI
- Traumatic cervical SCI (C4-C7)
- Ages 18-69
- AIS A or B



Primary Assessment: Safety  
 Secondary Assessment: Neurological Function (ISNCSCI exams)  
 Exploratory Assessments: SCIM, GRASSP



# SCiStar Study Enrollment & Cohort Progression



# Follow-Up Status for SciStar Study

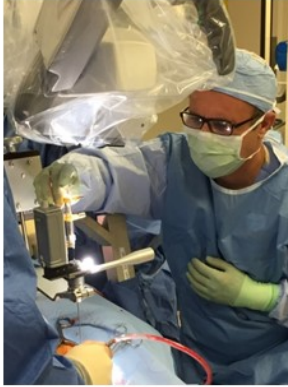
Cohort	# Subjects Administered OPC1	# Subjects with 12 Months Follow-Up	# Subjects with 2 Years LTFU	# Subjects with 3 Years LTFU
Safety Cohort 1 AIS-A 2x10 <sup>6</sup> Dose	3	3	3	3
Safety and Efficacy Cohort 2 AIS-A 1x10 <sup>7</sup> Dose	6	6	6	2
Safety and Efficacy Cohort 3 AIS-A 2x10 <sup>7</sup> Dose	6 <sup>a</sup>	6	2	-
Safety and Efficacy Cohort 4 AIS-B 1x10 <sup>7</sup> Dose	6	6	2	-
Safety and Efficacy Cohort 5 AIS-B 2x10 <sup>7</sup> Dose	4 <sup>b</sup>	4	-	-

<sup>a</sup> One subject enrolled in Cohort 3 received only the 1 x 10<sup>7</sup> dose due to an error during dose preparation

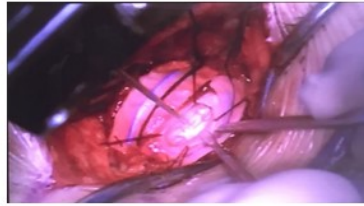
<sup>b</sup> One subject enrolled in Cohort 5 received only the 1 x 10<sup>7</sup> dose due to a very small spinal cord lesion

# OPC1 Injection Procedure

Shepherd Center



Rush University



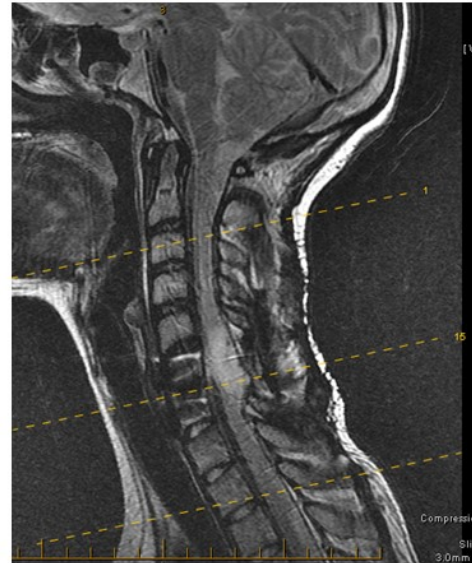
- Injections performed using a table-mounted syringe positioning device (SPD)
- Direct intra-parenchymal injection into the spinal cord lesion
- Single 50 $\mu$ L injection for both the 2M & 10M doses; Two injections for the 20M dose
- No intraoperative complications



## 12 Month MRI Results Support Durable Engraftment of OPC1 Cells

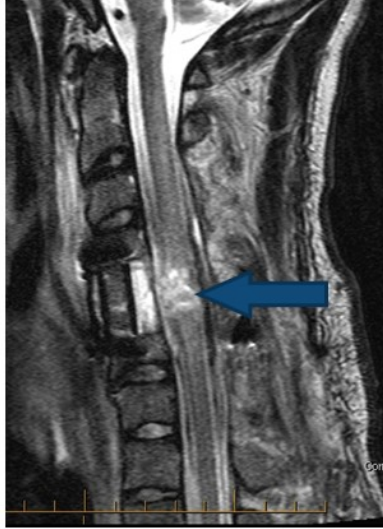
- Cavitation is estimated to occur in ~80% of spinal cord injury patients meeting SCiStar Study inclusion criteria
- 96% (24/25) of subjects had serial MRI scans at 12 months that indicated no sign of a lesion cavity
- The MRI results are consistent with formation of a tissue matrix at injury site, which we believe is supportive evidence showing that OPC1 cells have durably engrafted to help prevent cavitation at the injury site<sup>(1)</sup>

Cohort 2 subject  
365 Day T2 –weighted sagittal MRI



# Some Lesions May Be Too Severe for OPC1 Cell Survival

Pre-Injection Baseline



Day 365 Post-Injection



Failed  
graft with  
lesion  
cavity  
formation

- Large hematoma in spinal cord
- Most severe lesion at baseline
- Least favorable environment for survival of OPC1 cells

# SCiStar Study Primary Endpoint: Safety

## Tolerability Also Assessed

- **SCiStar Study primary endpoint was met:**
  - **Safety assessed by the frequency and severity of adverse events (AE) related to OPC1, the injection procedure, and immunosuppression with short-term, low-dose tacrolimus**
- **No concerning safety issues have been noted**
- **No intraoperative complications**
- **Immunosuppression with tacrolimus was well-tolerated**
- **No serious adverse events (SAEs) related to OPC1**
- **No subjects had worsening of neurological function post-injection**
- **No adverse findings on follow-up MRI scans**

# SCiStar Study Summary of Adverse Events

- Majority of SCiStar Study adverse events were mild to moderate in severity

All Treated Subjects (n=25)	AEs	SAEs
Total	534	29
Mild (Grade 1)	343	0
Moderate (Grade 2)	161	15
Severe (Grade 3)	30	14
Life threatening (Grade 4)	0	0
Death (Grade 5)	0	0
Related to OPC1*	1	0
Related to Injection Procedure	20	1
Related to Tacrolimus	11	1

\* The AE that was possibly related to OPC1 was a Grade 2 dysesthesia (unpleasant, abnormal sensation) that began 47 days post-injection and resolved by the Year 2 follow-up visit

## SCiStar Study Most Common SAEs

All Treated Subjects (N=25)	SAE #	Causality
<b>Total</b>	<b>29</b>	27 not related
<b>UTI</b>	<b>7</b>	Not related
<b>Urinary Sepsis</b>	<b>2</b>	Not related
<b>Pulmonary Embolus</b>	<b>2</b>	Not related
<b>Mental Status Changes</b>	<b>2</b>	Not related
<b>CSF Fluid Leak</b>	<b>1</b>	Related to Injection Procedure
<b>Infected Epidural Fluid</b>	<b>1</b>	Related to Tacrolimus

## SCiStar Study Top 5 Most Common AEs

All Treated Subjects (N=25)	AE #	Causality*
<b>Total</b>	<b>534</b>	402 not related AEs
UTI	60	60 not related
Decubitus Ulcer	37	37 not related
Hypokalemia	24	24 not related
Hypomagnesemia	16	8 related to tacrolimus
Headache	15	2 related to injection procedure

- \*Related to either OPC1 or injection procedure or tacrolimus
- Majority of AEs were mild to moderate in severity

# Neurological Function Assessment: International Standards for Neurological Classification of Spinal Cord Injury (ISNCSCI)

**INTERNATIONAL STANDARDS FOR NEUROLOGICAL CLASSIFICATION OF SPINAL CORD INJURY (ISNCSCI)**

Patient Name \_\_\_\_\_ Date/Time of Exam \_\_\_\_\_  
 Examiner Name \_\_\_\_\_ Signature \_\_\_\_\_

**RIGHT**

MOTOR KEY MUSCLES	SENSORY KEY SENSORY POINTS	
	Light Touch (L)	Pin Prick (P)
C2		
C3		
C4		
C5		
C6		
C7		
C8		
T1		
T2		
T3		
T4		
T5		
T6		
T7		
T8		
T9		
T10		
T11		
T12		
L1		
L2		
L3		
L4		
L5		
S1		
S2		
S3		
S4-5		

**UER** (Upper Extremity Right) **UEL** (Upper Extremity Left)  
 Elbow flexors C5  
 Wrist extensors C6  
 Elbow extensors C7  
 Finger flexors C8  
 Finger abductors (5th finger) T1

**LER** (Lower Extremity Right) **LEL** (Lower Extremity Left)  
 Hip flexors L2  
 Knee extensors L3  
 Ankle dorsiflexors L4  
 Long toe extensors L5  
 Ankle plantar flexors S1

(VAC) Voluntary anal contraction (Yes/No)  S4-5

RIGHT TOTALS: (56) (56) (56)

• Key Sensory Points

**LEFT**

MOTOR KEY MUSCLES	SENSORY KEY SENSORY POINTS	
	Light Touch (L)	Pin Prick (P)
C2		
C3		
C4		
C5		
C6		
C7		
C8		
T1		
T2		
T3		
T4		
T5		
T6		
T7		
T8		
T9		
T10		
T11		
T12		
L1		
L2		
L3		
L4		
L5		
S1		
S2		
S3		
S4-5		

**UEL** (Upper Extremity Left) **LER** (Lower Extremity Right)  
 Elbow flexors C5  
 Wrist extensors C6  
 Elbow extensors C7  
 Finger flexors C8  
 Finger abductors (5th finger) T1

**LEL** (Lower Extremity Left) **LER** (Lower Extremity Right)  
 Hip flexors L2  
 Knee extensors L3  
 Ankle dorsiflexors L4  
 Long toe extensors L5  
 Ankle plantar flexors S1

(DAP) Deep anal pressure (Yes/No)

LEFT TOTALS: (56) (56) (50)

**MOTOR SUBSCORES:** UER  + UEL  = UEMS TOTAL  (MAX 25) (25)  
 LER  + LEL  = LEMS TOTAL  (MAX 25) (25)

**SENSORY SUBSCORES:** RLT  + LLT  = RT TOTAL  (MAX 56) (56)    RPP  + LPP  = RP TOTAL  (MAX 56) (56)  
 RLTT  + LLTT  = LT TOTAL  (MAX 112) (112)    RPP  + LPP  = PP TOTAL  (MAX 112) (112)

**NEUROLOGICAL LEVELS:** 1. SENSORY  R  L   
 2. MOTOR  R  L

3. NEUROLOGICAL LEVEL OF INJURY (NLI)

4. COMPLETE OR INCOMPLETE?  (In complete injuries only)  
 Incomplete = Any sensory or motor function in S4-5

5. ASIA IMPAIRMENT SCALE (AIS)

ZONE OF PARTIAL PRESERVATION: SENSORY  R  L   
 MOTOR  R  L

This form may be copied freely but should not be altered without permission from the American Spinal Injury Association. REV 04/03

## Upper Extremity Motor Score (UEMS) Recovery in Cohorts 2-5

	+2 Motor Levels		UEMS Improvement	
	6 Months	12 Months	6 Months	12 Months
Cohort 2	2/6	4/6	9.7	12.3
Cohort 3	1/6	1/6	6.0	9.2
Cohort 4	1/6	1/6	5.5	6.7
Cohort 5	0/4	1/4	5.8	6.8
Cohorts 2-5	4/22	7/22	6.8	8.9 +/- 4.2

➤ Next step was to look all 22 subjects ranked by UEMS improvement



# Subjects with the Least Motor Recovery (Cohorts 2-5)

Subject	UEMS Change from Baseline to 12 mo	2 Motor level Gain Y/N	Cohort	Dose	Lot	Age	Injection Date Days from Injury	Baseline AIS	NLI Baseline
2207	7	N	5	20 million	M22	62	37	B	C4
2203	6	N	3	20 million	M25	45	31	A	C6
2105	6	N	3*	10 million	M25	19	20	A	C4
2004	5	N	4	10 million	M25	21	25	B	C6
2007	4	N	4	10 million	M22	55	38	B	C4
2307	4	N	5*	10 million	M22	19	38	B	C5
2303	3	N	4	10 million	M25	22	35	B	C6

- \* As previously noted on Slide 7, these two subjects received 10 million cells rather than the planned 20 million cells for cohorts 3 and 5
- Two subjects had cord compression after OPC1 injection (Subject 2307 at Day 7; Subject 2303 at Day 30)
- Subjects 2207, 2105, & 2007 had a C4 NLI (lowest intact neurological level) at Baseline
- Subject 2105 also had hematoma in spinal cord at baseline & failed OPC1 graft
- Subject 2004 focused on lower extremity rehab; regained normal bowel function

# SCiStar Study Preliminary Subset Analysis

Cohort	N	2 ML Gain	Mean UEMS Gain	Comments
Cohort 2	6	4	12.3	
Cohort 3	5	1	8.8	2105 had C4 NLI*, severe lesion and failed graft
Cohort 4	4	1	8.0	2303 had cord compression at Day 30 2007 had C4 NLI
Cohort 5	2	1	8.5	2307 had cord compression at Day 7 2207 had C4 NLI
Total	17	7	10.2 +/- 3.9	Mean is still 10.2 even if 2103 (C4 NLI in Cohort 2) is excluded

\* Neurological Level of Injury (NLI) is the lowest level with intact motor and sensory function

## SCiStar Study Evaluation of Change in UEMS (12 Months Post-Injection Versus Key Variables)

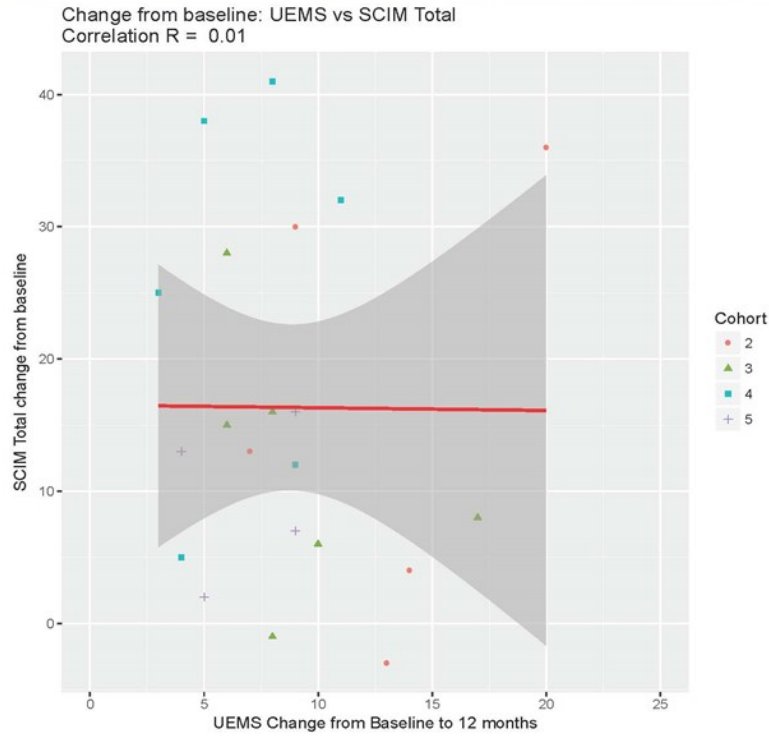
- Analysis performed for all 22 subjects in Cohorts 2-5 (except for Baseline NLI, which was only analyzed for subjects with a baseline NLI of C5, C6 or C7)

Key Variable	Correlation with UEMS Change from Baseline to 12 months
Age	p = 0.95
Gender	P = 0.86
Baseline AIS Grade	P = 0.02 (Better for AIS-A due to Cohort 2)
Baseline NLI (C5-C7)	C5: P = 0.22 C6: p = 0.39 C7: p = 0.13
Dose (10M or 20M cells)	P = 0.94
Number of days from SCI to OPC1 injection	P = 0.25
Manufacturing Lot of OPC1 (21 of 22 subjects received cells from Lot A or Lot B)	Lot A (n=7): P = 0.41 Lot B (n=14): P = 0.76

# Exploratory Assessment: Spinal Cord Independence Measure (SCIM)

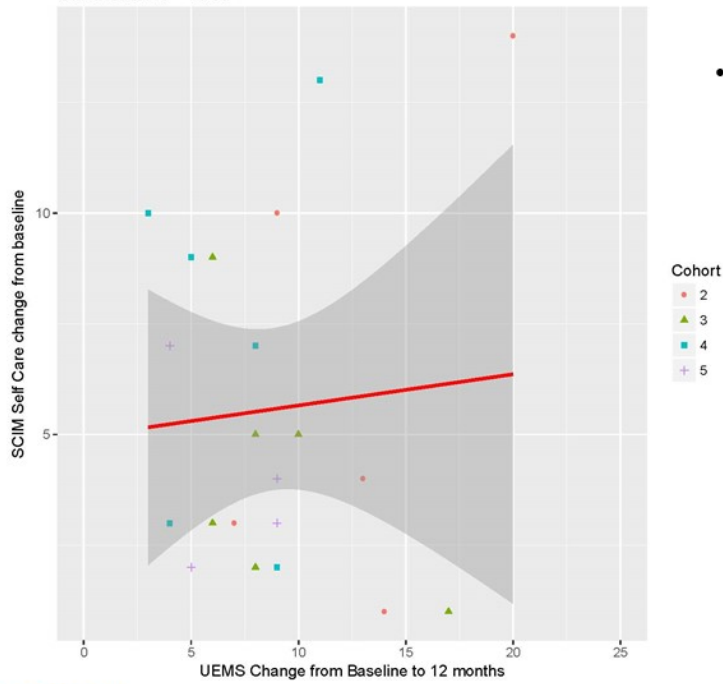
- SCIM is a “global” functional activities exam within the activity/function domain (does not cover pain, spasticity, or autonomic functions)
- Maximum score of 100 comes from 3 sub-scales within SCIM (self-care = 20 points, respiration and sphincter management = 40, mobility = 20)
- SCIM Self-care sub-score focuses on upper extremity activities and is therefore linked to assessments of cervical SCI
  - Feeding (3 pts)
  - Bathing: Upper body (3 pts), Lower body (3 pts)
  - Dressing: Upper body (4 pts), Lower body (4 pts)
  - Grooming (3 pts)

# Change in SCIM Total Score did not correlate with UEMS



# Change in SCIM Self-Care Subscore is Poorly Correlated with UEMS

Change from baseline: UEMS vs SCIM Self Care  
Correlation R = 0.08



- Will review with reordered SCIM data per the Rasch analysis used to develop the Spinal Cord Ability Ruler (SCAR)\* outcome measure to assess utility of SCIM in subsequent trials

# Summary

- The overall safety profile of OPC1 to date is excellent, and immunosuppression with tacrolimus was well-tolerated
- MRI scans are consistent with a very high rate (96%) of durable engraftment through 1 year post-injection
- Majority of subjects who received 10M or 20M OPC1 cells exhibited robust motor recovery in the upper extremities
  - In addition, 21/22 (95%) of subjects in Cohorts 2-5 improved at least 1 motor level on at least 1 side
- Two issues (C4 NLI; postop cord compression) that may negatively impact motor recovery are believed to be addressable in future studies
- These encouraging engraftment & motor recovery data warrant further evaluation in randomized, controlled studies
- Data from the SCiStar Study will help inform the design of future randomized studies with respect to inclusion/exclusion criteria, dose, and timing of administration

# Acknowledgments

## Asterias\*

Jane Lebkowski  
Katy Spink  
Susy Chen  
Maria Schaefer  
Sherin Halfon  
Oliver Tilk  
Rekha Nair  
Maggie Morysewicz  
Kevin Nishimoto  
Naomi Kautz  
OPC1 Team

## The Trial Participants

## Committees & Consultants

Data Monitoring Committee  
John Steeves  
Munish Mehra  
Mike Walker

## Clinical Investigators

Don Leslie  
Kevah Khajavi  
Richard Fessler  
James Young  
Steve McKenna  
Gary Steinberg  
Marco Lee  
Charles Liu  
Eric Horn  
Shekar Kurpad

## Funding

**CIRM**  
CALIFORNIA'S STEM CELL AGENCY





NYSE American: BTX

---

