






AST-OPC1 SCiStar Study  
Efficacy & Safety Update  
*January 24<sup>th</sup>, 2017*



# Enrollment Progress/Anticipated Completion

-  Dosing complete
-  Currently enrolling
-  Future enrollment

## AIS-A Cohorts

Cohort 1 – 2 million  
3 subjects

- Enrollment completed in August 2015

Cohort 2 – 10 million  
6 subjects

- Enrollment of 5th Subject completed in July 2016
- 6th subject dosed
- Five subjects have completed the 6 month visit & three of these subjects have also completed the 9 month visit

Cohort 3 – 20 million  
5-8 subjects

- First two subjects dosed
- Enrollment expected to be completed late Q1 2017
- 6 month data expected late Q3 2017

## AIS-B Cohorts

Cohort 4 – 10 million  
5-8 subjects

- First two subjects dosed
- Enrollment expected to be completed late Q1 2017
- 6 month data expected late Q3 2017

Cohort 5 – 20 million  
5-8 subjects

- Enrollment expected to be completed in Q3 2017
- 6 month data expected in Q1 2018

# Motor Recovery of Subjects Receiving AST-OPC1 Compared to That of Closely Match Historical Controls

## Matched controls

- Subset of SCI patients in the **EMSCI database\*** identified meeting matching criteria
- Time frames matched to baseline assessments of those in the SCiStar trial

\* **EMSCI** ([www.emsci.org](http://www.emsci.org)) is the most complete and most current SCI database available for comparison (> 3300 patients, ~300 new patients added annually)

- Actively managed database
- Best available ISNCSCI dataset

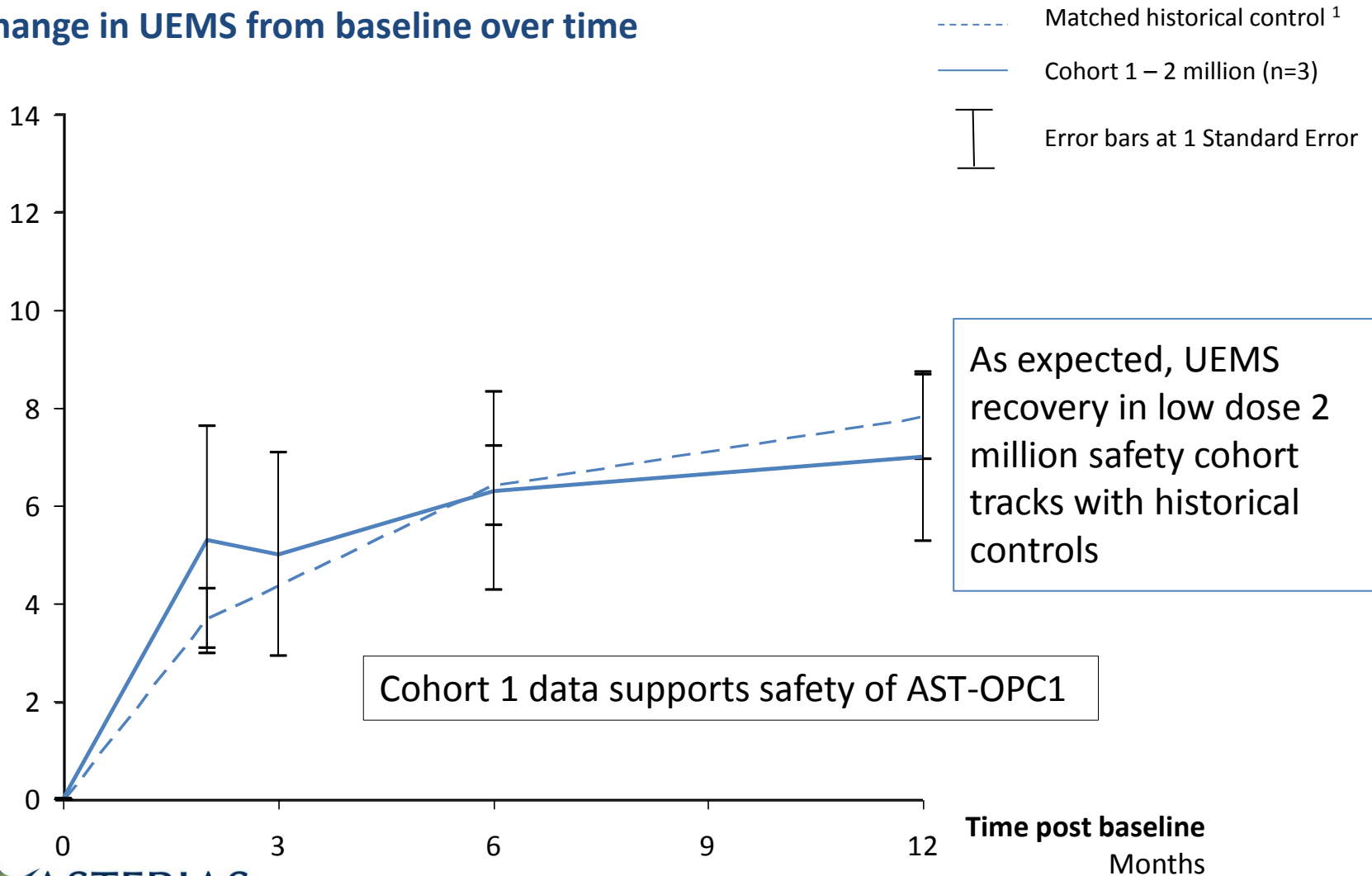
## Matching criteria

- Traumatic injury
- Baseline assessment between 16-40 days from injury
- AIS A at baseline
- Age 18-69
- NLI of C5-C7 at baseline
- UEMS at baseline 7-32

**Baseline includes 73 matched patients across multiple time points**

# Low Dose 2 Million Cell Cohort Has Motor Recovery Similar to Matched Historical Controls

## Change in UEMS from baseline over time



# Subjects Receiving 10 million AST-OPC1 Have Improved Motor Function as Measured by UEMS

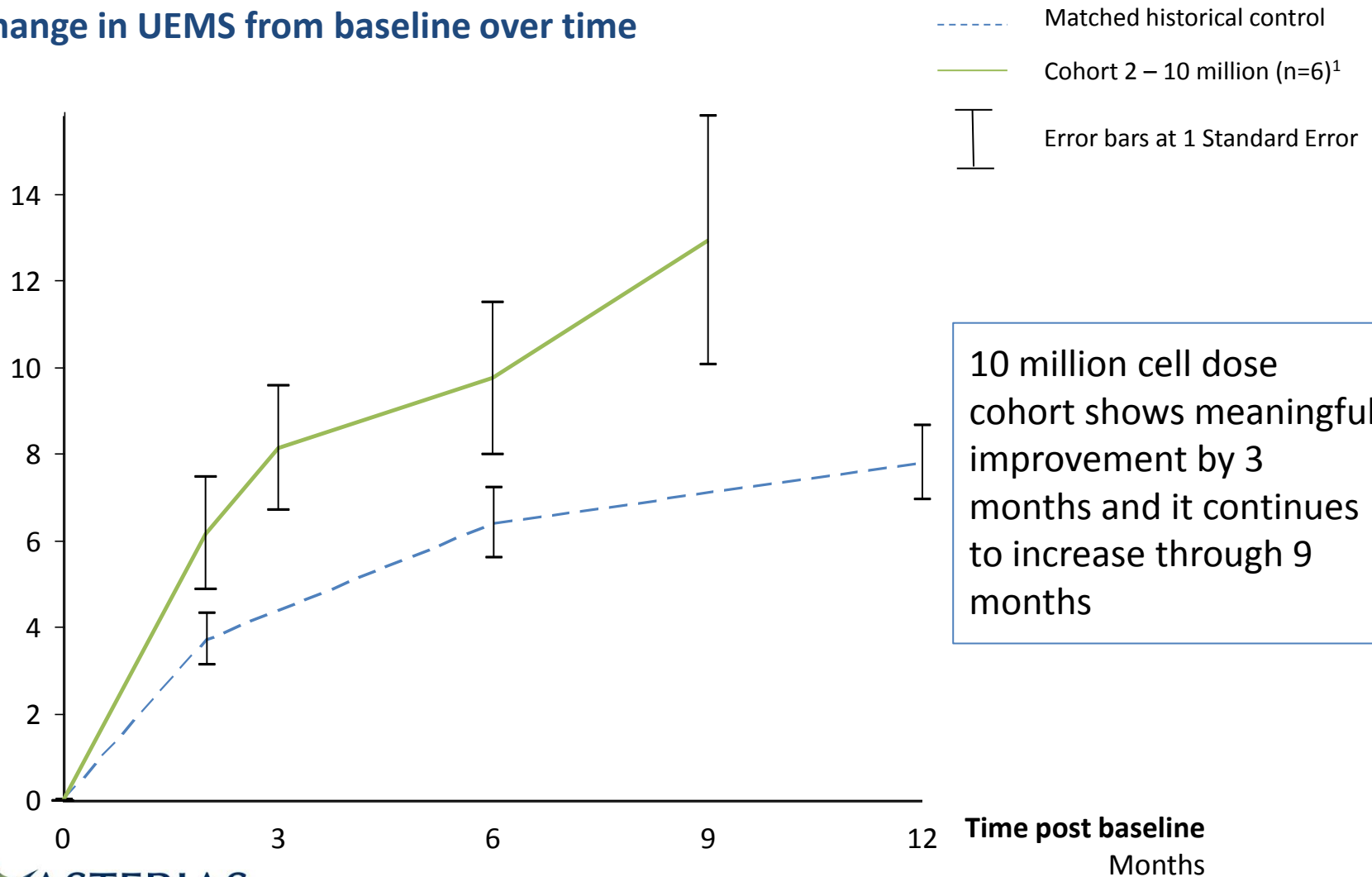
## AIS-A 10 million cell cohort data

	3 month	6 month	9 month
# patients	6	5	3
Average	8.2	9.8	13
Median	6.5	9	13
High	14	16	18
Low	5	6	8

- All subjects in Cohort 2 have exhibited improved upper extremity motor scores (UEMS) through last follow-up
- Maintained or continued improvement observed through 6 and 9 months post-treatment

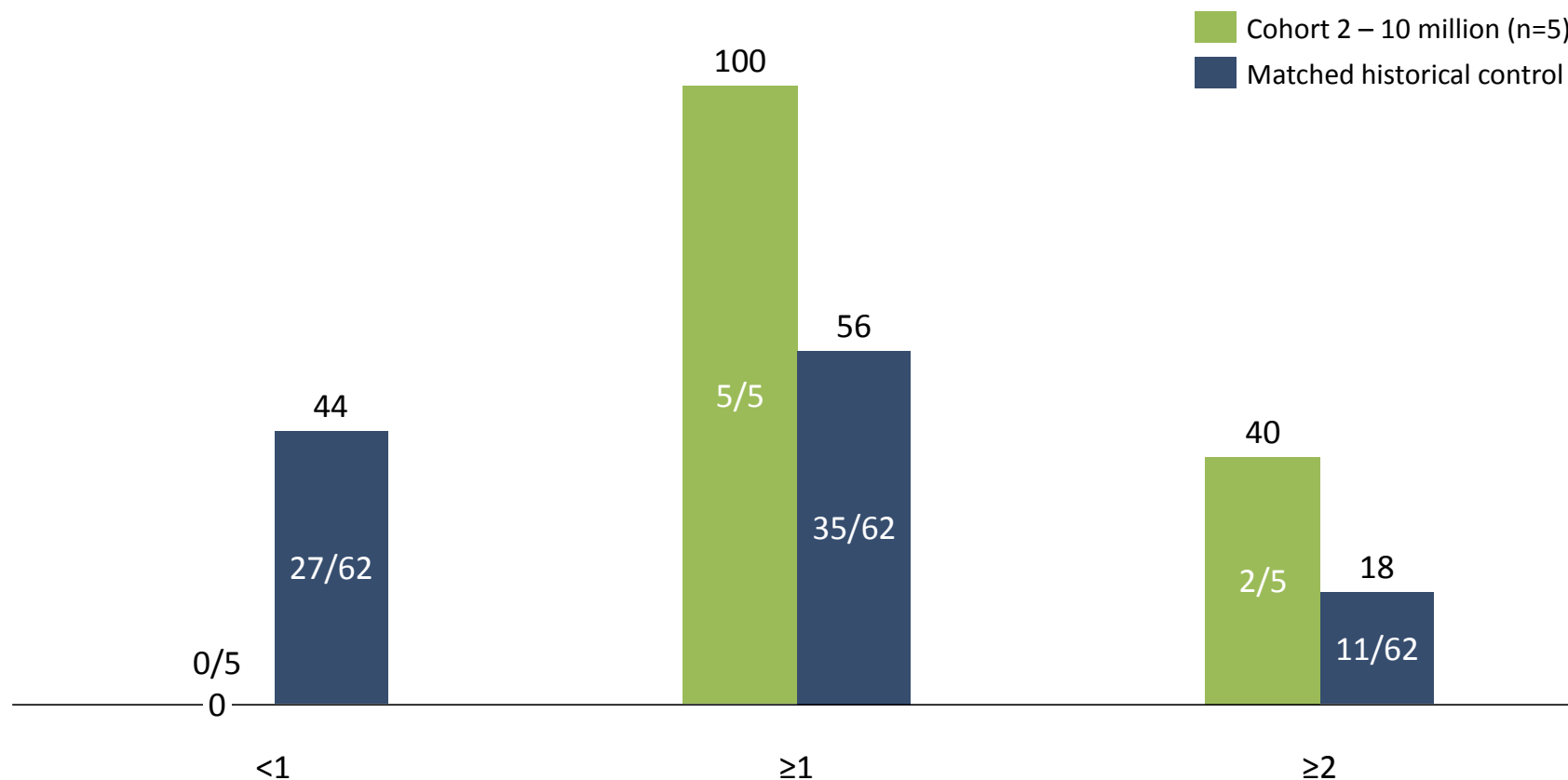
# AIS-A 10 Million Cell Cohort Experienced Greater UEMS Recovery than Matched Historical Control Group

## Change in UEMS from baseline over time



# AIS-A 10 Million Cell Cohort Shows Improved Motor Level Recovery vs. Matched Historical Controls

**Cohort 2 (10 million cells) motor level recovery vs. matched historical controls**  
Percentage of patients by recovery level as of last visit (6 or 9 months)



**Motor level improvement vs. baseline measurement**

# Safety Profile Remains Positive

- Safety profile from all AST-OPC1 patients enrolled to date remains positive through 6-12 months of follow up
- Safety of the injection procedure has been excellent
- Immunosuppression with tacrolimus has been well tolerated
- Safety profile of AST-OPC1 cells has been favorable, including no SAEs related to AST-OPC1 and no adverse findings on MRI scans to date



# Summary of Results To Date

- All subjects in Cohorts 1 & 2 have exhibited both improved upper extremity motor scores (UEMS) and improved motor levels relative to baseline
- Early improvements in motor function reported for Cohort 2 (10 million cells) in September 2016 have been maintained or further increased through last date of follow up
- UEMS improvement in Cohort 1 (2 million cells) was similar to matched controls which is indicative of safety in this low dose safety cohort
- Cohort 2 (10 million cells) has shown meaningfully greater UEMS improvement through 6- to 9-months of follow up, suggestive of a dose-dependent therapeutic effect
- Subjects in Cohort 2 have also shown a greater degree of motor level recovery than matched controls

# Plans for 2017

## Q1 2017

- Complete enrollment/dosing of AIS-A 20 million cell and AIS-B 10 million cell cohorts

## Q2/Q3 2017

- Engage in discussions with FDA on clinical development plan, potential accelerated development pathway for AST-OPC1, and possible “breakthrough” designation

## Q3 2017

- Announce AIS-A 10 million cell 12 month data, AIS-A 20 million cell 6 month data, AIS-B 10 million cell 6 month data
- Final patients enrolled in AST-OPC1 SCiStar Study (AIS-B 20 million cell cohort)
- Gain FDA agreement on plan for randomized, controlled trial of AST-OPC1 projected to begin in early 2018