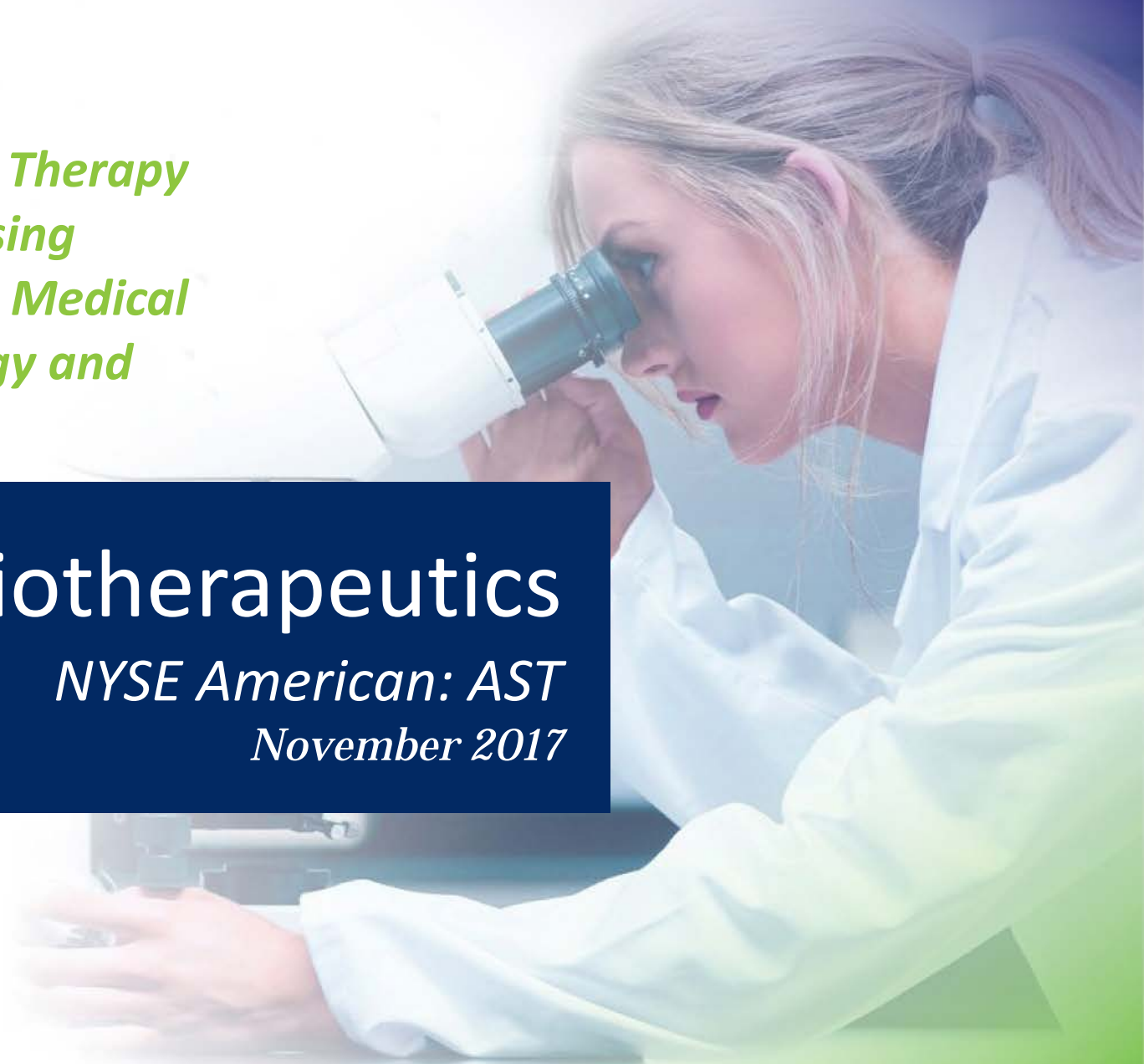


*Clinical-Stage Cell Therapy  
Programs Addressing  
Significant Unmet Medical  
Needs in Neurology and  
Oncology*

**Asterias Biotherapeutics**

*NYSE American: AST  
November 2017*








# Forward-Looking Statements

Statements pertaining to future financial and/or operating results, future growth in research, technology, clinical development, and potential opportunities for Asterias, along with other statements about the future expectations, beliefs, goals, plans, or prospects expressed by management constitute forward-looking statements. Any statements that are not historical fact (including, but not limited to statements that contain words such as “will,” “believes,” “plans,” “anticipates,” “expects,” “estimates”) should also be considered to be forward-looking statements. Forward-looking statements involve risks and uncertainties, including, without limitation, risks inherent in the development and/or commercialization of potential products, uncertainty in the results of clinical trials or regulatory approvals, need and ability to obtain future capital, and maintenance of intellectual property rights. Actual results may differ materially from the results anticipated in these forward-looking statements and as such should be evaluated together with the many uncertainties that affect the businesses of Asterias, particularly those mentioned in the cautionary statements found in Asterias’ filings with the Securities and Exchange Commission. Asterias disclaims any intent or obligation to update these forward-looking statements.

# Background Information

- A cell therapy company with lead programs in:
  - Neurology: Spinal cord injury
  - Immunotherapy: Lung cancer, AML
- Funding Partners:
  - California Institute of Regenerative Medicine
  - Cancer Research UK

# Clinical Programs – Development Pipeline

PROGRAM	PRECLIN	PHASE 1	PHASE 2	PHASE 3	Partners/Funding
<b>AST-OPC1</b> Spinal Cord Injury (subacute)					
<b>AST-VAC1</b> Leukemia (AML) Autologous					
<b>AST-VAC2</b> Lung Cancer Allogeneic					

A female scientist with blonde hair in a ponytail, wearing a white lab coat, is looking through a microscope in a laboratory. The background is bright and slightly blurred, suggesting a clean, professional environment. A dark blue banner is overlaid on the lower left side of the image.

# Spinal Cord Injury Program



# AST-OPC1: Introduction

- AST-OPC1 is a cellular therapy utilizing oligodendrocyte progenitor cells (OPCs)
- OPCs are found in the human body and are precursors to oligodendrocyte cells which, among other things, provide electrical insulation for nerve axons in the form of a myelin sheath
- AST-OPC1 administers OPCs into the body to supplement the body's own internal supply of OPCs with a non-patient specific supply of cells
- AST-OPC1 is made from a well-established, pluripotent embryonic stem cell line originally isolated in the 1990s



AST-OPC1 Injection Procedure

# SCiStar Study in Cervical Spinal Cord Injury

-  Dosing complete
-  Currently enrolling

## Study Design

- Phase 1/2a trial in progress
- 9 clinical sites currently participating
- AIS-A and AIS-B patients
- 2m, 10m, and 20m cell cohorts
- Primary Assessment: Safety
- Secondary Assessment: Motor Function Improvement
  - Motor Levels
  - Upper Extremity Motor Scores

## Enrollment Progress

- 4 cohorts enrolled to date
- More than 20 subjects enrolled to date
- Study enrollment to be completed later this year

Cohort 1 – AIS-A 2 million  
3 subjects

Cohort 2 – AIS-A 10 million  
6 subjects

Cohort 3 – AIS-A 20 million  
6 subjects

Cohort 4 – AIS-B 10 million  
6 subjects

Cohort 5 – AIS-B 20 million  
5-8 subjects

## Upcoming Data Readouts

- 6 mos. – Q118
- 12 mos. –Q318
- 6 mos. – Q118
- 12 mos. –Q318
- 6 mos. – 1H18
- 12 mos. –2H18

# Potential to Have Clinically Meaningful Benefit

- **Significant lifetime direct healthcare costs;** can reach \$5 million
- **Very high unemployment rate;** 63% of cervical injury patients are unemployed 8 years post-injury
- **Motor level improvements** translate into clinically significant improvements in ability to self-care and significant reductions in cost of care
- **Recovery of two motor levels** can save significant healthcare costs associated with paid attendant care

	Capability				
	C1-C3	C4	C5	C6	C7-C8
Bowel					
Bladder					
Bed Mobility					
Transfers					
Pressure Relief					
Eating					
Dressing					
Grooming					
Bathing					
Wheelchair					
Car Transport					
Daily Home Care	<b>24 hr Attendant</b>	<b>18-24 hr Attendant</b>	<b>6-12 hr Assistance</b>	<b>4 hr Housework</b>	<b>1 hr Housework</b>

Total Assist

Partial Assist

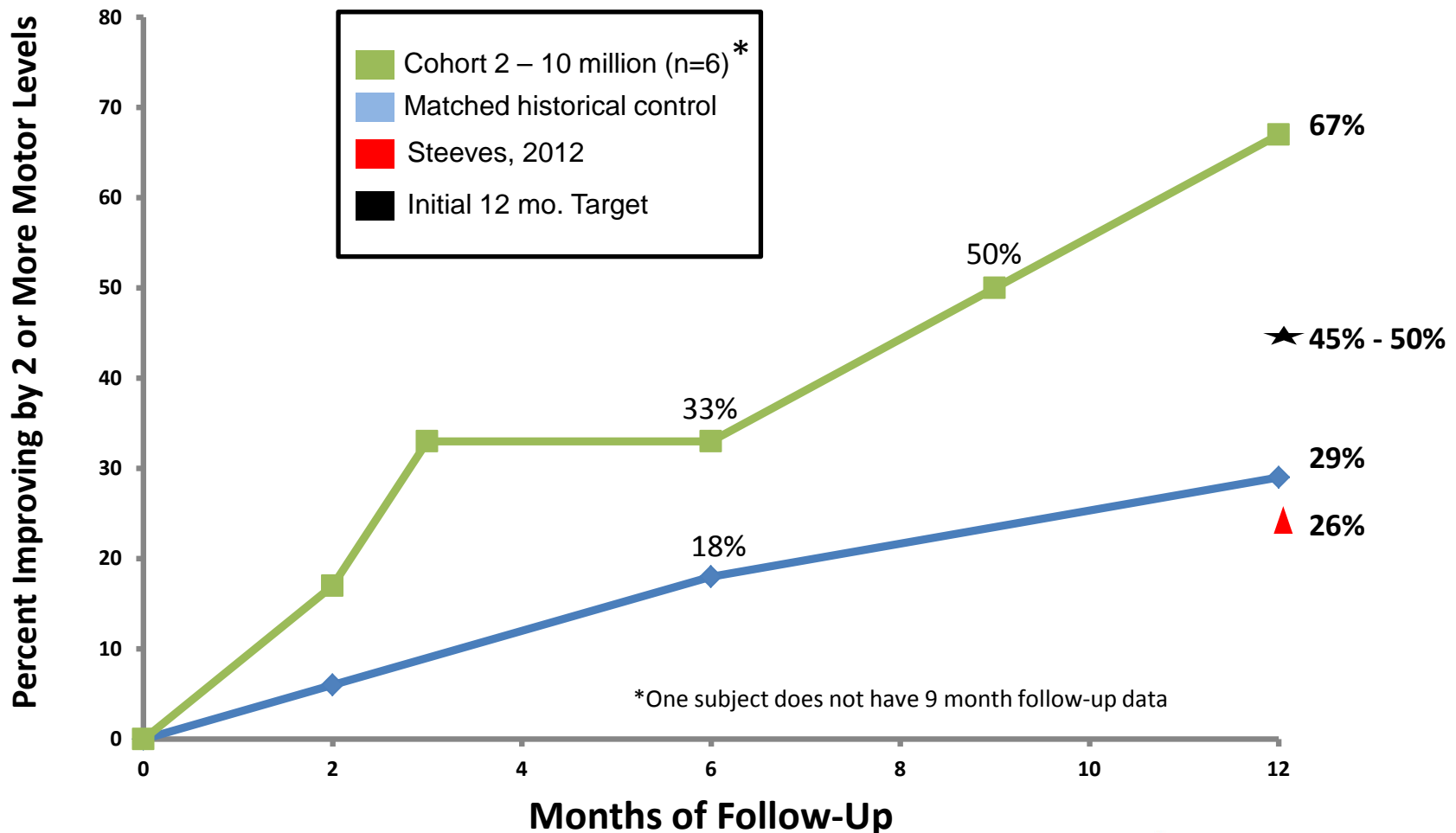
Independent





# Cohort 2 Two Motor Level Recovery at 12 Months

Cohort 2 (AIS-A 10 million cells) motor level recovery vs. comparators

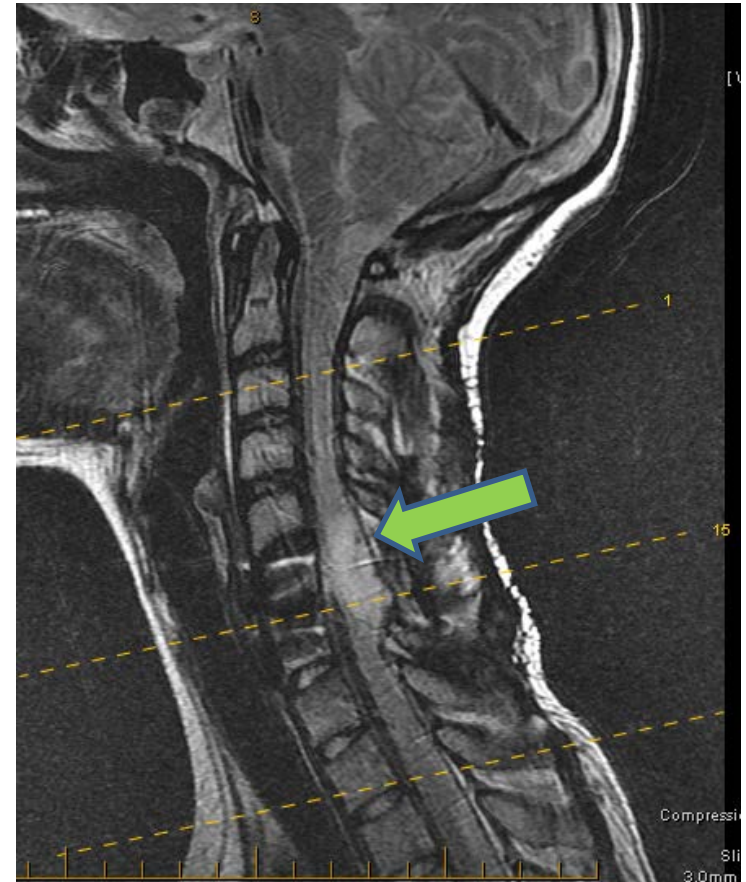


# 12 Month MRI Results Continue to Support Durable Engraftment of AST-OPC1 Cells

- Cavitation is estimated to occur in ~80% of spinal cord injury patients meeting SCiStar study inclusion criteria
- 100% (6/6) of Cohort 2 subjects had serial MRI scans at 12 months that indicated no sign of lesion cavities in any subject
- The MRI results are consistent with formation of a tissue matrix at injury site, which is supportive evidence showing that AST-OPC1 cells have durably engrafted to help prevent cavitation at the injury site<sup>(1)</sup>

- Green arrow shows the AST-OPC1 injection site in the sagittal plane
- Slightly bright signal is consistent with the presence of an AST-OPC1 graft

Cohort 2 subject  
365 Day T2 –weighted sagittal MRI



(1) Wirth et al, Exp Neurology 1995

# RMAT Designation

- FDA has granted the Regenerative Medicine Advanced Therapy (RMAT) designation for AST-OPC1
- The RMAT designation is granted to regenerative medicine therapies for which “preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs” for a “serious or life-threatening condition”<sup>1</sup>
- Upon RMAT designation, sponsors are eligible for increased and earlier interactions with FDA to help facilitate an efficient development program, including discussion of which approval pathways would be appropriate and the size of clinical trials
- The RMAT designation will help Asterias facilitate the expedited review of AST-OPC1 and its development
- Asterias will use the RMAT Designation to initiate formal discussions with FDA on the next study for AST-OPC1

## Companies with therapies receiving RMAT designation <sup>(2)</sup>



<sup>1</sup> 21<sup>st</sup> Century Cures Act, Section 3033; <sup>2</sup> FDA Submission to Congress re 21<sup>st</sup> Century Cures Act, June 6 2017

<sup>2</sup> This may not be an exhaustive list of companies with therapies receiving RMAT designation



# What's Next for AST-OPC1

1H'18

2H'18

## Data Updates

- Cohort 3 - 6 month data update
- Cohort 4 - 6 month data update
- SCiStar study 6 month update, including Cohort 5
- Cohort 3 - 12 month update
- Cohort 4 - 12 month update
- SCiStar study 12 month update, including Cohort 5

## Other 2018 Potential Catalysts

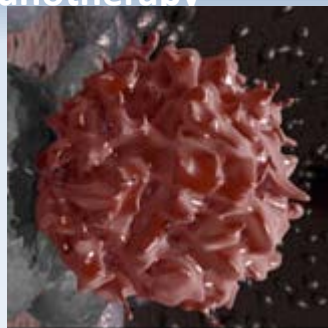
- Potential regulatory clearance for randomized controlled trial for AST-OPC1
  - Potential non-dilutive funding to partially offset costs for next trial
    - Potential partnering opportunities (Japan)

A female scientist with blonde hair tied back, wearing a white lab coat, is focused on looking through a microscope. The scene is set in a laboratory with a bright, clean background. A dark blue banner is overlaid on the lower left portion of the image, containing the text 'Cancer Immunotherapy' in white. The overall lighting is soft and professional, emphasizing the scientific nature of the work.

# Cancer Immunotherapy

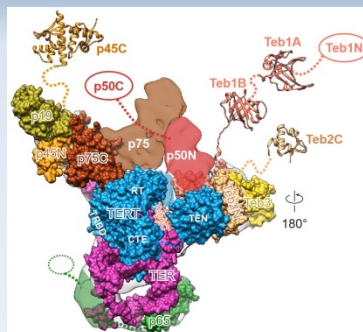
# AST-VAC is a Cell Immuno-Oncology Platform with the Ability to Universally Attack Cancer Cells

## Dendritic cell immunotherapy



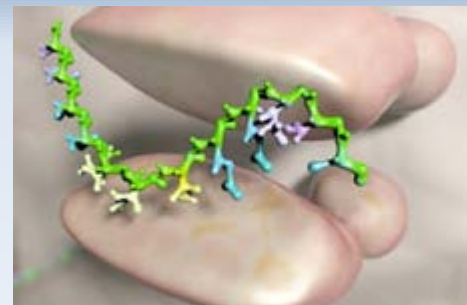
- Stimulates diverse immune responses to extend remission

## Telomerase antigen



- Universal cancer antigen, conferring proliferative ability
- Normal adult cells do not express telomerase, ensuring safety

## LAMP protein



- LAMP enhances antigen presentation by dendritic cells to increase immune response
- Licensed from Immunomic Therapeutics

**Two programs:**  
AST-VAC1 (autologous)  
AST-VAC2 (allogeneic)

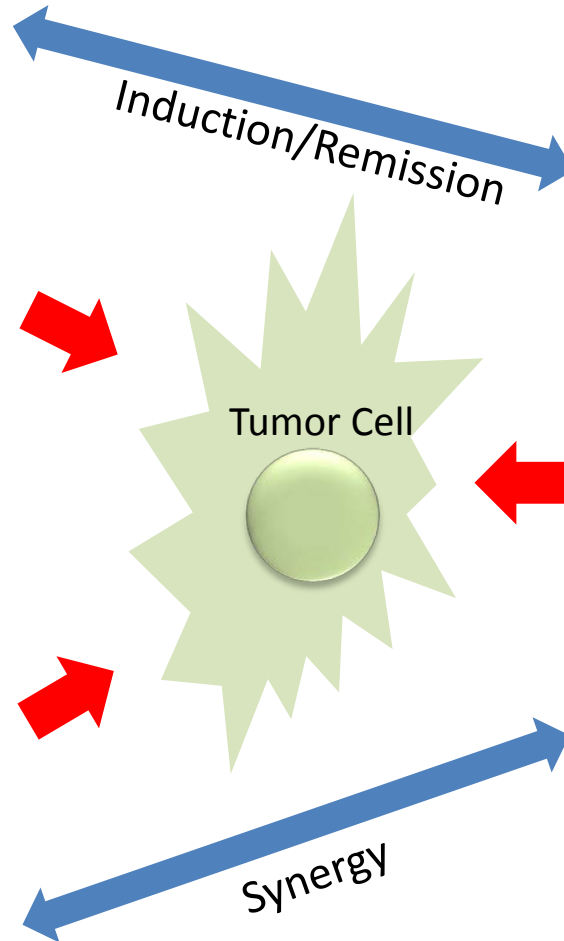
# VAC Platform Likely Synergistic with Other Promising Immunotherapies

## Car-T Cells

- Highly Engineered T Cells to Target and Kill Tumor Cells with Specific Targets
- Effective – targeting induction
- Relapses common (antigen escape)
- Toxicities – not a good option for maintenance

## Immune Checkpoint Inhibitors

- Antibodies/Biologics
- Enhance Immunogenicity and Immune-mediated Attack on Tumors
- Many Received Accelerated Approval for Specific Second Line Indications
- Efficacious only in a small subset of tumors



## VAC1 and VAC2

- Stimulate T cell Responses to Tumor by engineering mature dendritic cells
- POC data in AML supporting technology
- Low level of toxicities – potential positioning as maintenance therapy after patients enter remission with Car-T or other approach
- Potential to expand efficacy of ICIs by stimulating new T cell responses

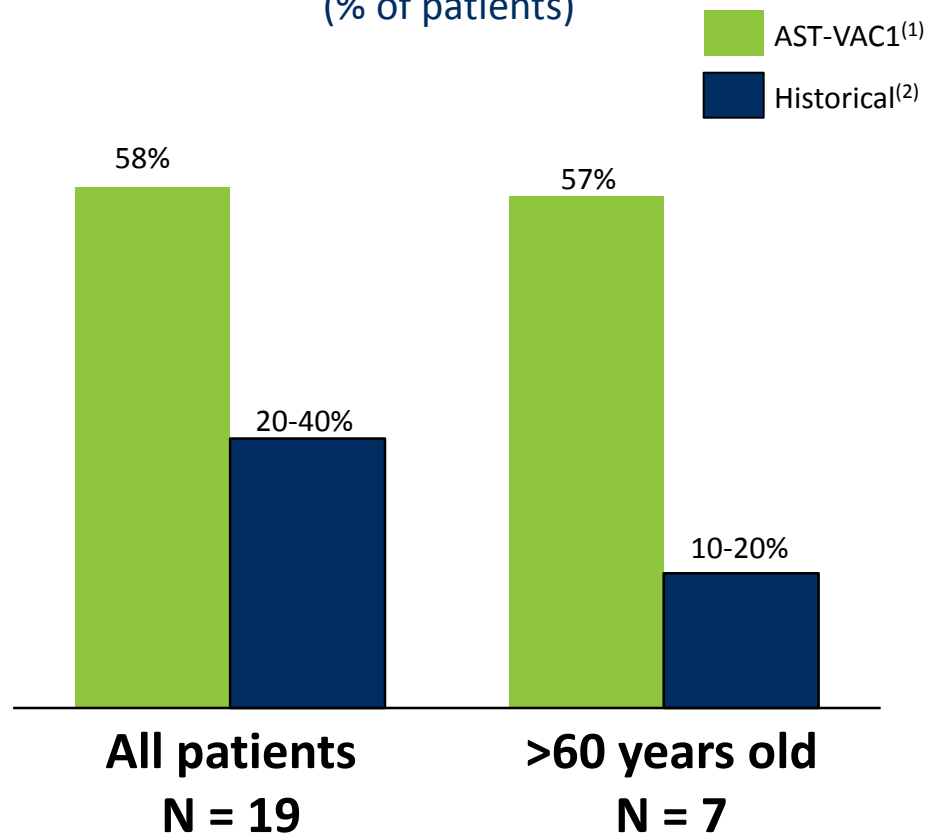
# AST-VAC1 Has Shown Clinical Success in Early Stage Trials and Provides Proof of Concept for AST-VAC2 Program

## Program Highlights

- Phase 2 Multicenter, open-label trial
- Treated 19 patients with AML in complete remission (CR)
  - Intermediate and high risk patients, evaluated by cytogenetics
  - N=7 in very high risk >60 year age group
- Robust safety in 40 patients treated
- Clear anti-telomerase immune response
- Process Development Underway to Improve TAT
- Potential Partnering Opportunity

## Relapse Free Survival at 52 Months

(% of patients)



(1) VAC1 Phase 2 results published: Cancer (2017) 123:3061.

(2) Mayer NEJM 1994; Rollig, J Clin Onc 2011

(3) ITP is commonly observed in AML patients and may not be linked to treatment with AST-VAC1 or any other therapies.



# Cancer Research UK Partnership to Execute Allogeneic AST-VAC2 Clinical Trial in NSCLC



## Upcoming study in non-small cell lung cancer (NSCLC) will:

- Establish safety of AST-VAC2 in resected and advanced cancer
- Assess the generation of anti-hTERT and anti-VAC2 Immune Responses Periodically Over 1 Year
- Investigate Initial Measures of Clinical Activity as Progression Over 2 Years

## Advantages of CRUK Partnership

- External validation
- Advancement of AST-VAC2 Program at minimal cost to Asterias shareholders
  - Estimated saving of >\$20M+
- Combine CRUK expertise in oncology drug development with Asterias expertise in cell therapy
- Access to network of CRUK investigators

## Status

- Received regulatory approval to initiate trial
- FPI in 2018

# Platform Benefits of POC Data from Phase 1 Study of AST-VAC2 in NSCLC

**Demonstration  
of safety and  
immune  
responses in  
NSCLC study**

## Enables:

Phase 2b/pivotal trials in NSCLC

Expansion of AST-VAC2 into additional cancer indications

Investigation of combination with checkpoint / other immune pathway inhibition

Amenability of platform to other neo-antigens

## Rationale:

- Safety data generated in adjuvant and advanced settings
- Telomerase expressed in >85% of all cancers
- Plays a critical role in cancer cell survival and proliferation
- Multiple preclinical studies suggest combining checkpoint inhibitors with immune activators will improve clinical responses
- DC platform can be used to present any antigen / multiple antigens