



Asterias **Biotherapeutics** (NYSE MKT: AST)

*Proprietary Regenerative
Medicine Platforms to
Address Significant Unmet
Medical Needs*



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’ Registration Statement on Form S-3 and Prospectus, as well as its other periodic reports, filed with the Securities and Exchange Commission. Asterias disclaims any intent or obligation to update these forward-looking statements.

Key investment highlights

Two transformative platforms

- **Dendritic Cell Immunotherapy**

- AST-VAC1: Autologous vaccine in Acute myeloid leukemia; planning Phase 3 study based on promising Phase 2 results
- AST-VAC2: Allogeneic vaccine with broad cancer applicability; entering Phase 1/2a in NSCLC: second generation approach; de-risked by VAC1 data
 - *Opportunity across multiple cancers, stages of cancer, and combinations*

- **Industry-leading Pluripotent Stem Cells**

- AST-OPC1 in Spinal cord injury: one of largest ever preclinical data packages; 3-4 years' safety data from FIH study. In second in man Phase 2A dose escalation study
 - *Mechanism of action applicable to MS, Stroke, ALS, and Leukodystrophies (e.g. Canavans disease)*

Partnerships with leading institutions

- \$14.3M in non-dilutive funding from California Institute of Regenerative Medicine (CIRM) for AST-OPC1 Phase 1/2a trial in spinal cord injury
- Partnered with Cancer Research UK (CRUK) to conduct AST-VAC2 Phase 1/2a for lung cancer with ~\$20-30M funded by CRUK

Multiple value inflection milestones over the next 12-24 months

Asterias 18 month milestone and catalyst outlook

Timing	Milestone / Catalyst / Event
2H'14 - completed	<ul style="list-style-type: none"> ✓ Establish collaboration with CRUK for AST-VAC2 trial in NSCLC ✓ Sign CIRM grant – triggers disbursement of CIRM funds
1H'15 – completed	<ul style="list-style-type: none"> ✓ Initiate AST-OPC1 Phase 1/2a clinical trial ✓ Dose first patient in AST-OPC1 Phase 1/2a clinical trial ✓ AST-VAC1 data presentation at ASCO
AST-VAC1	
H2'15	<ul style="list-style-type: none"> • Initiate process development for AST-VAC1
H2'16	<ul style="list-style-type: none"> • Regulatory clearance for AST-VAC1 Phase 3 trial in AML
AST-VAC2	
H2'16	<ul style="list-style-type: none"> • MHRA clearance for AST-VAC2 Phase 1/2a trial in NSCLC
AST-OPC1	
H2'15	<ul style="list-style-type: none"> • Safety data from 2M cell cohort • Dose escalate to 10M cell cohort
H1'16	<ul style="list-style-type: none"> • Complete enrollment of 10M cell cohort; safety data readout • Dose escalate to 20M cell cohort
H2'16	<ul style="list-style-type: none"> • 6 month efficacy data 10M cell cohort • Complete enrollment 20M cell cohort

Dendritic Cell Immunotherapy

Product: AST-VAC1

Platform: Autologous Dendritic Cell Immunotherapy

Status: Phase 3 planning

Indication: Acute myelogenous leukemia

Product: AST-VAC2

Platform: Allogeneic Dendritic Cell Immunotherapy

Status: Entering Phase 1/2a

Lead Indication: Lung Cancer

Asterias dendritic cell immunotherapy platform consists of two assets

AST-VAC1

- First generation, autologous dendritic cell telomerase vaccine
- Robust safety in 2 clinical trials totaling 41 patients
- Promising Phase 2 efficacy data in key unmet medical need of older AML patients
- Preparing for Phase 3 study to enable accelerated approval in older AML patients

AST-VAC2

- Second generation, allogeneic dendritic cell telomerase vaccine
- POC for safety and efficacy of telomerase dendritic cell vaccines from AST-VAC1
- Second generation allogeneic platform promises improved manufacturing and potential for improved efficacy
- Broad potential applications; lead indication in NSCLC
- Mode of action synergistic with immune checkpoint inhibitors

Dendritic Cell Immunotherapy

Product: AST-VAC1

Platform: Autologous Dendritic Cell Immunotherapy

Status: Phase 3 planning

Indication: Acute myelogenous leukemia

Product: AST-VAC2

Platform: Allogeneic Dendritic Cell Immunotherapy

Status: Entering Phase 1/2a

Lead Indication: Lung Cancer

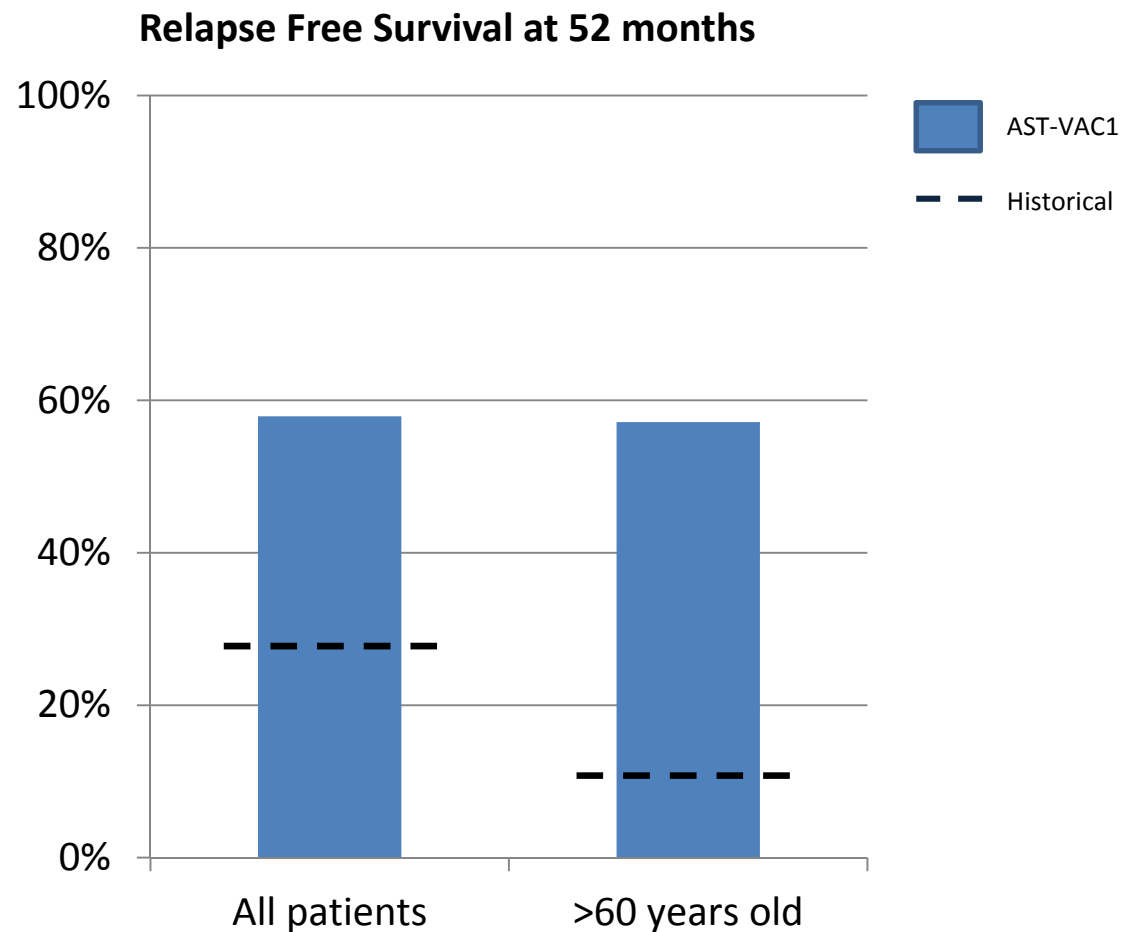
AST-VAC1 is an autologous dendritic cell telomerase vaccine

- **Robust safety in 2 clinical trials totaling 41 patients**
- **Promising efficacy data in key unmet medical need of older AML patients**
 - 57% of patients over 60 remained in remission at over 4 years after AST-VAC1 treatment, compared to historical rates of 10-20%
 - Potential to expand treatable population downstream to include lower risk AML and MDS patients
- **Presents potential late stage development opportunity**
 - Potential for accelerated approval if results can be reproduced
- **Strategic Alternatives to maximize shareholder value**
 - Develop VAC1 Internally at Asterias
 - Explore access to non-dilutive capital
 - Seek strategic development partnership for program

Phase 2 trial shows AST-VAC1 may extend remission in older and high-risk patients

Phase 2 trial highlights

- Included only intermediate and high risk patients, evaluated by cytogenetics
- All patients had already achieved CR
- Of 19 patients vaccinated during CR, only 1 possibly related SAE was reported
- Long term follow up showed relapse free survival at 52 months in 58% of patients, with similar success for both <60 and >60 patients



Source: Asterias results, presented ASCO May 2015; Cancer Research UK, "Statistics and Outlook for AML", accessed 12/15/14; physician interviews

AST-VAC1 fills a unique unmet need that will likely not be addressed by current pipeline therapies

Standard of Care in Younger and Low-risk AML:

1. Induction chemotherapy to achieve remission

2. (If tolerable) Consolidation

- High dose chemotherapy to prevent relapse
- Bone marrow transplant

Other therapies in development are primarily focused on increasing the number of patients who reach remission

AST-VAC1: The Unmet Need

- Older patients often cannot tolerate the co-morbidities associated with intensive chemotherapies and are not candidates for bone marrow transplants
- As a result, there is a key unmet need for therapies such as VAC1 that are well tolerated and can extend remission

Pipeline compounds could increase the market potential for AST-VAC1 by increasing the number of patients who achieve remission

AST-VAC1 is a late stage clinical asset and near term product opportunity for Asterias



- Potential for accelerated approval if Phase 2 results reproduced:
 - AST-VAC1 fills an unmet need as an alternative to current consolidation therapies
 - AST-VAC1 eligible for orphan drug status in AML
 - Surrogate end points (e.g. relapse free survival) may be used to accelerate trial timelines
- Exploring opportunities for non-dilutive funding and/or strategic partnership to fund development

Dendritic Cell Immunotherapy

Product: AST-VAC1

Platform: Autologous Dendritic Cell Immunotherapy

Status: Phase 3 planning

Indication: Acute myelogenous leukemia

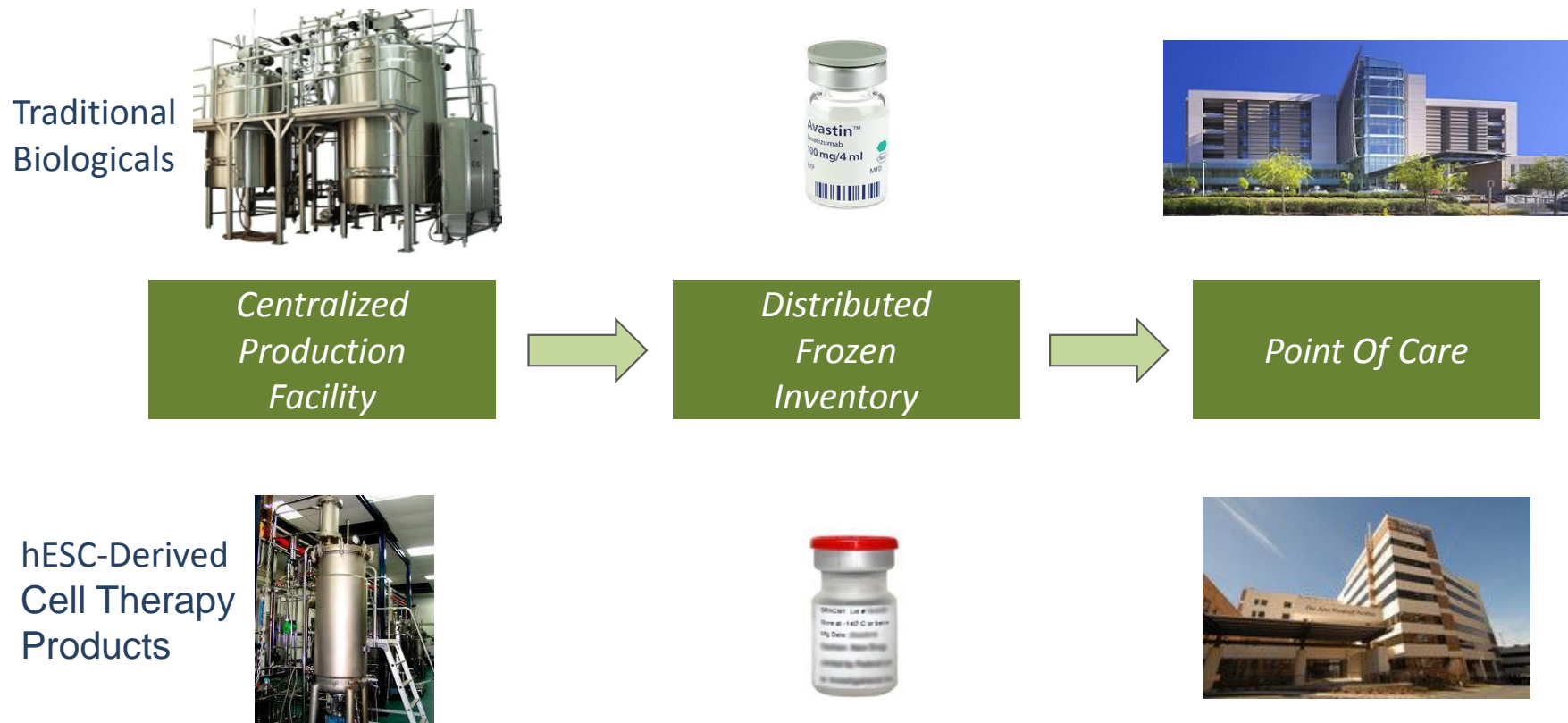
Product: AST-VAC2

Platform: Allogeneic Dendritic Cell Immunotherapy

Status: Entering Phase 1/2a

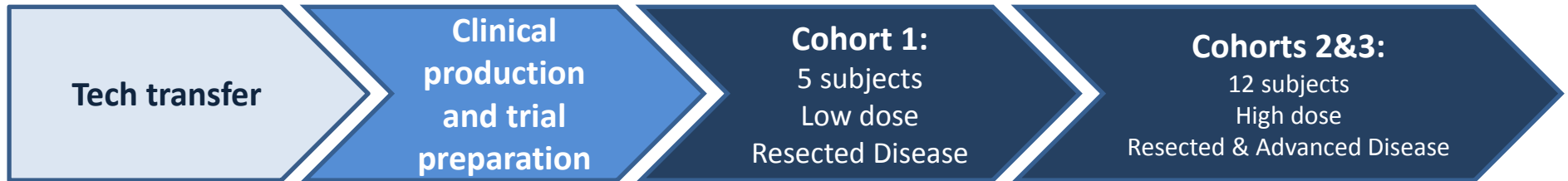
Lead Indication: Lung Cancer

Pluripotent stem cell platform for AST-VAC2 enables scalable production of allogeneic DCs with “point-of-care” distribution similar to protein therapeutics



- Using standard two tier banking system, entire product lifecycle can easily be supplied from a single MCB
- H1 cell line derived from excess IVF embryos, meets all ethical guidelines of NIH, UKSCB, etc

AST-VAC2 partnered with CRUK for phase 1/2a study in NSCLC



CANCER
RESEARCH
UK

★ **2016:**
Regulatory
clearance

- NSCLC selected as lead indication given high unmet medical need and responsiveness to immune therapies in previous trials
- Through partnership, Cancer Research UK (CRUK) pays full cost of GMP manufacturing, regulatory filing, and 29 patient Phase 1/2a study – Estimated Asterias savings of \$20-30M
- Asterias has first option to arising data in exchange for milestones and royalties, allowing Asterias to keep majority of value



Pluripotent Stem Cells

Product: AST-OPC1

Platform: Pluripotent Stem Cells

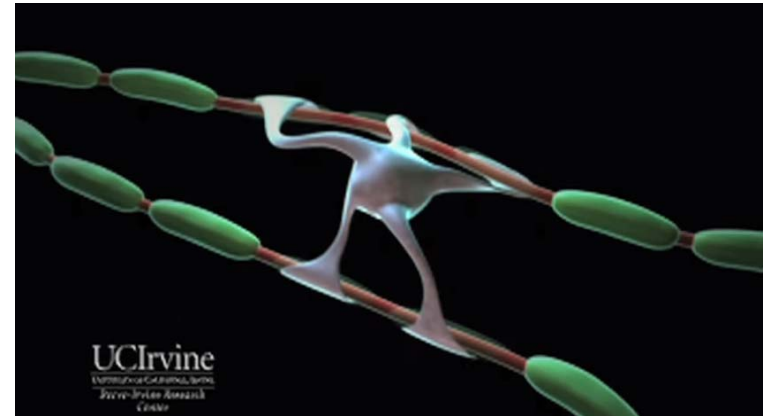
Status: Phase 1/2a

Lead Indication: Spinal Cord Injury

Based on our pluripotent stem cell platform, AST-OPC1 is being developed to address neurodegenerative diseases

OPC1 treatment

- OPC1 (oligodendrocyte progenitor cell) is a neural cell type based on our pluripotent stem cell platform
- Oligodendrocytes wrap nerve cells to facilitate the signal conduction and are particularly sensitive to the inflammatory environment post spinal cord injury (SCI)
- Goal of AST-OPC1 program is to replace these cells and improve motor function for SCI patients



SCI indication

- SCI affects 12,000 patients annually in the US with no currently approved therapies
- Multi-billion dollar annual market opportunity due to \$2-4M lifetime cost of patient care¹

Potential follow-on opportunities in MS, Stroke, and other neurodegenerative diseases

¹ National Spinal Cord Injury Statistical Center, Facts and Figures 2013
Video source: UC Irvine Keirstead lab; <https://www.youtube.com/watch?v=0g908rplos8>

OPC1 has produced strong preclinical and clinical results to date

One of the Most Comprehensive Preclinical Data Packages Ever Compiled

- Extensive data from 28 studies in >3,000 animals demonstrates:
- Significantly improved locomotor function in both cervical and thoracic SCI models
- No significant safety issues
- Extensive cavity filling and myelination
- Long term persistence of cellular grafts (>1 year)

Demonstrated safety in phase 1 trial

- Five subjects received 2M AST-OPC1 cells via surgery at 7-14d post-injury, followed for 3-4 years
- Clean safety profile observed – No serious adverse events related to surgery, AST-OPC1, or immunosuppression
- Monitoring through one year shows no evidence of immune responses to AST-OPC1
- MRI results in 4 of 5 subjects are consistent with prevention of lesion cavity formation

AST-OPC1 Phase 1/2a Trial: Design and Milestones

Trial design

- Surgical delivery at 14-30 days post injury
- Primary endpoint: safety
- Secondary endpoint: % patients recovering ≥ 2 motor levels on the ISNCSCI scale (Target: $\geq 40\%$)
- Open label trial enables use of adaptive design in expansion cohort
- Work of SCOPE enables comparison to robust historical outcomes databases

Cohorts

Scale
Dose

Cohort 1

3 subjects
2M cells

Cohort 2

5 subjects
10M cells

Cohort 3

5 subjects
20M cells

File IND amendment for study expansion based on initial safety data in Cohort 2

Expansion cohort*

Up to 27 patients
10 and/or 20M cells

Timeline and milestones

Q1 2015

- Study initiated

2H 2015

- Dose escalation for cohort 2

1H 2016

- Dose escalation for cohort 3

Initial efficacy readout (Cohort 2, 6 months) by 2H'16

*Subject to FDA clearance for expansion from 13 to 40 subjects

Work of the SCOPE consortium has defined clinical development path in complete cervical spinal cord injury

Target Product Profile for AST-OPC1 in Complete Cervical SCI:

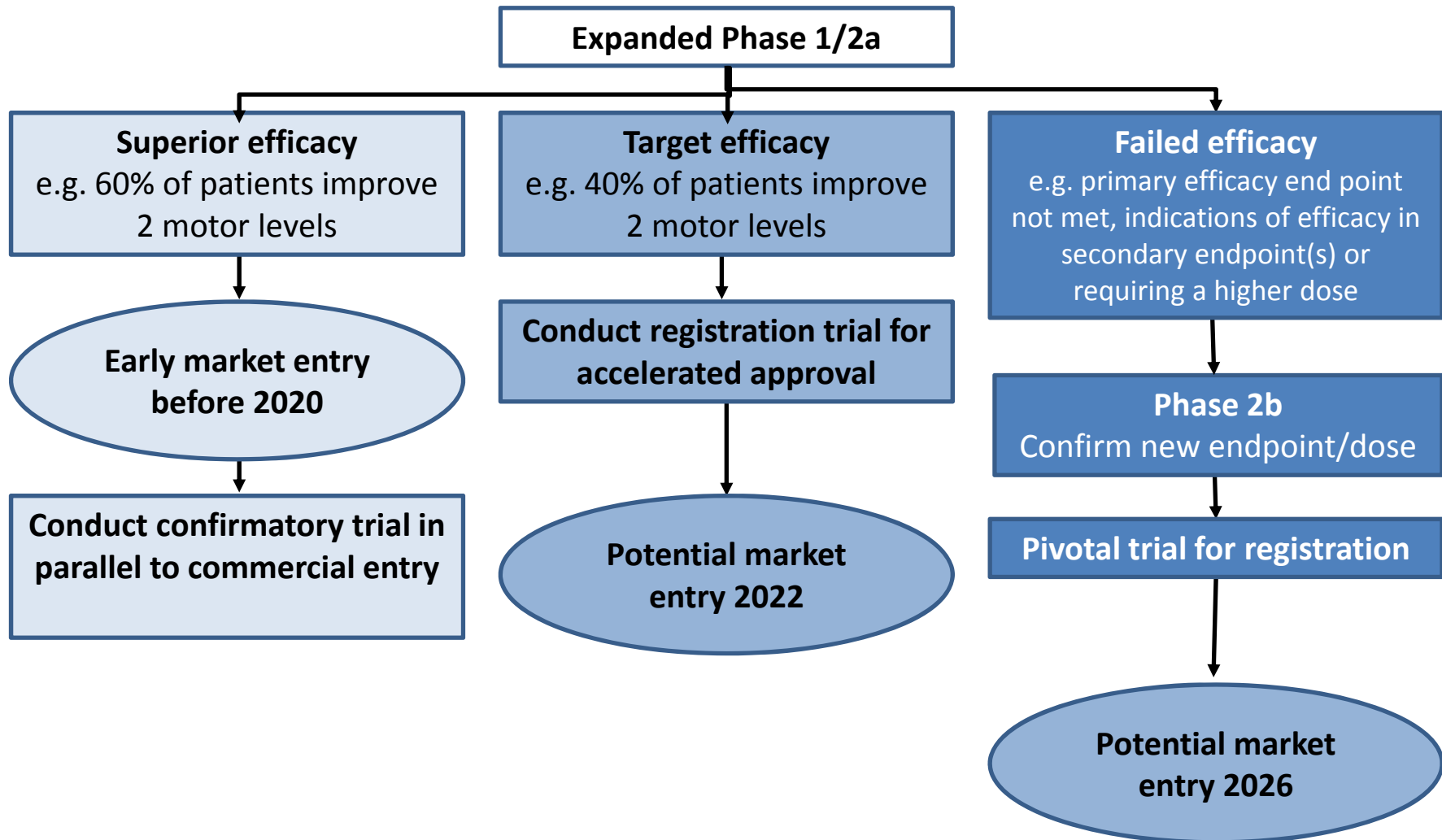
- Increase of $\geq 20\%$ in the percentage of patients regaining two or motor levels of function

Function	Capability				
	C1-C3	C4	C5	C6	C7-C8
Bowel	total assist	total assist	total assist	partial assist	independent
Bladder	total assist	total assist	total assist	partial assist	independent
Bed Mobility	total assist	total assist	partial assist	independent	independent
Transfers	total assist	total assist	total assist	independent	independent
Pressure Relief	total assist	total assist	partial assist	independent	independent
Eating	total assist	total assist	partial assist	independent	independent
Dressing	total assist	total assist	partial assist	independent	independent
Grooming	total assist	total assist	partial assist	independent	independent
Bathing	total assist	total assist	total assist	independent	independent
Wheelchair	total assist	total assist	total assist	partial assist	independent
Car transport	total assist	total assist	total assist	partial assist	independent
Daily Home Care	24 hr attendant	18-24 hr attendant	6-12 hr assistance	4 hr housework	1 hr housework

total assist
partial assist
independent

- Enables powering of trial with only ~200 subjects
- Translates into clinically significant improvements in ability to self-care, significant reductions in cost of care

Path to market for AST-OPC1 will likely be a function of strength of efficacy signal



Asterias Biotherapeutics, Inc

Issuer:	Asterias Biotherapeutics, Inc.
Exchange / Tickers:	NYSE MKT / AST
Shares outstanding:	37.6 M
Market cap:	\$148M ¹
Major institutional holders:	Biotime, Romulus, Fidelity, Scarsdale Equities
Key Financials :	<ul style="list-style-type: none"> • \$15.6M cash • \$10.7M marketable securities²
Guidance for 2015:	Expected net cash burn of \$15-17M

1 Based on share price of \$3.94 as of August 19, 2015

2 3,852,880 shares of BioTime (NYSE MKT: BTX) at \$2.77 as of August 19, 2015

Summary of Opportunity

- Potential for early entry into company with two potentially transformative therapeutic platforms:
 - Dendritic Cell Immunotherapy
 - Pluripotent Stem Cells
- Focused pipeline with three active development programs:
 - Late stage opportunity for AST-VAC1 in AML
 - Entering first in man opportunity in NSCLC with AST-VAC2
 - Extensive safety data, near term efficacy readouts for AST-OPC1 in SCI
- Significant opportunity to improve patient outcomes and impact costs of care in indications with high levels of unmet medical need
- Capital efficient model with ~\$40M of non-dilutive capital secured to date
- One year forward cash on hand and \$ 15.6M and \$ 10M in stock in the balance sheet
- Opportunity for major value creation

Asterias Executive Team: Business Focused, with Best-in-Class Cell Therapy Product Development Experience

Name	Title	Experience
Pedro Lichtinger	Chief Executive Officer	<i>Former President & CEO, Optimer Pharmaceuticals. 30 year career at Pfizer including as President of Global Primary Care and of Europe</i>
Jane Lebkowski, PhD	Chief Scientific Officer	<i>25 years experience in R&D of cell & gene therapies at Applied Immune Sciences, Rhone Poulenc Rorer, and Geron</i>
Katy Spink, PhD	Chief Operating Officer	<i>Former SVP, Cell Therapy Program Operations at Geron. Experience in biotech strategy, BD & program management and operations at Geron and McKinsey</i>
Ed Wirth, MD, PhD	Chief Medical Officer	<i>25 years experience in translational research of cell therapies and medical devices at University of Chicago, Geron, and InVivo</i>

- **Broad expertise in pharma and biotech development throughout the product lifecycle**
- **Track record of establishing value creating alliances**
- **Unmatched expertise in development of cell therapies**

