**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): May 2, 2017

**FORTRESS BIOTECH, INC.**
(Exact Name of Registrant as Specified in Charter)

| **Delaware** | **001-35366** | **20-5157386** |
|---|---|---|
| (State or Other Jurisdiction of Incorporation) | (Commission File Number) | (IRS Employer Identification No.) |

| **2 Gansevoort Street, 9th Floor, New York, New York** | **10014** |
|---|---|
| (Address of Principal Executive Offices) | (Zip Code) |

Registrant's Telephone Number, Including Area Code: **(781) 652-4500**

(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))
- ❑ 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.**

Attached hereto as Exhibit 99.1 and incorporated herein by reference is a presentation including an updated corporate overview of Fortress Biotech, Inc.

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

(d) Exhibits

| Exhibit No. | Description |
|---|---|
| 99.1 | Presentation of May 2017. |

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.

FORTRESS BIOTECH, INC.

Date: May 2, 2017

/s/ Lindsay A. Rosenwald
Name: Lindsay A. Rosenwald
Title: Chairman, President and Chief Executive Officer

Exhibit 99.1

# Corporate Presentation

FORTRESS BIOTECH

May 2017

# Forward Looking Statements

This presentation may contain "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934. Such statements include, but are not limited to, any statements relating to our growth strategy and product development programs and any other statements that are not historical facts. Forward-looking statements are based on management's current expectations and are subject to risks and uncertainties that could negatively affect our business, operating results, financial condition and stock price. Factors that could cause actual results to differ materially from those currently anticipated include: risks related to our growth strategy; risks relating to the results of research and development activities; our ability to obtain, perform under and maintain financing and strategic agreements and relationships; uncertainties relating to preclinical and clinical testing; our dependence on third party suppliers; our ability to attract, integrate, and retain key personnel; the early stage of products under development; our need for and continued access to additional funds; government regulation; patent and intellectual property matters; competition; as well as other risks described in our SEC filings. We expressly disclaim any obligation or undertaking to release publicly any updates or revisions to any forward looking statements contained herein to reflect any change in our expectations or any changes in events, conditions or circumstances on which any such statement is based, except as may be required by law.

# Fortress Biotech: Our Unique Approach

**What we do:** Acquire, develop and commercialize novel biopharmaceutical products in all stages of development and across multiple therapeutic areas directly within Fortress Biotech and through our subsidiaries.

**Our business strategy:** Build subsidiaries around marketed products and product candidates that create a pipeline providing our shareholders with a diversified long-term revenue stream.

**Product candidates**

| Marketed | Clinical Development | Pre-clinical |
|---|---|---|
| 4 | 18 | 11 |

# Fortress Biotech: Creating Opportunity

## Business Advantages

- Unique business model and company structure
- Seek out the best product candidates
- Move fast to get products to market
- Extensive experience in structuring deals
- Take advantage of time-sensitive opportunities
- Top tier, focused and experienced management team

## Financial Advantages

- Access to additional capital
- Efficient plan to fund subsidiaries
- Multiple revenue streams (sales, equities, royalties, fees)
- Super-majority voting shares of each subsidiary

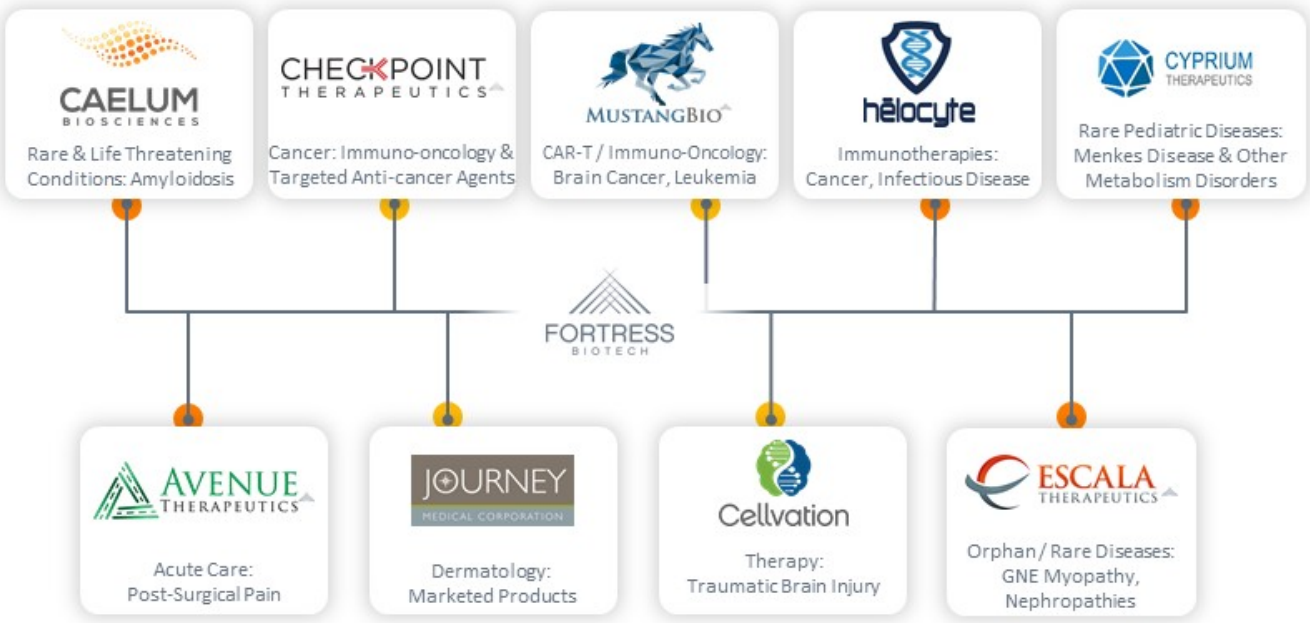
FORTRESS BIOTECH

# Experienced Leadership

| | |
|---|---|
| **Lindsay A. Rosenwald, MD**<br>President and CEO<br>Chairman of the Board | • Co-Portfolio Manager and Partner of Opus Point Partners, LLC<br>• Prolific and successful investor in the life sciences industry for over 20 years previously as Chairman of Paramount BioCapital |
| **Michael S. Weiss**<br>Executive Vice Chairman<br>Co-Vice Chairman of the Board | • Executive Chairman and CEO of TG Therapeutics<br>• Co-Portfolio Manager and Partner of Opus Point Partners, LLC<br>• Previously Chairman and CEO of Keryx Biopharmaceuticals |
| **George C. Avgerinos, PhD**<br>Senior Vice President, Operations | • Former Divisional VP, Global Process and Manufacturing Sciences, Abbvie<br>• Over 30 years experience in biopharmaceutical process development including leading Humira's™ process and manufacturing, world's biggest selling pharmaceutical product |
| **Lucy Lu, MD**<br>Executive Vice President<br>Chief Financial Officer | • Former Senior Analyst at Citi Investment Research<br>• Over 10 years of biotech equity research experience |
| **Eric K. Rowinsky, MD**<br>Co-Vice Chairman of the Board | • Currently serves on board of Biogen, Inc.<br>• Oncologist and former Chief Medical Officer at ImClone Systems, Inc.<br>• Advisor to academic, industrial and FDA advisory boards and has more than 300 peer-reviewed publications |

FORTRESS BIOTECH

# Subsidiaries' Relationship to Fortress




FORTRESS BIOTECH

# Provide Efficient Way To Develop / Commercialize A Product

**Identify Product Candidates**

We seek and identify new in-licensing opportunities in all therapeutic areas and all stages of development from:

- Academic centers
- Corporate entities
- Government health organizations

**Due-Diligence**

Perform extensive due diligence on product candidates using:

- KOLs
- Clinical data
- Market size
- Competition

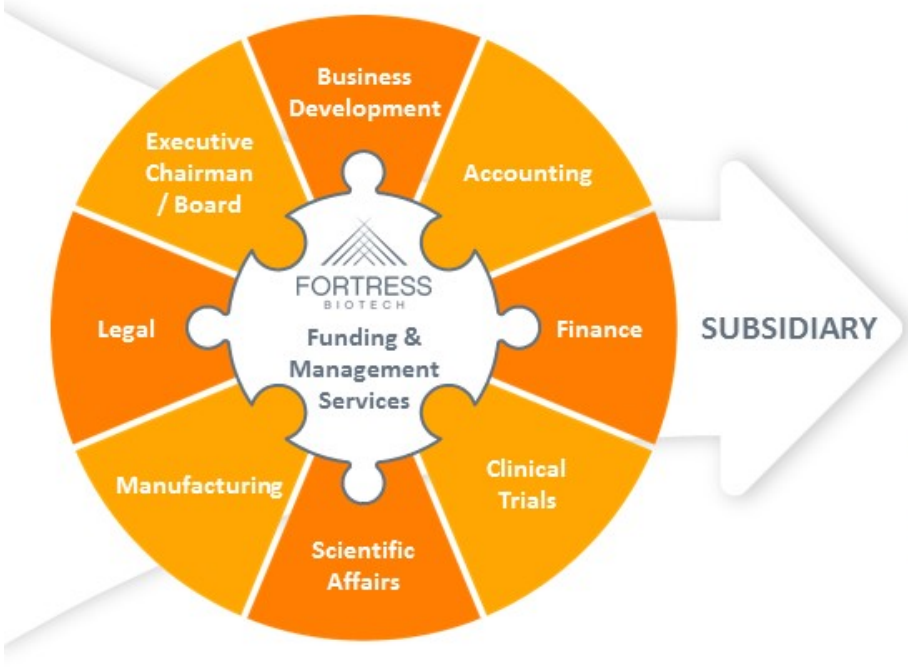
**In-License**

1. May create a subsidiary around product candidates / therapeutic areas
2. Fund and support research and development programs
3. Offer flexibility in deal structuring

Conventional licensing
Acquisitions
Partnerships
Equity arrangements
Joint ventures
Public / private financings
Option agreements

FORTRESS BIOTECH

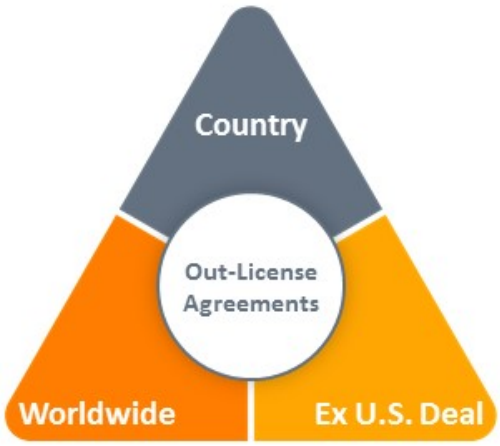
# Fortress: Offer Subsidiaries Accelerated Drug Development




1. Expertise in each therapeutic area
2. Knowledge of the development process
3. Pooled resources
4. Cost efficient operations structure
5. Top-tier focused management team
6. Access to capital

FORTRESS BIOTECH

# Out-License Strategy Adds To Market Size




- Supports portfolio / capital needs
- Provides revenue to find more assets
- Monetizes pipeline

# Hematology / Oncology Pipeline

| Subsidiary | Product Candidate | Indication | Stage of Development | | | | |
|---|---|---|---|---|---|---|---|
| | | | Preclinical | Phase 1 | Phase 2 | Phase 3 | Commercial |
| Caelum Biosciences | CAEL-101 | AL Amyloidosis | | | | | |
| Checkpoint Therapeutics | Anti-PD-L1 | Solid Tumor | | | | | |
| | Anti-GITR | | | | | | |
| | Anti-CAIX | | | | | | |
| | CK-101 EGFR Inhibitor | | | | | | |
| | CK-102 PARP Inhibitor | | | | | | |
| | CK-103 BET Inhibitor | | | | | | |
| Mustang Bio | MB-101 IL13Rα2-specific CAR | Malignant Glioma | | | | | |
| | MB-102 CD123 CAR | AML; BPDCN | | | | | |
| Fortress Biotech | CNDO-109 | AML; MDS | | | | | |

# Diversified Pipeline Across Different Therapeutic Areas

| Subsidiary | Product Candidate | Indication | Stage of Development | | | | |
|---|---|---|---|---|---|---|---|
| | | | Preclinical | Phase 1 | Phase 2 | Phase 3 | Commercial |
| Cellvation | CEVA101 | MNCs for Pediatric TBI | | | | | |
| | CEVA101 | MNCs for Adult TBI | | | | | |
| | CEVA102 | NextGen for Pediatric TBI | | | | | |
| | CEVA102 | NextGen for Adult TBI | | | | | |
| | CEVA-D | Bioreactor – Device | | | | | |
| Helocyte | Triplex | CMV Control Allo-Stem Cell | | | | | |
| | PepVax | CMV Control Allo-Stem Cell | | | | | |
| | Triplex | Kidney Transplant | | | | | |
| | Triplex | Liver Transplant | | | | | |
| | Triplex | Drive CMV Cell Therapy | | | | | |
| | Triplex | Post-Transplant in Pediatric ALL | | | | | |
| | Triplex plus Mustang CAR-T | Glioblastoma Multiforme | | | | | |
| | Triplex plus Mustang CAR-T | Hematalogical Malignancies | | | | | |
| | Pentamer | Congenital CMV | | | | | |
| Escala Therapeutics | ManNAc | GNE Myopathy | | | | | |
| | ManNAc | Nephropathies | | | | | |
| Avenue Therapeutics | IV Tramadol | Post Surgical Pain | | | | | |
| Cyprium Therapeutics | CUTX-101 | Menkes Disease | | | | | |
| | AAV-ATP7A Gene Therapy | Menkes Disease | | | | | |
| Journey Medical Corporation | Targadox | Acne | | | | | |
| | Ceracade | Eczema Emollient | | | | | |
| | Luxamend | Wound Cream | | | | | |
| | Dermasorb HC | Atopic Dermatitis | | | | | |

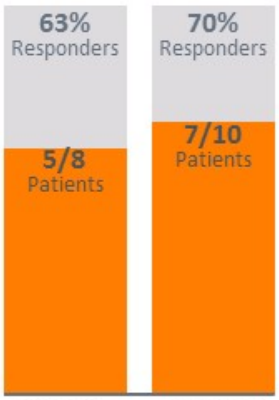
# Rare & Life-Threatening Conditions

CAELUM BIOSCIENCES

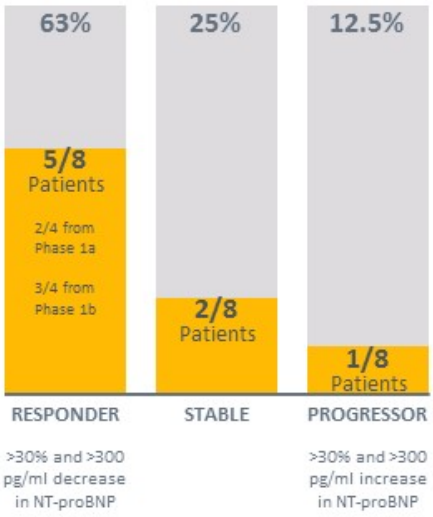
## A novel antibody in Phase 1b clinical trials being developed for patients with AL Amyloidosis

| Focus | Developing treatments toward rare and life threatening diseases that lack effective therapies |
|---|---|
| **Market Size** | 30,000-45,000 patients in the US and EU; 4,500 newly diagnosed patients per year<br>AL amyloidosis is the largest of systemic amyloidosis including both ATTR and AA |
| **Product Candidate** | CAEL-101, a pioneering antibody being developed to specifically target AL fibrils and dissolve amyloid deposits |
| **Clinical Trials** | Interim Phase 1 data of 21 patients, CAEL-101 is well-tolerated and safe showing no dose limiting toxicity: 67% of patients with organ response independent of light chain sub-type |
| **Milestones** | Phase 1a/1b expected to complete 2017<br>Phase 2 expected to commence 2018 |
| **Licensor & Scientific Advisor** | Columbia University: January 2017<br>Suzanne Lentzsch, M.D., Ph.D., Professor of Medicine at Columbia University Medical Center, Scientific Advisory Board Chair and Primary Investigator on Phase 1a/1b study |
| **CEO** | Michael Spector (25+ years of leadership experience in pharmaceutical and biotechnology) |

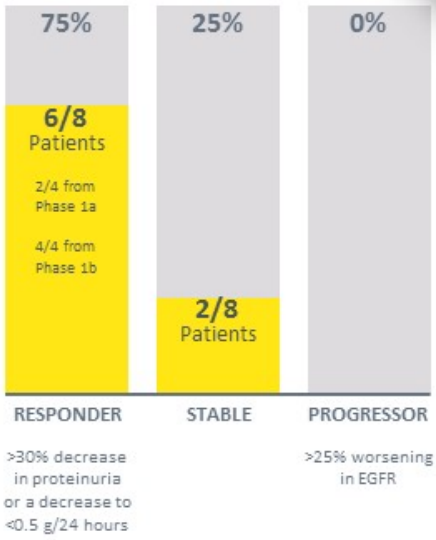
# CAEL-101 Phase 1a/1b Organ Response Rates




**Overall Responders**
**Best Organ Response**




**Cardiac Response Phase**
**1a & 1b (n=8)**




**Renal Response Phase**
**1a & 1b (n=8)**

# Immuno-Oncology

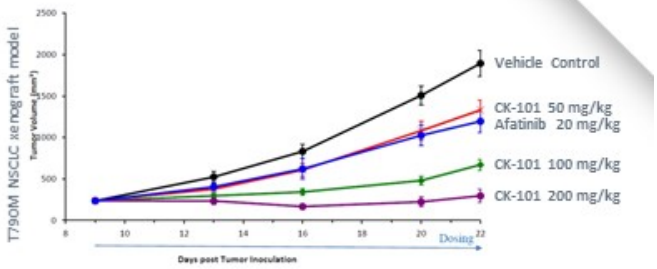
CHECKPOINT THERAPEUTICS

## Building a platform to combine targeted agents with immuno-oncology agents to maximize anti-cancer effect

| **Focus** | Acquire and develop novel immuno-oncology and targeted cancer agents alone and in combination to treat patients with solid tumors |
|---|---|
| **Market Size** | Anti-PD-(L)1 >$30B, Anti-GITR > $1B, CK-101 EGFR > $3B, CK-103 BET > $500M |
| **Product Candidates** | Two immuno-oncology "I/O" antibodies, licensed from Dana Farber<br>Four targeted anti-cancer agents |
| **Clinical Trials** | CK-101 (EGFR Inhibitor) Phase 1/2 study ongoing |
| **Milestones** | Mid- 2017: Anti-PD-L1 IND expected<br>2H 2017: CK-101 (EGFR Inhibitor) Phase 2 expected initiation<br>2H 2017: CK-103 (BET Inhibitor) target IND filing<br>2018: Anti-GITR target IND expected |
| **TGTX Collaboration** | Joint development of anti-PD-L1 and anti-GITR mAbs, and BET inhibitor program with Checkpoint developing solid tumor indications and TG in liquid tumors |
| **Funding** | ~$35M (12/31/16) to support development programs through 2018 |
| **CEO** | James Oliviero (15+ years of leadership experience in pharmaceutical and biotechnology, previously senior management of Keryx, achieving a new drug approval) |

# CK-101, 3rd Generation EGFR Pre-Clinical Efficacy

| $IC_{50}$(nM) | | | |
|---|---|---|---|
| Cell Line | A431 | H1975 | HCC827 |
| Mutation | EGFR Wild-Type | EGFR Mutant L858R / T790M | EGFR Mutant Exon 19 del |
| Afatinib | 34 | 23 | 1 |
| Tagrisso | 280 | 2 | 3 |
| CK-101 | 689 | 5 | 10 |




In vitro, CK-101 showed:

- Strong efficacy for T790M and other EGFR mutations
- Good selectivity for mutant over wild-type EGFR A431/H1975 ratio ~ 100 fold

In mice, CK-101 showed strong activity against T790M mutated NSCLC with increasing dose

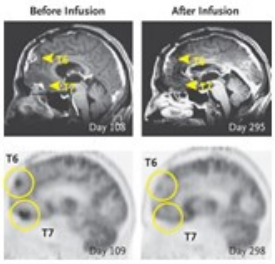
# Aggressive Forms of Cancer

MUSTANGBIO

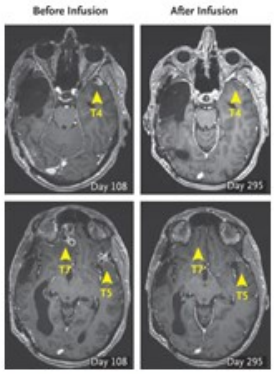
Robust CAR-T platform technology in partnership with pioneers in CAR-T technologies from City of Hope, recently raising a $94.5M private placement financing

| | |
|---|---|
| **Focus** | Two lead CAR-T programs targeting IL13Rα2 and CD123, for the treatment of Glioblastoma Multiforme and AML/BPDCN, respectively |
| **Market Size** | In the U.S., Japan and five major EU markets there are 30,000 newly diagnosed GBMs (malignant brain tumor) and 30,000 newly diagnosed cases of AML (acute myeloid leukemia) |
| **Product Candidates** | MB-101 IL13Rα2-specific CAR-T cells which have no current competition<br>MB-102 CD123-specific CAR-T cells which have been validated in ultra orphan indication |
| **Clinical Trials** | Two Phase 1 trials ongoing with preliminary safety data from at least 6 patients in both CAR-T programs |
| **Milestones** | Phase 1 data readouts early 2018 |
| **Licensor** | City of Hope |
| **Scientific Advisors** | Dr. Stephen Forman, City of Hope<br>Dr. Christine Brown, City of Hope |
| **Funding** | ~$94.5M (1/31/17) |
| **CEO** | Manuel Litchman, M.D. (20+ years of experience in pharmaceutical and biotechnology industry, including senior leadership positions in licensing, development and general management at Novartis and Arvinas LLC) |

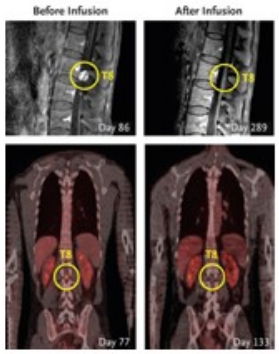
# Regression of Recurrent Multifocal Glioblastoma After Intraventricular Delivery of IL13Rα2-Targeted CAR T Cells



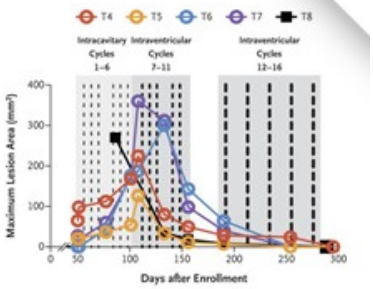

Sagittal MRI (top) and PET (bottom) of the brain




Axial MRI of the brain




All metastatic tumors in the spine were completely eliminated




Maximum lesion area for non-resected tumors 4 through 8 with their respective decreases over time

Clinical response was sustained for 7.5 months after the initiation of CAR T-cell therapy, and none of these initial tumors recurred. These results show that treatment with the CAR-T mediated a complete response.

Source: The New England Journal of Medicine. 2016;375:2561-9. 2016 Massachusetts Medical Society.

# Cytomegalovirus (CMV): Common Virus

## Three novel biologic immunotherapies (two in Phase 2) targeting billion dollar orphan market

| Focus | Develop novel immunotherapies for the prevention and treatment of CMV that can cause life-threatening disease in those with weak immune systems |
|---|---|
| Market Size | CDC estimates 50-80% infected with Cytomegalovirus (CMV) by age of 40<br>CMV in Allogeneic Stem Cell Transplant: U.S. Incidence ~8,000 / EU Incidence ~15,000<br>CMV in Allogeneic Solid Organ Transplant: U.S. Incidence ~8,000 / EU Incidence ~15,000 |
| Product Candidates | PepVax: HLA-restricted, single antigen CMV vaccine<br>Triplex: First universal, multi-antigen CMV vaccine |
| Clinical Trials | PepVax: Phase 2 ongoing, multi-center, double-blind trial for stem cell transplant (n=96) Phase 1b showed safe, effective and Published in Lancet Dec 2015<br>Triplex: Phase 2 ongoing, multi-center, double-blind trial for stem cell transplant (n=115) Phase 1 showed safe, immunogenic. Presented ASH 2015. Published in Blood Nov 2016 |
| Upcoming Milestones | Triplex: Phase 2 topline 100 day data by 2H2017<br>PepVax: Phase 2 topline data by 1H2018 |
| Licensor | City of Hope |
| Funding | Total budget (thru 1H2019): ~ $30M (Including $8M NCI grant funding) |
| CEO | Frank Taffy (15+ years of experience at Forest Labs and Life Tech in corporate development and operations) |

# Phase 1 Studies: Journal Publications

## Phase 1b
(Completed, Published in *The Lancet*)

| | |
|---|---|
| **Design** | Single-Center (City of Hope) Study in 36 Allogeneic HSCT CMV(+)Recipients Randomized (1:1) between Vaccine Arm (VA) and Observation Arm (OA) |
| **Dosing Schedule** | Two subcutaneous vaccinations after transplant<br>• Day 28<br>• Day 56 |
| **1° Endpoint** | Overall safe and well-tolerated<br>**Published in *The Lancet Haematology* (12/28/2015)** |
| **2° Endpoint** | • Increase in CD8+T-cells<br>• Reduced CMV Reactivation, 6% vs.33%,p=0.044<br>• Reduced Relapse, 6% vs. 28%, p=0.015<br>• Reduced Death, 0vs. 39% |

| | Vaccine (n=18) | Observation (n=18) |
|---|---|---|
| Patients with serious adverse events | 4 (22%) | 9 (50%) |
| Disease relapse | 1 (6%) | 5 (28%) |
| Death | 0 (0%) | 7 (39%) |
| CMV viraemia (≥500 gc/mL) | 1 (6%) | 6 (33%) |

## Phase 1
(Completed, Published in *Blood*)

| | |
|---|---|
| **Design** | Single-Center (City of Hope) Dose Escalation (three levels) in 24 Healthy Volunteers (CMV +/-) |
| **Dosing Schedule** | Two IM injections four weeks apart<br>Last patient dosed 4/2015 |
| **1° Endpoint** | Safe and well-tolerated in all dose cohorts<br>**Presented at *ASH* (December 2015)**<br>**Published in *Blood* (November2016)** |
| **2° Endpoint** | ↑pp65-, IE1-, IE2-specific CD8 and CD4 T-cells<br>**Particularly pronounced increase in T-cells in those with low baseline levels** |

# Rare & Fatal Pediatric Diseases

CYPRIUM THERAPEUTICS

## A novel therapy in Phase 3 clinical trial being developed for patients with Menkes Disease

| | |
|---|---|
| **Focus** | Developing novel therapies for the treatment of rare, fatal pediatric diseases, with initial focus on Menkes disease and related copper metabolism disorders |
| **Market Size** | Menkes disease is a rare X-linked pediatric disease caused by gene mutations of copper transporter ATP7A, which affects approximately one in 100,000 newborns per year. |
| **Product Candidate** | CUTX-101 (Copper Histidinate injection) is being developed to replenish copper levels in patients with Menkes disease. A preclinical AAV-based ATP7A gene therapy is being developed to deliver working copies of ATP7A to Menkes patients. Both programs have FDA Orphan Drug Designations. |
| **Clinical Trials** | In Phase 1/2 clinical studies conducted at NICHD, early treatment of Menkes patients with CUTX-101 led to an improvement in neurodevelopmental outcomes and survival. |
| **Milestones** | Natural History Study of untreated Menkes patients in 1H2017<br>FDA meeting to confirm regulatory pathway in 2017 |
| **Licensor & Scientific Advisor** | *Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD), part of the National Institutes of Health (NIH): March 2017 (CRADA & Exclusive License Agreement)<br>Stephen G. Kaler, M.D., Senior Investigator and Head, Section on Translational Neuroscience, Molecular Medicine Branch, NICHD<br>Principal Investigator for Menkes disease clinical studies |
| **CEO** | Lung S. Yam, M.D., Ph.D. (Senior Analyst, Opus Point Partners; BD Consultant involved in identifying and in-licensing of multiple assets to Fortress and affiliated companies) |

# IV Tramadol For Acute Post Surgical Pain

IV Tramadol, if approved, would be the only Schedule IV intravenous opioid in the U.S.

| Focus | IV tramadol for the treatment of post-surgical pain |
|---|---|
| **Market Size** | IV analgesics sells ~$1bn per year in the U.S.<br>IV acetaminophen sells >$250MM with ~3 to 4% of the unit volume |
| **Product Candidate** | Intravenous (IV) Tramadol, an opioid **without the typical side effects of narcotics**, for the treatment of moderate to moderately severe pain |
| **Regulatory Path** | 505b(2) |
| **Status** | Phase 3 ready |
| **Funding** | ~$30M to complete Phase 3 |
| **CEO** | Lucy Lu, M.D. (CFO, Fortress Biotech and previously Citi biotechnology equity research analyst) |

# Survey of Anesthesiologists: Favorable View of IV Tramadol

| Overall Impression | |
|---|---|
| Favorable initial impression of Tramadol as a potential new IV analgesic | 77% |

| Patients Taking | Switch To IV Tramadol | Add IV Tramadol |
|---|---|---|
| IV Morphine | 40% | 41% |
| IV NSAIDS | 26% | 37% |
| IV Acetaminophen | 24% | 35% |

Survey of 30 U.S. Anesthesiologists. Conducted through LEERINK and available upon request.

# Innovative Dermatology Products

JOURNEY MEDICAL CORPORATION

Team of industry experts successfully launched four dermatology products in 12 months

| | |
|---|---|
| **Focus** | Identify and commercialize innovative, differentiated prescription dermatology products through efficient and potent sales and marketing model |
| **Product Candidates** | Targadox *(doxycyline tablets)*: Severe acne<br>Ceracade *(skin emulsion)*: Atopic and various types of dermatitis<br>Luxamend *(wound cream)*: Wounds from superficial to full thickness and 1st and 2nd degree burns<br>Dermasorb HC *(hydrocortisone lotion)* Kit: Seborrheic dermatitis |
| **Market** | 5,000 top prescribing dermatologists |
| **CEO** | Claude Maraoui (25+ years commercializing dermatology products; previously Vice President of Sales at Medicis) |





FORTRESS BIOTECH

# CEVA101: Severe Traumatic Brain Injury

Cellvation

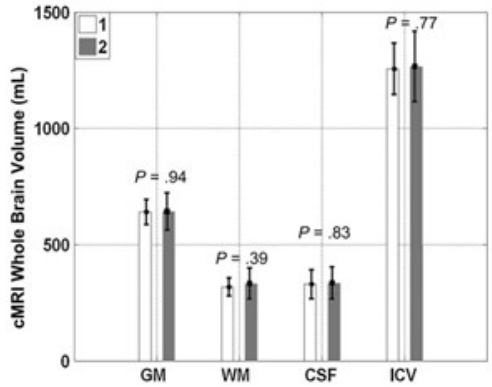
No approved reparative therapy for treatment of severe TBI. Now have CEVA101, a biologic, that minimizes the secondary injury associated with TBI.

| Focus | Develop novel biologic therapies for TBI treatment |
|---|---|
| **Market Size** | 200,000 adults / 50,000 children with TBI |
| **Product Candidate** | CEVA101: Autologous bone-marrow derived mononuclear cells |
| **Clinical Trials** | Two ongoing Phase 2 studies, one adult and one pediatric<br>Phase 1 in Adult TBI: Published in ***Stem Cells***, November 2016 |
| **Milestone** | Phase 2 data in Children by 1H2018, in Adults by 1H2019<br>Potential for accelerated approval in Japan<br>Potential for pediatric voucher |
| **Licensor** | Two technology platforms from University of Texas Health Science Center |
| **Funding** | NIH/DOD Grant Funding: $10M, low capital requirement |
| **CEO** | Frank Taffy (15+ years of experience at Forest Labs and Life Tech in corporate development and operations) |

FORTRESS BIOTECH

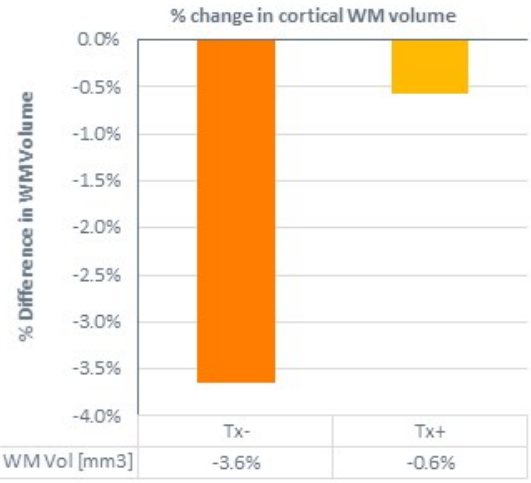
# Structural Preservation Through Cell Therapy

## CEVA101 Phase 1: Volumetric Preservation in Pediatric TBI




- Post-TBI MRI at Month 1 (Scan 1) versus Month 6 (Scan 2)
- Preservation of Grey Matter (GM), White Matter (WM), Intracranial Volume (ICV)

## CEVA101 Phase 1: Volumetric Preservation in Adult TBI



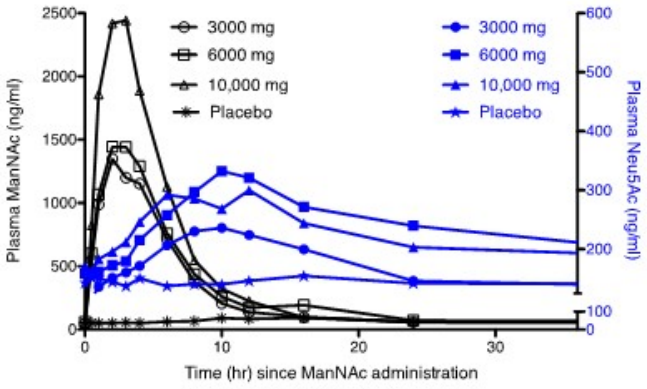
# Rare & Orphan Diseases

GNE Myopathy has no approved therapies. FDA granted ManNAc orphan designation.

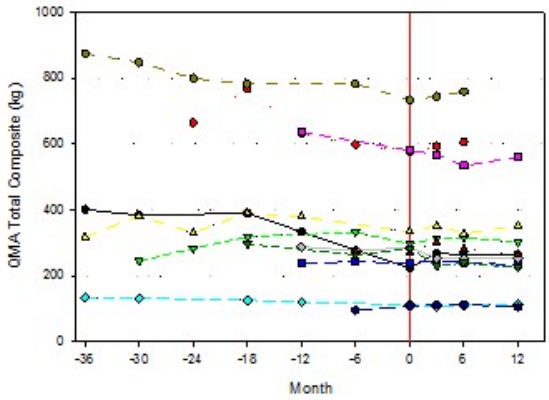
| | |
|---|---|
| **Focus** | Develop and commercialize b.i.d oral treatment for GNE Myopathy and primary podocyte nephropathies. No other company has this focus. |
| **Market Size** | GNE Myopathy: U.S. – 400 and WW – 2000 diagnosed<br>Nephropathy (including diabetic): ~220,000 WW |
| **Product Candidate** | ManNAc=N-Acetyl-D-Mannosamine, a naturally-occurring monosaccharide and precusor to sialic acid |
| **Clinical Trials** | In collaboration with NIH on 3 clinical studies GNE Myopathy: Natural History study ongoing ,Phase 2 open label ongoing, Phase 1 completed<br>Primary Podocyte Nephropathies: Phase 1 trial in progress (recruiting) |
| **Upcoming Milestone** | Phase 2 GNE Myopathy trial ongoing and Phase 3 planned for 2017 |
| **Licensor** | Acquired from New Zealand Pharmaceuticals Ltd which is the exclusive global supplier of ManNAc |
| **CEO** | Hootan Khatami, MD (12+ years of pharmaceutical and biotechnology experience at Genzyme/Sanofi, Roche/Genentech, Merck & Daiichi Sankyo) |

# ManNAc Clinical Data

## Phase 1 PK Results: ManNAc + Neu5Ac

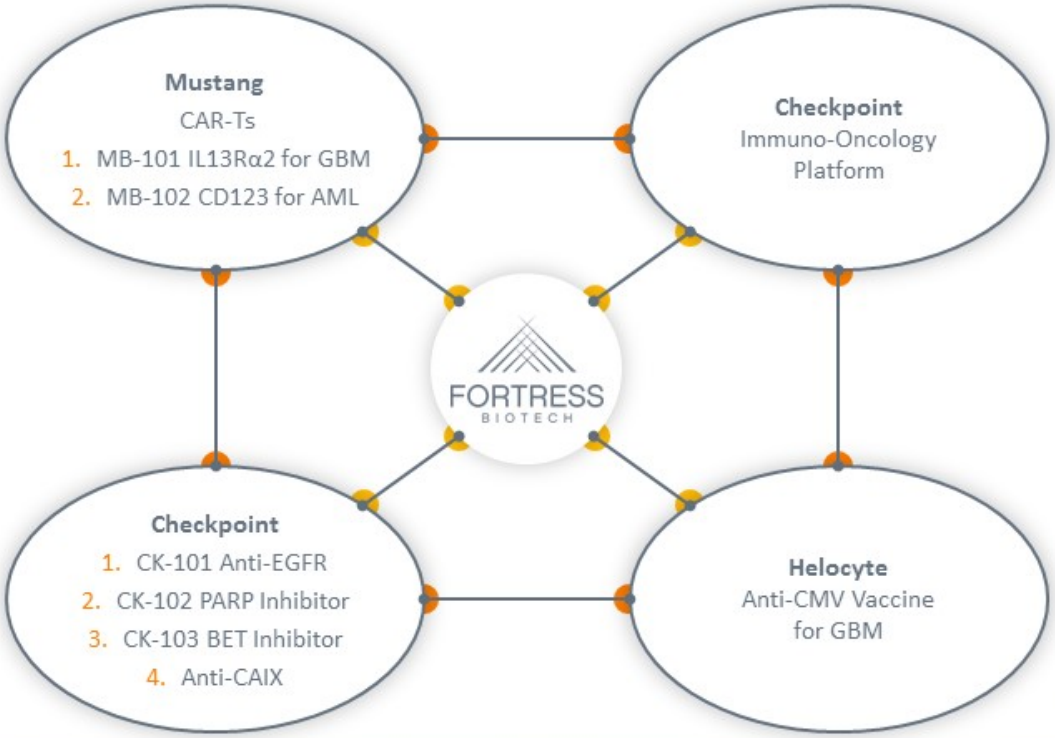



## Open Label Phase 2 Study




- 12 subjects on ManNAc were also previously in the Natural History Study
- Nearly all had evidence of preservation of muscle strength after 12 months on ManNAc

# Synergies Between & Among Subsidiaries



# Fortress Subsidiaries Are Creating A Pipeline of Therapies For Life-Threatening Diseases

| Accelerated Drug Development Model | Diversified Pipeline | Experienced, Proven Leadership |
|---|---|---|

# Corporate Presentation

FORTRESS BIOTECH

May 2017