

cellceutix™

Ticker: CTIX

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Biotech Showcase
11 January 2016



Forward Looking Statements

To the extent that statements in this presentation are not strictly historical, including Statements as to revenue projections, business strategy, outlook, objectives, future milestones, plans, intentions, goals, future financial conditions, future collaboration agreements, the success of the Company's development, events conditioned on stockholder or other approval, or otherwise as to future events, such statements are forward-looking, and are made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995.

The forward-looking statements contained in this presentation are subject to certain risks and uncertainties that could cause actual results to differ materially from the statements made. Factors that may impact Cellceutix's success are more fully disclosed in Cellceutix's most recent public filings with the U.S. Securities and Exchange Commission.

Management Team

Name	Title	Previous Affiliations
Leo Ehrlich, CPA	CEO, CFO, Board of Directors	Co-Founder of Cellceutix; Investor
Krishna Menon, PhD, DVM	President, CSO, Board of Directors	Co-Founder of Cellceutix; Eli Lilly
Daniel Jorgensen, MD, MPH, MBA	Chief Medical Officer	Pfizer, Pasteur Merieux Connaught
Ed Walters, MPH	Head of Clinical Operations	Allergan, Schering Plough, Eisai

Board of Directors: Leo Ehrlich, CPA; Krishna Menon, PhD, DVM; Barry Schechter, MD; Zorik Spektor, MD, Mark Tobin, MBA

Company Overview

- Exceptionally strong pipeline, novel mechanisms of action
- Three compounds in four clinical trials:
 - Brilacidin: Acute Bacterial Skin and Skin Structure Infections (ABSSSI)(phase 2b complete, preparing for phase 3), oral mucositis (phase 2)
 - Kevetrin: advanced solid tumors (phase 1), preparing phase 2 for ovarian cancer
 - Prurisol: psoriasis (phase 2)
- FDA designations for expedited drug development
 - Fast Track: Brilacidin-OM for oral mucositis
 - Orphan Drug: Kevetrin for ovarian cancer, Kevetrin for retinoblastoma
 - Pediatric Rare Disease: Kevetrin for retinoblastoma
 - Qualified Infectious Disease Product: Brilacidin for ABSSSI
- Experienced team with strong background:
 - Research: chemistry and biology
 - Development: clinical and regulatory
 - Expansion into other areas (commercial, business development, etc.)
- **Mission:** *To develop innovative compounds that address unmet medical needs*

Pipeline

Product	Indication	Pre-Clinical	Phase 1	Phase 2	Phase 3
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Infectious Disease

BRILACIDIN (intravenous)	Skin Infections (ABSSSI) ¹				
	Diabetic Foot Infections (IV & Topical) ²				
	Bone and Joint Infections				
TRIARYLS	Fungal Infections				
	Resistant Gram-negative Infections				
CTIX-1807	Carbapenem Resistant Enterobacteriaceae (CRE)				

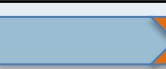

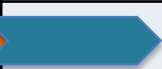
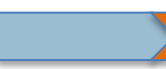
Cancer

KEVETRIN	Advanced Solid Tumors				
	Ovarian Cancer ³				
	Retinoblastoma ⁴				
	Acute Myelogenous Leukemia ⁵				
BRILACIDIN-OM	Oral Mucositis ⁶				

Pipeline (cont.)

Product	Indication	Pre-Clinical	Phase 1	Phase 2	Phase 3
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Dermatology

PRURISOL	Psoriasis				
BRILACIDIN	Hidradentitis Suppurativa ²				

Gastrointestinal

BRILACIDIN	Ulcerative Colitis/Proctitis ²				
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¹ Being developed under a Qualified Infectious Disease Product (QIDP) designation from U.S. Food and Drug Administration


² Leveraging safety data from Phase 2 or Phase 3 ABSSSI trials to later-stage trials in other indications

³ Being developed under an Orphan Drug designation from the U.S. Food and Drug Administration

⁴ Orphan Drug and Rare Pediatric Disease designations from the U.S. Food and Drug Administration

⁵ Proposed study. Trial is awaiting sponsor's funding and is subject to postponement, modification or cancellation based upon study financing.

⁶ Fast Track designation from the U.S. Food and Drug Administration

 Indicates data from previous studies being utilized to proceed to Phase 2 studies

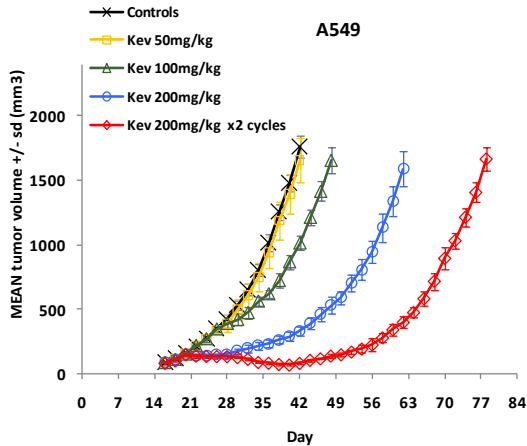
Cancer Program – Kevetrin Overview

FDA Orphan Drug designations for ovarian cancer and retinoblastoma; FDA Pediatric Rare Disease designation for retinoblastoma

- Unique Mechanism of Action (MOA)
 - Activity in wild type and mutant p53
 - Transcriptional dependent and independent
- Large therapeutic index
 - Potent anti-tumor activity even in drug resistant tumors
 - Excellent toxicity profile
- No development of drug resistance
- Non-genotoxic
- Small molecule; structurally distinct from other oncology agents
- Strong IP position; many novel compounds in patent application
- Efficacious in three drug-resistance tumor models
 - Dose-dependent anti-tumor activity
 - No resistance with second cycle
- Potential combination with radiation and with other drugs
- Simple API synthesis and formulation

Cancer Program – Kevetrin

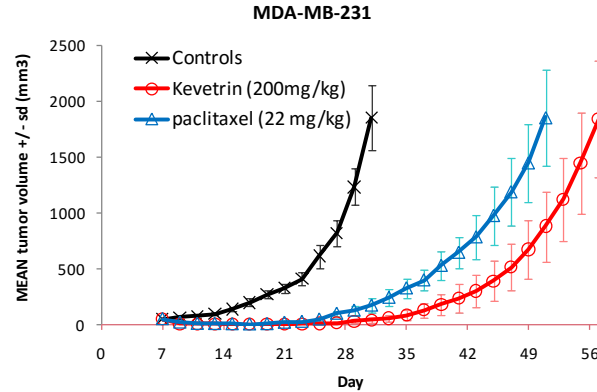
Anti-tumor Activity in Human Tumor Models



A549
lung adenocarcinoma

wild type p53

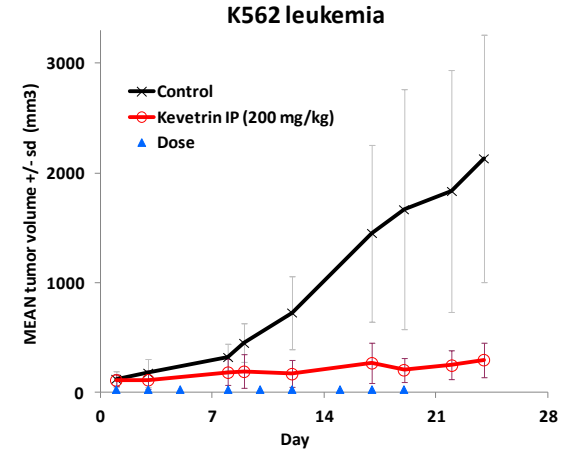
Reduced tumor growth in a dose-dependent manner



MDA-MB-231
breast carcinoma

mutant p53

Reduced average tumor volume by 98%
Taxol reduced average tumor volume by 91%



K-562
chronic myelogenous leukemia

p53 null

Reduced average tumor volume by 84%

Cancer Program – Kevetrin

Phase 1 Clinical Trial (in progress)



Dana-Farber Cancer Institute
Beth Israel Deaconess Medical Center (BIDMC)



- Phase 1 trial expanded into recently completed **11th cohort** at **750 mg/m²**
 - Subjects are late-stage cancer patients that have exhausted all other cancer treatment options
 - Trial has exceeded target enrollment and trial could be concluded. Cellceutix is collaborating with hospital and advisors on an advanced experimental phase in the trial. The trial has provided more than sufficient data for Cellceutix to proceed with Phase 2 trials for different indications, such as ovarian cancer, with a mid-stage trial planned for the first half of 2016.

- Pharmacokinetic parameters show approximate dose-dependent exposure; but similar terminal half-life, clearance, volume of distribution, as expected.
 - Parameters suggest rapid and extensive distribution from systemic circulation into tissues

- Only 1 dose limiting toxicity has been observed to date

- p21 is a biomarker for p53 activation
 - p21 increased by $\geq 10\%$ in 48% of the 31 evaluable patients
 - p21 increased in 11 (73%) of patients with gynecological cancers

Upcoming Clinical Trials

- Phase 1b/ 2 clinical trial: Ovarian Cancer – Orphan Drug Designated
 - Dosing planned to be multiple doses weekly based on PK. Phase 1 trial was only 1 dose weekly

- Phase 2 clinical trial: Acute Myelogenous Leukemia (AML)
 - Trial Design: Evaluate Kevetrin administered intravenously with Cytarabine administered either subcutaneously or intravenously
 - Trial currently awaiting funding by the sponsor and is subject to postponement, modification or cancellation based upon study financing

- Phase 1/2 clinical trial: Retinoblastoma
 - Orphan Drug designated, Rare Pediatric Disease designated
 - IND-enabling work underway

- Phase 1b/2 clinical trial: Drug Resistant Renal Cancer
 - Beth Israel Deaconess Medical Center; largest recipient of SPORE grant
 - Clinical study in renal cancer patients at BIDMC is under discussion based upon pre-clinical research

Cancer Program – Brilacidin for Oral Mucositis

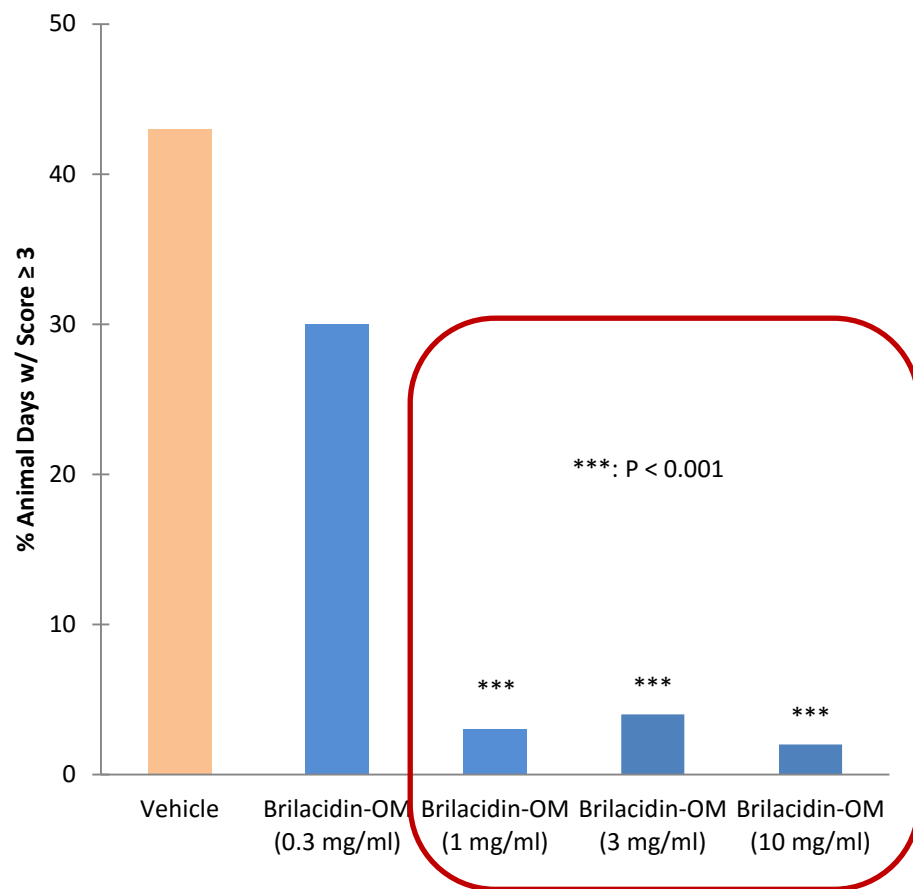
Oral Mucositis Overview

- Frequent complication of chemoradiation for head and neck cancers
 - May appear within 5 – 10 days of start of chemoradiation treatment
 - Can persist 1 – 6 weeks or longer depending on severity
- Painful and debilitating inflammation and ulceration; increases susceptibility to bacterial infections
 - Probability is increased with poor dental hygiene; any and all use of tobacco products; overall poor health; and previous chemoradiation treatment for head and neck cancers
- Patients unable to speak or eat
 - Often requires insertion of feeding tube
- Can be dose-limiting, leading to reduction/cessation of radiation and chemotherapy for cancer
- Severe cases require hospitalization
 - Increased overall cost of cancer treatment
- No currently approved medications for prevention of oral mucositis in this population



Brilacidin-OM Reduces Ulceration

Acute Radiation Model in Hamsters



Data presented at ASCO, June 2012

Study Design:

- Brilacidin-OM administered 3x/day as topical rinse @ doses of 0.3, 1, 3 or 10 mg/ml over 28 days

Results seen with Brilacidin-OM:

- **Reduced % animal days w/ ulcerative oral mucositis by >90%, from 42.7% to 2-4%**
- High statistical significance

Brilacidin for Oral Mucositis

Phase 2 Clinical Trial in progress under FDA Fast Track Designation

Study Design:

- Phase 2, Multi-center, Randomized, Double-blind, Placebo-controlled (10-20 sites in US)
- 60 subjects – 30 each of drug or placebo (Water for Injection)
- “Swish and spit” brilacidin 3x/daily for 7 weeks – 16 ml oral rinse
- First subject enrolled July 2015
- Interim analysis after 36 subjects (18 per group) by an independent data monitoring committee (DMC);
 - Targeted for second half of 2016

Primary endpoints:

Control and prevent oral mucositis in patients receiving chemoradiation therapy for Head and Neck Cancer

- To evaluate the efficacy of topically applied brilacidin vs. placebo in delaying the onset of severe OM (WHO Grade ≥ 3)
- To evaluate the safety and tolerability of topically applied brilacidin administered three times daily for approximately 7 weeks

Secondary endpoints:

- Assessing the ability of brilacidin to reduce the incidence and duration of ulcerative and severe OM (WHO Grade ≥ 2)
- Other secondary endpoints will include assessment of the following due to OM:
 - Mouth and throat soreness, analgesic consumption for pain
 - Use of gastrostomy tube for nutritional support
 - Number of unplanned office visits/ER visits for hospital admissions
 - Incidents of unplanned delays and/or breaks in chemoradiation therapy

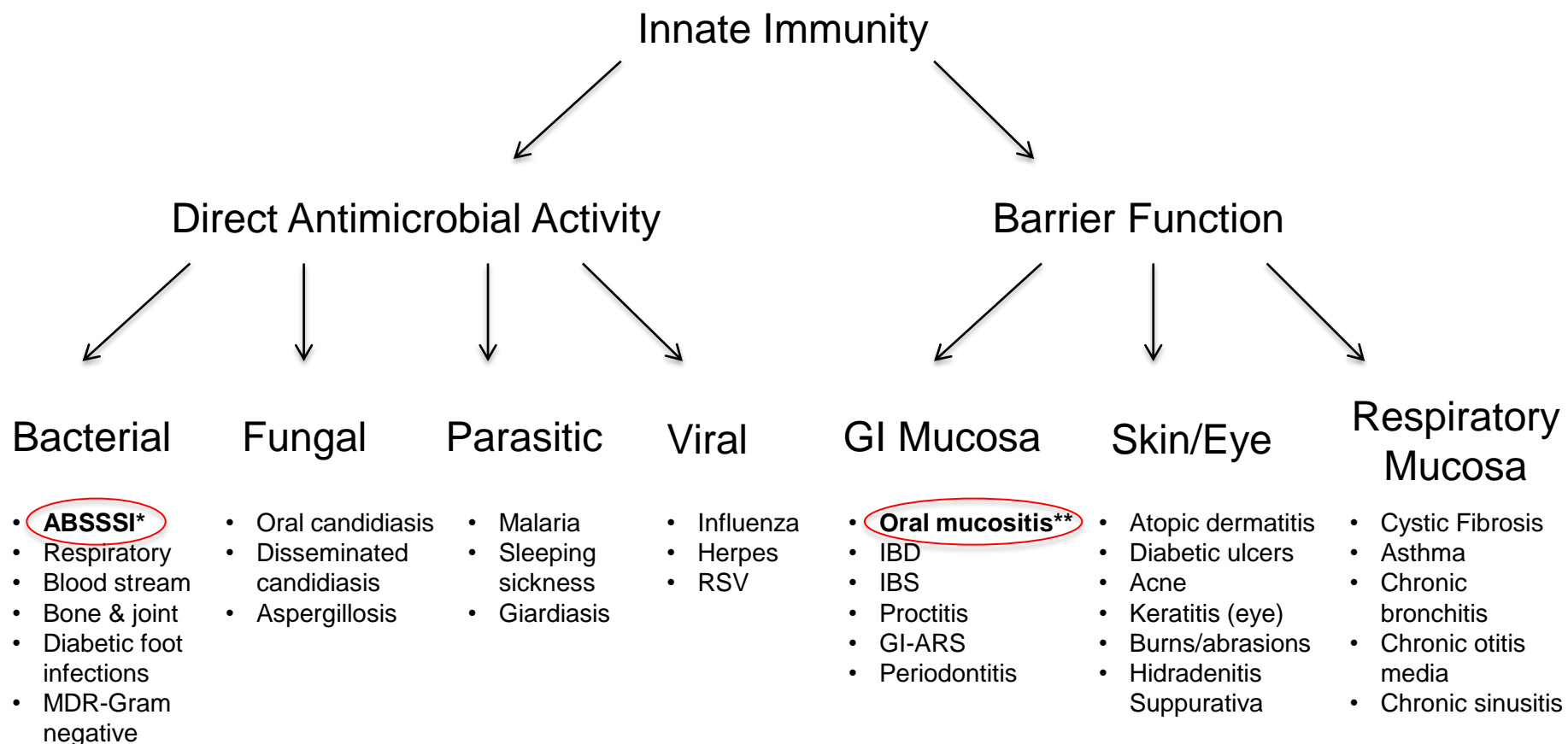
Brilacidin & Host Defense Protein (HDP) Mimics

Background & Rationale

- **Develop small non-peptidic, fully synthetic mimics of Host Defense Proteins (HDPs) as systemic and topical agents**
 - Novel approach for bactericidal activity
- **HDPs are small antimicrobial peptides**
 - Expressed widely in the animal kingdom
 - Produced in skin, mucosal surfaces, neutrophils
 - Target microbial membrane
- **First line of defense against bacterial invasion**
 - Part of innate immune system
 - Maintenance of epithelial barrier function
 - Regulate microbiota
 - Immunomodulatory activities – link innate and adaptive immunity
- **HDP dysfunction implicated in inflammatory disorders of skin and mucosal surfaces**
 - Inflammatory bowel disease, atopic dermatitis, acne, skin infections,...
- **Address Global Problem of Antimicrobial Resistance**
 - Gram-positive (e.g., MRSA) and Gram-negative (e.g., CRE) development programs
 - Identified by CDC and FDA as high priority pathogens

HDM Mimics for Other Indications

Platform Opportunities & Gateway Concept for HDP Mimics



*ABSSSI is gateway for antibiotic opportunities

**OM is gateway for anti-inflammatory opportunities

Brilacidin ABSSSI Market Opportunity

An FDA approved Brilacidin for ABSSSI has the potential to be the market leader in a \$1 Billion+ Market

	Brilacidin	Daptomycin	Tedizolid	Dalbavancin	Oritavancin	Telavancin
Company	Cellceutix	Merck	Merck	Pfizer	Medicines Co.	Theravance
Stage	Phase 3	Marketed	Marketed	Marketed	Marketed	Marketed
Drug Class	HDP mimic	Lipopeptide	Oxazolidinone	Lipoglycopeptide	Lipoglycopeptide	Lipoglycopeptide
Dosing	0.6 mg/kg x 1 (X mg maximum)	4 mg/kg q12h x 7 – 14d (ABSSSI)	200 mg qd x 6d (also PO)	Day 1: 1000 mg Day 7: 500 mg	1,200 mg x 1	10 mg/kg qd x 7 – 21d
Infusion Time	1 hour	2 minutes, or 30 minutes	1 hour	30 minutes	3 hours	1 hour

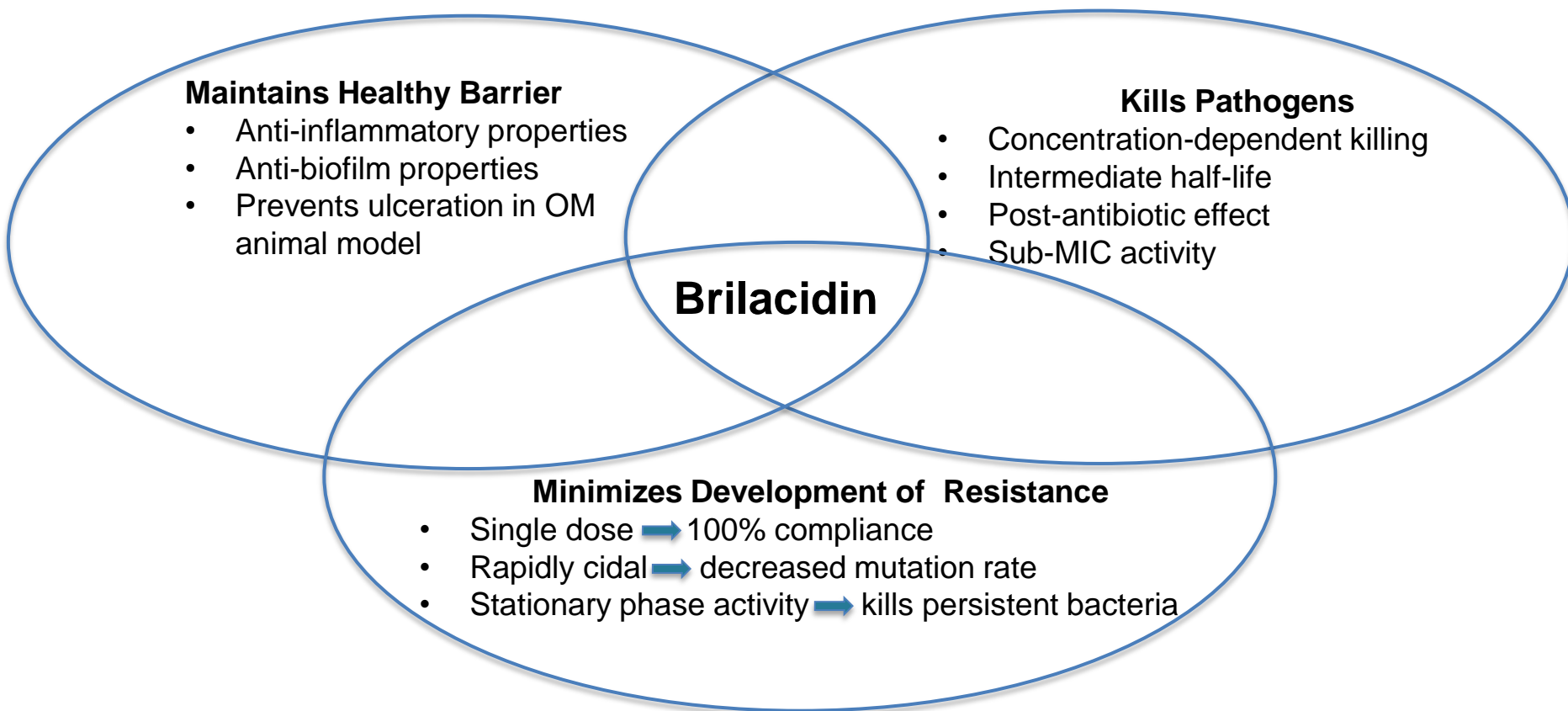
Daptomycin (brand name “Cubicin”) was developed Cubist Pharmaceuticals and generated \$1.05 billion in sales in 2014. Cubist also developed tedizolid (“Sivextro”) and earned FDA approval for ABSSSI in June 2014. In December 2014, Merck & Co. acquired Cubist in a \$9.5 billion deal.

Dalbavancin (“Dalvance”) was developed by Durata Therapeutics and received FDA approval in May 2014 for treating ABSSSI. In October 2014, Actavis agreed to acquire Durata for about \$675 million. Actavis became Allergan in March 2015, and is in the process of merging with Pfizer.

Oritavancin (“Orbactiv”) was developed by The Medicines Co. and FDA approved for ABSSSI in August 2014.

Telavancin (“Vibativ”) was developed by Theravance and FDA approved for ABSSSI (cSSSI) in September 2009.

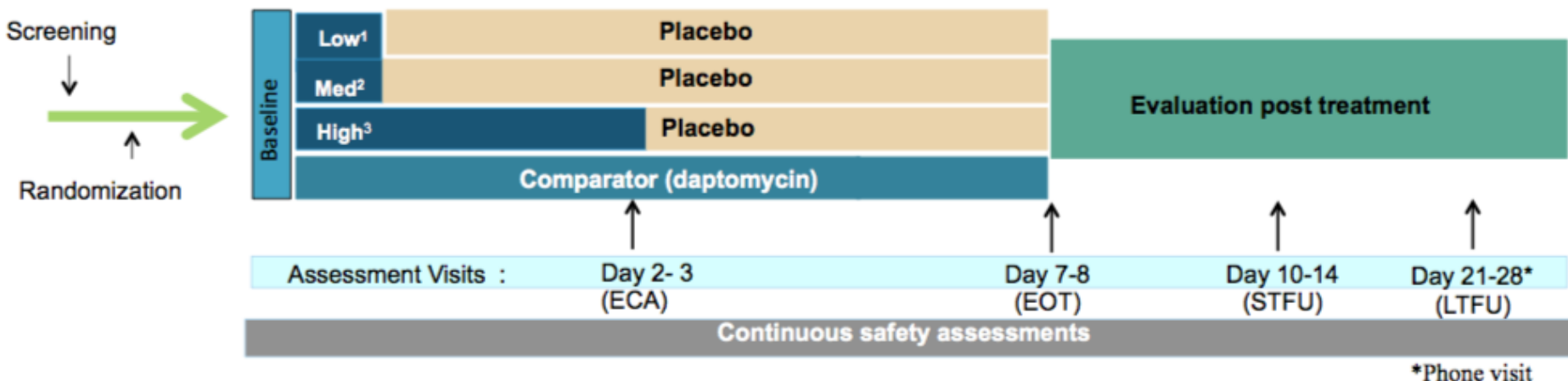
Gram-Positive Lead Compound: Brilacidin



Novel immunomodulatory antibiotics to treat infectious diseases and disorders of innate immunity

CTIX-BRI-204 Study (Phase 2b)

Study Design for ABSSSI



¹Low (0.6mg/kg single dose)

²Med (0.8mg/kg single dose)

³High (0.6 mg/kg D1; 0.3 mg/kg D2 & D3)*

*Highest total dose 1.2 mg/kg is less than lowest total dose of 1.6 mg/kg in 203 study

- **Trial conducted at 4 sites in U.S.**
- **Dosing: IV infusion 1x/day for 7 days**
 - 1 or 3 days on BRI + 4 or 6 days on placebo; or 7 days on daptomycin
- **215 patients, 4 arms, ~50 patients per arm**
- **ABSSSI definition (FDA Guidance, Oct 2013)**
 - ≥ 75 sq. cm² (redness, edema, and/or induration)
 - Wound, major cutaneous abscess, cellulitis/erysipelas
- **Early Clinical Response (48-72 hours)—FDA endpoint**
- **Clinical Response (Day 7-8; Day 10-14)—EMA endpoint**

CTIX-BRI-204 Study (Phase 2b)--Primary Endpoint

Early Clinical Response at 48-72 Hours*

	0.6 mg/kg IV x 1 Day (N=53)	0.8 mg/kg IV x 1 Day (N=53)	Brilacidin x 3 days (N=53)	Daptomycin (N=50)
N assessed	51	48	52	48
Clinical Response (%)	47 (92.2)	46 (95.8)	51 (98.1)	45 (93.8)
95% C.I.	(84.8, 99.5)	(90.2, 100)	(94.3, 100)	(86.9, 100)

ATS = All Treated/Safety Population
Pre-specified analysis population in statistical analysis plan for primary endpoint

Per FDA Guidance – ABSSSI (October, 2013)

*Data presented at ECCMID, April 2015

Brilacidin ABSSSI Program Summary

- Brilacidin was safe and effective in two Phase 2 studies
- Convenient single-dose regimen
 - Pharmacoeconomic advantages
- QIDP designation (November 2014) under the GAIN Act
 - Eligible for Fast Track and Priority Review
- Minimal potential for development of resistance
 - Novel class, no cross-resistance
 - Novel mechanism of action confers fitness disadvantage for bacterial resistance
 - Single dose removes non-compliance as driver for resistance
- Immunomodulatory, with anti-biofilm properties
 - May accelerate the healing process
- Phase 3 planning in progress
 - FDA End-of-Phase 2 Meeting in July 2015

Brilacidin is the first completely novel antibiotic to enter a phase 3 trial in ABSSSI in more than two decades.

Gram-Negative & Fungal Program Summary

HDP Mimics for MDR Infections, including CRE

- **Gram-negative activity evident in several structural series of small non-peptidic mimetics of host defense proteins**
- **2 series show low cytotoxicity, favorable PK properties and robust efficacy *in vitro* and *in vivo* against *Enterobacteriaceae* organisms**
 - CC-1807 is potently active against clinical isolates of *E. coli*, *K. pneumoniae* and *E. cloacae*, including MDR CRE strains
- **Additional preclinical efficacy studies are in progress**
 - Lung infection models
 - UTI and bacteremia
- **Chemical optimization of CC-1807 and additional analogs is continuing**
 - Expand coverage to *Pseudomonas* and *Acinetobacter* spp.
- **New triaryl analogs show promising broad spectrum and potent anti-bacterial and anti-fungal activity with low cytotoxicity**

Gram-Negative Program – CC-1807

HDP Mimics vs. MDR Enterobacteriaceae, including CRE

Activity vs. recent collection of clinical isolates

Organism (20 isolates)	MIC ₉₀ (µg/ml, CLSI)			
	CC-1807	Levofloxacin	Gentamicin	Meropenem
<i>Escherichia coli</i>	0.25	>4	32	0.015
<i>Klebsiella pneumonia</i>	1	>4	1	4
<i>Enterobacter spp.</i>	1	0.5	0.5	0.25

Activity vs. MDR* Enterobacteriaceae clinical isolates

Organism	MIC ₉₀ (µg/ml, CLSI)			
	CC-1807	Levofloxacin	Gentamicin	Meropenem
<i>Escherichia coli</i> (4 isolates)				
MIC range	≤0.06 – 0.12	>4	1 - >64	0.008 – 0.03
<i>Klebsiella pneumonia</i> (8 isolates)				
MIC range	0.25 – 1	>4	≤0.06 - >64	0.06 - >4
<i>Enterobacter cloacae</i> (3 isolates)				
MIC range	0.25 – 2	2 - >4	0.25 - >64	0.25 - >4
MDR Enterobacteriaceae (15 isolates)				
MIC range	≤0.06 – 2	2 - >4	≤0.06 - >64	0.008 - >4
MIC ₅₀	0.5	>4	64	0.5
MIC ₉₀	2	>4	>64	>4

*resistant to ≥ 3 antibiotic classes

Chronic Plaque Psoriasis

Psoriasis Market Opportunity:

For Mild and Moderate Chronic Plaque Psoriasis

- Huge market opportunity
 - Affects 7.5M Americans
 - Mild to Moderate is largest segment

- Chronic, autoimmune disease

- Problems with commercially available treatment options:
 - Side effects – sometimes severe
 - Lack of sufficient efficacy

Prurisol mechanism of action (MOA):

- Acts through immune modulation and PRINS reduction
 - Reduces IL-20
 - Reduces skin cell proliferation rate

Prurisol Attributes:

- Strong IP
- Small-molecule (<500 MW)
- Bioavailable
- Excellent in-vivo and in-vitro activity
- Efficacy in xenograft model
- Oral Dosing

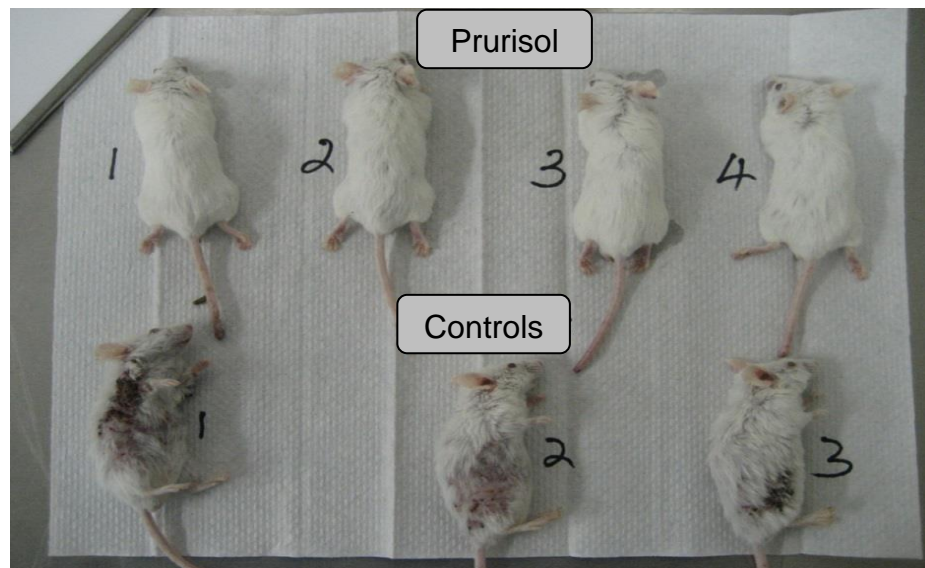
Animal Model of Psoriasis

Study design:

- Mice engrafted with human psoriatic tissue
- Group of 10 mice were treated with either:
 - 10 mg/kg of Prurisol orally once or twice a day for 21 days
 - 7.5 mg/kg methotrexate IP once/day
 - Untreated mice as controls
- Mice were followed for 180 days.

Results:

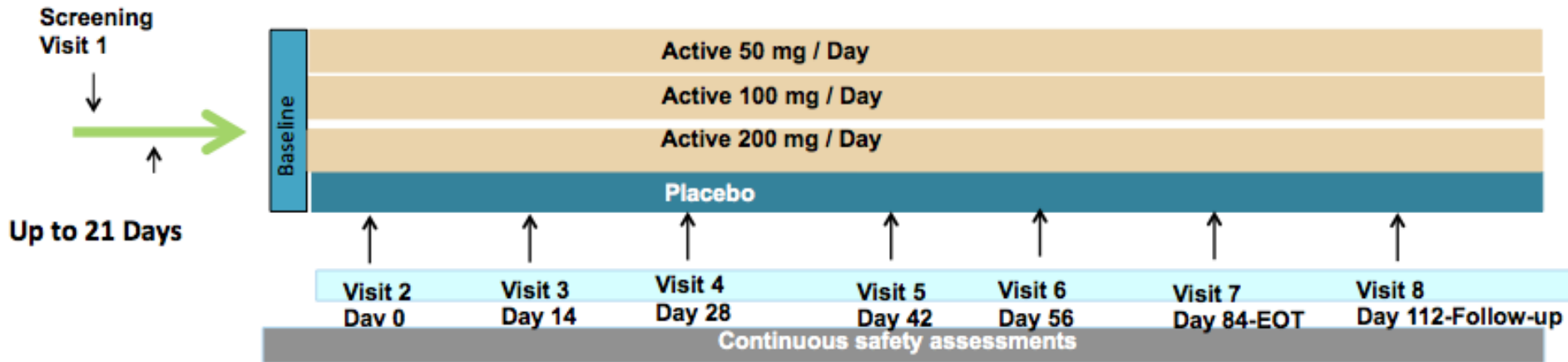
- Prurisol given at 10 mg/kg twice/day was shown to be more effective than methotrexate in reducing psoriatic skin lesions in a human xenograft model
- Prurisol can be given orally and is well-tolerated.



A variation of Prurisol in human xenograft model. The top row animals show a relatively clean coat with limited evidence of psoriasis. The bottom row shows the untreated control animals.

Dermatology Program – Prurisol

Phase 2 Trial Design



Dose Groups:

50 mg: AM: 1 Placebo & 1 Active PM: 2 Placebo
100 mg: AM: 1 Placebo & 1 Active PM: 1 Placebo & 1 Active
200 mg: AM: 2 Active PM: 2 Active
Placebo: AM: 2 Placebo PM: 2 Placebo

Primary efficacy endpoint is percentage of subjects with ≥ 2 point improvement in IGA rating [a rating of “clear” (0) or “almost clear” (1) per the IGA rating] at 84 days.

- Trial conducted at 10 sites in U.S.
- Randomized, Double Blind
- Prurisol (abacavir acetate) Tablets: 50 mg
- Dosing: Two (2) Tablets BID for 84 days
- 100 patients, 4 arms, ~25 patients per arm

Goals and Milestones for 2016

- Start of Kevetrin advanced study in ovarian cancer
- Start of Brillacidin ABSSSI Phase 3
- Start of Brillacidin for Ulcerative Proctitis proof of concept trial (Phase 2)
- Top line data on Kevetrin Phase 1 trial for advanced solid tumors
- Top line data on Prurisol Phase 2 trial for chronic plaque psoriasis
- Interim analysis for Brillacidin Phase 2 trial for Oral Mucositis (if enrollment milestone reached)
- Start of Retinoblastoma program, formulation and toxicity studies
- Additional ototoxicity studies with Brillacidin in different concentrations
- Gram- negative and anti-fungal progress
- Submission of grant application for study of Kevetrin in pancreatic cancer with renowned hospital.

cellceutix™

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Corporate Presentation
December 2015

