

cellceutix

Corporate Overview

February 2017

FIRST-IN-CLASS DRUG CANDIDATES

With dermatology, oncology, anti-inflammatory, and antibiotic applications

100 Cummings Center, Beverly, MA

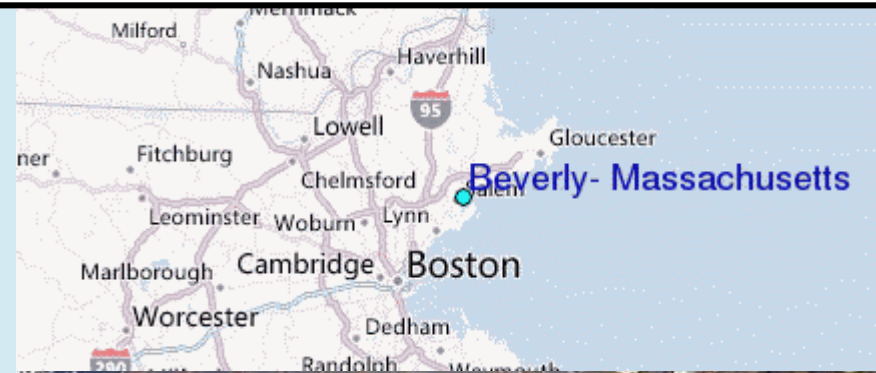
Ticker: CTIX

Cellceutix Corporation

100 Cummings Center







Suite 151B

Beverly, MA 01915



Corporate Focus and Management Team

Established in 2007, Cellceutix is a clinical-stage biopharmaceutical company dedicated to discovering and developing innovative compounds with **dermatology, oncology, anti-inflammatory and antibiotic applications**.

Name	Title	
Leo Ehrlich	Chief Executive Officer, Chief Financial Officer	Co-Founder; Investor
Krishna Menon, PhD, DVM	Chief Scientific Officer	Co-Founder 
Arthur P. Bertolino, MD, PhD, MBA	President, Chief Medical Officer	 
Jane Harness, MS, MP	VP, Clinical Sciences and Portfolio Management	 
LaVonne Lang, DrPH	VP, Regulatory Affairs	

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

Drug Candidates

Cellceutix has **three drug candidates**, each with first-in-class potential, advancing in mid-to-late stage clinical trials under various special FDA designations.

Prurisol

*Orally-delivered **psoriasis** drug candidate in a Phase 2b trial*
utilizing advantages of the 505(b)(2) development approach

Kevetrin

p53-activating drug candidate with three Orphan Drug designations
*starting a Phase 2a trial for **ovarian cancer***

Brilacidin

*Drug candidate in a **new antibiotic class** with unique immunomodulatory properties advancing in clinical trials under Fast Track designations*

Pipeline—Stages of Development & Special FDA Designations

Exceptionally strong pipeline, novel mechanisms of action

Drug Candidate	Indication	Preclinical	Phase 1	Phase 2	Phase 3
Prurisol	Psoriasis	→			
Kevetrin	Ovarian Cancer	→			
Brilacidin	UP/UPS*	→			
	Oral Mucositis	→			
	ABSSSI**	→			

Leveraging designations to expedite development, improve likelihood of drug approval, and gain added market exclusivity



Drug Candidate	Designation Type	Date Granted
Kevetrin	Orphan Drug Designation for Ovarian Cancer	July 2015
	Orphan Drug Designation for Retinoblastoma	November 2015
	Rare Pediatric Disease Designation for Retinoblastoma	November 2015
	Orphan Drug Designation for Pancreatic Cancer	January 2016
Brilacidin	QIDP*** Fast-Track Designation for ABSSSI**	December 2014
	Fast-Track Designation for Oral Mucositis	November 2015



*UP/UPS - Ulcerative Proctitis/Proctosigmoiditis

**ABSSSI - Acute Bacterial Skin and Skin Structure Infections

***QIDP – Qualified Infectious Disease Product

Cellceutix Anticipated Clinical Milestones

By Drug Candidate, Type of Event and Timeframe

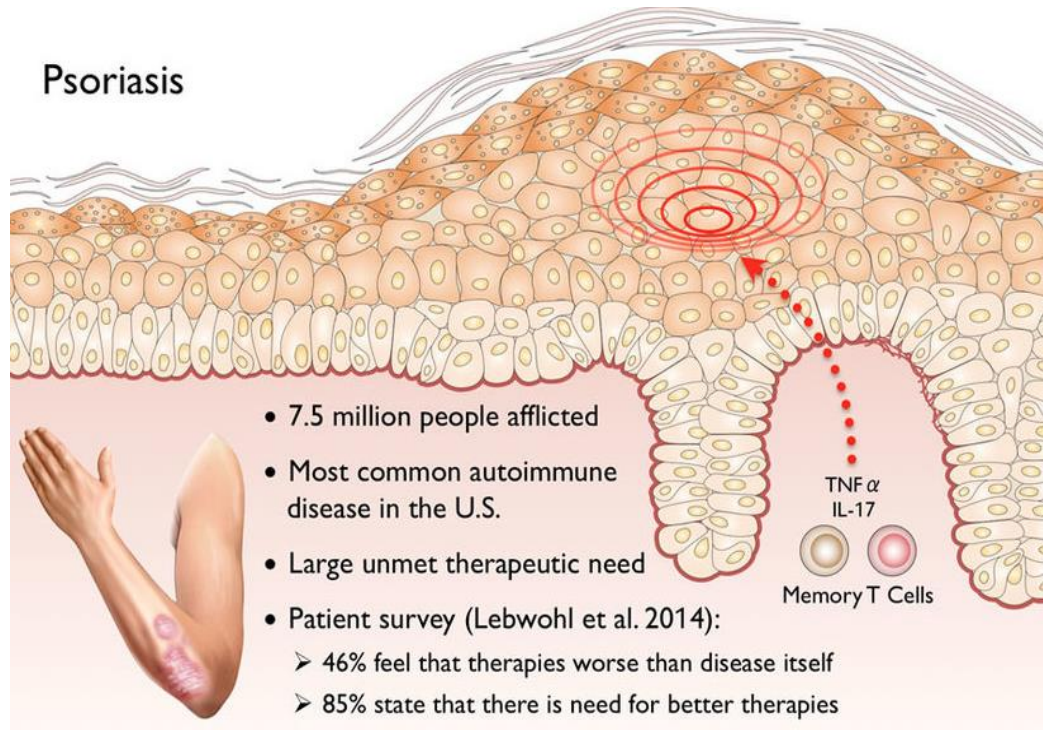
Drug Candidate			Event	Description	Period	Year	Delivered
Brilacidin	Kevetrin	Prurisol					
		X	Trial Progress	Psoriasis - Initiation Ph2b trial	4Q	2016	
X			Clinical Update	Ulcerative Proctitis - Interim analysis Ph2a trial	4Q	2016	
	X		Trial Progress	Ovarian Cancer - Initiation Ph2a trial	1Q	2017	
		X	Clinical Update	Psoriasis - Interim Analysis Ph2b	1H	2017	
X			Clinical Update	Oral Mucositis - Interim analysis Ph2	1H	2017	
X			Clinical Update	Ulcerative Proctitis - Complete Ph2a trial	1H	2017	
		X	Clinical Update	Psoriasis - Complete Ph2b trial	2H	2017	
X			Clinical Update	Oral Mucositis - Complete Ph2 trial	2H	2017	
	X		Clinical Update	Ovarian Cancer - PoC p53 modulation (Ph2a)	2H	2017	
X			Trial Progress	ABSSSI - Start Ph3 trial	*	2017	

*Timetable dependent on reaching SPA agreement with FDA

Prurisol

Psoriasis: Debilitating Chronic Disease That Affects Millions

84% of those with moderate-to-severe psoriasis report suffering discrimination and humiliation.



Overall Quality of Life among Psoriasis Patients



Sources:

<https://www.novartis.com/news/media-releases/largest-global-psoriasis-survey-shows-84-people-face-discrimination-and>

<http://www.cytherapharm.com/>

<http://journals.plos.org/plosone/article?id=10.1371/journal.pone.0052935>

Prurisol for Psoriasis

MOA and Attributes

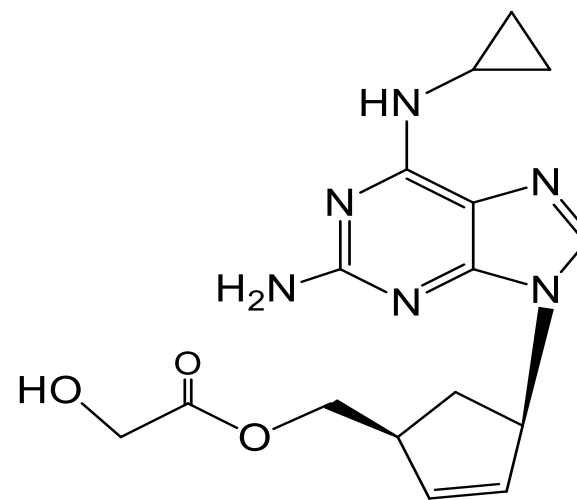
Mechanism of Action (MOA)

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

Attributes

- NCE with strong Intellectual Property(IP) and patent protections
- **Abbreviated development plan** utilizing advantages of 505(b)(2) development approach [reference drug: Abacavir]
- **Efficacy in Phase 2 trial in mild-to-moderate chronic plaque psoriasis**
- Oral dosing
- Small-molecule (<500 MW) (an ester of Abacavir)
- Bioavailable
- Excellent in-vivo and in-vitro activity
- Efficacy in xenograft model

*PRINS – Psoriasis-associated non-protein coding RNA induced by stress



Prurisol

[Abacavir glycolate]

Molecular formula: $C_{16}H_{20}N_6O_3$
Molecular weight: 344.37

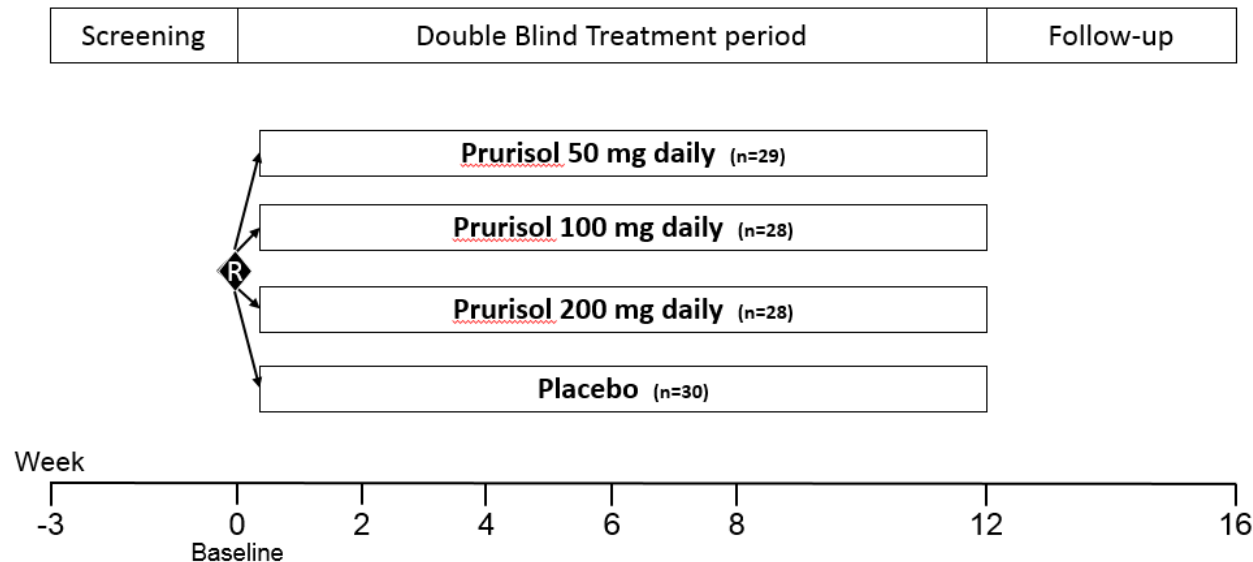
Prurisol for Psoriasis

CTIX-0002 – Study Design for Phase 2a Clinical Trial in *Mild-to-Moderate Plaque Psoriasis*

Primary efficacy endpoint: percentage of subjects with ≥ 2 point improvement in IGA rating at 84 days (12 weeks)

Investigator Global Assessment (IGA) rating: clear (0), almost clear (1), mild (2), moderate (3), severe (4), very severe (5)

- Randomized, double blind, parallel group, placebo-controlled
- 4 treatment groups, 1:1:1:1 randomization, 12 weeks treatment
 - Prurisol
 - 50 mg daily (50 mg AM)
 - 100 mg daily (50 mg AM & 50 mg PM)
 - 200 mg daily (100 mg AM & 100 mg PM)
 - Placebo AM & PM



- Trial conducted at 9 sites in U.S.
- 115 subjects, 4 arms, ~29 per arm
- Efficacy, Safety & PK

For study details, see

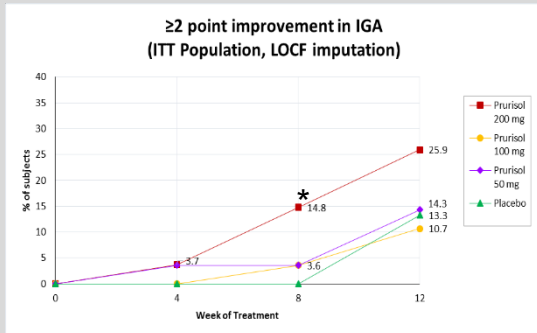
<https://clinicaltrials.gov/ct2/show/NCT02494479>

CTIX-0002: Efficacy Data

By group, Percentage of Subjects ≥ 2 -point Improvement in IGA Over Time

- Clinical improvement seen in 200 mg group as early as 4 weeks, with progressive treatment effect over time
- ≥ 2 -point IGA improvement (200 mg group) at Week 12 was:

25.9% subjects (ITT, LOCF)



Source: Table 14.2.1.1.1

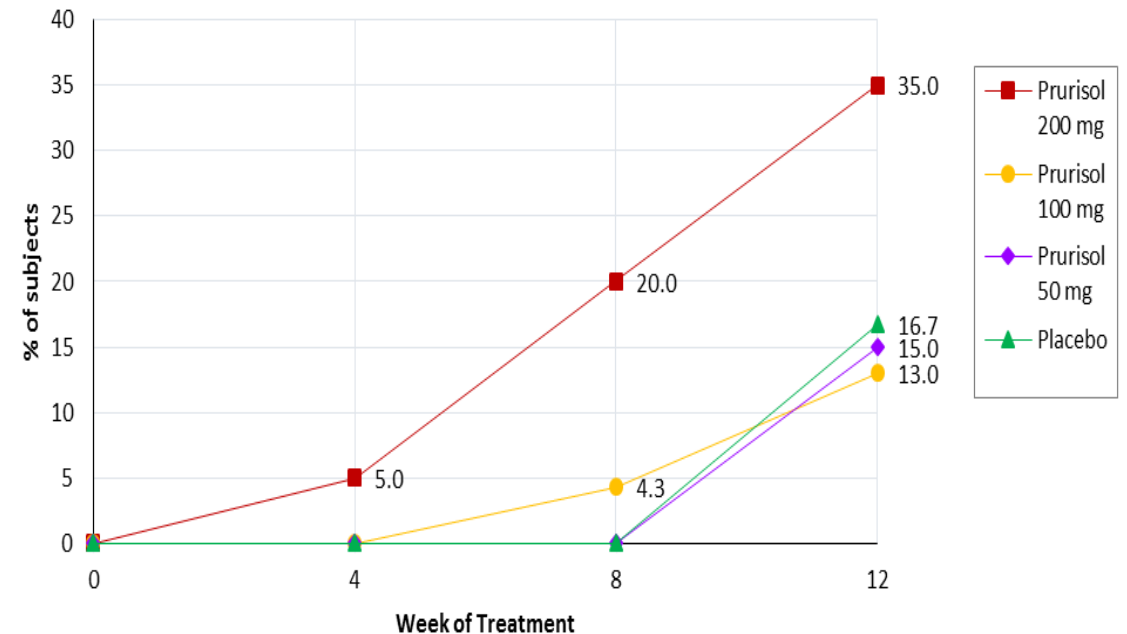
* $p < 0.05$ vs placebo

- Prurisol met the primary endpoint (a ≥ 2 -point IGA reduction) in 35% of all patients who received a dose of 200 mg per day (Per Protocol)**
- Prurisol was well-tolerated—just one Serious Adverse Event (SAE) occurred and it was in the 50 mg dose group
- PK results showed a dose-dependent increase in drug exposure and maximum plasma concentration

Among patients with the severest form of psoriasis in study, those having a baseline IGA score of 3 (“moderate”), the **primary endpoint was met in 46% of patients** who received 200 mg per day. These data were derived from the Per Protocol population.

35.0% subjects (PP)

≥ 2 point improvement in IGA
(PP Population)



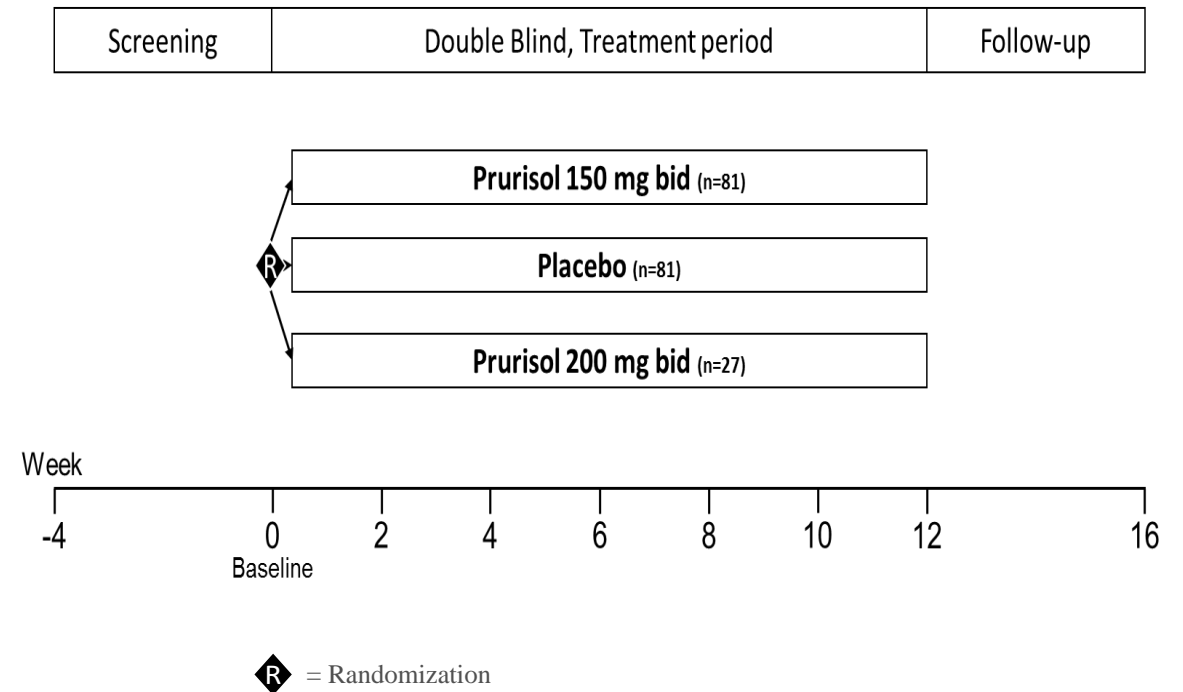
Source: Table 14.2.1.1.2 and Table 14.2.1.2.4

Prurisol for Psoriasis

Ongoing Phase 2b Clinical Trial in Moderate-to-Severe Plaque Psoriasis

- **Randomized, double-blind, parallel-group, placebo-controlled**
- **Treatment Groups**
 - Prurisol 300 mg: Pbo: Prurisol 400 mg
 - 3:3:1
- **Number of Subjects**
 - ~189
- **Treatment Duration**
 - 12 weeks (interim readout 2Q17)
- **Number of Sites (U.S.)**
 - ~25

Study Design Schematic



For study details, see








<https://clinicaltrials.gov/ct2/show/NCT02949388>

Prurisol for Psoriasis

Regulatory Plan Overview

Target NDA Approval 2020

Version 1.0: 01-Dec-2016

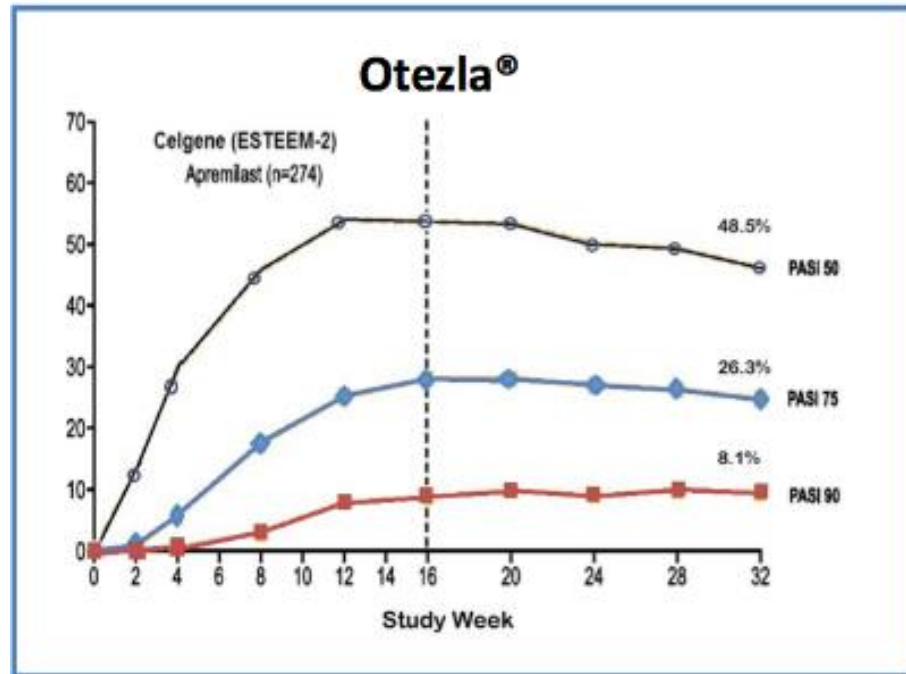
Year H/Q	2012	2013	2014		2015		2016				2017				2018				2019				2020			
			1H	2H	1H	2H	1Q	2Q	3Q	4Q	1Q	2Q	3Q	4Q	1Q	2Q	3Q	4Q	1Q	2Q	3Q	4Q	1Q	2Q	3Q	4Q
REG	Pre-IND mtg 		Submit IND 	EOP1 mtg 									EOP2 mtg 							Pre-NDA mtg 	Submit NDA 				NDA Approval 	
	505(b)(2) development approach [reference drug: Abacavir]																									

Prurisol for Psoriasis

Market Opportunity, Competitive Landscape

Otezla® is a Blockbuster Drug

Otezla®, the main potential oral competitor, demonstrates only moderate efficacy by week 16



Source: http://www.baystreet.ca/articles/research_reports/lifesci/Can-Fite%20BioPharma041216.pdf; Cellceutix research

Celgene Otezla® earned revenue of >\$1 billion in 2016 and expects \$1.5 billion to \$2 billion in 2017

Recent Psoriasis Deals

\$640 million



RORyt IL-17 modulation

\$790 million



SNA-based therapeutics

\$490 million



Monomethylfumarate prodrug

\$595 million



Anti-IL-23 mAb

cellceutix

Kevetrin for Ovarian Cancer

Program Summary

A p53-activating drug candidate granted multiple FDA Orphan Drug designations starting a Phase 2a trial for platinum-resistant ovarian cancer

- **Induces apoptosis** and shows potent anti-tumor activity
- **Multiple molecular targets and signaling pathways** targeted and regulated (modulates)
- **Non-genotoxic induction of Apoptosis**
- **Does not affect normal cell viability** at concentrations that kill tumor cells
- **Well-tolerated with minimal adverse effects** in the completed Phase 1 clinical trial

Current Perspectives

- **Ovarian Cancer (OC)** indication supported by Phase 1 solid tumor trial
- **p53 pathway modulation** to be measured in upcoming OC trial
- **Oral formulation** and delivery advances are underway. This better aligns with Kevetrin's short half-life and may provide for even better drug exposure and toleration

Kevetrin for Ovarian Cancer

Upcoming Phase 2a Clinical Trial in Late-Stage Ovarian Cancer

- **Kevetrin Therapy**

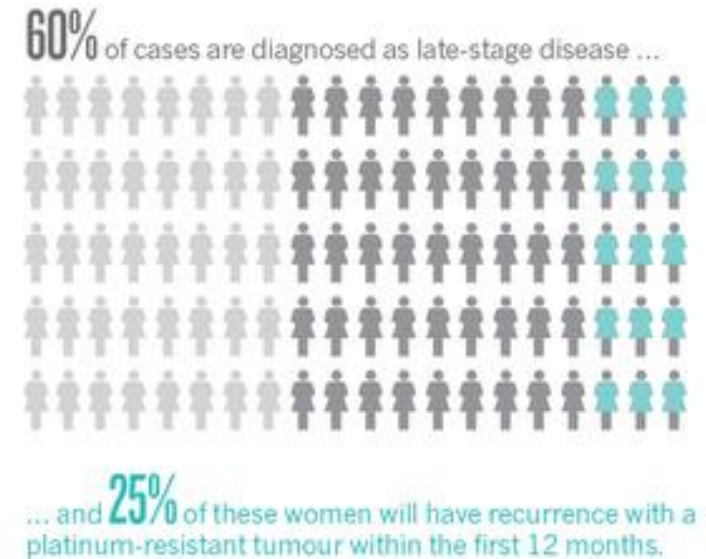
- Kevetrin (starting dose 250 mg/m²) 3 times/week over 3 weeks (dose escalation in 2nd cohort)
(5 subjects in each cohort)

- **Endpoints**

- Safety
- Efficacy based on RECIST criteria using scans
- PK

- **Proposed Biomarkers**

- *p53 (in tumor and ascites cells)*
- Pathways analyses via RNA sequencing
- Small RNAs and others



Goal: Establish p53 MOA directly in tumor cells

Multidisciplinary Programs - Brilacidin

Gastrointestinal

Inflammatory Bowel Disease:
Ulcerative Proctitis/Ulcerative Proctosigmoiditis
(UP/UPS)

Dermatology/Cancer

Oral Mucositis (OM)

Infectious Disease

Acute Bacterial Skin and Skin Structure Infections (ABSSSI)

Brilacidin

IBD—Ulcerative Proctitis and Ulcerative Proctosigmoiditis (Phase 2a Trial in Progress)

Study Design

- Open-label, sequential dose escalation
- Brilacidin (N= 6 per cohort) once daily for 42 days
 - Cohort A: 50 mg
 - Cohort B: 100 mg (currently dosing this cohort)
 - Cohort C: 200 mg

Objectives

Primary:

- Assess frequency of **clinical and endoscopic remission**
 - Brilacidin administered per rectum in subjects with active UP or UPS
 - 6 weeks of treatment



Healthy Colon

Ulcerative Colitis

Crohn's Disease

Interim Results in First Study Cohort

All **4 subjects evaluated** demonstrated a clinical response, measurable by the **Modified Mayo Disease Activity Index (MMDAI)**

- **Partial MMDAI (Day 42)**
 - **2 of 4** subjects achieved **full response** (100% reduction)
 - **2 of 4** subjects had **notable improvement** (50% reduction)
- **MMDAI (Day 42; 3 of 4 subjects completed endoscopy)**
 - **1 of 3** subjects achieved **full response** (100% reduction)
 - **2 of 3** subjects had **notable improvement** (50% reduction)
- **Patient Quality of Life**, as measured by the Short Inflammatory Bowel Disease Questionnaire (SIBDQ)
 - **Improved** after 6-week treatment with Brilacidin
- **Safety**
 - **Generally well-tolerated**
 - Subjects had stable normal vital signs
- **Drug Concentrations in Plasma**
 - All levels at all time points **below the lower limit of quantification** (ie, <100 ng/mL)
 - Consistent with **very limited systemic exposure** from administration per rectum by enema

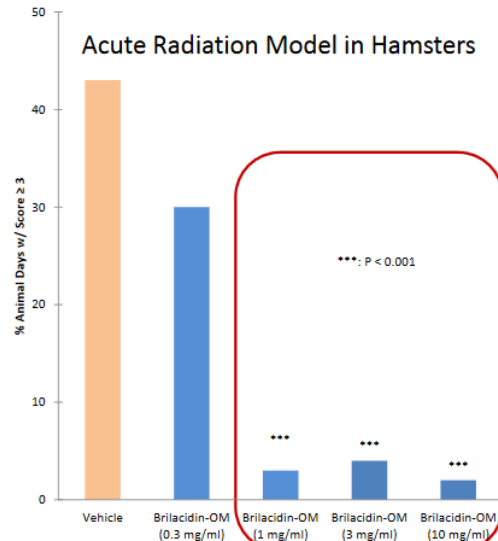
For current standard of care, see

<https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4876845/>

Brilacidin

Oral Mucositis—Animal Model Results (*Phase 2 Trial in Progress*)

Pre-Clinical



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

Phase 2 Trial

Study Design

- Phase 2, Multi-center, Randomized, Double-blind, Placebo-controlled (10 sites in US expanding to up to 20)
- 60 subjects – 30 each of drug or placebo (Water for Injection)
- “Swish and spit” Brilacidin 3x/daily for 7 weeks – 16 ml oral rinse
- Interim analysis after 36 subjects (18 per treatment group) by a Data Monitoring Committee (DMC); will review safety and efficacy results

Primary Endpoints

- *Control and prevent oral mucositis in patients receiving chemoradiation therapy for Head and Neck Cancer*
- Efficacy of topically-applied Brilacidin vs placebo in **delaying the onset of severe OM** (WHO Grade ≥ 3)
- Safety and tolerability of topically-applied Brilacidin administered three times daily for approximately 7 weeks

For pre-clinical work, see <http://cellceutix.com/wp-content/uploads/2013/11/ECCMID-2015-OM-poster.pdf>

For study details, see <https://clinicaltrials.gov/ct2/show/NCT02324335>

Clinical Predictability of the Hamster Oral Mucositis Models Studies by Dr. Stephen Sonis, Harvard *

	Effect in animal model % reduction	Effect in Clinic % reduction
ActoGenix AG013 (HTF-1)	33%	30%
SciClone SCV-07	33%	30%
Velafermin (hFGF-20)	37%	51%

*Cellceutix Clinical Advisor

BRILACIDIN 94%

Interim Phase 2 Results
Anticipated Q2 2017

Brilacidin

ABSSSI Phase 2b Clinical Trial Results

	Brilacidin 0.6 mg/kg IV x 1 day (N=53)	Brilacidin 0.8 mg/kg IV x 1 day (N=53)	Brilacidin x 3 days (N=53)	Daptomycin x 7 days (N=50)
Number 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)

Current Perspectives

- **Safe and effective in three Phase 2 studies**
- **Convenient single-dose regimen**
 - Pharmacoeconomic advantages
- **Efficacy comparable to 7-day regimen of robust comparator (Daptomycin x 7 days)**
- **QIDP designation (Nov 2014) under the GAIN Act**
 - Eligible for Fast Track and Priority Review
- **Minimal potential for development of resistance**
 - Novel class, with no cross-resistance
 - Novel mechanism of action confers fitness disadvantage for bacterial resistance
 - Single dose removes patient non-compliance as driver of resistance
- **Phase 3 planning in progress**
 - Response to Special Protocol Assessment (SPA) comments from FDA underway



*Acute Bacterial Skin and Skin Structure Infection

For the Phase 2b clinical trial of Brilacidin in ABSSSI, see <https://clinicaltrials.gov/ct2/show/NCT02052388>
Also see: [Comparative Mechanistic Studies of Brilacidin, Daptomycin, and the Antimicrobial Peptide LL16](#)

Intellectual Property and Patents

Strong Protections Across All Drug Candidates and Related Compounds

Prurisol and related compounds

- #US Patents granted
 - 1
- Prurisol Mfg method
 - Prov. pending
- Countries Granted
 - Various EU
 - Japan
 - Others

Kevetrin and related compounds

- # US Patents granted
 - 1
- # Patents pending
 - Others
- Countries Granted
 - Various EU
 - Japan
 - Others

Brilacidin and related compounds

- # US Patents granted
 - 9
- Brilacidin Mfg method
 - In-process
- Countries Granted
 - Various EU
 - Japan
 - Others

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