Corporate Overview

Arthur P. Bertolino, MD, PhD, MBA
President & Chief Medical Officer

FIRST-IN-CLASS DRUG CANDIDATES

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

Presented at: Biotech Showcase
San Francisco, CA, USA
January 9, 2017
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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.

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

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

<table>
<thead>
<tr>
<th>Drug Candidate</th>
<th>Indication</th>
<th>Preclinical</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
</tr>
</thead>
<tbody>
<tr>
<td>Prurisol</td>
<td>Psoriasis</td>
<td></td>
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<tr>
<td>Kevetrin</td>
<td>Ovarian Cancer</td>
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<tr>
<td>Brilacidin</td>
<td>UP/UPS*</td>
<td></td>
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<tr>
<td></td>
<td>Oral Mucositis</td>
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<tr>
<td></td>
<td>ABSSSI**</td>
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</table>

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

<table>
<thead>
<tr>
<th>Drug Candidate</th>
<th>Designation Type</th>
<th>Date Granted</th>
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<tbody>
<tr>
<td>Kevetrin</td>
<td>Orphan Drug Designation for Ovarian Cancer</td>
<td>July 2015</td>
</tr>
<tr>
<td></td>
<td>Orphan Drug Designation for Retinoblastoma</td>
<td>November 2015</td>
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<tr>
<td></td>
<td>Rare Pediatric Disease Designation for Retinoblastoma</td>
<td>November 2015</td>
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<tr>
<td></td>
<td>Orphan Drug Designation for Pancreatic Cancer</td>
<td>January 2016</td>
</tr>
<tr>
<td>Brilacidin</td>
<td>QIDP*** Fast-Track Designation for ABSSSI**</td>
<td>December 2014</td>
</tr>
<tr>
<td></td>
<td>Fast-Track Designation for Oral Mucositis</td>
<td>November 2015</td>
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</tbody>
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*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*

<table>
<thead>
<tr>
<th>Drug Candidate</th>
<th>Event</th>
<th>Description</th>
<th>Period</th>
<th>Year</th>
<th>Delivered</th>
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<tbody>
<tr>
<td>Brilacidin</td>
<td>X</td>
<td>Trial Progress</td>
<td><strong>Psoriasis</strong>- Initiation Ph2b trial</td>
<td>4Q</td>
<td>2016</td>
</tr>
<tr>
<td></td>
<td>X</td>
<td>Clinical Update</td>
<td><strong>Ulcerative Proctitis</strong>- Interim Analysis Ph2a trial</td>
<td>4Q</td>
<td>2016</td>
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<tr>
<td></td>
<td>X</td>
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<td><strong>Ovarian Cancer</strong>- Initiation Ph2a trial</td>
<td>1Q</td>
<td>2017</td>
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<td>X</td>
<td>Clinical Update</td>
<td><strong>Psoriasis</strong>- Interim Analysis Ph2b</td>
<td>1H</td>
<td>2017</td>
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<tr>
<td></td>
<td>X</td>
<td>Clinical Update</td>
<td><strong>Oral Mucositis</strong>- Interim Analysis Ph2</td>
<td>1H</td>
<td>2017</td>
</tr>
<tr>
<td></td>
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<td>2017</td>
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<tr>
<td></td>
<td>X</td>
<td>Clinical Update</td>
<td><strong>Ovarian Cancer</strong>- PoC p53 modulation (Ph2a)</td>
<td>2H</td>
<td>2017</td>
</tr>
<tr>
<td></td>
<td>X</td>
<td>Trial Progress</td>
<td><strong>ABSSSI</strong>- Start Ph3 trial</td>
<td>*</td>
<td>2017</td>
</tr>
</tbody>
</table>

*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.

Sources:
http://www.cytherapharm.com/
http://journals.plos.org/plosone/article?id=10.1371/journal.pone.0052935
**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]
- 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*
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
Prurisol for Psoriasis

CTIX-0002 – IGA Scores Over Time (200 mg group) & Topline Results

- IGA changes noted as soon as Week 2
- Progressive decrease of IGA scores to lower values over 12 weeks
- Time-based transition of original moderate & mild population to less psoriasis activity with emergence of almost clear and clear groups
  - At Week 12: 42.8% subjects (ITT) [and 55.0% subjects (PP)] achieved “clear” (0) or “almost clear” (1) in IGA

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

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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 analyses of all patients.

Source: Table 14.2.1.2.1 (ITT Population)

*Where % subject total <100%, basis is attrition without data imputation
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

2017 – An Inflection Point

Near-Term Value Drivers

- 2Q 2017 – Phase 2b Interim Data
- 3Q 2017 – Phase 2b Complete Top-line Data
- 4Q 2017 – End-of-Phase 2 Meeting with FDA

Broader Context

- Big Pharma Recognizes Value of Safe, Effective Oral Psoriasis Drug
- Cellceutix Actively Discussing Partnerships for Late-Stage Studies
- Targeting NDA Submission to FDA in 4Q 2019
Prurisol for Psoriasis
Market Opportunity, Competitive Landscape

**Otezla® Emerging as a Blockbuster Drug**

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

**Recent Psoriasis Deals**

- **$640 million**
  - Allergan
  - RORyt IL-17 modulation

- **$490 million**
  - Dr. Reddy’s
  - Monomethylfumarate prodrug

- **$790 million**
  - Vitae Pharmaceuticals
  - SNA-based therapeutics

- **$595 million**
  - XenoPort
  - Anti-IL-23 mAb

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

Celgene expects Otezla® to earn revenue of up to $1 billion in 2016 and $1.5 billion to $2 billion in 2017
Kevetrin for Ovarian Cancer
Program Summary

A p53-modulating 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 advanced 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 (approximately 2 hours, with the drug clearing the body within one day, though on average, between 8 and 10 hours) and may provide for even greater 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)

• Endpoints
  • Safety
  • Efficacy based on RECIST criteria using scans
  • PK

• Proposed Biomarkers
  • *p53 (in tumor and ascites cells)*
  • Plasma and ascites
    - CCL2 (MCP-1)
    - miRNA-27a
    - miRNA-1274b
    - miRNA-25
  • Additional tumor biomarker – miRNA-34a
  • Pathways analyses via RNA sequencing

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

**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/](https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4876845/)
Brilacidin
Oral Mucositis—Animal Model Results (Phase 2 Trial in Progress)

Pre-Clinical

![Graph showing Acute Radiation Model in Hamsters]

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

Clinical Predictability of the Hamster Oral Mucositis Models

Studies by Dr. Stephen Sonis, Harvard

<table>
<thead>
<tr>
<th>Model</th>
<th>Effect in animal model</th>
<th>Effect in clinic</th>
</tr>
</thead>
<tbody>
<tr>
<td>ActoGenix AG018 (HTF-1)</td>
<td>33%</td>
<td>30%</td>
</tr>
<tr>
<td>SciClone SCV-07</td>
<td>33%</td>
<td>30%</td>
</tr>
<tr>
<td>Valsfermin (bFGF-20)</td>
<td>37%</td>
<td>51%</td>
</tr>
</tbody>
</table>

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 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
- ts – 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

For study details, see [https://clinicaltrials.gov/ct2/show/NCT02324335](https://clinicaltrials.gov/ct2/show/NCT02324335)
Brilacidin
ABSSSI Phase 2b Clinical Trial Results

<table>
<thead>
<tr>
<th></th>
<th>Brilacidin 0.6 mg/kg IV x 1 day (N=53)</th>
<th>Brilacidin 0.8 mg/kg IV x 1 day (N=53)</th>
<th>Brilacidin x 3 days (N=53)</th>
<th>Daptomycin x 7 days (N=50)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Number assessed</td>
<td>51</td>
<td>48</td>
<td>52</td>
<td>48</td>
</tr>
<tr>
<td>Clinical Response (%)</td>
<td>47 (92.2)</td>
<td>46 (95.8)</td>
<td>51 (98.1)</td>
<td>45 (93.8)</td>
</tr>
<tr>
<td>95% C.I.</td>
<td>(84.8, 99.5)</td>
<td>(90.2, 100)</td>
<td>(94.3, 100)</td>
<td>(86.9, 100)</td>
</tr>
</tbody>
</table>

*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

- 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

Pre-treatment
Day 3
Day 10

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
Join Cellceutix in Reaching Milestones

2017 Goals = A Focus On Partnering Opportunities

Successfully Complete

• Phase 2b Trial of Prurisol for Psoriasis
• Phase 2a Trial of Brilacidin for UP/UPS (IBD)
• Phase 2 Trial of Brilacidin for Oral Mucositis
• Phase 2a Trial of Kevetrin for Ovarian Cancer
• FDA Meetings
  - EoP2 for Psoriasis
  - SPA for Brilacidin in ABSSSI

Meeting 2017 Milestones Sets the Stage to Potentially Initiate 5 Registration Studies in 2018!

OTCQB(symbol): CTIX; Share Price: ~$1.20; Market Cap: ~$150M;
Avg. Daily Vol: ~145k; Shares Outstanding: ~124M
Cellceutix Corporation
100 Cummings Center
Beverly, MA

January 2017

Ticker: CTIX