vitae
Pharmaceuticals

Company Overview

August 2016

Disclaimer Regarding Forward Looking Statements

This presentation includes statements that are, or may be deemed, "forward-looking statements", within the meaning of Section 27A of the Securities Act of 1933, as amended. All statements, other than statements of historical facts, included in this presentation regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans and objectives of management are forward-looking statements. In some cases, you can identify forward-looking statements by terms such as "may," "might," "will," "objective," "intend," "should," "could," "can," "would," "expect," "believe," "anticipate," "project," "target," "design," "estimate," "predict," "opportunity," "proposition," "strategy," "potential," "plan" or the negative of these terms and similar expressions intended to identify forward-looking statements.

These statements reflect our current views with respect to future events and are based on assumptions and subject to risks and uncertainties, including the clinical plans for VTP-43742, VTP-38543 and VTP-45489 and preclinical plans for our potential future product candidates, the anticipated top-line results from the Phase 2a proof-of-concept clinical trial for VTP-38543, the potential 12 week efficacy and safety for VTP-43742 and Vitae's projected cash runway. Actual results may be materially different. Certain information in this presentation is from previously published data and literature from prior psoriasis trials of other compounds. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Forward-looking statements include, but are not limited to, statements about: the timing and success of preclinical studies and clinical trials; the ability to obtain and maintain regulatory approval of our product candidates; the scope, progress, expansion and costs of developing and commercializing our product candidates; our expectations regarding the amount and timing of our expenses and revenue; the sufficiency of our cash resources, plans for the use of our cash resources and needs for additional financing; our ability to adequately manufacture our product candidates and the raw materials utilized therein; our ability to obtain and maintain intellectual property protection for our product candidates; our expectations regarding competition; the size and growth of the potential markets for our product candidates and the ability to serve those markets; the rate and degree of market acceptance of any of our product candidates; our anticipated growth strategies; the anticipated trends and challenges in our business and the market in which we operate; our ability to establish and maintain development partnerships; our ability to attract or retain key personnel; our expectations regarding federal, state and foreign regulatory requirements; regulatory developments in the United States and foreign countries and other factors that are described in the "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of our Annual Report on Form 10-K for the year ended December 31, 2015 and Quarterly Report on Form 10-Q for the quarters ended March 31, 2016 and June 30, 2016, which have been filed with the Securities and Exchange Commission (SEC) and are available on the SEC's website at www.sec.gov. All written and verbal forward-looking statements attributable to Vitae or any person acting on its behalf are expressly qualified in their entirety by the cautionary statements contained or referred to herein. Vitae cautions investors not to rely too heavily on the forward-looking statements Vitae makes or that are made on its behalf.

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Company Highlights

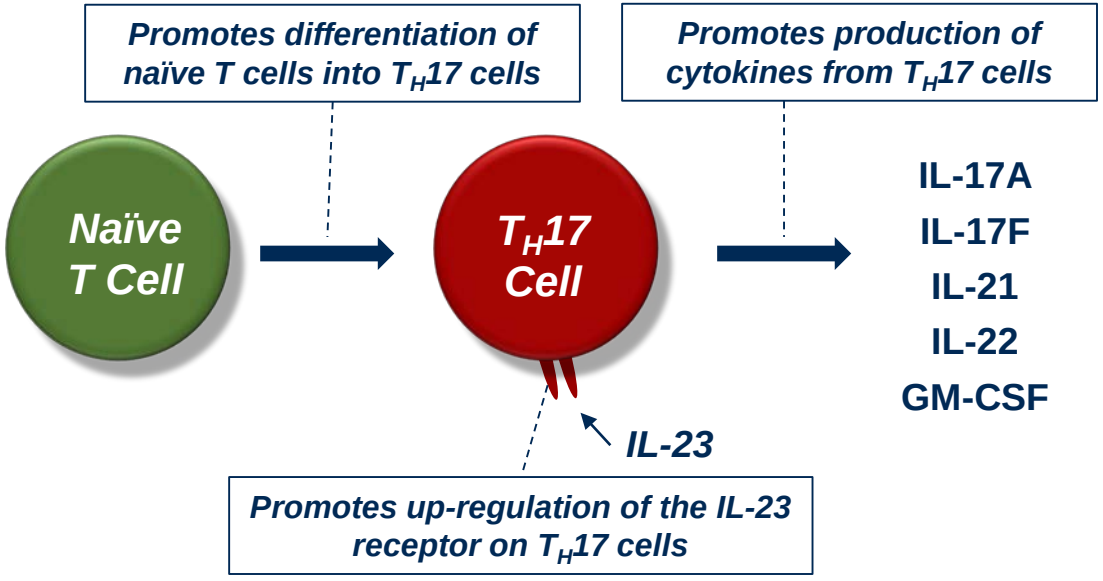
Platform with First-in-Class Wholly Owned Assets in the Clinic

- **VTP-43742: First-in-class oral RORγt inhibitor**
  - Positive Ph 2a proof-of-concept results announced in late 1Q 2016
  - 16 week Phase 2 trial in moderate to severe psoriasis expected to initiate in 4Q 2016
  - Wholly owned; expected patent protection at least into 2035
- **VTP-38543: Potential first-in-class topical LXRβ agonist**
  - Novel mechanism with potential to address significant unmet medical need in atopic dermatitis
  - Phase 2a proof-of-concept trial with 100 patients; top-line results expected 4Q 2016
  - Wholly owned; expected patent protection at least into 2033
- **VTP-45489: Leveraging global leadership in RORγt science**
  - VTP-45489, a RORγt inhibitor chemically distinct from VTP-43742
  - Company plans to initiate a Ph 1a single ascending dose clinical trial in 3Q 2016
- **Pipeline expansion driven by Contour®**
  - New pipeline programs generated by Vitae's proprietary discovery platform Contour®, positioning Vitae for incremental future value growth
- **$77.4 million cash and cash equivalents as of June 30, 2016; projected runway into 2H 2018**

RORγt – Attractive Target in Immunology

Master Regulator of Well Validated T_H17 Pathway

- The T_H17 immune system pathway is a major driver of autoimmune inflammation and tissue damage
- Novel IL-17 and IL-23 antibodies targeting the T_H17 pathway have shown clear superiority over widely used therapies currently in use
- RORγt is the master regulator of the T_H17 pathway; providing an attractive target for a small molecule approach



VTP-43742: Ph 1a Clinical Trial

Single Ascending Dose Trial Results

- **VTP-43742 completed Ph 1a in 3Q 2015**
  - Randomized, double blind, placebo controlled trial assessing safety, tolerability, pharmacokinetics and pharmacodynamics
- **VTP-43742 generally well tolerated**
  - All planned dose cohorts completed successfully; ranged from 30 mg to 2,000 mg
  - No dose limiting findings; no serious adverse events
  - No drug related clinical laboratory or electrocardiogram (ECG) abnormalities
- **Pharmacokinetics indicating once daily dosing**
- ***Ex vivo* biomarker assay demonstrated >90% inhibition of RORγt-mediated IL-17 production at multiple dose levels**

VTP-43742: Ph 1b Clinical Trial

10 Day Multiple Ascending Dose Trial Results

- **VTP-43742 completed Ph 1b in 4Q 2015**
  - Randomized, double blind, placebo controlled trial assessing safety, tolerability, pharmacokinetics and pharmacodynamics
- **VTP-43742 generally well tolerated**
  - Dose cohorts ranged from 100 mg/day to 1,400 mg/day
  - No dose limiting findings; no serious adverse events
    - *Some nausea and headache noted at top 1,400 mg dose; not dose limiting*
  - No drug related clinical laboratory or ECG abnormalities
- ***Ex vivo* biomarker assay demonstrated >90% inhibition of RORγt-mediated IL-17 production**
  - All doses exceeded IC_{50} over 24 hours
  - Four of the five doses exceeded IC_{90} over 24 hours

VTP-43742

Phase 2a Proof-of-Concept Trial Results

- **Clinical trial design**
  - Randomized, double blind, placebo controlled
  - 4 week treatment of 34 moderate-to-severe psoriasis patients
- **Positive efficacy signal**
  - Clear and consistent signal of efficacy from PASI score changes in both the 350mg and 700mg dose groups
  - Comprehensive biomarker data supported observed clinical results
  - PASI score improvement accelerated significantly in week 3, more in week 4
- **Generally well tolerated**
  - 350mg dose group safety and tolerability comparable to placebo
  - Transaminase elevations observed in some patients in the 700mg dose group
- **Results clinically validate RORγt target; support 'go' decision to advance VTP-43742 in clinical development**

VTP-43742

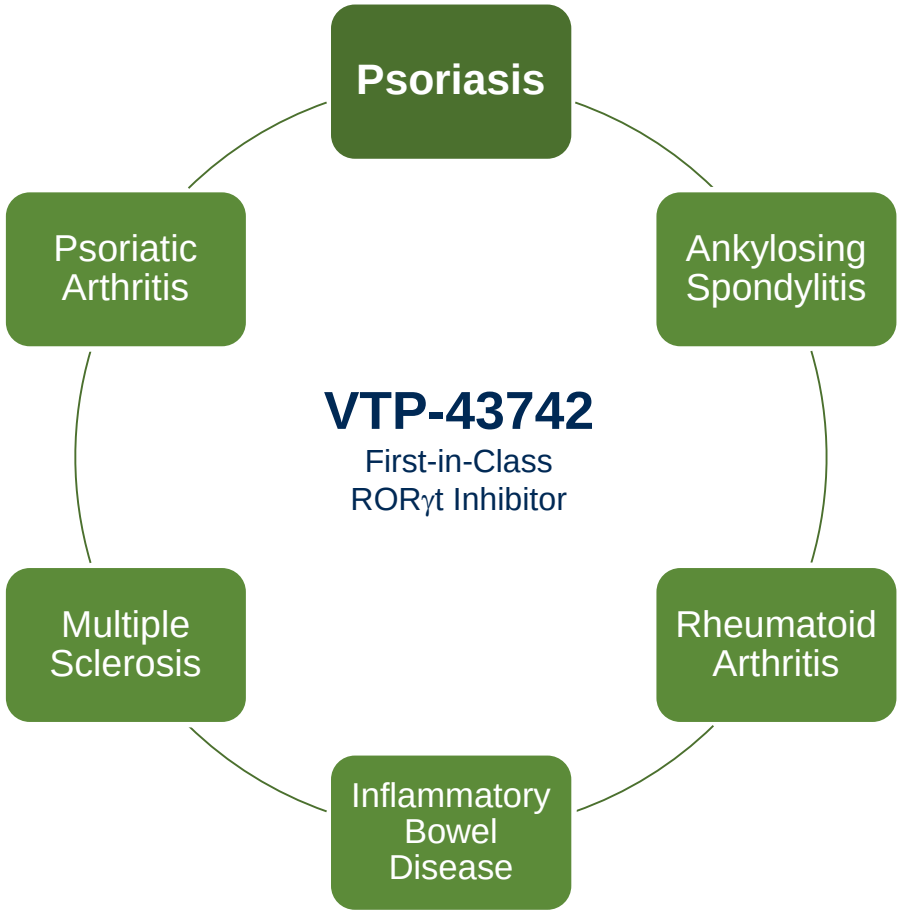
Next Phase 2 Trial Design: Key Objectives

1. **Establish the full efficacy potential of VTP-43742**
   - All currently available therapies require ≥12 weeks dosing to achieve full efficacy
   - In the 4 week Ph 2a trial with VTP-43742, clinical improvement accelerated significantly in week 3, even more in week 4; indicates potential for increasing efficacy beyond 4 weeks
   - Next Ph 2 trial with VTP-43742 will be 16 weeks duration
2. **Further elaborate the safety / tolerability profile of VTP-43742**
3. **Efficiently enable VTP-43742 to proceed into a pivotal trial next, if Ph 2 trial successful**

Development Goal: to develop VTP-43742 as a transformative 'best oral agent for the treatment of multiple autoimmune disorders, starting with psoriasis'

- i.e. highly effective, safe, well tolerated, once daily oral therapy

Beyond Psoriasis, Significant Potential in Multiple Additional Autoimmune Indications



- **Potential to address significant unmet medical need within multi-billion dollar autoimmune markets**
- **Potential to launch with specialized, highly focused salesforce**

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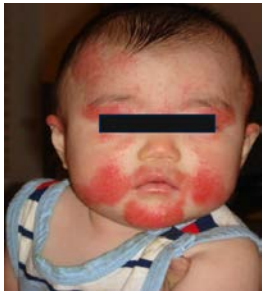
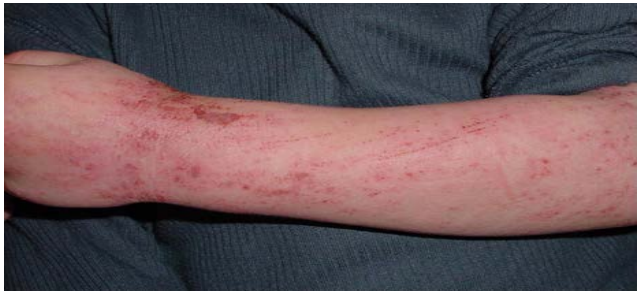
Atopic Dermatitis

VTP-38543

Atopic Dermatitis

- **Atopic dermatitis (AD) is the most common inflammatory disease**
  - Estimated to occur in 15-25% of children and 4-7% of adults
  - Estimated up to 32 million patients with AD in the US (~90% mild-to-moderate)
- **AD characterized by flares of intense itching, red and inflamed rash, dry skin**

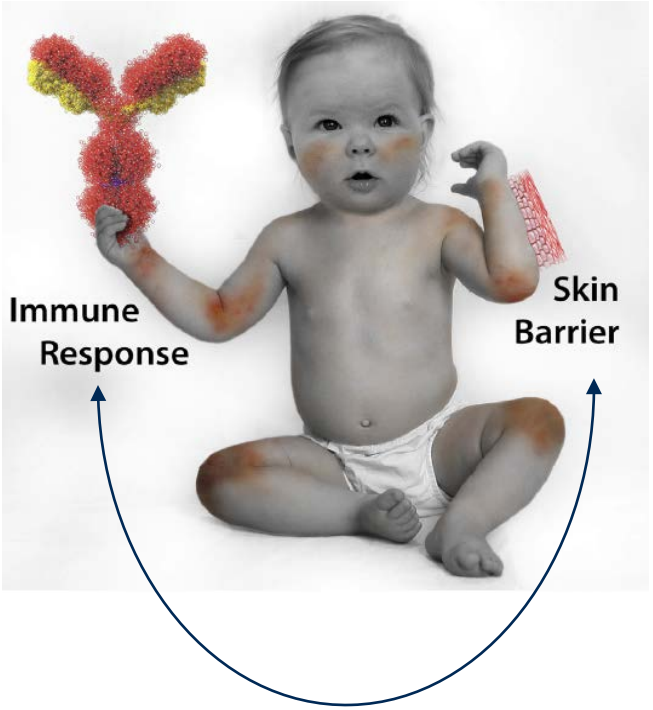




- **Itching and skin appearance have a major impact on patients (and families)**
  - **63%** report sleep impairment up to 2 hours daily; sleep loss affects school / work performance, often impacts other family members
  - **60%** of adolescents report school difficulties; teasing and bullying
  - **29%** report impaired personal relationships; high divorce rate observed
  - **71%** report feelings of exhaustion, frustration, resentment and helplessness
  - Health related quality of life impairment exceeds that in asthma and epilepsy, comparable to renal disease or cystic fibrosis

Atopic Dermatitis

Pathogenesis and Treatment Options



Drs. Leung and Guttman-Yassky, *J Allergy Clin Immunology,* October 2014

- **Intertwining mechanisms create pathology: skin barrier compromise and increased inflammatory response**
  - Either can be the initiating factor
- **Skin barrier compromise (outside→in)**
  - Genetic skin barrier defects (*e.g.* filaggrin) in up to 50% of AD patients, allowing irritants and allergens from environment in, triggering an inflammatory response
- **Increased inflammatory response (inside→out)**
  - Lesional over-expression of cytokines leads to broader immune response
  - Inflammatory response triggers itch-scratch cycle; scratching causes physical breakdown of skin barrier
- **Currently available AD drugs limited to anti-inflammatory action**
  - No therapies are available that address both aspects ; corticosteroids, the predominant therapy, actually thin skin with chronic use

Target Profile for Novel AD Therapy

LXR Biology Creates Potential Opportunity

Target profile for unmet need in AD:

- Addresses both the barrier function and inflammatory pathology of AD
- Very well tolerated; avoids the side effects of steroids / global immuno-suppressants
- Can be used for acute flares and as chronic therapy
- Topical, for targeted easy-to-use therapy on lesions from mild to severe
- Cosmetically elegant vehicle
- Potential to induce remission – treat existing lesions / prevent development of new lesions

LXRβ regulates a variety of skin functions required for epidermal homeostasis by orchestrating the expression of genes involved in lipid metabolism and inflammation

- **Barrier function effects**
  - ↑ epidermal lipid synthesis (SREBP1c)
  - ↑ lipid transport into lamellar bodies (ABCG1)
  - ↑ differentiation of keratinocytes into corneocytes
  - ↑ maturation of lamellar bodies and corneo-cytes into impermeable outer layer of skin (filaggrin, loricrin, involucrin)
- **Anti-inflammatory effects**
  - ↓ inflammatory gene expression in skin
  - Includes IL-1 and TNFα mediated pro-inflammatory molecules (IL-6, iNOS and COX-2)

Anti-Inflammatory Activity of VTP-38543

Similar to Ultra High Potency Corticosteroid

Mouse ear model of inflammation

① **Cross section of normal mouse ear**

② **Ear cross section after application of irritant (TPA) to induce inflammation**
(standard animal model of inflammation)

- Substantial edema and inflammatory cell infiltration observed

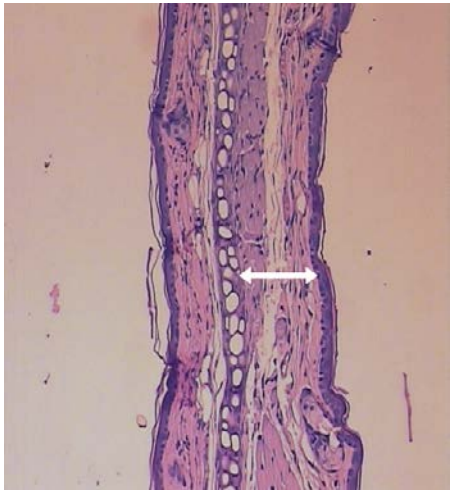
③ **Ultra high potency steroid clobetasol applied after TPA**

- Edema and inflammatory infiltrates decreased significantly

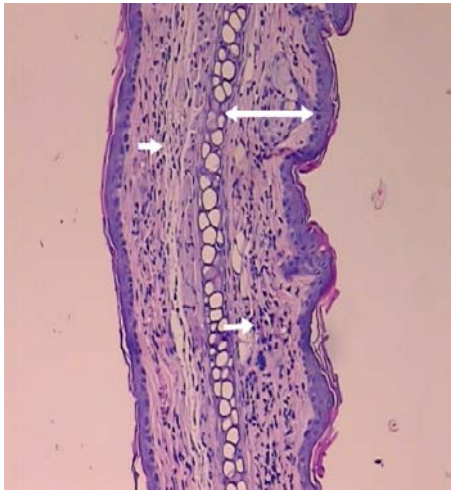
④ **VTP-38543 applied after TPA**

- Edema and inflammatory infiltrates decreased significantly; effect similar to clobetasol

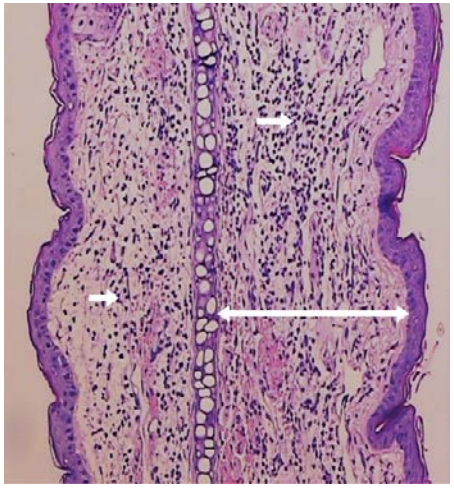
① **Vehicle + Vehicle**



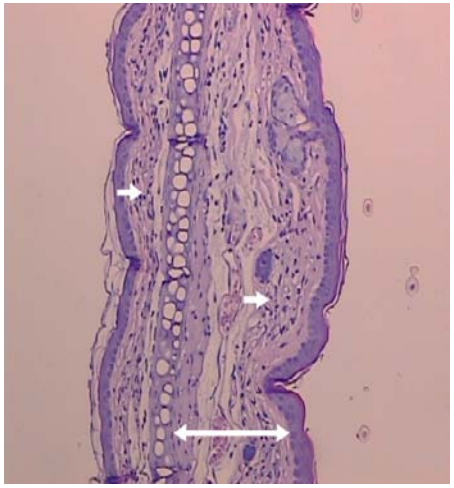
③ **TPA + Clobetasol 0.05%**



② **TPA (irritant) + Vehicle**



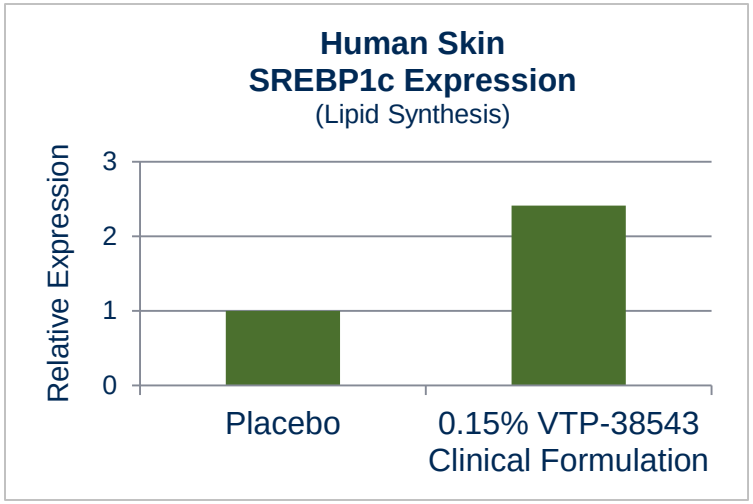
④ **TPA + VTP-38543 0.5%**

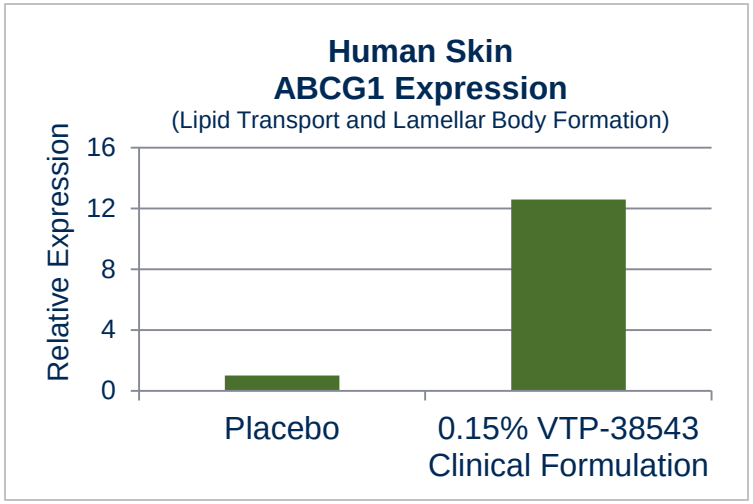


Barrier Function Activity of VTP-38543

Increases Target Gene Expression in Human Skin

- **LXR activation accelerates the return of barrier function following barrier disruption** (*e.g.* tape stripping)
  - stimulates keratinocyte differentiation to form the cornified outer layer of skin
  - stimulates all three key steps in lamellar body formation, to 'glue' corneocytes together with an impermeable lipid rich extracellular matrix
- **SREBP1c** (lipid synthesis) **and ABCG1** (lipid transport and lamellar body formation) **are genes essential to formation of the complex lipid rich matrix of the stratum corneum**
- **VTP-38543 topical formulation significantly increases expression of ABCG1 and SREBP1c genes in human skin in organ culture** (from cosmetic surgery)

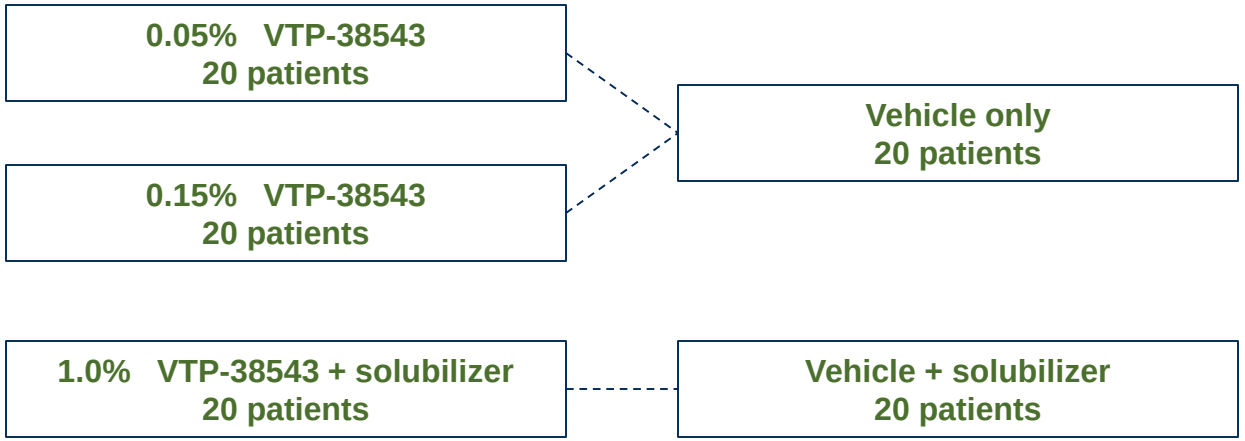




VTP-38543

Ph 2a Proof-of-Concept Trial Design

- **Randomized, double blind, placebo controlled trial with 100 mild-to-moderate atopic dermatitis patients** (IGA score of 2 or 3)
- **VTP-38543 applied topically twice daily for 4 weeks**



- **Topline clinical results expected in 4Q 2016**

VTP-38543

Ph 2a Proof-of-Concept Trial Clinical Endpoints

- **Physician assessment of clinical efficacy**

Scoring System	Acronym	Rationale	Description
Investigator's Global Assessment	**IGA**	US basis for approval	Simple **0-4 scale** per patient*
Severity Scoring of Atopic Dermatitis	**SCORAD**	EU basis for approval	Comprehensive, systematized scoring **0-103 point scale**
Eczema Area and Severity Index	**EASI**	Gaining use in US	Comprehensive, systematized scoring **0-72 point scale**

** IGA measures 'responders' - those achieving a rating of 0 (clear) or 1 (almost clear) and a minimum two point rating change*

- **Patient assessment of clinical efficacy**
  - Itching frequency / intensity and sleep disruption
- **Biomarker assessment of barrier and inflammatory markers**
  - Includes SREBP1c (lipid synthesis), ABCG1 (lipid secretion), keratin-16 and filaggrin (associated with keratinocyte differentiation) and IL-6 (pro-inflammatory cytokine)
  - Cell histology and immunohistochemistry changes from skin biopsies
- **Safety / tolerability and PK**

VTP-38543: Ph 2a Proof-of-Concept Trial

Expectations – Assessment to be Based on Totality of Data

- **High vehicle response rate is typical in atopic dermatitis trials** (vehicle moisturizing effect on dry, itchy skin)
  - In two phase 3 trials, Anacor's crisaborole demonstrated a 7.4 and 13.4 point difference vs vehicle respectively (33% responder *vs* 25% in AD-301, 31% *vs* 18% in AD-302)
- **With 20 patients per dose group, each patient has considerable impact on mean scoring results; statistical variability may not yield simple result**
- **Totality of data will determine next steps**
  - Physician assessment of efficacy (especially EASI & SCORAD which provide % change assessment)
  - Patient assessment of efficacy
  - Objective biomarker data – gene expression, cell histology
- **Following a review of the POC trial results, the next step could include advancing into a further clinical trial in patients with mild to moderate atopic dermatitis**

VTP-38543 Target Profile and Outlook

Parameter	Corticosteroids	Calcineurins	Crisaborole	VTP-38543
Anti-inflammatory	Yes	Yes	Yes	Trial ongoing
Barrier function effect	No	No	No	Trial ongoing
Vehicle elegance	Various	Cream / Ointment*	Ointment	Cream
Other	Can't be used chronically or on sensitive areas	Black box warning; stinging sensation on application		

** Cream formulation outsold ointment approximately 4:1 at launch*

- **Other skin barrier and inflammatory dermatologic conditions where an LXRβ agonist may be useful:**
  - Allergic and irritant induced contact dermatitis
  - Psoriasis
  - Photo-aging
    - *LXR signaling is down-regulated in cell-based models of photo-aging*
    - *LXR agonism inhibits UV-induced photo-damage and skin wrinkle formation in animal models*
    - *LXR KO mice exhibit a striking resemblance with the gene expression pattern of chronologically aged human skin*

Pipeline

Additional Assets Generated by Contour®

- **Contour® is Vitae's proprietary structure-based drug design** (SBDD) **platform**
- **Vitae applies Contour® to targets with these characteristics**
  - Known, relatively well validated biology
  - High unmet medical need
  - Specialty therapeutic category
  - Difficult-to-drug target with significant technical hurdles to overcome
- **Animal proof-of-principle achieved in a new target program**
- **Initial lead candidate selected to advance into pre-clinical development**

Company Summary

- **Multiple first-in-class, wholly owned assets**
  - Advancing in mid-stage clinical trials
  - Targeting attractive markets, with disease indications that have significant unmet medical need
- **Multiple clinical data read-outs expected within existing cash runway**
  - Runway expected to extend into 2H 2018
- **Discovery engine producing additional attractive assets**
- **Team committed to executing on Company's opportunities**