

**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): **April 3, 2017**

**AMICUS THERAPEUTICS, INC.**

(Exact name of registrant as specified in its charter)

**Delaware**  
(State or other Jurisdiction of Incorporation)

**001-33497**  
(Commission File Number)

**71-0869350**  
(IRS Employer Identification No.)

**1 Cedar Brook Drive, Cranbury, NJ**  
(Address of Principal Executive Offices)

**08512**  
(Zip Code)

Registrant's telephone number, including area code: **(609) 662-2000**

(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))

**Item 8.01. Other Events.**

On April 3, 2017, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing the completion of enrollment of the Company's ESSENCE Phase 3 Epidermolysis Bullosa Clinical Study. A copy of this press release is attached hereto as Exhibit 99.1.

The Company also provided a summary of its Epidermolysis Bullosa program of which a copy is attached hereto as Exhibit 99.2.

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

(d) Exhibits: The Exhibit Index annexed hereto is incorporated herein by reference.

Exhibit No.	Description
99.1	Press Release dated April 3, 2017 titled "Amicus Therapeutics Completes Enrollment in ESSENCE Phase 3 Epidermolysis Bullosa Clinical Study."
99.2	Presentation Materials — Epidermolysis Bullosa Program (April 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.

AMICUS THERAPEUTICS, INC.

Date: April 3, 2017

By: /s/ ELLEN S. ROSENBERG  
Name:

Ellen S. Rosenberg  
Title: General Counsel and Corporate Secretary



## Amicus Therapeutics Completes Enrollment in ESSENCE Phase 3 Epidermolysis Bullosa Clinical Study

*Target Exceeded with More than 160 Patients Enrolled*

*Top-Line Data on Track for 3Q17*

**CRANBURY, NJ, April 3, 2017** — Amicus Therapeutics (Nasdaq: FOLD), a global biotechnology company at the forefront of therapies for rare and orphan diseases, has completed enrollment in the ongoing Phase 3 clinical study (ESSENCE) of the novel topical medicine SD-101 for patients with all 3 major types of epidermolysis bullosa (EB) (Simplex, Recessive Dystrophic, and Junctional non-Herlitz EB). With the achievement of full enrollment, top-line data from this study are expected in the third quarter of 2017.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, “The completion of enrollment in our global Phase 3 ESSENCE study of SD-101 for epidermolysis bullosa is a significant accomplishment for our team at Amicus as well as for the EB community. This is the most advanced clinical study for EB, and we look forward to announcing top-line data from this study in the third quarter of this year.”

The FDA has granted Breakthrough Therapy designation for SD-101 based on results from a Phase 2a study for the treatment of lesions in patients suffering with EB. SD-101 is the first-ever treatment in clinical studies to show improvements in wound closure across all major EB types.

John C. Browning, Chief of Dermatology at Children’s Hospital of San Antonio, stated, “The full enrollment in this Phase 3 trial is a significant step forward as we look to advance new wound healing treatments for EB. With no currently approved treatment options, there is an urgent need among people living with EB, as well as their caregivers. There has been tremendous commitment among patients and families, advocacy organizations and study investigators in working alongside Amicus to raise awareness of and drive enrollment in this important study.”

The ESSENCE Study is a Phase 3 double-blind, placebo-controlled study that enrolled more than 160 patients who have a documented diagnosis of Simplex, Recessive Dystrophic, or Junctional non-Herlitz EB. To date, more than 95 percent of patients completing the 3-month primary treatment period have elected to continue in the open-label extension study.

### **About Epidermolysis Bullosa (EB)**

EB is a rare, genetic disorder that manifests as blistering or erosion of the skin, and, in some cases, the epithelial lining of other organs. EB is chronic, potentially disfiguring, and in some cases fatal. Individuals with EB have painful wounds and blisters that can lead to infection and scarring. There are many genetic and symptomatic variations of EB, but all forms share the common symptom of fragile skin that blisters and tears, sometimes from the slightest friction or trauma. There is currently no approved treatment for EB. Current standard of care consists of pain management and the cleaning and bandaging of open wounds to prevent infection.

### **About Amicus Therapeutics**

Amicus Therapeutics (Nasdaq: FOLD) is a global biotechnology company at the forefront of therapies for rare and orphan diseases. The Company has a robust pipeline of advanced therapies for a broad range of human genetic diseases. Amicus’ lead programs in development include the small molecule pharmacological chaperone migalastat as a monotherapy for Fabry disease, SD-101 for Epidermolysis Bullosa (EB), as well as novel enzyme replacement therapy (ERT) and biologic products for Fabry disease, Pompe disease, and other rare and devastating diseases.

### **Forward-Looking Statements**

This press release contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995 relating to clinical development of our product candidate and the timing and reporting of results from our clinical trial. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the potential goals, progress, and timing of results of our clinical trial, actual results may

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differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of our clinical study indicates that the product candidate is unsafe or ineffective; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that the clinical study could be delayed because we identify serious side effects or other safety issues; and the potential that we will need additional funding to complete our study. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2016. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

### **CONTACTS:**

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# Epidermolysis Bullosa Program

April 2017

## Safe Harbor Statement

*This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to clinical development of our product candidate and the timing and reporting of results from our clinical trial. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the potential goals, progress, and timing of results of our clinical trial, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of our clinical study indicates that the product candidate is unsafe or ineffective; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that the clinical study could be delayed because we identify serious side effects or other safety issues; and the potential that we will need additional funding to complete our study. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2016. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this presentation to reflect events or circumstances after the date hereof.*





# EB Program Overview

# EB Disease Overview

Rare, Devastating, Connective Tissue Disorder with No Approved Treatments

## Disease Overview

- Multiple genes cause disease
- Can affect internal organs
- Can be fatal
- Wounds can lead to life-threatening infections
- Diagnosis: infancy to adulthood

## Three Major EB Types

(~99% of EB Population)

SIMPLEX (75%)



DYSTROPHIC (20%)



JUNCTIONAL (5%)





# SD-101 – Patented High Concentration Allantoin

**Novel, Proprietary Topical Cream Promotes Healing of Wounds in EB and is Differentiated by Applicability for All Major EB Types**

<b>Active ingredient</b>	<ul style="list-style-type: none"> <li>Allantoin</li> </ul>
<b>RoA</b>	<ul style="list-style-type: none"> <li>Proprietary topical cream containing 6% allantoin, applied to entire body once daily</li> </ul>
<b>Proposed indication</b>	<ul style="list-style-type: none"> <li>All major EB types (Simplex, Dystrophic, and Junctional)</li> </ul>
<b>Phase of development</b>	<ul style="list-style-type: none"> <li>Phase 3 enrollment complete</li> </ul>
<b>Potential Triple-Targeting MoA<sup>1-8</sup></b>	<ul style="list-style-type: none"> <li>Anti-inflammatory, pro-collagen, anti-microbial</li> </ul>
<b>Formulation</b>	<ul style="list-style-type: none"> <li>Patented formula to deliver high concentration in highly stable, soluble form</li> </ul>



1. Araujo LU, et al. *Acta Cirurgica Brasileira* 2010;25:460-466.

2. Araujo LU et al. *Pharmazie*. 2012;67:355-360.

3. Frikeche J, et al. *Archives of Dermatological Research*. 2015;307:211-218.

4. Haag SF, et al. *Eur. J. Pharm. Biopharm.* 2014;86:227-233. 2015;70:155-164.

5. Couteau C, et al. *Arch Dermatol Res*. 2012; 304:817-821.

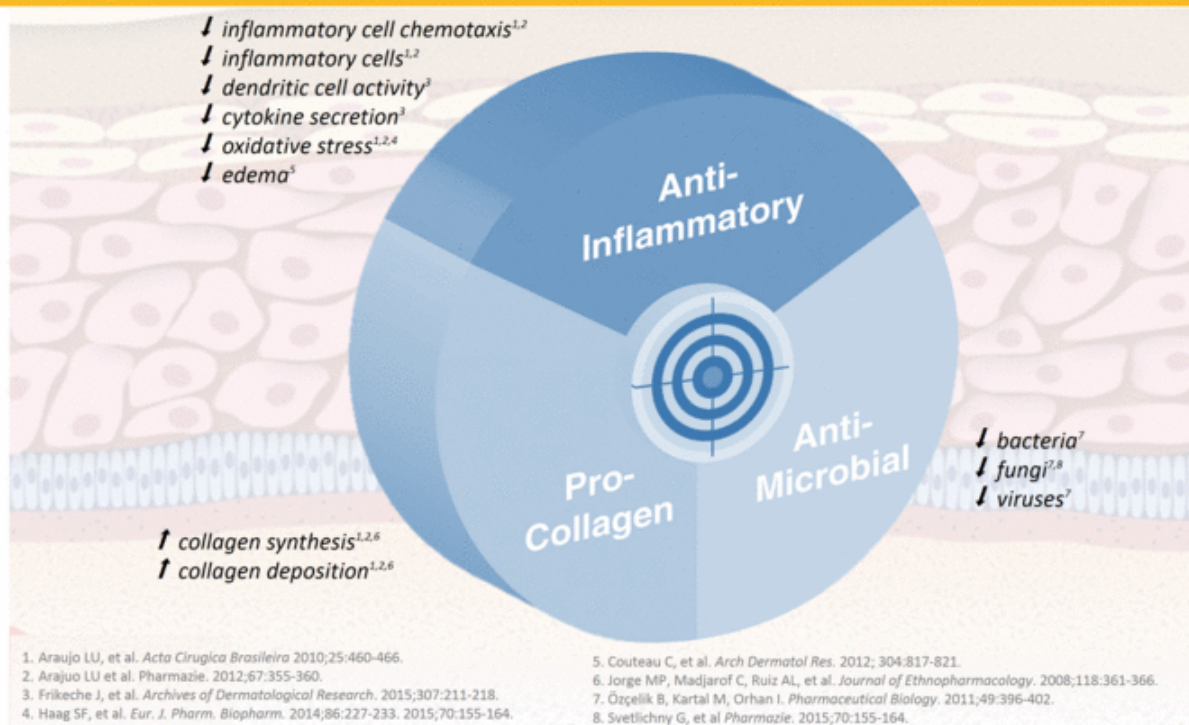
6. Jorge MP, Madjarof C, Ruiz AL, et al. *Journal of Ethnopharmacology*. 2008;118:361-366.

7. Özçelik B, Kartal M, Orhan I. *Pharmaceutical Biology*. 2011;49:396-402.

8. Svetlichny G, et al *Pharmazie*. 2015;70:155-164.

# Potential Mechanism of Action – Triple Targeting in Healing of Wounds

## Literature Describes 3 Main Mechanisms Contributing to Multi-Faceted Wound Healing Effects



# Proof of Concept Findings

## Phase 2 Results Informed Phase 3 Design

### Phase 2a Key Takeaways (SD-101 3%)



1-Year-Old Girl with EB Simplex at Baseline



Following 2 months of treatment with SD-101

**Breakthrough  
Therapy  
Designation**

### Phase 2b Key Takeaways (SD-101 6%)

- Faster time to wound closure
- Higher proportion with complete closure
- Reduction in total body surface area (BSA) of wounds
- Larger wounds (>10 cm<sup>2</sup>) showed widest separation versus placebo
- Daily administration generally safe and well-tolerated

**Informed  
Phase 3  
Study Design**

## Phase 3 Study - Delivering on Our EB Vision

Phase 3 Study Optimized for Success with Top-Line Data On Track for 3Q17



### SD-005 Study Design Optimized

- Sample size of up to 150 patients
- Larger baseline target wound size
- Time to wound closure endpoint elevated

### Status

- 95%+ participation in extension study
- Study overenrolled (>160 patients)
- Top-line data anticipated 3Q17





# SD-101 Clinical Data Overview

# U.S. Breakthrough Therapy Designation

## Positive Early Results from Phase 2a Study Led to Breakthrough Therapy Designation

- Open-label, 8-patient proof of concept study<sup>1</sup>
- Ages 6 months – 9 years
- All baseline target wounds  $\geq 10$  cm<sup>2</sup>
- SD-101 3% applied once daily for 3 months

### Key Findings

**87.5%**

of patients experienced complete closure of target wounds within 1 month

**57%**

reduction in affected body surface area by month 3

Daily administration generally safe and well-tolerated

### 1-Year-Old Girl with EB Simplex



Baseline



Following 2 months of treatment

1. Simplex (n=3), Junctional (n=3), Dystrophic (n=2)



## Phase 2b Design (Study 003)

### 3-Month, Double-Blind Treatment Period<sup>1</sup>

SD-101 6% (n=15)

SD-101 3% (n=16)

Placebo (n=17)

### Optional Extension (SD-004)

Open-Label SD-101 6%

42/44 Patients entered extension study

\$400K FDA Grant for Extension Study

#### Primary Efficacy Endpoint: Target Wound Healing at Month 1

- Baseline wound: Chronic ( $\geq 21$  days), size 5-50 cm<sup>2</sup>

#### Secondary Efficacy Endpoints Include:

- Time to target wound closure
- Change in Body Surface Area (BSA) of lesional skin

### 48 EB patients (age $\geq 6$ months)<sup>1</sup> - 1:1:1 Randomization - Daily Topical Application

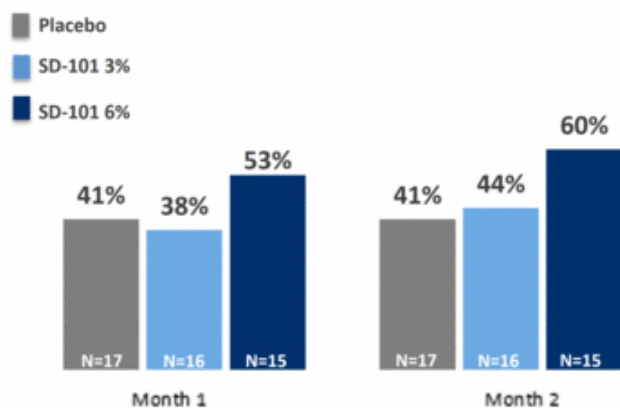
1. Assessments: 0, 14, 30, 60, 90 Days. 2. Initial Disease Severity: Mean target lesion size (cm<sup>2</sup>) 14.0 (range 5-39); mean lesional BSA: 19.4% (range 0.4-48%); mean wound age (days): 182 (range 21-1,639). EB types enrolled: Simplex (n=11), Dystrophic (n=29), and Junctional (n=8)

## Phase 2b Results

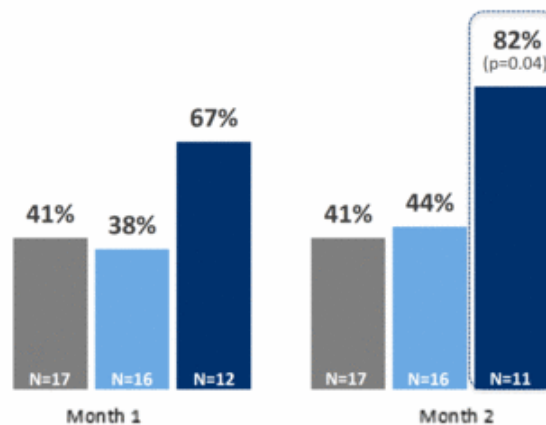
## SD-101 6% Demonstrated Higher Proportion of Complete Target Wound Closure

## ITT Population (n=48)

Proportion of Complete Target Wound Closure (%)

Evaluable Population<sup>1,2</sup> (n=45)

Proportion of Complete Target Wound Closure (%)



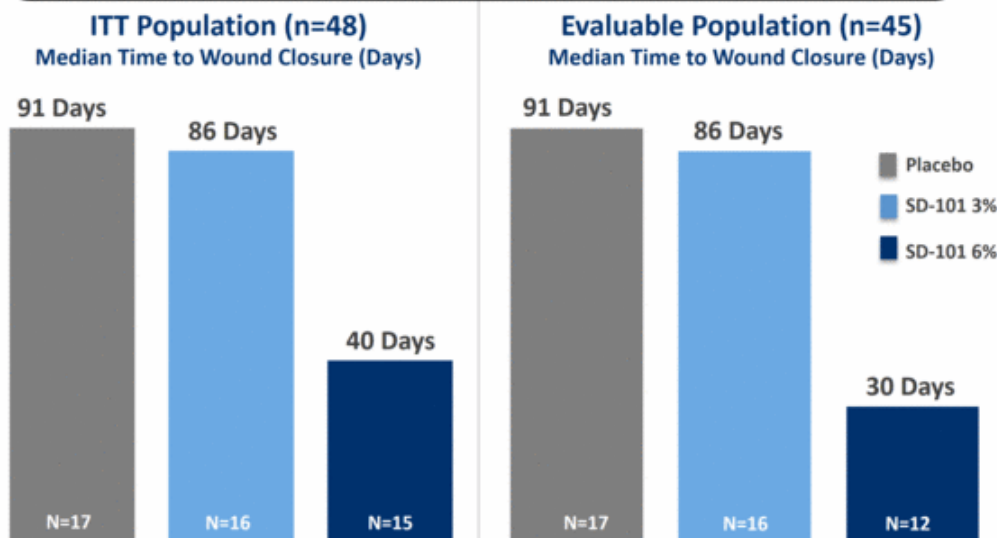
\*SD-101 6% vs placebo, unadjusted p=0.04

1. Excluded from Evaluable population: 1 patient (due to lost to follow-up), 2 patients (did not have single identified and qualified target lesion). 1 additional patient lost to follow up after Month 1 visit and is excluded from target wound assessment at later time points
2. Post-hoc analysis of patients with >10cm<sup>2</sup> wounds showed 2/4 (50%) response rate in 6% arm and 1/8 (12.5%) in placebo arm.

# Elevation of Time to Wound Closure Endpoint

## Statistical Analysis Plan (SAP) Near Final to Elevate Time to Wound Closure Endpoint<sup>1</sup>

### Median Time to Wound Closure in Phase 2b Study



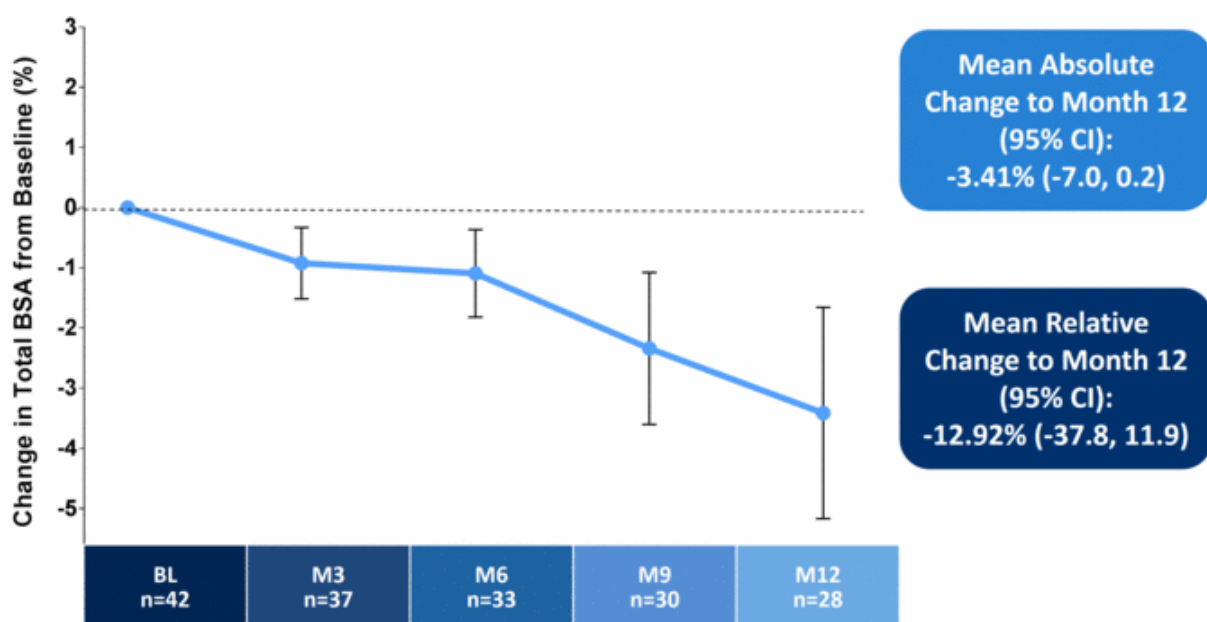
#### Time to Wound Closure

- Encouraging results in SD-101 Phase 2b study
- Measuring healing over time vs. one time point may further control for placebo response
- Results correlate with incidence of complete wound closure
- Statistical simulations indicate elevation of time to wound closure increases probability of study success

<sup>1</sup><http://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm071324.pdf>

# Phase 2b Extension (Study 004) Results

## Results on Total Body Surface Area (BSA) Affected by Wounds and Lesions



Note: Mean and SEM on change from baseline are plotted. Study 004 Total BSA baseline values are: N=42 Baseline population: 11.3. N=28 population used for Month 12 comparison: 10.9

# Phase 3 ESSENCE Study Design (SD-005)

## Study Design Optimized for Success

### 3-Month, Double-Blind Treatment Period

SD-101 6%

**>160 EB patients enrolled (age  $\geq$  1 month)**

*Baseline wound: Chronic ( $\geq$  21 days), size  $\geq$ 10 cm<sup>2</sup>*

Placebo

### Optional Extension (SD-006)

Open-Label SD-101 6%

>95% Participation in  
Extension Study  
(March 31, 2017)

Average Baseline Target  
Wound Size in Phase 3  
Population: **~20 cm<sup>2</sup>**  
(March 31, 2017)

#### Proposed Endpoints

- Time to target wound closure (elevated from secondary endpoint)
- Complete closure of target wound

#### Secondary Endpoints Include:

- Change in Body Surface Area (BSA) of lesions and blisters
- Patient-reported itching
- Patient-reported pain

Thank You

