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 28, 2017**

AMICUS THERAPEUTICS, INC.
(Exact name of registrant as specified in its charter)

Delaware	**001-33497**	**71-0869350**
(State or other Jurisdiction of Incorporation)	(Commission File Number)	(IRS Employer Identification No.)
1 Cedar Brook Drive, Cranbury, NJ		**08512**
(Address of Principal Executive Offices)		(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:

o Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

o Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

o Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

o Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company o

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. o

Item 8.01. Other Events.

On April 28, 2017, members of the management team of Amicus Therapeutics, Inc. (the "Company") will present posters related to its SD-101 program at the 76th Annual Meeting of the Society for Investigative Dermatology in Portland, Oregon. A copy of these posters is attached hereto as Exhibit 99.1.

Item 9.01. Financial Statements and Exhibits.

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

Exhibit No.	Description
99.1	Posters dated April 28, 2017 titled "Investigation of the Absorption of Allantoin From SD-101 in In Vitro Skin Models to Support Wound Healing" and "Characteristics of Patients With Epidermolysis Bullosa in the Phase 3 ESSENCE Study of SD-101."

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 28, 2017

By: /s/ ELLEN S. ROSENBERG
Name: Ellen S. Rosenberg
Title: General Counsel and Corporate Secretary

701

Investigation of the Absorption of Allantoin From SD-101 In Vitro Skin Models to Support Wound Healing

Paller AS[1], Nardi R[2], Do H[3], Reha A[3], Viereck C[3], Lagast H[3], Gault J[2], Castelli JP[3], Barth JA[3]

[1]Northwestern University Feinberg School of Medicine, Chicago, IL, USA; [2]Scioderm - An Amicus Therapeutics Company, Durham, NC, USA; [3]Amicus Therapeutics, Inc., Cranbury, N

INTRODUCTION

- Allantoin is a heterocyclic organic compound that has been investigated in wound healing, using formulations with minimal or unknown dermal penetration properties[1,2]
- SD-101 is a novel, proprietary, topical, allantoin-containing cream in development for the daily treatment of wounds caused by all major types of epidermolysis bullosa.[3] SD-101 has received Breakthrough Therapy designation from the US Food and Drug Administration[4]
  - Epidermolysis bullosa is a rare genetic disorder typically manifesting at birth as skin blistering/erosion and, in some cases, the epithelial lining of other organs, in response to minimal friction/trauma[5]
  - In a phase 2b study, patients with epidermolysis bullosa treated with SD-101 6% (SD-101 with 6% allantoin) demonstrated a higher rate of wound closure over a 1-month period than placebo-treated patients[3]
  - SD-101 6% is currently in phase 3 clinical development
- In vitro human cadaver and porcine models are recognized valuable tools to assess the skin absorption and to determine the pharmacokinetics of topically applied drugs[6-8]
- Separate preclinical studies of SD-101 with allantoin concentrations of up to 9% indicated no systemic absorption[9]

OBJECTIVE

- To investigate the skin absorption of 0.5%, 1.5%, 3%, 6%, and 9% concentrations of allantoin, the active ingredient in the SD-101 formulation, in skin models that mimic intact, broken, or blistered human skin

METHODS

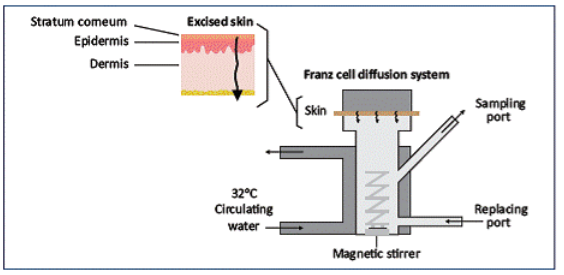
Models

- The skin absorption of 0.5%, 1.5%, 3%, 6%, and 9% concentrations of allantoin, the active ingredient in the SD-101 formulation, was investigated in 5 in vitro models:
  - Barrier-free to simulate delivery directly to the capillary bed
  - Unabraded porcine skin
  - Abraded porcine skin to simulate compromised skin
  - Intact (full thickness) human skin
  - Dermis-only human to mimic loss of skin barrier function due to broken skin

Skin Cadaver Preparation

- All human and porcine cadaver trunk skin without obvious signs of skin disease was stored at less than −70°C within 24 hours of death. On experiment day, the bagged tissue was thawed in 37°C water and rinsed to remove any adherent blood or material from the surface. Approximately 75% of the dermis was removed by dermatome or scalpel visually
  - Donor skin was cut into smaller sections and fitted on 0.8-cm² Franz diffusion cells. The diffusion cells were then placed between the epidermal chamber (route of drug application) and the dermal chamber, which was filled with magnetically stirred phosphate-buffered saline, and sampled at selected time points (Figure 1)
  - The permeability to tritiated water was determined prior to experimentation to assure skin integrity[10]

Figure 1. Schematic of the Skin Franz Cell Diffusion System[11]



Reprinted with permission from Kim KW et al. *Opt Express*. 2012;20(9):9476-9484.

Application

- Concentrations of SD-101 or vehicle (SD-101 minus active ingredient) were tested on ≥3 sections from 6 different skin donors (3 cadaver, 3 porcine) that were mounted in chambers designed to maintain skin at a temperature and humidity matching typical in vivo conditions
  - Each test product was applied at a target dose of 100 µL/cm² using a calibrated positive displacement pipette and then covered with 3 layers of medical-grade gauze

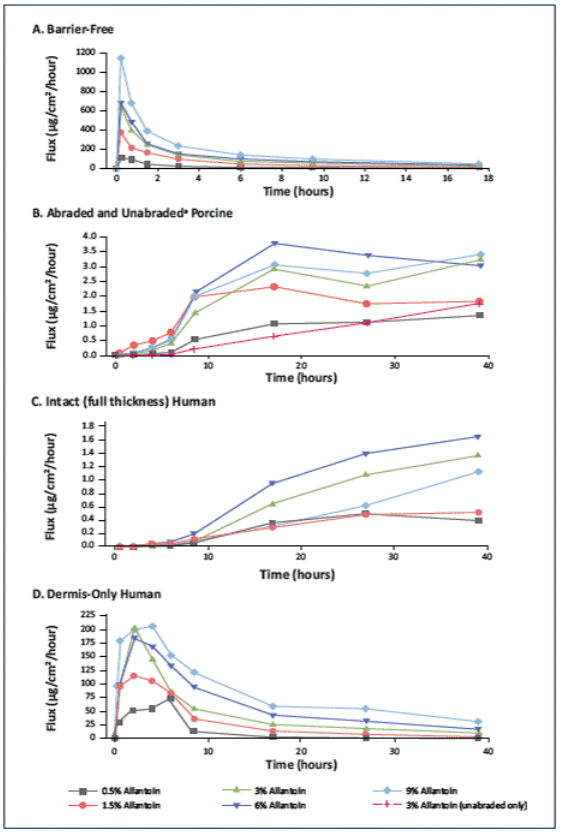
Sampling

- After SD-101 application, skin absorption (total absorption, rate of absorption, and skin content) was measured by monitoring the rate of appearance of drug in the solution bathing the inner surface of the skin
  - Samples were collected roughly 2, 4, 8, 12, 24, 32, and 48 hours after application and analyzed for allantoin using high-performance liquid chromatography with ultraviolet and mass spectrometry detection

RESULTS

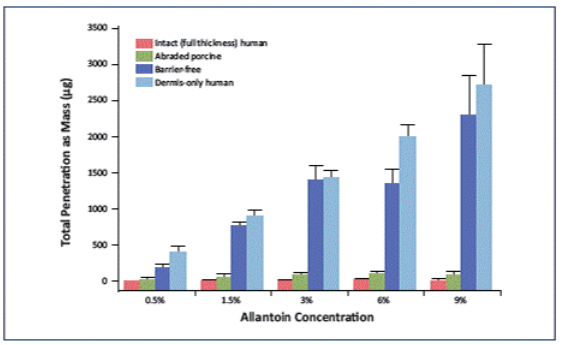
- In the SD-101 formulation:
  - There was evidence of skin absorption of allantoin in all models (Figures 2 and 3 and Table 1)
  - Skin absorption of allantoin was lowest in intact human skin (Figure 3 and Table 1)
  - Skin absorption increased with higher concentrations of allantoin in the dermis-only human model; uptake between the barrier-free and dermis-only human models was similar
  - Allantoin skin absorption in the human skin models was slow (≈8 hours for dermis-only), suggesting a long skin-exposure time (Figures 2C and 2D)

Figure 2. Skin Absorption of Allantoin (0.5% to 9%), a Component of SD-101, Within 40 Hours in Various Skin Models



Data are represented as mean from ≥3 replicates per formulation as µg/cm²/hour.
*Only 3% allantoin was tested in the unabraded model.

Figure 3. Total Skin Absorption of Allantoin Over 48 Hours From a Single Application



Data are represented as mean ± standard error from ≥3 replicates per formulation as total mass (µg).

Table 1. Total Skin Absorption of Allantoin (µg) Over 48 hours

Skin Model	Allantoin Conc		
	0.5%	1.5%	3%
Intact (full thickness) human	12.05 ± 1.88	13.23 ± 5.61	32.04 ± 11.
Abraded porcine	38.80 ± 18.34	68.91 ± 31.01	95.74 ± 25.
Barrier-free*	196.48 ± 45.30	792.25 ± 39.83	1399.49 ± 20
Dermis-only human	412.60 ± 96.30	910.51 ± 73.99	1434.19 ± 95
Unabraded porcine	Not tested	Not tested	38.64 ± 15.

Data are represented as mean ± standard error from ≥3 replicates per formulation as total
*Barrier-free study was conducted over 24 hours.

CONCLUSIO

- Allantoin, the active ingredient of the SD-101 formulati corneum, or skin, which may reduce wound formation
- In the SD-101 formulation, allantoin skin absorption in model was slow, suggesting a long skin-exposure time
- In damaged skin models that provide insight into the ab of allantoin increased significantly
- Substantial skin absorption of 6% allantoin occurred ov full-thickness human skin model, suggesting that SD-10 concentrations of allantoin than are currently used, is c
- In summary, these findings further support the therape treat wounds in a clinical setting and the ongoing phase bullosa (NCT02384460)

REFERENC

1. Becker LC et al. *Int J Toxicol*. 2010;29(suppl 2):845-975.
2. Xi H et al. *J Bacteriol*. 2000;182(19):5332-5341.
3. Paller A et al. Presented at: 42nd Annual Meeting of the Society for Minneapolis, MN.
4. Aggarwal SR. *Nature Biotechnol*. 2014;32(4):323-330.
5. El Hachem M et al. *Orphanet J Rare Dis*. 2014,9:76.
6. Franz TJ. *J Invest Derm*. 1975;64(3):190-195.
7. Franz TJ. In: Simon GA et al, eds. *Skin: Drug Application and Evaluat* Switzerland: Karger; 1978:58-68. *Current Problems in Dermatology;*
8. Schmook FP et al. *Int J Pharm*. 2001;215(1-2):51-56.
9. Amicus Therapeutics Inc. Investigators Brochure, Data on File.
10. Franz TJ, Lehman PA. *J Invest Dermatol*. 1990;94:525.
11. Kim KW et al. *Opt Express*. 2012;20(9):9476-9484.

ACKNOWLEDGM

Third-party medical writing assistance was provided by Apoth Therapeutics, Inc.

DISCLOSUR

Conflicts of Interest

AP is an investigator and a consultant for Amicus Therapeutics An Amicus Therapeutics Company and own stock in Amicus TI are employees of and own stock in Amicus Therapeutics.

Supported by Amicus Therapeutics, Inc.

Presented at the 76th Annual Meeting of the Society for Investigative Dermatology

696

Characteristics of Patients With Epidermolysis Bullosa in the Phase 3 ESSENCE Study of SD-101

Browning J[1], Bruckner A[2], Cornwall R[3], Lugo-Somolinos A[4], Paller A[5], Lagast H[6], Reha A[6], Gault J[7], Lenon W[7], Reklis L[7], Lazauskas R[6], M

[1]Texas Dermatology & Laser Specialists, San Antonio, TX, USA; [2]University of Colorado Denver, Aurora, CO, USA; [3]Cincinnati Children's Hospital, Cincinnati, OH, USA; [4]University of North Carolina School of
[5]Northwestern University Feinberg School of Medicine, Chicago, IL, USA; [6]Amicus Therapeutics, Inc., Cranbury, NJ, USA; [7]Scioderm - An Amicus Therapeutics Company, Durham, N

INTRODUCTION

- Epidermolysis bullosa is a rare, often severe genetic disorder characterized by mechanical fragility and blistering or erosion of the skin, mucosa, or epithelial lining of other organs, in response to little or no apparent trauma[1]
  - Often diagnosed in neonates; occurs in 19 per million live births in the United States as estimated from the National Epidermolysis Bullosa Registry, a cross-sectional and longitudinal epidemiologic study of patients with epidermolysis bullosa across the continental United States[1]
  - Subtypes differ by physical manifestations, genetic makeup, and prognosis[2]
  - Symptoms (blistering, scarring, disfiguration, pain) can vary in severity and may lead to premature death as well as major morbidities, including life-threatening infections, sepsis, and squamous cell carcinoma[1,3-6]
- SD-101 is a novel, proprietary, topical, allantoin-containing cream under investigation in clinical trials as a potential treatment for skin lesions associated with epidermolysis bullosa[7,8]
- In 2013, SD-101 became one of the first drug candidates to receive Breakthrough Therapy designation from the US Food and Drug Administration for the treatment of patients with epidermolysis bullosa[9,8]
- The efficacy and safety of SD-101 has been investigated in SD-003, a phase 2b, multicenter, randomized, double-blind, vehicle-controlled, dose-ranging, 3-month study (NCT02014376)[4]
  - Treatment with SD-101 cream containing 6% allantoin (SD-101 6%) demonstrated a higher rate of wound closure in patients with epidermolysis bullosa relative to treatment with vehicle
  - SD-101 6% was generally safe and well tolerated in patients with epidermolysis bullosa

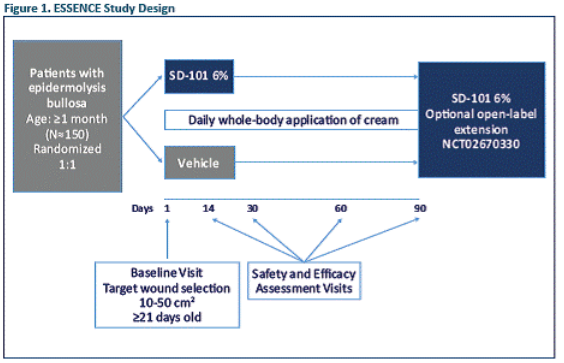
OBJECTIVE

- To describe the baseline characteristics of patients with epidermolysis bullosa enrolled in the ongoing ESSENCE trial as of February 2017

METHODS

- ESSENCE (SD-005; NCT02384460) is a phase 3, multicenter, randomized, double-blind, vehicle-controlled, ongoing study to assess the efficacy and safety of SD-101 6% vs vehicle (SD-101 0%) on lesions in patients with simplex, recessive dystrophic, or junctional non-Herlitz epidermolysis bullosa[8] (Figure 1)
  - The two primary endpoints are time to complete target wound closure and proportion of patients with complete target wound closure
  - Secondary endpoints include change in body surface area index (BSAi) of lesions and blisters, patient-reported itching, and patient-reported pain

Figure 1. ESSENCE Study Design



ESSENCE was initiated in Q2 of 2015, and topline results are expected in Q3 of 2017.
Q=quarter.

Key Inclusion Criteria

- Diagnosis of simplex, recessive dystrophic, or junctional non-Herlitz epidermolysis bullosa
- Age ≥1 month
- Target wound ≥21 days old and between 10 and 50 cm² in size

Key Exclusion Criteria

- Clinical evidence of local infection in the selected target wound
- Use of immunotherapy or cytotoxic chemotherapy ≤60 days before enrollment
- Use of any investigational drug or systemic or topical steroidal therapy ≤30 days before enrollment (inhaled steroids and ophthalmic drops containing steroids are allowed)
- Use of systemic antibiotics ≤7 days before enrollment
- Arterial or venous disorder resulting in ulcerated lesions

Application

- SD-101 6% or vehicle is applied topically once daily to the entire body as a thin layer for a period of 90 days. Patients/parents are taught how to apply the cream at first visit

Assessments

- During patient visits, the following evaluations are performed:
  - Baseline-selected target wound closure evaluation using ARANZ SilhouetteStar™. Complete target wound closure is defined as skin re-epithelialization without drainage
  - BSAi of lesional skin: percentage of total body coverage of epidermolysis bullosa–related lesions (blisters, erosions, ulcerations, scabbing, bullae, and eschars, as well as areas that are weeping, sloughing, oozing, crusted, and/or denuded)
  - BSAi of wound burden: percentage of total body coverage of epidermolysis bullosa wounds, defined as open areas on the skin (epidermal covering is disrupted)
  - Itch, using the Itch Man Pruritus Assessment Tool[10]
  - Pain, using the Face, Legs, Activity, Cry, Consolability (FLACC) scale for patients aged 1 month to 3 years and the Wong-Baker FACES® Pain Scale for patients aged ≥4 years. Each of the 5 categories in the FLACC scale is scored from 0 to 2, with a cumulative score ranging from 0-10. The Wong-Baker FACES® Pain Scale also ranges from 0 to 10. Higher scores indicate greater pain[11,12]

BASELINE RESULTS

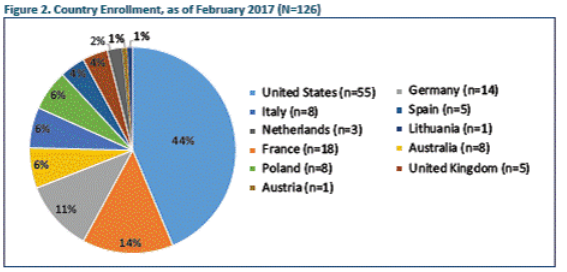
- As of February 21, 2017, ESSENCE was enrolling patients worldwide and included patients with a wide variety of ages and all major types of epidermolysis bullosa (Table 1 and Figure 2)

Table 1. ESSENCE Baseline Demographics and Characteristics, as of February 2017

Baseline	Total (N=126)
Age	
All patients, years (range)	15.1 ± 14.3 (0, 67)
0 to ≤1 month	0 (0)
>1 to ≤24 months	17 (13.5)
>24 months to ≤12 years	58 (46.0)
>12 to ≤18 years	15 (11.9)
>18 to ≤65 years	35 (27.8)
>65 years	1 (0.8)
Male	59 (46.8)
Race	
Black/African American	7 (5.6)
Asian	8 (6.3)
White	105 (83.3)
Unknown	5 (4.0)
Mixed	1 (0.8)
Epidermolysis bullosa subtype	
Simplex	14 (11.1)
Recessive dystrophic	85 (67.5)
Junctional non-Herlitz	27 (21.4)
Body mass index, kg/m²	
All patients (n=125)	17.4 ± 4.4
Age >1 to ≤24 months (n=17)	16.5 ± 4.2
Age >24 months to ≤12 years (n=57)	15.6 ± 3.7
Age >12 to ≤18 years (n=15)	16.7 ± 3.3
Age >18 to ≤65 years (n=35)	20.9 ± 4.2
Age >65 years (n=1)	20.1 ± N/A
BSAi of lesional skin, %	
All patients (n=124)	24.4 ± 19.4
Age >1 month to <8 years (n=42)	15.6 ± 14.8
Age ≥8 years (n=82)	28.9 ± 20.0
BSAi of wound burden, %	
All patients (n=124)	10.8 ± 11.0
Age >1 month to <8 years (n=42)	8.9 ± 11.5
Age ≥8 years (n=82)	11.9 ± 10.6
Target wound size, cm²	21.67 ± 27.5

Numbers reported as mean ± standard deviation (min, max) or n (%). The number of patients included in each analysis is noted if data were not available for all 126 patients.
BSAi=body surface area index; N/A=not applicable.

Figure 2. Country Enrollment, as of February 2017 (N=126)



- Patients enrolled in ESSENCE demonstrated a substantia

Table 2. Baseline Pain Scores

Pain Assessment
FLACC scale (age >1 month to ≤3 years)
Mean ± standard deviation
Median (min, max)
Wong-Baker FACES® Pain Scale (age ≥4 years)
Mean ± standard deviation
Median (min, max)

Both pain scales range from 0-10, with higher scores indicating greater pain.
FLACC=Face, Legs, Activity, Cry, Consolability; n/N=number of patients included in analysis/

- The medical history of patients enrolled in ESSENCE var medical conditions at baseline being pruritus and pain (

Table 3. Medical History by System Organ Class Reported in A

System Organ Class and Medical Condition
Gastrointestinal disorders
Constipation
Gastro-oesophageal reflux disease
Skin and subcutaneous tissue disorders
Pruritus
General disorders and administration-site conditions
Pain
Infections and infestations
Surgical and medical procedures
Blood and lymphatic system disorders
Metabolism and nutrition disorders
Injuries, poisonings, and procedural complications
Eye disorders
Congenital, familial, and genetic disorders

Data reported as n (%).

CONCLUSIO

- ESSENCE is one of the largest clinical trials of an investig with epidermolysis bullosa
- Patients enrolled thus far have substantial pain burden represent a range of disease severity (both in epidermo coverage of epidermolysis bullosa lesions), ages, and ge
- Top-line results for the phase 3 ESSENCE study are expe

REFERENC

1. El Hachem M et al. *Orphanet J Rare Dis.* 2014;9:76.
2. Fine J-D. *Orphanet J Rare Dis.* 2010;5:12.
3. Pagliarello C, Tabolli S. *Expert Rev Pharmacoecon Outcomes Res.* 2010;10(3):325
4. Brandling-Bennett HA, Morel KD. *Pediatr Dermatol.* 2010;27(1):25-28.
5. Mellerio JE et al. *Br J Dermatol.* 2016;174(1):56-67.
6. Montaudié H et al. *Orphanet J Rare Dis.* 2016;11(1):117.
7. Paller A et al. Presented at: 42nd Annual Meeting of the Society for Pediatric De
8. Aggarwal SR. *Nature Biotechnol.* 2014;32(4):323-330.
9. ClinicalTrials.gov Web site. https://clinicaltrials.gov/ct2/show/NCT02384460. Ac
10. Morris V et al. *J Burn Care Res.* 2012;33(3):419-424.
11. Merkel SI et al. *Pediatr Nurs.* 1997;23(3):293-797.
12. Wong DL et al, eds. *Wong's Essentials of Pediatric Nursing.* 6th ed. St. Louis, MO

ACKNOWLEDGM

The authors acknowledge the patients, their families, and epi
as well as the ESSENCE study investigators. Third-party medica
ApotheCom and was supported by Amicus Therapeutics, Inc.

DISCLOSUR

Conflicts of Interest

JB is an investigator and conducts clinical research for Amicus Th
and Regeneron, and is a speaker for Medimetriks and Promius. A
conducts clinical research for Amicus Therapeutics and Scioderm
and serves on advisory boards for Anacor and Pfizer. RC is an inv
Therapeutics Company and Amicus Therapeutics. AL-S is an inve
Scioderm - An Amicus Therapeutics Company. AP is a consultant
Amicus Therapeutics. HL, AR, and RL are employees of and own
Amicus Therapeutics. JG, WL, LR, and RN are employees of
Scioderm - An Amicus Therapeutics Company and own stock
in Amicus Therapeutics.

Supported by Amicus Therapeutics, Inc.

Presented at the 76th Annual Meeting of the Society for Investigative Dermatology