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.
- Often diagnosed in neonates; occurs in 1 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.
- Subtypes differ by physical manifestations, genetic makeup, and prognosis.
- Symptoms (blistering, scarring, disfigurement) can vary in severity and may lead to premature death as well as major morbidity, including life-threatening infections, sepsis, and squamous cell carcinoma.
- 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.
- 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.
- 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).
- 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 in patients with simplex, recessive dystrophic, or junctional non-Herlitz epidermolysis bullosa.
- 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.

Assessments

- During patient visits, the following evaluations are performed:
  - Baseline-selected target wound closure evaluation using ARAANZ Silhouette™.
  - Complete target wound closure is defined as skin re-epithelialisation without drainage.
  - BSAI of lesional skin: percentage of total body coverage of epidermolysis bullosa-related lesions (blisters, erosions, ulcerations, scabbing, bullae, and eczema, as well as areas that are not weeping, sloughing, oozy, 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™.
  - Pain, using the Faces, Legs, Activity, Cry, and Consistency (FLACC) scale for patients aged 1 month to 3 years and the Wong-Baker FACES® Pain Scale for patients aged 4 years.

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)

CONCLUSIONS

- ESSENCE is one of the largest clinical trials of an investigational drug conducted in patients with epidermolysis bullosa.
- Patients enrolled thus far have substantial pain burden and large chronic wounds, and represent a range of disease severity (both in epidermolysis bullosa type and extent of body coverage of epidermolysis bullosa lesions), ages, and geographic distribution.
- Top-line results for the phase 3 ESSENCE study are expected in Q3 of 2017.

REFERENCES

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DISCLOSURE

JF is an investigator and conducts clinical research for Amicus Therapeutics, Galderma, Valeant, Patagonia, and Regeneron, and is a speaker for MedImmune and Promius. AJ is a consultant and conducts clinical research for Amicus Therapeutics and Scioderm - An Amicus Therapeutics Company, and serves on advisory boards for Anacor and Pfizer. RC is an investigator for Scioderm - An Amicus Therapeutics Company and Amicus Therapeutics. AL-S is an investigator for Scioderm - An Amicus Therapeutics Company and serves on advisory boards for Anacor and Pfizer. RR is an employee of Anacor and Pfizer.

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