

**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): **August 30, 2015**

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 August 31, 2015, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing the entry into an Agreement and Plan of Merger (the "Merger Agreement"), dated August 30, 2015, by and among the Company, Scioderm, Inc. ("Scioderm"), Titan Merger Sub Corp., a wholly owned subsidiary of the Company, and Fortis Advisors LLC, as the Shareholders' Agent, providing for the acquisition by the Company of Scioderm (the "Merger"). A copy of the press release is attached hereto as Exhibit 99.1 and is incorporated herein by reference.

In addition, the Company will be providing supplemental information regarding the Merger in connection with a presentation to investors. The slides to be used in connection with this investor presentation are attached hereto as Exhibit 99.2 and are incorporated herein by reference.

The information required by Item 1.01, including a copy of the Merger Agreement, will be filed in a separate Current Report on Form 8-K.

The information contained in Exhibit 99.1 and 99.2 shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, nor shall it be deemed incorporated by reference in filings under the Securities Act of 1933.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

- 99.1 Press Release dated August 31, 2015
- 99.2 August 31, 2015 Conference Call Presentation Materials

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.

Date: August 31, 2015

By: /s/ WILLIAM D. BAIRD III

Name: William D. Baird III

Title: Chief Financial Officer

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EXHIBIT INDEX

| Exhibit No. | Description |
|--------------------|--|
| 99.1 | Press Release dated August 31, 2015 |
| 99.2 | August 31, 2015 Conference Call Presentation Materials |

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**Amicus Therapeutics to Acquire
Rare Disease Company Scioderm, Inc.**

***Scioderm's Lead Drug Candidate Zorblisa™ in Phase 3 Study for Rare Disease
Epidermolysis Bullosa (EB) Granted FDA Breakthrough Therapy Designation***

FDA Has Agreed to Rolling NDA Submission for Zorblisa Beginning in 4Q15

***Acquisition Advances Amicus Vision to Create One of the
World's Leading Rare Disease Biotechnology Companies***

Conference Call Today at 8:00 a.m. ET

CRANBURY, NJ, August 31, 2015 — Amicus Therapeutics (Nasdaq: FOLD), a biotechnology company at the forefront of therapies for rare and orphan diseases, and Scioderm, Inc. a privately-held biopharmaceutical company focused on developing innovative therapies for treating diseases with high unmet need, have signed a definitive agreement under which Amicus will acquire 100% of the capital stock of Scioderm, Inc.

Transaction highlights

- Excellent strategic fit with Amicus' patient-centric vision to develop and commercialize advanced therapies for devastating rare and orphan diseases
- Leverages Scioderm development team's EB expertise with Amicus' global clinical infrastructure to advance Zorblisa toward regulatory approvals and Amicus' commercial, patient advocacy and medical affairs infrastructure to support a successful global launch
 - Potential first-to-market therapy to address estimated \$1 billion+ global commercial opportunity
 - FDA breakthrough therapy designation based on positive Phase 2 proof-of-concept data
 - Phase 3 pivotal study (SD-005) is currently enrolling pediatric and adult EB patients across all major subtypes to support global regulatory approvals — data anticipated in 1H16
 - Well-defined global regulatory pathway — agreement on rolling NDA in U.S. and pediatric investigation plan (PIP) in Europe
- Creates a leading rare disease portfolio that is well-positioned to bring substantial value to patients and shareholders - potential for Fabry commercial product launch, EB marketing submissions, and Pompe Phase 3 study in 2016

“This acquisition is a major step forward toward our strategic vision and is transformative for the Epidermolysis Bullosa, or EB, community as well as the shareholders of Amicus and Scioderm,” said John F. Crowley, Chairman and Chief Executive Officer of Amicus and Board Member of Scioderm. “EB is a disorder that is utterly devastating and painful as it causes extremely fragile skin that blisters and tears from minor friction or trauma. In many children it leads to severe complications and a very early death. Amicus is committed now to advancing the tremendous mission of Scioderm's Co-Founder and CEO Dr. Robert Ryan, who we are proud to welcome to our senior leadership team at Amicus. We believe we are well-positioned to rapidly complete the clinical development of Zorblisa and to make Zorblisa commercially available for all EB patients as quickly as possible. When combined with migalastat for Fabry disease and ATB200 for Pompe disease, this acquisition solidly positions Amicus as a leading global rare disease company dedicated to bringing substantial value to patients and shareholders.”

Scioderm's lead product candidate Zorblisa is a novel, late-stage, proprietary topical cream and potential first-to-market therapy for EB. Zorblisa has established positive proof-of-concept in Phase 2 studies for the treatment of lesions in patients suffering with EB, and is currently being investigated in a Phase 3 study to support global regulatory approvals. Zorblisa was one of the first products to receive FDA breakthrough therapy designation in 2013, and was the first-ever treatment in EB clinical studies to show significant benefit in wound closure across all major EB subtypes.

Amicus estimates that EB may represent a potential \$1 billion+ global market opportunity based on third party market research. The current standard of care is palliative treatments which cost \$10,000 to \$15,000 per month, and mainly consist of bandaging, treating the open wounds to prevent infection and trying to manage patients' pain. An estimated 30,000 to 40,000+ people are currently diagnosed with EB in major markets.

“Amicus is a champion of the rare disease community that, together with Scioderm, understands our sense of urgency to see a treatment approved for EB,” said Brett Kopelan, Executive Director of the Dystrophic Epidermolysis Bullosa Research Association of America (DebRA). “The EB community will be well-served by the experience and broad, global capabilities that Amicus adds to Scioderm.”

“Both Amicus and Scioderm are wholly and passionately focused on patients with rare diseases, and share a common vision and similar values,” said Robert Ryan, Ph.D., President and Chief Executive Officer of Scioderm. “John Crowley has been a dedicated board member providing valuable counsel to Scioderm over the past several years, during which time he has been deeply involved with the EB community. This combination of Amicus and Scioderm is a major win for EB patients. With the added resources and expertise that Amicus provides for the Zorblisa program, we are more confident than ever in our potential for success and our ability to deliver significant benefits to patients and families living with the devastating effects of EB.”

The Transaction

Amicus will acquire Scioderm in a cash and stock transaction. At closing, Amicus will pay Scioderm shareholders \$229 million, of which \$125 million will be paid in cash and \$104 million will be paid through the issuance of 7 million newly issued Amicus shares. Amicus has agreed to pay up to an additional \$361 million to Scioderm shareholders in cash or stock upon achievement of certain clinical and regulatory milestones and \$257 million to Scioderm shareholders in cash or stock upon achievement of certain sales milestones. If Zorblisa is approved, EB qualifies as a rare pediatric disease and a Priority Review Voucher will be requested. If the Priority Review voucher is obtained and subsequently sold, Amicus will pay Scioderm shareholders the lesser of \$100 million or 50% of the proceeds of such sale. The transaction is subject to customary conditions, including the expiration or termination of the waiting period under the Hart-Scott-

Rodino Antitrust Improvements Act. The Boards of both companies have approved the transaction and the companies currently anticipate that the transaction will be completed in the third quarter of 2015.

Amicus intends to finance the acquisition through cash on hand and has a \$50 million debt commitment from Redmile Group. Leerink Partners LLC is acting as financial advisor to Amicus. Skadden, Arps, Slate, Meagher & Flom LLP is acting as legal counsel to Amicus. J.P. Morgan is acting as financial advisor to Scioderm. Cooley LLP is acting as legal advisor to Scioderm.

Based on the closing of the Scioderm acquisition, the anticipated debt financing and the forecasted spending on Zorblisa development, Amicus expects to end 2015 with \$200 million to \$225 million of cash on hand. Pro-forma cash post-closing is expected to fund the current operating plan (including Zorblisa) into 2017.

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, August 31, 2015 at 8:00 a.m. ET to discuss the proposed acquisition of Scioderm. Interested participants and investors may access the conference call at 8:00 a.m. ET by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international).

An audio webcast and slide presentation can also be accessed via the Investors section of the Amicus Therapeutics corporate web site at <http://www.amicusrx.com>, and will be archived for 30 days. Web participants are encouraged to go to the web site 15 minutes prior to the start of the call to register, download and install any necessary software. A telephonic replay of the call will be available for seven days beginning at 8:00 p.m. ET today. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); participant code 28588321.

About Epidermolysis Bullosa (EB)

Epidermolysis Bullosa (EB) is a chronic, rare genetic connective tissue disorder with no approved treatment options. EB is debilitating, disfiguring, and potentially fatal. There are many genetic and symptomatic variations of EB that all share the prevalent manifestation of fragile skin that blisters and tears from minor friction or trauma. Patients with the more severe forms of EB have generalized blistering and lesions affecting a substantial percentage of their bodies that can lead to infection and scarring, and, in severe cases, death. Internal organs and bodily systems can also be severely affected by the secondary complications and illnesses. There is currently no FDA approved treatment for EB. Current standard of care consists of bandaging and bathing the open wounds to prevent infection and trying to manage patients' pain. EB affects all racial, ethnic and genders equally.

About Zorblisa Phase 3 Clinical Trial (SD-005)

A Phase 3 multi-center, randomized, double-blind, placebo-controlled study (SD-005) in the U.S. and Europe is currently underway and expected to support registration globally. The study is currently enrolling individuals who are 1 month and older with a diagnosis of Simplex, Recessive Dystrophic, or Junctional non-Herlitz EB who have at least 1 target wound present for 21 days or more. Half the patients receive Zorblisa cream (also known as SD-101) and the other half receive placebo cream, applied topically once daily to the entire body for 90 days. The primary outcome measure is complete target wound closure within 2 months. Secondary outcome measures include 1) median time to complete target wound closure; 2) change in lesional skin at Month 2; 3)

change in itching at Day 7; and 4) change in pain at Day 7. Patients who complete the 90-day primary treatment period will be eligible to receive Zorblisa in an open-label extension study (SD-006). For more information please visit Scioderm's website at www.sderm.com.

About Scioderm

Scioderm is a privately held, clinical-stage biopharmaceutical company focused on developing innovative therapies to address diseases with high unmet need, including rare diseases. Scioderm was financed initially in 2013 by Morgenthaler Ventures and Technology Partners, followed by a subsequent financing that was led by Redmile Group and included the initial investors.

Ralph (Chris) Christoffersen, Ph.D., Chairman of the Board, noted that "Robert Ryan and the Scioderm team have done an outstanding job in bringing Zorblisa through both preclinical and clinical studies. It is a real pleasure to join with the excellent team at Amicus to continue the development and commercialization of this product which we believe will have a significant positive impact on the lives of EB patients and their families."

The company's lead therapy, Zorblisa (SD-101), is in Phase 3 development for treatment of the skin effects associated with Epidermolysis Bullosa (EB), a rare genetic connective tissue disorder. Scioderm was selected as a 2013 "Fierce Top 15" company by FierceBiotech, and considered one of the top 15 emerging companies in the biotech industry. The company is headquartered in Durham, North Carolina. Additional information about Scioderm can be found at www.sderm.com.

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq:FOLD) is a biotechnology company at the forefront of therapies for rare and orphan diseases. The Company is developing novel, first-in-class treatments for a broad range of human genetic diseases, with a focus on delivering new benefits to individuals with lysosomal storage disorders. Amicus' lead programs in development include the small molecule pharmacological chaperone migalastat as a monotherapy for Fabry disease, as well as next-generation enzyme replacement therapy (ERT) products for Fabry disease, Pompe disease, and MPS I.

Forward-Looking Statements

This press release contains, and the accompanying conference call and slide presentation will contain, "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to the planned acquisition of Scioderm, the expected financial impact and benefits to Amicus of such acquisition, and anticipated milestones and other expectations regarding Scioderm's product development activities, clinical trials and commercialization, preclinical and clinical development of Amicus' candidate drug products, the timing and reporting of results from preclinical studies and clinical trials evaluating Amicus' candidate drug products, financing plans, and the projected cash position for the Company. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "potential," "plan," "targets," "likely," "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. Such forward-looking statements are based upon current expectations that involve risks, changes in circumstances, assumptions and uncertainties. The inclusion of forward-looking statements should not be regarded as a representation by Amicus that any of its plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong. They can be affected by inaccurate assumptions Amicus might make or by known or unknown risks and uncertainties. Important factors that could cause or contribute to such differences include

risks relating to: the possibility that the transaction with Scioderm will not be completed; uncertainties as to the timing of the transaction; the possibility that the expected benefits of the transaction will not be fully realized by us or may take longer to realize than expected; future results of on-going or later clinical trials for Zorblisa; our ability to obtain regulatory approvals and commercialize Zorblisa following the closing; and market acceptance of Zorblisa. Also, with respect to statements regarding the goals, progress, timing and outcomes of discussions with regulatory authorities and the potential goals, progress, timing and results of preclinical studies and clinical trials, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in the business of Amicus, including, without limitation: the potential that results of clinical or pre-clinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities may not grant or may delay approval for our product candidates; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we will need additional funding to complete all of our studies and, our dependence on third parties in the conduct of our clinical studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding projections of the Company's cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. 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, 2014 2014 and our Form 10-Q for the quarter ended June 30, 2015. 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 Amicus undertakes no obligation to revise or update this news release to reflect events or circumstances after the date hereof. This caution is made under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995.

CONTACTS:

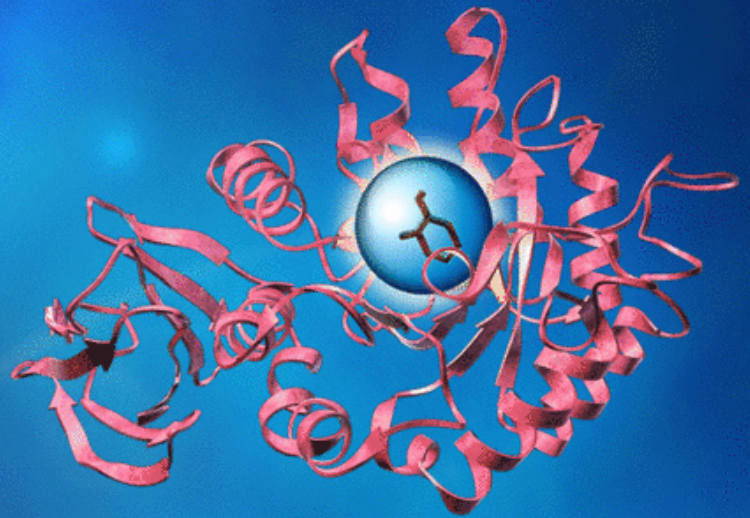
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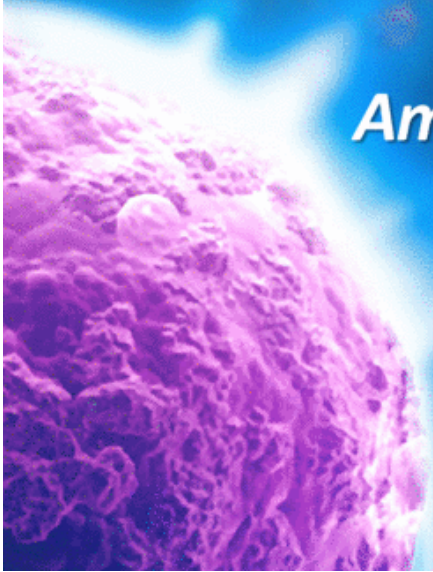
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Amicus Acquisition of Scioderm, Inc. Overview

August 31, 2015

*at the forefront of therapies
for rare and orphan diseases*



This presentation contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995 relating to the planned acquisition of Scioderm, the expected financial impact and benefits to Amicus of such acquisition, and anticipated milestones and other expectations regarding Scioderm’s product development activities, clinical trials and commercialization, and business, operations and financial conditions of Amicus including but not limited to preclinical and clinical development of Amicus’ candidate drug products, cash runway, and the timing and reporting of results from clinical trials evaluating Amicus’ candidate drug products. Words such as, but not limited to, “look forward to,” “believe,” “expect,” “anticipate,” “estimate,” “intend,” “plan,” “would,” “should” and “could,” and similar expressions or words, identify forward-looking statements. Although Amicus believes the expectations reflected in such forward-looking statements are based upon reasonable assumptions, there can be no assurance that its expectations will be realized. Actual results could differ materially from those projected in Amicus’ forward-looking statements due to numerous known and unknown risks and uncertainties, including the “Risk Factors” described in our Annual Report on Form 10-K for the year ended December 31, 2014 and our Form 10-Q for the quarter ended June 30, 2015. All forward-looking statements are qualified in their entirety by this cautionary statement, and Amicus undertakes no obligation to revise or update this presentation to reflect events or circumstances after the date hereof.

Amicus-Scioderm: Strong Strategic Fit

Five things we look for in an opportunity:



Satisfies all
of these
criteria

- ✓ Rare disease
- ✓ Devastating disorder
- ✓ First in class or best in class therapy
- ✓ Meaningful clinical benefit for patients
- ✓ Significant orphan patient population

Strategic Acquisition Aligned with Amicus Mission



Amicus Therapeutics is a biopharmaceutical company at the forefront of developing advanced therapies to treat a range of devastating rare and orphan diseases

Amicus-Scioderm: *Significant Value for All Key Stakeholders*

Acquisition Significantly Augments Amicus' Pipeline and Leverages Our Patient-Centric, Rare Disease Expertise to Benefit Epidermolysis Bullosa Community

Epidermolysis Bullosa (EB) is a \$1B+ potential market¹ with significant unmet need and no approved treatments

Zorblisa™ has Breakthrough Therapy Designation with Phase 3 study underway for all major EB subtypes

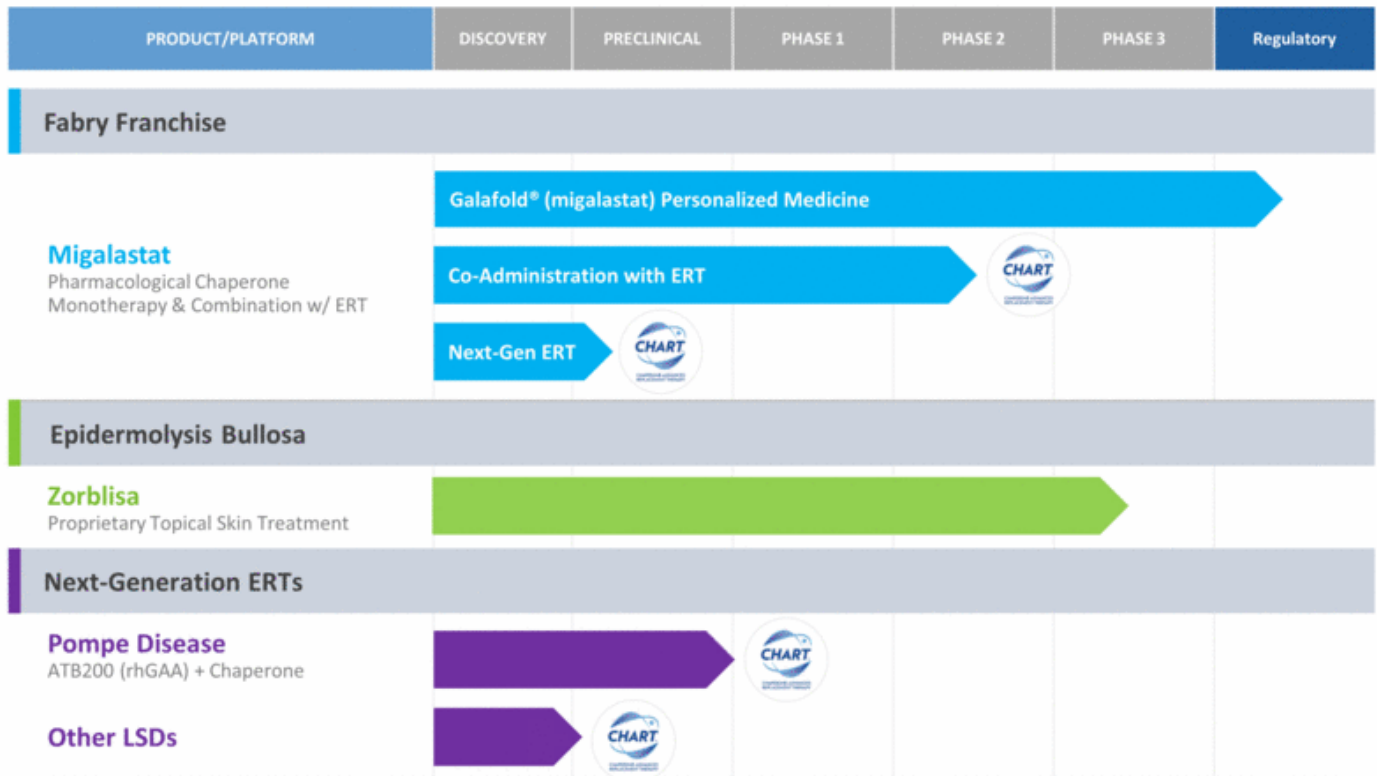
Leverages Scioderm's EB experience and Amicus' rare disease expertise to accelerate development for patients

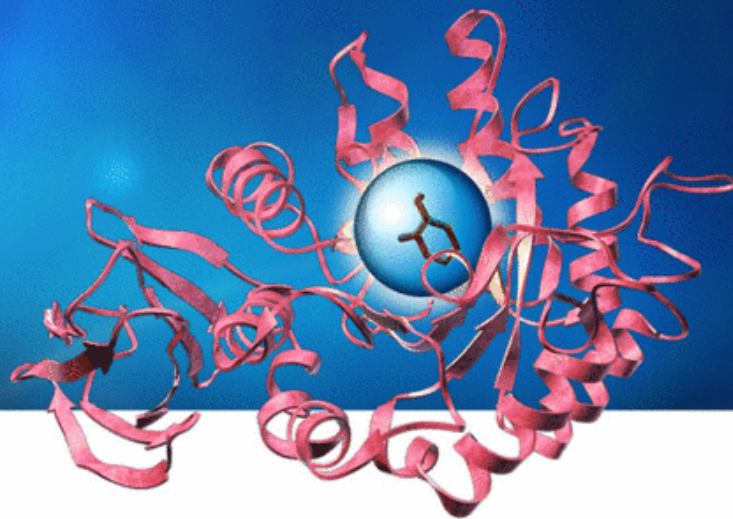
“

Amicus is a champion of the rare disease community, and together with Scioderm, understands our sense of urgency to see a treatment approved for EB. The EB community will be well-served by the experience and broad, global capabilities that Amicus adds to Scioderm”

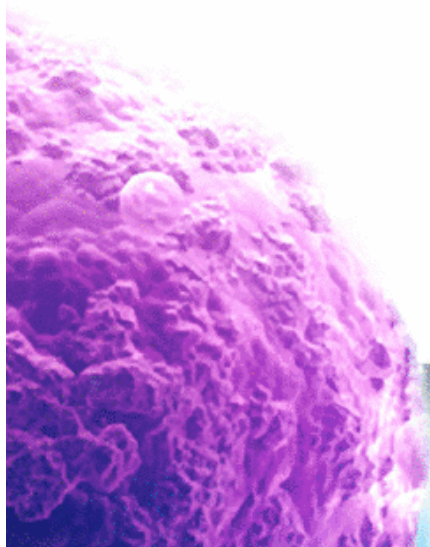
*- Brett Kopelan, Executive Director,
Dystrophic Epidermolysis Bullosa
Research Association of America (DebRA)*

Acquisition Strengthens Amicus' Pipeline





Epidermolysis Bullosa
“EB”



Epidermolysis Bullosa (EB)

Rare, Genetic Chronic Connective Tissue Disorder
with No Approved Treatment Options



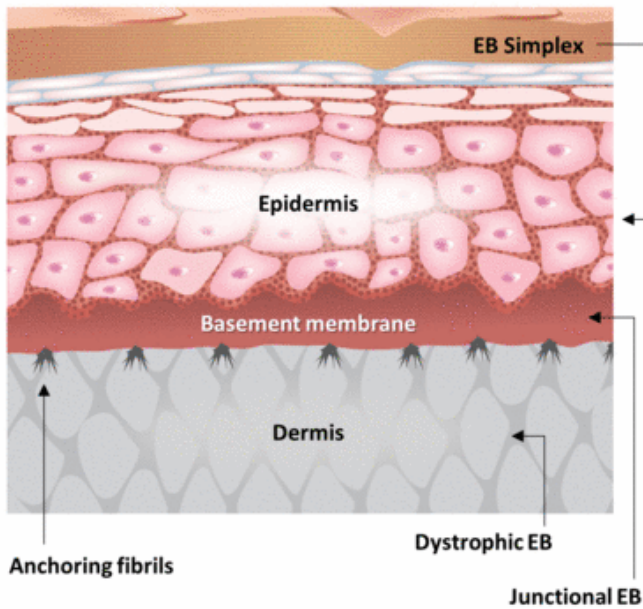
- Multiple genes cause disease which results in fragility of skin and can also affect internal organs
- Diagnosed from infancy to adulthood
- Severe blistering, open wounds and scarring in response to minor friction to the skin
- Disfiguring, excruciatingly painful, and can be fatal
- Palliative treatment (standard of care) ~\$10-15k/month (excluding hospitalizations)
- 30,000-40,000+ patients **diagnosed** in major markets¹

Three Major EB Subtypes

Three Major EB Subtypes Differ By Physical Manifestations, Genetic Makeup, and Prognosis

Skin structure

Sites of primary blister formation



EB subtypes

| Subtypes | Symptoms | Frequency | Mortality risk |
|-------------------|---|-----------|----------------|
| Junctional | <ul style="list-style-type: none"> External blistering Internal blistering (oral tract, internal organs) Severe complications can become fatal early in life | ~5% | |
| Dystrophic | <ul style="list-style-type: none"> External blistering Narrowing of esophagus Higher risk of aggressive skin cancer Associated with mortality | ~20% | |
| Simplex | <ul style="list-style-type: none"> Localized and generalized external blistering | ~75% | |

Zorblisa™ Overview

Patented High Concentration Allantoin with Breakthrough Therapy Designation

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

| | |
|-----------------------------|--|
| Active ingredient | <ul style="list-style-type: none">Allantoin |
| RoA | <ul style="list-style-type: none">Proprietary topical cream containing 6% allantoin, applied to entire body once daily |
| Proposed Indication | <ul style="list-style-type: none">All major EB subtypes (Simplex, Dystrophic and Junctional) |
| Phase of development | <ul style="list-style-type: none">Phase 3 ongoing (first to enter Phase 3 for EB)First to show significant benefit in wound closure in EB clinical studies |
| Proposed MOA* | <ul style="list-style-type: none">Impacts inflammatory response and promotes formation of epithelial and granulation tissueDirect bactericidal action demonstrated in vitroLoosens protein bridges (desmosomes) that hold together hyperkeratinized cells (e.g. in calluses) |
| Formulation | <ul style="list-style-type: none">Patented high concentration formulationLong-term stability of active ingredient and dose-related penetration of active ingredient in human dermal tissue |



*Margraf and Covey 1977; Meixell and Mecca 1966; Settle 1969; Meixell and Mecca 1966; Flesch 1958, Fisher 1981; Cajkovic et al., 1992, Medda 1976

Zorblisa Regulatory Pathway

Breakthrough Therapy Designation in US

US and EU Regulatory Pathways are Advanced and Well Defined

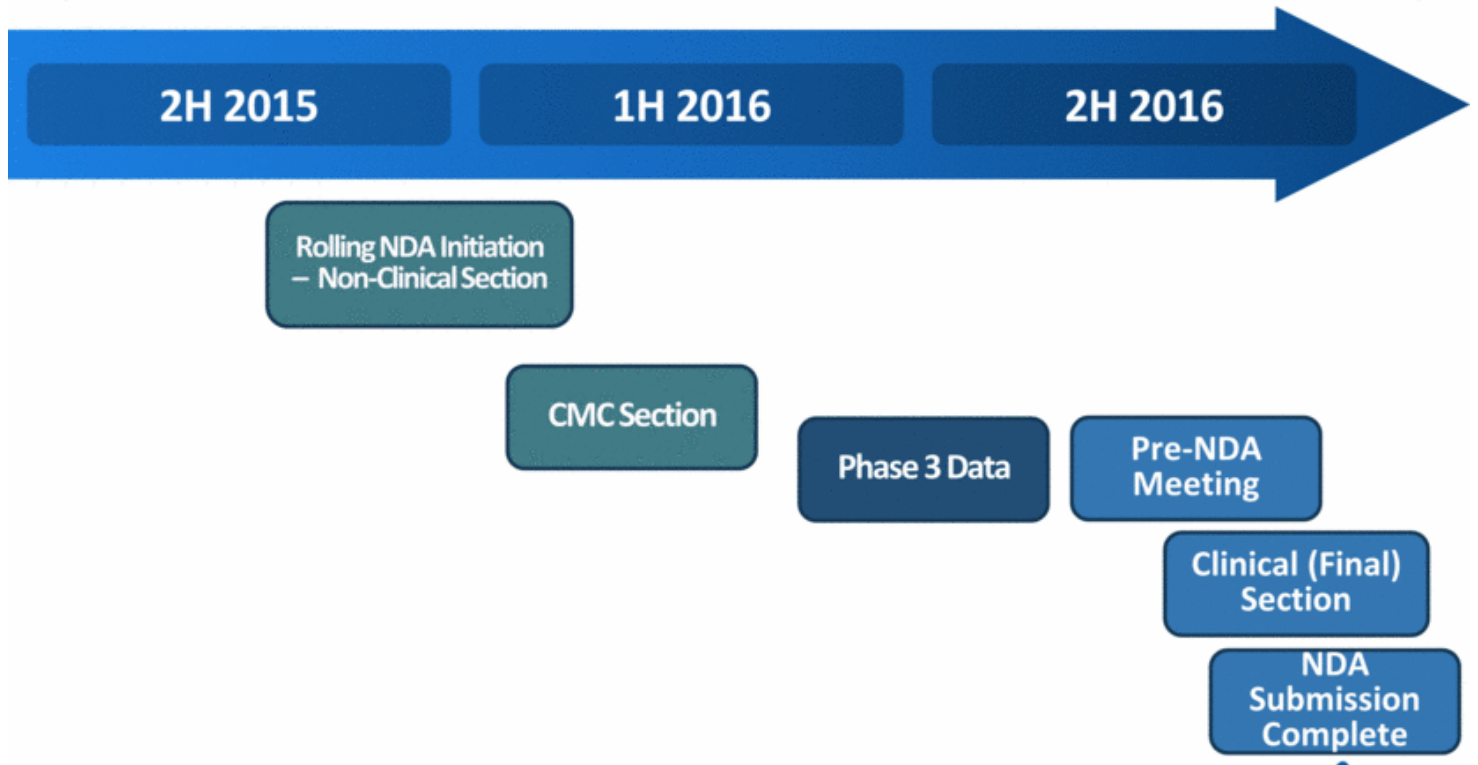
- Breakthrough Therapy Designation (BTD) based on Phase 2 POC
- Orphan drug designation
- FDA agreement on rolling NDA submission

- Orphan drug designation
- Approved Pediatric Investigation Plan (PIP)
- Defined registration pathway

ROW regulatory path to be based on EMA and FDA submissions

Zorblisa Rolling NDA Expected Timeline

U.S. FDA Agreed to Rolling NDA Submission; Expected to Commence in 4Q15



Scioderm Successfully Completed 48-Patient Phase 2b Study for Zorblisa (SD-003)

48 EB patients (age \geq 6 months)* - 1:1:1 Randomization - Daily Topical Application

Zorblisa 6%

Zorblisa 3%

Placebo

Open-Label Zorblisa (6%)

3-Month Double-Blind Treatment Period
Assessments: 0, 14, 30, 60, 90 Days

Optional Extension (SD-004)

Primary Efficacy Endpoint:

Target Wound Healing at Month 1

- Baseline wound: Chronic (\geq 21 days), size 5-50 cm²

Secondary Endpoints:

Change in Body Surface Area (BSA) of lesions and blisters; itching; pain

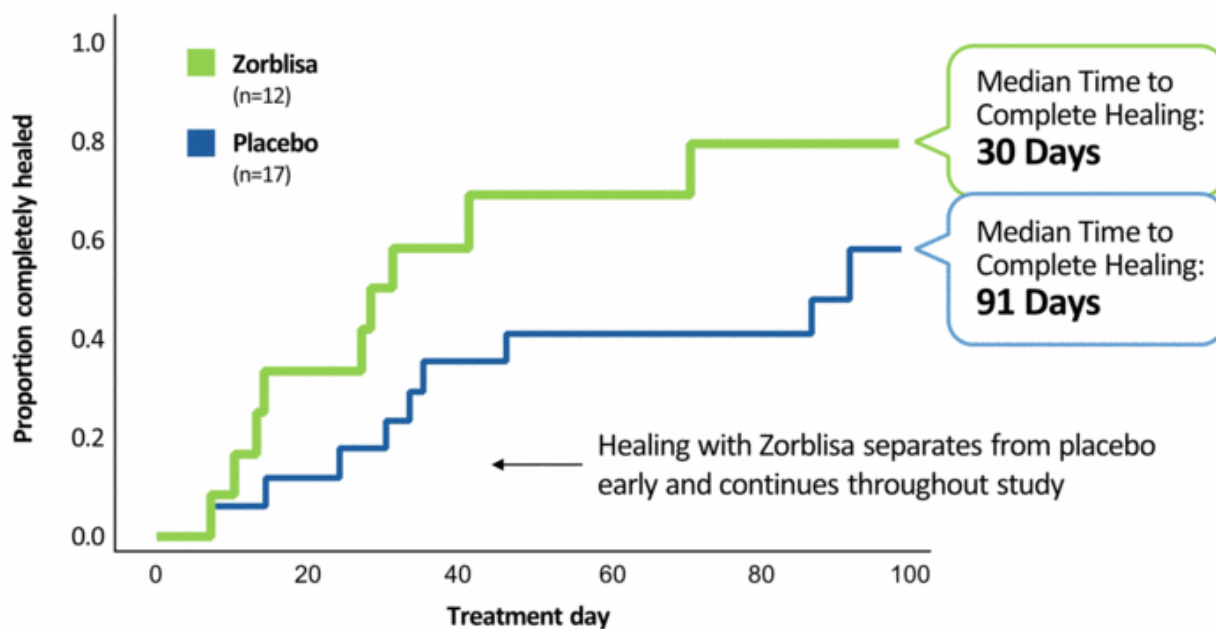
**Initial Disease Severity: Mean target lesion size (cm²) 14.0 (range 5-39); mean lesional BSA: 19.4% (range 0.4-48%); mean wound age (days): 182 (range 21-1,639)
EB Subtypes enrolled: Simplex (n=11), Recessive Dystrophic (n=29), and Junctional (n=8).*

Zorblisa Phase 2b Results

Evaluable population

67% REDUCTION in Median Time to Complete Wound Closure

Proportion with Complete Target Wound Closure Over 3 Months



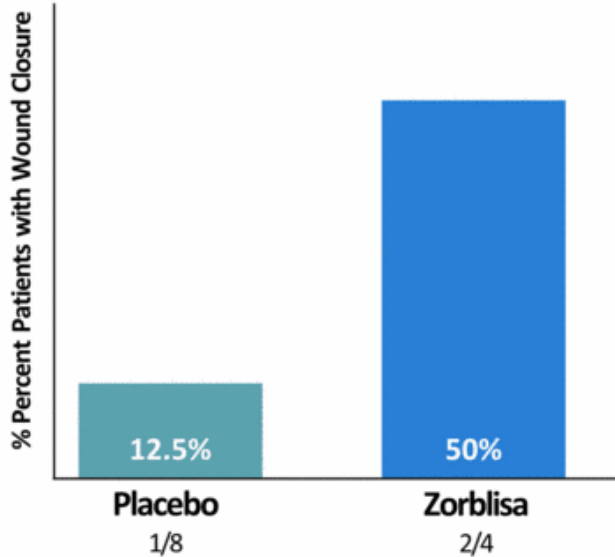
Zorblisa Phase 2b Results - Subgroup Analysis

Informed Phase 3 Primary Endpoint and Further Supported FDA Agreement on Rolling NDA

**Greatest Separation between Zorblisa and Placebo
at Month 2 in Subjects with Baseline Wounds $\geq 10 \text{ cm}^2$**

Complete Wound Closure

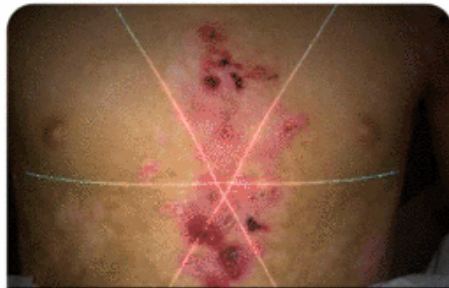
Month 2 Evaluable Population, Baseline Size $>10 \text{ cm}^2$



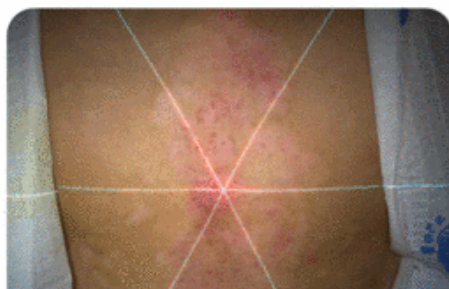
- Phase 3 primary endpoint: Target wound closure at Month 2
- Low rate of healing in placebo arm observed at Month 2 in wounds $\geq 10 \text{ cm}^2$
- Enhances ability to demonstrate treatment effect
- Phase 3 baseline target wound size: $\geq 10 \text{ cm}^2$
- Phase 3 study supported by US and EU regulatory authorities

Zorblisa Phase 2b Individual Patient Data

8 yr. old female with EB
(Recessive Dystrophic)



Baseline



2-Months Post-Treatment

3 yr. old male with EB
(Simplex)



Baseline



2-Weeks Post-Treatment

Zorblisa Phase 2b Safety Summary

- Treatment-emergent adverse events (TEAE) similar across treatment groups, including placebo
- No deaths and no severe TEAEs
- No serious adverse events reported in Zorblisa 6% group

Zorblisa Single Phase 3 Study Underway (SD-005)

Study Design Supported by Both FDA and EMA

Phase 3 Initiated in 2Q15 and Currently Already ~30% Enrolled

Zorblisa 6%

130 EB patients (age \geq 1 month)
1:1 Randomization - Daily Topical Application

Placebo

3-Month Double-Blind Treatment Period
Assessments: 0, 14, 30, 60, 90 Days

Open-Label Zorblisa (6%)

Optional Extension (SD-006)

Primary Efficacy Endpoint: Target Wound Healing at Month 2

- US and EU regulatory authorities agreed to target wound healing as primary endpoint
- Baseline wound: Chronic (\geq 21 days), size \geq 10 cm²

Secondary Endpoints

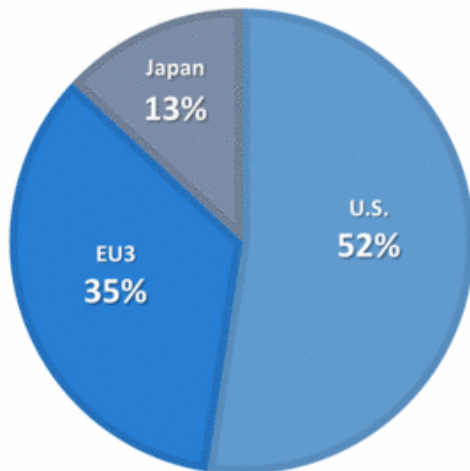
- Time to target wound closure; Change in Body Surface Area (BSA) of lesions and blisters; itching; pain

Potential \$1B+ Global EB Commercial Opportunity for Zorblisa

Significant Global Commercial Opportunity Supported by Profound Unmet Clinical Need, Strong Stakeholder Support and High Orphan Prevalence

Diagnosed EB Patients by Geography

(US, EU3, Japan)



Significant Unmet Clinical Need

- No approved treatments, opportunity for first-in-class
- Compelling proof-of-concept in meaningful endpoints
- Studied in all EB subtypes

Strong Stakeholder Support

- Physicians indicate usage in 100% patients due to product profile and urgent need
- Payers indicate support for broad reimbursement if approved

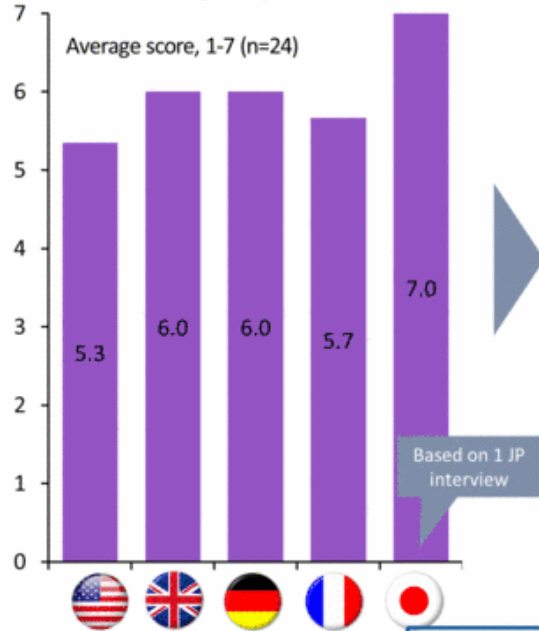
Large Commercial Opportunity

- 30,000 – 40,000 diagnosed in major markets
- Patients largely seen by neonatal wards, primary care physicians and dermatologists at major medical centers
- KOLs expect diagnosis rates to increase as EB is better characterized and awareness grows:
"...now we can identify the genetic lesion in 80% of the patients and we perform skin biopsy analysis in parallel..." – NE dermatologist

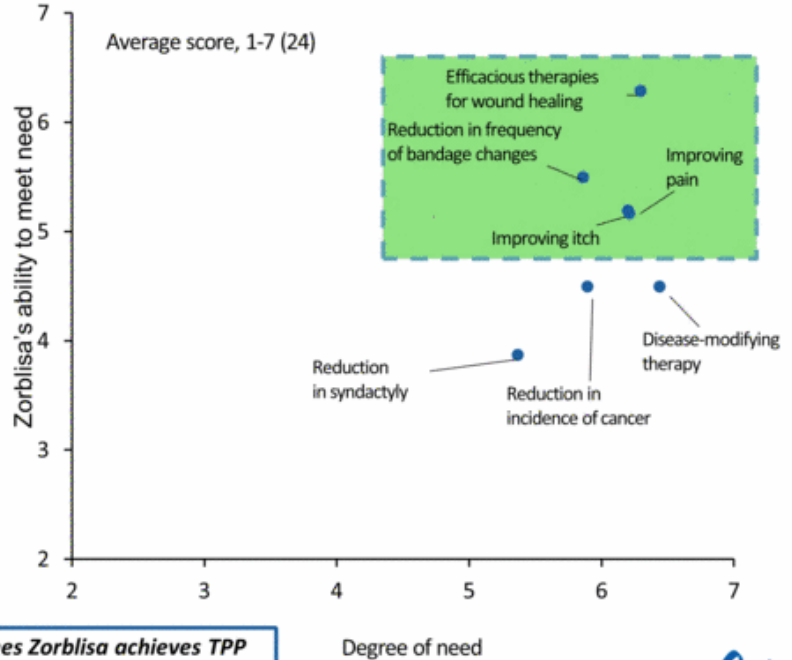
Positive KOL Feedback on Zorblisa Target Product Profile

Physicians Report They'd Use Zorblisa in 100% of EB Patients Based on High Perception of Phase 2 Data and Target Product Profile

Overall KOL perception of Zorblisa's TPP



Degree of unmet need versus Zorblisa's ability to meet needs



Source: Third Party interviews and analysis

NOTE: Assumes Zorblisa achieves TPP

Degree of need

Amicus Rare Disease Expertise

Amicus' Integrated Rare Disease Capabilities Will Accelerate the Development of Zorblisa for EB Patients

Clinical operations expertise in enrolling rare disease studies around the world

Experience partnering with rare disease patient communities through Patient Advocacy function

Senior leadership with track record of success in Fabry, Pompe, DMD, CF, and other rare diseases

Global regulatory expertise

Galafold global commercial organization will be leveraged to support potential Zorblisa launch

Acquisition Terms

Attractive Terms, Highly Structured Transaction

Transaction Highlights

| | |
|-------------------------|--|
| Consideration | <ul style="list-style-type: none">• \$229M upfront (\$125M cash/\$104M stock)¹• \$361M potential clinical and regulatory milestones (cash or stock)• \$257M potential commercial milestones (cash or stock)• Lesser of \$100M or 50% of sale price on potential future sale of Priority Review Voucher (may be awarded if Zorblisa approved) |
| Financial impact | <ul style="list-style-type: none">• Current cash, net of deal payment, debt financing, and added program development expenses expected to fund operating plan into 2017 |
| Financing | <ul style="list-style-type: none">• Funded using cash on hand and newly arranged \$50M debt from Redmile Group• Transaction not subject to financing contingency or shareholder approval |
| Timing | <ul style="list-style-type: none">• Closing expected in 3Q15 |

¹Subject to customary closing cash adjustments.

Significant Value Creation in Next 6-18 Months

Amicus Vision to Have One Product Launched (Galafold for Fabry), One Product Submitted to Regulators (Zorblisa for EB), and One Product in Phase 3 Study (ATB200 for Pompe) by YE16: Each with \$500M-\$1B+ Global Product Sales Potential

Pompe observational study initiation

Submission of non-clinical section of Zorblisa NDA

Pompe Phase 1/2 study initiation

Galafold NDA submission (Subpart H)

Galafold CHMP Opinion

Galafold EU launch

Submission of CMC section of Zorblisa NDA

Pompe Phase 1/2 interim data

Phase 3 Zorblisa data

Pompe Phase 3 study initiation

Submission of final (clinical) section of Zorblisa NDA

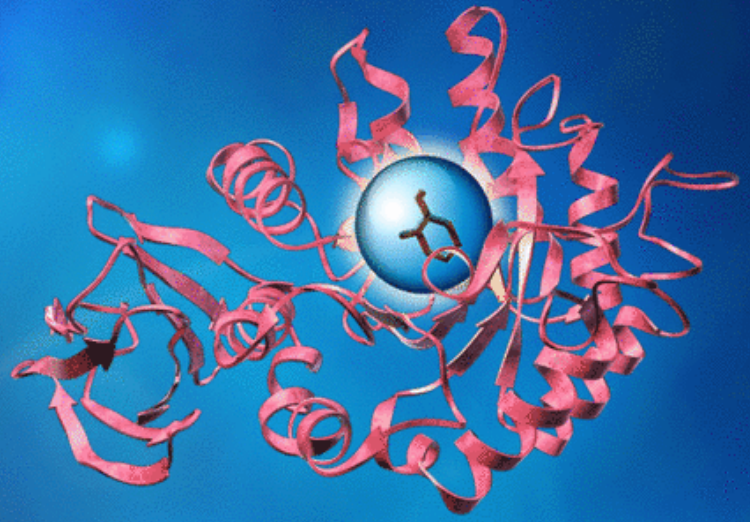
Galafold U.S. PDUFA Date

Zorblisa MAA submission

2H 2015

1H 2016

2H 2016



***Amicus Acquisition of Scioderm, Inc.
Overview***

August 31, 2015

*at the forefront of therapies
for rare and orphan diseases*