

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 9, 2016**

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 2.02. Results of Operations and Financial Condition.

On August 9, 2016, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing its financial results for the second quarter ended June 30, 2016. A copy of this press release is attached hereto as Exhibit 99.1. The Company will also host a conference call and webcast on August 9, 2016 to discuss its second quarter results of operations. A copy of the conference call presentation materials is also attached hereto as Exhibit 99.2.

In accordance with General Instruction B.2. of Form 8-K, the information in this Current Report on Form 8-K and the Exhibit shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing.

Item 9.01. Financial Statements and Exhibits.

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

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 9, 2016

By: /s/ Ellen S. Rosenberg
Ellen S. Rosenberg
General Counsel and Corporate Secretary

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EXHIBIT INDEX

Exhibit No.	Description
99.1	Press Release dated August 9, 2016
99.2	August 9, 2016 Conference Call Presentation Materials

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**Amicus Therapeutics Announces Second Quarter 2016
Financial Results and Corporate Updates**

Significant Progress with EU Galafold Launch and Regulatory Submissions

Enrollment in Pompe Phase 1/2 Clinical Study on Track with Initial Data Expected in 2H16

Elevation of “Time to Wound Closure” as Co-Primary Endpoint in Ongoing Phase 3 Study of SD-101 for Epidermolysis Bullosa

\$130 Million in Equity and Debt Proceeds Since March 31

CRANBURY, NJ, August 9, 2016 — Amicus Therapeutics (Nasdaq: FOLD), a global biotechnology company at the forefront of therapies for rare and orphan diseases, today announced financial results for the second quarter ended June 30, 2016. The Company also provided program updates and reiterated full-year 2016 net cash spend guidance.

“At Amicus Therapeutics our vision is bold — to build a leading global biotechnology company delivering meaningful benefits to people who are living with rare and devastating diseases,” stated John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. “To execute toward our vision, we are sharply focused on five key strategic priorities for the balance of 2016: 1) a successful Galafold launch in the EU; 2) multiple regulatory submissions for migalastat in the US, Japan and other global territories; 3) the advancement of our clinical programs in Pompe and epidermolysis bullosa, 4) a strengthened balance sheet; and 5) the expansion of our pipeline including a new biologics program for CDKL5. Today at Amicus we have never been in a stronger position with one of the best portfolios of potential first- and/or best-in-class medicines for rare and orphan diseases.”

Second Quarter 2016 Financial Results

- Cash, cash equivalents, and marketable securities totaled \$214.2 million at June 30, 2016 compared to \$165.9 million at March 31, 2016.
- Total operating expenses in the second quarter of 2016 increased to \$48.5 million compared to \$26.9 million for the second quarter 2015 primarily due to increases in commercial costs for the Fabry monotherapy program and the addition of the Phase 3 SD-101 program for epidermolysis bullosa (EB).
- Net loss was \$51.1 million, or \$0.40 per share in the second quarter of 2016, compared to a net loss of \$27.1 million, or \$0.27 per share, for the second quarter of 2015.

2016 Financial Guidance

Cash, cash equivalents, and marketable securities totaled \$214.2 million at June 30, 2016. The Company’s balance sheet was strengthened during the second quarter of 2016 with \$57.8 million in net proceeds under the existing at-the-marketing (ATM) financing facility and an additional \$30 million in debt under an existing debt facility. Following the second quarter close, the Company raised an additional \$39.3 million in net proceeds through the ATM facility and has raised the full \$100M allotted for the ATM facility. The Company expects to remain within the original 2016 net cash spend guidance of between \$135 million and \$155 million.

Program Highlights

Migalastat for Fabry Disease

Migalastat is an oral precision medicine intended to treat Fabry disease in patients who have amenable genetic mutations. As previously announced, the European Commission has granted full approval for migalastat, under the trade name Galafold™, as a first line therapy for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease (alpha-galactosidase A deficiency) and who have an amenable mutation.

International Launch and Expanded Access Programs (EAP):

-
- 21 patients (naïve and ERT-switch) are being treated with Galafold as of July 31, 2016 on a commercial basis in Germany, as well as through reimbursed expanded access programs (EAPs) including the temporary use authorization (ATU) in France.
 - Reimbursed EAPs are now available in 2 countries.
 - Reimbursement dossiers submitted and pricing discussions are now underway in multiple EU member states.

Regulatory Updates:

- Japanese new drug application (J-NDA) submission has been accelerated to the first half of 2017. As previously announced, Amicus has received written correspondence following a meeting with the PMDA. The J-NDA will be based upon data from completed clinical studies of migalastat without having to do another study.
- Discussions with the U.S. Food and Drug Administration (FDA) have been initiated and the Company expects to provide a U.S. regulatory update in the third quarter of 2016.

- Regulatory submissions have been completed in 4 additional territories outside the EU.

Anticipated Upcoming Fabry Disease Program Milestones:

- EU commercial reimbursement and EAP in additional territories
- Publication of Phase 3 Clinical Study 011 data
- Regulatory submissions in additional territories that accept the MAA as basis for submission
- U.S. regulatory update
- Fabry ERT cell line development and program update
- Japanese regulatory submission (J-NDA) on accelerated timeline

ATB200/AT2221 for Pompe Disease

Patient dosing is underway in a global clinical study (ATB200-02) to investigate ATB200/AT2221, a novel treatment paradigm that consists of ATB200, a uniquely engineered recombinant human acid alpha-glucosidase (rhGAA) enzyme with an optimized carbohydrate structure to enhance uptake, co-administered with AT2221, a pharmacological chaperone to improve activity and stability. The study is enrolling 3 cohorts of patients, including ambulatory ERT-switch patients (Cohort 1), non-ambulatory ERT-switch patients (Cohort 2), and ERT-naïve patients (Cohort 3).

Anticipated Upcoming Pompe Disease Program Milestones:

- Data from clinical study ATB200-02 in initial ambulatory ERT-switch patients (Cohort 1) on track by year-end 2016
- Additional ATB200-02 study data in naïve and non-ambulatory patients (Cohorts 2-3), as well as initial extension-phase data on ambulatory ERT-switch patients, throughout 1H17

SD-101 for Epidermolysis Bullosa (EB)

SD-101 is a novel, late-stage, proprietary topical treatment and potential first-to-market therapy for EB. SD-101 is currently being investigated in a registration-directed Phase 3 study (ESSENCE, also known as SD-005) to support global regulatory submissions. All (100%) patients completing the primary treatment period of the Phase 3 study have elected to continue in the open-label extension study.

SD-101 was granted FDA Breakthrough Therapy designation in 2013 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. The Company began a rolling NDA submission for SD-101 in the fourth quarter of 2015.

Amicus also held a series of discussions with the Dermatology Division of the U.S. FDA regarding proposed revisions to the statistical analysis plan (SAP) while remaining blinded to the Phase 3 ESSENCE study. Based on conversations with FDA and written communication received from the agency, the FDA has agreed to the Company's proposed revisions. Importantly, the FDA agreed that "Time to Target Wound Closure" may be elevated from a secondary endpoint to a co-primary endpoint (together with the previously specified primary endpoint "Proportion of Patients with Target Wound Closure"). Based on this feedback, the Company believes that study success could potentially be based on achievement of

one or both co-primary endpoints, assuming appropriate analytical methodology, and that the overall likelihood of study success has been improved.

Anticipated EB Program Milestones:

- Top-line data from the Phase 3 ESSENCE study of SD-101 (4Q16 or 1H17)

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, August 9, 2016 at 8:30 a.m. ET to discuss second quarter 2016 financial results and corporate updates. 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 slides can also be accessed via the Investors section of the Amicus Therapeutics corporate web site at <http://ir.amicusrx.com/events.cfm>, 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 11:30 a.m. ET today. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); participant code 53315677.

About Galafold™ and Amenable Mutations

Galafold™ (migalastat) is a first-in-class chaperone therapy approved in the EU as a monotherapy for Fabry disease in patients with amenable mutations. Galafold works by stabilizing the body's own dysfunctional enzyme, so it can clear the accumulation of disease substrate in patients who have amenable mutations. A proprietary *in vitro* assay (Galafold Amenability Assay) was used to classify more than 800 known GLA mutations as "amenable" or "not amenable" to treatment with Galafold. The current label includes all 269 GLA mutations that have been identified and determined to be amenable based on the Galafold Amenability Assay, which represent between 35% and 50% of the currently diagnosed Fabry population.

Healthcare providers in the EU may access the website www.galafoldamenabilitytable.com to quickly and accurately identify which mutations are categorized as "amenable" or "not amenable" to Galafold. Amicus expects to submit updates to the label as additional GLA mutations are identified and

tested in the Galafold Amenity Assay.

Important Safety Information

Treatment with GALAFOLD should be initiated and supervised by specialists experienced in the diagnosis and treatment of Fabry disease. GALAFOLD is not recommended for use in patients with a nonamenable mutation.

- GALAFOLD is not intended for concomitant use with enzyme replacement therapy.
- GALAFOLD is not recommended for use in patients with Fabry disease who have severe renal impairment (<30 mL/min/1.73 m²). The safety and efficacy of GALAFOLD in children 0–15 years of age have not yet been established.
- No dosage adjustments are required in patients with hepatic impairment or in the elderly population.
- There is very limited experience with the use of this medicine in pregnant women. If you are pregnant, think you may be pregnant, or are planning to have a baby, do not take this medicine until you have checked with your doctor, pharmacist, or nurse.
- While taking GALAFOLD, effective birth control should be used. It is not known whether GALAFOLD is excreted in human milk.
- Contraindications to GALAFOLD include hypersensitivity to the active substance or to any of the excipients listed in the PRESCRIBING INFORMATION.
- It is advised to periodically monitor renal function, echocardiographic parameters and biochemical markers (every 6 months) in patients initiated on GALAFOLD or switched to GALAFOLD.
- OVERDOSE: General medical care is recommended in the case of GALAFOLD overdose.
- The most common adverse reaction reported was headache, which was experienced by approximately 10% of patients who received GALAFOLD. For a complete list of adverse reactions, please review the SUMMARY OF PRODUCT CHARACTERISTICS.
- Call your doctor for medical advice about side effects.

For further important safety information for Galafold, including posology and method of administration, special warnings, drug interactions and adverse drug reactions, please see the European SmPC for Galafold available from the EMA website at www.ema.europa.eu.

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq: FOLD) is a 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.

(1)Company filings and Amicus estimates

Forward-Looking Statements

This press release contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, financing plans, and the projected cash position for the Company. 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 goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular 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 our business, including, without limitation: the potential that results of clinical or preclinical 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, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe or our other product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; and the potential that we will need additional funding to complete all of our 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, 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 we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

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Table 1

Amicus Therapeutics, Inc.
Consolidated Statements of Operations
(Unaudited)
(In thousands, except share and per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
Operating Expenses:				
Research and development	\$ 18,281	\$ 17,234	\$ 41,706	\$ 33,347
General and administrative	19,300	8,278	35,001	14,705
Changes in fair value of contingent consideration payable	10,186	100	13,338	1,100
Restructuring charges	8	26	58	36
Loss on extinguishment of debt	—	952	—	952
Depreciation	767	353	1,440	861
Total operating expenses	48,542	26,943	91,543	51,001
Loss from operations	(48,542)	(26,943)	(91,543)	(51,001)
Other income (expenses):				
Interest income	331	158	638	329
Interest expense	(1,055)	(338)	(2,000)	(710)
Other expense	(2,237)	(10)	(2,289)	(39)
Loss before income tax benefit	(51,503)	(27,133)	(95,194)	(51,421)
Income tax benefit	453	—	453	—
Net loss	(51,050)	(27,133)	(94,741)	(51,421)
Net loss – basic and diluted	\$ (0.40)	\$ (0.27)	\$ (0.75)	\$ (0.53)
Weighted-average common shares outstanding – basic and diluted	129,122,175	99,994,125	127,160,943	97,888,573

Table 2

Amicus Therapeutics, Inc.
Consolidated Balance Sheets
(Unaudited)
(in thousands, except share and per share amounts)

	June 30, 2016	December 31, 2015
Assets:		
Current assets:		
Cash and cash equivalents	\$ 63,656	\$ 69,485
Investments in marketable securities	150,494	144,548
Inventories	194	—
Prepaid expenses and other current assets	3,330	2,568
Total current assets	217,674	216,601
Property and equipment, less accumulated depreciation of \$14,284 and \$13,353 at June 30, 2016 and December 31, 2015, respectively	10,178	6,178
In-process research & development	486,700	486,700
Goodwill	197,797	197,797
Other non-current assets	1,657	1,108
Total Assets	\$ 914,006	\$ 908,384
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and accrued expenses	\$ 23,828	\$ 32,216
Contingent consideration payable, current portion	56,000	41,400
Other current liabilities	631	—
Total current liabilities	80,459	73,616
Deferred reimbursements	35,756	35,756
Due to related party	43,443	41,601
Unsecured notes payable	21,851	—
Contingent consideration payable, less current portion	220,300	232,677
Deferred tax liability	176,219	176,219
Other non-current liabilities	1,735	681
Commitments and contingencies		

Stockholders' equity:

Common stock, \$.01 par value, 250,000,000 authorized, 134,408,526 shares issued and outstanding at June 30, 2016, 250,000,000 shares authorized, 125,027,034 shares issued and outstanding at December 31, 2015	1,399	1,306
Additional paid-in capital	990,032	917,454
Accumulated other comprehensive loss:		
Foreign currency translation adjustment, less tax benefit of \$453 at June 30, 2016	842	—
Unrealized gain/ (loss) on available-for securities	201	(115)
Warrants	16,076	8,755
Accumulated deficit	(674,307)	(579,566)
Total stockholders' equity	<u>334,243</u>	<u>347,834</u>
Total Liabilities and Stockholders' Equity	<u>\$ 914,006</u>	<u>\$ 908,384</u>

FOLD-G



2Q16 Financial Results and Program Updates

August 9, 2016

2016: Significant Progress with Key Strategic Priorities

We Remain Sharply Focused on 5 Strategic Priorities for 2016 as We Continue to Build a Leading Global Biotechnology Company Focused on Rare and Devastating Diseases

2016 Strategic Priorities Achieved

- ✓ EU Approval of Galafold
- ✓ International commercial launch of Galafold
- ✓ Accelerated regulatory path for Galafold in Japan
- ✓ Dosing of Pompe patients with ATB200/AT2221
- ✓ Expansion of preclinical pipeline with CDKL5 program
- ✓ Completion of \$130M of financing

2016 Strategic Priorities for 2H16

- 1) Launching Galafold internationally
- 2) Pursuing Galafold regulatory approvals (U.S., Japan, and Rest-of-World)
- 3) Advancing clinical programs (Epidermolysis Bullosa (EB) and Pompe)
- 4) Maintaining a strong balance sheet
- 5) Building the pipeline



Galafold™ (Migalastat) Precision Medicine for Fabry Disease

International Launch Underway

European Commission Granted Full Approval for Galafold

Galafold Indicated for Long-Term Treatment of Adults and Adolescents Aged ≥ 16 years with a Confirmed Diagnosis of Fabry Disease and Who have an Amenable Mutation



Galafold™ (migalastat)

AMENABILITY TABLE

Search GLA Mutations

You can use this search tool to find out whether a specific GLA mutation has been classified as amenable to treatment with GALAFOLD™ according to the approved SmPC.

GALAFOLD™ is indicated for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease (a galactosidase α deficiency) and who have an amenable mutation.

Female patients have two GLA genes on two different chromosomes. The patient is considered amenable if the GLA mutations on either chromosome are amenable. Please utilize the appropriate search function to determine if the mutation or mutations on each chromosome are amenable.

PATIENT HAS SINGLE MUTATION

PATIENT HAS MULTIPLE MUTATIONS

Enter either a nucleotide or amino acid change.

For Nucleotide Change
Please use format c.AA-B or c.AAG for nucleotide sequence changes, where 'c' is optional. If indicates a number: A and B are letters. Examples: L123C or L23C.

For Amino Acid Change
Please use format p.AAG for protein sequence changes, where 'p' is optional. If indicates a number: A and B are letters. Example: gA2E

Download Amenable Reference Table
See the SmPC for full prescribing information

LAST Updated: 26 May 2016

Amicus Therapeutics

MIGALASTAT (MIGALASTAT) 123 mg

Galafold™

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“The evaluation of EMA’s Committee for Medicinal Products for Human Use (CHMP) was based on the results of two phase III clinical trials in about 110 patients with Fabry disease who had a genetic mutation which responds to migalastat. Galafold demonstrated its efficacy compared to placebo (a dummy treatment) and to ERT in a long-term comparative study.

- EMA Press Release

International Launch Update

EU Market Represents 34% of FY15 ERT Global Sales (\$1.2B)

GERMANY

ERT-treated patients : ~500 patients
~50% of diagnosed patients untreated
Galafold launched – initial patients on treatment

FRANCE

ERT-treated patients : ~375 patients
Multiple patients treated under ATU

UNITED KINGDOM

ERT-treated patients: ~450
Highly Specialised Technology (HST)



EU Launch Update

Successful Early Days of EU Launch with Naïve and Switch Patients on Galafold – Focusing on Patient Access and Country-by-Country Reimbursement Processes



LAUNCH IN GERMANY = IMPORTANT INDICATOR

- First Galafold Rx within 24 hours of EC approval
- Patient support program
- Experienced, high quality team
- Pricing dossier submitted

21

patients (switch & naïve) on reimbursed Galafold WW (7/31/16)

12

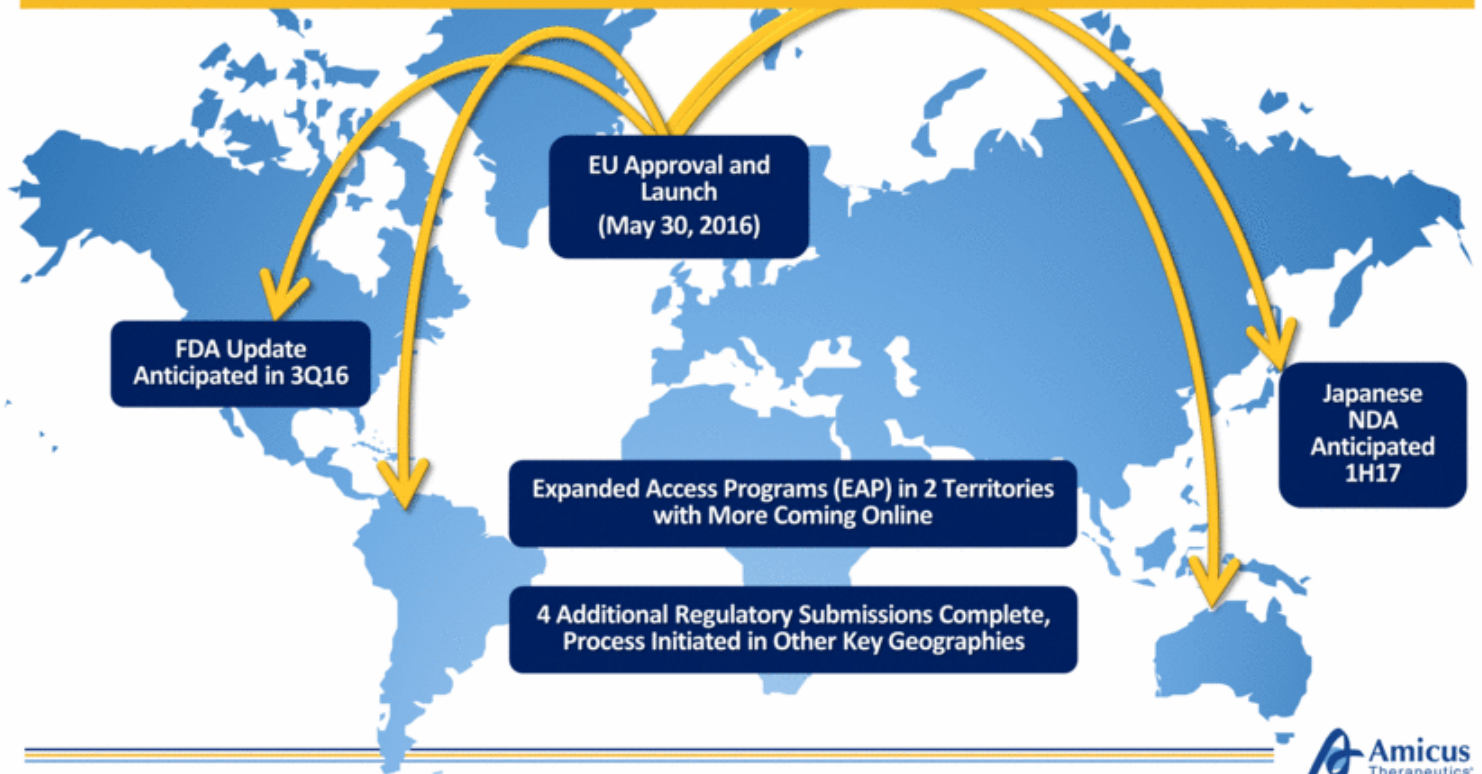
countries with active pricing discussions

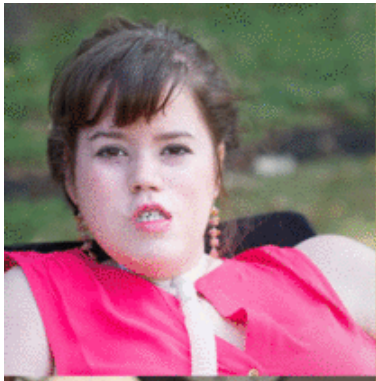
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countries with reimbursement (commercial and EAP)

Global Regulatory Strategy

Prioritizing Global Regulatory Submissions in Key Markets (US and Japan) with Additional Submissions Completed or Planned Based on EU Approval (MAA)





ATB200 Novel ERT for Pompe Disease

A Proprietary, Clinical-Stage Biologics Program

Pompe Clinical Study ATB200-02 Data Cascade

A Cascade of Data Points from 4Q16 through 2017 Offer Clear Parameters to Define Success and Differentiate ATB200/AT2221

Pompe Data Cascade 4Q16 Through 2017

Data in initial ambulatory ERT-switch patients (Cohort 1)

Additional data & initial extension data in Cohort 1

Data in ERT-naïve patients (Cohort 2)

Data in non-ambulatory ERT-switch patients (Cohort 3)

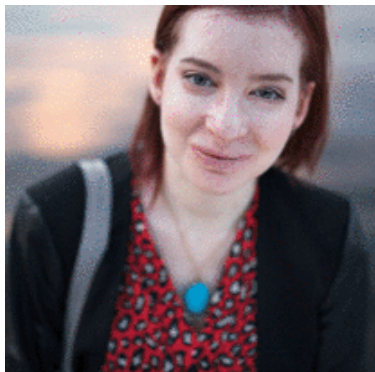
Additional extension study data (all Cohorts)

18-WEEK DATA

- Safety / tolerability
- Pharmacokinetics (PK)
- Biomarkers
- Immunogenicity

EXTENSION DATA

- Motor/pulmonary function



SD-101 for Epidermolysis Bullosa (EB)

Poised to deliver pivotal data for a
devastating rare disease

EB Program Update - Phase 3 ESSENCE Study (SD-005)

Following Recent Meeting with FDA, Amicus has Elevated Time to Wound Closure From Secondary to Co-Primary Endpoint. We Believe This Change Improves the Overall Likelihood of Study Success while ESSENCE Study Remains Blinded.



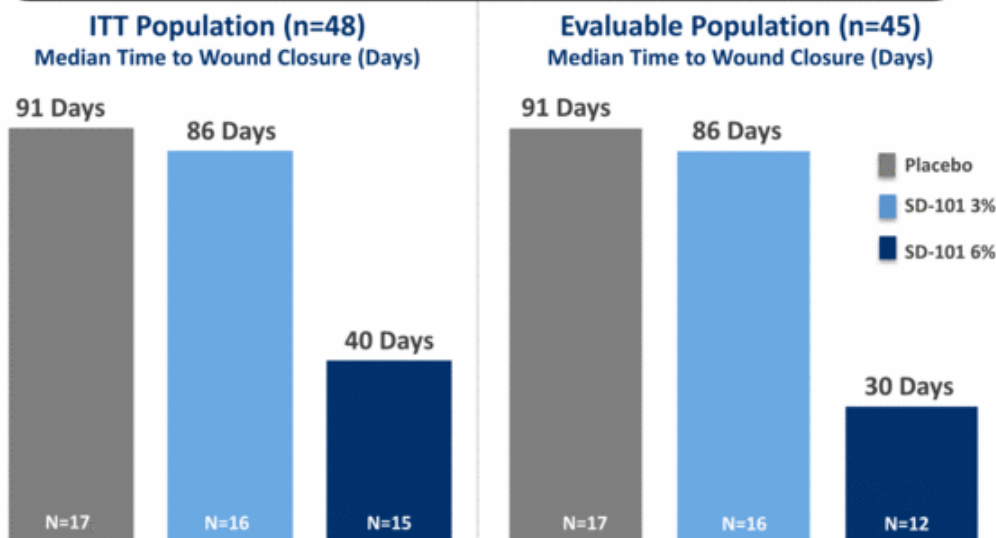
PHASE 3 ESSENCE STUDY STATUS

- >50% of target enrollment achieved
- 100% conversion to extension study (SD-006)
- Top-line Phase 3 data anticipated 4Q16/1H17

Elevation of Time to Wound Closure as Co-primary Endpoint

FDA 2006 Guidance Document¹ States Time to Wound Closure is an Acceptable Primary Efficacy Endpoint

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 addition of time to wound closure increases probability of study success

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

Phase 3 ESSENCE Study Design (SD-005)

Study Success Potentially Based on Achievement of One or Both Co-Primary Endpoints

3-Month, Double-Blind Treatment Period

SD-101 6%

~150 EB patients (age \geq 1 month)

Baseline wound: Chronic (\geq 21 days), size \geq 10 cm²

Placebo

Optional Extension (SD-006)

Open-Label SD-101 6%

100% Participation in
Extension Study
(August 1, 2016)

Average Baseline Target
Wound Size in Phase 3
Population: **~20 cm²**
(August 1, 2016)

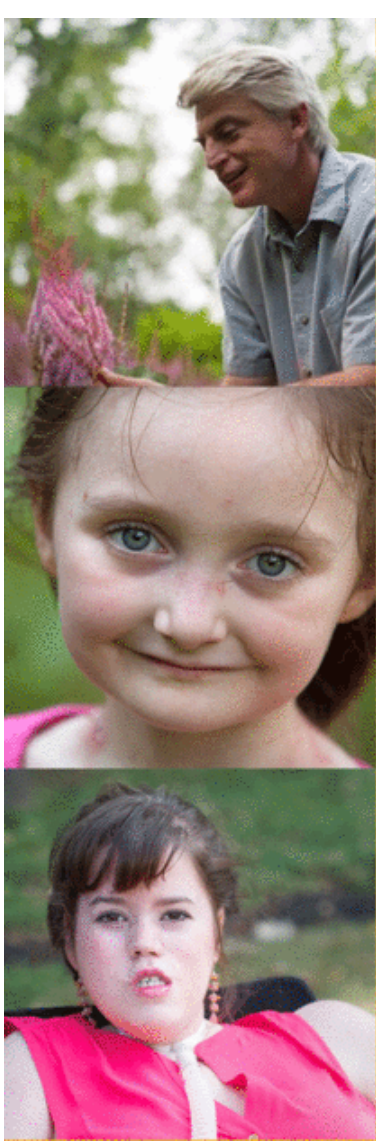
Co-Primary Endpoints

- Complete closure of target wound (previously specified primary endpoint)
- Time to target wound closure (elevated from secondary to co-primary)

Secondary Endpoints Include:

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

Covariates include age of patient and size of wound at baseline



Financial Summary & Milestones

Strong Balance Sheet to Invest in Rare Disease Pipeline

Strong Balance Sheet

Balance Sheet Strengthened with \$130M in Equity and Debt Proceeds Since March 31 with Cash Runway into 2H17

Financial Position	June 30, 2016
Cash:	\$214.2M
Debt	\$80.0M
FY16 Net Cash Spend Guidance:	\$135-\$155M (maintained)
Cash Runway	Into 2H17
Full Allotment Raised in ATM (average price per share: \$6.67)	\$100M (\$61.7M in 2Q; \$39.3M in 3Q)
Capitalization	
Shares Outstanding	134,408,526

2Q16 Select Financial Results

(\$000s)	June 30, 2016	June 30, 2015
R&D Expense	18,281	17,234
G&A Expense	19,300	8,278
Net Loss	(51,050)	(27,133)
Net Loss Per Share	(0.40)	(0.27)

Key Drivers of Value

3 Novel Product Candidates Each with \$500M to \$1B+ Market Potential

Fabry

- Galafold Precision Medicine (Small Molecule)
- EU Full Approval
- Launched in Germany (May 30, 2016)
- U.S. regulatory update anticipate 3Q16

Epidermolysis Bullosa (EB)

- Phase 3 Novel Topical Cream (SD-101)
- U.S. Breakthrough Therapy Designation
- Rolling NDA
- Phase 3 Data targeted in 4Q16/1H17

Pompe

- Novel ERT + Chaperone Treatment Paradigm
- Biologics Manufacturing
- Interim Data Anticipated in 4Q16

R&D Engine and Continued Business Development Activity

Thank You

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