

**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): **November 5, 2012**

**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))

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**Item 2.02. Results of Operations and Financial Condition.**

On November 5, 2012, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing its financial results for the quarter ended September 30, 2012. A copy of this press release is attached hereto as Exhibit 99.1. The Company will also host a conference call and webcast on November 5, 2012 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 Exhibits 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.

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**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: November 5, 2012

By: /s/ Peter M. Macaluso

Peter M. Macaluso

Secretary

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**EXHIBIT INDEX**

**Exhibit No.** **Description**

99.1 Press Release dated November 5, 2012

99.2 November 5, 2012 Conference Call Presentation Materials

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## Amicus Therapeutics Announces Third Quarter 2012 Financial Results & Corporate Updates

### *6-Month Results Expected 4Q12 and 12-Month Results Anticipated 1H13 from Phase 3 Fabry Monotherapy Study 011*

### *Additional Human Proof-of-Concept Demonstrated for Chaperone-Enzyme Replacement Therapy (ERT) Combination Platform*

CRANBURY, NJ, US, November 5, 2012 — Amicus Therapeutics (Nasdaq: FOLD), a biopharmaceutical company at the forefront of developing therapies for rare and orphan diseases, today announced financial results for the third quarter ended September 30, 2012. The Company also summarized recent and upcoming milestones and reiterated full-year 2012 operating expense guidance.

#### Key Highlights and Upcoming Milestones

- 6-month primary endpoint results expected 4Q12 and 12-month data anticipated 1H13 from first Phase 3 Fabry monotherapy study (Study 011)
- Achieved target enrollment in Second Phase 3 Fabry monotherapy study (Study 012)
- Announced updated results from Phase 2 extension study (Study 205) to evaluate investigational migalastat HCl monotherapy
- Completed enrollment in Phase 2 Study 013 to investigate migalastat HCl co-administered with ERT - updated results to be presented at American Society of Human Genetics (ASHG) on November 8, 2012
- Announced positive Pompe program updates - additional preliminary results for AT2220-ERT co-administration from Phase 2 Study 010 and *ex vivo* studies on ERT-related immunogenicity — Study 010 results from all 4 cohorts anticipated 4Q12

John F. Crowley, Chairman and Chief Executive Officer of Amicus stated, “The third quarter 2012 was excellent for Amicus. We continued to demonstrate our operational excellence in global clinical research in rare and orphan diseases through our advancement of multiple Phase 2 and Phase 3 studies in Fabry and Pompe disease. We are especially pleased with the recruitment success in our second Phase 3 Fabry Study 012. Finally, further clinical and preclinical proof-of-concept evaluating the direct combination of pharmacological chaperones with ERTs to both enhance ERT stability and reduce ERT immunogenicity will be a cornerstone of our pipeline and shareholder value going forward.”

#### 3Q12 Financial Results

The Company’s third quarter 2012 financial statements reflect updated revenue recognition accounting in conjunction with the expanded GSK collaboration (entered in July 2012). Payments received from GSK under the agreement are now being recorded in the “deferred reimbursements” account on the balance sheet. On September 30, 2012, the deferred reimbursements balance was \$27.2 million. This amount included \$4.5 million in cash reimbursed for shared global development costs of migalastat HCl during the third quarter 2012, and the \$22.7 million unrecognized balance of the upfront license payment and premium on equity. Prior to July 1, 2012, quarterly cash reimbursements were recognized as “research revenue” and the upfront payment was amortized each quarter in “collaboration revenue.”

For the three months ended September 30, 2011, Amicus reported total revenue of \$5.8 million, including \$4.1 million in research revenue and \$1.7 million in collaboration revenue. More information on the updated revenue recognition accounting, which does not impact cash, will be contained in the Company’s Form 10-Q for the quarter ended September 30, 2012.

Total operating expenses for the third quarter 2012 totaled \$16.9 million compared to \$18.9 million in the year-ago period on lower research and development expenses. Net loss attributable to common stockholders in the third quarter 2012 was \$16.3 million, or \$0.34 per share, compared to a net loss of \$9.8 million, or \$0.28 per share, in the year-ago period. The wider net loss versus the year-ago period is attributed to the change in revenue recognition under the expanded GSK collaboration.

#### Financial Guidance

Cash, cash equivalents, and marketable securities totaled \$106.2 million at September 30, 2012 compared to \$95.8 million at June 30, 2012 and \$55.7 million at December 31, 2011. The current cash position reflects an \$18.6 million equity investment from GSK related to the expanded collaboration and a \$3.5 million cash milestone payment received from GSK in the third quarter 2012. Amicus expects to end 2012 with at least \$90 million in cash, cash equivalents and marketable securities which is expected to fund its current operating plan beyond 2013. This projection includes quarterly reimbursement from GSK for shared development costs for migalastat HCl.

Amicus continues to expect full-year 2012 operating expenses within the higher end of the previously disclosed guidance range of \$37 million to \$43 million, net of anticipated cost sharing under the expanded Fabry disease collaboration with GSK. Amicus and GSK are funding 25% and 75% of the development costs, respectively, for migalastat HCl monotherapy and co-administration for full-year 2012. During the second half of 2012, Amicus and GSK will be responsible for 40% and 60% of the preclinical development costs, respectively, for the co-formulated chaperone-ERT product. Amicus will be responsible for all U.S. commercial activities for migalastat HCl upon approval, including pricing, marketing, patient access and reimbursement.

#### Program Updates

Amicus and GSK are co-developing all formulations of migalastat HCl under a global Fabry collaboration. Amicus has commercial rights to all Fabry products in the United States and GSK has commercial rights to all of these products in the rest of world. Outside the GSK collaboration, Amicus owns exclusive rights to the rest of its pipeline and applications of its platform technology.

### ***Migalastat HCl Monotherapy for Fabry Disease***

Migalastat HCl monotherapy is in Phase 3 development (Study 011 and Study 012) for Fabry patients with genetic mutations that are amenable to this chaperone monotherapy, as determined by a cell-based assay. Both studies are investigating oral migalastat HCl monotherapy 150 mg, dosed every-other-day (QOD).

Study 011 is a randomized, placebo-controlled study. During the fourth quarter 2012, Amicus and GSK expect to announce the primary endpoint analysis, based on interstitial capillary globotriaosylceramide (GL-3) as measured in kidney biopsy at 6 months. Results from the 6-month follow-up period in Study 011 are expected in the first half of 2013, which will include 12 months on migalastat HCl in the treatment group and 6 months on migalastat HCl in the placebo-crossover group.

Study 012 is the first clinical study to compare oral migalastat HCl to standard-of-care ERTs (Fabrazyme® and Replagal®). The open-label study randomized males and females with Fabry disease, who either to switch to migalastat HCl or remain on ERT. Amicus and GSK achieved target enrollment in Study 012 ahead of schedule. The primary outcome measure is renal function assessed by Glomerular Filtration Rate (GFR) at 18 months.

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Amicus and GSK also continue to evaluate patients who completed a previous Phase 2 study in an ongoing Phase 2 extension study (Study 205) of migalastat HCl. Updated results from Study 205 were presented at the American Society of Nephrology (ASN) Kidney Week 2012. A total of 17 patients completed Study 205 after a median treatment duration of 5.2 years (4.7 to 6.4 years) with migalastat HCl in the Phase 2 and Phase 2 extension studies. 16 of these patients are currently enrolled in a separate open-label extension study (MGM116041).

### ***Migalastat HCl in Combination with ERT for Fabry Disease***

During the third quarter 2012, Amicus and GSK completed enrollment in an open-label Phase 2 study (Study 013) to investigate the effects of a single oral dose of migalastat HCl (150 mg or 450 mg) co-administered prior to ERT (Fabrazyme or Replagal) in 23 males with Fabry disease. Positive preliminary results were announced in the first quarter 2012 and updated data will be presented at the American Society of Human Genetics (ASHG). Amicus and GSK, along with GSK's collaborator JCR Pharmaceutical Co. Ltd, also continue to advance preclinical studies of migalastat HCl co-formulated with JCR Pharmaceutical Co. Ltd's proprietary investigational ERT (JR-051, recombinant human alpha-Gal A enzyme).

### ***AT2220-ERT Co-Administration for Pompe Disease***

In October 2012, Amicus announced additional positive preliminary results from a Phase 2 open-label study (Study 010) to investigate four ascending dose cohorts of the pharmacological chaperone AT2220 co-administered with ERT (Myozyme® and Lumizyme®) for Pompe disease. In the first 3 dose cohorts, AT2220-ERT co-administration increased Pompe enzyme (recombinant GAA) activity. Study 010 is fully enrolled and results from Cohort 4 are anticipated by year-end. In parallel with Study 010, initial *ex vivo* studies completed using T cells from 50 healthy donor blood samples demonstrated that the addition of AT2220 may reduce the immunogenicity of Myozyme and Lumizyme. Results from these studies may help guide further investigation of the effects of AT2220 on immune response to ERT in future clinical studies.

### **Conference Call and Webcast**

Amicus Therapeutics will host a conference call and audio/visual webcast today, November 5, 2012 at 5:00 p.m. ET to review financial results and provide a corporate update. Interested participants and investors may access the conference call at 5:00 p.m. ET by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international).

An audio/visual webcast 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.

The slide presentation for today's conference call and webcast is also available in the Investors section of the Amicus Therapeutics corporate web site at <http://www.amicusrx.com>. 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 64424986.

### **About Amicus Therapeutics**

Amicus Therapeutics (Nasdaq:FOLD) is a biopharmaceutical company at the forefront of developing therapies for rare diseases. The Company is developing orally-administered, small molecule drugs called pharmacological chaperones, a novel, first-in-class approach to treating a broad range of human genetic diseases. Amicus' late-stage programs for lysosomal storage disorders include migalastat HCl monotherapy in Phase 3 for Fabry disease; migalastat HCl co-administered with enzyme replacement therapy (ERT) in Phase 2 for Fabry disease; and AT2220 co-administered with ERT in Phase 2 for Pompe disease.

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### **About Migalastat HCl for Fabry Disease**

Amicus and GSK are co-developing all formulations of migalastat HCl under a global Fabry collaboration. Amicus has commercial rights to all Fabry products in the United States and GSK has commercial rights to all of these products in the rest of world. As a monotherapy, migalastat HCl is designed to bind to and stabilize, or "chaperone" alpha-galactosidase A (alpha-Gal A) enzyme in patients with genetic mutations that are amenable to this chaperone in a cell-based assay. For patients currently receiving ERT for Fabry disease, migalastat HCl in combination with ERT may improve ERT outcomes by keeping the infused alpha-Gal A enzyme in its properly folded and active form.

Fabry disease is an inherited lysosomal storage disease that is currently estimated to affect approximately 5,000 to 10,000 people worldwide. Fabry Disease is caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A). The role of alpha-Gal A within the body is to break down a complex lipid called globotriaosylceramide (GL-3). Reduced or absent levels of alpha-Gal A activity leads to the accumulation of GL-3 in the affected tissues, including the central nervous system, heart, kidneys, and skin. This accumulation of GL-3 is believed to cause the various symptoms of Fabry disease, including pain, kidney failure, and increased risk of heart disorders and stroke.

### **About AT2220 for Pompe Disease**

AT2220 is an investigational, orally-administered pharmacological chaperone owned exclusively by Amicus. In published preclinical studies, AT2220-ERT co-administration resulted in significant increases in muscle rhGAA levels and decreases in glycogen levels in a mouse model of Pompe disease. Preclinical results to date also suggest that AT2220-ERT co-administration may mitigate ERT-induced immunogenicity by stabilizing the enzyme in its properly folded and active form.

Pompe disease is a lysosomal storage disease characterized by progressive skeletal muscle weakness and respiratory insufficiency. It is caused by a deficiency in GAA activity, which leads to accumulation of glycogen in tissues affected by the disease (primarily muscle). Pompe disease affects an estimated 5,000 to 10,000 individuals worldwide and is clinically heterogeneous in the age of onset, the extent of organ involvement, and the rate of progression.

### **Forward-Looking Statements**

This press release contains, and the accompanying conference call will contain, “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995 relating to 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, and the projected cash position for the Company. Words such as, but not limited to, “look forward to,” “believe,” “expect,” “anticipate,” “estimate,” “intend,” “plan,” “targets,” “likely,” “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. For example, 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 Quarterly Report on Form 10-Q for the quarter ended June 30, 2012. 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:**

Investors/Media:  
Sara Pellegrino  
spellegrino@amicusrx.com  
(609) 662-5044

**Table 1**

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Consolidated Statements of Operations**  
**(Unaudited)**  
**(In thousands, except share and per share amounts)**

	Three Months Ended September 30,		Nine Months Ended September 30,		Period from February 4, 2002 (inception) To Sept. 30, 2012
	2011	2012	2011	2012	2012
<b>Revenue:</b>					
Research revenue	\$ 4,138	\$ —	\$ 10,824	\$ 11,591	\$ 57,493
Collaboration and milestone revenue	1,660	—	4,980	6,820	64,382
<b>Total revenue</b>	<b>5,798</b>	<b>\$ —</b>	<b>15,804</b>	<b>18,411</b>	<b>121,875</b>
<b>Operating Expenses:</b>					
Research and development	13,711	11,499	36,455	39,226	304,846
General and administrative	4,841	4,995	15,963	14,909	128,158

Restructuring charges	—	—	—	—	1,522
Impairment of leasehold improvements	—	—	—	—	1,030
Depreciation and amortization	380	422	1,243	1,284	11,347
In-process research and development	—	—	—	—	418
Total operating expenses	18,932	16,916	53,661	55,419	447,321
Loss from operations	(13,134)	(16,916)	(37,857)	(37,008)	(325,446)
Other income (expenses):					
Interest income	31	92	136	235	14,308
Interest expense	(32)	(19)	(121)	(77)	(2,410)
Change in fair value of warrant liability	3,376	553	2,022	(1,941)	(1,041)
Other income	—	—	70	21	252
Loss before tax benefit	(9,759)	(16,290)	(35,750)	(38,770)	(314,337)
Benefit from income taxes	—	—	—	—	5,463
Net loss	(9,759)	(16,290)	(35,750)	(38,770)	(308,874)
Deemed dividend	—	—	—	—	(19,424)
Preferred stock accretion	—	—	—	—	(802)
Net loss attributable to common stockholders	\$ (9,759)	\$ (16,290)	\$ (35,750)	\$ (38,770)	\$ (329,100)
Net loss attributable to common stockholders per common share – basic and diluted	\$ (0.28)	\$ (0.34)	\$ (1.03)	\$ (0.88)	
Weighted-average common shares outstanding – basic and diluted	34,979,702	48,513,647	34,544,768	44,255,885	

**Amicus Therapeutics, Inc.**  
**(a development stage company)**  
**Consolidated Balance Sheets**  
*(Unaudited)*  
**(in thousands, except share and per share amounts)**

	December 31, 2011	September 30, 2012
<b>Assets:</b>		
Current assets:		
Cash and cash equivalents	\$ 25,668	\$ 24,273
Investments in marketable securities	30,034	81,942
Receivable due from GSK	5,043	3,184
Prepaid expenses and other current assets	5,903	3,077
Total current assets	66,648	112,476
Property and equipment, less accumulated depreciation and amortization of \$9,507 and \$8,080 at December 31, 2011 and September 30, 2012, respectively	2,438	5,293
Other non-current assets	709	442
<b>Total Assets</b>	<b>\$ 69,795</b>	<b>\$ 118,211</b>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable and accrued expenses	\$ 9,708	\$ 9,428
Current portion of deferred reimbursements	8,504	—
Current portion of secured loan	1,044	502
Total current liabilities	19,256	9,930
Deferred reimbursements, less current portion	18,999	27,235
Warrant liability	1,948	3,889
Secured loan, less current portion	—	398
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$.01 par value, 125,000,000 shares authorized, 34,654,206 shares issued and outstanding at December 31, 2011, 125,000,000 shares authorized, 49,360,659 shares issued and outstanding at September 30, 2012	407	554
Additional paid-in capital	299,285	385,042
Accumulated other comprehensive income	4	37
Deficit accumulated during the development stage	(270,104)	(308,874)
Total stockholders' equity	29,592	76,759
<b>Total Liabilities and Stockholders' Equity</b>	<b>\$ 69,795</b>	<b>\$ 118,211</b>



# 3Q12 Financial Results Conference Call & Webcast



***At the Forefront of Therapies for Rare and Orphan Diseases™***  
*November 5, 2012*

This conference call and presentation contain “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995 relating to business, operations and financial conditions of Amicus including but not limited to 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, the projected cash position for the Company, and business development and other transactional activities. 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-Q for the quarter ended June 30, 2012. 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.

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## Agenda

<b>Corporate Highlights</b>	John F. Crowley, Chairman & CEO
<b>Fabry Monotherapy Program Updates</b>	John F. Crowley, Chairman & CEO
<b>Chaperone-ERT Program Highlights</b> <i>Updated Pompe Results</i>	Bradley L. Campbell, Chief Business Officer David Lockhart, Chief Scientific Officer
<b>3Q12 Financial Results &amp; FY12 Guidance</b>	Chip Baird, Chief Financial Officer
<b>Upcoming Milestones/Concluding Remarks</b>	John F. Crowley, Chairman & CEO
<b>Q&amp;A</b>	John F. Crowley, Chairman & CEO Bradley L. Campbell, Chief Business Officer Chip Baird, Chief Financial Officer Pol F. Boudes, Chief Medical Officer David J. Lockhart, PhD, Chief Scientific Officer

Slide 3



## Amicus NJ Facility and Operations Intact and Fully Functional



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## Corporate Highlights

### Fabry Monotherapy Migalastat HCl

- ✓ Study 011: 12-month analysis plan completed – 6-month data expected 4Q12, 12-month data anticipated 1H13
- ✓ Study 012: target enrollment achieved ahead of schedule
- ✓ Updated Phase 2 extension study results presented

### Phase 2 Fabry Study Migalastat HCl Co-Administered with ERT

- ✓ Study 013: enrollment complete, updated preliminary results at ASHG 2012 (Nov. 8)

### Pompe Program AT2220 Co-Administered with ERT

- ✓ Phase 2 Study 010: positive preliminary results (Cohorts 1-3) at World Muscle Society – enrollment complete (4 Cohorts), results by YE12
- ✓ *Ex vivo* studies: AT2220 mitigates ERT immunogenicity

### Corporate and Financial Strength

- ✓ \$106.2M cash position on Sept. 30, 2012
- ✓ Flexibility to advance Fabry programs with GSK, rest of pipeline independently
- ✓ Transitioning into fully-integrated biopharmaceutical company within U.S. - full U.S. economics for all Fabry products upon approval

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## Global Phase 3 Registration Studies

### Both Studies Evaluating Migalastat HCl 150 mg, Every-Other-Day in Patients with Amenable Genetic Mutations

#### STUDY 011

- U.S. Registration Study
- Placebo-controlled
- 67 patients
- 6-month surrogate endpoint: kidney GL-3
- Potential for accelerated approval
- 6-month primary treatment period complete – data expected 4Q12
- 6-month open-label treatment extension data expected 1H13
- Ongoing treatment provided in Phase 3 extension studies
- 12-month analysis plan completed

#### STUDY 012

- Global Registration Study
- First clinical study to compare ERT to migalastat HCl
- 1.5: 1 randomization to migalastat HCl or ERT
- Target enrollment achieved ahead of schedule
- 57 patients now randomized (24 male / 33 female), final enrollment expected by YE12
- 18-month clinical endpoint: kidney function (GFR) – descriptive statistics

## Study 011 6 and 12 Month Analysis Plans

Study 011 Design (1:1 Randomization)			
Study Arm	Stage 1: Month 0-6		Stage 2: Month 6-12*
Placebo	Placebo	switch	Migalastat HCl
Treatment	Migalastat HCl		Migalastat HCl

- 6-month primary efficacy endpoint is interstitial capillary GL-3 (migalastat HCl vs. placebo) – results expected 4Q12
- 12-month descriptive comparisons proposed to support primary efficacy analysis – results anticipated 1H13
  - Placebo arm stage 1 (Placebo 6 months) vs. stage 2 (migalastat HCl 6 months)
  - Treatment arm stage 1 (migalastat HCl 6 months) vs. stage 2 (migalastat HCl 12 months)
  - Treatment arm stage 1 + Placebo arm stage 2 (pooled migalastat HCl 6 months) vs. placebo arm stage 1 (Placebo 6 months)
  - Additional safety data

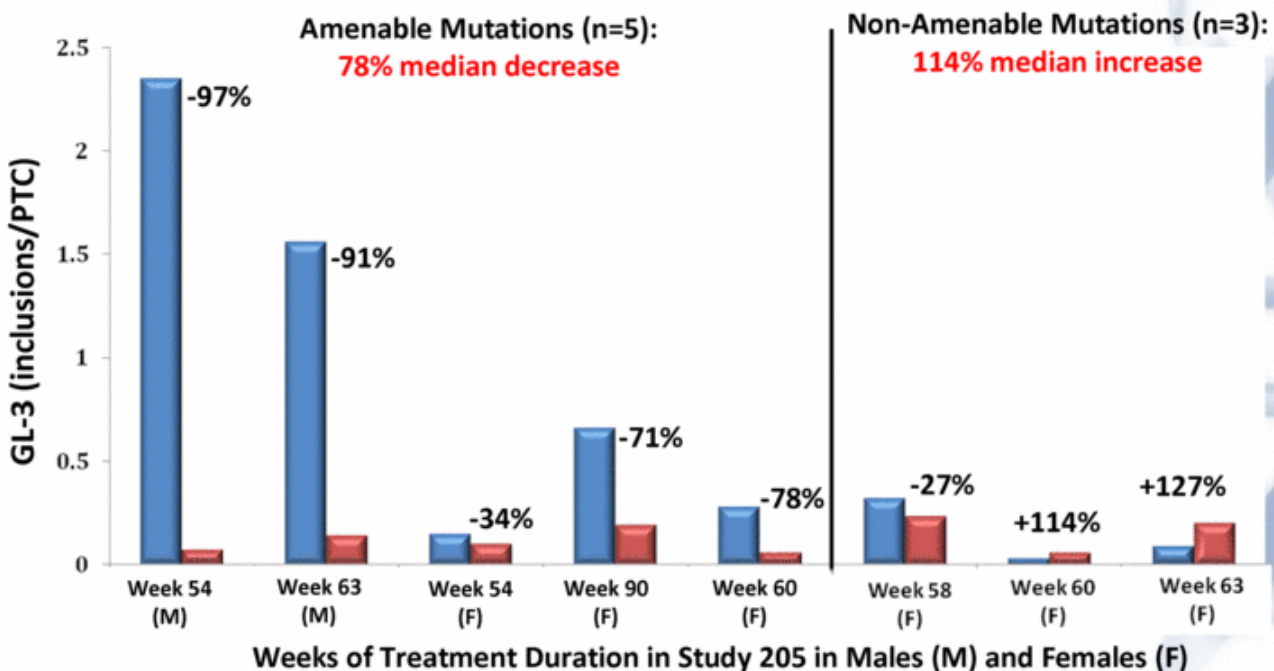
\*Additional 12-24 month open-label extension following Stage 2

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# Migalastat HCl Monotherapy for Fabry Disease

## Phase 2 Extension Study Update

Median 78% Decline in Kidney GL-3 Observed in Study Patients with Amenable Mutations



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### *Patient Disposition to Date (as of 10/31/12)*

**Long-Term Safety and Efficacy of Migalastat HCl in Patients Who Completed Previous Phase 2 Study**

27 Patients Enrolled in 4 Phase 2 Studies

26 Patients Completed Phase 2 Primary Treatment Period (12 or 24 Weeks)

23 Patients Completed Phase 2 Treatment Extension

23 out of 23 Patients Enrolled in Study 205

17 Patients Completed Study 205 (5.2 years median treatment duration)

16 Patients Currently Enrolled in Separate Open-Label Extension Study\*

## Study 011

### *Patient Disposition to Date (as of 10/31/12)*

**Low dropout rate and majority continuing in extension studies**

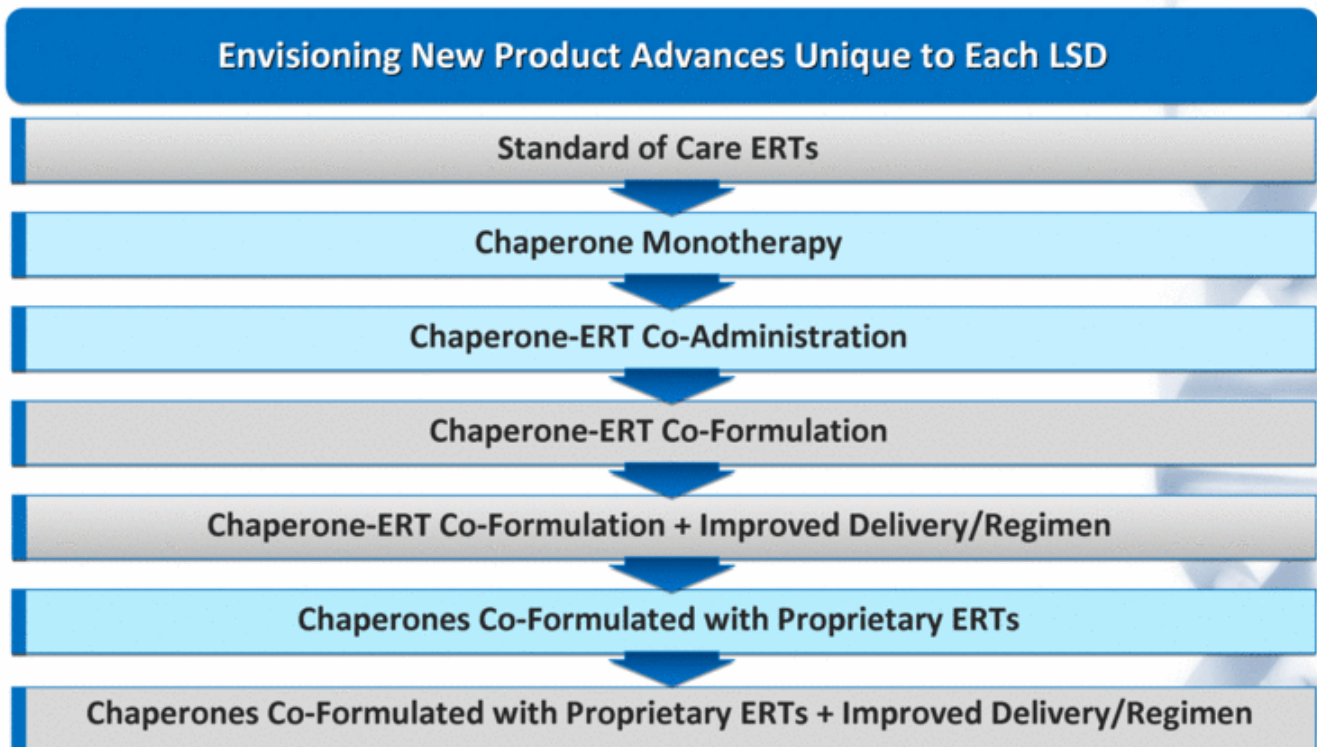
63 completed 6-mo. double-blind treatment period (~6% dropout rate)

63 continued in 6-mo. open-label treatment extension

51 to date completed Study 011 (6-mo. treatment + 6-mo. extension)

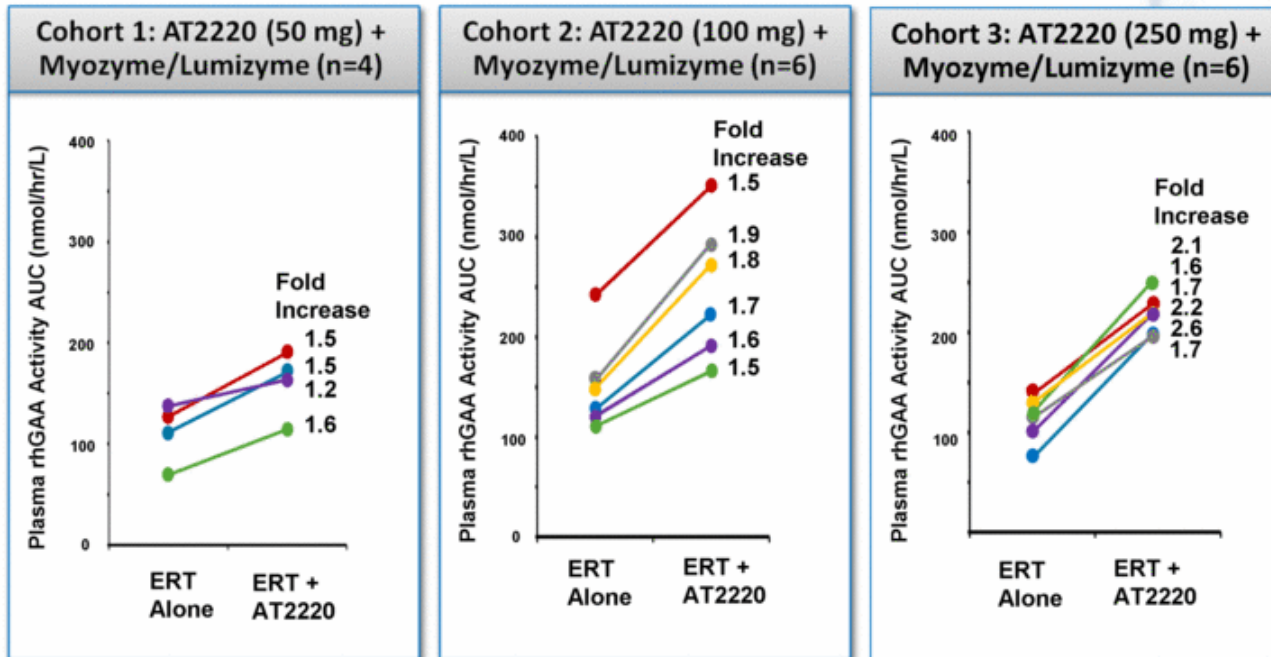
49 of 51 currently enrolled in both open-label extension studies\*

## *Multiple Paths Forward for Chaperone Therapy*



## Plasma PK Preliminary Data (n=16)

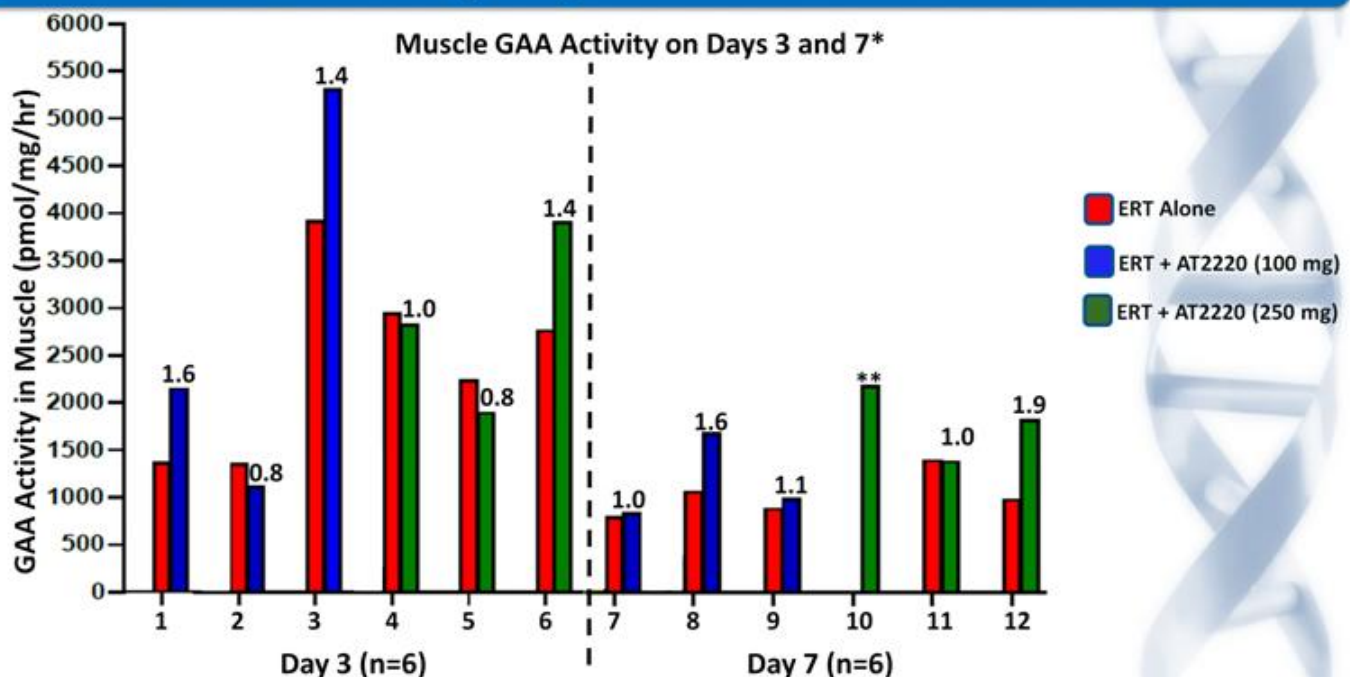
Co-Administration Increases Levels of Active Enzyme (GAA) in Plasma up to ~2.6-Fold vs. ERT Alone in First 3 Dose Cohorts of AT2220



Slide 12

## Needle Core Muscle Biopsies – Preliminary Data (n = 12)

Cohort 2-3 Muscle Biopsies Suggest Co-Administration Increases Enzyme Uptake into Muscle vs. ERT Alone



\*Ratios Relative to ERT Alone

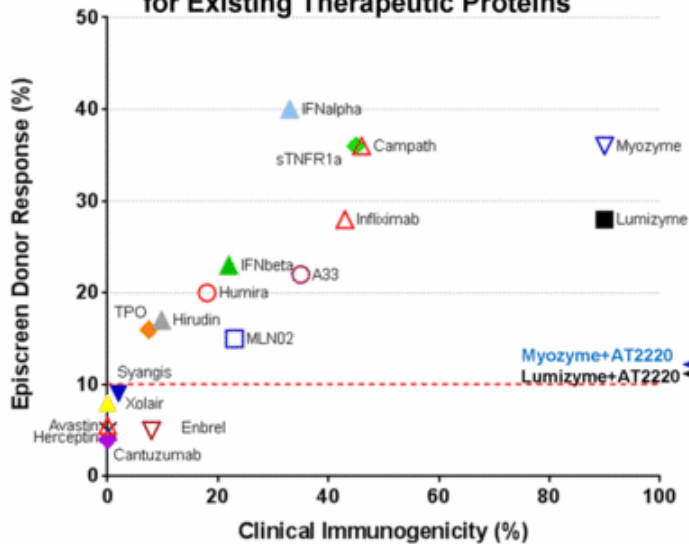
\*\*ERT Sample Insufficient for Determining Fold-Increase

Slide 13

## MDA Grant Supports Ongoing *ex vivo* Studies to Evaluate Immunogenicity of Pompe ERT +/- AT2220

### Episcreen™ Assays

Predictive of Clinical Immunogenicity for Existing Therapeutic Proteins



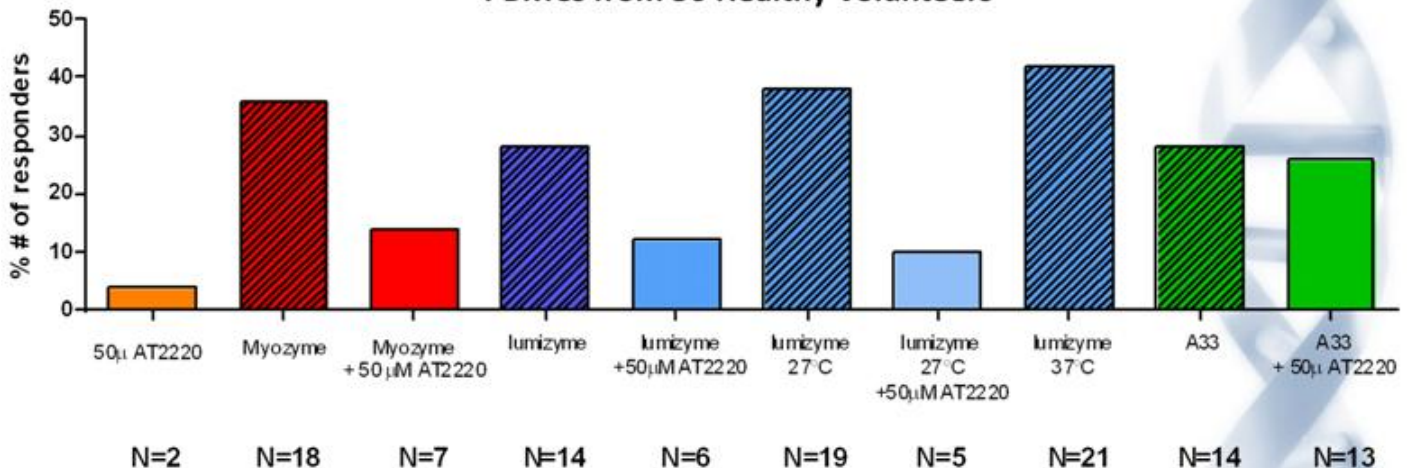
- Myozyme and Lumizyme among most immunogenic proteins assessed using EpiScreen
- AT2220 + Myozyme/Lumizyme significantly reduced T-cell responses in PBMCs from 50 healthy volunteers with different HLA types
- Next studies to evaluate T-cell response in patient-derived PBMCs from Study 010 (correlate HLA type, IgG titer and neutralizing antibody responses with T-cell stimulation index)
- Correlation between HLA type and immune response may help design future studies

# Pompe ERT-Related Immunogenicity

## Preliminary T-Cell Proliferation Results for Myozyme/Lumizyme +/- AT2220

### AT2220 Mitigates Pompe ERT-Related Immunogenicity *ex vivo*

#### PBMCs from 50 Healthy Volunteers



## Consolidated Statement of Operations (Unaudited) In thousands, except share and per share amounts

	3 Months Ended September 30,	
	2012	2011
<b>Revenue:</b>		
Research Revenue*	\$ ---	\$ 4,138
Collaboration revenue**	---	1,660
<b>Total revenue</b>	---	5,798
<b>Operating Expenses:</b>		
Research and development	11,499	13,711
General and administrative	4,995	4,841
Depreciation and amortization	422	380
<b>Total operating expenses</b>	16,916	18,932
<b>Loss from operations</b>	<b>(16,916)</b>	<b>(13,134)</b>
<b>Non-operating income</b>	<b>626</b>	<b>3,375</b>
<b>Net loss / net loss attributable to common stockholders</b>	<b>\$ (16,290)</b>	<b>\$ (9,759)</b>
<b>Net loss per common share – basic and diluted</b>	<b>\$ (0.34)</b>	<b>\$ (0.28)</b>
<b>Weighted-average common shares outstanding - basic and diluted</b>	<b>48,513,647</b>	<b>34,979,702</b>

\*Cash payments from GSK as part of cost sharing arrangement now recorded on balance sheet as deferred reimbursements

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\*\*Upfront GSK license payment previously amortized in collaboration revenue – unrecognized balance now in deferred reimbursements



## Impact from Contingent Future Milestones

Impacts Research and Collaboration Revenue Recognition, Effective 3Q12  
No Impact on Cash or Deal Economics

### Consolidated Statement of Operations (Unaudited)\*

	3 Months Ended	
	Sept. 30,	
	2011	2012
<b>Revenue:</b>		
Research Revenue	\$ 4,138	\$ 0
Collaboration and milestone revenue	1,660	0
<b>Total revenue</b>	<b>5,798</b>	<b>0</b>

Cash payments from GSK to Amicus as part of cost sharing arrangement (~\$4.5M in 3Q12)

Straight-line amortization of upfront license payment

Balance of unrecognized upfront license payment (~\$22.7M on 9/30/12)

### Consolidated Balance Sheets (Unaudited)\*

	Dec. 30, 2011	Sept. 30, 2012
<b>Assets:</b>		
Total Current Assets	66,648	112,476
Total Non-Current Assets	3,147	5,735
<b>Total Assets</b>	<b>69,795</b>	<b>118,211</b>
<b>Liabilities &amp; Stockholders' Equity</b>		
Total current liabilities:	19,256	9,930
Deferred reimbursements less current portion	18,999	27,235
Warrant Liability	1,948	3,889
Secured loan, less current portion	-	398
<b>Total liabilities</b>	<b>40,203</b>	<b>41,452</b>
Commitments and contingencies		
<b>Total stockholders' equity</b>	<b>29,592</b>	<b>76,759</b>
	\$ 69,795	\$ 118,211

\*In thousands

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## FY12 Financial Guidance

### ■ Cash position

- \$106.2 at September 30, 2012 vs. \$56.0M at December 31, 2011
- ≥ \$90M projected at December 31, 2012, expected to fund current operating plan beyond 2013

### ■ Balance sheet strengthened in 3Q12

- \$18.6M GSK equity investment
- \$3.5M development milestone received from GSK

### ■ FY12 OpEx guidance reiterated:

- Upper end of previous guidance range of \$37M - \$43M
- Net of anticipated Fabry cost-sharing

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## Building Shareholder Value

<b>Fabry</b>	✓ Phase 2 Study 013 Preliminary Co-Administration Data	Q1
	✓ Preclinical Chaperone-ERT Co-Formulation Results	Q3
	✓ Phase 3 Study 011 – 6-month primary treatment complete	Q3
	✓ Phase 3 Study 012 – target enrollment achieved	Q4
	▪ Phase 2 Study 013 – additional preliminary data	Q4
	▪ Phase 3 Study 011 – 6-month data	Q4
	▪ Phase 3 Study 011 – 12-month data	H1'13

<b>Pompe</b>	✓ MDA Grant to Investigate ERT Immunogenicity	Q1
	✓ Phase 2 Study 010 Preliminary Co-Administration Data	Q2
	✓ ERT Immunogenicity Preclinical Results	Q4
	✓ Additional Phase 2 Study 010 Co-Administration Data	Q4
	▪ Final Phase 2 Study 010 Co-Administration Data	Q4

<b>Parkinson's</b>	▪ Completion of additional AT3375 IND-Enabling Studies	Q4
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## Q&A

**John F. Crowley, Chairman & CEO**  
**Bradley L. Campbell, Chief Business Officer**  
**Chip D. Baird, Chief Financial Officer**  
**Pol F. Boudes, MD, Chief Medical Officer**  
**David J. Lockhart, PhD, Chief Scientific Officer**

