First Quarter 2017 Financial Results

May 4, 2017

Agios Conference Call Participants

Prepared Remarks

Introduction

- KENDRA ADAMS, Sr. Director, Investor & Public Relations

Business Highlights & 2017 Key Milestones

- DAVID SCHENKEIN, M.D., Chief Executive Officer

Clinical Development Progress

- CHRIS BOWDEN, M.D., Chief Medical Officer

First Quarter 2017 Financial Results

- ANDREW HIRSCH, Chief Financial Officer

Forward Looking Statements

This presentation and various remarks we make during this presentation contain forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include those regarding the Agios' plans, strategies and expectations for its and its collaborator's preclinical, clinical and commercial advancement of its drug development programs including IDHIFA® (enasidenib), ivosidenib, AG-881, AG-348 and AG-270; the potential benefits of Agios' product candidates; its key milestones for 2017; its plans regarding future data presentations; its financial guidance regarding the period in which it will have capital available to fund its operations; and the potential benefit of its strategic plans and focus. The words "anticipate," "expect," "intend," "potential," "milestone," "will," "on track", "upcoming" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from Agios' current expectations and beliefs. For example, there can be no guarantee that any product candidate Agios or its collaborator, Celgene, is developing will successfully commence or complete necessary preclinical and clinical development phases, or that development of any of Agios' product candidates will successfully continue. There can be no guarantee that any positive developments in Agios' business will result in stock price appreciation. Management's expectations and, therefore, any forward-looking statements in this presentation and various remarks we make during this presentation could also be affected by risks and uncertainties relating to a number of other important factors, including: Agios' results of clinical trials and preclinical studies, including subsequent analysis of existing data and new data received from ongoing and future studies; the content and timing of decisions made by the U.S. FDA and other regulatory authorities, investigational review boards at clinical trial sites and publication review bodies; Agios' ability to obtain and maintain requisite regulatory approvals and to enroll patients in its planned clinical trials; unplanned cash requirements and expenditures; competitive factors; Agios' ability to obtain, maintain and enforce patent and other intellectual property protection for any product candidates it is developing; Agios' ability to maintain key collaborations, such as its agreements with Celgene; and general economic and market conditions. These and other risks are described in greater detail under the caption "Risk Factors" included in Agios' public filings with the Securities and Exchange Commission. Any forward-looking statements contained in this presentation and various remarks we make during this presentation speak only as of the date hereof, and Agios expressly disclaims any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

Business Highlights & 2017 Key Milestones

David Schenkein, M.D., Chief Executive Officer

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Key Priorities & Expected Milestones

IDH

- Secure approval and co-commercialize IDHIFA® (enasidenib) for R/R AML in the U.S.
- Submit NDA for wholly owned ivosidenib in R/R AML by YE 2017
- Initiate Phase 3 combining ivosidenib and VIDAZA® in frontline AML in 1H 2017
- Complete enrollment of Phase 1 dose-escalation for AG-881 in glioma in 1H 2017

PKR

- Continue to demonstrate leadership in PK deficiency
- Finalize pivotal trial design for wholly owned AG-348 in PK deficiency in 3Q 2017
- Initiate a pivotal trial for AG-348 in PK deficiency 1H 2018

RESEARCH

- Advance next wave of research in three areas of expertise: cancer metabolism, rare genetic diseases and metabolic immuno-oncology
- Submit IND application for AG-270, development candidate targeting MTAP-deleted tumors, by YE 2017

First Quarter Highlights & New Announcements

ASCO & EHA DATA PRESENTATIONS

- Updated data from Phase 1/2 trial in IDH2m R/R AML to be presented at ASCO
- First data from ivosidenib cholangio Phase 1 expansion cohort to be presented at ASCO
- Updated data from AG-348 Phase 2 DRIVE PK study to be presented at EHA in June

FIRST QUARTER HIGHLIGHTS

IDH INHIBITORS
- FDA accepted IDHIFA® NDA for IDH2m R/R AML for Priority Review; Aug. 30 PDUFA date
- Completed enrollment in 125 patient expansion cohort for ivosidenib in R/R AML
- FDA granted ivosidenib Orphan Drug Designation for the treatment of cholangiocarcinoma

PKR ACTIVATOR
- FDA granted AG-348 Fast Track Designation for the treatment of patients with PK deficiency
- Completed enrollment of 258 patients in the PK deficiency natural history study being conducted with Boston Children's Hospital

RESEARCH
- Presented preclinical molecule data for potential treatment of MTAP deleted cancers at the Keystone Tumor Metabolism Meeting
- Celgene designated AG-270 for the treatment of MTAP-deleted cancers as a development candidate
- Entered into a licensing agreement with Aurigene to research, develop & commercialize small molecule inhibitors of an undisclosed cancer metabolism target

CORPORATE
- Completed follow-on offering resulting in gross proceeds of ~$287 million

Clinical Development Progress

Chris Bowden, M.D., Chief Medical Officer

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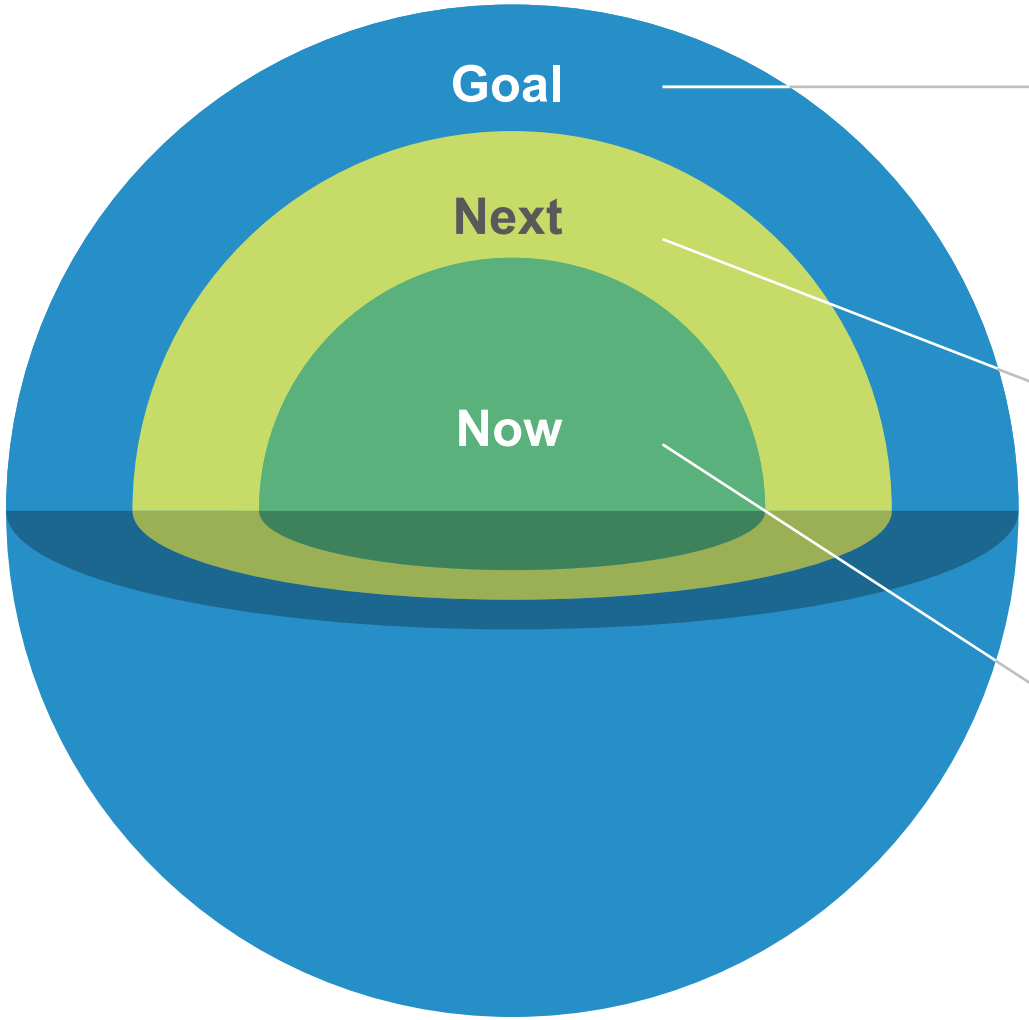
What's Possible for IDHm Patients

A Roadmap for Speed and Breadth



All IDHm patients screened and treated with an IDHm inhibitor for the entire course of their disease

Frontline AML

Combination trials

Maintenance

Other hematologic malignancies

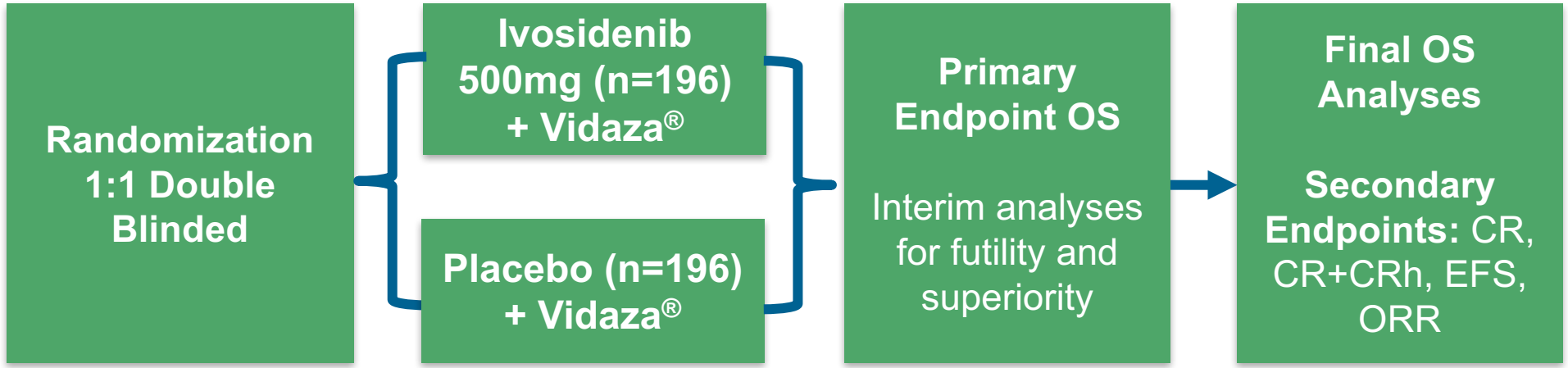
Relapsed/Refractory AML

- IDHIFA® NDA accepted; Aug. 30 PDUFA
- IDHIFA® Phase 1/2 data to be presented at ASCO
- Ivosidenib NDA filing by YE

Advancing Ivosidenib into Frontline Setting

AGIOS study for IDH1 mutation in acute myeloid LEukemia

Global Phase 3 Frontline IC-Ineligible IDH1m AML



Trial on track for 1H 2017 initiation

IC = intensive chemotherapy
Vidaza® is a registered trademark of Celgene Corporation

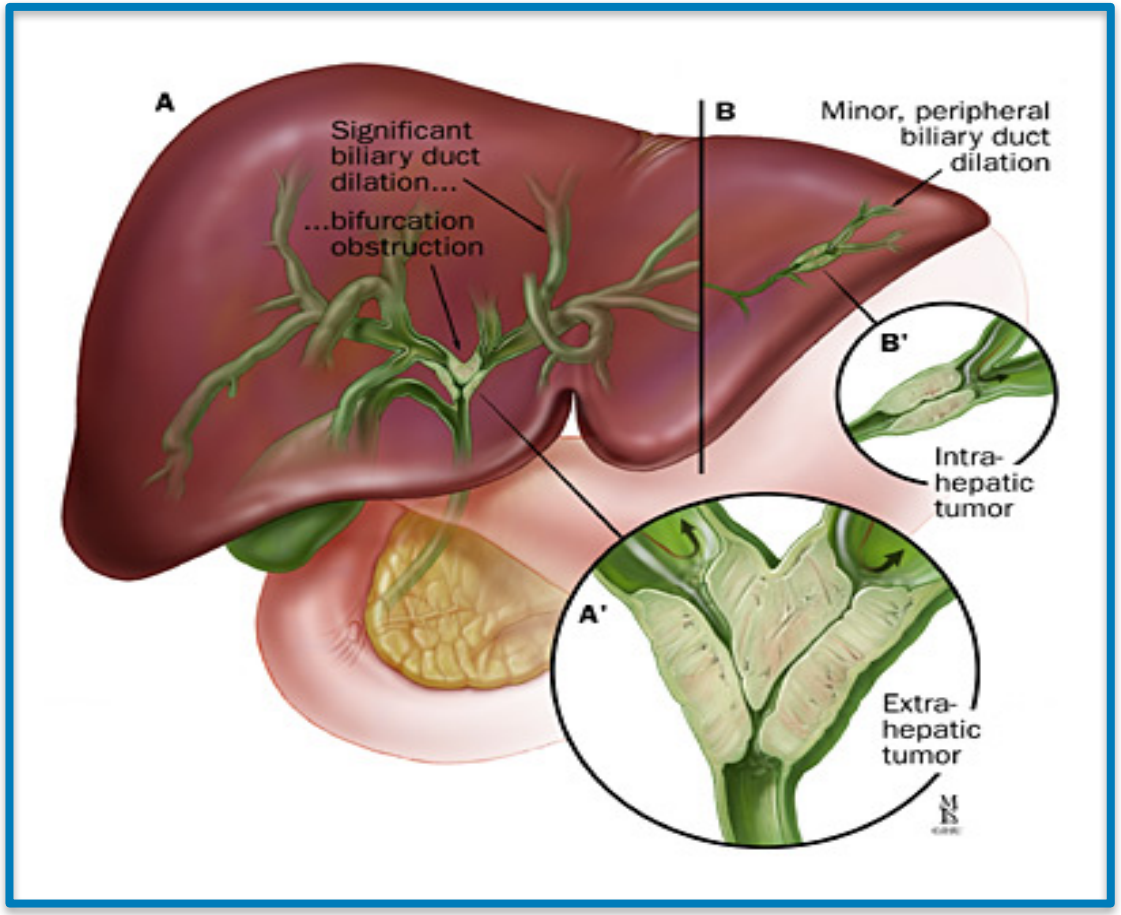
Phase 1 Combo Studies of IDHIFA® or Ivosidenib with 7+3 or Vidaza®
Ongoing

Phase 3 7+3 Combo Study
Planned

Multiple Novel-Novel Combo ISTs
Plan to Support

Cholangiocarcinoma Is a Rare Cancer of the Bile Duct and Liver

- 50% of cases occur within the liver (intrahepatic cholangiocarcinoma, IHCC)
  - 2,000-4,000 new cases per year in U.S.
  - IDH1 mutation frequency estimated at 11- 24%
  - 5 year overall survival ~9%
- Current treatments are inadequate
  - Surgery possibility curative, if not metastatic
  - Cisplatin plus gemcitabine standard of care for newly diagnosed metastatic disease



Multiple sources, including market research and SEER. Estimates will continue to evolve with additional future data

Registration-Enabling Phase 3 Cholangiocarcinoma Study

ClarIDHy

Global Phase 3 Previously Treated Advanced IDH1m Cholangiocarcinoma

2:1 Double Blind Randomization (n=186)

- **Ivosidenib Arm 500mg**
- **Placebo Arm**

Primary Endpoint: PFS

Crossover at time of progression

Secondary Endpoints: OS, ORR, safety, QoL

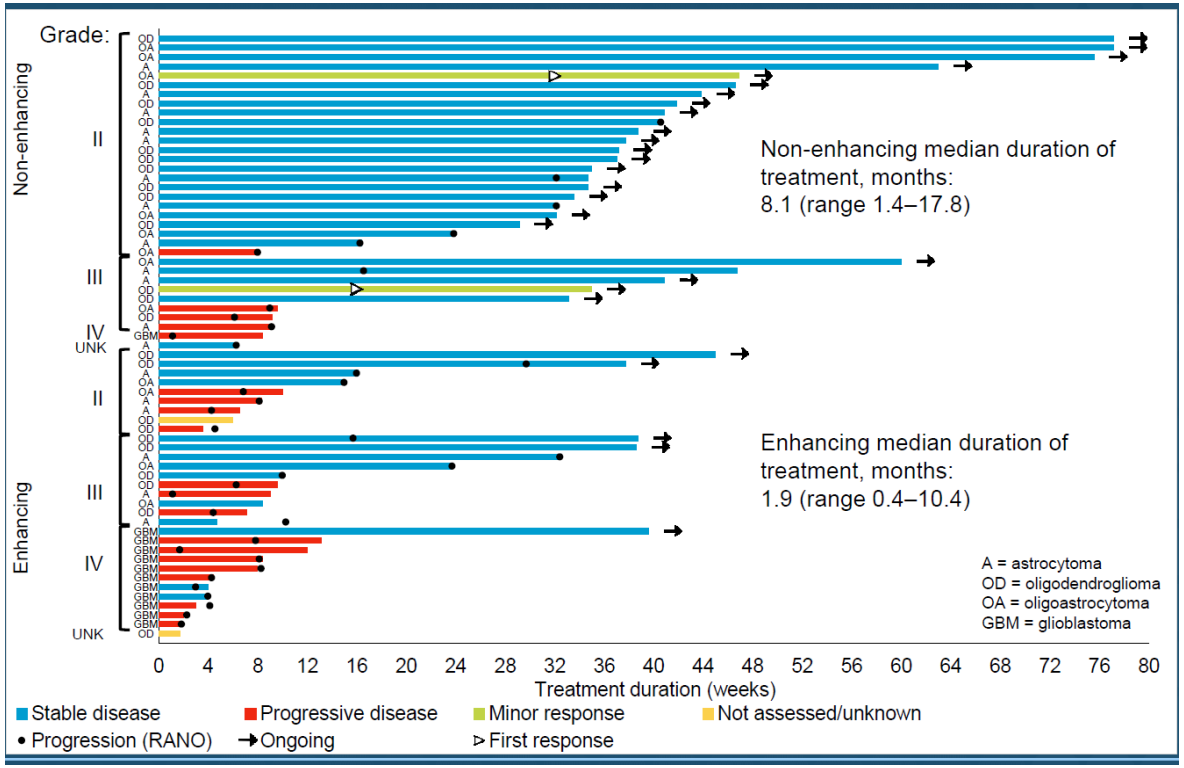
Phase 3 ClarIDHy trial initiated in December 2016;
Data from Phase 1 cholangiocarcinoma expansion cohort to be presented at ASCO

Encouraging Data with Ivosidenib Supports Clinical Development of IDH1m Inhibitor in Glioma

Ivosidenib

IDH1m Inhibition

→ Phase 1 Dose-Escalation and Expansion (Enrollment Complete)



Ivosidenib Data Presented at SNO 2016

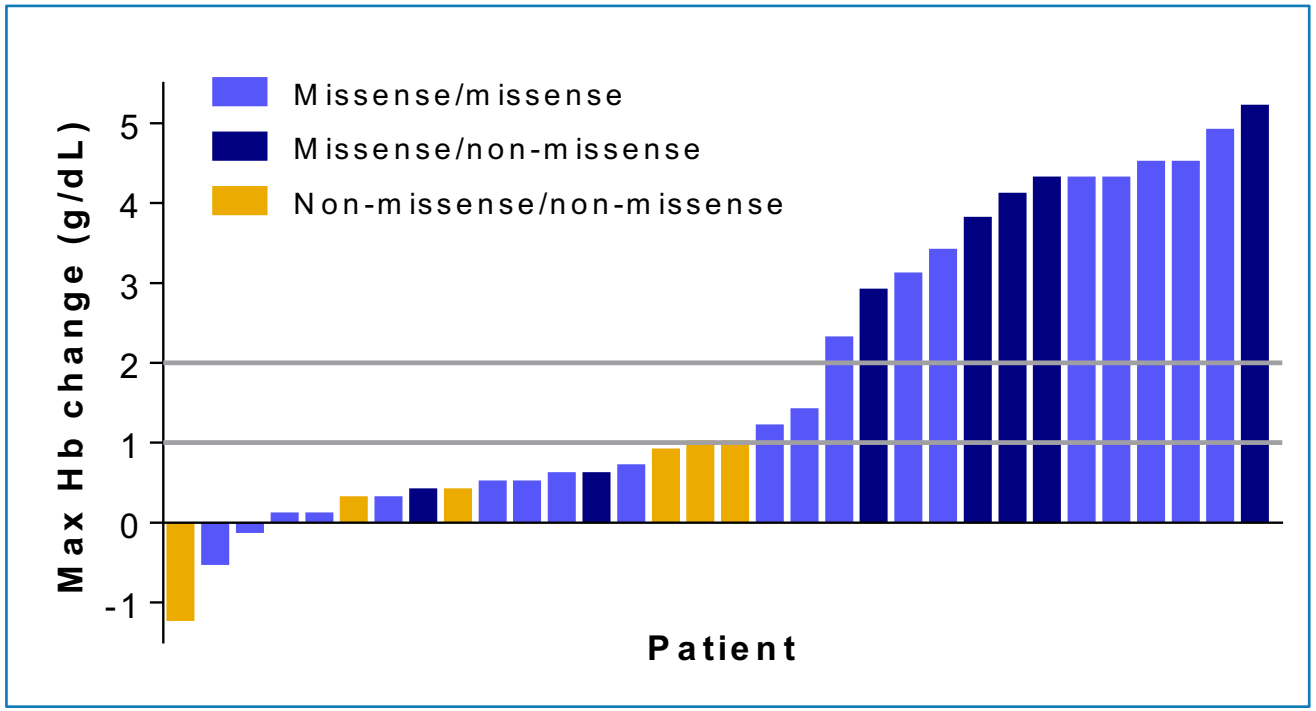
AG-881

High Brain Penetration, Pan-IDHm Inhibition

→ Phase 1 Dose-Escalation (Ongoing) → Potential Expansion

Updated data from ivosidenib Phase 1 glioma expansion cohort expected in 2H 2017

Compelling Proof-of-concept Data for AG-348, the First Disease Modifying Therapy for PK Deficiency



52 patients enrolled;
17 completed first 24 weeks, 15 in extension

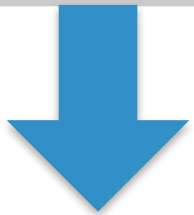
DRIVE PK Learnings
Robust hemoglobin increases in 15 / 32 patients; 15 / 26 patients with 1 or more missense mutation
Responses are rapid and sustained; median time to response of 1.4 weeks; mean max hemoglobin increase of 3.6 g/dL in responders
Majority of responders seen at doses ≤50 mg BID and as low as 5 mg QD
Well-tolerated beyond six months of dosing

Data presented at ASH 2016

Updated DRIVE PK data to be presented in an oral presentation at EHA

Key Considerations for AG-348 Pivotal Trial Design

Design Element	Considerations	Rationale
Patient Population	• Transfusion dependent adult (TD) • Non-Transfusion dependent adult (NTD)	• Goal to treat all adult patients
Size	• ~100 patients	• Rare disease
Dose	• Dose titration up to optimal hemoglobin response	• Majority of responders seen at doses ≤50 mg BID and as low as 5 mg QD
Endpoints	• Hemoglobin response (NTD) • Reduction in transfusion frequency (TD) • Patient-reported outcomes (PRO)	• Establish clinical benefit
Control	• Placebo controlled	• Evaluate PRO



Expect to initiate registrational program in 1H 2018

First Quarter 2017 Financial Results

Andrew Hirsch, Chief Financial Officer

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First Quarter 2017 Financial Results

Balance Sheet	March 31, 2017	December 31, 2016	March 31, 2016
Cash, Cash Equivalents & Marketable Securities	$503M	$574M	$356M
Total Assets	$557M	$619M	$396M

Statement of Operations	Three Months Ended March 31, 2017	Three Months Ended December 31, 2016	Three Months Ended March 31, 2016
Collaboration Revenue	$11M	$23M	$31M
Research & Development Expense	$63M	$65M	$44M
General & Administrative Expense	$15M	$15M	$11M

The R&D expenses reported for the three months ended March 31, 2017, December 31, 2016 and March 31, 2016 are reported net of cost reimbursements of $3 million, $1 million and $9 million, respectively.

~$270M net proceeds from follow-on offering;
Extends cash runway through at least the end of 2019

2017 – 2018

Commercial Stage Biopharmaceutical Company

2 approved precision medicines in AML

4+ clinical-stage molecules

3+ pivotal trials (IDH, PKR)

3 areas of research expertise

450+ employees

100% committed to helping patients

Delivering Our First Medicines to Patients

Team

Science

Discovery