Fourth Quarter and Full Year 2016 Financial Results

February 16, 2017

Agios Conference Call Participants

Prepared Remarks

Introduction

- RENEE LECK, Sr. Manager, Investor & Public Relations

2017 Vision & Key Milestones

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

Clinical Development Progress

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

Fourth Quarter and Full Year 2016 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 enasidenib, ivosidenib (AG-120), AG-881 and AG-348; 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," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "would," "could," "potential," "possible," "hope," "strategy," "milestone," "will," 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' Quarterly Report on Form 10-Q for the quarter ended September 30, 2016, and other filings that Agios may make with the Securities and Exchange Commission in the future. Any forward-looking statements contained in this 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.

2017 Key Milestones

David Schenkein, M.D., Chief Executive Officer

agios

January 2017

Delivering Our First Medicines to Patients

Team

Science

Discovery

Key Priorities & Expected Milestones

IDH

- Secure approval and co-commercialize 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 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 development candidate targeting MTAP-deleted tumors by YE 2017

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

Clinical Development Progress

Chris Bowden, M.D., Chief Medical Officer

agios

Anticipated 2017 Data Presentations

IDH Mutant Inhibitors in AML

- Present the first data from the expansion phase of the ongoing Phase 1 study of ivosidenib in R/R AML in 2H 2017
- Present the first data from the ongoing Phase 1b frontline combination study of enasidenib or ivosidenib with standard-of-care intensive chemotherapy in newly diagnosed AML in 2H 2017

IDH Mutant Inhibitors in Solid Tumors

- Present the first data from the expansion phase of the ongoing Phase 1 study of ivosidenib in advanced IDH1m cholangiocarcinoma in 1H 2017
- Present updated data from the expansion phase of the ongoing Phase 1 study of ivosidenib in advanced IDH1m low-grade glioma in 2H 2017

Rare Genetic Diseases

- Present updated data from AG-348 Phase 2 DRIVE PK study in PK deficiency in 1H and 2H 2017

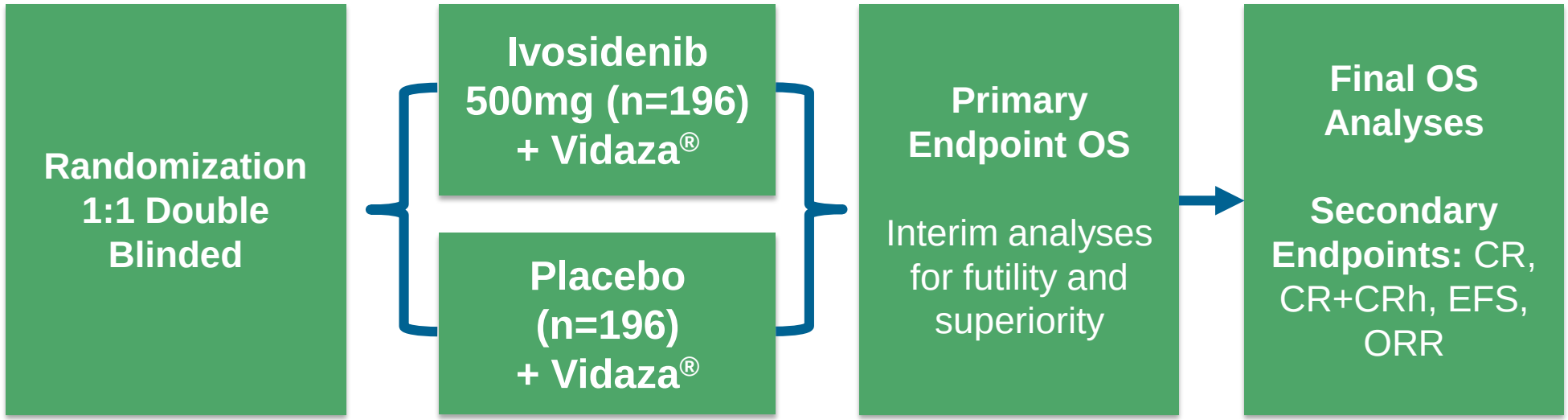
Cancer Metabolism Research

- Present updated preclinical data for the MTAP pathway development candidate at the Keystone Tumor Metabolism Meeting taking place March 5-9, 2017 in Whistler, British Columbia

Advancing Ivosidenib into Frontline Setting

AGIOS study for IDH1 mutation in acute myeloid LEukemia

Global Phase 3 Frontline IC-Ineligible IDH1m AML



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

Trial on track for 1H 2017 initiation

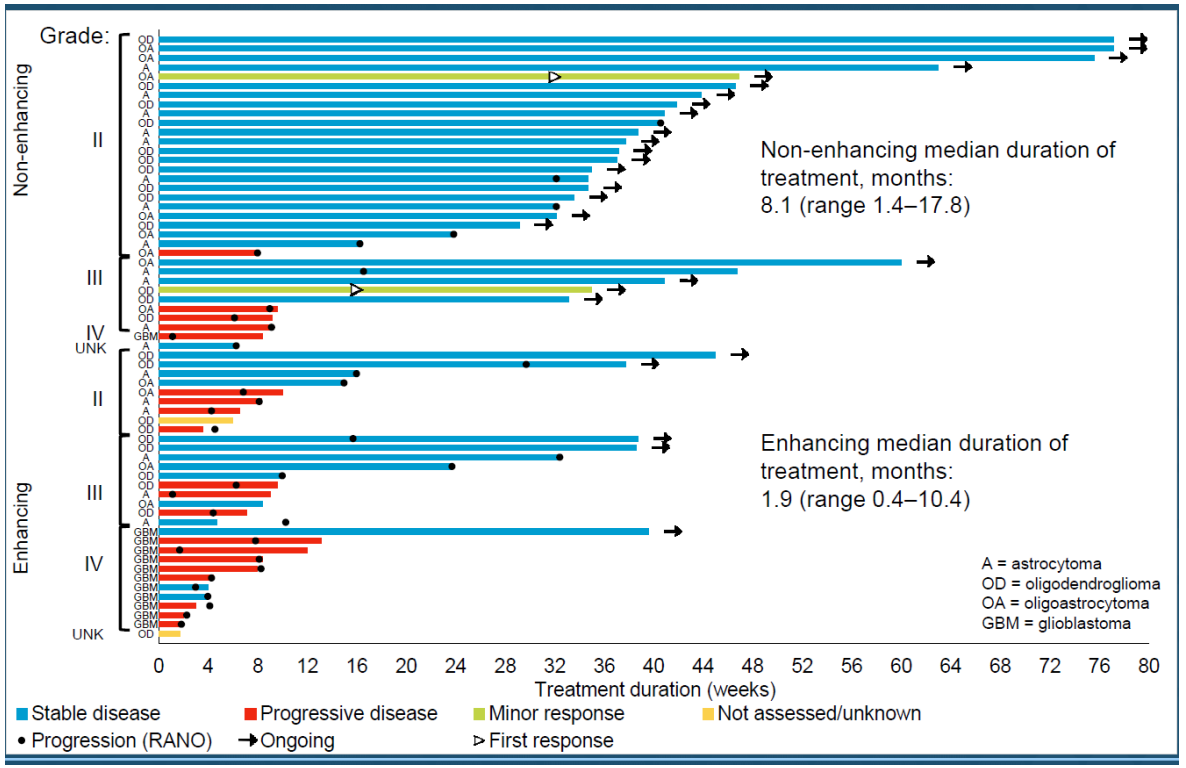
Encouraging Data with Ivosidenib Supports Clinical Development of IDH1m Inhibitor in Glioma

Ivosidenib

IDH1m Inhibition

→

Phase 1 Dose-Escalation and Expansion (Enrollment Complete)



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

Trial Initiated in December 2016

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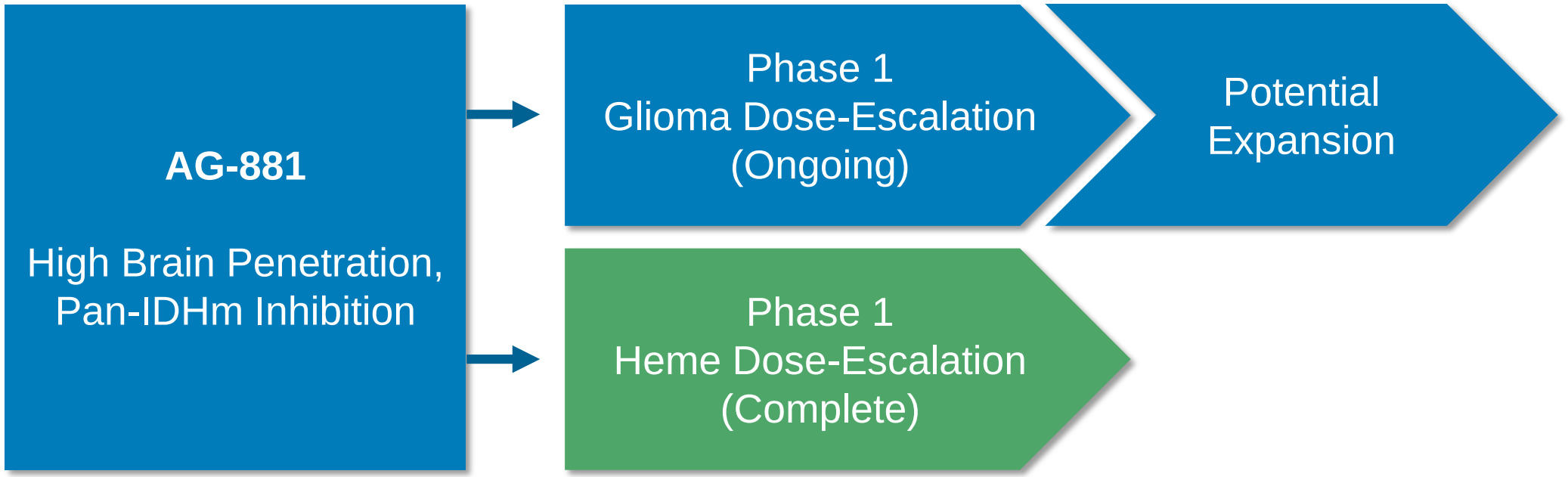
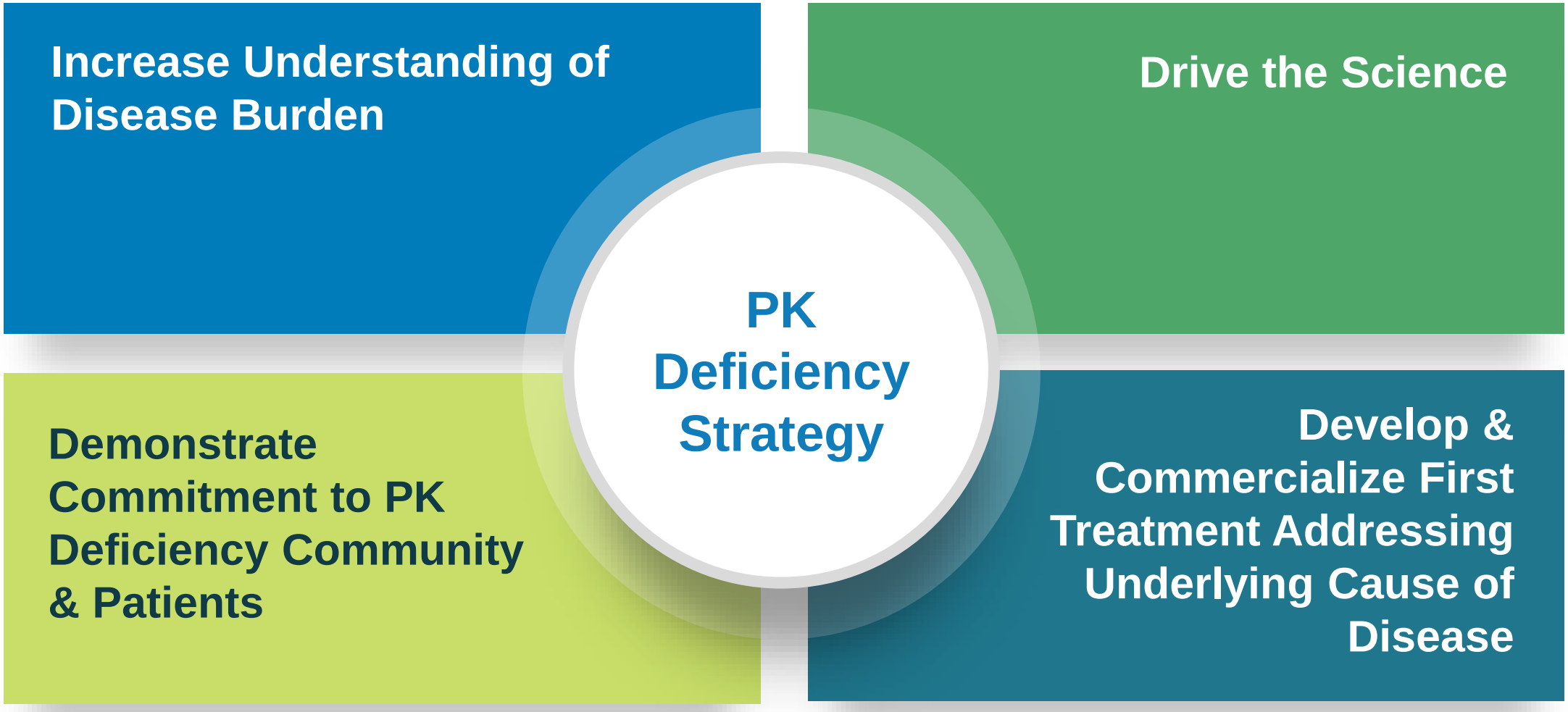
AG-881 Clinical Development

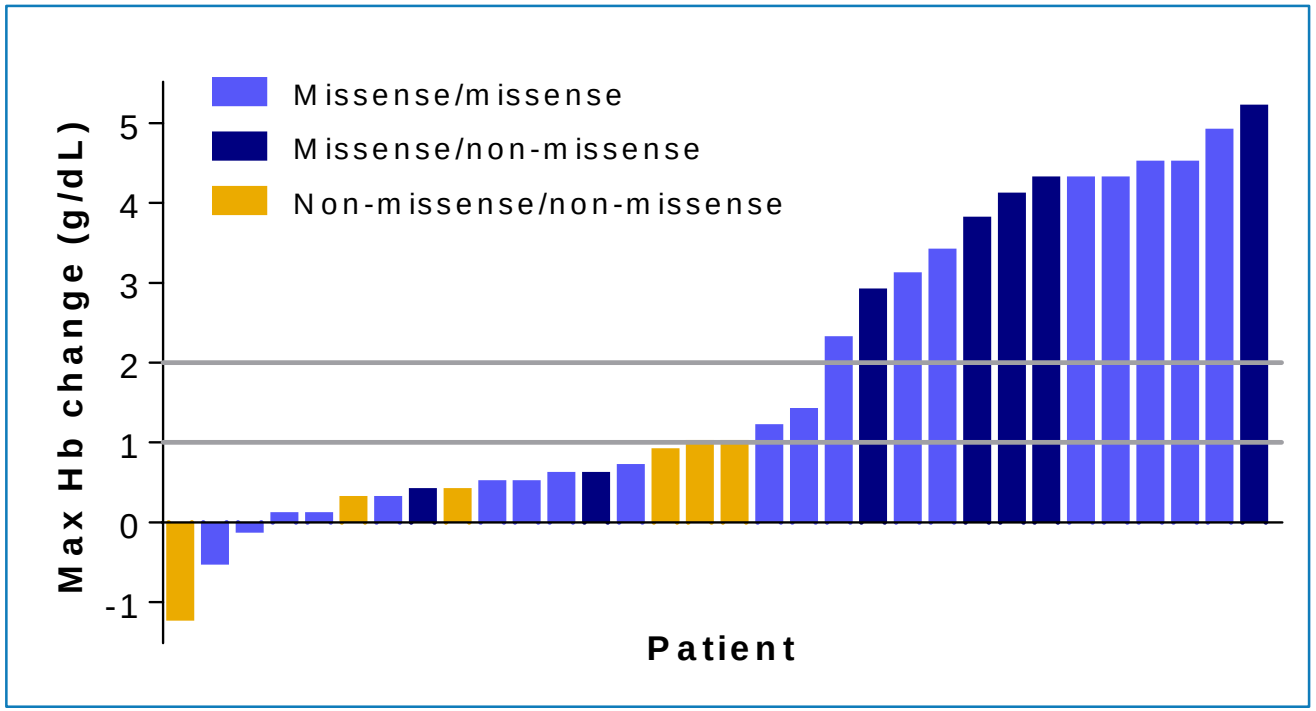


Continuing to explore additional doses in glioma

Agios Leadership in PK Deficiency



Compelling Proof-of-Concept 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

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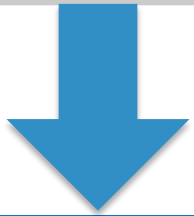
Key Considerations for AG-348 Pivotal Trial Design

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 pivotal study in 1H 2018

Full Year 2016 Financial Results

Andrew Hirsch, Chief Financial Officer

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Full Year 2016 Financial Results

Balance Sheet	December 31, 2016	December 31, 2015
Cash, Cash Equivalents and Marketable Securities	$573.6M	$375.9M
Total Assets	$619.1M	$420.1M

Statement of Operations	December 31, 2016	December 31, 2015
Collaboration Revenue (1)	$69.9M	$59.1M
Research & Development Expense (1)	$220.2M	$141.8M
General & Administrative Expense	$50.7M	$36.0M

(1) The R&D expenses reported for the twelve months ended December 31, 2016 and December 31, 2015 are reported net of cost reimbursements of $19.7 million and $25.2 million, respectively.

YE 2016 cash balance of ~$574M expected to fund operations through at least the end of 2018

2017 – 2018

Commercial Stage Biopharmaceutical Company

2	4+	3+	3	450+	100%
approved precision medicines in AML	clinical-stage molecules	pivotal trials (IDH, PKR)	areas of research expertise	employees	committed to helping patients

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