



Third Quarter 2017 Financial Results

November 1, 2017



Agios Conference Call Participants

Prepared Remarks

Introduction

- KENDRA ADAMS, Sr. Director, Investor Relations

Business Highlights & 2017 Key Milestones

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

Clinical Development Activities

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

Third 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 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 and 2018; 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," "goal," "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



Key Priorities & Expected Milestones

IDH

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

PKR

- ✓ Finalize pivotal trial design for wholly owned AG-348 in PK deficiency in 3Q 2017
- Continue to demonstrate leadership in PK deficiency
- Initiate pivotal program for AG-348 in PK deficiency in 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



Clinical Development Activities

Chris Bowden, M.D., Chief Medical Officer

ASH 2017 Data Presentations

IDH MUTANT INHIBITORS IN AML

- First data from the expansion phase of the ongoing Phase 1 study of ivosidenib in R/R AML
- First data from the ongoing Phase 1 frontline combo study of ivosidenib or enasidenib with 7+3 in newly diagnosed AML
- First data from the ongoing Phase 1/2 frontline combo study of ivosidenib or enasidenib with VIDAZA® in newly diagnosed AML

PKR ACTIVATOR

- Updated data from AG-348 Phase 2 DRIVE PK study in PK deficiency
- Updated data from PK Deficiency Natural History Study conducted with Boston Children's Hospital

Abstracts to go online today at 9a.m. ET

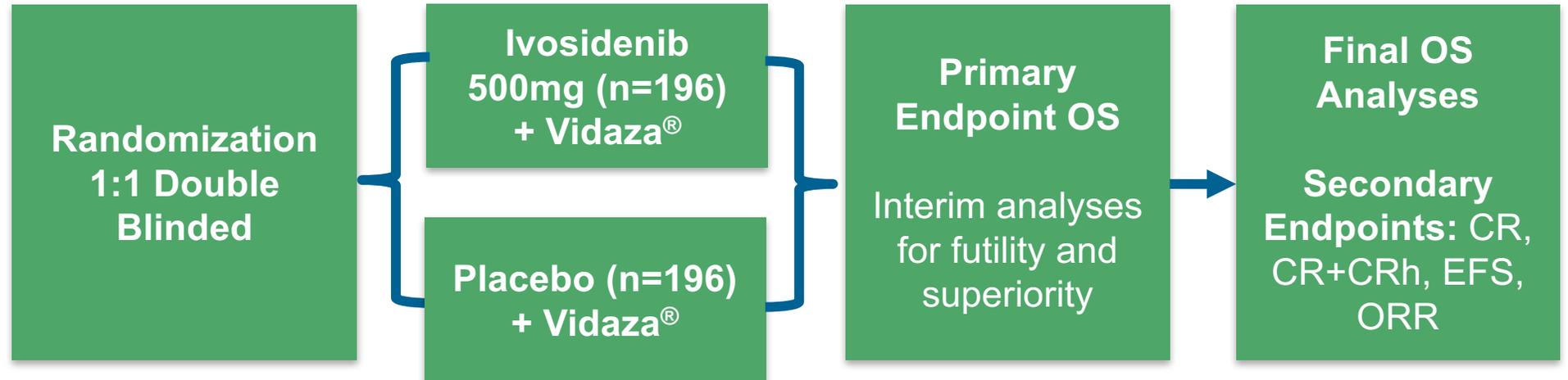


Advancing Ivosidenib into Frontline Setting



**Global Phase 3
Frontline
IC-Ineligible
IDH1m AML**

*Expect to complete
enrollment in 2021*



**Data from Phase 1 combo trials of
ivosidenib or enasidenib with 7+3 or
Vidaza® to be presented at ASH**

**Phase 3 combo study with
ivosidenib and 7+3 planned**

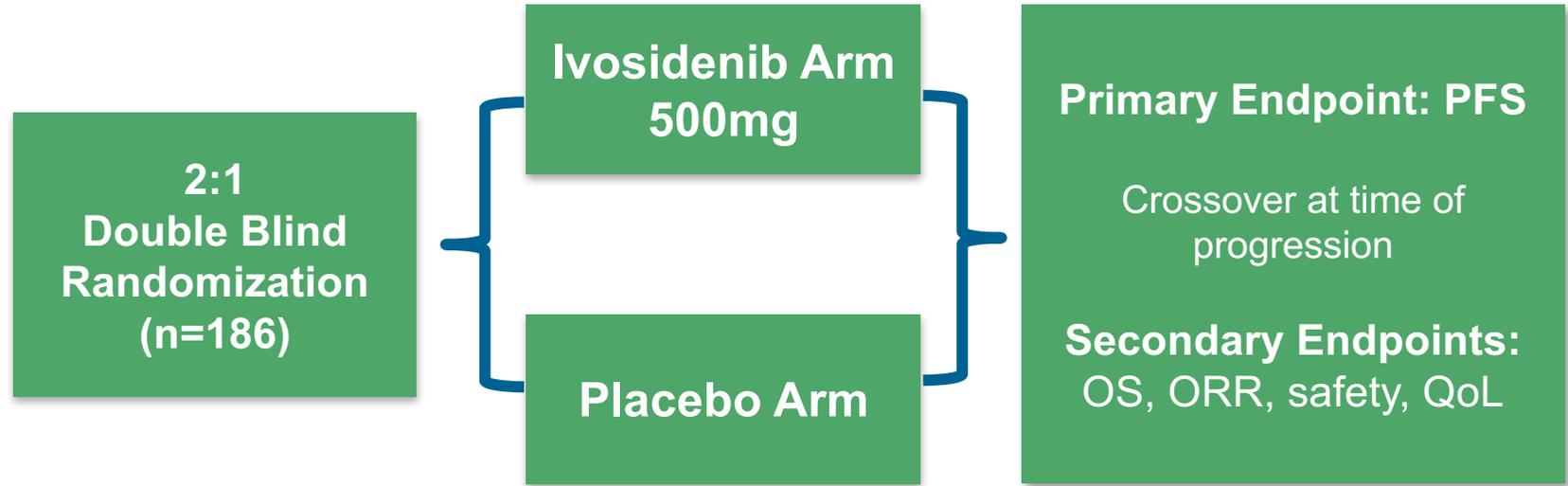


Registration-Enabling Phase 3 Cholangiocarcinoma Study



Global Phase 3 Previously Treated Advanced IDH1m Cholangiocarcinoma

Expect to complete enrollment in 2019



Ivosidenib Translational Data Presented at AACR-NCI-EORTC

- Demonstrate that ivosidenib induces morphologic and molecular changes in a subset of IDH1m cholangiocarcinoma patients
- Treatment with ivosidenib appears to be associated with increased PFS



Two IDHm Inhibitors in Clinical Development for Glioma

IVOSIDENIB IDH1m Inhibitor

- Updated Phase 1 data to be presented at SNO
- Will focus on low grade glioma with longer follow-up and additional volumetric imaging data from last year's meeting

**Phase 1
Dose-Escalation
and Expansion**

AG-881 Brain Penetrant, Pan-IDHm Inhibitor

- First preclinical data presented at AACR-NCI-EORTC in Oct.
- Data demonstrated AG-881 can suppress 2-HG in brain tissue in animal models
- Presentation of Phase 1 data planned for 2018

**Phase 1
Dose-Escalation**

**Dose
Expansion**

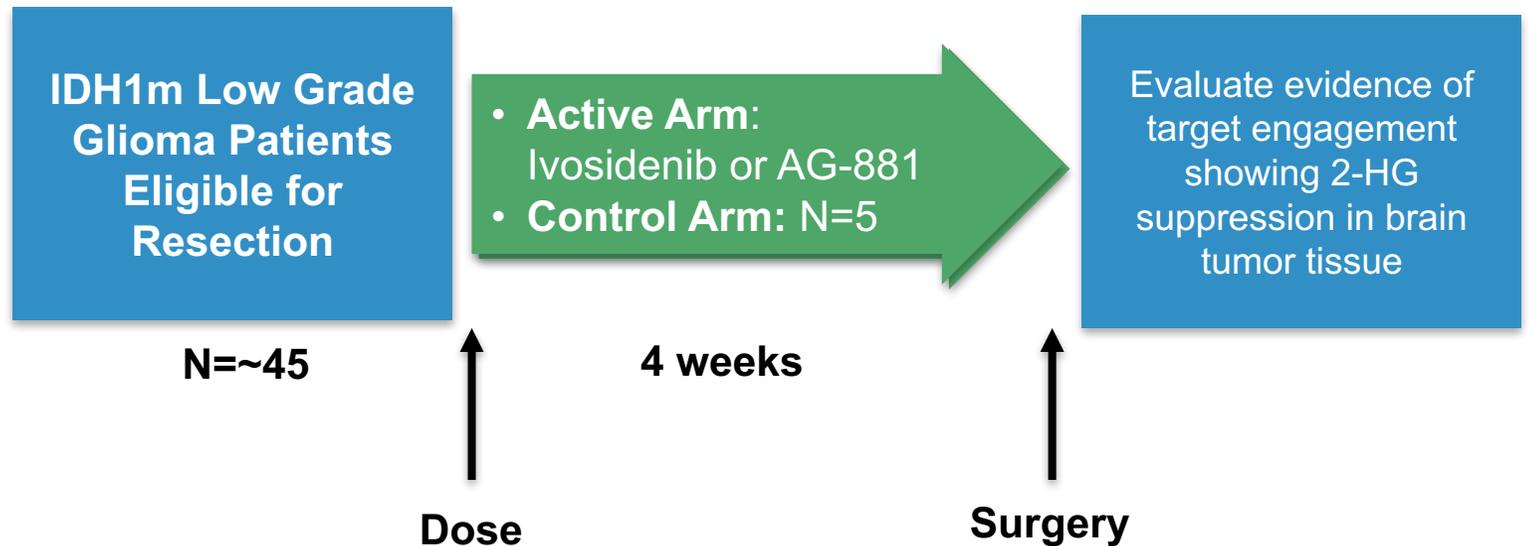
Regulatory input in 2018 will guide design of glioma pivotal trial



Design of Glioma Perioperative Study with Ivosidenib and AG-881

Study Objectives:

- Determine amount of drug penetration in the brain
- Confirm magnitude of IDH target engagement as measured by 2HG levels in brain tumor tissue
- Assess impact of IDH inhibition on differentiation and epigenetic profiles in tumor tissue
- Assess the safety of both molecules

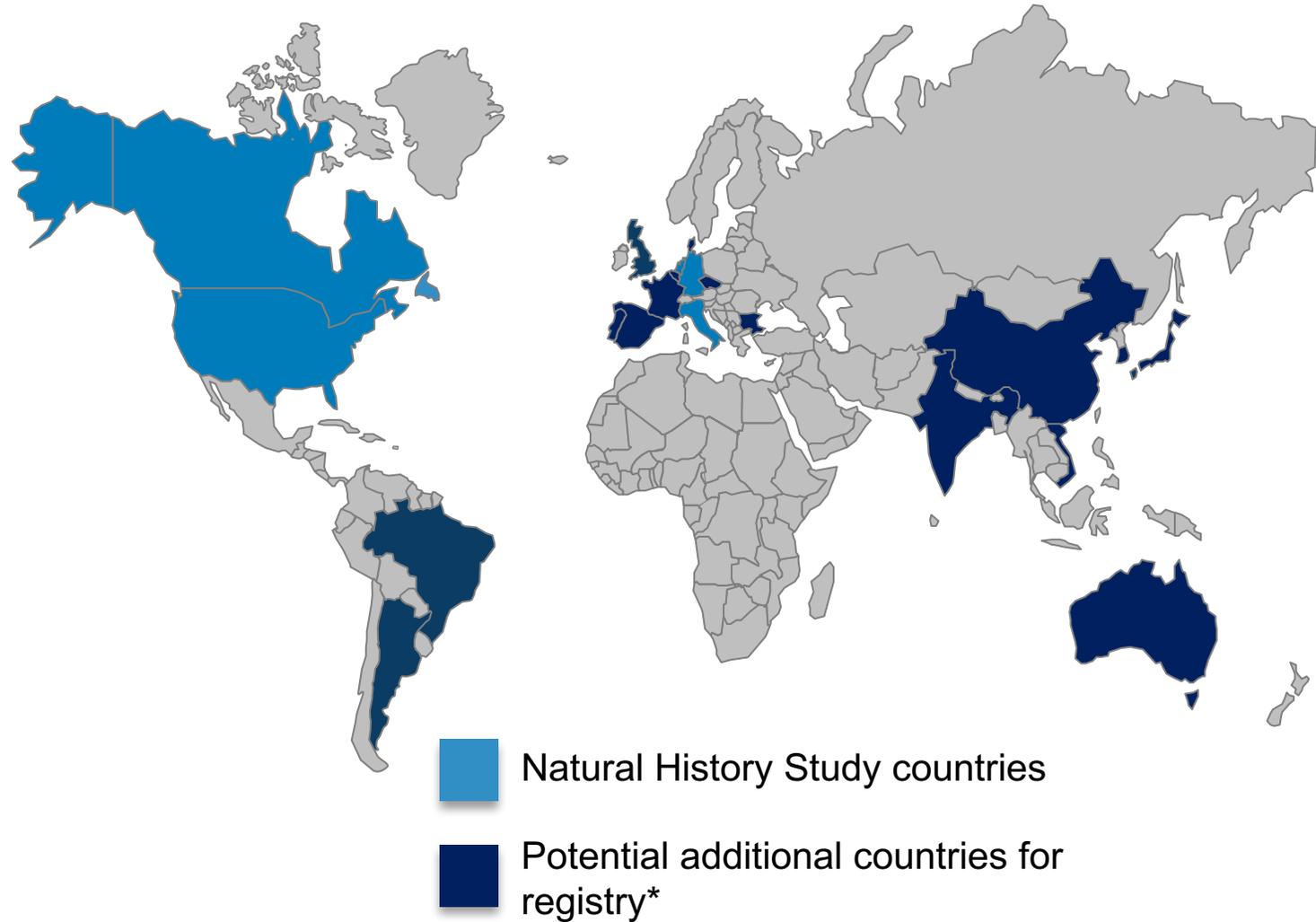


Plan to initiate perioperative study in 1H 2018



PK Deficiency Global Patient Registry

- Primary objective is to develop greater understanding of the longitudinal clinical implications of pyruvate kinase deficiency, including:
 - Natural history
 - Treatments and outcomes
 - Variability of clinical care
 - Disease burden
- Adult and pediatric patients eligible
 - Will be followed for at least 2 years
- Will include up to 20 countries, 60 sites



* This list is not exhaustive, and may change



Third Quarter 2017 Financial Results

Andrew Hirsch, Chief Financial Officer



Third Quarter 2017 Financial Results

Balance Sheet	September 30, 2017	December 31, 2016	September 30, 2016
Cash, Cash Equivalents & Marketable Securities	\$642M	\$574M	\$623M
Total Assets	\$687M	\$619M	\$672M

Statement of Operations	Three Months Ended September 30, 2017	Three Months Ended December 31, 2016	Three Months Ended September 30, 2016
Total Revenue	\$11M	\$23M	\$9M
Research & Development Expense	\$73M	\$65M	\$61M
General & Administrative Expense	\$17M	\$15M	\$12M

The R&D expenses reported for the three months ended September 30, 2017, December 31, 2016 and September, 2016 are reported net of cost reimbursements of \$1 million, \$1 million and \$4 million, respectively.

