Second Quarter 2017 Financial Results

August 8, 2017

Agios Conference Call Participants

Prepared Remarks

Introduction

- RENEE LECK, Sr. Manager, Investor Relations

Business Highlights & 2017 Key Milestones

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

Clinical Development Progress

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

Second 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; 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

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Now Approved in IDH2m Relapsed/Refractory AML

IDHIFA®
(enasidenib) tablets

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 Progress

Chris Bowden, M.D., Chief Medical Officer

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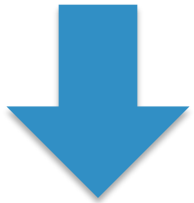
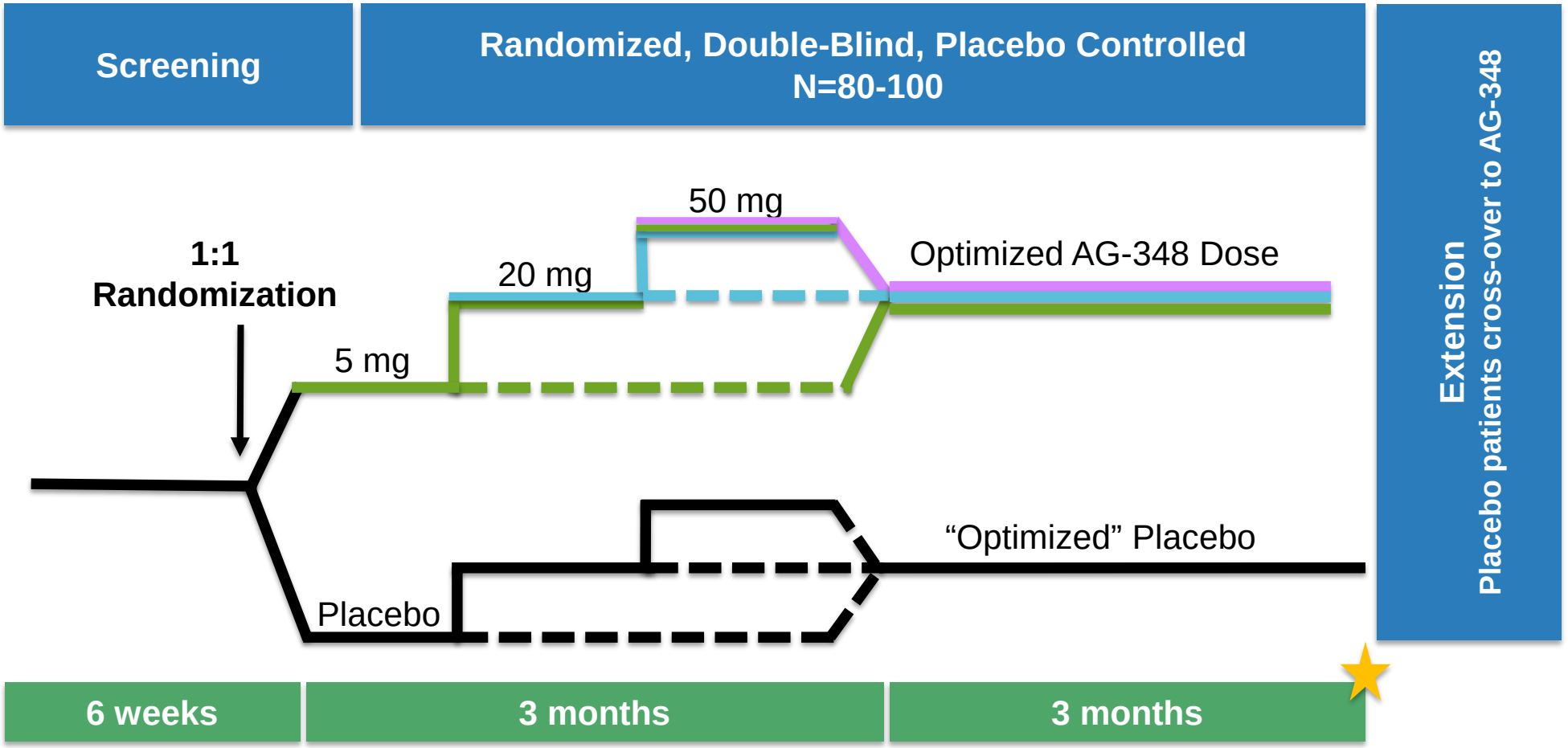
AG-348 Pivotal Program Design

Design Element	Non-Transfusion Dependent	Regularly Transfused
Patient Population	• Non-transfusion dependent adults • Baseline hemoglobin of ≤10.0 grams per deciliter • Excludes patients with two non-missense mutations and those homozygous for R479H	• Regularly transfused adults • Excludes patients with two non-missense mutations and those homozygous for R479H
Size	• 80-100 patients	• ~20 patients
Dose	• Dose titration up to optimal hemoglobin response (5, 20 or 50 mg twice daily)	• Dose titration up to optimal hemoglobin response (5, 20 or 50 mg twice daily)
Endpoints	• Hemoglobin response • Patient-reported outcomes (PRO)	• Reduction in transfusion burden • Patient-reported outcomes (PRO)
Control	• Placebo controlled	• Reduction of transfusion burden compared to patients' baseline



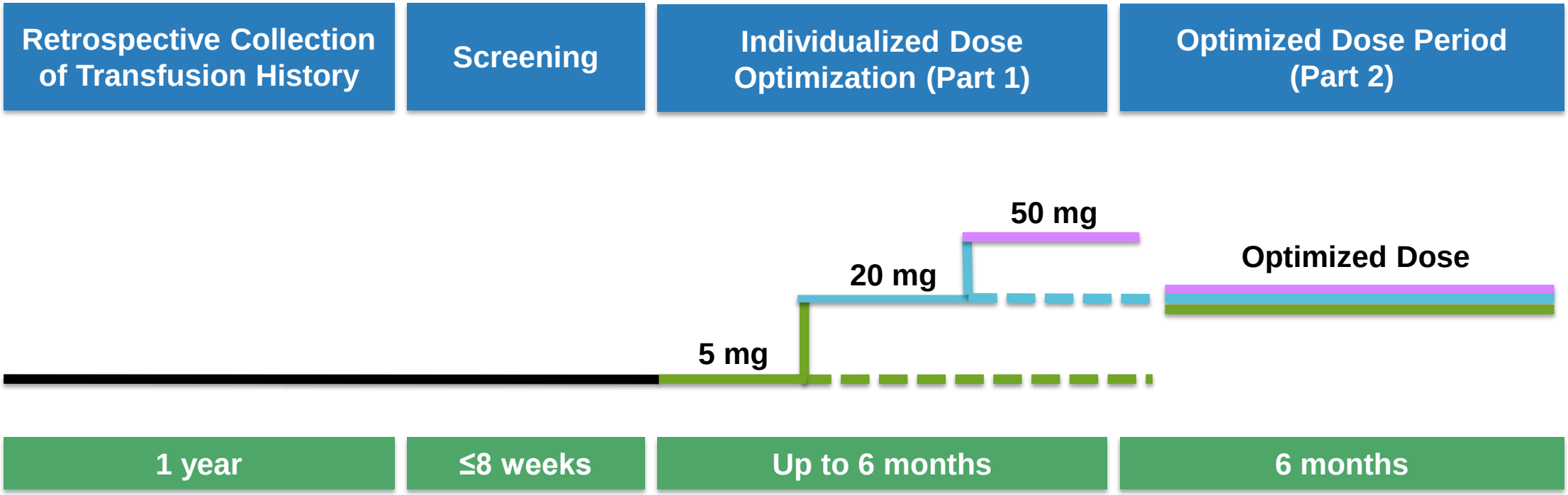
On track to initiate registrational program in 1H 2018

Trial Design for Non-Transfusion Dependent Patients



Primary Efficacy Endpoint: Proportion of patients who achieve at least a 1.5 g/dL increase in hemoglobin sustained over multiple visits

Trial Design for Regularly Transfused Patients

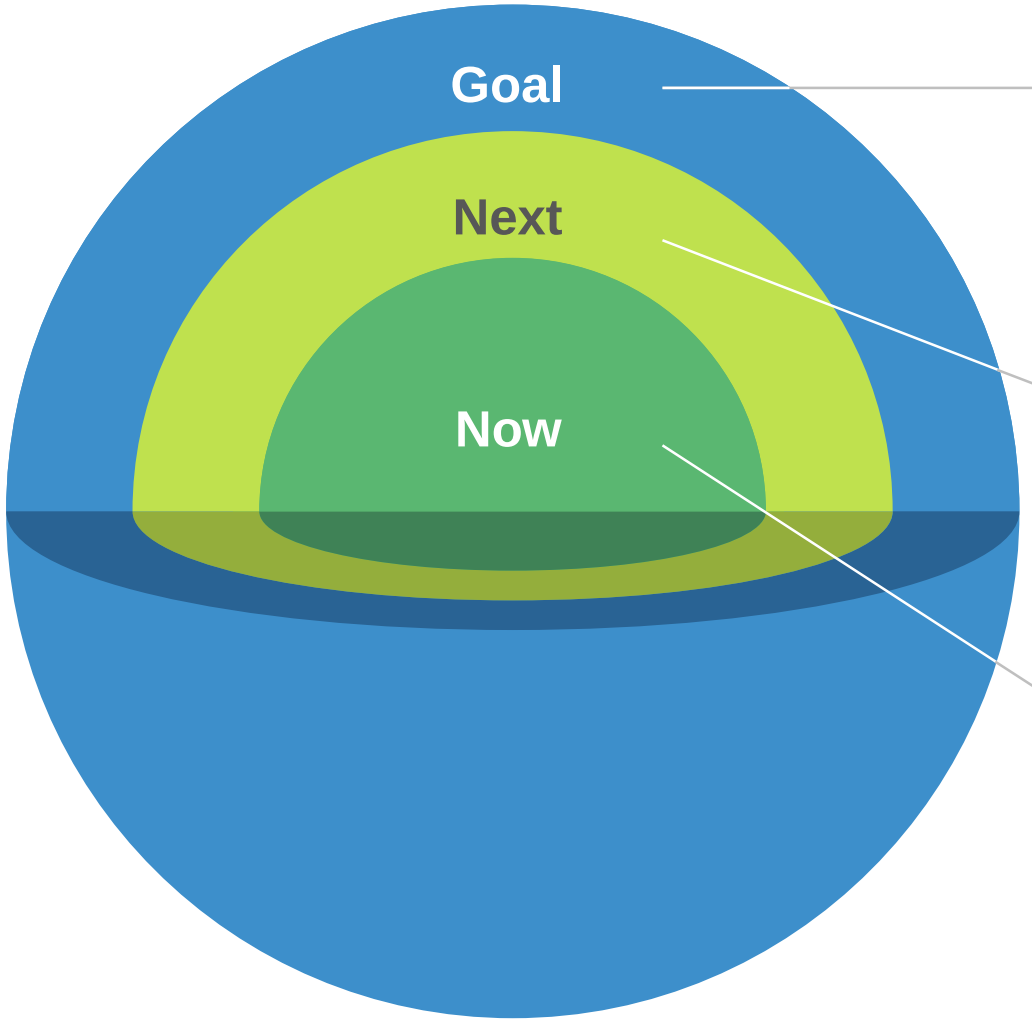


Approximately 20 regularly transfused patients who have required a minimum of 6 transfusions over the year preceding enrollment

Primary Endpoint: Reduction in transfusion burden over a 6 month period compared to the patient's transfusion history

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

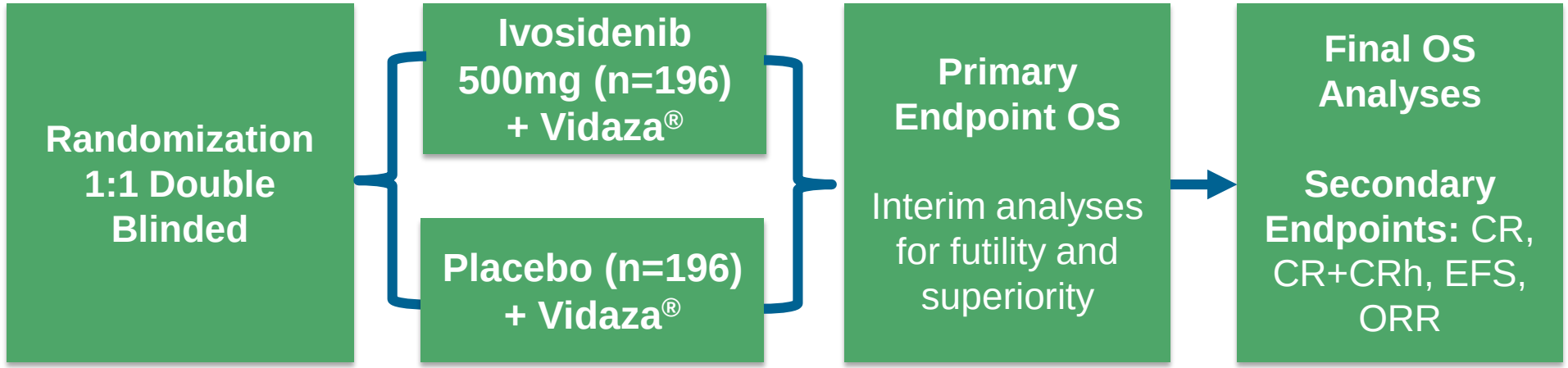
Relapsed/Refractory AML

- IDHIFA® approved in IDH2m R/R AML
- Ivosidenib NDA filing by YE
- Ivosidenib Phase 1 expansion data submitted for presentation at ASH

Advancing Ivosidenib into Frontline Setting

AGIOS study for IDH1 mutation in acute myeloid LEukemia

Global Phase 3 Frontline IC-Ineligible IDH1m AML

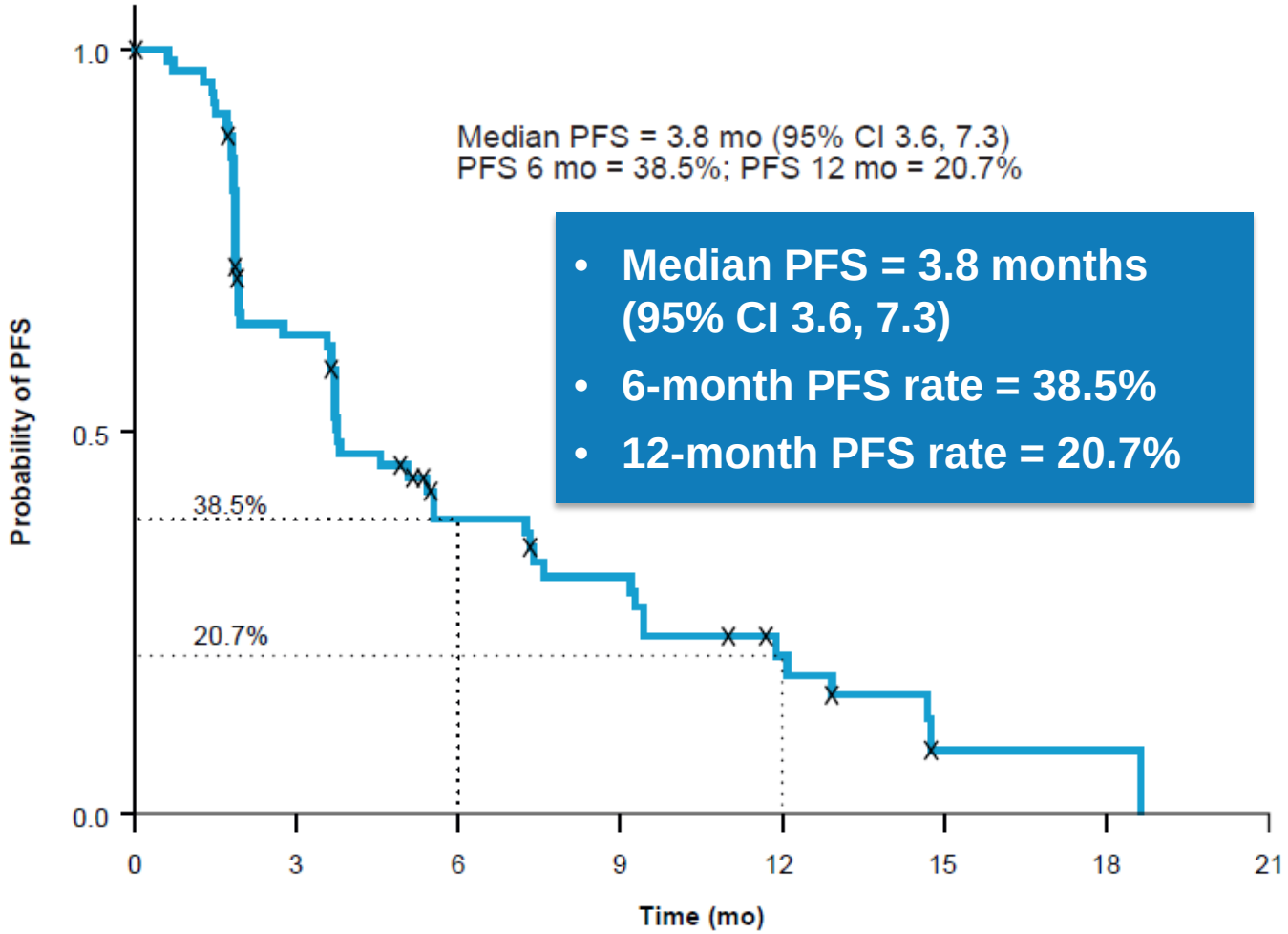


Initiated in June 2017

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

Data from Phase 1 combo trial of IDHIFA® or Ivosidenib with 7+3 submitted for presentation at ASH

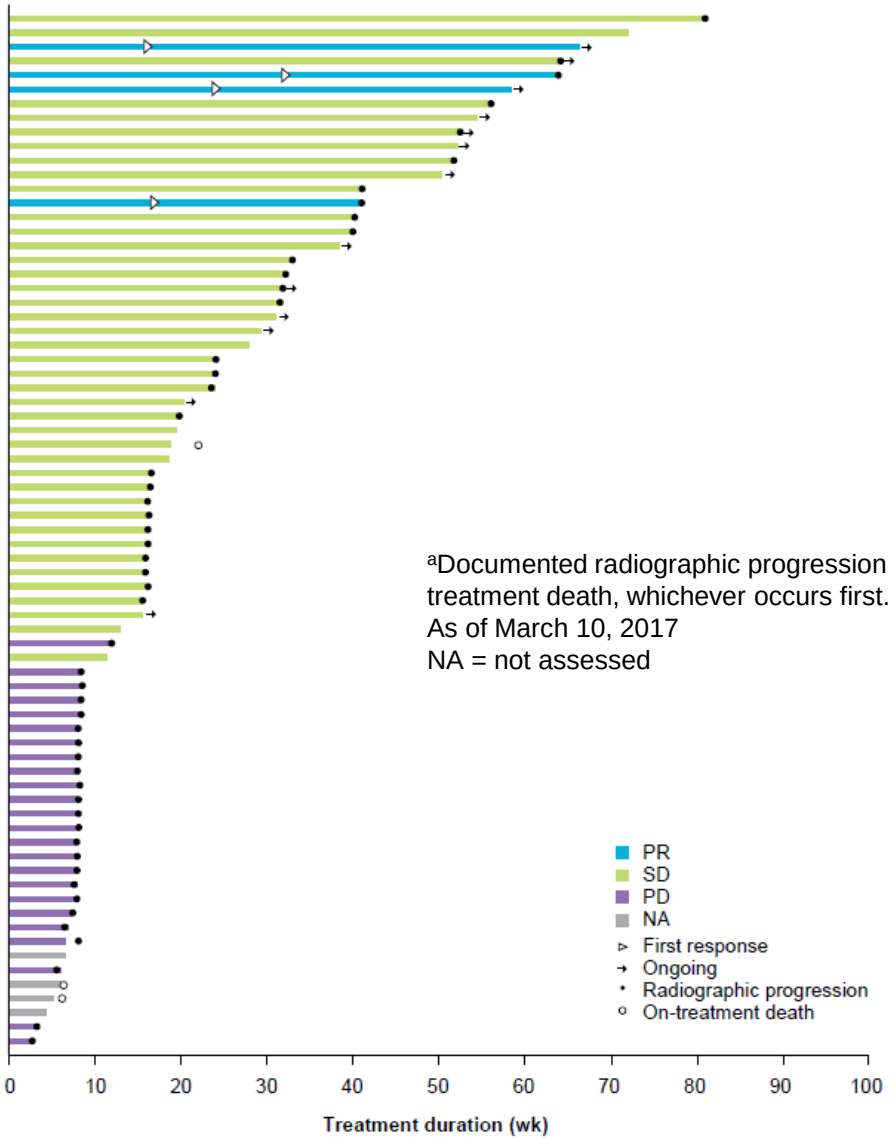
Ivosidenib Phase 1 Cholangio Data at ASCO – PFS & Duration Data



- **Median PFS = 3.8 months (95% CI 3.6, 7.3)**
- **6-month PFS rate = 38.5%**
- **12-month PFS rate = 20.7%**

18 of 73 (25%) censored. As of March 10, 2017
Median 2 prior therapies (range 1–5)

Cholangiocarcinoma Patients (N=74)



[a]Documented radiographic progression or on-treatment death, whichever occurs first.
As of March 10, 2017
NA = not assessed

Data presented at ASCO 2017

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

Second Quarter 2017 Financial Results

Andrew Hirsch, Chief Financial Officer

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Second Quarter 2017 Financial Results

Balance Sheet	June 30, 2017	December 31, 2016	June 30, 2016
Cash, Cash Equivalents & Marketable Securities	$716M	$574M	$512M
Total Assets	$761M	$619M	$558M

Statement of Operations	Three Months Ended June 30, 2017	Three Months Ended December 31, 2016	Three Months Ended June 30, 2016
Collaboration Revenue	$11M	$23M	$7M
Research & Development Expense	$80M	$65M	$51M
General & Administrative Expense	$16M	$15M	$13M

The R&D expenses reported for the three months ended June 30, 2017, December 31, 2016 and June 30, 2016 are reported net of cost reimbursements of $2 million, $1 million and $6 million, respectively.

Our Pipeline

CLINICAL PROGRAMS	INDICATION	DRUG DISCOVERY	EARLY STAGE CLINICAL DEVELOPMENT	LATE STAGE CLINICAL DEVELOPMENT	APPROVED	PRIMARY COMMERCIAL RIGHTS
IDHIFA® ***Enasidenib*** (IDH2m Inhibitor)	R/R AML				●	agios Celgene
	Frontline AML			●		Agios U.S. Co-promotion and Royalty
Ivosidenib (IDH1m Inhibitor)	R/R AML			●		agios
	Frontline AML			●		
	Cholangio			●		
	Glioma		●			
AG-881 (pan-IDHm Inhibitor)	Glioma		●			Joint Worldwide Collaboration agios Celgene
AG-348 (PK (R) Activator)	PK Deficiency			●		agios
RESEARCH PROGRAMS						
MTAP Program		●				agios Celgene Joint Worldwide Collaboration
CM Research Programs		●				agios
RGD Research Programs		●				agios
Metabolic IO Research Programs		●				agios Celgene Joint Worldwide Collaboration