



# Full Year 2017 Financial Results

February 14, 2018



# Agios Conference Call Participants

## Prepared Remarks

### Introduction

- KENDRA ADAMS, Sr. Director, Investor Relations

### 2018 Vision & Key Milestones

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

### Clinical Development Activities

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

### Fourth Quarter and Full Year 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®, ivosidenib, AG-881, AG-348 and AG-270; the potential benefits of Agios' product candidates; its key milestones for 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.



# 2018 Vision & Key Milestones

*David Schenkein, M.D., Chief Executive Officer*

# Setting the Stage for Building Long-Term Value

## 2017 Accomplishments Demonstrate Strength of R&D Engine

First drug approved (IDHIFA®) with a second close behind in R/R AML

Expansion opportunities for ivosidenib in frontline AML and solid tumors underway

First disease modifying treatment for PK deficiency ready for pivotal trials

Research productivity stronger than ever with 6<sup>th</sup> IND submission

Labs opened in 2009

2018 & Beyond

At least 3 approved medicines

Multibillion dollar commercial opportunity across clinical portfolio

Research engine primed to deliver multiple INDs over next 24 months



# Agios' Scientific Platform Demonstrates Remarkable, Reproducible Productivity

## DISCOVERY

**\$50-60M**

INVESTED IN DRUG DISCOVERY ANNUALLY



## SCIENCE



**40+**

PEER-REVIEWED PUBLICATIONS

## CULTURE



**400+** EMPLOYEES

**1** VISION



**10+**

CLINICAL TRIALS IN

**6** DISEASES

**1,000+**



PATIENTS TREATED IN CLINICAL TRIALS

**6**



INDs

**1<sup>ST</sup>**

MEDICINE APPROVED



**+**

**2<sup>ND</sup>**

NDA SUBMITTED



**+**

**3**

ADDITIONAL COMPOUNDS IN CLINICAL DEVELOPMENT



IN 4 YEARS SINCE FIRST PATIENT DOSED



# 2018 Key Milestones

## CANCER

- Secure approval and commercialize ivosidenib for IDH1m R/R AML in the U.S. in Q3 2018
- Submit ivosidenib European MAA in IDH1m R/R AML in Q4 2018
- Initiate Phase 3 frontline AML trial combining ivosidenib or enasidenib with 7+3 in Q4 2018
- Initiate glioma perioperative study with ivosidenib and AG-881 in Q1 2018
- Initiate AG-270 Phase 1 dose-escalation trial in Q1 2018

## RARE GENETIC DISEASES

- Initiate the ACTIVATE-T pivotal trial of AG-348 in regularly transfused PK deficiency patients in Q1 2018
- Initiate the ACTIVATE pivotal trial of AG-348 in PK deficiency patients not regularly transfused in Q2 2018
- Initiate global PEAK registry for adult and pediatric PK deficiency patients in Q1 2018
- Initiate AG-348 Phase 2 proof-of-concept trial in thalassemia in Q4 2018

## RESEARCH

- Submit IND for DHODH in Q4 2018
- Advance next wave of research in three areas of expertise: cancer metabolism, rare genetic diseases and metabolic immuno-oncology



# Clinical Development Activities

*Chris Bowden, M.D., Chief Medical Officer*



# Anticipated Key 2018 Data Presentations

**Updated data from expansion phase of the Phase 1 study of ivosidenib in IDH1m R/R AML submitted to ASCO**

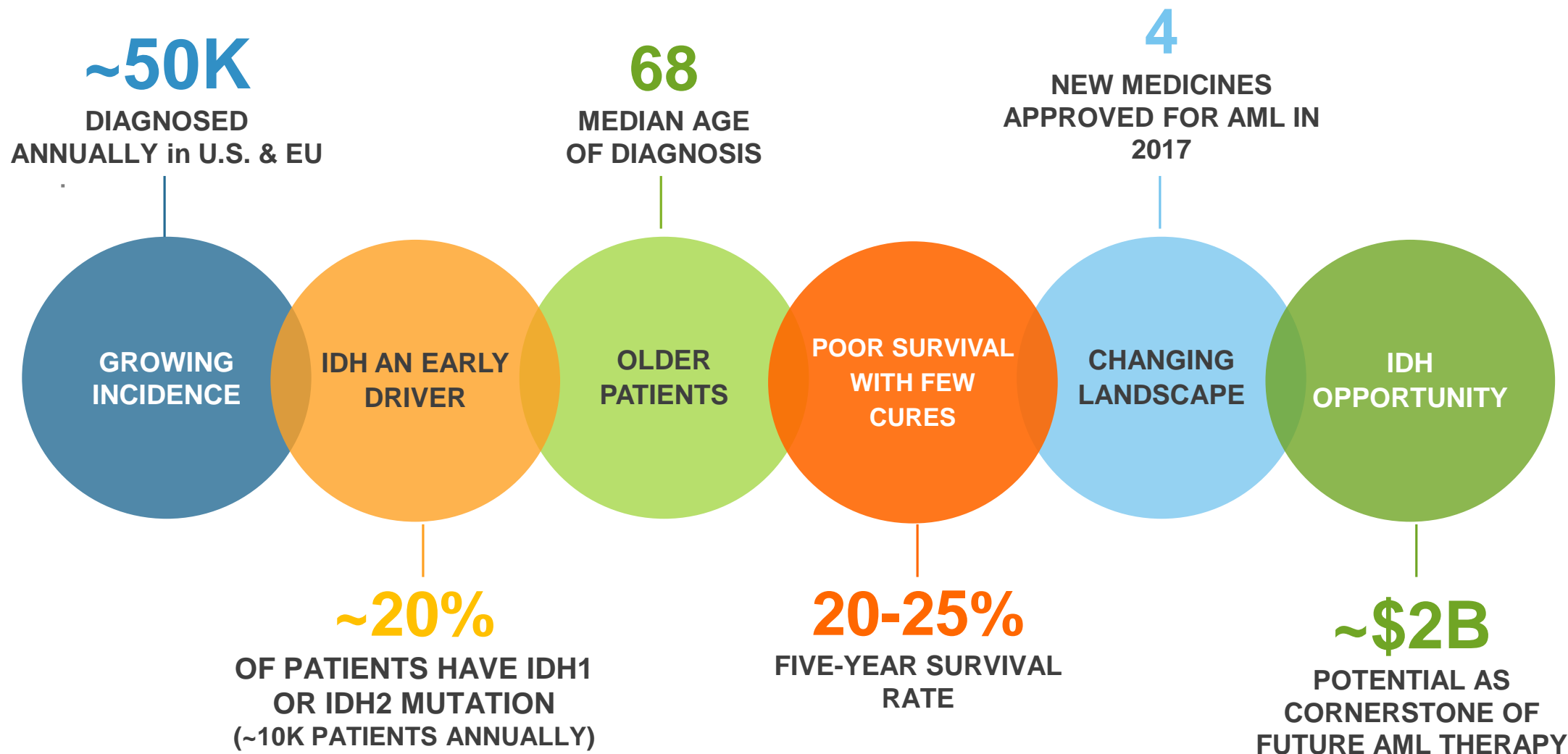
**Updated data from the Phase 1/2 combo trial of enasidenib or ivosidenib with VIDAZA® in newly diagnosed AML submitted to ASCO**

**First clinical data from the Phase 1 study of AG-881 in advanced IDHm positive solid tumors, including glioma, submitted to ASCO**

**Updated data from the Phase 1 combo trial of enasidenib or ivosidenib with 7+3 intensive chemo in newly diagnosed AML to be submitted to ASH**



# AML Landscape on the Brink of a Therapeutic Tidal Shift



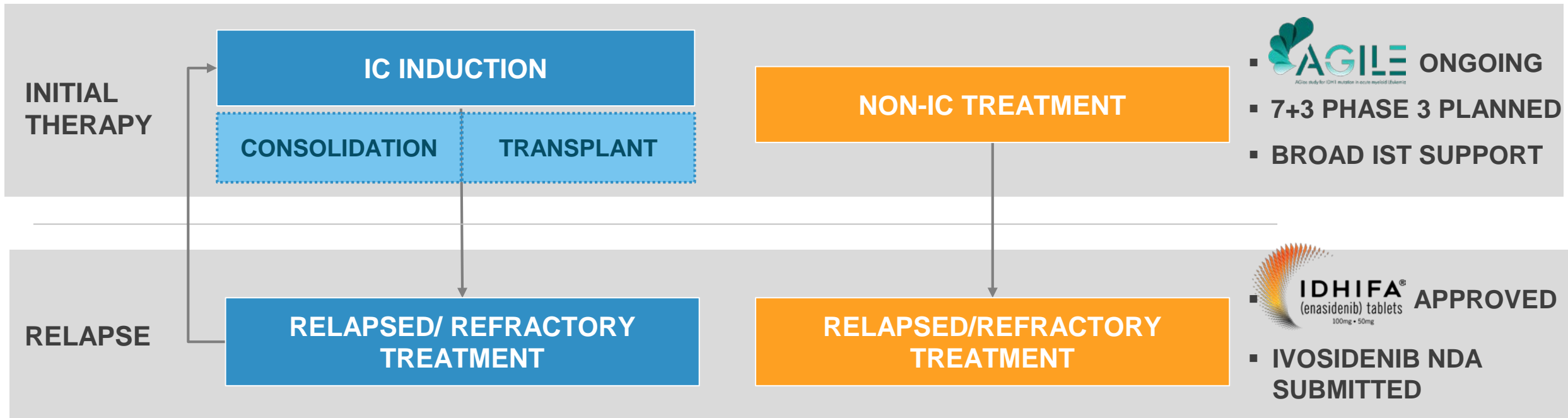
Sources: SEER. Cancer Stat Facts: AML 2015 and Epiphany EPIC oncology numbers; American Cancer Society. AML 2017.; Visser et. Al. Incidence, survival and prevalence of myeloid malignancies in Europe. Eur J Cancer. 2012 Nov;48(17):3257-66; Thomas ED, N Engl J Med. 1979 Mayer, N Engl J Med. 1994, Fernandez H, N Engl J Med, 2009; Kumar C. Genetic Abnormalities and Challenges in the Treatment of Acute Myeloid Leukemia. Genes Cancer. 2011; 2:95-107; AML O/S: Klepin, et al, JCO, 32, 2014



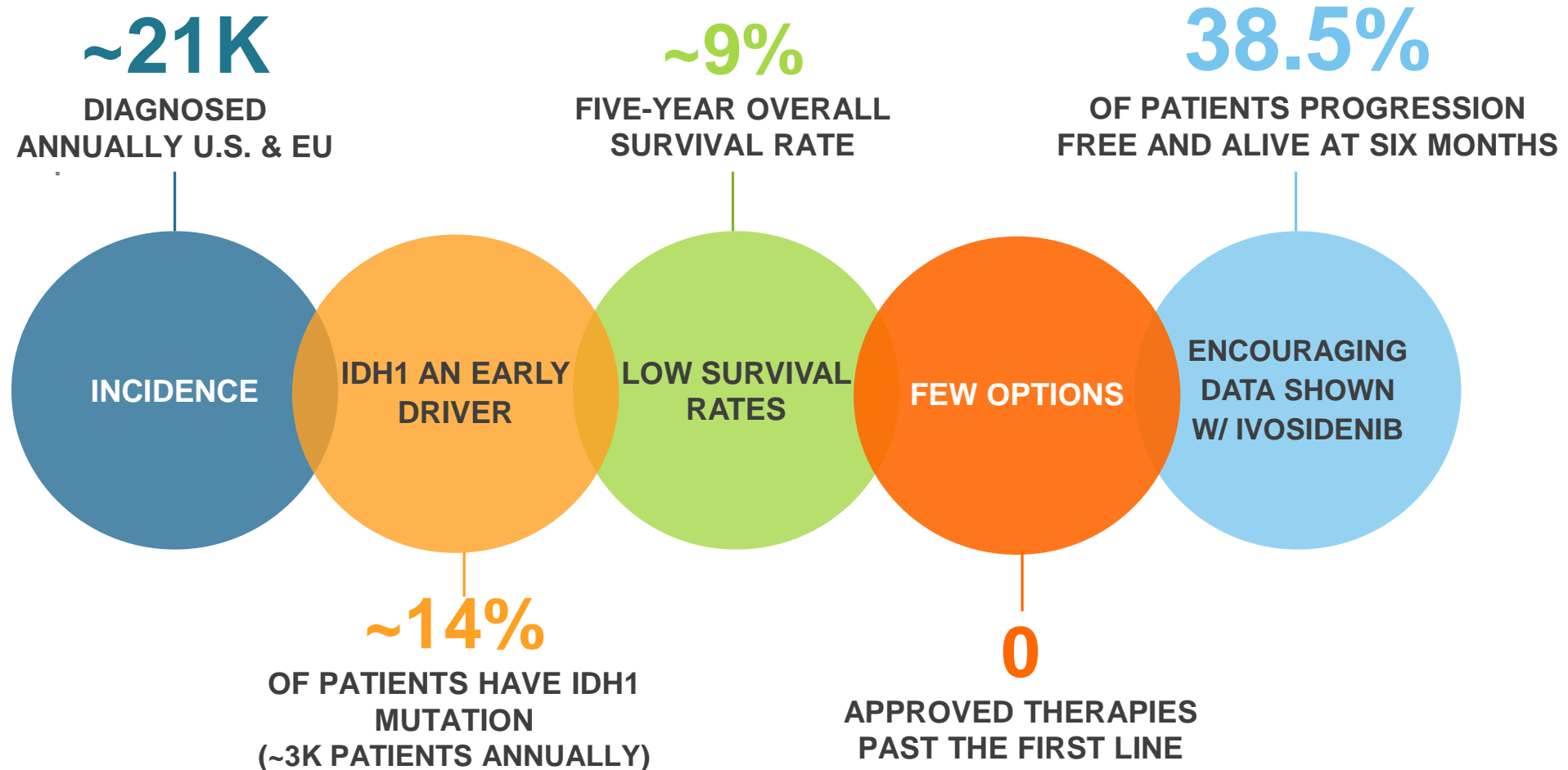
# Clinical Development of IDHm Inhibitors Spans All Treatment Lines to Become Cornerstone of Therapy

 **INTENSIVE CHEMO (IC)**  
~60-70% of AML Patients

 **NON-IC TREATMENT**  
~30-40% of AML Patients



# Opportunity for Ivosidenib in Cholangiocarcinoma: Devastating Disease with No Approved Targeted Therapies

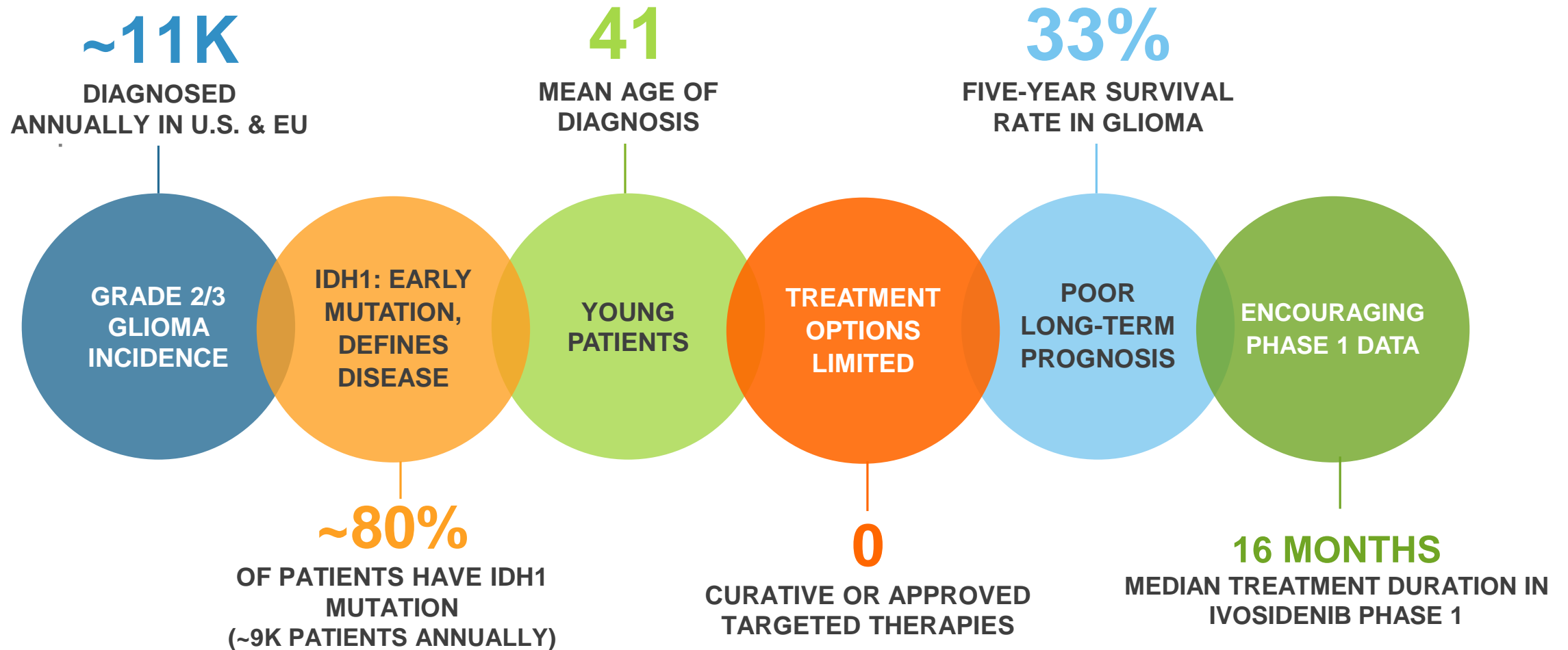


Sources: CDC National Program of Cancer Registries (NPCR); Epiphany Partners Epic Oncology; Decision Resources; Market Research; Borger DR et al. Oncologist 2012;17:72-9.; Kipp BR et al. Hum Pathol 2012;43:1552-8.; Goyal L et al. Oncologist 2015;20:1019-27.

**Global ClarIDHy Phase 3 in previously treated advanced IDH1m cholangiocarcinoma ongoing;  
Enrollment expected to complete in 2019**



# Low Grade Glioma: High Unmet Need Not Adequately Addressed by Chemotherapy or Radiation



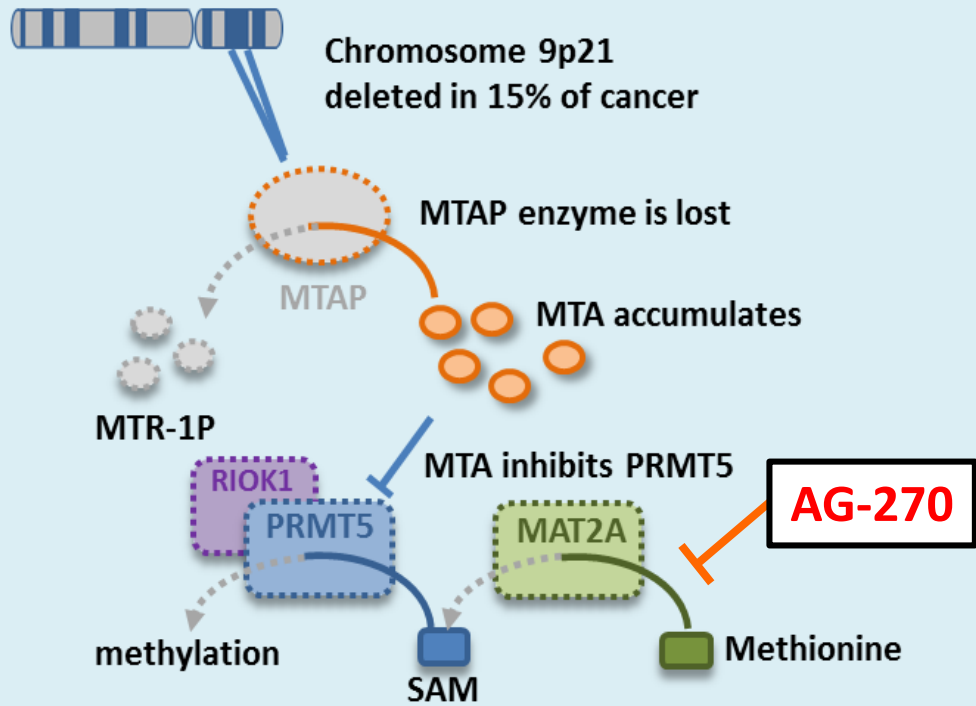
Sources: CDC National Program of Cancer Registries (NPCR); SEER. Cancer Stat Facts; Market research; CBTRUS (Central Brain Tumor Registry in the US); Neurosurg Focus. 2015 Jan; 38(1): E6.

**Perioperative study on track to start Q1 2018;  
Regulatory feedback to inform pivotal path**



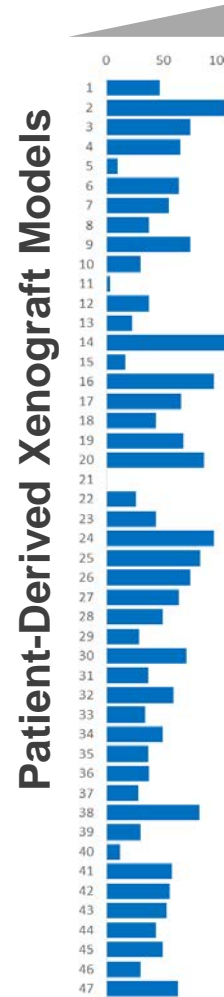
# AG-270 Active in Wide Variety of MTAP-deleted Cancer Models

## MTAP Deleted Cancer Cell

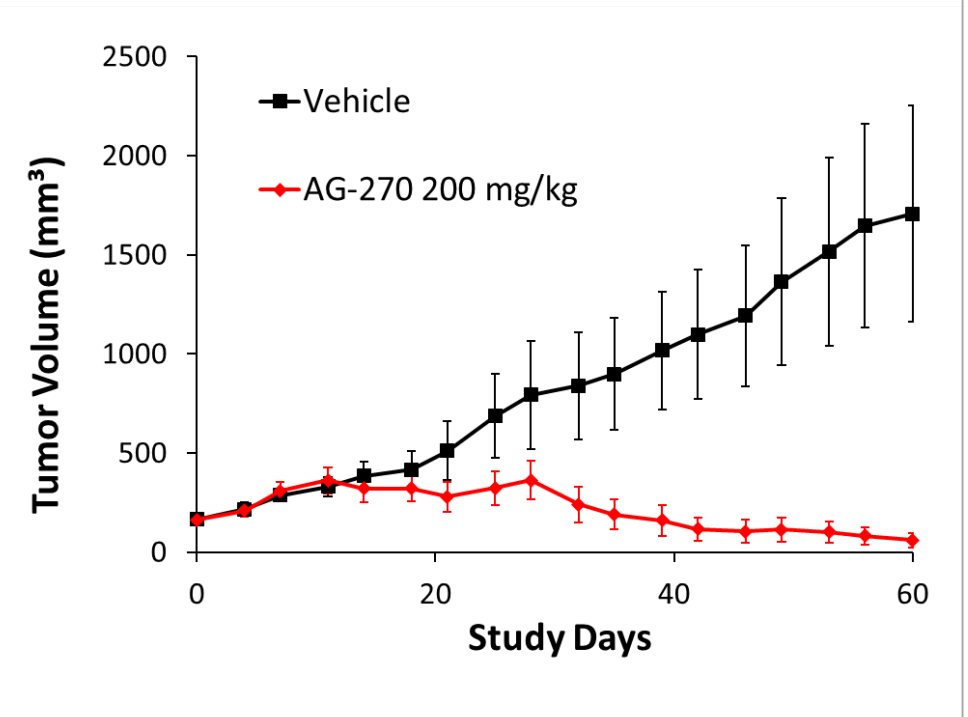


Agios publication: Marjon et al. Cell Reports 2016

Efficacy  
(%Tumor Growth Inhibition)



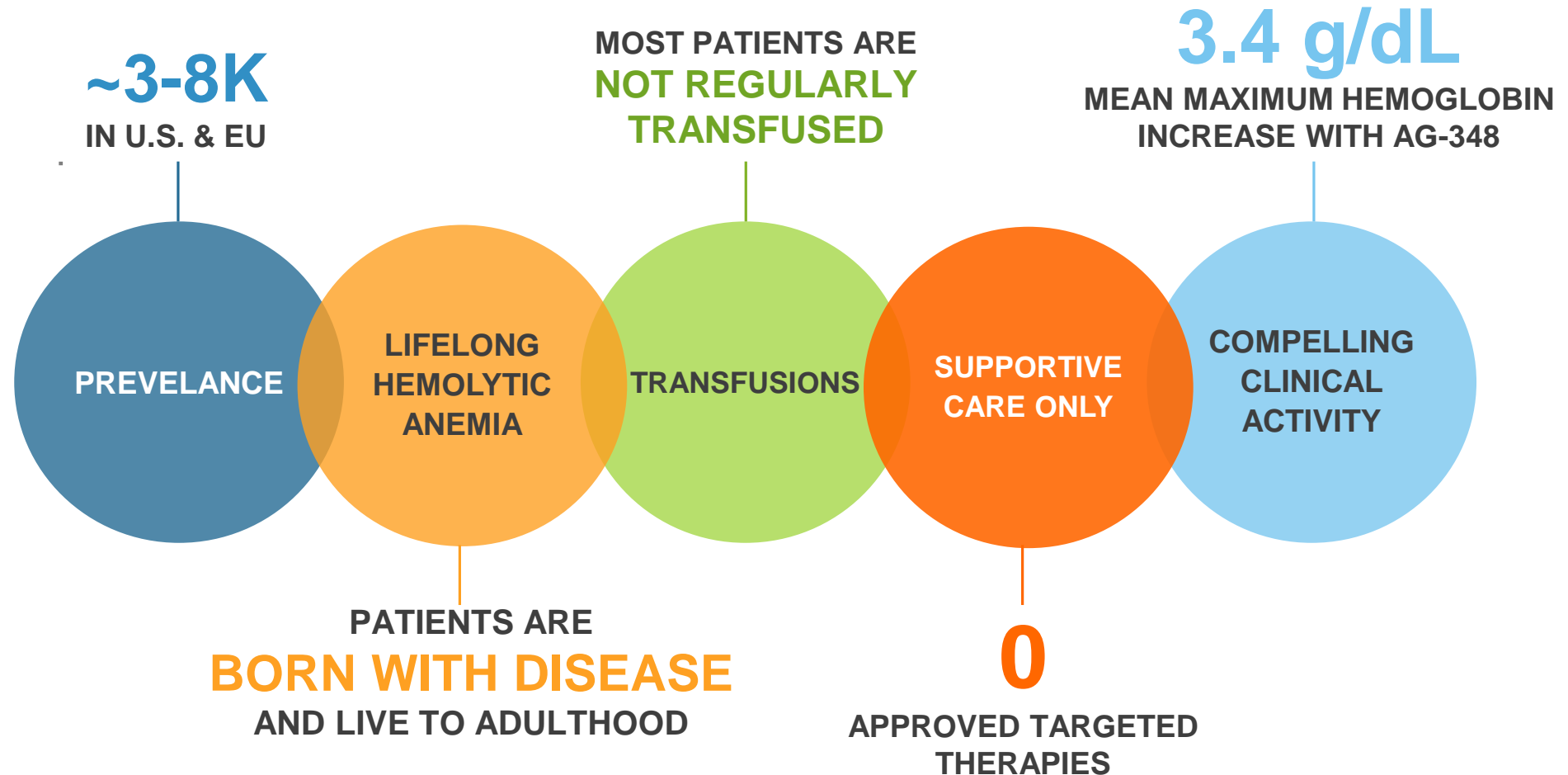
## MTAP-null NSCLC PDX model



First-in-human Phase 1 dose-escalation clinical trial to start Q1 2018



# Opportunity for AG-348 to be the First Disease-Modifying Treatment for PK Deficiency



Sources: Estimated prevalence range from ~1:20K to ~1:485K Grace R et al. *Am J Hematol* 2015;90(9):825-30; <sup>1</sup>Mohrenweiser HW *PNAS* 1981;78(8):5046-50; <sup>2</sup>Carey PJ et al. *Blood* 2000;96(12):4005-6; <sup>3</sup>Beutler E & Gelbart T *Blood* 2000;95(11):3585-8; <sup>4</sup>deMedicis et al. *Hum Hered* 1992;42(3):179-83; data presented at ASH 2017

Pivotal trials ACTIVATE-T to initiate in Q1 2018 and ACTIVATE in Q2 2018



# Full Year 2017 Financial Results

*Andrew Hirsch, Chief Financial Officer*





# Full Year 2017 Financial Results

| Balance Sheet                                    | December 31, 2017 | December 31, 2016 |
|--|-------------------|-------------------|
| Cash, Cash Equivalents and Marketable Securities | \$567.8M          | \$573.6M          |
| Total Assets                                     | \$614.4M          | \$619.1M          |

| Statement of Operations            | December 31, 2017 | December 31, 2016 |
|------------------------------------|-------------------|-------------------|
| Total Revenue                      | \$43.0M           | \$69.9M           |
| Research & Development Expense (1) | \$292.7M          | \$220.2M          |
| General & Administrative Expense   | \$71.1M           | \$50.7M           |

1) The R&D expenses reported for the twelve months ended December 31, 2017 and December 31, 2016 are reported net of cost reimbursements of \$7.8 million and \$19.7 million, respectively.

~\$516M net proceeds from January follow-on offering;  
Extends cash runway through at least the end of 2020

