Agios at J.P. Morgan Healthcare Conference

January 8, 2018

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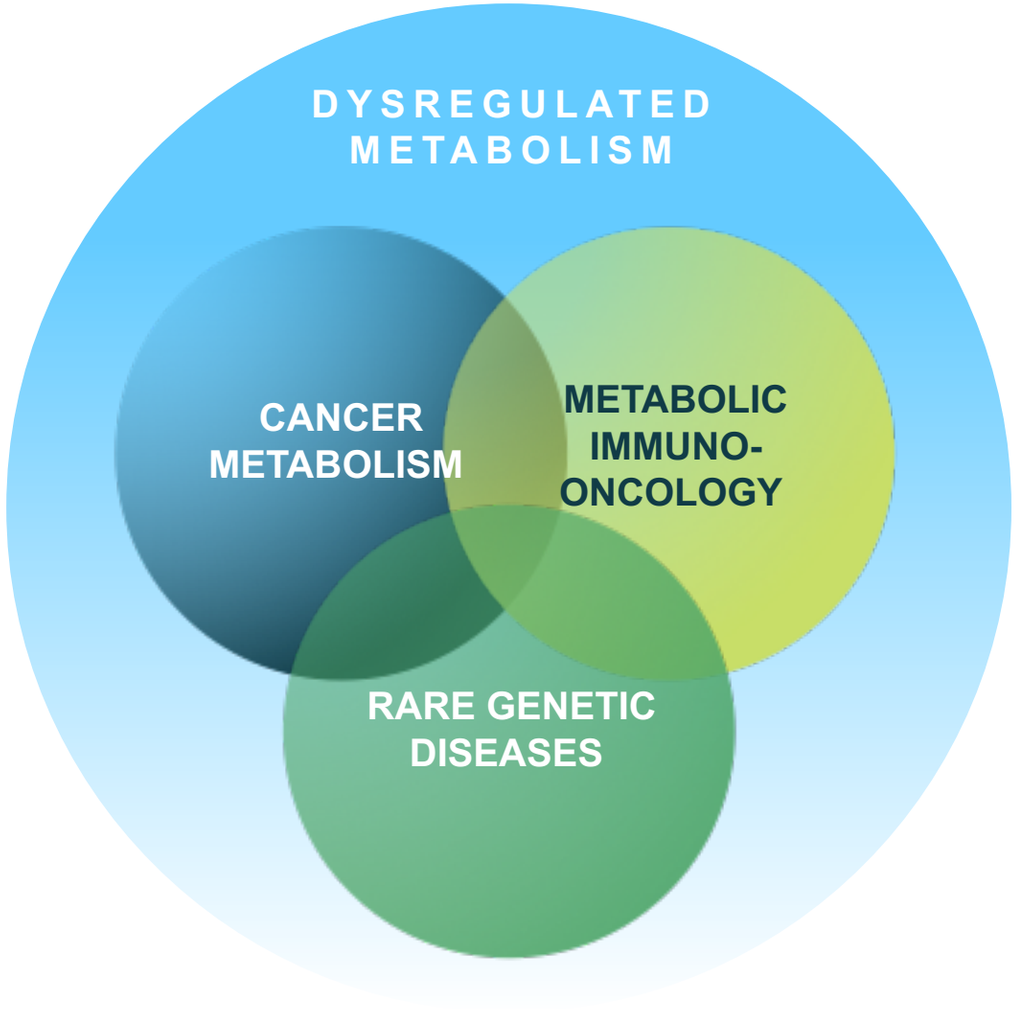
Chief Executive Officer, Agios

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 estimates regarding its balance of cash, cash equivalents and marketable securities for the year ended December 31, 2017; 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," "estimate," "expect," "intend," "milestone", "on track" "plan," "potential," 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.

Driven By a Clear Vision and Values



agios

Agios is passionately committed to applying our scientific leadership in the field of cellular metabolism to transform the lives of patients with cancer and rare genetic diseases.

Current Clinical Portfolio Has Potential to Benefit Large Number of Patients

ACUTE MYELOID LEUKEMIA	~10,000 IDHm Patients AML opportunity ~$2B
CHOLANGIO-CARCINOMA	~3,000 IDH1m Patients
LOW GRADE GLIOMA	~9,000 IDH1m Patients
PYRUVATE KINASE DEFICIENCY	~3,000 to ~8,000 Patients
MTAP-DELETED TUMORS	>100,000 MTAP Deletion Patients

Oncology patient numbers represent annual U.S. and EU incidence; PK deficiency represent U.S. and EU prevalence

Discovering Enasidenib Video

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

1ST MEDICINE APPROVED + **2ND** NDA SUBMITTED + **3** ADDITIONAL COMPOUNDS IN CLINICAL DEVELOPMENT

IN 4 YEARS SINCE FIRST PATIENT DOSED

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

Labs opened in 2009

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 6th IND submission

Labs opened in 2009

Setting the Stage for Building Long-Term Value

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2017 Accomplishments Demonstrate Strength of R&D Engine

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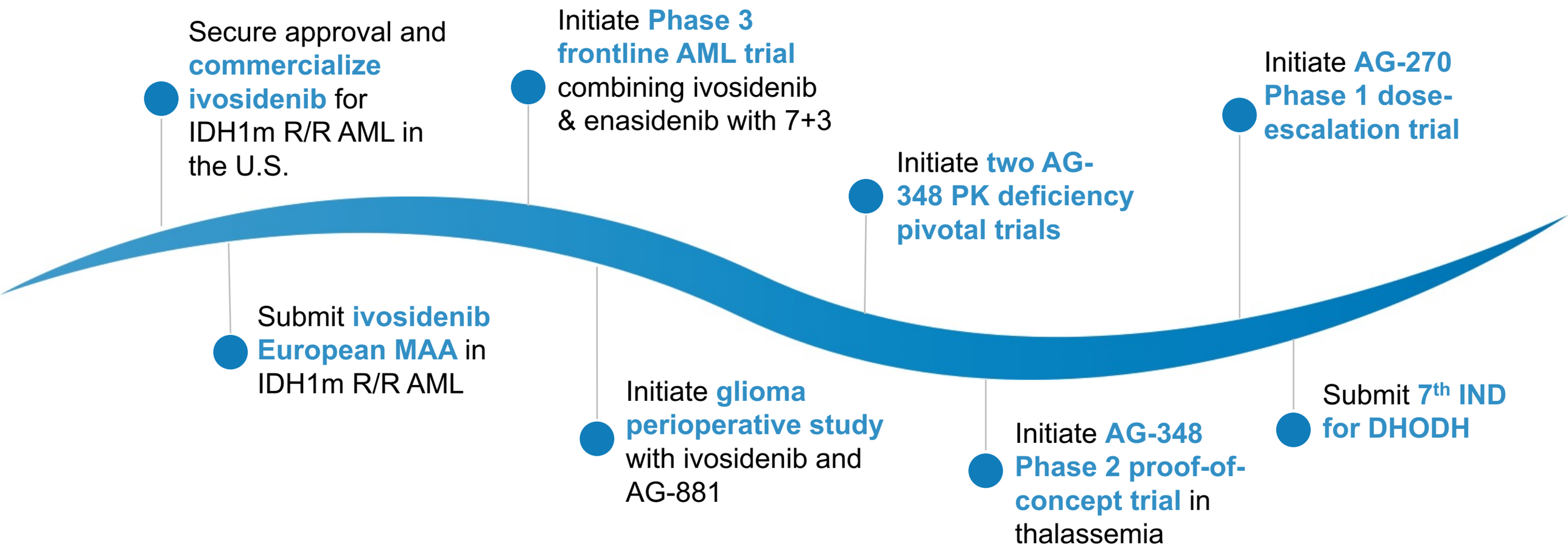
- Research productivity stronger than ever with 6th IND submission

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

2018 Key Milestones




CANCER
RARE GENETIC DISEASES
RESEARCH

CANCER

RARE GENETIC DISEASES

RESEARCH

Multiple Opportunities Across Hematologic and Solid Cancers Originating from Agios Research Platform

ACUTE MYELOID LEUKEMIA

- **IDH2m R/R**
  IDHIFA® Approved
- **IDH1m R/R**
  Ivosidenib NDA Submitted
- **IDH1m Frontline Non-IC**
  Ivosidenib + Aza Phase 3 (AGILE) Ongoing
- **IDHm Frontline IC-Eligible**
  Ivo/Ena + 7+3 Phase 3
  Q4 2018 Start
- **IDHm Frontline Non-IC**
  Ivo/Ena + Aza Phase 1 Ongoing
- **IDHm Frontline IC-Eligible**
  Ivo/Ena + 7+3 Phase 1 Ongoing

CHOLANGIOCARCINOMA

- **IDH1m R/R**
  Ivosidenib Phase 3 (ClarIDHY) Ongoing
- **IDH1m R/R**
  Ivosidenib Phase 1 Enrollment Complete

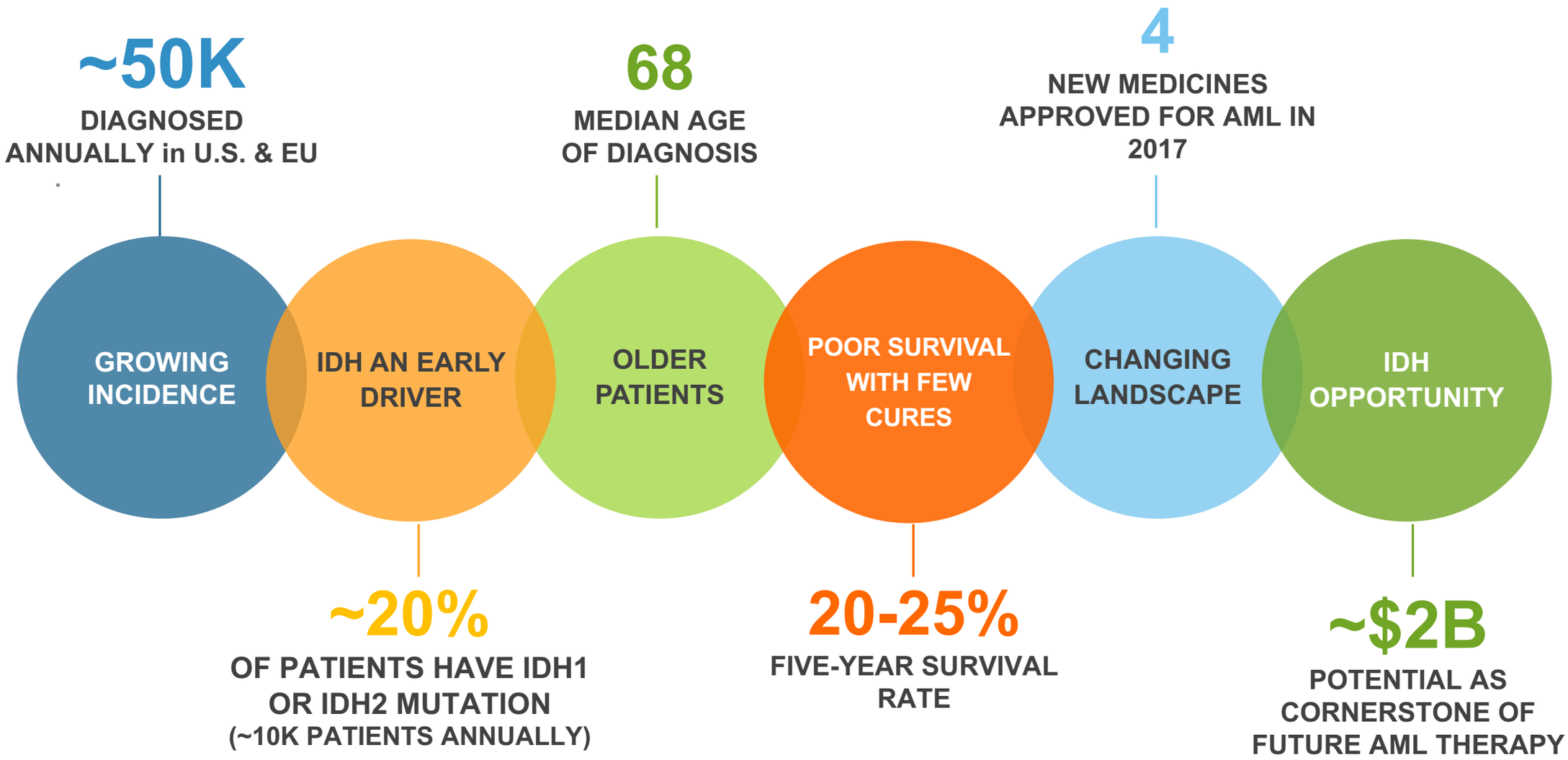
LOW GRADE GLIOMA

- **IDH1m**
  Ivosidenib & AG-881 Perioperative Study
  1H 2018 Start
- **IDH1m**
  Ivosidenib
  Phase 1 Enrollment Complete
- **IDH1m**
  AG-881
  Phase 1 Enrollment Complete

MTAP-DELETED TUMORS

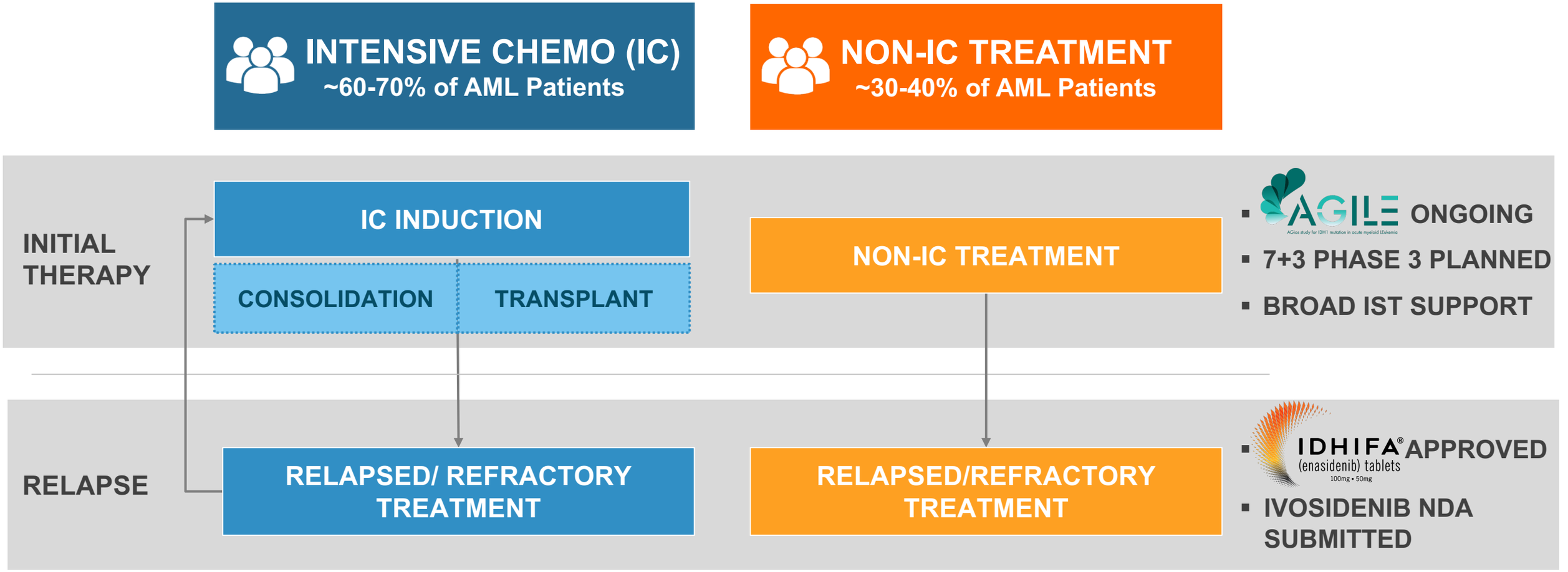
- **Multiple Tumor Types**
  AG-270 Phase 1 Study
  Q1 2018 Start

AML Landscape on the Brink of a Therapeutic Tidal Shift

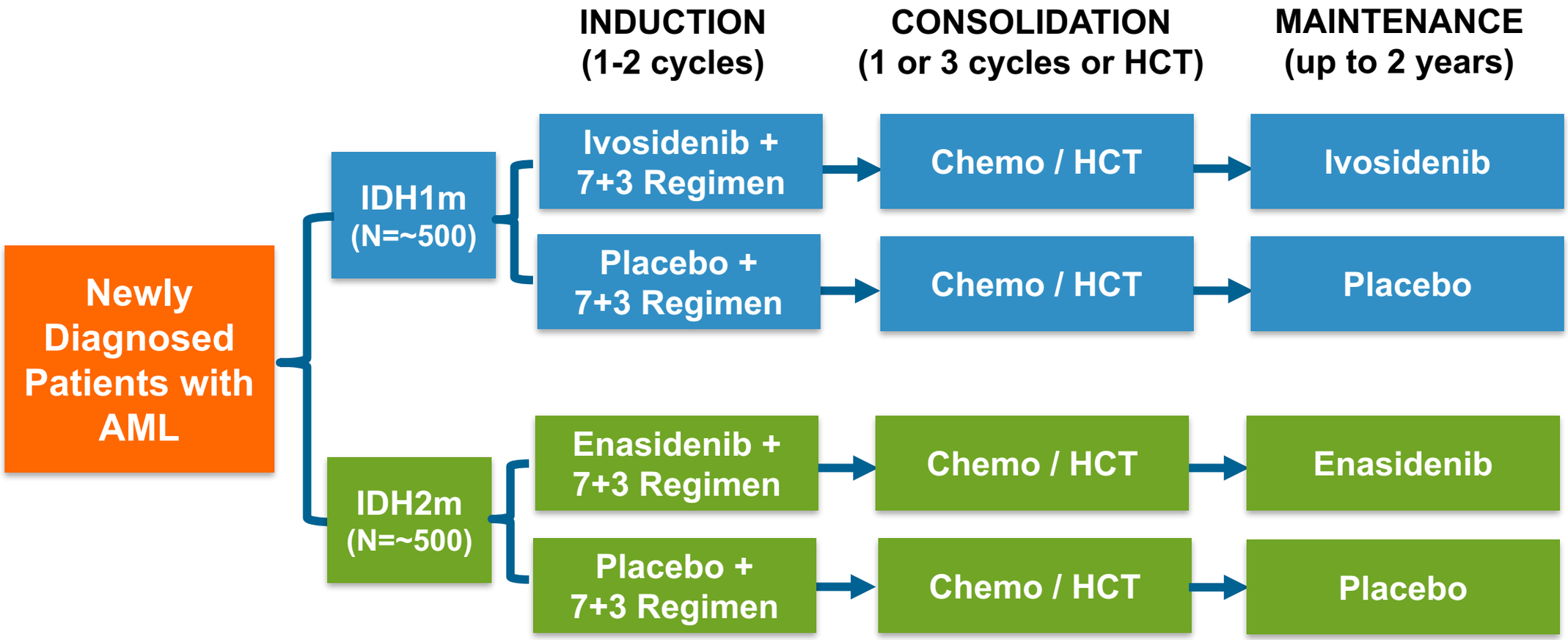


Sources: SEER. Cancer Stat Facts: AML 2015 and Epiphany EPIC oncology numbers; American Cancer Society. AML 2017.; Visser et. Al. Incidence, survival and prevalence of myeloid malignancies in Europe. Eur J Cancer. 2012 Nov;48(17):3257-66; Thomas ED, N Engl J Med. 1979 Mayer, N Engl J Med. 1994, Fernandez 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



Phase 3 Intergroup Frontline AML Trial in Collaboration with Celgene Beginning Q4 2018



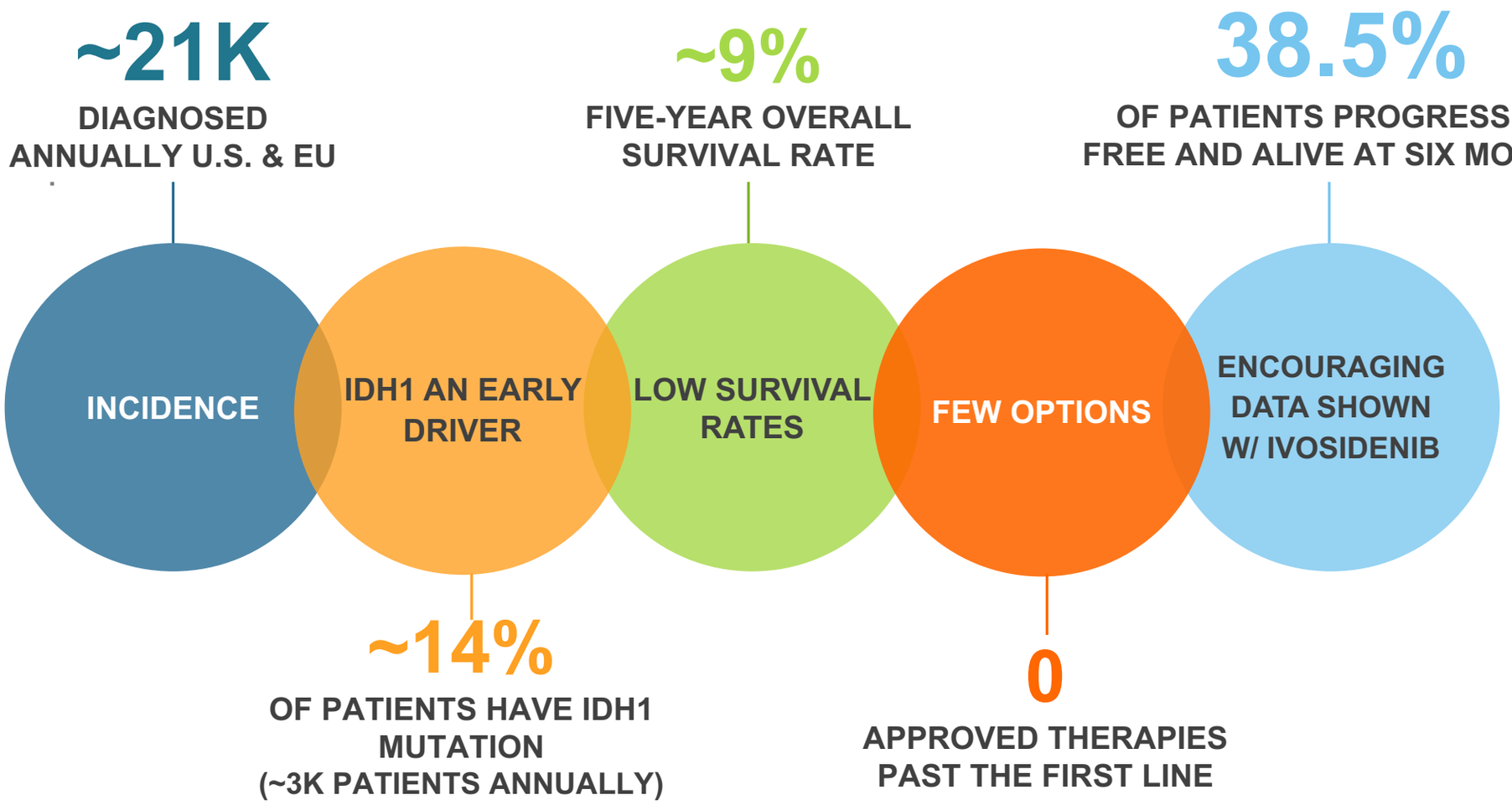
EFS primary endpoint; sponsored by HOVON and AML-SG

Leveraging Early Launch Success of IDHIFA®

Sales	Diagnostic Testing	Prescriber Base	Awareness	Commercial Infrastructure
Early IDHIFA® Success				
Q3 2017 sales **$7M**	IDH2m testing increasing: **~50% as of October**	**>250** unique prescribers	IDHIFA® awareness increasing: **~50% as of October**	Agios sales and MSL teams in the field **>1,200 customer interactions**
Impact for Ivosidenib Launch				
Increased physician experience with IDHm inhibitors	Expected continued rapid increase in testing rate	Increased physician experience with IDHm inhibitors	Strong IDH awareness already established	Experienced commercial team fully staffed Expanded sales team starts next week Comprehensive market access strategy in place

Revenues reported by Celgene; Market research as of Oct. 2017; Agios estimates

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

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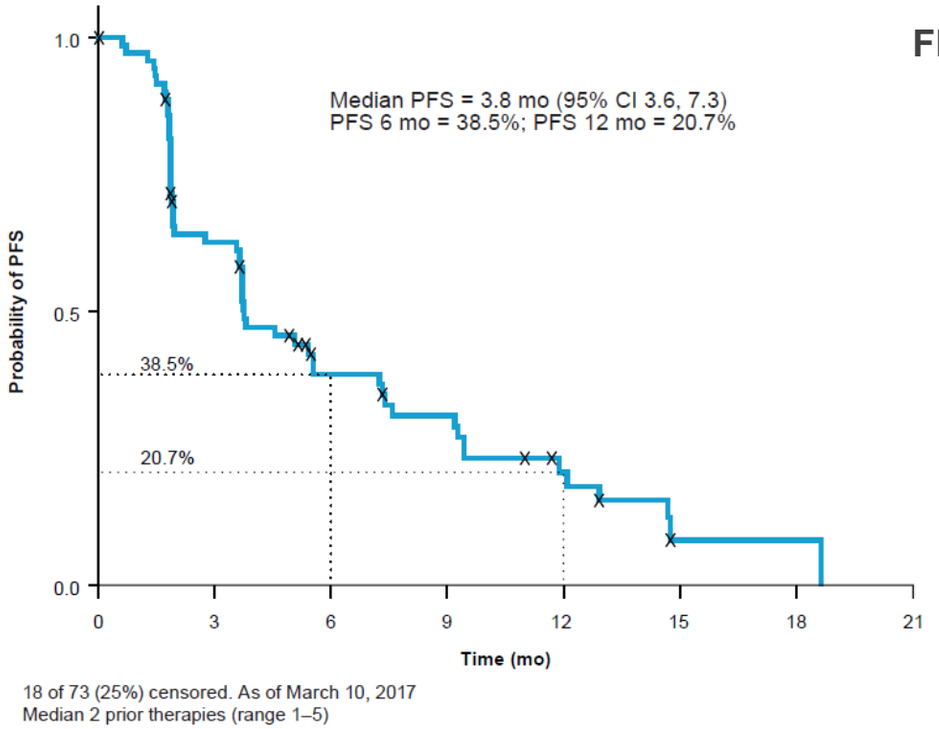


18 of 73 (25%) censored. As of March 10, 2017
Median 2 prior therapies (range 1–5)

Data from ASCO 2017

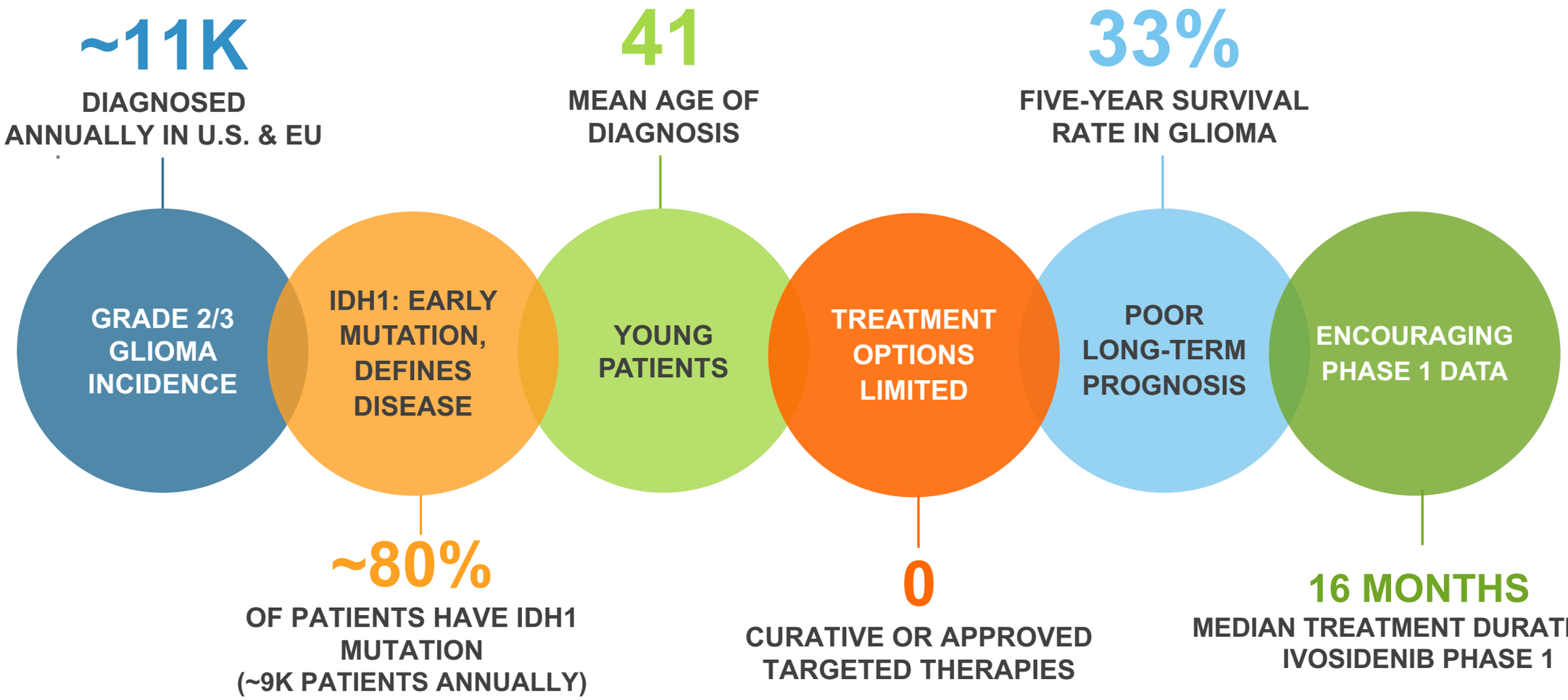
38.5%

OF PATIENTS PROGRESSION FREE AND ALIVE AT SIX MONTHS

ENCOURAGING DATA SHOWN W/ IVOSIDENIB

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

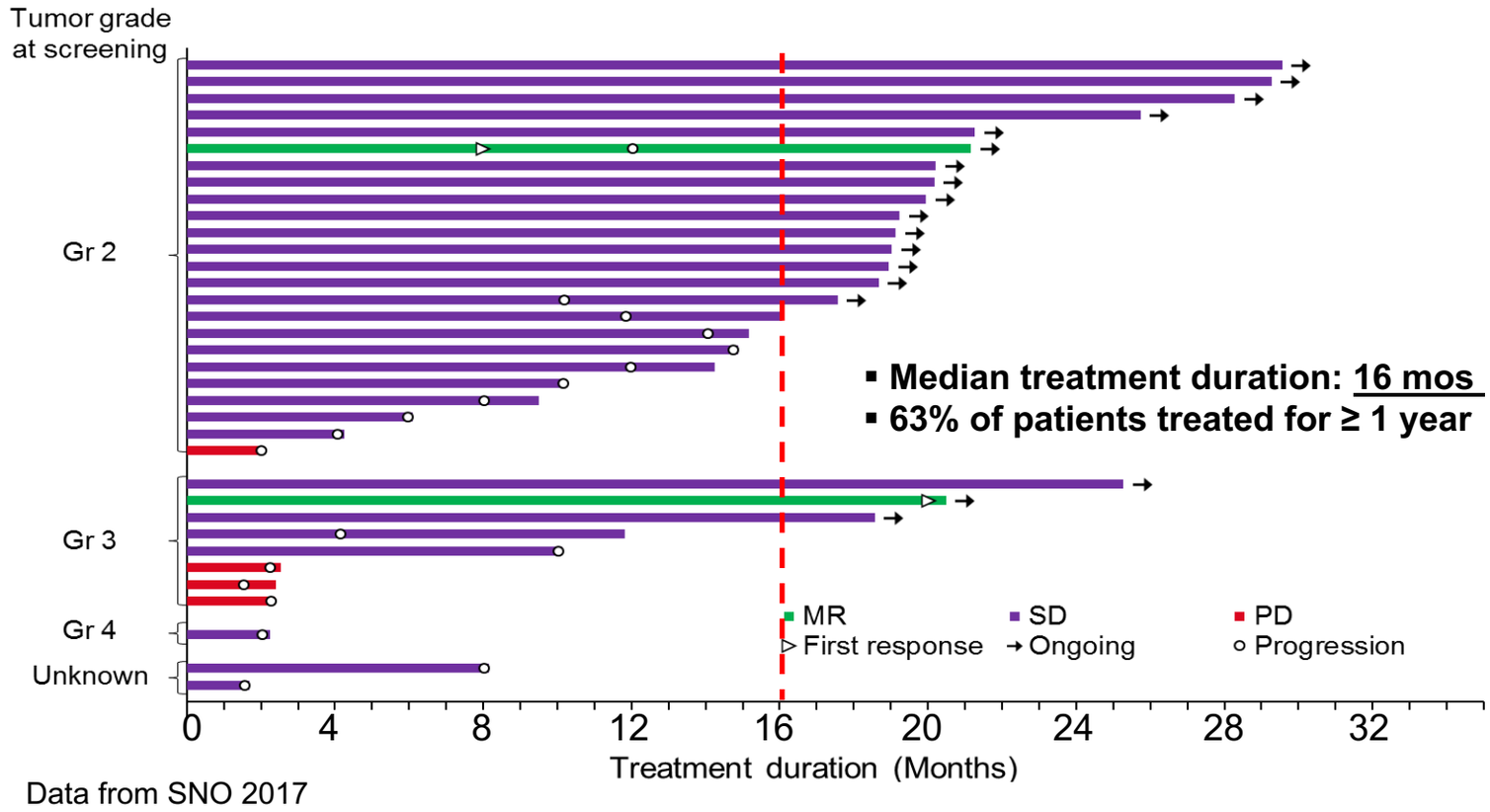
Low Grade Glioma: High Unmet Need Not Adequately Addressed by Chemotherapy or Radiation



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 1H 2018; Regulatory feedback to inform pivotal path

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



Data from SNO 2017

ENCOURAGING PHASE 1 DATA

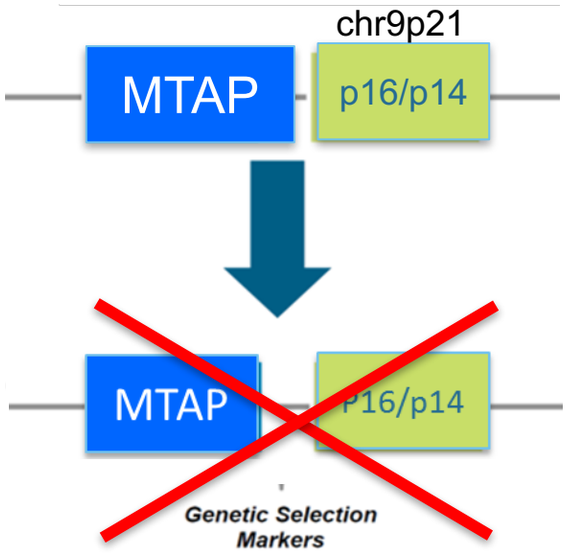
16 MONTHS

MEDIAN TREATMENT DURATION IN IVOSIDENIB PHASE 1

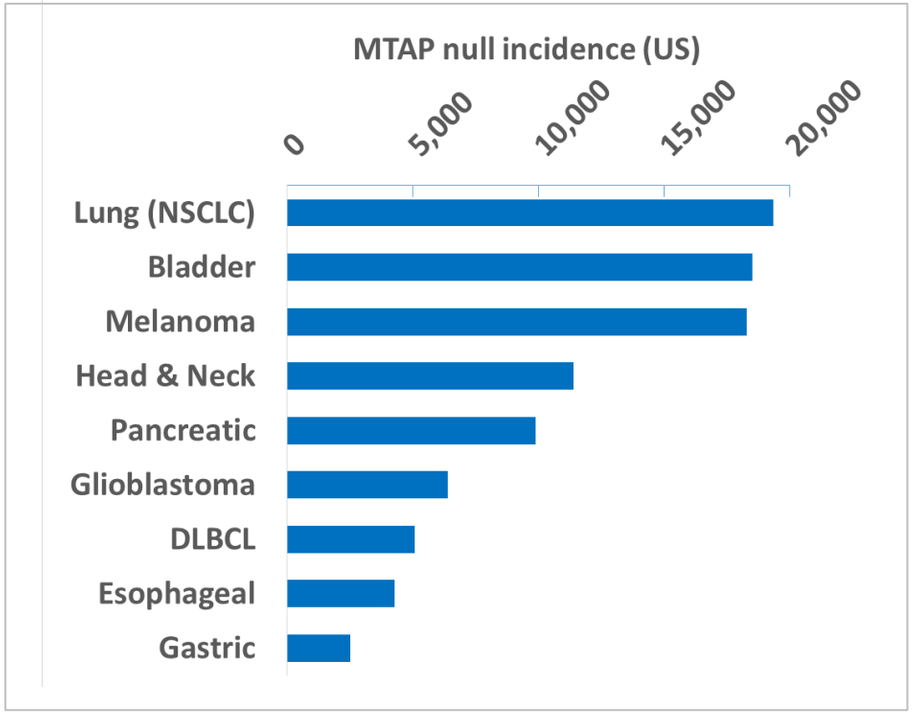
Perioperative study on track to start 1H 2018; Regulatory feedback to inform pivotal path

AG-270 Targets MAT2A in MTAP-Deleted Tumors

MTAP is the metabolic gene most frequently deleted in cancer because it is adjacent to a common tumor suppressor p16/p14



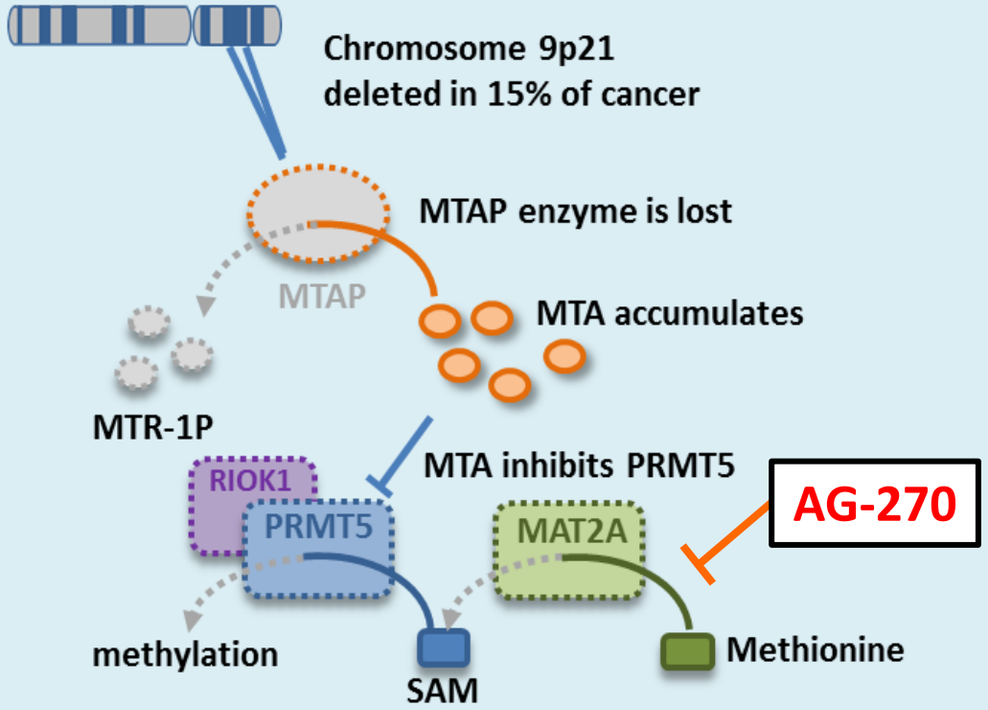
~98K new patients/year in U.S. with MTAP deletion



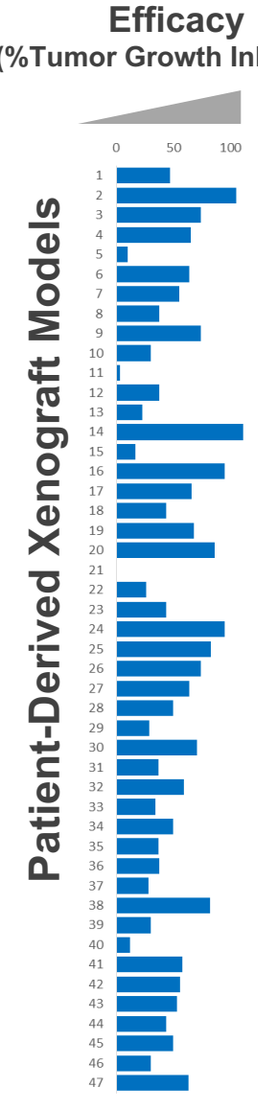
Sources: US Incidence data is from the NCI SEER; MTAP deletion frequencies are from Agios analysis of data from The Cancer Genome Atlas

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

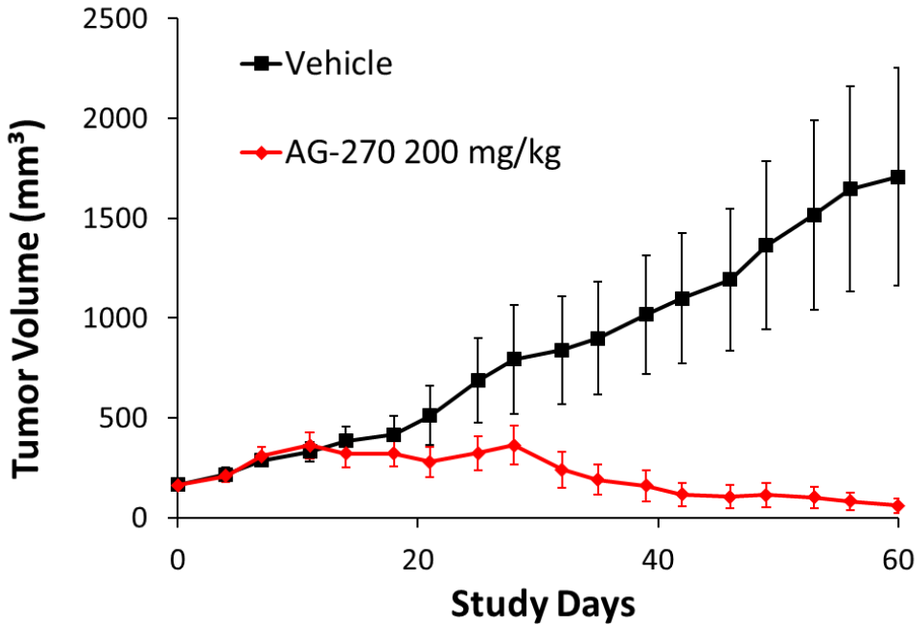
MTAP Deleted Cancer Cell



Agios publication: Marjon et al. Cell Reports 2016



MTAP-null NSCLC PDX model



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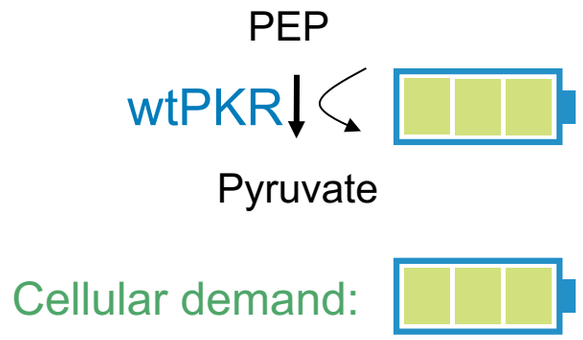
RESEARCH

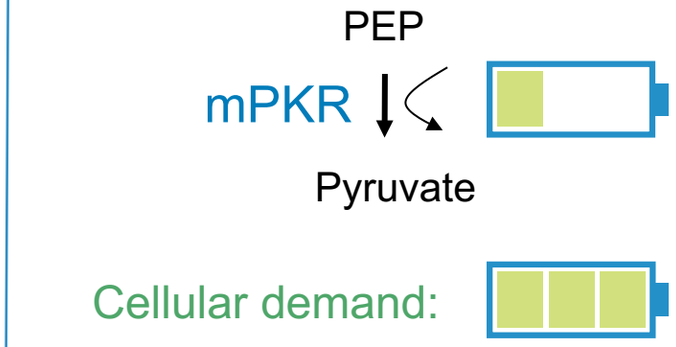
PK Activation Represents Opportunities Across Hemolytic Anemias

Normal Red Cell



ATP production meets demand

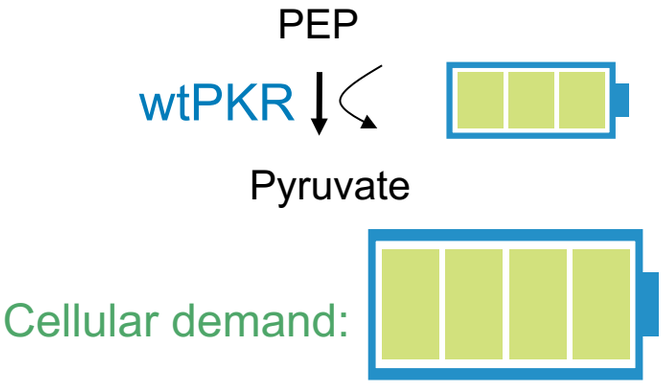
Pyruvate Kinase Deficiency



Inadequate production: ATP deficiency

✓ **Proof of concept achieved**

Other Hemolytic Anemias

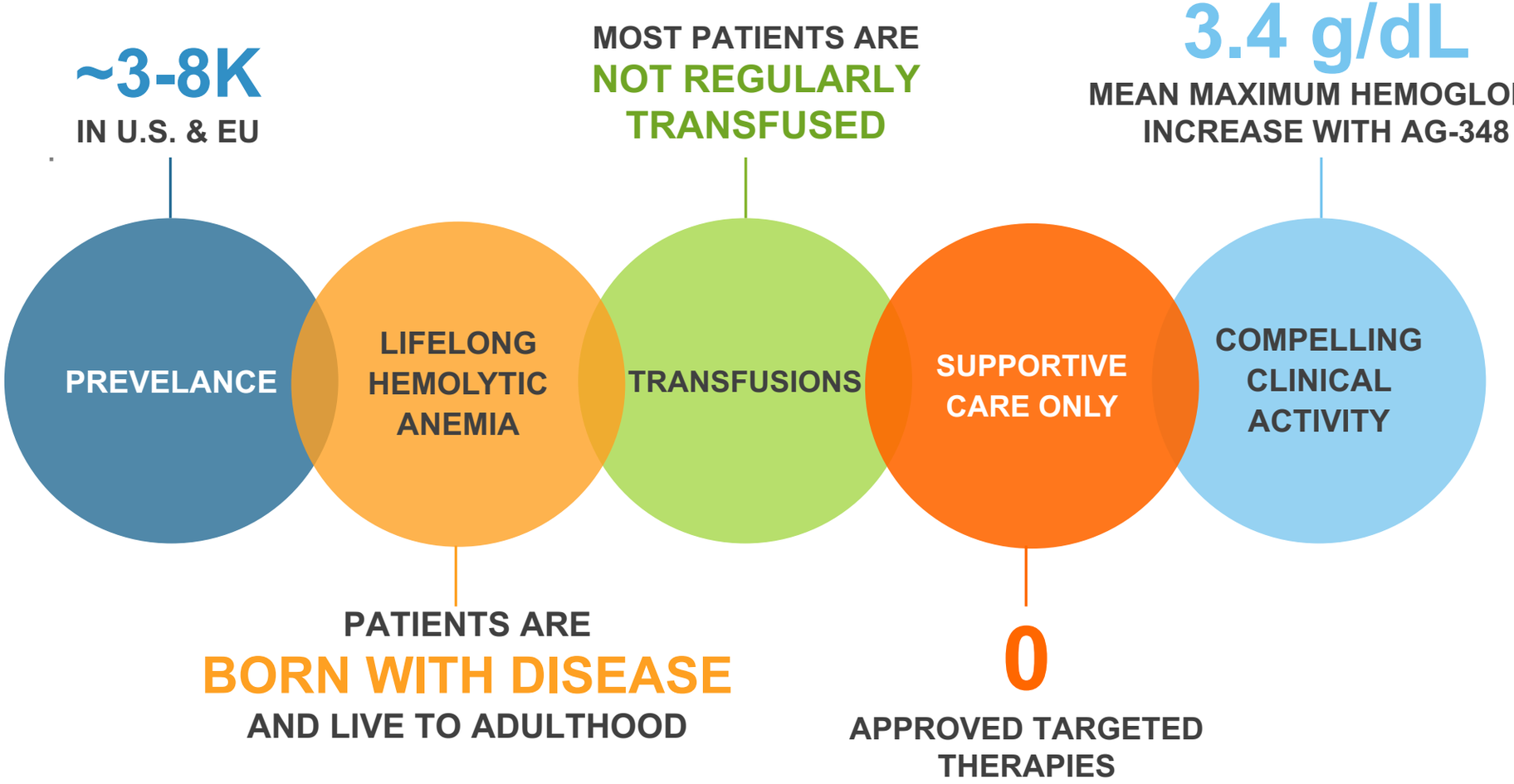


Increased demand: ATP deficiency

Thalassemia: Phase 2 proof-of-concept study to initiate Q4 2018

Sickle cell: Planning underway

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; [1]Mohrenweiser HW *PNAS* 1981;78(8):5046-50; [2]Carey PJ et al. *Blood* 2000;96(12):4005-6; [3]Beutler E & Gelbart T *Blood* 2000;95(11):3585-8; [4]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

PK Deficiency Carries Lifelong Burden

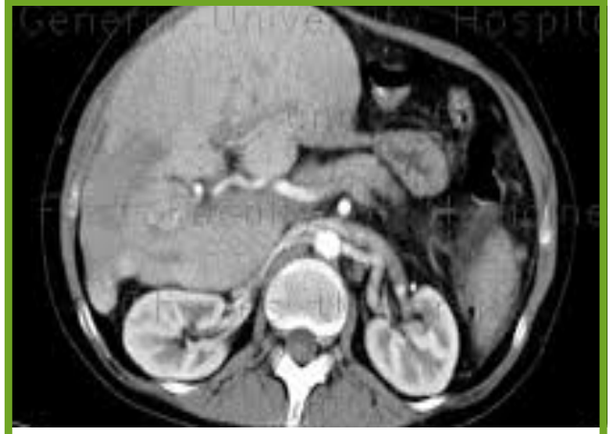
Infants

Jaundice, severe anemia, exchange transfusions

Toddlers, Children

Splenectomy leading to increased infection risk, antibiotic prophylaxis

Adults



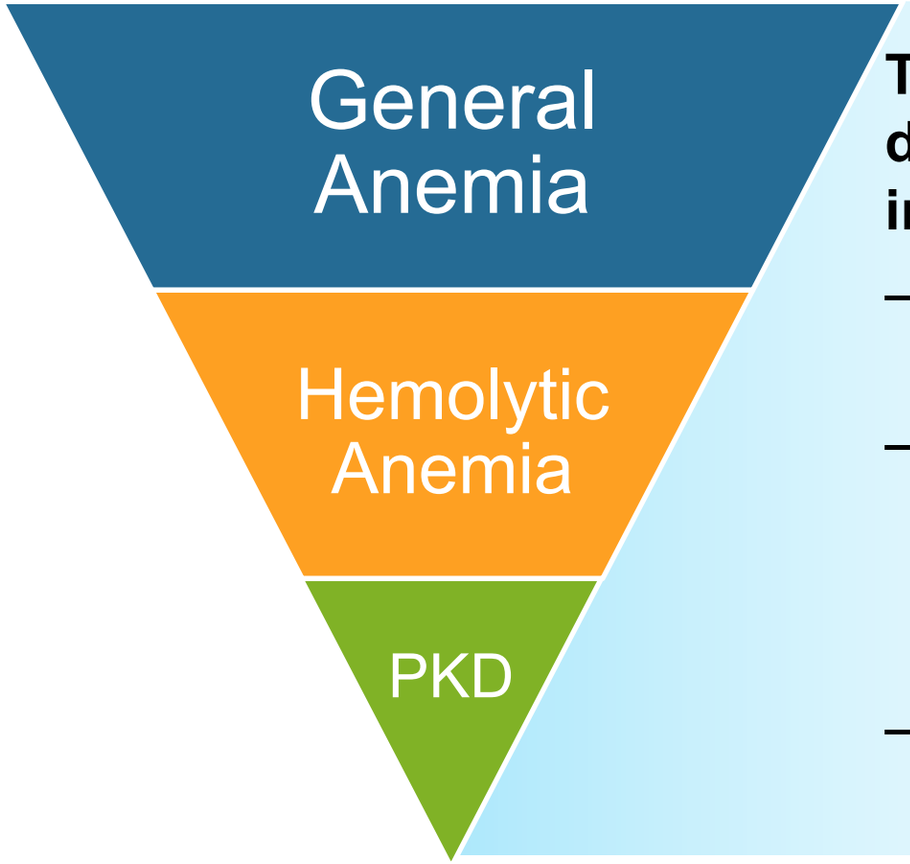
Iron overload leading to liver cirrhosis, cardiac and endocrine issues

Lifelong acute complications regardless of age/severity

- Splenectomy
- Transfusions
- Cholecystectomy
- Extramedullary hematopoiesis
- Pregnancy complications
- Hemolytic crisis
- Iron overload

Global PEAK patient registry opening Q1 2018

PK Deficiency Under Diagnosed: Patient Finding Efforts Focus on Outreach, Disease Awareness & Diagnosis



Testing for PK deficiency often incomplete due to:

- Lack of treatment options
- Spectrum of severity, defined differently among patients and physicians
- Inconsistent standard of care

Patient Finding Efforts

Social Media

Lowering Testing Barriers & Driving Differential Diagnosis

Disease Awareness

International Hematology Meetings & Thought Leader Engagement

Source: Physician Interviews; ClearView Analysis (Mar 2014); Trinity Analysis (Dec 2016).


CANCER
RARE GENETIC DISEASES
RESEARCH

Agios' Scientific Research Platform

DYSREGULATED METABOLISM

CANCER METABOLISM

- Inhibit key enzymes in ***cancer cell*** specific metabolic pathways to disrupt tumor cell proliferation and survival

RARE GENETIC DISEASES

- Restore defective metabolic pathways in ***disease cells*** that cause rare genetic disorders of metabolism

METABOLIC IMMUNO-ONCOLOGY

- Alter the metabolic state of ***immune cells*** to enhance the body's anti-tumor response

RESEARCH PLATFORM

Robust Preclinical Pipeline: Expecting Multiple INDs in 24 Months

Program	Project Stage: Target Identification	Target Validation	Drug Discovery	Drug Candidate
Oncology				
Genetically Defined Solid Tumor Target			●	
Heme Lineage: DHODH				●
Genetically Defined Heme Target			●	
Genetically Defined Heme Target		●		
Genetically Defined Solid Tumor Target		●		
Other Exploratory Programs	◐	◐		
Rare Genetic Diseases				
Program 1			●	
Program 2			●	
Program 3			●	
Program 4		●		
Other Exploratory Programs	◐			
Metabolic Immuno-Oncology (Celgene Collaboration)				
Target 1			●	
Target 2			●	
Target 3		●		
Other Exploratory Programs	●			

Celgene-Partnered Programs

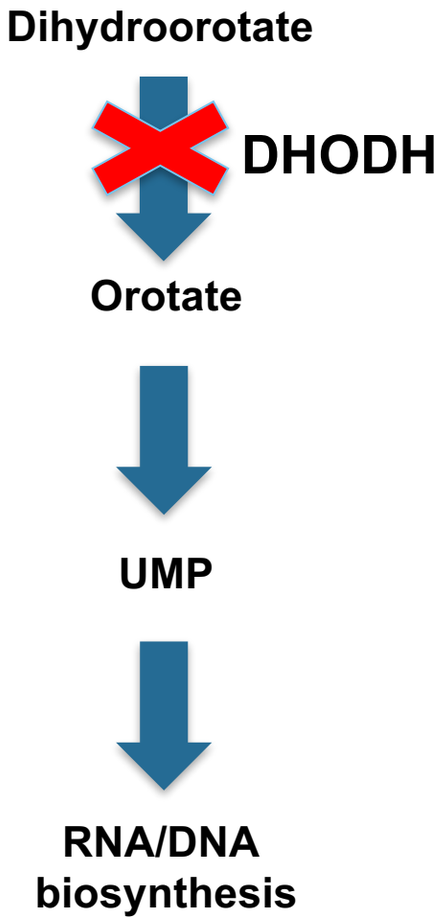
● Metabolic Target

● Non-Metabolic Target

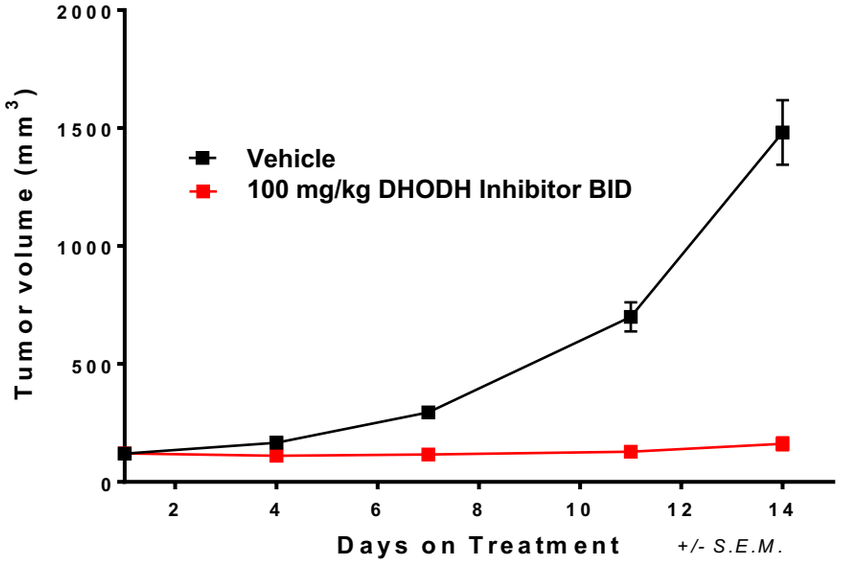
◐ Metabolic and Non-Metabolic Targets

DHODH Inhibitor Program IND Expected in Q4 2018

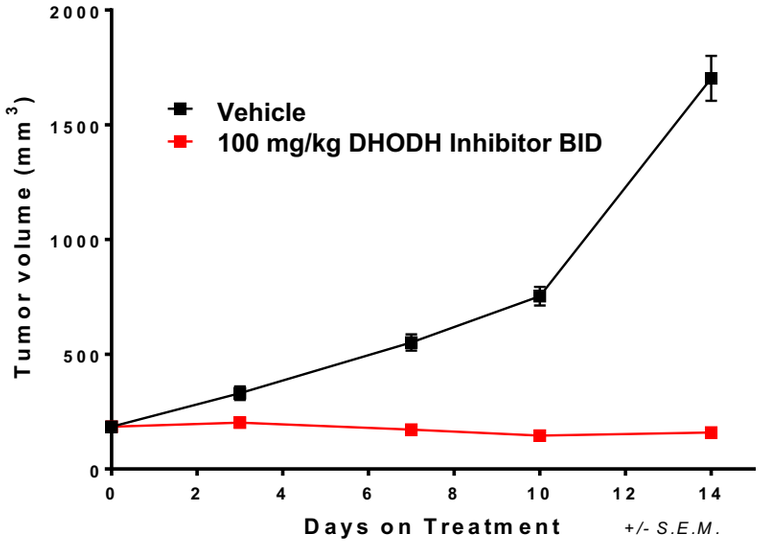
DHODH catalyzes a critical step in pyrimidine biosynthesis



Efficacy in MOLM13 **AML** model



Efficacy in OCILY19 **DLBCL** model



- Agios discovered a lineage-specific dependence on dihydrooroate dehydrogenase (DHODH) in hematologic malignancies (particularly AML and DLBCL)
- DHODH Inhibition is anticipated to differentiate from standard of care therapies
  - Activity in cancers that are resistant to standard-of-care chemotherapeutics
  - Mechanism of antitumor effect a combination of cell growth arrest and cellular differentiation

Sources: Sykes et al Cell 2016, Agios data on file

2018 Goals Set Stage for Building Long-Term Value

2018 GOALS

- Secure approval and commercialize ivosidenib for R/R AML in the U.S.
- Initiate Phase 3 frontline AML trial combining ivosidenib & enasidenib with 7+3
- Initiate two AG-348 PK deficiency pivotal trials
- Initiate AG-270 Phase 1 dose-escalation trial
- Submit ivosidenib European MAA
- Initiate glioma perioperative study
- Initiate AG-348 Phase 2 trial in thalassemia
- Submit 7th IND for DHODH

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

Thank You

Agios Company Retreat 2017