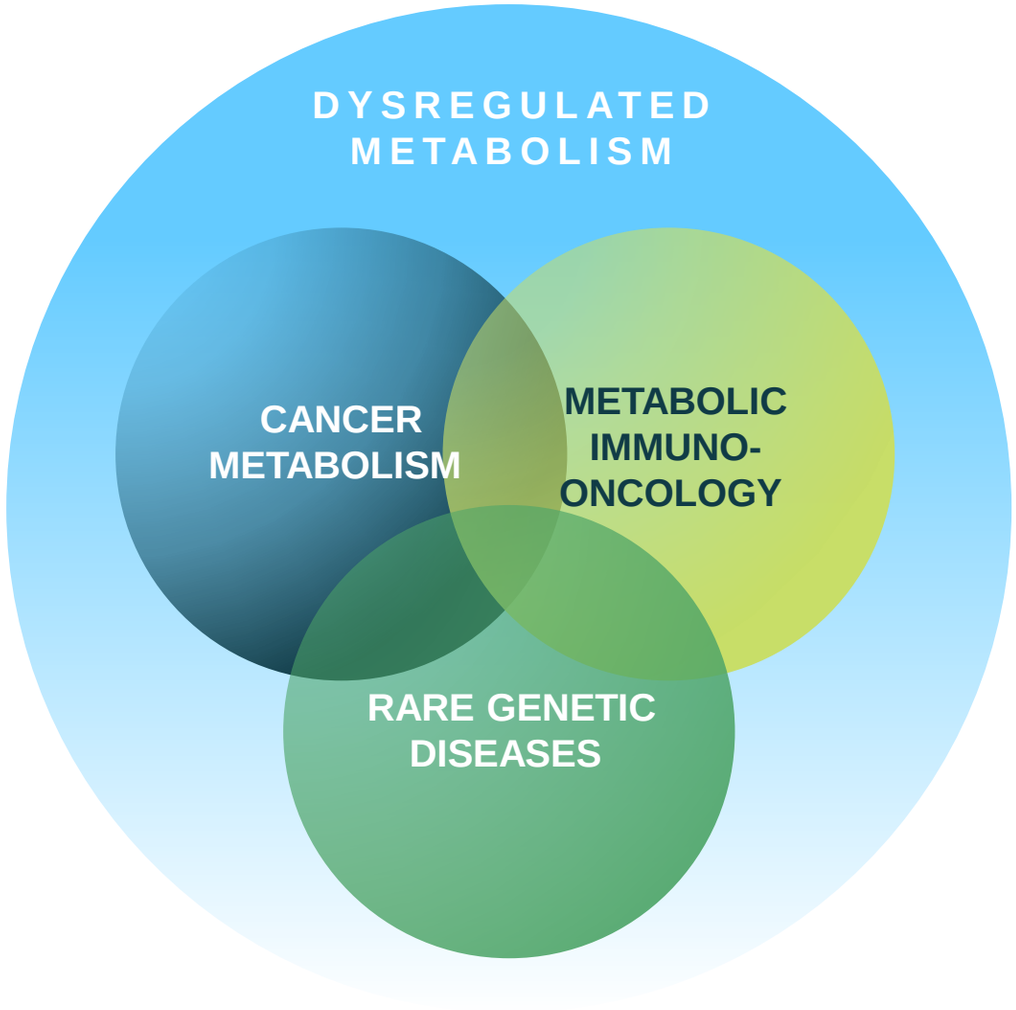
Needham Healthcare Conference

April 4, 2017

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-881 and AG-348; the potential benefits of Agios' product candidates; its key milestones for 2017; its plans regarding future data presentations; 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' Annual Report on Form 10-K for the year ended December 31, 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.

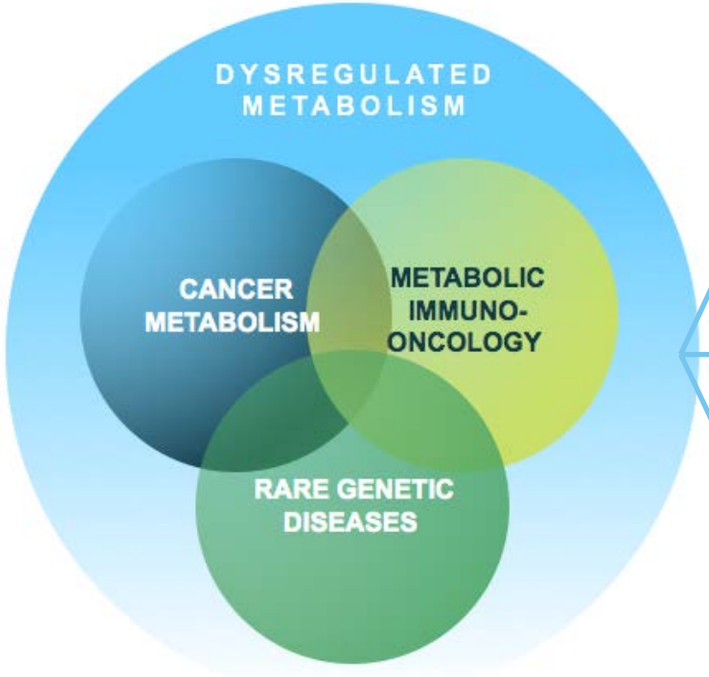
We Are Driven By a Clear Vision and Values…



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.

…Resulting in a Highly Productive Drug Development Engine…

Science	Discovery	Precision Medicine Candidates	Addressing Unmet Medical Needs
	**IDH**	**Enasidenib** (AG-221) **Ivosidenib** (AG-120) **AG-881**	**AML** **Cholangiocarcinoma** **Glioma**
	PKR	**AG-348**	**PK Deficiency**
	MTAP		

Demonstrated ability to rapidly translate novel biology into precision medicines in areas of high unmet need

…Building a Research-Driven, Commercial-Stage Biopharmaceutical Company

2017 – 2018

2 approved precision medicines in AML

4+ clinical-stage molecules

3+ pivotal trials (IDH, PKR)

3 areas of research focus

450+ employees

100% committed to helping patients

Delivering Our First Medicines to Patients

Team

Science

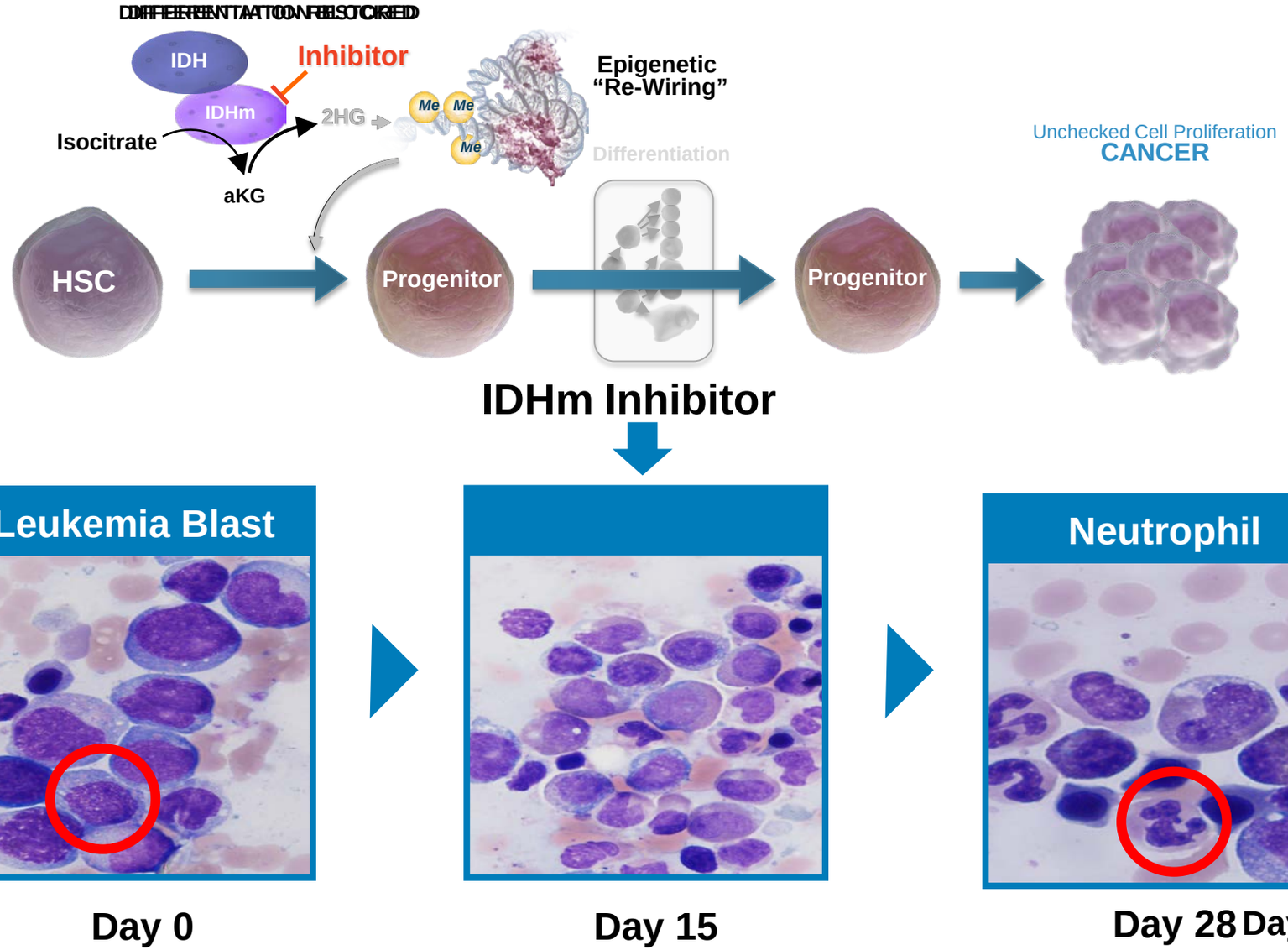
Discovery

IDH

PKR

MTAP

Differentiated MOA: Repairing an IDH Mutant Cancer Cell



Ivosidenib, EORTC, 2014

Mutation occurs early and persists throughout illness

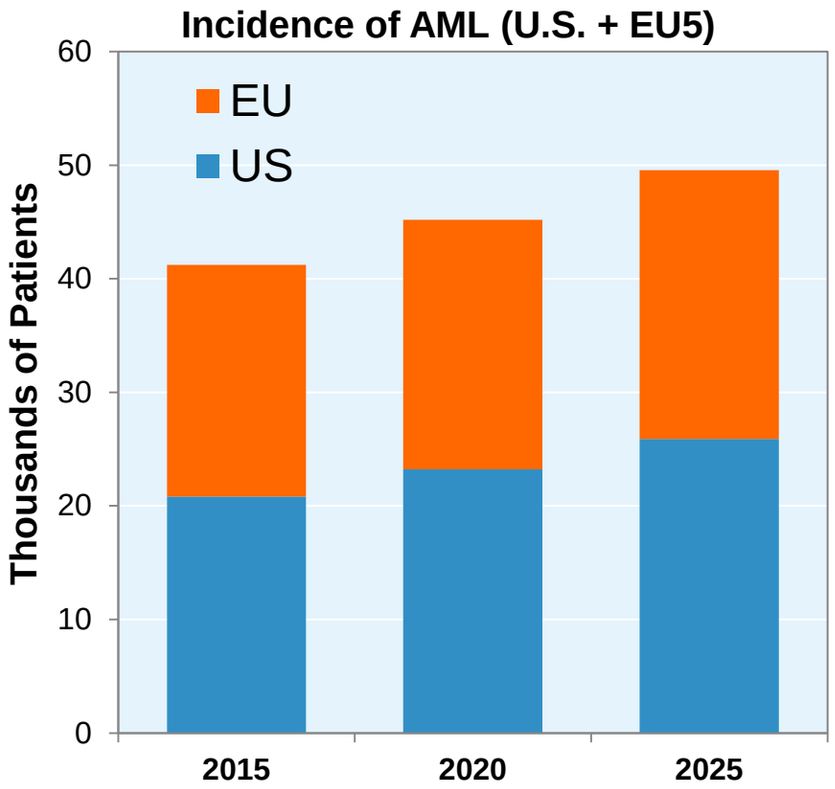
Our Vision for IDHm Inhibitors

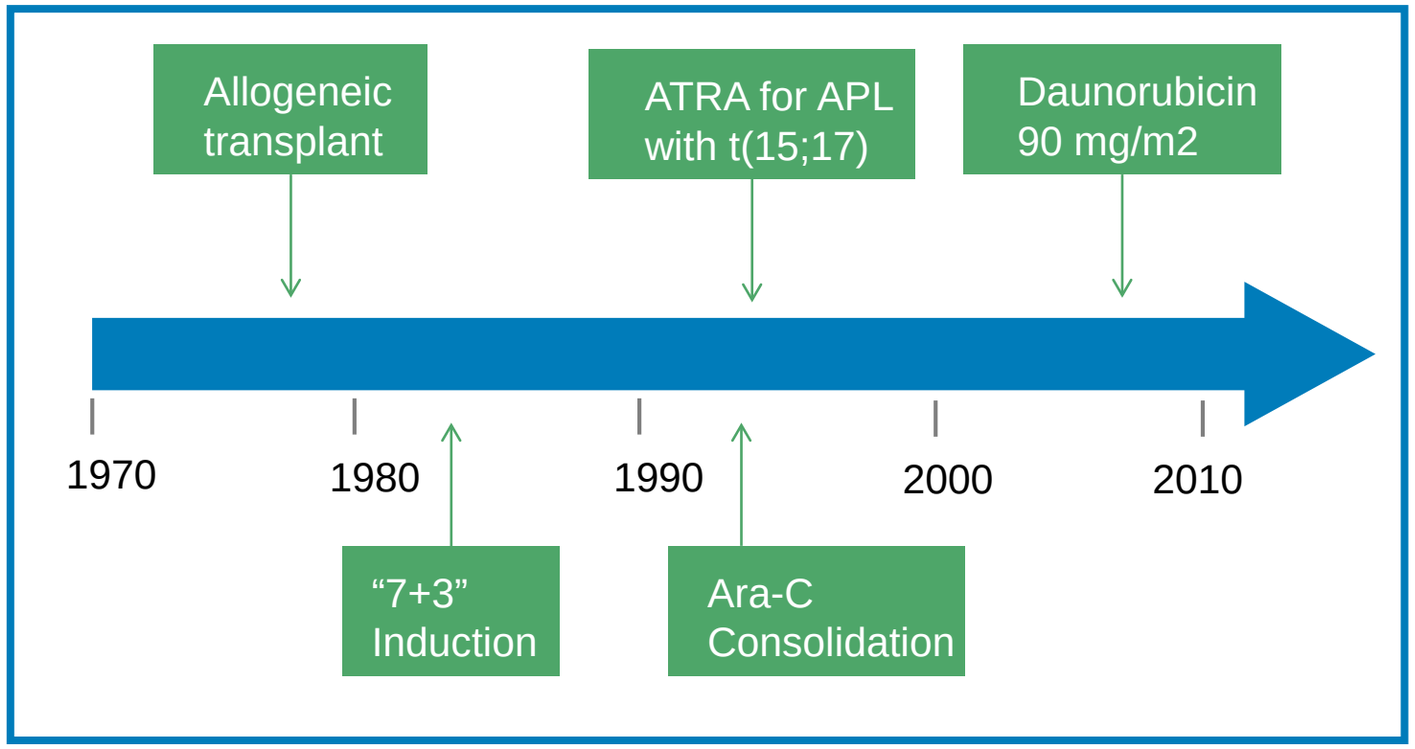
A Roadmap for Speed and Breadth

All IDHm patients screened and treated with an IDHm inhibitor for the entire course of their disease

Growing Unmet Need for AML Treatments

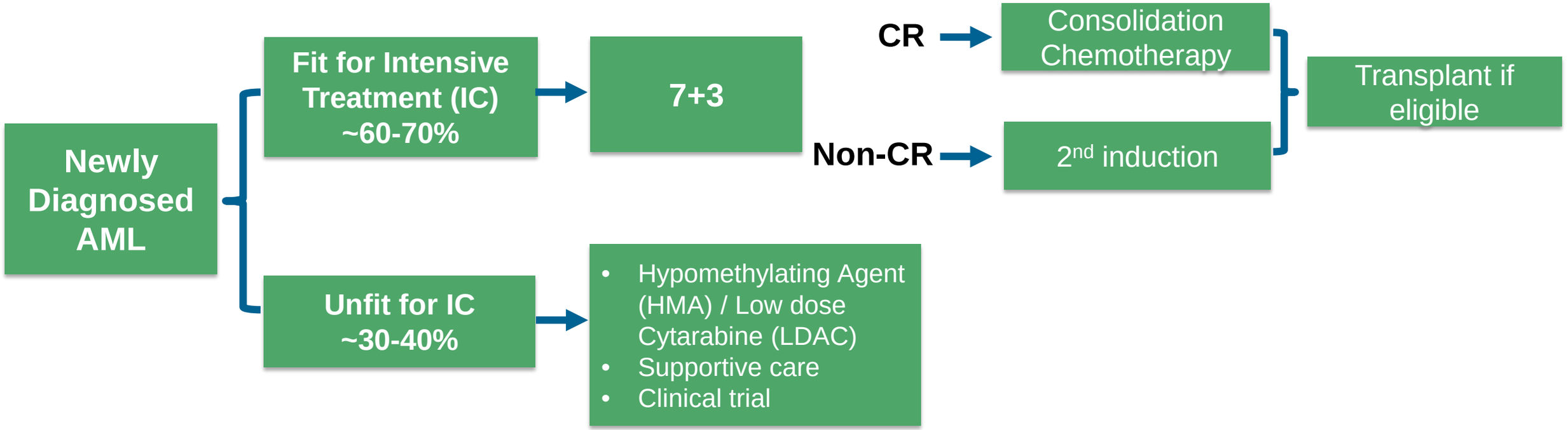
Growing unmet need in AML given rising incidence in U.S. and EU5
with aging population and lack of new therapies





Sources:
1) American Cancer Society: Cancer Facts and Figures
2) Visser et. Al. Incidence, survival and prevalence of myeloid malignancies in Europe. Eur J Cancer. 2012 Nov;48(17):3257-66
3) Epiphany Partners Epic Oncology
4) Decision Resources
5) Thomas ED, N Engl J Med. 1979 Mayer, N Engl J Med. 1994, Fernandez H,N Engl J Med, 2009

Shifting the Treatment Paradigm for AML with Precision Medicine



Acute Myeloid Leukemia (AML)		
IDH1m frequency	IDH2m frequency	5-year overall survival
6-10%	9-13%	20-25%

Multiple sources including market research SEER

Long-term goal: treatment across multiple lines with an IDHm inhibitor

IDHm Inhibitors in AML

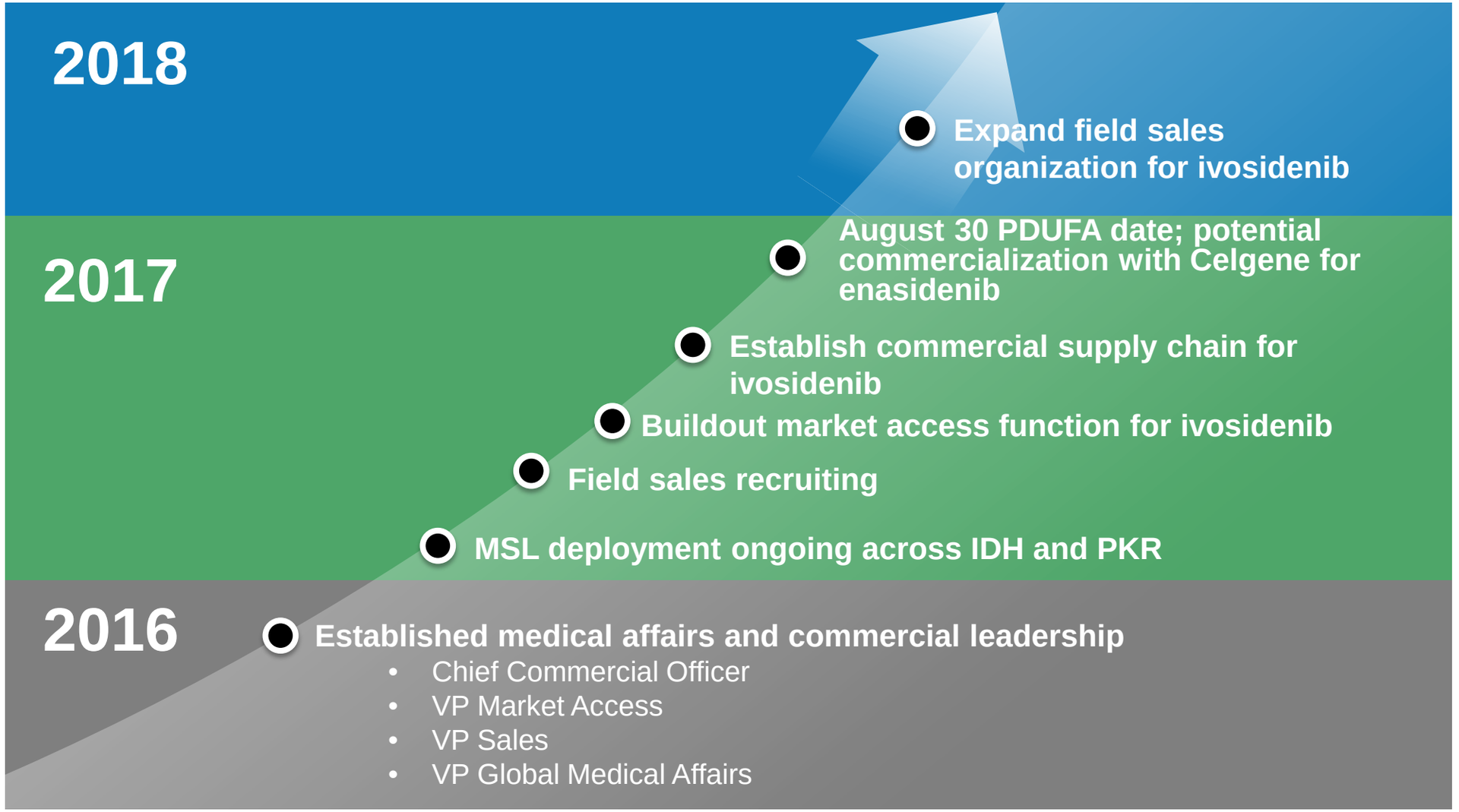
A Roadmap for Speed and Breadth

Relapsed/Refractory AML

- Enasidenib priority review; August 30, 2017 PDUFA
- Ivosidenib NDA submission expected by YE 2017

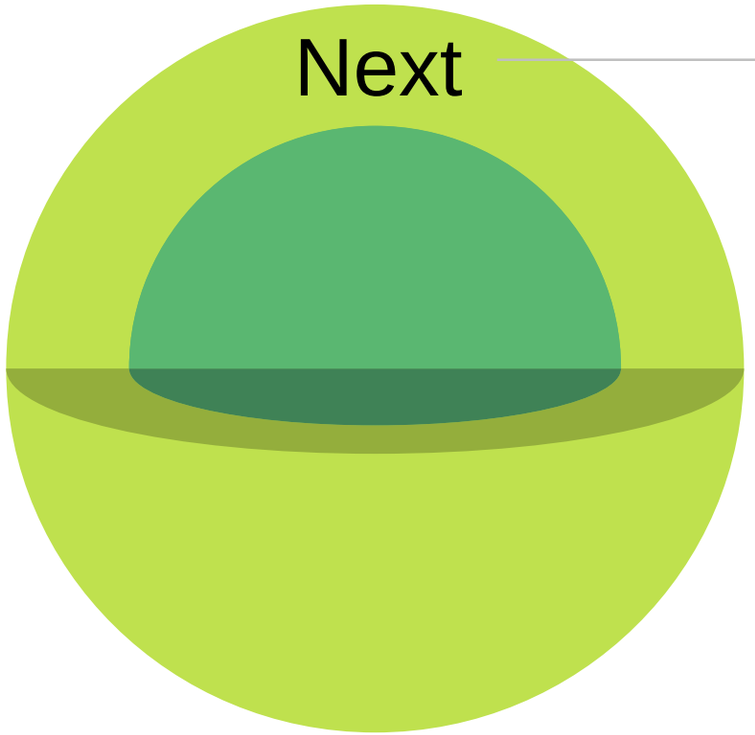
First data from ivosidenib Phase 1 expansion in R/R AML in 2H 2017

Building Commercial Capabilities to Ensure Success for Upcoming IDH Launches



IDHm Inhibitors in AML

A Roadmap for Speed and Breadth



Frontline AML

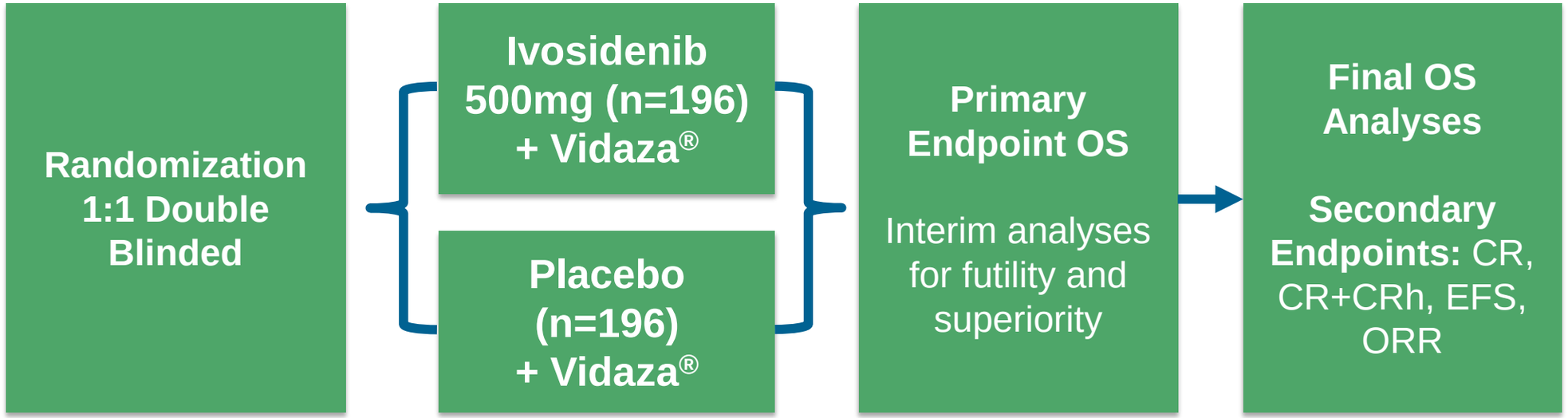
- Ongoing Phase 1 combo trials (Vidaza® or 7+3)
- AGILE: Ivosidenib + Vidaza® Phase 3
- Enasidenib / ivosidenib + (7+3) with maintenance

First data from Phase 1b frontline combination of enasidenib or ivosidenib with intensive chemotherapy in 2H 2017

Advancing Ivosidenib into Frontline Setting

AGIL
AGios study for IDH1 mutation in acute myeloid LEukemia

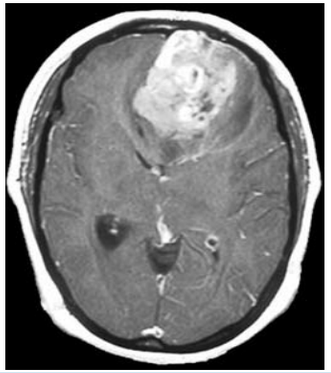
Global Phase 3 Frontline IC-Ineligible IDH1m AML

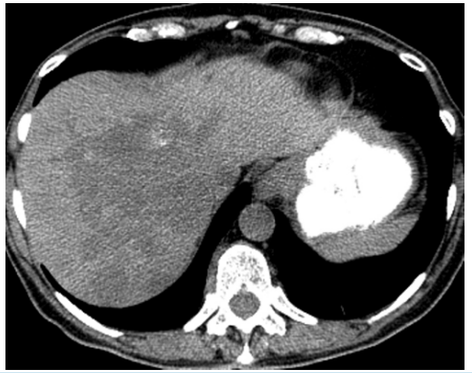


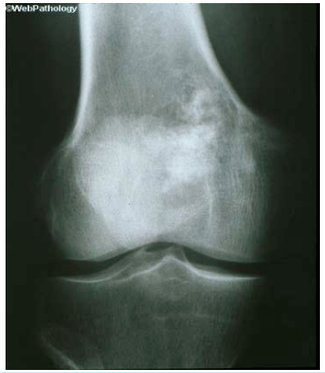
IC = intensive chemotherapy
Vidaza® is a registered trademark of Celgene Corporation

Trial on track for 1H 2017 initiation

Treating Solid Tumors with an IDH1m Inhibitor





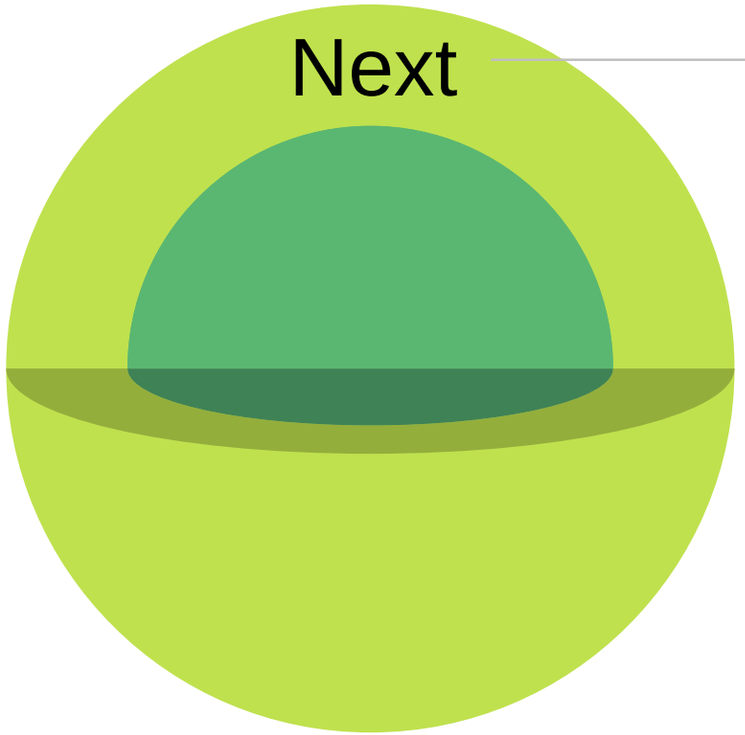


	Glioma	Intrahepatic Cholangiocarcinoma (IHCC)	Chondrosarcoma
	Low grade and 2^{ary} GBM	Bile ducts	Cartilage
Incidence (cases/year U.S.)	5,000	2,000 – 4,000	700 – 1,000
IDH1m frequency	68-74%	11-24%	40-52%
Treatment options	Surgery, XRT Chemotherapy	Surgery, Chemotherapy Liver transplantation	Surgery, XRT Chemotherapy
5-year OS	~32-68%*	~9%	~10-90%

Other solid tumor types include colon, melanoma, lung, ovarian.
Multiple sources, including market research and SEER. Estimates will continue to evolve with additional future data
*excludes primary GBM

IDHm Inhibitors in Solid Tumors

A Roadmap for Speed and Breadth



Solid Tumors

- ClarIDHy: Ivosidenib Phase 3 cholangiocarcinoma
- Ivosidenib and AG-881 Phase 1 glioma expansion

Ivosidenib cholangio expansion data in 1H 2017; Updated ivosidenib glioma data in 2H 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

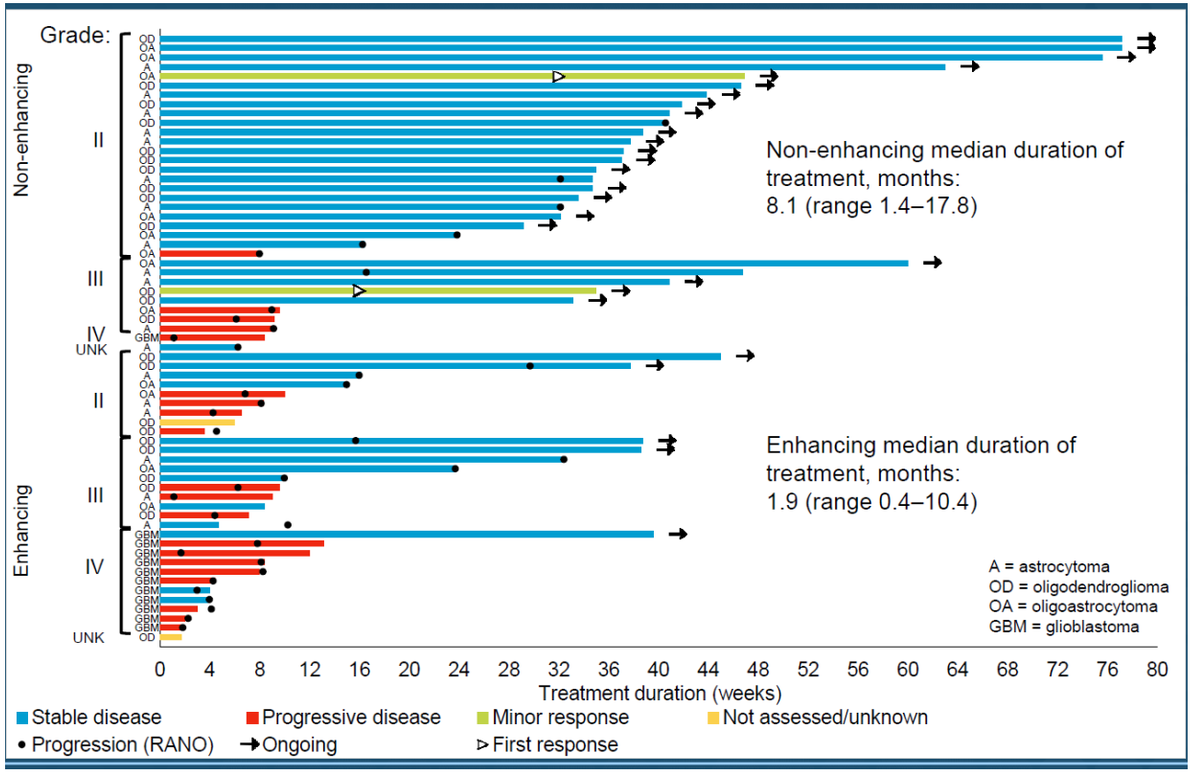
Trial initiated in December 2016

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

Ivosidenib

IDH1m Inhibition

→ Phase 1 Dose-Escalation and Expansion (Enrollment Complete)



Ivosidenib Data Presented at SNO 2016

AG-881

High Brain Penetration, Pan-IDHm Inhibition

→ Phase 1 Dose-Escalation (Ongoing) → Potential Expansion

IDH

PKR

MTAP

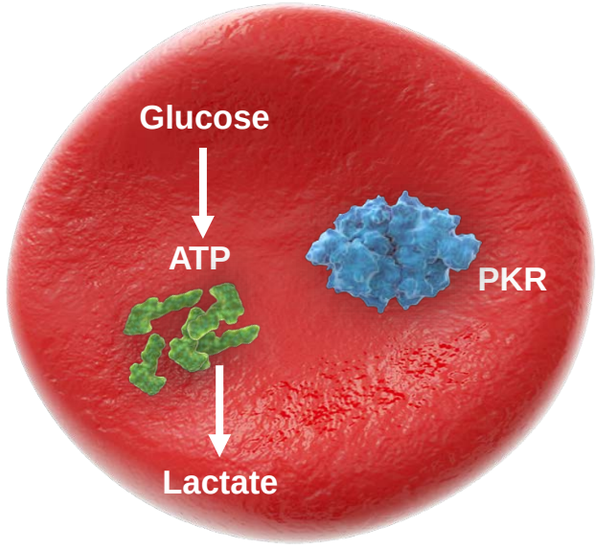
PK Deficiency Is a Rare Genetic Disease that Affects Red Blood Cells

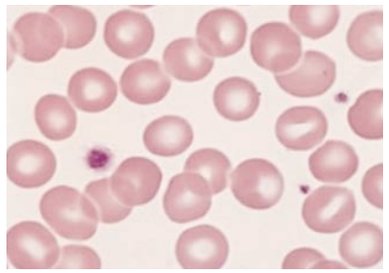
Rare genetic disease of erythrocyte pyruvate kinase

- PK deficiency often presents at birth with jaundice and can cause lifelong hemolytic anemia and associated morbidities
- Estimated prevalence ranges from ~1:20K to ~1:485K[1–4]

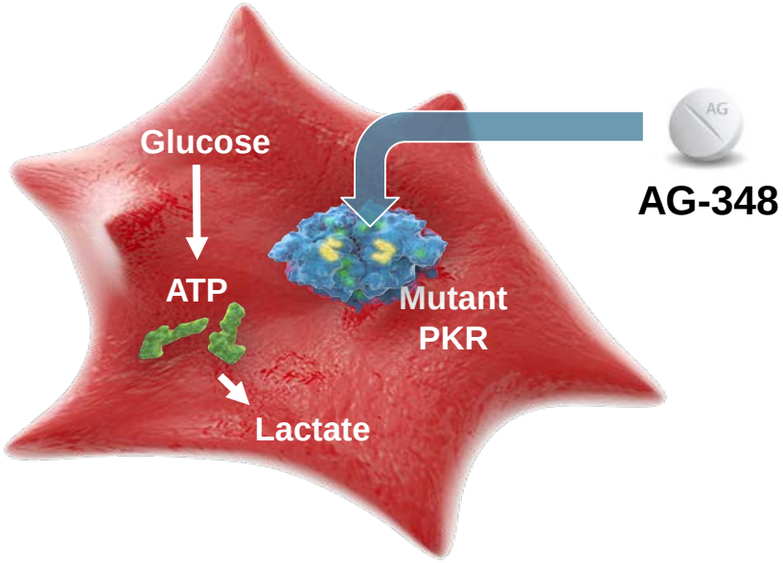
PKR regulates a crucial step in red blood cell metabolism and when mutated causes premature death of these cells

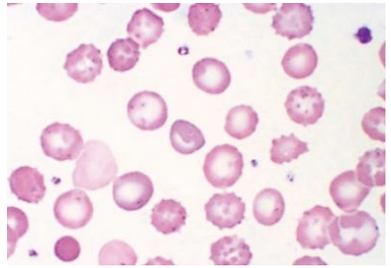
Healthy





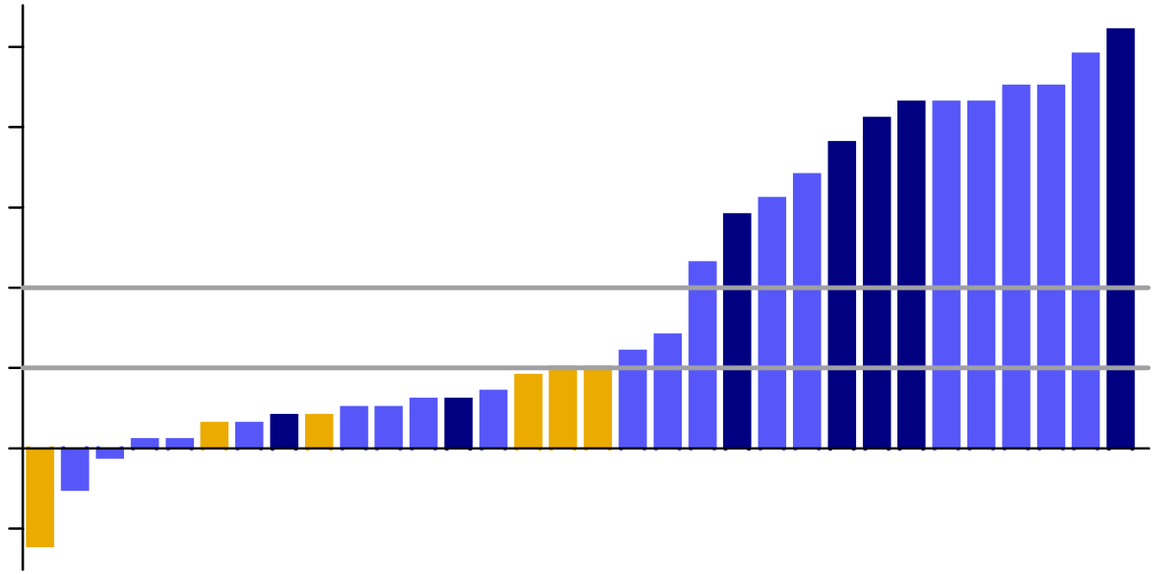
PK Deficiency





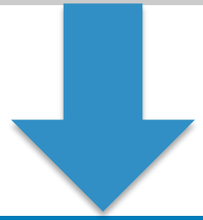
Grace R et al. *Am J Hematol* 2015;90(9):825-30; [1]Mohrenweiser HW *PNAS* 1981;78(8):5046-50; [2]Carey PJ et al. *Blood* 2000;96(12):4005-6; [3]Beutler E & Gelbart T *Blood* 2000;95(11):3585-8; [4]deMedicis et al. *Hum Hered* 1992;42(3):179-83

Compelling Proof-of-Concept for AG-348, the First Disease Modifying Therapy for PK Deficiency



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



Finalize pivotal trial design by 3Q 2017; Expect to initiate pivotal study in 1H 2018

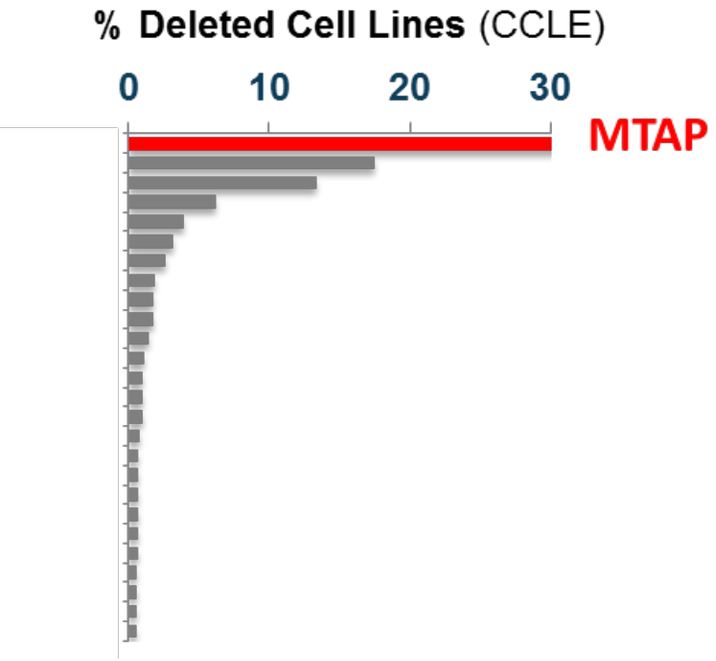
IDH

PKR

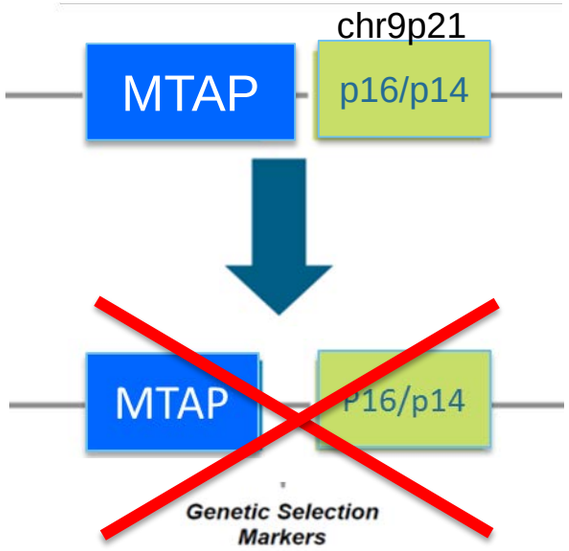
MTAP

MTAP-Deleted Tumors Constitute a Large, Genetically Defined Patient Population

MTAP is the metabolic gene most frequently deleted in cancer…

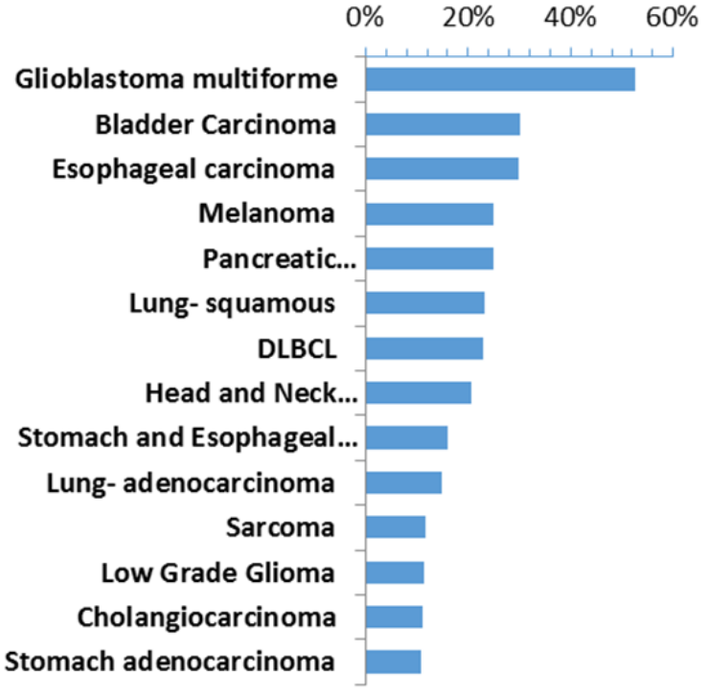


… because it is adjacent to a common tumor suppressor p16/p14…



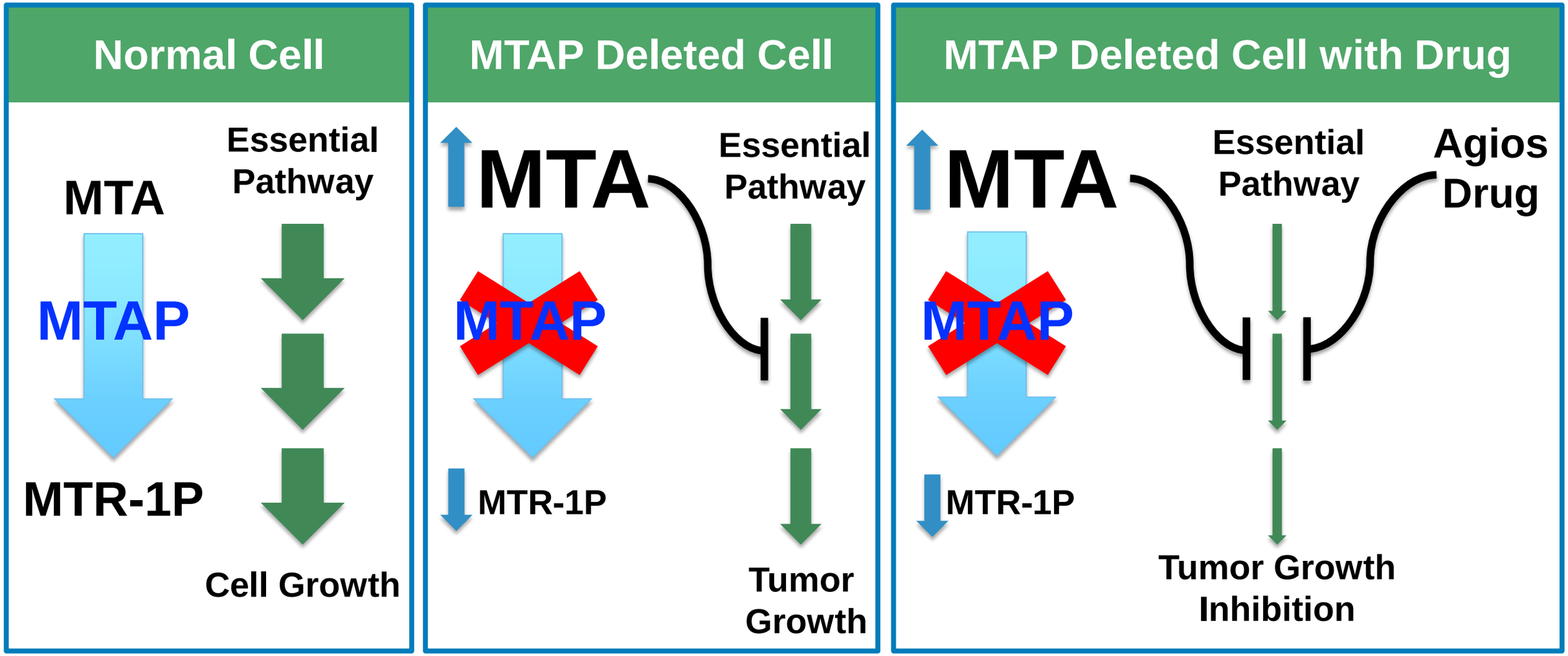
- p16/p14 tumor suppressor locus deleted in 15% of all cancers
- Metabolic gene, MTAP, is adjacent to p16/p14 & typically co-deleted

…resulting in ~98,000 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

Cancers with Deletion of MTAP Are Vulnerable to Inhibition



Development candidate for MTAP pathway selected under Celgene collaboration; IND expected by year-end 2017

Continued Investment in Agios' Research & Discovery 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 diseases of metabolism**

METABOLIC IMMUNO-ONCOLOGY

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

agios + Celgene

RESEARCH PLATFORM

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

- Submit IND application for development candidate targeting MTAP-deleted tumors by YE 2017
- Advance next wave of research in our three focus areas: cancer metabolism, rare genetic diseases and metabolic immuno-oncology

Building a Research-Driven, Commercial-Stage Biopharmaceutical Company

2017 – 2018

2 approved precision medicines in AML

4+ clinical-stage molecules

3+ pivotal trials (IDH, PKR)

3 areas of research focus

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