Third Quarter 2019 Financial Results

October 31, 2019

Agios Conference Call Participants

Prepared Remarks

Introduction

- KENDRA ADAMS, Vice President, External Communications & Investor Relations

Business Highlights

- JACKIE FOUSE, Ph.D., Chief Executive Officer

Clinical Development Progress

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

TIBSOVO® Commercial Update

- DARRIN MILES, Senior Vice President, U.S. Commercial & Global Marketing

Third Quarter 2019 Financial Results

- ANDREW HIRSCH, Chief Financial Officer & Head of Corporate Development

Forward Looking Statements

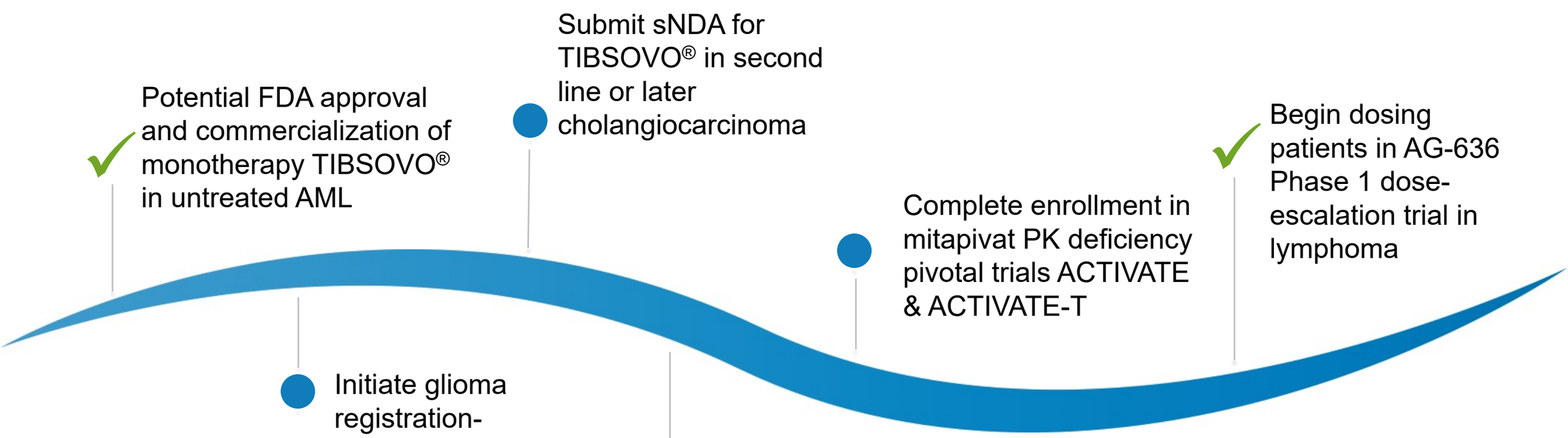
This presentation and various remarks we make during this presentation contain forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include those regarding Agios' plans, strategies and expectations for its and its collaborator's preclinical, clinical and commercial advancement of its drug development programs including TIBSOVO® (ivosidenib), IDHIFA® (enasidenib), vorasidenib (AG-881), mitapivat, AG-270 and AG-636; the potential benefits of Agios' product candidates; its key milestones for 2019; 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," "hope," "milestone," "plan," "potential," "possible," "strategy," "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 collaborators 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, the EMA or 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 CStone Pharmaceuticals; 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 Updates

Jackie Fouse, Ph.D., Chief Executive Officer

agios

2019 Key Milestones Position Agios for Long-term Value Creation



- ✓ Potential FDA approval and commercialization of monotherapy TIBSOVO® in untreated AML
- Initiate glioma registration-enabling trial with vorasidenib
- Submit sNDA for TIBSOVO® in second line or later cholangiocarcinoma
- Achieve proof-of-concept for mitapivat in thalassemia
- Complete enrollment in mitapivat PK deficiency pivotal trials ACTIVATE & ACTIVATE-T
- ✓ Complete AG-270 Phase 1 dose-escalation
- ✓ Initiate combination arms
- ✓ Begin dosing patients in AG-636 Phase 1 dose-escalation trial in lymphoma

Agios Clinical Pipeline

IC = Intensive Chemotherapy

CLINICAL PROGRAMS	INDICATION	DRUG DISCOVERY	EARLY STAGE CLINICAL DEVELOPMENT	LATE STAGE CLINICAL DEVELOPMENT	REGULATORY SUBMISSION	APPROVED	PRIMARY RIGHTS
TIBSOVO® **ivosidenib** (IDH1m inhibitor)	R/R AML		Phase 1 Dose-Escalation and Expansion		EU	U.S.	agios
	Frontline AML Monotherapy		Phase 1 Dose-Escalation and Expansion			U.S.	
	IC Eligible Frontline AML		Phase 1b 7+3 Combo	Phase 3 HOVON 7+3 Combo			
	IC Ineligible Frontline AML		Phase 1/2 Azacitidine Combo	Phase 3 AGILE Azacitidine Combo			
	Cholangio		Phase 1 Dose-Escalation and Expansion	Phase 3 ClarIDHy			
	Glioma		Perioperative Study				
IDHIFA® **enasidenib** (IDH2m inhibitor)	R/R AML			Phase 3 IDHENTIFY	EU	U.S.	agios Celgene Agios U.S. Co-promotion and Royalty
	IC Eligible Frontline AML		Phase 1b 7+3 Combo	Phase 3 HOVON 7+3 Combo			
	IC Ineligible Frontline AML		Phase 1/2 Azacitidine Combo				
Mitapivat (PKR activator)	Transfusion Independent PK Deficiency		Phase 2 DRIVE PK	Phase 3 ACTIVATE			agios
	Transfusion Dependent PK Deficiency			Phase 3 ACTIVATE-T			
	Thalassemia		Phase 2 Study				
Vorasidenib (brain-penetrant, pan-IDHm inhibitor)	Glioma		Perioperative Study	Phase 3 Study Planned for 4Q 2019			agios
	Solid Tumors		Phase 1 Dose-Escalation and Expansion				
AG-270 (MAT2A inhibitor)	MTAP-deleted Tumors		Phase 1 Dose-Escalation and Expansion				Celgene agios Subject to Celgene Option Joint Worldwide Collaboration
AG-636 (DHODH inhibitor)	Lymphoma		Phase 1 Dose-Escalation				agios

Agios Preclinical Pipeline

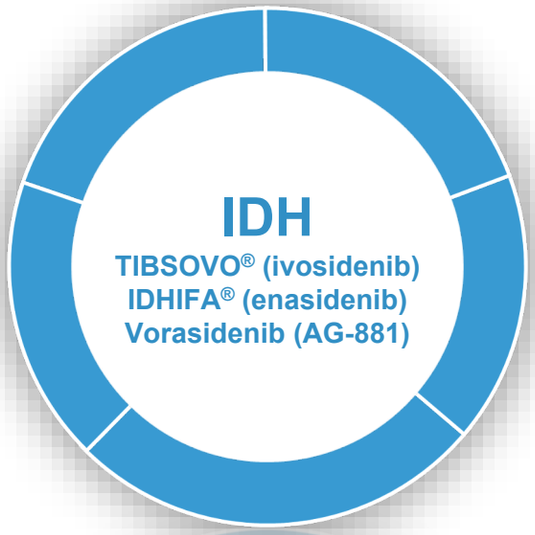
Program	Target Discovery	Target Validation	Drug Discovery	Drug Candidate
Oncology				
MAT2A Follow-Ons			●	
PTEN-mutant Solid Tumors			●	
Genetically Defined Heme Target			●	
Genetically Defined Heme Target			●	
Other Exploratory Programs	◐	◐		
Rare Genetic Diseases				
Pyruvate Kinase Activator Follow-Ons				●
Phenylketonuria (PKU)			●	
Erythroid Porphyria			●	
Friedreich's Ataxia			●	
Other Exploratory Programs	◐	◐		
Metabolic Immuno-Oncology (Celgene Collaboration)				
T-cell and Tumor Target			●	
Macrophage Target			●	
Macrophage Target		●		
Tumor Target		●		
Other Targets (T-cell, Macrophage, Tumor)	●	●		

● Metabolic Target ● Non-Metabolic Target ◐ Metabolic and Non-Metabolic Targets ▬ Celgene Collaboration

Clinical Development Progress

Chris Bowden, M.D., Chief Medical Officer

Productive Research & Discovery Engine Has Produced Four Key Targets with Multiple Disease Opportunities



AML

Low Grade Glioma

Cholangiocarcinoma

Chondrosarcoma

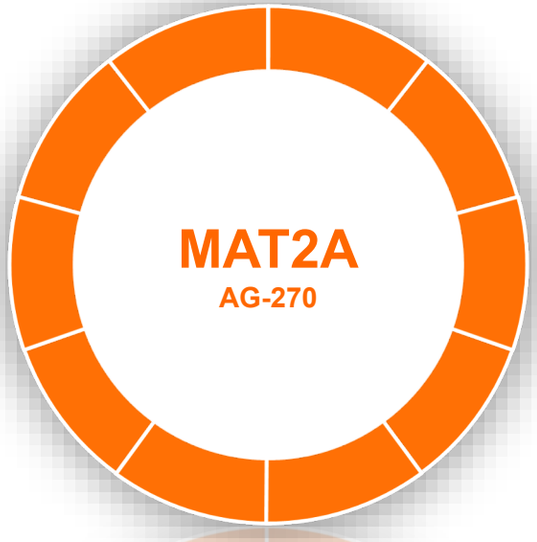
MDS

Adult PK Deficiency

Pediatric PK Deficiency

Sickle Cell Disease

Thalassemia



NSCLC

Bladder

Melanoma

Head & Neck

Pancreatic

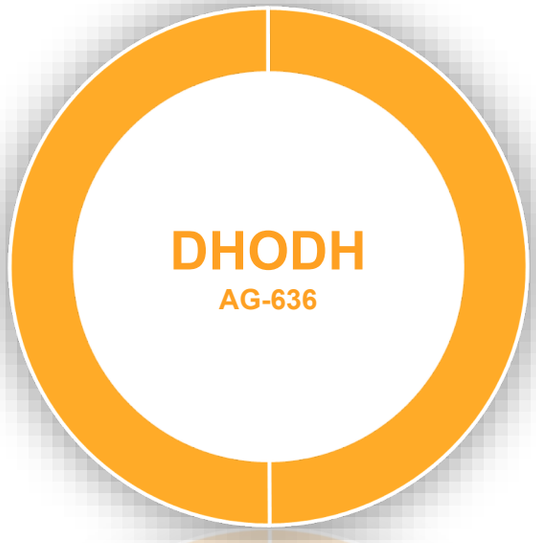
Glioblastoma

DLBCL

Esophageal

Gastric

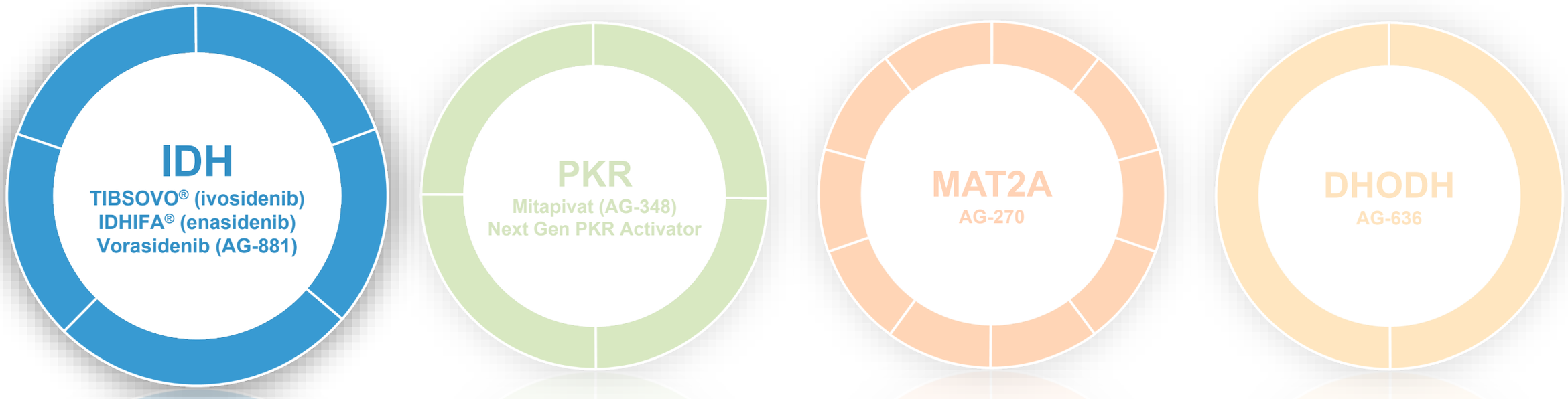
Mesothelioma



Lymphoma

AML

Productive Research & Discovery Engine Has Produced Four Key Targets with Multiple Disease Opportunities



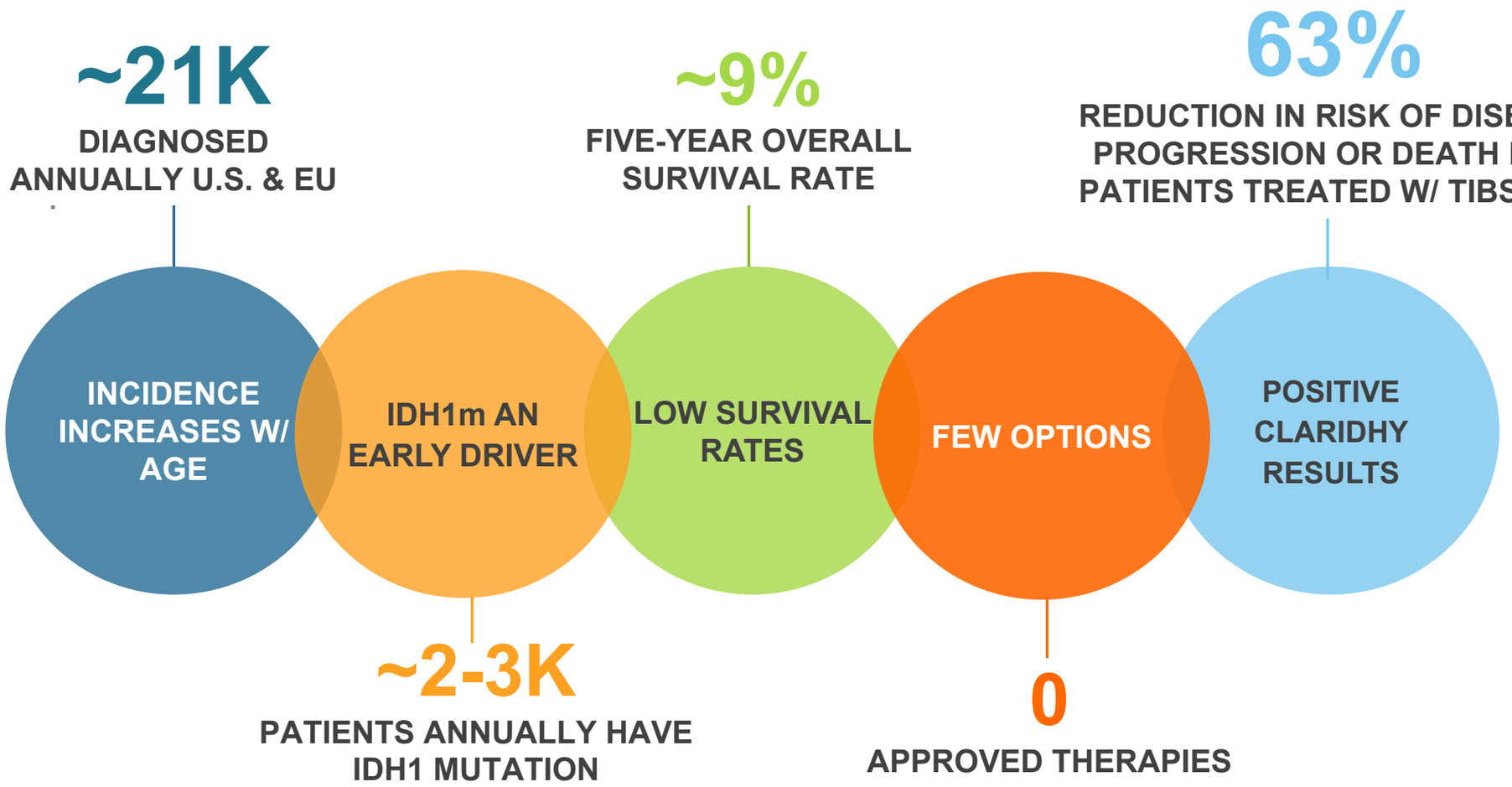
AML
Low Grade Glioma
Cholangiocarcinoma
Chondrosarcoma
MDS

Adult PK Deficiency
Pediatric PK Deficiency
Sickle Cell Disease
Thalassemia

NSCLC
Bladder
Melanoma
Head & Neck
Pancreatic
Glioblastoma
DLBCL
Esophageal
Gastric
Mesothelioma

Lymphoma
AML

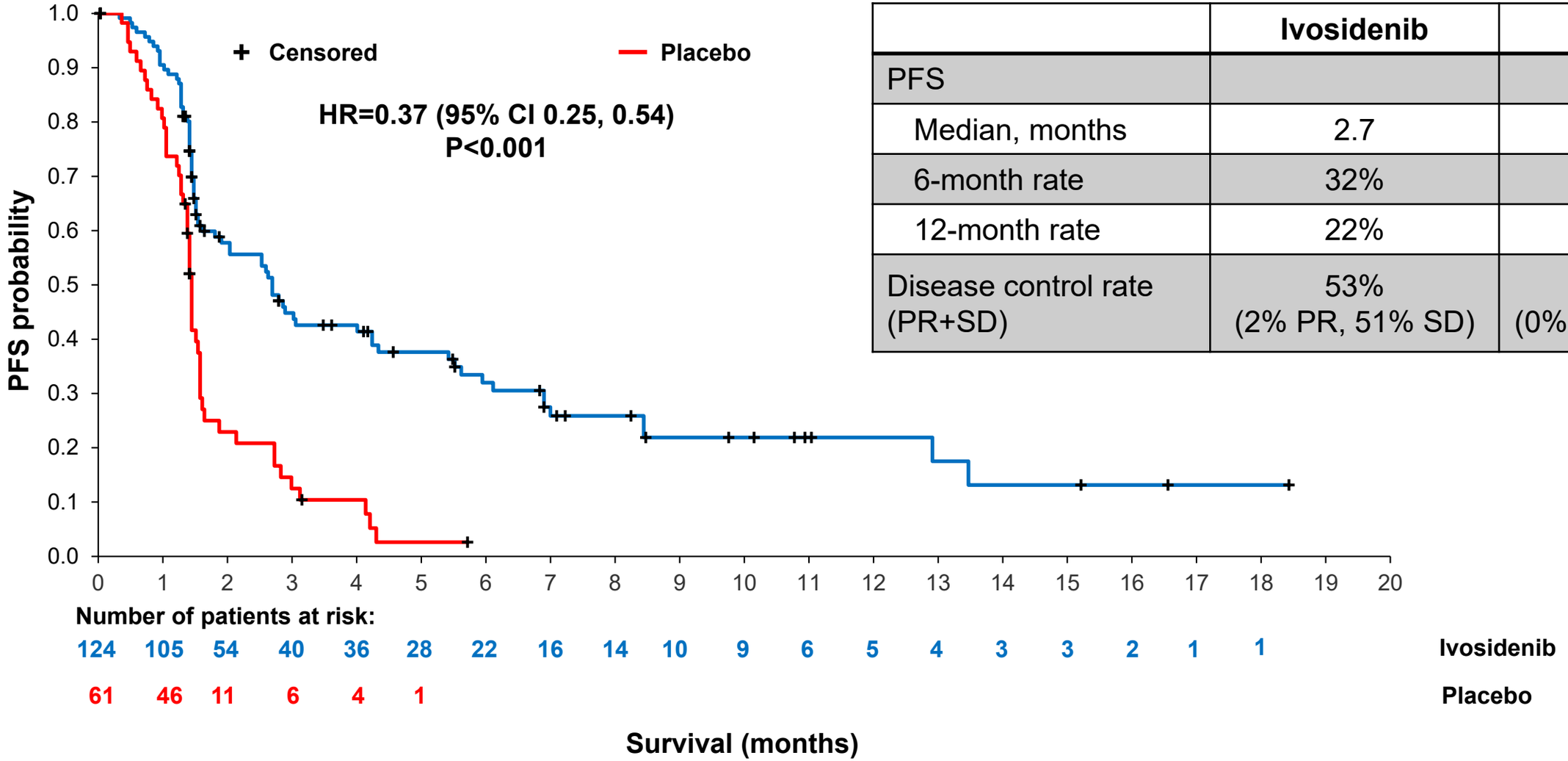
Plan to File sNDA for TIBSOVO® in Second-line or Later Cholangiocarcinoma by Year-end 2019



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; data from ESMO 2019

Phase 3 ClarIDHy Study Achieved Primary Endpoint, Demonstrating Statistically Significant Improvement in PFS

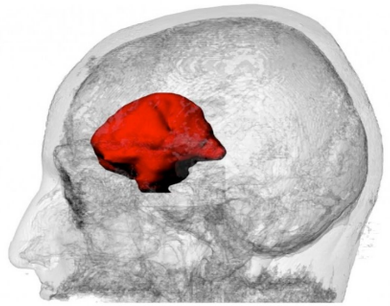
Safety Profile Consistent with Published Phase 1 Data in Patients with IDH1 Mutant Solid Tumors



	Ivosidenib	Placebo
PFS		
Median, months	2.7	1.4
6-month rate	32%	NE
12-month rate	22%	NE
Disease control rate (PR+SD)	53% (2% PR, 51% SD)	28% (0% PR, 28% SD)

NE=not estimable; PR=partial response; SD=stable disease; Data presented at ESMO 2019

Current Treatment Paradigm for IDHm Gliomas



SURGERY

GLIOMA DIAGNOSIS

IDH1-mutant Glioma (~80%)

WHO GRADE II

WATCH AND WAIT

WHO GRADE III

Radiotherapy → PCV (or temozolomide plus radiotherapy → temozolomide)

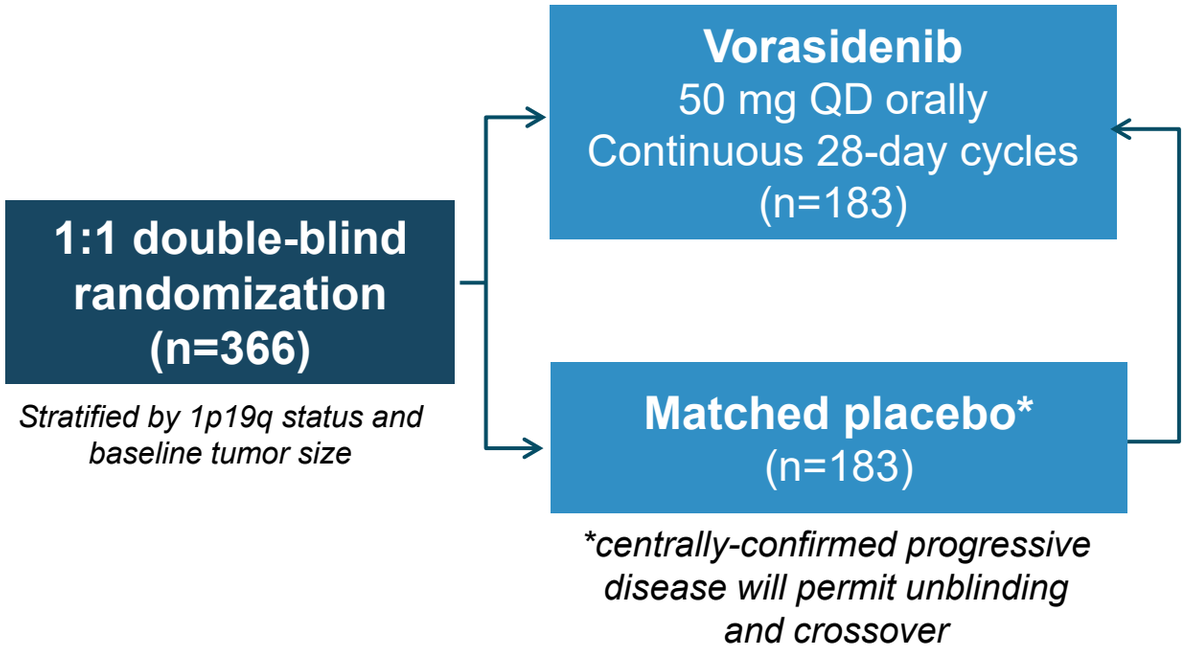
Sources: Steven Keating and Ahmed Hosny; N Engl J Med 2015; 372:2481-2498
PCV = procarbazine, lomustine and vincristine

Global Phase 3 INDIGO Study of Vorasidenib in IDH Mutant Low-Grade Glioma

INDIGO | INVESTIGATING VORASIDENIB IN GLIOMA

Key Eligibility Criteria

- ≥12 years of age
- IDH-mutated Grade 2 oligodendroglioma or astrocytoma per WHO 2016
- Prior surgery only
- Measurable residual or recurrent disease



Endpoints

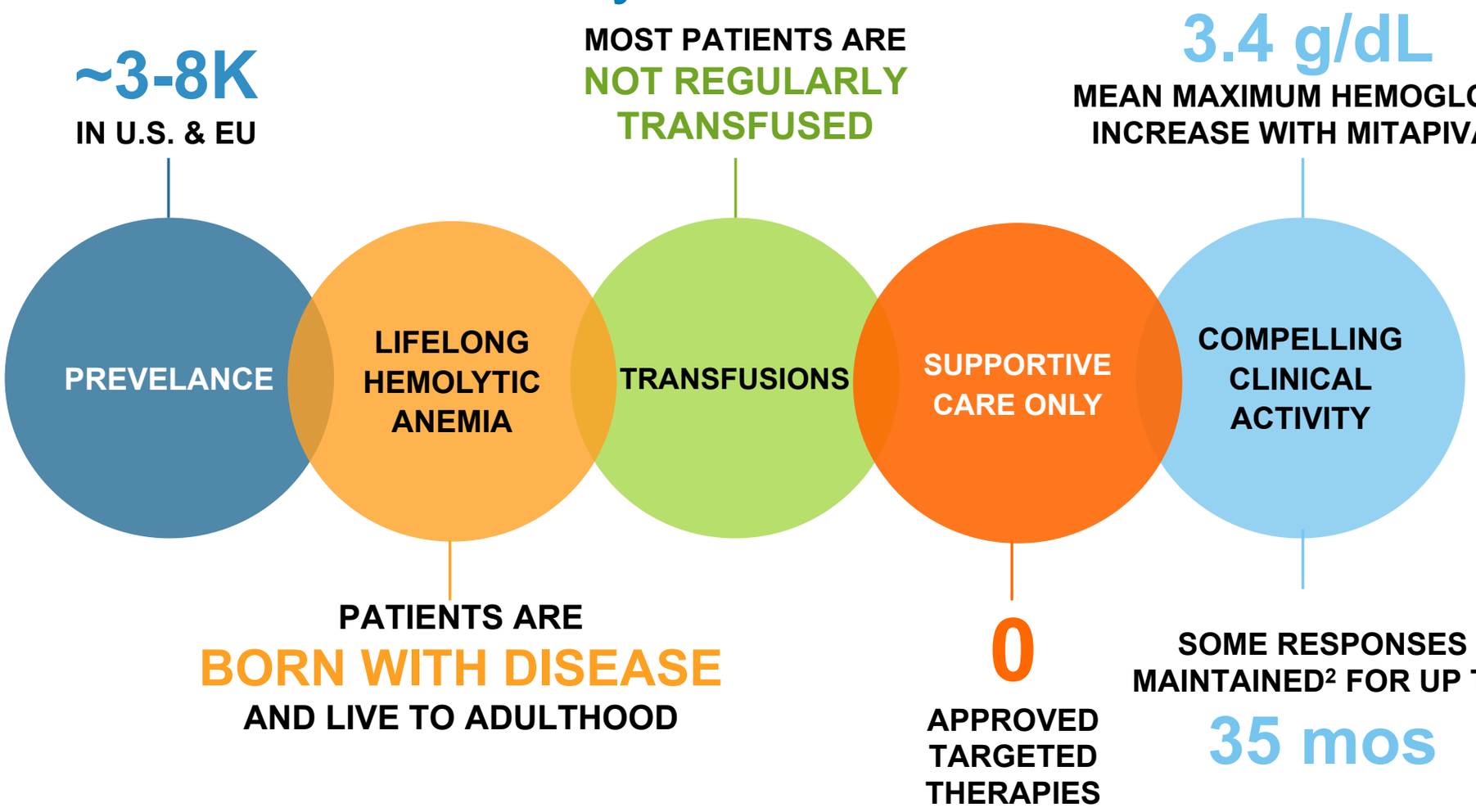
Primary: Progression free survival (by BIRC)

Secondary/Exploratory: Tumor volume, safety, ORR, OS, QOL, seizures, neuro-cognitive function, time to next intervention

BIRC = blinded independent review committee, ORR = overall response rate, OS = overall survival, QOL = quality of life, WHO = World Health Organization

Registration-enabling study on track to initiate by year-end 2019

Opportunity for Mitapivat 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; Grace R et al. *N Engl J Med 2019;381:933-44*

[1]Mean maximum hemoglobin increase of 3.4 g/dL in patients to had a >1.0 g/dL increase in haemoglobin on study; [2] 19 pts remain in the extension phase with a median treatment duration of 28.9 months [range 21.6-34.8]

New data from the extension phase of the Phase 2 DRIVE PK study of mitapivat in adults with PK deficiency accepted for presentation at ASH

Mitapivat Path to Approval

ACTIVATE

- **~80 patients** treated for 6 months
- **Primary endpoint:** portion of patients achieving 1.5 g/dL Hb increase over multiple visits

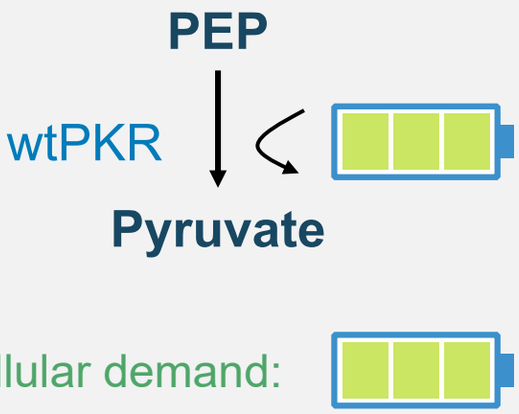
ACTIVATE-T

- **Up to 40 patients,** minimum of 6 transfusions 1 year before enrollment
- **Primary endpoint:** reduction in transfusion burden over 6 months compared to patient's transfusion history

- **Disease awareness**
- **Improving path to diagnosis**
- **Patient voice**

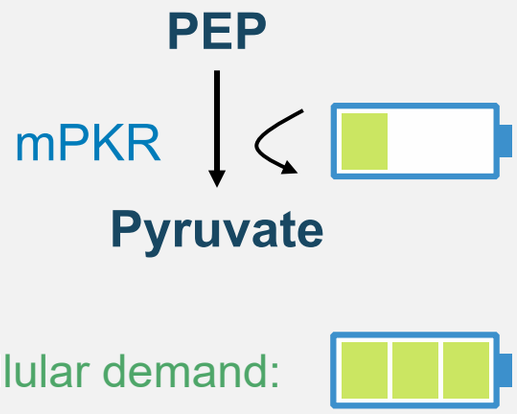
PK Activation Represents Opportunities Across Hemolytic Anemias

Normal Red Cell



ATP production meets demand

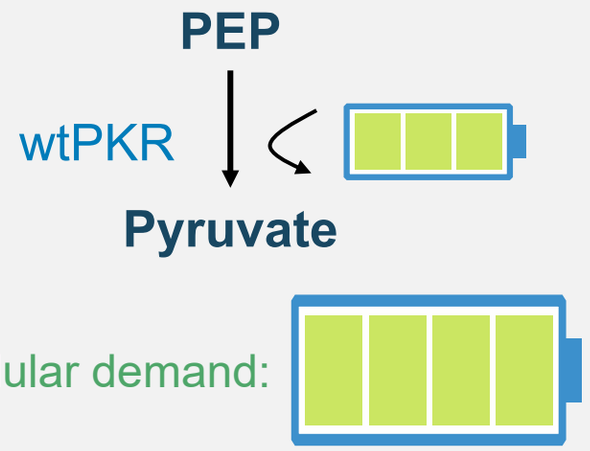
Pyruvate Kinase Deficiency



Inadequate production: ATP deficiency

✓ **Proof of concept achieved**

Other Hemolytic Anemias



Increased demand: ATP deficiency

Thalassemia Phase 2 initiated; NIH sponsored trial in sickle cell disease initiated

AG-270 Advancing to Next Phase of Clinical Development

SINGLE AGENT PHASE 1 DOSE-ESCALATION COMPLETE

39 treatment-refractory advanced solid tumors or lymphoma patients with MTAP/CDKN2A deletion

- ☑ AG-270 generates reductions in plasma SAM concentration and in levels of tumor SDMA at well-tolerated doses
- ☑ Safety profile well characterized
- ☑ MTD determined to be 200 mg QD

COMBINATION ARMS INITIATED

AG-270 + docetaxel in MTAP-deleted NSCLC (2nd line)
N = up to 40

AG-270 + nab-paclitaxel and gemcitabine in MTAP-deleted pancreatic ductal adenocarcinoma (1st or 2nd line)
N = up to 45

ClinicalTrials.gov Identifier: NCT03435250
Data presented at AACR-NCI-EORTC 2019

2019 Key Milestones & Data Presentations Position Agios for Long-term Value Creation

Key 2019 Milestones

- ✓ FDA approval and commercialization of monotherapy TIBSOVO® in untreated AML
- ✓ Initiate AG-636 Phase 1 dose-escalation trial in lymphoma in 1H 2019
- ✓ Complete AG-270 Phase 1 dose-escalation and select go forward dose
- ✓ Initiate expansion arms in the AG-270 Phase 1 study in Q3 2019
- Achieve proof-of-concept for mitapivat in thalassemia in 2H 2019
- Submit sNDA for TIBSOVO® in second line or later cholangiocarcinoma by YE
- Initiate glioma registration-enabling trial with vorasidenib by YE
- Complete enrollment in PK deficiency pivotal trials ACTIVATE-T and ACTIVATE by YE

Key Upcoming Data Presentations

- Updated data from the perioperative study of ivosidenib and vorasidenib accepted for presentation at the SNO Annual Meeting
- Data from IDH and PKR programs have been accepted for presentation at ASH, including:
  - New data from the extension phase of the Phase 2 DRIVE PK study of mitapivat in adults with PK deficiency
  - Important translational data from the Phase 1 study of TIBSOVO® and azacitidine in frontline AML

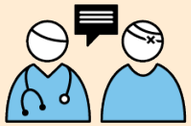
TIBSOVO® Commercial Update

Darrin Miles, Senior Vice President, U.S. Commercial & Global Marketing

agios

TIBSOVO® Q3 2019 Performance

$17.4M Net U.S. Sales of TIBSOVO®



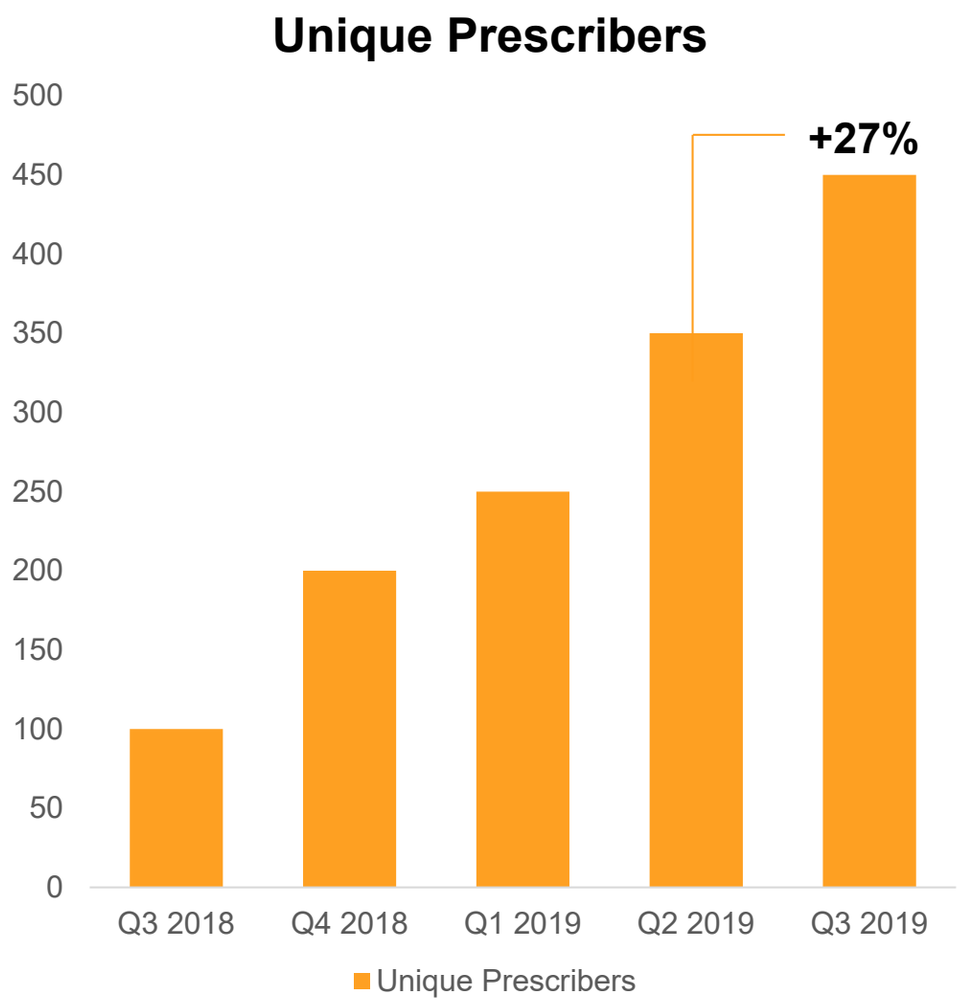
+90% Academic and Community Physicians Testing for IDH1/IDH2 mutations

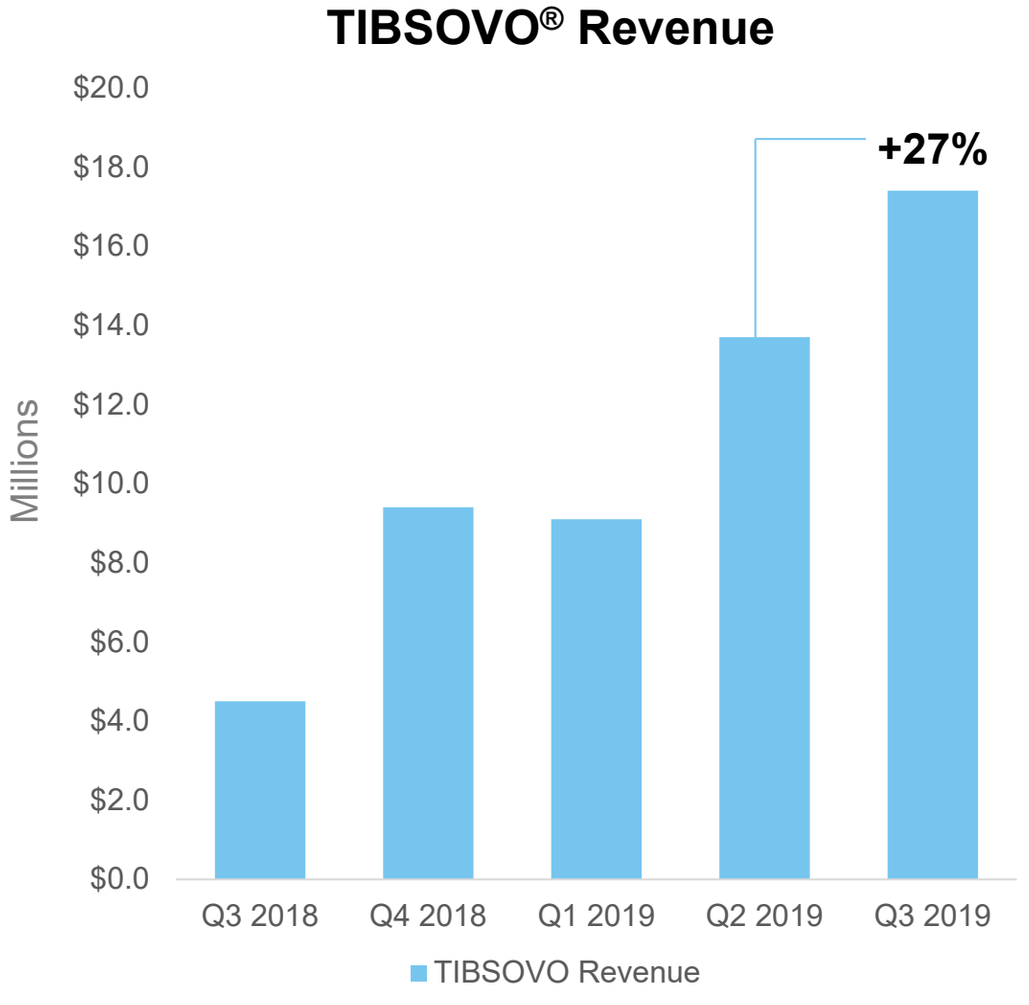
~450 Unique Prescribers; Continue to Broaden Prescriber Base

Increase in Treatment Duration to ~4-5 Months

Market research as of February 2019; Agios estimates

Demonstrated Ability to Drive Commercial Performance During First Year of the R/R AML Launch





Agios estimates

Third Quarter 2019 Financial Results

Andrew Hirsch, Chief Financial Officer and Head of Corporate Development

agios

Third Quarter 2019 Financial Results

Statement of Operations	Three Months Ended 9/30/19	Three Months Ended 9/30/18
Total Revenue	$26.0M	$15.2M
Collaboration Revenue	5.9M	8.7M
TIBSOVO® Net Sales	17.4M	4.5M
Royalty Revenue	2.7M	2.0M
Cost of Sales	0.4M	0.7M
Research & Development Expense	101.7M	82.6M
Selling, General & Administrative Expense	33.0M	31.1M

Balance Sheet	9/30/19	12/31/18
Cash, Cash Equivalents and Marketable Securities	$540.5M	$805.4M

September 30, 2019 cash balance provides runway through at least the end of 2020