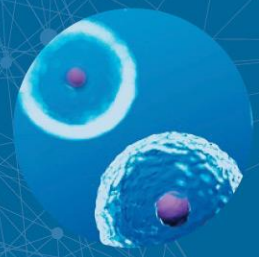
agios

Fourth Quarter and Full Year 2019 Financial Results

February 13, 2020



Agios Conference Call Participants

Prepared Remarks

Introduction

- HOLLY MANNING, Associate Director, Investor Relations

Business Highlights and 2020 Milestones

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

Clinical Development Progress

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

Commercial Update

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

Fourth Quarter and Full Year 2019 Financial Results

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

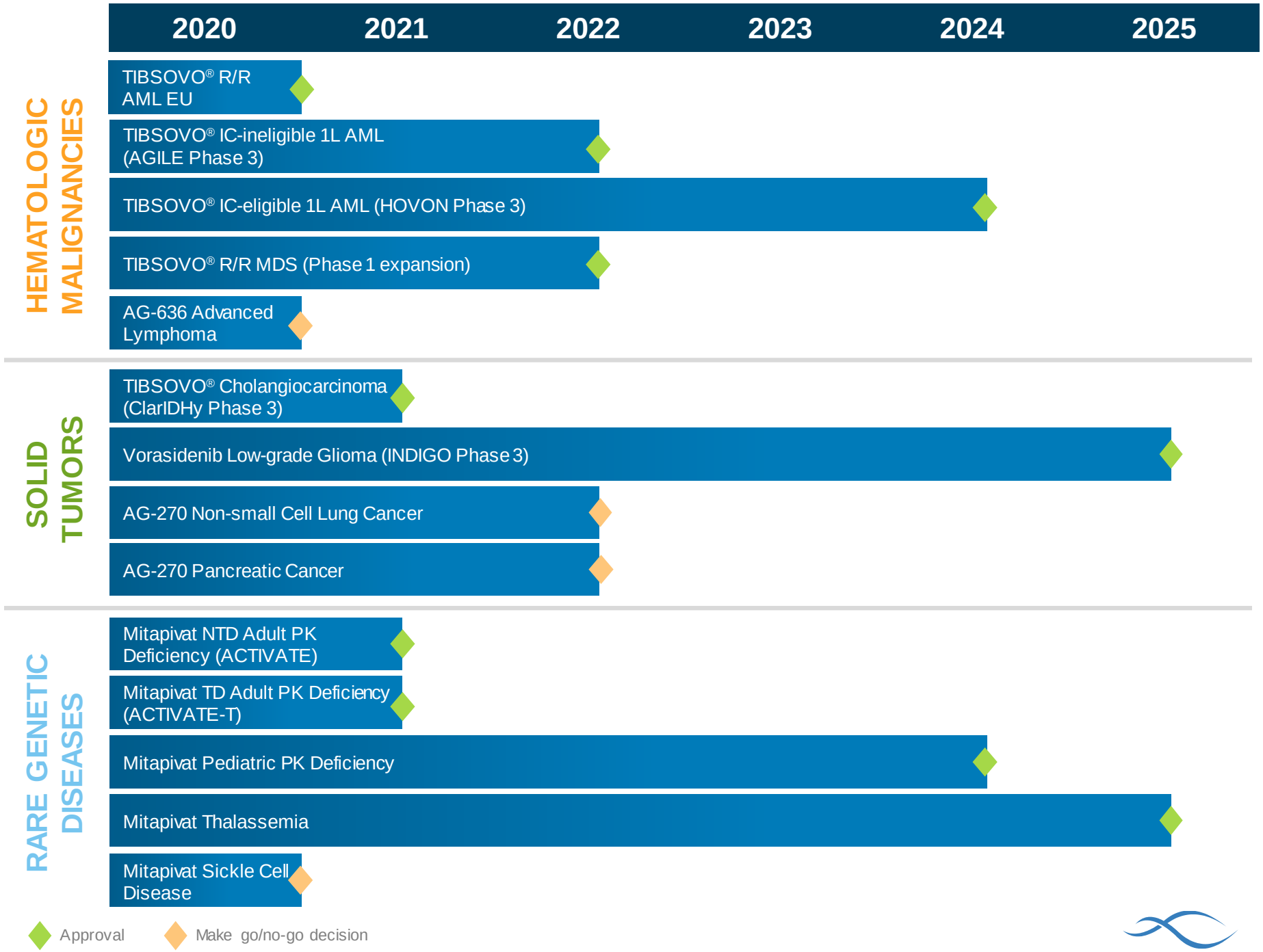
Forward Looking Statements

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), mitapivat, vorasidenib, AG-270, AG-636 and AG-946; the potential benefits of Agios' product candidates; Agios's strategic vision and goals for 2025; its key milestones and guidance for 2020; 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," "goal," "hope," "milestone," "plan," "potential," "possible," "strategy," "will," "vision," 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; 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.

Agios 2025 Vision: Focused Innovation. Ambitious Development. Transformative Treatments for Patients Across Three Focus Areas.

	NOW	2025
COMMERCIAL	2 MEDICINES	4 MEDICINES
LABEL EXPANSION	2 INDICATIONS	8+ INDICATIONS
PRODUCTIVE DISCOVERY ENGINE	4 MOLECULES IN THE CLINIC	6+ MOLECULES IN THE CLINIC
FINANCIAL	$105-115M EXPECTED U.S. TIBSOVO® 2020 REVENUE	CASH FLOW POSITIVE

Multiple Potential Near- and Long-term Value Drivers Across All Focus Areas



Agios 2020 Key Milestones

HEMATOLOGIC MALIGNANCIES

- Achieve full-year U.S. revenue for TIBSOVO® $105-115M
- Receive CHMP opinion for TIBSOVO® in mIDH1 relapsed/refractory AML
- Complete enrollment in AGILE Phase 3 trial of TIBSOVO® + azacitidine in frontline mIDH1 AML
- Complete enrollment in MDS arm of TIBSOVO® Phase 1

SOLID TUMORS

- File sNDA for TIBSOVO® in mIDH1 previously treated cholangiocarcinoma

RARE GENETIC DISEASES

- Topline data in PK deficiency from ACTIVATE and ACTIVATE-T
- Present data from mitapivat Phase 2 thalassemia study and finalize pivotal trial strategy in thalassemia
- Achieve proof-of-concept for mitapivat in sickle cell disease
- Initiate first-in-human study for next generation PKR activator, AG-946

RESEARCH

- Achieve at least 1 new development candidate

Clinical Development Progress

Chris Bowden, M.D., Chief Medical Officer

1. Malignant Hematology
2. Solid Tumors
3. Rare Genetic Diseases

PKR Activation Has Potential Broad Utility Across Hemolytic Anemias

~3-8K PATIENTS IN U.S. & EU

Pyruvate Kinase Deficiency

NTD Adult PKD	Phase 3 Enrollment Closed
TD Adult PKD	Phase 3 Enrollment Closed
Pediatric PKD	Pivotal Plan by YE

~18-23K PATIENTS IN U.S. & EU

β- and α-Thalassemia

NTD β- and α-Thalassemia	Phase 2
Thalassemia	Pivotal Plan by YE

~120-135K PATIENTS IN U.S. & EU

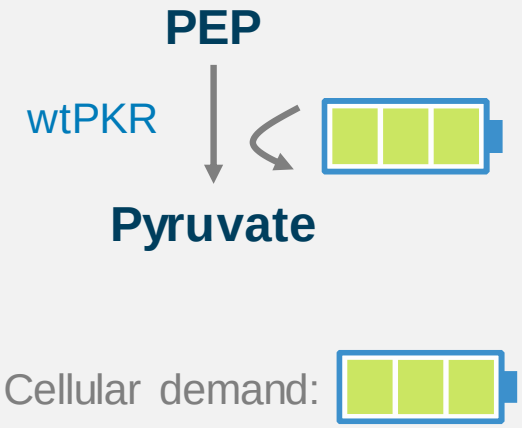
Sickle Cell Disease

Adult SCD	NIH CRADA

Source: Agios estimates, market research

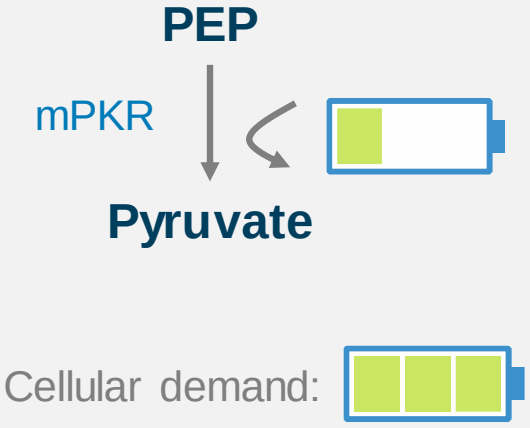
PKR Activation Represents Unique Mechanism of Action with Potential to Address Broad Range of Hemolytic Anemias

Normal Red Cell



ATP production meets demand

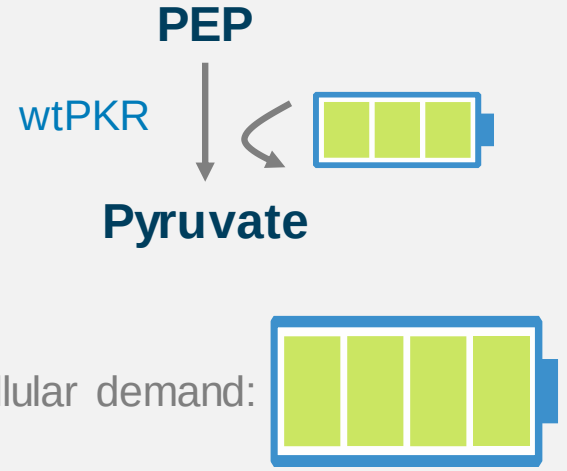
Pyruvate Kinase Deficiency



Inadequate production: ATP deficiency

- Proof-of-concept achieved
- Adult PK deficiency approval expected in 2021
- Pediatric PK deficiency pivotal strategy to be finalized in 2020

Other Hemolytic Anemias



Increased demand: ATP deficiency

- Thalassemia proof-of-concept achieved
- NIH sponsored trial in sickle cell disease ongoing

Clinical Proof-of-concept for Mitapivat Established in Non-transfusion-dependent Thalassemia

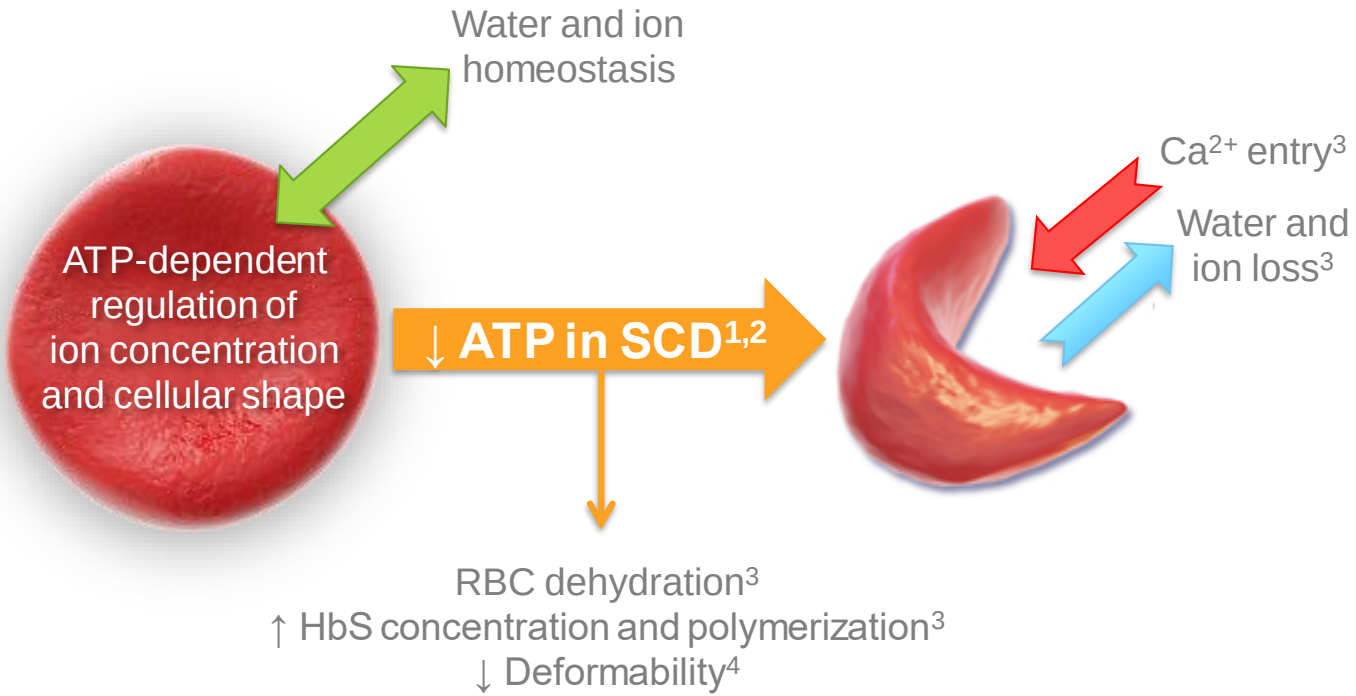
7 of 8 efficacy evaluable patients achieved a hemoglobin increase of ≥1.0 g/dL from baseline in at least one assessment (weeks 4 – 12)

In responding patients, the mean hemoglobin increase from baseline was 1.76 g/dL (range, 0.9 – 3.3 g/dL)

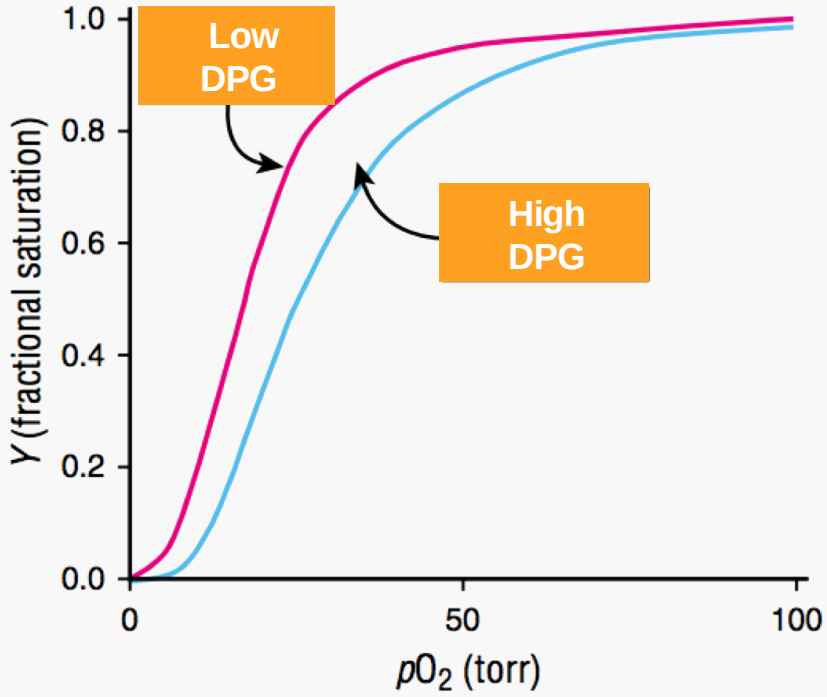
Majority of adverse events were Grade 1 or 2 and consistent with previously published Phase 2 data for mitapivat in patients with PK deficiency

Updated Phase 2 thalassemia data to be submitted for presentation at EHA and pivotal strategy to be finalized by YE 2020

Therapeutic Hypothesis for Wildtype PKR Activation in Sickle Cell Disease: 2,3-DPG and ATP Modulation Improves Anemia and Reduces Sickling



2,3-DPG Shifts the Oxygen Saturation Curve



ATP, adenosine triphosphate; HbS, sickle cell hemoglobin; RBC, red blood cell; SCD, sickle cell disease.

1. Palek J, Liu SC. J Supramol Struct. 1979;10(1):79-96. 2. Glader BE, et al. Br J Haematol. 1978;40(4):527-32.
3. Bogdanova A, et al. Int J Mol Sci. 2013;14(5):9848-72. 4. Park Y, et al. Proc Natl Acad Sci USA. 2010;107(4):1289-94.

Expect to establish proof-of-concept for PKR activation in sickle cell disease by mid-2020

Significant Growth Potential in Malignant Hematology

~4K PATIENTS IN U.S. & EU

IDH1 Mutant Acute Myeloid Leukemia (AML)

TIBSOVO®

R/R AML	U.S. Approval; MAA Under Review
1L Monotherapy	U.S. Approval
1L HMA Combo	Phase 3
1L 7+3 Combo	Phase 3

<1K PATIENTS IN U.S.

IDH1 Mutant Myelodysplastic Syndrome (MDS)

TIBSOVO®

R/R MDS	Phase 1 Expansion

~55K PATIENTS IN U.S. & EU

Mantle Cell and Diffuse Large B Cell Lymphoma

AG-636

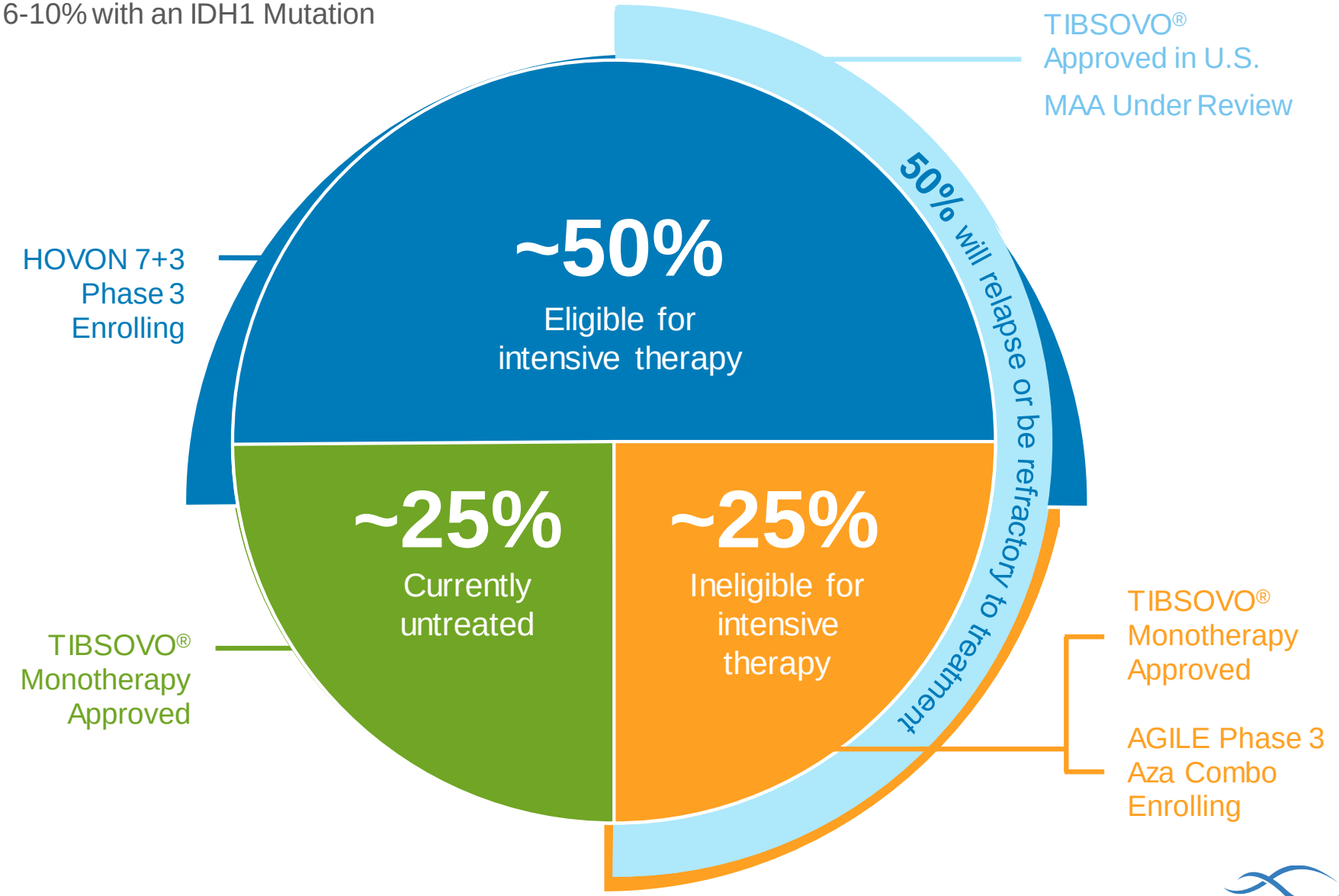
R/R Lymphoma	Phase 1

Source: Agios estimates, market research, SEER, MDS Foundation, Datamonitor

Advancing Toward Largest Opportunity for mIDH1 AML: Intensive and Non-Intensive Therapy Combinations

50K AML Patients Diagnosed Per Year in U.S. and EU

6-10% with an IDH1 Mutation



Sources: SEER. Cancer Stat Facts: AML 2015 and Epiphany EPIC oncology numbers; American Cancer Society. AML 2017.

Four Distinct Solid Tumor Opportunities Across Three Clinical Molecules

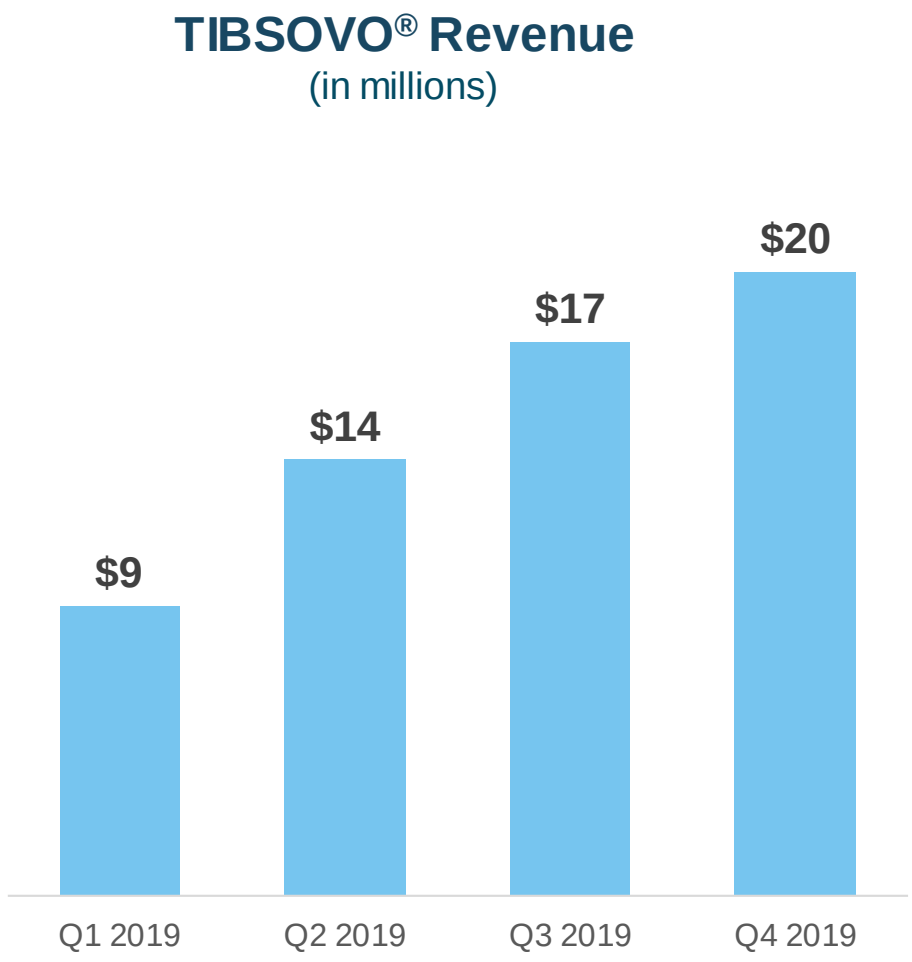
~2-3K PATIENTS IN U.S. & EU	~9K PATIENTS IN U.S. & EU	~9K PATIENTS IN U.S.	~10K PATIENTS IN U.S.
IDH1 Mutant Cholangiocarcinoma	**IDH Mutant Low Grade Glioma**	**MTAP-Deleted Non-Small Cell Lung Cancer**	**MTAP-Deleted Pancreatic Cancer**
TIBSOVO®	**Vorasidenib**	**AG-270**	**AG-270**
R/R Cholangio sNDA 2020	**Low-grade Glioma** Phase 3	**2nd Line NSCLC** Phase 1 Combo	**1st or 2nd Line Pancreatic Cancer** Phase 1 Combo

Source: Agios estimates, market research

TIBSOVO® Commercial Update

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

Successful TIBSOVO® Launch in R/R and Frontline AML Result of Focused Commercial Effort



$60M
Full Year 2019 Product Revenue

$105 – 115M
U.S. Net Sales Guidance for 2020

~515
Unique Prescribers as of Q4 2019

>1,000
Patients Treated Since Launch

Source: Agios estimates

Fourth Quarter and Full Year 2019 Financial Results

Andrew Hirsch, Chief Financial Officer and Head of Corporate Development

Fourth Quarter and Full Year 2019 Financial Results

Statement of Operations	Three Months Ended 12/31/19	Three Months Ended 12/31/18	Year Ended 12/31/19	Year Ended 12/31/18
Total Revenue	$35.4M	$30.0M	$117.9M	$94.4M
Collaboration Revenue	12.9M	18.4M	47.5M	73.3M
TIBSOVO® Net Sales	19.6M	9.4M	59.9M	13.8M
Royalty Revenue	3.0M	2.2M	10.5M	7.2M
Cost of Sales	0.3M	0.7M	1.3M	1.4M
Research & Development Expense	106.2M	93.8M	410.9M	341.3M
Selling, General & Administrative Expense	34.8M	31.9M	132.0M	114.1M

Balance Sheet	12/31/19	12/31/18
Cash, Cash Equivalents and Marketable Securities	$717.8M	$805.4M

December 31, 2019 cash balance provides runway through at least the end of 2021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Q&A