agios

First Quarter 2020 Financial Results

April 30, 2020

Agios Conference Call Participants

TOPIC	PARTICIPANT
Introductions	Holly Manning, Director of Investor Relations
Business Update & COVID-19 Response	Jackie Fouse, Ph.D., Chief Executive Officer
Clinical Development Update	Chris Bowden, M.D., Chief Medical Officer
TIBSOVO® Performance	Darrin Miles, Senior Vice President, U.S. Commercial & Global Marketing
First Quarter 2020 Financial Results	Andrew Hirsch, Chief Financial Officer & Head of Corporate Development
Joining for Q&A	Bruce Car, Ph.D., Chief Scientific 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 TIBSOVO® (ivosidenib), IDHIFA® (enasidenib), mitapivat, vorasidenib, AG-270, and AG-946; the potential benefits of Agios' product candidates; 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 benefits 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, without limitation: risks and uncertainties related to the impact of the COVID-19 pandemic to Agios' business, operations, strategy, goals and anticipated milestones, including its ongoing and planned research activities, ability to conduct ongoing and planned clinical trials, clinical supply of current or future drug candidates, commercial supply of current or future approved products, and launching, marketing and selling current or future approved products; 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.

COVID-19 Response Focused on Key Priorities

Our Patients

- Continued to provide uninterrupted commercial supply of TIBSOVO® and investigational medicines
- Ongoing access to myAgios™ patient support services
- Activated the clinical operations response team focused on clinical trial continuity

Our Employees

- Vast majority of employees are working from home
- Increased support and resources for employees during disruption
- Regular communication and implementing new ways of staying connected

Our Communities

- Organized a donation of Agios's PPE supply to local hospitals
- Raised more than $22,000 for The Greater Boston Food Bank
- Encouraged blood donations

Clinical Development Updates

Chris Bowden, M.D., Chief Medical Officer

What's New: **RARE GENETIC DISEASES**

PK DEFICIENCY

- Enrollment complete in mitapivat PK deficiency pivotal trials ACTIVATE (80 patients) and ACTIVATE-T (27 patients)
- ACTIVATE and ACTIVATE-T topline data now expected between YE 2020 and mid-2021 vs. previous guidance of YE 2020 based on anticipated challenges with clinical trial site access

THALASSEMIA

- Enrollment complete in Phase 2 trial of mitapivat in alpha- and beta-thalassemia (20 patients)
- Data on 13 patients have been accepted for presentation at EHA

SICKLE CELL DISEASE

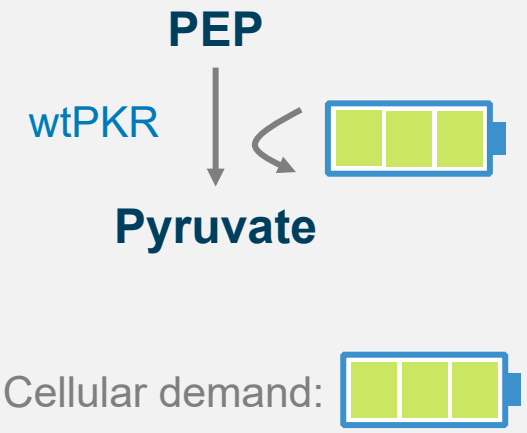
- NIH has amended the study to include a 100 mg BID dose
- New enrollment is paused during the COVID-19 pandemic
- The decision on proof-of-concept remains on track for mid-2020
- Data are expected to be submitted by NIH for presentation at ASH

AG-946

- Phase 1 study of AG-946, next-generation PKR activator, in healthy volunteers expected to initiate in mid-2020

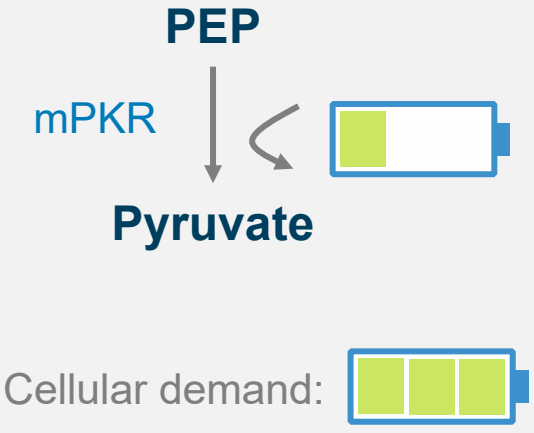
PKR Activation Represents Unique Mechanism of Action with Potential to Address Broad Range of Hemolytic Anemias

Normal Red Cell



ATP production meets demand

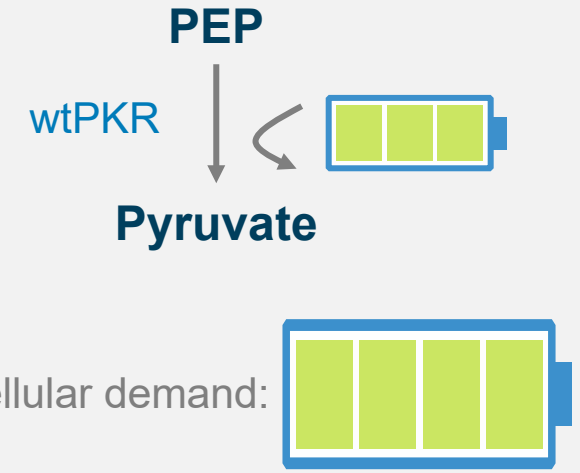
Pyruvate Kinase Deficiency



Inadequate production: ATP deficiency

- Proof-of-concept achieved
- Adult PK deficiency approval expected in 2022
- Pediatric PK deficiency pivotal strategy expected to be finalized YE 2020

Other Hemolytic Anemias

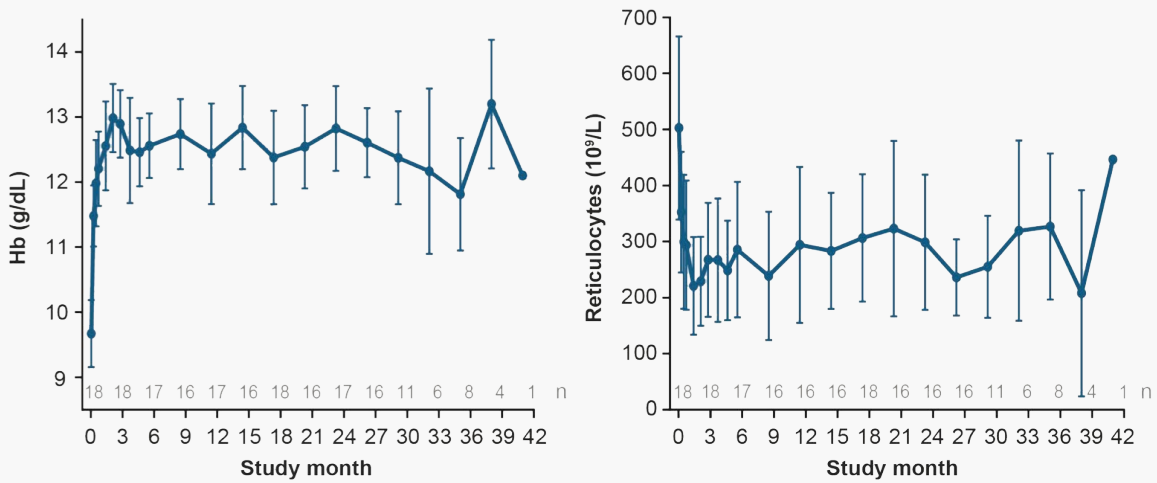


Increased demand: ATP deficiency

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

Mitapivat has Potential to be First Disease-modifying Therapy for Patients with PK Deficiency

Improvements in Hemoglobin and Other Hemolysis Markers Maintained for More Than 3 Years in Responding Patients from DRIVE PK Extension



Chronic daily dosing with mitapivat well tolerated for a median of 3 years and up to 42 months

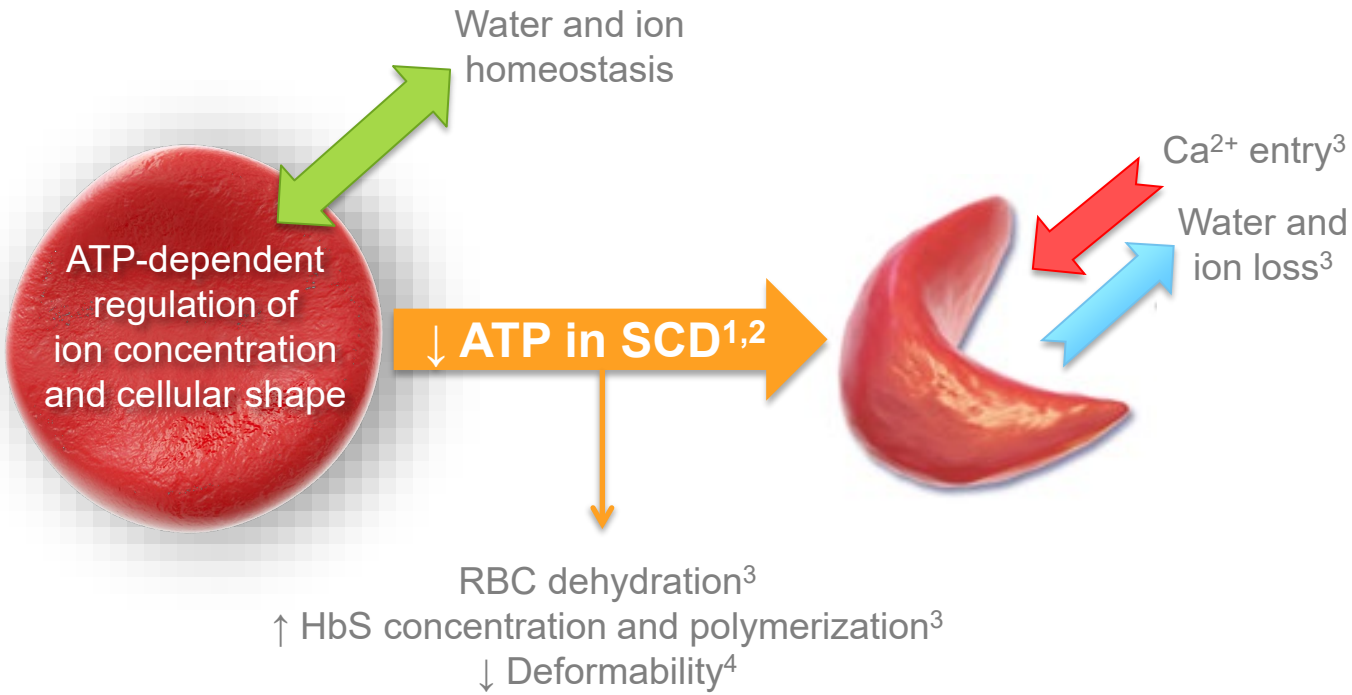
COMPLICATIONS & COMORBIDITIES REGARDLESS OF TRANSFUSION STATUS	SUPPORTIVE CARE ONLY	HIGH RISK OF IRON OVERLOAD
HIGHER LIFETIME RATES OF PULMONARY HYPERTENSION, OSTEOPOROSIS, AND LIVER CIRRHOSIS	**0** APPROVED THERAPIES	**38%** OF PATIENTS NOT RECEIVING REGULAR TRANSFUSIONS EXPERIENCE IRON OVERLOAD

Source: Data presented at ASH 2019; van Beers EJ, et al. Haematologica. 2019;104(2):e51-e53.

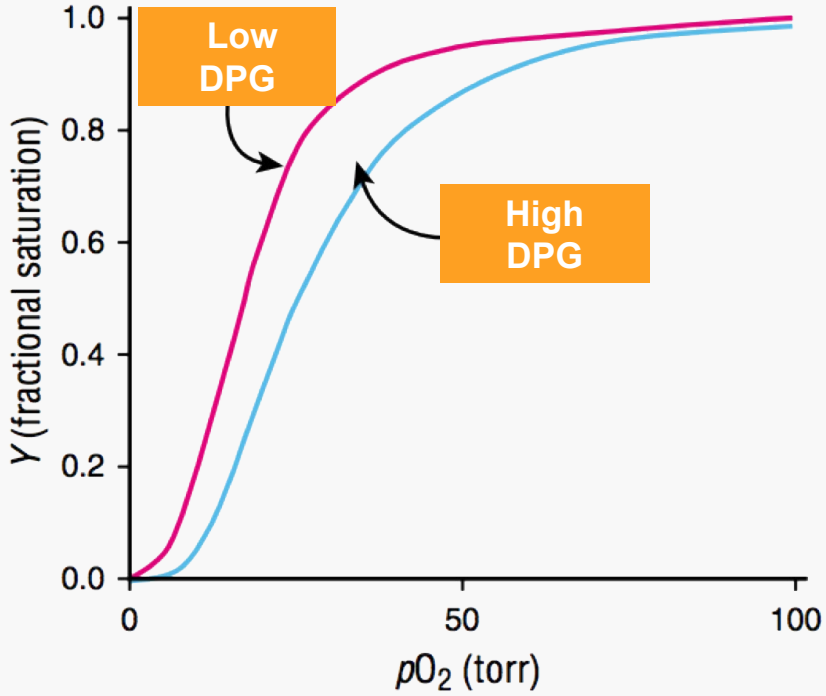
Clinical Proof-of-concept for Mitapivat Established in Non-transfusion-dependent Thalassemia

- 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 presented at EHA and pivotal strategy expected 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 make a decision on proof-of-concept for mitapivat in sickle cell disease by mid-2020

What's New: **MALIGNANT HEMATOLOGY**

AML

- EU regulatory process for TIBSOVO® in R/R AML remains on track with a CHMP opinion expected by YE 2020
- Phase 3 AGILE enrollment completion now expected in 2021 vs. previous guidance of YE 2020 due to COVID-19 related delays in site start-up activities and enrollment interruptions
- HOVON150 Phase 3 enrollment has slowed as a result of COVID-19 related delays in site start-up activities and enrollment interruptions

MDS

- MDS expansion arm enrollment completion now expected in 2021 vs. previous guidance of YE 2020 due to COVID-19 related delays in site start-up activities and enrollment interruptions

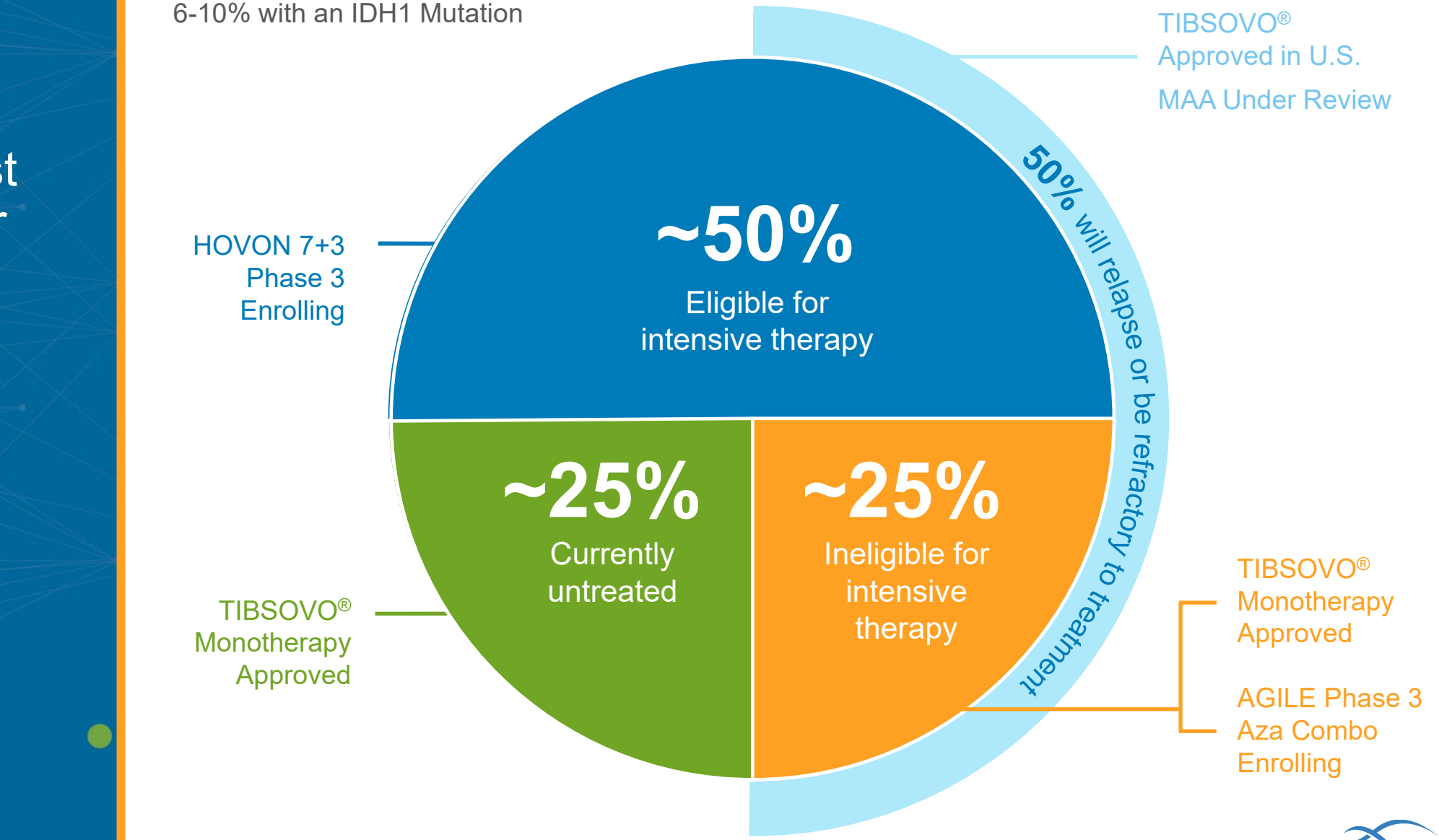
LYMPHOMA

- As a result of limited enrollment in the Phase 1 study of AG-636, a DHODH inhibitor, Agios will stop in-house development and evaluate partnering options

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.

What's New: **SOLID TUMORS**

CHOLANGIOCARCINOMA

- Mature OS data from ClarIDHy Phase 3 study of TIBSOVO® expected in mid-2020, but delays anticipated in collecting data from trial sites and executing the data cleaning process
- sNDA for TIBSOVO® in previously treated IDH1 mutant cholangiocarcinoma now expected between the end of 2020 and mid-2021 vs. previous guidance of YE 2020

GLIOMA

- Vorasidenib Phase 3 INDIGO trial enrollment has slowed as a result of COVID-19 related delays in site start-up activities and enrollment interruptions
- An update on timing of enrollment completion expected when site activation is complete and patient enrollment has returned to expected levels
- Updated data from the Phase 1 study of vorasidenib in non-enhancing low-grade glioma accepted for presentation at ASCO

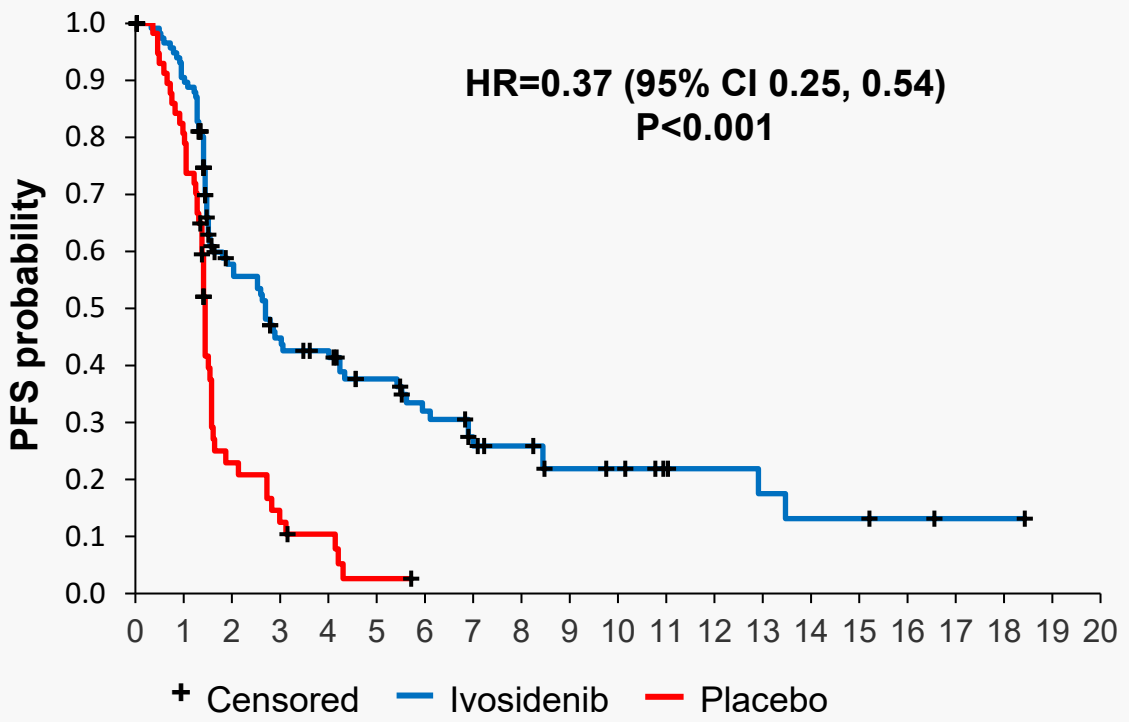
NSCLC & PANC

- Enrollment in the AG-270 Phase 1 combination arms has slowed as a result of COVID-19 related delays at trial sites
- A go/no-go decision is still expected no later than 2022

Established Utility of IDH Inhibition in Solid Tumors with Positive ClarIDHy Phase 3 Study of TIBSOVO® in Second-line or Later Cholangiocarcinoma

Mature OS from ClarIDHy Phase 3 expected mid-2020; sNDA planned between YE 2020 and mid-2021

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



LOW SURVIVAL RATES

~9%

FIVE-YEAR OVERALL SURVIVAL RATE

FEW TREATMENT OPTIONS

0

APPROVED THERAPIES FOR mIDH1 PATIENTS

POSITIVE CLARIDHY RESULTS

63%

REDUCTION IN RISK OF DISEASE PROGRESSION OR DEATH FOR PATIENTS TREATED W/ TIBSOVO®

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

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

SIGNIFICANT 2-HG SUPPRESSION

>90%

2-HG SUPPRESSION IN RESECTED mIDH1 GLIOMAS ACROSS ALL DOSES TESTED

IMPRESSIVE PRELIMINARY EFFICACY DATA

33% ORR

IN THE VORASIDENIB ARM OF THE PERIOPERATIVE STUDY

ENCOURAGING PHASE 1 DATA

22 mo.

MEDIAN TREATMENT DURATION IN VORASIDENIB PHASE 1

INDIGO | INVESTIGATING VORASIDENIB IN GLIOMA

1:1 double-blind randomization (n=366)

Stratified by 1p19q status and baseline tumor size

Vorasidenib
50 mg QD orally
Continuous 28-day cycles
(n=183)

Matched placebo*
(n=183)

*centrally-confirmed progressive disease will permit unblinding and crossover

Endpoints

Primary: Progression free survival (by BIRC)

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

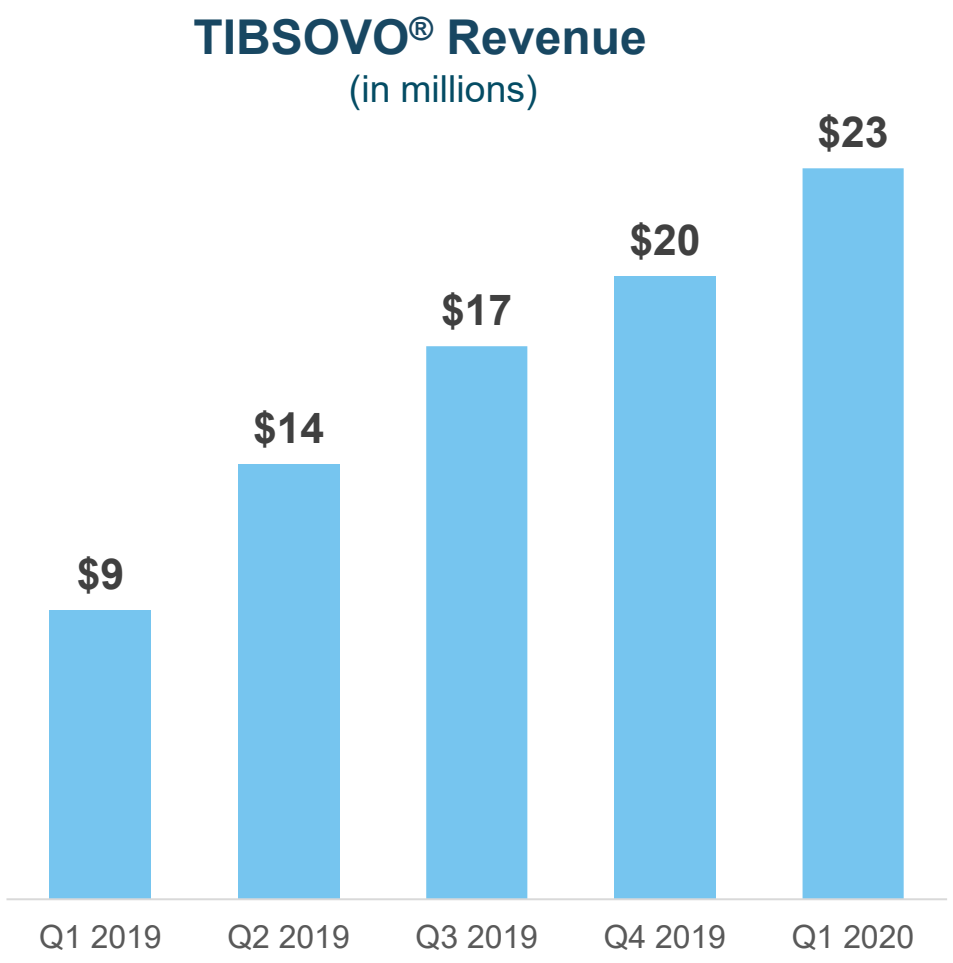
BIRC = blinded independent review committee, ORR = overall response rate, OS = overall survival, QOL = quality of life, WHO = World Health Organization; Source: Data presented at SNO 2019

Updated data from the vorasidenib Phase 1 accepted for presentation at ASCO

TIBSOVO® Commercial Update

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

Significant Q1 Growth Driven by Increased Demand in Both R/R and Frontline AML Segments and Expanding Customer Base



16% Growth
In Product Revenue Quarter-over-Quarter

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

25% Increase
In Unique Prescribers Quarter-over-Quarter

~1,400
Patients Treated Since Launch

Source: Agios estimates

Despite Potential Disruptions Related to COVID-19, Patient Access to TIBSOVO® Remains Uninterrupted and Performance Outlook Unchanged

- Current inventory is more than sufficient to meet anticipated demand for TIBSOVO® even if COVID-19 disruptions were to persist
- The TIBSOVO® distribution strategy is flexible which permits treatment to delivered directly to patients' residences if they are homebound for a prolonged period of time.
- myAgios™ Patient Support Services is fully functional and remains available to help patients with access, reimbursement, and financial assistance for TIBSOVO®
- Our field team has been working from home since mid-March and continues to engage customers remotely
- The urgency to treat patients with AML is high, and recently issued professional oncology patient care guidelines favor effective oral options with well tolerated safety profiles as healthcare professionals wrestle with the needs of COVID-19 infected patients

First Quarter 2020 Financial Results

Andrew Hirsch, Chief Financial Officer and Head of Corporate Development

Cash Conservation Efforts Extend Runway to the End of June 2022

- In order to conserve cash while supporting the execution of critical business objectives, Agios made the following decisions:
  - Cease in-house development of AG-636, a DHODH inhibitor in Phase 1 clinical development, and evaluating options for partnering this program
  - Pause select research programs, including the Friedreich's Ataxia program
  - Delay the start of certain longer-term clinical studies
  - Limit staff hiring and significantly reduce contract workforce
  - Pause certain infrastructure projects
- Additional savings anticipated as a result of reduced spending levels that will occur naturally due to the COVID-19 pandemic, such as travel expenses and clinical trial spend

Cash, cash equivalents and marketable securities as of March 31, 2020 were $613.1 million

First Quarter 2020 Financial Results

Statement of Operations	Three Months Ended 3/31/20	Three Months Ended 3/31/19	Year Ended 12/31/19
Total Revenue	$87.1M	$30.2M	$117.9M
Collaboration Revenue	61.1M	18.9M	47.5M
TIBSOVO® Net Sales	22.7M	9.1M	59.9M
Royalty Revenue	3.3M	2.2M	10.5M
Cost of Sales	0.5M	0.3M	1.3M
Research & Development Expense	91.3M	95.6M	410.9M
Selling, General & Administrative Expense	38.5M	31.8M	132.0M

Balance Sheet	3/31/20	12/31/19
Cash, Cash Equivalents and Marketable Securities	$613.1M	$717.8M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Q&A