agios

Q4 and YE 2020 Financial Results

February 25, 2021

Agios Conference Call Participants

TOPIC	PARTICIPANT
Introductions	Holly Manning, Director of Investor Relations
Business Update	Jackie Fouse, Ph.D., Chief Executive Officer
Clinical Development Update	Chris Bowden, M.D., Chief Medical Officer
TIBSOVO® Performance & Commercial Update	Darrin Miles, Chief Commercial Officer
Fourth Quarter and Year-end 2020 Financial Results	Jonathan Biller, Chief Financial Officer, Head of Legal & Corporate Affairs
Q&A	Bruce Car, Ph.D., Chief Scientific Officer

Important Information for Investors and Stockholders

Additional Information and Where to Find It

This communication relates to the proposed transaction involving the sale by Agios Pharmaceuticals, Inc. ("Agios") of its oncology business to Servier Pharmaceuticals, LLC. In connection with the proposed transaction, Agios filed with the U.S. Securities and Exchange Commission (the "SEC") a definitive proxy statement on Schedule 14A on February 11, 2021 and Agios commenced mailing the definitive proxy statement to its stockholders on February 12, 2021. BEFORE MAKING ANY VOTING DECISION, STOCKHOLDERS OF AGIOS ARE URGED TO READ THE DEFINITIVE PROXY STATEMENT REGARDING THE TRANSACTION AND ANY OTHER RELEVANT DOCUMENTS FILED OR THAT WILL BE FILED WITH THE SEC, AS WELL AS ANY AMENDMENTS OR SUPPLEMENTS TO THOSE DOCUMENTS, BECAUSE THEY CONTAIN OR WILL CONTAIN IMPORTANT INFORMATION ABOUT THE PROPOSED TRANSACTION. Investors and security holders will be able to obtain the documents (when available) free of charge at the SEC's website, at http://www.sec.gov, and Agios' website, at www.agios.com. In addition, the documents (when available) may be obtained free of charge by accessing Agios' website at www.agios.com under the heading "Investors" or, alternatively, directing a request to Holly Manning by email at holly.manning@agios.com or by calling 617-649-8600.

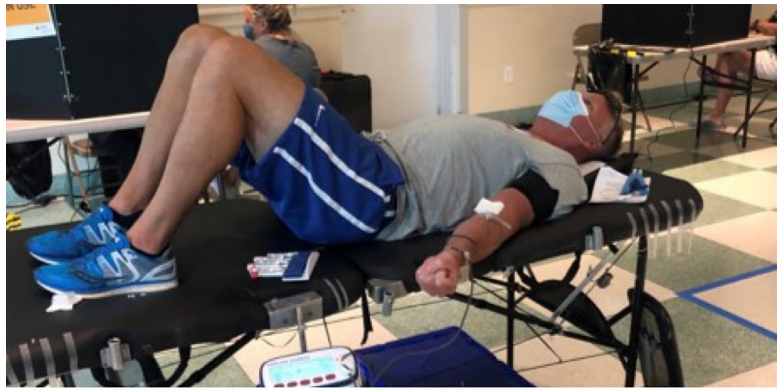
Participants in the Solicitation

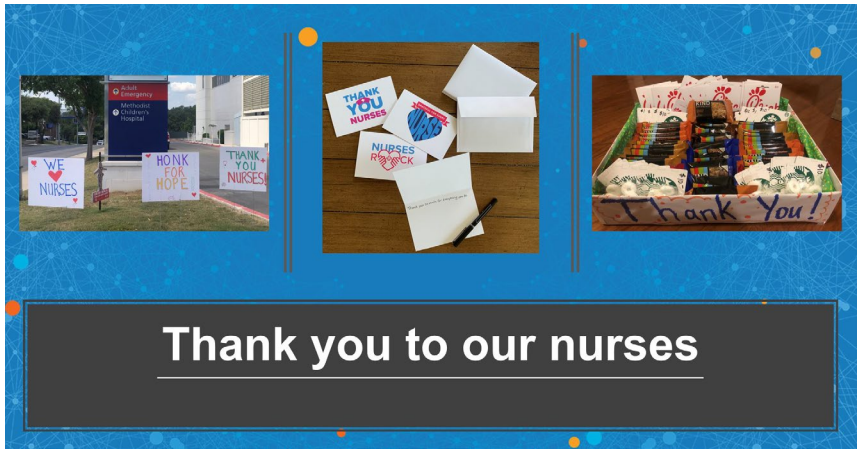
Agios and its directors and executive officers may be deemed to be participants in the solicitation of proxies from the holders of Agios common stock in respect of the proposed transaction. Information about the directors and executive officers of Agios is set forth in the proxy statement for Agios' 2020 annual meeting of stockholders, which was filed with the SEC on April 16, 2020, and in other documents filed by Agios with the SEC. Other information regarding the participants in the proxy solicitation and a description of their direct and indirect interests, by security holdings or otherwise, is contained in the definitive proxy statement and in other relevant materials filed or to be filed with the SEC in respect of the proposed transaction when they become available.

Forward Looking Statements

This communication contains forward-looking statements within the meaning of within the meaning of the Private Securities Litigation Reform Act of 1995, Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Such forward-looking statements include those regarding Agios' plans, strategies and expectations for the preclinical, clinical and commercial advancement of its drug development programs; the potential benefits of Agios' products and product candidates; Agios' key milestones and guidance for 2021 and strategic vision; its financial guidance regarding the period in which it will have capital available to fund its operations; expectations regarding the sale of Agios' oncology portfolio and associated return of capital to shareholders; and the potential benefits of Agios's 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. Management's expectations and, therefore, any forward-looking statements in this communication could also be affected by risks and uncertainties relating to a number of other important factors, including, without limitation risks and uncertainties related to: (i) Agios's sale of its oncology portfolio, including the occurrence of any event, change or other circumstance that could give rise to the termination of the purchase and sale agreement; the failure of Agios to obtain stockholder approval for the proposed transaction or the failure to satisfy any of the other conditions to the completion of the proposed transaction; the effect of the announcement of the proposed transaction on the ability of Agios to retain and hire key personnel and maintain relationships with its customers, suppliers, advertisers, partners and others with whom it does business, or on its operating results and businesses generally; risks associated with the disruption of management's attention from ongoing business operations due to the proposed transaction; the ability to meet expectations regarding the timing and completion of the proposed transaction, including with respect to receipt of required regulatory approvals; the failure of Agios to receive milestone or royalty payments under the purchase and sale agreement and the uncertainty of the timing of any receipt of any such payments; and the uncertainty of the results and effectiveness of the use of proceeds from the proposed transaction; (ii) 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; (iii) Agios' results of clinical trials and preclinical studies, including subsequent analysis of existing data and new data received from ongoing and future studies; (iv) 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; (v) Agios' ability to obtain and maintain requisite regulatory approvals and to enroll patients in its planned clinical trials; (vi) unplanned cash requirements and expenditures and competitive factors; (vii) Agios' ability to obtain, maintain and enforce patent and other intellectual property protection for any product candidates it is developing; (viii) Agios' ability to maintain key collaborations; and (ix) 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, including the risks and uncertainties set forth in Item 1A under the heading Risk Factors in our Annual Report on Form 10-K for the year ended December 31, 2019, our Quarterly Report on Form 10-Q for the fiscal quarter ended on September 30, 2020 filed with the SEC on November 5, 2020, our definitive proxy statement and other subsequent periodic reports we file with the SEC, which are available at http://www.sec.gov and our website at http://www.agios.com. While the list of factors presented here is considered representative, this list should not be considered to be a complete statement of all potential risks and uncertainties. Any forward-looking statements contained in this communication are made only as of the date hereof, and we undertake no obligation to update forward-looking statements to reflect developments or information obtained after the date hereof and disclaim any obligation to do so other than as may be required by law.

Thank you to the tremendous Agios team!





To maximize the value and promise of our diverse portfolio, we have made a deliberate choice of where to focus our efforts and investment

Expanding our genetically defined disease pipeline and mining the extensive, untapped potential in the PK franchise provides a clear path to success.

Selling our oncology portfolio to a capable and committed buyer allows us to maximize its patient impact.

By singularly focusing on genetically defined diseases and positioning our oncology portfolio for success with Servier, we can make the greatest impact.

The recently announced deal with Servier captures the full value* of the oncology portfolio facilitating the acceleration of our efforts in genetically defined diseases

As part of the definitive agreement with Servier for the acquisition of the oncology portfolio, Agios will receive:

1. Cash consideration of up to $2B, including $1.8B in upfront cash and a $200M milestone upon FDA approval of vorasidenib**
2. 5% royalties on U.S. net sales of TIBSOVO® from transaction close through loss of exclusivity
3. 15% royalties on U.S. net sales of vorasidenib from first commercial sale through loss of exclusivity

Agios plans to return at least $1.2B to shareholders; residual proceeds will be retained to achieve capital markets independence to fund the company through major catalysts and to profitability

**Risk adjusted*

***FDA approval of vorasidenib on or before January 1, 2027 with label permitting use as single agent in adjuvant setting for Grade 2 glioma with IDH1 or IDH2 mutation*

Q4 2020 & recent highlights

Genetically Defined Diseases

- Reported positive topline data from ACTIVATE and ACTIVATE-T, including statistically significant patient-reported outcomes data from ACTIVATE
- Received regulatory feedback on the mitapivat SCD development plan and finalized the Phase 2/3 clinical trial design
- Launched Anemia ID, a program providing no-cost genetic testing for patients with suspected hereditary anemias
- Presented updated Phase 1 data for mitapivat in sickle cell disease at ASH

Oncology

- TIBSOVO® net sales of $39M for the quarter and $121M for the year, exceeding $115M updated net revenue target
- Presented final data from the ClarIDHy Phase 3 study of TIBSOVO in cholangiocarcinoma at ASCO-GI

Corporate

- Promoted Darrin Miles to chief commercial officer
- Shareholder vote for Servier transaction scheduled for March 25

Clinical Development Updates

Chris Bowden, M.D., Chief Medical Officer

Positive data from both pivotal program in PK deficiency designed to support a broad label

ACTIVATE

- **Primary Efficacy Endpoint Achieved**: 40% of patients treated with mitapivat achieved a sustained hemoglobin increase of ≥1.5 g/dL compared to 0 placebo patients ($p<0.0001$)
- Treatment with mitapivat also demonstrated statistically significant improvements over placebo across pre-specified key secondary endpoints including: patient-reported outcomes (PRO) based on changes from baseline in pyruvate kinase deficiency diary (PKDD) score and pyruvate kinase deficiency impact assessment (PKDIA) score
- Safety profile was generally consistent with previously reported data

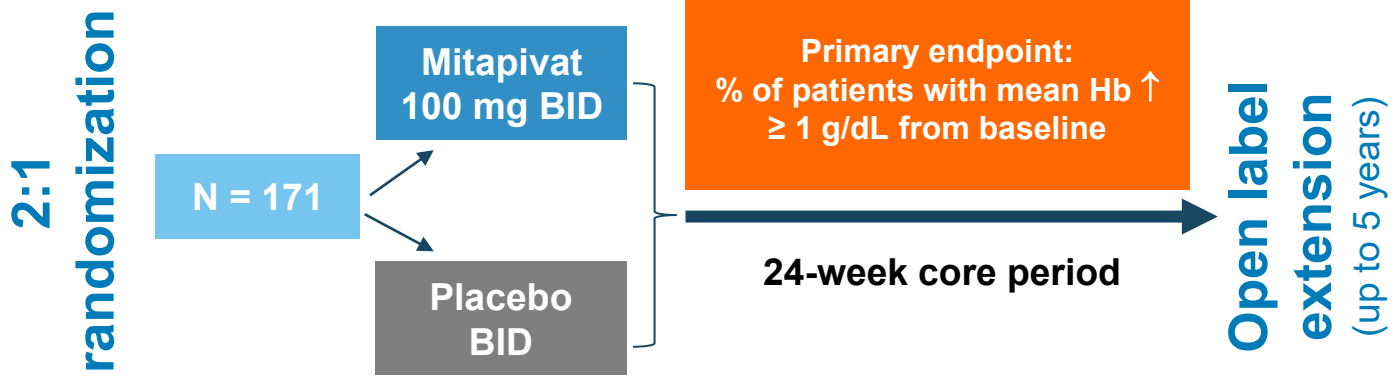
ACTIVATE-T

- **Primary Efficacy Endpoint Achieved**: 37% of patients treated with mitapivat achieved a ≥33% reduction in transfusion burden compared to individual historical transfusion burden standardized to 24 weeks (1-Sided $p=0.0002$)
- 22% of patients treated with mitapivat were transfusion-free during the 24-week fixed dose period
- Safety profile was generally consistent with previously reported data

Data from both trials, including PRO data, to be submitted for presentation at EHA

Two global, Phase 3, randomized controlled trials of mitapivat in thalassemia are planned for 2021

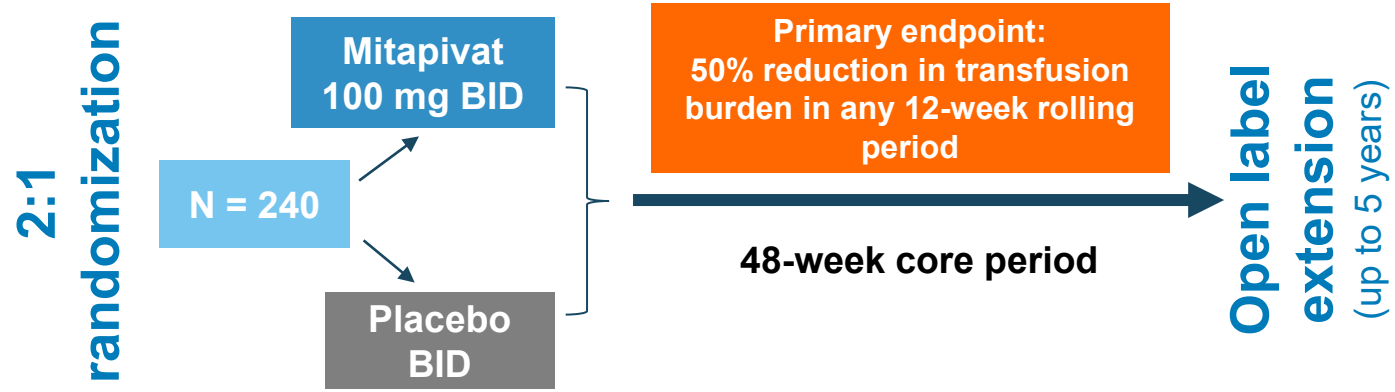
ENERGIZE



Key inclusion criteria

- ≥ 18 years
- β-thalassemia ± α-globin mutations, HbE β-thalassemia, or α-thalassemia (HbH disease)
- Non—transfusion-dependent defined as ≤5 RBC units during the 24-week period before randomization and no RBC transfusions ≤8 weeks prior
- Hb ≤ 10.0 g/dL

ENERGIZE-T



Key inclusion criteria

- ≥ 18 years
- β-thalassemia ± α-globin mutations, HbE β-thalassemia, or α-thalassemia (HbH disease)
- Transfusion-dependent defined as 6 to 20 RBC units transfused and ≤6-week transfusion-free period during the 24-week period before randomization

BID = twice daily; Hb = hemoglobin; HbE = hemoglobin E; HbH = hemoglobin H.

Phase 1 data supportive of advancing mitapivat to pivotal development in sickle cell disease

- Mitapivat is being evaluated in an ongoing Phase 1 trial in sickle cell disease under a Cooperative Research and Development Agreement (CRADA) with the U.S. National Institutes of Health (NIH)
- Updated data to be submitted for presentation at a medical meeting this year

Proof of concept demonstrated[1]

- Six of 11 efficacy evaluable patients (55%) achieved a hemoglobin increase of ≥1.0 g/dL from baseline
- Mean maximal hemoglobin increase among all efficacy evaluable patients was 1.3 g/dL; mean maximal hemoglobin increase among responders was 1.9 g/dL
- Dose-dependent increases in ATP and decreases in 2,3-DPG
- Decreases in total bilirubin, LDH and absolute reticulocyte count

- Mitapivat was generally well-tolerated. Safety profile was consistent with previously presented data in patients with PK deficiency and thalassemia

- Our collaborators at the University of Utrecht initiated an IST of mitapivat in sickle cell disease late last year
- Data to be submitted for presentation at a medical meeting this year.

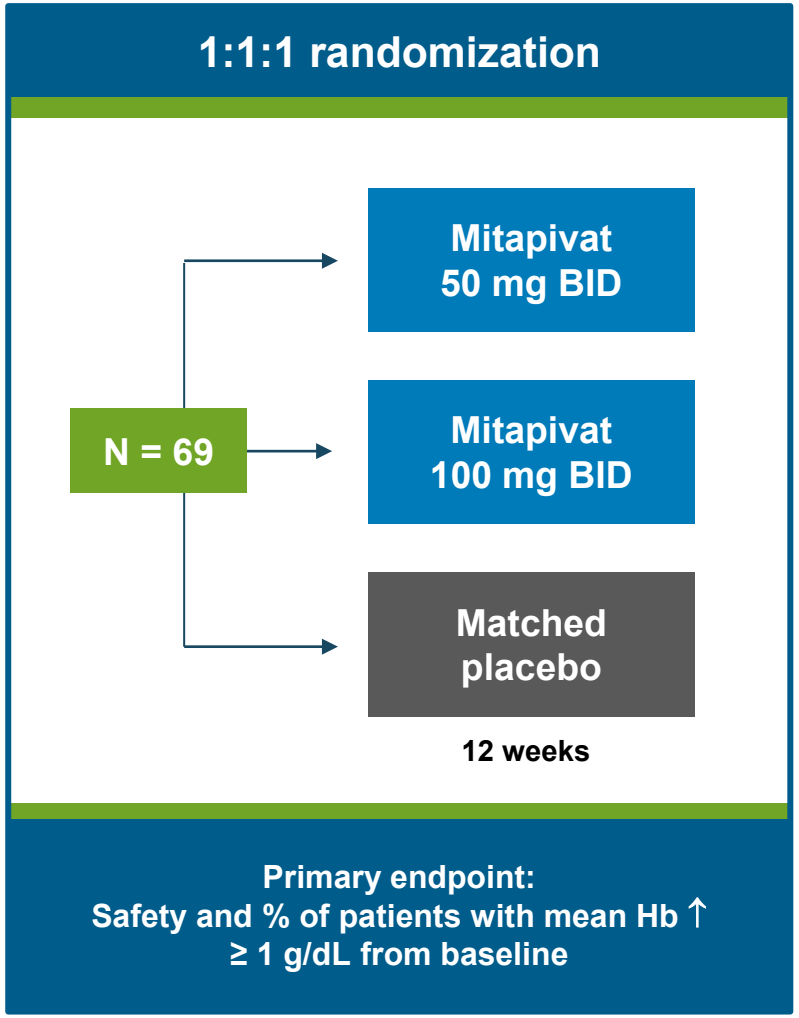
[1]Data as of October 6, 2020 cutoff; presented at ASH 2020

Pivotal program for mitapivat in sickle cell disease: Operationally seamless Phase 2/3 trial

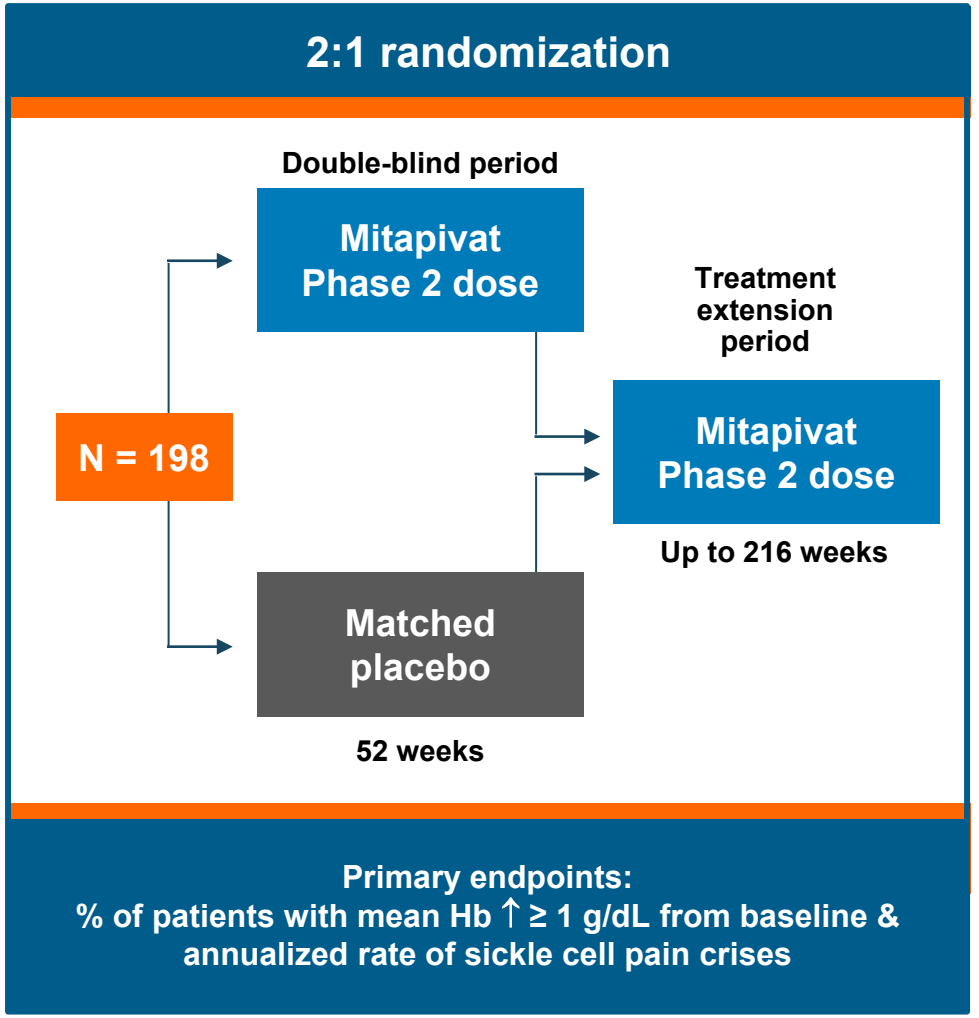
ENROLLMENT CRITERIA

- ≥ 16 years
- Had 2-10 sickle cell crises in the past 12 months
- Hb ≥ 5.5 and ≤ 10.5 g/dL
- Patients currently receiving treatment with voxelotor, crizanlizumab, or any other agent intended to increase Hb-oxygen affinity are excluded.
- Treatment with hydroxyurea is allowed.

PHASE 2



PHASE 3



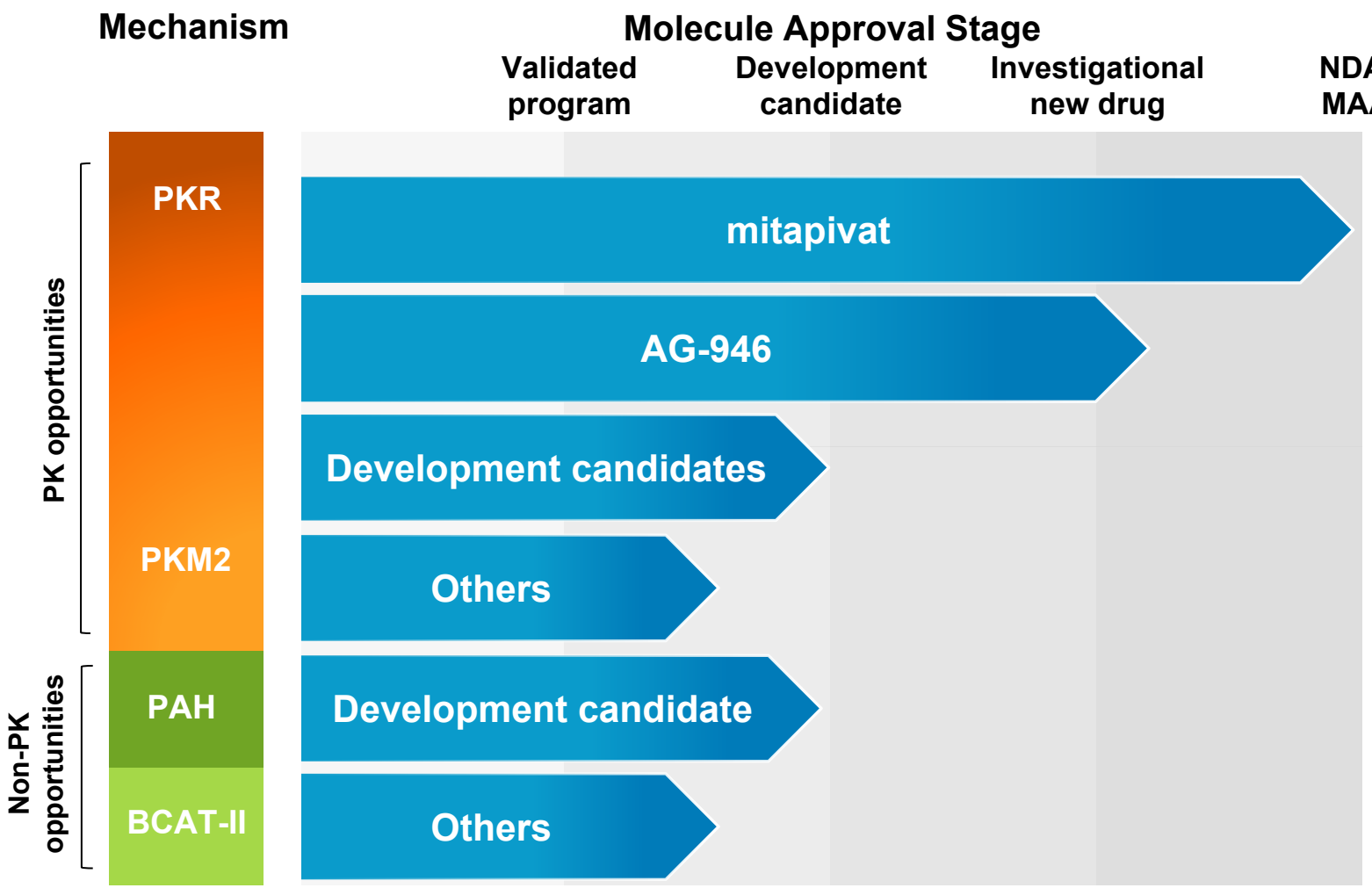
BID = twice daily; Hb = hemoglobin

In mitapivat, we are building a robust pipeline with the ability to rapidly expand to three indications

Mitapivat Pipeline Overview

Early Stage Clinical	Late Stage Clinical	Regulatory Submission	Near-Term Milestones	Anticipated Approval	
Non-transfusion Dependent (NTD) Adult PK Deficiency (ACTIVATE)		NDA filing in Q2; MAA filing in 2H 2021	Positive topline data from ACTIVATE announced in Dec.	2022	~3-8K PATIENTS IN U.S. & EU5
Transfusion Dependent Adult PK Deficiency (ACTIVATE-T)			Positive topline data from ACTIVATE-T announced in Jan.	2022	Pyruvate Kinase Deficiency
Non-transfusion Dependent Adult Thalassemia (ENERGIZE)			Finalized pivotal plan in Dec. 2020; Initiate pivotal study in 2H 2021	2025	~18-23K PATIENTS IN U.S. & EU5
Transfusion Dependent Adult Thalassemia (ENERGIZE-T)			Finalized pivotal plan in Dec. 2020; Initiate pivotal study in 2H 2021	2025	
Sickle Cell Disease			Finalized pivotal plan in Feb. 2021; Initiate pivotal study by YE 2021	2026	β- and α-Thalassemia
Pediatric PK Deficiency			Finalized pivotal plan in Nov. 2020		~120-135K PATIENTS IN U.S. & EU5
Pediatric Thalassemia			Planning in process		
Pediatric Sickle Cell Disease			Planning in process		Sickle Cell Disease

Significant opportunities exist beyond our initial pipeline focus



Indications

***Pyruvate kinase activation*: 20+ potential indications**

- Pyruvate kinase deficiency
- Sickle cell disease
- α, β−Thalassemia

- Hereditary spherocytosis **(~64K U.S. patients)**
- Anemia of low-risk MDS **(~50K U.S. patients)**
- Multiple hereditary hemolytic anemias

- Anemia of chronic kidney disease
- Retinal indications
- Diabetic retinopathy/nephropathy
- Hereditary myopathies
- Neurologic (ALS)

***Aminoacidurias/acidemia*: 6+ potential indications**

- Phenylketonuria **(~16,500 U.S. patients)**

- Methylmalonic acidemia
- Propionic acidemia
- Multiple rare inborn errors of metabolism

Anticipated 2021 key milestones

GDD PROGRAM MILESTONES

- Submit NDA in the U.S. for mitapivat in adults with PK deficiency in Q2
- Submit MAA in the EU for mitapivat in adults with PK deficiency in mid-2021
- Initiate two Phase 3 studies of mitapivat – ENERGIZE-T and ENERGIZE – in regularly transfused and not regularly transfused thalassemia in 2H 2021
- Initiate Phase 2/3 study of mitapivat in sickle cell disease by YE 2021
- Prioritize new PKR and PKM2 indications for clinical development in 2021

GDD DATA PRESENTATIONS

- ✓ Report topline data from the ACTIVATE-T study of mitapivat in regularly transfused PK deficiency in Q1
- Submit data from the mitapivat ACTIVATE and ACTIVATE-T studies for presentation at EHA
- Submit data from the mitapivat thalassemia Phase 2 study for presentation at EHA
- Submit data from ongoing clinical trials of mitapivat in sickle cell disease for presentation at medical meetings throughout 2021
- Submit data from the SAD/MAD cohorts of the AG-946 healthy volunteer study for presentation at a medical meeting by YE

CORPORATE

- Close the sale of the oncology portfolio to Servier following shareholder vote
- Complete share repurchases over 12-18 months post-close

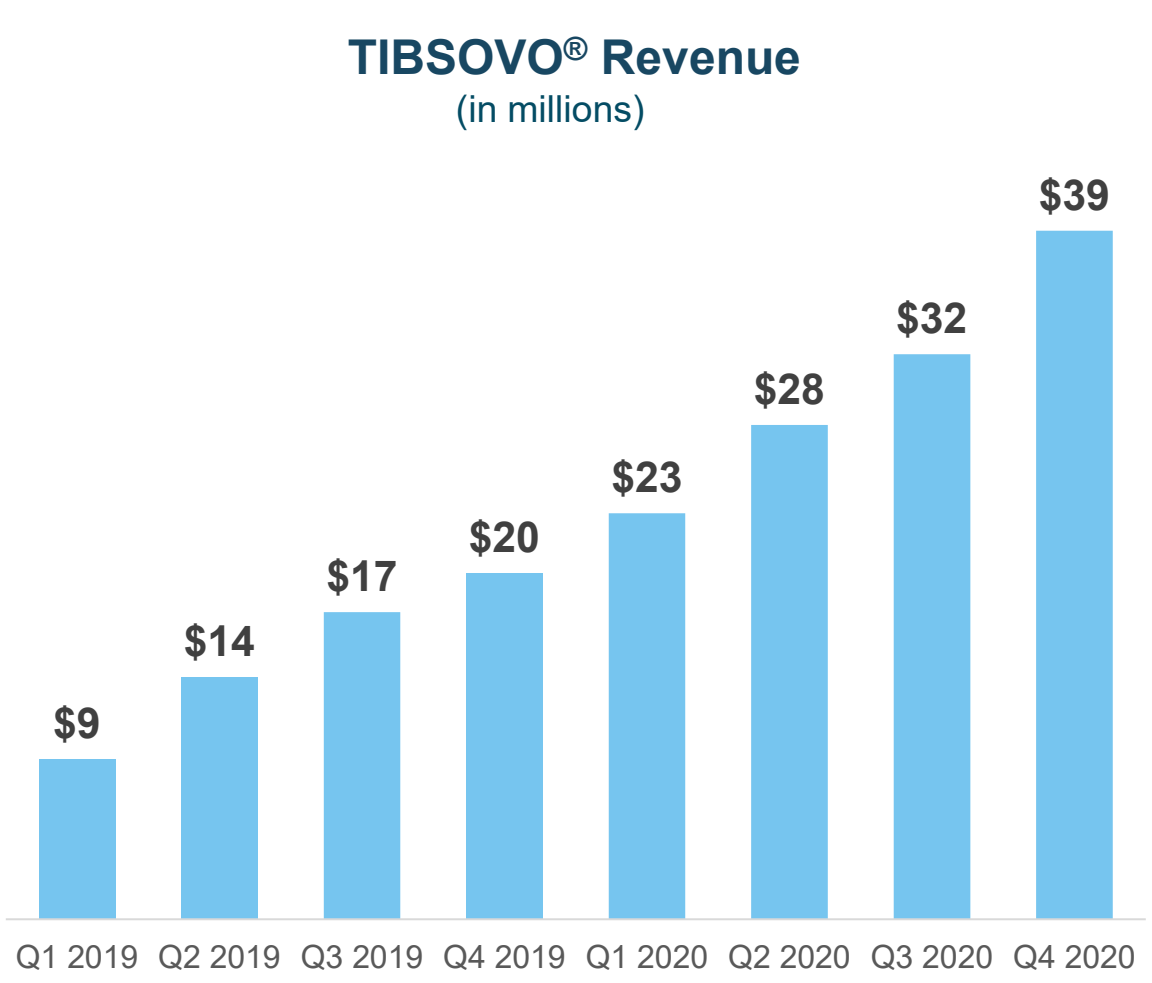
NEAR-TERM ONCOLOGY MILESTONES & DATA PRESENATIONS

- ✓ Present mature OS for ClarIDHy at ASCO GI on Jan. 17
- Submit sNDA for TIBSOVO in previously treated cholangiocarcinoma in Q1
- Achieve full-year revenue for TIBSOVO® of $160-170M
- Complete enrollment in AGILE by YE
- Complete enrollment in the MDS cohort of the Phase 1 study by YE

TIBSOVO® Commercial Update

Darrin Miles, Chief Commercial Officer

Q4 growth driven by increased demand in both R/R and frontline AML segments and expanding customer base



23% Growth
In Product Revenue Quarter-over-Quarter

$160-170M
U.S. Net Sales Guidance for 2021

15% Increase
In Unique Prescribers Quarter-over-Quarter

~2,127
Patients Treated Since Launch

Source: Agios estimates

Fourth Quarter and Full Year 2020 Financial Results

Jonathan Biller, Chief Financial Officer, Head of Legal and Corporate Affairs

Fourth quarter and full year 2020 financial results

Statement of Operations	Three Months Ended 12/31/20	Three Months Ended 12/31/19	Year Ended 12/31/20	Year Ended 12/31/19
Total Revenue	$44.0M	$35.4M	$203.2M	$117.9M
Collaboration Revenue	2.0M	12.8M	71.8M	47.5M
TIBSOVO® Net Sales	39.1M	19.6M	121.1M	59.9M
Royalty Revenue	2.9M	3.0M	10.3M	10.5M
Cost of Sales	1.0M	0.3M	2.8M	1.3M
Research & Development Expense	95.7M	106.2M	367.5M	410.9M
Selling, General & Administrative Expense	39.8M	34.8M	149.1M	132.0M

Balance Sheet	12/31/20	12/31/19
Cash, Cash Equivalents and Marketable Securities	$670.5M	$717.8M

December 31, 2020 cash balance provides runway to the end of 2022

Q&A