



Third Quarter 2018 Financial Results

November 1, 2018



Agios Conference Call Participants

Prepared Remarks

Introduction

- RENEE LECK, Associate Director, Investor Relations

Business Highlights & 2018 Key Milestones

- DAVID SCHENKEIN, M.D., Chief Executive Officer

Clinical Development Progress

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

TIBSOVO® Launch Update

- STEVE HOERTER, Chief Commercial Officer

Third Quarter 2018 Financial Results

- ANDREW HIRSCH, Chief Financial 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 IDHIFA® (enasidenib), TIBSOVO® (ivosidenib), AG-881, mitapivat, AG-270 and AG-636; the potential benefits of Agios' product candidates; its key milestones for 2018; 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," "believe," "could," "estimate," "expect," "hope," "intend," "may," "milestone," "path," "plan," "possible," "potential," "predict," "prepare," "project," "strategy," "will," "would," 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 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.



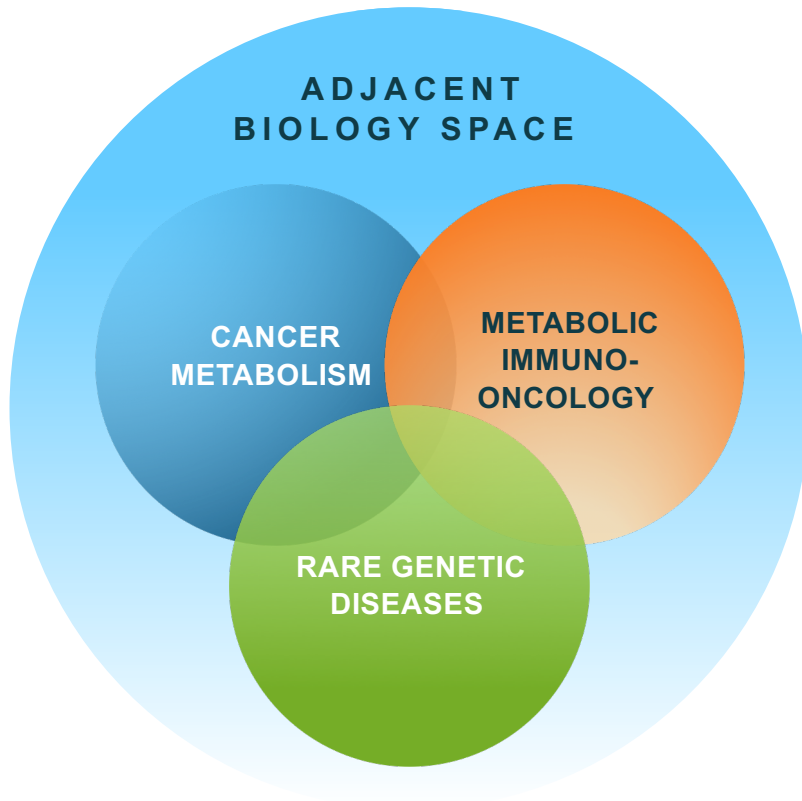


Business Highlights & 2018 Key Milestones

David Schenkein, M.D., Chief Executive Officer



Driven By a Clear Vision and Values



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.



2018 Key Milestones

✓ Secure approval and commercialize **TIBSOVO®** for IDH1m R/R AML in the U.S.

● Submit **TIBSOVO®** European MAA in IDH1m R/R AML

● Initiate **Phase 3 frontline AML trial HO150** combining ivosidenib & enasidenib with 7+3

✓ Initiate low grade glioma perioperative study with ivosidenib and AG-881

✓ Initiate mitapivat (AG-348) PK deficiency pivotal trial **ACTIVATE-T**; Initiate **ACTIVATE**













● Initiate mitapivat (AG-348) **Phase 2 proof-of-concept trial** in thalassemia

✓ Initiate **AG-270 Phase 1 dose-escalation trial** in MTAP-deleted cancers

✓ Submit **7th IND** for DHODH



Our Pipeline

CLINICAL PROGRAMS	INDICATION	DRUG DISCOVERY	EARLY STAGE CLINICAL DEVELOPMENT	LATE STAGE CLINICAL DEVELOPMENT	APPROVED	PRIMARY COMMERCIAL RIGHTS
IDHIFA® <i>enasidenib</i> (IDH2m Inhibitor)	R/R AML				●	  Agios U.S. Co-promotion and Royalty
	Frontline AML		●			
TIBSOVO® <i>ivosidenib</i> (IDH1m Inhibitor)	R/R AML				●	
	Frontline AML			●		
	Cholangio			●		
	Glioma		●			
AG-881 (pan-IDHm Inhibitor)	Glioma		●			
mitapivat (PK (R) Activator)	PK Deficiency			●		
AG-270 (MAT2A Inhibitor)	MTAP-deleted Tumors		●			 
RESEARCH PROGRAMS						
AG-636 (DHODH)			●			
CM Research Programs		●				
RGD Research Programs		●				
Metabolic IO Research Programs		●				 



Clinical Development Progress

Chris Bowden, M.D., Chief Medical Officer



Today's Clinical News

1. Plan to submit an a supplemental new drug application for single agent TIBSOVO® (ivosidenib) in newly diagnosed AML patients not eligible for standard treatment by the end of January 2019
2. Full enrollment for Phase 3 AGILE trial (ivosidenib combination with azacitidine in newly diagnosed AML patients ineligible for intensive chemotherapy) now expected to complete in 2020 vs. previous guidance of 2021 based on FDA agreement on event free survival primary endpoint
3. Mitapivat pivotal program (ACTIVATE and ACTIVATE-T) expected to complete enrollment in 2019



Multiple Opportunities Across IDHm Hematologic and Solid Cancers Originating from Agios Research Platform

ACUTE MYELOID LEUKEMIA	CHOLANGIOCARCINOMA	LOW GRADE GLIOMA	OTHER INDICATIONS
<p>IDH2m R/R <i>IDHIFA® (enasidenib) Approved</i></p>	<p>IDH1m R/R <i>ivosidenib Phase 3 (ClarIDHY) Ongoing</i></p>	<p>IDH1m <i>ivosidenib & AG-881 Perioperative Study Ongoing</i></p>	<p>MYELODYSPLASTIC SYNDROMES</p> <p>IDHm R/R <i>ivosidenib Phase 1 Enrollment Complete</i></p>
<p>IDH1m R/R <i>TIBSOVO® (ivosidenib) Approved</i></p>	<p>IDH1m R/R <i>ivosidenib Phase 1 Enrollment Complete</i></p>	<p>IDH1m <i>ivosidenib Phase 1 Enrollment Complete</i></p>	<p>CHONDROSARCOMAS</p> <p>IDH1m R/R <i>ivosidenib Phase 1 Enrollment Complete</i></p>
<p>IDH1m Frontline Non-IC <i>ivosidenib + Aza Phase 3 (AGILE) Ongoing</i></p>		<p>IDH1m <i>AG-881 Phase 1 Enrollment Complete</i></p>	
<p>IDHm Frontline IC-Eligible <i>ivo/ena + 7+3 Phase 3 (HO150) Q4 2018 Start</i></p>			
<p>IDHm Frontline Non-IC <i>ivo/ena + Aza Phase 1/2 Enrollment Complete</i></p>			
<p>IDHm Frontline IC-Eligible <i>ivo/ena + 7+3 Phase 1b Enrollment Complete</i></p>			



Fourth Quarter Clinical Data Presentations

**SNO oral presentation –
updated data from Phase 1 trial of
AG-881 in IDH1m solid tumors,
including glioma**

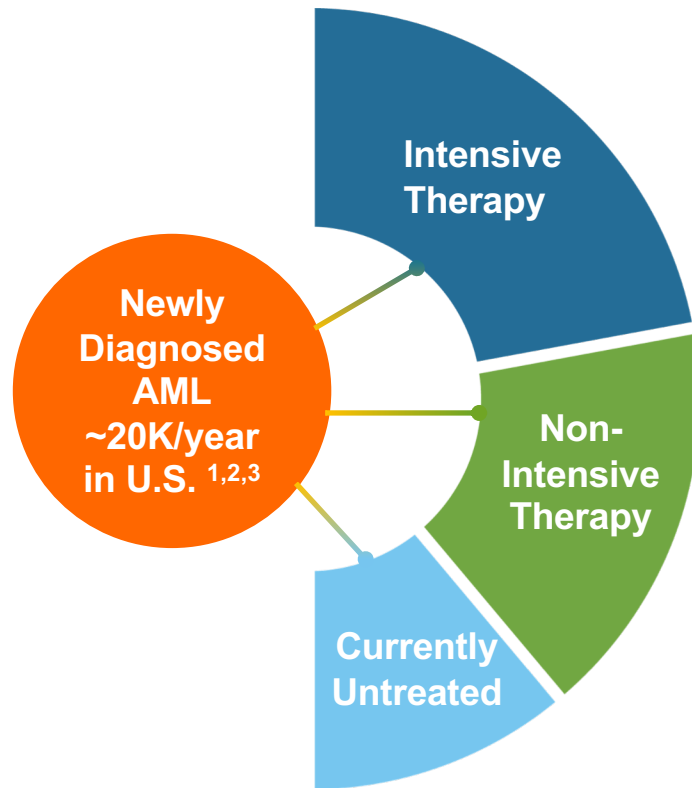
**ASH oral presentation –
updated data in untreated AML from
the Phase 1 study of ivosidenib in
IDH1m hematologic malignancies**

**ASH oral presentation –
updated data in IDHm newly
diagnosed AML from the Phase 1
combination trial of ivosidenib or
enasidenib with standard-of-care
intensive chemotherapy**

**ASH poster presentation -
updated data in MDS from the
Phase 1 study of ivosidenib in
IDH1m hematologic malignancies**



Broad Clinical Development Strategy in Frontline AML



- HOVON 150 Phase 3 planned
- Phase 1 combo study ongoing
- Broad IST strategy

- AGILE Phase 3 ongoing, EFS endpoint
- Phase 1 combo study ongoing
- Broad IST strategy

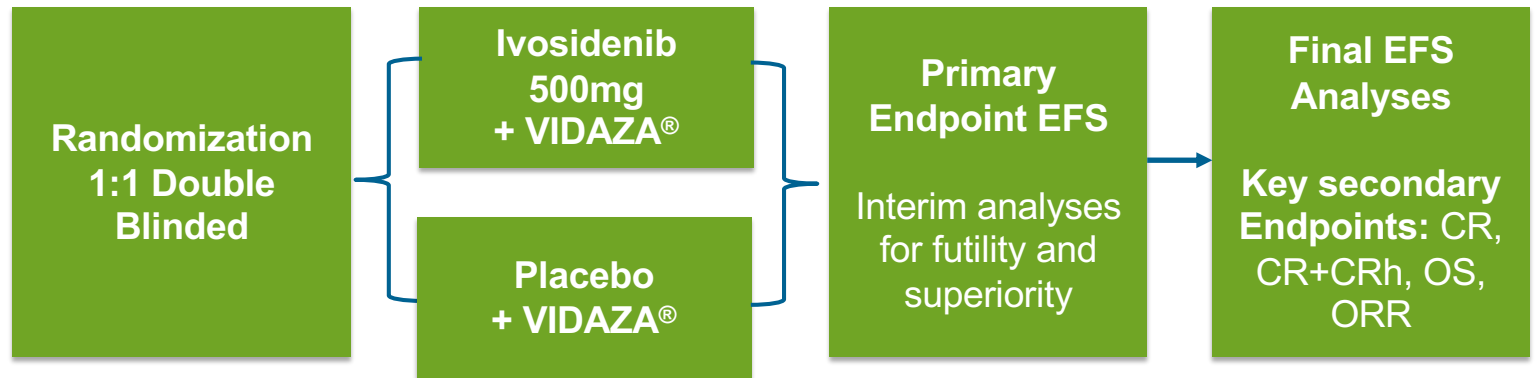
- sNDA submission expected by end of Jan. 2019



Phase 3 Frontline AGILE Ongoing



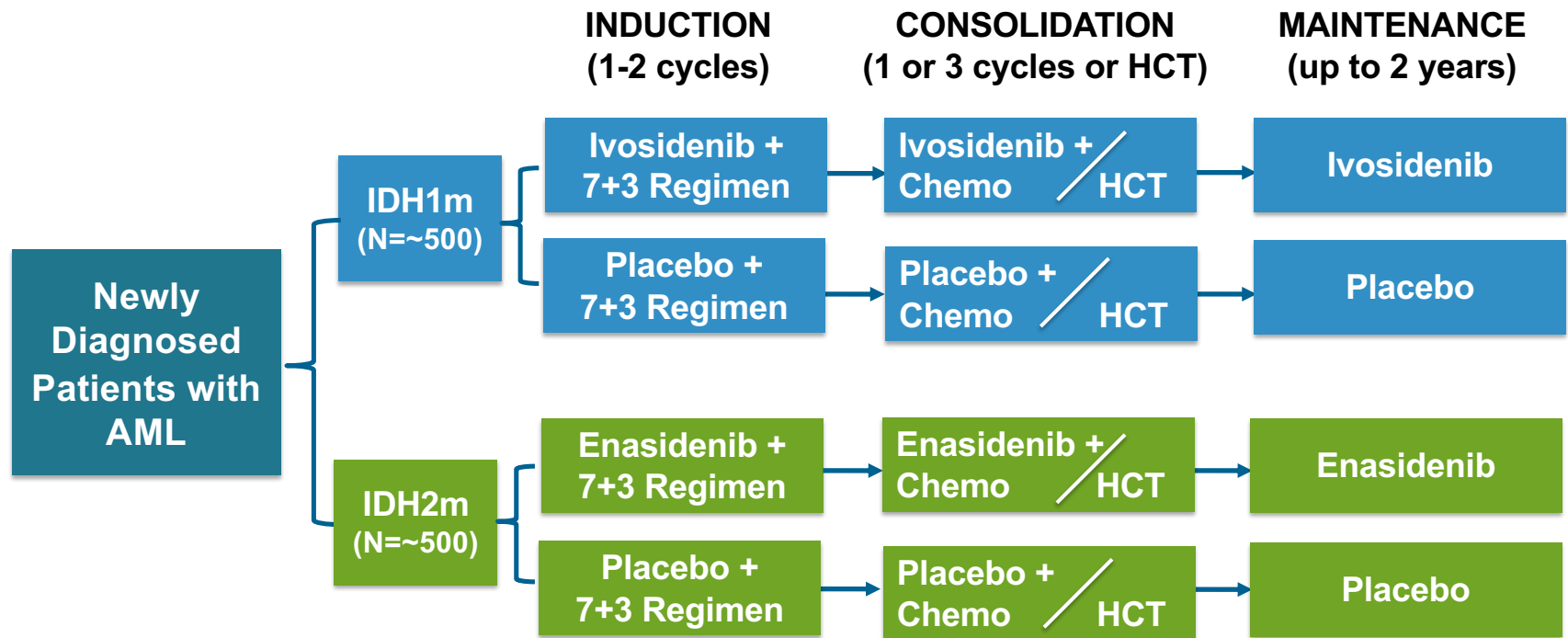
**Global Phase 3
Frontline
IC-Ineligible
IDH1m AML**



ClinicalTrials.gov Identifier: NCT03173248
IC = intensive chemotherapy
VIDAZA® is a registered trademark of Celgene Corporation



HOVON 150 Phase 3 Intergroup Frontline AML Trial in Collaboration with Celgene Planned for Q4 2018



EFS = Event Free Survival
HCT = Hematopoietic Cell Transplantation

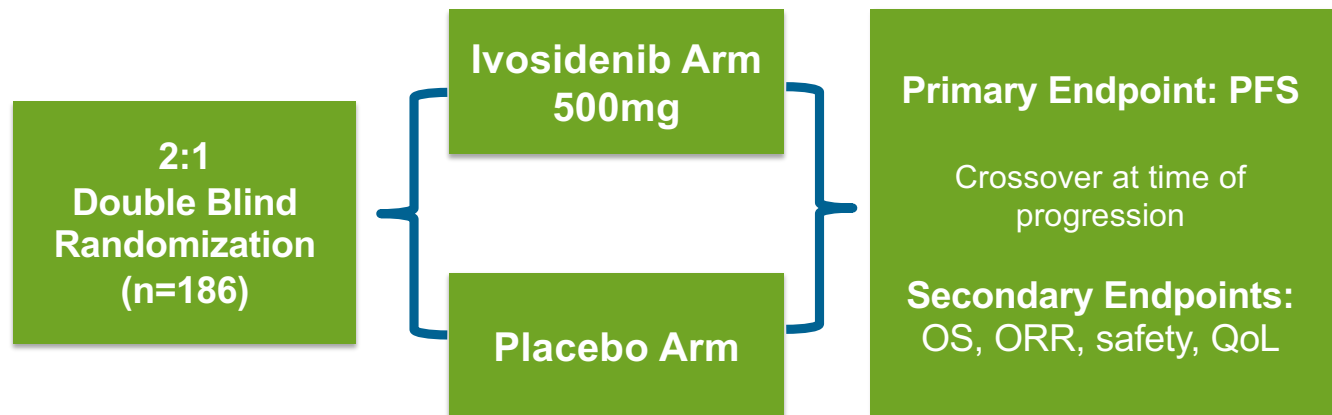
EFS primary endpoint; sponsored by HOVON and AML-SG



Registration-Enabling Phase 3 Cholangiocarcinoma Study Ongoing



Global Phase 3 Previously Treated Advanced IDH1m Cholangiocarcinoma (no more than 2 prior therapies)



The study has 96% power to detect a hazard ratio of 0.5 with a one-sided alpha of 0.025

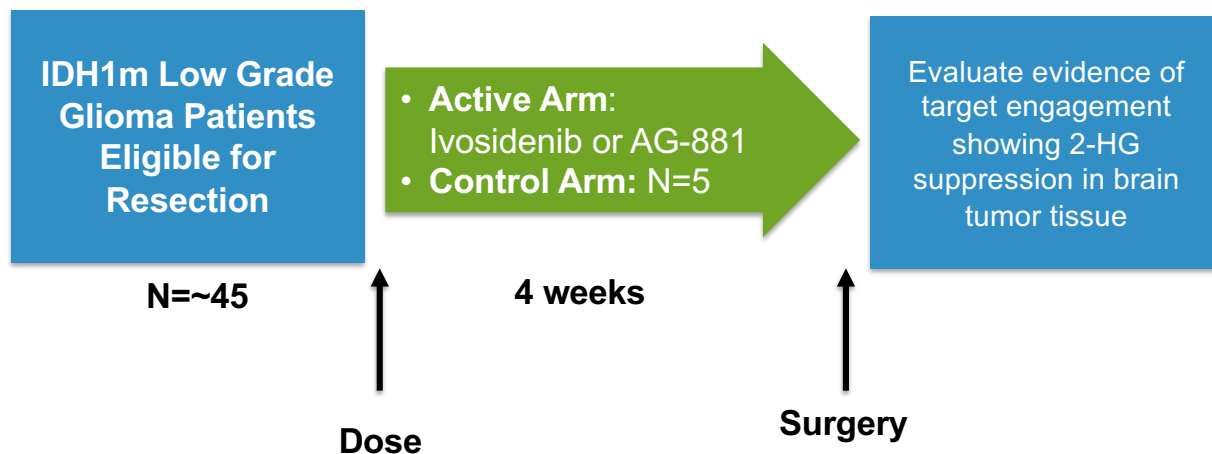
ClinicalTrials.gov Identifier: NCT02989857



Ongoing Phase 1 Perioperative Study with Ivosidenib and AG-881 Evaluating Evidence of Target Engagement

Study Objectives:

- Determine amount of drug penetration in the brain
- Confirm magnitude of IDHm target engagement as measured by 2HG levels in brain tumor tissue (pre-clinically 85% seen with ivosidenib & 98% with AG-881)
- Assess impact of IDHm inhibition on differentiation and epigenetic profiles in tumor tissue
- Assess the safety of both molecules

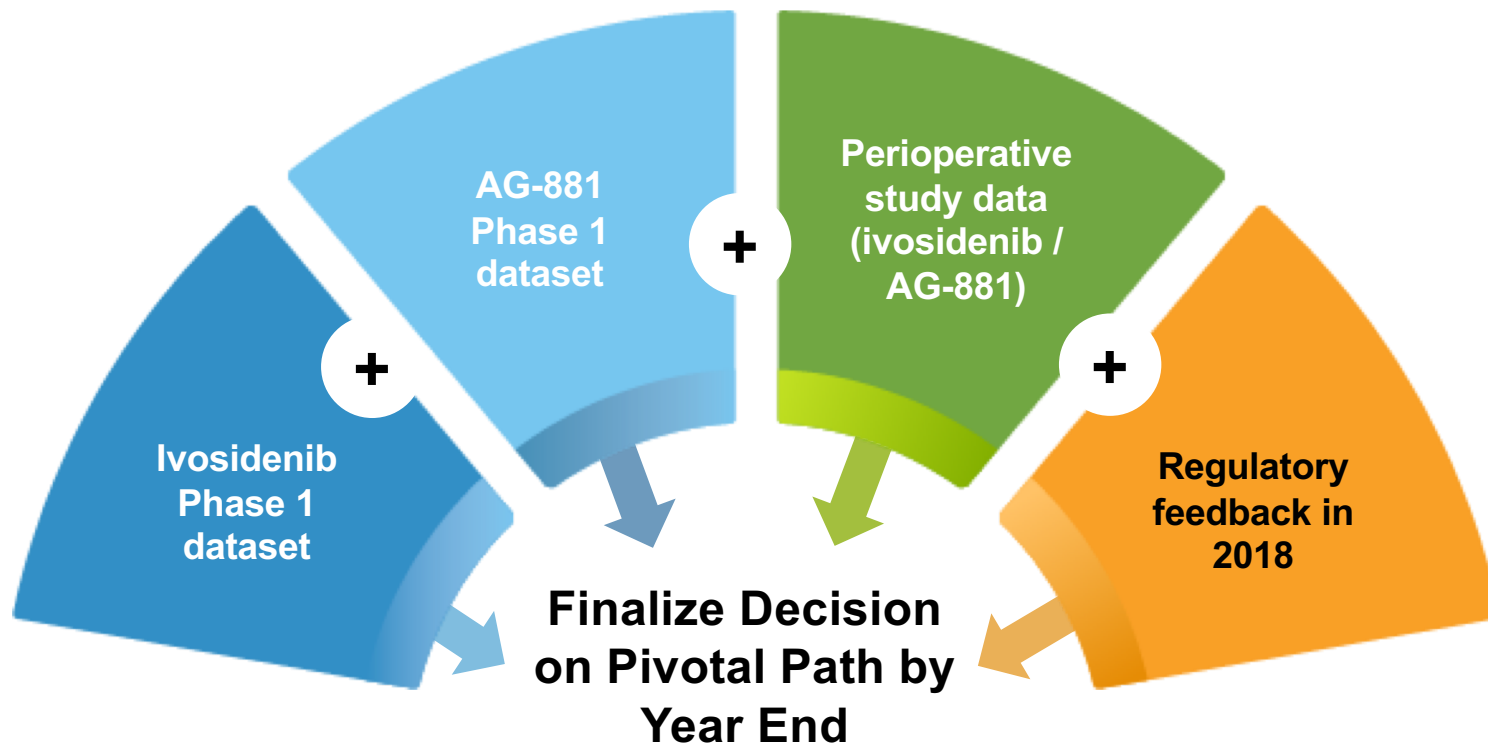


ClinicalTrials.gov Identifier: NCT03343197

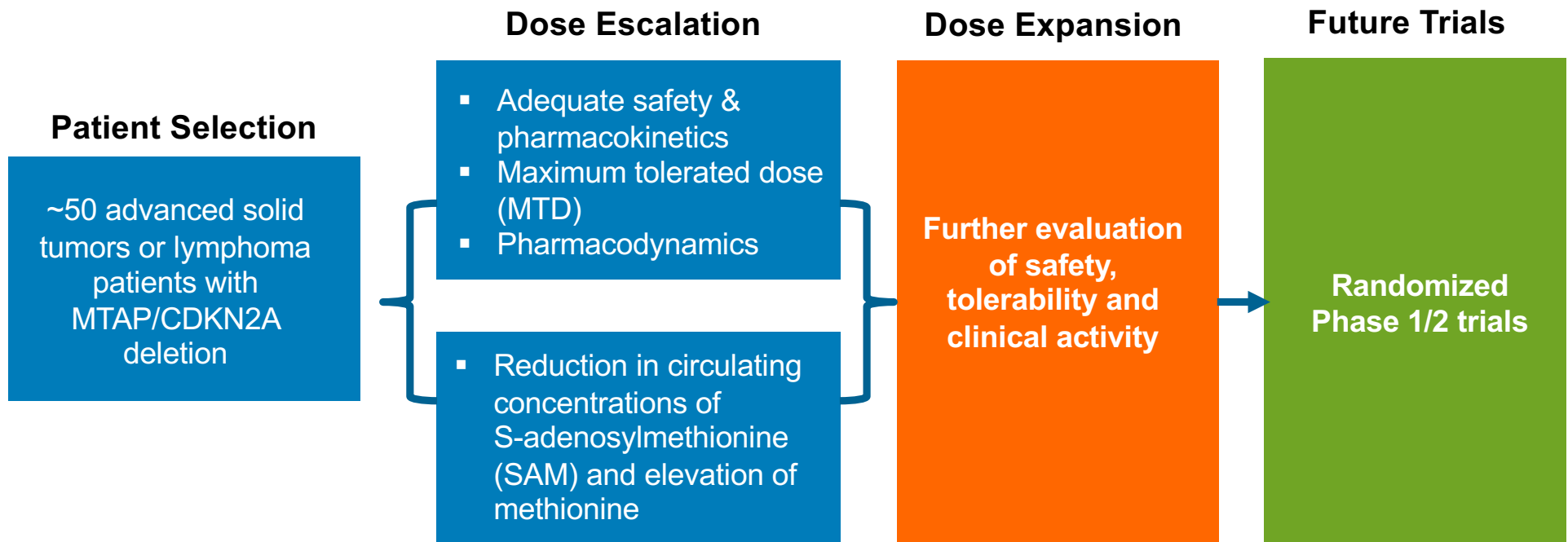
Trial initiated in Q1; Patients Enrolling



Next Steps in Glioma



AG-270 First-in-Human Phase 1 Clinical Trial

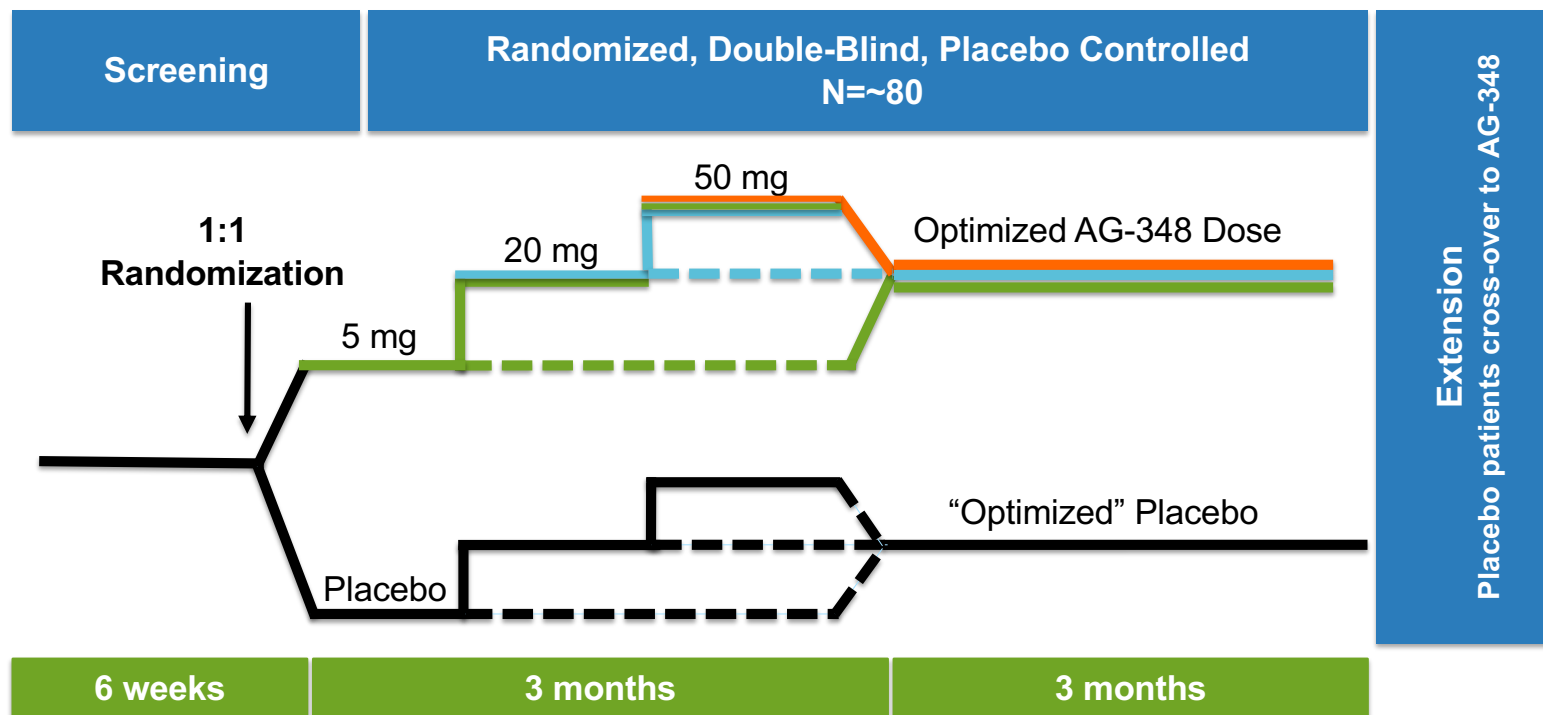


ClinicalTrials.gov Identifier: NCT03435250

Trial initiated and enrolling patients



Mitapivat (AG-348) ACTIVATE Trial for Non-Regularly Transfused Patients



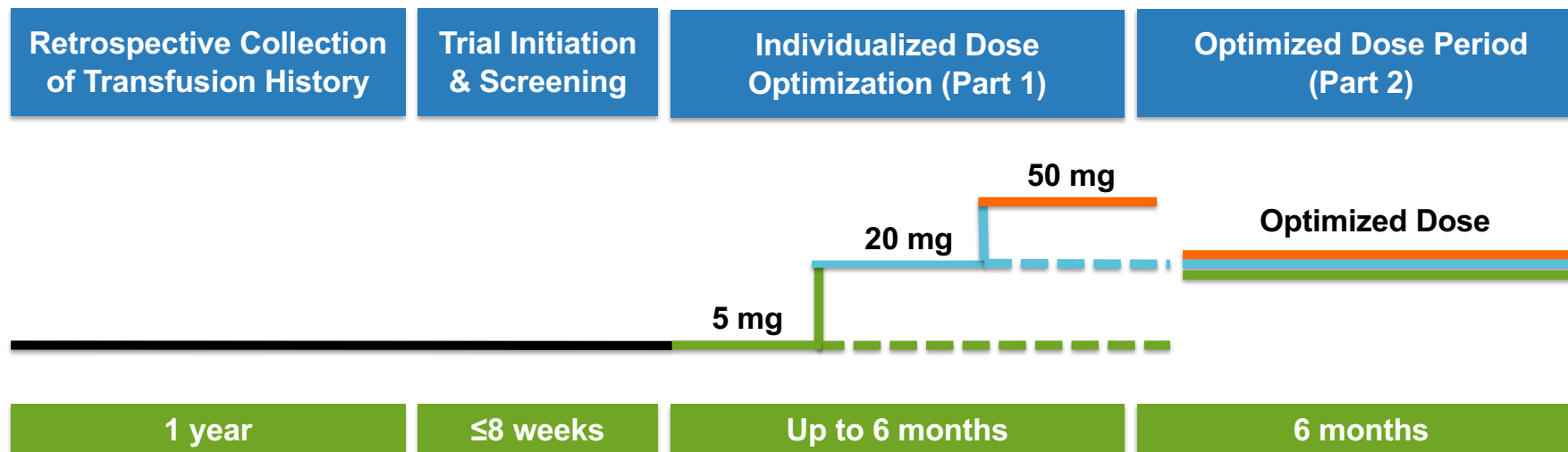
Primary Efficacy Endpoint:

Proportion of patients who achieve at least a 1.5 g/dL increase in hemoglobin sustained over multiple visits

Enrollment expected to complete in 2019



Mitapivat (AG-348) ACTIVATE-T Trial for Regularly Transfused Patients



Approximately 20 regularly transfused patients who have required a minimum of 6 transfusions over the year preceding enrollment

Primary Endpoint:

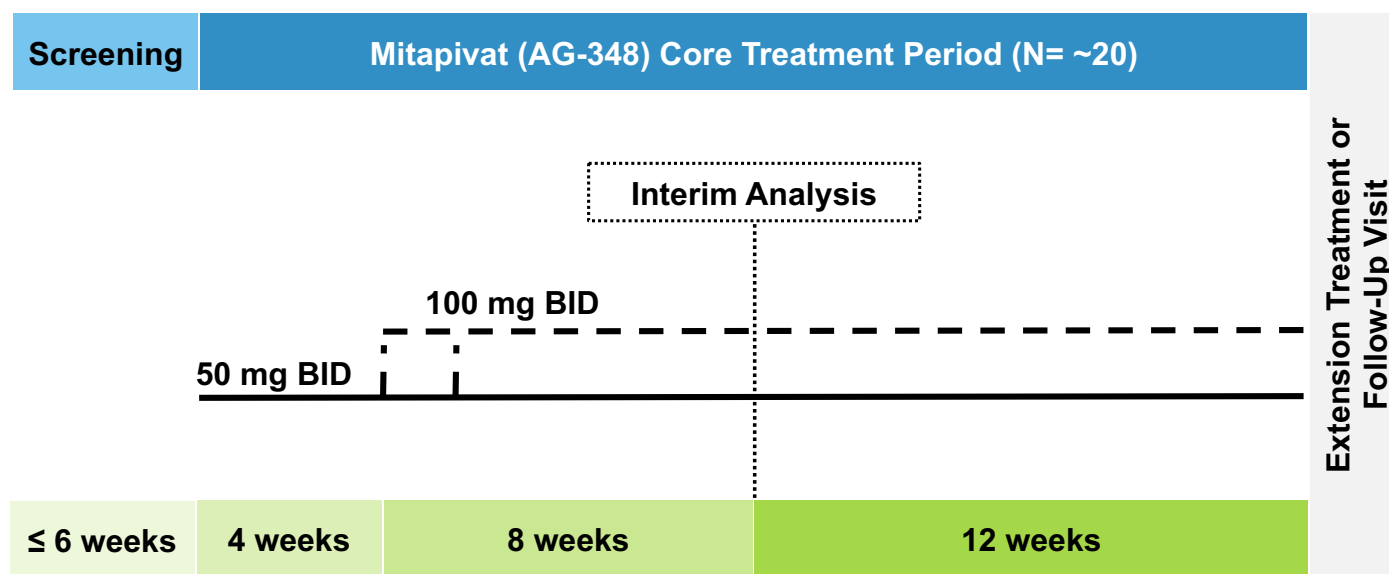
Reduction in transfusion burden over a 6-month period compared to the patient's transfusion history

Enrollment expected to complete in 2019



Thalassemia Phase 2 Proof-of-Concept in Non-Transfusion Dependent Adults

- Open-label trial in ~20 patients with hemoglobin < 9.0.
- Primary endpoint is hemoglobin response, using a definition of 1.0 g/dl over baseline at 12 weeks



On track to initiate by year-end 2018





TIBSOVO Launch Update

Steve Hoerter, Chief Commercial Officer



Strategic Imperatives for the TIBSOVO® Launch

Physicians test
for IDH1m



TIBSOVO® is
recognized as
the best option
for IDH1m+
R/R AML



Patients have
access to
TIBSOVO®



TIBSOVO® Launch Performance – Q3 2018



\$4.5M Net Sales



~80% Physicians Testing for IDH1/IDH2 mutations

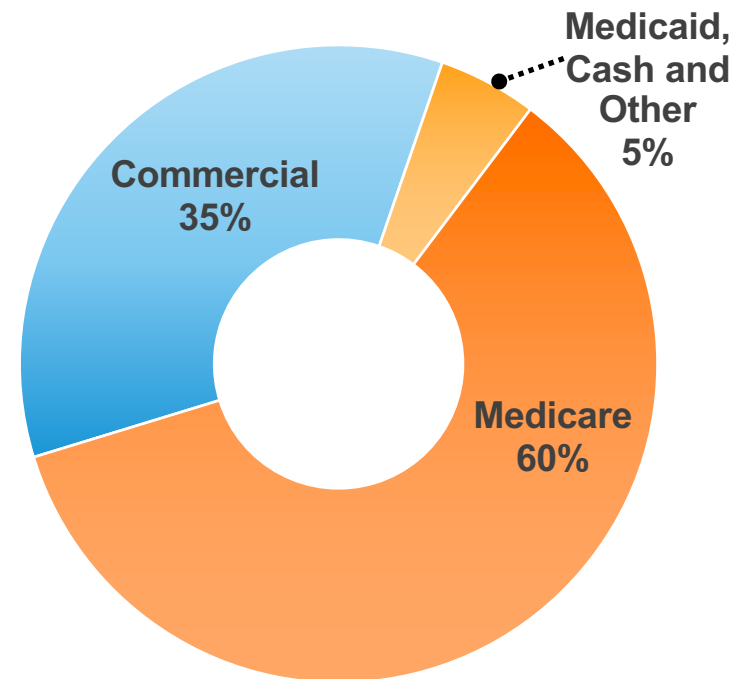


~2,000 Customer Interactions



100+ Unique Prescribers

Payer Mix: Specialty Pharmacy Channel





Third Quarter 2018 Financial Results

Andrew Hirsch, Chief Financial Officer



Third Quarter 2018 Financial Results

Statement of Operations	Three Months Ended September 30, 2018	Three Months Ended September 30, 2017
Total Revenue	\$15.2M	\$11.4M
Collaboration Revenue	8.7M	10.6M
TIBSOVO® Net Sales	4.5M	--
Royalty Revenue	2.0M	0.7M
Cost of Sales	0.7M	--
Research & Development Expense	82.6M	72.9M
Selling, General & Administrative Expense	31.1M	17.5M
Balance Sheet	September 30, 2018	December 31, 2017
Cash, Cash Equivalents and Marketable Securities	\$878.4M	\$567.8M





Q&A