Fourth Quarter and Full Year 2018 Financial Results

February 14, 2019

Agios Conference Call Participants

Prepared Remarks

Introduction

- KENDRA ADAMS, Senior Director, Investor & Public Relations

Business Highlights and 2019 Milestones

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

Clinical Development Progress

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

TIBSOVO® Launch Update

- STEVE HOERTER, Chief Commercial Officer

Fourth Quarter and Full Year 2018 Financial Results

- ANDREW HIRSCH, Chief Financial Officer

Forward Looking Statements

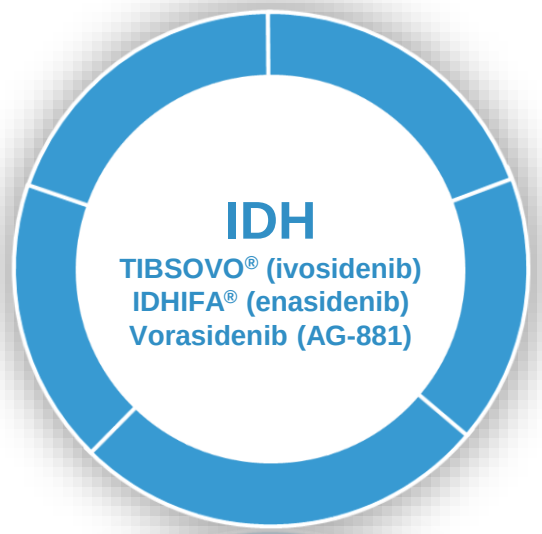
This presentation and various remarks we make during this presentation contain forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include those regarding Agios' plans, strategies and expectations for its and its collaborator's preclinical, clinical and commercial advancement of its drug development programs including 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 and 2019 Milestones

Jackie Fouse, Ph.D., Chief Executive Officer

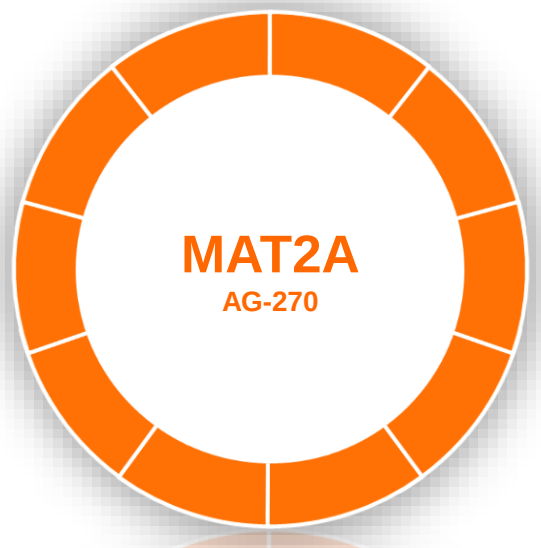
agios

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

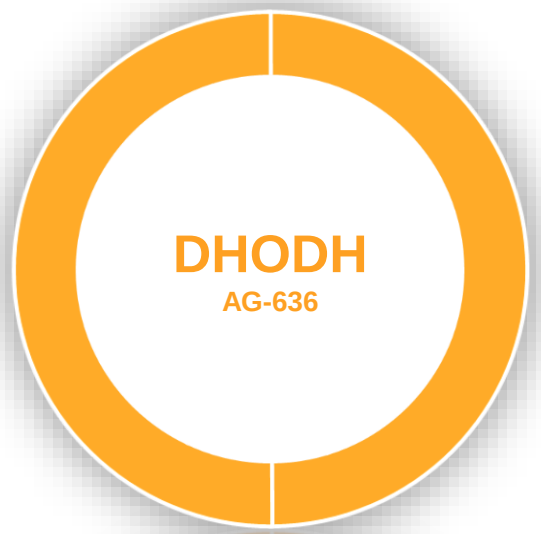


- AML
- Low Grade Glioma
- Cholangiocarcinoma
- Chondrosarcoma
- MDS

- Adult PK Deficiency
- Pediatric PK Deficiency
- Sickle Cell Disease
- Thalassemia

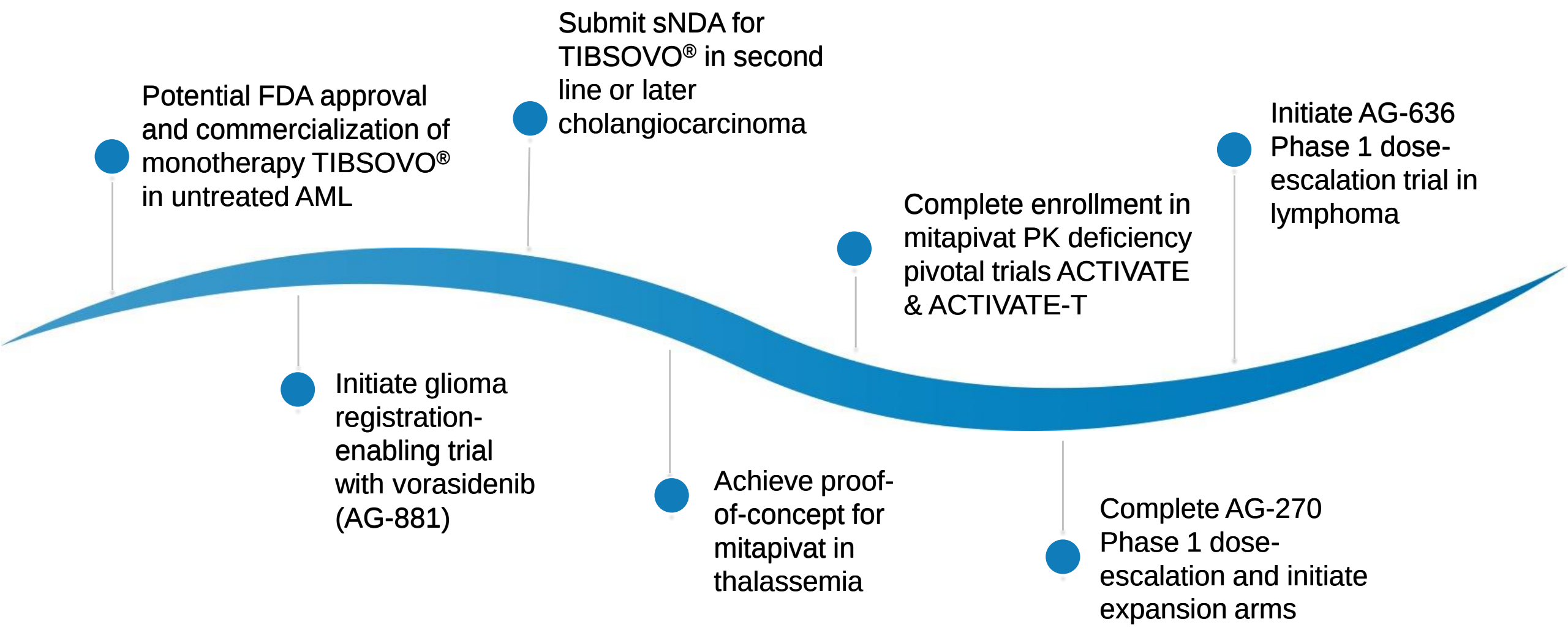


- NSCLC
- Bladder
- Melanoma
- Head & Neck
- Pancreatic
- Glioblastoma
- DLBCL
- Esophageal
- Gastric
- Mesothelioma



- Lymphoma
- AML

2019 Key Milestones Position Agios for Long-term Value Creation



Clinical Development Progress

Chris Bowden, M.D., Chief Medical Officer

What's New Today

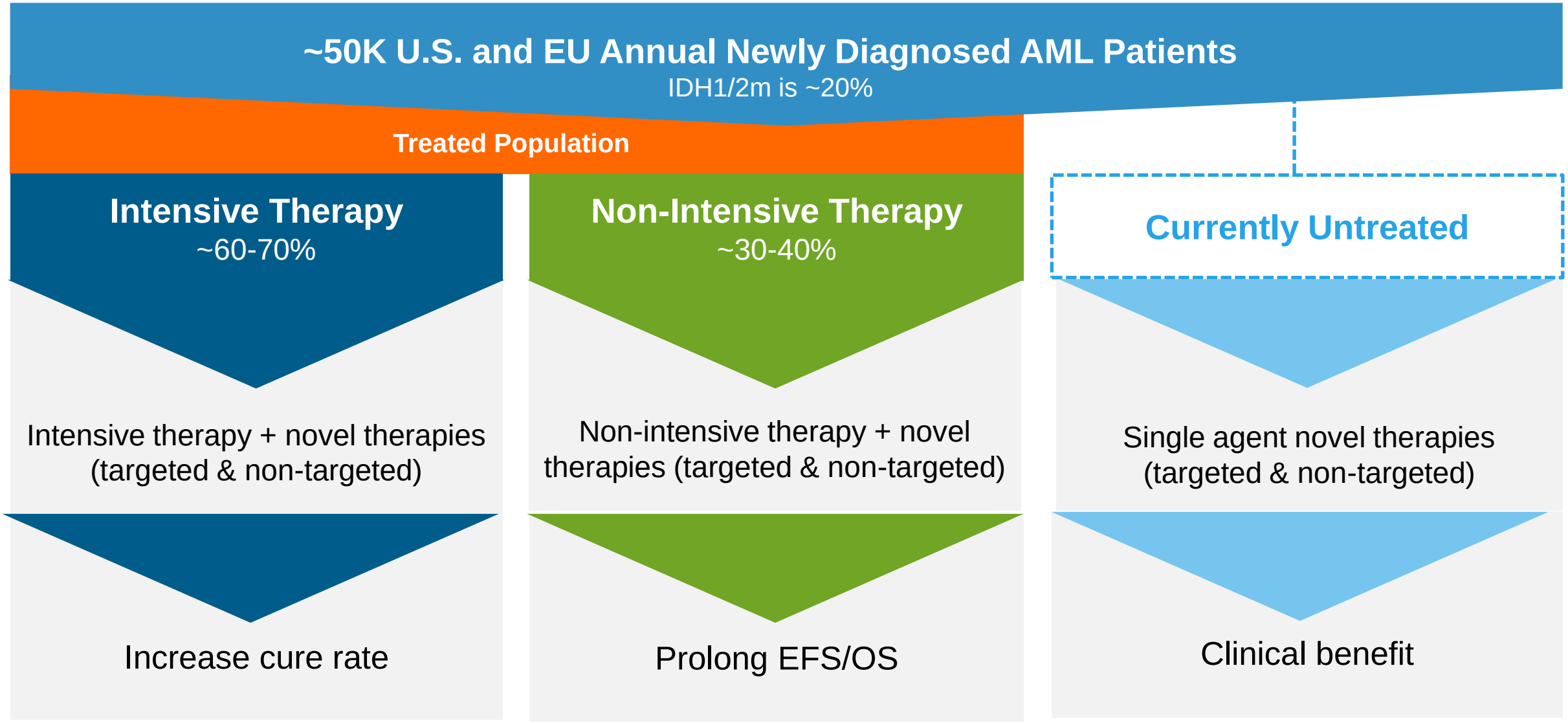
Key Business Updates

- Marketing Authorization Application for TIBSOVO® for the treatment of adult patients with R/R AML with an IDH1 mutation has been validated by the EMA
- ClarIDHy Phase 3 study of TIBSOVO® in second line or later cholangiocarcinoma with an IDH1 mutation now fully enrolled

Upcoming Data Presentations

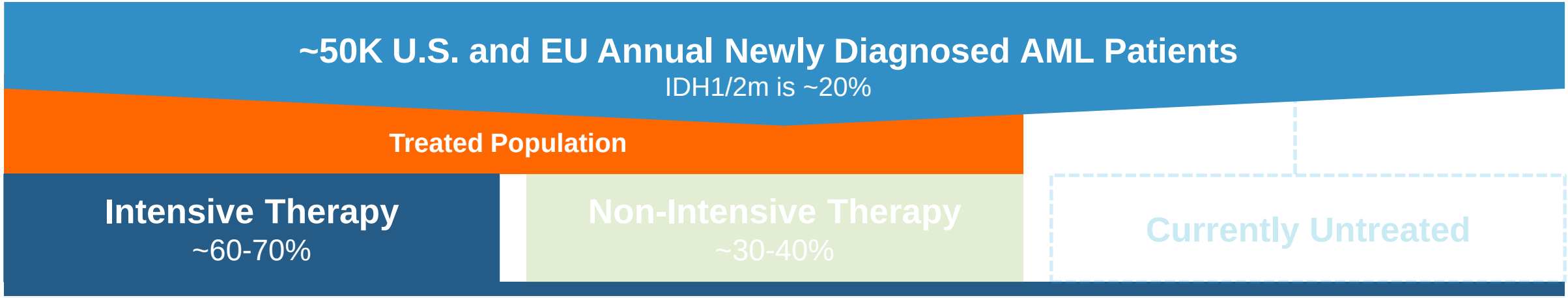
- Updated data from Phase 1 combo trial of TIBSOVO® with azacitidine in newly diagnosed AML to be presented at International Symposium on Acute Leukemias in Munich Feb. 24-27
- Data from perioperative 'window' trial with TIBSOVO® and vorasidenib in IDHm low-grade glioma submitted for presentation at ASCO May 31-June 4
- Preclinical data for AG-270 to be presented at AACR March 29-April 3

Shifting the Treatment Paradigm for Patients with Newly Diagnosed IDH1m AML



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

Encouraging Phase 1 Data in Combination with Intensive Chemo Supports Label Enabling Phase 3 Study



PHASE 1 7+3 COMBO DATA

(TIBSOVO® cohort)

- Median age 63 years
- 70% de novo; 30% sAML
- Safety consistent with previously reported data
- 91% CR+CRi/CRp rate for de novo patients (31 of 34)
- 80% CR+CRi/CRp rate for all patients (39 of 49)

NEXT STEPS

HOVON 150 AML / AMLSG 29-18 PHASE 3 STUDY

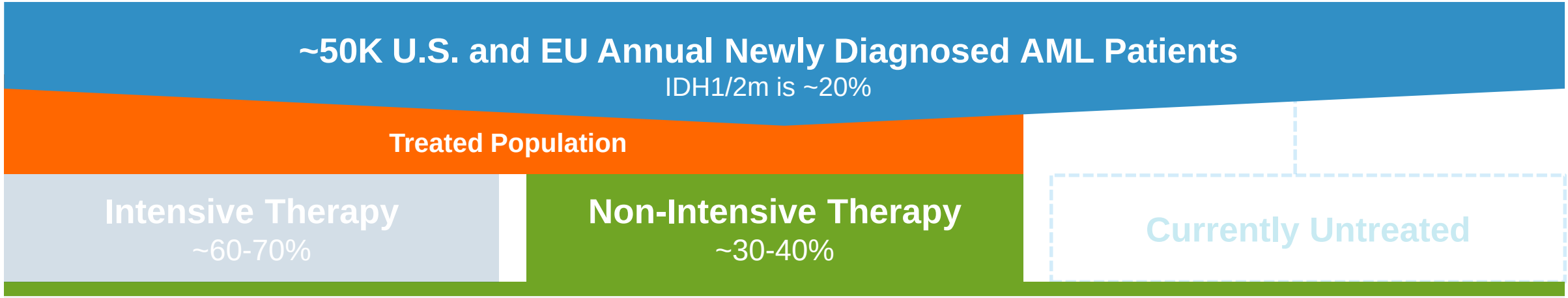
Planned for Q1 2019 Initiation

BROAD IST SUPPORT

VYXEOS™ Combination

Sources: SEER. Cancer Stat Facts: AML 2015 and Epiphany EPIC oncology numbers; American Cancer Society AML 2017; ASCO 2018; ASH 2018; VYXEOS™ is a trademark of Jazz Pharmaceuticals

Compelling Phase 1 Combination Data for Patients Ineligible for Intensive Chemo Suggests Potential to Extend EFS/OS



PHASE 1 AZACITIDINE COMBO DATA

(TIBSOVO® cohort)

Updated Phase 1 Data to be Presented at the Leukemia Meeting in February

- Median age 76 years
- Safety consistent with previously reported data
- 78% ORR (18 of 23)
- 65% CR/CRi/CRp rate (15 of 23)
- 44% CR rate (10 of 23)
- 17/23 patients remain on therapy as of data cut off (median of 5 treatment cycles)

NEXT STEPS

AGILE PHASE 3 STUDY

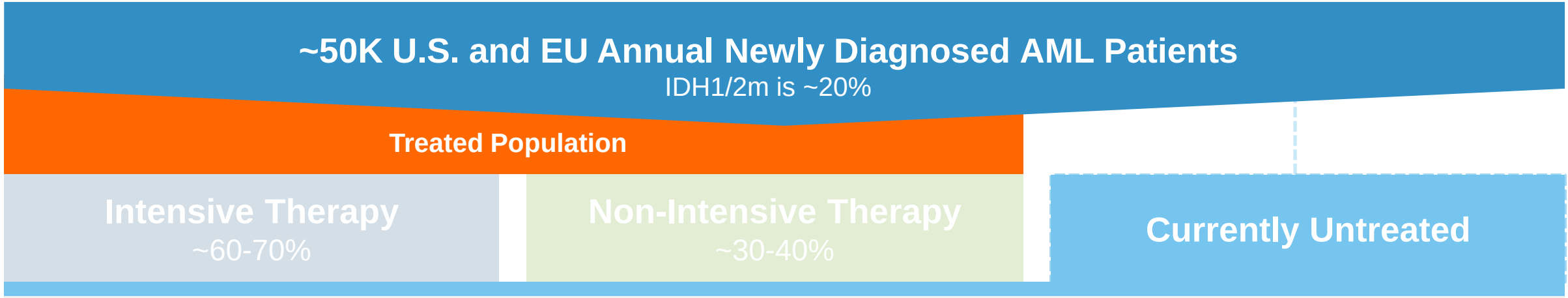
Enrollment Expected to Complete in 2020

BROAD IST SUPPORT

VENCLEXTA® Combination
XOSPATA® Combination
BEAT AML Master Trial

Sources: SEER. Cancer Stat Facts: AML 2015 and Epiphany EPIC oncology numbers; American Cancer Society AML 2017; ASCO 2018; VENCLEXTA® is a registered trademark of Abbvie; XOSPATA® is a registered trademark of Astellas

sNDA Submission Provides Potential to Offer Clinical Benefit to Patients with No Current Treatment Options



PHASE 1 SINGLE AGENT TIBSOVO® DATA

- Median age 76.5 years
- 79% sAML; 41% prior HMA
- Safety consistent with single agent data
- 58% ORR (19 of 33)
- 42% CR+CRh rate (14 of 33)
- 67% CR+CRh patients remain in response at 12 months

NEXT STEPS

sNDA APPROVAL

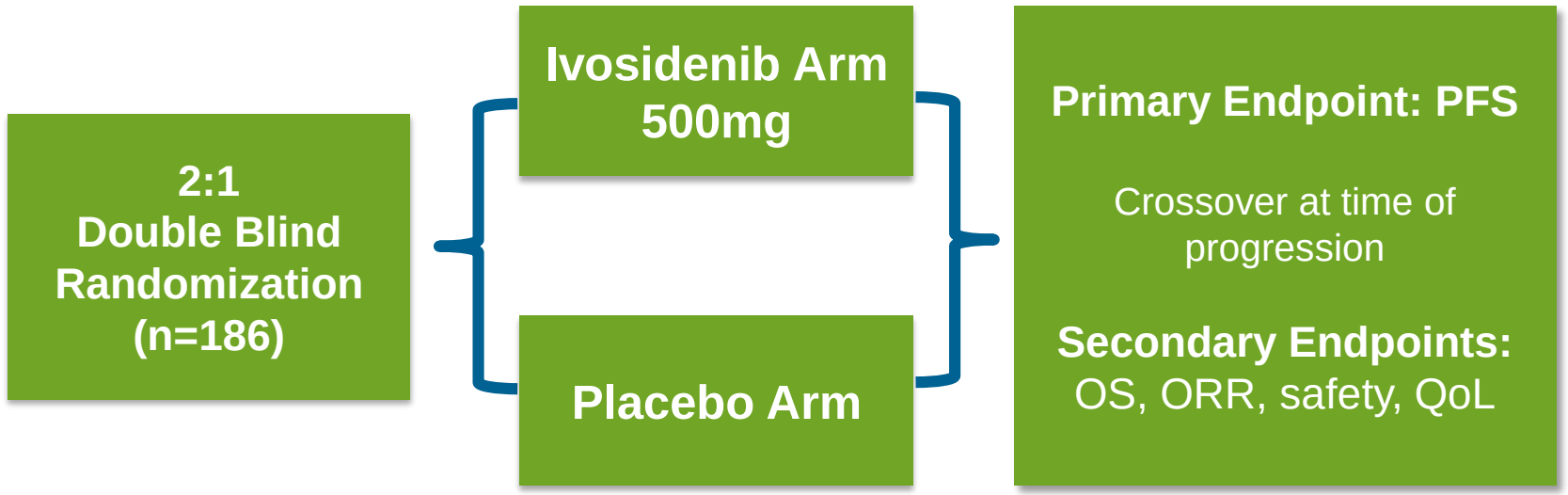
sNDA Submitted December 2018

Potential approval in 2019

Sources: SEER. Cancer Stat Facts: AML 2015 and Epiphany EPIC oncology numbers; American Cancer Society AML 2017; ASCO 2018; ASH 2018

Registration-Enabling Phase 3 Cholangiocarcinoma Study Fully Enrolled; Plan to File sNDA by Year-end

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

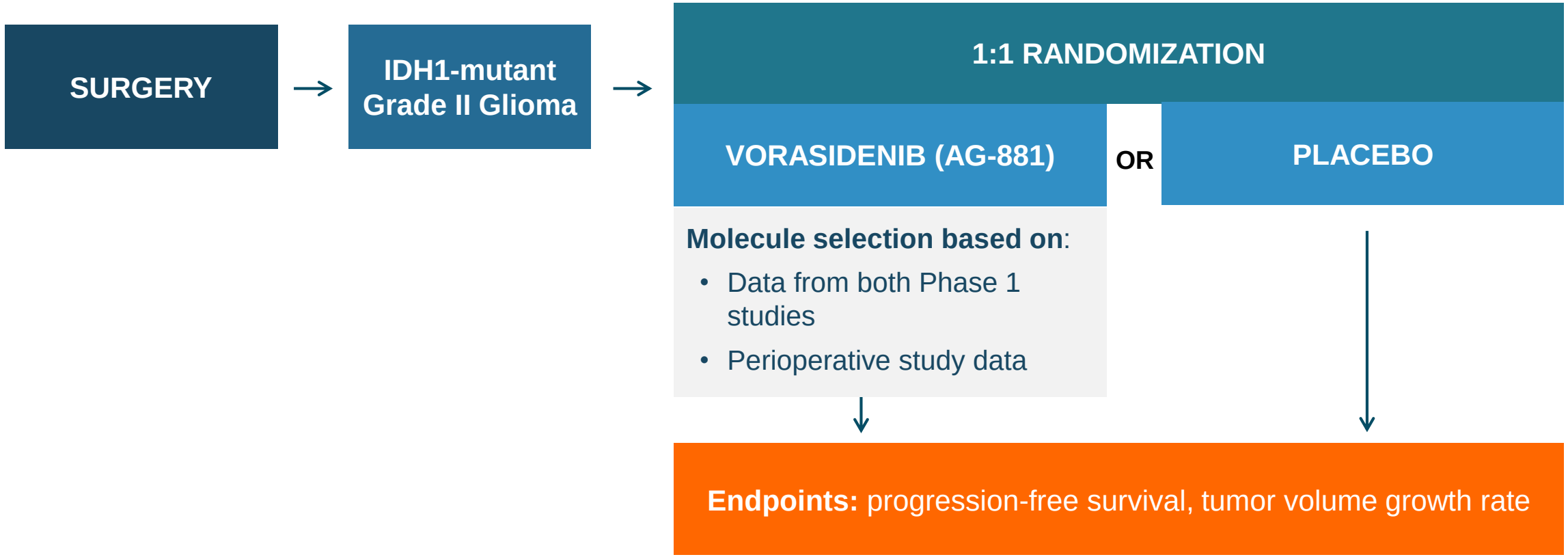


The study has 96% power to detect a hazard ratio of 0.5 with a one-sided alpha of 0.025

ClinicalTrials.gov Identifier: NCT02989857

Topline data from the Phase 3 ClarIDHy study of TIBSOVO® in IDH1m second line or later cholangiocarcinoma expected in 1H and full data to be presented in 2H 2019

Pivotal Path in WHO Grade II Glioma: Aim to Delay Progression to Chemotherapy and/or Radiotherapy



Registration-enabling Phase 3 study of vorasidenib to initiate by year-end 2019; Perioperative data submitted for presentation at ASCO

Mitapivat Path to Approval: Potential First Disease-Modifying Therapy for Adult PK Deficiency

ACTIVATE

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

ACTIVATE-T

- **~20 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**

Broadening the Opportunity for Mitapivat in Thalassemia and Pediatric PK Deficiency Patients

PHASE 2 THALASSEMIA STUDY INITIATED

- ~20 non-transfusion dependent adults
- Evaluating 50 and 100 mg BID
- Primary endpoint: hemoglobin response (1.0 g/dL increase over baseline at 12 weeks)
- Goal to achieve proof of concept in 2019

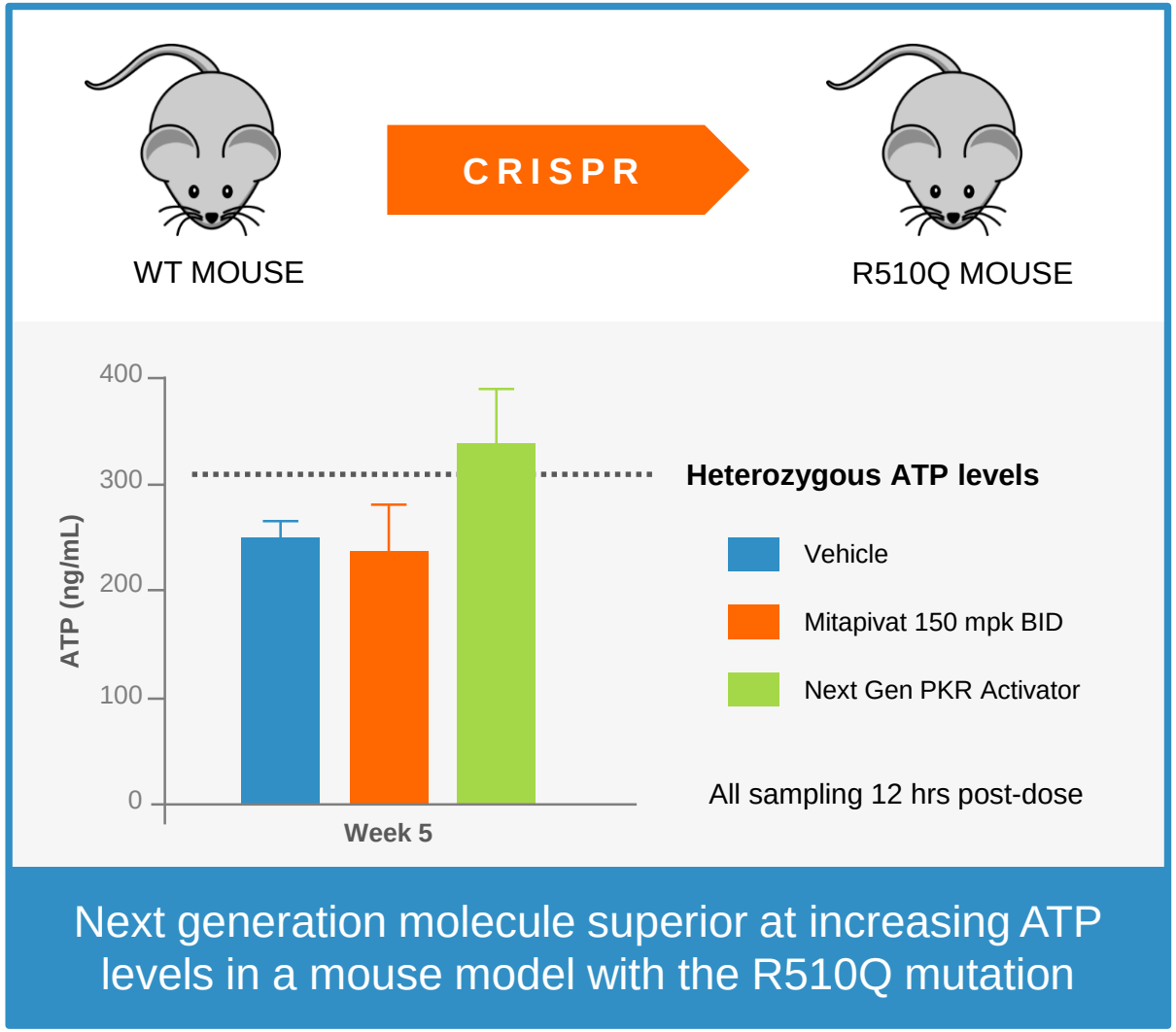
POTENTIAL PATH FORWARD FOR MITAPIVAT IN PK DEFICIENCY PEDIATRICS

- Safety and efficacy observed in DRIVE PK extension phase warrants evaluation of mitapivat in pediatric PK deficiency patients
- Juvenile toxicology studies underway
- Discussion with regulators planned for 2019
- Primary goal to develop mitapivat in a pediatric population

Committed to Continued Development of PKR Activators for the Treatment of Every Patient with PK Deficiency

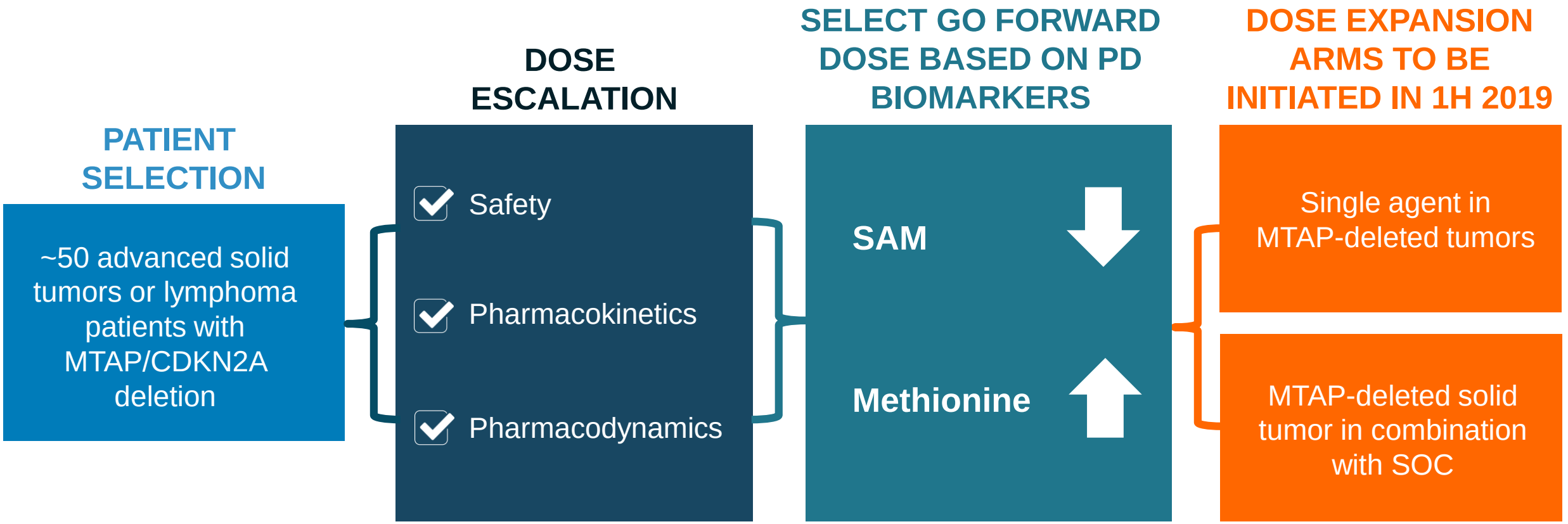
DEVELOPMENT CANDIDATE FOR A NEXT GENERATION PKR ACTIVATOR SELECTED

- More potent across a range of PKR mutations
- Address patients who do not have a sufficient response to mitapivat
- IND planned in next 12-18 months



Next generation molecule superior at increasing ATP levels in a mouse model with the R510Q mutation

Advancing AG-270 to Next Phase of Clinical Development



ClinicalTrials.gov Identifier: NCT03435250

Updated preclinical data for AG-270 accepted for presentation at AACR;
First clinical data from the Phase 1 to be presented in 2H 2019

Phase 1 Study of DHODH Inhibitor AG-636 in Lymphoma

DHODH catalyzes a critical step in pyrimidine biosynthesis

Dihydroorotate

DHODH

Orotate

UMP

RNA/DNA biosynthesis

LYMPHOMA

Phase 1 Study in Treatment Refractory Lymphoma

Planned for 1H 2019

Dose Escalation

- Determine MTD
- PK and PD to guide dose and schedule
- Safety and tolerability
- Evaluation of anti-lymphoma activity

Dose Expansion

- Confirm safety of Phase 2 dose
- Further assessment of anti-lymphoma activity

ACUTE MYELOID LEUKEMIA

Phase 1 Study in Treatment Refractory AML Planned

TIBSOVO® Launch Update

Steve Hoerter, Chief Commercial Officer

agios

Strong Launch in the Relapsed/Refractory Population Sets the Stage for IDHm Inhibitors as the Cornerstone of AML Therapy

~50K U.S. and EU Annual Newly Diagnosed AML Patients
IDH1/2m is ~20%

RELAPSED / REFRACTORY
~50% of Treated Patients

IDHIFA®
(enasidenib) tablets
100mg • 50mg

$68M
2018 Worldwide Net Sales
U.S. Co-commercialization with Celgene

TIBSOVO®
(ivosidenib) tablets

$13.8M
2018 U.S. Net Sales

MAA
Submitted & Validated

~80%
Physicians Testing for IDH1/IDH2 mutations

200+
Unique Prescribers as of YE 2018

 Sources: Market research as of August 2018; Agios estimates; SEER. Cancer Stat Facts: AML 2015 and Epiphany EPIC oncology numbers; American Cancer Society. AML 2017.

Fourth Quarter and Full Year 2018 Financial Results

Andrew Hirsch, Chief Financial Officer

Fourth Quarter and Full Year 2018 Financial Results

Statement of Operations	Three Months Ended 12/31/18	Three Months Ended 12/31/17	Year Ended 12/31/18	Year Ended 12/31/17
Total Revenue	$30.0M	$9.8M	$94.4M	$43M
Collaboration Revenue	18.4M	8.6M	73.3M	41.1M
TIBSOVO® Net Sales	9.4M	--	13.8M	--
Royalty Revenue	2.2M	1.2M	7.2M	1.9M
Cost of Sales	0.7M	--	1.4M	--
Research & Development Expense	93.8M	77.2M	341.3M	292.7M
Selling, General & Administrative Expense	31.9M	22.7M	114.1M	71.1M

Balance Sheet	12/31/18	12/31/17
Cash, Cash Equivalents and Marketable Securities	$805.4M	$567.8M

December 31, 2018 cash balance provides runway through at least the end of 2020

Q&A

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