First Quarter 2019 Financial Results

May 2, 2019

Agios Conference Call Participants

Prepared Remarks

Introduction

- KENDRA ADAMS, Vice President, External Communications & Investor Relations

Business Highlights and TIBSOVO® Commercial Update

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

Clinical Development Progress

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

First Quarter 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), 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 and Commercial Updates

Jackie Fouse, Ph.D., Chief Executive Officer

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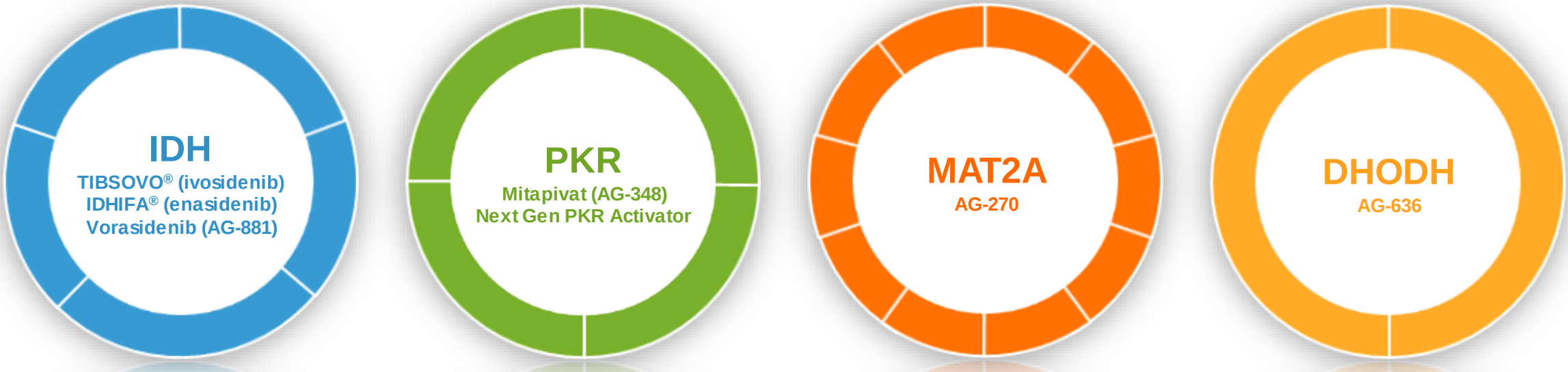
Productive Research & Discovery Engine Has Produced Four Key Targets with Multiple Disease Opportunities



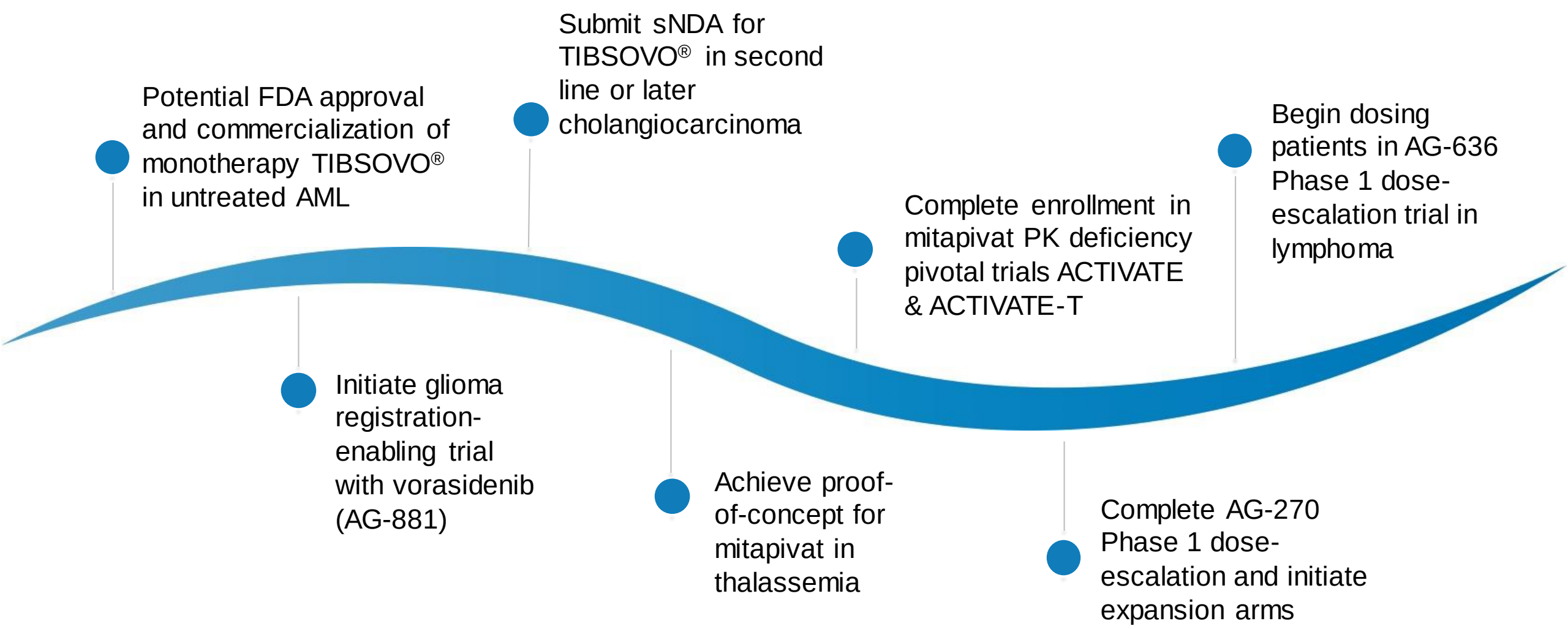
AML
Low Grade Glioma
Cholangiocarcinoma
Chondrosarcoma
MDS

Adult PK Deficiency
Pediatric PK Deficiency
Sickle Cell Disease
Thalassemia

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

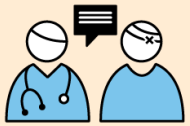
Lymphoma
AML

2019 Key Milestones Position Agios for Long-term Value Creation



TIBSOVO® Q1 2019 Performance

$9.1M Net U.S. Sales of TIBSOVO®



~90% Academic and Community Physicians Testing for IDH1/IDH2 mutations

~15% Increase in Unique Prescribers over Q4 2018

Commercial Team Launch Ready for Potential sNDA Approval in the Frontline

Market research as of February 2019; Agios estimates

Clinical Development Progress

Chris Bowden, M.D., Chief Medical Officer

What's New Today

Key Business Updates

- Commercial team is launch ready for TIBSOVO® sNDA approval by June 21
- HOVON/AMLSG Phase 3 7+3 combination study initiated
- ACTIVATE-T enrollment increased from 20 to 40 patients
- NIH-sponsored study of mitapivat in sickle cell disease planned to initiate in 2019
- First patient has been dosed in Phase 2 study of mitapivat in thalassemia
- Three planned expansion arms for AG-270 Phase 1 study now initiating in Q3
- Sites are open for the Phase 1 study of AG-636 in lymphoma

ASCO Data Presentations

- Data from first cohort in Phase 1 perioperative study of TIBSOVO® and vorasidenib in recurrent IDH1 mutant low-grade glioma
- Updated data from a Phase 1 study of single agent TIBSOVO® in IDH1 mutant newly diagnosed AML ineligible for standard therapies
- Updated data from the Phase 1 combination study of TIBSOVO® and azacitidine in newly diagnosed AML with an IDH1 mutation

What's Possible for IDHm Patients

NOW

- Relapsed/Refractory AML

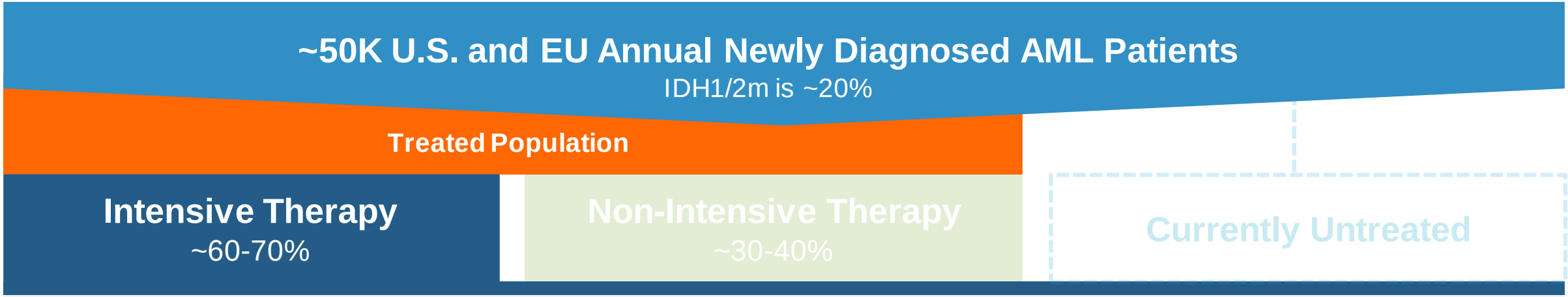
NEXT

- Newly diagnosed AML ineligible for standard treatment
- 2L Cholangiocarcinoma

FUTURE

- Low Grade Glioma
- IC-eligible frontline AML
- IC-ineligible frontline AML
- MDS
- Chondrosarcoma

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

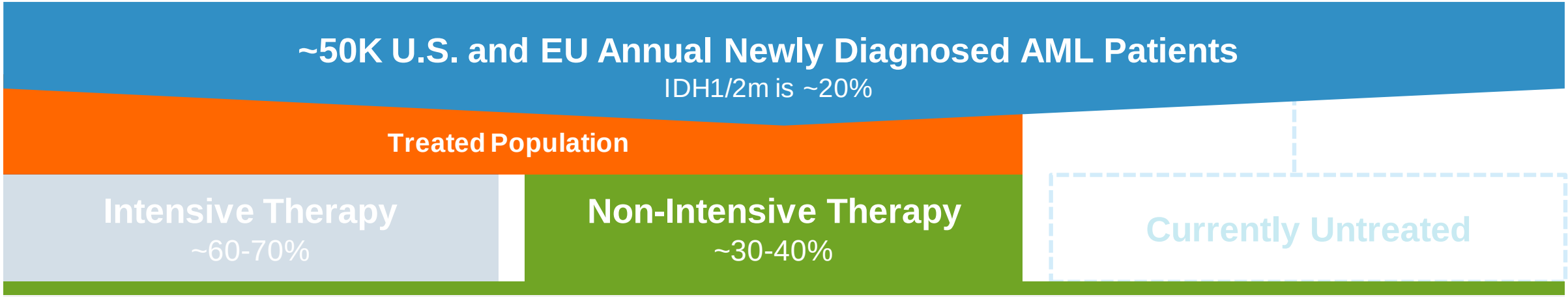
Trial initiated in Q2 2019

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 data to be presented at ASCO

- Median age 76 years
- Safety consistent with previously reported data
- 78% ORR (18 of 23)
- 65% CR/CRi/CRp rate (15 of 23)
- 57% CR rate (13 of 23)
- 82% 12-month overall survival rate

NEXT STEPS

AGILE PHASE 3 STUDY

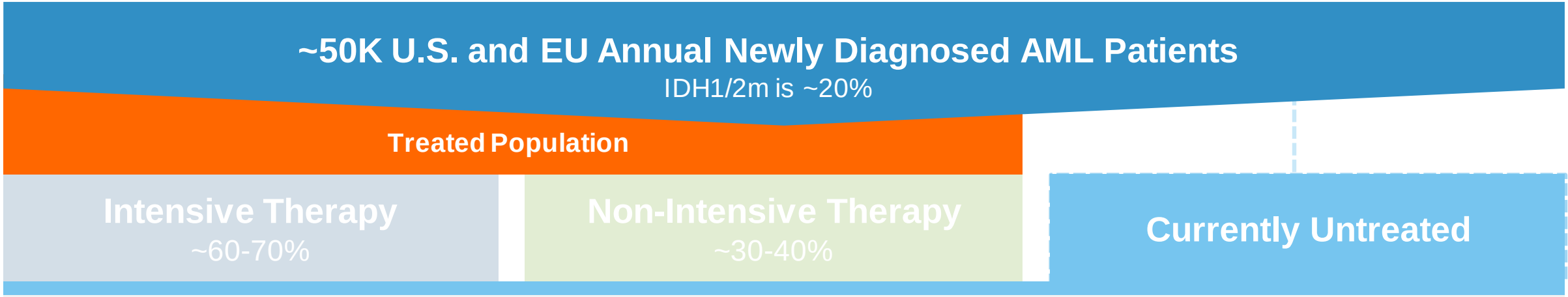
Enrollment Expected to Complete in 2020

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; Leukemia Meeting 2019; VENCLEXTA® is a registered trademark of Abbvie; XOSPATA® is a registered trademark of Astellas

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



PHASE 1 SINGLE AGENT TIBSOVO® DATA

Updated data to be presented at ASCO

- 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 accepted February 2019

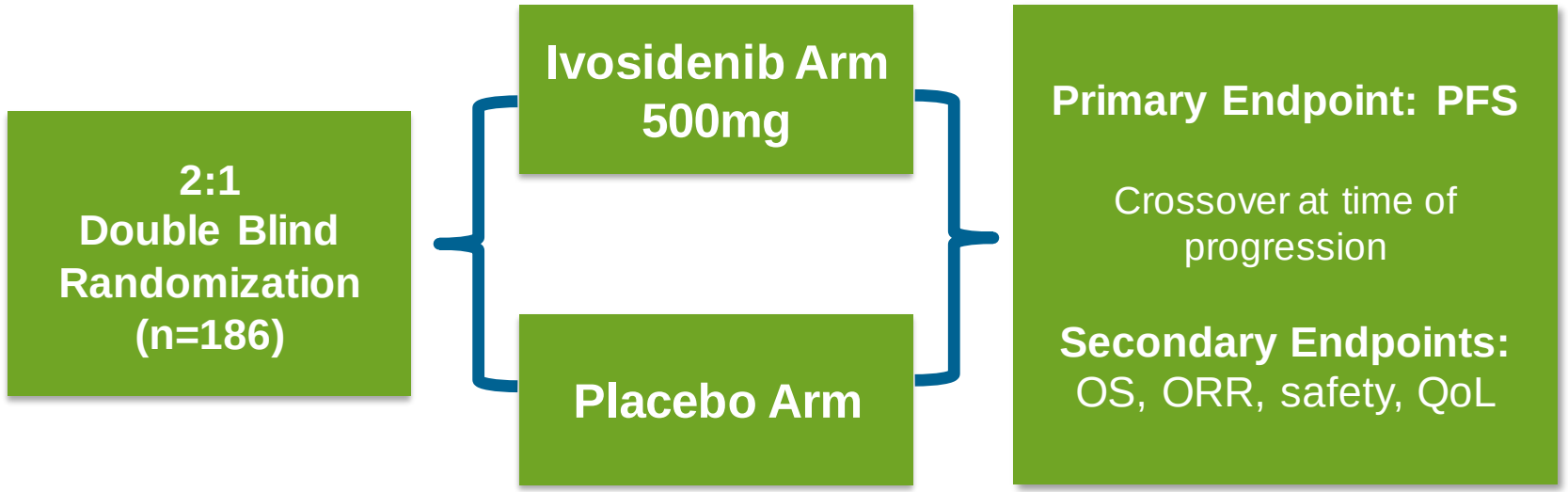
PDUFA date set for June 21, 2019

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

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

ClarIDHy

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

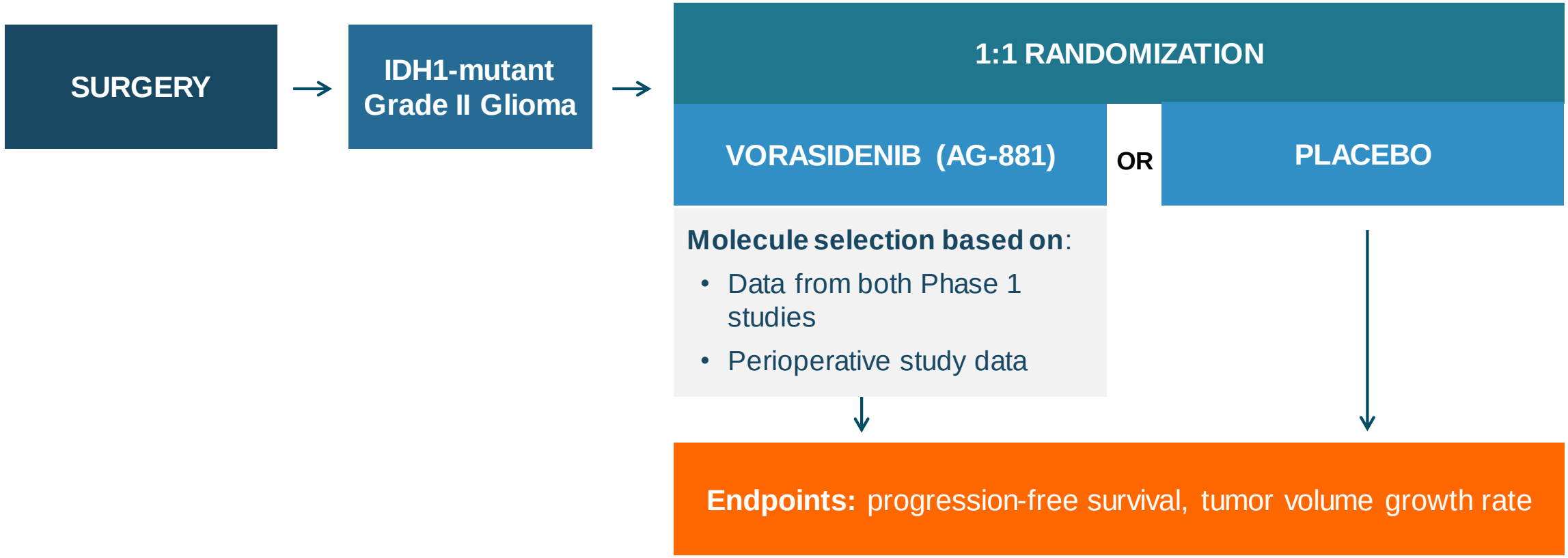


The study has 96% power to detect a hazard ratio of 0.5 with a one-sided alpha of 0.025

ClinicalTrials.gov Identifier: NCT02989857

Topline data from the Phase 3 ClarIDHy study of TIBSOVO® in IDH1m second line or later cholangiocarcinoma expected in Q2 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

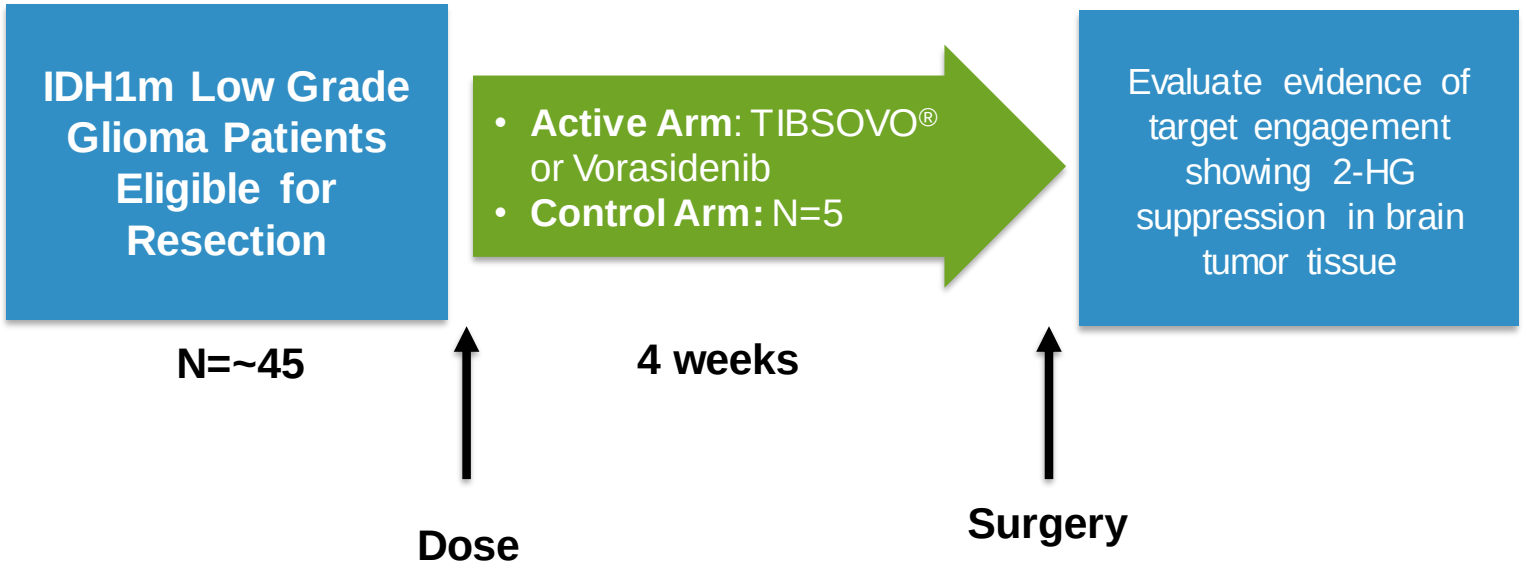


Registration-enabling Phase 3 study of vorasidenib to initiate by year-end 2019; Perioperative data submitted for presentation at ASCO

Phase 1 Perioperative Study with TIBSOVO® and Vorasidenib 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 TIBSOVO® & 98% with vorasidenib)
- Assess impact of IDHm inhibition on differentiation and epigenetic profiles in tumor tissue
- Assess the safety of both molecules



ClinicalTrials.gov Identifier: NCT03343197

First data from study to be presented at ASCO

What's Possible with PKR Activators

NOW

- **Adult PK Deficiency**

NEXT

- **Thalassemia**
  - First patient dosed in Phase 2 study

FUTURE

- **Pediatric PK Deficiency**
- **Sickle Cell Disease**
  - NIH-sponsored study to initiate in 2019

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

ACTIVATE

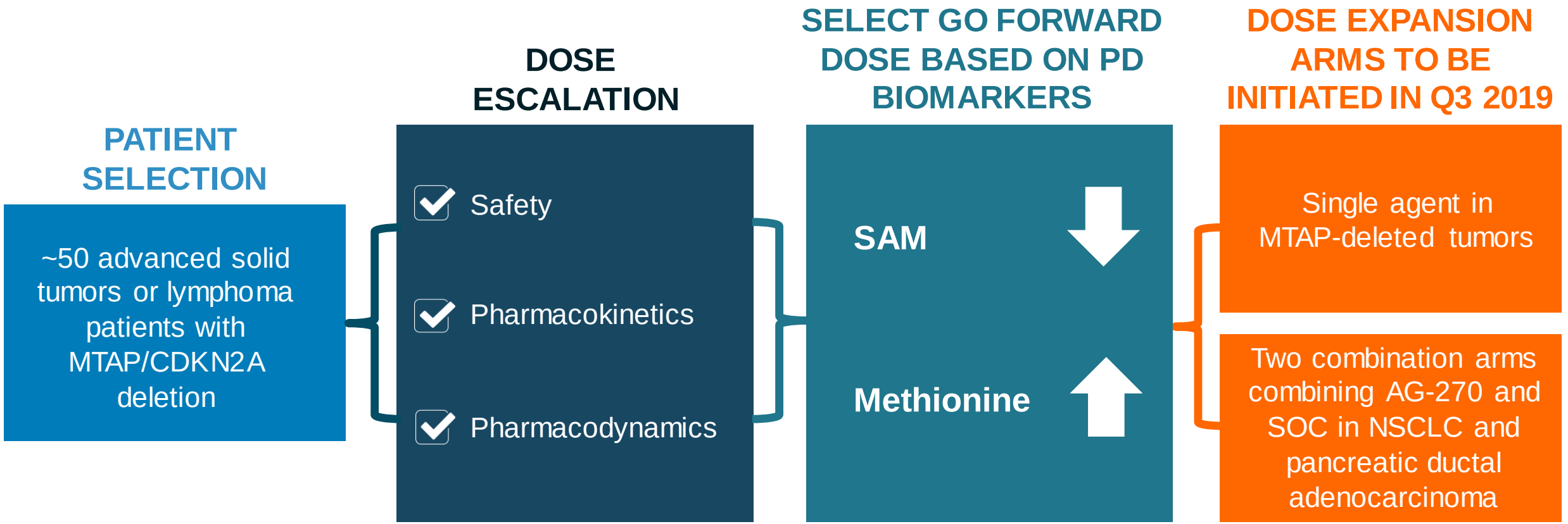
- **~80 patients** treated for 6 months
- **Primary endpoint:** portion of patients achieving 1.5 g/dL Hb increase over multiple visits

ACTIVATE-T

- **~40 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**

Advancing AG-270 to Next Phase of Clinical Development



ClinicalTrials.gov Identifier: NCT03435250

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

Sites Open and Screening Patients

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

First Quarter 2019 Financial Results

Andrew Hirsch, Chief Financial Officer and Head of Corporate Development

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First Quarter 2019 Financial Results

Statement of Operations	Three Months Ended 3/31/19	Three Months Ended 3/31/18
Total Revenue	$30.2M	$8.8M
Collaboration Revenue TIBSOVO® Net Sales Royalty Revenue	18.9M 9.1M 2.2M	7.3M -- 1.4M
Cost of Sales	0.3M	--
Research & Development Expense	95.6M	78.2M
Selling, General & Administrative Expense	31.8M	24.6M

Balance Sheet	3/31/19	12/31/18
Cash, Cash Equivalents and Marketable Securities	$707.8M	$805.4M

March 31, 2019 cash balance provides runway through at least the end of 2020

Q&A

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