Third Quarter 2015 Financial Results

November 5, 2015

Agios Conference Call Participants

Prepared Remarks

Introduction

- RENEE LECK, Sr. Manager, Investor & Public Relations

Corporate Strategy and Vision

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

Clinical Development Updates

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

Third Quarter Year Financial Results

- GLENN GODDARD, SVP Finance

Cautionary Note Regarding 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, including those regarding Agios' expectations and beliefs about: the potential of IDH1/IDH2 and pyruvate kinase-R mutations as therapeutic targets; the potential benefits of Agios' product candidates targeting IDH1/IDH2 or pyruvate kinase-R mutations, including AG-221, AG-120, AG-881, AG-348 and AG-519; its plans and timelines for the clinical development of AG-221, AG-120, AG-881, AG-348 and AG-519; its plans regarding future data presentations; its financial guidance regarding the amount of cash, cash equivalents and marketable securities that the company will have as of December 31, 2015, and the potential benefit of its strategic plans and focus. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "would," "could," "potential," "possible," "hope" 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 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 or the various remarks made 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 agreement with Celgene, and general economic and market conditions. These and other risks are described in greater detail under the caption "Risk Factors" included in Agios' Annual Report on Form 10-Q for the quarter ended June 30, 2015, and other filings that Agios may make with the Securities and Exchange Commission in the future. Any forward-looking statements contained in this presentation or in remarks made 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.

Corporate Strategy and Vision

DAVID SCHENKEIN, M.D.

Chief Executive Officer

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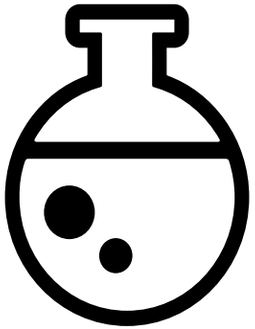
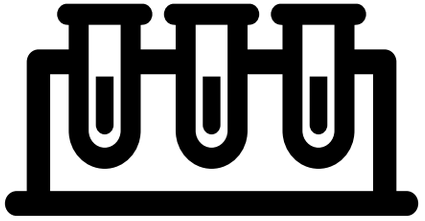
Agios 2015



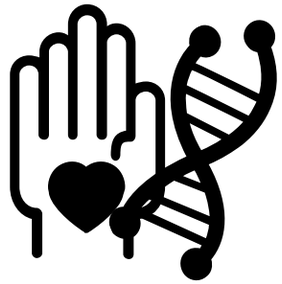
Five clinical stage investigational medicines with possibility to help a large number of genetically identified patients

Two investigational medicines accelerating towards approval and commercialization



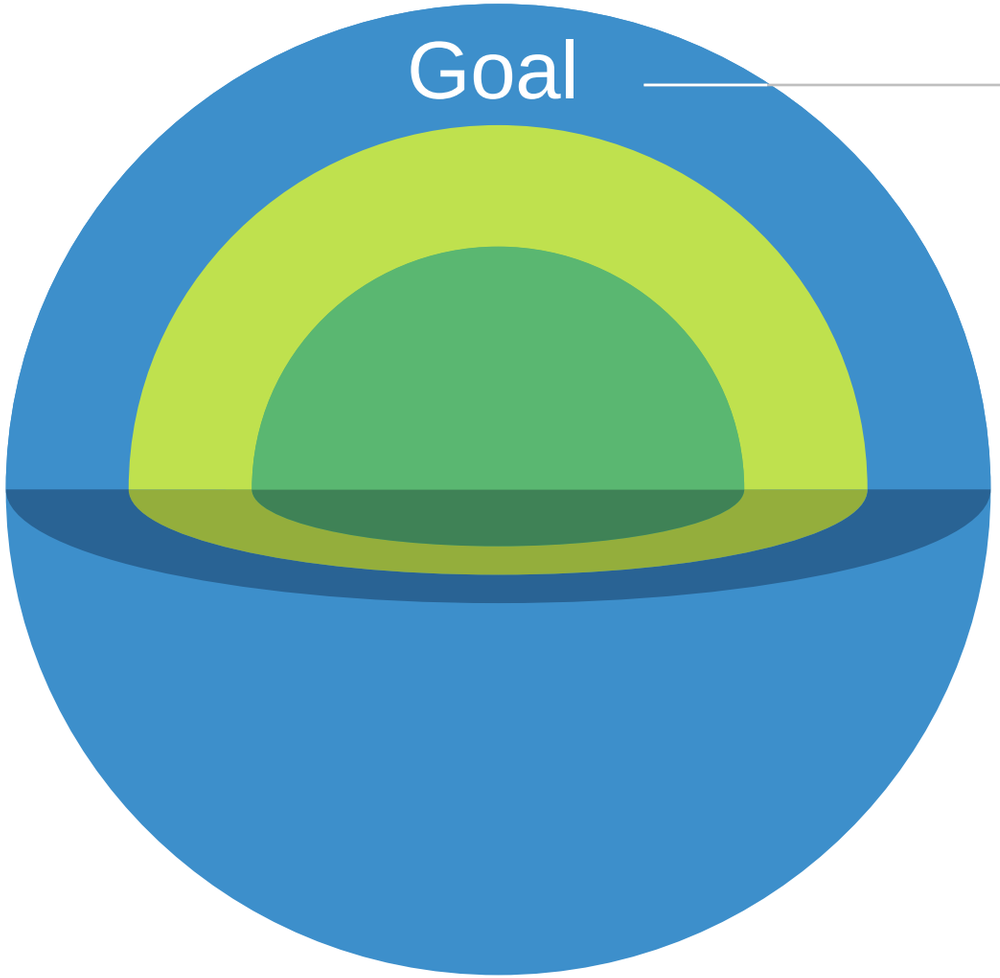
A robust and novel pre-clinical pipeline in both cancer and RGDs

An organization preparing for commercialization



A passion to help patients and follow great science

What's Possible for IDHm Patients

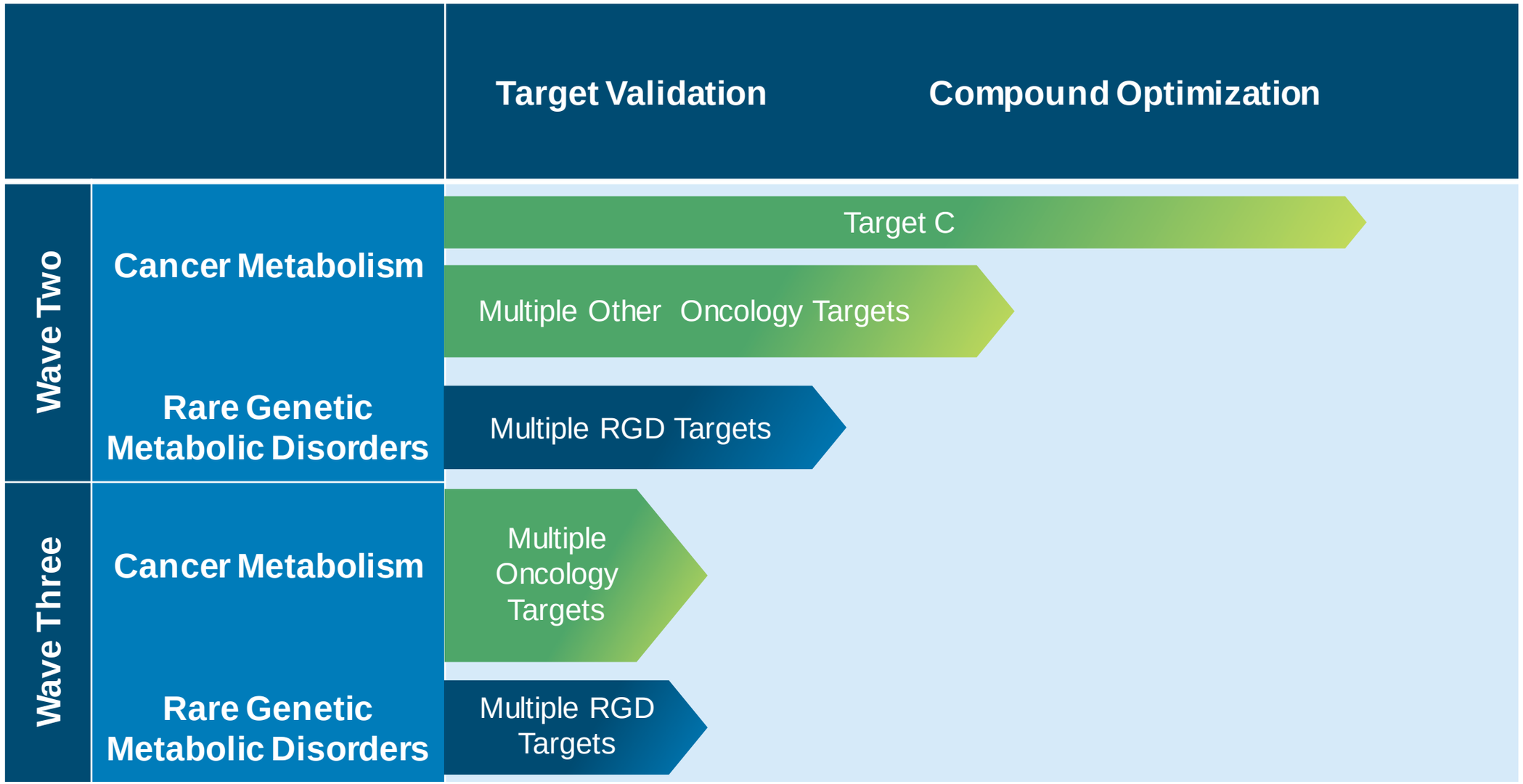


All IDHm patients screened and treated with an IDHm inhibitor for the entire course of their disease

Novel First-in-Class Clinical Portfolio

Candidate	Indication	Early Stage Clinical Development	Late Stage Clinical Development	Primary Commercial Rights
AG-221 (IDH2m inhibitor)	**R/R AML**	Phase 3		Agios U.S. Co-promotion and Royalty
	R/R AML	Phase 1 Dose Escalation → Expansion Cohorts		
	Frontline AML	Phase 1b Combinations (Q4'15)		
	Frontline AML	Phase 1/2 Combinations (Q1'16)		
	MDS/Heme Malig	Phase 1 Expansion		
	Solid Tumors	Phase 1 Dose Escalation		
AG-120 (IDH1m inhibitor)	**AML**	Phase 3 (1H'16)		agios (U.S. Rights) / Celgene (EX-U.S.)
	R/R AML	Dose Escalation → Expansion Cohorts		
	MDS/Heme Malig	Phase 1 Expansion		
	Frontline AML	Phase 1b Combinations (Q4'15)		
	Frontline AML	Phase 1/2 Combinations (Q1'16)		
	Solid Tumors	Phase 1 Dose Escalation		
AG-881 (pan-IDHm inhibitor)	**R/R AML**	Phase 1 Dose Escalation		agios / Celgene Joint Worldwide Collaboration
	Solid Tumors	Phase 1 Dose Escalation		
AG-348 (PK (R) Activator)	**PK Deficiency**	Phase 2 DRIVE PK		agios
AG-519 (PK (R) Activator)	**PK Deficiency**	Phase 1 (Q1'16)		agios

Novel First-in-Class Research Portfolio



Clinical Development Updates: Cancer Metabolism

CHRIS BOWDEN, M.D.

Chief Medical Officer

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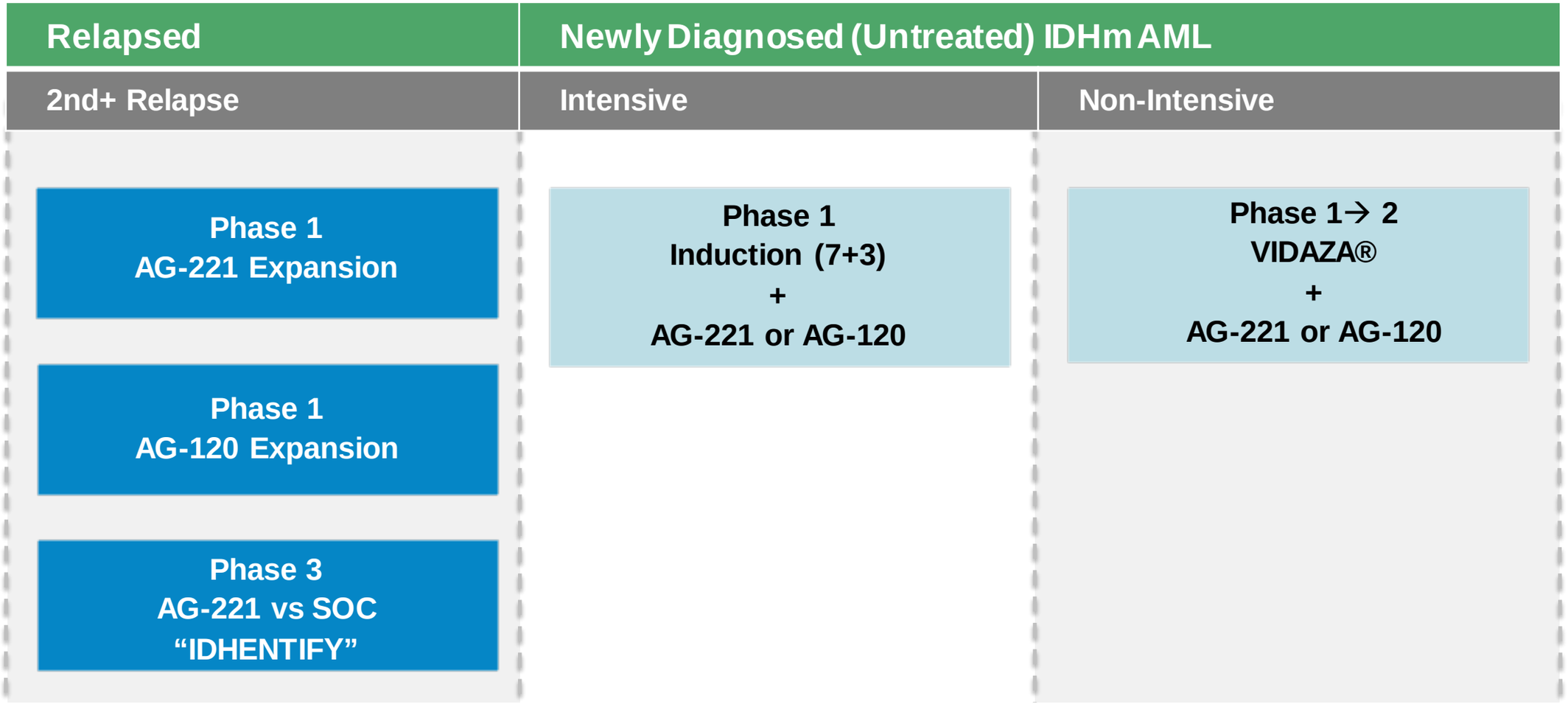
Development Program Targets Multiple Lines of Treatment from R/R to Frontline AML



IDHENTIFY: Global Phase 3 Study to Evaluate the Efficacy of AG-221 in IDH2m R/R AML

KEY INCLUSION

- ≥ 60 years
- IDH2m
- R/R AML after 2nd/3rd line

STRATIFIED BY

- Prior intensive therapy
- Prior refractory
- Prior HSCT

1:1 Randomization

AG-221/CC-90007

- Starting 100 mg QD
- AG-221 + BSC
- 28 day cycles

COMPARATOR

- Best Supportive Care (BSC)
- VIDAZA® + BSC
- Low dose Ara-C + BSC
- Intermediate Dose Ara -C + BSC

1° ENDPOINT

- OS

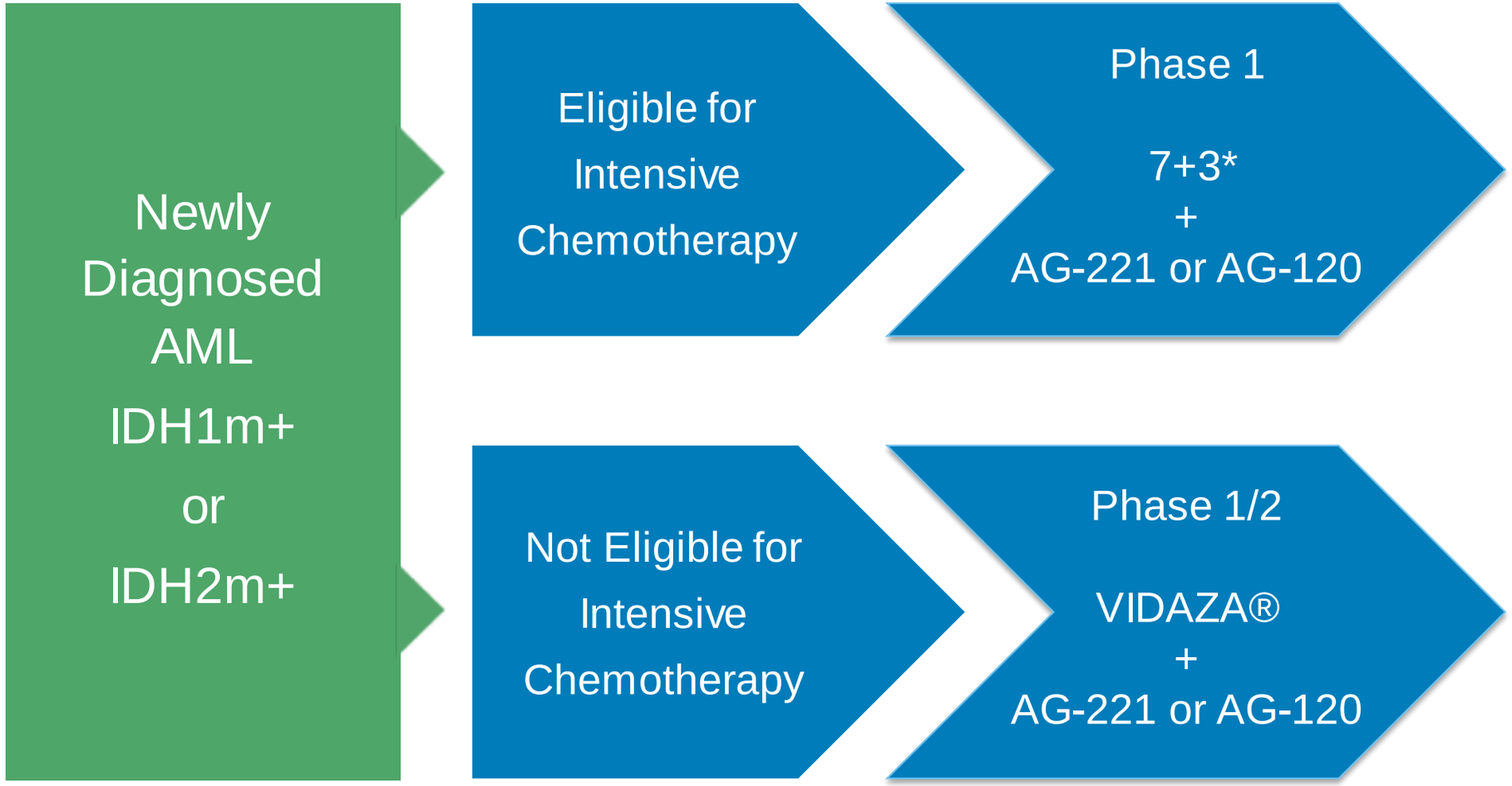
KEY 2° ENDPOINTS

- EFS
- ORR
- Duration of response
- 1-yr survival
- CR rate
- Safety

- **280 patients**
- **No crossover**

Phase 3 for AG-120 also planned for 1H16

Frontline Therapy: Novel Clinical Development Strategies



Potential Registration Trials

*7+3-Ara-C (Days 1-7), Daunorubicin or Idarubicin (D1-3)

What's Next at ASH

- 7 abstracts accepted
  - Four for AG-221 and AG-120, including new data from dose escalation and expansion cohorts
  - Three for PK deficiency program, including PK/PD data for AG-348 and natural history study findings

AG-120: Current Development Status in Advanced Solid Tumors

Phase 1
All IDH1m

Advanced hematologic malignancies

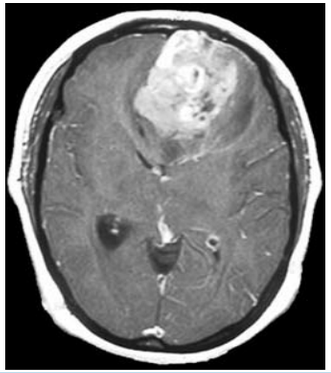
Advanced solid tumors

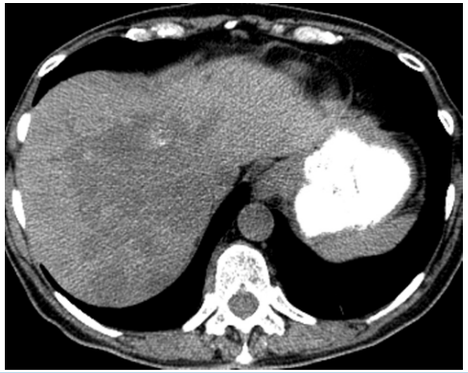
- IHCC
- Chondrosarcoma
- Glioma
- Other advanced solid tumors

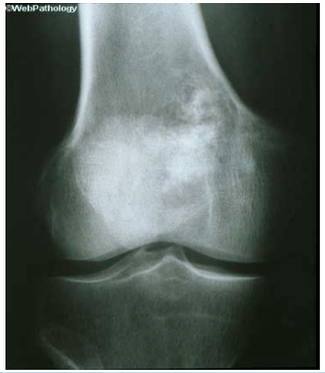
Phase 1 dose escalation study in advanced solid tumors

- Study initiated in March 2014
- First data to be presented at AACR-NCI-EORTC
- Assess clinical activity, safety and tolerability of AG-120 as single agent
- Administered orally in 28-day cycles
- Assess 2-HG levels, differentiation & efficacy

Goal is to Explore AG-120 and AG-221 in IDHm Solid Tumors







	Glioma	Intrahepatic Cholangiocarcinoma (IHCC)	Chondrosarcoma
	Low grade and 2^{ary} GBM	Bile ducts	Cartilage
Incidence (cases/year U.S.)	5K	2K – 4K	700-1000
Prevalence (U.S.)	24K	5K	--
IDH1m frequency	68-74%	11-24%	40-52%
IDH2m frequency	3-5%	2-6%	6-11%
Treatment options	Surgery, XRT Chemotherapy	Surgery, Chemotherapy Liver transplantation	Surgery, XRT Chemotherapy
5-year O/S	~32– 68%*	~9%	~10-90%

Other solid tumor types include colon, melanoma, lung, ovarian.
Multiple sources, including market research and SEER. Estimates will continue to evolve with additional future data
*excludes primary GBM

AG-881: Brain Penetrant, Pan-IDHm Inhibitor

Two Phase 1 Studies Ongoing

Phase 1
IDH1m or IDH2m

Advanced solid tumors, including glioma

- Study initiated in June 2015
- Assess safety, tolerability and clinical activity of AG-881 as a single agent
- Administered orally in 28-day cycles
- Assess 2-HG levels, differentiation & efficacy

Advanced hematologic malignancies

- Study initiated in August 2015
- IDH1m or IDH2m patients whose cancer progressed on prior IDHm inhibitor therapy eligible
- Purpose and dosing schedule same as above

Clinical Development Updates: Rare Genetic Metabolic Disorders

CHRIS BOWDEN, M.D.

Chief Medical Officer

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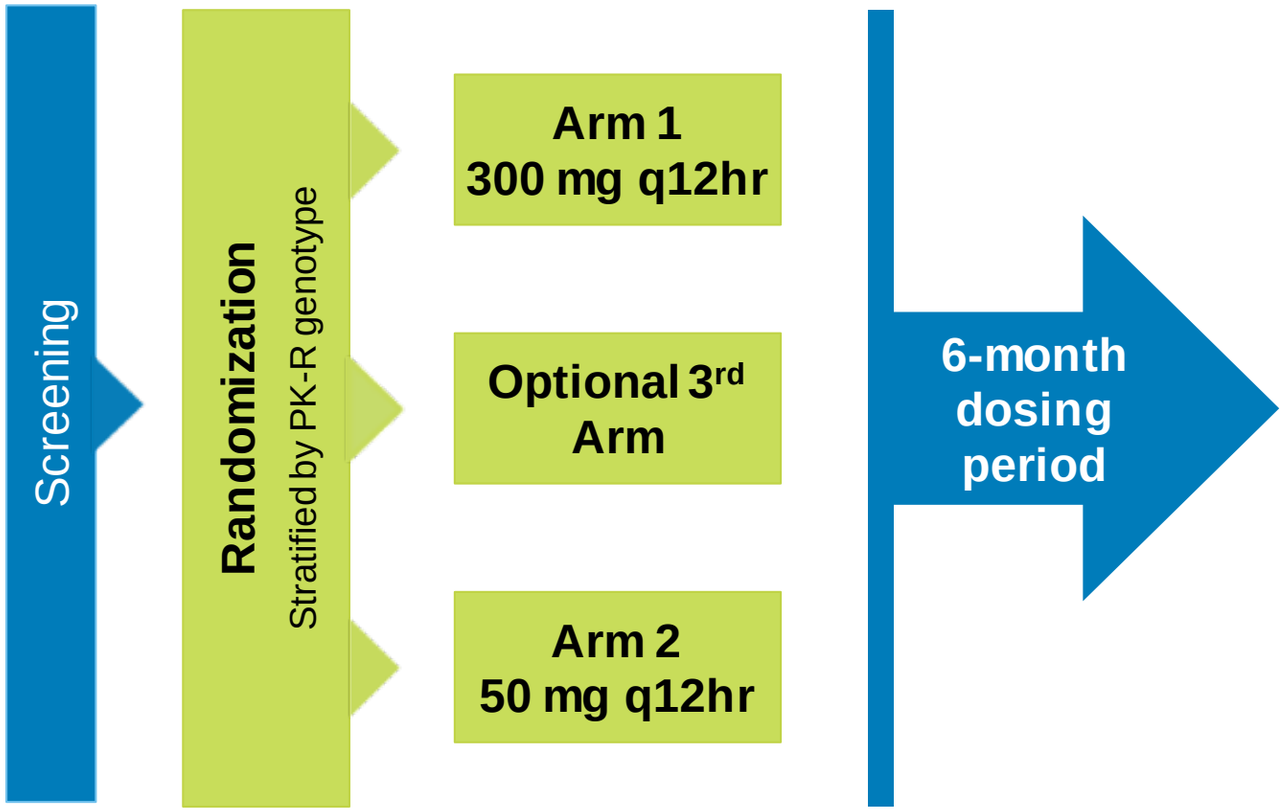
Global Phase 2 DRIVE PK Study Open and Enrolling

DRIVEPK

Transfusion-independent PK-deficient adults n=25 in each arm



Primary endpoints

- Safety and tolerability

Secondary endpoints

- PK of AG-348
- PD response: ATP, 2,3-DPG
- Indicators of clinical activity: hemoglobin, hematocrit, reticulocyte count, and other hematologic parameters.

Follow-on PKR Activator AG-519

Healthy Volunteer Study to Open in 1Q 2016

- AG-519 is a potent, highly selective and oral PKR activator
- Differentiated chemical structure vs. AG-348
- No activity against the aromatase enzyme
- Similar activity in vitro, in vivo and ex vivo relative to AG-348

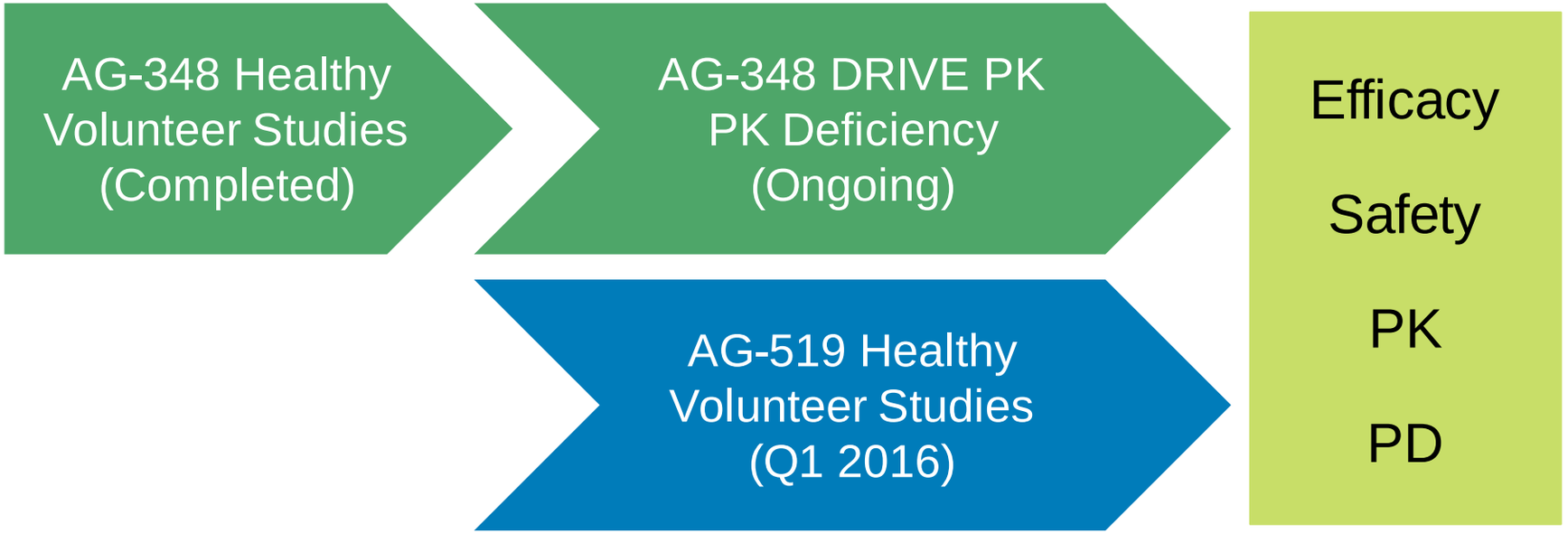
One protocol, two steps, healthy volunteers

Step 1: Integrated SD-MD

- 4 dose-ascending cohorts: 8 subjects per cohort (n=32)
- Placebo controlled (6A, 2P)

Step 2: Bioavailability and Food Effect Study

AG-519 Provides Optionality for Clinical Development



Pivotal Trials:

- Adults
- Adolescents
- Children

Clinical data from AG-519 and AG-348 trials will determine late stage development path

Natural History Study Designed to Inform Development

First data presented in June 2015 at EHA

Additional data to be presented at ASH in Dec. 2015

Key objectives:

- Understanding the disease, including range of symptoms and complications:
  - Transfusion burden
  - Patient reported outcome measures
  - Incidence and timing of splenectomy
  - Prevalence and treatment of iron overload
  - Prevalence of co-morbidities
- Identifying patients and treatment centers
- Capturing retrospective/prospective clinical data, QoL measures and genetic diagnostic information

Selected Third Quarter Financial Results & 2015 Guidance

GLENN GODDARD

SVP, Finance

agios

Third Quarter 2015 Selected Financial Summary

Balance Sheet	September 30, 2015	December 31, 2014
Cash, cash equivalents and marketable securities	$408.0M	$467.4M
Total Assets	$449.1M	$491.9M

Statement of Operations	September 30, 2015	September 30, 2014
Collaboration Revenue(1)	$5.5M	$33.9M
Research & Development Expense(2)	$36.0M	$25.5M
General and Administrative Expense	$9.9M	$5.2M

(1) Collaboration revenue decreased due to the July 2014 amendment of the 2010 collaboration agreement with Celgene requiring the application of new accounting guidance during the three months ended September 30, 2014.

(2) During 1Q15, the Company began offsetting R&D expense for amounts received from Celgene for reimbursement of costs related to our IDH programs. R&D expense reported for the three months ended June 30, 2015 is presented net of $7.8 million, compared to no offset for cost reimbursement for the comparable period in 2014.

Expect to end 2015 with cash position of more than $350M

Does not include any additional program-specific milestone payments

Making a Difference for Patients & Building Long-Term Value

Vision

Science

Team/Culture

Execution