Third Quarter 2016 Financial Results

November 3, 2016

Agios Conference Call Participants

Prepared Remarks

Introduction

- KENDRA ADAMS, Sr. Director, Investor & Public Relations

Third Quarter Highlights

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

Clinical Development Progress

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

Third Quarter 2016 Financial Results

- ANDREW HIRSCH, Chief Financial Officer

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 or other genetic mutations, including enasidenib (AG-221), AG-120, AG-881, AG-348 and AG-519; its plans and timelines for the clinical development of enasidenib (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, 2016; 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 agreements 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' Quarterly Report on Form 10-Q for the quarter ended June 30, 2016, 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.

Third Quarter Highlights

David Schenkein, M.D., Chief Executive Officer

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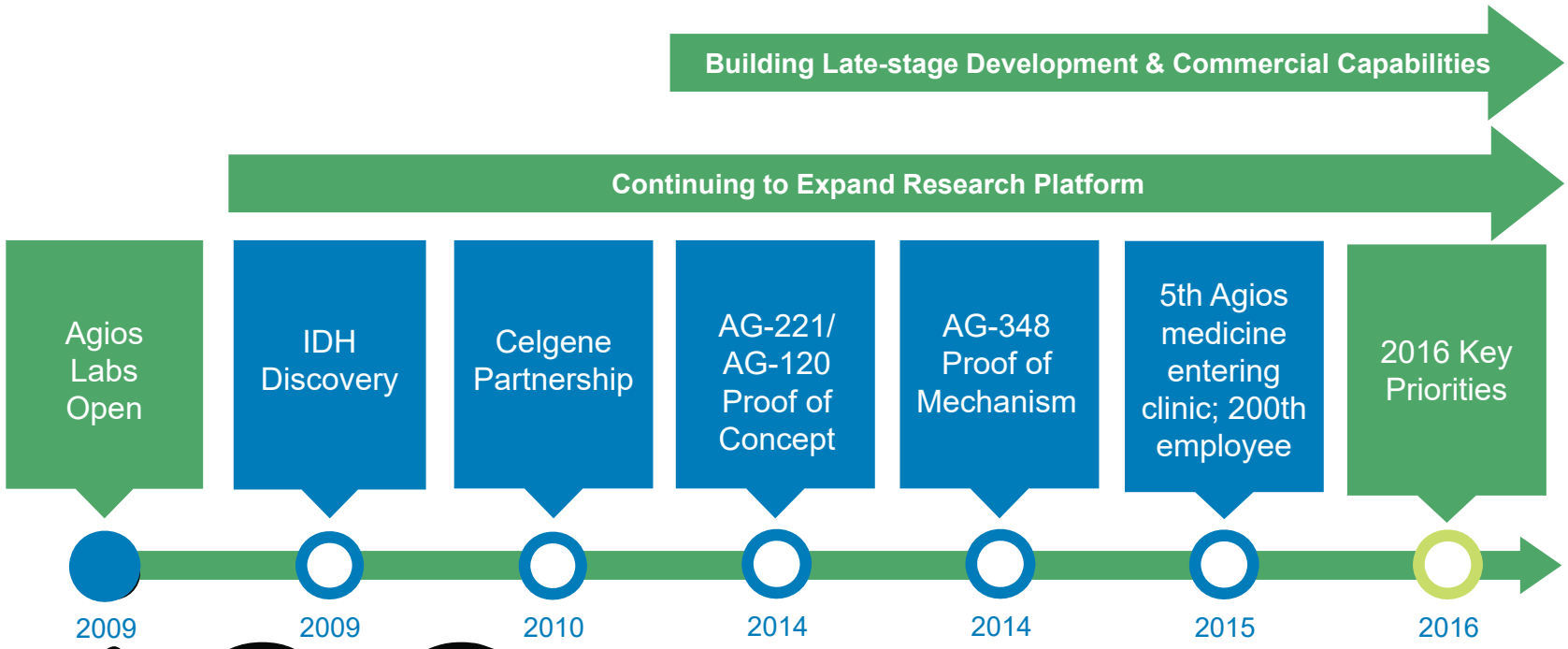
Building a Great Sustainable Biopharmaceutical Company



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Our 2016 Key Priorities: Maturing into a Late-stage Company

Rapid and broad late stage clinical development for IDHm inhibitors

Demonstrate clinical activity of PKR activators in patients

Advance research and initiate preclinical development of next wave research program

2016 ASH Presentations

Topic/Date	Title	No.
AG-348 DRIVE PK – Oral Sun, Dec 4, 5:45-6:00pm (4:30-6pm session)	Effects of AG-348, a Pyruvate Kinase Activator, on Anemia and Hemolysis in Patients With Pyruvate Kinase Deficiency: Data From the DRIVE PK Study	402
AG-120 Clinical – Oral Mon, Dec 5, 4:45-5:00pm (4:30-6pm session)	Determination of IDH1 Mutational Burden and Clearance via Next-Generation Sequencing in Patients With IDH1 Mutation-Positive Hematologic Malignancies Receiving AG-120, a First-in-Class Inhibitor of Mutant IDH1	1070
AG-221 MDS – Oral Sun, Dec 4, 9:30-9:45am (9:30-11am session)	Enasidenib (AG221), a Potent Oral Inhibitor of Mutant Isocitrate Dehydrogenase 2 (IDH2) Enzyme, Induces Hematologic Responses in Patients with Myelodysplastic Syndromes (MDS)	343
AG-519 Clinical – Poster Sat, Dec 3, 5:30-7:30pm	Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of Multiple Doses of AG-519, an Allosteric Activator of Pyruvate Kinase-R, in Healthy Subjects	1264
AG-348 RBC metabolism – Poster Sun, Dec 4, 6:00-8:00pm	Characterization of Metabolic Response to AG-348, an Allosteric Activator of Red Cell Pyruvate Kinase, in Healthy Volunteers and Pyruvate Kinase Deficiency Patients	2452
AG-519 PK/PD – Poster Sat, Dec 3, 5:30-7:30pm	Population Pharmacokinetics and Pharmacodynamics of AG-519, a Pyruvate Kinase Activator for the Treatment of Pyruvate Kinase Deficiency, in Human Healthy Volunteers	1263
NHS Iron overload – Poster Sun, Dec 4, 6:00-8:00pm	Iron Overload is Highly Prevalent in All Disease Severity States in Pyruvate Kinase Deficiency (PKD)	2430

Clinical Development Progress

Chris Bowden, M.D., Chief Medical Officer

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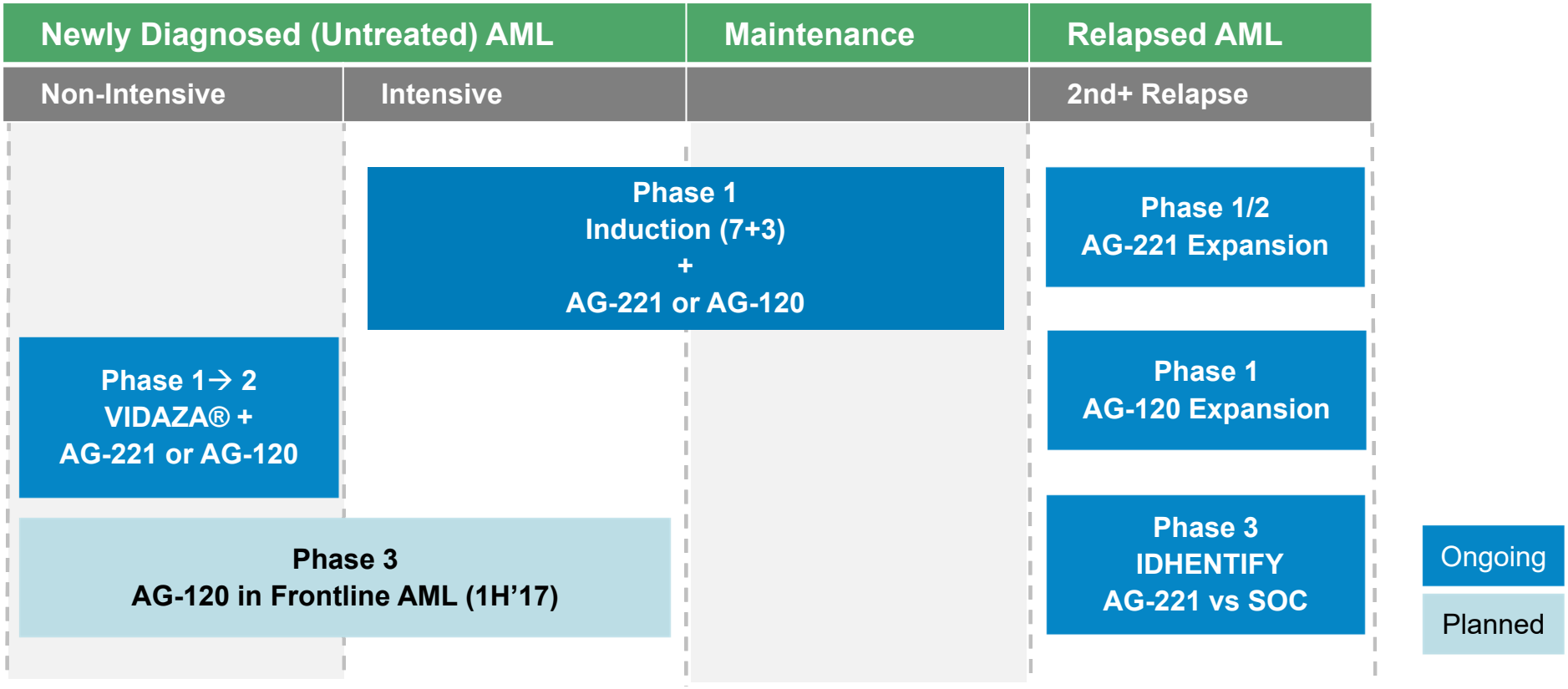
AG-120 ASH Presentation

Topic/Date	Title	No.
AG-120 Clinical – Oral Mon, Dec 5, 4:45-5:00pm (4:30-6pm session)	Determination of IDH1 Mutational Burden and Clearance via Next-Generation Sequencing in Patients With IDH1 Mutation-Positive Hematologic Malignancies Receiving AG-120, a First-in-Class Inhibitor of Mutant IDH1	1070

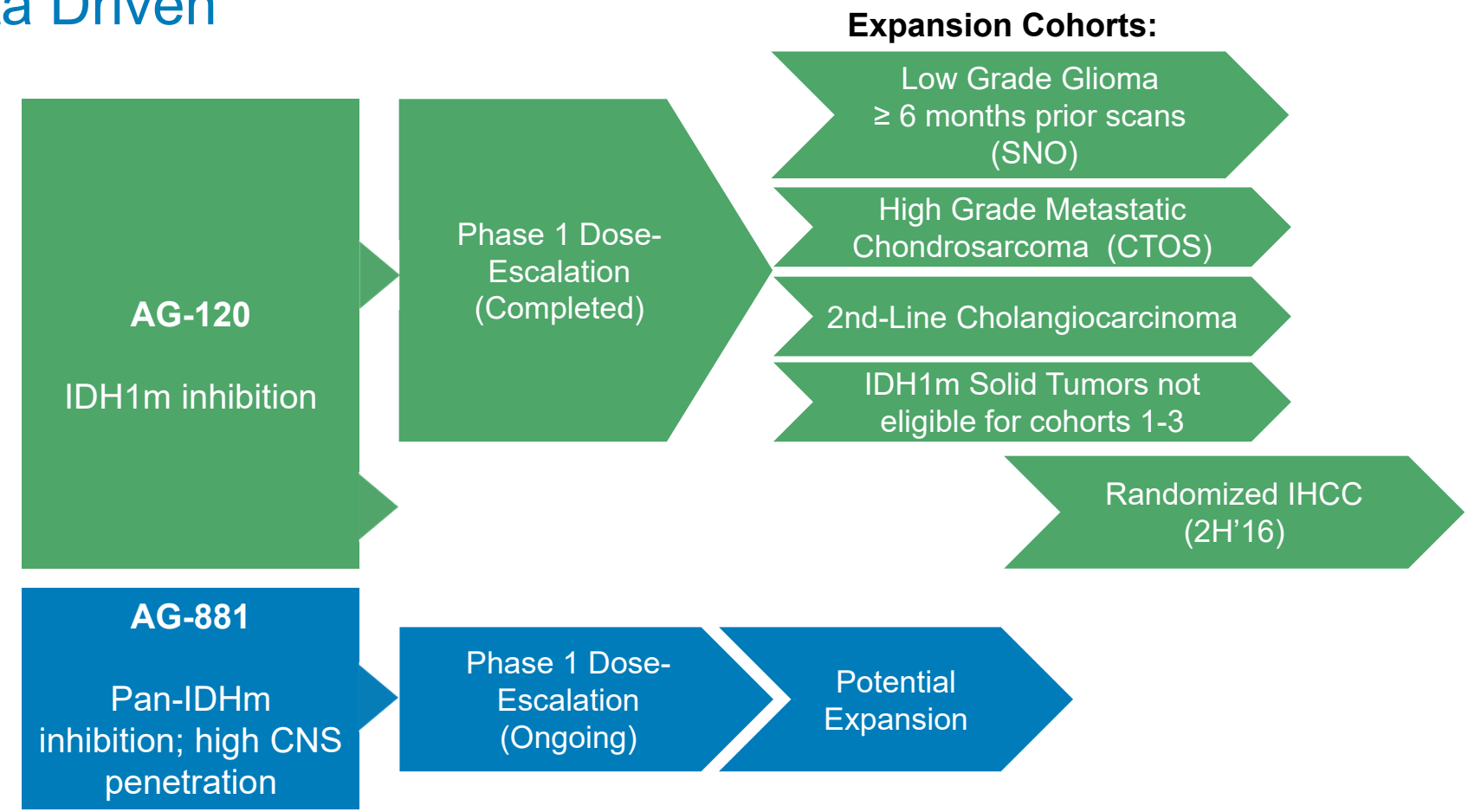
Data in the abstract show:

- Impressive single agent activity in relapsed/refractory IDH1m AML & favorable safety profile
- New molecular data demonstrate mutational clearance in some CR patients
- Additional patients & longer follow-up from dose escalation presented at meeting

Targeting Multiple Lines of Treatment in IDHm AML and Other Hematologic Malignancies



Clinical Development Path in IDH1m Solid Tumors Will Be Data Driven



DRIVE PK & AG-519 ASH Presentations

Topic/Date	Title	No.
AG-348 DRIVE PK – Oral Sun, Dec 4, 5:45-6:00pm (4:30-6pm session)	Effects of AG-348, a Pyruvate Kinase Activator, on Anemia and Hemolysis in Patients With Pyruvate Kinase Deficiency: Data From the DRIVE PK Study	402
AG-519 Clinical – Poster Sat, Dec 3, 5:30-7:30pm	Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of Multiple Doses of AG-519, an Allosteric Activator of Pyruvate Kinase-R, in Healthy Subjects	1264
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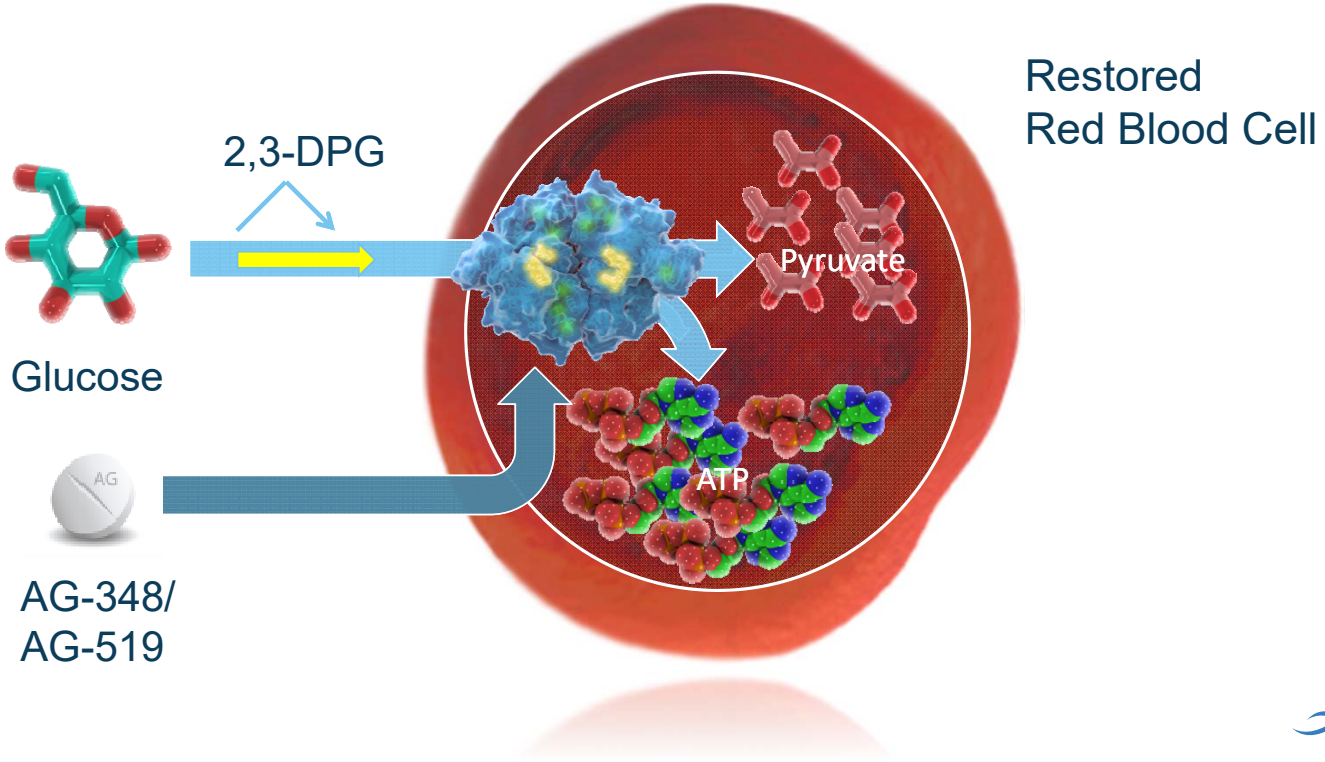
Data in the abstract show:

- DRIVE PK data demonstrate robust activity and support pivotal development of our PKR activators
- AG-519 demonstrates a favorable safety profile and robust dose-dependent changes in ATP and 2,3-DPG blood levels consistent with PKR enzyme activation
- Additional data & longer follow-up presented at meeting

DRIVE PK Metabolic ASH Presentation

Topic/Date	Title	No.
AG-348 RBC metabolism – Poster Sun, Dec 4, 6:00-8:00pm	Characterization of Metabolic Response to AG-348, an Allosteric Activator of Red Cell Pyruvate Kinase, in Healthy Volunteers and Pyruvate Kinase Deficiency Patients	2452

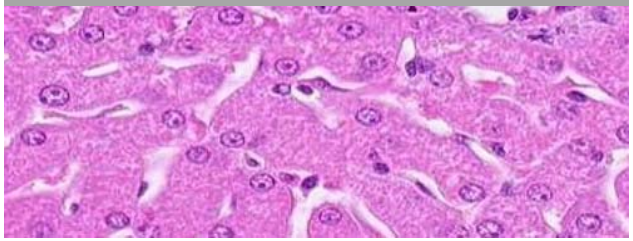
First data linking AG-348's impact on hemoglobin levels with activation of the PKR pathway in patients with PK deficiency



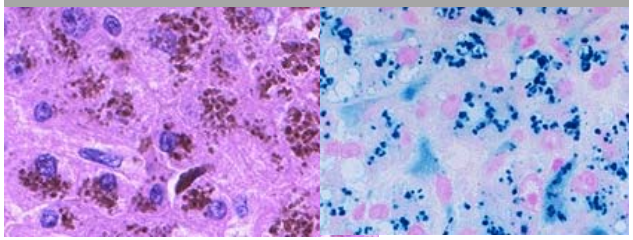
Natural History Study ASH Presentation

Topic/Date	Title	No.
NHS Iron overload – Poster Sun, Dec 4, 6:00-8:00pm	Iron Overload is Highly Prevalent in All Disease Severity States in Pyruvate Kinase Deficiency (PKD)	2430

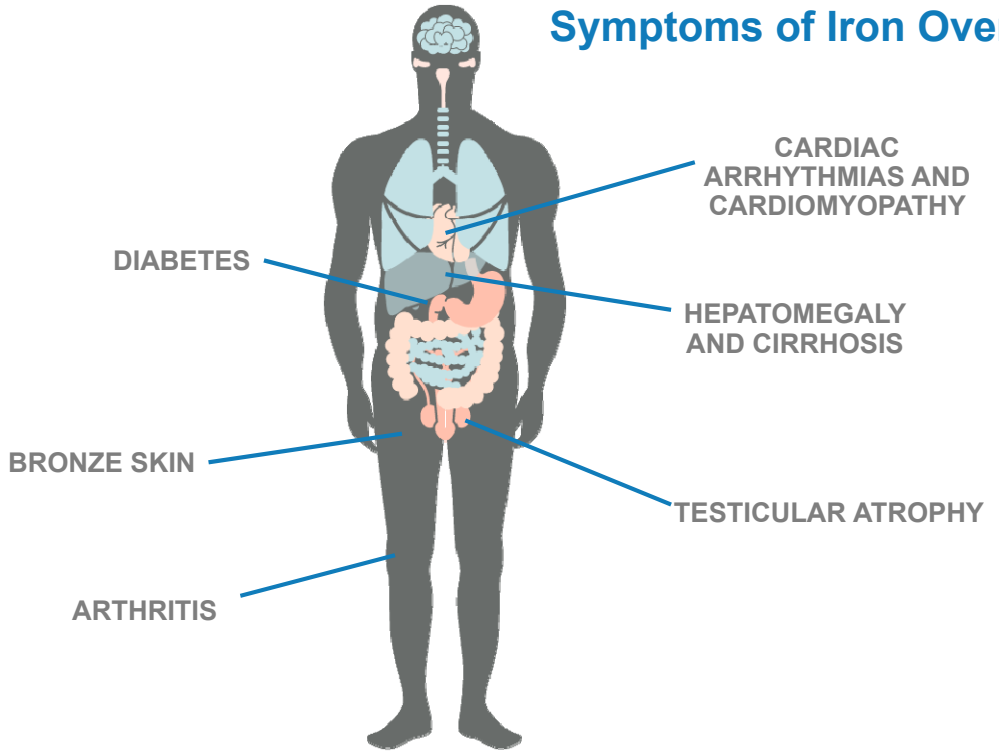
NORMAL LIVER



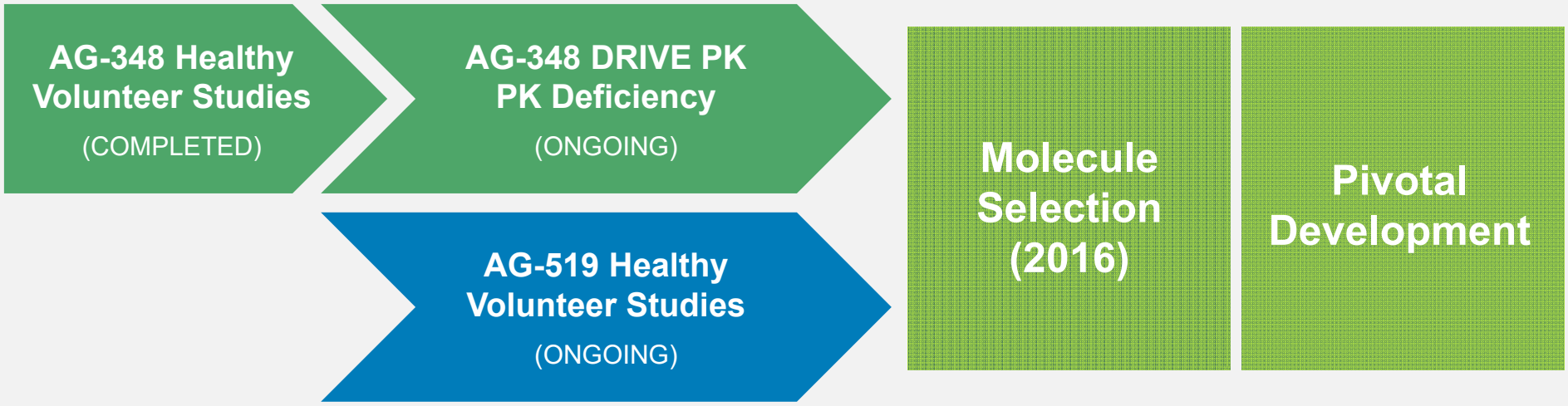
IRON DEPOSITION IN THE LIVER



Symptoms of Iron Overload



Proof-of-Concept Achieved, Moving Toward Pivotal Development



Our Pipeline

CANDIDATE	INDICATION	DRUG DISCOVERY	EARLY STAGE CLINICAL DEVELOPMENT	LATE STAGE CLINICAL DEVELOPMENT	PRIMARY COMMERCIAL RIGHTS
Enasidenib (AG-221) (IDH2m Inhibitor)	R/R AML			●	agios Celgene
	Frontline AML			●	
	MDS/HemeMalig		●		Agios U.S. Co-promotion and Royalty
AG-120 (IDH1m Inhibitor)	R/R AML			●	agios
	Frontline AML			●	
	MDS/HemeMalig		●		
	Solid Tumors		●		
AG-881 (pan-IDHm Inhibitor)	R/R AML		●		agios Celgene
	Solid Tumors		●		Joint Worldwide Collaboration
AG-348 (PK (R) Activator)	PK Deficiency			●	agios
AG-519 (PK (R) Activator)	PK Deficiency		●		agios
RESEARCH PROGRAMS					
MTAP Program		●			agios Celgene
Undisclosed CM Program		●			Joint Worldwide Collaboration
CM Research Programs		●			agios
RGD Research Programs		●			agios
Metabolic IO Research Programs		●			agios Celgene Joint Worldwide Collaboration

Third Quarter 2016 Financial Results

Andrew Hirsch, Chief Financial Officer

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Third Quarter 2016 Financial Results

Balance Sheet	September 30, 2016	June 30, 2016	December 31, 2015
Cash, Cash Equivalents & Marketable Securities	$623M	$512M	$376M
Total Assets	$672M	$558M	$420M

Statement of Operations	Three Months Ended September 30, 2016	Three Months Ended June 30, 2016	Three Months Ended September 30, 2015
Collaboration Revenue	$9M	$7M	$5M
Research & Development Expense	$61M	$51M	$36M
General & Administrative Expense	$12M	$13M	$10M

The R&D expenses reported for the three months ended September 30, 2016, June 30, 2016 and September 30, 2015 are reported net of cost reimbursements of $4.0 million, $6.0 million and $8.0 million, respectively.

Expect to end 2016 with cash position of more than $550M

Does not include any additional program-specific milestone payments

2016 Milestone Progress

IDHm Inhibitors

- ✓ Complete enrollment in AG-221 expansion arm
- Complete enrollment in AG-120 expansion arm in 2H
- First data from AG-120 dose-expansion cohort in low grade glioma expected 2H
- Initiate randomized study of AG-120 in cholangiocarcinoma in 2H

PKR Activators

- ✓ Present first data from AG-348 Phase 2 DRIVE PK and AG-519 Phase 1 healthy volunteer studies at EHA in June
- Present new findings from Natural History Study of PK deficiency in 2H
- Outline clinical development plans for PKR activators in beta-thalassemia in 2H

Research

- ✓ Published on MTAP cancer metabolism program
- Initiate preclinical development activities for MTAP cancer metabolism program

Thank you