



Fourth Quarter and Full Year 2015 Financial Results

February 18, 2016



Agios Conference Call Participants

Prepared Remarks

Introduction

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

2016 Corporate Priorities and New Announcements

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

Clinical Development Progress

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

Full Year 2015 Financial Results

- GLENN GODDARD, Senior Vice President, 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 or other genetic 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, 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 September 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.



2016 Corporate Priorities and New Announcements

David Schenkein, M.D., Chief Executive Officer



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



New Announcements & Key Milestones

PKR Activators

- First data from AG-348 Phase 2 DRIVE PK and AG-519 Phase 1 healthy volunteer studies to be submitted for presentation at EHA in June

IDHm Inhibitors

- Late-stage AG-221 and AG-120 IDH hematology program on track with 125-patient expansion cohorts enrolling and multiple frontline trials ongoing and planned
- First data from AG-120 dose-expansion cohort in low grade glioma expected 2H'16

Research

- Preclinical findings on a new research program focused on MTAP deleted cancers to be presented at the Keystone Symposia in February



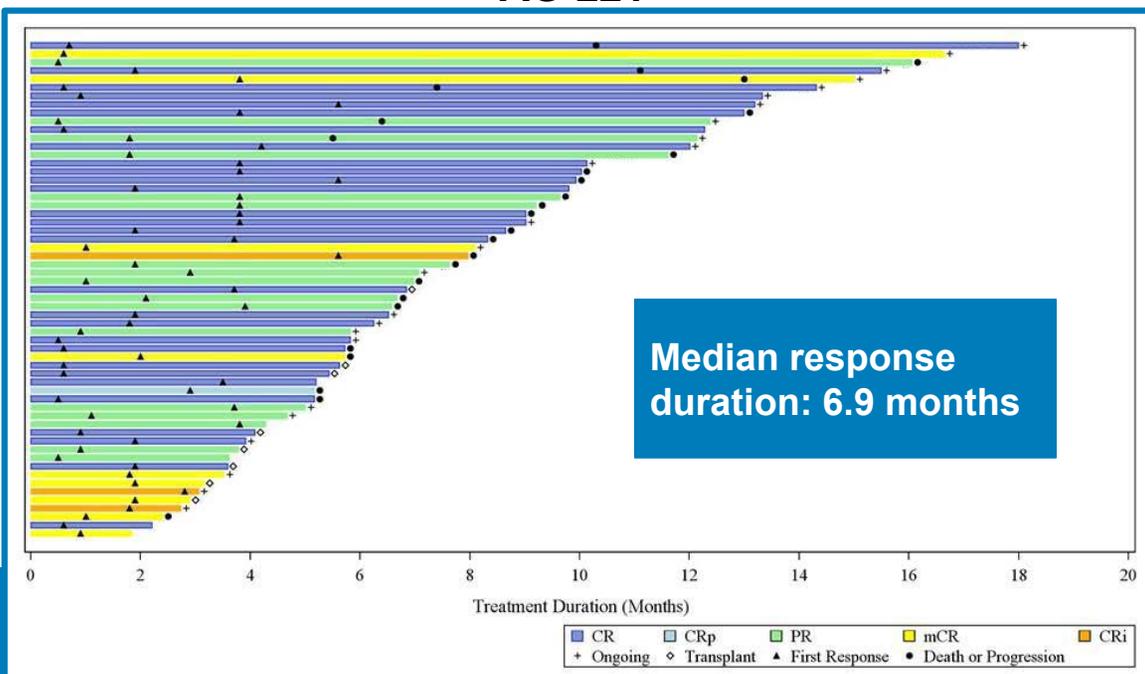
Clinical Development Progress

Chris Bowden, M.D., Chief Medical Officer

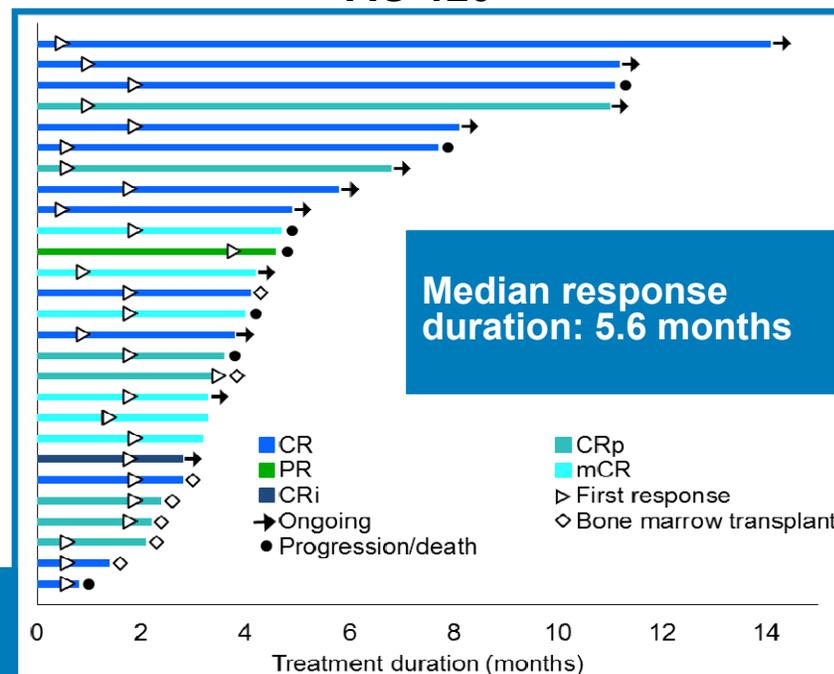


AG-221 and AG-120 Represent a New Treatment Paradigm in AML

AG-221



AG-120



First-in-Class, Oral, Potent, Selective, Reversible Inhibitors

- With ~300 patients treated, AG-221 and AG-120 demonstrate favorable safety profiles
- Impressive single agent complete and partial responses in relapsed/refractory IDHm AML
- Neutrophil and platelet improvements observed in non-CR responders and some patients with stable disease



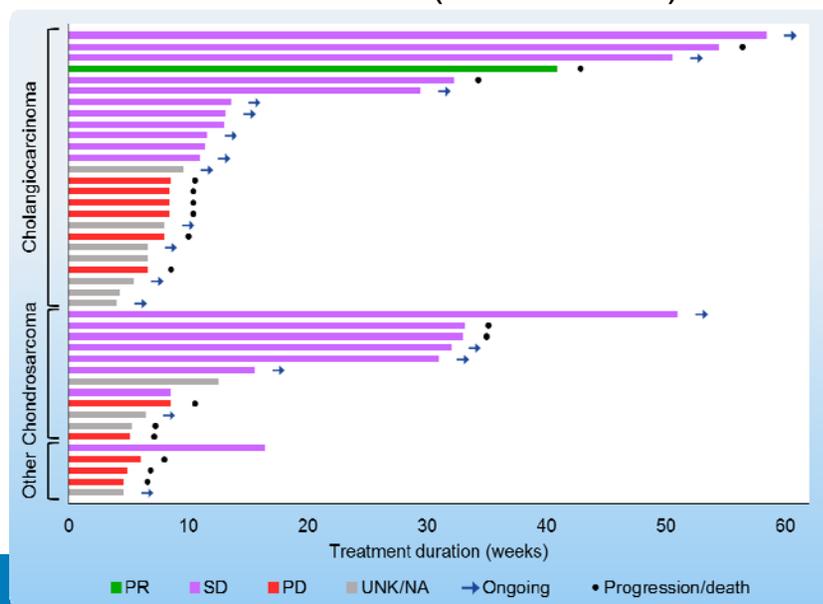
Targeting Multiple Lines of Treatment in IDHm AML and Other Hematologic Malignancies

Newly Diagnosed (Untreated) AML		Maintenance	Relapsed AML	MDS / Other Heme Malignancies
Non-Intensive	Intensive		2nd+ Relapse	Frontline to R/R
	<p>Phase 1 Induction (7+3) + AG-221 or AG-120</p>		<p>Phase 1/2 AG-221 Expansion</p>	<p>Phase 1/2 AG-221 MDS Expansion Cohort (2016)</p>
<p>Phase 1 → 2 VIDAZA® + AG-221 or AG-120 (1Q'16)</p>			<p>Phase 1 AG-120 Expansion</p>	
<p>Phase 3 AG-120 in Frontline AML (2H'16)</p>			<p>Phase 3 IDHENTIFY AG-221 vs SOC</p>	<p>Ongoing</p> <p>Planned</p>

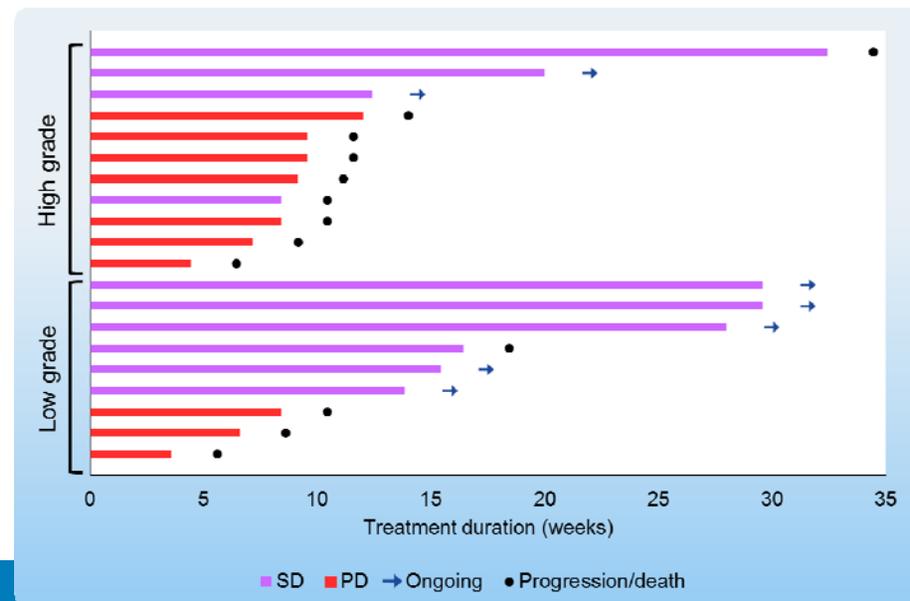


Encouraging AG-120 Phase 1 Data in Solid Tumors

Solid Tumors (Non-Glioma)



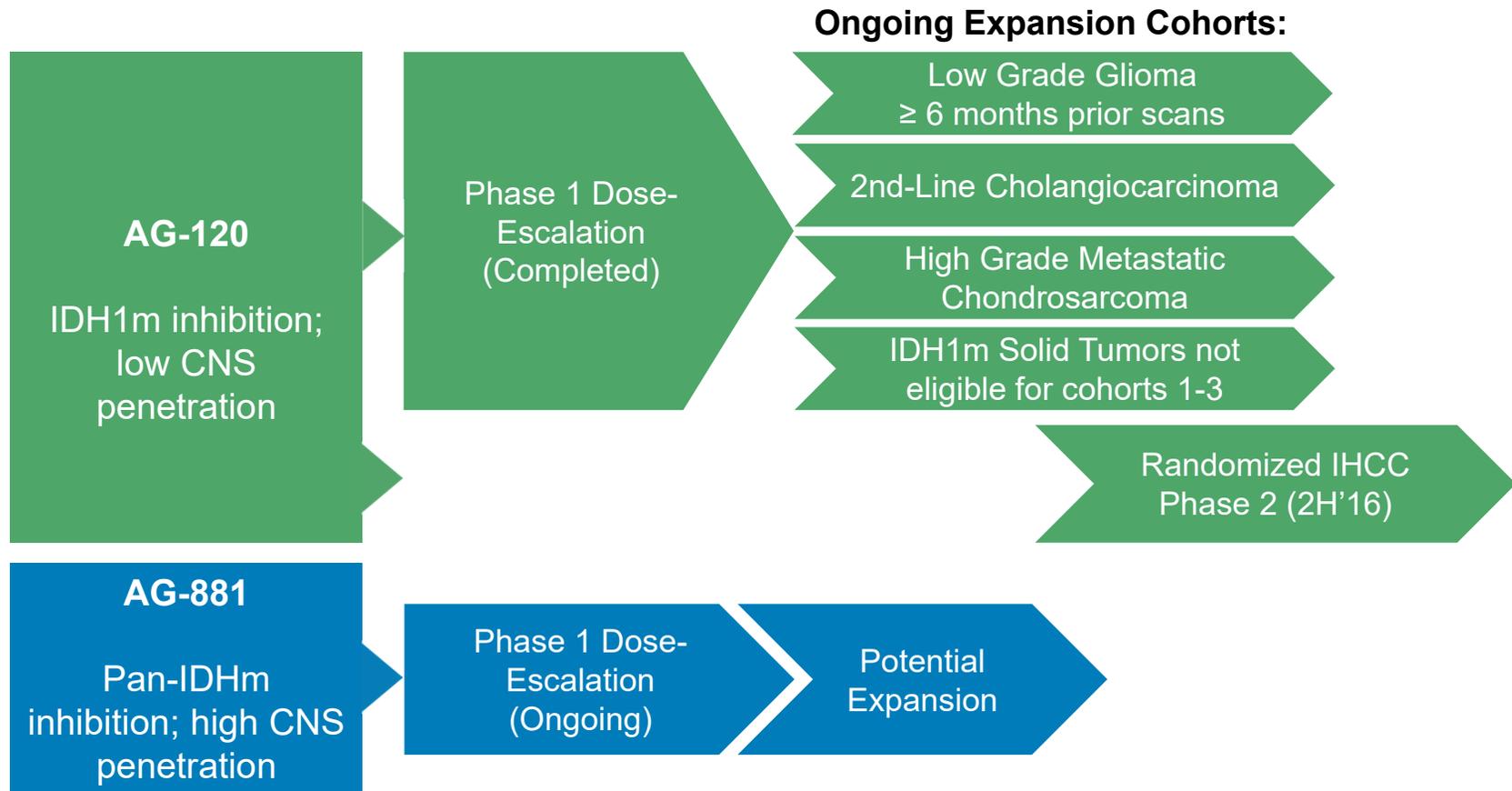
Glioma



- AG-120 well tolerated (no MTD) and showed signs of clinical activity
- Reductions in tumor volume observed in some glioma patients
- Favorable PK properties, inhibition of 2HG in tumor and reduction in proliferation markers



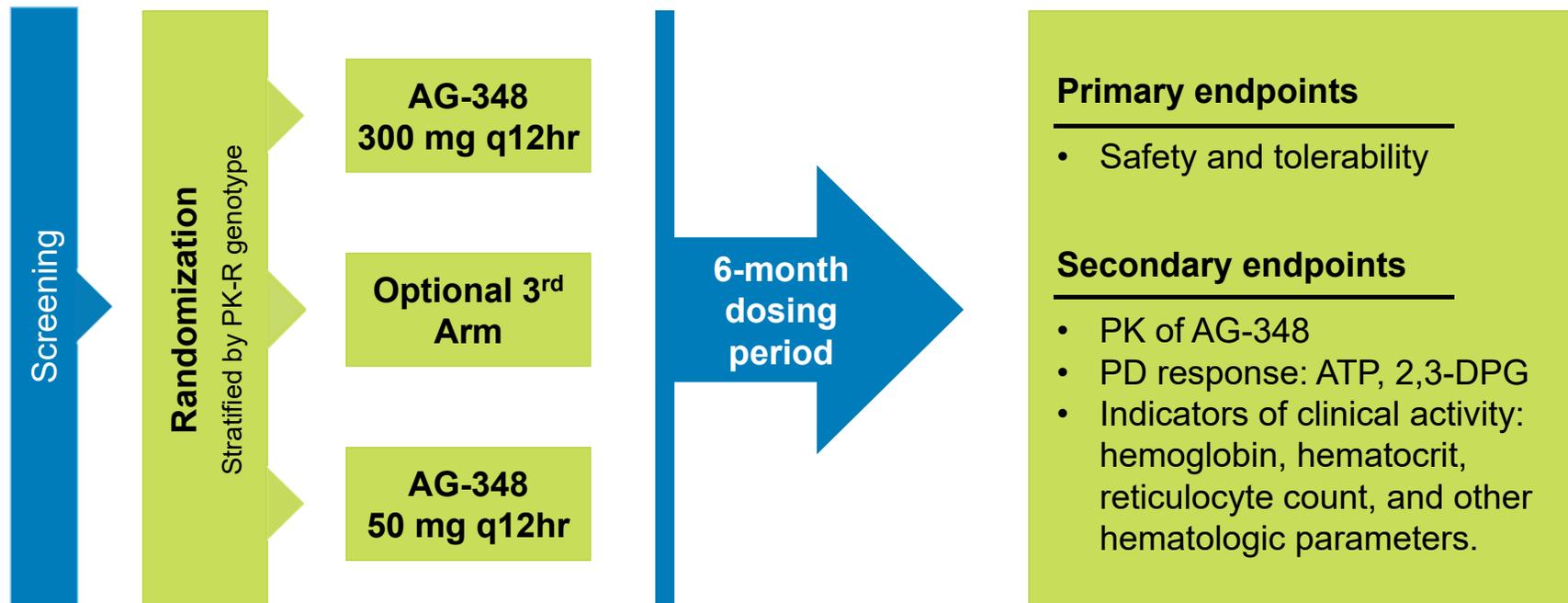
Clinical Development Path in IDH1m Solid Tumors Will Be Data Driven



AG-348 Global Phase 2 DRIVE PK Study Open and Enrolling



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



Submitting First AG-348 DRIVE PK Data for Presentation at EHA



AG-519 Healthy Volunteer Study Open and Enrolling

AG-519

- Potent, highly selective and orally bioavailable PKR activator
- Differentiated chemical structure versus AG-348
- No activity against the aromatase enzyme
- AG-519 has similar activity in vitro, in vivo and ex vivo (patient samples) relative to AG-348

One protocol, two steps, healthy volunteers

Step 1: Integrated SAD/MAD

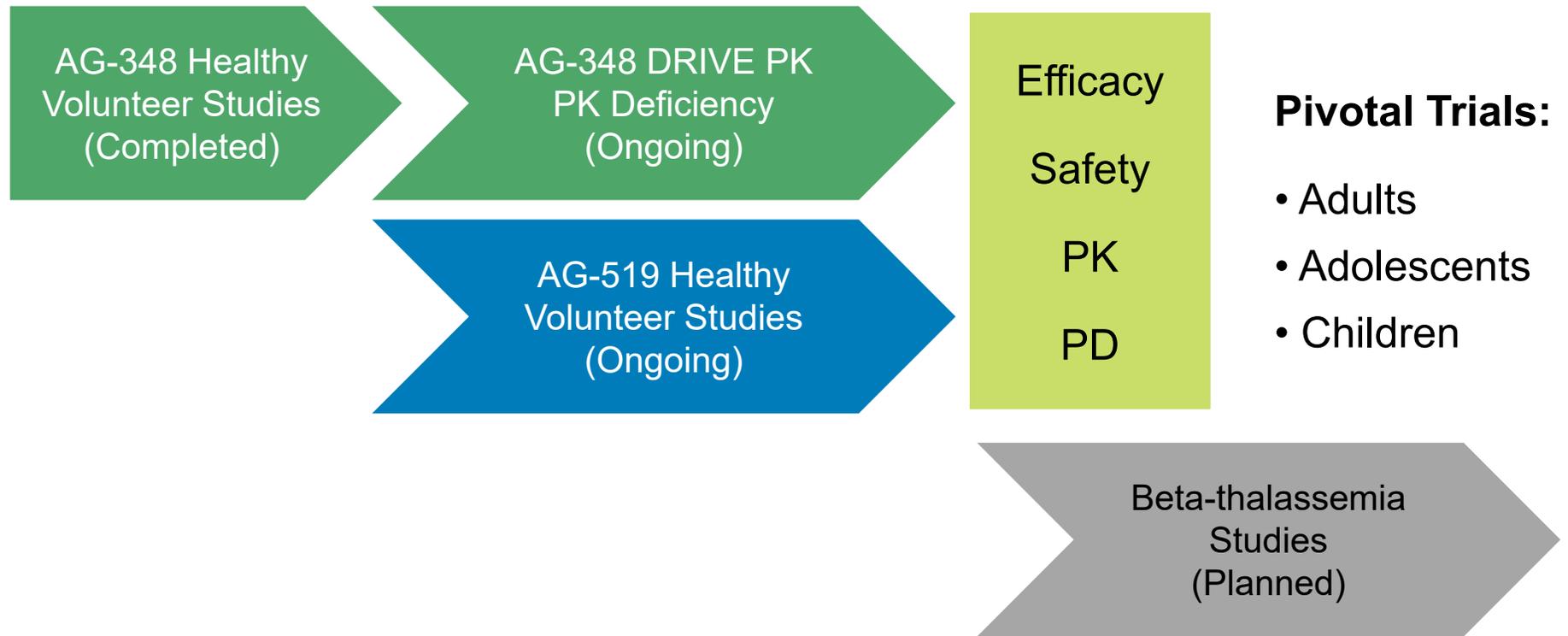
- 4 dose-ascending cohorts: 8 subjects per cohort (n=32)
- Placebo controlled (6A, 2P)

Step 2: Bioavailability and Food Effect Study

Submitting First AG-519 Phase 1 Data for Presentation at EHA



PKR Portfolio: Optionality for Clinical Development



Natural History Study Designed to Inform Development



Data presented at 2015
EHA and ASH meetings

Additional data
expected in 2H 2016

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



Full Year 2015 Financial Results

Glenn Goddard, Senior Vice President, Finance



Full Year 2015 Financial Results

Balance Sheet	December 31, 2015	December 31, 2014
Cash, cash equivalents and marketable securities	\$375.9M	\$467.4M
Total Assets	\$420.1M	\$491.9M

Statement of Operations	December 31, 2015	December 31, 2014
Collaboration Revenue (1)	\$59.1M	\$65.4M
Research & Development Expense (1)	\$141.8M	\$100.4M
General & Administrative Expense	\$36M	\$19.1M

Note 1 (Collaboration revenue and R&D expenses): Beginning in the first quarter of 2015, the Company began offsetting R&D expense for amounts received from Celgene for reimbursement of certain development costs incurred on Celgene's behalf related to AG-221 which were presented as gross collaboration revenue during 2014. In addition, beginning in the second quarter of 2015, the Company began offsetting R&D expense for amounts received from Celgene for reimbursement of certain development costs incurred on Celgene's behalf related to AG-120 and AG-881. The R&D expense reported for the twelve months ended December 31, 2015 is presented net of \$25.2 million of reimbursement compared to no offset for cost reimbursement for the comparable period in 2014.

Expect to end 2016 with cash position of more than \$180M

Does not include any additional program-specific milestone payments

New Announcements & Key Milestones

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2016 Milestones

IDHm Hematologic Malignancies

- Initiate Ph 1/2 combo study of AG-221 or AG-120 with VIDAZA® in 1Q'16
- Initiate MDS expansion arm for AG-221 in 2016
- Complete enrollment in AG-221 & AG-120 125-patient expansion arms in 2H'16
- Initiate AG-120 Ph 3 study in frontline AML in 2H'16
- Continue to enroll patients in:
 - AG-221 Phase 3 IDHENTIFY study
 - Phase 1b frontline combo study of AG-221 or AG-120 with intensive chemo
 - AG-881 Phase 1 dose-escalation and expansion study

IDHm Solid Tumors

- Present first data from AG-120 dose-expansion cohort in low grade glioma in 2H'16
- Initiate randomized Phase 2 study of AG-120 in cholangiocarcinoma in 2H'16
- Continue to enroll patients in:
 - AG-120 expansion phase of ongoing Phase 1 study
 - AG-881 Phase 1 dose-escalation and expansion study

PKR Activators

- Submit first data from AG-348 Phase 2 DRIVE PK study for presentation at EHA in June
- Submit first data from AG-519 Phase 1 healthy volunteer study for presentation at EHA in June
- Outline clinical development plans for PKR activators in beta-thalassemia in 2H'16
- Present new findings from Natural History Study of PK deficiency in 2H'16

Research

- Present preclinical findings on cancer metabolism program focused on MTAP deleted cancers at Keystone Symposia in 1Q'16
- Initiate preclinical development activities for the first molecule in the next wave of novel investigational medicines

