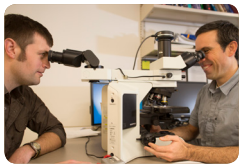
THE OTHER SIDE OF **POSSIBLE**

agios
NASDAQ: AGIO



QUICK GLANCE

Founded: 2007
IPO: July 2013
Ticker Symbol: AGIO

ANALYST COVERAGE:

Canaccord Genuity
Cowen
Credit Suisse
Goldman Sachs
JP Morgan
Leerink Swann & Company
Needham & Company
Oppenheimer & Co.
RBC Capital Markets
SunTrust Robinson Humphrey

VISION

Agios is passionately committed to applying our scientific leadership in the field of cellular metabolism to transform the lives of patients with cancer and rare genetic diseases.

LEADERSHIP TEAM

David Schenkein, M.D.
Chief Executive Officer

Steve Hoerter
Chief Commercial Officer

Scott Biller, Ph.D.
Chief Scientific Officer

Melissa McLaughlin
Human Resources

Chris Bowden, M.D.
Chief Medical Officer

Clive Patience, Ph.D.
SVP Technical Operations

Andrew Hirsch
Chief Financial Officer

Min Wang, J.D., Ph.D.
General Counsel

CONTACT INFORMATION

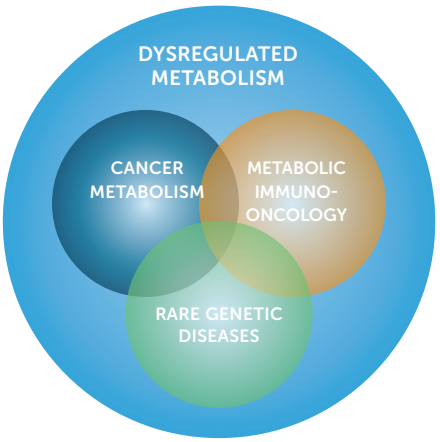
AGIOS:
88 Sidney Street
Cambridge, MA 02139-4169
617-649-8600
www.agios.com
info@agios.com

INVESTORS CONTACT:
Renee Leck, Senior Manager,
Investor & Public Relations
Renee.Leck@agios.com
617-649-8299

MEDIA CONTACT:
Holly Manning, Assoc. Director,
Corporate Communications
holly.manning@agios.com
617-844-6630

Unwavering Commitment to Science and Patients

Agios is a biopharmaceutical company passionately committed to applying our scientific leadership in the field of cellular metabolism to transform the lives of patients with cancer and rare genetic diseases. Metabolism is a complex biological process involving the uptake and assimilation of nutrients in cells to produce energy and facilitate many of the processes required for cellular division and growth. Agios believes that dysregulation of normal cellular metabolism plays a crucial role in many genetic diseases, and it is among the first in using cellular metabolism as a platform for developing potentially transformative medicines.



A Fundamentally Different Approach to Treating Cancer & Rare Genetic Diseases

Inspired by patients and frustrated by the limitations of conventional approaches to treatment, Agios advanced a novel path to treating cancer and rare genetic diseases by targeting cellular metabolism. Under this umbrella, Agios' work encompasses three distinct areas of research and development:

CANCER METABOLISM

Inhabit key enzyme in *cancer cell* specific metabolic pathways to disrupt tumor cell proliferation and survival

RARE GENETIC DISEASES

Restore defective metabolic pathways in *disease cells* that cause rare genetic diseases of metabolism

METABOLIC IMMUNO-ONCOLOGY

Alter the metabolic state of *immune cells* to enhance the body's anti-tumor response

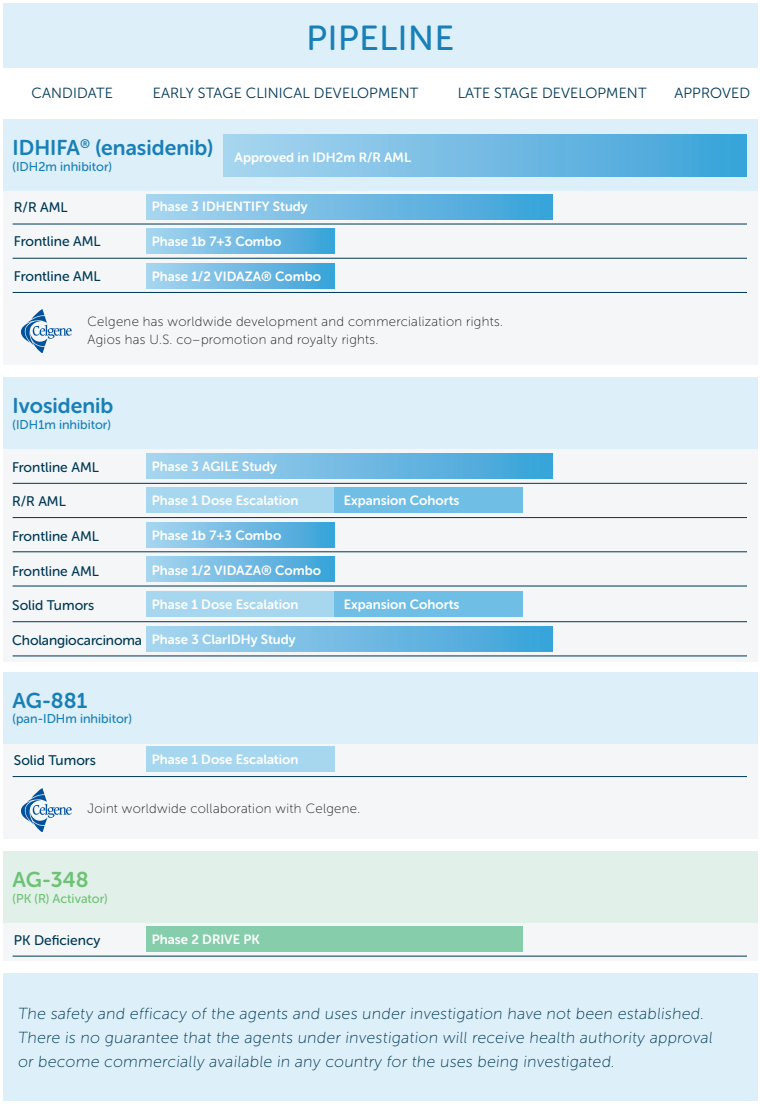
RESEARCH PLATFORM

Agios leveraged these capabilities to build a robust product engine to explore the metabolic differences between normal and diseased cells and identify new metabolic drug targets. This engine has enabled the company to discover proprietary, first-in-class, orally available small molecules as potential drug candidates for each of its novel programs. Agios' programs are focused on genetically identified patient populations and the clinical trials are biomarker-driven, allowing for a "precision medicine" approach, in which drugs are tested early among the patients who are most likely to respond.

Last updated October 2017

AGIOS: THE OTHER SIDE OF POSSIBLE

PIPELINE



The safety and efficacy of the agents and uses under investigation have not been established. There is no guarantee that the agents under investigation will receive health authority approval or become commercially available in any country for the uses being investigated.

IDH Mutant Inhibitors

Isocitrate dehydrogenase (IDH) 1 and 2 are metabolic enzymes that are mutated in a wide range of hematologic and solid tumor malignancies, including acute myelogenous leukemia (AML) and glioma, a type of aggressive brain tumor with poor prognosis. Normally, IDH enzymes help break down nutrients and generate energy for cells. When mutated, IDH enzymes create a molecule that we believe alters the cells' genetic programming, and instead of maturing, the cells remain primitive and proliferate quickly. Agios believes that inhibition of these mutated proteins may lead to clinical benefit for the subset of cancer patients whose tumors carry them. Agios has identified one approved and two novel investigational medicines that target the mutated forms of IDH1 and IDH2.

- **IDHIFA®** (enasidenib) is approved in the U.S. for the treatment of adult patients with IDH2 mutant positive relapsed / refractory AML as detected by an FDA-approved test. Celgene has worldwide development and commercialization rights for IDHIFA.
- **Ivosidenib** is a first-in-class, orally available, selective, potent inhibitor of the mutated IDH1 enzyme and is a highly targeted investigational medicine for the treatment of patients with cancers that harbor an IDH1 mutation, including AML, glioma and cholangiocarcinoma.
- **AG-881** is an orally available, selective inhibitor of the mutated IDH1 and IDH2 enzymes. In preclinical studies, it has shown to fully penetrate the blood-brain barrier, which has the potential to support ongoing development efforts to provide treatment options to patients with glioma. AG-881 is being developed in collaboration with Celgene.

PKR Activator

Pyruvate kinase, or PK, is the enzyme involved in the second to last reaction in glycolysis—the conversion of glucose into lactic acid—and is critical for the survival of the cell and has several tissue-specific isoforms (PKR, PKL, PKM1 and PKM2). The inherited mutations in PKR enzymes cause a deficit in cellular energy within the red blood cell, as evidenced by lower pyruvate kinase enzyme activity and a decline in ATP (adenosine triphosphate) levels and a build-up of upstream metabolites, including 2,3-DPG (2,3-diphosphoglycerate). This shift leads to a hematologic rare genetic disorder known as pyruvate kinase deficiency, or PK deficiency. The understanding of PK deficiency is still evolving, and there is no approved therapy to treat the underlying cause of the disease.

- **AG-348** is a wholly owned first-in-class, novel, oral activator of both wild-type (normal) and mutated pyruvate kinase-R (PKR) enzymes. AG-348 is being evaluated in a global first-in-patient Phase 2 study, DRIVE PK. DRIVE PK is an open label safety and efficacy trial in adult, transfusion-independent patients with PK deficiency.

Research

Research is the heart and soul of Agios, and the company continues to discover novel metabolic targets that meet a high bar for future development. In cancer metabolism and rare genetic diseases, Agios has started exploratory and early target validation for multiple programs, including a cancer metabolism program, AG-270, focused on MTAP deleted tumors. Immuno-oncology – exploiting the body's immune system to fight cancer – is one of the most promising current approaches to cancer therapy. There is increasing evidence that cellular metabolism plays an important role in modulating key components of the immune system. Agios' focus is on metabolic immuno-oncology, which aims to modulate the activity of relevant immune cells by targeting critical metabolic nodes, thereby, enhancing the immune mediated anti-tumor response. Leveraging its existing platform, Agios is able to add immuno-oncology to its ongoing efforts in cancer metabolism and rare genetic diseases.

Cautionary Note Regarding Forward-Looking Statements

This fact sheet contains 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 IDHIFA® (enasidenib), ivosidenib, AG-881, AG-348 and AG-270; the potential benefits of Agios' product candidates; 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 fact sheet 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; Agios' ability to maintain its key collaborations such as its agreements with Celgene; 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; unplanned cash requirements and expenditures; competitive factors; Agios' ability to obtain and maintain requisite regulatory approvals and to enroll patients in its planned clinical trials; Agios' ability to obtain, maintain and enforce patent and other intellectual property protection for any product candidates it is developing; Agios' ability to obtain the substantial additional capital required to execute its plans and strategies; and general economic and market conditions. These and other risks are described in greater detail in under the caption "Risk Factors" included in Agios' public filings with the Securities and Exchange Commission.in Any forward-looking statements contained in this fact sheet speak only as of the date of this fact sheet 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.