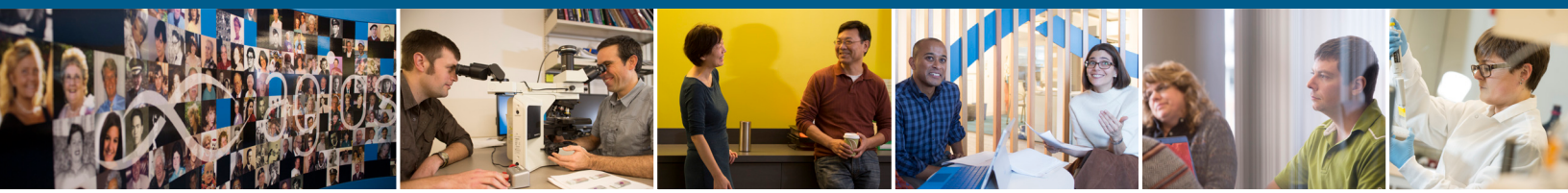
THE OTHER SIDE OF **POSSIBLE**

agios
NASDAQ: AGIO



## QUICK GLANCE

Founded: 2007
IPO: July 2013
Ticker Symbol: AGIO

## ANALYST COVERAGE:

| | |
|---|---|
| Canaccord Genuity | Leerink Partners |
| Citi | Needham |
| Cowen | Oppenheimer |
| Goldman Sachs | Piper Jaffray |
| Guggenheim Partners | RBC Capital Markets |
| JP Morgan | 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

Scott Biller, Ph.D.
Chief Scientific Officer

Chris Bowden, M.D.
Chief Medical Officer

Andrew Hirsch
Chief Financial Officer and Head of Corporate Development

Steve Hoerter
Chief Commercial Officer

Melissa McLaughlin
Chief People Officer

Clive Patience, Ph.D.
SVP Technical Operations

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

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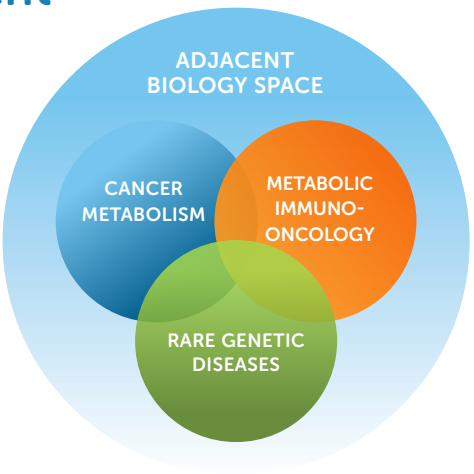
# Unwavering Commitment to Science and Patients

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**

Inhibit key enzymes in *cancer cell* specific metabolic pathways to disrupt tumor cell proliferation & survival

**RARE GENETIC DISEASES**

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

**METABOLIC IMMUNO-ONCOLOGY**

Alter *immune or cancer cell* metabolism to enhance the body's anti-tumor response

**TRANSLATIONAL SYSTEMS BIOLOGY 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 August 2018

## Pipeline

| CANDIDATE | DRUG DISCOVERY | EARLY STAGE CLINICAL DEVELOPMENT | LATE STAGE DEVELOPMENT | REGULATORY SUBMISSION | APPROVED |
|---|---|---|---|---|---|
| **IDHIFA® (enasidenib)** (IDH2m inhibitor) | | | | | |
| R/R AML | | | Phase 3 IDHENTIFY Ongoing | | U.S. |
| IC-Eligible Frontline AML | | Phase 1b Combo Ongoing | | | |
| IC-Ineligible Frontline AML | | Phase 1/2 Combo Ongoing | | | |
| Celgene has worldwide development and commercialization rights. Agios has U.S. co –promotion and royalty rights. | | | | | |
| **TIBSOVO® (ivosidenib)** (IDH1m inhibitor) | | | | | |
| R/R AML | | Phase 1 Dose-Escalation & Expansion Ongoing | | | U.S. |
| IC-Eligible Frontline AML | | Phase 1b Combo Ongoing | | | |
| IC-Ineligible Frontline AML | | Phase 1/2 Combo Ongoing | Phase 3 Agile Ongoing | | |
| Cholangio | | | Phase 3 ClarIDHy Ongoing | | |
| Glioma | | Perioperative Study Ongoing | | | |
| **AG-881** (pan-IDHm inhibitor) | | | | | |
| Glioma | | Perioperative Study Ongoing | | | |
| **Mitapivat** (PK (R) activator) | | | | | |
| PK Deficiency | | | Phase 3 ACTIVATE Ongoing | | |
| PK Deficiency | | | Phase 3 ACTIVATE-T Ongoing | | |
| **AG-270** (MAT2A inhibitor) | | | | | |
| MTAP-Deleted Tumors | | Phase 1 Dose-Escalation Ongoing | | | |

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

## Medicines

**TIBSOVO®** (ivosidenib) is approved in the U.S. for the treatment of adult patients with relapsed or refractory AML with a susceptible IDH1 mutation as detected by an FDA-approved test. TIBSOVO is wholly owned by Agios.

**IDHIFA®** (enasidenib) is approved in the U.S. for the treatment of adult patients with relapsed or refractory AML with an IDH2 mutation as detected by an FDA-approved test. Celgene has worldwide development and commercialization rights for IDHIFA.

## Preclinical Programs

Agios has led the field of cancer metabolism with its novel IDH and MTAP programs and continues to make important advances in the field of rare genetic diseases with its PKR program. These programs exemplify our strategy of applying our foundational expertise in cellular metabolism and precision medicine to translated science from our labs into first-of-their-kind experimental therapies. In addition to advancing our lead programs, we continue to discover novel metabolic targets that meet a high bar for future development across all three of our core focus areas: cancer metabolism, rare genetic diseases and metabolic immuno-oncology.

## Clinical Programs

**Mitapivat** is an investigational, wholly owned, first-in-class, novel, oral activator of both wild-type (normal) and mutated pyruvate kinase-R (PKR) enzymes. Mutations in PKR cause deficiencies in red blood cell glycolysis, which lead to a disease known as PK deficiency. Agios' pre-clinical work has demonstrated PKR activation has potential utility in other hemolytic anemias such as thalassemia and sickle cell disease.

**AG-881** is an investigational, 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-270** is an investigational first-in-class methionine adenosyltransferase 2a (MAT2A) inhibitor being evaluated in patients with advanced solid tumors or lymphoma with MTAP (methylthioadenosine phosphorylase) loss. MTAP is a metabolic enzyme that is deleted in approximately 15 percent of all cancers. MAT2A is a component of a novel pathway in MTAP-deleted tumors which, when modulated by small molecule inhibitors, results in robust anti-tumor activity. AG-270 is being developed in collaboration with Celgene.

### 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), TIBSOVO® (ivosidenib), AG-881, Mitapivat 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.